

Health Informatics

Karen M. Facey
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Ann N.V. Single *Editors*

Patient Involvement in Health Technology Assessment

Second Edition

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Karen M. Facey • Anke-Peggy Holtorf
Ann N. V. Single
Editors

Patient Involvement in Health Technology Assessment

Second Edition

 Springer

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ISSN 1431-1917

ISSN 2197-3741 (electronic)

Health Informatics

ISBN 978-3-032-11283-5

ISBN 978-3-032-11284-2 (eBook)

<https://doi.org/10.1007/978-3-032-11284-2>

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*For all those champions of patient
involvement in HTA who have inspired us.*

Foreword to the Second Edition

Patient involvement in health technology assessment (HTA) is no longer just an aspiration—it is a vital cornerstone of meaningful, ethical, and effective healthcare decision-making worldwide. As healthcare systems grow increasingly complex and resource allocation decisions more consequential, the voices of those most affected—the patients—must be at the centre of this process.

This second edition of *Patient Involvement in Health Technology Assessment* arrives at an important time, building on the solid foundation established in 2017. With 33 chapters authored by a diverse and dedicated group of patient involvement experts and patient representatives, this collection offers rich, multifaceted perspectives on patient involvement in HTA from around the globe. Since the first edition, the field has evolved significantly, and this updated volume reflects those advances with fresh insights, expanded experiences, and recognition of good practice characteristics.

Whether you come from settings where patient involvement is well-established or from places where it is still developing, this resource provides invaluable guidance, inspiration, and a platform for collaboration.

The lessons and case studies within these pages challenge us to rethink traditional approaches and to embrace patient perspectives not only as stakeholders but as active partners. Engaging patients enriches the quality, relevance, and legitimacy of HTA processes. It fosters transparency and trust, ultimately leading to healthcare decisions that are more equitable and responsive to real-world needs.

By sharing proven strategies alongside thought-provoking questions, this book empowers readers to innovate and tailor patient involvement approaches suited to their unique contexts. It is an invitation to learn from one another, to grow, and to work together towards health systems that truly reflect and respect the voices of the people they serve.

I am confident this book will serve as both a practical manual and a source of inspiration for policymakers, practitioners, researchers, and patient advocates globally. Together, we can move towards a future where patient involvement in HTA is standard practice, helping to shape better health outcomes for all.

Past President of HTAi
Raleigh, NC, USA

Rabia Sucu

Foreword I to the First Edition

If you're not involving patients, you're not doing HTA!

It's that simple. Patient involvement improves the quality, relevance, and value of HTA. It is difficult to conceive of health technology assessment being conducted in a meaningful way in the twenty-first century without the involvement of patients.

As the President and CEO of a Health Technology Assessment agency, and as Chair of the International Network of Agencies for Health Technology Assessment (INAHTA), I am a strong advocate for patient involvement in HTA.

Patient Involvement in HTA explores the rationales underlying patient involvement, provides research methodologies to produce patient-based evidence, describes how patient involvement is being achieved in different countries, and provides the point of view of various stakeholders. They go one step further by providing insight into how all stakeholders can contribute to making patient involvement more robust and meaningful. The book is truly an international collaboration with contributions from a virtual who's who of experts from INAHTA member agencies around the globe—not to mention patient organisations, academia, health technology developers, and health care funders.

Don't think you've done your part by buying this book. Read it. Learn from it. Let it spark discussions about what you, your colleagues, your organisation, and your networks can do to improve patient involvement in HTA. There are important messages for all stakeholders, and I will do my part to support and indeed implement many of the proposals identified in the book—within my agency and more broadly across INAHTA member agencies.

Facey, Single, and Hansen have given us an invaluable resource that can help us improve the quality and relevance of HTA. I encourage you to take up their call to action.

Canadian Agency for Drugs
and Technologies in Health (CADTH)
Ottawa, ON, Canada

Brian O'Rourke

Chair of the International Network of Agencies
for Health Technology Assessment (INAHTA)
Edmonton, AB, Canada

Foreword II to the First Edition

The development of new health technologies, be it at the scientific bench or the clinical bedside, involves the involvement of patients in one way or another. So, it seems profoundly intuitive that patient involvement also occurs during health technology assessment (HTA). However, the lack of meaningful ways for patients to participate and influence HTA is still very noticeable across the globe. This book places patient involvement in HTA within a strong scientific and policy context. It establishes a coherent case that the scientific basis of HTA is not robust without research to produce patient-based evidence and participation of patients in the HTA process. It describes credible methodologies to achieve it. By classifying the ways in which patients interact with HTA, providing their perspectives, identifying their preferences, and helping to uncover evidence uncertainties, the authors describe a starting point from which the HTA community can debate the opportunities and challenges of patient involvement. Everyone involved in HTA should read this book. It doesn't provide all the answers. But it will certainly stimulate those with a strong desire to ensure that HTA has face and construct validity to think what more they can, and should, do to incorporate patients' perspectives and experiences in their work.

Past President, Health Technology
Assessment International
Centre for Health Technology Evaluation,
National Institute for Health and Care Excellence
London, UK

Carole Longson

Preface to the Second Edition

The first edition of this book was driven by a desire to take stock of the literature and practice related to patient involvement in health technology assessment (HTA) to address a “gaping need for an academic text on this subject” and share current knowledge on methodologies and good practice. We soon realised that interest in the book extended well beyond students and researchers, with HTA bodies, technology developers (industry), and patient communities reading and using it. One patient organisation in China, Hongmian Charity Foundation, even took the incredible initiative of translating it so that they could apply it in their work. We’re delighted that this edition will be available Open Access and enormously grateful to organisations from all stakeholder groups that have donated to make this possible (as listed overleaf).

Along with a sense that the field was growing rapidly, this wider interest propelled us to commit to a second edition. Just as with the first edition, no one had time to do it. Between editions we faced the COVID-19 pandemic—experienced differently around the globe—but leaving us all with more challenging workloads. Despite this, over the past two years, 121 authors worked together to contribute the book’s 33 chapters using consistent terms and including case studies from jurisdictions which did not have patient involvement or HTA in 2017. No author or the editors were paid for this work, all contributed because they were keen to share their experience of patient involvement with others and want to read the new edition.

Within this edition’s chapters, you’ll see the evolution of patients in HTA, including as patient authors. Since 2017, our number of patient authors (patients, carers, or patient organisation representatives) has increased, with 25 (75%) of the chapters benefiting from at least one patient author. We’ve adopted a standardised approach in authors’ affiliations to highlight this aspect and support greater recognition of patient authorship.

Not everyone who made significant contributions to the first edition could join us on this second adventure. We especially acknowledge the absence of our 2017 co-editor, Helle Ploug Hansen. We remain incredibly grateful for the experience of working with her on the first edition and thank her for interrupting her retirement to contribute Chap. 10: Ethnographic Fieldwork.

In 2017, we somewhat rhetorically posed the question: “Can we assess the value of a health technology without involving patients?”. We trust that these chapters will challenge those who work without patient involvement to reconsider the quality and conclusiveness of their work. And for those of you already studying, working, or volunteering in this field, we hope this book will inform and inspire you to keep questioning, keep reflecting, keep caring, and keep going.

Edinburgh, UK
Basel, Switzerland
Sydney, NSW, Australia
August 2025

Karen M. Facey
Anke-Peggy Holtorf
Ann N. V. Single

Preface to the First Edition

The Birth of the Book

Can we assess the value of a health technology without involving patients? Many HTA bodies have no or only limited patient involvement in HTA. Their reasons are well documented: it's too expensive, it uses too many resources, it takes too long, it's too biased, it's hard to use in a scientific process, there is no established good practice, it lacks methods, it adds little, and it's just politics. While these concerns may be genuine, are they accurate or sufficient to overlook a potentially important source of information? Can we really know the value of most health technologies in the absence of knowledge of patients' needs, preferences and experiences?

As patient involvement has emerged and evolved in HTA, we have been challenged by others about its value and practical application. And perhaps because we come from diverse backgrounds (statistics, anthropology, stakeholder engagement) and different cultures (British, Danish, Australian), we've been challenging each other on these issues since we began exchanging ideas through Health Technology Assessment international (HTAi) more than a decade ago.

At the first HTAi annual meeting in Krakow in 2004, a panel session about patients' perspectives took place. It was the only place in the conference of HTA professionals, researchers and health technology developers where patient issues were mentioned. Karen M. Facey moderated the session, including presentations from Laura Sampietro-Colom and Helle Ploug Hansen. The discussion was lively and demonstrated the imperative for HTA to focus on patients and citizens. The following year, Karen and Laura established the HTAi Interest Group for Patient and Citizen Involvement in HTA. In 2008, at a pre-conference workshop, the Interest Group was challenged to explain what it meant by involvement—was it research into patients' perspectives or was it participation in the HTA process? After much reflection, in 2010, we published our answer in the paper 'Patients' perspectives in health technology assessment: A route to robust evidence and fair deliberation'. In it, we set out our belief that it was both research and participation, a definition informed by wider global involvement initiatives in policy but uniquely shaped by

healthcare and scientific tradition in HTA. That year, at the HTAi annual meeting in Dublin, patient issues began to gain attention in sessions outside those run by the Interest Group.

At the HTAi annual meeting in Washington in 2013, Timothy Wrightson from Springer Publishers told Karen M. Facey and Helle Ploug Hansen that something was missing. The HTAi society with its academics and HTA professionals lacked a book on patient involvement in HTA. Timothy asked if Karen and Helle could and would take the lead as editors on such a book. Like most people, Karen and Helle did not have the time to add a book to already heavy workloads, and they knew all too well how challenging a task it would be to bring together the diverse array of work in this field. They should decline. Instead, they discussed it with members of the HTAi Interest Group. Everyone thought the book was needed and several offered to write chapters, so Karen and Helle accepted the invitation from Springer. However, after creating the book's outline, contacting possible authors and reviewing abstracts, they realised that they needed one more person in the group of editors. So, Ann N.V. Single was contacted and accepted this challenge.

So why have we worked with more than 80 authors in five continents to produce this book? In truth, it was not just the gaping need for an academic text on this subject. We were also eager to spend time taking stock of the field, looking for answers in the many papers published and learning from the authors contributing chapters. Do we now have the research approaches? What are the best mechanisms to support patient participation? Can we use consistent terms and what do we mean by them? What are the consequences of our definition of patient involvement? How are HTA bodies involving patients? What are the implications of reduced budgets and rapid HTAs? Is patient involvement making a difference and how would we know? What needs to happen next? This book tries to give at least tentative answers to these questions.

Contributors

As HTA is interdisciplinary, it has been important to obtain different views about patient involvement in HTA. The contributors of this book come from a wide range of professions and positions in different organisations. They come from different countries and cultures, and authors are writing from their own perspectives and experiences. Although all of the authors manage the English language, they have faced many challenges on their writing journey, including unpacking the different meanings and practice behind common words and concepts and responding to insights found in other chapters. We have encouraged the authors to use a common language and style, creating consistency and continuity across the chapters to form a cohesive book that can be read from cover to cover, rather than a random selection of works. We do not necessarily agree with all the authors, but we welcome their views to challenge our assumptions and encourage debate and research in a field that is rapidly developing.

Since the beginning of 2015, the authors of the 37 chapters of this book have worked collaboratively with skill, thoughtfulness and tenacity together with their associates and the editors. Difficult questions have been asked and patience has been tested, but commitment has not waned. New ideas have been generated and existing ideas have been adapted to HTA, filling gaps in knowledge and competencies within patient involvement in HTA. Several authors had their own experiences of serious illness during the writing period and showed determination to complete their chapters. In particular, we offer our condolences to the family of Dr. Christin Andersson who passed away in December 2016.

Content and Organisation

The book is written for academics and HTA professionals. The conceptual, political and ethical rationales for patient involvement in HTA are explored in Part I, with specific research methodologies described in Part II. Part III then presents examples from a range of HTA bodies and stakeholders showing how patient involvement is achieved and plans for improving processes. It is our hope that this book will find its place in master's programmes, in PhD courses and in every HTA body. Furthermore, it is our hope that it will give those working in HTA the confidence to review the evidence for patient involvement and implement the methods that improve assessments.

Now, the book is here. We hope it will be 'A page turner'. At the same time, we hope you will return again and again to it to reach a deeper and more complex understanding of patient involvement in HTA that emboldens you to reflect and alter your own practice when involving patients.

Ashgrove, QLD, Australia
Edinburgh, UK
Odense, Denmark

Ann N. V. Single
Karen M. Facey
Helle Ploug Hansen

Acknowledgement

We recognise that patient involvement depends on patients and patient organisations and wish to acknowledge the countless patients, relatives, carers and volunteers who take part in research or participate in HTAs. We are indebted to you.

Open Access Funding We thank those organisations who financially contributed to enable the second edition of this book to be available as open access. These organisations had no input to the scope or content of the book.

Patient Organisations/Non-profit Organisations	European Patient Forum, Belgium Myeloma Europe, Belgium Patient Voice Initiative, Australia Philippine Alliance of Patient Organizations, The Philippines The Synergist, Belgium
Health Technology Assessment Bodies	Federaal Kenniscentrum voor de Gezondheidszorg (KCE), Belgium Health Technology Wales, UK National Institute for Health and Care Excellence (NICE), England, UK Zorginstituut Nederland (ZIN), The Netherlands
Academic Institutions	European Center of Pharmaceutical Medicine (ECPM), Switzerland
Health Technology Developers/Associations	Novartis, Switzerland UCB (Union Chimique Belge), Belgium European Federation of Pharmaceutical Industries and Associations (EFPIA), Belgium
Consultancies	Alira Health, France Patient Voice Advisors, USA

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About the Editors

Karen M. Facey, PhD is a visiting senior research fellow at the University of Edinburgh, chartered statistician and honorary member of the Faculty of Public Health. She has worked as a statistician for pharmaceutical companies and the UK Medicines Regulatory Agency. In 2000, she set up the first national health technology assessment (HTA) agency in Scotland and since 2003 has been an independent consultant. She is a member of the Scottish HTA appraisal committee, the SUSTAIN-HTA consortium and the Myeloma Patients' Europe Patient Evidence Research Advisory Committee. She has been active in HTA international (HTAi) for the past 20 years and was founding chairperson of the HTAi Interest Group for Patient and Citizen Involvement in HTA. She is on the editorial board of several journals and has research interests in HTA policy, patient involvement, rare diseases and real world data. In 2014, she was named as one of the top 100 practising scientists in the UK for her work on HTA and patient well-being.

Anke-Peggy Holtorf, PhD, MBA is Director of Health Outcomes Strategies GmbH with focus on value of health technologies and related policy decisions, health technology assessment, outcomes research, and health economics. She contributed to global projects including work in Low- and Middle-Income Countries or initiatives for involving patients along the technology lifecycle. She acts as Secretary of the Board (2024–2027) at Health Technology Assessment international (HTAi). She has published broadly on subjects related to evidence-based decision making and policies in healthcare or the methods for including the patient perspective in such decisions. As Steering Committee member and project coordinator for the Patient and Citizen Involvement in HTA Interest Group, she has co-led several projects (e.g., 'Patient and Citizen Involvement in HTA in LMICs', '360° Stakeholder Experiences with PI in HTA in Europe' and 'Patient Insight Research for HTA'). She teaches as Adjunct Faculty at the University of Utah and as guest lecturer in other university programmes.

Ann N. V. Single, MA is the CEO of Patient Voice Initiative, a patient-led not for profit working to ensure patients are seen, heard and valued in healthcare decision-making, especially HTA. She's worked in patient involvement in HTA for 25 years, first directing it within Scotland's HTA agency and then sharing and developing good practice in HTAi's Patient and Citizen Involvement Interest Group. From 2019 to 2023, she chaired this Interest Group and is now its Outgoing Chair and HTAi's first President (2025–2027) from the patient community sector. She is a faculty member of the International Patient Advocacy Management Masters Course at *Università Cattolica del Sacro Cuore* and a member of Rare Voices Australia's Scientific and Medical Advisory Committee. She served as a Reference Committee member on the Australian Government's HTA Policy and Methods Review (2022–2024). With a background in government and patient organisations, her focus is improving health outcomes by integrating patients and their knowledge in healthcare decisions.

Abbreviations

A4R	Accountability for reasonableness
ACE	Agency for Care Effectiveness
AETSA	Agency for Evaluation of Healthcare Technologies in Andalusia, Spain
AHP	Analytic hierarchy process
AHRQ	Agency for Healthcare Research and Quality (USA)
AI	Artificial intelligence
AiArthritis	International Foundation for Autoimmune and Autoinflammatory Arthritis
AIDS	Acquired immune deficiency syndrome
AIFP	Association of Innovative Pharmaceutical Industry
ALS	Amyotrophic lateral sclerosis
AQuAS	Agency for Healthcare Quality and Assessment of Catalonia, Spain
ASMR	Amélioration du Service Médical Rendu (France)
Avalia-t	Scientific-Technical Advice Unit, Galician Agency for Health Knowledge Management, Spain
BMA	Become the Miracle Association
BMI	Body mass index
BWS	Best worst scaling
CAB	Community advisory board
CADTH	Canadian Agency for Drugs and Technologies in Health (since 2024, Canada's Drug Agency)
CAMELOT	Cochrane qualitative METHodological LimitatiOns Tool
CAN	Consumer AdvoCare Network
CASP	Critical Appraisal Skills Programme
CDA—AMC	Canada's Drug Agency (until 2024, CADTH), L'Agence des Médicaments du Canada
CDE	Center for Drug Evaluation (Taiwan)
CEE	Consumer Engagement and Education (Singapore)
CEEU	Consumer Evidence and Engagement Unit
CEPS	Comité Économique des Produits de Santé (France)

CEU	Conseil pour l'Engagement des Usagers (France)
CGMS	Continuous glucose monitoring system
Chap.	Chapter
CHPTA	Center for Health Policy and Technology Assessment (Taiwan)
CHRD	Centers for Devices and Radiological Health
CINAHL	Cumulative Index to Nursing and Allied Health Literature
COI	Conflict of interest
CONITEC	National Committee for Health Technology Incorporation into the SUS; Comissão Nacional de Incorporação de Tecnologias no Sistema Único de Saúde (Brazil)
COSMIN	COnsensus-based Standards for the selection of health Measurement INstruments
CRM - CTG	Drug Reimbursement Commission (Belgium); La Commission de Remboursement des Medicaments/Commissie Tegemoetkoming Geneesmiddelen
CSO	Civil society organisation
CT	Commission de la Transparence, Transparency Committee France
CWG	Consumer Working Group
CXB	X-ray brachytherapy
DAC	Drug Advisory Committee (Singapore)
DCE	Discrete choice experiment
Digi-G	Law to accelerate the digitalisation of the healthcare system (Germany)
DL	Deep Learning
DMD	Duchenne muscular dystrophy
DoH	Department of Health
DRC	Drug Reimbursement Committee
D-RSC	Duchenne Regulatory Science Consortium
DUSC	Drug Utilisation Sub-Committee
EAR	Evidence Appraisal Report (Wales)
EATG	European AIDS Treatment Group
EC	European Commission
ECAB	European Community Advisory Board
ECTHA	European Collaboration for HTA
ECPC	European Cancer Patient Coalition
EEA	European Economic Area
EHC	Effective Health Care
EHR	Electronic health record
EIFFEL	Effectiveness of Interventions For Fatigue in Long term conditions
EMA	European Medicines Agency
EMBASE	Excerpta Medica dataBASE
EPF	European Patients' Forum
EQ5D	EuroQual 5 Dimensions Questionnaire
ESG	Environmental, Social and Governance

EU	European Union
EUCAPA	European Capacity Building for Patients
EUnetHTA	European Network for Health Technology Assessment
EUPATI	European Patients' Academy on Therapeutic Innovation
EuroCAB	European Community Advisory Board
EURORDIS	European Organisation for Rare Diseases
FDA	Food and Drug Administration (US)
Fig.	Figure
FEV	Forced expiratory volume
FPS	Federal Public Service
G-BA	Federal Joint Committee; <i>Gemeinsamer Bundesausschuss</i> (Germany)
GCP	Good clinical practice
GDPR	General Data Protection Regulation (Europe)
GRADE	Grading of Recommendations Assessment, Development and Evaluation
GRIPP	Guidance for Reporting Involvement of Patients and Public
HADEA	Health and Digital Executive Agency, European Union
HAS	Haute Autorité de santé (France)
HC	Healthcare
HCP	Healthcare professional
HCRW	Health Care Research Wales
Healthtech	Medical technologies
HERCULES	HEalth Research Collaboration United in Leading Evidence Synthesis
HHT	Hereditary haemorrhagic telangiectasia
HIPAA	Health Insurance Portability and Accountability Act (USA)
HIRA	Health Insurance Review and Assessment service (Republic of Korea)
HIV	Human immunodeficiency virus
HRQoL	Health-related quality of life
HSR	Health services research
HST	Highly specialised technology
HTA	Health technology assessment
HTA CCC	Health Technology Assessment Consumer Consultative Committee
HTACG	HTA Coordination Group (EU)
HTAi	HTA international
HTAR	HTA Regulation (European Union)
HTD	Health technology developer
HTW	Health Technology Wales
HVD	Heart valve disease
IACS	Institute of Health Sciences of Aragon, Spain
IAP2	International Association of Public Participation

ibid.	<i>ibidem</i> —Latin for ‘in the same place’, used when citing the immediately preceding reference in the same paragraph
ICER	Institute for Clinical and Economic Review (USA)
IETS	Instituto de Evaluación Tecnológica en Salud (Chile)
IHI	Innovative Health Initiative (previously Innovative Medicines Initiative)
IMI	Innovative Medicines Initiative (now Innovative Health Initiative (IHI))
INAHTA	International Network of Agencies for Health Technology Assessment
INESSS	Institut National d’Excellence en Santé et en Services Sociaux (Québec)
IPPOSI	Irish Platform for Patient Organisations, Science, and Industry
IQWiG	Institute for Quality and Efficiency in Healthcare; Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Germany)
IR	Implementing regulation
ISPOR	International Society for Pharmacoeconomics and Outcomes Research
IT	Information technology
JA	Joint Action
JCA	Joint Clinical Assessment (European Union)
JSC	Joint Scientific Consultation (European Union)
KCE	Kenniscentrum—Centre d’Expertise; Healthcare Knowledge Centre (Belgium)
KPI	Key performance indicator
KSJA	Korean Science Journalists Association
LAC	Latin American and Caribbean
LAPA	Latin America Patients Academy
LATAM	Latin America
LMIC	Low- and Middle-Income Country
LRS	Lower resource setting
LUSS	Ligue des Usagers des Services de Santé
MA	Marketing Authorisation
MaHTAS	Malaysian Health Technology Assessment Section
MAR	Minimum acceptable risk
MDIC	Medical Device Innovation Consortium
MEDLINE	Medical Literature Analysis and Retrieval System Online
Medtech	Medical technologies
ML	Machine learning
MLD	Metachromatic leukodystrophy
MoH	Ministry of Health
MoHW	Ministry of Health and Welfare (Republic of Korea)
MSAC	Medical Services Advisory Committee (Australia)
MSAC—ESC	Medical Services Advisory Committee—Evaluation Sub Committee

MSAC—PASC	Medical Services Advisory Committee—PICO Advisory Sub Committee
MTAC	Medical Technology Advisory Committee (Singapore)
NECA	National Evidence-based healthcare Collaborating Agency (Republic of Korea)
NER	Named entity recognition
NHI	National Health Insurance
NHIA	National Health Insurance Administration (Taiwan)
NHIS	National Health Insurance Service (Republic of Korea)
NHS	National Health Service
NICE	National Institute for Health and Care Excellence (England and Wales)
NIHDI	National Institute for Health and Disability Insurance (Belgium)
NIHR	National Institute for Health Research (England)
NLP	Natural language processing
OHTAC	Ontario Health Technology Advisory Committee
OMP	Orphan medicinal product
Osteba	Basque Office for Health Technology Assessment, Spain
OTA	Office of Technology Assessment (USA)
P+R	Pricing and reimbursement
PAPO	Philippine Alliance of Patient Organisations
PAQS	Platform for Continuous Improvement in the Quality of Care and Patient Safety
PARADIGM	Patients Active in Research and Dialogues for an Improved Generation of Medicines project
PBAC	Pharmaceutical Benefits Advisory Committee (Australia)
PBAC—ESC	Pharmaceutical Benefits Advisory Committee—Economics Sub Committee
PBRIS	Pharmaceutical Benefits and Reimbursement Scheme
PBS	Pharmaceutical Benefits Scheme (Australia)
PCIG	Patient and Citizen Involvement in HTA Interest Group (HTAi)
PCORI	Patient-Centered Outcomes Research Institute
PEL	Patient engagement lead
PEMDL	Philippine Essential Medical Device List
PFDD	Patient Focused Drug Development
PFMD	Patient Focused Medicines Development
PGHD	Patient-generated health data
PhilHealth	Philippine Health Insurance Corporation
PI	Patient involvement
PIA	Public Involvement Adviser
PICO	Population, intervention, comparator, outcome
PMR	Post market reviews
PNF	Philippine National Formulary
PO	Patient organisation
POS	Part-of-speech

PPG	Public Participation Group
PPI	Patient and Public Involvement
PPIE	Patient Public Involvement Engagement
PPISG	Patient and Public Involvement Standing Group
PPSC	Participatory Priority Setting Committee
PREFER	Patient Preferences in Benefit-Risk Assessments during the Medical Product Lifecycle (IMI Project)
PREM	Patient-reported experience measure
PROBE	Patient-reported outcomes, burdens and experiences
PROM	Patient-reported outcome measure
PPPS	The Patient Preference Project Sub-Committee (in PCIG)
PUT—RD	Protocole d'Utilisation Thérapeutique—Recueil de Données (France)
QALY	Quality-adjusted life-year
QES	Qualitative evidence synthesis
QOL	Quality of life
R&D	Research and development
RaDiOrg	Rare Disease Organisation (Belgium)
RCT	Randomised controlled trial
RedETS	Red Española de Agencias de Evaluación de Tecnologías Sanitarias y Prestaciones del Sistema Nacional de Salud, Spain
RETREAT	Review question, Epistemology, Time, Resources, Expertise, Audience and purpose, Type of data
RVS	Relative value scale
RWD	Real-world data
RWE	Real-world evidence
SA	Sentiment analysis
SAVR	Surgical aortic valve replacement
SBU	Swedish Agency for HTA and Assessment of Social Services
SCHARR	Sheffield Centre for Health and Related Research
SEC	Securities and Exchange Commission (Philippines)
Sect.	Section
SEED	Shaping European Early Dialogues
SEM	Service d'Évaluation des Médicaments (France)
SESCS	Evaluation Unit of the Canary Islands Health Service, Spain
SEU	Service pour l'Engagement des Usagers (France)
SG	Sub-group
SHE	HTA Stakeholder Engagement
SHI	Statutory health insurance (Germany)
SHTG	Scottish Health Technologies Group
SMA	Social media analysis (Chap. 7)
SMA	Spinal muscular atrophy
SMR	Service Médical Rendu (France)
SNHS	Spanish National Healthcare System, Sistema Nacional de Salud (Spain)

SOH	Secretary of Health (The Philippines)
SPAN	SingHealth Patient Advocacy Network
SRS	Spontaneous reporting system
START	STrAtegies for RelaTives (Wales)
SuRe Info	Summarized Research in Information Retrieval for HTA
SUS	Brazilian Public Health System, Sistema Único de Saúde
TA	Technology appraisal
TAVI	Transcatheter aortic valve implantation
TAVR	Transcatheter aortic valve replacement
TD-IDF	Term frequency-inverse document frequency
UETS	Healthcare Technologies Evaluation Unit of Madrid, Spain
UHC	Universal health coverage
UK	United Kingdom
US	United States
USA	United States of America
VCS	Voluntary and community sector
VPP	Vlaamse Patiëntenplatform
WECAN	Workgroup of European Cancer Patient Advocacy Networks
WGEDCD	Working Group for the Early Diagnosis of Celiac Disease Protocol; Spain
WHA	World Health Assembly
WHO	World Health Organization
WTP	Willingness to pay
XLH	X-linked hypophosphataemia

Part I

Conceptualisation

Chapter 1

Health Technology Assessment



Karen M. Facey and Neil Bertelsen

1.1 Introduction

This chapter explores the history of health technology assessment (HTA) and how it has evolved in terms of processes and methods, highlighting elements relevant to patient involvement to lay a foundation for the subsequent chapters of this book.

A health technology is any intervention to prevent, diagnose or treat medical conditions; promote health; provide rehabilitation; or organise healthcare delivery. This includes tests, devices, apps, medicines, vaccines, procedures, screening programmes, and care pathways (O'Rourke et al. 2020). HTA seeks to inform healthcare policymakers about whether, or how, a health technology should be made available in a health system. It is now often used to inform pricing and reimbursement decisions and so can affect patient access to an intervention or service. Given its influence, HTA needs to be rigorous and defensible and is internationally defined as a multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its life cycle. Through informing healthcare decision-making it seeks to support delivery of an equitable, efficient and high-quality health system (ibid.).

HTAs use systematic processes that are meant to critically assess all the available evidence about the impacts of using a health technology. This evidence generally comes from international research. This is followed by context-specific appraisal of the social, economic, legal and ethical implications of the use of the health technology

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K. M. Facey et al. (eds.), *Patient Involvement in Health Technology Assessment*,
Health Informatics, https://doi.org/10.1007/978-3-032-11284-2_1

compared to the current standard of care in a specific health system. This is not simply a scientific endeavour. It requires interdisciplinary deliberative discussion and value judgements about the best assumptions to be made given the evidence ‘uncertainties’ and the relevance of the evidence to the local health system.

HTAs may recommend the criteria or conditions for use or disinvestment of a health technology and so are subject to political, public and stakeholder scrutiny. As a result of these requirements, there has been pressure to involve those in the HTA process who have a specific interest in the health technology, particularly patients. This has been contentious in some jurisdictions due to concerns about potential bias and representativeness of patient input. However, implementation of systematic processes can overcome these issues and personal/committee experience of engaging with patients can demonstrate positive impacts and change perceptions (Part III). A further challenge relates to the scientific standing of qualitative social sciences research versus quantitative research (Chap. 3) that is promulgated through the evidence grading systems used in many HTA bodies, and reliance on cost utility models in some influential HTA jurisdictions. This means that HTA assessors and decision-making committees often don’t have the expertise to evaluate scientific evidence about patient impacts and may deem it as anecdotal.

1.2 Context and History

In 2001, it was stated that to build a quality health system, (whether funded by taxation, social insurance, personal insurance or private fees), services should be organised to use available funds efficiently to deliver effective, safe, person-centred care in a timely and equitable manner for the population they serve (Institute of Medicine 2001). Health systems have difficult choices to make about who to treat, with what intervention, in what setting and for how long (Newdick 2004). Such questions must be considered not just in the context of the individual patient but in terms of providing the best possible service to all potential users of the health system (Drummond et al. 2006, Chap. 2). Thus resource allocation questions often seek to maximise health gain of the population overall, recognising that there is an opportunity cost to any investment, i.e. giving up the possibility of funding an alternative intervention with that money (Metzler and Smith 2012).

Daniels and Sabin stated that resource allocation decisions in healthcare were “rife with moral disagreements and a fair, deliberative process is necessary to establish legitimacy and fairness of such decisions” (2008, p. 1). They argued that resource allocation:

1. Processes must be public (fully transparent) about the grounds for decisions
2. Decisions must rest on reasons that stakeholders can agree are relevant
3. Decisions should be revisable in light of new evidence and arguments
4. Should include assurance, through enforcement, that these three conditions are met.

In fact, three decades earlier, the United States’ Senate had noted that “a reasonable amount of justification should be provided before costly new medical technologies

and procedures are put into general use” (Office of Technology Assessment 1976, p. vii). As a result, the Office of Technology Assessment (OTA) created a report providing examples of medical technologies in the fields of diagnostics, implantable devices, vaccines, surgery, medicines and interventional procedures that illustrated the diversity in development, purpose and the use of medical technologies (ibid.). It noted that decisions about the use of such new technologies were often made on the basis of evidence about technical feasibility, safety and anticipated need or demand, but that wider consideration of impacts should be assessed, including implications for:

- Patients
- Patients’ families
- Society as a whole (environmental impacts, ethics, cultural values)
- Medical care system
- Legal and political systems
- The economy.

OTA (1976) stated that to systematically consider these wider impacts of medical technologies, a comprehensive form of policy research was needed to provide decision-makers with policy alternatives. The formal process of ‘technology assessment’, which had first been used to evaluate other forms of technologies in 1965, was suggested. Technology assessment was described as systematically examining the short- and long-term social consequences of the application or use of technology, considering unintended, indirect or delayed social impacts (ibid.).

OTA (1976) described the unique features of technology assessment as being:

- Based on an explicit analytic framework, specified in advance
- Comprehensive in scope, examining impacts on social, ethical, legal and other systems that may not be immediately obvious
- Carried out by a multidisciplinary group
- Able to explicitly identify the groups that would be affected by the technology and evaluate the impacts (and impacts of impacts) of the technology on each party.

The report (OTA 1976) outlined a list of questions to be considered for each potential area of impact, including for patients and their families. An example provided over 20 questions about the implications for patients and families of a new heart valve.

1.3 Development of HTA

1.3.1 *Spread of HTA*

OTA was abolished a few years after its establishment due to lack of funding (Banta and Jonsson 2009), but it stimulated technology assessment activities in other countries (ibid.). In Denmark, in 1982, and Sweden in 1987, national organisations were given responsibility to undertake systematic assessments of all forms of health intervention (including medical technologies, educational programmes,

organisation of care) to inform policy and practice (Sigmund and Kristensen 2009; Jonsson 2009). So, the ethos and processes of OTA's technology assessment were used under the new name of health technology assessment.

The proliferation of HTA in the past three decades is shown by the breadth of membership in the International Network of Agencies for HTA (INAHTA). INAHTA is a society for non-profit HTA bodies, with 53 members in 34 countries across five continents (INAHTA 2024). Furthermore, in 2014, the World Health Organization (WHO) issued a declaration recognising that HTA offers rigorous and structured research methodologies and transparent and inclusive processes. It recommended the use of HTA in all its member states to guide policies for rational and efficient use of medicines and devices to inform policies of universal health coverage and support sustainable health systems (World Health Organisation 2014) and continues to support development of HTA, particularly in low and middle income countries (ibid.).

1.3.2 The Determination of Value in HTA

The HTA process should be formal, systematic, and transparent, and use state-of-the-art methods to consider the best available evidence to determine the value of a health technology. (O'Rourke et al. 2020). The 'value' of a health technology is a comparative evaluation of the intended and unintended consequences of using a health technology compared to interventions currently used within a health system.

As HTA seeks to inform policy about the use of a health intervention in a national, regional or local context, the process of doing HTA is different in each healthcare system. HTA must be shaped to take account of the HTA body's responsibilities and legislative constraints, its ability to interact with decision-makers and stakeholders, the evidence likely to be available and policy drivers as outlined in Part III. As a result, there may be different HTA processes for different forms of health technology within a single health system.

All HTA bodies evaluate the clinical (comparative/relative) effectiveness of a health technology compared to the standard of care (so-called 'added benefit'), and its safety. Evidence is assessed from clinical trials, and in some cases real world data, and then value judgements are made about how well the evidence reflects local clinical practice. In some cases, added benefit may not be clear for the entire population, and so a sub-group may be identified in whom the added benefit is higher, or who have greater need for a new technology because they alternatives for them are more limited.

Some HTA bodies also evaluate economic considerations such as cost effectiveness (value for money), incremental cost effectiveness (additional cost per additional effect as compared to standard of care) and budget impact (total cost per year for all the patients treated for the indication with the new treatment compared to absence of a new treatment). Cost effectiveness is often in the form of a cost utility analysis that evaluates the long-term costs and consequences of the health

technology versus the current standard of care in that health system, requiring many assumptions to extrapolate anticipated effects in terms of disease progression over the lifetime of a patient in each treatment pathway. Another common approach is cost minimisation, especially for technologies making a claim of non-superiority. These analyses may compare the new technology to the lowest cost alternative reimbursed.

The integration of ethical enquiry into HTA is not widespread (Hofmann et al. 2014) but some HTA bodies, such as those in Canada and Sweden, include ethical analysis for specific products (HTAi Ethics Interest Group 2024). Organisational issues are often considered in HTAs of medical devices, whereas social and legal aspects are rarely explicitly investigated, but feature in the deliberative discussion about the local context.

Ultimately, the determination of value will vary depending on the perspective taken, the stakeholders involved, and the decision context (O'Rourke et al. 2020).

1.3.3 The HTA Process in Relation to the Health Technology Life Cycle

HTA is often delineated into:

- Assessment: critical review and scientific summary of the (international) evidence about relevant aspects of the health technology (Garrido et al. 2008)
- Appraisal: wider consideration of the assessment information, taking account of (local) values and other factors (ibid.).

The processes for assessment have been developed over decades and arise from the evidence-based medicine movement. However, HTA goes beyond evidence-based medicine to make recommendations on how a technology will be used in a specific healthcare system, whether it should be used routinely, for which patients and whether it will be fully reimbursed. For this, appraisal is needed which can be organised to take a two-tiered multi-stakeholder approach, enabling effective input of local experts for a specific technology and use of an independent advisory group providing consistency in decision-making. This creates the framework for a systematic deliberative approach (Oortwijn et al. 2022) that facilitates fair value judgments and trade-offs, given the evidence available, the needs of the patient population, and the constraints of the health system.

In the past decade, it has also been recognised that HTA may need to extend its remit beyond traditional assessment/appraisal boundaries, at a single point in the life cycle of a medicine, to influence other aspects of the life cycle of development and use of a health technology (Facey et al. 2015; Trowman et al. 2023). As Fig. 1.1 shows there are opportunities for patient involvement at all of these points in the life cycle.

HTA bodies are recognising the value of horizon scanning to have forewarning of health technologies in development that might have substantial impact on the

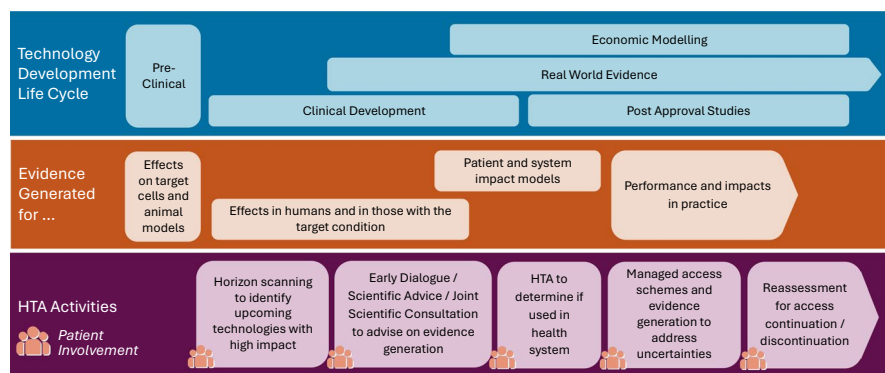


Fig. 1.1 HTA activities over the life cycle of health technology development

health system (International Horizon Scanning Initiative 2024). Processes to enable patient participation in these processes are developing and can support identification of the disruptive potential of a technology (Tipton et al. 2021, Chap. 30). An international review undertaken in 2023 identified that HTA bodies in the United Kingdom (UK), Canada and the international Euroscan system have processes for patient participation in their horizon scanning processes (Merlin et al. 2024, Table 18). This information has influenced a recommendation in the 2024 review of HTA processes in Australia to ensure key stakeholders, including patients, participate in the establishment of a national horizon scanning process. This should ensure its scope, audience, purpose, governance and outcomes are appropriately designed to support evidence-based recommendations that support improvements to health technology access and availability (Health Technology Assessment Policy and Methods Review Reference Committee 2024).

Meanwhile, processes to enable health technology developers to undergo Scientific Advice (Early Dialogue, Joint Scientific Consultation) with HTA bodies during technology development about their evidence generation plans have developed in some jurisdictions. Many of these processes go beyond considerations about the design of a confirmatory trial to address development of a new outcome measure or use of a new methodology, e.g. patient preference research (Chap. 9). These advice processes have matured and now more routinely include other decision makers such as regulators and stakeholders such as clinical and patient experts (Merlin et al. 2024). Learnings from these processes have been brought together in the Innovative Medicines Initiative (IMI) PARADIGM toolkit to support HTA bodies to routinely and systematically involve patient stakeholders in Early Dialogues (PARADIGM 2020).

If major uncertainties are identified during HTA appraisal that impact on decision making, there may be an opportunity to collect data post launch from the health system to resolve uncertainties through use of outcomes/performance based managed entry agreements. Originally it was thought that these agreements should be between the health technology developer and healthcare payer (Klemp et al. 2011),

but in recent years the value of multi-stakeholder input (including patient organisations) into the design and monitoring of data collection has proven valuable (Facey et al. 2021).

1.3.4 HTA Methods

HTA is founded on scientific research and seeks to answer clear, structured research questions about the implications (direct and indirect, intended and unintended) of using the health technology. Agreement on the parameters or the ‘scope’ of the HTA, is the foundation of any assessment and is often structured using the PICO framework from evidence-based medicine (Sackett et al. 1997):

- **Population** (who should be treated)
- **Intervention** (technical specification of health technology under study, how it will be given)
- **Comparator** (health technologies currently used in the health service(s))
- **Outcome** (what outcomes/impacts are important)

HTA bodies vary in the way in which they develop this scope. Some develop a draft scope for input from stakeholders (e.g. England, see Chap. 17), while others agree it internally, perhaps with the input of some experts, but it is not published. In some HTAs, the HTA bodies accept the PICO developed by the technology developers, which may or may not have had patient input.

Historically, HTA research questions were answered primarily by secondary research (systematic review of published literature with critical assessment of relevant studies or economic modelling drawing inputs from a range of research sources and expert elicitation). If no published literature could be found, primary research (undertaking new research) could be instigated (Kristensen and Sigmund 2007; Goodman 2014). In most countries, in the past two decades, focus has been placed on HTA using secondary research. However, there are well established primary research HTA organisations such as the UK National Institute for Health and Care Research (NIHR) HTA programme, the Dutch ZonMw and the Belgian Knowledge Centre (Chap. 15), and there is a growth of primary HTA research for digital technologies.

In the 1990s and early 2000s, collaborative HTA work was undertaken among HTA bodies in the EUR-ASSESS¹ and European Collaboration on HTA (ECHTA) projects (Banta et al. 1997; Jonsson et al. 2002). This was followed in 2006, by the European Commission-funded project to develop a European Network for HTA (EUnetHTA) and three subsequent Joint Actions² of European Union (EU) Member States. The centrepiece of this work from a methodological standpoint has been the *HTA Core Model*[®] (EUnetHTA Joint Action 2 2016), which has nine domains:

¹ Coordination and Development of Health Care Technology Assessment in Europe.

² Joint Actions are initiatives that are co-funded by the European Commission and Member States.

1. Health problem and current health technologies
2. Description/technical characteristics
3. Safety
4. Clinical effectiveness
5. Costs/economic evaluation
6. Ethical analysis
7. Organisational aspects
8. Patient and social aspects
9. Legal aspects.

The *HTA Core Model*[®] documentation is a detailed report of over 400 pages, which describes how each domain should be studied, including assessment elements (research questions) that might be relevant for each domain and methods to study those questions, by secondary or primary research.

In the past decade, the European Commission has funded HTA projects that have sought to develop new methods for HTA and to harmonise approaches across member states. INTEGRATE HTA presented a logic model to underpin evaluation of complex technologies and this evolved into the VALIDATE-HTA project to systematise integration of values into HTA (Oortwijn and Sampietro-Colom 2022). COMED considered specific assessment issues for medical devices (2021), IMPACT HTA Work Package 10 presented an appraisal framework for rare disease medicines (Facey et al. 2021) and HTx focused on the potential for personalised treatment to inform HTA (HTx 2024).

The uptake of these methods into practice is unclear. Nationally, HTA bodies have increased transparency of their systems by publishing process and methods guides (Haute Autorité de santé 2020; Canada's Drug Agency 2025, Part III) and a new project is underway to create HTA guidance for Low and Middle Income Countries (HTAsiaLink 2024).

A major advancement in HTA collaborative activities was achieved in 2021 with the adoption of European Union (EU) regulation for HTA to create high quality, timely Joint Clinical Assessments (JCAs) from 2025 (European Commission 2024). These JCAs are expected to inform national pricing and reimbursement decisions in each EU member state (Chap. 18). This was achieved after more than two decades of informal collaborative work in the EU on HTA methods (Banta et al. 1997; Jonsson et al. 2002) and three subsequent Joint Actions of EU member states (Chap. 18).

HTA has evolved from a process that mainly informed health systems on the value of a range of existing health technologies, where a wide range of evidence is available ('full HTA'), to one that assesses a single new technology to inform reimbursement/coverage decisions. This has changed the time at which the HTA happens, the duration of the HTA, and the evidence assessed ('rapid HTA'). Today, most HTAs occur at the point of market launch when the only evidence available is from clinical research developed for the regulatory submission, which may not be published (Facey et al. 2015). This change saw a shift away from systematic reviews of all published evidence to explore the wide-ranging implications of using the

technology to one focused on the evidence submitted by the new technology's developer with a focus on what this limited set of evidence could inform—clinical effectiveness and sometimes cost effectiveness (Watt et al. 2008). These now commonly-used rapid HTA processes lack the ability to assess the wider implications of using a health technology due to the limited evidence at market launch and the short timelines (Nielsen et al. 2011).

HTAs often find 'uncertainties' in the evidence due to:

- Outcomes that may not reflect clinical practice or demonstrate patient benefit
- Lack of data about the comparator of interest
- Limited evidence about the costs and impacts of the health technology and its comparator over the lifetime of a patient.

These uncertainties are often exacerbated in rapid HTAs, where evidence is only available from limited sources over a short period.

An appraisal or multi-stakeholder advisory committee will make value judgements about these uncertainties to agree the most plausible assumptions and estimates, and to consider the wider aspects not presented in the evidence such as ethical, legal and social issues (Hofmann et al. 2014). However, few HTA bodies are explicit about their scientific and social value judgements (Hofmann et al. 2014; Rawlins 2014).

As OTA (1976) identified, when there may be differences in values, as broad a group as possible should be involved in preparing the assessment, including adversaries on certain issues. Daniels and Sabin (2008) also indicated that decisions must rest on reasons that stakeholders can agree, and this has become more important as HTAs are used to inform reimbursement/coverage decisions that may limit patient access to health interventions. Most HTA bodies assume that value frameworks are implicit in their work, as national policies and legislation have influenced the HTA domains they assess and the manner in which they undertake their processes (e.g. for stakeholder involvement, modifiers to process for certain groups such as rare diseases). However, some HTA bodies publish their value framework, such as the Institute for Clinical and Economic Review in the USA (Chap. 26). It has been recommended that Australian HTA committees consult with stakeholders to develop an explicit values framework, which will provide guidance about the elements (beyond clinical effectiveness, cost-effectiveness and financial impact) that they consider (Health Technology Assessment Policy and Methods Review Reference Committee 2024).

Alongside a values framework that may influence the conduct of an HTA, it is important to consider the quality of the deliberations that occur in HTA to interrogate evidence, identify decision-relevant uncertainties and choose the best assumptions that fit with the health system. International collaborative work on deliberative processes in HTA has stressed the value of wide-ranging stakeholder and public involvement, at each stage of an individual HTA process, and for development of the HTA process. This work outlines procedures, activities, and events that support informed and critical examination of an issue and the

weighing of arguments and evidence to guide a subsequent decision (Oortwijn et al. 2022).

1.4 Patient Involvement in HTA

1.4.1 Defining Patient in Involvement Processes

In this chapter (and for the rest of the book), when referring to patient involvement, ‘patient’ is an umbrella term where the involvement processes may refer to anyone who has direct experience of living with the condition being studied in the HTA or who may be eligible to receive the technology (e.g. specific members of the public who might be invited for vaccination or to undertake a diagnostic intervention). This can include individuals who have had or have the condition, carers (sometimes called informal caregivers) and organisations that advocate for patients, such as not-for-profit patient or consumer organisations, self-help groups, user groups and patient associations. This does not include general members of the public or citizens who may use other services in the health system or someone whose primary interest is as a clinical expert.

In 2018, the European Patients’ Academy on Therapeutic Innovation (EUPATI) developed definitions of different forms of patients from the perspective of how their knowledge may be used by R&D, regulators and HTA (Table 1.1). These are an important starting point for HTA bodies to make sense of roles within patient communities and consider which form of patients could contribute best at each stage of HTA.

Table 1.1 Definitions of ‘patient’ (Hunter et al. 2018)

Term	Descriptor
Individual patients	Persons with personal experience of living with a disease. They may or may not have technical knowledge in R&D or regulatory processes, but their main role is to contribute with their subjective disease and treatment experience.
Carers	Persons supporting individual patients such as family members as well as paid or volunteer helpers.
Patient advocates	Persons who have the insight and experience in supporting a larger population of patients living with a specific disease. They may or may not be affiliated with an organisation.
Patient organisation representatives	Persons who are mandated to represent and express the collective views of a patient organisation on a specific issue or disease area.
Patient experts	Persons who have disease-specific expertise and the technical knowledge in R&D and/or regulatory affairs through training or experience, for example EUPATI fellows who have been trained by EUPATI on the full spectrum of medicines R&D.

EUPATI European Patients’ Academy on Therapeutic Innovation

1.4.2 A Policy Imperative?

Before considering the potential for patient involvement in HTA it is interesting to consider the evolution of healthcare policy imperatives for patient involvement.

One of the most striking examples of the impact of patient advocacy in healthcare decision making arises from the strong and informed advocacy of human immunodeficiency virus (HIV) patients in the late 1980s and early 1990s. This advocacy from a stigmatised, marginalised and desperate community changed the interpretation and application of medicine regulation by the US Food and Drug Administration. It challenged the existing regulatory framework to expand beyond protecting consumers from harm, to expediting access for potentially effective treatments in severe diseases and enabling patient/clinician choice about risk/benefit (Grossman 2016).

In the late 1990s, participation of patients at every stage of publicly-funded research was put into policy by some national governments, and principles to support this were developed (Telford et al. 2004).

Meanwhile, from a health system perspective, pillars of quality were defined as effectiveness, safety, efficiency, timeliness and equity, and patient centredness (Institute of Medicine 2001). These are building blocks that not only shape safe and high-quality health services, but also resonate with HTA, including the need for patient centredness. Indeed, policy rationales indicate that patient involvement in HTA can contribute to democratic, developmental, scientific and instrumental goals (Table 1.2) (Abelson et al. 2016).

1.4.3 Operationalising Patient Involvement in HTA

‘Involvement’ is a term that is used widely but may be understood in different ways in different countries and alternative terms such as engagement, participation and empowerment may be used (Barello et al. 2014) More recently, the term patient

Table 1.2 Theoretical goals of (public and) patient involvement in HTA (OHTAC Public Engagement Subcommittee 2015)

Theoretical goal	Description
Democratic	Achieving more informed, transparent, accountable and legitimate decisions about health technologies
Developmental	Increasing public understanding of health technologies and HTA and strengthening the public’s and patients’ capacity to contribute to health technology policy issues
Scientific	Promoting a more robust and comprehensive approach to HTA that incorporates social values and ethics, as well as patients’ problems, lived experiences, outcomes and preferences
Instrumental	Making better-quality decisions across all stages of the HTA process

experience data has emerged. This book presents the concept developed by the HTA international (HTAi) Patient and Citizen Involvement in HTA Interest Group that patient *involvement* in HTA encompasses two distinct but complementary ways in which HTAs could be strengthened by taking account of patients' perspectives (adapted from Facey et al. 2010):

- *Research* into patient aspects (patients' experiences, preferences, perspectives, needs)
- Patient *participation* in HTA (individual HTAs, methods, processes, governance, and policy).

Coulter (2004) stressed that as HTA involves value judgments, it should have greater patient and public participation. She stated that a patient-focused HTA would determine the types of questions that patients want to be answered and engage them in determining HTA priorities, designing and conducting assessments and appraisals, receiving and using findings from HTA and debating policy priorities and rationing. In 2010, Gauvin et al. provided a framework to consider the different levels of patient and public participation that could be used at every stage of HTA. This framework has been developed further in Chap. 4 to identify specific mechanisms of participation that have been used by the HTA bodies.

Coulter (2004) also stated that the HTA research process should include a variety of methods to determine the experience, views and preferences of wide groups of patients. *The DACEHTA Handbook on HTA* (Kristensen and Sigmund 2007) and the *HTA Core Model*[®] (EUnetHTA Joint Action 2 2016) present methods to obtain robust evidence about patients' perspectives and experiences, but this is within the context of full HTA. For the many HTA bodies that focus on the assessment of clinical and cost effectiveness, and who must do this in a rapid time frame, there are questions about how to undertake research into patient aspects (Chap. 3). Like all issues in HTA, planning is key and such research should be planned well in advance (Facey et al. 2010) and international, multi-disciplinary collaborations encouraged. Part II of this book presents methodologies for qualitative and quantitative research to understand the implications that living with a condition has on a patient and their family and the actual and perceived impacts of a new health technology.

In 2014, HTAi undertook an international Delphi process to create consensus on *Values and Quality Standards for Patient Involvement in HTA* (HTAi 2014) as presented in Table 1.3 and Box 1.1. The values clearly relate to either research or participation, but the quality standards may relate more to participation with the assumption that research has its own ethical standards.

Table 1.3 HTAi Values for Patient Involvement in HTA (HTAi 2014)

Value	Descriptor
Relevance	Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.
Fairness	Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.
Equity	Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.
Legitimacy	Patient involvement facilitates those affected by the HTA recommendations/ decision to participate in the HTA, contributing to the transparency, accountability and credibility of the decision-making process.
Capacity building	Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organisations to work together.

Box 1.1 HTAi Quality Standards for Patient Involvement in HTA (HTAi 2014)

General HTA process

1. HTA organisations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients.
2. HTA organisations designate appropriate resources to ensure and support effective patient involvement in HTA.
3. HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients' perspectives throughout the HTA process.
4. Patients and patient organisations are given the opportunity to participate in training to empower them so that they can best contribute to HTA.
5. Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.

For individual HTAs

6. Proactive communication strategies are used to effectively reach, inform and enable a wide range of patients to participate fully in each HTA.
7. Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.
8. For each HTA, HTA organisations identify a staff member whose role is to support patients to contribute effectively to HTA.

(continued)

Box 1.1 (continued)

9. In each HTA, patients' perspectives and experiences are documented, and the influence of patient contributions on conclusions and decisions is reported.
10. Feedback is given to patient organisations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.

An international research project undertook mixed methods research to explore enactment of patient involvement in HTA in a range of European jurisdictions using these Values and Standards as the basis for discussions (Holtorf and Bertelsen 2023). Although the Standards were developed over a decade ago, they were still found to be highly relevant and a helpful guide to any HTA body that is establishing or seeking to improve its processes for patient involvement in HTA. The evolution of patient involvement within HTA processes is outlined further in the country case studies presented in Part III of this book.

1.5 Discussion

As Part III of this book shows, HTA bodies vary widely in their roles and functions. Some undertake full HTA with systematic reviews of the literature; others perform more rapid HTAs relying on structured submission of evidence from health technology developers. Some have a remit to do the assessment, others do appraisal, some do both. Some assess individual health technologies in each HTA. Some assess a wide range of health technologies for a condition in one HTA. Some provide scientific advice to health technology developers, and some support collection of local real world data post-HTA to inform a future reassessment. Some reassess health technologies to determine continued coverage within a benefits package.

HTA appraisal committees judge the available evidence within the local social and political context, trying to create fair processes with consistent decisions that can be explained. As (Coulter 2004) noted the balancing act of individual needs versus population requirements cannot be left to 'experts' alone. Patients (and citizens) need to understand the choices confronting policymakers and have the chance to be involved in determining priorities and trade-offs, but this must be done in a manner that promotes fair decisions for all users of the health system (ibid.). Indeed, as Menon and Stafinski (2015) stated, patient involvement can help resolve uncertainties that arise in any HTA.

Patient involvement in HTA can help with the difficult value judgments that arise when clinical and economic evidence is limited, or added value is at the cusp of a pre-defined threshold for acceptance, by articulating the implications for patients and whether the identified effects are meaningful to them. The imperative for this has become stronger in recent years as health technology developers have focussed

their efforts on areas of high unmet need for which regulators have permitted expedited approval (Eichler et al. 2015; US Food and Drug Administration 2024). Precision medicines, treatments for rare diseases and advanced therapeutic medicinal products expose healthcare systems to higher risk as they not only yield a smaller evidence base but also come at a very high price. Furthermore, it is important to remember that for all forms of health technologies other than medicines, the evidence base has always been sparser and the way in which the patient engages with the technology may have a strong influence on outcomes.

HTA has been described as “a bridge between the world of research and the world of decision-making” (Battista and Hodge 1999, p. 1464). We would like to complement this image to explain that patient involvement provides the lights on the bridge. Patient involvement can inform the value judgments made in any HTA by elucidating the unintended and indirect impacts of the health technology, illuminating areas of unmet need, outcomes that matter to patients and informing determination of value.

1.6 Conclusion

This chapter began with a review of HTA showing that when it was developed more than 45 years ago, it was intended to assess all the implications of using a health technology, and explicit questions were developed for patients and families. As HTA has evolved and been used to inform reimbursement and coverage decisions, comprehensive assessments are less common, and in many jurisdictions, focus has been placed on clinical effectiveness and cost effectiveness. Systematic research on patients’ perspectives and experiences has often been replaced by processes to help patients participate in HTA. These elements of research into patient aspects and patient participation are complementary, and both are the basis for how HTAi would define patient involvement in HTA. This is important because patient involvement can elucidate patients’ experiences and perspectives about the intended and unintended consequences of using a health technology at different points in the HTA process. This can help interpretation of the clinical evidence, construct of economic models, and highlight and inform the value judgments that are inherent throughout the HTA process.

Acknowledgements The authors give thanks to the co-editors for their helpful comments on the draft of this transcript. All views expressed and any errors are entirely the responsibility of the authors.

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Chapter 2

Justice as an Ethical Rationale for Patient Involvement in HTA



**Roma Dhamanaskar, Julia Abelson, Frank Gavin, Lisa Schwartz,
and Meredith Vanstone**

2.1 Introduction

This chapter presents three forms of justice, namely procedural justice, social justice and epistemic injustice, as tools to help HTA practitioners justify and advance ethical patient involvement in HTA. Here, ‘patient’ refers to people who have experience living with a particular health condition within a particular healthcare system, and those who care for such individuals, often termed ‘carers’ or ‘caregivers’. In this volume, patient involvement is an umbrella term that includes (1) research into patient aspects (patients’ experiences, preferences and perspectives) and (2)

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patient participation, e.g. inclusion of patients in HTA committee deliberations (Chap. 1).

Practitioners seeking to involve patients in HTA must consider three essential questions:

1. Why should we involve patients? (justification)
2. Which patients should we involve? (representation)
3. How should we involve patients? (operationalisation)

In the following section, we briefly discuss the complexities inherent in addressing questions of justification, representation and operationalisation and show how these issues are intrinsically linked. In doing so, we make the case for using forms of justice, which can guide HTA practitioners through the challenges of doing patient involvement well. When applied together, these forms of justice can help practitioners navigate the ethical complexity of patient involvement in HTA including the existence of multiple groups with varying interests (public, patients, patient organisations, clinicians, researchers) and numerous methods for involvement (surveys, patient and patient organisation submissions, deliberative groups, advisory committees) (Gauvin et al. 2010, Chaps. 3 and 4). This chapter aims to introduce HTA practitioners to the value and practice of using forms of justice to help justify and operationalise patient involvement in HTA; we do not aim—nor do we think it is possible—to cover all the possible considerations that may emerge from applying these forms of justice. While we believe that procedural, social and epistemic forms of justice are particularly salient and useful to the field of patient involvement in HTA, we acknowledge the existence of other forms of justice that may also be relevant, including those stemming from Eastern and Indigenous philosophies and ways of knowing. We leave open the possibility of including additional forms of justice to enrich discussions about ethical patient involvement in HTA.

2.2 Why, Who and How—From Justifying to Operationalising Patient Involvement in HTA

There are multiple justifications for involving patients in HTA. Patient involvement can enhance the comprehensiveness of HTA processes by providing information that cannot be obtained from clinicians, researchers or other professionals (Abelson et al. 2016; Sandman et al. 2017; Wale et al. 2017). This information can better reflect the experiences and perspectives of patients and may help confirm, refute or add context to information from other sources. By attuning HTA processes to patient needs and preferences, organisations and practitioners can enhance the trustworthiness, legitimacy and support for HTA decisions among patients, professionals and the public (Abelson et al. 2016; Sandman et al. 2017; Wale et al. 2017). The goal is that patient involvement can ultimately lead to better decisions across all stages of HTA.

Patient involvement can also be justified from a democratic standpoint where patients have a right to shape HTA processes which will determine the health

technologies available to them (Abelson et al. 2016; Sandman et al. 2017; Wale et al. 2017). Their involvement can improve the transparency and accountability of HTA bodies and address power imbalances that favour clinical and economic input over input about patient experiences, values and preferences. The values-laden nature of HTA decisions also supports patient involvement. Decisions about which health technologies are deemed worthy of assessment, about the decision-making framework for HTA and about how technologies are funded and delivered are grounded in values (Wale et al. 2017). Patient involvement can help uncover the full range of values worthy of consideration in HTA processes.

As discussed in the subsequent sections, procedural justice, social justice and epistemic injustice each provide rationales for involving patients in HTA. However, these forms of justice prove their usefulness when tying rationales to downstream representation and operationalisation questions that HTA practitioners inevitably face. Representation requires careful consideration of which patients should be involved in HTA processes and why. It is impossible to include everyone with relevant experiences to contribute and this problem is further complicated by the subjective nature and diversity of patients' perspectives (Sandman et al. 2017). Can one or two patient members on an HTA advisory board or involved in an HTA Committee discussion adequately convey the diversity of lived experiences in a patient population? Can a patient organisation adequately represent the views of the patient population it aims to serve? Is the involvement of a smaller patient population impacted greatly by an HTA decision more important than the involvement of a less-impacted but larger patient population?

Finally, operationalising patient involvement requires consideration of how patients will be involved in the HTA process. Here, we stress that no single approach to involvement is necessarily superior and each has its tradeoffs. For instance, surveys may be well-suited to get the broad range of experiences across patient populations, but survey questions are sometimes framed by the priorities of HTA bodies rather than those of patients (Vanstone et al. 2019). In the following sections, we apply procedural justice, social justice and epistemic injustice to questions of justification, representation and operationalisation in patient involvement in HTA. We finish with an example showing how HTA practitioners can use these forms of justice to reflect on their patient involvement practices. We stress that the forms of justice should be applied together and not in isolation.

2.3 Procedural Justice as an Ethical Rationale for Patient Involvement in HTA

HTA is a procedurally intricate field with numerous frameworks and guidelines that outline what HTA processes should look like (Abelson et al. 2016; Canada's Drug Agency 2017; Oortwijn et al. 2022, Chap. 1). These frameworks may indicate a commitment to procedural justice, where procedures are implemented to ensure that decision-making processes are fair, appropriate and trustworthy (Dale et al.

2023; Daniels and Sabin 2002). This is essential because HTA decisions are values-based and reasonable people could disagree about them. Procedural justice seeks to ensure that where we cannot objectively determine whether a decision is correct or not, we can at least strive to ensure the process of coming to that decision is fair. Patient involvement, then, can be justified as a process that can enhance procedural justice in HTA, as opposed to HTA that relies solely on the views of clinicians, researchers and policymakers and their sense of patient values and preferences.

Operationally, a procedural justice lens would require that the procedures for patient involvement are consistent with certain fairness principles. However, exactly *what* these principles are is up for debate. Accountability for reasonableness (A4R) has gained broad acceptance as a framework to determine whether HTA procedures are fair (Daniels and Sabin 2002, Chap. 1). However, the A4R principles are designed to assess the fairness of decision-making processes broadly and not patient involvement specifically. Questions about representation arise because the A4R framework does not specify *who* should be involved in determining the underlying criteria by which HTA decisions are made, nor does it expressly suggest that patients should be involved. Gibson et al. (2005) also argue that A4R is inattentive to the power differentials between individuals involved in the decision-making process. This seems especially important when involving patients in HTA processes where patients can be tokenised or required to engage on the organisation's terms (Ocloclo and Matthews 2016; Glimmerveen et al. 2022).

There are numerous frameworks and standards that attempt to outline what patient involvement in HTA processes should look like (Abelson et al. 2016; Oortwijn et al. 2022, Chap. 1). These frameworks are useful because they are specific to patient involvement and can help HTA practitioners plan and evaluate their patient involvement activities. To an extent, the focus on procedures of patient involvement in HTA reflects one of the rationales for procedural justice—fostering the legitimacy of HTA decisions. Having standards and guidelines for involvement can help demystify the decision-making process, improving transparency and accountability (Abelson et al. 2016; Oortwijn et al. 2022; Hailey 2003). However, it is worth questioning whether procedural standards always advance procedural justice or whether they can lead practitioners down a narrow path of ticking check-boxes and setting rigid protocols.

An uncritical procedural focus can have unintended consequences. For example, does patient participation, which is happening in many countries as described in Part III of this book and which can take many forms, such as encouraging patient submissions and inviting patients to answer questions posed by a committee, always advance procedural justice in HTA? Do such limited engagements allow for the appropriate involvement of equity-deserving groups that may not be able to access such opportunities? Could research into patient aspects as described in Chap. 3 overcome some of these challenges? Do deliberative committees advance procedural fairness if they require professionals to deliberate *about* patient input rather than *with* patients directly or if they require patient members to navigate the practices of an HTA committee with little or no consideration for what they need to participate? In such a setting, are patients' perspectives valued similarly to professionals' perspectives and is research about patient aspects given similar attention

and weight to other types of research, like clinical trials and cost-effectiveness analyses? Where procedures are standardised, do they mainly reflect institutionally accepted or streamlined options for patient involvement rather than a commitment to having patients inform the HTA process?

While HTA bodies are limited in the time and resources available to conduct patient involvement, it is worth considering whether current procedures, especially those that have become entrenched within organisations, advance procedural justice or simply over-proceduralise the HTA process. When examining processes, practitioners may find it useful to ask whether procedures align with rationales like greater accountability and transparency that are rooted in procedural justice. However, as we show below, understanding social justice and epistemic injustice can help HTA practitioners uncover other essential considerations when judging the procedural fairness of patient involvement.

2.4 Social Justice as an Ethical Rationale for Patient Involvement in HTA

A social justice approach is concerned with how a process is attentive to groups that are systemically marginalised in society (Ruger 2004; Schoem 2014). In this chapter, we use the term equity-deserving groups, where equity has been denied to members of these groups due to historical injustices and long-standing barriers to accessing the healthcare system. At a systems level, a social justice focus in HTA would try to ensure that HTA decisions do not exacerbate social injustices and possibly even help improve equitable healthcare outcomes by promoting fair access to health technologies (Agboola et al. 2023; Axler et al. 2023; Ghinea et al. 2020). For example, digital health technologies may not be accessible to those individuals lacking digital health literacy or access to the internet (Institute for Clinical and Economic Review 2023). A social justice focus would ask how such individuals could still receive the best available standard of care.

It is also possible that decisions about *which* technologies are assessed by HTA bodies are determined by dominant groups that have the money and resources to influence policy (Ghinea et al. 2020; Denburg et al. 2020; Razavi et al. 2022). A social justice lens would ask us to consider why and how some technologies are proposed, championed, and supported for assessment over others, for instance by considering the role of health technology developers and the categories of technologies without sponsorship which may seldom be considered. Prioritising social justice would bring attention to the opportunity costs in HTA that have important equity implications, where investment in technologies that are expensive and fashionable (e.g. artificial intelligence) can lead to reduced investment in other technologies. Broader priority-setting decisions may warrant broader public involvement in HTA and the targeted involvement of communities that experience health disparities to determine the appropriate allocation of limited healthcare funds. Technologies that have the potential to reduce health disparities might be worthy of specific

attention, particularly in the ways they may be implemented to promote health equity (Benkhalti et al. 2021; Garfield et al. 2023).

At the patient involvement level, attention to social justice would require HTA bodies to question whether the current methods of involvement are able to reach equity-deserving communities (Agboola et al. 2023; Axler et al. 2023; Ghinea et al. 2020; Benkhalti et al. 2021; Garfield et al. 2023). It is well known that public participation in health system settings tends to favour those who are well-resourced—high income, well educated, usually retired, and, in Western countries, often white (Vanstone et al. 2019; Abelson et al. 2022; Sayani et al. 2021). Assumptions about who should be engaged and who has relevant knowledge may need to change when social justice considerations are applied. Beyond experience living with a particular illness or accessing a particular technology, identity markers like race, gender and income shape how illness and healthcare are experienced (Eilert et al. 2022; Paradies et al. 2015). Individuals with lived experiences of racism, sexism and other forms of disadvantage should be included for their unique and critical perspectives that others (even those with the same illness) may not have. Attention to social justice may require us to consider including those individuals who may not have had the opportunity to become patients due either to barriers to access or to an understandable mistrust of the healthcare system.

When thinking about representation, HTA practitioners should carefully consider which equity-deserving communities should be engaged for a particular HTA process. Selection could be informed by historical data about which communities are able to access certain technologies, which communities face barriers to access and which communities are uniquely impacted by a particular illness (Agboola et al. 2023). Awareness that certain communities are disproportionately underrepresented in health research would facilitate critical examination of existing research and could encourage new research into patient aspects informed by community members. Operationally, a social justice lens would be cognisant of the history of exclusionary and unethical research practices imposed on certain equity-deserving groups (Crooks et al. 2021; Scharff et al. 2010). Research would need to be undertaken carefully to ensure adequate representation and avoid perpetuating harms. A social justice approach would also question the often bureaucratic and professionalised processes for patient participation prevalent in HTA and call for more community-based participation. Involving members of equity-deserving groups may require additional time to foster trusting relationships between HTA organisations and community members who have experienced historical and ongoing harms.

2.5 Epistemic Injustice as an Ethical Rationale for Patient Involvement in HTA

Epistemic injustice is concerned with how knowledge is shared and valued in communicative practices (Fricker 2007). Epistemic injustice stands apart from procedural and social justice, as it is framed in the negative: as an *injustice*. In other words, epistemic injustice aims to describe what unjust conditions look like,

allowing us to consider how we may avoid or correct them. Epistemic injustice occurs when a person is treated unjustly in their capacity as a knower (ibid.). This happens through two forms of injustice: testimonial injustice and hermeneutical injustice (Box 2.1).

One justification for patient involvement in HTA is that patients have relevant

Box 2.1 Forms of Epistemic Injustice

Testimonial injustice: occurs when a person's knowledge is devalued because of their identity markers (e.g. as a patient, as a member of a particular race).

Hermeneutical injustice: occurs when a person is not able to share their perspective because their experiences cannot be adequately understood through existing knowledge and value systems (e.g. patient input might not be communicated clearly through the language that researchers and clinicians use) (de Boer 2021; Liabo et al. 2022).

knowledge to contribute to HTA processes (Chap. 4). Patient involvement can elucidate patient needs for a particular technology, tradeoffs for using that technology, patient-important outcome measures and the way a technology fits into a patient's overall experiences of the healthcare system (Wale et al. 2017). Excluding or devaluing these perspectives by committing testimonial and hermeneutical injustices undermines patients as relevant knowers. Practically, this can lead to poorer HTA decisions as relevant knowledge is not factored into decision making (Wale et al. 2022; Single et al. 2019). Ethically, it calls into question the point of doing patient involvement in the first place, if the knowledge contributions of patients deemed to be relevant knowers are not taken up in HTA processes.

Testimonial injustice in HTA can occur when patient perspectives are undervalued compared to technical perspectives. HTA may be ripe for such injustice as many different types of knowledge (clinical, economic, patient) have to be shared and factored into decision making, with no guidance on the appropriate and relative weight of each type of knowledge (Chap. 4). Patient contributions may be perceived as less credible or relevant to HTA decisions as compared to more authoritative forms of knowledge coming from clinical trials, cost-effectiveness analyses and clinician testimonials. These latter forms of knowledge have historically garnered legitimacy and power through their relationship to science and medicine as knowledge-making fields (Busfield 2017; Clancy et al. 2022).

Beliefs about which types of knowledge are *more* or *less* credible or not credible at all are pervasive and can become embedded within organisations. It is important to question how certain forms of knowledge, particularly knowledge offered by patients, may be differently valued (or devalued) depending on how HTA procedures are set up (Vanstone et al. 2019, Chap. 4). A practice that can help address testimonial injustice is epistemic humility, where patients and non-patients appreciate the knowledge contributions of all members of a decision-making process

(Dieleman 2015). Reflexivity, where HTA practitioners and committee members interrogate their own worldviews and potential biases about who is a credible knower and why, may also be useful (Vanstone et al. 2019).

Hermeneutical injustice can occur when HTA bodies are operationalised for the communication and use of clinical, economic or other technical information over patient input. For example, HTA committees with only one or two patient members may privilege norms of communication used by researchers and clinicians, or patients may feel uncomfortable sharing their viewpoints. Committees where patient input is collected through structured input forms or surveys may not ask questions that are most relevant to patients, the rigour underlying patient input may not be recognised, or patient contributions may not be deemed important enough to substantially influence recommendations (Vanstone et al. 2019). Patient involvement that happens late in the HTA process may be seen as tokenistic and patients may feel it is too late to contribute meaningfully (Wale et al. 2022).

While an understanding of epistemic injustice can be useful to identify unfairness in knowledge sharing practices, HTA practitioners must first consider *who* the relevant knowers are in a particular context. It could be those who have experience in a particular healthcare system, with a particular illness and/or with a particular health technology. Here we can see the entwinement of epistemic and social justice; those who are most likely to face barriers accessing a particular technology may also face barriers in participating in an HTA. Further, are individuals who respond to a survey or volunteer for an HTA committee necessarily those with the most relevant knowledge? Recognising the barriers and considering the personal resources and motivation required to participate in HTA processes, it is likely that those who can afford to contribute their time to such initiatives differ in substantial ways from those who cannot (Vanstone et al. 2019).

These unanswered questions about representativeness present opportunities for innovative practice. For example, during the scoping phase of an HTA, practitioners can brainstorm about which patient communities might be relevant knowers. This could be informed by available data about which communities are impacted by a particular condition or have accessed a particular technology in the past or in different settings. A social justice approach would also note that research has often excluded equity-deserving groups and certain communities may be perceived as being less impacted than they actually are (Sharma and Palaniappan 2021; Varma et al. 2023). Initial patient involvement efforts could focus not only on the HTA but also on who should be involved in the HTA and the different mechanisms of involvement that may be necessary to involve them meaningfully. This could allow practitioners to expand the list of relevant knowers to those not

previously considered, yielding new forms of research into patient aspects or communities for outreach. Recognising the problems of testimonial and hermeneutical injustice, HTA bodies can design processes to record which communities were involved, why they were involved, how they were involved and how their input informed decision making.

2.6 Discussion

Procedural justice, social justice and epistemic injustice offer important considerations when addressing questions of justification, representation and operationalisation in patient involvement in HTA. We intentionally address justification, representation and operationalisation together in this chapter because we see them as intimately connected; alignment between purpose and methods is central to ethical involvement. We hope this chapter will encourage HTA practitioners to think critically about their reasons for involving patients in HTA and connect those reasons to their decisions about which patients to involve and how.

Each form of justice offers unique criteria by which to justify and carry out patient involvement; however, none can be applied in isolation. The strengths of these forms of justice become apparent when they are applied together; prioritisation of one over the others can produce unjust outcomes. To exemplify how these forms of justice work together in practice, we will introduce a hypothetical case study and illustrate how an HTA practitioner may appraise the ‘justness’ of patient involvement using procedural justice, social justice and epistemic injustice. Please note that the forms of justice need not be used only as tools for appraisal or evaluation; they can also be used when planning and conducting involvement.

Consider a hypothetical example of an HTA process for drug A to treat disease X. The HTA process involves deliberation by a committee of 18 members: three patient members and 15 individuals from professional backgrounds. All committee members are engaged for a specific term and are involved in a range of assessments; none have disease X. Patient participation is facilitated through submissions from two large patient organisations for disease X, who conducted surveys, interviews and focus groups with patients with disease X. The committee makes recommendations through a majority vote.

To apply the forms of justice, an HTA practitioner may ask the following questions (Table 2.1).

Table 2.1 Justice considerations for example HTA process

Procedural justice considerations	• How do patient members contribute to committee deliberations? (related to epistemic injustice) – Fairness issues may arise if patients are not provided sufficient, comprehensible information about clinical, economic and other aspects to meaningfully contribute – Fairness issues may arise if patients are not provided training, mentoring and other tools needed to confidently participate in discussions – Professional members may overshadow patient members if the latter do not feel equipped or comfortable participating in deliberations – Professional contributions may be deemed more objective, relevant or credible than input from patient members • How are submissions by patient organisations discussed and considered by committee members? (related to epistemic injustice) – Fairness issues may arise if adequate time and value are not given to patient input – Information from clinical research and cost-effectiveness analyses may be considered more objective, relevant or credible than input from patient submissions • How are patient organisation submission templates designed? – Templates may not allow patients to decide how to share their lived experiences, especially if their input has a limited word count (related to epistemic injustice) • Who is likely to contribute to the patient organisation submission? – Patient organisation submissions require a significant amount of time, expertise, funding and motivation—patient organisations with more resources are more likely to submit • Groups representing equity-deserving communities may have fewer resources to involve their communities and submit input (related to social justice) • How is the HTA process and the impact of patient involvement reported?
Social justice considerations	• Are patient organisations required to report demographic details about whom they surveyed/interviewed? (related to procedural justice) – Patient organisations cannot be assumed to have diverse memberships and there may be little or no involvement of members of relevant equity-deserving groups – Certain communities are disproportionately impacted by disease X and targeted involvement of these communities may be needed • Where demographic details are not reported, involvement of these communities is unknown • Where demographic details are reported, is the level of involvement sufficient or is targeted outreach required? • Do templates ask specific questions about the involvement and perspective of equity-deserving groups? (related to procedural justice) – If not, equity issues and the unique experiences of equity-deserving groups may be ignored (related to epistemic injustice) – If not, patient organisations may not be incentivised to involve equity-deserving groups • Who are the groups being involved? – Patient organisations, especially those without previous exposure to HTA, might not be aware of a call for input unless it is shared with them and they may need support to understand what's required – Patient organisations with more resources are more likely to submit • Are all populations with relevant knowledge involved? (related to epistemic injustice) – Patient organisations involve only people with disease X, not members of the public or other patients who may be at risk or likely to develop the disease • This may be important for equity-deserving groups who are more likely to develop disease X

(continued)

Table 2.1 (continued)

Epistemic injustice considerations	• How are patient members without experience with disease X expected to contribute? – While patient members may still bring up important concerns relevant to patients or help the committee discuss the patient organisation submissions, they cannot be expected to contribute as people with lived experience of disease X • Two-way communication between patient members of the HTA committee and representatives of patient organisations may help facilitate mutual learning and understanding • How are patient organisation submissions solicited and considered? – Templates created by the HTA organisation without input from patients/ patient organisations may reflect organisational priorities (related to procedural justice) – Patient organisations may not adequately represent the patient population or be able to identify the specific patient population that is the subject of the HTA – Surveys created by the patient organisation without input from their members may reflect organisational priorities (related to procedural justice) – Patients may feel more comfortable sharing their perspectives in interviews and focus groups coordinated by a trusted patient organisation than directly through the HTA organisation

2.7 Conclusion

This chapter identifies three essential questions facing practitioners seeking to involve patients in HTA:

- Why involve patients? (justification)
- Which patients should or need to be involved? (representation)
- How should patients be involved? (operationalisation).

We show how applying procedural justice, social justice and epistemic injustice identifies and strengthens important justifications for patient involvement while highlighting areas of ethical complexity and opportunities for improved practice. We call attention to the interconnectedness of rationales for patient involvement with operational decisions about how to plan, facilitate and evaluate involvement processes. Clearly articulated rationales early on in patient involvement set an important standard for practitioners to continuously improve patient involvement processes in HTA. Together, procedural justice, social justice and epistemic injustice equip practitioners with a toolbox of rationales and operational considerations to help them think more critically about ethical patient involvement in HTA.

Acknowledgements The authors give thanks to the coeditors and external reviewer for their thoughtful comments on the draft of this transcript. All views expressed and any errors are entirely the responsibility of the authors. The ideas expressed here are from RD’s PhD work funded by a doctoral Canada Graduate Scholarship through the Social Sciences and Humanities Research Council. JA is an internationally recognised leader in public and patient involvement and professor in the Department of Health Research Methods, Evidence and Impact (HEI) at McMaster University. She has contributed to several projects about patient involvement in HTA with a particular focus on the use of deliberative methods. FG is a retired community college English teacher,

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Chapter 3

Research into Patient Aspects



Karen M. Facey , Neil Bertelsen, and Anke-Peggy Holtorf

3.1 Introduction—What Patient Aspects are Relevant to HTA?

From its inception in the 1970s, health technology assessment (HTA) was intended to systematically assess all the impacts of introducing a health technology, including the wider impacts on patients, carers, and their families (Chap. 1). This is important, because the experiences of patients, across many dimensions and life spheres (home, school, work, social activities, health service, etc) are a vital part of understanding the context and impact of health technologies (EUnetHTA Joint Action 2 2016, p. 348). Furthermore, administration of a health technology may place a burden on the patient and may change their abilities to function in their life spheres in positive and negative ways, and these will vary among patients (ibid.). This has been confirmed in the more recent, internationally agreed, definition of HTA. It notes that the dimensions of the value of a health technology compared to existing alternatives

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K. M. Facey et al. (eds.), *Patient Involvement in Health Technology Assessment*,
Health Informatics, https://doi.org/10.1007/978-3-032-11284-2_3

can include wider implications for patients, relatives, caregivers, and the population (O'Rourke et al. 2020).

At the outset, HTA was research-based and versatile, drawing on a range of interconnected scientific disciplines (health science, social science, natural science and the humanities) (Kristensen and Sigmund 2007). These disciplines could draw on a range of research genres to suit the initial research questions and those that evolved during the HTA. The focus was on synthesis of published articles, only performing primary research if knowledge gained from review of the literature was inadequate and of vital importance to the HTA (ibid). The new definition of HTA still notes that the HTA process “uses state-of-the-art methods to consider the best available evidence” (O'Rourke et al. 2020, p. 2). Our concern is that this concept of using the best available evidence is often applied to clinical and cost effectiveness considerations, but not to those that will help elucidate important patient aspects.

This chapter outlines how HTA can gather evidence to understand the wide-ranging perspectives, experiences, attitudes, preferences, values and expectations regarding health, illness, service delivery and technologies (EUnetHTA Joint Action 2 2016, p. 346). These perspectives may include feelings of hope, fear, uncertainty, and societal values (ibid.). The exploration might include patients' experiences of living with the condition and experiences of existing and new health technologies (Facey 2019). It may also highlight consequences on daily life such as required interaction with the technology, improvement in self-care, ability to work and engage in social activities, and resources required, including financial burdens (Kristensen and Sigmund 2007, Chap. 7; EUnetHTA Joint Action 2 2016). If patients do not have experience of a new technology they can still explain their preferences, needs and expectations of it (Facey et al. 2010). All these issues, we term ‘patient aspects’.

A review of the patient issues reported by HTA bodies identified individual, ethical, economic aspects, social and communication patient aspects (Lee and Sinding 2007) which led to the delineation of potential issues for research into patient aspects as shown in Table 3.1.

Even if some of these issues may be studied in other parts of an HTA, patients' perspectives on these aspects should influence that research and its evaluation.

Examples of research into patient aspects that have influenced HTAs are presented in Parts II and III. They show that such research can be integrated into evaluations of clinical and cost effectiveness to help scope an HTA and resolve uncertainties in evidence interpretation (Facey et al. 2018). HTA bodies may also produce evidence about patient aspects from primary (Chap. 27) or secondary research (Stewart et al. 2021) that is considered alongside clinical and cost effectiveness in an HTA.

More research is needed to further strengthen the evidence about patient aspects and the trust in this evidence, especially as since the first edition of this book, we have seen a decline in investment in research in this field. A reason for this could be a requirement for rapid evaluation of evidence when it is used to inform pricing and reimbursement decisions of new health technologies. As a result, HTA has become dependent on evidence submissions from health technology developers

Table 3.1 Examples of issues that might be explored in patient aspects (Kristensen and Sigmund 2007, Chap. 7)

Aspect	Examples
Individual	From a patient perspective does the technology affect: • Existential experiences such as insecurity, worry, hope, anxiety • Patient roles and stigmatisation • Courage to face life • Satisfaction • Use of one's own resources (self-care, empowerment)
Social	Direct or indirect influence on: • Family life • Work and training • Leisure time • Lifestyle/quality of life
Economic	Direct or indirect costs related to: • Work • Family life • Leisure time • Lifestyle/quality of life
Ethical	From a patient perspective are there any: • Ethical considerations • Ethical choices • Ethical dilemmas
Communication	Influence on: • Exchange of information • Patients' knowledge and understanding of the technology • Modified relations between the patient and health professionals • Involvement in decision-making.

(particularly for medicines), and it has narrowed its scope of assessment to quantitative evidence about clinical and cost effectiveness. In some cases, participatory approaches (Chap. 4) are used to bring in wider patient aspects, but few HTA bodies now include research into patient aspects in their processes: either undertaking it themselves or requiring it to be submitted by health technology developers. Furthermore, when a health technology developer undertakes research into patient aspects (Chaps. 30 and 31), HTA assessors may not be familiar with critically assessing such evidence and appraisal committees may not recognise its scientific integrity and so treat it anecdotally, rather than as robust evidence.

Fuelled by the prioritisation of clinical and cost effectiveness aspects in HTA, which are predominantly driven by quantitative methods, health technology developers have invested in quantitative and mixed-methods research to understand patient aspects (Chaps. 6, 9, and 31). Purely quantitative approaches have limitations such as looking at a limited set of predefined outcomes measures and a preference for standardisation rather than personalisation. Furthermore, focus on quantitative methods may also constrict the frame of analysis, and consequently, compromise the knowledge about a technology as it is used in its context. However, mixed-methods approaches that begin with qualitative approaches to shape quantitative research can be very valuable.

Hence, this chapter will put more emphasis on the less well implemented field of qualitative research by reflecting on the meaning of evidence and knowledge, and exploring the underpinnings and robustness of qualitative research. This will be complemented by an overview of quantitative methods and finally, a reflection on how research into patient aspects can be promoted in HTA.

3.2 Evidence for HTA

As Murphy et al. stated, “the goal of all research in HTA should be to establish knowledge about which we can be reasonably confident, and to provide findings that are relevant to policy makers and practitioners” (Murphy et al. 1998, p. iii). This requires a broad perspective on the systematic accumulation of knowledge as described by Plato’s ‘arch of knowledge’ that spans from induction to deduction with particulars (e.g. perceptions) providing the basis for universals (concepts, models, theories) that are supported by testable predictions and explanations (Sale and Thielke 2018).

Research may originate from a range of disciplines and produce ‘evidence’, but to be valuable and build knowledge it must be critically evaluated taking account of the research paradigm from which it emanates. In the medical field, the integrity of quantitative research to build knowledge is well accepted if its generation and reporting follows agreed upon norms, and even the role of qualitative evidence is also becoming more recognised (Williams et al. 2019). However, the fact that qualitative research is a valid systematic and reflective process for development of knowledge, using systematic processes with documentation of methods, critical reflection on the reflexivity of the researcher (Malterud 2001), peer review and external scrutiny (Nutley et al. 2013) is not well understood by many HTA decision makers.

Research that seeks to determine patients’ experiences, perceptions, needs, or attitudes about their care and health can be termed as patient-based evidence, (Staniszewska and Werkö 2021). This term was promoted in version 1 of this book, but uptake of this terminology in the HTA field has not been widespread and in the United States of America (USA) the use of the term ‘patient experience data’ is more common (US Department of Health and Human Services Food and Drug Administration 2020).

3.2.1 *The Rise of Patient Experience Data as Evidence in the Regulatory Setting*

As health technology developers’ submissions of evidence have become more important in HTA, it is important to understand the influencers of their evidence generation plans and, within those, any evidence that could be relevant to determine patient aspects. The requirements of the US Food and Drug Administration (FDA) are a key driver in any developer’s plans, and in recent years, they have encouraged

the inclusion of ‘patient experience data’ for medicines and medical devices as part of the agency’s Patient Focused Drug Development programme (PFDD). This approach has been written into statute in the twenty-first Century Cures Act (US Government 2016, Sect 3001(c)) stating that

patient experience data includes data that:

- (1) are collected by any persons (including patients, family members, and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and
- (2) are intended to provide information about patients’ experiences with a disease or condition, including (A) the impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy or clinical investigation, on patients’ lives; and (B) patient preferences with respect to treatment of such disease or condition

To support health technology developers in considering collection of patient experience data in their evidence generation plans, the FDA has released four PFDD guidance documents (ibid.). These documents guide the process from collecting comprehensive and representative input, using this to identify what is important to patients, ensuring that these important impacts are captured in clinical outcome assessments, and finally detailing how these outcome assessments aid regulatory decision-making. Furthermore, the FDA submission template for health technology developers now includes a section to highlight patient experience data within the dossier. The European Medicines Agency also intends to publish guidance on generation of ‘patient experience data’ that includes its reporting in a specific section of the evidence submission.

Such evidence should be of value but is not yet considered in the same way in HTA. However, we have some concerns with this definition of ‘patient experience data’ as it also includes reports of people’s health-related experiences, perceptions of their health status and needs, and preferences, which we see as distinct types of evidence (Chap. 13). Hence in this book we promote use of the more holistic term of ‘patient-based evidence’.

3.3 Qualitative Research

3.3.1 *Origins and Methods*

Qualitative research requires “the systematic collection, organisation and interpretation of textual material derived from talk or observation” (Malterud 2001, p. 483) to explore the meaning of social phenomena in their natural context. It is an enquiry process that involves in-depth exploration of human problems and phenomena (Majid and Weeks 2021; Sale and Thielke 2018) to produce data that relate to the social world, concepts and behaviours of people (Murphy et al. 1998). It enables exploration of the meanings people attach to experiences, the relationship between knowledge, experience and action and the social factors that shape these processes (Popay and Williams 1998).

In the field of medicine, such patient values and preferences are at the foundation of evidence-based medicine (Sackett et al. 1996). Furthermore, qualitative research has potential value to engage ‘hard to reach’ patients (Pope and May 1995) and explore patients’ articulated knowledge, experiences, attitudes, needs and desires. As Malterud states: “Qualitative research methods could help us to improve our understanding of medicine. Rather than thinking of qualitative and quantitative strategies as incompatible, they should be seen as complementary” (Malterud 2001, p. 483).

Qualitative research arises from disciplines such as anthropology, sociology, psychology and education (Hansen 2007), and findings are descriptions, notions or theories that are applicable in the specific setting of the research. Each of these disciplines has drawn from different theoretical positions and epistemological standpoints (philosophical understanding of knowledge) to inform their perspectives and research practices (O’Reilly and Kiyimba 2015). As a result a wide range of qualitative research methodologies exist such as thematic and template analysis, ethnography, grounded theory, narrative analysis, discourse analysis, interpretative phenomenological analysis, conversational analysis, facet methodology and action research (O’Reilly and Kiyimba 2015, Chap. 4). Of these, thematic analysis and grounded theory appear to be the analytical approaches most accepted for HTA, often through use of qualitative data analysis software such as NVivo (<https://lumivero.com/products/nvivo/>) or MAXQDA (<https://www.maxqda.com>).

The most commonly used approaches for collecting qualitative data in HTA are interviews and focus groups (Szabo et al. 2024). These allow in-depth exploration of a small number of issues, with questions adapted during each meeting to react to what the individual or group is sharing and the researcher’s emerging ideas. Interviews with individuals can be particularly beneficial when sensitive issues might be addressed, whereas focus groups aim to create a dynamic that encourages natural discussion that is able to draw out conflicting experiences and may give quiet individuals confidence to contribute, when they hear the views of others.

Chapter 10 expounds the value of ethnography to conduct interviews and participant observation and provides several examples of their use in HTAs. In other HTA settings, qualitative research may be undertaken with less scientific rigour, even so the findings from such activities can provide valuable insights to those responsible for the interpretation of clinical evidence and potential place of a technology in a care pathway (Chap. 27).

Reviewing qualitative research requires the expertise to recognise that the choice of methods used should have used theoretical considerations based on scientific theory (Hansen 2007). As a result terminology may be used differently across different approaches given their differing epistemological stances (Ring et al. 2010). Furthermore, an essential aspect of qualitative research, as outlined in Chap. 10, is that the researcher shapes the knowledge that is produced and so any evidence should critically reflect on what the role and influence of the researcher has been.

While the results from one qualitative study may be difficult to generalise, a synthesis of all relevant qualitative studies may draw on research from a wider range of settings (including different jurisdictions) and so can result in identification of common themes, as well as divergent views and experiences. A range of approaches are available and applied for such synthesis in healthcare. For instance, in Scotland

a study in 2010 suggested that critical interpretive synthesis, grounded theory, meta-ethnography, meta-interpretation, meta-study, meta-summary, qualitative cross-case analysis, thematic synthesis, realist synthesis and narrative synthesis were frequently used in the healthcare setting and should be considered for HTA (Ring et al. 2010). Chapter 11 brings that work up to date. It presents the latest methods for qualitative evidence synthesis that can be applied in HTA and explains how patients can participate in that research.

3.3.2 *Use of Qualitative Research in HTA*

In 1998, a comprehensive literature review concluded that qualitative research could provide valuable information about the implementation and impact of health technologies on patients (Murphy et al. 1998). In 2010, the potential value of qualitative research in HTA to illuminate experiences of living with a condition and the patient journey through a health system, including the effects of different treatments was promoted by Health Technology Assessment international (HTAi) (Facey et al. 2010). Others suggested it could help to identify barriers and facilitators to the delivery or implementation of new technologies in practice and explain why technologies may be more (or less) effective in some population groups than others (Ring et al. 2010). More recently, the potential of qualitative research to offer in-depth information about patients' experiences, needs, preferences and attitudes to health and care has been recognised (Szabo et al. 2024) for the rationales outlined in Box 3.1. Furthermore, as only a small number of participants are needed to produce this rich information, its value in rare diseases has been particularly noted (Facey and Hansen 2015).

Box 3.1 Rationales for Use of Qualitative Research in HTA (Szabo et al. 2024)

- Assessing acceptability, perceptions of benefit and safety, aspects of most or least merit
- Understanding perspectives and providing context (including experiences of condition and carer impact)
- Reaching groups other methods cannot reach
- Laying groundwork for subsequent quantitative work
- Contributing to economic model development such as modelling strategy or specific parameter inputs

A review of all National Institute for Health and Care Excellence (NICE) and Canadian Agency for Drugs and Technologies in Health (CADTH) medicine HTAs for a 2-year period from October 2019 (Szabo et al. 2024) identified that 83/107 NICE HTAs and 119/124 of CADTH HTAs included submissions from patient

organisations. Reports of the research undertaken were limited. In the NICE HTAs, 14 described qualitative data collection. Six of these explained analysis methods but only two scored more than 7/10 yes's on the Critical Appraisal Skills Programme (CASP) checklist (Long et al. 2020), the most commonly used tool for quality appraisal in health-related qualitative evidence syntheses. In CADTH, 24 included qualitative data, nine described methods, and seven scored more than 7/10 on the CASP checklist. The most common objectives of the qualitative research were to understand perspectives and provide context (e.g. patient experience of disease and carer/family impact) and to ascertain acceptability and subjective value of the new treatment. Methods were generally interviews and focus groups, using thematic analysis. The most deficient aspects of reporting were adequate consideration of the relationship between the researcher and participants, considering ethical issues and providing sufficiently rigorous data analysis. In the other patient organisation submissions, patient insights were obtained in a less formal manner via ad hoc input and surveys (Szabo et al. 2024).

As HTA has become more expedited, participatory approaches have taken precedence over qualitative research to understand patient aspects as can be seen in Part III of this book. However, its use to lay the groundwork for subsequent research has increased. This is particularly noticeable given the stricter methodologies in place to develop patient-reported outcome measure (PROMs) and as the basis for creating the constructs for trade-off discussions in patient preference studies (Chap. 9).

3.3.3 *Explaining Legitimacy*

In medical spheres, qualitative evidence is often confused with information that is not quantitative and has the connotation of being subjective and lacking facts (Malterud 2001). Major medical journals have been criticised by high-standing healthcare academics for not including such articles because they are considered unscientific and lacking utility (Greenhalgh et al. 2016) and difficult to generalise (Loder et al. 2016).

However evidence arising from qualitative research draws legitimacy from its underpinning intellectual traditions that go back 2000 years (Murphy et al. 1998). It encompasses a range of approaches that each have a clear theoretical basis, rigorous planning, techniques for analysis and rigour in reporting including critical reflection on researcher bias. Long-established methods exist to critically assess its scientific validity (Williams et al. 2022; Germini and Szabo 2023; Long et al. 2020) and a clear and simple guide is given in the *HTA Core Model*[®] (EUnetHTA Joint Action 2 2016, pp. 355–7). This is not well understood in HTA spheres and undermines the perceived value of the evidence arising from qualitative research.

Moreover, qualitative research can provide legitimate evidence. It can ensure that an HTA is informed by the values, beliefs, perspectives, experiences and preferences of the intended recipients of the technology (Majid and Weeks 2021) and is truly patient-focussed (Facey et al. 2018). It can provide information about the

context in which an HTA decision will be made and help clarify research questions, aid conceptualisation and be used to interpret, qualify or illuminate quantitative evidence (Murphy et al. 1998). However, for qualitative research to be considered credible, it must adhere to recognised quality and reporting standards, and focus on areas that quantitative methods cannot illuminate (Germení and Szabo 2023). Furthermore, HTA would benefit from staff and appraisal committee member education on the value of such evidence along with increased investment in, and engagement, with social scientists to develop methods that fit within HTA processes, as was done for economic modelling.

3.4 Quantitative Research

3.4.1 *Patient-Reported Outcome Measures (PROMs)*

Health outcomes can be influenced by an individual's experience of symptoms, their motivation, values, preferences and psychological state, by their social environment and economic situation (Holtorf 2022). Ideally, to obtain a holistic view of the impact of a new health technology, all these aspects should be considered based on multi-dimensional measures that are directly reported by the patients, the so-called PROMs. A wide range of PROMs exist. They differ by the reporting approach, constructs they measure, and attributes of the person that investigators hope to capture. One subgroup of PROMs aims to measure health-related quality of life and includes aspects relating to health status such as physical, mental and biological functioning (ibid.).

In the past decade, there has been major investment in research to develop disease-specific PROMs (Whittal et al. 2021). Input from patients and carers in the development of PROMs is key (Haywood et al. 2017) and is now often driven by patients (Chap. 6). Work is underway to measure outcomes reported by carers (Carlton et al. 2022) and 'spillover' effects in families (Henry et al. 2024). In some cases, these are already being used in economic evaluations for HTA (Pennington 2020).

Rigour in the development and analysis of PROMs that report quality of life has been partly driven by stringent regulatory requirements in the USA (US Food and Drug Administration 2022). A new PROM requires rigorous background research to identify relevant concepts for measuring the constructs of interest that are not captured in other validated instruments, creation of a clear questionnaire that can be understood by patients, piloting and validation, specific to the languages in which it will be deployed. The quality of a PROM can be evaluated by considerations of content validity, internal consistency, criterion validity, construct validity, reproducibility, inter-rater reliability, intra-rater reliability, responsiveness, floor and ceiling effects and interpretability (Holtorf 2022). Disease-specific PROMs have a growing role to play in relative effectiveness assessments as a complementary evaluation of

the impacts of a treatment alongside a clinical outcome. This is most notable in HTA bodies that rely on added benefit assessments, such as France and Germany, which now specify exactly how such measures should be analysed and the expected size of effect (Schlichting et al. 2022).

In contrast, generic PROMs can be used to compare patient reported outcomes across a range of diseases but are often criticised for being less sensitive for changes in a particular condition (Chap. 6). A subgroup of the generic PROMs are the index instruments such as the EQ-5D, which enable the calculation of the utilities that are the backbone of a cost utility analysis underlying many HTA recommendations (Meregaglia et al. 2020).

The choice of PROM depends on the purpose for which it is to be used. The EU-funded HTx project has undertaken extensive work on the use of PROMs in HTA and healthcare to create a PROM toolbox, including a web-based platform (<https://www.prom-select.eu/proms>) to help select a validated PROM.

A PROM may be deployed in a clinical trial (Katz et al. 2022), in a real life setting or as part of other surveys or registries (Skinner et al. 2018), and as for other areas of research rigour is needed in analysis of the data it generates. The analysis (and required sample size) must be pre-planned, and efforts must be taken to support data collection to overcome biases associated with missing data. Indeed, given the increasing importance of PROMs to both regulators and HTA bodies, it is more common for a PROM to be named as a secondary endpoint and part of a hierarchical testing process. In this function, it is mostly well validated PROMs that are used. To address both regulatory and reimbursement needs, a combination may be used of index instruments such as EQ5D, profile and construct-specific instruments that have been co-developed with patients, for example by the ICHOM organisation, and that explore disease specific outcomes of relevance to the patients.

Care must be taken in the evaluation of PROMs analyses and interpretations, as the way individuals report their own health status varies and may also depend on the elicitation method. It has been shown in some studies that men were more likely than women to report better health and that positive ratings tend to decline with age (Holtorf 2022). Furthermore, those who have lived with a condition for a long time often report similar quality of life to the age-matched population (response-shift phenomenon) (Nicod et al. 2023). In addition, authors often use the terms ‘quality of life’, ‘health status’, ‘functional status’, ‘health-related quality of life (HRQoL)’ and ‘well-being’ loosely and interchangeably without further reflection on the instrument, construct, or frame of reference. However, evidence from patient-reported outcomes still does not feature strongly in the evaluation of added benefit in an HTA in many countries. This is despite the proliferation of guidance and instruments, and the increased use of generic and disease-specific PROMs by health technology developers in clinical trials and by patient organisations. It may be that the diversity and multitude of instruments make it difficult for HTA bodies to understand each measure’s properties. Then challenges may arise in determining how to compare them to measures resulting from other instruments and outcomes. Furthermore, the use of PROMs in clinical trials comes with its own challenges such as high degrees of missing data due to low patient completion of questionnaires and hence, the risk of not demonstrating meaningful

treatment effects. As an HTA community, we need to explore these issues and work collectively to ensure PROMs produce valuable patient-based evidence that can be used effectively in the assessment of health technologies and that they are used in the HTA.

3.4.2 Patient-Reported Experience Measures (PREMs)

PREMs are another type of quantitative measure reported by patients. These are more extensively used in health systems and performance management to measure patients' experience of the process of care delivery, and so can be used as a feedback mechanism for service improvements (Kingsley and Patel 2017). As with PROMs, care must be taken in the selection of an appropriate measurement instrument, the data collection methods, cost of the exercise and valid analysis given the potential biases in those responding.

In HTA, PREMs may be useful sources of information for understanding the intersection between care pathways and patient journeys. In hospital-based HTA or the assessment of some digital health interventions, PREMs could be useful for verifying whether some of the expected patient benefits are realised.

3.4.3 Surveys

Questionnaires and surveys are commonly used by patient organisations to obtain specific information from their patient members for patient input into HTA (Chap. 4) and represent the views of a broader patient population than a single patient stakeholder representative. Although there are limitations with survey methodologies, insights gained through surveys can provide important contextual information for HTA processes. Using pre-stated categorised responses or rankings allows for easy quantitative analysis and presentation (HTAi Patient and Citizen Involvement in HTA Interest Group 2015).

3.4.4 Patient Preferences

Research into patient stated preferences arises from the field of economic evaluation as outlined in Chap. 9. It uses a rigorous, systematic approach that explores patients' views about the acceptability of trade-offs between treatment characteristics, or other attributes of health interventions. Patient preference information is particularly valuable to inform an appraisal committee about the relative importance of different aspects of health technologies. There has been increased interest in this form of research since the last edition of this book with:

- the EU Innovative Medicines Initiative (IMI) PREFER project publishing detailed guidance on the use of patient preferences over the life cycle of a medicine, including in HTA (Innovative Medicines Institute Prefer Project [2022](#)) which was pilot-tested with multiple myeloma.
- HTA professional societies such as HTAi and International Society for Pharmacoeconomics and Outcomes Research (ISPOR) running major projects on use of patient preferences in decision-making.

This expansion of interest in patient preferences in HTA is explained in Chaps. 9 and 31 and confirmed by HTA bodies in England (Bouvy et al. [2020](#)) and Germany (Chap. 19), indicating that such evidence could provide valuable insights to appraisal committees.

3.4.5 Collection and Analysis of RWD Through Wearables

An emerging class of evidence is generated by wearables as part of a structured study or clinical study. There remains a range of questions about the methodologies needed to interpret this evidence, the judgement needed to assess the validity of source data, and the challenges of rapidly changing hardware and software during data collection (Leyens et al. [2024](#)).

3.5 Generation and Use of Evidence by the Patient Community

In the years before systematic patient involvement in HTA, patient organisations were limited to ‘lobbying’ responses to a HTA recommendation. The advent of integrated patient participation in HTA has led to a partnership approach, where the patient community can bring evidence such as that discussed in this chapter, to substantiate the needs and experiences of patients within the context of HTA. While lobbying entails the attempts by individuals or interest groups to influence the decisions of government, a partnership approach implies cooperation to achieve a common objective (e.g. a comprehensive benefit-risk assessment).

The increase of patient participation in HTA has prompted some patient organisations to invest in activities to support their participation. This has not just covered increasing skills and capacities to understand how to participate in HTA processes, but has also led to major investment in research activities, sometimes called evidence-based advocacy. Research might range from creating data collection systems/measures either through registries or surveys, to contributing to methodological development in a disease area (such as developing a new PROM). This may be undertaken in a leadership role or in collaboration with clinicians, academics and health technology developers. Some examples are shown in Box 3.2.

Box 3.2 Patient Organisation Research Activities to Build Evidence for HTA

Examples of how the patient community is generating and using the evidence types explained earlier in this chapter:

- **Annual surveys** to their members—often obtain high response rates and include demographic information that allows analysis by subgroup for input to an HTA about a specific sector of the patient community
- **International disease registry** and development of a **PROM** in a rare disease—Gaucher disease (Collin-Histed et al. 2023)
- **Guidance on how patient groups can develop disease registries** (Wicks et al. 2023)
- The **Patient Reported Outcomes, Burdens and Experiences (PROBE) Project**—haemophilia (Skinner et al. 2018)
- Leading interdisciplinary research, including regulators and HTA bodies, to develop a **core outcome set** for the study of gene therapies in haemophilia (Iorio et al. 2018)
- The Patient Values Project has been a partnership between academia and a patient organisation to deliver **patient preferences** research of more than 1000 patients with different forms of colorectal cancer with the specific goal of identify trade-offs that can inform HTA (Marshall et al. 2023)
- Project Hercules has commissioned work from academia to resolve issues seen in past HTA appraisal for Duchenne Muscular Dystrophy (Chap. 6) resulting in a new **PROM**, a new state in the **economic model** and other inputs to the model including cost of illness.

The challenge is that few HTA bodies recognise the quality of research arising from such initiatives and do not yet have a mechanism for its submission as evidence, critical assessment and fair consideration alongside other evidence. As Part III shows patient participation approaches are dominated by a written template approach that assumes patient organisations will simply provide ‘input’. This may include word count limits that severely limit patient organisations’ ability to set out the rigour of any research they have commissioned or undertaken. Ethnographic observations of HTA meetings in the UK suggest that when rigorous research is undertaken by patient organisations (e.g. from long-standing national registries), it is not recognised as such by HTA assessors or appraisal committees. Instead, individual quotes gathered from patients are presented to appraisal committees in an ad hoc manner (Facey et al. 2021).

3.6 Discussion—Intertwining Evidence to Understand Value

3.6.1 *Robust, Published Research to Understand Patient Aspects (Call to Action)*

A focus on clinical trials has led to standards of evidence assessment, which imply hierarchies of evidence that privilege certain ways of knowing and attribute much higher value to evidence produced in randomised clinical trials or other quantitative research types, rather than appraising the whole spectrum of research, each with own merits and limitations.

As HTA is meant to systematically determine the value of a health technology from a range of perspectives (O'Rourke et al. 2020), we suggest this requires scientific methods grounded in curiosity, observation, interpretation and judgement (Torrance 2017). It should encourage the intertwining of a range of quantitative and qualitative research approaches for nuanced and evolving research questions, and triangulation of diverse studies to ensure key patient aspects are appropriately evaluated.

In 2019, the ISPOR HTA Council recommended that systematic reviews of qualitative and quantitative research into patient, carer and citizen issues were needed, alongside those undertaken for clinical and cost effectiveness (Kristensen et al. 2019). We wholeheartedly agree with this and go one step further to say that this is an activity that should be undertaken by the health technology developer at the outset of technology development, and this research should be published, so that other parties (including other developers, patient organisations and decision makers) can make best use of that evidence to continually build knowledge about a condition and a technology.

Qualitative and quantitative research can be seen to intertwine in the development of PROMs (Chap. 6) and patient preferences (Chap. 9). Here qualitative research is a precursor to the development of the quantitative measure or attributes table. Unfortunately, such qualitative research is not generally published and so there is duplication of effort and knowledge is lost.

All forms of research about patient aspects should be published to allow its reuse, where appropriate. This requires evaluation of the applicability of any research to the context in question and consideration of how the treatment landscape and care practices have evolved to ensure the research is still relevant.

3.6.2 *Integration of Multiple Evidence Types*

In recent years there has been a call for pharmaceutical companies to move from a siloed approach to evidence generation that considers regulatory, HTA, payer and clinical needs separately to a more integrated approach, which is also reflected in closer collaboration of regulatory and HTA bodies, for example via the

implementation of the EU HTA Regulation (Chap. 18). Much attention is being paid to generation and use of real world data (Curtis et al. 2023), but some have recognised the value of qualitative research in early product development. This includes patient preference research to provide important insights to drive technology research plans (Cook et al. 2019, Marsh et al. 2021, see also Chaps. 31 and 32).

Integrated approaches to evidence generation are particularly needed for technologies that aim to offer longer term benefit in areas of high unmet need that receive expedited regulatory approval. In these situations, the clinical evidence submitted is limited and a picture of value must be built from many different pieces of evidence. This requires not only evidence about the technology, but also about the impact and progression of a disease and challenges with current treatments. These patient aspects will not be evaluated in clinical trials but could provide insights that help to describe the relative value for the specific target patient population.

3.6.3 Enhance Capabilities of All Stakeholders to Generate and Assess Robust and Meaningful Research into Patient Aspects

Qualitative research activities provide deep insights into patients' experiences and perspectives. It must be carefully planned, executed, analysed, reported and critically assessed by experts to be considered as robust 'patient-based evidence' in the deliberative processes undertaken in HTA (Staniszewska and Werkö 2021). However, Germení and Szabo (2023) found that use of qualitative research in NICE and Canada's Drug Agency submissions was infrequent, and its reporting was often inadequate, particularly in relation to the reflections about the relationships between the participants and research and data analysis. This is important in HTA where concerns about bias and conflicts of interest arise with different stakeholders. Assumptions about the capabilities and motives of the generators of research need to be acknowledged, overcome, and addressed.

In terms of capabilities, when research comes from the patient community, there can be assumptions that the patient community do not have a rigorous understanding of the methodologies and may be biased to draw out positive experiences. And similarly, when research comes from the industry, there may be assumptions about potential bias in the research question and methodologies chosen. To move this field forward, we need an open discussion about standards of research, and the academic community needs to be a key partner in this, both to ensure that patient stakeholders have the support they need to generate robust research, and as a trusted third party when assessing the research from any stakeholder. Ensuring qualitative data are collected and analysed in a rigorous manner will maximise their usefulness in HTA (Germení and Szabo 2023). For this reason, patient organisations, and researchers—especially also researchers with experience in qualitative research—are advised to work together to generate high quality evidence that reflects the patient

perspective and can be used effectively in HTA. Likewise, quantitative research coming from registries, PROMs or preferences studies need to be co-produced, executed well and reported transparently.

In addition, HTA bodies need to build capacity and expertise to undertake and assess qualitative and novel forms of quantitative research so that it can inform their recommendations. Just as we would expect HTA bodies to employ staff trained in health economics to critically assess economic models, we question why few HTA bodies (or health technology developers) employ those with skills in qualitative research or preferences research.

3.7 Conclusion

It is essential that the rigour and value of a range of research approaches to explore patient aspects be better communicated to HTA bodies, and all stakeholders invest in its production and assessment. In many instances it is the best source for addressing what can be highly-charged political questions about impacts on patients, carers, and their families, and overcomes criticisms about the bias and lack of diversity of patient input arising from participatory approaches (Chap. 4). It also has an important role to play throughout technology development (Chaps. 31 and 32) and to provide disease context to an HTA committee that can illuminate the HTA bridge between evidence and policy. But for this, we need to make sure that research into patient aspects is generated robustly and shared widely, so that it builds a knowledge base that can be used by many parties. This would contribute to building trust in patient-based evidence so that it can be considered with equal informative weight to other forms of evidence that support the determination of value. Hence, it is imperative that all stakeholders capture, publish and share experiences of undertaking research into patient aspects in HTA. This would accelerate the cycle of learning around the types of research that best address HTA questions and what form of patient-based evidence can help resolve decision-relevant uncertainties. This would build trust and confidence in its ability to strengthen HTA recommendations and that would create real value!

Acknowledgements The authors acknowledge the ground-breaking work done by Dr. Helga Sigmund and Professor Helle Ploug Hansen over two decades ago. They developed processes for gathering robust evidence about patient aspects in Danish HTA and sought to explain the value of different research genres and the importance of a diverse understanding of knowledge. We also thank Professor Sophie Staniszevska and Dr. Sophie Werkö for their chapter in the first edition of this book, and their leadership to promote research that produces robust patient-based evidence in the HTA and clinical guideline community.

We are grateful to comments received from co-editor Ann Single. All views expressed and any errors are entirely the responsibility of the authors.

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Chapter 4

Patient Participation in HTA



Ann N. V. Single, Kate Morgan, and Karen M. Facey 

4.1 Introduction

In this book, patient involvement has been defined as encompassing research into patients' needs, preferences, experiences and perspectives, and the participation of patients in health technology assessment (HTA) (Chap. 1). This chapter sets out the rationale for patient participation in HTA, the nature of patient knowledge and describes current common practices stressing the need to define clear goals of participation. It takes stock of reported challenges, enablers and barriers, before revisiting Facey's (2017) mosaic of methods for patient participation in HTA, to update it in line with evolving practice and conceptualisation of patient participation. It concludes with a reflection on unresolved challenges in participation, especially equity and burden, before raising questions about the suitability of the current focus of participation activities in HTA.

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4.2 Defining Participation

Patient participation in HTA refers to a range of activities which enable patients to contribute their knowledge regarding their needs, preferences, experiences and perspectives before, during or after an HTA to influence individual HTAs, methods, processes, governance and policy. Spectrums of participation frequently range from a passive recipient level of participation associated with receiving information, such as the International Association of Public Participation's (IAP2) concept of 'inform' (International Association of Public Participation 2018) or Rowe and Frewer's 'communication' (2005) to control over decision making (see IAP2's 'empower'). However, in HTA, participating is usually associated with roles such as providing input (predominantly written, but also verbal) during consultations and appraisals, committee membership, and collaborating in dynamic processes with two-way communication. Participation's strength is its ability to respond to emerging issues (Staniszewska and Werkö 2017) and bring an unmediated, patient-constructed perspective. It is complementary to research into these aspects (see Chap. 3). We call this participation and research 'patient involvement' in line with work developed by the Health Technology Assessment international (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) (Facey et al. 2010; Facey 2017).

The use of the word 'patient' in this definition is a shorthand for people with direct experience of living with the condition being reviewed in the HTA or potentially eligible for the technology, as well as carers, patient and community organisations and informal support groups, such as online patient communities (see Sect. 1.4.1). However, we recognise the important and essential differences between individuals and these categories of people, so will be specific when required.

Depending on the field of study and country of origin, wording and terms used for participation vary and, in some cases, may be interchangeable, and, in others, one term may be a subset of another. For example, engagement and—increasingly frequently—patient experience data are often used to describe practices which meet our definition of participation, and some jurisdictions prefer the term consumer to patients. We acknowledge the variety of competing terms in this field and its impact on shared understanding and development. Therefore, when describing notions from published references, we may have altered terms to ensure consistency and throughout the chapter we endeavour to be specific in our definitions and descriptions.

When conceptualising the patient participation in HTA and its varied approaches, Facey (2017) chose not to draw on frameworks, such as Arnstein (1969) or IAP2 (2018), which conceptualise a hierarchical ladder of involvement with increasing levels of power in decision making. Instead, she adapted the work of Tritter and McCallum (2006) to propose a means of envisioning participation in HTA which is responsive to the local goals and settings. Tritter (2009) reflected that the ladder model assumes power is finite and that ceding power to one group diminishes power in another. His alternative view was that there are different kinds of knowledge and power in health policy, and that partnership and collaboration can result in a better outcome. The goal is not to hand over power, but to support sharing of knowledge

and expertise from a range of perspectives to inform the design of services. This knowledge sharing approach would seem a better fit for HTA which often operates with in tight legislative constraints and makes recommendations for the guidance of decision-makers. Additionally, unlike power-based ladders of involvement, the mosaic allows room for evolving practice such as the increase of co-design, co-development and co-production (see Chaps. 6, 14, 15, 17, 20, 23, 26, and 27) which expand on concepts such as partnership (Arnstein 1969) and collaboration (International Association of Public Participation 2018) with new framings of potential interaction. Here again, there is variation in the way these terms are defined. In 2018, the UK research funder outlined key principles for co-production in research which can usefully inform emerging practice in HTA:

- sharing of power—the work is jointly owned, and people work together to achieve a joint understanding
- including all perspectives and skills—make sure the research team includes all those who can make a contribution
- respecting and valuing the knowledge of all those working together on the research—everyone is of equal importance
- reciprocity—everybody benefits from working together
- building and maintaining relationships—an emphasis on relationships is key to sharing power (National Institute for Health Research 2021).

We will return to discussion of a mosaic of participation later in this chapter, but first, we will consider the rationale for patient participation in HTA, common approaches, barriers and enablers, and ongoing challenges.

4.3 The Rationale for Patient Participation in HTA

Academic arguments from ethics, philosophy of science, political science and social science have legitimised patient participation in HTA (Gauvin et al. 2011), especially highlighting drivers such as relevance due to HTA's role in taking account of the consequences of a new technology in a particular local setting and the value-laden nature of HTA. For example, the widely-adopted 2020 definition of HTA states that the “overall value <determined for a health technology in an HTA> may vary depending on the perspective taken, the stakeholders involved, and the decision context” (O'Rourke and Oortwijn 2020). This is a recognition that our perspective shapes the questions we ask, the evidence we then may need to answer these questions and the ultimate value we determine. For example, a patient is likely to ask different questions to a clinician or researcher about a new technology. The different ways the problem is constructed, for example a patient-constructed versus a scientific-constructed perspective of a technology or condition, together offer a richer picture of value. Patients are ultimately the end users or recipients of health technologies, and their opinion on what is valuable should hold weight and be an important guide for decision making and resource allocation.

Patient participation became an element of HTA in the early part of the 2000s when some HTA bodies recognised the imperative to include those affected by changes in health services, such as patients as well as healthcare professionals, in deliberations and decision making. One challenging aspect of this is that during the same period, HTAs were shortening and streamlining their processes to enable them to provide more timely recommendations. Other HTA bodies saw the value of patient participation in those jurisdictions and adapted approaches to suit their systems. Furthermore, greater scrutiny of HTA approaches led to calls for processes that enabled all stakeholders to contribute at a time of increasing awareness of the value of the knowledge, especially lived experience, of patients, carers and patient organisations. For example, the role of people living with HIV and their support organisations in accelerating innovation and improving care in the final two decades of the 1900s is well documented (Royles 2012, see Chap. 30 for further examples). In many regions, especially where participation is possible, patient communities have also become more organised. This includes increasing their knowledge and information-sharing about HTA and optimising the opportunity to engage with the process and decision-makers (including through patient evidence generation). This ‘learning by doing’ approach may have led to increased acceptance among decision-makers of the quality of patient involvement and utility of engagement throughout the HTA process.

Furthermore, as identified in Chap. 1, patient involvement in HTA is undertaken to achieve relevancy, fairness, equity, legitimacy and build capacity. See Chap. 2 for a detailed discussion of procedural justice, epistemic injustice and social justice as rationales for patient involvement in HTA.

These rationales give us the ‘why’ patient participation matters for HTA, but not the ‘so what’. What is it that patient participation is trying to achieve in HTA? Abelson et al. found in 2016 that few HTA bodies were explicit about their goals for patient participation. Instead, their focus was to describe the ‘how’, i.e. their process. Abelson et al. (2016) recommended that as with scientific objectives and methodology, the goal of patient participation should inform appropriate methods for participation. Their work theorised the four goals outlined in Box 4.1, which take account of the above rationales.

Box 4.1 Goals for Patient Participation in HTA (OHTAC 2015)

Democratic—Achieving more informed, transparent, accountable, and legitimate decisions about health technologies

Scientific—Promoting a more robust and comprehensive approach to HTA that incorporates social values and ethics, as well as patients’ problems, lived experiences, outcomes, and preferences

Instrumental—Making better-quality decisions across all stages of the HTA process

Developmental—Increasing public understanding of health technologies and HTA and strengthening the capacity of the public and patients to contribute to health technology policy issues

4.3.1 Democratic Goals and Social Participation

‘Nothing about us without us’ has been a long-standing catch cry among overlooked and disempowered communities, particularly in disability advocacy and among patient advocates. However, as a result of the rapidly increasing distrust in the institutions of government, medicine and science in communities around the globe, the rationale for democratic goals in HTA has become more evident since the beginning of the COVID-19 pandemic in 2020. Distrust, the unintentional and intentional spreading of false information (known respectively as misinformation and disinformation) and social polarisation can be viewed as significant risks to societies and their health (World Health Organization 2021). The pandemic also served to highlight and exacerbate existing social (including health) inequities within and across countries, with participation often cited as one means to increase accountability and reduce these inequities (*ibid.*).

Such was the concern about the disconnection between these institutions and communities that in 2024, the World Health Organization (WHO) adopted a resolution on social participation for universal health coverage (UHC) and well-being (World Health Organization 2024). The resolution re-affirms the 2023 UN General Assembly’s declaration which promotes participation and inclusive approaches in UHC to “systematically inform decisions” (*ibid.*) so that health policies and programmes are better able to “respond to individual and community health needs, while fostering trust in health systems” (*ibid.*). Central to the WHO definition of social participation is the concept of empowering people. As Boivin et al. (2024) highlight, the resolution gives government the responsibility to ensure this participation is adequately resourced and designed to have impact. The resolution also gives governments a political imperative to act which includes showing their progress every 2 years until 2030. Just as critically, it makes social participation core to health systems rather than ad hoc or an afterthought.

4.3.2 Scientific Goals and Patient Knowledge

How do we know that a new health technology is better value than an existing health technology? Overwhelmingly, HTA relies on data from clinical trials—preferably randomised controlled trials—to determine the relative/comparative effectiveness of a new health technology versus the current standard of care used in a health system. In some jurisdictions, economic evaluations of the long-term cost and consequences of using a health technology compared to the standard of care are also needed. For this, quantitative evidence from a wide range of sources is needed including natural history data to extrapolate longer-term effects and local epidemiological data to characterise the patient population. The evidence used to determine value in this way arises from a scientific and economic understanding of health and an economic goal to distribute resources according to a determination of value that

is common across all diseases, rather than the specific aspects of value relevant in a particular condition. Furthermore, it is limited by what can be, or has been, measured. If patients were not included when scoping the research, it is likely that some of the questions they might ask to determine the value of a health technology have not been included. Unexpected benefits or disbenefits may not be captured, and patients' perspectives about the relevancy of different aspects may not be sought when evaluating the relevance of the data. Therefore, evidence is missing or classified as not relevant. The research described in Part II can play a key role in addressing some of these deficiencies. In addition, patient participation can be essential for framing a context for the HTA of the burden of illness and current treatments in the local healthcare system, in validating or refuting assumptions, and interpreting the available evidence for the local setting.

This draws on the first HTAi value for patient involvement that identifies that patients have knowledge that is unique, and they should be supported to contribute to HTA to ensure its relevancy (HTAi 2014, see also Chap. 1). But what is the knowledge of patients? It is sometimes described as lived experience, but this may be understood as just one part of patient knowledge. Building on the health literature, Dumez and L'Esperance¹ (2024) offer a classification of patient knowledge informed by the three main sources of learning (the self, the system and their community) in six categories as outlined in Table 4.1.

Dumez and L'Esperance argue that this strengthened understanding of patient knowledge can “take us away from the issue of interests and preferences of involved parties, biases and evidence, and redefine care relationships around learning, dialogue, and knowledge exchange” (ibid. p. 183). This knowledge is insightful for understanding the consequences of using health technologies and interpreting the meaning of the scientific and economic data for the real world. For example, what does three to six months progression free survival actually mean for the patients? Patient knowledge frequently adds the following aspects to individual HTAs:

- Outcomes that matter most
- Experience and suitability of comparators
- Ease of use of a technology
- Quality of life, i.e. the daily reality of symptoms, side effects, burden of treatment
- Perspectives on trade-offs, such as willingness to experience particular side effects to gain particular benefits
- Impact and experiences of treatments
- Perspectives on location, method and timings of administration (e.g. oral vs IV)
- Unmet needs
- Local variation in service provision and pathways to diagnosis and care
- Consequences of starting and stopping a health technology
- Issues of recovery progress, e.g. ability and willingness to continue taking medication, hospitalisations, treatment supports

¹It is notable that this framing of patient knowledge arose from an experienced research partnership between a patient, Vincent Dumez, and a political scientist, Audrey L'Esperance.

Table 4.1 Classification of patient knowledge (Dumez and L’Espérance 2024)

Embodied knowledge	Sensory and grounded in bodily experience. The physical sensations (e.g. pain, energy level) and “perceptions of body and mind” (p. 179) that patients listen to make decisions about their health. It is a basis for defining life experience which may be at odds with other socially-constructed perceptions of the experience of ill health.
Monitoring knowledge	The capacity to identify with acuity physical, mental, and/or emotional signs either through formal tracking or self-awareness. It is gained “through the practice of the body” from learning to live with a condition and covers a wide range of areas from coping skills to using complex technologies.
Navigation knowledge	Learning by using the system. It comes from being lost and finding ways to access services and necessities, especially through frequent use of healthcare and services. It is not just about knowing what is available and where to find it, but also how to access it including how to present themselves and be seen as credible.
Medical knowledge	Gained from interacting with medical practitioners and broader information seeking. It empowers patients in decision making about their care, but also plays a role in enabling them to be seen as credible by healthcare professionals and make sense of their condition.
Relational knowledge	A set of explicit and situated knowledge that enables patients to know who to turn to within their community to access the care they need, to help them with disease management and support the achievement of their life goals. It requires a knowledge of roles, specialities and how they are co-ordinated.
Cultural knowledge	Includes the norms, values, symbols, constructions of reality, and worldviews that influence the experience of life and illness. It shapes what is considered as right or wrong and how to act accordingly. It shapes conceptions and preferences of health, need, care options and relationships.

- Issues of health sustainability, e.g. psycho-social quality of life, avoiding further disease, independence
- Financial consequences
- Impacts on carers and family
- Other non-health benefits and risks, such as stigma and societal participation (see for example, Berglas et al. 2016; Menon et al. 2015; Livingstone et al. 2021).

It is this patient knowledge that patient organisations can gather and provide to an HTA (see Chap. 30 for a wider description of the role of patient organisations).

4.3.3 Instrumental Goals and Participation in Shaping Processes

For patient knowledge to be acted on by HTA bodies, they first need to access it and hear it. Numerous barriers create obstacles to achieving this (see Sect. 4.5). Developing or co-designing processes for patient involvement in HTA may enable

HTA bodies and patients to better understand each other's context and needs, and in turn overcome some of these barriers. This can ensure that participation processes continue to develop and enable greater participation. This participation may inform how and when patients take part as well as what input is used, how it is used, and how its influence is reported. With increasing experience and confidence working with patients, patients have participated with HTA bodies in defining processes for patient participation in HTA (see Chaps. 14, 20, and 27), as well as HTA processes themselves in areas such as early access to technologies, assessment of specific technologies, such as medicines for rare diseases (Chap. 16) and interim reimbursement processes with mandatory real world data collection (Chap. 17). Other examples include, the Scottish Medicines Consortium (SMC) developing a Public Involvement Network (PIN) advisory group to ensure their public involvement processes are evaluated, informed and co-developed by both citizens and patients (Nabarette et al. 2023). A further example can be found in *Haute Autorité de Santé*, the HTA body in France, which involved the 85 members of the legislated federation of patient organisations, *France Assos Santé*, in the development of a new assessment process for early access to medicines and in designing how patients are involved (ibid.).

4.3.4 *Developmental Goals and Capacity Building*

Taking part in an HTA builds the process knowledge of a patient organisation that can enable them to hone future input. It may also lead to invitations to participate in other health system processes, including the organisation of the health service to deliver a new health technology. Training or provision of support (such as written information and dedicated HTA support staff) is key for patients to be able to take part effectively in HTA, for example to know how the process will work and what aspects of their knowledge will be useful. Additionally, training is required for HTA practitioners and committees to enable them to appropriately seek knowledge from patients and make sense of this knowledge so it can be integrated effectively into assessments and HTA deliberations. The role of the committee Chair is crucial to ensure fair and effective participation of patients at any meeting (Thomas and Cowl 2021).

Rather than relying on the traditional deficit model of education which focuses on teaching an audience and may make assumptions about knowledge, a strengths-based approach may be used. For instance, patient participation in capacity building programmes may help to connect training to existing knowledge and identify areas of interest. It can contribute to developing shared language and support shared learning between communities and the HTA body. This approach to capacity building may also support wider public understanding of HTA if patients take part in communication about HTA or a particular HTA, for example shaping plain language public summaries or patient organisations keeping members informed before, during and after an HTA. Part III of this book showcases a range of different patient

advisory bodies that support HTA bodies in developing their patient involvement processes and working with patient organisations to build capacity for optimal participation.

4.4 How Patient Participation Typically Occurs

The most common approaches for patient participation in individual HTAs are providing written comments on draft documents and submitting written information for specific assessments via a structured form. These structured templates (see Table 4.2) became a feature of HTA when rapid assessments were introduced in the early 2000s and have been adapted by HTA bodies globally. They are typically completed by patient organisations and provided as evidence to the HTA committee along with a summary of key points. However, in some jurisdictions (such as Australia, Taiwan, and Brazil) individual patients, carers and community members use forms to submit input to an HTA. Responses are then summarised or thematically analysed (such as in Brazil) and shared with the HTA committee. The input is likely to be highly variable, even when training or guidance is provided. This is in part because it is often not clear to patients what information will be useful, and they usually have limited information about the health technology being assessed. The SMC provides a plain language summary created by the health technology developer that summarises the evidence in their application—not including confidential cost information—for patient organisations. Adaptations of this have been trialled in England and Australia with positive feedback from stakeholders (Cook et al. 2021). In Australia, this included a simple guide to help organisations think critically about the information in the summary to identify what their knowledge could add to the evidence base. The HTAi PCIG provides templates for local adaptations for written submission forms and these plain language summaries (see <https://htai.org/patient-and-citizen-involvement/>).

Written submissions have been shown to add useful information to inform HTAs (e.g. Berglas et al. 2016), but they generally lack the dynamic, two-way communication associated with good participation. Dialogue can be enhanced if feedback is provided during their development by patient involvement HTA staff and if the submission is discussed with patient representatives who sit on the appraisal committee. However, this is not standard, and the real challenge of a written submission is that it occurs separately before appraisal deliberation has identified the key uncertainties where patient input might be most relevant. Additionally, HTA bodies often do not provide feedback on the usefulness of submissions or how they were taken account of in decision making and in relation to other inputs and evidence.

Since the first edition of this book, more attention has been paid to approaches that enable two-way communication and conversation, for example through participation in online or face to face meetings. This may take the form of membership of an HTA committee, usually with full voting rights. The role of these members is usually to bring a public or patient perspective to the discussion, rather than to

Table 4.2 HTAi patient group submission templates—Summary of key elements

Key areas	Elements
Impact of condition	• Challenging symptoms • Limitations to usual activities of daily living • Emotional and psychological issues • Impacts on family life • Financial implications • Impacts on carers • Groups of patients that have particular issues
Experiences with current health interventions	• Main health interventions currently used • Extent to which they control/reduce condition's most difficult aspects • Most important benefits of currently available interventions • Burden of taking currently available health interventions on daily life • Side effects that are distressing or difficult to tolerate • Financial implications to the patient and their family • Implications for carers • Areas the current health interventions do not address • Groups of patients that have particular issues with current interventions
Experiences with new health intervention (views from patients and carers who <i>have</i> received the technology being assessed)	• Main reasons for use of this health intervention compared with other interventions • Extent to which health intervention controls or reduces condition's most difficult aspects, e.g. symptoms, daily life, quality of life, fewer hospital visits • Limitations of the health intervention • Side effects that are difficult to tolerate and those that patients are willing to tolerate • Burden of taking the health intervention on daily life • Financial implications to patients and families • Impact of intervention on carers • Aspects of the health intervention that patients would like to change
Expectations of new health intervention (views from patients and carers who <i>have not</i> received the technology)	• Whether the studied outcomes are important to patients • Minimum level of improvement of the most important symptoms that patients would like to see • What patients would most like to see from intervention being assessed • Main reasons why health intervention being assessed may not be used • Perceived advantages and disadvantages

Adapted from HTAi (2015a, b)

represent a particular condition group. Additionally, some HTA bodies invite patient organisations and patients who have submitted input to the HTA committee meeting to answer any questions that arise and provide clarifications. Sometimes this involves providing a testimony or sharing patient stories, i.e. a personal narrative of the experience of a condition and or treatment which highlights lived experience and need. Participation can also occur in ad hoc meetings when there is a high degree of uncertainty in the application but high unmet clinical need in the community. Sometimes these meetings include the health technology developer. Other opportunities for interaction between patients and the HTA body include interacting with patient involvement units (including follow up questions and feedback on draft submissions), workshops and discussion groups. This participation can occur before, during and after HTA; including horizon scanning, early dialogues/scientific advice, topic selection, scoping, assessment, appraisal, agreeing managed entry arrangements and post market review. Most commonly it occurs at appraisal which places limitations on the influence of the patient knowledge.

In addition to individual HTAs, Gauvin et al. (2010) identified participation at the policy level, for example defining government obligations for healthcare services (see Chap. 16 for an example) and at the organisational level. At the organisational level, patients may take part in activities associated with governing the HTA body, developing processes and methods, developing patient involvement, and capacity building (Nabarette et al. 2023). In these settings, there is evidence of partnership and collaboration. This participation may be ad hoc, for example ‘expert patients’ taking part in the development of the INESSS strategic plan in Quebec and the *France Assos Santé* helping to define the process and criteria for early access to medicines in France. Other times, the relationship is systematic and ongoing such as Health Technology Wales Patient and Public Involvement Standing Group which determines the approach for patient involvement in each assessment (Chap. 27). Elsewhere, patients participate in governance on HTA body boards (Chap. 15).

4.5 Barriers and Enablers to Patient Participation in HTA

Hailey (2005) presented barriers to patient and public involvement in HTA that appeared still extant a decade later (Facey and Stafinski 2015) and continue to be relevant. These are presented in modified form in Table 4.3, updated with additional review references.

Where politics and science meet there will always be difficulties. However, despite the list of barriers being long, HTA bodies are developing processes to support patients to participate in HTA. These are captured in Table 4.4 according to the HTA domains or levels identified by Gauvin et al. (2010) with their research domain expressed as individual HTAs for clarity. Although these barriers and enablers were written from the perspective of HTA bodies, many are also relevant for patient organisations.

Table 4.3 Barriers to patient participation in HTA

Challenge	Issues for HTA bodies and patient communities
Relationships	• Time needed to develop a trusting, productive relationship • Lack of trust among patient communities towards government/political institutions (Dimitrova et al. 2022) and towards patient communities by HTA bodies • Power differences between patients and professionals (Boivin et al. 2014) • Turnover of people in HTA bodies and patient organisations
Resources	• Administrative • Financial • Support staff
Mechanism of participation	• Lack of comprehensive approach that sets goals of participation for each stage of HTA (Gauvin et al. 2015) • Often chosen by the decision-maker, who shapes it in a specific manner and so has control over the participation (Boivin et al. 2014)
Identifying a 'patient position'	• Recognising that there are differing values, expectations, environment, culture, genetics, experiences of patients in the health system, but that it is not possible to canvass all
Nature and extent of patient representation	• Difficulty defining which patients should be involved • Questions about representativeness • HTA body concerns about conflicts of interest due to potential influence of health technology developers • Difficulty reaching marginalised populations
Technical demands	• Lack of patient community knowledge/power/credentials/skills in scientific process and healthcare policy options • Difficulty for patient representatives in accessing materials or virtual meetings through unfamiliar IT systems or in formats that are not accessible • Inability for patient representatives to attend a meeting due to illness or declining health condition • Limited HTA body knowledge and proficiency in participation processes and insufficient authority to embed good practice, including inclusive content and tone at meetings
Training and education	• Lack of training and/or support developed specifically for patients with recognition of turnover among 'patient communities of interest' and patient organisation staffing. • Lack of education about patient knowledge, the use of patient insights and evidence of patients' needs, preferences, experiences and perspectives in decision making • Lack of training about optimal moderation to bring patient input into the deliberative process (Boivin et al. 2014)
Language	• Lack of access to support and information beyond jurisdictions' official language • Lack of understanding of HTA language among patient communities, and patient language among HTA professionals
Time demands and remuneration	• Time commitments • Competing priorities (especially for patient organisations) • Working to tight timetables • May increase time needed for appraisal • Payments that should be made to patients

(continued)

Table 4.3 (continued)

Critiquing and integrating	• Lack of concordance between issues that patients regard as important and those in which research has been conducted • Concern about methodology to balance qualitative and quantitative evidence and the role of costs, including questions about credibility of patient-based evidence • Devaluing of research into broader patient aspects in evidence hierarchies (Gauvin et al. 2011)
Use of patient input	• Unsure what to do with knowledge of patient representatives • Concern of tokenism • Concerns by researchers/clinicians that scientific debate gets softened by inclusion of patients' perspectives • Patient organisations concern about lack of impact and lack of feedback/transparency about how evidence from different sources is handled, weighed and valued and that others have more influence. • Managing conflicts of interest—real and perceived (Facey and Hansen 2011)
Lack of awareness of HTA processes	• The implications of HTA processes are not understood • Lack of awareness of HTA, its use, or how to participate
Few evaluations of patient involvement	• Limited good quality research to show that patient involvement makes a difference • Evidence that patient involvement improves quality of assessments (Gauvin et al. 2015)
Burden, benefit or risk	• Poor consideration of the impact on patients and patient organisations of involvement, including poor management of expectations. Benefit (for example, capacity building, learning, system change) should outweigh risk (for example, physical, emotional, spiritual, economic harm; Vanstone et al. 2019)

Adapted from Hailey (2005), Facey (2017), Single and Facey (2021) with additions of issues from patient community perspective from authors' experience and references for other additional content included in table

The scope and type of patient participation varies according to the power and authority of HTA (within or outside government, advisory recommendations, binding decisions on reimbursement, etc.) and the policies of the organisation that hosts the HTA body (Gauvin et al. 2011; Kreis and Schmidt 2013). However, several serious criticisms of patient participation continue to be largely unaddressed: power imbalances, the inclusion of marginalised populations, burden of providing input and transparency of impact.

When patient communities advocate for a patient voice in HTA, they are typically responding to a perception of exclusion and experience of disempowerment. The concept of voice is related to “agency, representation, power and authority of ‘having a voice’, and can be concerned with ‘claiming one’s voice’” (Keane as cited in Lawy 2017, p. 195). Voice is about being heard and recognised (Lawy 2017). It depends on the listener’s skills, such as openness (rather than making assumptions). Language is an important concept in voice. Those who have been traditionally excluded bring their knowledge using different language (ibid.). To have the credibility and legitimacy that Boivin et al. (2014) identified as important to influence

Table 4.4 Enablers of patient participation in HTA (Gauvin et al. 2010; OHTAC 2015; Boivin et al. 2014; Kreis and Schmidt 2013; Facey and Hansen 2011; Cleemput et al. 2019; Nabarette et al. 2023)

Context	Enabler	Description
Policy	Cultural and political context	- Legislate requirement for patient knowledge in health system design and delivery - Adopt national/regional policies that encourage and enable patient participation in health service design and delivery - Foster a collaborative and reflective culture to work with patients to improve governance processes and capacity building
	Critical mass of HTA bodies	Normalise patient participation in HTA with processes that can be shared and adapted
	Leadership	- Develop or champion HTA and political leaders who encourage or mandate HTA bodies to involve patients - Ensure HTA bodies are reviewed by their funders about the effectiveness of their approaches for patient participation
	Transparency	- Publicly and transparently report patient participation in HTA using metrics agreed between HTA body and patient community - Make HTA and decision-making processes transparent
Organisational (processes, governance, capacity building)	Rationale articulated	Agree and share principles, goals and objectives for patient participation at organisational level as well as roles and responsibilities
	A range of approaches	- Identify which of the following to involve and when based on goal and role: individual patients, carers, patient representatives, patient organisations, or umbrella organisations - Enable flexible and supportive approaches to take account of health and wellness, and/or competing priorities and demands in patient community
	Recruitment and support	- Document process for recruitment and selection strategies - Provide onboarding and/or mentoring/buddying - Provide a terms of reference for each committee/group and role descriptions - Provide a clear point of contact - Pay attention to relational aspects to ensure patients feel respected and legitimated in their expertise - Pay patient representatives for preparation and meeting attendance - Offer support, including recognition of mental burden for patients and their representatives
	Evaluation	- Review participation approaches against intended goals and objectives - Monitor satisfaction of all involved and provide a process for addressing concerns - Strengthen processes with feedback from all stakeholders - Feedback to all stakeholders on what was useful and why, what will or has changed

(continued)

Table 4.4 (continued)

Context	Enabler	Description
Individual HTAs	Careful recruitment and support	• Document process for recruitment and selection strategies for patient representatives • Provide onboarding and/or mentoring/buddying for patient representatives • Provide a terms of reference for each committee/group and role descriptions • Provide a clear point of contact for patient representatives • Pay attention to relational aspects to ensure patients feel respected and legitimated in their expertise • Pay patient representatives for preparation and meeting attendance • Offer support, including recognition of mental burden for patients and their representatives • Include organisations, groups and/or individuals that can best contribute the patient community knowledge relevant to the health technology • Offer training for patient representatives including opportunities for peer-to-peer learning • Offer HTA committee members training in patient expertise, the value patient perspectives bring and how to involve them effectively
	Sufficient time	• Provide patient communities with advance notice of upcoming HTAs that might be of relevance • Provide sufficient notification in the HTA process to allow patients and patient organisations to prepare their input in collaboration with others
	Appropriate materials	• Convey technical information to patient representatives and check to ensure understanding and/or refinements required for effective communication. • Convey process and logistics information to patient representatives and check to ensure understanding and/or refinements required for effective communication. • Offer administrative support (printing papers, etc) to patient representatives
	Inclusive meetings	• Offer patient representatives preparatory meetings to discuss areas where patient contributions are of most valuable • Take account of patients' and carers' needs regarding timing and location of meeting, including accessibility adjustments. • Train or appoint chairs to ensure HTA committee chairs are highly skilled in moderation to enable everyone to be heard in interdisciplinary discussions • Inform all HTA committee members of the role and expertise of each committee member • Dedicate space and time for input by patient representatives and sensemaking dialogue
	Clear reporting	• Report how patients were involved in the HTA and the difference they made

collective decisions, patients may translate their language to suit the language of medicine, science and HTA (Dumez and L'Espérance 2024). In the process, important nuances can be lost or omitted. This has implications for the most underserved patient communities whose language (and perspectives) may differ most from the HTA bodies and committees. The complexity of this communication benefits from conversations that support sensemaking and shared learning.

Developed from Weick's 1995 work on organisational responses to unexpected events and complex information (Weick 2006), sensemaking can be expressed simply as forming a plausible understanding in order to act. The multiple properties identified by Weick as at work in sensemaking hint at the challenges for patients and HTA professionals, i.e. sensemaking is "(i) grounded in identity construction; (ii) retrospective; (iii) enactive of sensible environments; (iv) social; (v) ongoing; (vi) driven by plausibility rather than accuracy; and (vii) focused on and by extracted cues" (Thurlow and Mills 2015, p. 246). So sensemaking depends on 'responsive interactions' with empathetic listening, paying attention, questioning each other, suspending assumptions, and expecting and dealing with misunderstanding (Jordan et al. 2009). It benefits from time to appropriately pace the conversation and grow trust (ibid.). This type of communication is likely to support the development of shared expectations, visions and understanding of how patient participation is understood throughout an HTA body that are the hallmarks of meaningful participation in HTA (Abelson 2018). However, it is difficult to envisage it within the rapid timelines and intensity of individual HTAs. But patient participation in HTA is not limited to individual HTAs. It can occur before or after an HTA and at the organisational and policy levels. It can be part of a longer-term relationship or less time constrained ad hoc activities (Nabarette et al. 2023). For a patient organisation or community with an ongoing interest in multiple technologies, this dialogue might develop within training workshops and process reviews in addition to interactions for specific HTAs. For those with high unmet need and infrequent technological innovation, participation before HTA could take the form of inclusion in horizon scanning, early dialogue or pre-submission meetings. Optimising opportunities for participation in HTA body activities outside the intensity of an individual assessment may be valuable for avoiding tokenism and growing the relationships patient communities often seek.

There are few examples of programmes or activities within HTA bodies which particularly seek to support the participation of marginalised or underserved communities. In addition, and despite increasing efforts of the patient community, difficulties also arise in generating data on and reflecting underserved patient populations in HTA patient submissions. Reasons for this include (not exhaustive) capacity, lack of definition of 'underserved' in different disease areas and difficulties reaching or engaging specific populations.

One example within an HTA body is the development of criteria to trigger engagement of local First Nations people in HTA in British Columbia (pre-conference workshop recorded presentation by Robert Bacigalupo and Selva Bayat, *Indigenous Perspectives, Knowledge and Ways of Knowing in Health Technology Assessment* HTAi Annual Meeting Adelaide, Australia, June 25, 2023). Another is

the establishment of a Patient and Community Advisory Committee at Canada's Drug Agency to help the HTA body understanding the impacts of its policies and activities (Berglas et al. 2021). When forming the committee, the Agency acknowledged the concept of intersectionality which recognises that different aspects of a person's identity can expose them to marginalisation and discrimination. So, the initial 12 members brought together lived experiences of "chronic illness, progressive illness, mental illness, trauma, traveling long distances for treatment, and caregiving to an ill child, parent, or spouse" (ibid. p. 1). Careful recruitment meant the 12 members could provide 100 perspectives (ibid.). ICER, in the United States of America (USA), has also taken the approach of forming a group, a patient council, to improve diversity in its work (Sect. 26.4). An individual HTA example is described in Sect. 26.3.1. See also ACE's approach to finding formal and informal patient organisations in Singapore (Sect. 23.2.1).

Shimmin et al. argue that patient participation

approaches that do not consider the simultaneous interactions between different social categories (e.g. race, ethnicity, indigeneity, gender, class, sexuality, geography, age, ability, immigration status, religion) that make up social identity, as well as the impact of systems and processes of oppression and domination (e.g. racism, colonialism, classism, sexism, ableism, homophobia) exclude the involvement of individuals who often carry the greatest burden of illness (2017, p. 1).

They propose reconceptualising participation through a health equity and social justice lens by incorporating a trauma-informed intersectional analysis. Ui Haq et al. (2023) provide a useful step by step guide to equity-centred engagement, which brings together a wealth of resources that may be a valuable starting point. Key steps to increased equity in patient participation include setting explicit goals and undertaking essential skills training, such as trauma informed practice.

Trauma can be a feature of living with ill health and experiencing its treatment. When patients' lived experiences are collected for HTA input (or any other involvement), or patients are encouraged to share their stories, we are potentially asking people to recall events that they may usually avoid dwelling on in order to cope, such as some of their most distressing experiences. This may result in patients using days when they have better than usual health, to focus on and share experiences from some of their worst days (sometimes known as giving up your best days to re-live your worst). This is rarely done within a framework of maximising safety and support.

The mental and physical burden of participation continues to lack serious consideration. Participation is resource intensive for patient organisations, often consuming their scarce resources without an understanding of the opportunity cost. While there has been some discussion regarding payment for patient time and input, a lack of transparency and evaluation of patient participation continues to hamper informed decisions about where and what participation is the best return on investment. It also may contribute to 'HTA fatigue' in some patient communities. Furthermore, the mental burden of participation is rarely discussed in the literature. Birrell and Godfrey's small study suggests this aspect of burden may be "grossly underestimated" (2023, p. 5) by patient organisations, HTA bodies and other stakeholders. It

points to the enormous sense of responsibility of those taking part and its impact on their health and wellbeing. The study describes a workload which includes caring for their members and community throughout the process, navigating the complexities of the HTA body, giving up other valued activities and wearing the financial consequences. The study begs an urgent response, including wider investigation.

4.6 Developing the Mosaic for Patient Participation in HTA

In addition to the enablers presented in Table 4.4, there are overarching issues to consider such as who to engage, when and how. As discussed previously, this is complex and cannot be addressed by a simple ladder based on a power hierarchy or a one size fits all approach given that HTA varies according to goals and settings, including political and social expectations and structures. In turn, the patient sector can be highly variable both globally and across condition groups. For example, some HTA participation focuses on patient organisations, while other bodies—out of necessity or preference—value the inclusion of online communities and individuals. In some jurisdictions there are opportunities for participation not only in HTA but in the research and development of technologies, horizon scanning and in early dialogue prior to phase 3 trials. But in other jurisdictions technologies are typically not able to be accessed until a year or years after the primary markets of the USA and European Union (EU), so these opportunities are rare or not available. Similarly, some HTA bodies focus on rapid assessments of technologies driven by applications from technology developers, while others focus on technologies of highest need. A multi-dimensional framework is needed that can be drawn on by any HTA body, whatever their context or current processes.

As an alternative to Arnstein's participation ladder, Tritter and McCallum (2006) suggested a two-dimensional 'mosaic' of participation to allow mapping and monitoring of patient participation, based on methods of participation, category of health service user and outcomes. The mosaic enables the totality of participation in HTA in a jurisdiction to be considered so that attention is drawn to how and where goals can meaningfully be met, rather than focusing on an abstract and untested notion of the most sought-after level of participation. Gauvin et al. (2010) used this concept to develop a framework for patient/public participation in HTA outlining eight levels of involvement for six 'publics' at each stage of HTA.

In 2017, Facey modified Gauvin's framework so that rather than depicting general levels of involvement, goals and specific approaches for patient participation can be suggested. This Table 4.5, has been further developed in consultation with the authors in Part III of both the first and second edition of this book, to take account of the four goals articulated by Abelson et al. (2016) and practices described in Part III.

The mosaic in Table 4.5 is not a statement of best practice or call for universalisation of particular approaches of participation. However, it can be a tool to support good practice. For example, an HTA body would be expected to use it for reference

Table 4.5 Mosaic of patient participation in HTA

When (activity/stage) <i>Examples and cross references to case studies in Part III</i>	Why (goals/values)	Who (to involve)	How (approaches to participation)
Policy			
Legislation for coverage policies <i>In Czechia, legislation for orphan medicinal products was developed and drafted with extensive input from patient representatives after an HTA training programme (Chap. 16).</i>	Democratic	• Relevant umbrella groups • HTA-experienced patient organisations • Individual patient or carer community leaders on this issue	• Working group membership • Public consultation (written input or meeting)
Legislation for patient participation in HTA <i>In Germany, legislation has supported comprehensive patient participation in HTA since 2004. This includes the right to request a decision, the right to consultation, including the right to be present when a decision is made, and the right to submit written statements or take part in hearings, and submit petitions, but not to vote. In 2024 legislation extended patient participation to the country's innovation fund (Chap. 19)</i> <i>See also Chap. 18 (EU)</i>	Instrumental	• Relevant umbrella groups • HTA-experienced patient organisations • Individual patient or carer community leaders on this issue	• Working group membership • Public consultation (written input or meeting)
Culture <i>In Belgium the HTA body carried out a study to describe possible models for involving the general public and patients in the healthcare decision-making process and assess their acceptability for stakeholders, including researchers and decision-makers (Chap. 15).</i> <i>See also Chap. 14 (Australia), Sect. 24.3.1 (Spain)</i>	Instrumental	• HTA staff • HTA committee members • Research and external evaluators • Relevant umbrella groups • HTA-experienced patient organisations • Individual patient or carer community leaders on this issue	• Assess acceptability and expectations of patient involvement through: – surveys – workshops – consultation on draft position statements – discussion groups – input into research design • Establishing collaborative networks

(continued)

Table 4.5 (continued)

When (activity/stage) <i>Examples and cross references to case studies in Part III</i>	Why (goals/values)	Who (to involve)	How (approaches to participation)
Organisational			
Governing the HTA body <i>In Belgium, representatives from two umbrella organisations are full voting members of the HTA body board (Chap. 15).</i>	Democratic	• Individuals with strong networks and experiences in patient communities, not representing a specific condition • Umbrella organisation representatives	• Public recruitment, selection criteria and appointment with full voting rights
Development of HTA processes <i>In France, 85 peak patient organisations (members of the legislated federation of patient organisations) took part in defining a new assessment process for early access to medicines and in designing how patients are involved (Sect. 4.3.3)</i>	Instrumental	• Umbrella organisations • Peak associations • HTA-experienced patient organisations • Representatives of communities most likely to be impacted	• Consultation on assessment methods • Working groups for co-development of new processes
Development of patient involvement processes <i>In Australia, a patient representative chair led a working group of patients and representatives from the HTA body and technology developers to oversee a co-design to enhance patient involvement in individual HTAs (Chap. 14). See also see Sect. 15.3.4 (Belgium) Sect. 17.3 (England) Chap. 20 (LAC), Sect. 23.3 (Singapore), Chap. 26 (USA), Chap. 27 (Wales)</i>	Instrumental	• Patient organisations • Standing committees or working groups including patients and their organisations	• Workshops and use of feedback from patient organisations to develop patient participation methods • Formally evaluate and research patient participation methods • Co-create or co-design guidance or guidebook for patient involvement • Publish a policy for patient participation that indicates patients' rights, opportunities, and process for participation • Evaluation of processes

Proposal of HTA topics <i>In the Philippines, any stakeholder has been able to nominate a topic since HTA began in 2019. In 2023, the civil society organisations co-designed a new topic proposal form with the HTA body to make it simpler, shorter and easier to complete with questions relevant to the patient and carer experience (Chap. 21).</i> <i>See also Sect. 19.3.2.1 (Germany), Chap. 20 (LAC), Chap. 22 (Republic of Korea), Chap. 23 (Singapore), Chap. 27 (Wales)</i>	Instrumental	Patient organisations Individual patients where no patient organisation exists	• Submission of potential topics online or via paper form (with support from HTA researchers to complete the form depending on content required) • Provide full support to draft topic submission for patient organisations and patients • Patient representatives in committees for topic selection • Opportunities to add information to topic submissions
	Instrumental	Individual patients and carers Informal support groups	• Group discussion (focus group, Delphi etc) with HTA researchers to identify potential topics
	Democratic	Interested members of the public Patient organisations Individual patients	• Public consultation on proposed topics and policy questions • Submission of potential topics online or via paper form
	Democratic	Patient organisation member	• Patient representative on selection committee for a themed call of topics

(continued)

Table 4.5 (continued)

When (activity/stage) <i>Examples and cross references to case studies in Part III</i>	Why (goals/values)	Who (to involve)	How (approaches to participation)
Building capacity for patients to contribute <i>In Singapore, the HTA body began the patient participation process with stakeholder mapping to identify all local patient groups and determine their capacity to meaningfully participate. A Consumer Panel was established to foster collaborative working relationships between patient groups and the HTA body and overcome local barriers to patient participation. Topic proposal and patient input processes as well as education resources are co-designed with patient groups (see Chap. 23). See also Chap. 14 (Australia), Sect. 16.5.1 (Czechia), Chap. 17 (England), Sect. 18.4.1 (EU), Sect. 19.3.3 (Germany), Chap. 20 (LAC), Chap. 22 (Republic of Korea), Sects. 24.2.3 and 24.3.3 (Spain), Chap. 26 (USA), Chap. 27 (Wales)</i>	Developmental	Patient organisations Individual patients and carers	• Co-design training programmes and events with HTA-experienced patient organisations via a standing committee or short life working group • Observation or participation in HTA committee meetings • Dedicate HTA staff to work on patient involvement and provide a named individual to contact for support • Ensure two-way feedback mechanisms in processes • Deliver training courses led by HTA staff • Contribute to training courses developed by patient groups • Support networking meetings of patient representatives who are participating in HTA or may do so in future • Promote trusted online training resources • Hold meetings in public • Support attendance at HTA conferences with travel grant • Payment for travel, loss of earnings, preparation of submission • Organise a buddying or mentoring system among patient representatives • Include patient representatives in conference organisation committees • Offer grants for projects related to HTA

Individual HTAs			
Scientific advice on study design <i>In EU Joint Scientific Consultations patients can provide input during the preparation of the draft JSC outcome document and exchange views with the HTA subgroup and technology developer in a meeting (Chap. 18).</i> <i>See also Chap. 17 (England)</i> HTA scoping/Protocol development <i>In Wales, a Patient and Public Involvement Standing Committee identifies a systematic, but bespoke, approach to patient involvement for each HTA based on the best mechanism for involvement in each specific case (Chap. 27)</i> <i>See also Sect. 14.2 (Australia), Sect. 17.2.2.2 (England), Chap. 18 (EU), Sect. 19.3 (Germany), Chap. 20 (LAC), Chap. 21 (Philippines), Chap. 24 (Spain), Chap. 26 (USA)</i> Primary or secondary research <i>In Spain, RedETS provided training for methodologists in significant and effective patient involvement methods to support the use of social and patient-related aspects in HTAs, including through literature reviews (Chap. 24).</i> <i>See also Sect. 15.3.2 (Belgium), Sect. 18.4 (EU), Chap. 20 (LAC), Chap. 28 (Malaysia and China), Chap. 27 (Wales)</i>	Scientific	Individual patients	• Patients considered equal experts in scientific advice meeting • Meeting led by HTA researcher to elicit issues to feed into scientific advice meeting
	Scientific	Patient organisations Individual patients Underserved patients	• Interviews, focus groups, workshops to identify keys issues to hone research questions and identify priority patient populations (e.g. with high unmet need)
	Scientific	Patient organisations	• Stakeholder consultation on draft scope/protocol and PICO framework
	Instrumental	Standing committee including experienced patient representatives	• Select appropriate approach for patient involvement from suite of approaches
	Scientific	Patient organisations	• Input to prioritising, commissioning, designing, conducting and reporting of research as per public research guidance INVOLVE (2024)

(continued)

Table 4.5 (continued)

When (activity/stage) <i>Examples and cross references to case studies in Part III</i>	Why (goals/values)	Who (to involve)	How (approaches to participation)
Patient input in advance <i>In Taiwan, an online platform was created for patient input when patient participation began in 2015. Since then, the platform has been re-developed with feedback from patients to make it easier to use and two patient representatives share the input at the HTA committee meetings (Chap. 25).</i> <i>See also Sect. 14.2 (Australia), Chap. 16 (Czechia), Sect. 17.2.2.3 (England), Chap. 18 (EU), Sect. 19.3 (Germany), Chap. 20 (Brazil), Chap. 21 (Philippines), Chap. 22 (Republic of Korea), Chap. 23 (Singapore) Chap. 26 (USA), Chap. 27 (Wales)</i>	Scientific	Patient organisations Individual patients Underserved patients	• Via clear submission template with supporting guidance and assistance from HTA staff • Surveys • Phone calls with individual patients • Interviews or discussion groups with opportunity to review report
Participation in a meeting <i>In Brazil, patients or patient organisations can be selected to provide testimonials at HTA plenary sessions. Pre-registration with the HTA body is mandatory for each HTA topic that interests patients. One presenter is chosen (with one alternate). Prerequisites for providing testimony include virtual training sessions to learn about HTA and the HTA body's processes. (Chap. 20)</i> <i>See also Sect. 14.2 (Australia), Sect. 17.2.3, (England), Sects. 19.3.2.2 and 19.3.2.3 (Germany), Chap. 20 (LAC), Chap. 21 (Philippines), Chap. 26 (USA)</i>	Scientific	Patient organisations Individual patients Underserved patients	• 5-min oral • Attendance at committee meetings to answer questions and provide clarification • Attendance at a pre-meeting, pre-appraisal or main appraisal meeting to describe experiences, answer questions, provide clarification, comment on discussions • Comment on formal record of the meeting to ensure patient input adequately captured

Consultation on draft report <i>In the USA, the patient community can provide feedback on draft iterations of the HTA research scope, model analysis plan, evidence report, policy recommendations, and patient-facing report summary. Feedback can be provided through virtual meetings, written input, or early review of the draft report prior to publication. The HTA body provides a formal response and rationale for the inclusion or exclusion of feedback received and publishes responses for all stakeholders to review alongside the revised report (Chap. 26)</i> <i>See also Box 17.3 (England) for example of addressing evidence gap at consultation, also Chap. 21 (Philippines), Chap. 24 (Spain)</i>	Scientific	Patient organisations	- Stakeholder review of draft reports to ensure all relevant patient issues have been included - Comment on clarity of draft recommendations
	Developmental	Patient organisations	Comment on clarity of draft recommendations
	Democratic	Underserved patients Patients Patient organisations	- Meetings to discuss draft findings - Response and rationale for inclusion/exclusion
	Scientific	Individual patients	Expert testimony to appraisal committee Dialogue in stakeholder meetings
Multi-stakeholder review/appraisal of evidence/development of recommendations <i>In Argentina, patient representatives collect information on behalf of patients for assessments and working groups and can also bring in patients with expertise relevant to a specific technology (Chap. 20)</i> <i>See Box 17.2 (England) for a 'lay' members reflections on enhancing the role.</i> <i>See also Sect. 14.3.2 (Australia), Sect. 17.2.3 (England), and Sect.19.3 (Germany), Chap. 20 (LAC), Chap. 21 (Philippines), Chap. 25 (Taiwan), Chap. 27 (Wales)</i>	Scientific	Patient organisations Patient/public/lay committee members	- Comments on issues that might have been misunderstood and discusses value of treatment to patients via participation at HTA committee meetings - Summarises patient issues, including patient input
	Developmental Democratic	Patient organisations Patient/public/lay committee members	Separate section of HTA report summarises patient aspects
	Developmental Democratic	Patient/public/lay committee members	Voting and non-voting member of appraisal or recommendation/decision-making committee
			(continued)

Table 4.5 (continued)

When (activity/stage) <i>Examples and cross references to case studies in Part III</i>	Why (goals/values)	Who (to involve)	How (approaches to participation)
Appeal <i>In England, patient organisations can appeal/request resolution. If there is an appeal meeting, patient organisation appellants are invited to attend (Chap. 17).</i>	Democratic	Patient organisations	• As a stakeholder according to defined appeal process, or to the courts
Communication <i>In the Republic of Korea, the HTA body actively engages patients in monitoring health topics on social media to identify information requiring verification and potential HTA topics. Working with patients, HTA findings are translated into plain language summaries which the patients help to disseminate on social media (Chap. 22). See also Sect. 17.2.5 (England), Chap. 21 (Philippines), Chap. 23 (Singapore), Chap. 26 (USA)</i>	Democratic Developmental	Patient organisations Patient/public/lay committee members	• Feedback on input, including how it was considered alongside other sources of evidence and expertise • Input to development of patient friendly summaries of HTA, including how patient input was taken account of (Patient Focused Medicines Development n.d.) • Input into dissemination processes • Co-design of plain language qualitative values framework to make values used in decision making more explicit
	Democratic	Patient organisations Individual patients	• Participation in media conference and preparation of media statements about HTA decision

Managed entry to health service <i>In England, when treatments are funded by the Innovative Medicines Fund, a managed access oversight group—including patient organisations—meets periodically for the duration of the agreement to check the quality and quantity of the data being collected and to discuss any issues that arise as the treatment is used (Chap. 17).</i>	Scientific	Patient organisations	• Act as ‘safe harbour’ to provide governance on data collection systems for managed entry agreements and inform reasonable protocol

Adapted from Facey (2017) with examples from this book
Chap. Chapter, EU European Union, LAC Latin American and Caribbean, PICO Population, Intervention, Comparator, Outcomes, Sect. Section of chapter, USA United States of America
Note that information contained in columns 2–4 captures a broader range of practice than the specific example in column one

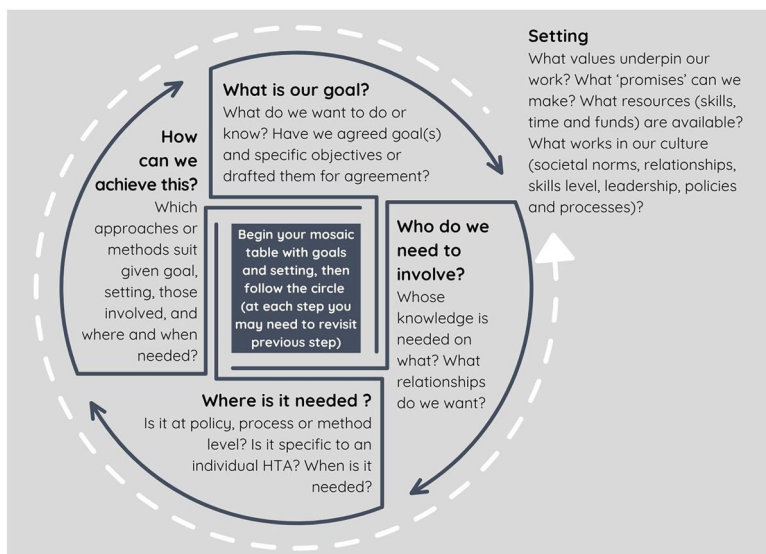


Fig. 4.1 How to use create your own mosaic of participation

co-producing its patient involvement framework—identifying overarching goals and specific objectives for participation at each step of the HTA process as well as at the organisational level, who to involve and how. Figure 4.1 set out how to operationalise the mosaic approach and Chap. 19 shows how the mosaic can be used by an HTA body.

4.7 Discussion

The literature and practice of patient participation in HTA has expanded rapidly in the early part of the twenty-first century. In HTA bodies where the practice has been established longest, there is evidence of its spread beyond individual HTAs to the wider activities of HTA bodies such as capacity building, process development, review, governance and legislative change. This suggests an increased understanding of the value of patient knowledge, trust in patient input and confidence in working constructively with patients.

However, there are some serious issues for the day-to-day business of participation in individual HTAs which often fail to demonstrate transparently the impact of participation, struggle to prioritise equity in engagement and possibly lack cognisance of the real burden and potential harm of practices. The rationale for using patient knowledge and perspectives in HTA is strong. But reasonable questions arise. Is the short, intense and stressful context of an individual HTA the best time for the collection of this knowledge? Or should HTA focus on ensuring this knowledge is captured earlier in the technology life cycle or at other opportunities such as when a promising treatment is in the pipeline for an area of high unmet clinical need? One of the reasons the

editors of this book in 2017 encouraged the distinction between input resulting from patient participation and evidence from research into patients' needs, preferences, experiences and perspectives was a concern that HTA bodies did not discern important differences in the two when seeking to use them in HTA. There was a concern that the dominance of written submissions risked this form of input being used as a proxy for appropriate evidence development. The lack of investment in research in the years since the first edition have likely contributed to an undue burden on patient organisations who may desperately employ their resources and membership to reveal a patient-constructed perception of value in a timeline that is better suited to interpreting a richer evidence base to identify its meaning for local patients.

While patient organisations still struggle to see if their input has had an impact in individual HTAs, participation in developing HTA processes, especially through co-design or co-production may be associated with greater satisfaction. In considering the current landscape with an increased attention to patient participation in technology research and development, and improved knowledge about participation approaches, we can think more critically about goals for participation (and patient involvement) and where in the life cycle of HTA and patient community landscapes they would best be addressed. This might include greater attention to democratic goals following the COVID-19 pandemic's lesson that approving a technology for use will not enable a society to enjoy the health benefits if those who provide the advice are not trusted. Social participation in health decision making plays an essential role in bridging policy-maker and community perspectives as well as educating and empowering patient communities (World Health Organization 2021). It might include a greater focus on equity with attention to those who do not take part. It might also honour the decades of patient knowledge contributed via written submissions by making these available as an enduring source of data. Finally, it could gain a better understanding of the burden of participation on health and wellbeing and implement plans to minimise this.

Participation's dynamic nature which can support shared learning inevitably continuously challenges us to review our practices and improve them. In the increasingly resource-constrained environments of even the largest HTA bodies and patient organisations the potential is to hone practice to the most appropriate and impactful areas.

4.8 Conclusion

Patient participation in HTA inevitably creates tensions due to entrenched assumptions about patient knowledge and the ongoing challenges of integrating patient-constructed understandings of health and value with those constructed in medical science and economics. However, uptake has steadily grown with increasingly well-articulated rationales, acceptance of the quality and utility of patient input and a large presence in the literature.

At its best, participation is two-way dialogue that may transform the opinions of both the decision-makers and patients, but its effectiveness depends on the fairness of the mechanism of dialogue and its competence and efficiency in achieving its intended

purpose. There is evidence that the one-way input of written submissions has influenced individual HTAs, however this impact is possibly increased by dialogue with dedicated HTA staff, and the shared understandings and expectations developed over a range of interactions within individual HTAs as well as potentially organisational and policy level participation. Participation requires a serious intent to maximise the relevant contribution (knowledge/views) from the maximum number of sources and depends on the selection of those involved, form of information elicitation and information sources. Then that input must be processed to ensure minimal information loss when combined with other information. Critically, to support capacity building, trust and ongoing participation, HTA bodies must be transparent about participation's impact and how this information was taken account of alongside other sources of evidence.

Incorporating Abelson et al.'s four goals for patient involvement into Facey's (2017) adaptation of Tritter and McCallum's mosaic of participation, along with the most recent examples of practice from Part III of this book, we see a wealth of opportunities for participation in HTA. With the mosaic conceptualisation, there is also the opportunity for HTA bodies and patient communities to consider which goals are most important and which approaches will best achieve them.

Ongoing issues of inclusivity, power imbalances, burden and transparency of impact, along with the use of participation to fill large gaps in the evidence, pose challenges and opportunities which should be central to the development of increasingly relevant and proportionate HTA.

Acknowledgements The authors would like to acknowledge the very helpful comments received from Anke-Peggy Holtorf as well as Part III lead authors for reviewing Table 4.5. We are especially grateful to the many patients and patient organisation representatives along with those leading patient involvement in HTA bodies who have shared their experiences, knowledge and expertise to inform our understanding. All views expressed and any errors are entirely the responsibility of the authors.

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Chapter 5

Discussion: Patients' Role, Representation and Epistemic Justice in HTA



Mark Sheehan

5.1 Introduction

The chapters in this Part are a testament to the progress in thinking and practice that has taken place in patient involvement in health technology assessment (HTA) over the past 20 or so years. In particular, they point to the vast steps from small-scale participation in research and evidence gathering to the situation now, in so many cases, where patients and patient organisations are active partners in the processes of understanding their conditions and potential treatments. Of course, this progress has not been limited to the evaluation of treatments, there have been similar steps in the ways in which patients are involved in the development and shaping of research and research programmes (Chaps. 30 and 31).

Arguably, these shifts and this progress can be connected in significant ways to broader social and global movements towards participatory, local and decentralised governance. Whatever their origin or their influences, in both research and HTA, these developments aim for overall better and more just healthcare provision (Rawlins 2014; Abelson et al. 2016). These participatory movements across society are now, in many cases, very well established and have a level of maturity that enables self-reflection and deeper consideration of the details and process that are in place, as well as their rationale (Sandman et al. 2017; Wale et al. 2017). HTA is no exception here. The legacy of the first edition of this book stands as a testament to precisely this maturity.

In what follows, I aim to build on the conceptual developments tracked in the four preceding chapters, to reflect back with critical eye, aiming at refinement and precision. I will consider and develop four key concepts from the previous chapters

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with a view to drawing distinctions within them that can further extend the rationale and practice of patient involvement in HTA.

The four parts which follow below examine, respectively, HTA and decision-making, the functional difference between patient and public involvement, the concept of representation as it features in HTA and the issue of epistemic justice in HTA. I suggest in the first section that it is important to attend to the distinction between the HTA process and the actual, ‘final’ decision to fund or provide a particular treatment. This distinction matters for who is involved in each part of the process and allows for a focus on the ways in which patients are involved in the assessment and appraisal rather than the decision. In the third section, I consider ‘representation’ in this context and point to two senses of representation—very broadly ‘descriptive’ and ‘political’—which is key to being clear about the role that patient contributors play and are expected to play in HTA processes. Finally, I pick up the discussion of epistemic injustice and, by reflecting on its contrary, epistemic justice, link it to questions about the contextually appropriate ways in which patient testimony, in particular, is managed.

5.2 HTA and Decision Making, Patients and the Public

To begin, Chap. 1 cites the internationally recognised definition of HTA:

HTA is a multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. The purpose is to inform decision making in order to promote an equitable, efficient, and high-quality health system. (O’Rourke et al. 2020, p. 824)

The key feature I explore briefly here is that between the first and the second sentences in this definition: between the process and the decision making. On this model, the HTA process is designed to prepare the ground for or to inform, in various ways, a decision about the ‘content’ of the health system. In this sense, the HTA process provides the evidence (broadly understood) for the decision. The distinction between the preparatory work and the decision itself is important for how we think about the relevant ethical issues and about who is involved in each part. This distinction provides an important nuance which can helpfully inform how we think about patient involvement in HTA: in particular, that patients and carers should, emphasising the work of Chap. 4, be central to proper evidence gathering processes but should not be directly part of the decision-making step. The latter step follows from considerations of justice and fairness in the decision step, to ensure that equal consideration is given to all patients and all treatments.

5.2.1 Decision Making and the HTA Process

Despite the clear conceptual separation between the process and the decision making described above, HTA processes can be variously related or ‘close’ to the actual decision making about the implementation of the technology. In some cases, the

HTA process itself culminates in a decision about funding or treatment provision, in some cases it results in a recommendation that forms part of a decision-making process and in others it is an independent of the decision making.

A few examples are useful. In the National Health Service (NHS), National Institute for Health and Care Excellence (NICE) Technology Appraisal (TA) Committee make funding decisions that determine provision in the healthcare system (Chap. 17). This is a clear example of an HTA process which results in a decision.¹ In Germany, the Institute for Quality and Efficiency in Health Care (IQWiG) goes through a comprehensive HTA and consultation process in developing its recommendations which are then considered by the Federal Joint Committee (G-BA) “in issuing coverage and payment directives” (Chap. 19). Importantly, the decision rests with the G-BA after the HTA process conducted by the IQWiG. Finally, in the US, the recommendations of the Institute for Clinical and Economic Review (ICER) have independent standing and are clearly designed to be used as credible evidence in a distinct decision-making process (Chap. 26).

In each of these cases, the distinction between the decision making and the HTA processes is clear, with the NICE TAs being a good example where the HTA process culminates in a decision.² The G-BA, like NICE TA Committees, is responsible for decisions which affect the budget and provision, and which make a difference to the whole population. These decisions essentially involve trade-offs between different parts of an overall budget: if one area is funded more significantly, another area or areas will not be.

This difference in decision making matters ethically because how we justify each decision, what counts as a fair or right decision and what counts as the best and most comprehensive HTA process are different. In particular, this matters for how we understand the contribution of patients to the HTA process that leads to the decision and to the decision itself.

A familiar kind of analogy is useful here. Suppose that we imagine a courtroom where a particular (safe and effective) treatment is, as it were, the defendant. The idea is that the ultimate question to be settled about the treatment is concerning its provision within the healthcare system. (It is thus not about the patients who would receive the treatment or the companies who provide the treatment—both are likely to have interests in this question, and both will have important contributions to make in various ways to the process of answering it.) Evidence and other relevant considerations must be brought into the court so that the judge and the jury can decide whether the treatment should be made available in the healthcare system. The right and fair judgement will be one which makes the treatment available when it is a good use of healthcare resources and does not allow access when it is not. On this

¹In Australia (Chap. 14), the Pharmaceutical Benefits Advisory Committee (PBAC 2025) makes recommendations for listing new medicines as a part of the Pharmaceutical Benefits Scheme but crucially, “no new medicine can be listed unless the committee makes a positive recommendation.” <https://www.pbs.gov.au/info/industry/listing/participants/pbac>.

²In the case of the PBAC, the decision is a recommendation which is then accepted (or not) by the Minister (and in some cases Cabinet), who has the final say. This makes the process not quite as immediately connected to the final decision but still an essential part of it.

analogy, the evidence both for and against providing the treatment should be presented objectively or presented with full disclosure of perspective. Evidence to demonstrate the added benefit of a new health technology should include how well the current technology works, what is wanted from a new technology (important outcomes), benefits and disbenefits of the new technology and how confident we are in that knowledge (strength of evidence). In some jurisdictions, cost and an assessment of the amount of benefit for that cost is also required. It should include an account of the needs of patients with the condition treated, including the nature and success (or otherwise) of treatment at meeting the needs of patients and enabling them to live better lives. It is important to notice here how the discussion in Chap. 4 illuminates and develops the complexities of this provision and the full range of knowledge and understanding involved. All of these things will allow the decision maker (the judge and jury) to make the relevant trade-offs between this treatment and the needs of the rest of the healthcare system.

In many kinds of HTA and decision-making processes, the inherent adversarial nature of the court of law will not be present quite so obviously—but it will be there to some extent. There are various ways to manage the adversarial nature of the trade-offs between the specific patient population and the overall population. The NICE model has been largely to build it into the process with the manufacturer making the case for the treatment and the External Assessment Group providing an (often critical) assessment of the presented evidence case (National Institute for Health and Care Excellence 2022). Of course, there is a clear sense in which the overall societal endeavour is a shared one which aims at the best care for all and so is one that we all should understand and appreciate.

In many respects, this is an uncomfortable analogy. Unlike in the courtroom case, there is no guilt in the healthcare resource allocation case—this is why, on this analogy, the ‘defendant’ is the treatment, and the judgement is between population level considerations about that treatment and the treatment of specific conditions using this technology. But these trade-offs occur whether they are explicitly decided or not.

This discussion distinguishes the HTA process from allocation decision making. There are a range of ways in which HTA bodies might be connected to decision making both formally and in practice but there are good ethical and justice related reasons why care should be taken in managing that connection.

5.2.2 Patients and the Public

What is most important about the courtroom analogy is the clear distinction between the decision-makers (the judge and jury) and those who are providing the evidence. In the analogy, patients and those who represent them are and should be on the evidence giving side, rather than on the judging side. In terms of evidence provision, a system which purported to give an account of the treatment which failed to fully and properly incorporate patient and carer evidence would be significantly deficient.

There is a good case for the stronger claim that this evidence should be driven by patients and patient focussed considerations. This case is well made in Chaps. 3 and 4 where the knowledge, understanding and experience that can be provided through the involvement of patients, carers and patient organisations is delineated.

McCoy et al. (2019) distinguish between the role that patients and the public, respectively can and should play in the context of involvement:

We use the term patient involvement to refer to the practice of involving individuals in health research or policy on the basis of their experience of a particular disease or condition, including its effects on daily life and its management. ... We use the term public involvement to refer to the practice of involving individuals in health policy or research based on their status as members of a relevant population — be they residents of a city, individuals served by a health system, or a national population. (p. 710)

This distinction draws on the particular characteristics that each broad category possesses and the corresponding roles that develop from those characteristics. It is important that these distinctions are not per individual: any person can be a member of the public or, with the relevant experience, fulfil the role of patient.

If we distinguish in this way, between the functional, contributing roles that are played here respectively by patients and the public, it is also clear that it is the public and not patients who are properly part of the jury. Justice, here understood as fair process, requires independence and lack of bias with a clear expectation that consideration will be given to the system as a whole (Daniels and Sabin 1997; Sheehan and Hope 2012). So, it is fitting that independent, public representatives are included in decision-making committees like NICE TAs and the G-BA and very often patient representatives for the particular conditions are invited to present or witness much of the discussion. Public members on HTA committees often have backgrounds in patient and community organisations and/or lived experience as patients and carers. This perspective may be valued in their roles. But the interest should—and typically is—managed as per other declared interests in HTA committees to ensure these members can act as independent representatives for the public in decision making rather than representatives for the health condition they are associated with. Patients with the condition in question, on the other hand, have a clear stake and so should not be a part of the 'jury'.

This distinction is one that can be lost or muddled and so is worth some emphasis and practical focus to ensure its widespread implementation.

5.3 Representation

Representation is a complex concept with multiple different senses and nuances. In the context of HTA processes, these senses become very important in understanding who is involved and in what capacity. These senses arise in a number of different ways which follow on nicely from the distinction drawn above between patients and the public. As I will develop here, when and how patients are representative is very different from the kind of representation we require of members of the public, when

they are involved in decision-making. These differences matter for what is asked and what is expected of those involved in each case.

Perhaps the most basic distinction relevant for the HTA context is that between ‘being representative of’ and ‘being a representative for’ (Pitkin 1967). ‘Being representative of’ is what we might call being descriptively representative or indicative: having certain characteristics that are shared with others. So, someone who is tall is in this sense representative of those who are tall and someone who has a particular health condition is again in this sense representative of others with that particular health condition. What matters for this sense of representation is a kind of descriptive similarity which picks out common features, experiences or characteristics. The individual that possesses the common feature can be taken to be representative of the others with that feature. When we conduct research into ‘patient aspects’ (Chap. 3), we are gathering descriptive information of all kinds about similarities and differences so that the whole picture can be represented and understood—this is descriptive representation.

‘Being a representative for’ a group is a very different sense and one that is partly captured by the idea of being a substantive political representative of a group. In this sense, a representative is someone who speaks for or is taken to speak for a particular group. Perhaps the paradigm case here is an elected member of parliament or a patient organisation spokesperson. Importantly this kind of representative can be elected, officially appointed or more simply be informally taken to have this role (Salkin 2024).

As is hopefully apparent, this distinction is particularly important in the HTA context. ‘Being representative of’ is a descriptive matter whereas ‘being a representative for’ is political and is importantly connected to authority and voice (Martin 2008; Salkin 2024). In one context, say, in speaking of the experiences of living with a particular condition it is important that the individual is descriptively representative, whereas in the role of advocate, it looks more important to be well placed to speak for the group of patients with the condition.

As we saw above there are two reasons that justify the involvement of patients in HTA processes: (i) the instrumental or evidentiary contribution that they bring to the process and (ii) the intrinsic importance of being included and having a say in things that matter. Also, as we saw above, the second of these reasons applies, if slightly differently, in the case of allocation decision making but there is a strong reason to involve the public, often as independent, in these decisions. In each case, and indeed, within cases there is good reason to attend to the kinds of representation that is important.

In the case of HTA, we require a process that does a good job of capturing all of the relevant evidence surrounding the treatment in question. Given this, it is straightforwardly clear that patients have a good deal to contribute but also, that different kinds of representativeness might matter. The HTA process is more complete if it includes a thorough, descriptive account of the nature of the condition, the needs of patients and the impact of the technology. Both qualitative and quantitative data will be important for this completeness and for the structuring and organisation of models, outcome measures and predictive tools (Williams et al. 2019; Rand et al. 2019;

Staniszewska and Werkö 2021; Germini and Szabo 2023). These strands of evidence gathering and shaping in the HTA process are comprehensively discussed in Chaps. 3 and 4. In this respect, descriptive patient representation will be more valuable if it contributes a range of different qualitative experiences of the condition and its treatment: in depth qualitative data will capture both similarities and differences linked by the idea of saturation. In quantitative terms, representativeness comes through the numbers and the model of the patient population: mean and outlying outcomes, for example, are ways of representing the population. In both of these cases, the involvement of patient representatives in the evidence gathering process (over and above participation) and its interpretation will lend authority to the data: in this sense patient representatives are speaking for the broader population as (informal) political representatives.

There is a good deal more to say about these distinctions between senses of representation and their role in HTA process and decision making. These refinements are important for clarifying roles and managing expectations both of those who would be representatives and those who would understand how and when patient or public representatives speak for others (or themselves).

5.4 Epistemic Justice and Injustice

In this last section of the discussion, I endeavour to expand on the idea of epistemic injustice which is applied to this context in Chap. 2. At its core, epistemic injustice involves a fairness claim which means that patients should be included in the development of evidence relating to the treatment (as those affected) or should be otherwise be privy to the decision-making process.

As noted in Chap. 2, broadly, epistemic injustice occurs when (i) an individual's knowledge (including their understanding and experience) is not given the credibility that it deserves (testimonial injustice) or (ii) an individual does not have the tools (including the concepts, language, or an appropriate forum) to express what they know (understand, experience) (hermeneutic injustice) (Fricker 2007). Importantly, the injustice occurs when the individual's knowledge is not treated as it should be. So, to be able to say that there is an injustice, we need an account of how the knowledge ought to be treated. And to have an account of how the knowledge ought to be treated, we first need to be clear about the kinds of knowledge that patients have and how this knowledge fits and ought to fit within the HTA process. The kinds of knowledge outlined in Chap. 4 alongside the methodologies for gathering and integrating this knowledge described in Chap. 3 and in the context of clinical and economic evidence provide the raw materials for exactly this kind of normative epistemic framework. Such a framework would yield clear standards for when testimonial injustice occurs—when an individual's knowledge or understanding is not treated according to the framework.

There is a third idea in the mix here: the idea of treating people respectfully, where this is a basic ethical requirement that one person owes to another. Importantly

this is about how they are treated as a person and is distinct from how credible their claims to knowledge are. Someone can be treated respectfully but their knowledge, their testimony, given no (and inappropriate) credibility—or importantly, they can be treated disrespectfully but their knowledge given full and proper (epistemic) credibility. The former is a case of testimonial epistemic injustice, but the latter is not. The latter, of course, is ethically problematic.

A useful example in this context concerns the role of individual, anecdotal patient experience in the formation of generalised claims about effectiveness. A patient's experience of their condition and its treatment leads them to see improvements in particular symptoms. However, there is significant evidence that the patient population generally, as assessed through reliable measures, does not see these symptom improvements. To disregard or downplay the individual patient's experience of symptom improvement in the context of making generalised claims about the treatment's effects is not a case of epistemic injustice. To treat the individual patient disrespectfully in flippantly or easily disregarding their experience is also, on the face of it, not a matter of epistemic injustice, it is a violation of common courtesy or respect for persons.³

There are similar kinds of examples in the comparison of individual patient 'success stories' in the face of significant clinical evidence to the contrary, particularly in the context of conditions with high levels of heterogeneity of symptoms. Disregarding or downplaying testimonials of the effectiveness of a treatment in a context where there is evidence of high heterogeneity and variability of response across the population does constitute epistemic injustice in the context of population level effectiveness. But this downplaying can in no way undermine the fact that for that individual the treatment may have had such a significant effect.

This last point shows how important it is for these matters of knowledge to be properly contextualised. It is easy for wires to be crossed here: science and medicine is inexact and variable, which is difficult to grasp and accept. It can be hard for the trialist or the economic modeller to accept an outlying case when the population data is clearly saying the opposite. Similarly, it can be hard for the patient or carer, who witnesses the incredible improvements in quality of life, to accept that their experience is an outlier.

Even if we agree that individual level data has a limited role in population level judgements, the case of experiential knowledge seems clearly to be valuable for HTA for all the reasons that qualitative evidence matters—to give us a clear sense of depth and meaning, to help shape our sense of value and need. These reasons give us a clear steer on how experiential knowledge ought to be treated, so the denial of experiences in the context of the value that they add, or the denial of their value are certainly instances of epistemic injustice.

³A non-HTA example: If I believe what a person says when the next bus will arrive but make rude remarks about their appearance and odour, I am being disrespectful of the person but epistemically 'just' (I am accepting their testimony because they are local). But if I am not rude and treat the person well (with respect) but I don't believe them about the bus timetable (because they are clearly a homeless person and what would they know), then I am being epistemically unjust but respectful of the person.

The focus so far in this part has been on testimonial justice and injustice. Recall that hermeneutical injustice occurs when individuals are not provided with an appropriate forum in which to express their knowledge. In the context of HTA, the framing of this discussion and those in this Part generally are precisely about dealing with the nature and importance of this provision. Notice that the framework suggested above goes some way towards protecting against hermeneutic injustice by providing exactly the tools by which patients and patient organisations have space for the expression of their knowledge. By involving patients in a thoughtful and appropriate way, in a way that is both meaningful to patients and relevant to the HTA process, we ensure that hermeneutical injustices are guarded against.

5.5 Concluding Remarks

As is evident from the preceding chapters in this Part, there has been significant progress in thinking about the important theoretical and conceptual aspects of patient and public involvement in HTA and health care resource allocation.

In this discussion, I have picked out a number of key concepts from the previous chapters that arise in this context and are worthy of further detailed discussion. Further refinement of these four concepts in practice in this context, I suggest will further enhance our understanding of the nature and justification of patient and public involvement in HTA. The distinction between a process which informs a decision and the decision-making process itself is an important one and one that can become very involved. However, as the analogy with the courtroom suggests, justice in decision-making does importantly rest on it being clear. This distinction has implications for involvement. Patients are essential for the development of evidence and its provision, so their role in HTA processes is hard to understate. At the same time, patient involvement in the decision-making process is deeply problematic for the justice of that process but the public do have an important role to play in this regard. In all of this, both representation and the distinction between epistemic justice and injustice are key ideas. We need to further reflect on how and who patients and the public represent in their roles in HTA and allocation. It is also clear that attention should be paid to how the evidence that patients present is treated and given appropriate space in the corpus of evidence assembled.

These reflections are intended to be a beginning and to gesture to ways in which practice can be refined but very much build on and out of the themes of the four chapters in this Part.

Acknowledgements I am very grateful for the patience and detailed, thoughtful comments from the editors. I am also grateful for the support of the Oxford NIHR Biomedical Research Centre. This work has been very much influenced by my experience on HTA committees in the UK, and my time on the Advisory Group for National Specialised Services and NICE's Highly Specialised Technology Appraisal Committee.

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Part II

Methodologies

Chapter 6

Patient-Led Collaboration for HTA Tools and Evidence Development: Project HERCULES



Emily Reuben, Josie Godfrey, Fleur Chandler, Juliet Warner, Jill Carlton, and Philip Powell

6.1 Introduction

This chapter describes the role of patients (in particular carers who are experts in patient advocacy) as leaders in Project HERCULES,¹ an international patient-led collaboration that has developed tools and evidence to support health technology assessment (HTA) for new treatments for Duchenne muscular dystrophy (DMD). This collaboration was initiated and is led by the patient community and has brought together patients and other experts (including clinicians, technology developer representatives, academics and HTA experts). The chapter explores when a patient-led collaboration for HTA may be appropriate and how the governance structure of Project HERCULES put patients at the heart of even the most technical work streams of the project. We look at the challenges in establishing Project HERCULES and the role of patients in the work on developing a validated measure for health-related quality of life (HRQoL), and in building a natural history model and disease-level economic model.

¹HEALTH Research Collaboration United in Leading Evidence Synthesis; <https://www.duchenneuk.org/project-hercules/>.

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6.2 The Case for Patient-Led Collaborations

HTA requires several disease-level inputs including a natural history model, HRQoL and cost of illness and various inputs to build economic models. In rare diseases there may be few clinical experts, little published evidence, and a limited pool of patients to provide this evidence. Building a robust evidence base can be expensive and time consuming, often prohibitively so for smaller biotech companies. Project HERCULES was established by Duchenne UK in response to the need for disease level tools and evidence to support HTA in DMD. The aim was to establish a patient-led collaboration to build a natural history model, economic model, HRQoL metric and build evidence on the burden and cost of DMD.

Project HERCULES is managed by Duchenne UK through a core team including those with lived experience of Duchenne as well as experience in HTA in rare diseases. Individual work streams are, where necessary, delivered by external vendors. An international multistakeholder Steering Group agrees the overarching work programme and members of this group advise on the work. Work is funded by Duchenne UK and pharmaceutical company members of the Project HERCULES Steering Group.

6.3 The Project HERCULES Approach

DMD is a serious genetic disease which is life-threatening and shortens the patient's life substantially. DMD is an X-linked disorder caused by mutations in the dystrophin gene and it is the most frequent muscular dystrophy in boys, affecting 1 in 3500 live births (Katiirji et al. 2013; Mendell et al. 2013) and 1 in 50 million girls. DMD is usually diagnosed before the age of six. The disease causes progressive and unyielding muscle weakness frequently identified in the early toddler years when the child begins to miss development motor milestones (Henricson et al. 2013; Connolly et al. 2013). Loss of ambulation occurs generally around the age of 12. Only a few DMD patients survive beyond the third decade; most die because of respiratory complications or heart failure due to cardiomyopathy (Ricotti et al. 2019; van Ruiten et al. 2017; Bushby et al. 2010; Birnkrant et al. 2018).

Some HTA bodies struggled to reach decisions about the first licensed product for DMD, for example it was two and a half years after licence that the National Institute for Health and Care Excellence (NICE) guidance on the use of Ataluren in DMD was issued (National Institute for Health and Care Excellence 2016). The high cost of drug development, poorly documented natural history of the disease, lack of disease specific and age-appropriate endpoints that truly represent disease burden and the challenges of recruiting patients are major factors in hindering development, approval and reimbursement of new drugs for rare diseases (Nicod et al. 2019).

Duchenne UK was a stakeholder in a NICE appraisal of a treatment for DMD and witnessed a lengthy process which, in part, was due to gaps in the disease level evidence base. Duchenne UK recognised that determinations of value were based on limited evidence and models of cost effectiveness that did not fully characterise the experience of patients and carers with DMD (National Institute for Health and Care Excellence 2016). Duchenne UK proposed that for a rare disease with a limited patient population, non-competitive collaboration across all interested developers with relevant experts (patients, clinicians, academics, HTA) would be efficient and reduce the demand on patients and families who might otherwise be asked to participate in multiple, similar surveys or focus groups (Hatswell and Chandler 2017). Duchenne UK invited pharmaceutical companies with a DMD product in development to a training day in February 2017 to explore modelling and HTA in DMD. This led to a proposal from the patient organisation to establish Project HERCULES to drive development of a single disease-level economic model (rather than different companies developing models for their own products) and a core suite of associated tools to support the development of patient-focused evidence to input to HTA value determinations, including:

- A natural history model
- A bespoke HRQoL preference-based measure
- Burden of illness data.

Companies were invited to sponsor the work and join the Project HERCULES Steering Group together with international patient organisations, clinicians, academics and HTA experts. Full details of current Steering Group membership can be found at www.duchenneuk.org/project-hercules/project-hercules-team/.

All work was on the disease level rather than product specific, reducing the risk of conflict of interest or compliance issues for any of the stakeholders.

Project HERCULES is led by Duchenne UK with a core team of three, consisting of: the joint Chief Executive Officer of Duchenne UK (a parent of a son with DMD); a senior technology developer health economist (also a parent of a son with DMD); and an expert in HTA for rare diseases (previously an Associate Director at NICE, the UK's reimbursement decision maker).

This patient leadership set the priorities so that the outputs reflected individual and family experiences of people living with DMD, and enabled access to data sources and expertise which may be inaccessible for individual researchers.

Members of the Steering Group were proposed by the patient organisation and once established additional members were proposed by these Steering Group members. In rare diseases there are limited experts, and they are usually well known to others in the field, making establishing the Steering Group a relatively simple process.

Insights from patient organisations and people with lived experience of DMD were included in all Steering Group meetings and these directed the evolution of the work, having a major impact on the development of the evidence and tools. The initial work programme was proposed by Duchenne UK and agreed by the Steering Group. As work progressed it became evident that the broader impacts on patients and their families need to be captured and additional pieces of work were commissioned to explore specific aspects such as the impact of using steroids or the impact of the disease on familial carers.

Project HERCULES was designed to meet the needs of international HTA processes and quality requirements in a way that remained patient focused. Depending on their areas of expertise, steering group members contributed to the work streams, which were co-led with academics and researchers (Fig. 6.1). All outputs were reviewed by the Steering Group in addition to patient organisations and individual patients and parents.

Project HERCULES welcomed active participation from all stakeholders. Pharmaceutical companies sponsoring the work programme were full members of the Steering Group. As the primary end users (along with HTA bodies and payers) of the outputs of Project HERCULES, their contributions have been essential in ensuring the tools and evidence not only reflect the lived experience of patients and families, but also have a clear practical use for submissions to HTA. Technology developer involvement has been carefully managed to ensure full compliance with the ABPI Code of Practice for the Pharmaceutical Industry (ABPI 2021) and avoid any anti-competitiveness or conflicts of interest. Likewise, the HTA perspective was represented regularly by NICE and experts in HTA and health economics research.

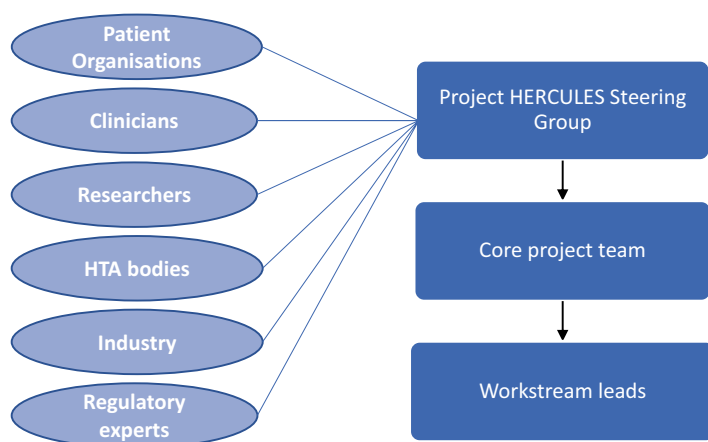


Fig. 6.1 Governance structure for project HERCULES

6.4 Targeted Methods for Patient Participation in Project HERCULES

As well as being patient led, via Duchenne UK, Project HERCULES incorporated active patient participation in all work streams. Duchenne UK recruited patients and parents to participate in the research through the use of social media and offline networks. This section outlines the different methods used to support effective participation in each work stream, which evolved as the research developed.

Economic models enable the assessment of the cost-effectiveness and impact on healthcare systems of new medicines, using clinical data, treatment costs, and health outcomes. They are typically developed by manufacturers for individual treatments to assess their cost-effectiveness and impact on healthcare systems once late stage clinical trials are underway. Economic models focus on health states that have a significant impact on clinical outcomes, HRQoL and/or healthcare resources and costs. The Project HERCULES economic model was constructed using the health states defined in the natural history model and burden of illness data. In an innovative step, the economic model was developed to be suitable for use in the assessment of any treatment for DMD, addressing cost effectiveness in multiple ways, and also to be used for treatments of specific symptoms as well as disease modifying treatments. It was validated by patients through advisory boards and Steering Group participation.

An iterative approach to developing the individual work streams meant that input from patients and informal carers could be used for multiple purposes, ensuring effective use of patients' and families' time.

6.4.1 *Natural History Model*

DMD has long been described along four health states: early ambulatory, late ambulatory, early non-ambulatory, and late non-ambulatory. These stages of progression are well-established in clinical guidelines on the diagnosis and management of DMD (Bushby et al. 2010; Birnkrant et al. 2018).

When Project HERCULES set out to build a natural history model for DMD, the patient leadership embedded lived experience in the project design and every stage of the model development, strengthening the input of patients and families to ensure that health states represented their lived experience and reflected a significant change in the clinical and/or financial burden of the disease.

The natural history model was built from a broad collection of clinical data in DMD in collaboration with the Duchenne Regulatory Science Consortium (D-RSC) (Conrado et al. 2019), part of the Critical Path Institute (2024). D-RSC is a non-profit consortium focused on regulatory science, which includes a US patient organisation, Parent Project Muscular Dystrophy and other individual patient/carers representation. Placebo data from completed clinical trials were also included. The Project HERCULES core team

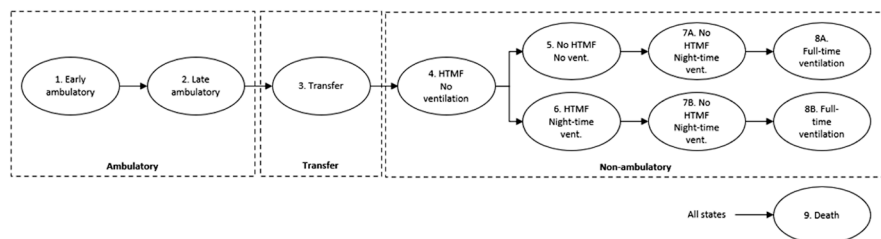


Fig. 6.2 Natural history model structure (Broomfield et al. 2024—<http://creativecommons.org/licenses/by-nc/4.0/>)

worked closely with the University of Leicester to ensure emerging findings were compared with real world experiences of patients and families through advisory board meetings and findings of the HRQoL work stream. Patient and clinical advisory groups reviewed data and provided input into definitions of health states.

Patient and family input was elicited at an initial advisory board meeting. During this meeting, it became clear that the standard distinction between late ambulatory and early non-ambulatory health states failed to adequately capture important aspects of DMD. Patients and relatives unanimously emphasised the importance of being able to weight bear and assist with transfers. They reported that the loss of this ability often triggers the need for additional support such as hoists, adapted vehicles and home adaptations, and can impact the carer (often the parent) financially, emotionally and physically. This input led to the inclusion of a transfer state between the late ambulatory and early non-ambulatory states in the natural history model, where patients can still weight bear but are no longer able to walk. This health state had previously been overlooked in data collection, natural history models and economic decision models.

Input from patients and families also identified more granular nuance in the non-ambulatory phase of DMD, including the loss of hand-to-mouth function alongside the introduction of night-time and thereafter full-time ventilation. The subsequent natural history model comprised nine main health states including death (see Fig. 6.2). Patient and family input was taken to confirm these health states for the natural history model at a second advisory board meeting (Broomfield et al. 2024).

These health states reflect states that have a significant impact on clinical outcomes, HRQoL, and/or healthcare resource use and the burden of illness. They informed the Project HERCULES HRQoL and burden of illness work streams, and the economic model (Woodcock et al. 2019).

6.4.2 Health-Related Quality of Life

Research into HRQoL was driven by both a recognised need within the Duchenne community to better measure quality of life in people with DMD and the known requirements from many HTA bodies for preference-based measures of HRQoL. The

objective was to develop a HRQoL patient-reported outcome measure (PROM) that is relevant to patients and allows estimation of utilities, an important measure for calculating quality-adjusted life years (QALYs). The QALY measure is an outcome measure that allows comparison of outcomes across multiple diseases and is the basis for cost utility analyses used by some HTA bodies. A systematic literature review, followed by application of COSMIN² methodology (Prinsen et al. 2018), demonstrated no suitable measure in DMD currently exists (Powell et al. 2020). Project HERCULES accepted the challenge to develop a new HRQoL preference-based measure in DMD. As well as being used to calculate QALYs, it was essential that the new PROM “measured what matters” to people living with Duchenne and that the patient voice was accurately represented (Powell et al. 2022). The measure was thus designed to have stronger content validity than any previous PROMs used in Duchenne. The novel measure became known as the DMD-QoL (Powell et al. 2021; Rowen et al. 2021).

Patient involvement was critical to the development of the DMD-QoL. At the outset, patient views were represented by Duchenne UK, contributing to the research aims and design of the project (i.e. to develop an outcome measure that captured what mattered to people living with DMD). The Project HERCULES core team worked closely with the University of Sheffield throughout this project, ensuring that each stage reflected lived experience, and jointly ran a patient and family advisory board to explore themes identified in patient interviews and surveys. A project-specific patient involvement group was established, which included adults with DMD and parents of children with DMD, who contributed throughout the project as research peers.

The development of the DMD-QoL consisted of three stages. In stage one, patient participation informed the design of semi structured interviews, including the areas of focus (see Supplementary File 1 of Powell and Carlton (2023) for topic guide) and appropriateness of topics for given age groups. Interviews were conducted with DMD patients and analysed to produce a draft framework for understanding HRQoL in DMD (Powell and Carlton 2023). This initial collaborative activity facilitated an active role for patient input into framing the research questions addressed during the patient interviews.

In the second stage, patients, clinicians and DMD carers assessed the face validity of the HRQoL framework, followed by a quantitative survey of a larger sample of patients, which was psychometrically analysed. At this stage, potential items for the DMD-QoL were ranked for their importance by patients and parents in an advisory group discussion using a traffic-light system facilitated by the research team (green = include in DMD-QoL, red = do not include in DMD-QoL, amber = uncertain, for discussion). Six people living with DMD and/or their carers contributed to this exercise, as well as five clinicians. The different perspectives employed in stage two informed the final item selection and highlighted differences in opinions between patients, families, and clinicians on proposed items for the questionnaire.

²Consensus-based Standards for the selection of health Measurement INstruments.

It was considered important that the instrument would be equally useful for clinical, patient related, and HTA purposes. Therefore, it was important to gain input from all perspectives and then weight the different perspectives in a way that ensured the patient perspective had a priority without diminishing the usefulness to other stakeholders. In the development of the DMD-QoL patient views were always prioritised. In the third stage, an online discrete choice experiment was administered to a general public sample to weight the different elements identified and thus generate UK utility values (Rowen et al. 2021). Throughout the project, collaboration with people with DMD helped to construct the measure so as to meet the needs of people with DMD and their families (Powell et al. 2019).

6.4.3 *Burden of Illness*

Project HERCULES commissioned work looking at the burden of illness to explore the impacts of DMD that patients, families and clinicians identified as most important to them. The core team helped identify the items of most relevance for patients and HTA bodies to inform this work stream.

A UK burden of illness study was conducted. The study set out to provide health state costs for the economic model by applying unit costs to estimates of annual resource use for patients in each health state. The Project HERCULES Steering Group, which included a patient representative and two mothers of individuals with DMD, reviewed and approved the initial study outline.

A sample of 18 neuromuscular specialists completed an electronic case report form for the next 10 eligible patients they consulted. The specialists identified and extracted direct medical resource use information from the patients' Electronic Health Medical Records for the preceding 12-month period. The patients were then invited to complete a bespoke Patient Public Involvement Engagement (PPIE) questionnaire to capture data for direct non-medical resource use and work productivity impact. The survey was designed with survey responses from clinicians, patients and families and discussions at Project HERCULES Steering Group meetings (Evans et al. 2019). Dependent on the age of the patient, the questionnaire was completed by the patient (16 years or older), the patient's primary carer (4–7 years old), or a combination of both (8–16 years old).

The study found that overall indirect costs were the main driver of total cost in people with DMD, with the lost opportunity cost of work being the main driver for indirect costs. This key finding was only possible due to the involvement of patients and their families and the use of a PPIE questionnaire.

Key issues identified through this work informed the later detailed review of routine NHS data sets (Morgan et al. 2024) and work exploring patient, family and clinician views of the burden of steroids involving online surveys to inform themes explored in a small group, two dyad interviews with patients and their parents followed by a focus group with patients, carers and healthcare professionals.

6.4.4 *Economic Model for DMD*

The Project HERCULES economic model provides a robust core economic model suitable for submission to HTA bodies that is representative of the patients' and their families' progression through DMD. The model structure adopts a Markov approach which captures patients' HRQoL, health care resource use and cost, and mortality associated with progression of disease.

While the technical details of economic models are likely to be inaccessible to those without expert training in the area, the input of patients and their families shaped the structure of the model and its core data set. The model health states reflect those in the natural history model, which were defined with the input of patients and their families. The model was developed iteratively with patient and family involvement in its review at Steering Group meetings. In addition, the core data set for the economic model was developed with patient and family involvement in the burden of illness and HRQoL work streams.

This approach resulted in a development process led by patients, family and clinicians to complement findings from the published literature and clinical trials.

6.5 Challenges for Project HERCULES

The collaborative approach taken by Project HERCULES was not without challenges (Box 6.1).

Box 6.1 Challenges of the Collaborative Approach

- Demonstrating the capability of Duchenne UK to deliver an ambitious programme of health economics research
- Establishing the governance and financial arrangements for Project HERCULES with technology developer sponsors unused to working with patient organisations as leaders in health economics research
- Embedding lived experience in every aspect of Project HERCULES
- Ensuring involvement and engagement in a rare and complex health condition, including making appropriate and necessary adjustments for meetings, patient days, etc.
- Representation and diversity (i.e. not all parts of the patient community were represented and why that is difficult to do)
- Managing patient burden—an ambitious work programme requested multiple inputs from some patients and families.

Establishing Project HERCULES required significant investment by Duchenne UK to establish a work plan and bring sponsors and Steering Group members together, initiating some work prior to signing contracts.

There was some initial scepticism from technology developers and other partners who had concerns that a patient organisation would not bring the expertise, skills and experience necessary to deliver an effective programme of health economics research. Duchenne UK sought early involvement from experts and ensured that specialists in HTA, rare diseases and health economics were included in the project leadership team. Commissioning early research from the University of Sheffield into HRQoL also helped demonstrate to stakeholders the commitment and ability to deliver high quality outputs.

The governance and financial arrangements were complicated by the requirements around technology developer funding to patient organisations. Interpretations around the rules governing this varied by company, leading to extensive negotiations with most sponsors, made more challenging by the need to ensure consistency across contracts. Ultimately all issues were resolved but the lengthy discussions led to inevitable delays in funding. Duchenne UK approached these discussions offering as much flexibility as possible to each company, within the framework of the overarching approach to Project HERCULES and the need to ensure no individual company received preferential terms.

Researchers often needed to learn to explain complex concepts in accessible language to a non-expert audience so that patients and families could contribute effectively. This included researchers learning to explain what aspects of a disease are important to HTA processes, the specific requirements as to how those aspects should be measured and some of the more technical elements of the project such as the economic model. Balancing input from patients and families with clinical, technology developer and HTA experts has also been challenging. Project HERCULES selected researchers in part by their readiness to work collaboratively with patients, families and other stakeholders by actively listening and responding to all the information received not simply seeking confirmation of what they expected to find.

Throughout Project HERCULES attention was given to minimising the burden on patients and families of participation in research. This included careful consideration of the amount of patient participation in individual work streams and the most appropriate methods for achieving this participation. Rare disease patients may find themselves burdened by repeated requests for information and a key aim of Project HERCULES was to avoid duplication and unnecessary information gathering wherever possible.

Minimising the burden on patients included ensuring patients and families were able to participate in the research at a time and location that would fit around their logistical requirements such as work, caring responsibilities or access requirements.

Ensuring a representative sample of patients and families was and remains challenging. We were aware that certain demographics are more likely to respond to surveys, requests for interviews and focus groups and despite efforts to address this,

the sample of patients was not fully representative of the whole population. More work is needed to ensure diversity of representation in health economics research, for example mothers were over represented in our engagement with families and the geographical, economic and ethnic diversity of those engaging in the programme could be improved.

Despite the challenges, Project HERCULES has consistently taken a patient-led approach that has had a clear impact on each work stream (Box 6.2).

Box 6.2 Impacts of a Patient-Led Approach

- Identifying the need for the research, scoping its work streams and creating an inclusive way of working for a wide range of stakeholders
- Enabling researchers to test assumptions against lived experience and develop their own understanding of the condition and care pathway
- Identification of meaningful disease stages including the previously overlooked state between the traditional stages of late ambulatory and early non ambulatory, reflecting patient and parent experiences of the importance of being able to weight bear
- Development of a bespoke HRQoL preference-based measure that better reflects the lived experiences of those with DMD as well as the views of clinicians and other experts
- An economic model that builds on the actual experience of patients and families and has been well received by HTA professionals
- A burden of illness study focusing on what is most important to patients and families
- The identification of future priorities that reflect what is most important to patients and families, including the impact of long-term high dose steroid use and the burden and quality of life of parents and family carers.

6.6 Discussion

Project HERCULES has demonstrated that patient communities can direct health economics research, working as peers with academics, clinicians, HTA experts and other stakeholders to ensure that the disease-level evidence used in HTA decisions reflects the lived experience of patients and families. Indeed, patient-led health economics research can improve the relevance of health economics research outputs. The evidence and tools are available to companies developing treatments for DMD, raising the quality of the evidence and its presentation to HTA, shortening time to patient access, saving the costs of duplication of effort by multiple companies, and making efficient use of a small patient pool.

Challenges in patient involvement in health economics research can be overcome and patient-led collaborations can help ensure that this is a priority for research, avoiding marginalisation of patients' contributions.

Duchenne UK embarked on an ambitious programme and made substantial financial and staff investments into this which were essential to ensuring its success, along with other key factors (Box 6.3).

Box 6.3 Key Success Factors

- Ensuring patients and families were leading the work as well as participating in the work
- Embedding subject matter expertise alongside lived experience in the project leadership
- Iterative and responsive communications with all stakeholders including regular updates for patients and families to explain the aims of Project HERCULES, progress being made, and the impact patient contributions made to this work
- Identifying and collaborating in all work streams with qualified research experts, who were willing to adapt their work processes to the required focus on patient needs and patient leadership
- A project management team that ensured work stream leads were committed to capturing patient and family perspectives, providing feedback on how the input had been used, and to working collaboratively where possible to ensure learnings from each work stream informed the other work streams.

The Project HERCULES approach could be used in other disease areas. Indeed, it has informed the development of collaborative programmes for HTA in other rare diseases, including Project Mercury for facioscapulohumeral muscular dystrophy (FSHD).

Collaboration with patients and families can completely change the perception of researchers, providing insights that allow evidence to be understood differently and directly affecting the outputs of each work stream.

Bringing stakeholders together in a non-competitive space can have many benefits for all stakeholders preparing for HTA in rare diseases (Box 6.4).

Box 6.4 Benefits for Stakeholders

- Access to a wider pool of expertise and evidence, maximising the impact of patient data and insights and ensuring that evidence reflects patients' and families' experiences
- Minimising the demands placed on patients, families, and clinicians to repeatedly share their evidence and insights to inform individual pharmaceutical company work programmes (particularly relevant in areas such as DMD which has a number of treatments in development)

(continued)

Box 6.4 (continued)

- Better quality tools and evidence to inform HTA decisions, developed independently from any single life sciences company
- Spreading financial burden across multiple organisations
- Developing disease level tools and evidence well in advance of HTA requirements, enabling them to be shared with HTA bodies and allowing a consistent approach to evaluating new treatments
- Avoiding duplication of effort across different stakeholder groups.

Patient organisations are well placed to lead these collaborations as experts in the impact of the condition(s) they represent. A patient organisation-led approach to building tools and evidence for HTA, in collaboration with qualified researchers and other stakeholders, can effectively ensure that the lived experience of a condition informs every aspect of health economics and outcomes research, leading to results that better reflect the impact of that condition, or a new health technology, on patients' lives. Such evidence can help in demonstrating value of new therapies in a patient relevant way.

Acknowledgements The research team would like to thank all the patients and families who contributed to this work at every stage, without whom it would not have been possible to develop evidence and tools for HTA that accurately reflect the experience of living with the impact of DMD.

Emily Reuben is co-Chief Executive Officer at Duchenne UK. Duchenne UK has received funding to support the work of Project HERCULES from Astellas Pharma Global Development Inc., BioMarin (UK) Ltd., Catabasis Pharmaceuticals Inc., Italfarmaco, Pfizer Inc., PTC Therapeutics International Ltd., F. Hoffmann-La Roche, Ltd. (Switzerland), Santhera Pharmaceuticals (Switzerland) Ltd., Sarepta Therapeutics Inc., Solid Biosciences LLC, Summit Ltd., Ultragenyx Pharmaceutical Inc., and Wave Life Sciences Inc. Fleur Chandler is an independent consultant who has been funded by Duchenne UK to support Project HERCULES, she is a member of Duchenne UK's Patient Advisory Board. Josie Godfrey is a consultant who has been funded by Duchenne UK to support Project HERCULES. Juliet Warner is a consultant who has been funded by Duchenne UK to support Project HERCULES. Jill Carlton is a Professor of Health Outcomes Research at the University of Sheffield, she has received funding from Duchenne UK for Project HERCULES. Philip Powell is Senior Research Fellow at the University of Sheffield, he has received funding from Duchenne UK for Project HERCULES.

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Chapter 7

Extracting Data on Patient Perspectives from Social Media



Alessio Luschi, Julie Polisena, and Ernesto Iadanza

7.1 Introduction

Randomised controlled trials (RCTs) are considered the gold standard for measuring the efficacy of a health technology and many current HTA practices focus on grading evidence derived from quantitative methods. These approaches undervalue a wider range of data and evidence that delve into patient aspects, which can be further explored for integration in HTA (Chap. 3).

Social media platforms (e.g. X/Twitter[®] Facebook[®], discussion forums, blogs, etc.) are used by patients, carers, and healthcare providers to share information, comments and support one another. As such, social media platforms may be considered one source of real world data (RWD) related to the experiences of patients or carers with the disease or specific health technologies (Polisena et al. 2021).

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7.2 Principles of Social Media Analysis

Social media analysis (SMA) is the process of gathering and analysing data from social media networks. It can provide the clinical community and decision-makers with novel insights on the issues associated with living with a condition, the benefits and side effects of using health technologies, as well as health service provisions for a patient in real-world settings. Standardised methods, such as natural language processing (NLP), computational linguistics, deep learning (DL), and machine learning (ML) can help automatise information retrieval and data mining of digital data (Polisena et al. 2021). Conversations about specific health technologies on social media are usually led by patients instead of clinicians and researchers. While social media is a relevant resource for identifying issues, which can be further explored with in-depth interviews or focus groups, its content could present a valuable asset in a detailed understanding of the patient's lived experience of a disease or use of health technologies (Street and Farrell 2017).

Known challenges of online forums involve the anonymity and representativeness of the social media users as the information collected may be difficult to validate and irrelevant to the patient population for which the health technology is intended. In addition, they may attract like-minded views and not be representative of the entire targeted patient population. Furthermore, SMA of patient conversations on public forums is more feasible as explicit agreements are needed to access patient-generated data in closed groups. Even though some biases may persist, SMA can address some of these challenges by applying standardised methods to review the information available on online patient forums (Street and Farrell 2017).

7.3 Stakeholder Perspectives on Use of Social Media Analysis in HTA

In September 2023, the Health Technology Assessment International's Patient and Citizen Involvement in HTA Interest Group (PCIG) published a study that engaged a multistakeholder group (i.e. from HTA producers and industry to patient advocates). It aimed to assess whether the evidence generated from SMA provides additional insights into patient experiences, preferences or needs to help inform HTAs (Holtorf et al. 2023a, b). The stakeholders agreed that SMA can be an important source of information to identify patient unmet needs and identify the potential benefits of a new health technology, which can be integrated into an HTA as well as inform various elements in the HTA process. Some examples include the suitability of clinical outcome assessments or patient-reported outcomes and the identification of trends in the patient experience dynamics (ibid.).

While the stakeholders saw potential value in SMA in revealing new or complementary information on the patient's needs, experiences, and behaviours, HTA producers stressed the importance of assessing the information obtained from the

online platforms in specific contexts to more accurately understand its potential value. Particularly, stakeholders highlighted concerns about SMA regarding legal and ethical requirements, such as privacy protection and the anticipated risks related to the data and research robustness (Holtorf et al. 2023a, b).

Due to the rapid and frequent developments in social media communications, the PCIG members conducted a scoping review of the published literature to inform the creation of guiding principles on the research tools, the data, and the ethical review process across the SMA lifecycle (i.e. from conceptualisation and scoping to reporting and follow-up). This framework is available for the researchers, patient communities, and HTA practitioners or committees to use to meet the ethical standards and the legal requirements of privacy protection needed to validate the SMA results and support HTA or healthcare policy decisions (Holtorf et al. 2023a, b). The guiding principles represent an initial step in the standardisation of SMA conduct and a baseline for future enhancements of the framework based on continued dialogues between HTA researchers or producers and ethics reviewers as well as advancements on this topic (ibid.).

While the incorporation of SMA in HTA processes is still limited, it has the potential to offer new and valuable insights into patient needs, experiences, and adherence behaviours, which normally would not be revealed in clinical trials or other data sources (Kalf et al. 2018; Wang et al. 2019; Shukla et al. 2020). These new insights can then have an important impact on HTA recommendations as well as public health policies. However, careful ethical considerations must be taken toward the further development of research methods, data interpretation, and engagement to minimise the risk of potential harm to those who post information on social media and to the people, who are the subject matter, in the posts.

7.4 Analysing Unstructured Patient-Generated Health Data Extracted from Social Media with NLP and DL

Unstructured *patient-generated health data* are spontaneously recorded or captured by patients or their carers (Wood et al. 2015). These data can be obtained from a variety of sources, including patient-led research networks, wearable smart devices, and social media platforms. Notably, platforms such as Facebook®, X/Twitter®, and patient networks have created several opportunities for patients and carers to exchange health-related information. Patients frequently use social media for knowledge advancement, social support, advice exchange, and to promote self-care and doctor-patient relationships (De Simoni et al. 2016). Consequently, this has resulted in a potentially rich, but analytically challenging, source of RWD.

In recent years, improved analytics approaches have made it easier to use these data in medical research. Techniques such as NLP paired with ML/DL now enable efficient processing of the various complexities inherent in social media data, spanning differences in terminology, duplicate posts, misspellings, and abbreviations

(Segura-Bedmar and Martínez 2015). Moreover, as opposed to manual categorisation, NLP algorithms can be developed to accurately and automatically identify features within posted content, such as adverse events, thereby enabling the analysis of large volumes of text-based posts (Liu et al. 2016). The Web-Recognizing Adverse Drug Reactions (WEB-RADR) project is part of the European Union's Innovative Health Initiative (IHI, previously Innovative Medicines Initiative) and aims to collect information on existing practices, guidance, and legal requirements for social media monitoring to identify potential safety issues related to adverse reactions in pharmacovigilance (Lengsavath et al. 2017). Applying neural networks and NLP approaches to mine structured data from the publicly available spontaneous reporting system (SRS) data sources might also increase evidence extraction, hence eliminating several logistical barriers associated with previous methodologies, and allowing for faster, real-time, and cost-effective data collection (Luschi et al. 2023).

In one example, Lee et al. demonstrated the importance of integrating multiple patient-generated data sources (i.e. social media and FDA public archival transcripts) to capture patient perspectives on specific topics. For instance, their work proposed two case-studies: opioid use disorder and pulmonary arterial hypertension. The findings demonstrated the value of social media in patient-centered research by providing unfiltered patient perspectives outside the clinical settings, capturing experiences of patients who may not participate in clinical trials, and offering real-time data on medication use, treatment barriers, and side effects (Lee et al. 2020a, b).

NLP is a subfield of artificial intelligence (AI) that helps programs to interpret and handle natural language data. This approach produces a computer program that can 'understand' language. Syntactic and semantic analysis are the primary methods for doing natural language processing tasks. The syntactic analysis examines natural language using formal grammar. Grammatical norms apply to categories and groups of words, not to individual words:

- Parsing: the process of analysing the strings of symbols in natural language following the norms of formal grammar
- Lemmatization: a text pre-processing technique to break a word down to its root meaning to identify similarities. For example, a lemmatization algorithm would reduce the word *better* to its root word, or lemme, *good*
- Stemming: a method in text processing that eliminates prefixes and suffixes from words, transforming them into their fundamental or root form. The main objective of stemming is to streamline and standardise words
- Text segmentation: the process of dividing written text into meaningful units, such as words, sentences, or topics
- Part-of-speech (POS) tagging: the process of marking up a word in a text (corpus) as corresponding to a particular part of speech based on both its definition and its context. A simplified form of this is commonly taught to school-age children, in the identification of words as nouns, verbs, adjectives, adverbs, etc.

Semantic analysis is the process of comprehending and evaluating the words, signals, tone, and structure of a sentence. This task examines the meaning or logic underlying the sentence. In real life communication, we comprehend what someone says mostly by intuition, language understanding, and tone (e.g. Named Entity Recognition, Relation Extraction, and Sentiment Analysis).

Named Entity Recognition (NER), is a crucial task in the field of NLP. It plays a fundamental role in extracting structured information from unstructured text data. NER involves identifying and classifying entities in text into predefined categories such as names of persons, organisations, locations, dates, and more. Performing NER is not free of challenges. Texts can be ambiguous, and named entities may not always follow conventional patterns. Variability in language, typos, and homonyms make the task complex. Handling multi-word entities and context-based classification is another hurdle.

Sentiment Analysis (SA) is an NLP technique that employs computational linguistics, information retrieval and data mining techniques to determine the emotional tone and positive and negative opinions in a body of free-text. In healthcare, it can be used to glean insights from both social media and clinical papers about the effectiveness of a treatment or drug (Denecke and Deng 2015), as well as for assessing the performance of maintenance in healthcare (Mascii et al. 2021). There are mainly two different approaches used for SA tasks: lexicon-based versus ML-based. Bag of Words is the most common lexicon-based approach. It builds a vocabulary from a corpus of documents and counts how many times the words appear in each document. Each word in the vocabulary becomes a feature and a document is represented by a vector with the same length of the vocabulary (a ‘bag of words’). TD-IDF (Term Frequency-Inverse Document Frequency) and word-embedding are, instead, two of the most common ML-based techniques. The former estimates the importance of a word by evaluating a score that increases proportionally to count, but it is inversely proportional to the frequency of the word in the corpus. The latter is a collective name for feature learning techniques where words from the vocabulary are mapped to vectors of real numbers. Google’s Word2Vec, Stanford’s GloVe, and Facebook’s FastText are the most famous ones. Comparing the outcomes of studies that used both the lexicon and ML approach (Jiménez-Zafra et al. 2019) with the outcomes of studies that used the first (Luo et al. 2019) or the second method (Du et al. 2017) (same topic and data sources) confirmed that the lexicon-based approach has significant drawbacks in evaluating the real sentiment in medical texts. However, despite the implemented method, the accuracy and automation of SA still remain a challenge (Polisena et al. 2021; Mascii et al. 2021).

Recent works have shown how DL can also be used to solve NLP tasks. DL models exemplifying the notion of unsupervised representation learning are the Autoencoders (AE). They became popular as an early tool to pre-train supervised deep-learning models, especially when labelled data was scarce, but still retain usefulness for entirely unsupervised tasks such as phenotype discovery (Shickel et al. 2018). AEs have been applied to biomedical fields for clinical relation extraction (Lv et al. 2016) and as outcome predictors (Miotto et al. 2016).

Transformer models, introduced in 2017 (Vaswani et al. 2017), have been developed to address NLP tasks and overcome the limitations of Recurrent Neural Networks (RNNs). Transformers make use of multi-headed self-attention to perform sequence-to-sequence learning tasks (Vaswani et al. 2017) and have become the DL model of choice for NLP problems (Wolf et al. 2020; Khan et al. 2022).

Transformers led to the development of pre-trained systems for Natural Language Understanding, such as the popular BERT (Bidirectional Encoder Representations from Transformers) (Devlin et al. 2019), and for Natural Language Generation (GPT—Generative Pre-trained Transformer) (Radford et al. 2018). They are trained with huge general-language datasets such as Wikipedia Corpus and Common Crawl and can be also fine-tuned to specific NLP tasks (Zhu et al. 2021). Biomedical and clinical NLP community researchers have been actively proposing BERT-based models to process biomedical and clinical text information effectively and efficiently. Successful biomedical and clinical BERT models include SciBERT (Beltagy et al. 2019), which is built on basic BERT to increase its performance on scientific data; BioBERT (Lee et al. 2020a, b), which is a domain-specific BERT model pre-trained on a large number of biomedical text corpora; ClinicalBERT (Huang et al. 2020), which is a language representation model to extract high-quality relationships between medical concepts from extensive clinical notes. Recent studies (Akkasi and Moens 2021) show that these specifically pre-trained models outperform classical models in extracting evidence from biomedical corpora.

The study by Timimi et al. (2019) highlighted how NLP analysis can identify real-world concerns that might otherwise go unreported in formal medical settings. The study applied NLP to online health community discussions, and authors analysed over 11 million posts from patients and carers to detect correlations between statin use and memory issues. It identified linguistic patterns, themes, and terminology used by patients focusing on memory loss, aphasia, cognitive impairment, and emotional changes.

7.5 Strengths and Limitations of the Described Approaches

Despite the ability of NLP to recognise and categorise information from unstructured data, certain conditions jeopardise the accuracy of such categorisations. Human language has evolved into something exceedingly sophisticated, diversified, and profound. Language is further complicated by spoken language(s), dialect, reference points (such as sarcasm), and numerous other factors. Computer science practitioners have made significant progress in training NLP models to identify negation (where phrases like ‘not’, ‘without’, ‘unseen’, ‘ruled out’, and others are used to prevent improper categorisation of traits in a record). For example, a record with the phrase ‘The patient experienced chest pain but has no history of heart disease’ would be appropriate for understanding the relationship between chest pain and heart disease. On the other hand, a record that states ‘The patient does not deny experiencing chest pain but has no history of heart disease’ would make an NLP

model struggle in diagnosing the patient. Computer scientists have been able to control for many negations like this by entering countless records into NLP tools, which are then reviewed and evaluated for validity. However, due to the richness and diversity of language, false positives and negatives may still arise.

While the discussed limitations must always be taken into account when applying NLP to unstructured data, NLP provides powerful insight into data that would otherwise be impossible to combine, aggregate, describe, analyse, and visualise without enormous effort, human resources, and costs. The use of NLP in a health-care and health sciences environment for the underdeveloped world is an excellent demonstration of utility. NLP is a possibility for analysing social media posts to extract patient emotions, satisfaction, and concerns about specific treatments, drugs, or healthcare services to inform policy-makers about unmet patient needs or areas where new health technologies might be necessary. NLP can aggregate and summarise these insights, which are crucial for HTA in understanding the value of a healthcare intervention from the patient's perspective. Besides, as social media offers insights from a wide demographic range that may otherwise not be captured, NLP can help structure and analyse this data to ensure that health policy decisions are more representative of diverse patient populations.

7.5.1 Ethical Issues

Recently, there has been a shift away from the widely held belief that ‘anything goes’ in SMA, toward a more sophisticated perspective that recognises both the existence and importance of the ethical and regulatory issues involved in applying NLP to social media for health research (Street and Farrell 2017).

When conducting SMA, it is crucial to maintain ethical integrity by following best practices at every stage of the research—preparation, execution, and communication. Researchers should engage in ongoing dialogue with ethics reviewers to ensure the protection of social media users, build trust, and foster continuous learning in line with the evolving media, technologies, and research methods (Holtorf et al. 2023a, b).

Research emphasises the importance of transparency in algorithm development, as well as the creation of an ethical framework to govern the appropriate use of social media for computational public health research. Most jurisdictions' current research regulation systems were established before the introduction of social media, leaving inadequate procedures to regulate computational SMA (Vayena et al. 2015). This assumption is supported by a qualitative study of members of the Research Ethics Committees in the United Kingdom. The study outlines the challenges faced by ethics committees in applying existing research ethical laws to computational activities, emphasising the importance of protecting research participants, including social media users, even when exploiting publicly available data (Hibbin et al. 2018). Eight principles have been outlined for NLP research using social media data in a recent set of practical guidelines (Benton et al. 2017). These include

the requirements that data should be identified for use in publications and presentations, that ethical approval or exemption should be obtained from an Institutional Review Board or Research Ethics Committee, and that caution must be exercised when linking data.

7.6 Conclusion

NLP provides a transformative framework for the analysis of unstructured, *patient-generated health data* from social media, offering critical insights that could provide valuable evidence about patient aspects to inform HTA. The ability of NLP to process vast volumes of text-based data, through techniques such as SA, named entity recognition, and relation extraction, enables the identification of key aspects of patient experience, needs, and outcomes that traditional data sources like RCTs or surveys often fail to capture.

Social media platforms offer a rich repository of RWD, reflecting patient experiences with diseases, treatments, and healthcare services in everyday contexts. By utilising NLP, these large and complex data sets can be efficiently analysed, providing a rapid and cost-effective means of gaining access to patient perspectives. The insights derived from this data complement more formal and structured sources, facilitating a more comprehensive understanding of the impacts of a health condition and how health technologies impact patients beyond controlled clinical settings.

Medical NLP tools are expected to become an even more important part of healthcare organisations in the future, acting as intelligent assistants for healthcare providers. As technology progresses and trends in various fields of medicine are further investigated utilising NLP techniques, the opportunities for leveraging NLP systems will only increase.

While NLP-driven social media analysis holds promise for enhancing HTA with patient insights, some limitations must be also considered. HTA bodies need to develop the skills to critically interpret evidence arising from SMA, as its variability and informal nature can pose challenges to traditional decision-making frameworks. Social media insights should be viewed as part of a broader patient involvement strategy, complementing, rather than replacing, established methods like surveys and focus groups. Additionally, the potential biases and non-representativeness of social media data require careful contextualisation to avoid skewed conclusions. For social media data to be effectively integrated into HTA, agencies must invest in training and tools to harness these insights responsibly and accurately.

Although considerable work remains to be done before NLP in healthcare reaches its full potential, its present uses have already shown enormous potential to improve patient care by analysing *patient-generated health data* obtained from social media platforms, as well as streamline healthcare processes, such as maintenance of medical equipment, adverse event identification and analysis.

Acknowledgements The authors would like to acknowledge comments received from the editors and thank Dr. Jacqueline Street and Lucie Farrell for their chapter in the first edition of this book. All views expressed and any errors are entirely the responsibility of the authors.

The authors received no specific grant from any funding agency in the public, commercial, or not-for-profit sectors.

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Chapter 8

Discussion: The Evolving Science of Patient Involvement



Nijole P. Tjader, Norah L. Crossnohere, and John F. P. Bridges

8.1 Introduction

Patient involvement in all forms of medical decision making has been growing over time. It is now well recognised that integrating patients' perspectives can improve the relevance of decision making across the entire medical product lifecycle. The emergence of this paradigm is accompanied by a shift in the range and rigor of approaches used to involve patients in diverse medical contexts. For example, the Patient-Centered Outcomes Research Institute (PCORI) has a Science of Engagement Initiative that has invested in systematically studying approaches for involving patients, carers, and broader healthcare communities to support research that is most meaningful to patients (Patient-Centered Outcomes Research Institute 2022). In regulatory science, the United States (US) Food and Drug Administrations' Patient-Focused Drug Development initiative has sought to create more opportunities for patients, carers, and advocates to express their experiences and perspectives to inform benefit-risk decisions (Perfetto et al. 2015; US Food and Drug Administration 2025). Public-private partnerships involving patient and community stakeholders have developed guidelines for inclusion of patient preference information to inform industry, regulatory groups, and health technology assessment (HTA) bodies internationally (PREFER 2024).

In this chapter, we discuss advancing methods and opportunities to involve patients in decision making in medicine, including in HTA. We explore novel approaches to maximise participation through the lens of two chapters in this

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volume: one which depicts the growing scientific and technical expertise of patient communities, and another which depicts the application of artificial intelligence (AI) to improve representation of diverse patient experiences into the evidence bases which inform HTA decision making. We also consider the implications of these chapters in the broader context of patient involvement in healthcare.

8.2 Growing Scientific and Technical Capacity of Patient Organisations

Modern patient organisations are increasingly conducting cutting-edge scientific work on even the most technical of topics. One example of this is Project HERCULES, described in detail in Chap. 6. Project HERCULES is an international, patient-organisation led collaboration to support HTA for treatments for Duchenne muscular dystrophy (DMD), a rare, genetic neuromuscular disease for which there is currently no cure. Project HERCULES was initiated by the patient organisation Duchenne UK following a prolonged two-and-a-half-year HTA process to establish National Institute for Health and Care Excellence (NICE) recommendations for the first licensed product for DMD in the UK. Several unique factors challenged NICE in their evaluation of this product, including the high cost of medicine development, poorly documented natural history of DMD itself, and a lack of disease specific and age-appropriate endpoints that represented disease burden. To build an evidence base to support future HTA decision-making on DMD products in the pipeline, Project HERCULES partnered with diverse stakeholders to generate tools, including a natural history model, a bespoke preference-based quality of life measure, burden of illness data, and a disease-level economic model.

Project HERCULES is unique among HTA initiatives in several ways. First, it provides an example of a patient organisation initiating the development of tools to support HTA. Traditionally, patient involvement in the HTA process has been incumbent upon HTA bodies to invite patient participation. HERCULES flips this script by itself initiating the activities, and then inviting regulators, researchers, clinicians, and members of industry to collaborate on the generation of tools to support HTA.

A second way in which Project HERCULES stands out is its demonstration of the technical and scientific expertise that patient organisations can today hold. Project HERCULES is an example wherein leaders from Duchenne UK were themselves technical experts in HTA and health outcomes research, as well as being parents of children with DMD. These dual perspectives have been essential in helping HERCULES achieve its mission, as the leadership have not only insiders' knowledge of how to influence these decision-making systems, but personal drive and a clear emotive imperative. Not all patient organisations will have this capacity

among their internal leadership. However, partnerships between patient organisations and academic groups can be used to support a mission to conduct rigorous scientific studies and build evidence bases that support the patient community. Examples of such patient organisation-academic partnerships are abundant in the literature and have informed a range of topics related to pre-market decision making, criteria for appropriate benefit-risk determinations, and pricing/reimbursement decisions (Lavelle et al. 2021; Tsai et al. 2021; Riley et al. 2023; Richardson et al. 2020).

Project HERCULES provides an important case study of an HTA initiative with strong patient involvement, and more effort is needed to make initiatives such as these scalable and mainstream. DMD is a space with several products already licensed and more in the pipeline. As such, there is some financial incentive by drug developers to support HTA efforts. In the absence of these financial incentives, such as for those condition areas for which products are earlier along in the pipeline, there may be need to find alternative funding mechanisms for this type of work. The need for funding and specialized personnel to support like this raises questions about the feasibility of this scope of work for other patient organizations. Additionally, it is also uncertain if this level of investment is always *needed* to ensure appropriate levels of patient participation. Other initiatives led by patient organisations and conducted in partnership with academics have demonstrated potentially more efficient approaches to assessing the appropriateness of established processes in HTA decision making. For instance, members of a US-based DMD patient organisation, Parent Project Muscular Dystrophy, partnered with academic researchers in the US to measure patient and parent preferences for emerging DMD treatments (Crossnohere et al. 2022d). As a part of this work, they sought to assess the acceptability of the generic health-related quality of life measure EuroQol-5 dimension (EQ-5D) to the DMD community. The prevailing notion at the initiation of the study was that the EQ-5D measure, which is typically used to assess health status and make comparisons across conditions, did not reflect the challenges of life that had the greatest impact on DMD patients and families. As such, the patient organisation, in partnership with an academic research team, went on to conduct a multi-national study which evaluated the acceptability of the EQ-5D. While *a priori* the intent of the study was to demonstrate that the measure was not appropriate for measuring health status in DMD, findings from a series of assessments, including those exploring content validity, face validity, and correlation with disease-specific measures, demonstrated that the measure performed well at documenting many burdens of DMD (Crossnohere et al. 2021). In this way it challenged an emerging notion that established legacy measures could not be used to capture the experiences of patients (Crossnohere et al. 2022c; Powell et al. 2022). In doing so, it raised questions regarding how to determine when the use of these measures is appropriate and sufficient, and when additional resources and engagement efforts would be needed to supplement HTA deliberations.

8.3 AI and Patient Involvement

AI is challenging all aspects of science, medicine, and daily life. AI includes both machine learning models and natural language processing (NLP), which can be used in ways that can enhance, and in some cases replace, human-performed processes. US physicians named addressing administrative burden such as medical documentation to be the greatest area of opportunity for AI in a 2025 survey of US physicians by the American Medical Association (2025) and patients found AI chatbots to be an empowering resource for nonjudgemental feedback on sensitive topics in a recent systematic review (Aggarwal et al. 2023).

AI can also be applied to support patient involvement broadly in health. AI-based tools can be valuable to support existing approaches for healthcare research, and they are also leading to a new branch of research that can only be done with AI support. AI can be used to ‘listen’ to patients’ communication and is increasingly used in medical research to support administration and evidence generation (such as transcribing meetings and recordings) and evidence synthesis (such as assisting in systematic reviews). AI can also be used to understand patients by assisting in mining diverse data sources such as medical records, billing data, wearables, and other big data in health. While the term ‘real-world data’ (RWD) can be used to describe many types of big data collected in routine care in health, AI can be applied to non-health sources such as social media, telecommunications data, and purchasing/financial data. Chapter 7 presents one potential use of AI to foster patient involvement through NLP studies that could be enlightening for HTA and medical research more generally by identifying patients’ perspectives. The emergence of AI in health research challenges traditional practices and methods for patient involvement that need to be explored.

Chapter 7 provides details of how NLP can extract structured data from unstructured text. It can be used to identify emotional tone and sentiment, identify medical concepts from clinical notes and support the prediction of patient outcomes. Though NLP has technical shortcomings due to the complexity of language, these tools are a valuable way to interpret patient-generated health data from social media that could not otherwise be interpreted without enormous human effort. The authors of Chap. 7 suggest that NLP may offer benefits over structured patient-reported outcome measures, such as avoiding participant attrition for follow-up surveys.

Even with highly accurate NLP, applying it to content on social media can be challenging to interpret because social media platforms are a unique context. Tufekci (2014) eloquently described use of social media for research as comparable to using fruit flies as model organisms in biology; neither is an absolutely accurate representation of humans, but both are useful for the ways that they can be used in research that could not be feasible through other methods. Algorithms are built into social media platforms, shaping what content is visible to the users and leading to bias of how the users engage with social media. Content is not always generated in earnest, as AI-generated content and sponsored content are prominent. It has been recommended not to draw conclusions from social media research alone, but instead to also use complementary conventional approaches (ibid.).

Applying NLP to social media has increasingly raised ethical and regulatory concerns. The ethical guidelines developed before the existence of social media are not adequate to ensure the protection of research subjects, even when using publicly available data. Ethical recommendations include performing de-identification of social media data, obtaining institutional review board or research ethics committee approval, and exercising caution when linking data (Chap 7, Holtorf et al. 2023b). Use of direct quotes from social media is particularly challenging as individuals can be easily re-identified (Ayers et al. 2018). There is also a lack of consensus on patients' perspectives on the acceptability of these technologies and use of the data collected. HTA experts state concerns about transparency of how AI algorithms operate and potential harmful model biases (Goodman and Treloar 2024). The ethical, legal and social implications of AI in engagement research will need to be managed in future best practices for AI approaches to be widely adopted in a way that balances stakeholder protection with benefits.

Chapter 7 points out that using NLP for data already generated is not a license to abandon participation in research. Approaches for involving patients in the development and evaluation of medical products, such as those outlined in Chaps. 29, 30, 31 and 32, are well-established and can be applied to guide the use of AI in HTA (Crossnohere et al. 2022b). Still, a review of frameworks for the use of AI in medicine found that end-user participation is not standard in the development, evaluation, and surveillance of AI (Crossnohere et al. 2022a). Knowledge gaps around AI are an obstacle to acquiring participant input in study design (Adus et al. 2023) and the key to overcoming this obstacle is use of educational resources that prime discussion rather than overwhelm the participant (Crossnohere et al. 2022b). Methods such as participatory research (Arevian et al. 2018), deliberative processes (Staniszewska et al. 2021), and preference studies (Seo et al. 2022; Crossnohere et al. 2022d) are effective platforms that could be applied to inform NLP studies of patients (Crossnohere et al. 2022b).

8.4 Expanding the Use of AI in HTA

There is substantial enthusiasm and potential for the use of NLP studies of patients' perspectives despite concerns about data quality, risk of generating false or misleading outputs known as hallucinations, and regulatory concerns. HTA may be informed by the unique types of research that can only be done with AI in the future. Social media research is uniquely suited to characterise populations with potentially stigmatising conditions because it provides complex self-reported data without the burden of a qualitative interview. One example is a recent study of a Facebook support group of Malaysian women with endometriosis, which identified the ways that this population sought peer support and the limitations of support provided by health-care providers (Wilson et al. 2020; Holtorf et al. 2023a). Social media often includes the time and location of a post, providing epidemiological data to consider in analyses, as seen in a thematic analysis of Weibo posters' attitudes about COVID-19 with

consideration for concurrent regional outbreaks (Li et al. 2020; Holtorf et al. 2023a). Apart from primary data, NLP may be used to organise data from published studies of patient involvement, facilitating new reviews and meta-analyses.

The high barrier to entry of conducting NLP research will be an ongoing limitation for its future applications in HTA. NLP is efficient at parsing large volumes of data, but tremendous upfront investment is needed to conduct studies. Researchers often face obstacles to access data from healthcare systems and use it to train NLP due to justified concerns about data governance, privacy, and security (Ng et al. 2025). New secure infrastructures may be needed, as were developed for NLP to follow the US Health Insurance Portability and Accountability Act (HIPAA) law regarding privacy of healthcare data (ibid.), and this expense may be prohibitive for many organisations for which such regulations apply. Apart from simply acquiring data, training and fine-tuning an NLP model takes time, effort, and expertise. High-quality NLP training data is available for languages that have previously been used extensively in AI research, such as English and Mandarin Chinese, which are referred to as high-resource languages (Pakray et al. 2025). Conversely, low-resource languages lack high-quality training data, even though many such languages are widely spoken, such as Arabic and Portuguese (ibid.). Without better accommodation for low-resource languages, NLP methods may perpetuate inequity of who benefits from healthcare research.

8.5 Conclusion

Patient involvement continues to advance through new research methods and approaches, yielding valuable findings in healthcare research and HTA. It is important to develop new methods to incorporate patients' perspectives because this can lead to research questions that are most valuable to the patient. The studies described in this chapter are highlighted for their innovative approaches. In the case of Project HERCULES, the innovation came from using a patient-led multi-stakeholder collaborative approach. A patient organisation initiated the effort and invited industry, clinicians, and academics as collaborators, inverting the normal workflow of research. This has led to research that incorporates the patient voice at every step. Conversely, the AI methods and example studies described in this chapter ascertained patients' perspectives through a wide variety of data sources, often indirectly. AI will continue to be a valuable source of support for established research methods, such as transcribing qualitative interviews. AI is also engendering new research, such as data mining of social media and medical health records content, that would not otherwise be possible. There are ongoing challenges to AI-driven studies, such as ethical research conduct, accuracy, and incorporating participant engagement. Greater discussion is needed to address these challenges and refine best practices.

Acknowledgements We thank the co-editors for their insightful feedback. NT has no patient or industry funding. NC has served as a paid and unpaid consultant on patient engagement and preference studies for patient organisations and the pharmaceutical industry. JB has served as a

paid and unpaid consultant on patient engagement and measurement of patient preferences for numerous patient organisations, pharmaceutical and medical device industry, and contract research organisations. JB holds an Innovation in Regulatory Science Award from the Burroughs Wellcome Fund.

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Chapter 9

Patient Preference Research to Inform HTA



Deborah A. Marshall, Carina Oedingen, Barry Stein, and Simon Fifer

9.1 Overview of Various Patient-Reported Measures

As described previously (Chaps. 1, 3, and 4), ‘patient involvement’ is an overarching term for research into patient aspects regarding needs, preferences, experiences and perspectives, and patient participation in health technology assessment (HTA) (individual HTA, overarching processes and HTA policy).

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With a view to patient-centred care and bringing the patient voice into decision making, information reported by patients is increasingly important in the context of patient-centred care (Bridges et al. 2023; Reed and Lavezzari 2016) and there has been growing interest and application of methods to elicit patient preferences and apply patient preference information in decision making at all levels of research and care delivery. Patient preferences are sometimes confused with patient-reported outcome measures (PROMs) and patient-reported experience measures (PREMs). Although all are patient-reported measures, each of these measures differ. PREMs refer to the patient's experience with their own health state, the setting in which healthcare is delivered (structure) and the interactions between patients and providers (process). PREMs can be any report of the patient's personal experience with their health and the healthcare services they have received. In contrast, PROMs capture the health status and the impact of healthcare on the health status of patients (outcomes) (Gilmore et al. 2023; Mühlbacher and Stolk 2023). PROMs can be any report of the status of a patient's health condition (realised outcome) that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else.

In comparison, patient preferences can be used to value a broader range of aspects of care, beyond clinical outcomes such as survival and quality of life, to include aspects of care delivery and non-health outcomes (e.g. mode and administration of treatment). Patient preference research has received increasing attention in the health literature and in decision making with the increased focus on patient-centred care and an emphasis on measuring benefits that matter to patients (Bridges et al. 2023; Mott 2018; Reed and Lavezzari 2016; Soekhai et al. 2019a).

Patient preference information has been defined as qualitative or quantitative assessments of the relative desirability or acceptability to patients of features that differ among alternative health states, health interventions, or health services (US Food and Drug Administration 2016). Desirability reflects preferences for positive outcomes or features (benefits) and acceptability reflects the degree of aversion to negative outcomes or features (harms or risks). In contrast to PREMs and PROMs, patient preference information offers a measure of what a patient wants in terms of desirability and acceptability of these features and also offers the possibility to capture the relative importance of these features and patient priorities, and the trade-offs that a patient might make amongst the features.

Patient preference data inform the (relative) importance of product or service characteristics from the patient's perspective and can also provide insights regarding heterogeneity of preferences in different patient populations (with the caveat that the sample is sufficiently large and diverse, and the data collected capture relevant variables related to the preference heterogeneity). Both qualitative and quantitative preference research can be conducted to understand the relative importance of a product or service. Qualitative research (Chap. 11) is also critical to informing the design of quantitative patient preference surveys in which trade-offs among attributes are elicited from respondents through a series of questions (Coast et al. 2012; Hollin et al. 2020; Vass et al. 2017).

9.2 Main Methods Used for Measuring Patient Preferences

In health, the term preference research is often used to refer to an array of qualitative and quantitative methods. Qualitative methods include interviews and focus groups asking about patient preferences for different treatments. Quantitative methods include:

- time-trade-off and standard gamble methods for estimating health state utilities,
- structured weighting, e.g. using rating, ranking exercises, swing weighting or analytic hierarchy process (AHP),
- stated preferences, e.g. using best-worst scaling (BWS), conjoint analysis, and discrete choice experiment (DCE) surveys, and
- revealed preferences (derive preferences from actual observed market behaviours).

Various catalogues have been created to capture and categorise these alternative patient preference methods (Ho et al. 2016; Medical Device Innovation Consortium 2022; Soekhai et al. 2019a).

Patient preference research in health is motivated in general by the desire to promote patient centredness in healthcare and can be used to inform clinical and health policy decision making (Ho et al. 2016; US Food and Drug Administration 2016; Soekhai et al. 2019b). Quantitative stated patient preference research includes valuation methods grounded in utility and consumer theory and the psychology of choice to understand how patients value different aspects (attributes) of an intervention (Louviere et al. 2000). Stated preference research examines trade-offs between multiple attributes used to describe an intervention, and the effect of changes in the attributes' levels on choice. Utility values are derived from the choice responses using econometric analysis methods. In contrast, structured weighting techniques such as AHP derive preferences from pairwise comparisons between two attributes, and relative statements regarding the importance of (or preference for) an attribute (level) are elicited. The weight of each attribute in the set is calculated from the pairwise comparisons of all weights.

Systematic reviews of the literature over time have reported that DCEs, and more recently, BWS methods are the most commonly applied approaches for measuring patient stated preferences in health from which preferences are derived from surveys of patients based on choices (Clark et al. 2014; De Bekker-Grob et al. 2012; Marshall et al. 2010; Soekhai et al. 2019a; Veldwijk et al. 2023). Thus, this chapter focuses on DCE and BWS.

DCEs are an attribute-based measure of value that examine trade-offs among attributes of a healthcare intervention. In DCEs, respondents choose between alternatives in a series of questions called 'choice tasks'. Each alternative differs and includes features ('attributes') of the decision (i.e. treatment side effects, dosing, chance of success) (Bridges et al. 2011; Lancsar and Louviere 2008). The way participants respond to the choice tasks reveal the weight respondents attach to attributes of the treatment or intervention and thus infer respondent preferences through

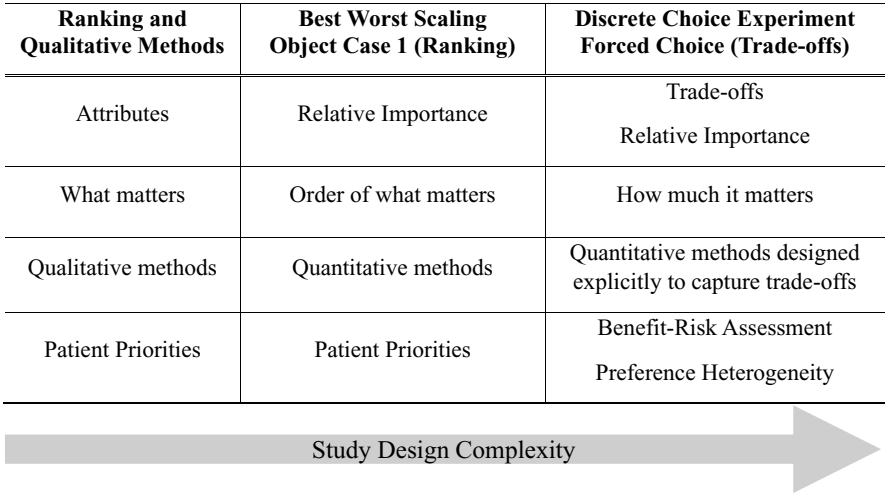


Fig. 9.1 Most common types of stated preference methods in health

an indirect utility function. These weights can be used to estimate marginal rates of substitution that reveal the trade-offs respondents are willing to make between attributes (Hauber et al. 2013; Soekhai et al. 2019b).

Evidence about patient preferences derived from DCEs can be used to value patient experiences alongside clinical outcomes, value health states in economic models and predict uptake or acceptability of new services (Vass et al. 2017). DCEs also provide potentially useful information within a HTA decision-making context: what characteristics (attributes) are important, how important these are, trade-offs between characteristics, monetary measures of value and predictions of take-up rates and acceptability of interventions, medicines or services (Bouvy et al. 2020).

More recently, BWS surveys, specifically Object Case 1, have been applied to capture patient preferences in terms of relative importance and rank and prioritise attributes of an intervention (Flynn et al. 2007; Hollin et al. 2022; Mühlbacher et al. 2016). In BWS Object Case 1, respondents complete a survey in which they choose both the best and worst items in a series of questions (‘choice tasks’) containing different subsets of the items each containing the same number of items. The responses reveal the relative importance of each item (Fig. 9.1).

9.3 Types of Questions that Can Be Answered and Types of Preference Studies Used to Address Them

Patient preference studies can be used to answer a broad number of questions. We identified six common research questions relevant to HTA decision making that patient preference studies can be used to answer (see Table 9.1). These include

Table 9.1 Types of research questions answered by patient preference studies

Research question	Preference method	Context	Use cases	Benefit for HTA	References
Relative attribute importance What matters most to patients? How do patients trade-off benefits with risks/ costs?	DCE	When treatment profiles vary (e.g. by efficacy, safety, quality of life outcomes, etc.) and there is a lack of evidence or uncertainty about how patients trade-off benefits vs risks/costs	1. Comparing a new treatment vs an existing treatment (comparator). Determine if the new treatment is preferred, by how much and what is driving preference 2. Comparing multiple treatment options	Score and compare treatments by mapping real treatment characteristics to the patient value framework identified by the model (sometimes referred to as Patient Value Mapping)	Fifer et al. (2020), Gonzalez (2019)
Non-health benefits What value do patients place on non-health benefits?	DCE	When a new treatment has non-health benefits that are not captured by other evidence (e.g. clinical outcomes or quality-adjusted life-years (QALYs))	1. A new treatment has a different mode of administration compared to existing treatments (e.g. measure the value of oral vs injection treatments) 2. A new treatment has a different frequency of administration compared to existing treatments (e.g. measure the value of monthly vs weekly injections)	Understand the value of wider patient experience alongside other evidence (clinical outcomes)	Fifer et al. (2018), Marshall et al. (2018)
Predicted uptake What is the predicted uptake of a new treatment?	DCE	When there is uncertainty around what proportion of patients would prefer to use a new treatment over existing treatments	1. Measure uptake for a new treatment that has not entered the market	Measure the acceptability of new treatments and potential budget impact	Fifer et al. (2024), Marshall et al. (2009, 2016a)

(continued)

Table 9.1 (continued)

Research question	Preference method	Context	Use cases	Benefit for HTA	References
Willingness to pay (WTP) How much are patients WTP for a new treatment or feature of treatment?	DCE	When we need to convert patient value (benefits) into monetary measures	1. Measure how much additional benefit a new treatment brings to a market and translate that benefit into monetary measures	WTP estimates can be used in a Cost Benefit Analysis (CBA) framework	Deal et al. (2013), Fifer et al. (2018), Johnson et al. (2014), Marshall et al. (2016b, 2019)
Maximum acceptable risk (MAR) What is the greatest increase in harms/risks which a patient would accept for a given benefit?	DCE	When a new treatment has significant benefits but also significant harms/risks and there is no evidence of the risk tolerance of patients for a specific benefit	1. Measure how much risk patients would be willing to accept to gain an additional benefit (e.g. % increase in risk of side effects for an additional year of average survival)	1. Maximum level of risk that patients are willing to accept 2. Measure how much risk patients would be willing to accept to gain an additional benefit (e.g. % increase in risk of side effects for an additional year of average survival)	Hauber et al. (2009, 2013), Ho et al. (2015)
Endpoint prioritisation How do patients prioritise various study endpoints?	BWS	When there is a lack of evidence or uncertainty about how patients value endpoints (e.g. primary vs secondary vs surrogate endpoints)	1. A new treatment has a number of endpoints used in the trial (clinical benefits and risks, quality of life, etc.). Determine or measure how patients prioritise these endpoints	Interpret relevant endpoints through the lens of the patient. Provides context to decision making	Hazlewood et al. (2016), Hollin et al. (2022), Tsai et al. (2018), Vaanholt et al. (2018)

measuring relative attribute importance, evaluating non-health benefits, predicting uptake, measuring willingness to pay (WTP), calculating maximum acceptable risk (MAR), and endpoint prioritisation. The table summarises which preference method best matches each research objective, the context where this type of evidence would be valuable, common examples or use cases, how preference outcomes would be beneficial in HTA decision making, and lastly providing a supporting reference.

Each research question can be applied to measure aggregate patient preferences. More sophisticated preference models can be used to measure variation in patient preferences (known as preference heterogeneity) (Vass et al. 2022; Zhou et al. 2018). This is particularly important in HTA decision making because it is not expected that all patients have the same preferences in most health contexts (e.g. measure whether groups or segments of patients have different preferences). If systematic differences in preferences between populations can be demonstrated, HTA committees can use this information to see how certain decisions would impact or align with different segments of patients.

9.4 How Patient Preferences are Currently Used in HTA

In recent years, the increased attention on patient preferences in clinical practice guidelines (Krahn and Naglie 2008; Whitty et al. 2017) and academic research (Soekhai et al. 2019b) was reflected in the demonstratable increase in the use of patient preferences among regulatory decision-making bodies in different countries. In an era where sustainability and innovation may be seen to be in conflict and where clinical trial endpoints are challenged, patient perspectives in HTA integrating quality of life, patient experiences, and preferences are fundamental in drug reimbursement decisions. Moreover, as HTA bodies enter into international collaboration agreements with other HTA bodies, for example under the European Regulation on HTA coming into effect in 2025 (Chap. 18), input from patients and patient organisations will become increasingly important. Consequently, regulatory and HTA bodies have been exploring the use of patient preference data (Benz et al. 2019).

In the United States (US), for example, the Food and Drug Administration (FDA) has provided guidance on how to use patient preference information in benefit-risk assessments (US Food and Drug Administration 2016). Furthermore, the increasing commitment to patient focused drug development by the US FDA and patient participation in translational research, provides a significant opportunity to improve the clinical trials enterprise and enhance participation by patients and patient organisations in the work of trial sponsors (US Food and Drug Administration 2025). The European Medicines Agency (EMA) also assessed the feasibility of incorporating patient preferences into their processes (Postmus et al. 2016).

Patient preference research is also mentioned as a potential method in the guidelines of the Australian Pharmaceutical Benefits Advisory Committee (Commonwealth of Australia 2016). In England and Wales, the National Institute for Health and Care

Excellence completed a two-year exploratory study concluding that patient preferences might be able to generate relevant evidence for HTA decision making (Bouvy et al. 2020). The Institute for Quality and Efficiency in Health Care in Germany explored the application of patient preference data to inform relevant endpoints of a technology conducting case studies on the use of quantitative patient preference data for reimbursement decisions (Hummel et al. 2012; Mühlbacher and Sadler 2017).

This interest from regulatory bodies in the use of patient preference data in HTA requires consensus on where patient preferences can provide valuable input into HTA decision making with which advantages and disadvantages over current practices. Patient preferences can be informative for HTA throughout all stages of the product life cycle (such as product development, regulatory decision making, marketing authorisation, HTA or reimbursement decision making and real-world evidence generation) to address a range of questions faced by decision-makers. This can include areas where multiple treatment options are available, where there is uncertainty about which treatment is most beneficial, or where the patient's perspective is crucial for understanding the impact of the condition and its treatment on their quality of life (Jackson et al. 2019). Bouvy et al. (2020) identified three main application areas of patient preference data from the perspective of HTA bodies:

1. identification of priorities and relevant endpoints in early drug development,
2. benefit-risk assessment in regulatory approval, and
3. complementing clinical trial evidence for informing the HTA recommendations or policy decisions.

Traditionally, HTA processes focus on clinical effectiveness, while some HTA bodies also evaluate economic considerations such as cost-effectiveness (Mott 2018) but do not explicitly consider (quantitative) patient preferences.

Patient preference data can be integrated through approaches such as trade-off assessment between clinical benefits and side effects, preference share, estimation of QALY gains and cost-benefit analysis as shown in the transcatheter aortic valve implantation case study example (Marsh et al. 2021). Despite growing interest, it remains unclear how patient preferences can be used in HTA and the extent to which it can reshape and influence HTA decisions (e.g. accepted as stand-alone evidence to clinical and economic effectiveness or as additional evidence to clinical effectiveness only).

Thus, patient preference data can be used as evidence to support alignment of HTA decisions with patient preferences and needs (patient-centredness) resulting in improved treatment outcomes and patient's quality of life (Case study example Box 9.1). Furthermore, preference evidence can guide HTA bodies in prioritising resources and funding for interventions that are valued highly by patients. HTA bodies are enabled to make more informed decisions about which healthcare interventions should be prioritised and funded. This ensures that resources are allocated to interventions that are most valued by patients.

HTA bodies will remain primarily interested in health technologies for which added health benefits were demonstrated for the whole patient population compared to currently available treatments. Patient preference studies can reveal how relevant

these demonstrated benefits are for the patients living with the disease. HTA bodies will be most interested in using patient preference studies when these studies are carefully targeted to address research questions which are relevant to HTA, when they offer guidance and a standardised path on how to effectively balance patient preference evidence with clinical and economic evidence, and when the content and format of these preference studies can support consistent interpretation as well as application in HTA (Bouvy et al. 2020).

Box 9.1 Case Example 1: Use of Patient Preference Study Findings in HTA from a Discrete Choice Experiment for Heart Valve Diseases

Heart valve disease (HVD) is a broad term used to describe diseases and conditions affecting the heart's valves (Marwick et al. 2023). Both surgical aortic valve replacement (SAVR) (Hibino et al. 2023) and more recently less invasive procedures, such as transcatheter aortic valve implantation (TAVI), are becoming more common (Baron et al. 2017). Techniques are safe and effective, however TAVI is less established than SAVR. On the other hand, it is also less invasive and associated with faster recovery and fewer complications (Leon et al. 2010; Kolkailah et al. 2019; Mack et al. 2019).

Fifer et al. (2024) conducted a DCE to understand patients' preferences for HVD treatments, including benefit risk trade-offs for heart valve procedures, with a focus on the effects of invasive vs minimally invasive procedures to mirror SAVR and TAVI. The DCE was funded by Edwards Lifesciences, a medical device company specialising in artificial valves. Relative attribute importance, as well as predicted preference share was calculated from the DCE model, and participants were grouped by their prior experience with heart valve procedures into: (i) no procedure experience; (ii) invasive procedure experience; or (iii) minimally invasive procedure experience.

There is a great deal of heterogeneity in HVD patient preferences, even when treatment outcomes appear similar. HVD patients demonstrated a slight overall preference for the minimally invasive procedure over the invasive procedure. Patients' choices were most influenced by the durability of the valve and likelihood of independence post-procedure, irrespective of their prior procedure experience. When all treatment attributes were held constant and the effect of invasiveness of the procedure was isolated, patients were more likely to choose the treatment they had already had experience with (an 'experience effect'). However, predicted preference share analysis demonstrated that when the attributes reflected the profile of the TAVI/SAVR procedure (its safety and efficacy results), preference for TAVI was so strong it reversed the influence of prior treatment experience, and all patient organisations were predicted to choose TAVI over the SAVR profile.

These results can inform HTA decision-makers about what features of HVD procedures patients value most, to ensure appropriate treatments are available that align with patient goals and values (i.e. long valve durability and fast recovery).

9.5 Recommendations and Guidance from International Consortia and Expert Groups

International consortia and expert groups working in the field of patient preference research have published recommendations on why, when and how to access and use patient preferences in HTA. This section presents three separate sets of recommendations from the:

1. International Society for Pharmacoeconomics and Outcomes Research (ISPOR),
2. Innovative Medicines Initiative Patient Preferences in Benefit–Risk Assessments during the Medical Product Lifecycle (IMI-PREFER) project, and
3. Health Technology Assessment International (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) Patient Preference Project Subcommittee (PPPS).

9.5.1 ISPOR

ISPOR has produced several international expert Task Force reports that are consensus-based good research practices for methods to study patient preferences which have been widely cited and applied in the literature. The first of these, published in 2011 (Bridges et al.), reported on a range of methods that included those that used rating, ranking and choice-based stated preference approaches to assess profiles that varied in their attributes and levels. The Task Force provided broad guidance on good research practices by suggesting a structure to guide the development, analysis, and publication of conjoint analyses in healthcare studies, without necessarily endorsing any one approach. The good research practice guidance included a 10-step checklist covering: (1) research question; (2) attributes and levels; (3) construction of tasks; (4) experimental design; (5) preference elicitation; (6) instrument design; (7) data collection plan; (8) statistical analyses; (9) results and conclusions; and (10) study presentation. The second and third Task Force reports focused on choice-based methods, and the key methodological aspects of experimental design and statistical analysis of DCEs (Johnson et al. 2013; Hauber et al. 2016). Together, these Task Force reports provide guidance for planning, designing, conducting and analysis of high-quality patient preference studies to build capacity for researchers generating patient preference information and for users to interpret consistently findings from preference studies.

The fourth Task Force report shifted attention from the methodological aspects of stated preferences to the important aspects of how patient preference studies can be used for decision making in a wide variety of contexts, including HTA (Bridges et al. 2023). This report provides good practices for increasing the usefulness and impact of patient preferences in decision making captured as five key elements: context, purpose, population, method and impact (Table 9.2).

Table 9.2 Good practice elements to increase the usefulness and impact of patient preference studies in decision making

Element	Context	Key aspects to consider
1. Context	Assessing the who, what, where, why and when about decision makers and decision making	• Identifying the most relevant individuals and organisation who are involved in making the decision • Identifying what types of decision/s can be informed by conducting a preference study • Identifying where you can interact with decision makers • Identifying why decision makers may need the preference study and/or find it useful • Identifying the optimal time to conduct a study and share it with decision makers
2. Purpose	Determining how a preference study can be most useful to decision makers and other stakeholders	• Clarifying how the study can be relevant to decision makers and/or in decision making • Communicating how the study can help address decision makers' need(s) • Being transparent about how the researcher intends the study to be used by decision makers • Developing resources that help decision makers use the study findings in decision making • Providing assistance to decision makers and having allies who can also support decision makers
3. Population	Identifying to whom the study findings will be applied and how they can best be engaged	• Identifying who will be affected when decision makers apply the findings • Clarifying how the study sample was identified to reflect the relevant affected population • Clarifying how patients/groups are engaged in the conduct of the preference study • Partnering with patients/groups to ensure their needs are being addressed by the study • Sharing the study findings to relevant patients/groups and communicating how data was used

(continued)

Table 9.2 (continued)

Element	Context	Key aspects to consider
4. Method	Describing how the study method/s chosen and the protocol was shared and explained	• Explaining which methods were considered when designing the study and why • Explaining how and why specific methods were chosen for the preference study • Sharing the study protocol broadly with decision makers, and/or other stakeholders • Demonstrating how input from decision makers, patients and stakeholders was addressed • Providing resources for decision makers, patients and other stakeholders about the methods used
5. Impact	Thinking and acting broadly to identify and assess the intended and unintended impacts	• Identifying all the intended and unintended impacts from the study on decision making • Assessing the usefulness of the study and the intended and unintended impacts • Replicating the study and testing if findings are generalisable to other populations • Disseminating the work broadly and capturing how it might be impactful in other ways • Fostering the implement or application of the study findings in real-world contexts

Adapted from Bridges et al. (2023)

9.5.2 *IMI-PREFER Project*

IMI-PREFER was a joint project from 2016 to 2022 undertaken by 33 public and private partners representing academic institutions, pharmaceutical companies, health technology agencies, and patient organisations in Europe and the US. The expert and evidence-based recommendations provided guidance on the design, conduct, and use of patient preference research in decision making throughout the medical product life cycle for health technology developers, regulatory authorities, health technology and reimbursement agencies (PREFER Consortium 2022). The four recommendations listed below are the ones most relevant to HTA.

9.5.2.1 Value of Patient Preferences: When to Incorporate Patient Preferences?

Patient preferences can be useful for informing preference-sensitive decisions. However, it is important to decide when patient preference research may benefit decision making. Patient preference data can be used as stand-alone data or as supplementary information together with clinical data. The preference data can inform the choice of endpoints by showing what is most important to patients and how much it matters as well as patient acceptability of trade-offs between different medical product characteristics (Whichello et al. [2020](#)).

9.5.2.2 PREFER Framework: Which Steps are Needed to Conduct Patient Preference Research?

IMI-PREFER provided a step-by-step framework of the required stages for preference research based on three overall components: the organisation (study objectives and timing); design (study population, method selection and analysis planning); and conduction (data collection, analysis, and interpretation). The framework is intended to facilitate the discussion about best practices for preference research and complement other published best practices and checklists (PREFER Consortium [2022](#)).

9.5.2.3 Working with Key Stakeholders: When Should Patients and Other Stakeholders Participate?

Getting stakeholders to participate early in preference research is crucial for its success. Patients should participate as research partners or advisors through the entire process, from planning the study until the dissemination (Smith et al. [2023](#)). Regulators and HTA bodies should be consulted to make sure that the research adds value and delivers results that can be integrated in the decision-making process (Van Overbeeke et al. [2020a](#)). This approach can increase the relevance, appropriateness, feasibility, and acceptability of patient preference research ensuring that it provides useful information for stakeholders.

9.5.2.4 Areas for Future Research: What Is Next in Patient Preference Research?

Future areas of research were identified that could not be addressed by the IMI-PREFER group. These include how to incorporate results from patient preference research, to compare different preference elicitation methods or to determine if, and to what extent, internal validity and data quality can be incorporated in preference research, as well as to explore measurements of external validity for different preference methods (PREFER Consortium [2022](#)).

9.5.3 HTAi PCIG Patient Preference Project Subcommittee (PPPS)

HTAi is a multi-stakeholder professional society that seeks to develop and promote HTA internationally. Its PCIG runs a range of projects to develop patient involvement in HTA. One such project is led by the PPPS to enhance the use of patient preference evidence in HTA decision making. The PPPS is made up of volunteers from around the world, including patients, academics, preference researchers, health technology developers and HTA representatives. The HTAi project's early work focused on assessing the current landscape regarding use of patient preferences in HTA. This work identified a gap in knowledge which led to a four-part educational patient preference webinar series that started in 2020 (HTAi 2021).

In 2022, the PPPS conducted an online survey with 40 members of HTA bodies and affiliated organisations, across 12 countries, to identify and validate the current barriers to implementing and adopting patient preferences in HTA decision making processes (Hilgsmann et al. 2024). Survey results showed that the leading reasons why patient preference studies were not submitted by technology developers in most HTA assessments were time and resource constraints followed by a lack of clarity on how preference evidence was used in the HTA and what impact it may have on decisions.

Extending this work, the PPPS conducted a series of in-depth collaborative workshops with HTA representatives from five different countries to facilitate mutual learning and reflection on ways that patient preference data can be better integrated into HTA (Germeni et al. 2024). Outcomes from the workshops further highlighted a lack of awareness and understanding on the use of patient preferences in HTA. Workshop participants agreed that patient preference evidence would enhance decision making if submitted alongside other types of evidence, however, despite appreciating the value of these methods they were still unsure of where the evidence fits and how it could be used in the decision-making context.

In light of these findings, the HTAi PCIG subcommittee conducted mock deliberations with members of various HTA bodies (via an online workshop series) where patient preference information is presented as evidence to be included in the HTA deliberative process, alongside standard clinical and cost effectiveness evidence. The mock deliberation workshops highlighted both opportunities and challenges in integrating patient preference information into the HTA processes. While patient preference information was acknowledged as valuable evidence, its influence in decision-making varied. Variation in responses was predominantly linked to the experience and background of the participant (e.g. clinician or health economist) rather than systematic differences between the countries. In general, participants understood how patient preference information could be integrated with the clinical evidence (e.g. interpreting the clinical data from the patient perspective). In contrast participants found it difficult to comprehend how patient preference information could be integrated with standard cost-effectiveness evidence. Further

research is planned with committee members to address these issues before developing more formal guidance.

Final results of the project will be disseminated as a ‘how to guidebook’ with the aim of improving the likelihood that patient preferences can and will be used more frequently in HTA decision making.

9.6 Methodological Challenges of Using Patient Preferences in HTA

Evidence from patient preference research studies can provide useful information for HTA bodies. However, while the use of patient preference research studies in HTA is evolving it is not standard practice yet. There remain methodological challenges in meeting the 11 recommendations issued by the FDA for valid scientific evidence for patient preference information or any clinical/nonclinical information in a regulatory submission. These recommendations address: patient centredness; representativeness of the sample and generalisability of results; capturing heterogeneity of patients’ preferences; established good research practices by recognised professional organisations; effective communication of benefit, harm, risk, and uncertainty; minimal cognitive bias; logical soundness; relevance so that critical aspects of harm, risk, benefit, and uncertainty are included; robustness of analysis of results; study conduct; and comprehension by study participants (US Food and Drug Administration 2025).

Amongst this list of requirements, potential key barriers to the broader use of patient preference research studies in the context of HTA applications include:

- measuring the preferences of a representative sample of adequate size so that the study results can be reasonably generalised to the population of interest,
- capturing heterogeneity of patients’ preferences for benefit-risk trade-offs that reflects a broad array of potential factors (e.g. sex, gender race, ethnicity, socio-economic, life circumstances, personal tolerance for risk, and experiences with their disease,
- effective communication so that patients understand and cognitively process the health outcomes, and the benefits, harms, risks, and uncertainties associated with the benefit-risk trade-offs captures in the patient preference study.

There is ongoing debate on how it might be possible to incorporate patient preference data in a systematic, standardised, and quantitative manner in HTA decision making (Van Overbeeke et al. 2020b). Huls et al. (2019) identified 37 unique research issues that are hindering the integration of patient preferences into HTA; mainly conceptual, normative, procedural, methodological and practical issues. Conceptual issues include how patient preferences should be defined, while normative issues encompass questions about whose preferences should be elicited (patients with or without treatment experiences, carers, patient representatives). Procedural

issues were found to be HTA specific and concern what weight should be given to patient preference data, when to include those data in the process, what impact these data can have on decision making, and how the quality of patient preference data can be evaluated. Lastly, methodological issues were related to the internal and external validity, reliability, generalisability, and how patient characteristics affect preferences (Huls et al. 2019).

If the current guidance on the design, conduct and analysis of patient preference studies were applied, this would likely help overcome many these concerns (Bridges et al. 2011; Johnson et al. 2013; Hauber et al. 2016; Bridges et al. 2023). The Case Study in Box 9.2 is an example which follows the FDA recommendations for designing, conducting and reporting a patient preference study in the first study designed to obtain quantitative patient preference evidence in a regulatory approval decision.

Box 9.2 Case Example 2: Use of Patient Preferences from a Discrete Choice Experiment of Weight Loss Devices for Regulatory Approval Decision (Adapted from US Food and Drug Administration 2025)

This was the first study designed to obtain quantitative patient preference evidence to inform regulatory approval decisions by the FDA Center for Devices and Radiological Health (CDRH). The study evaluated trade-offs among effectiveness, safety, and other attributes of weight-loss devices using discrete choice experiments to quantify the importance of safety, effectiveness, and other attributes of weight-loss devices to obese respondents. Respondent choices yielded preference scores indicating their relative value for attributes of weight-loss devices in this study. The study showed that a substantial portion of obese patients would accept the risks associated with a surgically implanted device if they lost a sufficient number of pounds. For example, to accept a device with 0.01% mortality risk, a risk tolerant patient will require about 10% total body weight loss lasting 5 years.

Patient preference evidence was used to make regulatory decision making more patient-centred. In addition, they captured the heterogeneity of patient preferences allowing market approval of effective devices for risk tolerant patients. CDRH is using the study tool to define minimum clinical effectiveness to evaluate new weight-loss devices. The methods presented can be applied to a wide variety of medical products. Studies like this may provide information on the relative importance of certain device attributes to patients as well as how benefits and risks are weighted, enabling more patient-centric decision making and potentially informing the design and analysis of clinical trials.

Key aspects

- Patient preference evidence was used to make regulatory decision making more patient-centred.

(continued)

Box 9.2 (continued)

- The sample included more than 500 patients drawn from an online panel that was designed to represent a cross section of the US population. The study sample had similar demographic characteristics to those of obese patients in the US population. The sample size was planned to capture a wide spectrum of patient preferences and provided better representativeness of the US obese population than anecdotal remarks or small focus group studies.
- The study was designed to measure quantitative patient preference heterogeneity and conduct preference segmentation. The study's stratified sampling by Body Mass Index (BMI) ensured that estimates were precise across the whole BMI range of interest. Moreover, the study used a preference elicitation method that not only allowed investigators to identify and divide patients into different segments by patients' risk-tolerance level but also provided the estimated percentage of patients that would prefer receiving the device to the status quo.
- Design, conduct, and analysis of the study followed good research practices endorsed by a recognised professional organisation (ISPOR).
- Research conducted at the study design stage and during the face-to-face interviews with patients helped ensure that the survey instrument was patient-centred, the communication of benefits, harms, risks and uncertainty was clear, and the format of the questions would keep potential cognitive bias to a minimum.
- Rigorous internal validation tests were conducted to make sure the data quality was sufficiently high.
- The benefits (weight loss amount and duration, improvement in comorbidities), risks (mortality, adverse events, and hospitalisation), and key attributes (type of surgery, diet restrictions) of the device were carefully defined so that the trade-off among the benefits and risks would be comprehensible to patients, healthcare professionals, and the FDA.

Another consideration in the use of patient preference data within the HTA appraisal process is the time and costs involved in conducting stated preference surveys such as DCEs or BWS (Object Case 1). Many HTA assessments rely on the review of existing evidence—that is, they are based on secondary data with limited time (and resources) to collect primary data. As such, it is unlikely that preference studies would be conducted as part of many assessments. However, they could be part of the evidence base submitted by health technology developers, especially in collaboration with academic experts to address the quality and robustness of the patient preference study design.

Responsibility for collecting patient preference evidence remains an unresolved issue. Research suggests that HTA bodies believe patient organisations should be responsible for collecting such evidence (Hilgsmann et al. 2024). Despite having lived experience, knowledge and connections with patients, many patient organisations lack resources to conduct these types of studies.

While health technology developers may have the resources to fund or conduct patient preference research studies, such evidence will be perceived as less robust due to the perceived conflict of interest. Collaboration between patient organisations, patients, carers, academic researchers, clinicians, and HTA body representatives as early as possible in the drug or device development process can provide an opportunity for patient organisation-led research on patient preferences. These studies require unrestricted funding from multiple sources to protect against perceived bias.

9.7 Conclusion and Discussion: Areas for Future Research

Several areas of future research have been identified in the previous sections of this chapter. However, the inclusion of all relevant stakeholders in patient preference research is a common feature crucial to its success. From the outset of the research, regulators, HTA bodies and health technology developers should be consulted to determine which evidence gaps can be addressed with patient preference research, how patient preference research can add value and be integrated practically into the decision-making process. This highlights the need to explore alternative approaches in the context of current HTA processes and more case studies on how to use and interpret patient preferences in HTA. Moreover, patients and patient organisations should participate as research partners or advisors throughout the entire process including the planning, development and testing of the research to ensure the relevance, appropriateness, feasibility, and acceptability of the research. Additional consideration must be given to patient preferences being heterogenous and fluid over time.

Researchers and HTA bodies alike need to gain more experience with patient preference research and explore how its results can add value to HTA alongside other types of evidence. Evaluating how best to incorporate it in assessments and deliberative processes will be key to expanding its use. Where HTA bodies have the resources and capabilities, there may be opportunities for patient organisations to lead the research with the guidance of expert researchers.

Patient preferences research can provide added value to the decision-making process and help ensure that the drugs and devices truly meets the needs of patients, however there is more work to be done to ensure its place in the deliberative processes in HTA.

Acknowledgements We would like to thank Nicolle Hua and Karis Barker for their help with editing and referencing. We also would like to thank the co-editors for their helpful comments on the draft of this book chapter. All views expressed and any errors are entirely the responsibility of the authors. No funding was received by any authors for the work on this book chapter.

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Chapter 10

Ethnographic Fieldwork



Tine Tjørnhøj-Thomsen and Helle Ploug Hansen

10.1 Introduction

Ethnographic fieldwork is a robust research methodology to study patients' experiences, perspectives and practices, and, therefore, particularly valuable for health technology assessment (HTA) (Chap. 3). In this chapter, we introduce ethnographic research in relation to patient involvement in HTA. We consider fieldwork, which includes participant observation, ethnographic interviewing, and writing field notes. We also address the analytical work following data collection. We close the chapter discussing what we consider to be important dimensions of knowledge production including HTA.

10.2 Ethnographic Research and HTA

Ethnographic fieldwork is a specific research design developed in anthropology to explore the dynamic relations between social worlds and cultural systems, and how social actors think, act and interact. Ethnographic fieldwork encompasses a range of research methods, the principal being participant observation and ethnographic interviews. Conducting fieldwork implies that the researcher joins the people under study where they live or work for a period to observe, take part and experience their everyday life (Malinowski 1961; Amit 2000). Within anthropology and related disciplines, there is a long tradition of observing and participating in informants'

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(patients, consumers, carers, etc.) everyday life, talking and listening to what they say, thereby gaining knowledge about their perspectives and experiences as well as their activities, social interactions, and relationships. Thus, ethnographic fieldwork implies the researcher's engagement with the people under study and their involvement in the process of knowledge production.

To our knowledge, there are only a few HTA studies that have included ethnographic fieldwork in their research design. Among these is a Danish HTA which assessed the ward rounds at a Danish Hospital as a social practice and investigated the implications of the ward round for the different actors including both staff and patients. The methods used were observational studies, focus group interviews, individual interviews and time registration (Willemann et al. 2006). Another Danish HTA investigated how patient involvement in relation to heart rehabilitation and arthritis treatment could be shaped and developed. The empirical study took place in units for heart rehabilitation and arthritis treatment, where the researcher observed several consultations and conducted qualitative interviews with patients, nurses and doctors (Jacobsen et al. 2008). Both studies combined various forms of interviewing and participant observation. The latter enabled the researcher to document social practices and interactions between health professionals and patients, as well as their surroundings, thereby gaining insights into contextual issues not mentioned in interviews. In a Canadian study, the researchers used institutional ethnography including fieldwork with participant observation and interviews to take people's experiences as a 'point of entry' to understand organisational processes in an HTA setting. The Canadian Agency for Drugs and Technologies in Health (CADTH) implemented some of the proposed measures to develop their processes for public and patient involvement in HTA (Bidonde et al. 2021).

According to the article 'Patients' perspectives in health technology assessment: a route to robust evidence and fair deliberation' by Facey et al. (2010), the most commonly used qualitative methods "for generating evidence to determine patients' perspectives are individual in-depth interviews and focus group interviews" (ibid. p. 37). The authors emphasise that participant observation can "supplement" interviews (ibid.). Firstly, because what people *say they do* and what they *actually do* can be contradictory and secondly, because participant observation is useful for an understanding of the physical, social, cultural and economic context in which patients live or are receiving care.

Often, the researcher stays with people under study for a longer period of time (e.g. 3 or 6 months). This is seldom possible when doing an HTA. However, a shorter fieldwork (e.g. 1–3 weeks) may provide insights and knowledge to the HTA in understanding the social world of the patients. For instance, how they perceive and act in relation to health technologies such as screening, home dialyses versus hospital dialysis, ostomy bags or patient education. In this way, a short fieldwork study can do much more than just supplement different forms of interviews. Even though a patient may have valuable experiential knowledge about a specific illness, condition or health technology that can be explored in an individual interview, knowledge and perspectives are not only told, but they are also acted out in social interaction and social context. When, for instance, people use a blood pressure monitor to check their blood pressure at home, they may operate it in ways that adjust to their everyday life routines

and may not therefore follow the doctor's instruction. In addition, some patients may be taciturn or not responsive to interview techniques. Hence, we cannot obtain patients' perspectives only by interviewing them, even though interviews like all forms of conversations are important for getting patients' perspectives, attitudes, belief and expectations about health, illness and health technologies (Facey et al. 2010). Since patients' perspectives both influence and are influenced by social relationships, it is crucial also to understand how patients and their close relatives act and interact in different life- and healthcare situations.

10.2.1 Access and Gatekeepers

A methodological and ethical problem for all researchers is that of recruiting and gaining access to relevant participants and, in the case of ethnography, to acquire a place among them in a relevant setting, space or community. Access is not definitively solved, by gaining permission from, for instance, a hospital administrator or consultant to follow patients through investigations and treatment. It is a necessary, but not sufficient for including patients or clinical staff in a study. Patients, carers, and healthcare professionals are also gatekeeping their own lives, picking out what they want to disclose to the researcher. Negotiating access and consent is an ongoing activity. The researcher must continuously explain the purpose of the study and the reasons for their presence (Tjørnhøj-Thomsen and Whyte 2008). Access is an integral part of the social relations and forms of exchange that an HTA researcher enters to gain empirical insight into the patients' experiences with a specific health technology (Tjørnhøj-Thomsen 2003). In addition, researchers (and HTA bodies) can gain important insights from reflecting on the motives that patients may have for participating in an HTA as well as from the barriers they set up (ibid.). Getting access also implies ethical approval, i.e. obtaining informed consent and ensuring confidentiality. Research must adhere to national and international guidelines regarding the risks and benefits associated with the study as well as protection, storage and destruction of the data provided by the participants. In Denmark, as in other EU countries, any research involving personal data must comply with General Data Protection Regulation (GDPR) (<https://eur-lex.europa.eu/eli/reg/2016/679/oj>) and undergo evaluation by the Danish Research Ethics Committees. Additionally, researchers are strongly recommended to submit their studies for assessment by the Research Ethics Committee at their respective universities.

10.3 Participant Observation

Participant observation entails two interrelated forms of research activities. *Participation* implies engagement with the people and activities under study whereas *observation* implies distance and a more passive stance from the researcher.

The researcher performs both activities in turn and to different degrees. The idea is that by being there and taking part in people's lives, the researcher gains an opportunity to experience and understand what the social world looks like and how the context (e.g. the ward, the hospital, the home, the organisational division of labour, the technology etc.) frames the ways in how people think and act (Spradley 1980; Tjørnhøj-Thomsen and Whyte 2008; Atkinson 2015). What the researchers observe, sense and thereby come to know (Wolcott 1994) depends, therefore, on the nature of their participation, the place they are allotted in the empirical context and the social situations and activities they engage in.

Observation does not mean merely to *watch*, *see* or *look* at something — but also includes other senses such as smell, taste, hearing and touch. It nevertheless requires methodological reflections about from where, what and how the researcher observes (or senses), and the researcher's aims (Tjora 2006; Wolcott 1994). For that reason, the preparation of an observation guide may be just as important as preparing an interview guide. Such a guide can support the researcher to strategize carefully about what to observe, with what purpose and from where (activities, surroundings, symbols, social interactions, who-does-what, etc.) The researcher may, for example, first apply an *open* observation strategy to provide a broad-spectrum insight into what is going on in a particular health technology setting and the characteristics of the setting. Thereafter, one may apply a more focused strategy by observing particular social situations including the interplay between places, actors, and activities (ibid., Kawulich 2005).

10.3.1 Social Situations

The first step in doing fieldwork is to locate a social situation (or situations) that is relevant for the study and accessible for the researcher (Spradley 1980). In an HTA, such a situation could be a patient in home dialysis or patients in a rehabilitation programme. Spradley identifies three major features of all social situations *place*, *actors* and *activities*.

Box 10.1 Describing a Social Situation (Spradley 1980)

Place relates to the physical place or places of study (including physical surroundings, social atmosphere, objects, furniture and decor).

Actors relate to the people who are present in the situation.

Activities is a set of acts between the people who are present (e.g. communication, health professionals supporting patients with daily hygiene and shared decision-making).

Paying empirical attention to these features is an important guide for the participant observer in HTA situation. It emphasises the importance of not only defining the relevant situation for the HTA, but also of following the informants in and

through different situations. Thereby, the researchers can learn how the patients' experiences, views, perceptions, and preferences change with situation, context and time. Spradley adds six more dimensions of social situations, which can be used to guide participant observation in an HTA, thereby enhancing the researcher's possibility of generating nuanced and rich data.

Box 10.2 Six Dimensions of Social Situations (Spradley 1980)

Objects: physical things that are present (e.g. medical devices, apps monitors or screening apparatus)

Acts: single actions that people do

Event: what characterises the set of related activities that people carry out

Time: the sequencing that takes place over time

Goal: the thing that people are trying to accomplish

Feelings: emotions expressed

In summary participant observation can provide knowledge about patients' everyday life with a specific health technology, which they may take for granted in everyday routine situations and forget to talk about in interviews or do not find relevant. If the researcher wishes to obtain knowledge of how patients (or their carers and relatives) talk about staying in hospital, live with a rare disease, use a new telehealth app or a new medicine, interviews or focus group discussions are relevant choices of methods. But if researchers also want to understand how the patients, carers, or relatives act, interact and live with a specific condition and manage different health technologies, knowledge about context becomes important (Hansen et al. 2011). Such knowledge is best obtained by participant observation.

10.4 Ethnographic Interviewing

During fieldwork, researchers engage in informal and spontaneous conversation with people about what they are doing and thinking. This approach provides rich and relevant data about diverse perspectives. Ethnographic interviewing is distinct from other forms of interviewing, because it is conducted in and informed by the context of the fieldwork. It is more formal and planned and carried out with specific informants. While it shares some characteristics with everyday conversation, an ethnographic interview has an *explicit purpose* defined by the researcher and follows specific agenda. For instance, repetitions and asking questions are integrated parts of the process (Spradley 1979, pp. 55–57).

Generally, interviewing is important in exploring how patients articulate experiences, perspectives, attitudes, needs and desires in relation to a specific health technology. Interviews can be conducted as a 'face to face' individual interview, walk-and-talk interviews (Carpiano 2009; Garcia et al. 2012), focus group interview, telephone and/or

online interviews. The forms of interviews chosen depend on the research questions, the topic, the conditions and preferences of the informants. In order to conduct an ethnographic interview, the researcher needs to prepare thoroughly and consider the interview context in relation to the distinctive characteristics of the ethnographic fieldwork. This can be achieved by developing an interview guide or arranging the interview around a theme, a picture, a photograph or a video. Ethnographic interview may also be open or unstructured taking the form of a sickness or life history interview (Spradley 1979; Denzin and Lincoln 2011). Interviews may last for a short period of time, for instance, 10–20 minutes, or they may last for hours (Jeppesen et al. 2015).

The researcher can choose between different techniques to record the interviews. In most cases the interview is recorded in a digital form. This gives the researcher an opportunity to engage in the actual interview situation with full attention.

10.4.1 Ethnographic Questions

It may be relevant to create an interview guide to maintain focus in the ethnographic interview. We suggest that such a guide be based on Spradley's description of the ethnographic interview (ibid. p. 67). This would begin with greetings, explaining the purpose of the interview, checking informed consent, outlining the agenda and right to withdraw then asking a range of questions.

10.4.1.1 Descriptive Questions

Spradley divides descriptive questions into five major types of questions: *grand tour*, *mini tour*, *example questions*, *experience questions* and *native-language questions* (ibid. pp. 86–87). In HTA with a focus on patient involvement, we suggest that the first four types of questions are relevant.

10.4.1.2 Grand Tour Questions

Typically, grand tour questions ask the person to generalise, including patterns of events, typical situations and typical subjects. These questions often start with phrases such as 'Could you describe ...', for example, 'Could you describe a typical day at home using your telehealth app?' or 'Could you tell me about how you usually contact the nurse' (ibid. p. 87).

10.4.1.3 Mini Tour Questions

When the patient answers, comments and discusses grand tour questions, a richness of description will often open up for an in-depth investigation of more detailed aspects of the experience (ibid. p. 88). If the HTA researcher, for instance, asked the

patient during the grand tour ‘Could you describe a typical day at home using your telehealth app’, this could lead to a mini tour question, such as ‘Could you describe how you used your telehealth app yesterday?’. Grand tour and mini tour questions are in many ways similar to each other. The difference is that mini tour deals with smaller units of experience than grand tour questions.

10.4.1.4 Example Questions

In some HTAs the grand and mini tour questions will be sufficient. However, using phrases such as ‘Could you give me an example of what you do if your telehealth app does not work?’ may often be very helpful to make the patients tell more and even narrate their answers.

10.4.1.5 Experience Questions

In most HTAs concerned with patient involvement, the researcher or the patient will be interested in talking about their experiences with a treatment, a new device, symptoms, etc. The researcher may, for instance, open such a dialogue by saying ‘Could you describe how you feel about using the telehealth app at home four times per day?’ or ‘Could you tell me about how you experienced the information session about the app at the outdoor clinic?’

Summarising, the ethnographic interview always depends on contextual dimensions, such as the interview setting, the behaviour of the interviewer, the responses from the interviewee, the interview length and the positions and roles undertaken by the interviewer and the interviewee. It is important to consider that it is perhaps through the interview the patient becomes aware of their emotions, views, needs and life challenges and puts them into words. Therefore, the knowledge produced through ethnographic or other kinds of interview is co-constructed knowledge based on and through the researcher’s intervention.

10.5 Field Notes

During a fieldwork with participant observation and ethnographic interviews, the researchers’ experiences, and observations are recorded and stored as *field notes*. Field notes are descriptions of social situations including description of places, actors and activities and a record of what people say and do and how they interact (Emerson et al. 1995). Field notes are part of the empirical material that subsequently becomes the object of analysis, and they often provide good cases or illustrations for the analytic points in the HTA assessment report (Tjørnhøj-Thomsen and Whyte 2008, p. 109). It is seldom possible to take comprehensive notes during participant observation or interviews. The researcher may only have time and space for scratch notes or writing down keywords and memos. Later it is important to

write a full and coherent account of the situation. Taking notes is also an important tool for critical reflection. When re-reading the notes, the researchers may ‘discover that there are aspects that they must ask about in greater depth at a later point’ (ibid. p. 112), or the notes may spark ideas for theoretical and analytical work. It may be useful to distinguish between the following three forms of notetaking (Bernard 1994).

Descriptive notes, where the researcher makes thorough and uninterpreted descriptions of the setting and the people under study, including what people do and say. These notes are used in making so called ‘thick descriptions’.

Methodological notes, in which the researcher registers and reflects on whether the chosen methods prove relevant or useful in the HTA setting or not, and in what ways and why they can be adjusted during the study. They may enhance methodological reflections and knowledge and help to create transparency and validity.

Analytic notes, which concern concepts and theoretical ideas or perspectives that emerge during the fieldwork and may be used in the ongoing analysis.

10.6 Considerations on the Analytic Process

In ethnographic research, data generation and analysis are closely interlinked, which implies that the analysis begins during fieldwork (Atkinson 2015, pp. 14–15). Writing is an integral part of the analysis. It is, for instance, through writing, and (re-) reading field notes that ideas for the analysis and sense-making surface and can inform further research to contribute to the HTA. After the fieldwork the analysis requires that the researcher reads through the material (i.e. transcribed interviews and field notes) several times to get an overall impression of what is at stake and to become familiar with the empirical material. The researcher thus engages with the material by sorting, organising and coding it with the purpose of developing, conceptualising more general cross-cutting themes. Various software tools, such as NVivo, may assist the analytical process. The analytical work described above is often referred to as a *thematic* analysis (e.g. Braun and Clarke 2021). The identification of themes may be driven by the HTA questions and theoretical interests; however, it is crucial also to include unexpected or contradictory themes and findings. If feasible within the specific HTA, it can be relevant to make comparisons between findings from other studies and bring them in dialogue with relevant theories.

10.7 Discussion

As shown in this chapter ethnographic fieldwork, participant observation, and interviewing provide robust in-depth research into patient aspects that can inform HTA. Patients are people with experiences of illness and health technologies, experts in their own particular life situation, and they also involve themselves in research for different reasons (which may also be relevant to explore). Knowledge

of patients' own lived experiences is crucial in an HTA. However, the notion of patient involvement calls for further critical and reflexive consideration.

Firstly, it is important to keep in mind that patients do not form a homogenous group and may have diverging interests and positions as experts in their disease or representatives of a disease or a disability.

Secondly, it may be important to consider the differences between patients' and researchers' specific contributions to an HTA. We raise this, because we sometimes meet the assumption that conducting interviews and participant observation does not require special training and expertise when compared to, e.g. statistical or epidemiological research. The drive for patient participation, such as patients conducting interviews or joining the analytical work may result in a de-valuing of the expertise and yearlong training (in e.g. data generation, critical analysis, and theory) qualitative researchers brings to the field.

Thirdly, researchers working in HTA settings and among people or issues with which they are familiar or have prior knowledge need to be attentive not to take the familiar for granted. There is a risk that researchers who are familiar with the setting forget, neglect or avoid asking questions to what seems obvious, thereby leaving cultural or setting-specific notions or assumptions about the specific HTA implicit and unexplored.

Finally, we suggest—with inspiration from the Danish anthropologist Kirsten Hastrup—that researchers involved in HTA consider three aspects of knowledge and knowledge production in the HTA: namely *critical conceptual work*, *relational dimensions*, and the *selective and reductive characteristics of knowledge* (Hastrup 2004).

Critical conceptual work means that the researcher needs to reflect on the use and meaning of concepts, words, and categories. The knowledge produced in an HTA always reflects the meanings inherent in the concepts used, whether it refers to researchers' perspectives or patients' perspectives. Therefore, concepts, categories and words have an impact on the knowledge production (Hastrup 2004). Also, the notion of technology needs to be defined and described in an HTA, since not only cultural perceptions of 'technology' but also the nature of the particular technology (e.g. whether it is medical equipment or rehabilitation) under assessment will influence the design and choice of methods used.

Relational dimensions are another important aspect to consider. Hastrup states that knowledge "has become—and must be—acknowledged (implicitly, at least) as *relational*, both in the sense that it attaches itself to relations between people or between people and objects and in the sense that it emerges within a dialogical field" (Hastrup 2004, p. 456, original italics). The ethnographic researcher gains knowledge, as also shown in this chapter, by engaging in social relations in the field (among the people under study) as well as in the academic institution. That is why knowledge can never be objective in the classical meaning of the concept; it is always partial, positioned and situated (Harraway 1988).

The selective and reductive characteristics of knowledge means that knowledge is always both reductive and selective: "It is reductive because it renders empirical complexity and messiness in clear, but therefore also more limited, propositions

about the world: It is selective, because for it to be knowledge, it must disregard some information” (Hastrup 2004, p. 456). A critical reflection and discussion of the knowledge produced in HTA about, for instance, patients’ perspectives and experiences needs therefore to address the reducing and selecting activities embedded in the knowledge production. If, for example, a patient representative is participating in an HTA, the researchers must reflect on to what extent and how the knowledge gained from this individual representative represents a larger group of patients’ experiences with the technology. Hansen and Street (2017) discussed in the first edition of this book some of the different terms used in relation to patient involvement in HTA including patient as experts, patient advocates or representatives of a disease and patients as consumers. It is, therefore, important that the HTA researchers continuously raise a critical and reflective stance towards the knowledge produced through, for instance, ethnographic fieldwork, participant observation and ethnographic interviews. This requires that the HTA researchers develop the necessary skills for undertaking this specific research design.

10.8 Concluding Remarks

Ethnographic fieldwork has not been extensively utilised in relation to HTA, likely because it is considered time-consuming or because its great potential within the field of HTA is still not fully recognised. In this chapter, we have attempted to demonstrate that ethnographic fieldwork can provide important insights into patients’ experiences of their illness or use of a health technology, thereby contributing with important new knowledge to inform HTA.

Firstly, because ethnographic fieldwork including participant observation and ethnographic interviews may reduce the methodological limitations of only conducting semi-structured interviews in obtaining patients’ experiences and perspectives by providing nuanced insights into what people do and how they interact with others and the surroundings in particular contexts.

Secondly, because ethnographic fieldwork optimises the possibilities for ethically sound involvement of the patients.

Thirdly, because ethnographic fieldwork can provide nuanced, comprehensive, and scientific insights to improve HTAs.

It is crucial to explore how patients (and their carers) act, react and interpret health technologies. Patients’ perspectives and experiences are emerging, relational and shifting. Therefore, there is a need for enhancing methodological and epistemological reflections and discussions about future development of ethnographic fieldwork in relation to HTA and patient involvement. The potential use of ethnographic fieldwork will be highly relevant in relation to the assessment of new screening procedures, telehealth solutions and collaboration between different sectors such as hospital, municipalities, and general practice. Furthermore, ethnographic fieldwork would be of importance for exploring how technology is working in local settings.

Acknowledgements Tine Tjørnhøj-Thomsen and Helle Ploug Hansen have written this manuscript together and contributed equally to the different sections in the manuscript. The authors would like to acknowledge comments received from the editors. All views expressed and any errors are entirely the responsibility of the authors.

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Chapter 11

Qualitative Evidence Synthesis



Andrew Booth and Kate Fryer

11.1 Introduction

Qualitative evidence synthesis (QES), also known as qualitative systematic review or qualitative meta-synthesis, offers a way to present patients' attitudes, beliefs, feelings and experiences as originally captured by individual qualitative research studies (Flemming and Noyes 2021). By using multiple studies, rather than a single study, researchers are able to avoid being over-influenced by that isolated study. In representing multiple perspectives from different contexts they can potentially contribute to the transferability of findings.

The experience or perspective of even one patient can be influential within the context of an important healthcare decision. By harnessing multiple patient perspectives, where diverse opinions and attitudes are organised within a single qualitative research study (Tong and Craig 2016), and, then, brought together with multiple similar studies within a synthesis, a research team is able to tap into multiple diverse patient values, experiences and preferences. True, multiple patient perspectives could be collected by commissioning original primary research that studies those targeted by a new treatment or programme. However, that costly alternative may take a prohibitive amount of time; a QES trades the relevance of data from a specific context for an understanding of the perspectives of numerous stakeholders from diverse contexts (Booth et al. 2019a). In recent years, several health technology assessment (HTA) bodies have mobilised the collective richness of multiple qualitative studies. Working from an assumption that, at least to a certain

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extent, qualitative research findings are ‘transferable’ (Finfgeld-Connett 2010) they have welcomed emerging methods that have produced such syntheses ‘rapidly’ without losing the rich insights to be gained through the QES approach (Majid and Weeks 2021).

The term ‘qualitative evidence synthesis’ has been popularised by the Cochrane Qualitative and Implementation Methods Group as a distinctive label applied broadly to the synthesis of very different types of qualitative data, extending to patient postings on online bulletin boards or narrated patient real-life experiences recorded from interviews (Flemming and Noyes 2021). While commonly the term refers to systematic reviews of research that have used recognised methods of data collection and analysis of qualitative data, as documented in journal articles, book chapters and dissertations and theses, developments and expansions in methods continue to unfold.

This chapter explores the versatile ways that QES can be applied within HTA and how patients can participate in this research. It looks at different types of QES and how they have been used within time-sensitive decision-making contexts such as HTA. The chapter concludes by looking at how data from qualitative research can be placed alongside quantitative data (for example from clinical effectiveness or cost effectiveness evaluations) in an HTA.

11.2 Developments in Qualitative Evidence Synthesis

QES started to appear in the literature in the latter half of the 1990s when a landmark meta-synthesis sought to extend “the analysis of individual research studies beyond individual experience to incorporate dominant system beliefs and health system ideologies” (Paterson et al. 1998, p. 57). A decade later a chapter on qualitative evidence appeared in the *Cochrane Handbook of Systematic Reviews of Interventions* (Noyes et al. 2008). However, the first stand-alone Cochrane QES was not published in the Cochrane Library until 2013. While dispersed adoption is evidenced in HTA bodies such as the National Institute for Health and Care Excellence (NICE) in the UK, Canada’s Drug Agency (CDA-AMC) and the World Health Organization (2021) such progress has frequently faced challenges from the apparent discomfort of those more familiar with the quantitative paradigm when encountering the interpretative nature of qualitative evidence—with the HTA review team, in effect, offering interpretations (by the team) of interpretations (by the primary authors) of the experiences of research participants.

At the beginning of the 2020s, interest expanded to include rapid qualitative evidence syntheses. HTA organisations were at the forefront of this rapid approach to use of synthesized qualitative evidence (Ring et al. 2010), being attracted by the timely incorporation of patient experiences within the decision-making process (Majid and Weeks 2021; Campbell et al. 2019). During the COVID-19 pandemic the first Cochrane rapid QES was produced within 25 days as part of the WHO

response (Biesty et al. 2020). Several HTA bodies now include rapid QESs within their HTA reports. Such is the prevalence of QESs that it has become feasible to produce overviews of QES (mega-syntheses) where QESs, not primary studies, are subject to synthesis and analysis (Carroll et al. 2022).

It is appropriate at this juncture to see the next phase in QES development signalled by the planned publication in 2026 of the first ever *Cochrane-Campbell Handbook of Qualitative Evidence Synthesis* (Noyes and Harden 2026, In press). In particular, this forthcoming handbook displays a pervasive approach to both patient and public involvement and to equality, diversity and inclusion. It also manifests a methodological concern with reflexivity and with purposive sampling of studies.

11.3 Using QES to Build Reliable Patient-Based Evidence in HTA

There are at least three main ways in which a QES, which can be used for multifarious HTA purposes (e.g. acceptability and implementation) and perspectives (e.g. policy makers, clinicians, public) can be harnessed to provide robust patient-based evidence to inform decision-making:

1. a QES may be a mechanism by which to present evidence about patients' experiences and perspectives of relevance to an HTA
2. patients may participate in the QES and provide insights that impact other parts of the HTA process
3. a QES may be an appropriate design for studying the evidence base around patient participation processes and methods.

11.3.1 *Spotlighting Patients' Perspectives*

From their earliest years, QES in health care have offered a vehicle for otherwise disenfranchised patient groups (Booth 2016; Park et al. 2020). As Toye and colleagues observe: "Affirming a person's experience and allowing an empathetic interpretation of their story is not an adjunct [i.e. optional extra], but integral to care" (Toye et al. 2013, p. e835).

As with our featured Effectiveness of Interventions For Fatigue in Long Term Conditions (EIFFEL) synthesis (See Box 11.1), the National Health Service (NHS) HTA programme of the United Kingdom (UK) makes frequent use of QES methods. For example, a very large team of researchers produced the EMPoWER evidence synthesis to explore the needs of very young children with mobility limitations (Bray et al. 2020). Spanish HTA reports, published as journal articles, include qualitative evidence syntheses that capture the prenatal perspectives of

women living with type 1 diabetes (Toledo-Chavarri et al. 2023) and explore the implementation of care for people with generalised anxiety disorder (Toledo-Chávarri et al. 2020).

The Swedish Agency for HTA and Assessment of Social Services (SBU) is another champion of QES methodology as evidenced in reports on conflicting attitudes between clinicians and women regarding caesarean section (Johansson et al. 2023) and experiences of eating disorders from the three diverse perspectives of patients, family members and health care professionals (Gustafsson et al. 2021). Other productive agencies for this stream of evidence are CADTH (now Canada's Drug Agency—AMC) in Canada, as exemplified for the nuances of experiences with the extramedical use of buprenorphine (Sud et al. 2022), and the Scottish HTA agency with the apt title “*placing the patient at the centre of chronic wound care*” (Fearn et al. 2017).

11.3.2 Enhancing the HTA Process and Product

Patients' perspectives are crucial in evaluating health technologies (Gagnon et al. 2021). While funders increasingly require patient participation in research, many studies engage in superficial involvement (a ‘tick box exercise’) rather than meaningful participation (Mitchell et al. 2023). UK NIHR Include guidelines emphasise quality patient participation throughout research processes, challenging researchers to find diverse participants and effectively communicate concepts (Witham et al. 2020). Findings from an updated systematic review show that patient participation in HTA typically occurs through consultation rather than direct involvement (Gagnon et al. 2021). Throughout this chapter, we reflect upon how patients can be included in each stage of the QES process.

Within HTA, QES is considered alongside clinical effectiveness, network meta-analysis, and cost-effectiveness reviews. The EIFFEL study exemplifies this approach, using patient focus groups to guide QES research on fatigue across medical conditions (Leaviss et al. 2025). These groups meet three times to discuss methodology, interpretation, and outputs, with sessions co-facilitated by researchers and individuals with lived experience. Mixed approaches to synthesis continue evolving, either integrating quantitative and qualitative data separately or reviewing mixed-methods primary studies.

Within an HTA context, a QES is generally considered alongside other components such as a review of clinical effectiveness, a network meta-analysis and a review of cost effectiveness. HTA may extend to ask ‘what works for whom under what circumstances’ using a specific methodology, such as realist synthesis. Box 11.1 offers a recent example of such a compound review commissioned by the UK National Institute of Health and Care Research (NIHR) HTA Programme.

Mixed approaches to synthesis remain under development; mixed methods reviews may juxtapose quantitative trial evidence with qualitative data in

Box 11.1 EIFFEL: Patient Involvement in Interventions for Fatigue (UK NIHR HTA Programme) (Leaviss et al. 2025)

A patient focus group, conducted within a larger health technology assessment, is used to guide an accompanying qualitative evidence synthesis by helping researchers to understand how people with lived experience view the relationship between fatigue and different medical conditions. This guides the larger programme in deciding when it is appropriate to combine evidence from different conditions. Secondly, it helps researchers to understand how people with lived experience make sense of the evidence from research. The wider programme team is thus able to make recommendations for the QES based on the acceptability of different treatments.

Six focus groups represent diverse medical conditions and diverse communities. Each group meets three times: first for a general introduction to the process, then to discuss methodological decisions in relation to the conduct of the evidence synthesis, and finally to contribute to the interpretation of findings and content of outputs.

Focus groups are co-facilitated by a researcher with expertise in PPI work and two study co-applicants with lived experience of fatigue. Groups use a blend of face to face and online (but not hybrid) format chosen to maximise representation of people with different needs and capabilities.

Analysis is at two levels: first to meet the specific PPI needs of the larger evidence synthesis project and second to provide deeper understanding, transferable beyond this study about the perception of fatigue across different medical conditions.

separate syntheses and then seek to integrate the two types of evidence, or, alternatively, they may seek to review only mixed methods primary studies (Noyes et al. 2019).

11.4 Undertaking a Qualitative Evidence Synthesis

Although several methodological choices exist in relation to the actual synthesis process most QESs share common review phases that resemble, at least to a certain degree, corresponding phases in the systematic review of effectiveness data (Table 11.1). The following section summarises the main features of this process; expansive detail of each phase can be found in the *Cochrane-Campbell Handbook of Qualitative Evidence Synthesis* (Noyes and Harden 2026). These QES, as described, are either undertaken within an HTA body by an internal review team (e.g. NICE within the UK) or by contracted external researchers within a national HTA Programme (e.g. NIHR within the UK), henceforth identified singly as ‘the review team’.

Table 11.1 Comparison of the phases of a qualitative evidence synthesis and a systematic review

	Qualitative evidence synthesis	Systematic review
1.	Development of clearly formulated review question	Formulate the problem
2.	Scoping the literature	
3.	Identifying included studies	Literature search
4.	Initial assessment of study reports	
		Data extraction
		Critical appraisal of studies (quality assessment)
5.	Analysis and synthesis (at a descriptive, study level)	
6.	Preliminary synthesis (at a descriptive thematic level)	
7.	Full synthesis (at an analytic thematic level)	Data synthesis
8.	Dissemination	Presenting results (writing the report)
9.	Whole process considerations (reflexivity, patient focus, equality, diversity and inclusion)	

11.4.1 *Development of Clearly Formulated Review Question*

Reviews usually start with a clearly formulated review question. This helps to define the size of the task logistically and the scope of the topic conceptually. For a review of clinical effectiveness the epidemiology-based four-part Population-Intervention-Comparison-Outcomes formulation is typically sufficient (Eriksen and Frandsen 2018). However, qualitative evidence synthesis tends to require more components. A five component formulation, Setting-Perspective-Interest, phenomenon of-Comparison-Evaluation is frequently used (Riesenberg and Justice 2014) although complex interventions may benefit further from the introduction of a Time element and a distinction between the immediate context for an intervention (Setting) and the wider socio-political-legal-environment Context. The seven component PerSPE(c)TiF formulation is proposed for use in complex interventions as typified by WHO public health guidelines (Booth et al. 2019b). A clearly formulated question also helps in determining the eligibility criteria, the search strategy (Frandsen et al. 2021) and the selection of the data extraction elements.

Two characteristics are specific to question formulation for QES; first, the review question is more a ‘compass’ rather than the ‘anchor’ associated with effectiveness reviews (Flemming and Noyes 2021). As the review team follows up initial leads, they may unearth further lines of inquiry—in a similar way to inquiry in primary qualitative research. Secondly, a review team may be interested in qualitative data that extends beyond the experience of an intervention, particularly as a health technology may be novel and qualitative research scarce. The QES may have to examine patient experiences of a condition, both with and without any intervention, and may consequently be broader in scope than the effectiveness question (Lorenc et al. 2012).

Patient participation is often critical at this stage; to explore patient priorities and to ensure that the questions identified by researchers reflect patient concerns. There

is a mismatch between what generally receives funding and what patients, carers, and the public would like to see examined (Crowe and Giles 2016). Patients may also be able to aid setting the parameters of the questions, by reflecting on their lived experience of health conditions (Gierisch et al. 2019). The James Lind Alliance undertakes priority setting exercises on a large scale, with methods adapted by individual initiatives such as Born in Bradford (Rahman 2022), and the Deep End Research Alliance (<https://sites.google.com/sheffield.ac.uk/dera/research-priority-setting?authuser=0>), for smaller scale exercises with underserved communities.

11.4.2 Scoping the Literature

Increasingly it is recognised that, unlike for systematic reviews of effectiveness, it is not always necessary to identify and synthesise all studies in order to create a sufficiently rich interpretation of a phenomenon of interest, whether that be a condition or an intervention. It is argued that ‘more of the same’ studies add little to a synthesis and so searching for a diverse sample is to be privileged. This interest in the ‘disconfirming case’ alongside other sources of variation opens up a full array of sampling methods from qualitative research (Benoot et al. 2016).

Scoping of the literature allows a review team to map the important variables for context, population and time and place characteristics. A qualified information specialist can identify key data sources and determine whether included studies belong to a single discipline (requiring similar databases e.g. MEDLINE, EMBASE, CINAHL, PsycInfo) or multiple disciplines (requiring diverse databases to represent each discipline and multidisciplinary databases e.g. Scopus and Web of Knowledge). The potential contribution of book chapters, theses and other types of grey literature should not be overlooked. Regional databases or institutional repositories may also be important for region-specific topics (Stansfield et al. 2012).

11.4.3 Identifying Included Studies

Once the HTA review team has articulated the review question and set its conceptual. Logistical and terminological limits, the team proceeds to formal identification of relevant literature.

The unrivalled coverage of MEDLINE means that a review team may identify up to 90% of included studies from a well-constructed MEDLINE search (Booth 2016). However, recent research suggests that Scopus may well occupy first place in limited ‘high-yield’ database searches (Frandsen et al. 2019). Retrieval of qualitative research often proves challenging. CINAHL is also considered essential while EMBASE, PsycINFO, Sociological Abstracts and Social Sciences Citation Index (Web of Knowledge) feature prominently in QES search methods.

Certainly, it is important not to rely on conventional subject searching of bibliographic databases but to use numerous supplementary techniques such as backward and forward citation searching, handsearching of relevant journals such as *The Patient; Health Expectations; Value in Health; Social Science and Medicine; Culture, Medicine, and Psychiatry; Research Involvement and Engagement*; and *Sociology of Health and Illness* and contact with authors and experts (Papaioannou et al. 2010). Websites of national patient organisations may also yield useful information.

‘Clusters’ of related studies add thicker contextual detail and a richer conceptual understanding (Booth et al. 2013b). Qualitative sources may include pilot studies, feasibility studies and process evaluations as well as ‘sibling’ studies that run alongside a higher-profile trial. Health Services Research PubMed Queries (<https://www.nlm.nih.gov/nichsr/hedges/search.html>) offers a rapid search facility for scoping qualitative research topics or related topics. Supplementary search strategies (citation searches, co-citations and ‘related articles’ searches) increase retrieval yield and therefore should be included, whenever time allows (Frandsen and Eriksen 2023).

Given that quantitative studies outnumber qualitative studies by as many as 10:1 it is helpful to choose an appropriate search filter that seeks references with a high number of qualitative-specific words or phrases. For example, phrases such as ‘semi-structured interview’ are commonly encountered in the context of qualitative research. Predesigned filters exist for retrieving qualitative research studies from the main databases (Wagner et al. 2020). However, it may be equally useful to use hedges of key terms associated with a patient and public involvement (Rogers et al. 2017) or quality of life (InterTASC Information Specialists’ Sub-Group (ISSG) 2024). Short lists of qualitative terms may perform comparably to an expansive list (Frandsen et al. 2019). However, this requires testing across a greater range of review topics and literatures. The SuRe Info: Summarized Research in Information Retrieval for HTA Google site (<https://sites.google.com/york.ac.uk/sureinfo/home>) maintains an updated digest of current knowledge about qualitative sources and strategies (Booth et al. 2021).

Some research teams have involved patients in screening titles and abstracts to establish whether they should be included in the final review, and to observe on how they might be categorised. Careful consideration needs to be given as to how research studies are presented to patients, so that the terminology and style is clearly understandable without sacrificing nuance and rigour. For example, the Fairsteps study developed ‘vignettes’ from studies, to aid both practitioners and patients to decide whether or not a study should be included within the final review (Jackson et al. 2023).

11.4.4 Initial Assessment of the Study Reports

The fourth phase involves an initial assessment of study reports. After preliminary reading and re-reading, the QES team forms a picture of the literature and how it is structured. Theories, either explicitly stated or implicitly referenced, start to become

apparent (Booth and Carroll 2015b). Such conceptual frameworks may become a useful vehicle for data extraction through framework synthesis (Booth and Carroll 2015a).

At this stage review teams typically undertake quality assessment of the included studies using one from over a 100 available checklists. The Critical Appraisal Skills Programme (CASP) checklist, designed for assessment of a single paper by clinicians not for use in syntheses, has been used widely (Long et al. 2020). In 2024, a new tool, CAMELOT, for use alongside GRADE-CERQual was launched for further user testing and ongoing refinement (Munthe-Kaas et al. 2024).

11.4.5 Analysis and Synthesis

Choice of synthesis is a key decision for any HTA review team. It is determined how much time the team has and whether they are experienced in qualitative research and synthesis. QES methods are plentiful with over 15 different approaches to synthesis having been documented. The RETREAT criteria (Box 11.2) have been developed to help review teams choose the most appropriate method of synthesis (Booth et al. 2018).

Box 11.2 Considerations When Selecting a Method of Qualitative Evidence Synthesis (RETREAT Mnemonic)

Review Question

Epistemology

Time

Resources

Expertise

Audience and Purpose

Type of Data

Cochrane has identified three main synthesis methods from which thematic synthesis and framework synthesis are believed to be the most straightforward and most amenable to rapid approaches (Noyes et al. 2018). Meta-ethnography has an established pedigree but may not be suitable for producing recommendations for practice. Essentially, it seeks to interpret studies rather than simply aggregating them, with the intent being to generate a new theory or ‘line of argument’ to explain all the studies (France et al. 2014). The Campbell Collaboration has accepted a further method, meta-aggregation although this has been criticised for not being sufficiently interpretative.

Constant comparison is used to identify patterns and similarities across reports. Refutational findings must be reconciled (Booth et al. 2013a). At this point the

review team may conduct quality assessment, either using a single generic assessment tool or checklist or a battery of checklists designed for individual types of study (Carroll and Booth 2015). They must consider the extent to which the synthesis and its findings are based on robust qualitative studies (Carroll et al. 2012).

Patients can contribute to the interpretation of findings. Commentators attest to the value for the evidence analysis process in capturing patient values and experiences while, at the same time recognising that successful involvement faces key organisational challenges such as time, financial resources, and expertise required to communicate with the patients (Abelson et al. 2016).

11.4.6 Preliminary Synthesis

Preliminary synthesis (stage 6) involves organisational procedures such as categorising, tabulation and the creation of mind maps. Patients may inform this preliminary work, as in the previously featured EIFFEL study, where patients supply observations on perceived similarities or differences between their experiences of fatigue in different medical conditions and thus to influence how data ultimately is organised and presented (Box 11.1). The review team explores relationships both within and between studies. Ultimately the hands of the review team may be tied to choice of method based on whether available data is conceptually rich and contextually thick. If this is the case, then a greater number of choices is available including meta-ethnography. Where qualitative data sources offer minimal data, as in thin case study reports published in professional journals, the potential for a rich interpretation is compromised. Thin data is unlikely to be able to sustain meta-ethnography. More superficial approaches, such as thematic synthesis, become an appropriate alternative.

Typically, an HTA review team defaults to thematic synthesis unless a framework (from an included study or conceptual paper) is readily identifiable (Dixon-Woods 2011). Outputs from some methods of synthesis (including *Thematic Synthesis* and *Framework Synthesis*) are “more directly relevant to policymakers and designers of interventions than the outputs of methods with a more constructivist orientation (*such as meta-ethnography*) which are generally more complex and conceptual” (Barnett-Page and Thomas 2009, p. 9). Should the data be rich enough and thick enough then the team can switch from thematic synthesis to meta-ethnography, using the work already completed from their preliminary synthesis as the first stage of a more complex and interpretative meta-ethnography.

11.4.7 Full Synthesis

All QESs, regardless of synthesis method, provide an accompanying narrative and supporting evidence tables. Typically, these start with data on the study characteristics within Excel or some spreadsheet alternative. The verbatim extracts from

participants and the accompanying observations from the study authors are either extracted into an extraction form or coded within a qualitative analysis package. The principal idea behind all synthesis is being able to ‘spot’ patterns—whether these are common findings across multiple studies or an isolated context or study which proves an exception to a ‘rule’.

11.4.8 Dissemination

HTA QES are typically produced as chapters within a larger HTA report with only a proportion of these proceeding to publication in peer-reviewed journals. Ultimately, the intended audience should shape the methods chosen for dissemination. Numerous options exist for visual presentation of data (Bianchim et al. 2024). It is important to consider how patients may be able to benefit from findings, and how best to present these. Plain language is essential, and creative use of illustrations and videos may be beneficial. Patients can contribute to producing plain language resources, and advise on channels of dissemination. The review team assesses the strengths and limitations of the review itself and of the body of included studies. Optimally, all stakeholders are consulted so that emerging findings become an organic product of knowledge co-creation. In recent years synthesis findings have been presented as first person statements, as poems and in the form of artworks, further emphasising the versatility of QES methods. However, stakeholders may not recognise the entirety of the synthetic findings given that they often possess only a fragmented, yet valid, perspective.

11.4.9 Whole Process Considerations

Throughout the process the multidisciplinary team brings together their different perspectives not for consensus, as for multiple reviewers in an effectiveness review, but for divergence and interpretive richness (Booth et al. 2013a). Reflexivity, and the related concept of positionality, require that review team members consider how their own role as researchers impacts on both process and product (Downe et al. 2019) and how they have positioned themselves in relation to the studies that they are reviewing. It has been recommended that reflexivity (See Chap. 3) be both prospective, at the beginning of the review, and retrospective in assessing how each person has contributed to the review (Glenton et al. 2022). Particular examples are available in the journals literature or within the Cochrane protocol and review QES template (Glenton et al. 2021). Even though the QES is iterative and recursive this does not exempt it from needing careful documentation of methods and decisions to increase confidence in the findings (Benoot et al. 2016).

The movement of QESs into HTAs and guideline production has served as a stimulus to for development of the GRADE-CERQual four-component approach

that assesses individual review findings for adequacy, coherence, methodological limitations and relevance. Assessments of the findings from a QES are designed to parallel GRADE, whereby quantitative findings have previously been assessed against four corresponding components.

11.5 Conducting Time-Sensitive QES

Prior to the COVID-19 pandemic, several HTA bodies were experimenting with rapid QES formats (Campbell et al. 2019). In actuality, limited time is only one factor that might require an alternative to a full conventional QES. For example, HTA bodies in low- and middle-income countries may decide to conduct reviews across fewer databases or may limit included studies to readily available full-texts. As public-funded agencies HTA bodies carry a strong moral imperative to ensure that all research into patient aspects is published for reuse and wider benefit. During the pandemic, large numbers of rapid reviews were undertaken, both quantitative and qualitative. Subsequently, expanded methodological guidance on rapid reviews has been produced by organisations such as Cochrane (Booth et al. 2024).

Overviews of QESs may offer an option for topics well-covered by existing QESs, such as obesity, HIV, and pain. Others have proposed the model of the living QES, periodically updated as evidence becomes more prevalent (Carmona et al. 2023). These three different variants (rapid QES, overviews of QES and living QES) are all considered in a separate chapter of the *Cochrane-Campbell Handbook of Qualitative Evidence Synthesis* (Noyes and Harden 2026).

11.6 Integrating Qualitative with Quantitative Data

Given the multiple facets covered by the core HTA model it is unsurprising to see that the integration of quantitative and qualitative data remains the outstanding methodological challenge. Such integration allows a review team to produce evidence products to inform complex HTA problems. Linking mechanisms, either overarching methodologies or practical methods, can be employed to harness patient perspectives to effectiveness data (Noyes et al. 2019) (Table 11.2).

These methods for integrating quantitative and qualitative data remain tentative with few worked examples. At present the QES as a stand-alone chapter within an HTA remains the dominant model and, in doing so, sidesteps methodological difficulties.

Table 11.2 Mechanisms for linking patient perspectives to effectiveness data

Methodology	Methods
Use realist synthesis (what works under what circumstances for whom)	Identify a conceptual framework from the literature as a scaffold for both types of data
Use meta-narrative to explore how quantitative and qualitative studies relate to disciplines and paradigms	Use an internally generated framework from stakeholder consultation
Use qualitative comparative analysis (truth tables) to explore how qualitative factors exert their influence on quantitative outcomes	Use a simple matrix to juxtapose themes (qualitative) with outcomes (quantitative)
	Use a programme theory as a causal map
	Construct a logic model and map quantitative and qualitative data against it.
	Perform subgroup analyses to bring quantitative and qualitative data together for particular subgroups

11.7 Discussion

Since the preceding edition of this book, QES has maintained its place as one of the fastest growing areas of research synthesis methodology. Adoption by health technology organisations, although steady, has not yet matched this accelerated pace. Valuing the patient experience in society requires incorporation of patient values and perspectives in the decision-making process. Without the essential contribution of patients many health technologies may not be fit for purpose or used as intended.

The relationship between QES and patient and public involvement should never be uni-directional—patients and the public can be actively involved in improving the design, analysis and salience of QES (Oliver et al. 2015)—even extending to co-production where feasible (Merner et al. 2023). While it may be very challenging to manage the timing, extent and nature of patient involvement within a QES, particularly within a time-constrained context, there is heightened realisation that this time is particularly well-spent. This chapter attempts to demonstrate and give examples of best practise in this area.

11.8 Conclusion

As with other methods of synthesis, QES is limited by the quality of reporting of primary studies. Primary research questions from included studies may only map partially to the broader concerns of a QES or such studies may only contribute minimal amounts of relevant data. Innovations, such as rapid QES and overviews of

QES continue to demonstrate the versatility and feasibility of QES methods. Grading recommendations in a GRADE-comparable manner using the GRADE-CERQual approach, and developing a transparent approach to quality assessment through CAMELOT may help to put qualitative synthesis on a comparable footing to its quantitative counterparts.

Acknowledgements Both authors acknowledge membership of the National Institute for Health and Care Research (NIHR) funded project Effectiveness of Interventions For Fatigue in Long term conditions (EIFFEL) project. Principal Investigators: Professor Chris Burton and Dr. Jo Leaviss.

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Chapter 12

Evaluation of Patient Involvement in HTA



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12.1 Introduction

This chapter aims to provide an overview of current practices regarding the evaluation of patient involvement in HTA. Patient involvement is considered an essential component of HTA and there are some guidelines and processes to support it within certain HTA bodies (e.g. Silva et al. 2022; Toledo-Chávarri et al. 2021), as outlined in Part III. With the increased involvement of patients in HTA processes, the question of assessing its impacts also arises. A previous survey among HTA bodies showed a scarcity of rigorous evaluations of patient involvement in HTA (Weeks et al. 2017). Other work explored the impact of patient involvement as reported by various stakeholders (Lopez Gousset et al. 2023). Evaluating patient involvement initiatives is crucial to strengthening the evidence base supporting the value that patient involvement brings to the HTA process and products, as well as its impact

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on decisions regarding health technologies and services, and ultimately on health outcomes. Evaluations of patient involvement in HTA also provide a mechanism to improve the way that patient involvement is done and experienced, and help determine good practices.

12.2 The Imperative to Evaluate Patient Involvement in HTA

In the last two decades, patient involvement has emerged as an imperative for more informed, transparent, accountable and legitimate decisions about health technologies (Facey et al. 2010; HTAi 2014; Werkö and Staniszewska 2021). However, its benefits remain hypothetical in the absence of formal evaluation of patient involvement in HTA.

A systematic review of 31 studies published between 2009 and 2019 (Gagnon et al. 2021) identified two main ways to involve patients or public in HTA. First, patients or their representatives are involved in research to generate evidence about their perspectives, experiences or preferences about a health technology or a clinical intervention. The second way is direct participation of patients in one or several steps of the HTA process: topic identification and selection, prioritisation, formulation and scoping of the evaluation question, evidence assessment and dissemination and implementation of HTA recommendations (see also Chaps. 3 and 4).

Evaluation is required to examine the process and impact of patient involvement in HTA from different stakeholders' perspectives (patients, HTA bodies, healthcare providers, managers, policymakers), but few studies have examined this to date (Gagnon et al. 2021; Gauvin et al. 2014; Mason et al. 2020). A key argument against evaluating patient involvement is that as users of healthcare, patients have the right to participate in decisions related to services provided and therefore should be involved regardless of the impact (Kamaruzaman et al. 2021; Staley 2015). Thus, there is a moral obligation to involve patients as the ultimate end users of health technologies (Esmail et al. 2015). Nevertheless, Staley (2015) suggests that evaluating patient involvement remains essential to improve how it is done and to ensure it has the desired impact, which could vary depending on the specific context and purpose of patient involvement. Furthermore, patients themselves are interested in seeing the impact of their participation to ensure the time and funds they invest are wisely spent and really make a difference in the process.

To date, few published studies report on the evaluation of patient involvement activities in HTA. An international survey of patient involvement in HTA (Weeks et al. 2017) found that only seven HTA bodies had evaluated their patient involvement activities. The dimensions that were evaluated were participant satisfaction, process and impact. A scoping review found only six studies that focused on the evaluation of patient involvement in HTA (Mason et al. 2021). Four of these studies assessed the contribution of patient experience to HTA recommendations, one assessed the impact of direct patient participation in HTA committees, and one evaluated the impact of multiple forms of patient involvement. Evaluation methods

included quantitative analyses of funding decisions, qualitative analyses based on interviews with HTA committee members and patient representatives, and reviews of HTA reports. The authors conclude that the evaluation approach depends on how the impact of patient involvement is defined.

In the systematic review by Gagnon et al. (2021), varied short-term impacts of patient involvement in HTA were identified. The most common result associated with patient involvement was raising awareness of HTA committee members about patient preferences to inform decision-making at different stages of the HTA process. Similarly, the Patient and Citizen Involvement in HTA Interest Group (PCIG) of the Health Technology Assessment International (HTAi) collected stories of impact from different stakeholders' perspective (Lopez Gousset et al. 2024). The most frequently reported impact, highlighted in 19 of the 24 stories, was the role of the patient perspective in enhancing the interpretation of data by HTA bodies, providing contextual understanding and identifying outcomes important to patients.

12.3 Frameworks and Considerations for the Evaluation of Patient Involvement in HTA

This section reviews current frameworks proposed for the evaluation of patient involvement in HTA, discusses some requirements for evaluation identified in the literature and reflects on associated challenges.

12.3.1 Frameworks to Evaluate Patient Involvement in HTA

Most studies evaluating patient involvement in HTA are carried out in the absence of an evaluation framework that would inform the choice of evaluation criteria (Mason et al. 2021). In that sense, a logic model could be useful, as it provides an organised representation of the different components of the intervention and the relationships between them (Kellogg Foundation 2004). Furthermore, the lack of frameworks specific to patient involvement in HTA makes difficult the articulation of evaluation goals. For instance, the mosaic of patient participation in HTA presented in Chap. 4 provides a comprehensive framework to guide patient involvement practices and could also inform the selection of criteria and indicators for evaluating these practices. Thus, for a meaningful evaluation of patient involvement in HTA, it must be clear what the goals are and from whose perspective (Chap. 4).

In addition, there is a lack of clearly identified relationships among the process and outcome variables of interest. These gaps are not specific to HTA but are common in the literature on patient and public involvement in the health field in general. For instance, Abelson et al. (2010) found that much of the empirical public engagement evaluation work is done without any framework that defines the theoretical

basis for the engagement process or the relationships among involvement mechanism and process or outcome variables of interest.

The framework proposed by Oliver et al. (2008) highlights key features that could influence the process and outcome of patient involvement in HTA. It considers:

- The type of patients involved (individuals or members of consumer groups)
- The initiator of the involvement process
- The degree of involvement (patients are consulted, collaborate or control the process)
- The choice of deliberation methods (qualitative or quantitative methods)
- The methods for eliciting and aggregating values (such as voting, scoring or ranking).

However, this framework does not include indicators of the outcomes and impact of patient involvement strategies.

Dipankui et al. (2015) developed a framework to assess patient involvement in HTA inspired by logic models used in programme evaluation to illustrate the relationships between the resources, activities, outputs and outcomes of a programme (Fig. 12.1). It maps the different types of outcomes related to patient involvement and distinguishes short- and medium-term results from long-term impact, acknowledging the importance of the evaluation timeframe. It also considers dimensions related to the process of patient involvement in HTA and the context in which this involvement takes place.

This framework incorporates three main components:

1. key elements of a logic model (Kellogg Foundation 2004)
2. evaluation criteria of patient involvement in HTA based on a general framework for evaluating public involvement (Rowe and Frewer 2005)
3. contextual factors highlighted by Abelson et al. (2010).

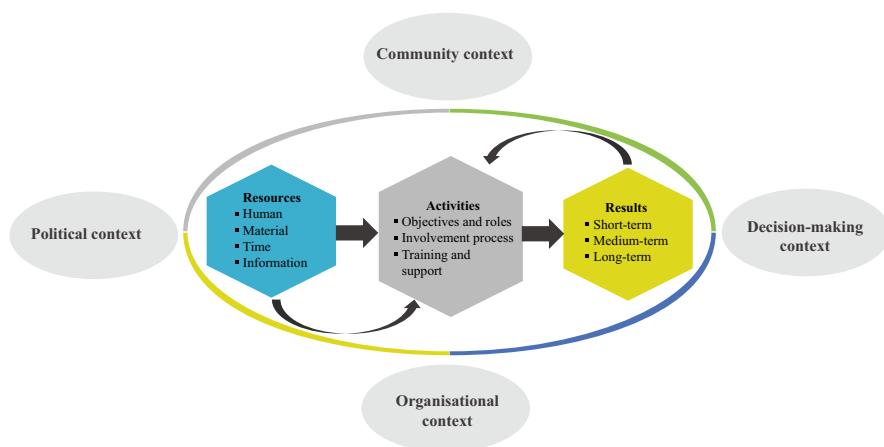


Fig. 12.1 Conceptual framework to evaluate patient involvement in health technology assessment

This framework was applied to the evaluation of patient involvement in the assessment of alternative measures to restraint and seclusion in psychiatry and in long-term care facilities for the elderly (Dipankui et al. 2015). As a generic framework, it considers most of the dimensions proposed in other frameworks for evaluating patient and public involvement in health decisions and could be used by HTA bodies to evaluate patient involvement in their activities.

Hamilton et al. (2021) identified key themes for evaluating the quality of initiatives to engage patients and family carers in decision-making across healthcare systems. Each theme is useful when assessing the structure, process or outcome of engagement initiatives within the organisation and they are relevant for evaluation at different points during and after engagement initiatives.

Based on the analysis of stories from 24 stakeholders with relevant experience, Lopez Gousset et al. (2024) propose a framework to characterise the impact of patient involvement in HTA. The domains of impact captured under this framework cover:

- impact on basis of HTA result or recommendation
- impact on HTA body
- impact on patient participants.

This framework, which includes a set of codifiers under each domain, can support transparent tracking and stimulate awareness of the value of involving patients in HTA. It is anticipated that this framework could be broadened to include additional domains specific to other stakeholders (healthcare providers, technology developers) through further research.

The Patient Engagement Monitoring and Evaluation framework was proposed by the PARADIGM consortium, a public–private multi-stakeholder partnership co-led by the European Patients’ Forum and The European Federation of Pharmaceutical Industries and Associations (Vat et al. 2021). This framework aims to support patients, patient organisations, technology developers, regulatory bodies and HTA bodies in monitoring the progress and impact of patient participation in the drug development lifecycle. It proposes five components, subdivided into 16 sub-components, as well as relevant metrics to monitor each of these elements. In Spain, this framework was used and adapted to develop a methodology for evaluating patient involvement in HTAs (see Chap. 24).

More studies applying these frameworks are needed in HTA to build a stronger evidence base on the process and outcomes of patient involvement and to identify the strengths and weaknesses of evaluation frameworks.

12.3.2 Definition of Concepts

The literature on patient and public involvement in general provides some criteria against which participation mechanisms are evaluated and there have been some attempts to use a definition of ‘effectiveness’ as a starting point (Abelson et al. 2010).

This echoes the situation in the field of HTA where some evaluation criteria are specified but often without distinguishing those related to the results and those related to the process. It is thus important to clearly define evaluation objectives and indicate whether they refer to the process or the impact of patient involvement in HTA.

Clearly defining each concept used also allows for more rigorous evaluation of patient involvement in HTA. For instance, most authors do not specify what they mean by impact, and in general, when they talk about impacts, they describe short-term outcomes. Short-term outcomes should be attainable within 1–3 years, while longer-term outcomes should be achievable within a 4- to 6-year timeframe and should be reflected in impact occurring within about 7–10 years (Kellogg Foundation 2004). Difficulty arises in defining what is meant by impact in HTA (Wortley et al. 2015). Impact can have different meanings from the perspective of an HTA body or that of a patient organisation (Livingstone et al. 2021). In this sense, defining ‘impact’ as a change perceived to be the result of patient involvement in HTA, as proposed by Lopez Gousset et al. (2024), could allow a better adaptation of the evaluation to the specific goals of patient involvement in HTA.

12.3.3 Consideration of the Context

Another aspect to consider is the context related to patient involvement in HTA. In fact, a growing number of researchers are recognising the significant role played by contextual variables that lie outside of the involvement process. According to Brett et al. (2014), the context refers to the environment in which involvement is undertaken and considers whether the right conditions are in place for patient involvement to work. Contextual variables can include funding, policy, physical environment or the attitude of those involved. With respect to HTA, Saarni et al. (2011) note that technologies and HTA are not executed in a vacuum, but always in a social and value context. These factors can have a significant shaping effect on how participants and policymakers view the involvement initiative, and how it is implemented. For instance, a study described how the organisational culture could influence the readiness of the Belgian HTA body for the future implementation of patient involvement (Cleemput et al. 2020, Chap. 15). Organisational change for patient involvement in HTA is also discussed in Chaps. 30 and 32. Figure 12.1 highlights four context areas that should be considered when evaluating patient involvement in HTA.

12.3.4 Different Methods and Techniques Used to Solicit Patient Evidence

The research methodologies outlined in Part II indicate how patients’ perspectives and experiences can be studied in a robust scientific manner. Systematic approaches are now needed to agree how this patient-based evidence and patient input that is

submitted directly by patients should be used in HTA. The Patient Engagement Monitoring and Evaluation framework (Vat et al. 2021) proposes specific metrics to monitor and evaluate several aspects of patient engagement in medicine development that could be applied to the broad field of HTA.

The variety of approaches that are employed to obtain patient-based evidence and patient input in HTA challenges our ability to compare different patient involvement strategies. At the moment, there is very limited research comparing the effectiveness of different approaches for evaluating patient involvement in HTA (Gagnon et al. 2015a). However, a ‘one size fits all’ approach is probably not the best option, and a combination of qualitative and quantitative methods could be more appropriate, adapted to the particular purpose of patient involvement in HTA (Mason et al. 2021).

In conclusion, four main considerations, identified from the literature, could improve the evaluation of patient involvement in HTA:

1. Validated frameworks to evaluate patient involvement in HTA
2. Clear definition of concepts
3. Consideration of the context
4. Appropriate methods and techniques to undertake patient involvement.

These considerations lead us to identify the most common challenges to evaluating patient involvement in HTA and to propose strategies that could answer these challenges in the following section.

12.4 Challenges Related to Evaluating Patient Involvement in HTA

The main challenges identified regarding the evaluation of patient involvement in HTA are grouped into conceptual, methodological and practical challenges. These challenges also offer avenues to consider for developing the field of patient involvement in HTA and thus strengthening its value.

12.4.1 Conceptual Challenges

Brett et al. (2014) report that patient involvement constitutes a complex intervention that requires appropriate evaluation. However, some preliminary steps are necessary before conducting an evaluation. Defining the purpose of the evaluation is an important prerequisite. Researchers wishing to design evaluation studies must first address questions such as: *Why is an evaluation being done? What will be done with the results? Who wants these results and how will they be used?*

The second conceptual challenge relates to the terms used when referring to patient involvement in HTA. We need to use a common terminology when referring to key evaluation concepts such as process, outcomes, impact or effectiveness. Additionally, the terms ‘patient’, ‘consumer’ or ‘service user’ are often used interchangeably in the literature.

A third conceptual challenge is the development and/or adaptation of theoretical and conceptual frameworks for guiding the evaluation of patient involvement in HTA, and their validation in different contexts. Some existing frameworks can offer a comprehensive and integrated approach, although they are not developed specifically for this field.

For instance, the framework proposed by Esmail et al. (2015) for evaluating patient involvement in research can be used as a starting point to identify relevant dimensions related to the process, context and impact of patient involvement in HTA. Table 12.1 builds on the dimensions of this framework in relation to the context, process and impact of patient involvement that could apply to the field of HTA.

12.4.2 Methodological Challenges

The diversity of ways in which patient involvement can be incorporated into HTA processes and for specific HTAs makes it difficult to compare any involvement mechanism to another. Thus, several methodological challenges must be addressed,

Table 12.1 Dimensions to consider in the evaluation of patient involvement in HTA

Dimensions related to		
Context	Process	Impact
Adequate resources and funding	Representativeness and diversity of patients involved	Patient empowerment
Favourable political environment and regulatory framework	Degree of patient involvement and choice of deliberation methods	Dissemination, translation and uptake of the results
Nature and attributes of the sponsoring organisation(s)	Access to sufficient and adequate resources to facilitate patient engagement	
Characteristics of the patients involved	Patient satisfaction with their engagement	Democracy and accountability
Availability of training for patients and researchers	Timing and frequency of engagement	Moral and ethical considerations
Nature of the topic being deliberated	Fairness and transparency of the process	
Time allocation	Definition of the patients’ role and the purpose of involvement	
	Definition of impact and effectiveness in patient involvement Adequate strategy in relation to the desired form of involvement	

Adapted from Esmail et al. (2015)

from the selection of the most appropriate evaluation design to the consideration of results generalisability and theoretical/conceptual contribution. From the literature on patient involvement in HTA, there are no recommended designs to evaluate such initiatives and no guidance on which indicators to use and how to interpret them (Staley 2015). Such methodological developments are greatly needed to improve the rigour of patient involvement evaluations. Qualitative and quantitative methodological approaches used to generate patient-based evidence in HTA could also be used to assess the impact of patient involvement from different stakeholder perspectives. For instance, Chap. 3 discusses the notion of patient-reported experience measure (PREMs) and patient-reported outcome measure (PROMs), which could be used to assess the impact of involving patients in HTA on health outcomes that are meaningful for patients. Qualitative methods, such as ethnographic fieldwork (Bidonde et al. 2021, Chap. 10), provide rich data to understand the experience of patients and other stakeholders involved in the HTA process.

Qualitative methods are often more appropriate for evaluating an intervention that cannot be isolated from its context. According to Staley (2015), one of the main weaknesses of the current evidence of the value of patient involvement is the lack of detail about the context in which it takes place, thus limiting our understanding of ‘why’, ‘when’ and ‘how’ the involvement has made a difference. The realist evaluation approach adopted by Wilson et al. (2015) offers an interesting avenue to better consider the relation between the context and the mechanisms that lead to outcomes. The GRIPP (Guidance for Reporting Involvement of Patients and Public) checklist (Staniszewska et al. 2011) provides a useful tool to ensure that reporting of results includes a detailed description of where, when, how, why and for whom patient involvement was conducted.

Patient involvement contributes to the transparency of the HTA decision-making process, as presented by PCIG’s Value and Standards for Patient Involvement in HTA (HTAi 2014). However, ensuring transparency presents another practical challenge, inviting questions about access to submitted documentation and records about how this information was used and how it influenced the basis for HTA recommendation. Legal standards around the sharing of confidential materials present a major challenge to increasing transparency. Lopez Gousset et al. (2024) suggest improving transparency of HTA processes by publishing information about who is invited to testify and how testimonies were considered in decision-making. Common understanding and language of what transparency entails would facilitate its evaluation in the context of patient involvement in HTA.

12.4.3 Practical Challenges

With respect to the practical challenges associated with the evaluation of patient involvement in HTA, it is important to consider who is engaged, who they represent, what role they play, at what stages of the HTA process and using what types of participation mechanism (Gauvin et al. 2014). Some models have been proposed to guide

HTA stakeholders in their patient involvement processes (Gagnon et al. 2015a; Vat et al. 2021), which could help to identify the key components of the evaluation.

Transparency on process, quality and impact is essential to foster trust and meaningful participation of patients as stakeholders in the HTA process. However, lack of dedicated resources and insufficient time can be significant challenges to assessing quality and effectiveness of patient involvement. In consequence, while evaluation is important, evaluation frameworks must be practical and easily implementable in order to be used routinely.

12.5 Discussion and Conclusion

While patient involvement in HTA is increasing, rigorous evaluation of its implementation and value is still needed to ensure that it fulfils its objectives. Thus, there must be clearly defined goals for involving patients in HTA such as those outlined in Chap. 4, which could in turn inform the evaluation questions and the methods selected. It is also important that terminology related to patient involvement in HTA is defined, and that involvement is transparent, and its methods informed by the best evidence. As HTA producers and patients can understand and practise patient involvement in different ways based on individual ideologies, circumstances and needs, the simple question of the choice of indicators of the success of patient involvement depends on the different perspectives of the key stakeholders (Fudge et al. 2008).

Typical scientific evidence based on experimental designs is currently lacking on the effectiveness of patient involvement interventions on healthcare and health outcomes. As proposed by Drummond et al. (2013), we need to promote the use of rigorous methods for combining and synthesising the findings from qualitative studies. However, alternatives to experimental methods are needed to provide evidence on the added value of involving patients in HTA and to consider factors that contribute to the success of different patient involvement in HTA (Craig 2008).

Evaluating current experiences is necessary to promote meaningful patient involvement in HTA. As the field evolves, the knowledge base regarding its evaluation and value is likely to expand rapidly. The value for money and sustainability of patient involvement initiatives in HTA need to be further investigated. This chapter presented key frameworks and requirements for evaluation and some of the challenges associated with the conduct of rigorous evaluations. Overall, there are many aspects to consider when evaluating patient involvement in HTA, and this chapter offers some practical guidance on how to conduct appropriate evaluations of patient involvement in HTA.

Acknowledgements We would like to acknowledge the contribution of Mylène Tantchou Dipankui and Deirdre DeJean who co-authored the previous version of this chapter. We are grateful to Karen M. Facey and Ann N. V. Single for their helpful comments.

Marie-Pierre Gagnon receives funds from various organisations to conduct research on patient and public engagement in health research. No funding was received in relation to the content reported here.

Aline Silveira Silva reports volunteer and consultancy work in patient involvement for patient groups and industry. No funding was received in relation to the content reported here.

Veronica Lopez Gousset reports volunteer and consultancy work in patient involvement for government, patient groups, and industry. No funding was received in relation to the content reported here.

Ana Toledo Chávarri reports volunteer and consultancy work in patient involvement for patient groups and industry. No funding was received in relation to the content reported here.

Anke Peggy Holtorf is employed by Health Outcomes Strategies GmbH, which contracts with a range of organisations including industry. No funding was received in relation to the content reported here.

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Chapter 13

Discussion: Making Sense of Patients' Perspectives, Experiences, and Involvement in HTA



Pascale Lehoux and Jaime Jimenez-Pernett

13.1 Thinking Qualitatively About Patients' Perspectives, Experiences, and Preferences

Qualitative research methods have been developed several decades ago and have been applied to further knowledge in many social scientific disciplines (e.g. sociology of health and illness, healthcare management, health policy, and knowledge synthesis). This chapter recognises that a mature and rich qualitative research scholarship can deepen our understanding of the strengths, limitations, and comparative relevance of different methods for eliciting patients' perspectives and for contextualising the use of qualitative evidence in HTA. It thus adopts a qualitative thinking perspective to reflect on four chapters in Part II of this book. Three of them (Chaps. 9, 10, and 11) provide readers with clear guidance on the ways in which specific qualitative and quantitative methods can elicit patients' perspectives, experiences, and preferences, while a fourth chapter (Chap. 12) takes stock of how patient involvement in HTA can be assessed. These chapters make more explicit the need to consider the epistemological underpinnings of the 'patient's voice' as well as the contexts in which such a perspective is being articulated.

The current chapter is comprised of four sections. It critically discusses the kinds of qualitative evidence that HTA can integrate (1), summarises key lessons from the

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four chapters (2), identifies methodological challenges that lie ahead (3), and formulates take-home messages for a rigorous use of qualitative evidence in HTA (4).

13.2 What Kinds of Patient-Based Evidence Qualitative Methods Generate?

Almost three decades ago, Murphy and colleagues produced an exhaustive monograph on the role of qualitative methods in HTA. For these authors, there are some problems in HTA (as introduced in Part I of this book) that “cannot be fully resolved using quantitative methods alone, and there are some circumstances in which qualitative methods represent the technically superior option” (Murphy et al. 1998, p. 87). In our view, what provides qualitative research a ‘competitive advantage’ has to do with its key general features set out in Box 13.1.

Box 13.1 Key Features of Qualitative Research

- A focus on the perspective of the study participants (be they managers, providers, or patients)
- An in-depth description of the research context
- A holistic view on the phenomenon of interest
- An emphasis on processes
- A flexible study design that is responsive to emerging findings.

Typically, qualitative research is suited to address ‘why’ and ‘how’ research questions, and it does so by recognising the centrality of subjectivity in human action:

Health technologies are applied by people (be they doctors, nurses, technicians or patients) to other people (usually patients). One of the distinctive features of a human action is that it is meaningful. People act on the basis of what they believe to be true rather than what may be objectively true (Murphy et al. 1998, p. 87).

Because their object of inquiry is permeated by subjectivity, qualitative researchers have developed theoretical frameworks and methodological tools to handle rigorously their informants’ subjectivity as well as their own subjectivity (better than their quantitative colleagues, we would be tempted to say). Such scholarly advancements have relied on a stronger command of the relationship between the ‘knower’ and the knowledge being produced (i.e. reflexivity), and on social scientific theories, which are necessary to make sense of meanings and social interactions. An epistemological framework defines what counts as knowledge, how such knowledge can be produced, what is knowable, and why it should be known. Because “the

choice of theory, although often unacknowledged, shapes the way practitioners and researchers collect and interpret evidence” (Alderson 1998, p. 1007), qualitative research that is not firmly grounded in a social scientific epistemology suffers from severe limitations (perhaps like a quantitative study of poor quality that has insufficient power to detect a statistically significant change).

For Murphy et al., qualitative research brings an important contribution to HTA “whenever the context in which a health technology is to be implemented can be expected to have an impact upon the outcome of that technology” (1998, p. 87). Qualitative studies can clarify the organisational, political, and sociocultural dimensions that affect the diffusion of technology in different settings and its real-world effectiveness. This ability to consider contextual issues is also a key asset when it comes to assessing patient involvement in HTA.

In the practice of HTA, where knowledge syntheses prevail, it is useful to differentiate the distinct contributions of three types of evidence:

1. context-free scientific evidence (such as the knowledge generated through a randomised controlled trial on the efficacy and safety of a new technology)
2. context-sensitive scientific evidence (such as the knowledge generated by an implementation study that examines regional variations using a sociology of innovation framework)
3. experiential evidence, which refers to the expertise, views, and realities of stakeholders (Culyer and Lomas 2006, p. 5).

At the Institut national d'excellence en santé et en services sociaux (INESSS), different knowledge sources are integrated within knowledge syntheses through a process that combines scientific, experiential, and contextual data (INESSS 2021). Relying on INESSS' multidimensional value appraisal framework, which examines clinical, populational, organisational, economic, and sociocultural aspects, multiple knowledge sources “are analysed and synthesised, using quantitative, qualitative or mixed methods of synthesis” and “are then organised by dimension to inform the deliberation about what is known and the associated limitations, uncertainties and ethical issues” (ibid. p. 5). Drawing on different types of evidence and on a comprehensive definition of value makes it possible to include the patient voice in HTA syntheses and within deliberative processes.

13.3 Patient-Based Evidence for HTA: Key Lessons from the Four Chapters

The four chapters address different methods to deliver evidence on patients' perspectives and evaluate HTA bodies' patient involvement strategies. Below, we address successively each chapter's key contributions and critical lessons for the international HTA community to ponder.

13.3.1 Measuring What Matters to Patients Is as Important as Qualitatively Thinking About Why Their Preferences May Vary

The chapter by Marshall and colleagues (Chap. 9) describes the main methods that can be used to elicit patient preferences and how such research can inform HTAs. Though the authors stress that preferences can be researched using both qualitative and quantitative methods, they focus on quantitative methods.

The strength of their quantitative focus is to clarify how patient preferences research can support two important analytical tasks:

1. identifying what aspects of a healthcare intervention matter to patients, which may lead to a measurable definition of *what is important*
2. appraising how much each aspect of that intervention matters in relation to other aspects, which may lead to rank-ordering different aspects of an intervention according to their *relative importance*.

The authors suggest that patient preferences can be used at different stages in the development of a new health technology and in HTAs to inform a broad range of decisions. Recognising that many HTA bodies rarely conduct primary research, the authors underscore that ‘it is unlikely’ that HTA bodies would themselves produce such evidence, but it could be submitted by health technology developers or patient organisations.

In contemporary societies, the amplitude of variations across patient groups’ preferences may depend upon the sociocultural and ethical aspects of the health technology undergoing an HTA. It remains unclear the extent to which “insights regarding heterogeneity of preferences in different patient populations” (Sect. 9.1) may inform policy or reimbursement decisions. Measuring benefits that matter to (most) patients may not always lead to a straightforward course of action, especially when public policies aim to consider equity, diversity, and inclusion. We thus concur with Marshall and colleagues for whom “there is more work to be done” to properly integrate patient preferences research in HTA deliberative processes.

In principle, deliberation should bring the patient’s perspectives, preferences, and experiences to a collective level: what matters is not to collect a range of views, but to bring these views in a common good-spirited dialogue (Bombard et al. 2011; Degeling et al. 2015). The goal of a deliberative process is to reflect on and ponder what a just and reasonable public policy recommendation may entail, addressing both uncertainty—lack of knowledge—and ambiguity—the potential for multiple interpretations (Cairney et al. 2016). As such, it should explicitly support the expression of challenging viewpoints and learning opportunities for all participants (Abelson et al. 2010; de Vries et al. 2011). Deliberative methods may prove physically and/or emotionally burdensome, and this creates a formidable tension when the aim is to gather the views of individuals who are afflicted by health problems. As Giacomini and Cook underscore, a deliberation “affects the meanings of social experiences” while it seeks to “translate these experiences for persons who might

not otherwise understand each other's perspectives well" (Giacomini and Cook 2000, p. 480). Hence, a deliberation may also be transformative, which is a puzzling issue when one seeks to design (and later assess) a deliberative process (Carman et al. 2014). It might be entirely legitimate that some opinions remain unchanged if, at the same time, mutual learning between participants takes place (Black et al. 2011; Lehoux et al. 2009). Overall, qualitatively thinking about why patient preferences may vary across sociocultural groups is as important as knowing what matters to most patients.

13.3.2 Ethnography Is Not Just About What Patients Say

For Tjørnhøj-Thomsen and Hansen (Chap. 10), ethnography is first and foremost concerned about the patient's everyday life and context, not just about what they say about a particular technology. Because ethnography pays attention to the social situation in which the use of a technology unfolds, it leads to a detailed understanding of how particular settings influence its real-world effectiveness. Hence, ethnography goes well beyond the knowledge one may obtain from conducting individual interviews or focus groups since it is designed to capture holistically the interactions between the technology, patients, and their social surroundings, including providers, insurers, employers, neighbours, family, etc.

This chapter underscores how aiming to develop a single approach to generate patient-based evidence is unsound and would divert the attention from a key object of inquiry in HTA: technology's impact on patients. The nature of the technology—that is, whether its use is lifesaving or not, whether it supports chronic or acute care, or whether it involves small or large trade-offs in one's personal life—will influence how the ethnographer approaches patients' experiences. Summarising ethnographic studies can be precious in HTAs that take seriously sociocultural and ethical issues because it highlights the “dynamic relations between social worlds and cultural systems” (Sect. 10.2), which often vary from one patient group to another.

Considering that some patients may be ‘of a few words’ but certainly not without intent, expectations, or emotions, the time ethnographers patiently invest in the field is directly proportional to the completeness of their analyses. Just like focus groups are not a cheaper and quicker means to increase one's ‘n’ in a qualitative study, ethnography is not a simple data collection task, but a primary research endeavour per se (and rarely practiced in HTA bodies). When critically reflecting on focus group research that we had conducted with patients, we argued that such a method does not “derive epistemological authority simply because of the identity of its participants” (Lehoux et al. 2006, p. 2103). This implies recognising that patients do not arrive with “pre-formed ideas that just need to be skilfully elicited or discovered. Nor do they share in any straightforward way their knowledge or naively endorse all knowledge claims put forward by others” (Lehoux et al. 2006, p. 2103). Likewise, Tjørnhøj-Thomsen and Hansen rightly tear to pieces the assumption according to which qualitative research would not require special training and expertise. An

ethnographic method must be thoroughly understood to be rigorously applied (and vice versa). Tjørnhøj-Thomsen and Hansen stress that patients “may take for granted” (Sect. 10.3) care-related tasks they routinely accomplish and thus may not see as relevant to talk about in interviews. Hence, a well-prepared observation guide, i.e. informed by social sciences, should help to “strategise carefully about what to observe, with what purpose and from where” (Sect 10.3).

A rigorous, in-depth understanding of what works and does not work for patients in different settings is very precious when policymakers and practitioners are looking for ways to adequately implement new technologies that raise challenges for patients. This is why, despite the time it takes, ethnographic research is likely to provide results with a profound and lasting usefulness, thereby supporting the broader mission of HTA. Tjørnhøj-Thomsen and Hansen underscore that patients’ perspectives are not static and evolve through social circumstances, and this type of uncertainty needs to be well recognised in HTAs. Like healthcare professionals, patients are “gatekeeping their own lives, picking out what they want to disclose to the researcher” (Sect. 10.2.1). Such gatekeeping points to the incompleteness of the experiential knowledge individuals may share with others, not only because of the tacit components embedded in experiential knowledge but also because talking about one’s experience reveals certain things about one’s identity (this is also true for clinicians).

Today, as we are surrounded by social media and digital communication tools, HTA practitioners may need to reflect on the way these technologies transform access to experiential knowledge. For instance, there are many topics in HTA that raise social desirability or sensitive issues (e.g. sexual health, substance use), that require recruiting over a very large area (e.g. rare diseases, discriminatory practices based on gender, disability, or ethnicity) or that call for the ability to secure collaboration with hard-to-reach groups (e.g. migrants, stigmatised lifestyles). Yet, the vast amount of data social media may provide pushes researchers to use quantitative analytical strategies that fall short of fulfilling the essential qualitative research features we introduced earlier. Overall, we concur with Tjørnhøj-Thomsen and Hansen and doubt that there are any quick and cheap alternatives to ethnography.

13.3.3 Qualitative Evidence Synthesis Is Here to Stay

In Chap. 11, one finds an enlightening and state-of-the-art description of Qualitative Evidence Synthesis (QES). This chapter describes the different types of QES, the versatile ways in which they can be applied within HTA, and how they have been used to inform various time-sensitive policy decisions.

The intent shared by most QES reports is to provide a nuanced understanding of how patients interact with service providers in various contexts. This is achieved “by harnessing multiple patient perspectives” and bringing together diverse findings within a single synthesis. As QES explicitly seeks to account for multiple perspectives from different healthcare settings, it goes beyond summarising patients’ experiences in each context. For instance, such syntheses may aim to draw meta-theoretical

links between different types of qualitative studies. Their bibliographic search strategies thus require a strong command of the specificities of social scientific publication databases. Given the knowledge and resources now available for HTA practitioners, for an HTA body not to engage in QES would signal an ideological rather a methodological decision.

One piece of the methodological puzzle that Booth and Fryer clarify very well in Chap. 11 is the distinction between a synthesis of the patients' experience of a condition and a synthesis of their appreciation of the outcomes of a particular technology or service. Whereas the former will necessarily be broader in scope and require some theory building, the latter may prove much more focused and potentially less time-consuming.

It remains surprising for the authors that HTA bodies may still be reluctant to integrate QES given the multidisciplinary aspects covered by the HTA Core Model® (Chap. 1). The increasing number of meta-ethnographies (one form of QES) published every year can be understood because of the sheer number of primary studies that are already 'out there'—and hard to ignore—and of the natural connection between qualitative research and the patient's perspective. The good news for HTA bodies is that QES methodologies have made an enormous leap forward in the past two decades, exemplified by the international efforts Booth and Fryer describe. Interestingly, while each individual published qualitative study may never have been intended to be generalisable, a solid QES methodology may contribute to increase their policy relevance. A well-designed QES can help to piece together why and how variations across contexts occur. Throughout the review process, a multidisciplinary team should not aim for consensus but for "divergence and interpretive richness" (Sect. 11.4.9), and this stands in contrast with the expected role of reviewers in a systematic review.

Though we may doubt that patient participation at every step in QES is necessary (and compatible with a proportionate approach in HTA), Booth and Fryer clearly explain how patients can participate throughout the whole synthesis process. One stimulating idea that stems from their decision to do so is to highlight that the interpretation and dissemination of QES findings could benefit from conceptual maps, illustrations, or videos to better support patient participation at these steps. This observation seems even more important considering that patient participation may remain at risk of being a 'tick box exercise' that betrays the very goal of patient participation in HTA. Given the resources now available for QES, HTA bodies should be encouraged to expand their methodological toolbox and recruit scientists well-trained in qualitative methods.

13.3.4 Knowing 'Why' and 'How' Patient Involvement in HTA Makes a Difference

Gagnon et al. provide in Chap. 12 an excellent overview of current practices regarding the evaluation of patient involvement in HTA. There is a lack of rigorous evaluations, even if it has been recognised as an essential component of HTA a decade or

so ago. The chapter thus carefully reviews key frameworks and requirements for conducting more rigorous evaluations of patient involvement in HTA and their associated challenges.

This chapter emphasises the importance of being clear about ‘why’ an HTA body may wish to evaluate its patient involvement activities and ‘how’ the results will be used. This may partly explain why the authors find a variety of approaches being employed to obtain patient-based evidence and patient input in HTA. This variety challenges the ability to compare different strategies and identify which may prove more effective for certain topics or policy contexts. Though the authors do not recommend a ‘one size fits all’ approach and agree that a combination of qualitative and quantitative methods may work best, they stress that qualitative methods are well-suited for evaluating an intervention that “cannot be isolated from its context” (Sect. 12.4.2). A lack of attention to contextual issues limits current understanding of ‘why’, ‘when’, and ‘how’ patient involvement makes a difference or not.

For Gagnon et al., the fact that evaluation frameworks vary, and the types of outcomes reported too seems problematic. But one may wonder the extent to which this is a major problem and for whom: researchers, patient organisations, HTA bodies, or HTA funders? Perhaps the heterogeneity is compounded by the ambiguity inherent to the reasons why HTA bodies implement patient involvement strategies and then approach the impact such involvement can or should have on their evidence synthesis processes and outcomes.

This chapter brings to the HTA community’s attention the need for: (1) validated patient involvement evaluation frameworks; (2) clearly defined concepts; (3) context-sensitive evaluation methods; and (4) patient involvement strategies adapted to the participants and the task-at-hand. Interestingly, these four considerations closely resonate with those found in the early literature on deliberative methods. For Abelson et al. (2010, p. 10), deliberative methods were increasingly being applied in the health field, but their evaluation was “carried out in the absence of any guiding frameworks” that would have defined the theoretical basis for the process, the expected relationships between the mechanisms set in place, and the outcomes of interest. Popay and Collins (2014) and colleagues later called for making explicit the ‘intervention theory’ of the involvement strategy, that is, describing the ways in which a particular approach to involving patients is supposed to lead to the expected effects. Hence, we may hope that previous recommendations made to further the informed development of deliberative methods in HTA will be integrated within the steadily evolving field of evaluation of patient involvement in HTA.

13.4 What Future Methodological Challenges Lie Ahead?

Considering the time and resource constraints that characterise HTA bodies, conducting qualitative primary research is rarely an option. Synthesising qualitative evidence is more common. Yet, a HTA body must secure human resources who possess such research skills and experience. HTA practitioners who are generally

trained to perform quantitative systematic reviews must learn how to read critically qualitative studies and be able to meaningfully extract from social scientific studies evidence that is relevant to patients' perspectives, preferences, and experiences. They may also have to develop methodological skills in the design of syntheses that integrate both kinds of evidence. When it is not feasible to conduct QES, for instance, in the case of emerging technologies, performing succinct qualitative primary research may prove insightful. Short ethnographic fieldwork or other qualitative methods could be indicated in those circumstances. It would provide at the same time a great opportunity to engage patients in the research process itself.

Among the methodological challenges that lie ahead for the HTA community, is determining the extent to which patients can be properly consulted through digital means. The use of audiovisual material-based tools may succeed in supporting informed online deliberations among nonexperts (Lehoux et al. 2014, 2016). Nonetheless, it remains important to foster inclusiveness and meaningful involvement of all participants, and maximise the opportunity to democratically learn from each other and co-produce rigorous patient-centred knowledge (Khodyakov et al. 2016). In our view, the risk of not doing so is to increase civic inequalities (Lehoux et al. 2016, p. 13; Marques 2009).

13.4.1 Take-Home Messages for a Rigorous Use of Qualitative Evidence in HTA

Beyond knowing how to choose and apply the right methods, one needs to unpack the epistemological underpinnings of patient-based evidence. As Chaps. 9 and 10 point out, patient preferences and patient experiences are two different objects of inquiry. For Tjørnhøj-Thomsen and Hansen, patients' experiences are "emerging, relational, and shifting" (Sect. 10.8) and this observation has tremendous implications. It brings us back to the task of defining what counts as experiential knowledge, how such knowledge can be produced, what is knowable, and why it should be known.

Rigorous qualitative researchers refrain from "positing an apparent consensus as *the* patient's view" and explicitly recognise that participants' views remain "the result of context-dependent social interactions wherein perplexing dynamics contribute to the creation of dominant narratives" (Lehoux et al. 2006, p. 2103). Further research on ways to elicit patient's perspectives, experiences, and preferences is likely to deliver the epistemic diversity a deliberative democracy requires.

Until then, the credibility and legitimacy of a HTA body will be increased if it can consistently show a strong command of qualitative research findings, which is not a mere *technical* challenge (see Chap. 3). Rigor in the use of qualitative evidence requires a strong ability to *think qualitatively* and therefore to reflexively address the participants' and one's own subjectivity.

13.4.2 *Concluding Remarks*

In this chapter, we argued that the use of qualitative research in HTA cannot reasonably be considered an emerging topic anymore. The qualitative research scholarship is vast, mature, and dynamically responsive to the digital world in which our societies evolve. It possesses a clear competitive advantage over quantitative methods for making sense of the patients' perspectives, preferences and experiences since it can clarify why, how, and in what context patient-centred evidence-informed healthcare may flourish.

Yet, the integration of qualitative research into HTA calls for specific knowledge, know-how, and skills, e.g. methodological expertise that must be rigorously acquired and applied. It also often requires a social scientific 'lens', otherwise one may be trying to piece together different data fragments without using a meaningful framework. Henceforth, there is neither a 'quick and dirty' way to become a solid QES producer, nor a simple trick to produce rigorous and non-complacent patient-based evidence. One should therefore resist ill-informed demands for diluted evidence.

HTA as a field must stay true to the rich, complex, and at times conflicting realities of patients. Fulfilling this aspiration requires, beyond the necessary allocation of time and resources, a reflexive, theory-informed, and rigorous integration of qualitative data to account for technology's role in patients' everyday life and context.

Acknowledgements Pascale Lehoux's salary is covered by INESSS and the Université de Montréal. Jaime Jimenez-Pernett receives his salary from the University of Granada.

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Part III
Jurisdictional Approaches and
Stakeholder Views

Chapter 14

Australia—Co-designing Enhanced HTA Processes with Patients



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14.1 Context

Across the health and medical research sector in Australia, ‘consumer’ is used to refer to ‘people who have lived experience of a health issue’ and so includes, but is not limited to, patients, families, carers, friends and members of the general public (National Health and Medical Research Council 2018).

The use of the term, however, is not without tension. Some patients feel less visible when described as a consumer. Similarly, the experience and needs of patients with a particular condition cannot be represented by a single health consumer (irrespective of their personal experience of a specific condition). Input received for Health Technology Assessment (HTA) Committee considerations from consumers (and other external stakeholders such as healthcare providers) is therefore categorised to ensure that during committee discussions, it is clear who has: lived experience of a condition; and who has direct experience of receiving the health technology under assessment as opposed to other consumers—including family, friends and carers and also members of the public (Street et al. 2020). For

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consistency in this chapter, the term consumer will be used when referring to Australian processes or outputs and patient will be used otherwise.

Consumer involvement has been an element of HTA processes for several decades, as previously described (Wortley and Wale 2017). In 2019, the HTA Consumer Evidence and Engagement Unit (CEEU) was formally established within the Department of Health and Aged Care (now known as the Department of Health, Disability and Ageing and referred to as the Department) to strengthen and support consumer participation. The CEEU aims to further consumer involvement within the Australian HTA system and works closely with HTA committee members. This model has increased the diversity of participation, enabled unfiltered narratives of perspectives and experiences, and steadily enhanced the learning curve of the work around patient and consumer involvement in the Australian HTA system.

14.2 Current Structures for Patient Participation

There are multiple HTA committees with consumer members that seek consumer input in their decision-making processes in Australia. At a national level, there are two principal HTA committees: the Pharmaceutical Benefits Advisory Committee (PBAC) for medicines and vaccines and the Medical Services Advisory Committee (MSAC) for medical services, diagnostics, devices and highly specialised therapies (HSTs); both with associated subcommittees. The principal committees provide advice to the Minister for Health, Disability and Ageing around subsidisation of health technologies. Table 14.1 highlights some of the primary engagement opportunities that are currently available in the system for these HTA committees. Notably, the addition of stakeholder meetings (2014) and consumer hearings to the PBAC process (2017) provided valuable strategies to support patient participation, particularly for conditions where there is unmet clinical need and limited experience of a treatment in Australia.

Table 14.1 Current patient participation opportunities with principal Australian HTA committees

Committee	Current patient participation opportunities
PBAC (Medicines and vaccines)	PBAC Consideration: any consumer or organisation can provide their views about medicines or vaccines on each PBAC agenda item via the online ‘consultation hub’. There are 8 weeks to make comments for most submissions. Where consumers cannot access the web survey, emails and letters are accepted. Patient and other stakeholder comments are provided to the PBAC and discussed. A summary of individual comments received and the complete submissions from organisations are provided (with personal information redacted) to the PBAC and applicant. Patient and other stakeholder comments are summarised in the PBAC minutes and noted in the Public Summary Document. The Drug Utilisation Sub Committee (DUSC) of the PBAC also seeks consumer input for utilisation analysis reports to assist patients and other stakeholders to better understand how medicines are used once subsidised. Consumer Hearings: Participation is by invitation and mainly take place between patient or consumer organisations and PBAC representatives. They take place when deemed necessary by PBAC where there is a need for greater consumer input prior to the PBAC meeting on an agenda item. Stakeholder Meetings: convened by PBAC where certain criteria are met. A range of stakeholders may be invited, and this usually includes consumer organisations and individual patients with the disease or condition. These meetings may occur prior to or post a PBAC meeting.
MSAC (Medical services, diagnostics, devices, HSTs)	Application and Population, Intervention, Comparator, Outcome (PICO) development: The PICO is ratified by the MSAC PICO Advisory Sub Committee (PASC). Once an application has been deemed suitable for assessment, the application form is provided to clinical and consumer organisations for targeted consultation. The application form is also published on the MSAC website for any stakeholder to provide comment prior to the PASC meeting. Once ratified, the PICO confirmation is published and includes a summary of patient input received. Evaluation Sub Committee consideration: An assessment report is developed by the applicant or contracted by the department (in the case of non-commercial applicants, which may include consumer organisations). The Evaluation Sub Committee (ESC) considers the assessment report and considers consultation input received prior to the meeting. MSAC Consideration: The application and PICO remains on the MSAC website and is available to inform consultation on MSAC applications. Comments can be submitted through the online ‘consultation hub’ or can be submitted by letter or email. Stakeholder meetings may also be held where further input is needed, which may include patient or consumer organisations. Consultation input is discussed at the MSAC meeting. A consumer summary providing a plain language rationale for MSAC’s advice is included in the Public Summary Document that is made available on the MSAC website.

MSAC Medical Services Advisory Committee, PBAC Pharmaceutical Benefits Advisory Committee

14.3 Spotlight on Areas of Patient Participation in HTA

14.3.1 Considering Individual Patient Comments

A key feature of the Australian HTA process is the ability for individuals and organisations to provide input for consideration by the HTA committees. Both types of input have a role to play in confirming, contradicting or complementing the submissions the committees appraise.

An additional important contextual element for considering how the HTA committees interact with patients and other stakeholders is the direct contact and communications afforded between Chairs and external organisations and groups for other activities and events. The various mechanisms available for patients to interact directly with the HTA committees and Chairs in a meaningful and open manner allows development of rapport and trust. This flows to how HTA committees can then directly interact with relevant consumer representatives and get to the ‘heart’ of issues, facilitating the formal HTA Committee processes separately.

Input from individuals provides additional depth and understanding for committees, beyond what is presented within a company application or by a collective patient group or organisation. Open and inclusive processes for obtaining individual patient input requires connections with patient communities and an accessible framework for individuals to have sufficient understanding and trust of the processes, willingness to engage and flexible options for how that engagement can be supported. Once received, input is then read, considered and, where necessary, summarised. This requires adequate resourcing and skilled capacity within the structures to achieve consistent and accurate formats for summarising individual comments, with consideration of what is most useful for committee deliberations.

The tendency to report on the quantity of consumer comments received, as opposed to the content or impact, may risk overlooking the value of the content and context. Work with stakeholders over time has emphasised the value of inputs relevant to HTA decision-making processes, rather than ‘petitioning’ an advocacy position with the Committees.

Over the past 5 years there has been a focus on improving the quality of input received and reducing the number of ‘petition-style’ comments. Some success has been achieved in this area. This is partly due to the development of an online survey with targeted questions housed on the ‘Consultation Hub’, introduced in 2021 for PBAC and in 2024 for MSAC.

The majority of consumer inputs are received from patients, some with experience of the new technology or those who wish to access it, their family members/carers and other interested members of the public. It is also important to note that while high numbers of individual comments can provide informative detail for committee deliberations, a lower number of inputs received for an item does not necessarily mean that there is less interest in a technology. For example, lower numbers of input may be expected when:

- the patient population is small (e.g. rare diseases) or is representative of disadvantaged/vulnerable groups
- the consumer organisation lacks capacity
- applications are not relevant to a single condition (e.g. MSAC may consider policies or services relevant to the Australian population such as population level screening interventions).

In these situations, it is harder to actively engage with specific groups, and the role of individual submissions is even more important to help understand potential impacts on those who may receive the health technology, in addition to implications for the health system. It is noteworthy that in some of these situations there has been important inputs requested of specific clinicians and their patients directly to support Committee deliberations.

14.3.2 The HTA Consumer Consultative Committee

The HTA Consumer Consultative Committee (HTA CCC) was established in 2017 as a non-statutory committee, to act as an advisory group to the Department on consumer perspectives in HTA matters. It is comprised of the consumer committee members from each of the HTA committees and subcommittees. The formation of the HTA CCC enabled consumer committee members to be brought together, allowing for support of members in their HTA roles and a strengthening of evidence-informed consumer engagement. The CEEU provides support to the HTA CCC and works closely with its members.

The HTA CCC provides advice on improving communication, collaboration, engagement and opportunities for consumer participation in HTA processes. The HTA CCC is seen as valuable as it provides an avenue for mentoring and capacity building and helps the Department to more proactively consider how consumer roles should be considered and developed over time, and for future recruitment purposes.

Since its inception, the HTA CCC has contributed to supporting and developing consumer participation. These outputs include hosting workshops and meetings with consumer organisations (including virtual symposia aimed exclusively at consumers and consumer organisations), participation in international HTA forums, and exploring the benefits of mentoring to upskill and support consumer members in their role on HTA committees (Walker et al. 2021). Other examples of the impact of the HTA CCC includes influencing strategic policy development, e.g. the addition of an extra *Terms of Reference* for post market reviews to consider the perspective of consumers using the medicine of interest (Department of Health and Aged Care 2024a).

A notable activity developed by the HTA CCC, with representatives of patient organisations and the CEEU team, was a consultation series called *Conversations for Change*. The consultation, conducted during 2022/23, informed the CEEU on

priorities to support consumers and consumer organisations engaging with HTA processes. It was specifically designed to ask participants (consumers, health professionals and HTA researchers) *what, when* and *how* consumer views and experiences can best be provided during HTA processes. Five interdependent themes emerged from the consultation, centred around consumers being informed and engaged, supported and knowledgeable, included in HTA processes and methods, actively participating and, equal partners (Department of Health and Aged Care 2023). The theme of ‘equal partners’ was considered the aspirational outcome which will continue to develop with the realisation of the other objectives stated.

14.3.3 Co-designing an Enhanced Consumer Engagement Process

A further body of consumer specific work, developed from an element of a 5-year strategic agreement, 2022–2027 (Department of Health 2021), between the Commonwealth Government and Medicines Australia (the peak body for the research-based pharmaceutical industry in Australia), built on the *Conversations for Change* findings, a House of Representatives parliamentary inquiry (Standing Committee on Health, Aged Care and Sport, Parliament of Australia 2021) and the National Medicines Policy (Department of Health and Aged Care 2022). This work entailed a co-design of an enhanced consumer engagement process to capture consumer voices in respect to applications to list new medicines on the Pharmaceutical Benefits Scheme (PBS), the Australian Government-funded programme for subsidising medicines (Department of Health and Aged Care 2024b).

A Co-design Working Group (CWG) was established, comprised of consumer nominees, government and medicines industry representatives and chaired by an elected consumer lead from the group. All consumer members were identified through an open, national expression of interest process and selected by peer-review. The project was facilitated by an independent academic group based within a university programme and supported by the CEEU team within the Department. The CWG sought consultation on its draft recommendations from consumer, industry and department stakeholders.

A report of ten recommendations emerged from this work, titled *Enhance HTA: An Enhanced Consumer Engagement Process in Health Technology Assessment* (Department of Health and Aged Care 2024c). These reflect the report’s vision of:

Australians’ diverse health care experiences and needs are understood, and consumer engagement is integral in HTA decision-making.

Three themes supported the recommendations, and key foundational elements were identified as critical to implementation of the recommendations (Box 14.1). These elements recognised that for consumers to be integral to HTA processes from early and throughout the health technology pathway, a consumer engagement framework should be developed, supported by investment in systemic change that would be

required to resource enhanced consumer engagement processes. Crucially, the recommendations should be strengthened by HTA policies and methods.

Box 14.1 Themes and Foundational Elements for Implementation Emerging from the Enhance HTA Report (Department of Health and Aged Care 2024c)

The Enhance HTA report is driven by three core values:

- Partnership—consumers actively partner and participate in HTA processes
- Evidence and Input—consumer evidence is integral to decision making
- Transparency—consumers are informed and HTA processes are clear.

Three elements are identified as critical to implement the report's recommendations:

- Investing in systemic change
- Strengthening HTA policies and methods
- Establishing a consumer engagement framework.

The ten recommendations proposed complementary enhancements to support earlier and continuous consumer participation and to facilitate the integration of consumer evidence and input into HTA processes (Table 14.2).

While the proposed recommendations are intended to apply to the PBAC processes, it has been recognised that they should also enhance consumer engagement across the entire health technology pathway, from clinical research through to post-market review. Involving patients and consumers earlier in the health technology pathway and ensuring information about the assessment under HTA consideration is available and accessible (plain language) for meaningful submissions have, for some time, been recognised within the Australian HTA setting as elements requiring improvements. Earlier participation in clinical trials presents challenges for Australian patients due to the limited number of later stage trials conducted locally. As the landscape changes around this, it is likely that there will be a corresponding change in the quality and volume of individual and group submissions. Further, such recommendations may be relevant and transferable to other HTA committees such as the MSAC.

Another element of the Australian Government and Medicines Australia strategic agreement, the HTA Policy and Methods Review (Department of Health and Aged Care 2024d), has synergies and alignment with the co-designed enhanced consumer engagement process. The resulting report, *Accelerating Access to the Best Medicines for Australians Now and into The Future* (Health Technology Assessment Policy and Methods Review Reference Committee 2024) has recommended comprehensive, multifaceted, reforms to Australia's HTA arrangements more broadly. Patient and consumer organisations were consulted in the

Table 14.2 Summary of recommendations from the Enhance HTA report (Department of Health and Aged Care 2024c)

Recommendation	Details
Transparent communications and timely notifications	Provide transparent communications and timely notifications to enhance the clarity of HTA processes and enable timely consumer engagement.
Centralised and expanded consumer support	Coordinate centralised and expanded consumer support to facilitate engagement across the health technology pathway.
Consumer identification	Develop a process for consumer identification to expand the diversity of consumers engaged in HTA processes.
Resources and training	Provide accessible resources and training to support equitable consumer engagement in HTA.
Elevate consumer evidence and input	Elevate consumer evidence and input for consideration in HTA deliberations and decision-making.
Early and continuous collaboration	Establish guidance to enable early and continuous collaboration between stakeholders.
Consumer-identified items for HTA Committees’ considerations	Further develop processes to enable consumer-identified items for HTA Committees’ considerations.
Consumer feedback loop following HTA Committee recommendations	Establish a consumer feedback loop following HTA Committee recommendations to provide insight into how consumer input has been used to inform the assessment of health technologies.
Consumer digital portal	Develop a consumer digital portal to connect consumers with information and resources required for engagement in HTA processes.
Consumer-focused horizon scanning	Ensure consumer engagement is informed by consumer-focused horizon scanning processes and opportunities.

development of these recommendations, which included 51 patient and community organisations providing a joint response to the Review consultation as further discussed in Chap. 29. This self-initiated patient collaboration is indicative of the evolving maturity of the patient sector and demonstrates the sector’s ongoing progression towards the aspiration of ‘equal partners’ with other stakeholders within the Australian HTA setting.

Examples of specific recommendations of the HTA Policy and Methods Review which are relevant to patients, and aligning with the Enhance HTA recommendations, included:

- Publishing plain language summaries
- Improving the HTA website including the development of a dashboard
- Developing a stakeholder engagement framework
- Improving the involvement of consumers in HTAs

Of note, the HTA Review Implementation Advisory Group was established by government in November 2024 to guide the response to the recommendations of the report (Department of Health and Aged Care 2024e). This multistakeholder group includes representatives from government, industry, consumers and clinical practice

to enable all key sectors to collaboratively progress the work of reform within the HTA setting.

14.4 Discussion

Patient and consumer participation in the Australian HTA process has evolved over the past 5 years and continues to do so. Engagement with patients and consumers has become more interactive and the frequency and depth of engagement with the Department and HTA committees has grown. Further, there has been an increase in patient and consumer organisations in Australia working directly with patients and consumers themselves; independent of third-party commercial entities. This indicates maturation across the whole of the Australian health and medical research sector and is bringing increased transparency and trust between patients and consumers and key health system stakeholders. With activities such as the HTA Policy and Methods Review, and the co-design project considering enhancement of consultation processes, the current period is dynamic and an enabling environment for further consumer-led innovations.

It is acknowledged that there are still areas to be improved, particularly in terms of increasing awareness of the HTA system and opportunities for engagement and providing feedback to patients and consumers on how their input was used. And while the recognition of the importance of patient participation in the HTA processes in Australia is undeniably a positive move, this is likely to bring change and challenges with additional expectations for the HTA system. Workforce skills and capacity need to be developed and increased over time, to ensure ongoing working relationships are sustained with the various stakeholders involved. This also needs to happen in such a way that does not add to system complexity and administrative burdens to ensure that access processes for appropriate health technologies are as efficient as possible, and optimal for meaningful stakeholder participation.

14.5 Conclusion

In Australia there is no longer need for debates regarding whether patient and consumer input matters, as this is universally recognised by Commonwealth HTA committees, the Department and the Australian Government. Their responsiveness has played an important role in increasing a focus on patient participation throughout the national HTA processes, with the visibility and impacts of patient input evolving markedly since 2019. Following the establishment of the HTA CCC in 2017, the formation of the CEEU in 2019, the launch of the Consultation Hub in 2021, and *Conversations for Change* series in 2022/23, the Australian Government is establishing formal frameworks for meaningful consumer involvement in HTA processes and decision-making. The findings and recommendations from the 2023/24 HTA

Review and the Enhance HTA co-design project further established patients and consumers within HTA structures and activities. The participation of patients in the development of these reports and the relevance of resulting recommendations indicate support for strengthening and embedding future patient involvement in all stages of HTA processes in Australia.

Acknowledgements We would like to acknowledge the contributions made to this chapter by Bella Beach-Mills (Departmental Officer in the CEEU). We would also like to recognise the hard work of previous and current members of the HTA CCC and other individuals and organisations that have worked closely with the CEEU, the Department and HTA Committees.

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Chapter 15

Belgium: Partnering with Patients for HTA Research



Célia Primus-de Jong, Irina Cleemput, Marie Dauvrin, Laurence Kohn, Sabine Corachan, Marit Mellaerts, Hester M. van de Bovenkamp, and Roland Bal

15.1 Context

Belgium has over 20 years' experience in health technology assessment (HTA). The creation of the Drug Reimbursement Committee (DRC), in accordance with the Royal Decree of 21 December 2001 (Royal Decree 2001), marked the first steps in formalising procedures for assessing the appropriateness of drug reimbursement (Cleemput and Van Wilder 2009).

The Belgian Health Care Knowledge Centre (**KCE**, which stands for *Federaal Kenniscentrum voor de Gezondheidszorg/Centre fédéral d'expertise des soins de santé*) performs HTAs that are not directly related to concrete reimbursement requests from medical product developers, but gives advice to decision makers in healthcare, including the National Institute for Health and Disability Insurance (NIHDI) outside this specific context, on all types of health technologies or interventions (Fig. 15.1).

Involvement of healthcare professionals, experts and decision-makers as stakeholders has been part of KCE's HTA process since the beginning, but patients were only formally involved since 2015, with the appointment of a patient representative in the Board of KCE. Systematic patient involvement in HTA required a cultural change

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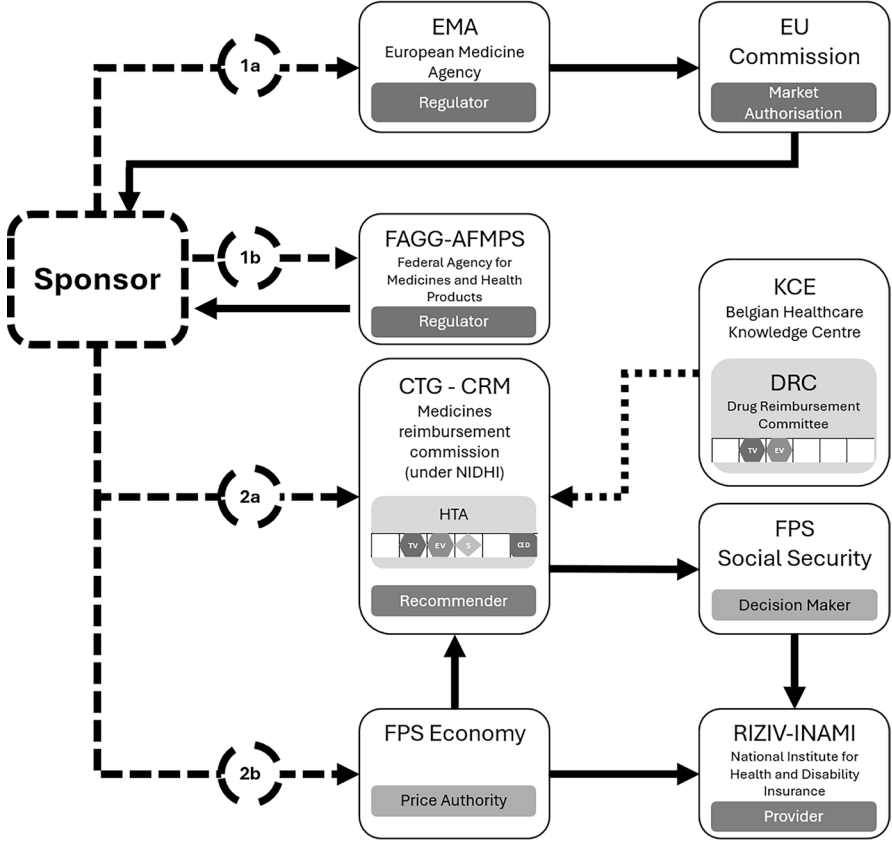


Fig. 15.1 Belgian reimbursement process of a HTA. (Allen 2017, CC-BY)

within the organisation. KCE has been working on creating the conditions for better patient involvement since 2012, starting with the publication of a report on stakeholder involvement in KCE work processes (Piérart et al. 2012). A year later, it surveyed 80 stakeholders in the healthcare sector (Christiaens et al. 2013) on the acceptability and feasibility of citizen and patient participation in health policy (see Sect. 15.3.1).

15.2 Current Structures for Patient Involvement in Belgium

Patients’ desire to play an active part in the decision-making process in healthcare began in the 1990s, in a general context of citizen emancipation that had taken hold in many Western countries (Denis and Teller 2011). The voice of patients can be heard through various channels: through individual patients, through patient organisations, or umbrella organisations representing multiple patient groups (Cleemput

et al. 2019). Patients can have different hats: patients as citizens, patients as experts and patients as representatives. Depending on the group, a specific role and related activities can be defined (Table 15.1).

The term ‘patient’ in the table above can be understood as:

- relatives or informal carers (non-organisational/individual), carers such as the parent of a sick child
- patient-experts: experts by experience with knowledge about their disease and about scientific approaches for policy research (trained or acquired through frequent involvement in research projects)
- patient representatives: either of specific patient groups (e.g. cancer survivors, haemophilia patients) or representatives of patients in general (e.g. collaborators of an umbrella organisation of patient organisations).

In addition to the ‘classic’ patient representatives cited above, sickness funds in Belgium also play the role of representatives of healthcare users in several decision-making bodies.

Table 15.1 Types of patient representatives defined in KCE process note (Cleemput et al. 2019)

Types of patient representative	Definition
Individual patients with individual experiential knowledge	Patients recruited for their personal experience, belonging or not belonging to or representing a patient organisation or other organised form of patient representation—random selection
Individual patients with collective experiential knowledge	Patients with collective knowledge based on contacts with other patients, either through a patient organisation or through an informal gathering of patients (e.g. as moderator of a Facebook group or spokesperson of a group of people). These patients do not formally represent a patient organisation—Purposive selection
Patient organisations	Formal gathering of patients (and professionals) aiming at advocating, supporting and promoting patient issues and rights. The size, the degree of formalisation, the presence of healthcare professionals and the objectives—Among others—Vary from association to association. Patient organisations usually concern a specific disease, health condition or symptom.
Sickness funds	Sickness funds represent patients as health care consumers under the term of national health insurance. They represent all the patients registered to their funds and are—In a certain sense—Influenced by a specific ‘ideological’ perspective (socialist, liberal, Christian, independent) although differences are more historic than really influencing the current practices.
Patient umbrella organisations	Patient umbrella organisations regroup numerous patient organisations and aim at advocating for patient rights from a general perspective—Without referring to a specific disease, health condition or symptom. Patient umbrella organisations can also represent patients for whom no association exist. It should be noted that not all patient organisations are members of patient umbrella organisations.

Belgium now has more than 400 patient organisations (with more volunteers than employees with or without financing) and three national umbrella organisations of patient organisations: the *Ligue des Usagers des Services de Santé* (LUSS) for the French-speaking community, the *Vlaamse Patiëntenplatform* (VPP) for the Dutch-speaking community and the *Patienten Rat und Treff* (PRT) for the German-speaking community. In addition, the *Patient Expert Center* (PEC) plays a transversal role at the national level by training patients to become expert patients in their disease-specific areas, patients who will be involved in healthcare research and policies.

These different types of patient representatives can contribute their expertise to several decision-making bodies at all levels of Belgian authority concerned with health (federal, regional, community and local). For example, the LUSS and VPP are represented in decision-making bodies of the NIHDI, the Drug Reimbursement Commission (CRM/CTG) or the Federal Medicines Agency. In addition, LUSS and VPP also collaborate with research centres in Belgium that engage in patient involvement in research: universities, high schools with curricula related to the field of health and social sciences, public research centres, and non-academic research centres or initiatives.

15.3 Patient Involvement at KCE

15.3.1 A Story of Organisational Culture

KCE was established by a programme law in 2002 and started its activities in 2003. As a semi-governmental agency, it works independently from any particular governmental institution: the agency is entirely financed by public means. Its mission is to perform policy preparing research in the healthcare and health insurance sector. It gives advice to policymakers about how they can obtain an efficient allocation of limited healthcare resources that optimises the quality and accessibility of healthcare.

The initial idea was to limit the mission of KCE to HTA but the need for broader policy supporting research led to adding supplementary domains to KCE expertise. Therefore, KCE has three domains of research: Health technology assessment (HTA), Good clinical practice (GCP) and Health services research (HSR) (Cleemput and Van Wilder 2009). Figure 15.2 summarises the steps that led to the actual KCE model of patient involvement. The model was co-designed with the patient umbrella organisations (see Sect. 15.3.2).

Over a 10-year period (2012–2022), KCE introduced initiatives demonstrating its commitment to involving patients in its HSR and HTA research projects:

- In 2012, KCE carried out a study with the aim of describing the various possible models for involving the general public and patients in the healthcare decision-making process at different levels and assessing their acceptability for stakeholders in Belgium, including researchers and decision-makers (Box 15.1).

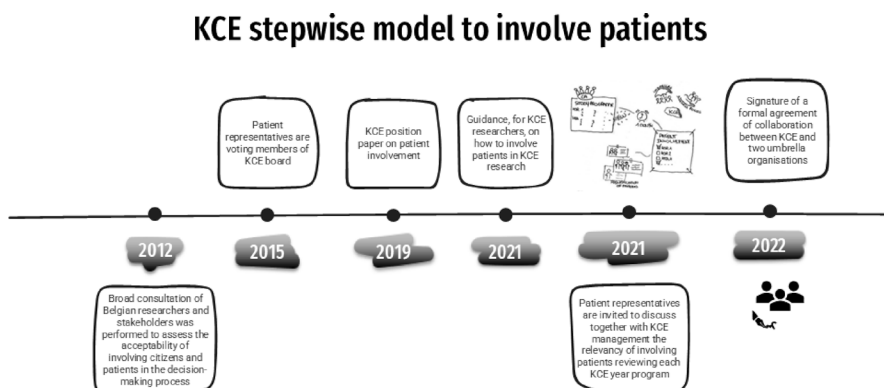


Fig. 15.2 KCE stepwise model to involve patients in its research

- Since 2015, representatives of the four Belgian umbrella organisations of patients' associations (LUSS, VPP, PRT and RaDiOrg) are full members of KCE Board (2 permanents and 2 substitutes).
- In 2019, KCE published a position paper stating its commitment to involve patients more systematically in its research. This was followed by the publication of a methodological guide for KCE researchers (see Box 15.1).
- In 2022, a yearly agreement was signed to formalise the collaboration with the umbrella organisations
- Since 2021, the participation of patients is anticipated before the start of certain studies (see Sect. 15.3.2).

Box 15.1 KCE Position Paper and the Creation of a Methodological Guide

At the beginning of KCE activities, while patients were occasionally involved in research projects, their role was not structured systematically. In its position paper (Christiaens et al. 2012), KCE outlines its stance on patient participation, exploring its methods and limits, and establishing a 'roadmap' for more systematic inclusion.

Rationales for Patient Participation:

1. Ethical: Patients have a moral and democratic right to participate in research that affects them. Their lived experiences provide unique perspectives often missing from scientific approaches.
2. Instrumental: Involving patients enhances the relevance of research, builds trust in outcomes, and facilitates the implementation of recommendations by addressing patients' real needs.

(continued)

Box 15.1 (continued)*KCE's Approach:*

KCE advocates for an 'integrated consultative approach', involving patients regularly across the research cycle. The specifics of who, how, and when are detailed in an operational guide. Final decisions must balance patients' input with societal objectives.

Final Position and Recommendations:

- Involve patients in all relevant research phases
- Provide adequate resources, training for researchers and patients, and regular process evaluations
- Document patient contributions transparently in reports
- Develop methodologies to maximise the relevance and impact of patient participation while respecting constraints.

Practical Guidance and Next Steps:

The KCE 340 report (Kohn et al. 2021), titled *Process Note: Patient Involvement in KCE Research* provides practical advice for integrating patients at different stages of research projects. It identifies potential barriers and proposes solutions to actively engage patients. A complementary practical guide offers techniques to make patient participation effective and engaging, and applicable to other groups as well.

Both documents are designed as living tools to be updated based on future experiences. They underline the importance of patient participation in enhancing the quality and relevance of health research, reaffirming KCE's commitment to more patient-centered policies.

Box 15.2 Description and Results of KCE Study Models for Citizen and Patient Involvement in Health Care Policy Part I: Exploration of Their Feasibility and Acceptability

In 2012, KCE carried out a study (Christiaens et al. 2013) with the aim of describing the various possible models for involving the general public and patients in the healthcare decision-making process at different levels and assessing their acceptability for stakeholders in Belgium, including researchers and decision-makers. To achieve these two objectives, a literature review (to describe the different models of involvement) and a Delphi survey (to assess acceptability) were carried out. The Delphi survey consisted of two rounds in order to reach a consensus on the acceptable models of involvement. Participants invited were respected experts on decision-making processes for reimbursement in the context of the Belgian healthcare system.

Eighty stakeholders participated in both rounds of the Delphi survey. Consensus was defined as 65% or more agreement on a proposal and a maximum of 15% disagreement/unimportance.

(continued)

Box 15.2 (continued)

This wide consultation raised a strong desire to involve patients in both decision making and at operational levels. According to the majority of stakeholders taking part in the Delphi panel, citizens and patients could be involved at different levels and with varying degrees of intensity. The raised arguments in favour of patient involvement in HTA related to the increased relevance of the scope of the studies, expertise with data collection, potential to bring in fresh ideas for the study design, access to survey participants, validation of data analyses, and better adherence to recommendations.

15.3.2 Co-Design of Involvement Processes

Throughout the research process, KCE researchers involve a range of stakeholders, including patients.

The first consultation of the umbrella organisations and the sickness funds takes place after the approval of the year's programme by the KCE Board. Since 2021, KCE management invites several patient representatives¹ to a meeting in order to identify the projects that could possibly benefit from patient involvement. This meeting is also a first brainstorming on the type of collaboration to perform. Several weeks in advance, they receive the list of the studies approved by KCE Board, with a description of the context, objective and planned working method for each study. They are then asked to give their opinion on the relevance/necessity of involving patients in each study, by applying a score: score one when patient involvement is absolutely necessary; score two when patient involvement is nice to have but not necessary and score three when not necessary or unclear. Of the 13 study projects included in the research programme for 2024, eight (five HSR projects and three HTA projects) were considered to require patient involvement. Studies that do not involve patients generally concern highly technical subjects such as hospital financing. In research projects, patient representatives are either consulted—through a targeted approach—or fully integrated in the project teams as research partners. Hence, at KCE patient participation means much more than filling questionnaires or participating in interviews or focus discussion groups. Patient participation implies an active role of patient representatives and of patients in the study development. This is put in place through two different approaches. The targeted approach consists in asking patients about specific aspects of the study (e.g. to test a survey before it goes online and to give feedback to the KCE researchers on its readability); they are consulted at a specific time and generally only once. A second approach is a research-integrated approach (referred as an embedded collaboration) where

¹Four umbrella organisations (LUSS, VPP, PRT and RaDIOrg), sickness funds (as representatives of the healthcare user in general) and an independent public institution that fights discrimination and promotes equality (Unia).

patients give their opinion and share their knowledge throughout the research process, at different agreed timepoints.

Besides the annual meeting, additional collaborations take place (especially with LUSS and VPP, but sometimes with specific patient organisations) according to the study's needs. The type of collaboration is further specified during the development of the project plan by the KCE experts. The suggested approach is presented to the umbrella organisations. Once a project has formally started (i.e. at the internal kick-off meeting), the umbrella associations help to identify and/or contact the appropriate patient organisation(s) (or individual patients if no patient organisation exists) to involve in the study project. These will take on a role as external advisors, as other external experts or stakeholders, or as study participants if primary data collection is needed.

In addition, umbrella associations can be involved in the advisory committee of the project (if one is established) to ensure appropriate communication with patients (for instance, to review data collection tools and methods, but also provide support to patients involved in KCE projects if they feel this need).

Umbrella associations are also involved directly as experts or as stakeholders in KCE projects, when the topic concerns patients in general, or for a transversal health issue, rather than patients with a specific condition. In that case they are invited to provide input like other external experts.

Finally, umbrella associations are invited at the end of the study to help disseminate the results through their own communication channels. If the press release produced by KCE is not sufficient, they help to summarise the main messages in plain language to make them accessible to the broader public.

15.3.3 Integrating Patient-Based Evidence: Qualitative Research

At KCE, patient participation is facilitated through specific tools and approaches such as structured consultation at different stages of the research cycle, patient advisory committees, practical tools for patient engagement (e.g. guides and frameworks), training for researchers and patients to optimise collaboration, and clear communication strategies to ensure inclusivity and effectiveness. Approaches like embedded consultation, and user-led decision-making are used depending on the context and goals of the research.

Beyond involving patients through these means, experiential evidence that was generated through qualitative research methods can be used. In qualitative research, integrating experiential evidence is recognised as a valuable methodological approach, enabling a deeper understanding of lived experiences. KCE uses qualitative research methodologies, including the integration of experiential data, to enhance the comprehensiveness and richness of its analyses. However, the KCE does not categorise the use of collected experiential data (such as filled questionnaires) as patient involvement as such. Indeed, KCE has established specific tools

and approaches for involving patients in research projects. These tools and methods are described in the process note provided to KCE researchers, outlining strategies and protocols for effectively involving patients in the research process (Kohn and Christiaens 2012).

Until now, the tools and methods described in the process note have only been used in HSR projects, but not in any of the HTA studies carried out at KCE. However, patients have been actively involved as patient research partners in HSR projects. For example, HSR project *Somatic health care in a psychiatric setting* (Jespers et al. 2021) was one of the first KCE experiences in research partnership with patients.

15.3.4 Evaluation of Participation

As already explained, KCE involves patients at every stage of its research process, from scoping to validation of reports to the Board and their dissemination. KCE aims to evaluate its patient participation methods by collecting patient feedback on how they experience this participation (e.g. Do they consider the participation to be meaningful? Are they sufficiently prepared by KCE researchers before taking part in a study? Is the format of stakeholder meetings always adapted to their condition, and do they have any say in the matter? Finally, how are they debriefed at the end of each study project?) KCE is currently developing approaches to collect answers to these questions.

Three areas of improvement are currently tested at different moments of the research study:

- Welcoming patients: working with researchers and other stakeholders is not always easy for patients. They do not always feel legitimate enough to take part in the evaluation. One of the main reasons for this is that the subjects are sometimes a little too technical and patients, apart from their own lived experience of the health condition, may not have the technical background to contribute all the discussions taking place. KCE should prepare patients to ensure a valuable contribution to the research. To do this, it is advisable to encourage meetings prior to the assessment, rather than only sending a set of documents, and to assess the patient's level of understanding of the issue with them.
- The format of the collaboration: collaborating with patients means working with people who have an illness (sometimes disabling). It is therefore essential to work together on a collaborative format that is adapted to the patient.
- End-of-project feedback: at the end of each research project, KCE researchers organise a feedback meeting with management to debrief on how the evaluation went. The same type of debriefing should be carried out systematically after each collaboration.

15.4 Reflections

Patient involvement at KCE followed a stepwise development. First, a broad consultation of Belgian researchers (included KCE ones) and stakeholders was performed in order to assess the acceptability of involving citizens and patients in the decision-making process. Then, a KCE position paper on patient involvement and a practical guidance were published on how to involve patients.

Today patient representatives participate in the KCE board, and an agreement for formal collaboration is signed with the patient umbrella associations. From the topics proposal to the validation process, patients are involved in different steps. Patient representatives are invited to discuss the forthcoming work programme of KCE in order to assess the necessity and required level of patient involvement in each research project.

Despite having progressed tremendously, there is still room for improvement, particularly in terms of welcoming, post-study feedback and format of meetings, which need to be adapted to suit patients' conditions.

Acknowledgements The authors wish to thank all patient representatives who actively contribute to the work of KCE. Moreover, KCE researchers are acknowledged for their efforts to involve patients appropriately in their work and KCE management for supporting patient involvement at KCE in terms of processes as well as in terms of resources. Special thanks to our KCE knowledge and information systems manager Patrice Chalon. None of the authors have declared a conflict of interest with respect to the content of this chapter.

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Chapter 16

Czechia: Patient Involvement in HTA



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16.1 Introduction—Patient Involvement in Czechia

The Czech Ministry of Health (the Ministry) developed a *Strategic Framework for Health 2030* (Ministry of Health of Czech Republic 2020), that included patient involvement as a specific goal. It set out how patient involvement should be established and mainstreamed in a range of areas in the healthcare system by 2030.

Following a range of collaborative efforts between patient organisations, the Ministry and the Association of Innovative Pharmaceutical Industry (AIFP), a dedicated patient involvement unit was established in the Ministry in 2017 and a permanent advisory group, the Patient Council, was established to systematise patient involvement. The ultimate goals were to increase transparency, legitimacy, and quality of decision-making processes. In 2022, the reimbursement process for orphan medicinal products (OMPs) became the first legally defined process to involve patients, building on collaborative work that had been underway for many years.

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Initially there was no national umbrella patient organisation in Czechia that would be a legitimate and representative partner to the Ministry, providing a unified voice of patients and providing a self-governed platform for discussion among patient organisations. The platform for meetings of patient representatives was provided by the AIFP Academy for Patients Organisations and later, by the Ministry. The Ministry and AIFP signed a memorandum of understanding and organised support for patient organisations to develop their capacity in areas they requested including aspects related to professionalisation and networking, and to ensure their stability. In 2021, a National Alliance of Patients Organisations was created by a group of patient organisations supported by a European Economic Area Grant, a programme designed by the Ministry of Health. The Alliance is still growing in membership.

This chapter describes how the processes for patient participation in the OMP reimbursement process were developed and enacted, and reflects on learnings from establishing these processes and the expansion of patient participation to other areas of reimbursement.

16.2 HTA and National Public Reimbursement of OMPs

In Czechia, health technologies are reimbursed based on legal rules established in the Public Health Insurance Act (1997). The reimbursement procedure for medicinal products for outpatient use follows an administrative procedure led by the State Institute for Drug Control (the Agency). The Agency uses HTA to assess evidence about the relative effectiveness, safety, unmet need, cost effectiveness and budgetary impact of a product and then makes decisions about reimbursement, determining the maximum prices and reimbursement price and conditions of reimbursement. A specific pathway for the reimbursement of OMPs was introduced in 2022 to ensure systematic, timely and equitable access for patients and improve the predictability of the costs of such medicines to the system. The pathway is intended for OMPs, where incremental cost-effectiveness ratios exceed the usual cost-effectiveness threshold used for other medicines, and therefore, previously, reimbursement would only be granted by exception for individual named patients, not for a specified population (Decker et al. 2023).

In comparison to other reimbursement pathways, the process for OMPs has been augmented to include a determination of value in which there is:

- Cost effectiveness analysis that considers a societal perspective, but the resulting incremental cost-effectiveness ratio (ICER) is not considered against a financial threshold (ICER is not decisive in OMP procedure compared to the usual procedures, where the granting of reimbursement is driven by cost-effectiveness)
- Separation of the assessment, appraisal and decision-making remits between three bodies (the Agency, Ministry's Advisory Body and the Ministry)
- Formal participation of patient organisations and medical associations as participants in administrative proceedings and members of the Advisory Body.

The new explicit legal criteria for appraisal involve:

- Consideration of the wider-societal impact of the OMP, including impact on both public health insurance system and public social care system
- Evidence for benefits in improving quality of life
- Healthcare infrastructure required to utilise the OMP.

The newly introduced processes for patient and clinical expert participation bring new inputs and ways to assess value using the above-mentioned new criteria, complementing the evidence in the health technology developer’s application. The methods for patient participation vary throughout the process. First, relevant patient organisations (formalised associations of patients whose conditions can be treated by the OMP) are lawful parties to the administrative procedure run by the Agency, with the right to contribute input and comments in the assessment phase, and appeal against the final decision on reimbursement. Given the characteristic of OMPs—small patient population, uncertainty of clinical outcomes, limited knowledge about the patient population and its size, etc.—it is hoped that patient input will bring additional information and unique knowledge to the assessment. This would include information about living with the condition and its impact on everyday life, estimated number of patients based on patient registries or any patient experience evidence. Secondly, patients are represented equally to other stakeholders in the Advisory Body panel that undertakes the appraisal (Fig. 16.1).

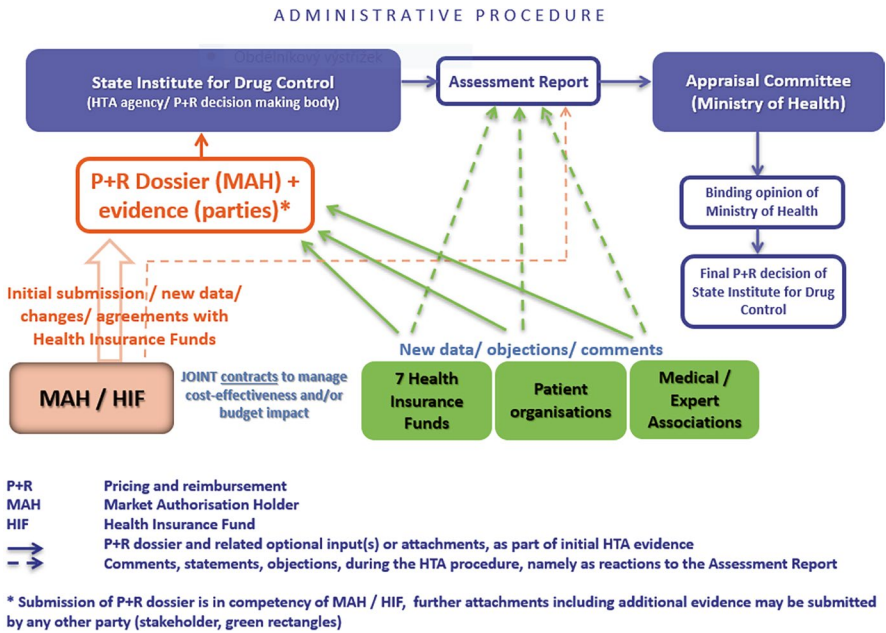


Fig. 16.1 Pricing and reimbursement procedure for OMPs since 2022 (State Institute for Drug Control 2024, 2025)

16.3 Patient Participation in Drafting the Legislation

In drafting the law, two elements were crucial to support meaningful patient involvement. First, patient representatives directly participated in drafting the law. Secondly, an intensive training programme was created and provided to patient representatives during the drafting process and afterwards.

Since the establishment of a dedicated unit at the Ministry, the principle is systematically used that any legislation affecting patients should be created with patients participating at the earliest stage possible. The already established Patient Council served to ensure patients were directly involved in drafting the law. The Patient Council created a working group on systems of reimbursement of medicinal products. The working group discussed and reached consensus on issues that arose during the development of the law. These views were presented by two elected patient representatives (chairs of the working group) to the All-Stakeholder Working Group that contributed to the drafting of the law. The patient working group usually held meetings before the All-Stakeholder Working Group. The final draft legislation was then reviewed by the whole Patient Council as part of a regular commenting procedure.

A training programme for patients on HTA was established at the Ministry in collaboration with AIFP in response to the evolving role of patients in the HTA process. Its primary objective was to prepare patient representatives for this new role by equipping them with fundamental knowledge of HTA, providing an overview of international approaches to patient involvement, and explaining the specifics of the pricing and reimbursement process in Czechia (Sect. 16.5.1). As discussions on the new law progressed, the training was honed, became a platform for dialogue, enabling patients to consult experts on legislative proposals and actively contribute to shaping policies for effective patient engagement in HTA. A key enabling factor was a collaboration with HTA expert Karen Facey who advised the Ministry and patient representatives on HTA approaches for OMPs, approaches to patient involvement and processes that might be suitable for Czechia including a workshop during which patient representatives worked on the customisation of a patient input template tailored to the Czech context.

16.4 Patient Participation in the Reimbursement Procedure for OMPs

Two methods of patient participation are currently used in the HTA of OMPs, which cover the assessment and appraisal phase. In the assessment phase, the law assigns the rights of ‘a party’ to patient organisations affected by the treatment if they fit the legally stated criteria for patient organisations (relevant patient organisation). If no patient organisation is listed within the indication, an umbrella patient organisation, Rare Diseases Czech Republic usually acts as a party within the process. Such

patient organisations are entitled to make their own submissions to the Agency using a standardised patient input template. Like other parties (e.g. medical associations), they have all other procedural rights including the opportunity to comment on the assessment report and the right to appeal the final decision. The second method of participation is the participation of representatives of patient organisations not affected by the assessed treatment in the Advisory Body at the Ministry serving as an appraisal committee member.

16.4.1 Defining Eligible Patient Organisations

To participate in the process, patient organisations must comply with the legal definition of a patient organisation, as an organisation that is entitled to represent patients as outlined in the Healthcare Services Act (2011/[2021](#)). Approved patient organisations are then listed publicly by the Ministry, which issues a certificate declaring compliance with legal requirements.

The definition was created after a 4-year consultative process led by the patient involvement unit in the Ministry, in cooperation with patient organisations in open forums and later also at the Patient Council. The criteria published by the European Patients' Forum ([2017](#), [2024](#)) and the European Medicines Agency ([2018](#)) were reviewed as part of this work.

From these a set of criteria were piloted and refined over many iterations. The criteria were also piloted as part of the process for becoming a member of the Patient Council. One of the most important conditions is the representativeness and legitimacy of the organisation, ensuring that the decision-making power lies in the hands of patients themselves, and that the organisation has a mandate to represent them. This mandate is derived from the structure of the organisation, where patients elect members of statutory or other decision-making bodies to represent them or choose their representatives.

The statutory definition was embedded into the Healthcare Services Act, and, along with the procedure for listing an organisation as eligible to participate in administrative procedures where required by law, it constitutes a complete part of the Act.

The criteria from EMA are compared with the final version in the Healthcare Services Act in Czechia (Table [16.1](#)).

The definition became the target of advocacy efforts by a small number of patient-focused organisations that provide services to patients (where patients are not in positions of authority and do not have membership rights), who sought to broaden its scope to include a wider range of entities. The original proposal by the Ministry of Health was based on EMA criteria, with the primary requirement that eligible organisations must be membership-based organisations (in Czechia 'associations') in which patients, as members, hold a decisive share in the management of the organisation, i.e. they possess majority decision-making power (criteria of legitimacy, structure, and accountability, see Table [16.1](#)). This requirement was

Table 16.1 Definition and eligibility criteria for patient organisations (European Medicines Agency 2018; Healthcare Services Act 2021/2024 Part Thirteen Section 113f)

Detailed criterion	European Medicines Agency	Czechia—Healthcare Services Act
Definition of patient organisation	Patients' organisations are defined as not-for-profit organisations which are patient focused, and where patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies.	Patient organisation is a registered association whose main activity is to assist patients and protect their rights and interests and whose members are usually persons with a specific illness or disability, their close ones (<i>as defined by law</i>) or their representatives under the Civil Code, and whose members have a major influence on the activities of the association. A patient organisation shall also mean an association whose members are associations which meet the conditions referred to in the first sentence and which elect the members of its statutory body. A patient organisation may also be an institute or a charitable organisation whose main activity consists in assisting patients and protecting their rights and interests, and where patients, close persons or their representatives under the Civil Code have a demonstrably significant influence on the activities of such an institute or charitable organisation.
Legitimacy	The organisation should, in principle, be formally established in one of the Member States of the EU/EEA. Organisations not formally established in an EU/EEA Member State may apply to become 'EMA eligible organisations' if they provide additional information that they have specific focus and carry out activities in the EU.	Registered association ... whose members are usually persons with a specific illness or disability, their relatives (close ones) or their representatives under the Civil Code, and whose members have a major influence on the activities of association. A patient organisation shall also mean an association whose members are associations which meet the conditions referred to in the first sentence and which elect the members of its statutory body. This applies <i>mutatis mutandi</i> to an institute or charitable organisation.

(continued)

Table 16.1 (continued)

Detailed criterion	European Medicines Agency	Czechia—Healthcare Services Act
Mission/objectives	The organisation shall have its mission/objectives clearly defined and should agree to have it/them published on the EMA website.	Main activity is to assist patients and protect their rights and interests; has been active in the field of providing assistance and support to patient and the protection of patients' rights and interests for at least 12 months.
Activities	The organisation shall have, as part of its activities, a specific interest in medicinal products which should be documented (e.g. through a report published on the organisation website).	
Representation	The organisation shall be representative of patients or consumers or healthcare professionals throughout the EU/EEA. Organisations already registered at Community level, e.g. in the EU Health Forum, the Council of Europe, are considered to adequately represent patients or consumers or healthcare professionals for involvement in EMA activities.	Registered association whose members are usually persons with a specific illness or disability, their relatives (close ones) or their representatives under the Civil Code, and whose members have a major influence on the activities of association. This applies <i>mutatis mutandi</i> to institute or charitable organisation.
Structure	The organisation should have governing bodies which are elected by their members, who shall be patients, or consumers, or their elected representatives or healthcare professionals	A: Members of the statutory and supreme body, if this body is not a general meeting, are elected by the members of the association, who are usually persons with a specific illness or disability, their relatives ('close persons' as defined by law) or their representatives according to the Civil Code, B: an institute or a charitable organisation whose main activity consists in assisting patients and protecting their rights and interests, and where patients, persons close to them or their representatives under the Civil Code have a demonstrably significant influence on the activities of such an institute or charitable organisation.

(continued)

Table 16.1 (continued)

Detailed criterion	European Medicines Agency	Czechia—Healthcare Services Act
Accountability and consultation modalities	Statements and opinions of the organisation should reflect the views and opinions of its members and adequate consultation procedures with those members should be in place. In particular, the organisation should ensure that the appropriate flow of information is in place to allow dialogue both ways: from and towards its members	Patients have demonstrably significant influence on the activities (initially a decisive share on the management of the organisation), members of the statutory and supreme body, if this body is not a general meeting, are elected by the members of the association. This applies <i>mutatis mutandi</i> to institute or charitable organisation.
Transparency	The organisation shall disclose to the EMA its sources of funding both public and private by providing the name of the bodies and their individual financial contribution, both in absolute terms and in terms of overall percentage of the organisation budget.	Publish its accounts and sources of funding on its website

EU/EEA European Union/European Economic Area, *EMA* European Medicines Agency

amended already during the legislative process, expanding the definition to include organisations where patients are not in a position of members with corresponding rights, but can still be regarded as having “a decisive share in the management of the organisation” (Healthcare Services Act as amended in 2021, Sect. 113f). Two years later, this condition was further broadened, replacing the requirement for a majority vote in decision-making with the more flexible criterion of having “a major influence on the activities of the association” (Healthcare Services Act as amended in 2024, Sect. 113f). As a result, the criteria were diluted, allowing a broader range of organisations to qualify.

16.4.2 Patient Organisation Submissions in Assessment Phase

The Agency notifies relevant patient organisations about new OMP procedures and asks for their input via a patient input template within 30 days of initiation of the procedure. Later in the procedure they have 15 days to comment on the draft assessment report issued by the Agency. This mechanism was effectively utilised for example when the assessment report was revised following comments submitted by the patient organisation Czech Cystic Fibrosis Association and the relevant medical association. The initial proposal by the Agency restricted reimbursement eligibility to patients with values for forced expiratory volume 1 (FEV1) of 40% or higher, as clinical data supporting the efficacy of elexacaftor–tezacaftor–ivacaftor modulator treatment were only available for this population. However, the Cystic Fibrosis Association opposed this limitation and advocated for reimbursement without an

FEV1 threshold restriction, emphasising the need for broader patient access. While the medical association provided arguments based on clinical data, the Cystic Fibrosis Association contributed essential patient-reported evidence, highlighting the significant improvements in quality of life and the reduction in exacerbations and antibiotic use among patients with FEV1 values below 40%. The submission further incorporated testimonials from individual patients, illustrating the tangible benefits of the treatment even in those with more advanced disease.

The patient input template was created by contextualising the Health Technology Assessment international (HTAi) template (see Chap. 4) for OMPs in Czechia (HTAi 2025). This was done in collaboration with the patient involvement unit in the Ministry and patient organisations during the patient training programmes and finalised by the Agency. In this template, patient organisations are asked to provide information on the impact of the disease on their life (i.e. the problems faced in personal and professional life by patients and carers), experience with current therapies, expectations of the new medicine, etc. They may also include comments on the conditions of reimbursement, particularly the indication limitations. The Agency must address patient input in its assessment report and typically does so by incorporating relevant information with a reference directly into the text of the assessment report. Additionally, all submitted patient input, along with other evidence, is included in the file provided to the Advisory Body as annexes to the assessment report. However, this inclusion occurs without explicit commentary or discussion by the Agency regarding its consideration.

Groups that do not fulfil the legal conditions to be considered as a patient organisation may also provide a patient submission, including information on themselves and connection to the cause. Such groups, however, do not have the rights of a party to the procedure and the Agency does not have to address their input in the assessment report.

16.4.3 Advisory Body Membership

The appraisal and *de facto* decision-making phase for OMPs are performed at the Ministry. The appraisal is conducted by the Advisory Body established in 2022. In this part of the reimbursement procedure, the Advisory Body appraises whether the reimbursement of the OMP is in line with the public interest, using assessment criteria listed by law. These consider benefits for patients and the impact on the public health insurance's financial sustainability. The Advisory Body consists of four stakeholder groups: government, health insurance companies, patient organisations and medical associations. These groups nominate approximately six to eight representatives, who create a pool of persons to take part in the process. For each procedure, the Advisory Body panel is created from people drawn from the pool, based on Ministry rules, and taking into account the assessed disease, conflicts of interest and their practical availability in the given time. In the panel, all stakeholders are represented equally with two members each.

Each member of the Advisory Body must provide a Declaration of Interest and a Confidentiality Agreement. The personal and professional interest towards the subject matter is assessed for each process. To eliminate an inherent conflict of interest, patients are represented by individuals from patient organisations which are not associated with the particular disease. The same rule applies to clinical experts. Members are appointed by the Minister of Health for 3 years (re-appointment is possible). To ensure fair representation of patients, the patient representatives can be selected only from patient organisations listed by the Ministry as fulfilling the conditions provided by the law. The nomination is performed via the Patient Council in consultation with Rare Diseases Czech Republic. The nominee can be a patient, carer or patient advocate. There is currently no distinction between these types of patient representatives in the Advisory Body once they have been nominated by an eligible patient organisation.

The assessment report created by the Agency is available to the Advisory Body along with the complete documentation containing submissions of all parties. Through a multi-stakeholder deliberation, the Advisory Body appraises the technology following the list of criteria. The patient representatives do not have a specific role different from the other members of the panel, it is nevertheless preferable if they focus on the impact of the disease and assessed medicine on the life of patients, carers and families (or ‘close ones’) and bring patients’ perspectives into the discussion. Taking into account the recommendation of the Advisory Body, the Ministry then issues a binding opinion, which is then formally transformed by the Agency into a final reimbursement decision after a communication with the health technology developer (applicant).

16.5 Supporting Patients to Participate in HTA

Along with the political will that championed patient participation in the development of the OMP reimbursement legislation, there was a need to prepare patients for their future role. A training programme for patient representatives was introduced. However, to ensure sustainability of the participation, additional support is needed, notably systematic and transparent financial support of patient organisations in conjunction with their professionalisation.

16.5.1 Training Programme

In 2018, almost four years prior to the legislation coming into effect, a comprehensive training programme was launched by the Ministry patient involvement unit and AIFP, which both organisers contributed to equally in terms of funding and programme development. Speakers were chosen to ensure impartiality of the content. The courses consisted of five intensive training days given consecutively plus 2 days after a

6-month period. The course covered the public healthcare system and its financing, reimbursement procedures, and HTA, including patient and clinical expert involvement. The lecturers included experts from academia, the Agency, the Czech Pharmacoeconomics Association, the Ministry and independent HTA experts.

Courses have been held annually since 2018. Over time, the curriculum of the course was adjusted to better meet the needs of patient representatives and the evolving health system, HTA and involvement landscape and the capacity of organisers. The number of training days was reduced, mainly due to the availability of other educational options and the decrease in national public funding for the activity. Other educational activities were developed within the AIFP Academy for Patient Organisations, the Ministry, and more recently established EUPATI (2025) courses in Czechia, that also contain a 1-day HTA module created in cooperation with the Agency. Completing the educational HTA course was a desirable, but not essential criterion, for appointment to the Advisory Body and its panels. Given the existing tools created by patient organisations themselves, the Ministry is considering holding courses for patient organisations in this field only on their specific role in the procedure led by the Agency and the Ministry.

16.5.2 Other Enabling Conditions—Financing, Remuneration, Plain Language

Sustainable and effective patient participation is only realistic and sustainable in an environment that creates conditions for the stable functioning of patient organisations. One of the most important of these conditions is funding that ensures stability, whilst maintaining the impartiality and independence of the patient organisation (Hunter et al. 2018). Some HTA bodies do not permit participation of patient organisations that have funding from health technology developers, but in Czechia a portfolio of funding from public and private sources is encouraged to ensure sufficient, diverse and transparent financing, and overcome potential conflicts of interest.

Public funding of patient participation is an important aspect to consider and in Czechia, the Ministry remunerates patient representatives and clinical experts for participating in Advisory Body meetings. In 2020, research and advocacy activities undertaken by patient organisations, including collecting patient experience data (patient-based evidence, Chap. 3), were added as activities that could be supported by ministerial grant schemes. In 2021, a programme of European Economic Area Grants (EEA Grants) was launched for a 3-year period to support professionalisation and stabilisation of patient organisations. In 2025, following extensive preparatory work, financial support for patient organisations was incorporated among the activities eligible for funding under the newly established General Benefit Activities Fund, which will be established within health insurance companies. This amendment to the Public Health Insurance Act is scheduled to take effect in 2026.

Other enabling conditions include the existence of support structures, such as dedicated personnel in public institutions providing support to patient organisations, ensuring the quality of participation. The patient involvement unit at the Ministry of Health originally consisted of four full-time employees responsible for designing and administering patient participation processes. This included managing the Patient Council and its working groups, facilitating their role in commenting on legislation and strategies developed by the Ministry of Health, coordinating communication between patient organisations and the Ministry's departments and units, organising meetings, conferences, and other events, as well as designing and delivering educational programme and other initiatives aimed at ensuring meaningful patient participation. From 2021 to 2024, the unit expanded with an additional four full-time equivalent employees under the Patient Hub project, funded by the EEA Grants. This initiative provided a unique educational, co-working, and networking space—both virtual and physical—to strengthen the capacities of patient organisations, which were simultaneously supported through an open call within the EEA Grants. Most employees of the unit are patients or former/current patient advocates, bringing not only strong motivation but also a deeper understanding of patient needs, which is presumed to enhance communication with patient organisations and better outcomes. Conflict of interest is actively managed. Currently, the unit is expanding its scope beyond patient participation to include broader public engagement, including participation of people receiving healthcare for a condition which is not chronic.

The accessibility of the process and materials is also important, namely the use of plain language where possible. Since 2020, the Agency has been publishing plain language summaries of assessment reports for new medicinal products and new indications on the Agency's website. Adjustments were also made to the structure and language of the assessment report provided to the Advisory Body, learning from experience of the new reimbursement procedure and needs of its members.

16.6 Reflections

At the time of writing, after 3 years' experience of patient participation in the OMP reimbursement process, we do not have sufficient data to fully evaluate the process. However, it is already possible to reflect on several benefits and enablers as well as potential areas for improvement.

Among clear benefits and enablers is the existence of a training programme. This helps patient representatives fulfil their role effectively and has aided the acceptance of patient participation as a new element by other stakeholders, especially in passing the law, while the legislators and public needed to be assured that patients will be able to take up their new role. Their capability and expertise were later demonstrated during the meetings of the Advisory Body and acknowledged by representatives from the Ministry and other stakeholders, who requested a training for all members of the Advisory Body following the success of training for patient representatives.

A necessary requirement is ongoing purposeful and open communication between stakeholders, namely the Agency, the Ministry and the patient community. This helps to build and sustain trust which is essential in driving cultural change.

The collaboration and unity of the patient community was crucial in the negotiations introducing patient involvement into the law. One of the most frequently discussed points was the legal definition of patient organisation written into the legislation that gave them rights as a ‘party’ entitled to represent patients in the process. Organisations that were not associating patients or led by patients, although they provided services or support to specific groups of patients, were excluded from the proposed definition. Several of those advocated against it and were partially successful (Sect. 16.4.1). This disagreement was confusing to the public and legislators, impacting the perception of unity and trustworthiness of the patient sector, which jeopardised the acceptance of the law. The discussion on whether and how to include organisations that do not directly associate patients, but provide services to them remains ongoing, especially in relation to patients that are not able to represent themselves and for whom these organisations offer unique insight into their needs.

During the initial stages of implementation, a collaborative and solution-orientated approach was needed by all stakeholders. For example, the materials provided by the Agency and other parties were amended according to the needs of new subjects to ensure effective exchange of the information required for assessment.

Managing conflicts of interests is another challenge for future consideration. The participation of relevant patient organisations and medical associations only during the assessment phase helps to eliminate inherent conflicts of interest in the decision making. However, it also limits the possibility of the Advisory Body to consult relevant experts beyond obtaining the assessment report, containing parties’ submissions.

Another aspect to investigate is the possible different quality of representation of patients in the Advisory Body when they are represented by a person that is personally living with a condition, opposed to a representation by a carer. Perceptions and goals of these groups may differ.

Financial constraints continue to pose a barrier to patient involvement. While there were several partial instruments to supplement other revenue sources in patient organisations’ financial portfolios (Sect. 16.5.2), these efforts have been either temporary or insufficient to secure long-term, sustainable patient engagement.

16.7 Next Steps

The reimbursement process of OMPs is the first to create a process in which patient representatives can participate, and it has done this in a secure fashion via legislation. This serves as a pilot for patient participation in other reimbursement processes in Czechia, such as the reimbursement of highly innovative medicines, medical

devices and other health technologies. Therefore, the next step is an evaluation of the current practice and a proposal to adapt it to the specifics of other processes.

At the time of writing, crucial work is in progress to establish a mechanism for financing of patient organisations to ensure their sustainable and high-quality involvement in healthcare processes. There has been an evolving discussion among stakeholders, mostly the patient organisations, government, health technology developers, and public health insurance companies, on how to achieve this goal. The discussions, including options such as a shared fund or contributions of public health insurance funds, are ongoing. The schemes may differ according to the aim of support—there may be different means to support patient participation and provision of services to patients. The task is complicated due to scarce resources, but necessary to sustain meaningful patient involvement. The most recent advancement is the newly established possibility to support the activities of patient organisations from the General Benefit Activities Fund of the health insurance companies, a mechanism that was adopted in 2025 and is scheduled to enter into force in 2026.

Acknowledgements The authors would like to acknowledge the stakeholders and colleagues who participated in the creation of this process: representatives of patient organisations, employees of Ministry of Health, State Institute for Drug Control, Association for Innovative Pharmaceutical industry, experts from Czech Association for Pharmacoeconomics and especially Karen Facey and her colleagues from HTAi.

The authors would further like to acknowledge the editors of this publication for their extensive expert support and a possibility to share our experience.

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Chapter 17

England: Continuously Improving Processes for Patient Involvement Throughout HTA



Laura Norburn, Mark Rasburn, Heidi Livingstone, and Stella O'Brien

17.1 Introduction

The National Institute for Health and Care Excellence (NICE) is established in English law to provide national guidance and advice to improve health and social care. NICE's role is to help practitioners and commissioners to get the best care to patients, fast, while ensuring value for taxpayers in England (NICE 2024a). From its inception in 1999, NICE has supported the participation of patients and carers (informal caregivers) in shaping its guidance, of which HTA is one example. NICE's approach to patient involvement is outlined in its policy (NICE 2013), and ambitions for having a best practice approach to involvement and engagement are laid out in its strategy for involving and engaging people and communities (NICE 2024b).

This chapter describes NICE's approaches to patient involvement across all HTA guidance types. This includes the role of patient organisations and individual patients. The staff, training, templates, and written support resources NICE provides to support patient involvement are also described. The role of the 'lay member' and its critical function in building the bridge between HTA committees and patients is explored, with first-hand experience shared. Continuous improvement in how NICE involves patients and captures their impact is an important element of NICE's patient involvement work and this chapter highlights new approaches NICE has taken, working with patient organisations, to gain deeper insights into the experiences of patients and carers.

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17.2 How NICE Supports Patient Involvement

NICE has a dedicated team, the People and Communities Team, which supports and advises on patient and public involvement across all its work programmes. The team helps identify, train and support the ‘lay’ people and patient organisations involved in each piece of guidance. They also provide written materials to support patient organisations and individuals to participate (NICE 2024c) and evaluate NICE’s involvement approaches with a view to improving their quality.

The team’s work is supported by NICE’s public involvement policy (NICE 2013), strategy (NICE 2024b), NICE’s HTA process and methods manual (NICE 2022d), and its core principles as set out in its Charter (NICE 2024d). The strategy introduces new, flexible ways of involving and engaging people in NICE’s work with a focus on impactful involvement, tailored approaches, developing an innovative culture where NICE tests and learns alongside people and communities, greater partnership working, and embedding a culture of curiosity for involvement and engagement across NICE. Highlighted in this chapter are some early developments in implementing the strategy in HTA.

The terminology used within this chapter is consistent with the terminology used throughout the rest of this book. Some of these terms used are not consistent with those used by NICE within its processes. For example, the term ‘patient input’ is used in Sect. 17.2.3 whereas NICE would describe this type of information as ‘patient evidence’. At NICE, the term ‘evidence’ is used to cover a wide range of information sources from formal published research (both qualitative and quantitative), expert submissions and commentary, through to individual testimony and experience. As NICE takes forward its strategy for involvement the terminology used to describe the people NICE works with is also evolving, moving away from the term ‘patient’ and ‘lay member’ to more fully reflect the people and communities NICE works with.

17.2.1 *NICE’s Approach to Patient Involvement in HTAs*

NICE HTA guidance is developed by independent advisory committees. Usually, committees meet in a virtual format using online meeting software. Hybrid (in-person and online) meetings take place a few times per year to help maintain committee cohesion.

Patient involvement in these programmes seeks to embed patients’ unique perspectives on living with and being treated for their illness, condition, or disability, and their experiences of care. The committees make recommendations across a wide range of clinical topics. Patient involvement in HTAs at NICE has been developed iteratively, following best practice, over the past 25 years. Where possible the approach aligns with the Health Technology Assessment international (HTAi)

Patient and Citizen Involvement in HTA Interest Group (PCIG) Values and Standards for Patient Involvement in HTA (HTAi 2014).

17.2.1.1 Health Technologies at NICE

NICE's HTA programmes make recommendations about the funding and use of medicines, medical technologies and procedures for England's National Health Service (NHS). NICE's HTA programmes are focused in two main areas:

- medicines: technology appraisals (NICE 2024e)—medicines with a marketing authorisation (Medicines and Healthcare products Regulatory Agency 2025) and highly specialised technologies (HST) (NICE 2024f)—medicines for very rare diseases
- healthtech: medical technologies (NICE 2024g)—new or existing medical devices and digital technologies, and diagnostics (NICE 2024h)—new diagnostic technologies.

An additional programme, interventional procedures (NICE 2024i), looks at the safety and efficacy of new and innovative surgical procedures, but does not consider issues of clinical or cost effectiveness.

To give a sense of scale, in the 2023/24 financial year NICE published 93 TAs and HSTs, and 51 pieces of healthtech guidance. Each of those pieces of guidance was developed with patient involvement included. Figure 17.1 presents a summary of the NICE HTA process, the opportunities for patient participation at each stage and the support offered to facilitate that involvement.

17.2.2 The Role of Patient Organisations in HTA

17.2.2.1 Identifying Patient Organisations

At the beginning of a HTA, NICE identifies and contacts organisations that have a remit relevant to the HTA in question. National patient organisations are identified from existing internal databases, internet searches, and the Charity Commission (Charity Commission for England and Wales 2025).

17.2.2.2 Participation in Scoping

Topics are scoped using the population, indication, comparator, outcome (PICO) framework (Chap. 1). Patient organisations are invited to comment on a draft scope document and, in some cases, to attend a workshop if the topic is an area that NICE has not evaluated before or if there are uncertainties about the evaluation. Their insights are sought on the following key aspects:

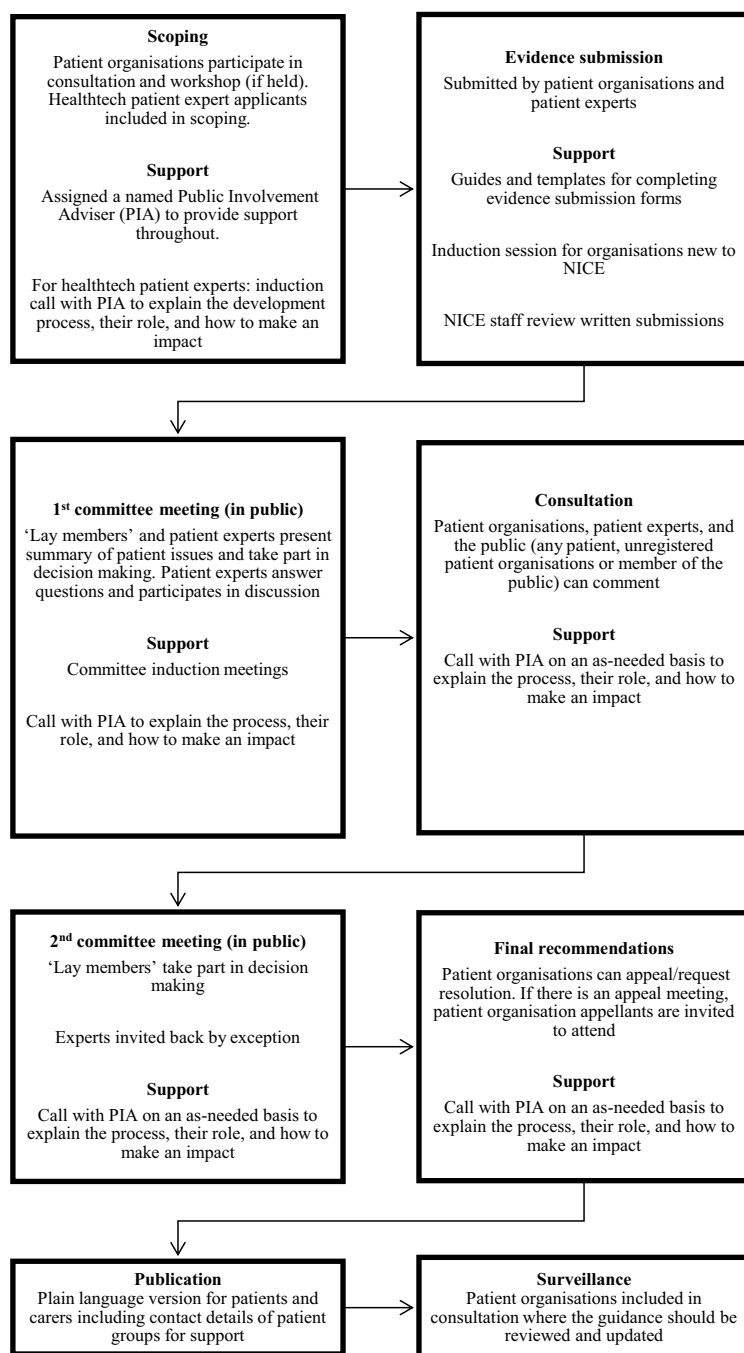


Fig. 17.1 Summary of the NICE HTA process—patient participation support

- outcomes of importance to patients
- quality of life issues which may not be captured by conventional measures, and
- tolerability, acceptability, and ease of use of the new medicine or technology compared to treatment currently available.

17.2.2.3 Submitting Input to Committees

For medicines appraisals patient organisations are routinely invited to submit input via a template (NICE 2024j). A guide written specifically to support patient organisations in writing their submission is also provided (NICE 2021a). The patient input in the submission is included in the slide presentation delivered at the committee meeting by the lead ‘lay member’. The submissions from all stakeholders are published as part of the evidence at the consultation stage of the process. Evidence submissions from patient organisations are being incorporated into healthtech evaluations, building on the learning from submissions in medicines appraisals.

In 2022, NICE began to introduce the *Summary of Information for Patients*, adapted from the work of the HTAi PCIG (Health Technology Assessment international 2021), into the medicines appraisal process. The summary is a plain English summary of a company’s evidence submission, provided by the company itself using a template provided by NICE (2022b). Initial feedback from patient organisations and patient experts has been positive, and at the time of writing a review of the summary is being undertaken to identify areas for improvement and further embed its use.

Box 17.1 Enhancing Patient Input—Digital Therapy for Chronic Tic Disorders and Tourette Syndrome

In 2024, NICE evaluated digital therapy for chronic tic disorders and Tourette syndrome, as an Early Value Assessment. These assessments are a new part of NICE’s healthtech programme and allow for the rapid assessment of promising digital technologies, devices and diagnostics. The resultant evaluation is not published as full NICE guidance but gives a signal to the NHS in England about the technology and, as part of the assessment, an evidence generation plan is developed to support the collection of data to produce the evidence that would be needed for a routine NICE guidance recommendation (NICE 2024k).

Tic disorders and Tourette syndrome was a topic area new to NICE, and the conditions have significant effect of quality of life for people living with them. To fully understand this impact NICE worked with a patient organisation, Tourettes Action, to develop a survey to send to people living with tic disorders and Tourette syndrome. The survey questions captured the physical, behavioural, social and emotional impact of tics and Tourette syndrome and people’s experiences of treatment, including

(continued)

Box 17.1 (continued)

digital therapies. The survey was live for just under 6 weeks and generated 1508 responses.

Although the committee would receive analysis of the survey data to consider, as part of the commitment to test new approaches in NICE's strategy for involvement and engagement, the People and Communities Team took the qualitative data from the survey responses and created four composite digital stories. The stories were narrated by volunteers, overlain with images sourced from people with tic disorders and Tourette syndrome. The stories were played as a video at the committee meeting where the digital therapies were discussed and brought to life the impact of tics and Tourette syndrome on people's quality of life. Also present at the committee were two specialist committee members with lived experience of caring for young people with tics and Tourette syndrome, and a representative from Tourettes Action, also with lived experience as a carer who joined the meeting as a patient expert.

All committee members who responded to a feedback survey after the meeting agreed that the patient input helped to determine the recommendations ($n = 8$). They highlighted that the patient input gave them deeper insights into symptoms and personal and family impact as well as the degree of social isolation and delays in obtaining a diagnosis and accessing services that people with tics and Tourette syndrome experience.

The views and experiences of people with tics and Tourette Syndrome were reflected in the guidance for the digital therapies both in terms of patient considerations and equality considerations (NICE 2025). The two specialist committee members and the patient expert also contributed to the evidence generation plan for the digital technologies to ensure that capturing patient evidence is a key part of developing the evidence base as the digital technologies are used in the NHS in England.

Emma McNally, CEO of Tourettes Action, reflected on her involvement in the topic: "The charity receives daily contact from individuals with Tourette syndrome and their families, who are struggling to obtain a diagnosis and the necessary treatment. Many within the community feel neglected, as though no one is listening or helping them access NHS services. For some, the only option—if financially feasible—is to pay for private treatment. Being involved with the work at NICE and contributing the patient voice was incredibly meaningful to me. It felt validating and provided a platform for the community to be seen and heard. The survey and accompanying video played a vital role in advancing the patients' perspectives, it gave the community hope that changes are potentially on the horizon." (Personal correspondence).

17.2.2.4 Support for Patient Organisations

The People and Communities Team support patient organisations throughout the course of an HTA. The team contacts organisations who have not been involved in a NICE HTA before to explain the role of NICE, how the patient organisation can take part, and to encourage contributions and offer support as needed. Organisations are sent support materials at the relevant stages of the process and offered an introductory meeting. Regular induction meetings are held for patient organisations which they can register to attend at any point during their work with NICE.

The team also offers support to patient organisations to help them develop their submissions, including producing a guide to making a submission and reviewing and commenting on draft submissions to improve their quality and impact.

The NICE voluntary and community sector (VCS) network (NICE 2024l), set up and run by the People and Communities Team in partnership with VCS and patient organisations, provides a space for organisations to learn more about NICE, share expertise and knowledge, and support each other.

17.2.3 The Role of Individual Patients in HTA

17.2.3.1 Input from Patient Experts

For most HTAs, experts are invited to attend the committee meeting to give testimony and to answer questions from a patient or clinical perspective (NICE 2021b). Patient experts are either nominated by patient organisations (medicines) or can express an interest via open recruitment (healthtech). The committee chair selects two patient experts from the nominations received. For medical technology HTAs, patient experts are invited where patient input is likely to bring unique insight—for example where patients are the users or operators of the technology, or where patient outcomes and preferences are particularly important. An example of this was the assessment of the GaitSmart rehabilitation exercise programme for gait and mobility issues. The patient expert for this topic was able to give their direct experience of the GaitSmart digital gait assessment and the personalised exercise programme developed by an app following the assessment (NICE 2024m).

Patient experts are invited to provide a personal written statement (NICE 2017), but do not take part in the committee's final decision-making processes. These statements are published together with the other submitted evidence.

Experts attend the committee meeting as individuals rather than as representatives of any organisation. Ideally one of the patient experts will be someone with the condition relevant to the treatment or technology being evaluated, preferably someone using the new intervention. They provide an in-depth perspective on their individual experience. The other expert usually works or volunteers for a patient organisation and is able to offer the perspectives of a broad range of patients with the condition, and their carers.

The patient experts give the committee a unique insight into what it is like to live with a condition, and its impact on their life, their family, and their ability to work. In addition, they provide insight to the benefits, risks, tolerability, side effects and ease of use of the medicine or technology. The outcomes patients consider to be important may also differ from the clinical outcomes measured in the clinical trials and those incorporated into the cost-effectiveness analysis.

17.2.3.2 Support for Patient Experts

Each HTA has a named adviser from the People and Communities Team assigned to the topic. The adviser works with the patient experts throughout their involvement with NICE, supporting them in making a statement, should they wish to, and in preparing to attend a committee meeting. Committee meetings can be daunting due to issues such as the:

- number of people in the meeting
- use of technical terms
- extensive discussion about clinical and cost effectiveness compared with discussion of patients' issues.

The People and Communities Team has produced written guides (NICE 2022a) explaining what happens before, during and after a committee meeting and outlining the patient expert role. Online briefings are offered ahead of the committee meeting creating the space for patient experts to ask any questions they may have. A public involvement adviser meets patients before the committee meeting starts and provides support as needed. The committee chair and the 'lay members' play an important role in ensuring the individual patients feel relaxed and confident about participating in the meeting.

Following a committee meeting the adviser remains in touch to offer any support the patient expert may need. This can include guidance on the next stages of the HTA process and a post-committee meeting to explain any key issues arising from the committee discussion.

Individual patients and 'lay members' are offered a payment for their attendance, as well as reimbursement of their expenses (NICE 2024n).

17.2.3.3 Role of the Committee 'Lay Member'

Each committee has two 'lay members' who are decision makers with equal status to the other members. The 'lay members' do not 'represent' any particular group or community of patients, but bring a broad patient perspective to the committee's decision-making. Although all committee members need to consider many aspects of all decisions, the 'lay members' ensure that issues raised by patients are heard and reflected in the committee's recommendations. For each medicine HTA, one of the 'lay members' takes 'lead' responsibility, summarising and presenting the relevant patient input and evidence. Other committee members act as equivalent leads

on the clinical and economic evidence. In Box 17.2 one of NICE's 'lay members' explains how they have approached their role and the steps they have taken to amplify the patient voice in committee meetings.

Box 17.2 Enhancing the Role of the 'Lay Member'—Stella O'Brien

Prior to volunteering with NICE, I was a member of several peer-to-peer patient support forums. Sometimes there were bitter exchanges about NICE's decisions about medicines and the perception that committee members must have disregarded everything they'd heard from the clinical and patient experts.

Before I joined as a 'lay member', I noted that the 'lay' presenter's visible public contribution was to read a single slide. The slide mostly repeated information about the condition and symptoms that a clinical member had already presented during the introduction. The sole addition was to quote some brief lines from the clinical and patient expert submissions.

The written and verbal evidence from the patient experts ranged from scant to impassioned and insightful. Some experts reported that they felt intimidated by the formality of the committee meetings, and that their input felt restricted to small sections after the presentation and discussion.

I was concerned that the perceived restriction made it difficult for the experts to engage fully with the committee. With the support of the People and Communities Team and my Chair, I introduced modest changes. Before meetings I asked patient experts for permission to introduce some talking points from their submissions that they could elaborate, highlighting points where their evidence would be helpful to committee decision making. I held this conversation with the patient experts during my presentation, rather than leaving them waiting until after the entire, complex presentation was finished.

To help patient experts feel more at ease and to lessen the formality and potential overwhelm of attending a large meeting, we reassigned presenter roles. The 'lay' lead now opened the presentation, presenting the background, the current standard of care and treatment pathway, the intervention, the patient and carer perspective and a summary of the clinical experts' evidence and equality considerations. The 'lay' lead could have a dialogue with the patient expert about their lived experience. We invited the experts to comment and discuss the material in these sections as we moved through and before I handed over the presentation to my lead team colleagues for the key clinical and economic issues.

The enhanced 'lay member' role is standard for all the technology appraisal committees.

We're now designing the next set of improvements to the process to help patient organisations and experts to leverage their insights and assist committee's decision making to bring faster access for treatments to patients. 'Lay members' are working with NICE to improve the accessibility of the committee papers and materials so that committees can hear from a greater diversity of patient experts. Together, we're strengthening the epistemic justice and patient-centredness that is at the heart of NICE's decision making and guidance.

17.2.4 Consultation

The draft recommendations made by HTA committees are published on the NICE website for public consultation to seek views on whether the draft recommendations are an appropriate interpretation of the evidence considered.

Patient organisations and patient experts are encouraged to comment on the extent to which the draft recommendations have taken account of the evidence from patients' perspectives. Even where a patient organisation or expert agrees with the draft recommendations it is important for them to respond to consultations otherwise NICE only hears from those who disagree.

Where there are evidence gaps or uncertainty, patient organisations can help to provide evidence which helps to resolve those uncertainties. Box 17.3 explains how the patient organisation XLH UK provided patient evidence to help fill the evidence gaps around the role of carers in supporting people with X-linked hypophosphataemia (XLH).

Box 17.3 Filling the Evidence Gap During Consultation

In the appraisal of burosumab for treating X-linked hypophosphataemia (XHL) in adults [TA993] (NICE 2024o) the committee were uncertain as to the number of unpaid carers (often a family member) a person with XLH would need, the impact of on quality of life of caring for someone with XLH and if burosumab would affect that quality of life. During the consultation period the patient organisation XLH UK ran a survey to help answer those questions and provide an understanding of the impact on carers. One hundred and sixteen people responded, with 24 of those being patients who had been prescribed burosumab. Their responses showed a reduction in the number of carers needed and the number of carer hours required whilst on treatment (NICE 2024p). The survey responses gave the committee a greater understanding of the impact of XLH on carers to inform their decision making.

17.2.5 Publication

Following consultation, the committee meets for a second time and a final draft version of the guidance is produced. If no appeal or resolution request is received, or if the grounds for appeal or resolution are not upheld, the recommendations are published on the NICE website as formal guidance to the NHS. The underpinning evidence and submissions obtained throughout the process are published alongside the guidance.

A plain language version of each evaluation is also published, known as *Information for the Public*. Included within it, are contact details for relevant patient organisations, for patients looking for further information and support.

17.2.5.1 Managed Access Agreements

Some medicines are recommended by NICE as part of a managed access agreement. This enables patients to access the treatment for a limited time while further data is collected. Access is funded by the Innovative Medicines Fund (NHS England 2024a) or Cancer Drugs Fund (NHS England 2024b). The managed access agreement is made between the company and NHS England and includes a data collection agreement and a commercial access agreement. At the end of the period specified in the agreement, NICE reconsiders the treatment and decides if it should be recommended for routine use in the NHS in England.

For treatments funded by the Innovative Medicines Fund, a managed access oversight group meets periodically for the duration of the agreement to check the quality and quantity of the data being collected and to discuss any issues that arise as the treatment is used. Patient organisations can have an important role in contributing to the oversight group. For example, Muscular Dystrophy UK played a key role in supporting the data collection agreement for Duchenne muscular dystrophy as part of the oversight group, to inform NICE's final decision at the end of the managed access agreement (NICE 2023). They also played a crucial role in informing and supporting the patient community during the managed access period.

17.3 Evaluating Patient Involvement in HTAs

Evaluation helps NICE to learn from the experiences of patients and patient organisations and improve how we involve and work with them. NICE conducts routine evaluation with patient experts, 'lay members', and patient organisations through questionnaires. Actions resulting from the feedback are shared across NICE teams to help improve the experiences and impact of the people who take part in NICE's work.

The HTAi quality standards (2014) emphasise the importance of feedback in helping patients and patient organisations to understand how their input has been used and the impact it had on committee decision making. The People and Communities Team routinely surveys committees who develop healthtech guidance to understand how they have used patient evidence and input to help identify improvements. This approach has been extended to HST evaluations and includes providing feedback to patient organisations from the committee and NICE teams. NICE prioritised providing feedback to patient organisations taking part in HSTs as the evaluations look at medicines for very rare diseases where evidence is less available, where there are often uncertainties in terms of cost effectiveness, and where patient organisations are often very small and volunteer-led. The MPS Society shared their feedback from NICE with their members noting that they were "immensely proud to have been entrusted with representing the patient voice and to hear that our submission met all the requirements of the committee" (MPS Society

2022). NICE has now started to roll out providing feedback for some technology appraisals and is looking to expand the approach as it becomes more embedded.

17.4 Evolving the Approach to Patient Involvement in HTA

NICE has been involving patients and patient organisations in HTAs for over 25 years. However, the HTA landscape and the environments in which NICE and the organisations we work with is continually changing. NICE therefore continues to evolve and improve how patients and patient organisations are involved in HTAs so that NICE recommendations can continue to benefit from their crucial input.

17.4.1 Supporting Small Organisations

There are many small organisations that bring the unique experience of living with a condition. This can be particularly the case for rare and ultra rare conditions. Because there is a limited understanding of the natural history of rare diseases in particular, and little data available, it is vital for NICE to understand those voices and the challenges of living with a condition. Often the voices which are vital for NICE to hear from are the voices who find it most difficult to work with NICE.

Small organisations are frequently set up by family members of people with a particular condition who are passionate about helping others. They are often juggling running the organisation alongside paid work and caring responsibilities. These organisations are unlikely to have staff with expertise or experience of contributing to NICE's work and often have extreme pressures on their resources.

Additionally, a negative decision by NICE makes organisations feel they had not participated 'well enough'. This is particularly challenging for organisations set up by parents of a child with a rare condition to help other families in similar situations, as they are committed professionally, personally and emotionally to their work; it is more than just a job to them.

Prompted by feedback from, and in collaboration with Genetic Alliance UK, NICE worked with seven patient organisations to think collectively about how NICE could make sure that voices were valued in a way that takes into account the skills and capacity of small organisations and minimises the financial and emotional burden.

A number of areas were highlighted to make working with NICE both less resource intensive and kinder, including:

- earlier notice of a medicine coming to NICE
- provision of earlier support and training to prepare the organisation

- development of a menu of flexible options for participation so that organisations can choose the best way for them to contribute the views of the people they represent.

Additionally, the organisations suggested that NICE looked to Wales' approach of conducting online surveys with patients as an alternative to formal patient organisation engagement (see Sect. 17.4.2).

Improvements to the 'lay' lead role (see Box 17.2) and establishing a fixed point in the agenda for the patient experts to be asked questions before the rest of the committee meeting continues, brings patient issues to the fore early in the meeting to set the scene and ensure the patient experts have an early opportunity to be heard.

Following committee meetings, NICE now holds meetings with patient and clinical experts for either rare disease or high-profile evaluations—such as those with significant unmet need or high levels of public interest—to explain decisions and next steps to help ease the worry and burden of an initial negative recommendation.

Any amendments to processes which help small organisations participate will also help other patient organisations, although more support is always likely to be needed by small organisations. It is important that the level of support and participation is determined by the organisation and not by NICE.

17.4.2 Medicines HTA: Introducing Surveys Direct with Patients

NICE heard from small organisations that online questionnaires direct with patients would help NICE to gather patient input in a way that placed less burden on the organisation. NICE worked with patient organisations, HTA bodies in Wales who had experience of this approach, and technical staff within NICE to explore this method of input for medicines HTAs.

Two approaches were piloted, with the aim of increasing the amount and quality of patient input:

- Developing the online questionnaire using the Summary of Information for Patients (NICE 2022c) to inform the questions where there was no disease-specific patient organisation. The resulting report and data were intended to be included in the evidence presented to committee
- Developing a jointly branded questionnaire collaboratively with the patient organisation and implementing a data sharing agreement to share the raw data so that the organisation could also use the data to for other purposes such as writing a journal article.

In both cases surveys were distributed by an umbrella patient organisation and analysed by NICE. Both pilots supported patient input into a medicines HTA and reduced the resource required from patient organisations. However, each pilot had

constraints. The first pilot did not have the benefit of patient organisation expertise to develop the questionnaire. The second pilot took longer than envisaged because the data agreement needed to be drawn up and agreed by both organisations. It was important to understand that this approach would not work for all patient organisations. Some patient organisations would not want NICE to conduct a survey or partner with them. Other organisations were concerned whether there would be a hierarchy of evidence depending on whether NICE conducted a survey or whether it was conducted by a patient organisation without NICE. NICE will continue to develop this approach by:

- evaluating what has been achieved so far
- developing a checklist and criteria, as Wales has (Chap. 27), to guide decisions as to when to consider this approach and who to include in making that decision
- sharing mutual learning with patient organisations about the approach to inform future developments.

17.5 Looking to the Future

NICE's work to improve participation through developing more tailored options for patient organisations to input into HTAs is an exciting development. Supported by our 3-year strategy (NICE 2024b), we will continually innovate and work collaboratively with patient organisations and individual patients to design options for involvement that can be tailored to organisations' resources and NICE's needs. We will also work to ensure that diverse voices that are often marginalised or under-represented have a stronger influence in NICE's work by developing new ways to engage with those patients and organisations in a way that works for them.

Acknowledgments We would like to acknowledge the previous authors of this book chapter, Victoria Thomas, Lizzie Thomas and Gillian Leng for their foundational work and contribution to patient and public involvement at NICE. We would also like to acknowledge Emma McNally, Chief Executive Officer at Tourettes Action for her work with NICE on the survey and patient stories for tic disorders and Tourette syndrome, and Louise Fish, former Chief Executive of Genetic Alliance UK for her input into the work supporting small organisations.

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Chapter 18

European Union: Patient Involvement Model in HTA Under the European HTA Regulation



Margaret Galbraith, Valentina Strammiello, and Anne Willemsen

18.1 Introduction

Patient involvement in European health technology assessment (HTA) is essential for understanding and assessing wider implications of coverage and reimbursement decisions for patients, their relatives, carers, and the general population (Dimitrova et al. 2022; Elvsaaas et al. 2021; Hunter et al. 2018). As of January 2025, Regulation (EU) 2021/2282 (HTAR) is expected to facilitate patient involvement in European joint work on HTA, as it is the first European-wide legislation enforcing patient involvement in HTA work. This expectation is supported by findings from the Health Technology Assessment international (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) which found that having a government mandate may help to motivate patients to participate in HTA (Holtorf et al. 2024).

The process for patient involvement in European HTA has evolved through the different iterations of the European Network for Health Technology Assessment (EUnetHTA 2006–2023) and in particular during the past 10 years. EUnetHTA has concluded and paved the way for the application of the HTAR in January 2025. Tertiary legislation, so-called Implementing Regulations (IR), provide further details on all aspects of the joint work foreseen under the HTAR. While these IR will provide more clarity on both the mandatory and optional timepoints for patient

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involvement, the exact process and format for patient involvement in both Joint Clinical Assessments (JCA) and Joint Scientific Consultations (JSC) may not become fully evident until put into practice in 2025. At the time this chapter was drafted five of the six required IR had been adopted, with those covering JCA for Medical Devices yet to be finalised. Table 18.1 below lists all the IR under the HTAR. The date for the last IR has been added during the last proofing of the chapter (European Commission 2025a, b).

The HTAR is applicable in all 27 Member States and observer status is given to European Economic Area (EEA) countries Iceland and Norway. Its overarching goal, as addressed in recital 54, is to increase quality, consistency, data availability, reduction of duplication and improve patient access to health technologies (European Commission 2021). Joint work covers a range of activities in addition to JSC and JCA including: the development of methodologies and procedures, and horizon scanning to identify the relevant technologies on which the joint work should be conducted. However, this chapter focuses only on JSC and JCA.

Importantly, the remits for HTA at the European and national levels are different. In accordance with the subsidiarity principle addressed in article 5 of the Treaty on the European Union (European Commission 2016), the JCA report focuses solely on the clinical assessment without any conclusions on added value or decision making, whereas at the national level, the technology will be appraised leading to reimbursement and pricing decisions thus considering national specificities. This difference in remit also means there is a different purpose for patient involvement on the European joint work compared to the national HTA activities. Therefore, any patient involvement in European joint work does not preclude but can complement patient involvement in a national setting. Although the HTAR does not have the mandate to describe national procedures, the JSC and JCA could both benefit from expert input on a national level too, for example during scoping for individual JCAs. Based on the IR for JCA for Medicinal Products as well as experiences from EUneHTA Joint Action

Table 18.1 Implementing regulations under the HTAR

Implementing Regulations	Status
Joint Clinical Assessment of Medicinal Products for Human Use ((EU) 2024/1381)	Adopted on 23/05/2024
Implementing Regulation on exchange of information with the European Medicines Agency ((EU) 2024/2699)	Adopted on 18/10/2024
Implementing Regulation on conflict-of-interest rules under the EU Health Technology Assessment Regulation ((EU) 2024/2745)	Adopted on 25/10/2024
Joint Scientific Consultations on Medicinal Products for Human Use ((EU) 2024/3169)	Adopted on 18/12/2024
Joint Scientific Consultations on Medical Devices and In-Vitro Diagnostic Medical Devices ((EU) 2025/117)	Adopted on 24/01/2025
Joint Clinical Assessment of Medical Devices and In-Vitro Diagnostic Medical Devices ((EU) 2025/2086)	Adopted on 17/10/2025

EU European Union

3 (JA3) and the EUnetHTA 21 Service Contract (EUnetHTA 21), suggestions are given in this chapter on where national input can take place and how it can be used.

This chapter explores the history and methodology for patient involvement throughout the different EUnetHTA Joint Actions and EUnetHTA 21, leading to a discussion of the envisioned process under the HTAR and the identification of challenges or gaps in the planned process. During EUnetHTA, different terminology was used: what are now known as JSC were referred to as Scientific Advice, Early Dialogues, and Parallel Consultations. For simplicity all are referred to here as JSC. Similarly, what are now referred to as JCA were known as Relative Effectiveness Assessments, Joint Assessments, or Rapid Effectiveness Assessments, are referred to as JCA in this chapter.

18.2 Evolution of Patient Involvement in EUnetHTA

Through the various iterations of EUnetHTA the process for patient involvement in European HTA evolved, specifically during the past 10 years (Galbraith et al. 2022). In the early years of EUnetHTA, the focus was on exploring grounds for collaboration in HTA and common methodology. While some of the pilot JSC and JCA included patient involvement, this was not the primary intent and the form of involvement varied (Børlum Kristensen 2012; EUnetHTA 2021; Garrett et al. 2022; Ruether et al. 2022). In the early years of EUnetHTA, the HTA Core Model was used which contains specific domains for patients' and social aspects to be addressed (Ørtenblad et al. 2017). The issues and topics shown in this model used on a high level the questions raised in the patient input template as developed by HTAi and adapted by EUnetHTA (2023; HTAi 2014).

From EUnetHTA JA3 onwards, attention shifted to proof of concept and, on a structural level, ensuring patient involvement in both JSC and JCA while still allowing for different methods of patient involvement to be developed and piloted. EUnetHTA 21 allowed for solidification of patient involvement procedures, in particular the patient input template, into a more robust procedure and supporting templates. This procedure forms the basis of discussions on the evolving approach to patient involvement under the HTAR.

18.2.1 Patient Involvement in EUnetHTA JSC and JCA

The core of today's JSC and JCA procedures, including the processes for patient involvement, results from all pilots conducted during EUnetHTA and the SEED Consortium (Shaping European Early Dialogues 2014–2015), all (co-)financed by the European Commission. Table 18.2 shows the total number of JSC and JCA conducted in EUnetHTA and SEED as well as the number of JSC and JCA that involved

Table 18.2 Number of JSC and JCA conducted and number of products that had patient involvement (EUnetHTA 2021; Galbraith et al. 2022; Willemsen et al. 2022)

	Joint Scientific Consultation (JSC) N (n): JSC produced (JSC with patient involvement)			Joint Clinical Assessment (JCA) N (n): JCA produced (JCA with patient involvement)		
	Medicinal Products	Medical Devices	Total	Medicinal Products	Medical Devices ^a	Total
Before JA3 ^{b,c}	17 (8)	5 (4)	22 (12)	7 (1)	8 (3)	15 (4)
JA3 and beyond	44 (39)	1(0)	45 (39)	20 (17)	28 (13)	48 (30)
Total	61 (47)	6 (4)	67 (51)	27 (18)	36 (16)	63 (34)

JSC Joint Scientific Consultation, JCA Joint Collaborative Assessment, JA3 European Network for Health Technology Assessment (EUnetHTA) Joint Action 3, SEED Shaping European Early Dialogues

^aMedical Devices for JCA in EUnetHTA were named Other Technologies, as they not only included medical devices but also included for example screening technologies, medical and surgical interventions

^bOnly the Rapid Relative Effectiveness Assessments are considered, as these are more alike the JCA under the HTAR

^cThe JSC numbers are deduced based on a review of the signed attendance sheets from all JSC conducted during JA2 and SEED as well as the final report for SEED (unpublished due to confidential content)

patients. A differentiation is made for the EUnetHTA projects before JA3 (i.e. JA2 and SEED), as these were considered pilots.

In JSC, the patient involvement process began by enabling an individual representative from an umbrella organisation and/or patients to participate directly as experts in the procedure. As of JA3, the process evolved to a multi-level approach based on the HTAi questionnaire (HTAi 2014) and allowed for different levels of national level and European level input. National level input was based on the national procedure in each member state with patients being involved early in the JSC process via a guided interview based on the HTAi questionnaire. This national input was shared with the other participating HTA bodies for information, but these patients did not participate in any meetings other than with the national HTA bodies. Multiple HTA bodies still include this step when elaborating their national positions.

In JCA, the process for involving patients followed a different evolutionary track. Initially, it started with the involvement of (umbrella) organisations via a consultation process on the JCA, also in addition to the involvement of individual experts if they could be identified (EUnetHTA 2015; Kleijnen et al. 2014). As of JA3, the focus was on involvement of individual patients which later was extended to patient organisations via standardised questionnaires (Elvsas et al. 2021). Although the HTA bodies conducting the JCA were involved in the identification and selection of patients, the focus of the process was on patient involvement at the European level, and it was not requested on a national level.

In EUnetHTA 21, the standardised questionnaire template was further revised and tested to harmonise the patient input received during JSC and JCA (EUnetHTA 2023). Although the template was requested to be completed in English or in the language of the JSC or JCA assessors (Elvsaaas et al. 2021), the JA3 version was translated to all official EU languages to facilitate patient involvement.

During JA3 and EUnetHTA 21, the JSC and JCA Secretariats were responsible for the identification and selection of patients. Additionally, the Secretariats performed a support function throughout the process by accompanying participating patients. For example, they explained the process to the patients, provided support with the declaration of interest and confidentiality form, guided the patients in what was expected from them, and highlighted important aspects of documentation received from health technology developers (Galbraith et al. 2022; Garrett et al. 2022; Willemsen et al. 2022).

A key EUnetHTA experience concerned the challenge of involving only individual experts, both for clinical experts as well as patients, and how to generalise the opinion of a single expert. Table 18.3 shows the other challenges that were experienced during EUnetHTA regarding patient involvement. This finding is supported by Ørtenblad et al. (2017) who advocated the importance of transnational use of joint work and argued that patients' and social aspects, as defined in the HTA Core Model, are to a large degree dependent on the context in which the input is provided. While in JSC, for confidentiality reasons, only individual patient experts could be involved, for JCA an alternative process was explored. This led to creating public questionnaires for patient organisations on lived experience and patients' expectations for a new product without providing access to the health technology developer's dossier, with the aim that these responses could synthesise perspectives of all members. Concluding on the experiences of EUnetHTA, for JCA it is considered ideal to include individual patient expertise as well as input from (a number of) patient organisations. For JSC, the EUnetHTA experience underscored the need to involve patient experts not necessarily with scientific expertise but with European knowledge (e.g. knowledge beyond the situation in their own member state) of living with the condition and of standard of care in other national contexts.

EUnetHTA 21 allowed for solidification of both patient involvement procedures and the patient input template into a robust procedure and the production of supporting templates that could be used under the HTAR. These deliverables underwent an extensive public consultation with responses received from many stakeholder groups. Building upon learnings from JA3, EUnetHTA 21 recommended that individual patients acting as European experts¹ should have broader knowledge of the situation in EU and not only their own member state (EUnetHTA 2023). Unfortunately, the production of JSC and JCA during EUnetHTA 21 was limited and therefore did not provide sufficient opportunities to pilot and refine the solidified engagement procedures and input templates.

¹HTAR recital 45: "Patients, clinical experts and other relevant experts should be selected for their subject matter expertise and act in individual capacity rather than any particular organisation, institution or member state".

Table 18.3 Challenges in patient involvement

Challenge	JSC	JCA
Identification of experts in the specific indication of the product under JSC or JCA was challenging, due to the reason that there were too specific populations or characteristics to be considered, or the patient’s burden of disease was too high to participate	X	X
Extremely short deadlines for patient involvement impacted the successful identification of patients, but also the willingness of patients to be involved	X	X
Conflict of interest must be transparent and can limit participation of the expert but has not been an issue for the ‘actual’ patient (i.e. patients that are not lead members of an organisation) in EUnetHTA	X, but less of an issue	X
Generalisability of the opinion of a single patient was very complex, as there are constraints and different interpretations, cultural differences, health system differences etc., that imply the patient opinion is not necessarily representative of all patients in Europe. It has been difficult to identify patients with knowledge greater than their own national perspective	X	X
Time compensation and remuneration	X	X
A lack of understanding of HTA and the purpose of a JSC and JCA and what patients can contribute to this	X	X
Language barrier as all work at the European level is conducted in English	X	X

JSC Joint Scientific Consultation, *JCA* Joint Clinical Assessment, *EUnetHTA* European Network for Health Technology Assessment, *HTA* Health Technology Assessment

18.3 Patient Involvement Process Envisioned Under the HTAR

The Coordination Group on HTA is established under the HTAR and is composed of Member State representatives, particularly from HTA bodies. Article 3 of the HTAR specifies the tasks of the Coordination Group and one of their main tasks is to issue guidance for the joint work (European Commission [n.d.](#)). More specifically, Article 3.7(j) of the HTAR states, “the Coordination Group shall: ensure appropriate involvement of stakeholder organisations and experts in its work”. Furthermore, the HTAR specifies on a high level certain time points for patient involvement in JSC and JCA, which are to be defined mostly in the respective IR. At the time of writing this chapter, the IR for the production of JCA Medical Devices was not yet publicly available, but it is assumed that the process for patient involvement will be similar as noted in the IR for JCA for Medicinal Products.

Next to involving patients as individual experts in joint work, the HTAR requires the creation of a Stakeholder Network and states in Article 29 that this network will “support the work of the Coordination Group and its subgroups upon request” (European Commission [2021](#)). This network was created in early 2023 based on a

call for applications by the European Commission, and after a second call for application in fall of 2024, the Network is now composed of 71 organisations with a multi-member state perspective, and two observing organisations. The membership is composed of representatives from patient associations, consumer organisations, non-governmental health organisations, health technology developer associations, and health professionals' associations (European Commission 2025a, b).

18.3.1 *Process for Patient Involvement for JCA*

The HTAR describes the patient involvement process in JCA as follows:

- Article 8(6): “The designated subgroup shall initiate the scoping process in which it identifies the relevant parameters for the assessment scope (...) The scoping process shall also take into account information provided by the health technology developer and input received from patients, clinical experts, and other relevant experts.”
- Article 11: “The subgroup shall ensure that patients are involved in the assessment process by being given the opportunity to provide input on the draft reports”.

The IR on JCA of Medicinal Products provides further clarity on these procedural steps, as well as the steps required for the identification and selection of patients (European Commission 2024). Importantly, the IR states in Article 6.5 that priority shall be given to “patients (...) who have expertise, covering several Member States, in the therapeutic area of the joint clinical assessment” (European Commission 2024). In addition to input from a patient expert (individual), the IR also allows in Article 8 to seek input from patient organisations via the Stakeholder Network. This procedure could provide input from an organisation, recognising patients as a community.

The IR provides a set of mandatory tasks, especially around the identification of potential experts by the HTA secretariat and selection of experts by the relevant subgroup (SG). Whilst these procedural details are now known, future guidance should be developed by the HTA Coordination Group (HTACG) to define what type of input is requested and how this input can be shared. Involvement of experts is dependent on a review of potential Conflict of Interest (COI) carried by the experts. A COI framework is detailed in a dedicated IR.

Figure 18.1 shows the timepoints during the JCA in which patient involvement and involvement of stakeholder organisations could take place. It addresses the mandatory steps in bold, as well as the optional steps in which the JCA subgroup can decide to involve patients.

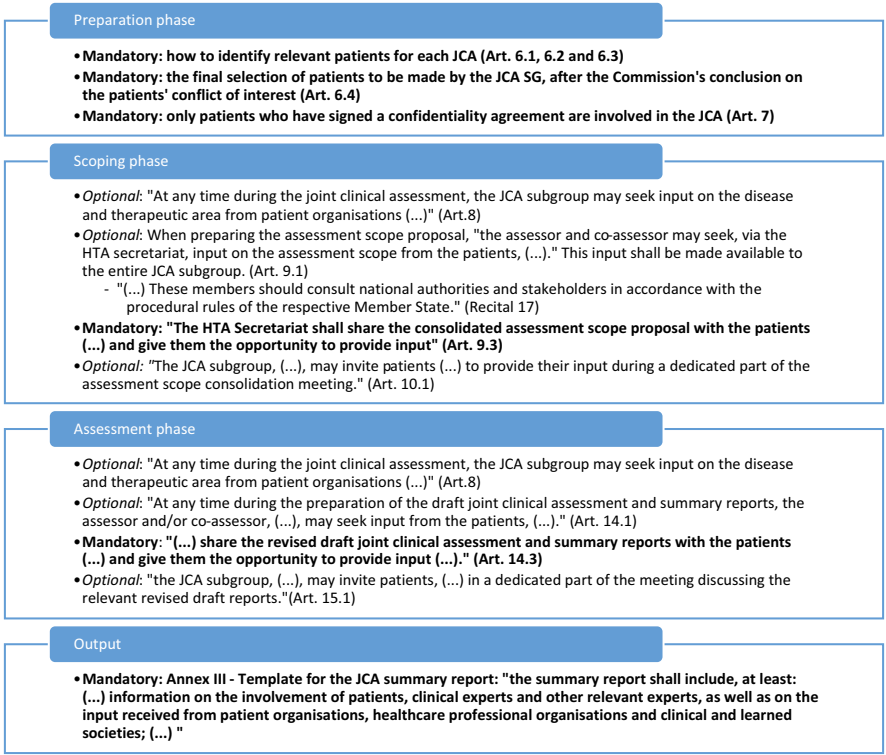


Fig. 18.1 Mandatory and optional steps for patient involvement in JCA as outlined in the JCA IR (as described by European Commission 2024)

18.3.2 Process for Patient Involvement for JSC

For JSC, the HTAR specifies in article 18(6) that “patients (...) are given an opportunity to provide input during the preparation of the draft joint scientific consultation outcome document” and in article 18(7) that “the designated subgroup shall organise a (...) meeting for an exchange of views with the health technology developer and patients (...)” (European Commission 2021). According to article 20.1(b), the HTAR shall detail procedural rules on “the selection and consultation of stakeholder organisations and patients (...) in joint scientific consultations” (European Commission 2021). The IR on JSC for Medicinal Products describes a process for patient involvement that closely aligns with what was done under EUnetHTA 21.

Figure 18.2 portrays the JSC process as described in the HTAR. Further details are provided in the JSC guidance documents.

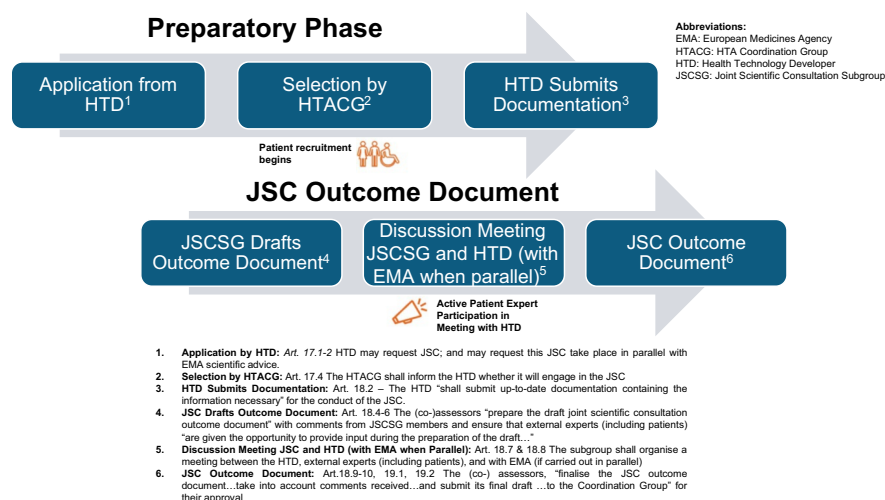


Fig. 18.2 Overview of the JSC Process as described in the HTAR (European Commission 2021)

18.4 Patient Organisation Preparation for EU and National Input

18.4.1 Training Funded by the European Commission

The European Commission has financed two complementary projects through the EU4Health programme aimed at preparing patients to participate in HTA, particularly in the context of the HTAR.

The 2-year European Capacity Building for Patients (EUCAPA)² project aimed to empower patients with the knowledge and skills to effectively participate in HTA and advocate for themselves and their communities. EUCAPA does this by offering three different types of training targeting patients with different levels of expertise and expectations. EUCAPA training include sessions on the basics of HTA, patient experts' role in HTA, methodological aspects and soft skills (EUCAPA n.d.).

The 3-year HTA4Patients project³ aims to enhance and update the education, training, and information provided by the EUPATI Open Classroom and Toolbox platform on HTA. It does so by specifically addressing the HTAR and its impact for patients through the development of dedicated training modules on patient participation in JSC and JCA. The training is in English, with translations being made available in Czech, French, German, Greek and Spanish by August 2025.

²Led by European Organisation for Rare Diseases (EURORDIS), the European Patients' Forum (EPF), and UMIT TIROL—University for Health Sciences and Technology.

³Is run by the European Patients' Academy on Therapeutic Innovation (EUPATI).

18.4.2 Other Preparations

Regardless of whether it is individual patients or patient organisations who participate on the national or European level, familiarity with the HTAR and its IRs is necessary as this allows them to have a basic understanding of what to expect and is expected of them. Although the regulations are legal documents and sometimes difficult to read, comprehension can be facilitated by attending one of the online trainings mentioned above or offered within different Member States. Additionally, there may be other, more general training opportunities, such as the open academy provided by EURORDIS.⁴ The knowledge acquired in such training will enable a more rapid response to calls for participation under the HTAR, but perhaps also at the national level. It will hopefully also enlarge the community being available for patient involvement throughout Europe.

A key learning of a patient involvement simulation workshop held by European Patients' Forum in December 2023 was that even though national patient organisations are not expected to directly contribute to European HTA, they should familiarise themselves with the tools provided under the HTAR. For example, find out how their organisation is represented within the Stakeholder Network, e.g. via an umbrella organisation, and initiate contact with that organisation (European Patients' Forum 2023). Likewise, it is important for patients and patient organisations to understand the national HTA process for patient involvement as this facilitates timely involvement when requested. It was also suggested that patient organisations should build up their repositories to facilitate the timely identification of appropriate patient experts when needed. To support and foster patient involvement in HTA, the European Patients' Forum is planning to create a mechanism to support interactions and exchange of information with national patient organisations.

Particularly in the case of patient involvement in JSC, it is recommended that patients and patient organisations become familiar with JA3 and EUnetHTA 21 methods until detailed procedural guidance on patient involvement is available under the HTAR and the respective IR.

18.5 Discussion

The HTAR only mandates the procedures and rules for undertaking joint HTA work (including patient involvement) at the European level. This does not diminish the necessity of patient involvement at the national level. As the decision-making process on pricing and reimbursement will stay at the national level, it is essential to involve patients in the appraisal process.

⁴Learn with the Open Academy—EURORDIS Open Academy.

It remains to be discussed how the HTA bodies can efficiently involve national patients in a JSC and JCA (Lips et al. 2022), due to barriers around confidentiality and tight deadlines. Such national interactions are important complements to the European involvement.

In addition to the IR for JSC and JCA, the HTACG should adopt procedural guidance and templates for interviews or questionnaires in the future to support patient involvement in joint work. Lastly, more guidance is required on how patient input could be used and more transparency is needed regarding its use in joint work (Elvsaaas et al. 2021). While the literature on research into patient aspects (Chap. 3) is substantial, it may be necessary to employ experts or train (co-)assessors of joint work in the specific qualitative and quantitative approaches described in Part II of this book to obtain and interpret patient input meaningfully (Facey et al. 2010; Lehoux and Williams-Jones 2007). Knowing these gaps is critical to fully understand the EU process for patient involvement and how this is supported by any relevant national activities. It is recommended that when developing the required patient involvement procedures and templates for joint work, not only should they build on the EUnetHTA 21 deliverables, but best practices available in Europe should also be considered and patient organisations should be consulted. For example, the 360° Stakeholder Experiences with Patient Involvement in HTA in Europe project (Holtorf et al. 2024) and the document released in June 2024 signed by 11 different patient organisations (Cancer Patients Europe et al. 2024) could serve to inform future processes. Figure 18.3 below provides an overview of the ten recommendations.

Patient involvement practices and the support provided to patients for their involvement varies significantly across Europe (Dimitrova et al. 2022). The procedures defined under the HTAR should serve as a best practice for those member states which do not have national patient involvement procedures in place and as stated by Lips et al. (2022) encourage those who already involve patients to

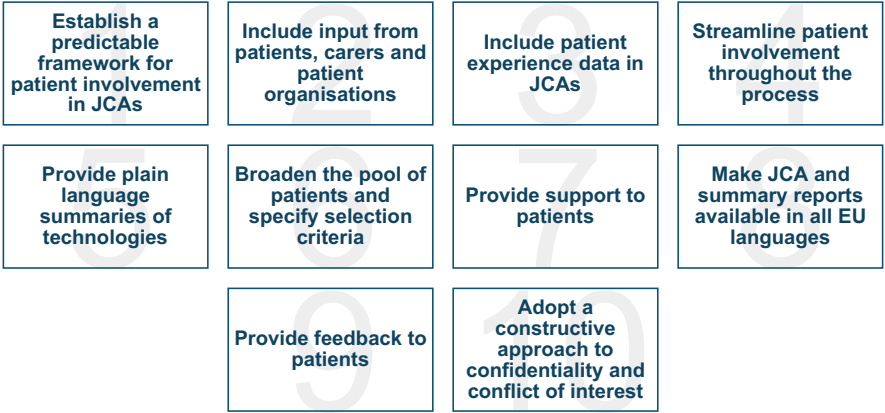


Fig. 18.3 Ten key recommendations from patient organisations on JCA under the EU HTAR. (Developed from Cancer Patients Europe et al. (2024))

continue and further improve their patient involvement practices. Additionally, the EUnetHTA-EMA joint workplan allowed for an exchange of experiences and methodologies on patient involvement. This collaboration led to better understanding of the expectations for involving experts in their respective procedures but recognised there are still different procedures and policies in place that could benefit from further alignment, for example a remuneration policy for patient involvement (EMA and EUnetHTA 2023).

Sustainability of patient involvement is crucial too, to ensure there is sufficient and growing capacity and knowledge in the patient community. The burden of disease must be considered for any tasks allocated to patients and carers and they should decide in what way they feel most comfortable participating (e.g. via interview or written input). Likewise, language is a critical factor impacting equity in patient involvement, be it the language in which procedures and templates are available, or the language in which patient input is accepted. During JA3, the patient input templates were made available in all official EU languages for JCA and translated into three languages for JSC. However, the HTAR foresees no such translation and input will only be accepted in English. This likely has an impact on the selection of patients, and any non-English language may therefore become an exclusion criterion and will also likely impact the ability of patient organisations to respond to calls for stakeholder contribution.

Although during EUnetHTA efforts were made to include patient input in the JSC and JCA reports using different approaches, e.g. via a summary in the report or via inclusion of the input in an annex, more work is needed to render the utilisation of this input more transparently in JSC outcome documents and JCA reports under the HTAR. Furthermore, much of EUnetHTA's experience centres on medicinal products. While a number of JCA were conducted on medical devices, only one JSC was conducted on a medical device (excluding JA2 and SEED pilots). This translates to less experience in involving patients in JSC for medical devices, a domain where patient involvement is already sometimes tricky given that a patient may not know that a device is being used and therefore cannot report any direct experience or implications.

Similar to EUnetHTA and national HTA procedures, under the HTAR it is not intended that patient experts or patient organisations participate in scientific and technical reviews. De Wit et al. found that patient input reaches beyond their lived experience of the disease to include "their experiences with the healthcare system and their experiences with fellow patients and patient organisations. This source of knowledge complements the scientific knowledge of researchers" (2020). Therefore, it is more relevant and appropriate for patient experts to review if their input is used correctly and if their disease and experience of living with the disease are correctly captured. Finally, in preparation for the planned review of the HTAR until January 2028, a proper evaluation process is needed for patient involvement in joint work and relevant performance indicators need to be identified.

18.6 Conclusion

The HTAR recognises the importance of involving both external experts (e.g. clinical experts and patients) with relevant in-depth, specialised expertise and organisations (e.g. patient organisations and scientific societies) to provide input on joint work while preserving the scientific integrity, independency, and quality of the joint work by developing rules on conflict-of-interest management. While the HTAR and the respective IRs are only mandated to lay down the procedures and rules for the European activities, different timepoints for both the JSC and JCA production can be identified in which national HTA bodies can be encouraged to seek national patient involvement.

More work remains to be done in further developing the procedures and templates for patient input in the HTAR joint work, and attention should be paid to already existing best practices. Involving patients is a learning process for all and the “aim should be to make this experience fruitful and productive, and to ensure all stakeholders feel that their time and resources are well spent” (Holtorf et al. 2024). Likewise, it may be useful to train assessors in the collection of patient input in order to insure consistency. It is of utmost importance that the sustainability of patient involvement, in terms of capacity, awareness, understanding, resourcing and language is sufficiently considered given the large volume of JSC and JCA expected to be conducted under the HTAR and their potential impact on the lives of European patients.

Acknowledgements We thank all our colleagues in the HTACG and the relevant Subgroups, as well as those who participated in the EUnetHTA 21 working group on patient involvement. We also extend our thanks to all the patients and patient organisations who participated in JSC and JCA under EUnetHTA as well as those who responded to public consultations under EUnetHTA 21 and the HTAR. Finally, we thank our colleagues Judith Fernandez (HAS) and Diana Delnoij (ZIN) for their guidance and review in the development of this chapter.

Disclaimer The content of this chapter represents the views of the authors only and is their sole responsibility; it cannot be considered to reflect the view of their agency nor the European Commission. MG was responsible for the Early Dialogue Secretariat in JA3, is a member of the EHT SG and is a member of the EUCAPA advisory board and HTA4Patients project expert panel. AW was leading the JCA Secretariat under JA3 and EUnetHTA 21 and is currently co-chair of the JCA SG under the HTAR. VS represented the patient perspective in EUnetHTA JA 2 Stakeholder Forum and is now representing EPF in the HTA Stakeholder Network. Between 2016 and 2024 VS was part of the HTAi PCIG Steering Committee.

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Chapter 19

Germany: Patient Involvement in HTA



Sabine Haefner, Martin Danner, and Susanne Bethge

19.1 Introduction

This chapter presents how Germany supports patient involvement in health technology assessment (HTA) and decision-making processes in the Federal Joint Committee (*Gemeinsamer Bundesausschuss*, G-BA) and the Institute for Quality and Efficiency in Healthcare (*Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen*, IQWiG), which drafts HTA reports commissioned by the G-BA. It outlines processes for incorporating individual and collective patient experiences through participation processes and research, and it shows how Germany has set a legal framework for patient participation. It explains how patient experiences are introduced by patient organisations that have been identified as *relevant organisations* by lawmakers and that have been granted participation rights including means for capacity building. It presents a range of mechanisms for participation according to the mosaic for participation (Chap. 4). In particular, it illustrates how patient organisations use their rights by filing requests for HTAs and decision-making regarding benefit coverage and by identifying patient representatives with different perspectives to take part in assessment and appraisal processes. In conclusion, the chapter reflects on issues of evaluation and future challenges.

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Approximately 90% of the people living in Germany are insured under statutory health insurance (SHI) (National Association of Statutory Health Insurance Funds 2024). The SHI is based on the basic principles of solidarity, medical necessity and benefit in kind. Everyone insured under SHI has the same right to necessary medical care regardless of age, disease or the amount of premiums paid.

19.1.1 Self-Government

Further characteristics are the principles of subsidiarity and *self-government*. The legislators only define the framework for medical care. In many areas, policy making is up to the so-called joint self-government to determine the scope of services provided by SHI funds and to organise healthcare delivery. The joint self-government enables joint decision-making including bodies with representatives from the health insurance funds and care providers (physicians, dentists, physiotherapists and hospitals). They are required by law to provide “needs-based and equitable care for insured persons according to the current generally accepted state of medical knowledge” (Social Code Book V. Section 12 1992). The “medical care of insured persons must be adequate, sufficient, and expedient, must not go beyond what is necessary, and must be cost-effective and of high professional quality” (ibid., Social Code Book V. Section 70 1999). HTAs play a key role in defining these requirements.

19.1.2 Mandate of G-BA and IQWiG

The G-BA is the highest decision-making body of the joint self-government. It is responsible for the assessment, appraisal and decision-making regarding various technologies within SHI, in particular coverage of medical diagnostic and therapeutic methods or pharmaceuticals. Appraisal includes consideration of assessments commissioned to the IQWiG or undertaken within G-BA.^{1,2} Moreover, the G-BA develops regulations on organisational and quality aspects of healthcare. G-BA stipulations take the form of directives that are legally binding for all statutory health insurance funds, care providers and insured persons (Fig. 19.1).

¹ The tasks of the G-BA are extensive. For more information on the G-BA, also in English, please visit www.english.g-ba.de/ and <https://patientenvertretung.g-ba.de/en>.

² For more information on the IQWiG, also in English, please visit www.iqwig.de.

The legal status of the G-BA (Federal Joint Committee)

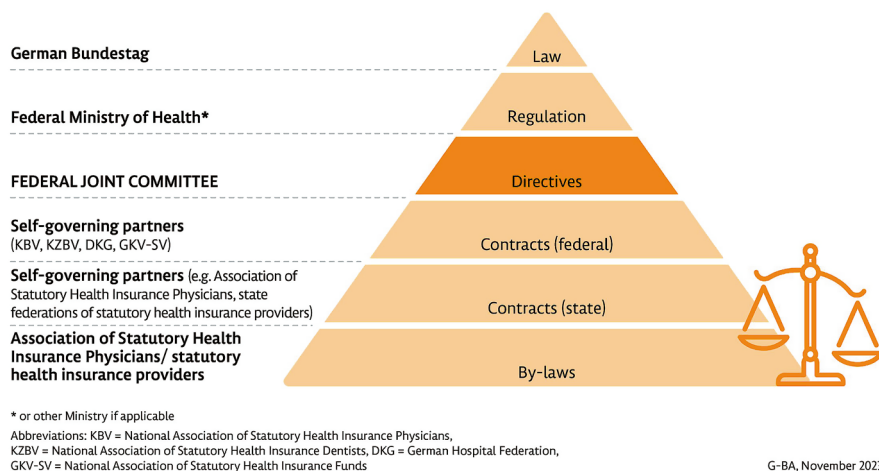


Fig. 19.1 The legal status of the G-BA (Federal Joint Committee) (G-BA 2023)

19.1.3 Patient Participation

Since 2004, the legislation has supported comprehensive patient participation in the G-BA and the IQWiG and both have now more than 20 years of experience. It includes participation of individual patients or their carers (informal caregivers) as well as representatives of patient groups, advocacy groups or consumer organisations. All possible patient perspectives are covered as described by HTAi (2024). The persons participating are chosen and appointed as *knowledgeable persons (patient representatives)* by *relevant patient organisations* (Sect. 19.2.2.1) into the bodies of G-BA. The diversity of individual and collective perspectives during appraisal is intended, and the terms *knowledgeable person* and *patient representative* are used alternatively.

Currently, around 250 *patient representatives* are active in G-BA committees.

19.2 Rationale of Patient Participation in G-BA and IQWiG

19.2.1 Starting Point for Lawmakers

It is no coincidence that patient participation became part of HTA and other decision-making processes of the G-BA and IQWiG.

19.2.1.1 Improving Quality, Efficiency and Cost-Effectiveness

The primary aim of a 2003 legislation was to maintain the basic principles of SHI in the context of an ageing society and advancements in medical care, without rationing services. Greater efficiency and better quality in medical care should lead to more cost-effective services overall (SHI Modernisation Act 2003).

The G-BA was established as the decision-making body, thus replacing its predecessor organisations (Draft SHI Modernisation Act 2003a), and the IQWiG founded as an independent HTA institute (Draft SHI Modernisation Act 2003c).

19.2.1.2 Self-Responsibility and Patient Sovereignty

However, the Modernisation Act also put some services into the category of ‘self-responsibility’, meaning that the patients must pay for some services themselves or make co-payments. At the same time, ‘patient sovereignty’ should be promoted with measures, such as the right to demand an understandable invoice for medical (in-kind) services or to choose cost refund instead of in-kind benefits.

On the macro-level those affected by SHI, the patients, became participants in decision-making processes. This was considered by the lawmakers as the only way more ‘self-responsibility’ could be asked of them (Draft SHI Modernisation Act 2003b).

19.2.2 The Scope of Patient Participation

The *SHI Modernisation Act* and the *Patient Involvement Act* from 2003 have formed the scope of patient participation in self-government bodies.

19.2.2.1 Relevant Patient Organisations

The Federal Ministry of Health has recognised four *relevant patient organisations* comprising national umbrella organisations and meeting further requirements (Patient Involvement Act 2003).

The four *relevant organisations* are the German Disability Council (Deutscher Behindertenrat 2016), the National Association of Patient Advisory Centres (Bundesarbeitsgemeinschaft der PatientInnenstellen 2016), the German Association of Self-Help Groups (Deutsche Arbeitsgemeinschaft Selbsthilfegruppen 2016) and the Federation of German Consumer Organisations (Verbraucherzentrale Bundesverband 2024). With their member patient organisations, they cover a broad spectrum of diseases and disabilities. This allows for the participation of persons directly concerned, meaning (chronically ill) patients, persons with disabilities and their informal carers. They also include organisations that advise patients. The

broad and highly differentiated competence of the various organisations ensures that *patient representatives* can be identified for each specific issue.

The organisations have non-profit status and have to disclose their financing to prove that their work is impartial and independent. *Patient representatives* from member organisations with limited funding from health technology developers can be involved.

19.2.2.2 Participation Rights

The *relevant patient organisations* were granted the statutory rights to take part in (*consultation right*) and to initiate decision-making processes (*right to request a decision*) in the G-BA. The *consultation right* includes the right to be present when a decision is made. They further were given the right to submit *written statements* or take part in *hearings* regarding decisions of other self-government committees (Social Code Book V. Section 140f 2015d) or the assessments of the IQWiG (Social Code Book V. Section 139a 2015c).

19.2.2.3 Knowledgeable Persons: The Patient Representatives

To exercise their *consultation rights*, the *relevant patient organisations* mutually nominate *knowledgeable persons* (*patient representatives*) with the relevant competence.

A *patient representative* is identified by a *relevant patient organisation* or one of their member organisations. They have to be voluntarily or professionally active within a patient organisation. Depending on the subject of consultation, the competence ranges from having individual or collective patient experience within the indication of an assessed technology, to being able to provide a broader knowledge of German healthcare system from a patient perspective. At least half of the *knowledgeable persons* should themselves be directly affected (Social Code Book V. Section 140f 2015d).

The number of appointed persons in the committees, subcommittees and working groups is limited by the number of persons sent by the health insurance funds (Social Code Book V. Section 140f 2015d; Patient Involvement Act 2003).

19.2.3 Assessment of Rationales

Taking the HTAi values for patient involvement in HTA (2014) as a basis, the lawmakers' rationale for patient participation in the G-BA can therefore be summarised as follows:

- **Relevance** The *relevant patient organisations* and their member organisations represent both a wide variety of indications and care areas as well as regions

throughout Germany. This allows relevant patient experience and perspectives to become part of HTAs.

- **Fairness** The *relevant patient organisations and their patient representatives* can contribute to the process by having the same right as other stakeholders to initiate a decision-making process (*right to request a decision*) and to take part actively in all consultations and sessions (*consultation right*). Nevertheless, often they do not feel like equal partners as they have limited resources regarding professional and financial support, and do not have the right to vote in the G-BA.
- **Equity** Through relevant contributions from the patient perspectives, patient participation helps to reduce underuse, overuse and misuse of health services, to promote quality and more efficient services. This can be considered as contribution to equity within SHI.
- **Legitimacy** Patient participation has contributed to more transparency of G-BA decisions, e.g. by discussing and explaining procedures and decisions within patient organisations. The credibility of G-BA decisions could be further improved, if published justifications of decisions put more emphasis on included patient perspectives (Sect. 19.4.2).
- **Capacity building** The right to initiate HTAs and legally binding decisions by request means that patient organisations can actively contribute to healthcare. Taking the limited resources into account, patient participation nevertheless includes financial, organisational and professional support as well as training (Sect. 19.3.3). Empowerment, influence and co-operation of patient organisations and *patient representatives* are promoted.

19.3 Patient Participation in HTA in the G-BA and IQWiG

19.3.1 Patient Participation Throughout the HTA Process

The decision-making process of G-BA can be described with the assessment and appraisal model. Assessments are conducted by a G-BA committee. But generally, the G-BA commissions the IQWiG to produce an HTA report. Appraisal of the results takes place in the G-BA subcommittees and working groups where resolution proposals are drafted for the decision-making body which takes a decision in a public session. *Patient representatives* and patient organisations are involved in the entire process in the G-BA.

The IQWiG includes patients or *patient representatives* for the preparation of HTA reports according to its methods papers (IQWiG 2023) (Fig. 19.2)

As an example, we use a request of the *relevant patient organisations* for HTA and benefit coverage of the *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* from 6 September 2012 (Relevant Patient Organisations 2012) in Table 19.1 and Sect. 19.3.2.

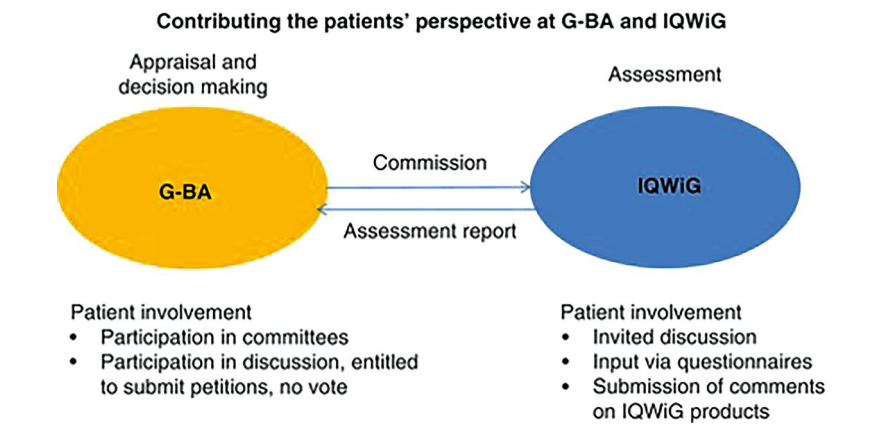


Fig. 19.2 Contributing the patients' perspectives at G-BA and IQWiG (Wieseler 2015)

Table 19.1 Patient participation in G-BA HTA and decision-making process

HTA phase	Relevant patient perspectives	Type of patient participation mechanism
Topic proposal and request by patient organisations		
Indications of deficiencies in care Inclusion into SHI services	Medical care aspects Patient safety Benefit in patient-relevant endpoints (mortality, morbidity, quality of life) For example: <i>Congenital heart disease most common organ malformation of newborns</i> <i>Need of fast intensive care of newborns with critical congenital heart disease</i> <i>Improved survival with early diagnostic</i>	Any patient group can <i>propose topic</i> to relevant patient organisations For example, <i>Federal Association of Children with Heart Disease</i> <i>The G-BA patient involvement specialist team</i> supports patient groups to develop a draft request meeting G-BA conditions for HTA Relevant patient organisations decide to submit the <i>request</i> to the G-BA
Topic selection and initiation of HTA (G-BA)		
Acceptance of request, clarification of topic, prioritisation	Collection of detailed information on the topic Healthcare relevance for patient groups (e.g. disease burden, anticipated benefit, available alternatives)	Patient representatives participate in G-BA committees, with support from G-BA patient involvement team For example, <i>representatives from the Federal Association of Children with Heart Disease as well as patient representatives from other organisations in the area early diagnosis of children</i>

(continued)

Table 19.1 (continued)

HTA phase	Relevant patient perspectives	Type of patient participation mechanism
Public notice of assessment and appraisal	Initial evaluation of topic (PICO)	Inter alia: umbrella organisations of self-help groups and patient representatives <i>Statement</i> via questionnaire
Commission to IQWiG for HTA report	Specification of HTA questions For example, <i>earlier identification and better prognosis of congenital heart disease?</i>	Patient representatives: <i>participation</i> in G-BA committees
Early phases of HTA report (IQWiG) (IQWiG 2015)		
Research question	Patient-relevant endpoints Subgroups, if needed	Patients, affected persons: <i>consultation</i> via interviews For example, <i>patient representatives from Federal Association of Children with Heart Disease, parents' initiative of children with heart disease cologne, Herzkind e.V.</i>
Preliminary report plan	Patient-relevant endpoints Inclusion and exclusion criteria	Public, including patient organisations: <i>written comments (hearing)</i>
Preliminary report	Methodical approach and findings	Public, including patient organisations: <i>written comments (hearing)</i>
Appraisal and decision (G-BA)		
Appraisal	Medical relevance, disease burden, access, alternatives	Patient representatives: participation in G-BA committees Relevant patient organisations: may submit (dissenting) draft decision as request, if applicable
Decision on inclusion as SHI service in public session		Patient representatives: participation Presentation of position or request in public session

Adapted from Figure 5 with example from the assessment and appraisal of the *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* (G-BA 2016b)

G-BA Federal Joint Committee (*Gemeinsamer Bundesausschuss*), IQWiG Institute for Quality and Efficiency in Healthcare (*Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen*), PICO Population, intervention, comparator, outcome

Many requests and proposals of the *relevant patient organisations* for HTA and benefit coverage can be found at the homepage of the G-BA patient involvement specialist team (<https://patientenvertretung.g-ba.de/antraege/>).

**Box 19.1 Example Request Made by Relevant Patient Organisations:
Inclusion of Spinal Muscular Atrophy in the Extended Newborn
Screening**

Spinal muscular atrophy (SMA) is a neuromuscular disease that is responsible for voluntary movements of the muscles such as crawling, walking, head and neck control, and swallowing. It is a relatively common ‘rare disease’. Approximately 1 in 6–10,000 newborns is affected and approximately one in 45 people are carriers of the disease. SMA affects all muscles in the body and, in particular, damage to the respiratory muscles leads to early death in the most common and severe type 1. It is known from clinical trial data that the earlier therapy is started, the more effective it is.

As part of newborn screening, every newborn in Germany is routinely examined for diseases that can be detected through an early examination and thus treated in a targeted manner. It aims to detect congenital disorders of the metabolism, the hormonal, blood and immune systems and also the neuromuscular system in newborns at an early stage and to ensure immediate initiation of therapy. For this purpose, a drop of blood is taken from the newborn’s heel after the parents’ informed consent. The blood is then examined in specialist laboratories. Unfortunately, the SMA test was not included in this screening.

Therefore, in 2018, the *relevant patient organisations* submitted an application to include the test into standard care. As already shown in Table 19.1, an identical HTA assessment was initiated in the G-BA based on the application. IQWiG was commissioned to carry out the benefit assessment. The IQWiG thereby also involved those affected by the German Society for Muscular Patients, among others. The consultations on evidence were then carried out on the basis of all available documents in the G-BA. Patient representatives from the *Deutsche Gesellschaft für Muskelkranke* (German Society for Muscular Diseases) took part in the consultation process.

In 2020, the SMA test was finally added to the newborn screening in Germany is now financed by all health insurance companies. This means that affected infants can be diagnosed before the first symptoms of SMA appear and can therefore receive life-saving therapy as early as possible.

(Please find all relevant documents at the following links: <https://patientenvertretung.g-ba.de/antraege/methodenbewertung/muskelschwund/> or IQWiG—Neugeborenencreening auf Spinale Muskelatrophie <https://www.iqwig.de/projekte/s18-02.html>)

19.3.2 *Involvement Mechanisms*

Table 19.1 shows patient participation rights and opportunities throughout the HTA process. They involve different actors.

19.3.2.1 Initiation of HTA

The right to request a decision of the *relevant patient organisations* goes beyond a topic proposal that could be considered or not. A request is a formal procedural right. Topics addressed in a request must be discussed, and a formal decision must be made about whether to go ahead with the topic or not.³ However, once the conditions for an HTA have been met by a request, the G-BA has to initiate the assessment and appraisal procedure, take the topic forward as an HTA and at the end of procedures has to decide about benefit coverage.⁴

Conditions for an HTA are specified in the G-BA rules of procedure. The request has to include a description and substantiated justification with information on the benefits, the target population, medical necessity and cost-effectiveness of the method to be discussed, as well as comparisons to methods already available. Available studies must permit a discussion of the topic, and the request must specify the topic's level of urgency.

Topic proposals are discussed within patient organisations first. Any patient organisation can propose a topic to the *relevant patient organisations*, regardless of its size, funding or skills. There is no paper form for requests, but the G-BA patient involvement specialist team has been established to support with drafting requests. It can review information and existing evidence and estimate the chances of success. On the basis of the collected information, the four *relevant organisations* decide whether to submit a request.

Our example *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry* was proposed by the Federal Association of Children with Heart Disease, a member organisation of the German Disability Council. The Association had support from medical experts, such as the German Association of Paediatric Cardiology. The G-BA patient involvement specialist team reviewed the information and drafted the request according to G-BA conditions, and after internal discussion and decision, *the relevant patient organisations* submitted the request to the G-BA.

³The G-BA is required to discuss requests in the next session of the relevant committee, and, if no decision is possible, define the further proceedings (Social Code Book V. Section 140f 2015d).

⁴Prioritisation is restricted: a decision on acceptance of a request for HTA of non-medicine technology has to be made after 3 months, and process shall be finished after 3 years (Social Code Book V. Section 135 2015b).

19.3.2.2 Patient Participation During the HTA Process

Appointments of patient representatives by the *relevant patient organisations* to G-BA committees are dependent on the experience and competence of the *patient representative* as well as on a committee's agenda. Each appointment is *topic related*, depending on the individual's knowledge and experience. If a committee works on several issues simultaneously, several persons will be appointed to cover all topics. In addition to these *topic-related* representatives, the *relevant patient organisations* delegate *permanent patient representatives* with methodological or general expertise. These persons have the additional task to ensure the continuity of the representation of interests, coordinate and support the topic-related representatives and act as a liaison until the G-BA takes a decision. The number of *patient representatives* per committee ranges from 1 to 12. In our example the *relevant patient organisations* appointed two topic-related *patient representatives* from the association of children with child disease into the G-BA. The topic was discussed in the responsible subcommittee 'Methods Assessment' and its working group 'Children' where *patient representatives* from other patient organisations also permanently work on topics of early diagnosis of children.

Conflicts of interest Before an appointment, the *patient representatives* have to fill out a questionnaire to disclose any potential personal conflicts of interest or those of their organisation. If any conflict of interest is present, the *relevant patient organisations* do not appoint the person to a committee at all. Representatives who are appointed must furthermore fill out a statement with the G-BA to disclose any interest regarding the specific topic under discussion. This disclosure statement can be viewed by all committee members, and all committee members (not only *patient representatives*) have to provide this (G-BA 2014).

19.3.2.3 Patient Participation at the IQWiG

Consultation At the start of a commissioned benefit assessment, IQWiG grants additional participation opportunities that are not guaranteed by law. This is to allow patient perspectives to be taken into consideration for the formulation of the research question and further assessment. This participation takes the form of a consultation or a questionnaire with persons affected who can be patients or their relatives with individual experience, as well as *patient representatives* with collective experience. The recruitment takes place via the *relevant patient organisations* and their member organisations, or the IQWiG can contact those persons directly (IQWiG 2015, 2017).

In the course of our example *patient representatives* from the Federal Association of Children with Heart Disease, the Parents' Initiative for Children with Heart Disease of Cologne and *Herzkind* have been consulted regarding the quantity of benefit (quality of life), alternatives, possible negative consequences for parents and children, treatment options as well as efficiency.

Conflicts of interest Each person involved at the IQWiG must also fill out a form to disclose any potential conflict of interest. The IQWiG publishes whether a question was answered with ‘yes’ or ‘no’ (IQWiG 2015, 2017).

Statements The law ensures the right of *relevant patient organisations* to submit written statements at various stages of an HTA assessment in the IQWiG (Social Code Book V. Section 139a 2015c), e.g. after the publication of preliminary report plans and preliminary reports. However, the IQWiG opens the hearing procedures to the whole public including private persons.

Research The IQWiG has conducted research with two pilot studies in order to provide evidence about patients’ preferences (Chap. 9). These approaches have not become regular instruments of patient involvement in HTA under the responsibility of the G-BA but are often part of evidence-based selection and evaluation of scientific literature, e. g. in early benefit assessment of medicinal products.

19.3.3 Measures to Support Patient Participation

Patient participation in the G-BA and IQWiG is mainly based on volunteer work by *patient representatives*. Therefore, supportive measures are required to be able to exercise rights effectively.

Statutory measures:

- Reimbursement of travel expenses, compensation for loss of earning up to a maximum sum and lump sum as representation allowance (Social Code Book V. Section 140f 2015d).
- The G-BA patient involvement specialist team is tasked with organisational and content support (Social Code Book V. Section 140f 2015d): this includes methodological and legal advice, help with consulting documents, organisation of meetings and support with the nomination procedures for *patient representatives*. Training is also provided by the G-BA Medical Consultancy Department.
- Embedding patient participation and procedural rights in the G-BA by-laws (Social Code Book V. Section 91 2015a).
- Barrier-free access to offices and rooms, consultation documents, website and organisation of meeting assistance (Gesetz zur Gleichstellung behinderter Menschen 2002; G-BA 2008).

Additional measures:

- Transparent internal appointment and collaboration rules for the *relevant patient organisations* and *patient representatives*.⁵
- Participation in events, also as speakers, facilitators or participants in discussions.

⁵By-laws of the patient representative coordination committee of 21 November 2012, not published.

- In 2016, the G-BA patient involvement team established an online portal for communication, joint work and education (G-BA [2016a](#)).

19.3.4 Patient Participation in the Innovation of (Statutory) Healthcare: The Innovation Fund

In order to ensure and continuously develop high-quality medical care for all, German insured persons across all sectors, the Innovation Fund was set up at the G-BA in 2016 with a financial volume of €200 million per annum. The Innovation Fund supports research projects that test innovative approaches into SHI and want to gain new insights that go beyond current standard care. Since its founding in year 2016, the Innovation Fund has helped to make an adequate contribution to improving healthcare. More than 700 research projects have been funded. As part of the statutory self-administration, patient representatives have been involved in all funding processes and decisions since its founding and has become increasingly important over time (Berghöfer et al. [2020](#); Innovation Fund [2024](#)).

Since 2021, the patient representation has not only been active in the internal procedures, but also in the funding announcements. Since then, applicants have been called to integrate the opportunity for patients to participate in the spirit of participatory research. This can occur throughout the entire project, among other things: in the form of advice, participation, collaboration or control in the projects. Structural integration of patient participation in projects ensures that the projects are geared to the needs of patients. The new law to accelerate the digitalisation of the healthcare system (Digi-G), which came into force in March 2024, will perpetuate the Innovation Fund and further develop patient participation as it is established as a new legal funding criterion that has to be addressed by all applicants (Innovation Fund [2024](#)).

Before this new law, patient participation in corresponding research projects has been voluntary. This is absolutely new, that patient participation is required by law (and on the initiative of the federal government of Germany). This gives patient participation the same importance as for example the participation of a health insurance company.

19.4 Evaluation and Challenges

19.4.1 Issues in Evaluating Patient Participation

Still, no comprehensive scientific evaluation of patient involvement in the G-BA and IQWiG has been conducted. Challenges for evaluation are the various forms and actors of participation, as well as the political aspects influencing G-BA decisions.

Requests for an HTA are published on the G-BA website as soon as a decision-making process is initiated, so that after some time, even years, the impact could be

examined by comparing the intent of the request with the G-BA decision and further implementation into healthcare.⁶

The circumstances surrounding how *consultation rights* are exercised by *patient representatives* in the G-BA committees are more difficult to evaluate. The workflow is a joint consultation in the preparatory committees aimed at achieving a consensual reconciliation of interests among the various healthcare stakeholders. Individual statements expressed or submitted by the *patient representatives* in the meetings become part of the discussion and can influence the votes of the other persons involved and thus also decisions. But this influence can hardly be evaluated. The contributions of the *patient representatives* might be recorded in the minutes, but the consultations and the relevant documents of the non-public meetings are confidential.

No minutes are taken of consultations on patient-relevant endpoints that the IQWiG conducts with persons affected (IQWiG 2015).

What remains are pertinent quotes by the impartial chairs of the G-BA. The impartial chair, Josef Hecken, confirmed in an interview: “I consider the involvement of the patient representatives in its current form to be exceptionally helpful because it brings the specific perspectives of the patient into the discussion in a highly responsible manner” (Gottfried and Kessen 2012). On the occasion of 20 years of patient participation in the G-BA, the impartial chairman as well as the boards of the pillar organisations and the institutes of the G-BA paid tribute to patient participation in welcoming messages for an anniversary publication. The enrichment through the advice provided by people with their own patient experience as well as the high level of expertise, empathy and professionalism are emphasised, which have contributed to the quality and legitimacy of the G-BA’s decisions (<https://patientenvertretung.g-ba.de/wer-wir-sind/20-jahre-patientenvertretung/>).

19.4.2 Future Challenges

Patient participation in the G-BA including *relevant patient organisations* and appointed *patient representatives* with different perspectives makes it possible, not only to reflect the overall acceptance of HTA and decision-making from a citizen’s point of view but also to include patient experience and to organise patient advocacy.

The establishment of the patient involvement specialist team in the G-BA in 2008 has become a powerful support. However, significant tasks fall to the patient organisations. These include, in particular, recruiting volunteer *topic-related patient representatives* for the indications addressed in an HTA and appointing *permanent patient representatives*.

⁶Requests and documented contributions by *relevant patient organisations* and *patient representatives* can be found on the G-BA website (www.g-ba.de). The website of the G-BA patient involvement specialist team (<https://patientenvertretung.g-ba.de>) also includes all requests formally submitted by the patient representatives, along with the consultation proceedings.

So far, the state has shied away from providing large subsidies for patient organisations out of a concern of intervening in the traditional balance of interests between health insurance funds and care providers. A statement in a ruling by the Federal Constitutional Court on 10 November 2015 has attracted much attention. The court stated that it cannot be ruled out that the constitutional legitimacy of the G-BA as self-governing committee might be missing, if it regulates with high intensity the affairs of others who are not permitted to contribute to the process (Bundesverfassungsgericht 2015). This has been commented on in the jurisprudential literature: “Better consideration of the interests and rights of insured persons, patients, and third parties affected belongs at the top of the political agenda” (Gassner 2016). In 2021, the governing parties agreed to strengthen patient participation. However, this has so far been limited to the patient representation being given a right of veto. This is viewed rather critically by patient organisations as a destructive instrument. They call for a more far-reaching increase in patient involvement (Die maßgeblichen Patientenorganisationen 2023).

Over the past years, lawmakers have assigned more and more decision-making power to the G-BA. Its decisions have a huge impact on healthcare, and they are being closely observed by the Federal Ministry of Health, politics, courts and the public.

Decisions of the G-BA are published on G-BA’s homepage including rationales and documentation of the HTA process. But more and more, the acceptance of decisions will also depend on the ability and commitment of the G-BA to report how patient perspectives have been determined, involved and considered in each assessment and appraisal.

Acknowledgements The authors would like to acknowledge comments received from Ann Single, Karen Facey and Dr. Anke Peggy Holtorf while revising this chapter and also Susanne Teupen for her valuable comments.

Conflict of Interest The authors declare that there is no conflict of interest.

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Chapter 20

Latin America and the Caribbean: Evolving Approaches to Involving Patients in HTA



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20.1 Introduction

Latin America and the Caribbean (LAC) is a region of 33 countries with a broad cultural, political, economic, and social diversity (CEPAL 2024). Likewise, population health outcomes across the countries or social subgroups are heterogeneous. While countries like Chile and Uruguay show life expectancy at birth of 80.7 and 77.1 years, Haití and Guyana only reach 64.1 and 65.7 years, respectively (WHO 2019). The determinants for these outcomes are multifactorial and include basic living conditions and violence (González-Pérez et al. 2017; Dávila-Cervantes and Pardo-Montaña 2018), non-communicable chronic conditions, and health expenditure (Roffia et al. 2023).

While variation exists across the region in the extent of access to health services and technologies, significant budgeting constraints mean that high-cost technologies are often not cost-effective in this region (Gutierrez et al. 2023; Presidencia de la Republica Dominicana 2024). To guide reimbursement and access to technologies that are likely to produce the most health benefits and to not waste resources on

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technologies that are not cost effective (Giedion et al. 2024), several LAC countries have started to characterise the value of new technologies through health technology assessment (HTA) (O'Rourke et al. 2020). This is expected to result in more rational and fairer decisions, especially when social representatives are included in collecting and deliberating the evidence. There is a growing consensus that the participation of patients or patient representatives in these steps contributes to better processes and decisions (Menon and Stafinski 2011).

Generally, countries first build their internal HTA capacity to perform the technical work of evidence collection and evaluation. Social or public participation in HTA only happens as systems and processes mature. Effective patient participation faces several challenges, such as identifying relevant representatives who understand the remit of HTA and the technical information and can effectively contribute to patients' perspectives alongside payers, clinicians, and government officials.

This chapter explores the current status of patient involvement in HTA systems and processes in ten LAC countries. More detailed case studies of Colombia, Chile, and Brazil illustrate some successes and continuing challenges for HTA patient participation in the region.

20.2 Overview of HTA in LAC

Many LAC countries have implemented HTA processes in the past decade to support resource allocation and healthcare prioritisation (Pichon-Riviere et al. 2020). Like the region, there is much diversity in HTA approaches and experience, including concerning public participation and research into patient aspects, as summarised in Table 20.1. In some cases, like Brazil, HTA has become institutionalised over time and now includes broad stakeholder participation, including patients, as one of the region's more structured and complete HTA systems (Lima et al. 2019). In others, like Mexico and Panama, HTA capability is relatively advanced but without a mechanism for patient or public participation. Other countries are somewhere in between. In our analysis, patient participation is in the early implementation stages

in Argentina and Peru. Colombia and Chile are unique in their progress and are detailed later in the chapter. Costa Rica is in the design stages because a commitment has been made and a mechanism drafted, but it has not yet been made public. Ecuador and Uruguay (as noted in Chap. 30) appear to be in the developmental stages of patient participation with some initial signs of progress, and new mechanisms will likely soon be considered in those countries.

Most HTA systems in LAC claim to be committed to socially participatory processes, but in practice, public participation and patient involvement is limited, and clinical and payer experts dominate the process. Most countries in the region still face significant challenges formally involving patients, which means the influence of these important stakeholders in HTA is limited and primarily consultative (Alcaraz et al. 2022). Chapter 28 discusses the challenges in low- to middle-income countries. Many challenges apply to LAC, including fragmented health systems where HTA is only used for certain subgroups, limited patient-level data, misperceptions that patients are unqualified, biased, or both, and few trusted mechanisms (or capacity) to identify and recruit patients. In addition, when patients are consulted, their social value judgments about a health technology can clash significantly with the biomedical paradigm used by doctors, statisticians, and economists (Espinoza et al. 2019). In this context, two priorities exist simultaneously: to develop mechanisms to involve patients and to ensure patients are capable of being involved meaningfully.

Despite these challenges, new opportunities and frameworks are emerging, as evidenced in Table 20.1 and the following case studies of Colombia, Chile, and Brazil. After all, only a few LAC countries have fully developed HTA capabilities. As experience continues to build and as international consensus continues to mount around incorporating real-world evidence (RWE) such as patient-reported outcomes (PROs) or the patients' lived experience or 'patient journey', patient involvement is anticipated to continue to grow in the region as data sources and capabilities widen and improve (García Martí et al. 2023).

Table 20.1 HTA systems, public participation, and patient involvement processes in LAC (Authors' compilation)

Country	Lead agency	Patient involvement mechanism	Status	Source(s)
Argentina	CONETEC was created by the Ministry of Health in 2018 and became a decentralised unit within the Ministry in 2023	Four elected patient representatives serve on the Patient Working Group (<i>Mesa de Pacientes</i>), representing the following areas: oncology, non-communicable chronic diseases, infectious diseases, and rare diseases. They serve 2-year terms. Coordinated by CONETEC, the working group helps identify relevant patient and carer experts for each HTA project, advises the technical teams doing the assessments, and participates in other agency working groups to represent the patient's voice on topic selection, prioritisation, and process transparency. The patient representatives have a voice and vote in the technical working group. Patient representatives collect information on behalf of patients for assessments and working groups and can also bring in patients with expertise relevant to a specific technology. Patient and carer input is especially valued on equity (is there anything about the technology that can close or widen a gap?) Public consultation at the beginning of an HTA includes patients in defining the scope of the assessment. Patient representatives are also included in the financing and coverage group. CONETEC is working to increase patient submissions to inform the prioritisation of topics, but up until April 2024, 371 patients or carers had 'bolstered the analysis' in 38 HTA reports.	Early implementation	CONETEC website: https://www.argentina.gob.ar/salud/conetec/convocatorias ANEXO II. <i>Manual de Procedimiento para el funcionamiento de la CONETEC</i>. Participación de Pacientes en la CONETEC. August 2023. Pages 46–56. https://www.argentina.gob.ar/sites/default/files/anexo_ii_manual-procedimiento-funcionamiento-conetec.pdf. (Donato 2024)

Brazil	CONITEC was created in 2011 for the SUS universal health system	<i>Perspectiva do Paciente</i> initiative allows for patients or patient organisations to be selected to provide testimonials at HTA plenary sessions. Pre-registration with CONITEC is mandatory for each HTA topic that interests patients. One presenter is chosen (with one alternate). Prerequisites for providing testimony include virtual training sessions to learn about HTA and CONITEC processes. Patients and patient organisations are involved in proposing HTA topics and providing input during public consultations. CONITEC surveys specific patient organisations for input as needed. Patient representatives on Brazil's National Health Council (CNS) may also participate in CONITEC's three thematic committees. A plain language version of each technical report is made available to the public during the public consultation period. Qualitative Evidence Synthesis has at times been used to increase understanding of patient perspectives, especially for rare disease technologies.	Implementation	CONITEC website: https://www.gov.br/conitec/pt-br/assuntos/noticias/2024/outubro/perspectiva-do-paciente-atualizacao-das-orientacoes-pode-ser-consultada-no-site-da-conitec 2024. <i>Perspectiva do Paciente</i>: atualização das orientações pode ser consultada no site da Conitec. November 11, 2024. CONITEC website: https://www.gov.br/conitec/pt-br/assuntos/participacao-social/perspectiva-do-paciente/orientacoes-perspectiva-do-paciente. Orientações <i>Perspectiva do Paciente</i>. October 4, 2024. Brígida de Souza (2024)
Chile	Ministry of Health (MSP) and affiliated agencies conduct HTA internally to inform health system policies and regulations. A multi-agency National Commission for HTA (<i>Comisión Nacional de ETESA</i>) was established in 2013 to propose a centralised, multistakeholder HTA institute. Discussions are ongoing.	Patient input is solicited as needed by the Ministry of Health and related institutions depending on specific HTA projects. Patient organisation representatives have seats on the oversight and topic selection committees to implement the <i>Ricarte Soto Law</i> (LRS) that provides coverage for high-cost diseases based largely on HTA appraisals. Patient organisation representatives serve on a civil society advisory council that advises the country's health superintendency on public policy programmes.	Implementation (but only under LRS)	<i>Biblioteca del Congreso Nacional de Chile</i>. Resolution 738. Comisión Ciudadana de Vigilancia y Control. June 29, 2017. https://www.superdesalud.gob.cl/app/uploads/2017/07/articles-16215_recurso_1.pdf <i>Superintendencia de Salud</i> website: https://www.superdesalud.gob.cl/tax-gestion-institucional/participacion-ciudadana-614/consejo-de-la-sociedad-civil-6145/

(continued)

Table 20.1 (continued)

Country	Lead agency	Patient involvement mechanism	Status	Source(s)
Costa Rica	The Costa Rican Social Security Fund (CCSS) and affiliated agencies and departments announced in 2024 the creation of an HTA institute. A National HTA Commission has been in place since 2011.	An HTA Review Committee within CCSS will promote public participation in HTA. The Committee will include one patient representative selected and trained by the Federation of Patient NGOs. A second consumer representative will be selected by the National Council of Health Interest Groups (<i>Consejo Nacional de Juntas de Salud</i>). Representatives will be able to identify additional patients to be involved in HTA, depending on the topic of the appraisal.	Design	Personal Communication
Colombia	IETS was created in 2011 as a non-profit with the Ministry of Health and other government health agencies as key members.	IETS published a guide for public participation in HTA in 2014 but without specific roles for patients or patient organisations. Anyone can propose appraisal topics and comment on draft HTA findings and reports. IETS also has an online training module. An IETS manual for patient participation with more explicit mechanisms has been drafted but not yet published. IETS employs qualitative research expertise and has staff who have undertaken training in using this research into patients' needs and experiences in HTA.	Early implementation	IETS <i>Manual de Procesos Participativos</i> . Bogotá D.C.: IETS; 2014. https://www.iets.org.co/Archivos/65/Manual%20Participacion.pdf . IETS Video: <i>Presentación del Manual de Participación de Pacientes</i> https://youtu.be/kImIWrchA?si=lp8BAVWMw7z-1QxX See Sect. 24.2.4
Ecuador	Ministry of Public Health (MSP) conducts HTA via an internal department (<i>Dirección Nacional de Evaluación de Tecnologías Sanitarias</i>).	MSP can solicit HTA input from the public, patients, or patient organisations on a case-by-case basis. Effective citizen participation in health is a goal within the current system. An initiative is still being developed to formalise public participation, including possible specific mechanisms for patient participation.	Deliberation	MSP website. <i>Ecuador y Reino Unido comparten experiencias sobre la Evaluación de Tecnologías Sanitarias para fortalecer el Sistema Nacional de Salud</i> . February 2024. https://www.salud.gob.ec/ecuador-y-reino-unido-comparten-experiencias-sobre-la-evaluacion-de-tecnologias-sanitarias-para-fortalecer-el-sistema-nacional-de-salud/ .

Table 20.1 (continued)

Country	Lead agency	Patient involvement mechanism	Status	Source(s)
Uruguay	AETSU was created by law in 2020 and established in 2021. The Ministry of Public Health (MSP) administers the finances of the agency, but AETSU analyses are independent of MSP.	AETSU does not have an explicit HTA patient involvement mechanism. In June 2023, AETSU's regulatory basis was revised to include interactions with external stakeholders, including patient organisations. In November 2024, the agency hosted a workshop with patient organisation leaders to get initial input on participation mechanisms in the agency's HTA processes.	Deliberation	MSP Decree: AETSU Ajuste de Reglamentación. June 26, 2023. https://aetsu.org.uy/wp-content/uploads/2024/08/DECRETO-241-021-26-06-2023.pdf . AETSU website: <i>AETSU impulsa el concepto de "Paciente Experto" en un taller nacional</i> . November 25, 2024. https://aetsu.org.uy/aetsu-impulsa-el-concepto-de-paciente-experto-en-un-taller-nacional/

CONETEC Comisión Nacional de Evaluación de Tecnologías de Salud (Argentina), CONITEC Comissão Nacional de Incorporação de Tecnologias no Sistema Único de Saúde (Brazil), SUS Sistema Único de Saúde (Brazil), CNS Conselho Nacional de Saúde (Brazil), ETESA Evaluación de Tecnologías Sanitarias (Chile), LRS Ley Ricarte Soto (Chile), CCSS Caja Costarricense de Seguridad Social (Costa Rica), NGOs non-governmental organisations, IETS Instituto de Evaluación Tecnológica en Salud (Colombia), MSP Ministerio de Salud Pública (Ecuador), CENETEC: Centro Nacional de Excelencia Tecnológica en Salud (Mexico), CSG Consejo de Salubridad General (Mexico), CSSP Caja del Seguro Social de Panama, CETES Centro de Evaluación de Tecnologías en Salud (Peru), INS-MINSA Instituto Nacional de Salud—Ministerio de Salud (Peru), IETS/ Instituto de Evaluación de Tecnologías en Salud e Investigación, EsSalud Seguro Social de Salud del Peru, AETSU Agencia de Evaluación de Tecnologías Sanitarias de Uruguay, MSP Ministerio de Salud Pública (Uruguay)

20.3 Case Studies

20.3.1 Colombia

Colombia faces considerable challenges in financing quality healthcare and managing the adoption of new, high-cost technologies while striving to provide universal healthcare coverage. Its HTA agency, the *Instituto de Evaluación Tecnológica en Salud* (IETS), was launched in 2011 to assess and appraise the evidence to support the health ministry's decision-making regarding technologies in the guaranteed health benefits package (Cuadros and Gallego Ortiz 2021). Although the IETS is only advisory, its technical capacity has grown with responsibilities that include a key support role for agencies like the *Cuenta de Alto Costo* (High-Cost Account), which manages catastrophic illness funds and runs specialised disease programmes (ibid.), or with the health ministry's effort to determine which health technologies should be excluded from coverage with public resources (Minsalud 2023).

IETS lists different stakeholders, including patients, in its 2014 manual describing a mandatory process for HTA-related patient and public consultation (IETS 2014). However, the manual does not describe or define any specific role for patients or consumers other than a consultative one to provide feedback on IETS preliminary findings or draft HTA reports. Nevertheless, IETS has an ongoing effort to involve patients more explicitly.

In 2020, with funding from the Latin America Patients Academy (LAPA) and technical expertise from the international wing of the UK's National Institute for Health and Care Excellence (NICE International), IETS began drafting an HTA patient participation guide (McKee 2020). A listening exercise with expert patient leaders was fundamental to developing the guide to better understand patient and patient organisation perspectives on access to health technologies in the Colombian healthcare system. IETS also widely surveyed patient organisations to gauge their interest in HTA and brainstorm mechanisms for participation and input. This focus group and survey initiative subsequently paved the way for a working relationship between patient leaders and IETS to finalise the draft patient participation guide and to establish a training and capacity-building programme in HTA and pharmacoeconomics for patients and patient organisation representatives.

Indeed, training for patients and carers is available on the IETS website, and general principles for transparency and conflict-of-interest management are also outlined there (IETS n.d.). The patient participation guide has not yet been published, but in its current draft form, it is explicit about how patients can be involved in all HTA stages, including topic selection, evidence synthesis, report drafting, recommendations, and results dissemination (Ordoñez and Fuertes 2020). The draft guide does not mandate patient participation, but by indicating the many ways IETS evaluations could include patient representatives, the manual should become an important reference and resource to promote patient participation in future HTA efforts. Additionally, IETS employs qualitative expertise which can be used to integrate research into patient and carer needs, experiences, and perspectives in HTA (see Sect. 24.2.4).

20.3.2 Chile

Patient participation in HTA emerged formally in the Chilean health system with the implementation of a 2015 law to regulate access to high-cost health technologies, known as the *Ley Ricarte Soto*. The law and the health coverage programme it inspired is named after Ricarte Soto, who championed a patient movement seeking financial protection for families affected by catastrophic illnesses. Given the law's origins, the process of gradually incorporating diseases and their medicines for government coverage included citizens and patients from the outset to help identify, evaluate, and prioritise technologies and help oversee the entire programme (Biblioteca del Congreso 2016).

The process governing the implementation of the new programme was outlined in a resolution from the Chilean Ministry of Health (Diario Oficial de la República de Chile 2016). Identifying technologies to appraise begins with an open call to the public to solicit health technology evaluation requests. The ministry then publishes a short list of candidate technologies for its internal department to conduct an abbreviated HTA appraisal of each. These HTA reports are then referred to a multistakeholder commission of clinicians, public health experts, and patients to decide on the final list of technologies to be prioritised. With two seats on this commission, patients have both a voice and a vote in identifying the high-cost technologies the programme will cover.

In addition, citizens and patients are given 5 days to provide input on the commission's final suggested list of technologies for programme coverage. While this is not direct input on the HTA appraisals, it is an important opportunity for the broader patient community in Chile to voice concerns and issues that must be addressed officially by health authorities before programme adoption. Finally, the Ricarte Soto programme created a macro-level commission for ongoing monitoring and oversight. This 12-person commission includes seats for four patient representatives, each elected to participate via votes from patient-based civil society organisations.

In summary, there is little doubt the Ricarte Soto law and coverage programme has enhanced patient participation in health care decisions informed by HTA in Chile. Despite this, some research has demonstrated a high number of instances of technical disagreement between what HTA analysis recommends and what eventually becomes implemented (Armijo et al. 2022). Although there is a transparent and highly participative process for patients to weigh in on what should be covered, important challenges remain. There is also a long-running discussion in Chile about centralising all government-led HTA efforts into a national agency or institute to support and guide evidence-based healthcare resource decision making (Lavín et al. 2017). Should this idea gain further momentum and become a reality, the experience of the Ricarte Soto programme could be important in determining the level of patient participation adopted as part of any new government-wide HTA institute or agency.

20.3.3 Brazil

The *Comissão Nacional de Incorporação de Tecnologias no Sistema Único de Saúde* (CONITEC) was established by law in 2011 and is responsible for the HTA process in Brazil. Specifically, CONITEC advises the Brazilian Ministry of Health on whether a given health technology should be incorporated, excluded, or altered within the *Sistema Único de Saúde* (SUS) universal public health system based on recommendations informed by HTA (Caetano et al. 2023). In general, the HTA process in Brazil has broad stakeholder participation in most phases and has become one of the region's more complete health technology evaluation systems (Lima et al. 2019).

Opportunities for public participation include time-limited consultations of the public on CONITEC recommendations supported by plain language technical reports. In addition, anyone from the public can suggest topics for evaluation. In both respects, patients and patient organisations have been frequent contributors to the process. In one review of CONITEC processes over 5 years, almost half of all submissions during the consultation phase were from patients, their carers, and patient organisations (Silva et al. 2019). One can also infer substantive patient participation in the public consultation phases of HTA since at least one study has shown that CONITEC does appear to be influenced when there is a high volume of contributions submitted by patients or their families, more frequently changing a 'no' to a 'yes' when the evaluation concerns potential SUS incorporation of a medicine (De Freitas Lopes et al. 2023). Between 2012 and July 2024, CONITEC received more than 406,000 submissions for 782 public consultations (Brigada 2024). From 2021 to July 2024, the agency conducted thematic content analysis for 65 public consultations for CONITEC members to review prior to a final decision (ibid.)

Additionally, in 2022, CONITEC piloted the use of qualitative evidence synthesis (see Chap. 11) for some HTAs and guidelines to increase the consideration of patient and carer perspectives. These have been especially used for rare disease technologies with five of the six syntheses completed by July 2024 being for rare diseases (Systemic lupus erythematosus, familial amyloidotic polyneuropathy, mucopolysaccharidosis type 1, mucopolysaccharidosis type 2 and cystic fibrosis (Brigada de Souza 2024)). To the extent patients and patient organisations have seats on the *Conselho Nacional de Saúde* (CNS), a multisectoral health advisory body comprised of public sector health consumers and workers, this is another opportunity for patient participation in HTA since CNS has a seat on each of the three main CONITEC working committees. Nevertheless, this mechanism via the CNS is widely considered insufficient for significant or effective patient participation (Silva et al. 2019).

A more direct mechanism for patient participation in HTA in Brazil is patient testimony provided at CONITEC plenary sessions on a person's lived experience with the health condition or technology in question. Patients with asthma participated in the CONITEC plenary process to review inhalers in 2014 and 2015 (Single

et al. 2019). By 2021, CONITEC made these patient testimonials a more official part of the beginning of the HTA appraisal process (Portugal et al. 2021; Meneses et al. 2024). This patient perspective initiative (*Perspectiva do Paciente*) standardised the patient participation process by asking patients, carers, or patient organisations to register in response to each new HTA topic to be deliberated. One registrant plus an alternate are then chosen either by consensus (if fewer than 30 registrants), by lottery (if more than 30), or via direct outreach by CONITEC to known parties in the case that no one registers or if the pool of registrants is deemed inappropriate by CONITEC staff. Chosen patient participants are provided training on how CONITEC functions and how to access the virtual platform on the day of the meeting.

It is important to note that since the inception of *Perspectiva do Paciente*, CONITEC has continuously revised portions of the process over the years to make it more efficient and transparent (Meneses et al. 2024). The most recent guidance was provided in October 2024 (CONITEC 2024a, b). There is also research underway to know how these patient-level contributions improve the process and impact CONITEC and health ministry decision making (Oliveira et al. 2024). CONITEC and its stakeholders support the inclusion of the patient perspective, and there is an overall strong consensus on the fundamental role and value of such input in HTA in Brazil.

20.4 Discussion

LAC countries differ regarding health outcomes, health system structures and procedures, and health resource allocation decisions. Several countries have HTA systems, but only a few have institutionalised HTA with robust methods and processes. Patient involvement-specific mechanisms in those more established settings are evolving out of a pre-existing commitment to public participation in HTA.

Several challenges exist, such as public or patient understanding of the technical information and sustainable mechanisms allowing regular citizens to add value alongside payers, clinicians, public health experts, and government officials. Understanding what is at stake for their constituents, patient leaders of organisations across LAC are generally willing to learn and build capacity to participate in HTA if provided the training and growth opportunities necessary.

Indeed, a common characteristic among countries in LAC is the relative strength of patient organisations as key stakeholders. For example, patient participation in HTA in Chile came from patient advocacy for a new law and programme prioritising civic participation in health coverage decisions. Brazil, Colombia, Argentina, Mexico, and Peru also have relatively strong patient advocacy movements, and there is a general desire among many patient organisations in those countries to be consulted on HTA recommendations and included in developing them. Of course, continued educational outreach and training efforts to build the capacity of patient advocates to understand health economics and HTA methodologies are critical (Espinoza et al. 2019). Where bottom-up demand for HTA participation can meet

top-down willingness, openness, and capacity to include patients as key stakeholders, patient participation has the best opportunity to grow.

There is one interesting caveat to the relative strength of patient advocacy across LAC. In countries where the health authority is traditionally weak or weakened by current political trends, patient advocacy can overwhelm health governance channels, resulting in interest-driven health resource allocation. In Chile, this has been labeled as the “legal path to priority setting”, where new laws promoted by patient organisations and supported by lawmakers are implemented to favor one disease or health technology over others (Espinoza et al. 2023). In other jurisdictions, this is related to the judicialisation of healthcare access, where individuals appropriate legal means to obtain a technology somewhat regardless of health system coverage rules and regulations (Borges 2018). In this context, it is essential to note that these advocacy and access tactics are not what patient involvement in HTA is about. In contrast, consistent and fair patient involvement mechanisms may help to overcome other mechanisms that foster inequity. In any case, continued research is needed on these dynamics across the LAC region as they relate to HTA systems and their increasingly important role in health resource allocation and prioritisation.

20.5 Conclusion

This chapter examines the current status of patient participation in HTA in LAC. Some countries have mechanisms and standards for patients and patient organisations to participate, while others have yet to establish systems for public input at any level. A few countries are in the early implementation stages of patient participation mechanisms, while others are in the design or deliberative stages. Patient participation is the dominant approach. LAC countries can learn from the experience and progress achieved in Colombia, Chile, and Brazil.

Current political trends, the relative strength of key health stakeholders, and international dynamics that continue to shape HTA methodology are all essential factors in the adoption of patient involvement mechanisms. All things considered, effective patient involvement across Latin America is expected to grow over time as long as HTA systems and processes continue to mature.

Acknowledgements We extend our heartfelt appreciation to the following individuals for their insights and contributions through interviews. Their perspectives and expertise have been instrumental in shaping this work, and we sincerely appreciate their time and dedication.

Martin Petrocco (Patient Organisation, Argentina); Verónica Stasiak (Patient Organisation, Brazil); Priscila Torres (Patient Organisation, Brazil); Cecilia Rodriguez (Patient Organisation, Chile); Adriana Robayo (HTA/MoH, Colombia); Jorge Garcia (Patient Organisation, Colombia); Luz Victoria Salazar (Patient Organisation, Colombia); Lorena Mesa (HTA/MoH, Colombia); Karla Ruiz de Castilla (Patient Organisation, Peru); Rosina Hinojosa (Research, Peru); Elizabeth Guambo (HTA/MoH, Ecuador); Hugo Marin Piva (HTA/CCSS, Costa Rica); Alexandra Nuñez (Patient Organisation, Costa Rica).

There was no funding for writing this book chapter or conducting the underlying research.

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Chapter 21

The Philippines: Systematising Patient Participation in HTA



Tisha Isabelle M. de Vergara, Eunice U. Mallari, Anne Julienne Genuino-Marfori, Sheena Jasley G. Samonte, Patrick Wincy C. Reyes, and Christopher Munoz

21.1 Context

In 2019, the Universal Health Care (UHC) Act was signed. It mandated Health Technology Assessment (HTA) as “a fair and transparent priority setting mechanism for the development of policies and programs, regulation, and the determination of a range of entitlements” (Republic of the Philippines, *Republic Act* No. 11223 Section 34.8 2019) in recognition of the role of evidence-informed policy and decision making in the pursuit of UHC in the Philippines.

To facilitate this, the HTA Council, an independent advisory body composed of a Core Committee and seven Subcommittees was created in October 2019 with the overall role of providing guidance to the Department of Health (DOH) and the Philippine Health Insurance Corporation (PhilHealth) on the coverage of health technologies to be publicly funded. Among the voting Core Committee members is the Citizen Representative who brings in the perspective of the patient and carers which adds balance to the different fields of expertise and considerations in HTA as recommendation develops. The HTA Division was created as a supporting office for

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the HTA Council in carrying out its mandates. Specifically, it provides technical, secretariat and administrative support, and generates evidence through the conduct of internal assessments and the management of externally commissioned assessments.

As part of institutionalising HTA in the country, the Administrative Order 2020-0041 or *The New Implementing Guidelines on HTA to Guide Funding Allocation and Coverage Decisions in support of UHC* was created and issued (Republic of the Philippines 2020). The *Process Guide and Methods Guide* were developed to provide the specific guidelines on the general steps and methodological standards of HTA. Upon the establishment of a functioning HTA system at the DOH, the HTA Division's operations were transitioned to the Department of Science and Technology (DOST) in March 2023.

During the COVID-19 pandemic, the Philippines was placed under a state of public health emergency in early 2020. The newly established HTA process and methods were tested through a 'baptism by fire.' The overall shift in the national health priority to respond to COVID-19 demonstrated significant challenges and barriers in conducting quality assessments in the shortest possible time while ensuring effective stakeholder involvement activities.

These limitations did not impede collaborations with Civil Society Organisations (CSOs). Part of the webinar series to introduce the HTA implementation guidelines in the country was tailored to CSOs and the general public. Several virtual meetings were conducted to facilitate their participation in focus group discussions and key informant interviews for Ethical, Legal, Social, and Health Systems Impact (ELSHI) assessments of several health technologies for COVID-19. These aimed to explore patient preferences and values, and associated aspects with or without the use of a specific health technology. In 2022, the *Guidelines on the Application of Philippine Social Values on HTA* (Philippine Health Technology Assessment 2022) was released which details Filipino social values that shall be considered in the formulation of HTA Council recommendations. Specifically, it recognises subpopulations that need special considerations (i.e. children and older adults, women, sexual/gender minorities, disability and special conditions, resource-poor, geographically isolated and disadvantaged areas, and Indigenous Peoples).

The HTA process in the Philippines envisions a participatory approach encompassing various stakeholders, including among others, the general public, CSOs, healthcare providers, industries, and researchers. It intends to ensure that all are involved and contribute to the relevance, acceptability, and legitimacy of healthcare decisions (see Table 21.1). Although the process guide and methods guide provide information on which parts of the HTA process these stakeholders may be involved in, details on the engagements are not thoroughly presented. There are current efforts to define stakeholder participation in the ongoing revision of the HTA implementing guidelines through public consultation. In the topic nominations, we crafted a special form for CSOs and the public which gives more emphasis to lived experiences, rather than getting scientific evidence about the health technology. In addition, the HTA Stakeholder Engagement (SHE) Framework is being developed

Table 21.1 Roles and responsibilities of stakeholders per HTA Process Step (General Track) (DOST HTA Philippines SHE Framework 2024 [draft])

Technology assessment process step	Roles of external stakeholders
Topic nomination	Submit topics for HTA
Topic prioritization	Provide needed information or prioritisation criteria for HTA topics Submit feedback during the public consultation on the prioritised HTA topics
Topic assignment	Submit an HTA research proposal for their topic
Finalisation of research questions	Provide insights and information
Protocol development	Provide insights and suggestions to the protocol
Topic assessment	Provide data to support clinical, economic, health system, ethical, legal, and social impact assessments
Review of preliminary assessments	None
Evidence appraisal and preliminary recommendation	Provide their views and inputs in the HTA Council meetings (by invitation)
Provisional recommendation	
Stakeholder consultation/feedback	Submit comments or feedback to the HTA Council provisional recommendation
Finalisation of the recommendation	None
Decision by the Secretary of Health (SOH) or PhilHealth Board of Directors	None
Dissemination	Contribute to dissemination of policy briefs and SOH-approved HTA Council recommendations

HTA health technology assessment, SOH Secretary of Health

to classify the types and levels of participation across the different HTA activities and how to operationalise them. This ensures that the engagements are consistent, effective and meaningful for an HTA process that is responsive to the present and future needs of the health sector. For consistency, we adopted the term ‘patient participation’ for this chapter to refer to our patient engagement activities.

21.2 Current Structures for Patient Participation

Several mechanisms are put in place to ensure meaningful participation of CSOs in HTA: (1) asynchronous feedback; (2) topic assessments; (3) public consultations; and (4) partnership with CSOs’ activities related to HTA.

The levels of stakeholder participation in the Philippine HTA are categorised into (a) informative, (b) consultative, and (c) collaborative, which may occur simultaneously across different HTA process steps. *Informative* refers to collecting or receiving information from stakeholders, while *consultation* is a form of open participation

wherein all stakeholders are invited to provide comments and suggestions to the HTA process or a particular topic. Lastly, *collaboration* is a more direct participation in HTA through the conduct of assessments and facilitation of joint dialogues or partnerships. Facilitation of invitation through open call or by invitation may be done depending on the process track step, and are conducted, either via online platforms or face-to-face events.

As part of *informative participation*, HTA Philippines accepts asynchronous feedback through document-based participation on topic nomination submissions, assessment reports, and issuances (e.g. DOST HTA Philippines Topic Nomination and Prioritization Guidelines, 2023). For *consultations*, stakeholders may provide feedback for the draft priority HTA topics list and provisional HTA Council recommendations. Meanwhile, targeted primary data collection (e.g. interviews, discussion groups) are conducted and summarised to inform ELSHI assessments. At the *collaboration* level, partnership activities with CSOs were initiated through the Philippine Alliance of Patient Organizations (PAPO). These include capacity-building activities (e.g. topic nomination workshops, HTA guideline dissemination forums, patient dialogues) and other promotional activities (e.g. World Patient Safety Day).

21.3 Spotlight on Selected Aspects of Patient Participation

21.3.1 History of PAPO Involvement in HTA Philippines

Since 2011, PAPO has advocated for UHC and patient welfare and amplified the representation of patient voices in issues that concern Filipino patients. As a *Securities and Exchange Commission (SEC)*-recognized umbrella organisation, PAPO partnered with various government agencies, private corporations, and non-governmental organisations. Additionally, PAPO engaged in various government-led efforts including patient advocacy events, capacity-building workshops, and policy-making councils (e.g. Cheaper Medicines Act, Patient Safety, Cancer, and Anti-Deposit Law).

Further, PAPO helped to recruit participants to serve as resource persons during data gathering via discussion groups for HTA Philippines' COVID-19 assessments. PAPO also helped bring together CSO representatives to take part in the consultation, training, and dissemination of the open HTA Topic Nominations process. Nominations from patients or organisations without SEC registration and with limited capacity are channelled through PAPO. In recognition that some CSOs have limited access to clinical evidence, HTA Philippines collaborates with PAPO to supplement the CSOs' nominations with evidence via correspondence with medical societies. This has facilitated a higher quality of CSO-nominations and promoted an inclusive HTA process for all stakeholders. The improved inclusivity is demonstrated by the increasing number of topic nominations by CSOs (see Fig. 21.1).

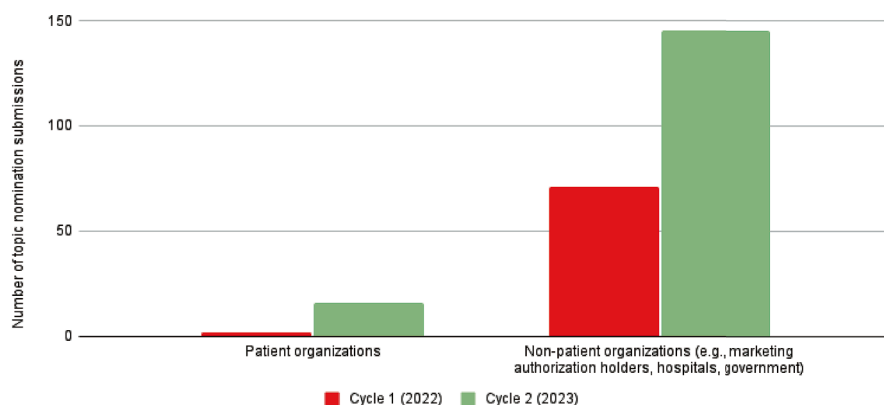


Fig. 21.1 Number of submissions by nominator type for Cycle 1 and Cycle 2 of General Track. (Source: HTA Philippines topic nominations tracker)

HTA Philippines continues to closely collaborate with PAPO and other stakeholders for the continuous improvement of meaningful patient participation activities in the HTA process.

21.3.2 *Topic Nomination and Selection*

In 2022, HTA Philippines opened the first cycle of topic nominations for all stakeholders in the country. This included CSOs, for whom we initially created a separate nomination form that was lengthier than the current version, with Filipino and English translations made available separately. Experiences during this first cycle of topic nominations highlighted some issues, including underestimation of working timelines due to the large volume of nominations, the inadequacy of the prioritisation criteria parameters that did not give sufficient advantage to neglected diseases (e.g. rare diseases and rare cancers), and the need to increase exposure of the public to HTA process. Stakeholders also indicated the need for a more tailored process that took their lived experiences into account.

In response, in 2023, HTA Philippines released a new *Topic Nomination and Prioritization Guidelines* along with the revised HTA nomination forms, including simpler, shorter and easier to complete nomination forms for CSOs and the general public. Heavily technical questions were removed and the questions focused more on the personal experiences of patients with the disease and the use of health technology. The English and Filipino versions were integrated into a single form for convenience and straightforwardness. Cognisant of the time and resources spent on completing the forms, an acknowledgement by the HTA Philippines is included in the nomination form signifying its commitment to ensuring all submissions are duly taken into account for an informative prioritisation and appraisal process among

HTA staff and committee members and HTA Philippines reports. The reports also note how patient inputs helped shape HTA Council resolutions on these process steps. On another note, patients are enjoined to give their best effort in providing notes on what the HTA Philippines has to know as well as sources for their information or supporting evidence including “web surveys, social networking, focus groups, patients/ CSOs’ records, one-to-one conversations with those in clinical trials, and patient stories” (DOST 2023a). The questions asked, reflect the content areas often included in written submission templates for HTAs in other jurisdictions (e.g. Chap. 4, Table 4.2) and are summarised in Table 21.2. HTA Philippines received positive feedback on its ease of use during the dissemination activity. Henceforth, the intention is to produce the form in various Philippine languages and by partnering with CSOs, HTA Philippines aims to produce forms that are accessible to individuals with visual or hearing impairment, among others.

A series of consultations was performed in co-designing the new HTA Topic nomination form with representatives from various CSOs (e.g. diabetes, rare diseases, lung diseases, skin conditions, cancer). During the consultations, feedback was gathered from patients on the draft nomination form to ensure its relevance and applicability to the local setting. During a second dissemination event, stakeholders simulated topic proposals with the finalised nomination form for validation.

Consequently, during the topic nomination in 2023, there was an increase in: (1) total nominations received from 73 (2022) to 161 (2023); (2) number of CSO-nominations from two (2022) to 16 topics (2023); (3) number of CSO-nominators from two organisations in 2022 to 10 organisations in 2023. Two out of the 16 CSO-nominations were prioritised for HTA in 2023. A preliminary list of priority topics is released for public consultation and proponents may submit appeals on their submitted topics. In response, HTA Philippines provides a summary of prioritisation scoring and its rationale to the proponents. Stakeholders will be informed of the final list of prioritised HTA topics through an official issuance published at the HTA Philippines website and social media page. Currently, with the direction of aligning HTA efforts more closely with the UHC, there is a working proposal for topic identification to be led by DOH and PhilHealth along with stakeholder consultations which shall include patients.

21.3.3 Targeting Underserved Communities Individuals

Due to lockdown measures and mobility restrictions during the COVID-19 pandemic, all communications and processes, including assessments were moved online. Social media and other online communication platforms were used for data gathering, especially among underserved communities and individuals. As an example, the assessment on the self-administered antigen test (Yang et al. 2024) looked into the experiences of healthcare workers and the public including at-risk groups (i.e. CSOs for chronic illnesses, human immunodeficiency virus). Focus group discussions were conducted in English and Filipino using the preferred

Table 21.2 Summary of *Topic Nomination Form for Patient Groups/Civil Society Organisations/General Public* (DOST 2023b)

Key areas	Elements
Name and type of health technology	• Type: Drugs/vaccines; clinical and medical device; preventive and promotive health; medical and surgical procedure; traditional, complementary and integrative medicine; other health technology (e.g. digital health)
Reasons for nominating health technology	• Alternative intervention to an existing drug, vaccine or device financed by the government (DOH and/or PhilHealth) needed due to clinical or financial reasons. Examples of situations where this might apply: – Drug, vaccine or device cannot be used in a subset of patients/CSOs due to possible intolerable side effects or adverse effects – Drug, vaccine or device listed in the PNF/PEMDL is not affordable or is difficult for patients/CSOs to access • No existing drug, vaccine or device for condition • Existing drug, vaccine or device, but with no existing government financing support yet for this disease (unmet need) • First of its kind or innovative and impactful in comparison to all existing drugs, vaccines or devices available in market for this condition
Patients/CSOs who will use health technology.	Details such as age, gender, ethnicity, subgroups, level of risk/severity, place of residence or other determinants relevant to the health technology
Setting	In-patients, out-patients or both
Burden: What is the effect of the condition or disease in the quality of the daily life of the patient or CSOs?	• Aspects of condition that are most challenging (e.g. symptoms, loss of ability to work, loss of confidence to go out, inability to drive, social exclusion) • Frequency of occurrence of medical condition (e.g. intermittent, sporadic, episodic, permanent) • Activities that patients or CSOs find difficult or are unable to do • Aspects of condition most important to control (e.g. symptoms that limit social interaction or ability to work such as difficulty breathing, pain, fatigue, incontinence, anxiety) • Support required for daily living (physical or emotional) • Challenges in managing main condition (e.g. main symptoms, potential symptoms, most common visible signs, mental health challenges, life expectancy) when patients or CSOs also have other medical conditions • Self-stigma or discrimination in society (how?)
Burden: How does the condition or disease affect carers?	• Challenges faced by family, friends and/or carers • Impact on family, friends and/or carers daily life (e.g. emotional/psychological effects, fatigue, stress, depression, physical challenges).

(continued)

Table 21.2 (continued)

Key areas	Elements
Cost of the disease	• Hospitalisation? If yes, how long? • Surgery? If yes, enumerate the surgeries • Outpatient care (e.g. maintenance medicines, regular testing)—if yes, describe outpatient care needed by patient • Indirect cost of care (e.g. loss of productivity, cost of transportation, cost of carer) if yes, describe the indirect costs incurred
Unmet need	• To your knowledge, are there treatments locally available to manage this condition? Describe these treatments. • Are there any challenges in accessing services related to the management of this condition? • Challenges from consultation, screening, diagnostics and treatment, in terms of any of the following: – Financial capacity to access services (any medical services accessed via local or international donations) – Geographical accessibility to health facilities (e.g. medical services done locally or internationally) – Availability of actual services or healthcare workers to provide such services among others
Experience with current therapies	• How well are patients or CSOs managing their condition with currently available therapies or home remedies (e.g. any form of medical intervention such as medicines, rehabilitation, counselling, hospital interventions, or home remedies) • How does current therapy or home remedy help address or manage condition? (is it preventive, promotive, curative/ rehabilitative, diagnostic, non-curative but life prolonging, life sustaining; provide temporary relief, emergency care; palliative action?) • Does patient need assistance of healthcare professionals or caregivers in administration of therapy? • How do current therapies benefit patients or CSOs? • To what extent do current drugs, vaccines or devices control or reduce most challenging aspects of condition? • What are the difficulties encountered by patients or CSOs in complying with currently available therapies (e.g. difficult to administer or receive, interruption to work, side effects from therapies that are difficult to tolerate, stigma, clinic visits, and need for weekly blood tests)? • Concerns about long-term use of current therapy?

(continued)

Table 21.2 (continued)

Key areas	Elements
Experiences with nominated therapies	• How does the nominated health technology make a difference in patients or CSOs’ lives? Response may be first-hand experience or information acquired from reading or experience of someone you know • Advantages and disadvantages of nominated health technology compared with current therapy in terms of: Mental and emotional health – Ease of use – Improvement of symptoms and quality of life – Side effects especially those which are new and are difficult to tolerate – Impact in use of healthcare services (e.g. fewer visits to hospitals) – Accessibility and availability – Financial implications (e.g. cost of health technology, other cost incurred to specific to the technology) – Adherence to treatment – Impact to carers
Additional information	Any additional information not covered in sections above which you think are relevant to prioritise this topic

CSO Civil Society Organisations, *PNF* Philippine National Formulary, *PEMDL* Philippine Essential Medical Device List

platform of the participants. One of its key findings was streamlining of self-reporting wherein participants expressed the need for centralised reporting and systematic patient information systems.

Furthermore, evidence briefs containing key information related to the health technology and the HTA Council recommendations were published for topics that were assessed during the COVID-19 pandemic, when the process was still new.

In addition, HTA Philippines plans to disseminate the revised HTA Implementing Guidelines and SHE Framework through face-to-face and online dissemination forums in different areas in the country. The dissemination activities are particularly targeted to reach local CSOs and the general public. Subsequently, HTA Philippines plans to conduct regular patient participation activities and establish a solid platform for regular involvement with the help of local CSOs.

21.4 Reflections

Current efforts for patient participation have been effective and reliable in ensuring that HTA remains participatory and reflective of the views and experiences of patients. Since we are currently working towards being an attached independent agency to DOST, which is the final phase to our institutionalisation, this implies bigger office support that can also facilitate more activities and partnerships with

patient groups and citizens. This requires flexible government funding mechanisms to gather support from external organisations and allow HTA Philippines to sustain their participation. A variety of training programmes on topics such as conducting evidence appraisals and other methodologies will be done to build capacity in CSOs towards a more comprehensive understanding of HTA and increased participation in consultations. Nonetheless, the continuous development of HTA in the country, along with the revised Philippine HTA Implementing Guidelines and SHE Framework, prompts the strengthening of the established partnership with all stakeholders gearing towards more collaborative participation.

Acknowledgements The authors would like to acknowledge the invaluable contributions of previous HTA Division staff who helped in the writing of the SHE Guide: Joshua S. Santillan, Jayne Eunice U. Yang, and Faisal H. Jackarain. The authors also express their gratitude to all the members of the HTA Council and HTA Division who are involved in all patient-participation activities. Lastly, the authors would like to acknowledge helpful comments received from the editors and reviewers while revising the original manuscript. All views expressed and any errors are entirely the responsibility of the authors.

Funding No funding was received for writing of the manuscript or the underlying research.

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Chapter 22

Republic of Korea: Tackling Misinformation with Patients in HTA



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22.1 Context

South Korea is a prime example of a nation that has successfully implemented Universal Health Coverage (UHC) within a short period through a single, unified National Health Insurance (NHI) system in 2000. The UHC system faces the constant challenges of balancing the demand for healthcare services with the availability of limited resources. These challenges necessitated the institutionalisation of health technology assessment (HTA) in Korea, making benefits list decisions explicit through effectiveness or cost-effectiveness assessments (Oh et al. 2023). For pharmaceuticals, Health Insurance Review and Assessment service (HIRA) reviews the dossiers submitted by the company. Based on HIRA's review, the Ministry of Health and Welfare (MoHW) decides whether the drug will be covered by NHI. National Health Insurance Service (NHIS) then negotiates the price with the company. For non-pharmaceutical technologies, National Evidence-based healthcare Collaborating Agency (NECA) conducts systematic reviews to assess safety and effectiveness. This evidence, along with input from HIRA, informs the MoHW's decision on NHI coverage. Then HIRA/MoHW determines the relative value scale (RVS) for the technology.

Meanwhile, the Korean healthcare delivery system is characterised by patient choice. While this feature including selection of providers and private insurance alongside social insurance can improve access to care, there is a risk of overusing health services unless patients are equipped with correct information. Korea also exhibits high rates of internet connectivity, smartphone use (1st), and social media engagement (2nd) among 27 developed nations (Taylor and Silver 2019). This digital landscape has democratised access to health information, but YouTube's

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user-generated content raised reliability concerns. A Korean celebrity promoting an unproven cancer treatment (dog anthelmintic) via social media exemplified the rapid spread of misinformation (Yoon et al. 2022).

Critical health literacy is an essential skill for individuals to navigate the ever-growing landscape of health information. This ability empowers them to discern reliable sources and make informed decisions. A survey result in Korea also indicates that effective information filtering is a crucial yet underdeveloped skill within the population (Park 2024).

A knowledge gap exists between the public regarding the reliability of medical information published in social media. NECA has gone beyond policy by undertaking HTA activities tailored to public needs through engaging them in the process. It helps to empower patients to make informed healthcare decisions by addressing information needs and providing accurate information.

22.2 Current Structures for Patient Participation in Korea

NECA has pioneered patient participation in HTA since its inception in Korea, progressively integrating it across all stages—identifying and prioritising topics, undertaking assessments, disseminating findings, and evaluating the impact. Furthering the commitment to patient and public participation, NECA established the Public Participation Group (PPG) in 2017. The PPG, initially composed of patient advocates, later expanded to include a wider range of citizens, ensuring diverse perspectives are considered. In 2023, NECA incorporated social value considerations like patient preferences, treatment experiences, and other relevant factors into health technology reassessments through patient focus group interviews. These initiatives demonstrate NECA's dedication to ensuring patient voices are heard throughout the HTA process.

The NHIS, inspired by the National Institute for Health and Care Excellence's (England and Wales) Citizen Council, established the Participatory Priority Setting Committee (PPSC) in 2012. This committee serves as another example of citizen participation at the health insurance coverage stage in HTA. Composed of 30 citizens, the PPSC initially reviewed information from experts and recommended priority revisions for the benefits list (Oh et al. 2015). Later, the PPSC's focus shifted to providing general health insurance policy guidance.

Since 2009, patients have been able to directly suggest healthcare issues as topics for HTA through a public topic suggestion track in NECA. In the remaining chapter, we will highlight an approach that has been developed recently in partnership with the PPG as a new way to inform topic selection for HTA.

22.3 Highlight: Involving the Public and Patients in Monitoring and Prioritisation of HTA

22.3.1 Monitoring Health-Related Social Media Communication

The vast amount of health information on social media, while empowering patients, can also lead to unnecessary healthcare spending and anxiety due to conflicting or misleading information. This unfiltered nature can infringe on individuals' right to health. To overcome these challenges, effective health information management is crucial (Klerings et al. 2015). In response, NECA PPG initiated social media health information monitoring in 2021. Its goals were to:

1. Identify and filter misleading health information, particularly regarding unproven treatments or miracle cures, and
2. Promote evidence-based health information dissemination to improve public health literacy.

To identify misleading health information, the PPG monitored Korea's top search channels: internet portals (online communities) and YouTube (Choi et al. 2020). They focused on information related to medicines or treatments lacking trusted evidence for safety and effectiveness. With 65 members actively monitoring social media for a month, the PPG collected 82 pieces of health information for further analysis.

22.3.2 Training the Participants

Before commencing the activity, the PPG members participated in training sessions to familiarise themselves with social media monitoring methods. The training focused on equipping participants with evidence-based health literacy skills by introducing criteria for identifying false health information, such as claims lacking trusted evidence or those based on personal anecdotes. To emphasise the prevalence of misleading information, researchers presented a case study revealing that nearly 30% of YouTube videos discussing a specific dewormer contained inaccurate information (Yoon et al. 2022). They also outlined common characteristics of false health information, such as a lack of credible sources and skewed presentations of outcomes. Additionally, the training provided specific monitoring tips, including methods to assess channel credibility and utilise YouTube filters to gauge content reach.

22.3.3 *Social Media Monitoring and Topic Identification*

The monitoring templates included a checklist outlining key aspects of the health information being monitored. These aspects included:

- 1. information regarding treatments
- 2. assertions lacking strong evidence but presented as generally beneficial or harmful to health
- 3. assertions made by non-experts (e.g. people without medical backgrounds).

Additionally, participants received a guideline (Table 22.1) to help them assess the information’s suitability for further analysis.

Following an analysis of 82 data points on medications and treatments, eight high-priority topics for HTA were identified. This process excluded information on dietary supplements and folk remedies. To evaluate these topics, a new vignette format was developed. Unlike traditional expert-driven HTA vignettes, these new versions incorporated metrics like view and comment counts for online information (YouTube videos and blog posts) to assess potential public impact. Additionally, patient responses from online communities, relevant news articles, and academic literature were included to gauge social interest and the potential severity of harm associated with the monitored information.

Table 22.1 A guideline for identifying false information: characteristics of misleading content

#	Category
1	The information is pervasively spread primarily on social media platforms such as YouTube, Facebook, or Instagram, by non-expert individuals, often lacking the necessary qualifications
2	It is difficult to verify the identity of the individual sharing the information
3	The claim lacks trusted evidence and relies solely on the personal experiences of a patient or a citizen
4	The information contains assertions about the effectiveness of a treatment method, potentially misleading the public as they contradict the assessments made by government agencies or experts
5	A non-expert claims their assertions are based on statistical data or papers from a renowned academic journal, but lacks the expertise to properly interpret the data or research
6	The information exhibits bias towards a particular viewpoint. This can manifest by solely presenting positive stories about a treatment method or focusing exclusively on negative experiences
7	The individual promotes conspiracy theories, suggesting without evidence that the government or pharmaceuticals companies are orchestrating them

22.3.4 *Setting Evaluation Criteria for HTA Topic Prioritisation*

To prioritise the HTA topics identified from the monitored data, a comprehensive evaluation process was established. This involved reviewing existing criteria used to assess the credibility of local and international media information. Sources included fact-checking guidelines from the American Press Institute and media fact-checking criteria developed by Seoul National University in Korea. Additionally, standards set by the Korean Academy of Medical Sciences for health information authentication (Shin et al. 2006) and evaluation criteria for potential health information harm (Ryu 2004) were considered. Based on this review, five key evaluation criteria were established for topic prioritisation (Table 22.2).

An expert committee, comprised of HTA methodology specialists from NECA and science journalists from the Korean Science Journalists Association (KSJA), then evaluated the initial HTA topics using these criteria. Through this rigorous process, two final HTA topics were chosen: ‘Saw Palmetto for benign prostatic

Table 22.2 Evaluation criteria for HTA topic prioritisation

Criteria	Item
Need for verification	Is the disease of high interest to the public?
	Is there societal confusion due to conflicting opinions among experts?
	Does the topic require verification by NECA, a specialised institution for HTA?
Feasibility of verification	Can this information be verified through systematic reviews or expert deliberations?
Veracity of information/ potential for misleading or deceptive manipulation	Is there a risk of bias in interpreting monitored information through selective evidence extraction?
	Is the monitored information based on subjective claims rooted in personal experiences?
Potential impact	Based on media type, views, comments, etc., has the monitored information generated a significant amount of discussion or mentions?
Potential harms	Can the monitored information potentially cause any of the following harmful effects on people’s behavior and actions?
	1. Physical: Could the information lead to significant physical harm by encouraging readers to delay or forgo necessary medical treatment, or by promoting treatments with serious adverse effects?
	2. Emotional: Could the information induce exaggerated hope or excessive anxiety by prompting readers to make a self-diagnoses without medical judgment, potentially leading to significant psychological harm?
	3. Economic: Could the information lead to significant financial losses for patients and consumers by encouraging unnecessary purchases of products or services, or by promoting unnecessary second opinions from healthcare professionals?

hyperplasia patients’ and ‘high-dose intravenous vitamin C in cancer patients’. NECA assessed safety and effectiveness for these chosen topics.

22.3.5 Dissemination of HTA Results

The inclusion of journalists from KSJA as an important stakeholder of HTA in the evaluation process provided valuable insights, enabling us to select HTA topics with greater public interest. As a result, when the research on the two selected topics—Saw Palmetto and intravenous vitamin C—were completed, the HTA findings garnered significant media attention and achieved wider dissemination compared to other HTA research. The results were broadcast on prime time on nationwide terrestrial television channels. Additionally, a video clip of the news was uploaded to the media’s YouTube channel, contributing to nationwide dissemination that would have been unlikely without collaboration with the media. Furthermore, approximately 34 articles on the HTA results were published online through various news outlets, with the findings continuing to be cited in related articles. A survey of 1000 Koreans revealed that over 70% of individuals rely on internet news articles, TV, and radio news for information and knowledge, with TV and news articles being the most trusted sources (Park 2024). Although the exact impact of media broadcasts on dissemination has not been measured, it suggests that the HTA results reached a broad audience.

Not only that, to make the HTA results more accessible to the public, the research abstracts were rewritten in plain language with the participation of PPG members, as explained in the following section.

22.3.6 From HTA Draft to User-Friendly Summary

The PPG played a crucial role in HTA beyond just identifying topics through social media monitoring. They also contributed to improving public health literacy by participating in the creation of plain language summaries of the findings.

Seven PPG members, who engaged in social media monitoring for HTA topics, were selected to refine plain language summaries drafted by researchers. Armed with a custom checklist, the group dove into the summary, dissecting its topic, difficulty level, clarity, and length. They identified challenging terminology, suggested missing information, and raised questions. This collaborative effort led to revisions that transformed the initial draft into user-friendly plain language summaries. Sharing the final version with all seven participants yielded positive feedbacks. All members reported a significant improvement in their ability to understand and utilise health information. One participant even remarked, “Discussing HTA results cleared up misunderstandings I had about medical information”. The plain language summaries were uploaded on NECA’s official website.

22.4 Discussion

Engaging the public within HTA in tackling misinformation offers a unique opportunity to directly incorporate health needs and perspectives of people. This approach, aligning with the World Health Assembly resolution on social participation for universal health coverage, health and well-being, provides an effective mechanism for promoting health by reflecting the priorities of the public (including patients and carers) and relevant feedback through their active engagement (World Health Assembly 2024). PPG members provide invaluable insights into the real-world health needs and challenges of the public. By reviewing their submissions, a comprehensive understanding of these issues was possible, leading to the development of more targeted interventions. Integrating health information monitoring with HTA enhances the ability to reach a wider audience, promoting rational decision making and a more responsive healthcare system.

Patient participation in HTA offers a promising path forward, but uncertainties remain regarding its depth and most effective role. Limited training infrastructure presents a hurdle, yet this initiative demonstrates the power of successful PPG engagement in HTA. It empowers patients to make informed decisions about safe and effective treatments, fostering a collaborative approach to healthcare governance that transcends traditional expert-driven models.

However, to ensure the programme's long-term success, key challenges need to be addressed. Developing a robust and systematic training programme is essential to equip PPG members with the necessary expertise to meaningfully participate in HTA. Additionally, dedicating staff and budgetary resources is crucial for smooth programme operation and long-term viability.

Overcoming these challenges and refining the PPG model is crucial for paving the way for even deeper patient involvement into HTA processes. This could include exploring innovative training methods, leveraging technology to facilitate collaboration, and developing metrics to measure the impact of patient participation on HTA outcomes. Ultimately, a future where patients are active partners in shaping healthcare decisions can be achieved through continued investment and strategic development of the PPG programme.

22.5 Conclusion

The PPG's approach to HTA goes beyond simply educating and enabling patients' participation. By equipping members with social media monitoring skills to identify content lacking strong scientific evidence, NECA integrates HTA directly into people's lives, empowering them to navigate the ever-growing health information landscape. Furthermore, plain language summaries make the HTA findings easily comprehensible and communicable to the public. This approach empowers patients, traditionally positioned solely as recipients of healthcare research, to take a more

proactive role. By actively participating in HTA, patients can make informed healthcare decisions and protect themselves from unsubstantiated healthcare services.

With continued investment in education and active participation, NECA empowers the PPG's potential to evolve into a body of respected opinion leaders within the healthcare sector. As PPG member expertise expands, HTA results that incorporate patient preferences and social values will be increasingly used to shape a broader spectrum of healthcare decision-making processes.

This patient-centered approach is not just empowering; it is laying the groundwork for a future where patients are active partners in shaping healthcare research, decision-making, and implementation.

Imagine a future where HTA results routinely incorporate patient preferences and social values, such as considering treatment side effects that may significantly impact quality of life. Through continued investment in education and fostering active participation, NECA is paving the way for this future, where HTA becomes a powerful tool for optimising healthcare delivery and empowering patients to make informed choices, ultimately leading to better healthcare outcomes for all.

Acknowledgements This project marks a novel attempt to establish a systematic approach to identifying HTA topics by incorporating social media monitoring, promoting patient participation and addressing misinformation related to health technology. While this effort constitutes a valuable step toward advancing patient-centered and socially responsive HTA practices, it would not have been possible to achieve meaningful outcomes without the invaluable support and contributions of many individuals throughout its development.

We would like to express our sincere gratitude to Dr. Ji Eun Choi and Dr. Sangjin Shin, whose continued support and keen interest were instrumental to the successful realisation of this project. We are also profoundly grateful to Dr. Yunjung Kim and Dr. Jae Kyung Suh for their rigorous research on the selected HTA topics, which contributed significantly to the depth and impact of the project's findings.

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Chapter 23

Singapore: From Stakeholder Mapping to Continuously Improving Patient Participation in HTA



Fiona Pearce, Ping-Tee Tan, and Josie Liow

23.1 Introduction

In Singapore, national health technology assessment (HTA) efforts are focused on informing healthcare funding decisions and improving patient access to cost-effective treatments. The Agency for Care Effectiveness (ACE) was established in 2015 to conduct HTAs for medicines, vaccines, gene therapies, and medical devices; negotiate prices for health technologies that are commensurate with their value; and support Ministry of Health (MOH) advisory committees in making evidence-based funding recommendations to drive appropriate care. Standardised HTA methods and processes have been developed in line with international best practices, adapted to Singapore's context, and updated periodically to ensure that they remain relevant (ACE 2023, 2024a).

This chapter describes how patient participation processes were co-developed with local patient organisations in 2022 and embedded into the HTA framework to improve the applicability of ACE's work to patients in Singapore. It also highlights the challenges of patient participation in a setting where patient organisations have limited experience influencing healthcare policy decision making. The resources ACE provides to support patient participation, including dedicated staff, a patient participation process and methods guide (ACE 2024b), and various plain language educational tools are also described.

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At ACE, the term ‘patient input’ describes input received from patients, their carers, representatives from patient organisations, and members of the public who use healthcare resources. Collectively, they are referred to as ‘healthcare consumers’ in the local context; however, for the purpose of this chapter, the term ‘patients’ will be used to align with the terminology used in the rest of this book. The terms ‘patient participation’ and ‘patient involvement’ are both used in Singapore to describe opportunities for patients to take part in ACE’s work; however, to align with the definitions in this book, the term ‘patient participation’ will be used in this chapter. The terms ‘patient organisation’ and ‘patient group’ are used interchangeably in Singapore, however, the latter typically refers to an informal network, alliance or online disease-specific community rather than a legally registered group which operates at a national or regional level.

23.1.1 Healthcare Financing and HTA

Singapore’s healthcare financing system is built on a philosophy of shared responsibility among individuals and families, insurers, and the government. It comprises government subsidies, compulsory individual healthcare savings accounts (MediSave), risk-pooling via both voluntary private and mandatory government health insurance plans (MediShield Life), out-of-pocket contributions from patients, and a government endowment fund (MediFund) that acts as a safety net for the needy (Pearce et al. 2019).

In the public healthcare sector, subsidies and financial assistance are provided to eligible patients for medicines (drugs) and medical technologies that have been recommended by the Drug Advisory Committee (DAC) or Medical Technology Advisory Committee (MTAC). Both committees are chaired by the MOH Director-General of Health with members comprising clinicians, pharmacists, and experts in regulatory affairs, healthcare finance, and healthcare services. Patients or carers are not currently represented in the committees.

Funding decisions made by DAC and MTAC are informed by ACE’s HTA reports which assess the unmet clinical need of patients; the clinical effectiveness and cost effectiveness of health technologies compared to existing standards of care; and the budget impact associated with funding the health technologies. MTAC also consider organisational feasibility to take account of the potential impact a health technology could have on the health system when used in clinical practice. Both committees may also deliberate other factors such as social, cultural, and ethical issues when making funding recommendations (ACE 2023, 2024a).

23.2 Supporting Patient Participation Initiatives at ACE

While stakeholder participation is an integral component throughout the HTA process, prior to 2022, only clinicians were consulted to ensure that ACE's HTAs reflected local clinical practice and addressed patients' needs.

Today, ACE is committed to understanding the priorities and preferences of patients and their carers to improve the quality of its work and ensure that it remains relevant for the people most affected by it. In 2021, a dedicated workstream, Consumer Engagement and Education (CEE), was established to encourage patient participation across ACE's work programmes. CEE helps identify and support patients, carers and patient organisations contribute testimonies and lived experiences to inform ACE's HTAs. The team prepares a survey for each HTA topic to gather patients' and carers' inputs, and collates all responses for consideration by MOH advisory committees. They also co-develop plain language materials with patient organisations to facilitate patient participation throughout the HTA process. Factsheets about different medical conditions and healthcare policies are also developed at the conclusion of select HTAs to improve health literacy, and equip patients with information to make shared decisions with their clinicians about their healthcare needs.

23.2.1 Stakeholder Mapping

One of the key challenges when first establishing patient participation processes in Singapore was to identify local patient organisations and relevant volunteer groups that could contribute to ACE's work. In the absence of a centralised database, a comprehensive stakeholder mapping exercise was undertaken in 2021 by searching the Singapore Charity Portal,¹ hospital websites, Google search engine, and social media platforms. Identified groups were screened in line with specific selection criteria (Box 23.1) and contacted via phone and email to discuss their remit and interest in participating in HTAs (Tan et al. 2023).

Box 23.1 Patient Organisation Selection Criteria

Organisations or informal groups based in Singapore representing one or more medical conditions were included. The following organisations were excluded because their activities are not directly related to ACE's remit:

- Activity centres for the elderly
- Schools for children or adults with special needs
- Traditional Chinese medicine societies

(continued)

¹ www.charities.gov.sg

Box 23.1 (continued)

- Academic or research groups
- Referral networks that link patients to relevant charities
- Fundraising and distribution charities
- Humanitarian aid or disaster relief organisations
- Organisations that solely provide legal assistance, transportation services, befriending or hotline services for patients
- Dormant organisations that could not be contacted.

One hundred and six patient organisations and groups were identified, covering 20 conditions, with membership ranging from 8 to 3500 patients and carers. Forty eight percent of the patient organisations were registered charities, while the remaining were either patient groups affiliated with public hospitals (39%) or informal groups founded by local patient advocates (13%). Most had not heard of ACE, and were not familiar with the role of HTA in healthcare decision making.

23.2.2 *ACE Consumer Panel*

In April 2022, a Consumer Panel comprising members² from local patient or voluntary organisations across a broad range of conditions was established to represent the collective voice of patients and carers in Singapore (Teo et al. 2024). It is the first long-standing engagement of patients by MOH and serves as an example to other government divisions on how to meaningfully involve patients at the organisational level. Members provide strategic advice to ACE and MOH advisory committees on priority setting, and propose opportunities to foster collaborative working relationships between ACE and patient organisations, and overcome local barriers to patient participation. They also play an integral role in championing ACE's work within their local networks, and provide advice on appropriate communication strategies to meet patients' information needs.

Although members are selected to represent their specific organisations or health conditions, they are also expected to provide their own personal views based on their experience engaging with the Singapore healthcare system. Members are appointed for a 2-year term, with an opportunity to serve additional terms.

²Consumer Panel membership is available at www.ace-hta.gov.sg/about-us/our-council-and-expert-panels.

23.3 Co-development of Patient Participation Processes

In 2022, ACE co-developed processes with local patient organisations to encourage participation in three key areas:

1. **Topic selection:** Suggesting which health technologies (topics) should be evaluated by ACE for funding consideration (Koh et al. 2024)
2. **Patient input:** Providing lived experiences about medical conditions, treatments, and unmet needs to inform ACE's HTAs and funding recommendations made by the MOH advisory committees for different health technologies
3. **Educational resources:** Providing feedback on resources co-developed by CEE, patient organisations and clinicians about different medical conditions, health technologies, and healthcare policies.

Draft processes were determined by conducting a literature review of well-established patient participation frameworks from overseas HTA agencies and scientific communities, including the International Network of Agencies for Health Technology Assessment (INAHTA 2021) and HTA International Patient and Citizen Involvement in HTA Interest Group (HTAi 2014) to identify best practices. The processes were then discussed with the ACE Consumer Panel and select patient organisations to help contextualise them to meet local patients' preferences and identify any barriers to implementation. Final processes were described in a process and methods guide (ACE 2024b) and in a series of factsheets and infographics which highlight key steps in plain language.³ Continuous process improvements in response to feedback from patients, and providing regular advice to patient organisations on how their inputs have been used to inform HTAs and funding decisions, have increased patient participation in ACE's work and improved the quality of inputs received.

23.4 Barriers and Enablers to Patient Participation

Patient participation in HTA was largely unknown to patient organisations when the CEE workstream was first established. There was uncertainty about how to do it well, in a way that constituted genuine partnerships and involved a broad range of patients. Perceived barriers were identified with the ACE Consumer Panel, and solutions were proposed to overcome them and ensure any approaches adopted were inclusive, patient-centred, and not tokenistic (Table 23.1). Initial patient participation processes had flexible approaches, so they could be piloted for select HTAs, then fine-tuned based on experience, to meet patients' needs.

³All factsheets about patient participation in HTA are available at <https://www.ace-hta.gov.sg/Patients-And-Community/opportunities-for-patient-involvement>.

Table 23.1 Initial barriers to patient participation and solutions implemented to overcome them

Initial barriers	Reasons	Solutions implemented
Limited awareness about HTA and the importance of patient participation	– Limited understanding by all stakeholders (payers, clinicians and patient organisations) of the value of patient participation in shaping healthcare policies and decisions – Lack of awareness among patients about HTA and their potential role in contributing evidence to inform assessments – Patient participation is perceived as tokenistic – Insufficient education and resources dedicated to informing patients about HTA and opportunities for participation	– Built trusting, productive relationships between ACE and patient organisations – Established ACE consumer panel to champion HTA and build awareness and trust of ACE's work and opportunities for patient participation – Patient organisations that have not been involved in ACE's work before are contacted by CEE to explain the role of ACE and how patients can participate in HTAs, and determine what level of support is needed – Plain English summaries of HTA guidance to explain funding decisions, and the information that informed the recommendations – Targeted educational resources and training to raise awareness about HTA and the important role of patients in the process – Established formal mechanisms for meaningful patient participation in HTAs for drugs, gene therapies and wearable or home-based medical devices – Allocated resources for capacity building among patient organisations
Cultural and linguistic diversity	– Singapore's multicultural society presents challenges in effectively engaging patients from diverse cultural backgrounds – Language barriers can hinder effective communication and participation, particularly among non-English-speaking populations – Cultural differences in healthcare beliefs and practices may impact the willingness of patients to engage in HTA processes	– Tailored communication strategies to address cultural and linguistic barriers – Patient surveys available in English and Chinese – Religious organisations help to reach minority patient groups – Carers or family members can provide evidence on behalf of patients

(continued)

Table 23.1 (continued)

Initial barriers	Reasons	Solutions implemented
Power dynamics and institutional barriers	– Hierarchical structures within healthcare institutions may marginalise patients' voices in decision-making processes – Lack of formal mechanisms for meaningful patient participation in healthcare decision making outside of ACE – Limited representation of patients' perspectives in decision-making committees, with dominance of medical professionals and policymakers	– Patients' lived experiences are recognised by ACE and its committees as an equally important source of information alongside clinical and economic evidence – Training for internal and external stakeholders on the value of patient input
Capacity and resources	– Patient organisations have limited capacity for patient participation within HTA framework – Meaningful patient participation requires time for ACE to develop collaborative relationships with patient organisations and ongoing effective communication and support – Lack of HTA training developed specifically for patients – Insufficient support for patients to effectively participate in HTA processes – Lack of dedicated funding for patient organisations to engage in HTA activities and represent patients' interests	– ACE proactively approaches all relevant patient organisations early in the HTA process to give them sufficient time to capture patient input from their members – Targeted training and self-directed learning modules on HTA are available for patients to address information needs – Dedicated ACE staff support patient organisations when providing input and help them meet submission deadlines

(continued)

Table 23.1 (continued)

Initial barriers	Reasons	Solutions implemented
Accessibility and equity	– Socioeconomic factors may impact access to HTA information and opportunities for patient participation – Limited access to healthcare services and information among marginalised and vulnerable populations – Inequities in access to technology and digital platforms for engaging patients in HTA discussions and consultations	– Wider contact improved by using social media platforms – ACE works with clinicians to find patients to provide testimonials if a relevant patient organisation is not available. Seeking patient referrals through clinicians also helps identify informal patient groups set up by clinical care teams – Patient survey is available in different formats (email, online form, hard copy, sent via apps) to make it more accessible to a broader range of patients
Data protection and ethical challenges	– Concerns about data privacy and confidentiality may deter patients from participating in HTA activities – Managing conflicts of interest	– Survey responses are anonymous to protect the identity of patients – Financial conflicts of interest are captured for all stakeholders, including patients, who participate in the HTA process, however, if a conflict is noted, it does not preclude the testimonial from being considered

Using patient input to inform HTAs and decision making was also unfamiliar to the ACE technical teams and MOH advisory committee members. General sentiments suggested that they were skeptical whether it was sufficiently robust to consider alongside clinical trial data. Though many were aware of the use of patient input in overseas settings, a shift in mindset was needed to recognise its value locally. As the number of HTAs with patient input continues to increase, staff and committee members now appreciate the different, yet complementary, knowledge and insights that patient input offers compared to traditional clinical studies. Such information also ensures that the outcomes that matter most to patients are brought to the attention of committee members and inform decisions that are relevant to the people most affected by them.

23.5 Value of Patient Input

Since May 2022, patients have been able to provide input to inform HTAs for drugs. The scope of patient input was expanded in 2023 to include non-drug topics including gene therapies and wearable or home-based medical devices. At least one patient testimonial was received for 85% of HTAs in the first year, increasing to 89% in the

second year. The number of patient responses varied depending on the condition under evaluation, the capacity of patient organisations to collate information from their members, and the interest of patients to participate in the process (Tan and Pearce 2024). Patient input addressed uncertainties in the scientific evidence and helped decision-making committees understand how different conditions affect patients and their carers, the outcomes that matter most to patients, and the benefits and disadvantages of existing and new treatments (Box 23.2).

Box 23.2 Value of Patient Input

Patients and their carers have provided important evidence to inform ACE's HTAs and helped decision-makers:

- Understand how different medical conditions affect patients, their carers, and families
- Identify unmet needs and treatment preferences of patients
- Understand the benefits and disadvantages of different health technologies
- Understand patients' expectations for new treatments
- Identify health outcomes that are important to patients
- Determine if the outcomes measured in clinical trials and economic models are relevant to patients in Singapore
- Fill gaps or address uncertainties in the scientific evidence
- Identify if there are any issues affecting a patient's ability to use or access treatments.

Local patient organisations also value the opportunity to participate in the HTA process and consider it to be a meaningful and important activity for their members. Some patient organisations have even appointed dedicated staff and resources to coordinate patient input into HTAs.

The SingHealth Patient Advocacy Network (SPAN) has worked closely with ACE since 2021 to provide the patient's perspective in healthcare decision-making. To ensure dedicated resources for the regular engagements, an internal workgroup comprising patient advocates was formed to support the collaboration. It has been an enlightening experience sharing personal insights from our patient and caregiver journeys, and using our knowledge to provide feedback, with the aim of improving healthcare quality. The partnership has empowered patient advocates to take on active roles in shaping healthcare policies by offering their voices as users of healthcare. Involving the patient's voice in the review of disease-specific factsheets was also a significant move in enabling patients and families to make informed healthcare decisions, thereby improving health literacy. We are certainly in the right direction towards healthcare that is truly for patients, by patients.

—Josie Liow, Co-Chair SingHealth Patient Advocacy Network (SPAN)

23.6 Capacity Building and Future Directions

Patient input has improved the quality of ACE's HTAs by providing decision makers with a better understanding of patients' needs, preferences, lived experiences and expectations so that their funding recommendations are relevant to the people most affected by them. While patient participation in HTA is still in its nascent period in Singapore, more than half of the patient organisations identified during the stakeholder mapping exercise have already provided input into ACE's HTAs. This is testament to their trust in the process, and the efforts CEE has made to raise awareness of available patient participation opportunities and support patients who would like to be involved.

Future efforts to build on this initial success will focus on providing targeted HTA training to upskill patients through a series of free, self-directed learning modules. Topics covered in the first tranche of modules have been proposed by patient organisations based on the information needs of their members. Measuring the impact of patient input in HTAs will also be a key focus in coming years to improve the transparency of decision making, establish metrics to systematically quantify the value of patient input, and provide case studies that showcase the impact local patient organisations have had on healthcare decision making.

Acknowledgements All views expressed and any errors within this article are entirely the responsibility of the authors.

Funding There was no funding used for writing the manuscript or performing the underlying research.

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Chapter 24

Spain (RedETS): Collaborating on Methodologies for Patient Involvement in HTA



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24.1 Spanish National Healthcare System

The Spanish National Healthcare System (SNHS), known as the *Sistema Nacional de Salud*, is founded on core principles of universality, equitable access, financial solidarity, and free availability. Funded primarily through taxation, the SNHS extends healthcare services to almost all residents of Spain, ensuring nearly universal access to medical care regardless of individuals' economic or social standing. This extensive accessibility to healthcare has significantly contributed to an increase in life expectancy, which currently stands at 83.2 years—the highest within the European Union (Bernal-Delgado et al. 2018; OECD 2023).

The SNHS is characterised by its decentralised structure, with 17 regional entities known as autonomous communities overseeing the management and delivery of healthcare services within their respective regions. Despite this decentralised approach, the SNHS adheres to common national guidelines and standards

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established by the Ministry of Health. Key features of the SNHS encompass a wide array of services, ranging from primary care to specialised medical attention, and hospital services. Additionally, the SNHS places a significant emphasis on preventive care and public health initiatives aimed at promoting healthier lifestyles and preventing illnesses. Overall, the SNHS has a crucial role in safeguarding the health and well-being of the Spanish population, embodying the principles of equality, accessibility, and solidarity in healthcare provision (Bernal-Delgado et al. 2018).

In Spain, the legal framework promotes patient participation in the decision making related to the SNHS. The Spanish Constitution (España. *Constitución Española* 1978), the country's highest law, underscores this commitment in Article 9.2, mandating public authorities to foster citizen participation in the political sphere. Similarly, the General Health Law (España. *Ley 14/1986*, article 5 1986) establishes the right to citizen participation and commits Public Administrations to facilitate its implementation. According to the Law Regulating Patient Autonomy and Rights and Obligations regarding Clinical Information and Documentation (España. *Ley 41/2002*), healthcare services must guide users by specifying their rights and obligations, available services, characteristics of the healthcare centre or service, staffing, facilities, and technical resources, providing all users with information on participation guidelines, as well as suggestions and complaints mechanisms. Furthermore, Law 16/2003 on Cohesion and Quality of the National Health System establishes provisions for the coordination and collaboration among public health

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administrations to ensure citizens' right to health protection, emphasising equity, quality, and societal engagement (España. *Ley 16/2003*). This legislation specifically addresses citizen involvement in health affairs, emphasising the respect for individuals' autonomy in decision-making and the collective consideration of their expectations as users of the system, fostering the exchange of knowledge and experiences (Plataforma de Organizaciones de Pacientes 2022).

24.1.1 *Health Technology Assessment in Spain*

Health technology assessment (HTA) is a key component in Spain by continuously informing the update of the SNHS common services portfolio, as it supports decision making regarding the inclusion of technological advances that have demonstrated safety, efficacy, and efficiency, as well as decisions related to disinvestment or the appropriate use of health technologies (España. *Real Decreto 63/1995 1995*; España. *Ley 16/2003*; España. *Real Decreto 1030/2006*). The Ministry of Health coordinates the Spanish Network of Agencies for Assessing National Health System Technologies and Performance (RedETS, *Red Española de Agencias de Evaluación de Tecnologías Sanitarias y Prestaciones del Sistema Nacional de Salud*), which, since its creation, has aimed to provide evidence-based information to enhance the equity, cohesion, and sustainability of the healthcare system (España. *Real Decreto 852/2021*). The primary objective of RedETS is to inform national decisions regarding the integration and utilisation of non-pharmaceutical health technologies, with a particular focus on medical devices and procedures, to promote an efficient and high-quality healthcare system. Additionally, RedETS seeks to foster a culture of evaluation to support its effective incorporation into healthcare management and daily clinical practice within the SNHS, while efficiently addressing evaluation needs through network cooperation (Varela Lema et al. 2018).

RedETS consists of two General Directorates from the Ministry of Health and eight HTA agencies, which are diverse in their functional and administrative structures, trajectories and resources. Despite this diversity, the Network operates cohesively with standardised methodologies that ensure a common quality framework for HTA processes. As part of this effort, RedETS develops methodological documents and procedures to guide the preparation of HTA reports.

The *RedETS Strategic Plan 2022–2025* outlines the network's strategic direction in addressing the challenge of becoming a benchmark for providing comprehensive responses to evaluation needs related to population health through the promotion and cooperation in the assessment of technologies at both national and international levels (España. *Real Decreto 852/2021*). The plan was developed in the context of the COVID-19 pandemic, which reshaped governmental and healthcare priorities (España. *Real Decreto-ley 16/2012*). Additionally, it coincides with rapid advances in health innovation, significant growth in digital technologies, and the transformative potential of artificial intelligence, machine learning, and robotics. The plan also aims to align RedETS' activities with the new framework for permanent and

sustainable cooperation in HTA at the European level, as outlined in Regulation (EU) 2021/2282.

The strategic plan revolves around four axes designed to respond to the scenario outlined: (1) Governance; (2) Coverage of SNHS needs; (3) Participation; and (4) Transparency and communication (España. *Real Decreto* 852/2021 [2021](#)). In the interest of transparency and stakeholder engagement, the strategic plan was made available for public consultation, allowing interested parties to contribute their perspectives (España. *Real Decreto* 852/2021 [2021](#)).

24.2 Current Structures for Patient Involvement in RedETS

RedETS has unevenly involved patients in its evidence-based outputs since the official constitution of the network in 2012, not only in HTAs but also in Clinical Practice Guidelines (Rodríguez and Espallargues [2014](#); Grupo de trabajo de implicación de pacientes en el desarrollo de Guías de Práctica Clínica [2013](#)). In HTA, noteworthy initiatives include the development of recommendations to tailor report content for better patient accessibility and readability (Hermosilla-Gago and Grupo de Expertos de las Agencias de Evaluación de Tecnologías Sanitarias de España [2011](#)), as well as the analysis of the first patient engagement experiences (Rodríguez and Espallargues [2014](#)).

However, in 2017, RedETS published an official public statement supporting patient participation in the HTA process, recognising the need and value of active participation and collaboration of patients, caregivers, and users in improving health technology-related decisions within the SNHS. The statement highlighted the unique knowledge, perspectives, and experiences that patients contribute, which can provide essential insights into the HTA process. It also emphasised that understanding and leveraging this knowledge allows for more precise alignment with patient needs while enhancing sustainability, transparency, accountability, and democratisation of the decision-making process (RedETS [2017](#)). This public statement marked the starting point for establishing the current structures for patient involvement within the network, with the *Patient Involvement Strategy* and the Patient Involvement Interest Group at its core.

24.2.1 RedETS Patient Involvement Strategy

RedETS Patient Involvement Strategy aimed to define actions that would enable its agencies and units to incorporate patient participation and overcome existing barriers. It was developed iteratively, beginning with a synthesis of the literature, followed by a qualitative study and a Delphi consultation with national stakeholders. The key findings and conclusions from these three processes contributed to

shaping a proposal for patient involvement in HTA within the context of RedETS activities, considering its specific needs and resources. The proposal was submitted to the members of the RedETS Governing Council, which consists of a Presidency, Vice-Presidency, Technical Secretariat from the Ministry of Health and the heads of the evaluation agencies or units. The Council is responsible for managing and coordinating the network, establishing common working procedures, and providing a methodological framework. Following an online discussion, minor changes were made to finalise the document (Toledo-Chávarri et al. 2016, 2019).

The strategy was implemented between 2017 and 2024 in three phases: (1) Piloting patient participation in HTA and enhancing patient capacity; (2) Broadening the participation of patients, and strengthening internal capacity through medium-term actions, and 3) Consolidating and mainstreaming patient involvement on a long-term basis. The main actions of RedETS’ *Patient Involvement Strategy* are outlined in Table 24.1.

Table 24.1 *Patient Involvement Strategy* in RedETS: short, medium, and long-term actions (Table 4, Toledo-Chávarri et al. 2019)

Actions proposed to be carried out in the short term (2017)	
Piloting patient participation in HTA and building patients’ capacity	1. Pilot patient participation in HTA reports by incorporating 2 or 3 patient representatives into the Development Group 2. Create and disseminate materials to inform and train patients about HTA and how to contribute to their different phases 3. Develop templates and forms to facilitate patient participation (e.g. conflict of interest declaration) 4. Include patient representatives in the review and development of the patient version and dissemination of the HTA report
Actions proposed to be carried out in the medium term (2018–2019)	
Broadening the participation of patients, and building internal capacity	1. Train methodologists in significant and effective patient involvement methods 2. Incorporate considerations for social and patient-related aspects into all HTA reports through literature review and engagement with expert patients and their organisations 3. Actively disseminate calls for public consultation of HTA reports to organisations affected by the assessed technology
Actions proposed to be carried out in the long term (2021–2024)	
Consolidate and mainstream patient involvement	1. Integrate patient participation into all HTA reports 2. Expand patient participation using a combination of methods to cover all possible contributions 3. Establish mechanisms to incorporate patient contributions and document them transparently 4. Ensure representativeness and diversity in the composition and number of participants in panels and consultations 5. Evaluate the impact of patients’ contributions 6. Conduct annual information and training sessions for patients and representatives

24.2.2 *RedETS Patient Involvement Interest Group*

The establishment of the Patient Involvement Interest Group in December 2017, during the RedETS Annual Conference, was a key initiative to support the implementation of the RedETS' Patient Involvement Strategy. Endorsed by the Spanish Ministry of Health and the RedETS Governing Council, the group includes at least one representative from each of the eight agencies and units within the network. Currently, it comprises 30 members from all HTA agencies, bringing together professionals from diverse disciplines such as psychology, sociology, anthropology, nursing, pharmacy, biology and medicine. The experience and capacities for patient involvement vary across HTA bodies, reflecting the unique expertise within each agency.

The group holds online plenary meetings every 2–3 months, complemented by an annual face-to-face meeting, typically held alongside the RedETS' Annual Conference. Additionally, sub-working groups meet independently according to their project timelines. The group's main priorities are collectively defined in coordination with the RedETS Governing Council and the Ministry of Health.

The group's core activities focus on training, collaborative learning, and coordinated actions across the network. In terms of training, workshops, working sessions, experience-sharing initiatives, and consultations are organised to enhance technical capacities among HTA researchers, fostering the integration of patient involvement in HTA processes. For example, RedETS has launched webinars and workshops focused on systematic reviews of qualitative and mixed-method studies. Regarding collaborative work, the group aims to develop tools, methodologies, and procedures that facilitate patient participation and promote their active participation in specific projects. Notable past initiatives included the development of a methodological manual on patient involvement in HTA and the creation of a decision flowchart to ensure meaningful patient participation in HTA processes (Toledo-Chávarri et al. 2020a). The most recently developed methodological document focused on evaluating patient involvement in Health Technology Assessment reports, underscoring the group's commitment to continuous improvement and advancing research in this area.

The group's inaugural project involved assessing various patient participation experiences from the perspective of researchers, culminating in a published study (Toledo-Chávarri et al. 2020b). For this analysis, each agency selected a case study, completed a structured questionnaire, and shared their findings for discussion within the Interest Group. As outlined in Table 24.2, the study highlighted that while patient involvement has a significant impact on HTA reports, challenges persist, particularly in recruitment and capacity building.

Table 24.2 Characteristics of patient involvement processes evaluated (Toledo-Chávarri et al. 2020b)

HTA agency/unit: report first author and year	Product type (assessed technology)	Main objective of patient involvement	Design	Patient characteristics <i>n</i>	Phase (s)
Avalia-t: Triñanes Pego (2018)	HTA therapeutic identification of depression in young people program	To include patient perspective about depression and the program	Direct involvement in working group; focus groups (one with adolescents, one with families)	One father in protocol review; five adolescents; and five parents in focus groups	Protocol review; assessment
UETS: Jurado López (2017)	HTA TAVI	To know how patients can participate in personalised decision to use TAVI in cases of severe aortic stenosis when valve replacement is needed	Semi-structured telephone individual interviews	24 patients with TAVI and family members	Assessment
Osteba: Bayón (2018)	HTA nipple-areola complex micropigmentation	To include the perspective of women affected with breast cancer about the nipple areola complex micropigmentation	One interview and two focus groups	Nine breast cancer affected women	Assessment
SESCS: Brito García (2017), del Pino Sedeño (2017), Vallejo Torres (2017)	HTA platelet-rich plasma (PRP), early detection of disease-related malnutrition and pneumococcal vaccines	To understand the perspectives, experiences and preferred outcome measures for patients regarding assessed technologies	Direct involvement in working group; survey; literature review	Patient organisations invited but did not participate in protocol and draft report review. 10 people with chronic wounds (survey)	Protocol and draft review, assessment; outcome selection
SESCS: WGEDCD 2018	Evidence-based protocol celiac disease early diagnosis	To include patient perspectives regarding celiac disease diagnosis	Direct involvement in working group; survey	Representatives from three patient organisations and 4048 patients (survey)	Protocol and draft review; assessment

(continued)

Table 24.2 (continued)

HTA agency/unit: report first author and year	Product type (assessed technology)	Main objective of patient involvement	Design	Patient characteristics <i>n</i>	Phase (s)
AETSA Baños Álvarez (2018)	HTA leadless pacemaker	To know which were the most important outcomes for patients with leadless pacemakers	Semi-structured interviews and a panel vote for preferred outcome measures	Five patients with cardiopathy or associated problems, noncandidates for leadless pacemaker implantation	Outcome selection
AQuAS; Puigdomènech-Puig (2021)	Methodological Report methods for assessment of health apps and other mHealth solutions	To explore the perspective and preferences regarding assessment of health apps and other mHealth solutions	Focus group	Nine app users	Assessment

AETSA Agency for Evaluation of Healthcare Technologies in Andalusia; AQuAS Agency for Healthcare Quality and Assessment of Catalonia; Avalia-t
Scientific-Technical Advice Unit, Galician Agency for Health Knowledge Management; WGEDCD Working Group for the Early Diagnosis of Celiac Disease;
Osteba, Basque Office for Health Technology Assessment; SESCS Evaluation Unit of the Canary Islands Health Service; TAVI transcatheter aortic-valve
implantation; UETS Healthcare Technologies Evaluation Unit of Madrid

24.2.3 *Training for Patients*

RedETS has also invested in development of training for patients to increase their participation in HTA. The primary goal of the training initiatives has been to empower patients and citizens to participate meaningfully in HTA processes. This effort involved the creation of educational materials, their implementation as a face-to-face course, and the virtualisation of content into an online Moodle course (<https://moodle.org>).

To achieve this, a multidisciplinary working group comprising researchers from RedETS agencies and units developed a document that provides information about HTA and patient involvement (Vicente Edo et al. 2020). This training material was accompanied by the additional publication of summarised versions of its seven chapters, which have been translated into co-official languages as Catalan, Basque, and Galician (Vicente Edo et al. 2021a, b, c).

These training materials served as the foundation for the face-to-face course, and they were virtualised in November 2020. In November 2021, the online course was officially launched on Moodle, offering concise yet comprehensive information about HTA processes and the role of patients. The course covers key topics such as HTA procedures, regulatory frameworks, essential health research concepts, and the importance of both qualitative and quantitative methods. Additionally, practical guidance is provided to equip patients and citizens with the necessary knowledge to effectively contribute to HTA activities. The pilot phase of the online training course took place during the first quarter of 2022, with ongoing evaluation of its effectiveness within the RedETS framework. The course is now available to patients involved in RedETS reports through the websites of various RedETS agencies and units, ensuring wide accessibility and continued patient empowerment in the HTA process.

24.2.4 *International Training for HTA Professionals*

The HTA Unit of the Community of Madrid (UETS), in coordination with the Colombian Institute of Technology Evaluation (IETS) (see Chap. 20), the Institute of Health Sciences of Aragon (IACS), and the Canary Islands Institute for Technology Evaluation (SESCS), organised a comprehensive training program aimed at equipping HTA professionals from Latin American agencies with the necessary skills to incorporate patient involvement in HTA processes. This initiative was conducted with the support of the RedETS Patient Involvement Interest Group and formed part of the Plan for Transfer, Exchange, and Management of Knowledge for the Development of Spanish Cooperation in Latin America and the Caribbean.

By facilitating this knowledge-sharing initiative, RedETS and its partners reinforced their commitment to strengthening international collaboration and promoting patient-centred approaches across diverse healthcare systems, ultimately contributing to the advancement of HTA practices in Latin America and the Caribbean.

24.3 Spotlight on Selected Aspects of Patient Involvement in RedETS

24.3.1 Networked Promotion of Patient Involvement

As a network, RedETS has the capacity to leverage both top-down and bottom-up approaches in the development and execution of its patient involvement strategy. From a top-down perspective, RedETS establishes general guidelines, policies, and strategic objectives that provide a common framework for all member agencies and units. These guidelines can be crucial in ensuring coherence and alignment across the network, fostering consistency in the actions and initiatives undertaken. On the other hand, from the bottom-up approach, RedETS can encourage active participation of its members in identifying needs, generating ideas, and implementing targeted actions. This allows for greater flexibility and adaptation to the specific conditions and contexts of each participating entity, while also enhancing the legitimacy and acceptance of the strategies and decisions adopted. Being part of a collaborative network offers the advantage of fostering strong connections among stakeholders involved in HTA. This structure facilitates collaboration and exchange of knowledge, experiences, and best practices among different actors, enriching the assessment process and promoting more informed and robust decision making. It also aims to increase transparency regarding stakeholders and the HTA, because the resulting process and contributions will be included in the HTA protocol and in the final report. Furthermore, as a network, RedETS can act as a meeting point and communication platform to facilitate the coordination of efforts and consolidation of resources for the benefit of all participants.

By joining forces and resources, RedETS can amplify the impact and influence of its activities and decisions in the field of HTA. Collaboration can enable its members to address their tasks more effectively, harness economies of scale, and achieve results that would be difficult to attain individually. Additionally, operating as a network enhances RedETS' visibility and credibility at both national and international levels, reinforcing its role as a key player in the HTA landscape.

24.3.2 Patient Involvement Methods

RedETS developed and implemented a methodology for patient involvement in HTA in the form of an algorithm. This methodology, created collaboratively by the Patient Involvement Interest Group alongside patient organisations, integrates direct participation with the generation and utilisation of patient-based evidence (Toledo Chávarri et al. 2021). Patient-based evidence, understood as knowledge derived from patients' attitudes, experiences, perspectives, perceptions, needs, or preferences regarding their care and health, can be gathered through qualitative,

quantitative, or mixed-method approaches, depending on the scope and objectives of each assessment (Chap. 4).

Published in 2021, this methodology became an integral part of the standardised framework that all RedETS agencies must adhere to. Each HTA research protocol must outline the patient involvement strategy guided by the algorithm. Moreover, the methodology and results of patient participation must be documented in the final report to ensure transparency and accountability.

The algorithm (Fig. 24.1) begins by indicating that patients should be invited to participate in all HTA. First contact with patient representatives by the RedETS Secretariat is made through the dissemination of the annual work plan to the largest patient umbrella organisations with an open invitation to participate and to promote involvement among their partner organisations.

According to the algorithm, the initial step to incorporating patient participation in each HTA is to identify patient organisations and/or expert patients who are part of the population affected by the implementation of the technology under evaluation. Expert patients are individuals with experiential knowledge about their health condition, either from their own experiences of illness or from the collection of collective experiences. Several sources of identification and recruitment of participants are proposed, including specific patient organisations, advisory councils, patient parliaments, and healthcare professionals. Which patients or expert patients are suitable for participation in a specific HTA will primarily depend on the scope and population under consideration. These patients receive a formal invitation to get involved in an HTA and are asked to sign an informed consent, a confidentiality agreement and a conflict-of-interest declaration to ensure ethical and responsible participation.

The algorithm also specifies the stages at which patients can contribute to the HTA process. Patient representatives or expert patients may be involved in all phases of an HTA as part of expert panels or working groups. However, a minimum level of participation is required in at least three critical stages of the process:

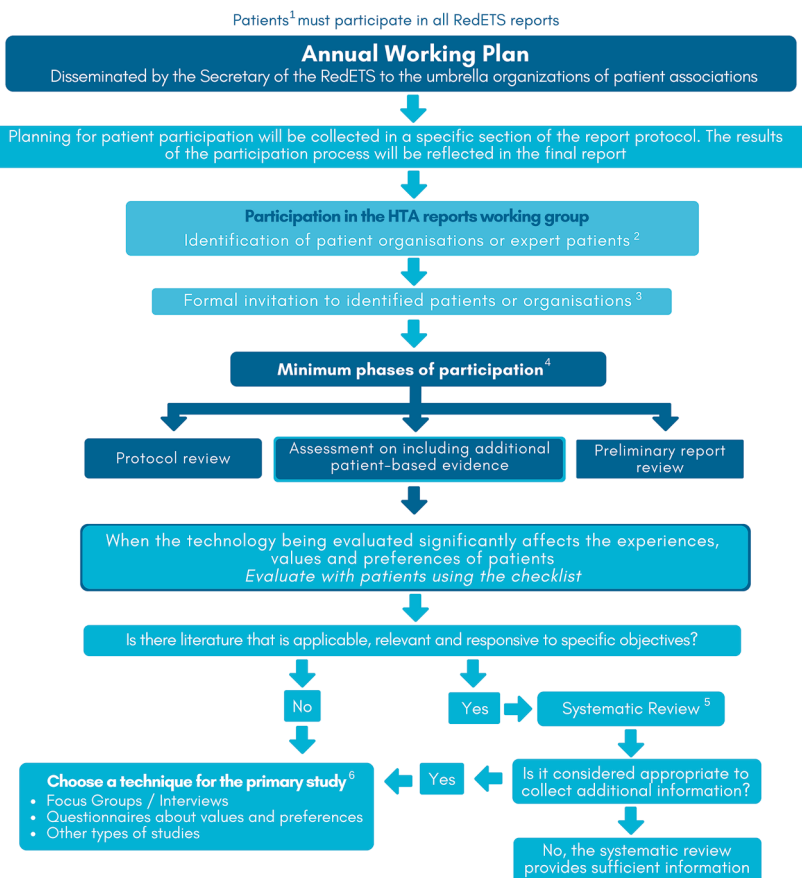
1. **Review of the HTA research protocol**—patients provide input on the study design to ensure their perspectives and concerns are considered from the outset
2. **Assessment of patient-based evidence inclusion**—patients contribute to evaluating the relevance and inclusion of additional evidence reflecting their experiences and insights
3. **Review of the preliminary report before publication**—patients help ensure that the final report accurately reflects their views and concerns prior to dissemination.

By integrating patient participation into these key phases, RedETS ensures that patients' voices are heard and valued throughout the HTA process, ultimately contributing to more patient-centered healthcare decisions.

In the development of the research protocol, patient representatives or expert patients can contribute in various ways. Patients can propose outcome measures that are meaningful from their perspective and define their relative importance in the decision-making process. Additionally, they can help identify affected populations

Flowchart to inform patient involvement in HTA reports of the RedETS

Guide for decision-making on patient participation in Health Technology Assessment (HTA) reports produced within the framework of the Spanish Network of Health Technology Assessment and National Health System Benefits Agencies (RedETS)



1. The term "patients" is used inclusively, encompassing individuals with a health condition, representatives from patient organizations, users of the healthcare system, caregivers, or members of the general public. Examples can be found in: Participación de los pacientes en la Evaluación de Tecnologías Sanitarias: manual metodológico

2. Expert patients are considered to be those who possess experiential knowledge about their health condition, either from their own experience with the disease or from gathering collective experiences

3. It is desirable to involve at least 2-3 patients per Health Technology Assessment (HTA) report. Several possible recruitment methods have been identified, including umbrella or specific patient organizations, advisory councils, patient parliaments, healthcare professionals, etc. For reports evaluating technologies without implications for the experiences, values, and preferences of individuals with specific health conditions or diseases, umbrella organizations may be invited to participate.

4. If they accept the invitation to participate, patients can add and prioritize the outcome measures of interest to them, identify the affected population or subgroups, share their experiences, values, and preferences regarding the pathology and/or evaluated technology, assess the impact of the technology in real-world contexts and its acceptability, and contribute to drafting or developing recommendations using GRADE[®] frameworks, among other contributions.

5. Reviews of qualitative or mixed-methods studies can be conducted to assess patients' experiences, values, and preferences. The GRADE-CERQual approach and methodological developments of Cochrane Qualitative & Implementation Methods Group can support the development of these reviews.

6. Approval from the Research Ethics Committee of the relevant agency/unit must be requested when required by the study entity.



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Fig. 24.1 Flowchart to inform patient involvement in HTA reports of the RedETS. (Source: Figure 1, Toledo Chávarri et al. 2021)

and specific subgroups that may experience distinct impacts from the technology under evaluation, among other contributions. During the evaluation process, patients provide valuable insights by sharing their experiences, values, and preferences related to their health condition and/or the assessed technology. They can assess the real-world impact and acceptability of the technology, even when no direct patient-based evidence is collected. The preliminary report review can be carried out by patients under the same conditions as other reviewers, ensuring their perspectives are considered in the finalisation of the report.

When the assessed technology significantly influences patient experiences, values, or preferences, a review or generation of evidence on these aspects is expected. Patients should actively participate, alongside the working group, in the decision-making process regarding the need for additional efforts to incorporate patient perspectives through the collection or production of patient-based evidence. If it is deemed necessary to include such evidence, the preferred methodological approach is to conduct a systematic literature review, provided that existing studies are available to address the specific research questions. Systematic reviews of qualitative or mixed-method studies—incorporating both qualitative and quantitative data—are generally useful in capturing diverse patient perspectives.

In cases where relevant literature is unavailable and conducting primary research is feasible, agency researchers may undertake primary studies to address specific objectives. These studies can explore patients' experiences, values, and preferences regarding their health conditions and the proposed technologies through methodologies such as focus groups, interviews, and surveys. If primary studies are conducted, approval from the respective Research Ethics Committee of the agency or unit is required, depending on institutional policies.

Patients can also participate in other phases of the HTA process, as outlined in the RedETS methodological manual (Toledo-Chávarri et al. 2016) and other international reference sources (Facey et al. 2017). For instance, in some RedETS agencies, patients contribute to the development of recommendations, where their involvement plays a crucial role in the interpretative process of translating evidence into actionable recommendations.

Examples of use cases of the algorithm are provided in Box 24.1.

Box 24.1 Use Cases

Case 24.1 Water birth

Women with prior experiences of water birth were consulted to identify outcome measures that reflected their values and preferences. These included autonomy during labour, pain relief, and overall satisfaction with the birthing process. Women highlighted barriers and facilitators to water immersion that were not initially considered, such as access to appropriate facilities and the availability of trained staff. This input was crucial in ensuring the research

(continued)

Box 24.1 (continued)

plan and protocol addressed the most relevant aspects from a patient-centred perspective (Reviriego Rodrigo et al. 2022).

Case 24.2 *Ethical, legal, organisational and social aspects of tele-neurology*

A focus group to define the scope of the assessment of the ethical, legal, organisational and social aspects of tele-neurology was conducted. Patient organisations among other stakeholders participated in the discussion and then reviewed the research questions to be included in a scoping review. Research questions focused primarily on aspects as warranting accessibility to tele-neurology care, considering patient perspectives and preferences or protecting privacy and confidentiality of consultations in the context of the Spanish healthcare systems (Torres Castaño et al. 2023).

24.3.3 *Evaluation of Patient Involvement*

The latest initiative that RedETS has embarked on is to establish a methodology for the evaluation of patient involvement (Toledo Chávarri et al. 2023). The evaluation has as its first objective the promotion of transparency and accountability. A secondary goal is to serve for the constant improvement, expansion and deepening of the participation of patients in RedETS. The initial step for the development of the evaluation methodology involved examining existing literature through both systematic and manual searches to identify precedents adaptable for use within RedETS. After careful consideration, the *Patient Engagement Monitoring and Evaluation Framework* emerged as the most suitable candidate for adaptation to RedETS's framework due to its versatility, currency, and adaptability (Vat et al. 2021).

With this robust evaluation methodology in place, RedETS aims to systematically enhance transparency, accountability, and ongoing improvement in patient participation. The evaluation strategy is structured around two key components:

1. **Annual Assessments:** Targeted questionnaires are administered to agencies, coordinators, and participating patients to gather valuable feedback and identify areas for enhancement
2. **Quinquennial In-Depth Case Studies:** Conducted every 5 years, these studies build on the insights from annual evaluations, providing a deeper analysis of patient involvement practices and informing strategic improvements.

This dual-approach evaluation framework is designed to ensure the sustained reinforcement, expansion, and integration of patient participation within RedETS, aligning with the network's commitment to continuous progress and stakeholder engagement. Currently, RedETS is developing technical procedures to implement the evaluation.

24.4 Discussion

Being part of a network like RedETS can have a significant impact on promoting collaboration, knowledge exchange, and amplification of activities related to patient involvement in HTA. In Spain, the highly decentralised healthcare system, with each autonomous community holding substantial responsibilities for healthcare delivery and policy making, results in a diverse and complex healthcare landscape. RedETS helps bridging these regional differences ensuring greater cohesion and consistency carrying out HTA with harmonised methodologies, including those related to patient involvement.

The methodology developed by RedETS for patient involvement serves as a standardised framework for equipping its members with the necessary guidance to ensure systematic and meaningful patient participation. Since its publication, the algorithm has been adopted as the official RedETS methodology for patient involvement. Its implementation is further strengthened through capacity-building activities led by the Patient Involvement Interest Group, ensuring that stakeholders are well-equipped to engage effectively. Additionally, RedETS' Secretariat at the Ministry of Health has integrated patient involvement as a core element of the official assessment protocol template, embedding it into routine HTA processes.

RedETS Patient Involvement Interest Group continues reflecting on how to improve patient involvement and addressing the barriers that are encountered along the way. The main challenges faced by group revolve around ensuring the sustainability of long-term activities and effectively disseminating the knowledge and projects developed across the entire RedETS network. Moving forward, the group aims to further strengthen capacity-building efforts, standardise patient involvement processes across projects, and continuously assess and refine participation strategies to enhance their impact and effectiveness. The group is currently working on addressing ethical challenges and exploring initiatives such as the establishment of a patient registration system and the creation of a dedicated patient forum.

Looking ahead, the implementation of the EU HTA Regulation (EU HTAR) is expected to significantly influence patient involvement practices in Spain (see Chap. 18). The regulation's emphasis on Joint Clinical Assessments and collaboration among member states presents an opportunity to align patient involvement methodologies at the European level, enhancing the quality, consistency and comparability of HTA outputs. However, the EU HTAR (European Union 2021) also poses significant challenges, particularly in ensuring meaningful participation within such a complex and diverse regulatory landscape. RedETS' extensive experience in fostering networked collaboration positions it well to contribute to and adapt within this evolving framework. The Patient Involvement Interest Group is actively working to promote non-tokenistic participation in European assessments, reflecting on how existing methodologies and procedures should be adapted or transformed to meet the demands of Joint Clinical Assessments. Furthermore, the group is closely monitoring which European assessments require adjustments to better fit the local Spanish context, considering factors such as access to healthcare

technologies and the unique values and preferences of Spanish patients. Strategies for incorporating this contextualisation and defining the role of patients in the process are also being explored.

RedETS' patient involvement methodology aims to enhance the sustainability, accountability, and democratisation of the HTA process. A key objective is to achieve a greater impact of patient participation on the scope and content of RedETS HTA reports, while also ensuring representation of the diverse patient population within the National Health System. Overcoming the challenges and barriers to effective patient participation remains a priority. To balance these goals with the fast-paced demands of HTA, RedETS has sought a feasible and pragmatic approach that integrates patient participation within tight timelines without compromising on openness, equity, and long-term capacity-building efforts. The recently developed evaluation framework for patient involvement will play a crucial role in assessing the extent and effectiveness of the methodology's implementation, providing valuable insights for continuous improvement.

In conclusion, RedETS' efforts to embed patient involvement in HTA represent a significant step towards democratising the process. By prioritising patient voices and systematically evaluating their participation, RedETS ensures the inclusion of diverse perspectives and strengthens the responsiveness, equity, and patient-centred focus of the healthcare system. Future efforts should continue to refine processes, embrace emerging methodologies and innovations, and further enhance patient participation to ensure patients' contributions have a tangible and meaningful impact on healthcare decision making.

Acknowledgements We would like to acknowledge the collective efforts of the RedETS' Patient Involvement Interest Group. Presently, the group comprises 30 individuals from 8 agencies and has been coordinated by Ana Toledo Chávarri working from the Servicio de Evaluación y Planificación del Servicio Canario de Salud (SESCS) between 2017 and 2024. The members and their respective agencies are as follows: Anabel Granja Domínguez, Carmen Martín Gómez, Máximo Molina Linde, Soledad Benot López, and Trinidad Sabalet Moya from Evaluación de Tecnologías Sanitarias de Andalucía (AETSA); María José Vicente Edo, Analfa Abt Sacks and Patricia Gavín Benavent from Instituto Aragonés de Ciencias de la Salud (IACS); Alezandra Torres Castaño, Andrea Duarte Díaz, Lilisbeth Perestelo Pérez, Vanesa Ramos García, and Yolanda Álvarez Pérez from SESC; Edurne Gallastegui Calvache, Maria-Dolors Estrada Sabadell, and Laura Llinàs Mallol from Agència de Qualitat i Avaluació Sanitàries de Catalunya (AQuAS); Begoña Rodríguez Ortiz de Salazar and Matilde Palma Ruiz from Agencia de Evaluación de Tecnologías Sanitarias del Instituto de Salud Carlos III (AETS-ISCH); Ainhoa Jausoro Zubiaga, Anaïtz Leunda Iñurritegui, Eva Reviriego Rodrigo, Josune Domínguez García, Marta López de Argumedo González de Durana, and Nora Ibargoyen Roteta from Servicio de Evaluación de Tecnologías Sanitarias-Fundación Vasca de Innovación e Investigación Sanitarias (BIOEF-OSTEBA); Paula Cantero Muñoz, Patricia Gómez Salgado, Lucinda Paz Valiñas and Yolanda Triñanes Pego from Agencia Gallega para la Gestión del Conocimiento en Salud. Unidad de Asesoramiento Científico-técnico (Avalia-t, ACIS); and Blanca Novella Arribas and Pilar Loeches Belinchón from Unidad de Evaluación de Tecnologías Sanitarias de la Comunidad de Madrid (UETS).

We sincerely thank Ann N. V. Single and Anke Peggy Holtorf for their valuable insights and constructive feedback.

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Chapter 25

Taiwan: Patient Participation in HTA



Li Ying Huang, Yi-Hua Kung, and Brenda Yen

25.1 Enhancing Deliberative Processes Within Taiwan's National Health Insurance Programme

Taiwan introduced its unified National Health Insurance (NHI) programme in 1995, gaining international recognition for its affordable premiums and low out-of-pocket costs, covering more than 99% of the population (Cheng 2015). Since 2007, Taiwan has conducted health technology assessments (HTAs) to assist the National Health Insurance Administration (NHIA) in shaping reimbursement policies for new drugs, medical devices, and health services. These assessments consider the comparative effectiveness, ethics, and cost-effectiveness within the financial framework of the NHI programme. The Division of HTA operates under the Center of Drug Evaluation (CDE). The enactment of the second-generation National Health Insurance Act in 2013 further integrated the HTA process into Taiwan's legislative framework, enhancing the transparency and inclusivity of the Pharmaceutical Benefits and Reimbursement Scheme (PBRs) Committee by allowing two patient representatives to participate in its appraisal meetings (Kao et al. 2019; Chen et al. 2021; Center for Drug Evaluation n.d.).

Building on this foundation, on December 27, 2023, the Ministry of Health and Welfare's NHIA celebrated the launch of the Center for Health Policy and Technology Assessment (CHPTA), which commenced operations on January 1, 2024. This marked a significant advancement in Taiwan's approach to HTA, further solidifying its commitment to enhancing its HTA framework (National Health Insurance Administration 2024a, b).

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K. M. Facey et al. (eds.), *Patient Involvement in Health Technology Assessment*,
Health Informatics, https://doi.org/10.1007/978-3-032-11284-2_25

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25.2 Current Structures for Patient Participation

To enable patients to participate in the decision-making processes regarding the inclusion of new drugs and new special materials in NHI coverage, the Patient Opinion Sharing Platform for New Drugs and New Medical Devices was launched in 2015. On October 1, 2021, the platform's functions were further enhanced, allowing patients to share their opinions on the use in the expanded indications. Patients are welcome to share their medication experiences and express their opinions so that the NHI Administration can make relevant decisions that meet their needs.

The online platform enables the submission of various types of information, including how the information was gathered, personal experiences with the condition and treatments, expectations from new treatments, impact on carers with or without new treatments, and other relevant comments. This input is collected at least 30 days before the PBRS Joint Committee is scheduled to discuss the application. The CDE/HTA team retrieves all opinions received via the platform, summarises them, and incorporates them into the HTA report, which is published before the PBRS Joint Committee meeting.

25.3 Spotlight on Selected Aspects of Patient Participation in HTA

25.3.1 Evolution of Patient Participation Over the Past 5 Years

Since 2015, various mechanisms have enabled patients to contribute to Taiwan's HTA and reimbursement processes actively. Patients share their experiences through an online platform before the PBRS Joint Committee meetings. The CDE/HTA team compiles these insights into the HTA reports and engages in preliminary discussions with key stakeholders. These meetings include the participation of two patient advocates and representatives of condition-specific patient groups. From May 2015 to October 2024, this inclusive approach has facilitated the adoption of 75 new technologies (45 oncology, 28 non-oncology, and two medical devices), with insightful patient contributions acknowledged and discussed before the PBRS Joint Committee meetings. These contributions are publicly available on the NHIA website, demonstrating a commitment to transparency and patient-centered care. The CDE/HTA team held 24 meetings between October 2019 and February 2024.

Over the years, significant progress has been made in collecting patient feedback through collaborations between patient organisations and the community. Initially, opinions were gathered from only one or two patients. Currently, most patient feedback comes from patient organisations that independently collect views from their members using various online platforms. The following provides a case study.

25.3.2 Case Study: Spinal Muscular Atrophy (SMA) Patient Voices

On February 4, 2023, the CDE/HTA team received the following inputs from the NHIA platform:

- 18 submissions from the Become the Miracle Association (BMA) patient organisation, including one that was compiled from focus groups with 100 patients and 200 carers and another one that reflected the experiences of 70 patients with SMA and their families
- Feedback from 10 individual patients
- Insights from 5 carers.

25.3.2.1 Experience of Receiving Treatment

According to the BMA, approximately 40 patients with SMA have been given compassionate use of nusinersen (Spinraza) and reported to have had gastrointestinal symptoms and rashes shortly after treatment. However, most studies reported that these adverse effects disappear over time. Patients with SMA reported that their muscular system has improved functions in swallowing, chewing, digestion, and mobility. In the physical therapy sessions, they felt stronger reflexes and more energetic than before. Other patients mentioned choking far less often, and even when they choked, they were able to cough up the phlegm. They also reported improvement in the motion of their fingers and were able to sit in a wheelchair without needing to lie down. Patients with SMA reported having improved medical conditions and mobility, and enjoying life more, leading them and their families to remain hopeful about the future.

25.3.2.2 Current Medical States

Patient organisations indicated that these patients experienced improvements in their health conditions after nusinersen treatment. This reduced the family/non-family carer burden while giving hope to patients and their families, as they were now taking medication. Several patients mentioned that, without taking medications, they simply kept track of any changes in muscular strength, lung function, and scoliosis while undergoing physical therapy to slow down muscular atrophy and deterioration. Patients without active treatment may show signs of deterioration and loss of muscle strength and may even be unable to breathe.

Quality of life among adult patients Several patients described how they have become unable to care for themselves because of muscle deterioration. Consequently, they now require assistance in bathing, toileting, and turning over in bed. Patients with SMA also experience difficulty in finding a job, as there are still many companies in Taiwan that fail to provide an accessible and disability-inclusive workplace.

In addition to financial issues, lack of mobility makes patients feel depressed and uncertain about their future when they are unable to move around or engage in outdoor activities, let alone enjoy their lives. As patients require a lot of assistance from their family members, they are stressed and fearful of 'being a burden'. Carers must keep an eye on patients to monitor whether they are well while tolerating emotional instability due to the patient's deterioration.

Expectations of new treatment Patients who require assistance in daily living hope that the treatment helps stop or slow the pace of deterioration while improving their muscular strength and mobility so that they can care for themselves. Other patients hoped to stop using the respirator or feel less pain. Some patients hope that this drug will be added to the NHI package to reduce the financial stress caused by medical treatment, which would also help improve their quality of life. Patients with severe symptoms hope to improve their muscular strength and protect the heart and lungs from rapid deterioration so that they can take better care of themselves. Patients with mild symptoms hope not to experience a worsening of symptoms, while reducing scoliosis after treatment. They hope not to undergo physical therapy, stop being wheelchair-bound, and find a new job. Most adult patients live independently for long periods. The patient organisations wanted to receive better treatment for scoliosis after the oral drug was added to the NHI package.

These insights were summarised and presented during the appraisal meeting, providing context on patients' lived experiences, unmet needs, and treatment expectations. Although clinical and economic evidence ultimately guide all recommendations, patient perspectives help inform the committee's discussion, shape the understanding of disease burden, and are documented in the HTA report to ensure transparency. Even in some cases where the final decision is not to recommend the medicine, patient input remains a crucial part of the assessment process as it contributes to a more holistic and patient-centered evaluation.

25.4 Reflections

25.4.1 *Current Practice and Areas for Future Focus*

Taiwan's experience of integrating patient participation into the HTA framework demonstrates that impactful outcomes are achievable even with resources that are considerably more constrained than in many Western nations. Taiwan's approach may serve as an exemplary blueprint for middle-income countries that aim to incorporate patient participation into HTA processes. Given the interdisciplinary nature of HTA, gathering perspectives on patient involvement from global audiences is crucial. Although patient involvement is vital for understanding the needs of the intended beneficiaries, issues related to the timeliness of engagement and resource allocation persist. Further initiatives are required to bolster the contribution and visibility of patient feedback in the decision-making framework.

25.5 Discussion

25.5.1 Challenges

In Taiwan, patient participation in HTAs and reimbursement decisions are strongly supported by the NHIA, with the CDE/HTA team facilitating this process. Since 2015, various mechanisms, including an online platform, have enabled patients to share their experiences before PBRs Joint Committee meetings. These insights are compiled into HTA reports and discussed with key stakeholders, including patient advocates and condition-specific group representatives. Meeting documentation and recordings are made publicly accessible on the NHIA website to promote transparency.

Internationally, organisations such as the National Institute for Health and Care Excellence (NICE) (Chap. 17), Canada's Drug Agency (Canadian Agency for Drugs and Technologies in Health [n.d.](#)), the Scottish Medicines Consortium (Scottish Medicines Consortium 2017), and the Pharmaceutical Benefits Advisory Committee (Chap. 14) have adopted similar methods to gather patient inputs, reflecting the global recognition of incorporating patient perspectives into HTA processes. However, several challenges remain to be resolved. The tangible impact of patient participation is not always clear and the number of patients using the online platform is limited. This could be due to a lack of awareness among patient organisations. Additionally, both HTA bodies and patient organisations require more personnel. With the PBRs Joint Committee agendas released only a week in advance, patient representatives have limited time to prepare feedback thoroughly.

Furthermore, one of the key challenges is that the current platform for collecting patient input is primarily limited to patients with catastrophic illnesses, mainly including cancer and rare diseases. As a result, patients with other chronic conditions, like diabetes, are unable to express their perspectives through the platform.

25.5.2 The Way Forward

To address these challenges, CDE has been actively enhancing the patient feedback platform and encouraging patient organisations to provide their concerns during decision making. Through regular patient communication meetings held annually, CDE collects feedback from patient organisations on platform functionality, data collection, and the presentation of opinions, making improvements accordingly. Recently, these meetings were held in March and August 2024, where patient groups provided valuable suggestions to optimise the current system.

Currently, in close collaboration with the NHIA, CDE is working towards a gradual enhancement of the patient feedback platform. In the future, subject to available resources and personnel, CDE aims to expand the scope of patient input collection to include more patients with chronic diseases such as those with diabetes and psoriasis, rather than limiting it to cancer and rare diseases. Additionally, recognising

the time constraints faced by patient representatives, CDE will continue exploring more flexible feedback submission mechanisms, such as early agenda notifications for patient organisations, to help them better prepare and provide more valuable input.

CDE has also learned from international practice, such as the patient expert mechanism used by NICE in the UK, to encourage patient participation in joint appraisal meetings and to enhance transparency and increase patient engagement in the process.

In 2025, Taiwan made further progress in patient involvement in the PBRs process. For the first time, one of the two insured representatives appointed by the NHIA was a patient advocate with voting rights. Although decisions in PBRs meetings are generally made by consensus rather than formal voting, this development marks a meaningful step towards institutionalising patient representation. In addition, two patient representatives continue to attend the meetings as observers and actively contribute to discussions.

To further address these challenges, the CDE/HTA team has adopted HTAi protocols tailored to patient groups (2014). With support from the NHIA, CDE has revised the online questionnaire to include a broader range of participants (National Health Insurance Administration *n.d.-a*, *n.d.-b*). These efforts have transformed the platform into a more effective tool for collecting patient perspectives and feedback, ensuring that patient participation better reflects the diverse experiences and needs of various patient groups.

Moving forward, CDE will continue to optimise the patient feedback platform, including improving platform functionality, enhancing patient response guidelines, and exploring more effective mechanisms for patient input collection. The goal is to ensure that patient voices play a stronger role in the HTA decision-making process.

25.6 Conclusion

Taiwan's integration of patient participation into the HTA framework shows that impactful outcomes are achievable even with limited resources. Taiwan's approach serves as a model for other middle-income countries. The ongoing refinement of strategies, including an online platform for patient input and group discussions, is crucial. Despite the challenges in engagement timeliness and resource allocation, further initiatives are needed to enhance the visibility and impact of patient feedback on decision-making.

Acknowledgements The authors thank the National Health Insurance Administration (NHIA) and Ministry of Health and Welfare (MOHW) for their financial support. No funding was provided for preparing this manuscript.

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Chapter 26

USA: Patient Involvement at the Institute for Clinical and Economic Review



Catherine K. Fischer, Kathleen A. Arntsen, Mark W. Skinner,
Maria J. Kefalas, and Tiffany A. Westrich-Robertson

26.1 Context

The Institute for Clinical and Economic Review (ICER) uses the term ‘patient engagement’ for activities described in this book as ‘patient participation’. For consistency, we are adopting the term ‘patient participation’ for this chapter when not referencing ICER’s Patient Engagement Program or process by its formal name. ICER uses the term ‘patient community’ to describe the group of relevant individual patients, carers, patient organisations, and affiliated groups representing patients for a given drug review. ICER invites both formal patient organisations and informal groups of patients to participate in our reviews. For the purposes of this chapter, all formal and informal groups will be referred to as ‘patient organisations’.

ICER functions as an independent not-for-profit that conducts health technology assessment (HTA) in the United States (US). ICER mainly reviews prescription drugs and occasionally non-drug health interventions, and produces evidence

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reports that include comparative clinical effectiveness and cost-effectiveness analyses. As an independent non-governmental institute, free from financial conflicts of interest, ICER produces non-binding recommendations for decisions related to drug pricing and insurance coverage. ICER's Governance Board, holding fiduciary responsibility and providing strategic counsel to ICER's leadership, is composed of a diverse group of stakeholders including patient and consumer representatives. Importantly, ICER does not have regulatory authority to approve, price, or provide insurance coverage for drugs. The role of ICER is substantially different than that of public HTA institutions in other countries where comparative effectiveness research and cost considerations are standard practice in the approval and reimbursement of new drugs.

Consequently, there is less familiarity with and acceptance of ICER's work by the general public in the US, creating unique challenges for ICER and its ability to build trust and relationships with patient community partners. As a result, there is broad misunderstanding about ICER's role in the US which has generated fear and confusion about ICER's intended mission. Coupled with the fact that before 2020, ICER did not have a dedicated patient participation team, patient involvement in ICER reviews often felt strained, with patient communities finding the process to be overly complex and burdensome. The overall sentiment improved considerably once ICER was able to invest in a dedicated staff to formalise a Patient Engagement Program (discussed in Sect. 26.2) that offers onboarding for new patient organisations, increased opportunities and compensation for input, and patient-facing resources.

ICER's mission to achieve fair pricing, fair access, and future innovation aims to address the fundamental problem with drug pricing and patient access in the US: that in the absence of drug price regulation, manufacturers often choose to price their products based on what the US market will bear rather than on the benefit provided to patients. When new drugs enter the market with high price tags, unsupported by the evidence of benefit, public and private insurers often respond by restricting access (e.g. narrowing eligibility, enforcing step therapy) in order to afford coverage for its members. Patients and families, then, pay the price for these decisions with rising insurance premiums, unaffordable insurance coverage, and ultimately worse health outcomes from not being able to access or afford high-quality care.

ICER's vision is sustainable access to high-value healthcare for all Americans, and this cannot be achieved without effectively involving patients in the decisions that directly affect their ability to access treatment. Participation in ICER's review process offers one avenue to elevate the patient voice and inform HTA recommendations about pricing, access, and future research.

26.2 Current Structures for Patient Involvement

Founded in 2006, ICER has always welcomed patient community input into its reviews, however, only in 2020 was ICER's Patient Engagement Program formalised with the publication of ICER's 2020 Value Assessment Framework (Institute for Clinical and Economic Review 2020). Since then, ICER has also published its updated 2023 Value Assessment Framework including significant additions to its Patient Engagement Process, all of which are described below (Institute for Clinical and Economic Review 2023a).

26.2.1 Early Notification and Onboarding

Upon selection of a new topic, the ICER patient engagement lead (PEL) conducts a review of national and regional disease-specific patient advocacy organisations, as well as relevant social impact or umbrella organisations. The PEL reaches out via email to the selected organisations with an early notification (4–6 weeks prior to public launch of the review) and invitation to an introductory call. Those interested can join for a one-hour call with the PEL and Program Manager to learn about:

1. ICER, its mission, and role in the US healthcare system
2. ICER's patient participation process and opportunities for input
3. The importance and impact of patient participation in an HTA review.

If the organisations are still interested in participating following the introductory call, the Program Manager will communicate the review timeline, all upcoming milestones, and opportunities for input.

26.2.2 Process Overview

The ICER review process typically spans 9 months and actively involves the patient community from start to finish. The Patient Engagement Process can be simplified into three distinct phases: Share Your Lived Experience, Respond to ICER's Work, and Participate at the Public Meeting. Figure 26.1 below illustrates the flow from phase to phase, and the opportunities ICER offers for patient community input.

26.2.3 Share your Lived Experience

Patients and patient organisations can share their lived experience through scoping calls (virtual meetings to comment on the population, intervention, comparators, and outcomes of focus) with ICER's research team or through written or public

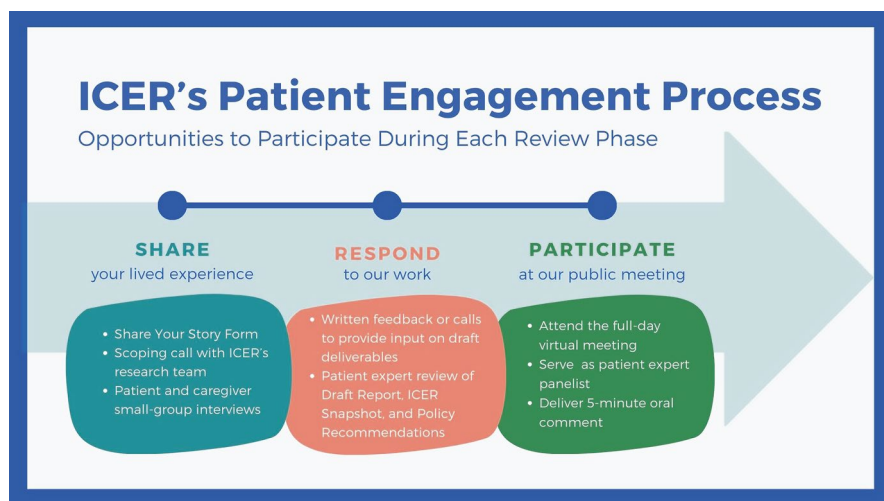


Fig. 26.1 The three phases of ICER's Patient Engagement Process

comments (Institute for Clinical and Economic Review 2023b). In addition to inviting patient organisation participation, ICER also requests assistance from the patient organisations to be connected with individual patients and carers to capture the full depth and diversity of individual lived experiences with a disease. This may include a better understanding of symptoms and existing treatment side effects, as well as the varying preferences and hopes for what a new treatment could offer. Following its 2023 Value Assessment Framework update, ICER offers the *Share Your Story Form* (an online five-question form about the impact, challenges, and overall lived experience) as well as small-group interviews for individual patients and carers to provide input when not affiliated with a formal organisation (ibid.).

The patient lived experience is summarised within its own section in the ICER report titled *Patient Community Insights*. A separate section, *Benefits Beyond Health*, captures important considerations from the patient community that are not measured in clinical trials or otherwise reflected in the current evidence base.

26.2.4 Respond to ICER's Work

ICER offers different touchpoints for the patient community to provide feedback on draft iterations of its research scope, model analysis plan, evidence report, policy recommendations, and patient-facing report summary (Institute for Clinical and Economic Review 2023c). Feedback can be provided through virtual meetings, written input, or early review of the draft report prior to publication (ibid.). ICER provides a formal response and rationale for the inclusion or exclusion of feedback received through public comments on the draft report. These responses are published for all stakeholders to review alongside the revised report.

26.2.5 *Participate at the Public Meeting*

Near the end of ICER's process, a public deliberative meeting is convened to which ICER invites both the patient organisations as well as individual patients and carers to participate as panellists or oral commenters. These patient perspectives are critical to informing the independent appraisal committee that deliberates and votes on the clinical effectiveness and value of the treatment under review. Patient community perspectives help the appraisal committee contextualise the evidence presented and signal to decision-makers how drug pricing, insurance coverage, and future research should prioritise and address the challenges faced by the patient community.

26.2.6 *Compensation*

Along with the 2023 Value Assessment Framework update, ICER has committed to compensate patients and carers who contribute extensively to the review process (Institute for Clinical and Economic Review [2023a](#)).

Individual patients and carers are compensated \$100 USD for their participation in the small-group interviews. Patient experts who review the draft report are compensated \$500 USD for their time and effort. Patient representatives who participate as panellists during the public meeting are compensated \$500 USD for their expertise and contributions.

26.2.7 *Final Touchpoints*

Following the public meeting, the final section of ICER's report includes key policy recommendations for decision makers—patient organisations, public and private payers, manufacturers, regulators, researchers, and clinical societies—that are often shaped by the contributions of patient expert panellists during the deliberation. The final report is accompanied by a patient-facing report summary, called the *ICER Snapshot*, which highlights key findings, policy recommendations, and the impact of patient community involvement.

The review closes with a post-review debrief call offered to the most engaged patient organisations as an opportunity to reflect on the entire review and provide feedback to ICER on its process, communication, and other aspects of patient involvement. The feedback received helps inform and enhance ICER's approach to future reviews.

26.3 Case Examples of Patient Involvement in ICER Drug Reviews

Three specific case studies below describe the impact on HTA from the involvement of patient communities advocating for lupus nephritis (2021), haemophilia (2022), and metachromatic leukodystrophy (2023).

26.3.1 *Partnering with the Lupus Nephritis Patient Community*

The first ICER review to undergo the formalised Patient Engagement Program was the review of two novel drugs for lupus nephritis, an autoimmune disease that causes inflammation and damage to the kidneys (Tice et al. 2021). Initial outreach to the community included invitations to three lupus-focused patient organisations ranging in structure from a smaller volunteer-led group to larger organisations, all with a national presence and reach. Given the increased prevalence of lupus nephritis among Black and Hispanic women, a health equity-focused organisation reached out to ICER to provide input on this important perspective.

The three leading lupus organisations, by the time of the ICER review, had already participated in a Patient-Focused Drug Development session with the Food and Drug Administration (FDA). This collaboration resulted in the publication of the *Lupus: Patient Voices* report which features data and anecdotes illustrating the lupus lived experience, from symptoms to daily impacts to experience with treatments (Lupus and Allied Diseases Association et al. 2018). This publication proved timely and relevant at the outset of the ICER review to identify outcomes of interest to the lupus community. In addition, early calls with and public comments from the patient organisations also emphasised important patient insights; ICER heard of how lupus nephritis impacted mainly women during child-bearing age with current standard of care negatively impacting their fertility and ability to plan for a family. The impact of lupus nephritis on communities of colour was also emphasised, prompting ICER to conduct subgroup analyses that helped draw attention to the disparities in outcomes between white and non-white patients (Tice et al. 2021). These patient insights shaped the deliberations during the public meeting, with an emphasis on reducing health inequities and expanding diversity in clinical trials, considerations that impacted ICER's key policy recommendations.

Although participation in ICER's process was time-consuming, especially for the smaller volunteer organisation, the high level of participation was claimed to be worthwhile; the report conclusions, deliberative process, and policy recommendations were greatly influenced by the priorities and perspectives of the lupus patient community. Those engaged also shared the importance and value of participation in ICER's process in order to elevate the voice of people living with lupus (Arntsen et al. 2022). Patient involvement (Table 26.1) was seen as the pathway to producing ICER assessments that are relevant to those who are directly affected by the condition and report conclusions (ibid.).

Table 26.1 Patient involvement and impact on ICER’s 2021 review of lupus nephritis therapies (Tice et al. 2021)

Patient involvement	Four organisations participated in various parts of the process; two patients and one patient advocacy representative served as panellists at the public meeting; two patients provided testimonies at the public meeting
Data received	<i>Lupus: Patient Voices</i> report (Lupus and Allied Diseases Association et al. 2018)
Patient insights	1. The most bothersome symptoms included fatigue, joint pain, and muscle pain 2. Downsides of current treatment included side effects, number of pills and treatments taken each day, and the cost of treatment 3. Reducing the need for high-dose steroids was important given the number of resulting negative side effects and harms 4. Going into an infusion centre to receive treatment during the COVID-19 pandemic was concerning 5. The young age of onset of lupus nephritis has a huge negative impact on patients’ ability to work, to have children, and to advance in their careers 6. The impact of lupus nephritis on communities of colour and disparities in health outcomes deserves attention
Impact of patient involvement	The ICER report included a more thorough and accurate reflection of the diversity of the patient lived experience with lupus nephritis. ICER’s economic model incorporated the steroid-sparing effects of the treatments. The voting questions for public deliberation were updated to include the patient-important outcome of “preservation of kidney function” given its impact on patients’ ability to have children

26.3.2 Partnering with the Haemophilia Patient Community

Haemophilia is an inherited bleeding disorder characterised by the inability for the blood to clot properly (Tice et al. 2022). ICER’s 2022 review of gene therapies for haemophilia A and B marked the third review of haemophilia therapies in the previous 6 years, with the repeated involvement of certain patient organisations leading to well-established and collaborative relationships (ibid.). ICER’s 2018 and 2020 reviews were focused on treatments for haemophilia A specifically (Rind et al. 2018, 2020); the 2022 review allowed ICER to expand community outreach to the haemophilia B community.

As a sophisticated and research-focused patient community, the leading patient organisations had developed a set of core outcome measures critical to evaluating the comparative effectiveness and value of haemophilia gene therapies. Upon launch of ICER’s 2022 review, the patient community directed ICER to the results of this coreHEM outcome set (Iorio et al. 2018), an international multi-stakeholder project (including HTA bodies) that convened a panel of experts to identify six core outcomes: frequency of bleeds, factor activity level, duration of expression, chronic pain, mental health status, and utilisation of the healthcare system (direct costs). These core outcomes were integrated into ICER’s scope of research for the gene therapy review.

Leveraging the coreHEM framework, leading patient advocates and researchers also conducted a study that demonstrated an increase in quality of life for patients receiving a one-time therapy versus repeated prophylactic administration (Benton et al. 2023). This quality-of-life gain from reducing lifetime treatment burden was an important input into ICER’s economic model; inclusion of this utility increment resulted in a higher value-based price for each of the gene therapies under review. It is rare for such patient utility studies to be conducted and made available for application into ICER’s assessment, marking a transformative moment in the research-informed strength and impact of the haemophilia community’s involvement with ICER.

In addition to the findings from these two research efforts, ICER’s understanding of the haemophilia lived experience was enriched by the compounding of patient insights gained over the three reviews (Rind et al. 2018, 2020; Tice et al. 2022). The 2022 review was the first to offer small-group patient interviews and therefore enhanced the depth of ICER’s summary of the outcomes and priorities most important to the community, especially with regard to the potential harms and uncertainties that come with novel therapies. The public deliberation and key policy recommendations were grounded in this robust appreciation for the daily impacts of haemophilia as well as long-term considerations for the safety and durability of gene therapy. Table 26.2 provides additional context for how the haemophilia community’s involvement in the 2022 review shaped ICER’s analysis and recommendations.

Table 26.2 Patient involvement and impact on ICER’s 2022 review of haemophilia A and B gene therapies (Tice et al. 2022)

Patient involvement	Three patient organisations participated in various parts of the process; three individual patients participated in small-group interviews; two patient experts served as panellists at the public meeting; three individual patients provided testimonies at public meeting
Data received	coreHEM core outcome set (Iorio et al. 2018), patient utility gain from reducing treatment burden (Benton et al. 2023)
Patient insights	1. Participation is the outcome that matters most to patients—ability to participate in family life, recreational activities, school activities, and work activities without restriction 2. Significant impact of permanent joint damage from prior bleeds on quality of life 3. Regular factor infusions are an enormous burden to patients and to their carers 4. Reluctance to try new therapies given historical harms from new therapies 5. Financial toxicity associated with high-cost haemophilia treatments remains a barrier to fair and equitable access 6. Significant burden on participants during the first 6- to 12-month period post-gene therapy
Impact of patient involvement	ICER’s scope integrated outcomes identified through the coreHEM project. ICER’s economic model included a utility input generated by the haemophilia patient community resulting in a higher value-based price for the gene therapies under review. The ICER report proposed alternative ways to calculate a fair price for a one-time gene therapy when compared to a lifetime of expensive chronic care. ICER’s key policy recommendations emphasised the financial toxicity faced by patients for factor replacement therapy and called on the government to step in and ensure more reasonable pricing

26.3.3 Partnering with the Metachromatic Leukodystrophy Patient Community

In 2023, ICER reviewed a gene therapy (Lin et al. 2023) for metachromatic leukodystrophy (MLD), a rare, fatal genetic disorder affecting young children and characterised by the progressive loss of motor and cognitive function. The ICER team heard from three patient organisations and several parents who were carers for their children with MLD. Patient community input was received through virtual meetings with the ICER research team as well as submissions to ICER’s *Share Your Story Form*, a short online form to collect perspectives on the impact and experiences with MLD.

With the unique circumstance that the gene therapy under review was already approved in Europe, patient community input included not only the US perspective, but insights from the experiences of families who had accessed this therapy in clinical trials in Europe. Interestingly, the patient organisations involved in this ICER review had recently undergone a similar HTA review experience with the National Institute for Health and Care Excellence (NICE) in England (National Institute for Health and Care Excellence 2022). The organisations, therefore, came to the ICER review with much more familiarity with the HTA process. The MLD community’s overall understanding of ICER’s objectives for the gene therapy review made the collaborations more efficient and less burdensome for the patient organisations. Table 26.3 describes the insights gained and impact of the MLD patient community’s participation in the 2023 ICER review.

The MLD review was one of the first reviews to include an *ICER Snapshot* which summarised the evidence report results in a more accessible way for the patient community (Institute for Clinical and Economic Review 2023e). The ICER team received positive feedback on the intent and execution of this Snapshot showcased in Fig. 26.2.

Table 26.3 Patient involvement and impact on ICER’s 2023 review of a gene therapy for MLD (Lin et al. 2023)

Patient involvement	Three organisations participated in various parts of the process; six carers (parents of children with MLD) participated in small-group interviews; two carers served as panellists at public meeting; three carers provided testimonies at public meeting
Data received	Video of Patient Experience of Gene Therapy for MLD (CureMLD 2022)
Patient insights	1. Delayed diagnosis affects child’s eligibility for treatment and clinical trials 2. Parents experience a physical toll from lifting their children who have lost motor function and often need to modify their homes and buy wheelchair vans to accommodate the child’s disabilities 3. Cognitive and mobility difficulties result in the need for individualised education plans and additional help at school 4. Newborn screening is a key focus of advocacy to identify MLD before symptom onset for faster access to therapy 5. Navigating insurance coverage is time consuming and delays in care could worsen outcomes for children 6. Families face a large financial impact of MLD, raising equity concerns for those with lower incomes or no insurance

(continued)

Table 26.3 (continued)

Impact of patient involvement	Parents and patient organisations shared that the ICER review helped elevate attention for MLD. Public comment on ICER’s draft report led ICER to adjust the evidence rating for the gene therapy in favour of greater patient benefit. ICER’s policy recommendations request that payers provide wraparound coverage (transportation and housing costs) given that most patients will need to travel to receive the gene therapy

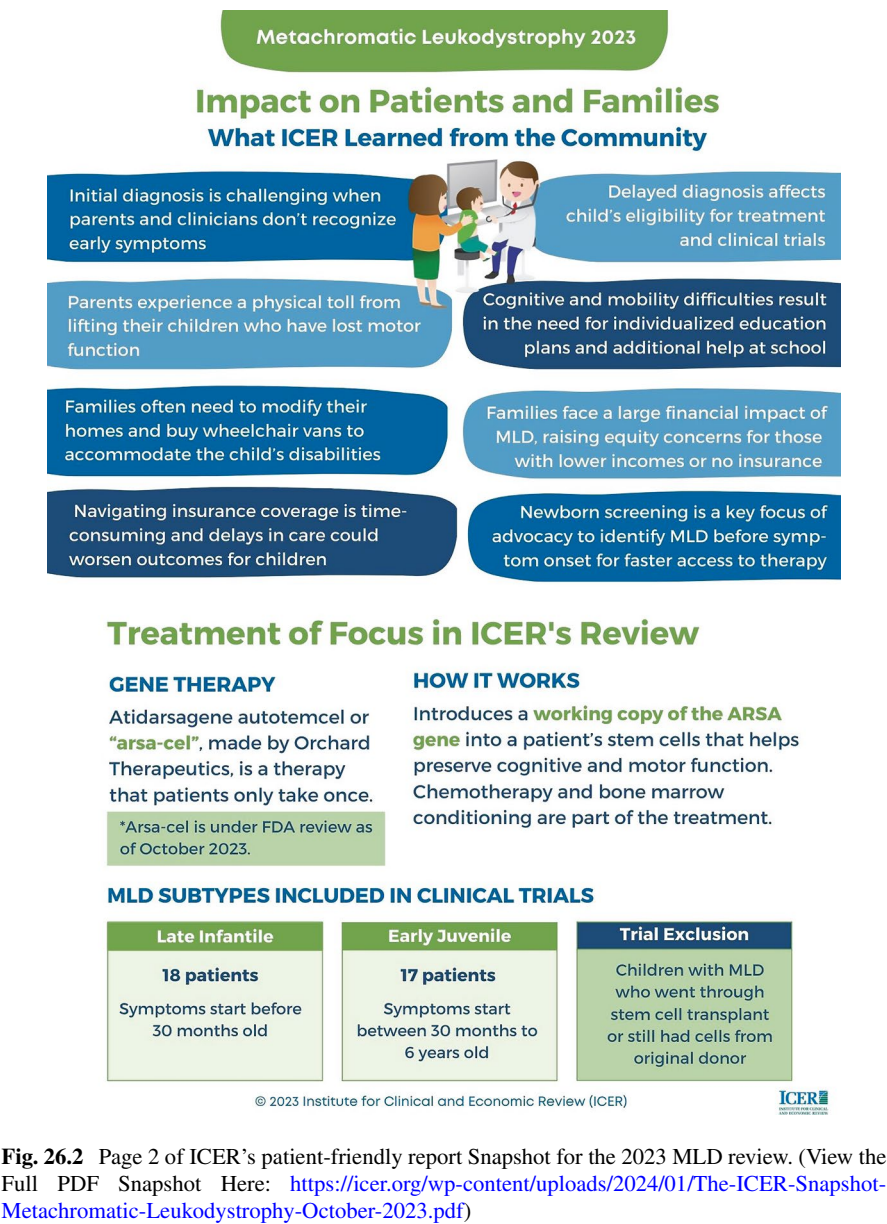


Fig. 26.2 Page 2 of ICER’s patient-friendly report Snapshot for the 2023 MLD review. (View the Full PDF Snapshot Here: <https://icer.org/wp-content/uploads/2024/01/The-ICER-Snapshot-Metachromatic-Leukodystrophy-October-2023.pdf>)

26.4 Creation and Progress of ICER's Patient Council

Since 2022, ICER has been working toward identifying and recruiting patients to serve in an advisory role on the Patient Council (Council). The first 18 months were dedicated to briefing interested candidates on ICER's background and process, and learning from them, in part, about the patient community's perception and concerns about ICER's role and process. This lead-in time was critical to developing a deeper understanding of the root of many tensions that often exist between ICER, patient organisations, and affiliated groups. ICER and Patient Council members agreed to a shared objective (Box 26.1) and action plan following a series of virtual meetings and an in-person meeting moderated by an external independent facilitator. In the early phase of Council development, having a neutral facilitator was critical to allow the Council members and ICER to independently voice their expectations and concerns, and co-create an action plan for the Council that met the needs of both parties.

Box 26.1 Objective of the Patient Council

To help patients and the public understand the value and impact of their involvement, and improve the diversity of people that are included and heard in the process of the evaluation of health treatments.

Over the 3-year term (2023–2026), the Patient Council aims to:

1. Evaluate ICER's current process of patient participation to identify gaps and opportunities for improved communication and participation
2. Ensure that the HTA process is inclusive of a diversity of experiences
3. Review ICER's Patient Portal and any future public facing documents to ensure accessibility and inclusivity.

Following the Council's unanimous approval of its primary goals, the launch of the Patient Council was formally and publicly announced in July 2023, with the Council description and member profiles featured on ICER's website (Institute for Clinical and Economic Review 2023d). The Council comprises five patient representatives with a range of expertise in HTA and experience with ICER, offering a variety of perspectives for evaluation of the ICER process. Of note, Patient Council members are participating as individuals, and are not representing the views of their affiliated organisations.

Through Council nominations and voting, a Patient Council member was selected to serve as the Council Co-Chair with ICER's Director of Patient Engagement serving as the ICER Co-Chair. Establishing this formal governance has been integral to building trust amongst Patient Council members and ICER.

Through regular quarterly meetings, ICER has benefited from the Council's candid review and constructive feedback on a variety of different projects related to the

main objectives listed above. The 2024 Patient Council Progress Report (Institute for Clinical and Economic Review [2024a](#)) was developed to reflect the Council's activities from July 2023 through June 2024 and demonstrate the progress and impact of the Council's contributions. A few examples of projects and outcomes are provided below:

- The Patient Council participated in a strategic discussion of the proposed restructuring of ICER's Patient Portal (Institute for Clinical and Economic Review [2021](#)), the webpage dedicated to describing ICER's Patient Engagement Process and guidance for participation. Their feedback led to the development of a new infographic to introduce the patient community to ICER and its process in a simplified, visually appealing format.
- With the goal of bridging the divide between ICER and patient communities, Patient Council members invited ICER's Director of Patient Engagement to attend their organisations' meetings and present to their community members. One such example includes an HTA 101 lecture given for the Knowledge = Empowerment Classroom hosted by the International Foundation for Autoimmune and Autoinflammatory Arthritis (AiArthritis [2023](#)). These introductory presentations raised awareness about ICER's process and facilitated early education about opportunities for community participation.
- The Patient Council's recommendations led ICER to supplement its patient community onboarding process with an *Introduction to ICER Guide* (Institute for Clinical and Economic Review [2024b](#)). The Patient Council also advised on ICER's new patient-friendly report summaries, called the *ICER Snapshot*, to ensure accessibility of ICER's process. Patient organisations who have had access to these new guides and summaries have provided positive feedback regarding the increased clarity and understanding of ICER's patient participation process, report findings, and the impact of their participation.

The expanded network and expertise of the Patient Council has further allowed ICER to leverage this partnership to address the current gaps in knowledge and understanding about the HTA process in the US.

26.5 Reflections

26.5.1 *Impact of Education, Communication, and Process Improvement*

Given the context of HTA in the US, further education on the goals and methods of HTA is critical to the efficiency and effectiveness of patient participation. The lupus community's participation required much more time and effort on their behalf to educate patients about the goal of ICER's drug review in order to create interest in participation. Contrastingly, the haemophilia community's repeated collaboration

with ICER and the MLD community's recent experience with NICE reduced the initial burden of onboarding, both for the participating organisations and for ICER's PEL.

ICER's relationships with patient community partners experienced much growth between 2020 and 2023, with feedback from these partners owing to many of the process updates implemented in the *2023 Value Assessment Framework* (Institute for Clinical and Economic Review 2023a). Remarkably, through our post-review debrief calls with patient organisations following an ICER review, there has been a notable shift toward improved understanding of ICER's process and decreased reports of the burden of participation in ICER assessments. These positive collaborations are likely a result of the combination of new onboarding materials, patient-facing guides, *ICER Snapshots*, financial compensation for intensive participation, and overall flexibility in accepting feedback in different formats. From the ICER perspective, the implementation of small-group patient and carer interviews has been a rich source of the diversity of lived experience, otherwise unattainable through a review of existing clinical evidence.

26.5.2 Impact of Long-Term Partnerships

ICER's prior reviews of haemophilia treatments helped highlight important gaps in the evidence that the haemophilia community worked diligently to address. This early evidence generation comes as a great benefit both to the patient community and to ICER, as the review team can incorporate this new evidence into its clinical and economic assessment. Given the short and rigorous timeline from topic selection to final report, patient organisations do not often have the time or resources to conduct research for incorporation into the ICER review. The three-time haemophilia review series demonstrate the rare example of how a trusted partnership over time between ICER and the patient community can help guide evidence generation and patient-informed HTA recommendations.

26.5.3 Impact of ICER's Patient Council

The Patient Council serves to provide feedback on ICER's patient participation processes from the perspective of patients with varying levels of HTA expertise. This external perspective helps ensure ICER's materials and communication are inclusive, accessible, and tailored to the unique needs of each patient community. The Patient Council and its advisement of ICER's patient participation strategy also provides a new mechanism through which communication to patient organisations is enhanced, community outreach is diversified, and impact of involvement is better conveyed. ICER's Governance Board receives regular reports of the work and feedback from the Patient Council.

26.5.4 The Future of ICER's Patient Engagement Program

Future areas of focus for ICER's Patient Engagement Program include methods of expanding community outreach and involvement in existing ICER initiatives. The first focus area is proactively building relationships with patient communities who may be unfamiliar with ICER. Even if no ICER review is in the immediate future, there is benefit in developing patient community relationships early on without the urgency of a review timeline. Such relationships could improve inclusivity of a new community's perspective for other initiatives at ICER, or even foster a call for relevant evidence generation should a new drug be entering the pipeline. Secondly, ICER's process for patient involvement in drug reviews has been streamlined and continuously fine-tuned for years. Borrowing from the existing process for drug reviews, ICER also has the opportunity to formally develop its process for patient involvement for other health policy projects focused on promoting fair insurance coverage and access for patients.

The three case examples profiled in this chapter all reflect partnership with well-established patient communities. There remain challenges in patient involvement, especially among groups less familiar with HTA or ICER's process, which demands continued development of ICER's Patient Engagement Program. ICER's hope is that the process improvements already achieved along with the contributions of the Patient Council and learnings from recent participation on reviews, will make patient involvement on an ICER assessment more feasible for less established or rare disease patient communities in the future.

26.6 Conclusion

The case examples of the lupus nephritis, haemophilia, and MLD reviews emphasise how ICER's updated Patient Engagement Program has allowed for increased understanding of HTA, collaborative partnerships, and a trusted channel for constructive feedback on the process for patient involvement in drug reviews. More broadly, investment in the Patient Council has shown early advantages in helping address the common gaps and tensions between ICER and the patient community, serving as a mechanism to affect change at the organisational level.

Acknowledgements The authors would like to thank all patients, carers, and patient organisations from the lupus nephritis, haemophilia, and MLD communities who participated on their respective ICER reviews. We would also like to thank all members of ICER's review teams that incorporated patient input into the 2021 lupus nephritis report, 2022 haemophilia report, and 2023 MLD report. The authors also acknowledge the contributions from all members of ICER's Patient Council who were instrumental in the creation of and impact achieved by the Council's advisory role. We also thank Sarah K. Emond, CEO & President of ICER, for her review and comments on the original manuscript.

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Chapter 27

Wales: Developing Bespoke Approaches to Patient Involvement in HTA



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27.1 Context

Health Technology Wales (HTW) is the national health technology assessment (HTA) body for evaluation of non-medicine technologies in the national health service (NHS) and social care service in Wales, United Kingdom (UK). It was founded to support a strategic approach to the identification, appraisal and adoption of non-medicine technologies in Welsh health and care settings. Established in 2017, HTW has strong support from the Welsh Government. Collaborating with partners across health and social care, HTW prepares HTAs based on reviews of the published literature to evaluate effectiveness and cost effectiveness, and has a wide-ranging patient involvement process to ensure patients' perspectives and experiences are considered systematically in HTA using appropriate methods.

This chapter outlines how a bespoke approach to patient involvement was developed for each HTW appraisal. This could include secondary and primary research that is the co-creation of participatory approaches with patient organisations. The early challenges of engaging with patient organisations during the COVID-19 pandemic are discussed and the resulting modifications to create a more flexible process of involvement are explained. Three case studies show the flexible approach, and the discussion reflects on the value of the approach and potential for further patient involvement in HTW processes.

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27.2 Mechanisms for Patient Involvement

HTW processes for patient involvement were developed in the year following the establishment of HTW in 2018. The *UK Standards for Public Involvement in Health and Social Care Research* (National Institute for Health Research et al. 2019), and *Participation Cymru* (now updated WCVA 2025) provided the policy setting to establish the HTW patient involvement process. Learnings were also elicited from other organisations such as the Scottish Health Technologies Group (2024), the National Institute for Health and Care Excellence (NICE 2019), those presented in the first edition of this book (Facey et al. 2017) publications on putting patients at the heart of HTA (Facey et al. 2018) and Health and Care Research Wales (2019).

The first activity undertaken was to establish a Patient and Public Involvement Standing Group (PPISG) to oversee development and use of HTW processes and tools for undertaking effective patient involvement in appraisal. Members recruited to this group had experience and knowledge of patient involvement, had been involved in research as a patient, were affiliated with UK patient networks and charities and had expertise in varying backgrounds including mental health, social care, the Welsh Government, genetic and rare diseases, cancer, HTA and health research.

Two members of the PPISG are also appointed to sit as Public Partners on HTW's Assessment Group, and two on HTW's Appraisal Panel, to (a) ensure appropriate patient involvement was being undertaken to a high standard per appraisal and (b) to ensure the perspectives of patients and carers are given due consideration in guidance decisions. The terms of reference are published for the group (Health Technology Wales 2019) and regularly reviewed.

Taking account of the range of non-medicines technologies that HTW could be appraising (from diagnostic tests performed without the patients' knowledge through to patient-controlled devices, such as monitors, apps and other wearable devices) it was decided that a carefully considered approach to patient involvement was needed. This would shape the process of involvement for each individual health technology, ensuring that it was proportionate to the value patient involvement was likely to bring, make best use of published research and limited patient organisation resources (particularly apparent in a country like Wales with a population of 3 million). Therefore, an initial suite of patient involvement mechanisms was agreed. The current suite is presented in Table 27.1 as they appear in the Mechanism Tool (Health Technology Wales 2022), which is used by PPISG for each HTA. See Table 27.1.

These mechanisms are supported by tools for patient organisations, including a submission form and associated guide, a template for questionnaires to be issued by patient organisations, and guidance on undertaking focus groups. These tools have undergone several amendments as processes have evolved, including becoming suitable topics from the social care sector in April 2022. They are available on the HTW web page (Health Technology Wales 2024). Completed submission forms are included in summary in the main body of the evidence appraisal under a dedicated section for patient evidence entitled *Patient, Carer and Family Evidence*, and included in full as an appendix. Patient evidence obtained by other methods, such as

Table 27.1 Patient and Public Involvement Mechanism Tool—Second part outlining mechanisms for involvement

Suggested method of involvement (tick all that apply)	Tick if required
Public Partner Input	*
Identification of patient/carer experiences and perspectives within clinical effectiveness review	*
Summarise easily accessible reports on relevant patient/carer issues (e.g. HCRW, Welsh Government, other HTA reports)	
Targeted patient/carer issues search to identify published literature	
Early stage patient group/organisation participation (topic identification, scoping)	
Key stage patient/group/organisation participation	
Focus groups	
Additional patient organisation involvement—i.e. at appraisal panel	

HCRW Health Care Research Wales, *HTA* health technology assessment
*1 and 2 are undertaken for every HTA

questionnaires, focus groups, etc. are written up into reports by the Patient and Public Involvement (PPI) Manager and included in full under the *Patient, Carer and Family Evidence* section. The patient evidence is presented at Appraisal Panel by a patient and/or service user representative or by the Public Partner. There is dedicated time for discussions of the patient evidence built into the structure of the Appraisal Panel and the PPI Manger is present to ensure that this evidence is given due consideration.

27.2.1 Role of PPISG in Determining an Optimal Approach

Determining the form of patient involvement for a topic is a multifaceted decision that considers how the technology will be used, the level of patient interest, if a patient organisation can be identified and if research into patient aspects is known to exist and likely to influence the HTA. These issues are discussed systematically for each HTA by the PPISG via a standard set of questions presented in the first part of the Mechanism Tool (Health Technology Wales 2022).

Contributing to these discussions are HTW health researchers, HTW’s PPI Manager, HTW’s Project Management team and the PPISG. An HTW researcher begins by giving a short presentation on the technology, the proposed uses, the patient population and the evidence appraisal scope. PPISG members can then ask questions before the aspects associated with that technology are discussed, then the potential mechanisms for involvement are discussed as outlined in Table 27.1 and an approach is agreed. The PPISG may also make specific recommendations about how the involvement should be conducted (e.g. running a focus group with patients from the national cancer centre, engaging with a specific patient organisation to co-develop a questionnaire for its members). Employed during the scoping period of an

HTA appraisal, the tool seeks to ensure the correct balance between the benefits of seeking patient participation versus the burden of contribution, ensuring that any patient involvement activities undertaken are fit for purpose, appropriate, meaningful and value-driven.

As the patient involvement approach is undertaken, PPISG is kept informed of progress, can advise on any adaptations needed and has oversight of the patient involvement submissions for each appraisal. PPISG may also support focus groups and educational activities with patient organisations.

27.3 Structured but Flexible Approaches

The initial pilot of the Mechanism Tool and associated processes and tools for patient involvement were undertaken from October 2019 and into Spring (northern hemisphere) 2020, at which point the COVID-19 pandemic severely impacted processes and capacity of patient organisations and HTW staff.

The most common mechanism of involvement at the time was the Patient/Carer Submission form. It was adapted from that created by SHTG, covering the health condition or social care need, current treatments and practices and the impacts, or potential impacts, of the health or social care technology.

From the outset, a record was kept of responses from patient organisations invited to complete the Patient/Carer Submission form, along with their feedback on HTW's patient involvement processes and appraisal outcomes. The feedback indicated that some patient organisations could see the value of completing a Patient/Carer Submission form to contribute to an HTA appraisal but advised it could be time consuming, resource intensive and difficult to justify considering their care commitments. This often led patient organisations to decline participation. Additionally, some patient organisations had never heard of HTW nor had any experience in contributing to an HTA and found the prospect daunting.

To address this feedback and encourage participation, it was recognised that a bespoke, flexible approach to patient involvement was needed for each topic. It became apparent that patient organisations often had established processes in place for engaging with their patient networks that could be utilised to gain the same information that would be collected in the Patient/Carer Submission form. Therefore, HTW began to invite patient organisations to an open conversation at the start of participation to discuss how they felt best able to contribute information. After HTW staff explained the purpose of the appraisal, the health or social care technology and the kind of information HTW was seeking from patients, the patient organisation would then advise what they considered to be the best approach. Working in partnership with patient organisations in this way, HTW was able to benefit from collaborative approaches such as:

- Repurposing previous submissions to another HTA organisation in the UK for a Welsh demographic

- Use of existing patient data: testimonials, feedback, stories, reports, etc. readily available on the patient organisation's website or archives
- Co-production of surveys and questionnaires for a patient organisation to issue to members
- Organisation of focus group(s) with the patient organisation for their members.

This has led to a flexible approach to supporting patient organisation to participate, using a co-production approach, with substantial support from HTW. The patient organisation helps shape the process to be taken and an agreement is made regarding roles, responsibilities, and deadlines. The patient organisation has final say on the submission information that goes into the detailed HTA report (the Evidence Appraisal Report (EAR)), in the same way as they would have done if they had completed the Patient/Carer Submission form. This allows the patient organisation to comment on such things as the preferred language of a patient community, inclusivity needs, realistic deadlines, correct reporting of patient views, etc. This results in an increased level of participation from the patients themselves.

This flexible approach also enables adaptation to address issues that emerge. On occasion, it may not be possible to deliver the originally chosen mechanism of involvement. So HTW staff, in liaison with the patient organisation and PPISG, would consider mitigation approaches such as identifying other patient organisations (perhaps UK-based ones) exploring alternative routes to patient communities (such as via clinics, local groups and amenities, social media sites) using translation services, using online tools, searching specific issues in the literature, etc.

Although this process is flexible, it is important to ensure consistency in this flexibility. So, this approach is written into the *HTW Patient Involvement Standard Operating Procedure*.

Three examples are presented to show this flexibility during an appraisal with different forms of patient involvement for different types of health and social care technologies.

27.3.1 Example 1: Flash Glucose Monitoring

HTW first published guidance on the use of flash glucose monitors in 2018 before the establishment of the PPISG and patient involvement processes. The recommendation was made that the devices showed promise, but the evidence was not sufficient to support adoption. The topic was reassessed in 2021 and PPISG used the Mechanism Tool taking account of the patient involvement approaches used in the first edition of the HTA. Patient aspects had been identified in the clinical effectiveness literature and so a full search of the literature for qualitative papers on patients' experiences was undertaken. Additionally, Diabetes Cymru agreed to complete a Patient/Carer Submission form. The submitted form and the results of the literature search were both included in the appraisal, and this was presented to the Appraisal Panel by a patient representative. Both forms of patient evidence provided unique

Table 27.2 Flash glucose monitoring

Topic	Healthcare sector	Mechanism of patient involvement	Published	Guidance outcome
FreeStyle Libre flash glucose monitoring for the management of diabetes	Primary care	1st HTA: Identification of patient aspects from clinical effectiveness literature search	2018	Not recommended
		2nd HTA: Full literature search for patient experiences and perspectives; patient/carers submission received from diabetes Cymru	2021	Recommended for routine adoption

insights to the challenges of living with diabetes and particularly highlighted the high personal and social costs of finger-prick testing for glucose levels. The burden, fatigue, social isolation, stigma and impacts on family and work relationships associated with finger-prick testing were greatly reduced with the use of flash glucose monitors. The inclusion of this testimony from patients in the evidence appraisal, and the personal accounts presented at committee, helped members to see the full benefit to patients of these devices. Along with new clinical and cost-effectiveness evidence, the result of the appraisal was such that the 2021 guidance recommended routine adoption (Health Technology Wales [2021a](#)). This result was welcomed by the patient community. See Table [27.2](#).

27.3.2 Example 2: Contact X-Ray Brachytherapy

As Wales has a range of cancer patient organisations, PPISG recommended pursuing a Patient/Carer Submission for contact X-ray brachytherapy (CXB) in rectal cancer (also called Papillon technique). HTW invited several cancer patient organisations, but most were unable to contribute. During searching, a patient support group advocating for the use of CXB, called the Papillon Patient Support Group, was identified and approached for a Patient/Carer Submission. The submitted form primarily reported clinical and economic outcomes and was used to help inform the clinical and cost-effectiveness evidence but could not be used as patient input. However, the Papillon Patient Support Group website contained detailed patient testimonies that included valuable insights on patient values, experiences with being diagnosed with cancer and having the CXB treatment. After gaining permission, HTW used these patient testimonials to write a summary report which was included in the appraisal in the place of the Patient/Carer Submission form. Additionally, TENOVUS (a cancer research charity) reached out to HTW to ask if they could be involved. HTW consulted with them, and identified that an important aspect, not yet captured in the evidence, was the impact of a stoma on a person and their family. An

Table 27.3 Contact X-ray brachytherapy

Topic	Healthcare sector	Mechanism of patient involvement	Published	Guidance outcome
Low energy contact X-ray brachytherapy (CXB) for the treatment of early stage rectal cancer (Papillon)	Secondary care	First approach: Patient/Carer Submission form	Not used	
		Adapted approach: Patient testimonials; patient survey conducted with TENOVUS (medical research charity)	2024	Recommended for patients who are suitable for surgery

online survey, adapted from the HTW documents, was created with the purpose of capturing people’s experiences with cancer and stoma bags. Responses were gathered and anonymised by TENOVUS who submitted this information to HTW, and it was then written up as part of the appraisal. The patient testimonies and the survey results showed how significant an impact a stoma can have on a person’s life, their ability to take part in activities, and their relationships, and how this can be avoided by having the CXB treatment. This evidence made a strong impression on the appraisal committee. The results of this appraisal recommend CXB for people who are fit for surgery (Health Technology Wales 2024). This has been well-received and a paper on this appraisal has been submitted for publication (see Table 27.3).

27.3.3 Example 3: STrategies for RelaTives (START)

PPISG recommended pursuing a Patient/Carer Submission from the Alzheimer’s Society for this intervention to support carers of those with dementia. The Alzheimer’s Society proposed a different approach using their Dementia Voices network that had been developed during the COVID-19 pandemic. HTW was invited to take part in two online focus groups that met regularly; one with people with dementia and their families and one specifically for people who care for people with dementia. Additionally, the Alzheimer’s Society proposed that a survey could be developed and circulated to their wider patient network to gain further evidence to supplement the focus group findings. HTW agreed to undertake the analysis of the results of the questionnaire and focus groups and produce a report, which the Alzheimer’s Society reviewed and approved (Table 27.4). This report was included in the appraisal. It was during this HTA that HTW adapted the patient involvement tools so they would be appropriate for use in social care. The patient evidence highlighted the challenges of caring for a relative with dementia and the substantial impact it has on the carer’s own health and wellbeing. Carers are often alone in managing the care of their relative while also having to continue to look after the rest of their families, and often are unable to process their stress, sadness and grief

Table 27.4 Strategies for relatives

Topic	Healthcare sector	Mechanism of patient involvement	Published	Guidance outcome
STrAtegies for RelaTives (START) intervention to improve the mental health of carers of people with dementia ¹²	Social care	First approach: Patient/Carer Submission Adapted approach: Patient focus groups Patient survey	2021	Recommended for routine adoption

for a long time. This was discussed at great length during the appraisal committee, and the importance of offering continued support, even if the caring has stopped, was heavily stressed. The result of the appraisal was the recommendation that START should be provided for all carers of people with dementia (Health Technology Wales [2021b](#)).

27.4 Discussion

When deciding how to conduct patient involvement in HTA, there are many considerations to keep in mind, but among the most important is the need for a consistent and evidence-based approach to ensure fairness and equity for all potential collaborators. The use of tools not only directs the course of the participation and research by aiding decisions but also, provides a clear justification and a record for decisions and actions undertaken. However, even with the use of well-established tools, there is still a need for flexibility and collaboration in the pursuit of patient involvement.

Despite detailed knowledge of the health technology and its potential impacts on patient populations, it is never possible for HTA organisations to be fully aware of what patients value, how patients of specific communities prefer to be addressed, how patients prefer to interact and how to optimise questions to adequately capture all potential insights patients may have. A patient organisation often has this knowledge, as well as other unique insights into the communities they support, and often know patients, their families, and situations in detail. Therefore, they are aptly placed to bridge the gap between HTA organisations and patient communities and can offer unique insights into how HTA organisations can best gather the information they need for appraisals. This significantly improves the quality of patient input and evidence gathered while minimising the impact on the patients themselves.

The value of contributing to HTA is something that patient organisations and their associated patient networks appreciate. However, while patient organisations

want to support bringing patient expertise into HTA, they sometimes are not able to do so using traditional involvement methods or approaches. Lack of time, resources, understanding, and confidence are the most often reported barriers. Giving patient organisations extra support such as offering meetings, writing up reports, organising data, etc. can ensure that patient organisations are able to take part in an appraisal where otherwise they would not. This encourages patient organisation participation without applying undue pressure, while improving their knowledge of HTA and health technologies and building trust. In our experience, this increases their ability and willingness to take part in future appraisals. Included with this is recognition on the part of the researchers that there may be some emotional aspects to manage, particularly if discussing sensitive health concerns and experiences during interviews or focus groups. Adequate preparation, including the provision of quiet spaces, giving participants an exit route, and providing follow-up support, are key to managing emotional aspects effectively.

Lastly, recognising that plans may need to change during participation can ensure that patient involvement takes place even when difficulties arise. It is possible for alternatives to be explored or for arrangements to be made to accommodate challenges as they develop. These flexible, but structured, approaches improve the experience of the patient organisation, the HTA outcomes, and the quality of the patient input and evidence received.

However, flexible approaches to patient involvement cannot be undertaken without support from the top level of an HTA organisation. Working to such a process requires dedicated patient involvement resources, such as full-time patient involvement staff, the expertise and guidance of advisory groups such as the HTW PPISG, support from all members of the HTA team, financial backing, commitment from the appraisal committee to consider patient inputs and feedback from all those involved to support process improvement. Patient involvement is an organisational commitment that is most successful when its value is understood, and its efforts are supported by all stakeholders. It may not always be possible for an HTA organisation to support individualised, flexible approaches for patient involvement, and HTA resources need to be taken into consideration when constructing patient involvement processes. In this situation, there should be careful prioritisation of topics that are particularly challenging and would most benefit from patient involvement, rather than using a one-size-fits all approach.

It is also important that roles and responsibilities are clearly defined and agreed at the start of participation. HTA organisations need to be transparent about what they can or cannot help with, the information being sought, what is not relevant to the appraisal, and the potential outcomes of the appraisal. This helps manage patient organisation and patient expectations to avoid disappointment or misunderstanding.

27.5 Conclusion

It is important to establish a clear process for patient involvement within an HTA process, but for non-medicine and social care technologies that cover a wide range of interventions, this needs to be shaped for each HTA topic and setting. This includes flexible approaches to working with patient organisations to help obtain patient input that would otherwise not have been available. Participation with HTW has been more successful since the introduction of these changes. As a result, patient organisations find these processes to be more worthwhile. Flexibility ensures that participation is proportionate and suited to the available capacities of patient organisations and capabilities of patients (taking account of limitations that their condition or needs may cause) a fully collaborative experience, responsive to challenges and changing circumstances, valuable to all stakeholders, appropriately planned, transparent, and accountable.

HTW is committed to achieving excellence through continual improvement. There are several areas where HTW's patient involvement process could continue to expand, but these need to be discussed with patient organisations to ensure further burden is not placed on them, such as:

- Including patient organisations in the discussions related to the Mechanism Tool
- Engaging patient organisations in the scoping and protocol development for topics
- Furthering efforts to feedback to patient organisations who engage with appraisals
- Furthering the evaluation of the impact of patient involvement in HTW Guidance and adoption of HTW Guidance
- Development of training for patient organisations that meets their needs
- Patient involvement in other HTW activities such as topic identification and audit.

Patient involvement would not be possible without the dedication and hard work of patient organisations and their networks. Their commitment to representing patient interests, to advocating for patients, and working with researchers and HTA bodies, is at the heart of successful participation. HTW is proud to have worked with, and learned from, such excellent organisations to which we owe our thanks.

Acknowledgements Alzheimer's Society, Diabetes Cymru, TENOVUS, The Papillon Patient Support Group, Members of the HTW PPISG 2019–2023: Louise Baker, Kiana Collins, Catherine Evans-O'Brien, Emma Hughes, Sian Jones, Alan Meudell, Sarah Peddle.

Funding Statement HTW is funded by the Welsh Government. No additional funding was provided for writing this chapter.

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Chapter 28

Perspectives

from Low- and Middle-Income Countries

on Patient Involvement in HTA



Debjani Müller, Anke-Peggy Holtorf, and The PI-in-LMIC Group

28.1 Introduction

According to the 1978 *Alma-Ata Declaration on Primary Healthcare*, individuals and communities have the right and duty to be involved in the planning and delivery of healthcare services both collectively and individually (Hall and Taylor 2023). Active participation or co-creation can help identify and address enablers and barriers to individuals' compliance to treatment programs or facilitate communication (Arumugam et al. 2023). However, despite critical demand for improving healthcare accessibility and health outcomes in low- and middle-income countries (LMICs), patients, citizens,¹ and carers are rarely consulted or encouraged to participate in health policy changes or decision-making processes. For example, a scoping review examining strategies for engaging patients and the public in health systems and communities in Sub-Saharan Africa concluded that patient

¹ A person who is a member of a particular country and who has rights because of being born there or because of being given rights, or a person who lives in a particular town or city (Source: Cambridge Dictionary 2025a).

The PI-in-LMIC Group at HTAi: The members of the PI-in-LMIC Group are listed in the acknowledgement.

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involvement in health service design, research, or technology value assessment was mostly superficial or tokenistic, rather than strategic (Ankomah et al. 2021).

Meanwhile, in high-income countries a trend exists towards involving patients and carers as active participants, advisors, collaborators, and members of decision-making bodies in healthcare systems in different aspects, including health research, policymaking, governance, regulation, and training of health professionals (Vanstone et al. 2023). Empirical evidence suggests that public involvement in healthcare planning and implementation may improve the health system performance and ultimately, health outcomes (Cook et al. 2019; Simkhada et al. 2021; de Sousa et al. 2019; Miah et al. 2021; Makaula et al. 2019). Active stakeholder involvement in shaping healthcare policy and decision processes serves to recognise specific needs and contextual conditions of different community segments, particularly those of socially disadvantaged groups such as women, children, elderly, adolescents, indigenous populations, and ethnic communities (Luxford et al. 2011).

A World Health Assembly (WHA) resolution, WHA77 (World Health Assembly 2024) recognises the critical role that social participation can play in fostering mutual respect and trust, which in turn enhances the resilience, responsiveness, and equity of health systems. It calls for the (a) strengthening of public sector capacities to facilitate the design and implementation of meaningful social participation, (b) allocation of adequate and sustainable public sector resources to support effective social participation, (c) facilitation of capacity strengthening for civil society to enable diverse, equitable, transparent and inclusive social participation; and (d) support of related research, piloting projects/programmes, and their monitoring and evaluation to facilitate implementation of social participation. Countries are obliged to report progress biannually until 2030.

Another, WHA resolution, WHA67.23 (World Health Assembly 2014) requires all countries to introduce health technology assessment (HTA) processes in their national health systems. The HTA definition specifies that the "... wider implications for the patient, relatives, caregivers, and the population" (O'Rourke et al. 2020, p. 188) are to be included. Among LMICs, however, the extent to which HTA exists and is used to inform healthcare decision-making varies considerably, and even more so the provision for patient involvement or patient and citizen/community² involvement. This book promotes a definition of patient involvement in which there are two complementary pathways that are ideally used in parallel: participation of patients in HTA processes, and research on patient needs, experiences, or preferences that can contribute patient-based evidence to HTA (Chap. 1). However, access to data, methodologies, and capacity for research into patient aspects (Chap. 3) in LMICs is limited.

A pilot study (Holtorf et al. 2021) on the state of HTA and patient and citizen/community involvement in HTA in Brazil, Indonesia, Nigeria, and South Africa compared specific patient and citizen involvement pathways. Involvement

²People living in one particular area or people who are considered as a unit because of their common interests, social group, or nationality (Source: Cambridge Dictionary 2025b).

frequently remained ad hoc, lacking a specific process in place, depending upon the existence, capability, and capacity of advocacy groups in selected disease areas. Collaboration with global patient and citizen/community involvement initiatives using adapted tools and guidance—for instance, by the health technology assessment international (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) or the first and second ed. of this book—for local use could accelerate implementation of patient and citizen/community involvement in HTA and thus, the decision-making process. For instance, the Brazilian HTA body includes patient representatives in its committees responsible for evaluating the incorporation of new health technologies into the public healthcare system (Carvalho et al. 2019; Holtorf et al. 2021; Chap. 20).

Starting from a multi-stakeholder discussion process, this chapter aims to further reflect on the current state of patient involvement in HTA in LMICs, including its values and barriers and possible strategies for improvement.

28.2 Strategies for Informing the Perspectives on Patient Involvement in LMICs

Given the different degree and characteristics of HTA in LMICs, the limited level of patient involvement and a shortage of relevant publications, a collaborative initiative of the PCIG and the HTA in Developing Countries Interest Group (DCIG) of HTAi, a Patient Involvement (PI)-in-LMIC³ Group was formed to collate information with a diverse group of experts with knowledge and prior experiences with HTA and patient involvement in their respective settings. This group comprising of patient representatives, HTA researchers, health policy experts, and health services researchers from various LMICs worldwide was formed through a call for participation disseminated via the HTAi Review and existing mailing lists of the PCIG and the DCIG.

In January 2024, using a preparatory survey, these participants were asked for their experiences with HTA and patient involvement in HTA in their respective countries. The survey results, comprising inputs from 20 respondents, were distributed to all survey participants and other stakeholders who had registered for one of the two workshops about patient involvement in HTA in LMICs. We scheduled two workshops to accommodate different time zones and ensure suitable timing for all participants. Participants added their interpretation, views, and critical commentaries to the 12 high level value aspects proposed through the survey.

In the workshops (web-based, using the MIRO[®] collaboration platform), the results from the survey on the value of patient involvement and its barriers were further detailed and scrutinised. Subsequently, the participants started to ideate possible interventions to address and overcome the barriers.

³PI-in-LMIC is the Patient Involvement in Low and Middle Income Countries Group within HTAi. The members are listed in the acknowledgements.

28.3 Value of Involving Patients in HTA in LMICs

All PI-in-LMIC Group members confirmed that they saw value in involving patients in HTA for a range of reasons as described in Table 28.1. The most significant benefit was that patients have unique knowledge that is of relevance to HTA due to experiencing the consequences of the disease and using health technologies in their daily life. The consideration of patients' experiences and needs was perceived to improve decision quality and equity. In certain countries, such as Brazil, participation is a legal right of patients, and perceived to increase transparency and hence, accountability of authorities.

Table 28.1 Rationale for patient involvement in HTA in LMICs^a

Rationale	Justification
With experience of the consequences of the disease and technologies, patients have unique knowledge of relevance to HTA	• Real-world experience of living with the disease, how it was diagnosed, and using treatments in the living and care context • Feasibility of treatment algorithm or relevance of clinical study design: Therapies that require unacceptable compromise from the patients may not be used to their full potential (leading to foregone opportunity to improve the health of the patient and unnecessary waste in the healthcare system).
Better decision quality and improved equity	• Knowing the variety of the needs and priorities of patients may lead to decisions being more relevant for the patients concerned by the decision • Examples for negative impact on equity in LMICs is discrimination in access related to ethnicity, gender, age or geography
Patients have a right to participate in the process if decisions are made that affect them	• As ultimate beneficiaries, patients should be consulted for every process relevant to their care • For many health technologies, it is the patient who makes the final choice of using it and, in many LMIC settings, of paying for it, at least partially.
Authorities (or fund-holders) must be held accountable to spend the scarce resources cost-effectively	• Increased transparency about processes and decisions related to reimbursement/financing of technologies allows more means to hold authorities accountable or appeal • Efficiency and sustainability of outcomes and the healthcare system can only be achieved with accountability for the process and results of HTA, supported through practicable measures that are consistently applied.
Improved process and decision legitimacy	• Giving patients the opportunity to participate in the assessment and appraisal process together with other stakeholders may improve legitimacy of the results from the patient perspective • Enhances inclusive and person-centred approach (patient, user, beneficiary) in healthcare decision making.
Enhanced transparency through public documentation and reporting	• For effective involvement, it is necessary to document the processes, evidentiary bases, reports, and results. This leads to more overall transparency and accountability for HTA (see also 'accountability' above).

(continued)

Table 28.1 (continued)

Rationale	Justification
Value to patients is an important part of HTA	• A (patient-facing) healthcare intervention that does not meet any unmet need of patients and does not improve patients' health outcomes or ability to live with the disease is unlikely to deliver value to the healthcare system • Value to patients should be considered consistently throughout the HTA lifecycle, starting already with horizon scanning, topic selection, or research scoping • For technologies that increase value to other stakeholders, it must at minimum be shown that they do not negatively impact value to patients (e.g. through worse health outcomes).
Improved health literacy of patients and the patient community	• Preparing to participate in the HTA and healthcare decision processes, improves the knowledge of the processes and the understanding of the disease or health technologies • The positive effect on health literacy can be multiplied through the patient organisations • Fosters a sense of shared responsibility and ownership
Shape and implement priorities for allocating healthcare resources	• Especially in LMIC settings, decisions require trade-offs and patient organisations can play an important role in optimising the mix of accessible health technologies through participating in the decision process and co-creating the solutions
Better acceptance and implementation of the HTA recommendations	• HTA decisions cannot always satisfy everybody. Participatory processes that include high transparency and good communication (reporting, explaining rationale for decision) can help to achieve a better acceptance by all stakeholders. Patient organisations are more co-operative if they understand and can accept the rationale for decisions • In addition, they can help the implementation of such decisions by communicating with the affected patient populations or serving as intermediaries between stakeholders
Reduce uncertainties through better interpretability of evidence	• Patient input can sometimes help the researchers or appraisal committees to better understand the relevance or importance of certain aspects, especially in LMIC settings, which are not represented in the clinical studies • Real word data that were generated in the local environment can strengthen/complement patient input
Improved patient adherence with therapies	• Better understanding and acceptance may improve the willingness to adhere to the therapy • Patient involvement in HTA can help to deprioritise therapies that are unlikely to be adhered to by patients because, for example, they don't fit into the context of living or culture

HTA health technology assessment, LMIC low and middle income country

*The sequence of the rationales in Table 28.1 was determined by the importance that was attributed through a ranking exercise

However, some enabling factors need to be considered, which are categorised as essential, beneficial, or aspirational conditions in Table 28.2 according to their level of urgency.

It is essential to establish meaningful patient involvement through transparent processes that evolve through feedback and experience. In addition, patients being involved should receive adequate information about the HTA, HTA processes, the purpose of their participation, and the technology under review. To enable mutual learning, it is desirable that participants understand their contribution to HTA

Table 28.2 Enabling factors for the expected rationale for involving patient in HTA in LMICs as derived from the responses of workshop participants

Essential	Desirable	Beneficial	Aspirational
• Create patient involvement processes that learn and can be improved based on evaluation and feedback • (Patient) stakeholders have a clearly defined (published) and meaningful role in the process • Patients receive adequate information about the HTA, the processes, the purpose of their participation, and the technology under review • Address risk of conflict of interest transparently without restricting likelihood of participation • Confidential proprietary data/information are handled appropriately • For public expenditure decisions, recognising value to patients is in balance with the value to the public	• Patients receive feedback on how their input was used in the assessment and in the results • Legal framework for patient participation • Evaluation of patient involvement (impact and process) is undertaken in the local context • Transferability and adoption of HTAs across borders for LMICs are carefully deliberated • Increase representation and activation of the patient community through local networks/ organisations	• Practicable methods for capturing representative patient perspectives and including them in the HTA process in LMICs • HTA objectives include accountability to patients • Frameworks for reporting and evaluating patient involvement in HTA must be established and agreed upon by the local stakeholders and policy makers • Well-designed effective approaches for communication and knowledge translation with the patient stakeholders (e.g. co-creation) • Examples are published that demonstrate the broader impact of patient involvement	• Overcome ‘paternalistic’ decision structures and empower patients to participate in healthcare decisions • Value-based decision making, and participatory processes become part of the culture • Methods allow for reflecting the full range of perspectives, experiences, and priorities • Patient participation in the deliberation, the formation of the HTA recommendations, and the decision processes • HTA for all: Addressing all patient populations and sub-populations (including public, private, and self-coverage) • Multi-stakeholder dialogues foster shared understanding of the respective perspectives and responsibilities in the implementation of the HTA recommendations

HTA health technology assessment, *LMICs* low and middle income countries

processes, content, and output. Other enabling factors include legal frameworks, representativeness, activation of the patient community, and well-designed communication and knowledge translation.

Although health outcomes have improved in LMICs in the past decades, shifting health needs and growing public expectations place increased pressure on health systems for providing more access to more healthcare solutions (Kruk et al. 2018). This calls for health systems, that respond to changing population needs. One channel for improving quality and proximity is to involve patients in their own healthcare decisions. Patient empowerment not only supports informed choices but also improves health literacy and therapy adherence (Wale et al. 2021). Involving patients and citizens helps to prioritise services that are culturally sensitive and tailored to their specific needs and preferences. In LMICs, the cognisance of traditions, beliefs and practices may help to promote health-seeking behaviours and adherence (De Ver et al. 2011). For instance, the Malaysian HTA body (MAHTAS) (see Box 28.3) invited a public representative for an assessment of prostate cancer screening whose input shed light on the program acceptability and perceived impact on society and patients (Kamaruzaman et al. 2021).

28.4 Barriers and Challenges for Patient Involvement in HTA in LMICs

PI-in-LMIC Group members identified practical challenges in the implementation of patient involvement based on their individual experiences in their respective country or jurisdiction (Fig. 28.1). The findings from the PI-in-LMIC Group correlate with a report from the Central and Eastern European countries, which exposed 25 potential barriers originating from either HTA bodies or the patient community (Dimitrova et al. 2022). Some of these barriers are especially common in LMICs, such as fragmentation of healthcare systems or a limitation of HTA to decisions regulating access to health technologies only for sub-populations.

Other barriers are less specific to LMICs. For example, patient perspectives are sometimes perceived as merely anecdotal and biased sources of evidence, or patients and the public are considered unqualified to make substantial contributions to the HTA process (Abelson et al. 2016; Drummond et al. 2020; Gauvin et al. 2011). Furthermore, concerns remain that patient and citizen/community involvement may not be sufficiently 'representative' and will bias decisions by individual interests taking precedence over fairness (Abelson et al. 2016; Kamaruzaman et al. 2021). The lack of formal recruitment criteria and processes (Kamaruzaman et al. 2021) or cultural tensions within the HTA bodies (Abelson et al. 2016; Drummond et al. 2020) may further limit the practice of patient involvement.

The implications and underlying reasons of such barriers, or how they could be addressed, must be considered in the context of the respective jurisdiction, or setting. In general, barriers are related to different levels or determinants of the healthcare system, including policy-level issues, the HTA organisation or processes, patient organisations, country culture or its value framework, or additional factors

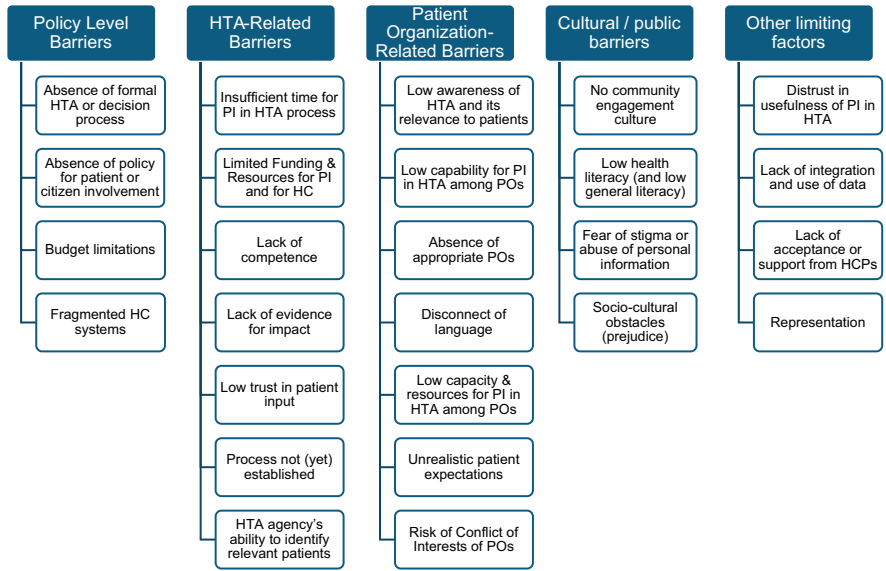


Fig. 28.1 Barriers for patient involvement in HTA as identified by the members of the PI-in-LMIC Group. *HC* healthcare, *HTA* Health Technology Assessment, *PO* patient organisation, *PI* patient involvement, *HCP* healthcare professionals

such as a general distrust in the competency of patients, inadequate data integration, or low acceptance or support from stakeholders. Addressing these barriers necessitates good knowledge of the determinants and stakeholders and, most likely, requires bridge-building for better consensus and collaboration.

28.5 Possible Initiatives to Foster Patient Involvement in HTA in LMICs

Table 28.3, compiled from the workshop inputs, lists possible actions for addressing the previously identified barriers. Like barriers, these are classified by their target level, i.e. change at policy level, in HTA processes and preparedness, preparedness of patient organisations, or public initiatives for cultural change and awareness building. The interventions in this list are neither exhaustive nor to be initiated simultaneously. The purpose of this list is to serve as an idea ‘menu’ of possible interventions, which could be considered and adopted collaboratively ideally by multi-stakeholder consortia that aim to promote patient involvement in HTA in their countries.

Examples for such initiatives and approaches serving as ignition points already exist in LMICs around the world as described in other chapters of this book (Chaps. 21 and 22, Boxes 28.1, 28.2, 28.3, and 28.4). Sharing, exchanging, and publishing experiences—permitting learning across borders—can stimulate the overall knowledge and tools for activities that are especially effective for fostering participatory processes in LMICs.

Table 28.3 Possible initiatives or approaches to address current barriers for patient involvement (PI) in HTA obtained from responses of participants

Measure	Action details	Barriers addressed
Policy level improvement options		
Establish formal frameworks for HTA	• If not existing yet: formulate a strategic HTA act and support its realisation with proper resources and budget • Alignment Ministry of Health, policy, funders (roundtable discussions, regular information, feedback loops, progress measurement) • Identify high level oversight committee: Policy makers and leaders with sufficient understanding	• No HTA/legal framework • Lack of HTA adoption
Champion for participatory HTA	• Identify and support 'champions' for participatory HTA in the country	• Lack of HTA adoption
Budget for HTA and patient involvement (PI) in HTA	• Reserve a budget for HTA body that allows a meaningful conduct of HTA (and PI) • Dedicated fund raising for establishment of HTA and PI that fits the context (e.g. on regional level) • Funding mechanisms of governments to support PI and capacity building	• Lack of budget • No HTA
Data integration	• Formal act to monitor and store patient data across the country	• No local national RWD
Strengthen HTA by building on WHO leadership	• Building on the WHA declaration on HTA (World Health Assembly 2014) to foster HTA implementation through cross country collaboration, regional networks, and centres of excellence • International drive for more participatory processes e.g. WHA resolution (World Health Assembly 2024)	• Absence of patient/citizen/ community involvement (PCI) culture • Limited political will
Patient and citizen involvement framework	• Foster participatory processes in general in social policies. Consider how to address social dynamics and inequities (e.g. vulnerable populations, gender, employment) in PI/HTA strategies	• Absence of PCI culture • Fragmentation of HC
Align decision stakeholders	• Communication and dialogue for better alignment related to HTA and patient and citizen involvement	• Fragmentation of HC
Regional centres of excellence	• Identify and foster regional resource centres for providing methods for patient and citizen/community involvement in HTA • Foster the formation of patient organisations and umbrella organisations in regionally relevant disease areas, potentially through engaging community groups	• Absence of PCI culture • Limited political will • Lack of expertise

(continued)

Table 28.3 (continued)

Measure	Action details	Barriers addressed
HTA related improvement options		
Increase public awareness	• Publish methods, learnings, and specific assessment aspects to increase awareness and interest in the academic environment • Publish HTA reports on HTA organisation website, so that they are accessible to public (e.g. summary reports in plain language) • Present HTA organisation in public media and in healthcare related conferences	• Low awareness of HTA
Repository/pool of patient experts	• Stakeholder mapping and creation of a pool of patient experts or organisations who are easily accessible for consultations or other activities related to PI in HTA • Build and promote up a repository of relevant and capable patient stakeholders in country	• HTA body's lacking ability to identify relevant patients
Dedicated PI functions (staff)	• Dedicated staff for PI activities are responsible for building up and improving processes, championing PI within the organisation, and support HTA researchers in all aspects related to PI	• Lack of competence and resources for PI • Processes not established
Build HTA capabilities and capacity	• Train young researchers and engage them in HTA (e.g. exchange programs, teaching HTA at university) • Train HTA technicians and staff for PI in HTA in their organisation, processes, reporting, evaluating, and their responsibility related to HTA • Education and awareness of expected benefits of patient and citizen/ community involvement • Information to improve capabilities of patients to give input in HTA and to manage expectations	• Lack of competence for PI

(continued)

Table 28.3 (continued)

Measure	Action details	Barriers addressed
Create an enabling HTA culture and processes	• Leadership initiative to change HTA culture and improve attitude towards PI • Create frameworks and methodologies that facilitate patient and stakeholder participation in HTA in terms of expectations, opportunities, roles, methodologies, and processes and that are adapted to the needs/limitations of patients (e.g. materials, participation schedule, infrastructure, etc.) • Work with patients to develop the frameworks and/or processes (e.g. stakeholder workgroup, stakeholder advisory board, PI committee)	• Lack of trust • Resistance • Resource management • Processes are not patient friendly
Stepwise adoption and implementation	• Both patient organisations and HTA researchers need to learn to work effectively with each other. Therefore, starting with limited steps for engagement and creating a learning organisation and exchange will help both patient and HTA stakeholders to learn how to work with each other while feeling comfortable with the changes. This could mean to start with certain aspects (e.g. written submission opportunity or consultation on report) and expand step by step based on experience	• Lack of evidence for impact • Low trust in patient input • Process not (yet) established
Strengthen communication	• Improve communication and interaction and networking by HTA organisation with patient organisations and other HTA stakeholders • Feedback to patient stakeholders on learnings from PI in HTA • Announce opportunities for PI early, e.g. to umbrella organisations	• HTA body's ability to identify patients • Low awareness of HTA • Time constraints
Evaluation and learning process	• Improve evaluation of process quality and impact of patient participation in the HTA process to understand which methods work well and what needs to be improved	• Lack of evidence for impact • Low trust in patient input • No established process.
Adapt to LMIC context	• Strategies and processes need to resonate in the respective LMIC context • Adaptation and development of processes to be applicable and meaningful in the local context	• Processes are not suitable to local context.

(continued)

Table 28.3 (continued)

Measure	Action details	Barriers addressed
Accessible language	• Creation of target formats that allow for using language that is accessible to the patient stakeholders	• Disconnect between HTA and patient language.
Patient Organisation related improvement options		
Training for patients	• Adequate training for motivated patients (or people) on topics related to HTA and healthcare decision making by: – Umbrella PO or PO network – Public health university or institute – HTA organisation • Train patients for participation in the HTA or decision process • Train patients on how to set up POs in their countries • Build up a resource Centre (books, publications, good practices, case studies, etc.) that is accessible to interested patients	• Low capability for PI in HTA among POs • Distrust in patient capabilities
Proactive preparation for PI in HTA	• Proactively plan for strategic participation in HTA at PO level (which technologies, how, who) – Resource planning – Build a communication and collaboration base with HTA organisation – Identify and motivate members to participate	• Low capacity and resources for PI in HTA among POs • Time constraints • Lack of PO readiness • HTA not able to find patients
Build up channels for participation	• Liaise with government officials at district and state level to generate awareness	• Low willingness or openness for PCI
Seek funding	• Apply to national/international funding initiatives for PO in LMICs – Liaise with other supportive stakeholders or potential collaborators (academic researchers, HTA, clinical)	• Lack of resources/funding
Build patient organisation networks	• Develop a network to support POs on education and costs and capacity building – Efficient use of resources – Cross-PO learning and support • Liaise with other patient organisations with experience/interest in HTA to share and learn from each other • Discussion/collaboration on regional level between POs	• Low capability for PI in HTA among POs • Low visibility or recognition of POs as competent partners
Cross-stakeholder liaison	• Liaisons across stakeholders in HC to increase awareness and collaborate (patients, clinicians, researchers, policy makers)	• Low general awareness of HTA • Distrust in usefulness of PI in HTA

(continued)

Table 28.3 (continued)

Measure	Action details	Barriers addressed
Strategic independence	• Demonstrate independence by diversifying the organisation's financial support and having a transparent framework to cooperate with the industry to create transparency and increase credibility	• Risk of conflict of interest
Patient needs/experience data	• Proactively understand PO members (e.g. regular surveys) – Identify patient needs and patient experiences among members – Identify specific communities with special needs	• Data are perceived as stronger than 'words' • Time constraints • Distrust in usefulness of PI in HTA • Lack of data
Cultural/public improvement options		
Target socio-cultural context	• Transferability often depends on socio-cultural acceptability • Be aware of the importance of the socio-cultural context when trying to change behaviours and traditions or to transfer learnings from other environments	Low adoption in local context
Awareness campaigns	• Increased information to the public on the purpose of HTA and role in healthcare access for people in country – Public media (print, tv, audio) – Social media – Adapted to the country's socio-economic context • Showcase case examples to show relevance to all parts of the population	• Low adoption in local context • Absence of appropriate POs
Education system	• Integrated health and healthcare system related topics into the education system Public health, relevance to individual health, and personal responsibility as school subjects Integrate subject of public health in related university programs (e.g. medicine, nursing, education)	• Lack of cultural preparedness for PCI
Foster dialogue on all levels	• Public events with patients, carers, and public to improve cultural openness for dialogue and to understand local barriers, needs, and priorities	• Lack of cultural preparedness for PCI

CoI Conflict of Interest, *HC* Healthcare, *HCP* Healthcare provider, *HIV/AIDS* Human Immunodeficiency Virus/Acquired Immunodeficiency Syndrome, *HTA* Health Technology Assessment, *IT* Information Technology, *LMIC* Low- and Middle-Income Country, *PCI* Patient and Citizen/Community Involvement, *PI* Patient Involvement, *PO* Patient Organisation, *RWD* Real World Data, *RWE* Real World Evidence, *WHA* World Health Assembly, *citizens* residents of a country or geographic area, *community people* having common interests because of being part of the same social, regional or national group

Box 28.1 Patient Involvement in HTA in Chile

In Chile, the Ministry of Health (MoH) uses HTA to inform the update of one of the national (universal) health benefit plans for the country focused on high-cost medicines and technologies. Two representatives of patient groups are selected through a participative process to attend the deliberative commission, which makes the final recommendation to MoH (Chap. 20).

Patient involvement in HTA contributes to:

- (a) higher legitimacy of the process; and
- (b) a different perspective, which may affect the value measurement (e.g. health benefits) or modify previous perceptions regarding health-related issues.

Example of patient and citizen involvement

- The Ricarte Soto Law (the legal foundation for patient participation in the HTA process for high-cost drugs) was created in response to the social movement ‘the march of patients’, organised by patient organisations to request a systematic HTA process with a deliberative decision discussion including two patient representatives (Ministerio de Salud 2015, Chap. 20).

Box 28.2 Patient Involvement in HTA in Brazil

Decisions at CONITEC, the HTA agency in the MoH, for both the public and private health systems are made at multi-stakeholder plenaries of the National Health Council (representing patients, healthcare partners, POs, patient and citizens advocacy groups, nongovernmental organisations, healthcare professionals, and the health industry sector). A patient (as a member of the National Health Council) together with a panel of other experts is involved in the deliberation and the decision. Like other stakeholders, patients can initiate a technology submission. Public consultation is open to all members of the population. (Holtorf et al. 2021, Chap. 20).

Patients and citizens as the ultimate beneficiary and an end-user of the health systems have a constitutional right in Brazil to participate in healthcare expenditure, policy, and management decision making. They can influence healthcare decisions both through formal and informal pathways. The National Commission for incorporation of health technologies to the Sistema Único de Saúde (SUS, Brazilian unified health system) makes specific calls to patients to provide a statement on the technology under review to the panel.

Examples of patient involvement

- (a) Since 2002, involvement occurs several times monthly during CONITEC (public health service HTA organisation) and COSAUDE (private health

(continued)

Box 28.2 (continued)

service HTA organisation) meetings. The objective of patient and citizen/community involvement is to provide users' perspectives within the public and private spheres of the national health system. In addition, all Brazilian citizens can be involved via public consultation and testimonials (Cazarim et al. 2017),

- (b) The assessment of fast-acting insulin analogues for selection to the SUS of Brazil in 2017 comprised submissions, public consultation and involvement in the committee deliberation, and final decision. By fostering collaboration among patient associations, the medical society, and digital influencers specialising in diabetes, it was possible to reverse negative perception of the implementation (ibid.).

In some African healthcare systems, patient organisations or community health workers, who are close to the patients and their lived experience, can shape health policies or healthcare decision making by raising awareness of safety issues and the effective use of quality medicines, advocating for increased funding, improving prominent healthcare issues, or emphasising self-care or community-based care. This was one of options that was mentioned during a workshop exploring the opportunities for patient involvement in HTA in South Africa. This workshop brought together patient, regulatory, provider, and research stakeholders to form a sub-Saharan patient involvement collaboration network for advancing patient involvement across the region. Similarly, a multi-stakeholder workshop was conducted by the MAHTAS (see also Box 28.3) during the HTAsiaLink Conference 2023 to explore ways to involve patients in HTA across Asia.

Stakeholders can review and comment on HTA findings before publication, hence increasing transparency and credibility of HTA reports; likewise, Box 28.2 provides such an example from Brazil. Finally, patient organisations can also be instrumental in the implementation or communication of HTA recommendations, decisions, or guidelines as seen in the example from the Philippines (Chap. 21).

Training the patient community is a frequently proposed step to improve the readiness of the patient community for participation. Enhancing the capacity of patients through fit-for-purpose training to understand and interpret technical evidence and the use of validated tools for facilitating participation, empower patients to surpass tokenistic representation in decision-making committees.

However, there are other actions that can help improve the readiness for patient involvement in HTA. For example, Jakab et al. (2023) recommended a range of measures for improving patient involvement in HTA in Central and Eastern European countries such as education of HTA researchers, revision of HTA guidelines, resourcing, training, and budget provisions.

In addition, research-based approaches may form supportive groundwork for starting stakeholder involvement in HTA. For example, a study in the Lebanon explored the knowledge of patient organisation and payers about HTA to set the base for gaps to be addressed (Al-Harakeh et al. 2021). Likewise, the HTA body of

Uruguay recently surveyed patient organisations on the knowledge and expectations about HTA and their willingness to be involved (research in progress, personal communication). In Malaysia, the HTA body uses surveys to specific patient groups to collect information on the patient perspective (Box 28.3).

Box 28.3 Examples of Patient Involvement in HTA in Malaysia

MAHTAS (Malaysian HTA section, MoH) has the mandate to conduct HTA to inform decision-making concerning procurement, reimbursement, implementation, and disinvestment before introduction of health technologies in MoH health facilities. Patients' perspectives are captured in HTA, using surveys and other methodologies. Increasingly patient representatives:

- (a) attend HTA, and clinical practice guidelines councils
- (b) participate in the expert committee for a specific HTA topic; and
- (c) act as external reviewers.

Clearly defined processes for stakeholder involvement can help to enhance transparency and reduce uncertainties leading to better acceptance of decisions by patients. Patients can influence healthcare decisions by becoming committee members that either make decisions or provide input for the decision. Such participation should reduce direct advocacy efforts towards decision-makers.

Examples of patient and citizen involvement

- In 2020, patients were involved in HTA by responding to a survey for a Pre-dialysis Education Program for advanced chronic kidney diseases before its introduction in Malaysia. Survey findings were incorporated in the HTA which subsequently led to the launch of a national pre-dialysis education program in Malaysia taking account of patients' inputs about requirements for successful program implementation.
- In 2023, patients and carers were involved in qualitative research. A focus group for continuous glucose monitoring systems (CGMS) for diabetes in Malaysia was formed. The focus group explored patients' perceptions about CGMS considering benefits, barriers, and recommendations. The input was successfully incorporated into an HTA of CGMS and presented to the Council meeting chaired by Director General of Health in Malaysia. This HTA recommendation will inform the decision to provide CGMS to certain groups of diabetes patients.

An example of data collection driven by a patient organisation is given in Box 28.4. Next to collecting data, this patient organisation also engaged to increase the awareness on good processes for patient involvement in HTA by translating the first edition of this book into Mandarin language published by Tsinghua University Press in 2022 (Facey et al. 2022).

Box 28.4 Example from China of Influencing a Listing/Coverage Decision Through Patient Experiences Research

House086 (a lymphoma patient organisation in China) published a white paper on the demographic characteristics, experience of diagnosis and treatment, disease burden and quality of life of follicular lymphoma patients in China in 2020. The data aided in estimating the socio-economic costs associated with the condition and the results.

The research was submitted to the healthcare negotiating team and ultimately, Obinutuzumab was included in the National Reimbursement Drug List. The research was a collaboration between House086, the Jockey Club School of Public Health and Primary Care, the Chinese University of Hong Kong, and the New Sunshine Charity Foundation.

28.6 Discussion

Patient and citizen/community involvement in HTA in LMICs is still in its infancy. This may be due to culture, political structure, lack of resources, or lack of guidance from institutions and advisors who are helping to build healthcare structures for universal health coverage in LMICs and typically use forms of adapted HTA processes from their home countries. When establishing a functioning healthcare system, giving people a voice in shaping research or assessments of health technologies may be perceived by some leaders as subordinate, premature or, at worst, a threat to their academic and professional competency.

However, examples from health services research suggest that patients and citizens, as beneficiaries and users of current and emerging health technologies can effectively partner with healthcare professionals and other stakeholders to plan healthcare services and policies (Nabarette et al. 2023; Greenhalgh et al. 2019; Wicks et al. 2018). Box 28.5 provides examples of emerging accounts demonstrating potential power of patient and citizen/community involvement in LMICs.

Patients or community representatives may help to assess the available evidence about using a health technology in the context of local conditions and the lived reality of the people in the country, such as cultural, economic, geographic, or community-dependent circumstances. With this, researchers or appraisal committees may better comprehend the relevance, importance, burden, or impact of living with the disease or using the technology locally (Sehmi and Wale 2022; Wale et al. 2021). However, it must be recognised that methods of participation currently used do not yet deliver their full potential. For instance, the systematic review analysing 27 publications concluded that, despite the use of a wide range of patient and citizen involvement mechanisms, participation in priority setting and decision making, occurred with limited depth of engagement and equity considerations (Arthur et al. 2023).

Consequently, in the absence of formal ways to participate, patients (organisations) may use other means to raise their voice which may not necessarily improve equity or justice in the country's health system (see Box 28.5). In this sense, the resolution on social participation for universal health coverage, health and well-being by the WHA (World Health Assembly 2024) may help to increase the receptiveness of healthcare systems and policies for improved mechanisms of patient involvement.

Box 28.5 Informal Ways For Influencing/Changing Health Coverage Decisions as Reported by the PI-in-LMIC Group

- Advocacy, lobbying, media, social media
- Litigation
- Public protests, public speaking
- Closed-door discussions/negotiations
- Use of political democratic processes

Stakeholders including donor organisations, academic or policy advisors or health technology developers should be held accountable for healthcare quality. Yet their goals and mandates may not fully align with population needs. Transparent oversight mechanisms supported by an equity-driven HTA can mitigate this disparity. Involving communities and patients, encouraging partnership and collaboration, assessing impact on all, including vulnerable populations, or tying funding or regulatory approval, and HTA recommendations to demonstrated efforts to address various population needs could help address evolving healthcare challenges and priorities.

The PI-in-LMIC Group highlighted barriers and poorly functioning practices related to patient and citizen/community involvement. Overcoming such barriers may require LMIC-specific solutions which could be better addressed by sharing learnings on barriers and possible solutions. HTA's purpose, potential implications on healthcare delivery and outcomes, and how patients and citizens can participate in the assessment process and implementation of the recommendations or decisions must be communicated. Therein, training, guidance, and plain-language information are instrumental. However, there is no single-best participatory mechanism available. The authors of the World Health Organisation's handbook on social participation in universal health coverage (2021, p. 15) observe "context and content-dependent advantages and dis-advantages" of different participatory mechanisms. They conclude that participation mechanisms should always follow a needs-driven selection process. Or, as described in in this book (Chaps. 4 and 33), the objective for involving patients should drive the selection and shaping of the methods, means, and practices.

A culture and processes that support patient participation are lacking in many LMICs. Patient communities and organisations are often limited to participation in

HTAs relating to specific disease areas, such as some oncological diseases, HIV/AIDS, or occasionally selective rare diseases. Hence, identifying patients who can contribute is difficult. However, proactive identification and mapping of key patient and community groups interested in emerging health technologies may help to close this gap, and training patient organisations about HTA methods and local processes may support and sustain participation.

Important elements for fostering good practices and promoting trust in patient involvement in LMICs include transparency, reporting patient or community input and how it was used and evaluating what worked and what needed improvement. Additional research into patient aspects specifically in LMICs could help to highlight the particularity in the lower resource and cultural context and their relative importance in increasing resilience and sustainability in these jurisdictions. Herein, evidence of what happened, how it happened, and its impact may have confirmative effects and incentivise others to follow.

28.7 Way Forward

Cross country collaboration and regional centres of excellence can boost patient involvement by establishing and communicating case examples, fostering good practices, and capturing learnings. Identifying HTA champions in these regions, who are aware of the social-cultural requirements in addition to the clinical and economic facts, can assist establishing HTA that meets the country's context and setting and incorporates patient involvement. The fit to the local context and its capability will be a major predictor of success.

Best practices from other jurisdictions should be tested for local acceptance, feasibility, and relevance. For this, a step-by-step approach with open communication or co-creation (for example, in the form of a patient involvement committee or advisory board) with internal and external stakeholders (such as patients) can be instrumental.

Acknowledgements We would like to thank all members of the PI-in-LMIC Group for continuing to contribute to this work. The following individuals have contributed to this work through participation in the survey and workshop or consultation of the report: Aliya Naheed (Research, Bangladesh); Vanessa Pirolo Vivancos (Patient Organisation, Brazil); Andrea Brigada de Souza (HTA/MoH, Brazil); M. Sharmila A. Sousa (Research, Brazil); Dr. Manuel Espinoza S. (Research & Health Policy, Chile); Zhang Jin (Jenny) (Patient Organisation, China); Lorena de Pilar Mesa (HTA/MoH, Columbia); Samuel Agyei Wiafe (Patient Organisation, Ghana, West Africa, Africa); Denny John (Research, India); Abhirup Dutta Majumdar (Research, India); Dr. Yogesh K. Gurav (Research, India); Dr. Sita Ratna Devi (Patient Organisation, India); Dr. Leenus Taflina A E (Research, India); Alexandr Kostyuk (HTA/MoH, Kazakhstan); Dr. Nur Farhana Mohamad (HTA/MoH, Malaysia); Dr. Izzuna Mudla Mohamed Ghazali (HTA/MoH, Malaysia); Dr. Amal Yassine (Research, Morocco); Rolf Hansen (Patient Organisation, Namibia); Adeyemi Adebawale Sylvester (Pharmacist, Nigeria); Christopher Muñoz (Patient Organisation, Philippines); Sheena Jasley G. Samonte (HTA/MoH, Philippines); Adrian Goretzki (Patient Organisation, Poland); Mark Brand (Health Policy Advisor, South Africa); Lauren Pretorius (Patient Organisation, South

Africa); Maria J. Vicente-Edo (HTA/MoH & Advisor, Spain); John V Amany (Patient Organisation, Uganda); Andrea Gimenez (HTA/MoH, Uruguay); Warren Simangolwa (Consultant; Patient and citizen/community involvement expert, Zambia); David Palacios (Patient engagement expert and consultant, USA). No funding was required for the research underlying this chapter and the writing of it.

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Chapter 29

Discussion: Perspective of an HTA Appraisal Committee Chair in France



Pierre Cochhat

29.1 Introduction

Health Technology Assessment (HTA) synthesises information about medical, economic, social, organisational impact and ethical aspects related to health technology use. HTA primarily serves patients and so requires their active participation. In France, HTA is founded on principles of science, independence, and transparency, with the goal of advancing healthcare and informing the government on reimbursement decisions. It encompasses medical devices, digital health technologies, medicinal products, diagnostic and therapeutic procedures, and public health decisions. The assessment follows specific regulations and relies on specific clinical or health economic criteria (Baba et al. 2025). This chapter focuses on the appraisal of clinical effectiveness of medicines in France and the evolution of patient participation.

Patients and patient organisations (patients) are encouraged to provide input throughout the medicine's development cycle, from the design of clinical trials (company) to HTA (*Haute Autorité de santé*, HAS). To ensure objectivity, individual perspectives and emotionally driven patient inputs are discouraged. Rather than individual views, the contributions should reflect a consolidated perspective of patients, often elaborated collaboratively with other stakeholders, including other patients, health providers, and regulatory agencies (Haute Autorité de santé 2024c). However, the role of healthcare professionals within patient organisations varies and requires careful consideration, as the primary added value of patient input lies in patient experience rather than an academic expertise. In HTAs in France, patient participation is managed by the Users Engagement Council, a dedicated department within the French National Authority for Health (HAS).

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K. M. Facey et al. (eds.), *Patient Involvement in Health Technology Assessment*, Health Informatics, https://doi.org/10.1007/978-3-032-11284-2_29

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29.2 Evaluation of Medicinal Products

The evaluation of medicinal products follows evidence-based medicine principles (Chap. 1), incorporating both ‘quantity of life’ and ‘quality of life’ endpoints, tailored to the specific medication and disease characteristics (Fig. 29.1). As defined by Sackett et al., “Evidence-based medicine is the conscientious and judicious use of current best evidence from clinical care research in the management of individual patients” (1996, p. 71).

The HAS conducts medicinal product evaluation through two distinct phases. The *Assessment Phase* is a technical evaluation conducted by the Medicinal Product Assessment Office (*Service d’Évaluation des Médicaments*, SEM) and performed by approximately 40 HTA project leaders (pharmacists and physicians). It is based on comprehensive data submitted by pharmaceutical companies.

The subsequent *Appraisal Phase* is conducted by the Transparency Committee (*Commission de la Transparence*, CT), which is composed of 26 healthcare professionals (physicians, pharmacists, methodologists; 20 with voting right) and three experts from patient organisations (two with voting rights). All voting members have equal voting power and experts from a patient organisation can significantly influence outcomes in close decisions. Meetings are usually held bi-weekly (except during holiday periods).

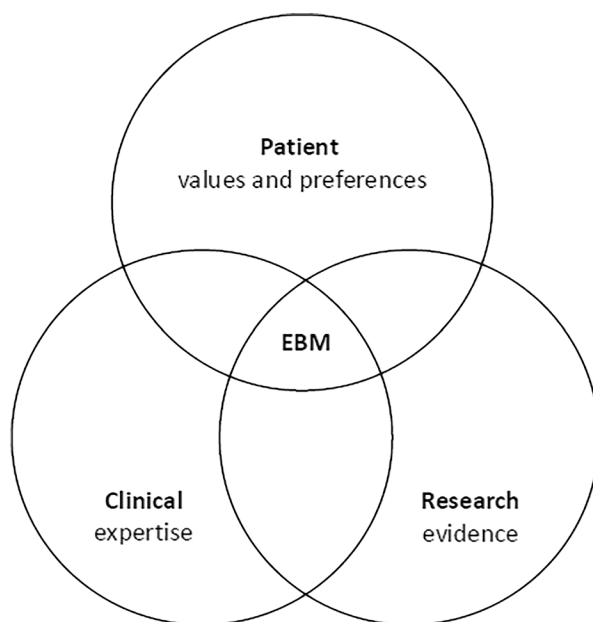


Fig. 29.1 Graphic interpretation of Sackett et al.’s (1996) concept of evidence-based medicine combining research evidence, clinical expertise and patients’ values and preferences

Under common law, medicinal product evaluation employs two key scoring mechanisms:

- The Clinical Benefit (*Service Médical Rendu*, SMR) evaluation considers five legally defined criteria: (i) disease severity, (ii) clinical efficacy and safety, (iii) product purpose, (iv) position in treatment pathway, and v) public health impact. Based on these criteria, the SMR assessment determines reimbursement. When rated as insufficient, no reimbursement is granted. A sufficient rating leads to one of three reimbursement levels: minor (15%), moderate (30%) or substantial (60%) clinical benefit. Complementary insurance may cover the remaining costs, with patients responsible for a limited co-payment.
- The Clinical Added Value (*Amélioration du Service Médical Rendu*, ASMR) assessment directly influences medicine's pricing through a five-level rating system. These ratings range from Level I, indicating major improvement, through Level II (important improvement), Level III (moderate improvement) and Level IV (minor improvement), to Level V, signifying no improvement over existing treatments. The ASMR rating serves as a key factor in pricing negotiations conducted between the interministerial committee (*Comité Économique des Produits de Santé*, CEPS) and the pharmaceutical company.

For innovative medicines, the HAS board may grant early access authorisation following CT evaluation based on four (post-marketing authorisation) to five (pre-marketing authorisation) essential criteria: (i) the benefit/risk ratio is favourable, (ii) the targeted disease must be serious, rare or disabling in nature, (iii) the medicinal product must address an established unmet medical need, (iv) there must be demonstrable urgency where treatment access cannot be delayed, (v) the medicine must show evidence of potential innovation when compared to the current standard of care.

29.3 Role of Patients in Medicinal Product Evaluation

Patient participation in medicinal product evaluation in HAS occurs in two ways: as an expert (e.g. in a committee) and as a stakeholder.

Before any medicinal product evaluation, patients are invited to submit their perspectives through an online form (Haute Autorité de Santé [2024a](#)), which is accompanied by a comprehensive guide (Haute Autorité de Santé [2019](#)) that helps patients address:

- Disease impact on quality of life for patients and caregivers
- Currently available treatments
- The medicine under evaluation (comments on proposed indication, experience with the medicine, expectations, and concerns)

- For early access applications (position on authorisation criteria and proposed patient data collection)
- Request for in-person hearing
- Summary
- Additional comments
- Methodology used.

A CT expert from a patient organisation presents this written contribution, after consulting with patients if needed. For each product, contributions from several different patient organisations are possible. The patients' report follows the project leader's general presentation and precedes the clinical experts' testimony. Patient representatives participate fully in discussions before the CT votes. Patients also help design real world evidence (RWE) studies that HAS requests, which are required in approximately 10% of cases.

For the early access procedures, patients can submit written contributions and also request in-person hearings. Their input accompanies the CT report to HAS board members for discussion and voting on authorisation. Patients also assist in designing RWE questionnaires (*Protocole d'Utilisation Thérapeutique—Recueil de Données*, PUT-RD) required for all early access applications (importantly prior to marketing authorisation); around half of all PUT-RD have generated comments from patients.

Patients can access upcoming CT session agendas, including both standard and early access dossiers. However, access to complete technical documentation is limited, due to the technology developers' confidentiality requirements to prevent premature data dissemination. The CT Executive Group meet weekly, including the president, the two vice-presidents, one methodologist and one expert from a patient organisation along with Medicinal Product Assessment Office members. With their growing experience, patients are encouraged to elaborate adequate information and contribute throughout all medical specialties to the medicinal product evaluation.

29.4 Training of Experts from Patients Organisations

The contribution of patients described above finds its origins in the continuous strengthening of user rights in France established in French law in 2002 and sometimes translated into English as a policy of 'health democracy'. The HAS included in its 2019–2025 strategy a direction entitled "Making user engagement a priority" (Haute Autorité de Santé 2022); herein, user means patients and patient organisations. As such, it is committed to "Systematising the integration of the user point of view in all methods and work" (ibid. Objectif 2.2. p. 15–16). In this spirit, the contribution of user organisations has been established as routine since 2017, among other guarantees offered for taking user expectations into account, there is also: early participation in framework notes, registration in the work program, participation in working groups, and inclusion in review groups. In this context, the patient

participation in the assessment of health technologies has been strengthened by the creation of two structures: the Users Engagement Service (*Service pour l'Engagement des Usagers*, SEU), and the Users Engagement Council (*Conseil pour l'Engagement des Usagers*, CEU). This department and deliberative body have contributed to the establishment of the user organisation contribution process. The department also engages in dialogue with the contributing organisation to support it in formulating its contribution, and in providing feedback on the final decision.

The public involvement department has elaborated, in collaboration with patients, training support for patient organisations, to explain (i) the role of HAS in the medication pathway from clinical research to market access, and (ii) each step where patients may interact with HAS (Low et al. 2023). Other training sessions are offered by patients at the French level by national union of accredited users organisations (*France Assos Santé*) or at the European level (EUPATI 2025).

29.5 Patient-Reported Outcome Measures and Patient-Reported Experience Measures

Measuring care quality is essential for defining improvement initiatives and demonstrating progress in healthcare delivery. This approach, traditionally applied as practice self-assessment by professionals, is enhanced by incorporating patient perspectives. It may come from patient submission, from some clinical trial evidence, and from specified RWE studies driven by the HAS.

Three types of measures assess patient-perceived care quality: (i) Patient-Reported Outcome Measures (PROMs) evaluate care outcomes, (ii) Patient-Reported Experience Measures (PREMs) assess the care experience and (iii) patient satisfaction questionnaires measure how well expectations are met (Chap. 3).

PROMs and PREMs serve multiple purposes in clinical practice and research, enhancing patient-provider communication, enabling personalised care approaches, and facilitating effective health monitoring. While increasingly adopted in routine clinical practice, their implementation remains insufficient.

29.6 Quality of Life Assessment

Quality of life (QoL) assessment, a crucial component of PROMs, should be integrated into both clinical trials and RWE studies.

QoL assessment is particularly significant in evaluating medicine's effectiveness. Its relevance may vary based on therapeutic context. For example, QoL impacts from severe adverse events may carry different weight in last-line versus first-line cancer treatment, and considerations like palatability of the medicine may have

varying importance between paediatric and adult populations. This reflects a concept of 'QoL dose/load'.

QoL measurement plays a crucial role across a broad spectrum of medical conditions. This encompasses cancer management, chronic conditions such as migraine and hypersomnia, respiratory diseases, psychiatric disorders, osteoporosis, etc; the assessment is equally important in dermatological conditions, neuromuscular diseases, chronic infections, and various other long-term conditions that significantly impact patients' daily lives.

QoL assessment takes a comprehensive approach, considering not only the direct impact on patients but also the effects on their family members. This includes evaluating the impact on siblings in paediatric cases, spouses and children for adult patients, carers for dependent individuals. The assessment process typically utilises two types of instruments. The first category includes generic instruments such as EQ-5D, which comprehensively evaluated five key dimensions: mobility, self-care, usual activities, pain/discomfort and anxiety/depression (EuroQol 2025). The second category comprises disease-specific instruments, such as the KCCQ for heart failure assessment (KCCQ-12 2012). Many of these tools can be used according to the clinical context, and the door should be open to innovative measures, provided they have been scientifically validated.

29.7 Public Consultation

Public consultation forms an integral part of HAS initiatives, encompassing various aspects of healthcare delivery. These consultations cover the development of clinical guidelines, best practice recommendations, and new methodological approaches. They also extend to vaccination strategies and the creation of decision support tools, such as for protecting infants from severe bronchiolitis (Haute Autorité de santé 2024b). These comprehensive public participation processes serve a vital role in ensuring that HAS's approaches and recommendations are acceptable to both the general population and specific target groups affected by these healthcare initiatives.

29.8 Expression of Disagreement

Patients may challenge medicine evaluation outcomes. Written communications raising questions or concerns always receive responses and may lead to in-person meetings. The Users Involvement Council manages these exchanges.

Patients can alert HAS to specific concerns beyond standard medicine evaluation issues. These often involve previously underestimated adverse effects of medications or classes of medicines, where patient impact was perceived to be inadequately assessed. While such alerts may not directly affect ongoing evaluations, they can

influence future practice recommendations and guidelines, as recently occurred with certain antibiotics and vaccines (Haute Autorité de santé [2024b](#)).

29.9 Patient Participation at the European Level

The European HTA Regulation (HTAR) implementation follows a progressive timeline. It began with new molecules in oncology and advanced therapy medicinal products (ATMP) in January 2025, followed by rare diseases and orphan medicinal products in 2028, and extending to all remaining medicinal products by 2030 (Chap. 18).

The assessment phase will consist of two main components: the Joint Scientific Consultation (JSC) and the Joint Clinical Assessment (JCA). Both processes will integrate patients input to ensure appropriate definition of target populations and outcome relevance, though specific mechanisms remain under development. While the European Union's 27 member states will share harmonised evidence-based JCAs, the overall assessment will remain under national jurisdiction. The JCA will utilise the PICO framework (Patient, Intervention, Comparison, Outcomes), with substantial participation of patients throughout each stage particularly in defining outcomes and patient expectations. Patients will also be involved in terms of global healthcare sustainability (Chap. 18).

At the national level in France, the existing appraisal procedure for patient participation will continue unchanged. HAS remains committed to maintain patients' perspectives in its evaluations, preserving current organisation contribution procedures for both standard and early access applications. The organisation may seek input from national experts and stakeholders during evaluation framework development to ensure comprehensive inclusivity, while ongoing discussions with patients address consultation formats and relevance.

29.10 Prospects for Further Steps

There is a global trend in increasing participatory healthcare (World Health Assembly [2024](#)) and putting more emphasis on patient relevant outcomes such as QoL assessment which will also be mirrored in the French HTA processes. In some diseases, QoL should be regarded as a primary outcome criterion, such as migraine, obesity, and last-line treatment in oncology when overall survival is low. The selection of such primary endpoints, and even the choice for secondary endpoints hierarchisation, should be done collaboratively with expert patients. In addition, their role is crucial in defining 'outcomes' in the PICO approach of the European HTAR and also in any kind of real-world data/evidence, registry and database.

Looking toward future developments, patient involvement in HTA processes requires expansion and enhanced validation. Despite notable progress in

incorporating patient input in medicine evaluation (Tringale et al. 2022), certain aspects need strengthening (e.g. disease-specific questionnaires) while others require new development approaches (e.g. Artificial Intelligence to better define specific populations). HAS's fundamental principles of science, independence and transparency should permeate all levels of patient involvement to strengthen credibility, professionalism, and rigor.

To achieve these goals, the participating patients must meet several key requirements as outlined in Box 29.1. In practice, challenges in patient involvement can be the differentiation between advocacy and participation for patient organisations and for individual patients the separation between their experiences and their emotions. The support and preparation by the SEU and the more experienced patient organisations help managing these challenges.

Box 29.1: Requirements that Patients Need to Fulfil to Qualify for Being Involved in HTA Processes by HAS

- **Absence of conflict of interest**, for example, due to collaboration with pharmaceutical companies, to reduce potential risk of bias.
- **Independence from scientific societies** while maintaining collaborative relationships.
- **Absence of political interests** that would increase the risk of bias.
- **Objectivity in reporting**, for example on decisions from countries with different healthcare contexts.

Patient representatives involved in HTA activities should undergo comprehensive training in clinical trial methodology, health economics, healthcare law, public health, and communication. This educational foundation enables more effective dialogues with experts, assessors, and media representatives. Additionally, experienced patient representatives can take responsibility for coordinating input from a diverse range of patients and expanding the participation of expert patients who possess specific training and background expertise. This coordination extends to managing disagreements between patients through evidence-based consensus building approaches.

29.11 Conclusion

In France, patient participation in HTA has evolved significantly, particularly through patient input in the HAS evaluation processes. Patient participation has become essential in medicinal product evaluation, moving beyond simple consultation to structured participation in decision-making through the Transparency Committee and early access procedures. The development of formal frameworks for

patient involvement, including contribution guidelines and participation in real-world evidence studies, demonstrates HAS's commitment to meaningful patient participation. With the upcoming implementation of European HTA Regulation, patients' role will expand further but must maintain the core principles of science, independence, and transparency. While significant progress has been made in incorporating patients' perspectives, there is a clear need for enhanced professionalisation and specific training of patient representatives in areas such as clinical trial methodology, health economics, and public health. Success in this evolution requires careful balance between patient advocacy and scientific rigor, while ensuring independence from various stakeholders. Looking ahead, the continued development of patient participation in HTA will require ongoing adaptation of evaluation methods, stronger training programs for patient members, and improved mechanisms for incorporating patient-reported outcomes. This evolution in HTA methodology, emphasising both scientific evidence and patient experience, sets a foundation for more comprehensive and patient-centred healthcare decision-making in the future.

Acknowledgments Joëlle André-Vert (Head, Patient Involvement Unit, French National Authority for Health), Nathan Guerrier (Medical advisor, Patient Involvement Unit, French National Authority for Health), Charlotte Masia (Head, Medicinal Product Assessment Office, French National Authority for Health), Bertrand Mussetta (Project Leader, Medicinal Product Assessment Office, French National Authority for Health), Christian Saout (Member of the board, French National Authority for Health, President of the Social and Medicosocial Committee, Chair of the Council for Patient Engagement) and Jean-Pierre Thierry (Medical Advisor at *France Assos Santé*, French National Union of Registered Associations of Health System Users) for their outstanding review.

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Chapter 30

The Evolving Role of Patient Organisations in HTA



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30.1 Introduction

Patient organisation involvement in health technology assessment (HTA) and research has evolved in line with an increased understanding among patient organisations, HTA bodies and health technology developers of the need to be informed by patient knowledge. Patient organisations have varied remits and may fall anywhere within broad continua of size, funding, and reach. For example, some represent large and geographically diverse communities of patients and patient organisations for different conditions such as umbrella organisations. Others may have small, local memberships. Some have reliable funding and paid professional staff, others rely on volunteers and available skills. Some have long histories of working with HTA bodies, others have no knowledge of HTA or no organisational memory of HTA involvement. These variations mean patient organisations differ in

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K. M. Facey et al. (eds.), *Patient Involvement in Health Technology Assessment*, Health Informatics, https://doi.org/10.1007/978-3-032-11284-2_30

not only what they contribute to HTA, but also how they prioritise HTA involvement. An area where all patient organisations can play a critical and unique role is contributing patient knowledge to inform decision making in HTA. Umbrella patient organisations often have HTA experience and a broad overview of the standard of care and knowledge of the unmet needs and burden of treatment for their patient population and relevant sub-groups across different geographies. This positions them strongly for involvement across the health technology lifecycle, including horizon scanning, and participation in HTA policy and process development.

As summarized in Table 4.1, Dumez and L'Espérance (2024) identify patients as the source of embodied, monitoring, medical, navigational, relational and cultural knowledge. Because patient knowledge has been traditionally undervalued or excluded by science (see epistemic injustice, Chap. 2), this knowledge is usually missing in the traditional HTA evidence or sponsor submissions (Chap. 3). To build relevant, accurate and appropriate HTA input, patient organisations may need to understand and interpret data to identify the consequences of the disease or the current care pathways for their community and highlight or address gaps in the evidence-base. They may co-produce evidence with researchers to demonstrate their communities' needs, preferences, experiences and perspectives, or—if they have the necessary methodological competencies—collect data themselves and generate well-informed input to HTA that is based on community insights. This task requires a close connection to the patient community, the ability to understand science as well as synthesise and communicate their members' unmet needs, cross-sectoral challenges and patient pathways, so interventions and decision making can be based on lived experience insights and evidence.

Nevertheless, building such an ability benefits from scientific knowledge relevant to the disease area, data literacy, evidence-based advocacy skills, financial and human resources capacity, fit for purpose guidance and support, and last, but not least, opportunities for regular access to training and information for the concerned community.

30.2 Patient Organisation Advocacy in HTA

Patient organisations have played a crucial role in shaping HTA. Their advocacy efforts have focused on promoting more patient-centred healthcare by integrating patient perspectives into research and healthcare policy, including individual HTAs and HTA policy and processes.

Patient involvement in medicine approval and access issues originated in the 1980s and 1990s. HIV/AIDS activists responded to the public concern over the high prices of medicines developed in the 1990s that were aggravated by delays in access that were costing lives. Similarly, the cancer patient community advocated to accelerate access to new therapies that were crucial to managing life-threatening diseases. The involvement of patient organisations in HTA is essential as the value of a new technology cannot be understood without capturing what matters most from the end

user perspective. This has prompted patient organisations to engage with health services to discuss the value and cost of treatments (Bedlington et al. 2017).

At the international level, global, regional, and national patient organisations as well as umbrella patient organisations participate in various international forums, such as Health Technology Assessment International (HTAi), the global society for professionals involved in HTA, and the International Society for Pharmacoeconomics and Outcomes Research (ISPOR), a leading professional organisation for health economics and outcomes research. Patient organisations are well-represented within HTAi. At its 2023 Annual Meeting, patients, carers and their organisations represented 10% of attendees. HTAi's Patient and Citizen Involvement in HTA Interest Group (PCIG), has facilitated the development of tools for patient input, methodology and fostered collaboration and knowledge exchange to improve patient and citizen involvement since 2005 (Holtorf and Bertelsen 2023).

Participation in these international forums has contributed to the acknowledgement among HTA bodies in several jurisdictions of the need for patient involvement in HTA. The sections that follow show on one side examples of meaningful patient involvement, on the other one the regional variations identified across the globe.

30.3 Patient Organisation Involvement in the HTA Life Cycle: Examples of Participation in Horizon Scanning

While patient organisations increasingly seek early engagement in the HTA life cycle, the reality in many jurisdictions is that their formal participation is invited only for the later stages of the HTA during the evidence collection, consultation on reports, or contributions to the appraisal deliberations, and this is often in the form of written input. However, as patient organisations mature, many argue that the time and resources spent at the end of the HTA processes would be more effectively spent earlier in the research and discovery process to ensure that patient and carer needs are met, patient-relevant data is collected, and thus fewer uncertainties will remain to be resolved at the HTA table (Low 2015). Where horizon scanning exists, it is potentially the earliest opportunity for patient organisations to engage in dialogue with an HTA body about health technologies under development.

Given the need for lifesaving and life changing health technologies, patient organisations may be highly attentive to research and development (R&D), have processes for monitoring pipelines and play an active role in identifying promising products. They can select technologies of interest and prioritise the most promising ones. The results of their horizon scanning could be shared with regulators and/or HTA bodies, as patients can anticipate the emergence of a medicine by three to five years, whereas HTA bodies usually look at a two-year horizon. Criteria used by patients could be added to the ones used by HTA bodies to complete their process.

The early identification of products in R&D was pioneered in HIV treatment advocacy in the 80s which highlighted the value of this early involvement,

especially for improving patient outcomes (Epstein 1995). The identification of HIV by Françoise Barré-Sinoussi and colleagues in 1983 (Barré-Sinoussi et al. 1983) attracted a lot of interest from HIV advocates around the world. Advocates from Europe, USA, Canada, Australia joined forces to find information and created the ‘HIV Pipeline’: a list of products in R&D, with scientific references and a grid to classify them according to the criteria advocates had selected (Houÿez 2004).

Box 30.1: Examples of Patient Organisation Led Horizon Scanning

Project Inform, 1985: led by Martin Delaney, Brenda Lein, David Evans and Ben Cheng. Starting in 1985, Project Inform continuously informed people living with HIV about research. An example of the HIV pipeline by Ben Cheng in 2002 can be found here: <https://www.thebodypro.com/article/hiv-pipeline-chart-drugs-development>

Act Up-Paris, 1991: reviewed the literature for HIV protease inhibitors, and 12 were identified. Advocates reviewed clinical trials from Phase I to III (saquinavir, zidovudine, zalcitabine). One trial for zidovudine (M94-247 from Abbott Laboratories), was designed by Spencer Cox, from Act Up-New York (Bernstein 2012). On January 28, 1996, results of the M94-247 trial were presented at the CROI conference: a 58% mortality reduction compared to placebo was seen. Zidovudine received FDA approval on February 29, and a Compassionate Use programme started in France on March 1. There, the Ministry of Health had not anticipated this situation and had no information on policies (e.g. on reference prices) in other countries. In fact, patients were better informed on the soon-to-arrive lifesaving HIV treatments than authorities in charge of price negotiations (Dalgallarrondo and Urfalino 2002).

European AIDS Treatment Group (EATG): established a Community Advisory Board (CAB; European Community Advisory Board, ECAB) that met several times per year with developers. In a report published in 1997, they had identified 130 products including 18 that were of particular interest to them. Out of these 18 products, nine were authorised; eight failed (negative Phase III trials); and one received negative feedback from the FDA hearing (November 1, 1999); no product was authorised that had not been followed up by the ECAB.

EuroCAB: when developing a health technology, developers can institute working relations with patients in a collaborative setting. EURORDIS and EUPATI-Spain have instituted a programme of Community Advisory Boards, EuroCAB (Global Cab). EuroCAB invites developers to sign a Charter for Collaboration with Patients in Clinical Research and provide guidelines together with a mentoring and training programme. CABs help set the agenda with the developer on a multitude of topics (design, end-points, feasibility, consent, site selection, quality of life (QoL) and patient-reported outcome measures (PROMs), compassionate use, pricing, relative efficacy, etc.). As of 2024, 11 disease-specific CABs exist, and others are

(continued)

Box 30.1 (continued)

in formation. Evaluating surveys from 26 distinct CAB meetings with 23 companies, having looked at 44 studies of 39 products, shows that this form of shared decision-making is ethical and effective in shortening development times, accelerating recruitment and improving retention by making research more patient relevant. If the study results are positive, the partnering results in higher quality results, faster approval, and accelerated access.

In rare diseases, patient organisations are generally more recently established compared to HIV groups. In a survey of the European Organisation for Rare Diseases (EURORDIS) member organisations, 80% were created after 1981. Inspired by CABs created by HIV/AIDS organisations (see Box 30.1), similar structures were created for some rare diseases. In these CABs, patient advocates organise their own desktop research as described in Box 30.2.

Box 30.2: Examples of Rare Disease Patient Organisation Led Horizon Scanning**Case 1: Hereditary Haemorrhagic Telangiectasia (HHT)**

European HHT patient groups partner with the US Cure for HHT Research Network that gathers researchers, clinicians, engineers and patients. The European HHT CAB, composed of 11 members, uses the International Society for the Study of Vascular Anomalies to conduct systematic literature searches using seven keywords proposed by medical engineers and biologists. Their current interest includes an ultrasound portable device to replace MRI, for an earlier diagnosis in children. They also monitor the development of 15 medicines.

Case 2: Niemann Pick disease (Acid sphingomyelinase deficiency)

Different subtypes exist (A–B, C) that require different treatments. The most severe cases are eligible for organ transplant. As of 2023, only one product has been authorised. Products in R&D include stem cells, symptomatic treatments, lipid lowering drugs and this R&D is monitored by patients. The Spanish patient group uses different sources, including the International Niemann Pick Alliance, the Carlos III Institute and the Spanish National Research Council.

Case 3: Amyotrophic lateral sclerosis (ALS)

119 medicines in development for ALS are designated as orphan drugs at the FDA and 69 at the EMA. In the Netherlands, the ALS patient group compiles a dashboard of the most promising medicines (six products in 2023) with the ALS Foundation, the Ministry of Health and the Zorginstituut Nederland (HTA). A volunteer analysed data from 404

(continued)

Box 30.2 (continued)

clinical trials, for more than 100 products, including failures. Meetings with Utrecht University, subscription to 10 medical journals of interest, and contacts with developers led to refining the list to 13 products of high priority. Discussions on platform trials are now in progress with research organisations, HEALEY in the USA (nine products), and TRICALS in Europe (six products).

30.4 Role of Patient Organisations in HTA Across Regions

While global efforts to promote patient involvement in HTA are led by multi-stakeholder networks such as HTAi PCIG, at the regional and national level, the involvement of patient organisations in HTA varies widely, reflecting the diversity of healthcare systems, policies, and cultural contexts. In some jurisdictions, patient organisations have played a role in influencing HTA processes, leading to greater recognition of patient perspectives in decision making

The experience and knowledge that patient organisations have generated through participation in well-developed and established HTA systems, can be shared with peer organisations operating in other regional contexts, with the caveat that tools such as templates and strategies, promoted as best practices need to be adapted to local contexts and recognise cultural specificities.

30.4.1 North America

In Canada, HTA is conducted by two organisations, Canada's Drug Agency (CDA)—formerly Canadian Agency for Drugs and Technologies in Health (CADTH)—and *Institut national d'excellence en santé et en services sociaux* (INESSS). Patients inserted themselves into the HTA process from the outset. In 2010, CADTH announced a process for patients to make direct submissions to the HTA process, using a template to share their perspectives on the burden of disease, experiences with existing therapies, and actual or anticipated impact of new therapies on patient-relevant clinical outcomes and quality of life. In 2016, a study analysing patient input in 30 CADTH assessments, stressed the importance of patient perspectives in interpreting evidence toward making assessments and informing recommendations. Despite the positive results, in 2017 patient organisations claimed that the system still lacked meaningful impact (Wong-Rieger 2017). This prompted CADTH to introduce a series of improvements for patient involvement such as:

- full patient submissions (not just summaries) to all members of review committees
- opportunities for patient submitters to comment on draft recommendations
- integration of patient submissions to the final published recommendations

- appointment of patients (not public representatives) on the scientific review committee
- patient members on advisory and work groups, patient position on the board of directors; and, most recently,
- opportunity for live patient testimony at the scientific review committee.

The HTA organisations in the United States of America (USA), unlike those in most other countries, currently do not conduct assessments on behalf of public (or private) healthcare programs or plans, but they can have significant impact on payers. The nonprofit Institute for Clinical and Economic Review (ICER) has developed a Patient Engagement Guide to help patient organisations and patients to make written submissions, participate in public meetings, and comment on draft reviews (see Chap. 26). Similarly, the publicly funded Agency for Healthcare Research and Quality (AHRQ) invites patients through its Effective Health Care (EHC) Program and Technology Assessment Program to provide input, comment on reviews, and participate in advisory groups.

30.4.2 *Latin America (LATAM)*

Patient organisations have been involved systematically in HTA, in at least four countries in LATAM: Argentina, Brazil, Chile and Colombia. Roles are variable and include well-established participation in individual HTAs in Brazil and the shaping and improvement of patient participation processes in Colombia (see Chap. 20). In Argentina, four patients—drawn from rare, oncology, chronic and chronic transmissible diseases organisations—are involved throughout the HTA process (FIFARMA 2023). Silva (2024) notes that as the region has transitioned to democracy, there has been evidence of experimentation with participatory governance, including different ways of involving patients in decision making such as HTA.

Similarly to other regions, a survey of stakeholders in 10 LATAM countries identified an undervaluing of patient involvement by HTA bodies and transparency of information and methodology as barriers to increasing patient involvement (FIFARMA 2023).

30.4.3 *Asia Pacific*

In Asia Pacific, patient organisations experience high degrees of variation in terms of their involvement in HTA. HTA has a formal role in at least 14 countries in the region and is undertaken informally in a further 12 countries (Mundy and Maddern 2023; Teerawattananon et al. 2019; Sivalal 2009). Some HTA bodies in the region

have established participation processes (Chaps. 14 and 25), others are evolving quickly to include patients in their processes (Chaps. 21, 22, and 23), and others have not yet prioritised patient aspects in the HTA.

Despite their growing recognition in HTA, patient organisations encounter numerous challenges, including time and resource constraints. For instance, patient organisations in the Asia Pacific region have reported a general lack of transparency regarding the process of patient involvement, as well as insufficient support from HTA bodies. Therefore, enhanced guidance and feedback from HTA bodies are instrumental to guarantee that patient organisation knowledge influences reimbursement decision-making (Single et al. 2021).

In Australia, where HTA was introduced initially for medicines in 1993, there are many well-established patient organisations who regularly participate in HTA and advocate for improvements to the process. This includes taking part in the co-design of the patient involvement process to support better and earlier engagement in HTA (Australian Government Department of Health and Aged Care 2024) and the HTA Policy and Methods Review which included a joint response from 51 patient and community organisations demonstrating unprecedented collaboration as well as support for the proposed options to reduce time to access, and increase transparency, patient engagement, equity and the use of research into patients' needs, preferences, experiences and perspectives (Health Technology Assessment Policy and Methods Review Reference Committee 2024). For further details see Chap. 14. Since the Review's publication more than 70 patient organisations have formed an informal alliance which has agreed actions to not only drive the implementation of recommendations but ensure the patient perspective is included. This has included successfully advocating for a transparent process for appointing patient representatives to the Implementation Advisory Group and proactively agreeing criteria for these appointments.

Further north in the region, Taiwan has a well-established process for involving patients and patient organisations, including input to individual HTAs, representation at committee meetings, improving HTA processes such as the submission template and developing training (see Chap. 25). Recently, patient involvement in HTA has been introduced in Singapore. One of the key steps to its introduction was the mapping of patient organisations across the country by the HTA body (Chap. 23).

Although patient organisations in the Republic of Korea are not involved in the HTA process for medicines, they successfully advocated for expanding insurance benefits for medicines (Single et al. 2021) and have a role in non-medicines HTA (Chap. 22). Meanwhile, in the Philippines, the Philippine Alliance of Patient Organizations successfully advocated for a patient position on the HTA decision-making council (Wong et al. 2023, see also Chap. 21). A 2021 study suggested patient organisations in New Zealand, the Republic of Korea and Taiwan wanted to participate throughout the HTA process, see Table 30.1

Table 30.1 Stages of HTA where patient organisations would like to see more participation

Stage of HTA	Republic of Korea (n = 8)	New Zealand (n = 9)	Taiwan (n = 26)
Topic proposal	4	4	3
Topic selection	2	5	7
Scoping of assessment	4	5	11
Submitting input	7	6	12
Membership of expert committee	6	5	6
Presentation to expert committee or hearing	6	6	8
Consultation on draft advice/recommendation report	3	6	12
Help write or disseminate patient-friendly report of advice/recommendations	4	5	14
Submit information for appeals	4	5	7
Other	1	1	0
I don't know	0	2	3

Adapted from Single et al. (2021)

30.4.4 Africa

While the practice of HTA in Africa is limited, several initiatives are underway to drive its adoption. Among those, patient organisations have been playing a key role in advocating for its adoption. See Chap. 28 for an example of patient organisations working with other stakeholders in the sub-Saharan region to develop a call to action. In addition to HTA being used to a limited degree for informing reimbursement decisions in African countries, only a few disease areas such as HIV, cancer, tuberculosis, or sickle cell disease have representative patient organisations. They mostly focus on education, awareness, peer to peer support, and access to treatment to overcome the limitations of the healthcare systems (Holtorf et al. 2021). However, with progress towards universal healthcare coverage and increasingly formalised use of HTA in decision making, patient and community involvement can become more influential as pioneering initiatives demonstrate. For example, in two consecutive conferences in 2023 and 2024, key stakeholders in Morocco came together under the leadership of the Ministry of Health and Social Protection to agree on eight recommendations related to the role of the patient’s voice in health systems (SMEPS 2023, 2024).

30.4.5 Europe

In many European countries, patients can be involved in different ways in HTA, but the processes and guidance for the involvement varies widely. To better understand the current state of involvement and the experiences of these different practices,

HTAi PCIG worked with the European Patients' Forum (EPF) and the European Patients' Academy for Therapeutic Innovation (EUPATI) from 2021 to 2023 (Holtorf and Bertelsen 2023). The results revealed a wide variation in the processes and intensity of patient involvement and highlighted several areas for improvement. The study produced a series of recommendations that emphasise the importance of involving patients at an organisational level, providing appropriate training and accessible information, and encouraging two-way communication. This includes providing feedback on how patient input has been incorporated into HTA reports and subsequent recommendations or decisions (Holtorf et al. 2024).

A distinctive feature of the European Union (EU) is the collaborative approach of its Member States, particularly in addressing the clinical aspects of HTA as described in Chap. 18. EUnetHTA, a collaboration between European HTA bodies since 2005 and precursor of the current European-level activity, provided an opportunity for patient organisations to influence patient involvement methodologies and frameworks in Europe. Patients were consulted through representation by umbrella organisations on the Stakeholder Forum. EUnetHTA has progressively shifted its focus to collaborating on the production of HTA information and early dialogue with companies, thereby increasing the involvement of patient umbrella organisations and individual patients. Building on this involvement experience, patient organisations were vocal in the negotiations on the Regulation (EU) 2021/2282 on HTA (HTAR), which institutionalised this voluntary collaboration in a common framework for joint work at EU level (European Commission 2021). Umbrella organisations such as EPF or EURORDIS advocated for the inclusion of patient representatives in the coordination group of HTA bodies (European Patients' Forum 2018; EURORDIS 2018). Although this provision was not included in the final agreement, European patient organisations have been given a formal role as members of the HTA Stakeholder Network (Chap. 18). National patient organisations and umbrella organisations continue to contribute within and outside the HTA Stakeholder Network to improve the conditions for patient involvement and its quality in the implementation of the HTAR.

Beyond HTAR and involvement in individual HTAs, there is more evidence of patient organisations participating in HTA at an organisational level, i.e., shaping policy, governance and HTA methods and processes. Examples include the involvement of patient organisations in drafting the legislation for rare disease treatments in Czechia (Chap. 16), the work of the French Federation of Patient Associations in the development of an early access process for medicines, and representatives of peak patient organisations in the development of an HTA process for ultra-orphan medicines in Scotland (Nabarette et al. 2023).

In addition to the cooperation between EU Member States on the clinical aspects of HTA, patient involvement at national level will remain crucial. Patient organisations provide insights that are essential for understanding the organisational, ethical and social dimensions of healthcare systems within each Member State—perspectives that cannot be captured by evidence from interventional clinical trials alone. In this context, EU cooperation under the HTAR has the potential to enhance the

expertise and skills of HTA researchers in all EU Member States to engage effectively with patient organisations.

30.5 Capacity Building

Patients involved in HTA can share first-hand perspectives on patient preferences, value of treatment and currently unmet medical needs for patients and carers (Hunter et al. 2018). Successful patient participation however still faces several challenges. On the patient side, the main issues include the lack of understanding of decision-making processes, the lack of knowledge and guidance in evidence-based advocacy, and the lack of resources that could be dedicated to support capacity building, and therefore, patient representation (Dimitrova et al. 2022). Patient organisations have a key role to play in supporting and coaching patients to ensure they feel equipped and able to meaningfully contribute in HTA processes. To this purpose, patient organisations may develop training programmes and materials for those that might be involved in HTA in any way, ensuring the decision-making process is understood and the mechanism for involvement is clear.

In the European Union, the implementation of the HTAR, applicable as of 2025, is expected to increase patients' roles compared to most existing European national frameworks (European Commission 2021). In this perspective, the European Health and Digital Executive Agency (HADEA) has funded two projects aimed at training patients (EU4Health 2022): the European Capacity Building for Patients (EUCAPA) and HTA4Patients. EUCAPA offers three training programmes, combining online and on-site training tailored to the needs of patients likely to be called to participate in HTA (EUCAPA 2023), while HTA4Patients provides an e-learning training course as well as interactive online training sessions on HTA (EUPATI 2024). However, the recognition of the importance of training patients in HTA in Europe predates the HTAR, and several capacity building initiatives can already be found in the past decades. In 2010, EURORDIS created a dedicated HTA programme, training approximately 40 to 50 rare diseases patient advocates per year (EURORDIS 2024). EUPATI, initiated as a public-private partnership led by EPF and now established as an independent foundation, is recognised as a prominent example of a capacity-building programme and provides online educational materials on therapeutic innovation, as well as an extensive module on HTA and patient participation. In the Czech Republic, the Academy of Patient Organisations (APO) offers a training programme, covering topics such as reimbursement systems, evaluation of efficacy, safety and cost-effectiveness. The Irish Platform for Patient Organisations, Science, and Industry (IPPOSI) or Beacon for Rare Diseases also offer online modules and/or toolkits on HTA. In Germany, patient organisations provide support to non-trained patients on behalf of the HTA body, by illustrating their involvement, and facilitating patient participation within HTA processes.

In Canada, an initial training on HTA for patient organisations was delivered by the Consumer AdvoCare Network (CAN) in 2003 (CADTH 2003). In 2006, in

collaboration with the University of Alberta, CAN began hosting HTA trainings that included patient organisations and other stakeholders, such as policymakers, pharmaceutical representatives and healthcare professionals. These trainings included interactive workshops and simulation exercises in HTA decision-making (Low et al. 2023).

The Canadian Drug Agency (CDA) has hosted webcasts covering various HTA topics, including patient participation processes, and organises an annual symposium to which patients are invited and sessions are dedicated to patient aspects. However, while CDA recognises the need to provide regular training specific to patient participation in HTA, such training for patients and patient advocates is not readily available (CDA 2024). As far back as 2012 (Secor 2012), independent reviewers of the patient input process have called for quarterly training sessions for patient groups and public members of the HTA process.

Other HTA bodies—such as NICE, and the French *Haute Autorité de Santé* (HAS)—have training materials available.

Entities such as Latin America Patients Academy (LAPA) and Patient Voice Initiative (Australia) are also examples of non-profit organisations having developed training or capacity building programmes in specific regions. An alternative approach to capacity development was taken by a patient organisation in China that worked with a university to translate the first edition of this book in Mandarin (Facey et al. 2022), before using some of the methodology to develop evidence regarding health technologies.

Overall, educational training for patient involvement in HTA is widely acknowledged as necessary. However, despite a mix of training programme formats and content, training programmes organised directly by patient organisations remain scarce, mostly as these organisations face competing priorities and may not have the time, staff, or financial resources needed to develop and conduct high quality training programmes.

30.6 Future Directions

The role of patient organisations in the healthcare landscape has expanded and so has their role in HTA. Patient organisations are increasingly recognised as important stakeholders in HTA processes, with their perspectives and expertise valued alongside those of healthcare professionals, regulators, and industry representatives. Their roles range from advocating for consistent and genuine patient involvement, transparency in decision-making processes, and effective pharmacovigilance and communication, to becoming institutionalised partners. Nevertheless, despite the recognised value they bring to healthcare systems, patient organisations are increasingly confronted with challenges. In some jurisdictions, perceived Conflict of Interest (COI) regarding funding sources represents a concrete barrier to patient organisations involvement, with most criticism directed at organisations exclusively funded by pharmaceutical companies, especially if one technology developer is the sole funder. As highlighted in the project *Patient Involvement in HTA in Europe*:

Recommendations from a 360° Perspective View of Current Patient Involvement Practices, the uncertainty and volatility of funding sources generate a conundrum due to the discontinuity of capacity invested in HTA, resulting in the loss of organisational memory within patient organisations (Holtorf and Bertelsen 2023; Holtorf et al. 2024). However, funding sources can easily be disclosed and the ongoing concerns about conflicts of interest for patient organisations is sometimes perceived by patient organisations as masking a polarised or even adversarial mindset in HTA (i.e. HTA body vs technology developer with the patient perspective assumed to support one side or the other), and a lack of education in the broader HTA community about the value of patient knowledge and the critical need for it when determining value.

As highlighted in the Capacity Building section, patient organisations' involvement in HTA across the whole life cycle of HTA often competes with their other priorities, such as providing support and assistance to individuals and strategic initiatives (Birrell and Godfrey 2023). Patient and patient organisation involvement generate a high burden, especially in terms of its impact on workloads and resource use. In addition, those involved (patients or staff of patient organisations) often experience considerable emotional stress in the process of delivering input to inform an HTA recommendation. This might include having strong personal connections to patients or lived experience of the condition and need. As a result, due to limited time and resource capacity, patient organisations with experience in HTA are often confronted with the need to review their practice to focus on activities where they can make the biggest difference for their members. For example, this may lead some to focus their efforts on engaging in research and development, horizon scanning, and/or co-producing or investing in research into patients' needs, preferences and experiences which may be able to be used for a variety of HTAs and policy areas.

Moreover, while HTA organisations are collaborating to harmonise processes, timelines, and data sharing to improve efficiency and rationalise limited resources, patient organisations are yet to realise the same opportunity of harmonising their activities globally to achieve the same efficiencies given their far more limited resources. Challenges to harmonisation amongst patient organisations arise mostly from the lack of infrastructure or over-arching network for data sharing. The fragmentation of efforts generates the risk of a perceived competition amongst patient organisations and, occasionally, with companies and professional consultants operating with the mandate to represent the patient voice. Given the increased demand for patient organisations to engage in HTA from horizon scanning to disinvestment, the need for them to work together regionally and globally has never been more pressing.

Acknowledgements The authors would like to acknowledge helpful comments received from Karen Facey and Anke-Peggy Holtorf. All views expressed and any errors are entirely the responsibility of the authors.

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Chapter 31

Patient Involvement Throughout the Medicine Life Cycle: A Pharmaceutical Industries' Perspective



Anke-Peggy Holtorf, Michaela Dinböck, Alexandra Moutet,
and Elin Haf Davies

31.1 Introduction

The pharmaceutical industry conducts research to discover, develop and eventually deliver better therapeutic options for patients. More systematic attention to the perspective of patients and other stakeholders by organisations regulating whether, how, and by whom medicines can be used, reimbursed, or prescribed, shapes the future of the pharmaceutical market (US Food and Drug Administration 2016; US Food and Drug Administration 2018; Mavris et al. 2019; Murphy et al. 2021) and necessitates the pharmaceutical industry to consider the implications for their assets.

Consequently, the pharmaceutical industry must adopt—throughout the entire product development—the same vista driven by the experiences of the future users or beneficiaries of the products, the people living with the disease. Will the therapy address important patient needs not yet addressed by currently available therapies? What are patients' expectations compared to those of other stakeholders such as clinicians? Does the therapy fit into the living context of the target patients? Will the clinical trial setting and protocol be relevant and feasible to participants? Do they

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have preferences that are relevant for the development and, later, for their everyday use?

Techniques to explore the answers to these questions through what some in industry call ‘the patient voice’ (Chap. 4) include patient, carer and family participation in advisory boards or their input to study plans and protocols, social media research (Chap. 7) or other methods for eliciting the patient perspective from individual representatives.

In addition, research methods facilitate the broader representation of the patient perspective (Chap. 3). Research into patient aspects aims to collect patient experience data/patient-based evidence, for example through social media research (Chap. 7), from apps and wearables used by the patient at home, or ethnographic methods (Chap. 10). Researchers develop patient-reported outcomes measures to integrate them as patient-relevant endpoints to the clinical studies. Patient preference studies (Chap. 9) help to explore priorities of patients and the acceptability of trade-offs.

An integrated end-to-end plan driven by a company strategy that prioritises patient value is likely most effective in yielding products that meaningfully improve the life of patients (Cavaller-Bellaubi et al. 2021; Algorri et al. 2023). To ensure that patient experiences, needs and preferences are well understood, addressed, documented, and have impact throughout the product development, preparation must happen long before dossier submission (Holtorf and Cook 2018; Cook et al. 2019).

We will outline how insights from patients or carers about living with the disease, use of current treatments and what they would like to see changed in future can be integrated along the life cycle of medicines to shape trial design, improve evidence generation and inform the value of the future products. In addition, we will explore how pharmaceutical companies can manage the changes that are required for strategically implementing these new approaches in their organisation.

31.2 Embedding Patients’ Perspectives Along the Entire Medicine Life Cycle

The typical life cycle of pharmaceuticals depicted on the top line of Fig. 31.1 comprises:

- A discovery process
- Product and evidence development during the preclinical and clinical phases
- Submission of evidence for receiving a market authorisation from the regulatory authorities
- Review by HTA bodies to inform the decisions on listing or coverage at market entry or soon after
- Commercialisation with further data collection and incremental product improvements
- Disinvestment at the end of the commercialisation (Moseley et al. 2020; Singh et al. 2023).

Traditionally, researchers, clinical scientists, clinical operations, regulatory or medical affairs managers, brand leads, and product managers determined these

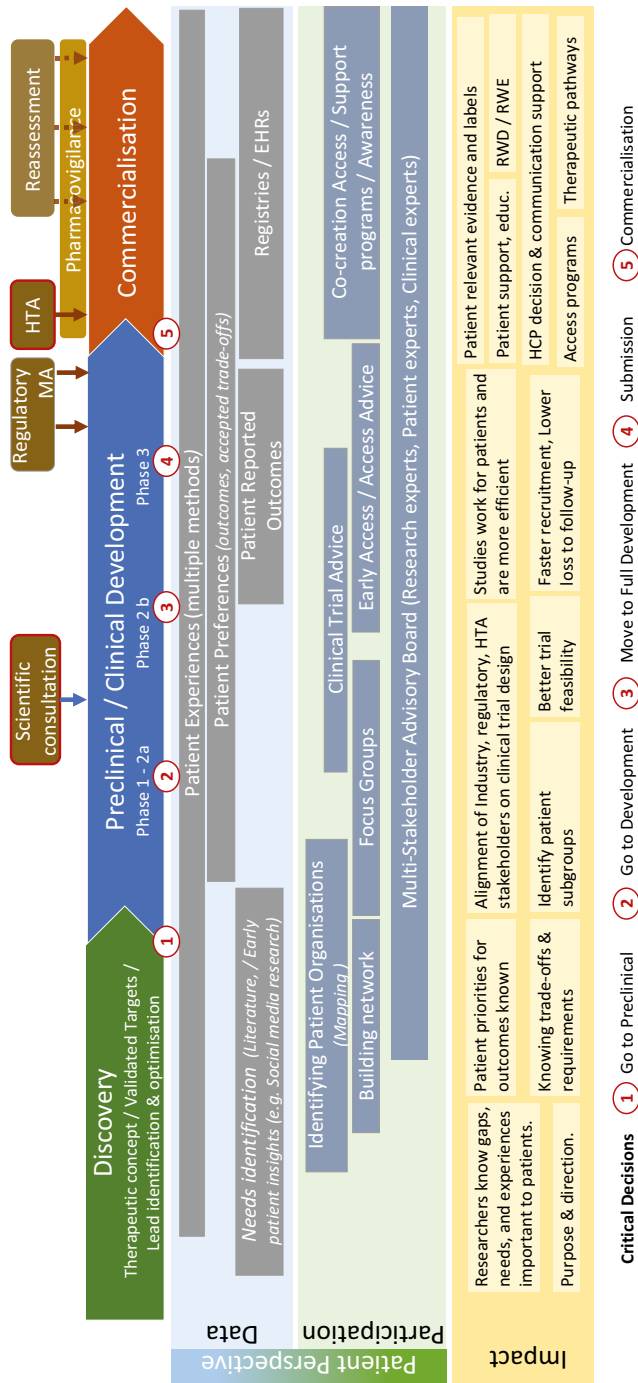


Fig. 31.1 Integration of patients' perspectives throughout the medicine life cycle using a range of participation and data collection approaches. *MA* Marketing Authorisation, *HTA* Health Technology Assessment, *EHR* Electronic Health Records, *HCP* Healthcare Professional, *RWD/RWE* Real World Data/Evidence

processes without interacting with patients before entering the market. At defined steps, referred to as decision points, the information and data accumulated were critically reviewed and scrutinised by senior decision boards composed of similar expertise, who decided whether to invest further in the product or to disinvest. The ‘customers’ perceived as important were not the future users or patients themselves, but the clinicians and future product prescribers. Direct contact with the patient community was somewhat limited due to concerns about legal, moral, or ethical conflicts, and the belief that physicians were best able to report patient needs. Patients contributed to the development process only as clinical trial participants, with all contact being via the site investigator.

Many useful medicines have emerged through this established process. However, data and the products may not always have convinced patients (e.g. because end-points and product labels were not relevant in their life). Medicines therefore may not be used to their full potential, be wasted, and/or product performance in the market may remain below expectations (Fuller et al. 2021). In addition, many patients who could benefit from the product may never get access or not use it in a way to maximise its benefits.

A novel approach driven by patients’ needs targets the final beneficiaries as soon as the therapeutic concepts emerge in industry medicine’s authorities such as the US Food and Drug Administration (FDA) (see Box 31.1). Two new activity chains reflecting the importance of patients’ perspectives are integrated into product development: (1) research aiming to highlight all aspects (dimensions and impacts) related to patients’ experiences of living with the disease, their current care and which outcomes are important to them, and (2) supporting patients to participate in a range of ways (Holtorf and Cook 2018). Both activities—research into patient aspects (Chap. 3) to generate patient experience data for the FDA (US Food and Drug Administration 2021) or patient-based evidence for HTA and participation in medicine’s development to integrate patients’ perspectives—can complement each other. Research can be contextualised (e.g. national vs international or specific sub-populations), co-created, triangulated, and/or validated through dialogue and partnerships. The representativeness of critical knowledge gained through participation may be confirmed through rigorous qualitative and quantitative methods, where such research is conducted. The mix of methods most suitable to generate a robust package of patient-based evidence is determined by the disease area, the properties of the technology itself, the expected benefits and tolerability, and the accessibility of published data on patient preferences and experiences and of the beneficiaries.

Box 31.1 Changing Perception of Patient Expertise at FDA

“It turns out that what is really bothering the patient and what is really bothering the doctor can be radically different things patients are true experts in their disease.”

“It’s clear you have to start with an understanding of the impact of the disease on the people who have it, and what they value most in terms of alleviation before you set up a measurement and go forward with truly patient-focused drug development.” (Dr. Janet Woodcock at the PDUFA V Clinical Outcome Assessments Public Workshop, 1 Apr 2015).

Beyond better preparedness for regulatory or HTA reviews, Cook et al. (2019) demonstrated with examples from a variety of disease areas (non-alcoholic steato-hepatitis, obesity, dry eye disease), that generation of patient experience data/patient-based evidence early in the life cycle helps to inform early discussions and alignment with the HTA and regulatory bodies on the design of the studies for the phase 3 development.

The impact of integrating representative patient perspectives along the entire life cycle can include motivational, directional, adaptive aspects (researcher motivation, purpose and direction, considering patient relevant endpoints; see bottom row of Fig. 31.1). Importantly, it also contributes to improved efficiency, e.g. better trial feasibility, faster trial recruitment, lower loss to follow-up (Crocker et al. 2018; Stergiopoulos et al. 2020). This directly translates into reduced development cost, faster time to market and differentiated patient relevant labels, relevant information, and support programmes (Getz 2015; Levitan et al. 2018). For company affiliates, enhanced knowledge on patient perspectives and critical national or regional factors that may influence the effective use of the product, may further support access and commercial success.

As with any research throughout product development, robust methods and good research practices must be applied and systematically documented to maintain transparency and support its use as high-quality evidence with authorities. For patient participation industry compliance with national legislative restrictions and international good practice guides must be followed (Haerry et al. 2018; Algorri et al. 2023; Almeida et al. 2024). A variety of methods that can be applied systematically during the innovation and development process are summarised in Table 31.1.

Table 31.1 Examples of methods for exploring the patient perspective for elements in the product development and innovation process

	Participation	Research
Strategic/research priorities	• Strategic/Organisational Advisory boards • Represented in leadership team	• Gap and needs analysis (Holtorf et al. 2018; Cook et al. 2019)
Needs analysis	• Co-creation of research • Patient partner in team or patient advisor	• Ethnography (Chap. 10) • Observational research – Social media research (Chap. 7, Holtorf et al. 2023) – Ethnography (Chap. 10) – Real world data analysis • Preference studies (Chap. 9) • Focus groups or online bulletin boards • Interviews and/or surveys
Burden of disease	• Testimonies • Patient partner in team or patient advisor	• Burden of disease studies (Chap. 6) • Patient-based evidence, patient experience data (Chap. 3) (US Food and Drug Administration 2018) • Burden/cost of disease analysis/modelling from the patient perspective • Digital approaches (e.g. through wearables) for data collection and analysis (Chaps. 7 and 3) • Focus groups, online bulletin boards • Interviews and/or surveys
Target product profile (TPP)	• Consultation • (Co-creation) • Councils or (group-) interviews to critically review TPP	• Include patient evidence specific to target population/subpopulations for TPP items • Patient preference studies (priorities, outcomes, acceptable trade-offs) (Chap. 9)
Study design	• Study advisory board • Co-creation • Councils, interviews to review trial designs, inclusion criteria, or trial material • Consultation	• Preference studies (Chap. 9) • Clinical outcomes assessments including Patient-reported outcomes (US Food and Drug Administration 2021)
Product design	• Workshop • Design thinking workshops	• Focus groups and/or interviews • Observational research – Ethnography (Chap. 10) – Time in motion studies (Nickman et al. 2007)
Cost-effectiveness	• Advisory board	• Patient centric cost data (Widrig and Tag 2014 p. 591) • Cost of disease; modelling from patient perspective (Bilvick Tai et al. 2016)

(continued)

Table 31.1 (continued)

	Participation	Research
Confirmatory/concept testing/user acceptance	• Consultation • User testing, (group-) interviews	• Survey • Patient experience studies (US Food and Drug Administration 2021, Chap. 3) • Patient satisfaction
Awareness building	• Dissemination via conferences, media, public or social media	• Peer reviewed publications
Inform stakeholders on technology and evidence	• Plain language summaries (Cook et al. 2021)	• Patient experience dossier/ repository (Bertelsen et al. 2023)
Post-authorisation performance and iterative improvement	• Advisory board	• Real world data collection and analysis (registries, electronic health records, wearables) • Drug utilisation review • Adverse event reporting (by patients) • Patient satisfaction survey • Patient preference studies

A few principles as outlined in Box 31.2 should be considered to guide the design, planning, and conduct of such approaches.

Box 31.2 Principles for Exploring Patient Perspectives Throughout Medicine Life Cycle

The following are principles for exploring patient perspectives, needs and experiences (patient aspects):

- All methods are interactive with direct participation from patients and are ideally co-designed, co-analysed, and interpreted with patients
- Most approaches to address research questions through participation or research start with desktop research (literature review, database analysis, existing repositories of previous research or related information)—this reduces the risk of repeating previous research and helps to improve the design of the interaction or research
- Observational research means systematic observation and recording of behaviours or events in their natural settings without manipulating variables, including watching and documenting activities or characteristics without interfering with or controlling the environment.

The choice of methods to apply will depend on the objectives of the research (the research question), disease area, the pre-existing knowledge, access to the respective patient community as well as time and resource constraints. It is important to start early in pre-clinical development to establish existing knowledge about the

patient experiences. Further knowledge generation should follow a patient involvement strategy with clearly defined objectives and plans, resembling the approach suggested in Chap. 4. The objectives (why) will determine the who, how, and when of the involvement activities. Such a targeted approach will help to use resources of the technology developer and the patient community most effectively and to build mutual trust through transparent collaboration.

Chapter 3 calls for more transparency through publication of research by industry into patient experiences. However, a company may choose for justified business-relevant reasons not to publish results immediately. Health technology developers should strive to make all research results relating to the patient experiences and perspectives accessible, for example by publication. To support this, solutions should be created to protect business-critical parts of information until the product is commercialised or withdrawn from the development or commercialisation process. Broad and sustainable access to patient-based evidence internally can enhance the implementation across the company (e.g. as a patient experience dossier or on data platforms).

One important adaptation supporting the exchange and communication with patients is a change of mindset. As a research-based industry with a focus on targeting the scientific and clinical community, all reports and much of the communication are traditionally characterised by highly technical scientific-clinical language. Patients and carers articulate their experience from a different angle and in different words and therefore may find it difficult to understand or to apply the technical language to their lived experience. Some companies complement reports with a plain language summary that reflects the content of the report in words understandable and accessible to patients (Cook et al. 2021). Under European Union (EU) regulation (EU Regulation 536/2014 Article 37), plain language summaries are now mandatory for clinical study reports. Following an initiative of the Scottish Medicines Consortium, guidance for the production and use of such plain language summaries has been developed and piloted by a multi-stakeholder Patient and Citizen Involvement in HTA Interest Group (PCIG) project subcommittee (Cook et al. 2021). However, there is only limited specific guidance (European Commission 2021a; NHS Health Research Authority 2025) by HTA bodies for technology developers that defines good practices for plain language summaries.

For developing health technologies, a failure to demonstrate a relevant improvement to the people living with the disease may mean failure of the entire investment (Bignami et al. 2011). An area, where the urgency of involving patients and carers is obvious and particularly important are rare diseases or complex diseases with a small number of people living with the disease, few clinical specialists who are familiar with patients' needs, limited data on natural history and varied standards of care that can be used as comparators. People living with the disease, often experience delayed diagnosis and varied treatment pathways. Hence, they are knowledgeable experts in navigating their healthcare system. Moreover, patient organisations often establish international networks for information exchange and collaboration (e.g. NORD, EURORDIS) and hence, can contribute critical knowledge to efficient development of new medicines.

31.2.1 Patient Focus Means Organisational Change for Pharmaceutical Companies

Integrating patients' perspectives throughout innovation and commercialisation in the pharmaceutical industry—from early on and along the entire life cycle—requires a significant 'cultural' change (du Plessis et al. 2017). Researchers, developers, management, investors, and marketers must now expand the view, as decision-critical, to go beyond prescribers and to consider the health ecosystem as a whole—including the HTA researchers, the national decision bodies, and the patients or carers as the final users or beneficiaries. The required shift of mindset and organisational change comprises alignment across the organisation, training, monitoring of implementation and impact, reinforcement, removal of cultural or perceived barriers particularly those that arise on scientific grounds due to the medical constructs of health (Boutin et al. 2017).

But can pharmaceutical companies become truly patient-centric? Increasing patients' influence in business decisions may unsettle some stakeholders in the organisation especially if they are remote from patients and driven by other, seemingly opposing criteria such as shareholder value or timely maximisation of return on investments. Risk adversity or the perception of compliance or other risks such as those related to ethics, legal, transparency, data privacy, reputation, or social media activities may be expressed through overly strict compliance rules and other internal regulations that moderate and limit interactions with patients (Dews et al. 2023). To involve patients meaningfully and systematically at high quality, an environment must be created that offers a transparent framework to the employees and stakeholders (ibid.). Guidance for developing such frameworks is emerging (PFMD 2018; Deane et al. 2019; Faulkner et al. 2021; Feldman et al. 2021).

Changing business processes requires impetus and reinforcement from both company leadership and development teams. Frameworks must be established that define the rationale for participation (why), and the what, the when and how. Support and guidance enhance their adoption by teams. Mandatory patient criteria complementing each decision point (see Fig. 31.1) along the product life cycle can ensure that only assets with the required patient-based evidence can advance.

Building the methodological expertise for generating high quality evidence and for enabling effective patient participation necessitates initial investment. This investment into integrating patients' perspectives can more than pay for itself by reducing the cost and length of clinical trials, reducing the risk of failure due to low patient compliance or persistence in the clinical trial, or later, through faster access to the market and better long-term positioning of the product in the market by meeting true patient unmet needs (Levitan et al. 2018). Patient involvement is an investment in good decision making.

A crucial element of taking a strategic approach to involving patients is continuous reflection on the frameworks and practices. Such reflection can help in implementing these practices across the organisation and improving them in an agile way informed by the feedback and learnings (Feldman et al. 2021; Dews et al. 2023).

Table 31.2 Categories of key performance indicators to demonstrate use, progress and value of patient involvement along the pharmaceutical value chain

KPI	Description
Involvement activity	Have we involved patients systematically over the life cycle of the medicine’s development (e. g. activity tracker)?
Quality of involvement	How was the mechanism of involvement perceived by participants (e.g. feedback questionnaires)?
Output	What were the learnings from each patient involvement activity?
Outcome	How was the patient input and patient-based evidence integrated into decisions (e.g. trial design and conduct changes implemented, how did patient preferences research impact drug development plans)?
Impact	Did the involvement activity result in change (e.g. accelerated trial recruitment, increased diversity)?

Indicators need to be included that help to measure and communicate the quality and impact of the interactions (Robinson and Gorbenko 2022).

Pharmaceutical companies should choose such measures—or key performance indicators (KPIs) related to quality, effectiveness, and impact—to demonstrate progress and value. The different categories of KPIs to be considered are described in Table 31.2.

Frameworks for measuring impact are emerging, for example from the European PARADIGM consortium (Vat et al. 2020). Which indicators are meaningful, however, will depend on the strategic and operational objectives, the purpose of measuring, and the target audience (Gorbenko et al. 2022; Robinson and Gorbenko 2022; Klein et al. 2025). How consequently they are implemented and applied by pharmaceutical companies may be limited by the complexity of the measures.

Despite variability in the measurements, transparent quantitative and qualitative KPIs for patient focus along the medicine life cycle may support the systematic collection of patient input and patient-based research and their consideration at each stage of drug development. Finally, KPIs help accelerate organisational change by demonstrating the value of the involvement and thus reducing resistance.

In addition, there is a need for changing attitudes. For example, it needs to be clear that involving patients means bringing their perspectives into the organisation (listening) instead of educating or informing patients to share the company perspective of the product value. Furthermore, a two-way dialogue requires the openness to partner with patients and to provide feedback to them about how their input has informed decisions of processes.

This change in the organisation can be supported by ensuring the role of the control and risk functions (e.g. compliance) is enabling, so that risks (of involvement and non-involvement) are evaluated and mitigated through procedures that foster appropriate interaction (dialogue, partnering, research) and eliminate perceived risks or challenges of inappropriate influence.

Those companies who succeed in aligning internal attitudes, will achieve an integrated approach across departments faster. Consistent communication, training and guidance will also support cross-departmental and cross-geographic understanding and alignment of the internal workforce.

To move from traditional relationship focused participation (e.g. presentation of patient testimonies, patient workshops and symposia) to the delivery of robust collection of patient experience data (US Food and Drug Administration 2021, see also Sect. 3.2.1) including direct patient input into the medicines lifecycle (participation) and patient-based evidence (Chap. 3), the company's patient involvement specialists must transition from relationship-dependent interactions only to adopting scientifically recognised data-based approaches—such as patient preferences research—and apply them to the decision criteria along the lifecycle including development, value dossier, and commercial messaging (IMI PREFER 2025).

Data-based and actionable feedback will be instrumental in maintaining and strengthening the relationships with the patient stakeholders. Fair representation principles support meaningful interaction with patients and ensure broad relevance across communities. Beyond functioning as the engine of change, implementing, measuring, and reporting progress in patient focus, selected measures or indicators may benefit the company's reporting related to Environmental, Social, and Governance (ESG) aspects, that are receiving increased attention from investors and public stakeholders alike. More specifically, patient involvement can directly contribute to the social aspect of ESG reporting (Novartis 2024) and initiatives are going on to develop good practices for this (PFMD 2024).

The extent of progress in patient involvement among pharmaceutical companies is variable. Some are hesitantly testing the waters, a smaller few are taking bolder action by radically changing their processes. Zvonareva et al. (2022) categorised patient involvement in drug development into two dimensions, the depth (setting up research and development programmes, co-designing studies, finetuning details) and the intensity of participation (patients leading, partnering with patients, involving patients, consulting patients). They observed that participation often relates to fine-tuning or co-designing studies without impact on the actual research design. However, they found no report of patients leading the research (highest level of participation) and only few examples of a partnership approach (ibid.). Despite high expectations for patient involvement, only a few companies fully commit and apply the same rigidity to patient involvement as to other processes (see also Sect. 31.2.3). Gaps typically include organisational goal setting, measurement of progress, and improvement cycles.

31.2.2 Contextual Challenges

As in industry, the global landscape for patient involvement in both regulatory and HTA processes is scattered and far from being aligned or fully established (Dinboeck et al. 2024). While there is a move for greater collaboration among the authorities across countries, for example the Joint Clinical Assessment across countries in Europe (European Commission 2021b), strong national policies with independent dedicated processes for patient involvement are maintained (Part III). Without universally accepted external standards for integrating the patient perspective, industry

must rely on their own standards and frameworks for an integrated approach. Existing standards from other sources such as the Values and Quality Standards developed by the PCIG (Box 1.1) or Arnstein's ladder of participation (1969) may support industry stakeholders and offer orientation. However, the lack of accepted standards across the organisation remains a challenge for company-wide implementation.

One barrier that exposes companies and patients to risks when engaging with each other is the potential that later in the life cycle regulatory or HTA authorities may perceive conflicts of interest. Most HTA bodies ask for declarations of interests and give guidance on when patient stakeholders are perceived to be at risk for conflict of interest. Some exclude any individual or organisation that has been involved in drug development, others set limits on the percentage of financial support or the limitation of action period between the industry and the HTA involvement, some require interests to be declared and documented (National Institute for Health and Care Excellence 2022) and will assess potential conflicts for each specific case. Pharmaceutical companies should be aware and judicious of these rules and provide for sufficient protection for patients to ensure their involvement in subsequent steps is not undermined carelessly.

Of great concern are also the remuneration and refund of expenses to patients for the time they invest in providing advice to companies or product teams throughout the product life cycle which is subject to laws or regulations. Remuneration for involvement efforts (fee for service) should not be confused with patient organisation funding (sponsorships, grants and donations). As there is little public funding for patient organisations to be involved in public health related activities (supporting patients, educating, awareness building, conducting patient-related research, or participating in research, regulatory or HTA processes) patient organisations often seek funding from technology developers. Despite most companies having governance frameworks that mandate transparency reporting and not being the sole source of funding, there is a concern by decision-makers that patient organisations will be biased by this funding.

Like all other stakeholders, patients should be reimbursed for their time and expertise. However, these payments must be appropriate, legitimate and lawful and not perceived as biasing, unduly influencing, or even corrupting (Novartis n.d.). Orientational guidance for fair payment mechanism (Fair Market Value) has been proposed by the National Health Council in the USA (NHC 2021) or the Patient Focused Medicines Development (PFMD) initiative (Finnegan 2022) building on previous collaboration with WECAN (Workgroup of European Cancer Patient Advocacy Networks) (Geissler 2019).

There is little consensus across HTA bodies on the expectations for industry in involving patients or whether patient involvement includes participation or relies on the use of patient experience data/patient-based evidence, or a combination of both, and how to report this evidence (Bertelsen et al. 2023). Despite some interest in patient experiences or patient preferences, not much is implemented as a standard for how these should be measured, submitted, or considered/evaluated in the HTA or regulatory process (Janssens et al. 2023).

Likewise, HTA dossier formats for submissions by health technology developers lack dedicated space for presentation of research into patient aspects (Chap. 3) or summarisation of methods of patient participation and the difference it has made. With health technology developers investing extensively in collecting data for the patient perspective and engaging with the patient community over a long time, it is a waste of effort and resources—particularly that of the patients—not to submit this evidence to the HTA within the formal submission dossier. Reviewing the evidence on patient insights, data, and learnings generated by the developers with patients during the innovation processes would allow HTA bodies to better understand how patient perspectives have influenced medicine’s development and identify gaps to focus their own involvement processes. This was also recognised in Australia’s 2024 HTA Policy and Methods Review (see Chap. 14) with the clear recommendation to the Australian Government that guidelines and all materials should be updated to clearly define “how both consumer evidence (research into patients’ needs, preferences, experiences and perspectives) and consumer input arising from engagement processes may be integrated into HTAs” (Health Technology Assessment Policy and Methods Review Reference Committee 2024 p. 162).

This chapter calls for better alignment and dialogue between HTA bodies and industry throughout the product life cycle to ensure that the right products are developed with the right trial design and endpoints to enable HTA bodies to assess the benefits and value to the patients and the healthcare system meaningfully and effectively.

31.2.3 Making a Difference in People’s Perceptions of Medicines

In 2019, the Boston Consultancy Group explored high-level patient needs and how well pharma companies are perceived to meet them in the USA, Great Britain, Germany, China, and Japan (Lubkeman et al. 2020). The expectations were consistent across all regions, which should encourage the industry to develop global patient involvement strategies that are implemented across all markets. The most important items relating to patient involvement were:

- Trials that reflect real world and outcomes that matter to patients
- Providing transparent information
- Seeking patient input
- Communicating trial outcomes, including patient reported outcomes
- Involving patients in product creation
- Engaging patients via sustained relationships.

A striking performance gap in these expectations was noted with high variability among the 17 different global companies rated. Doctors indicated in this study that, if given a choice, they are more likely to prescribe products from companies that are putting more focus on patient-needs in their research and development programmes.

In Australia, CaPPRe and Patient Voice Initiative published a report, based on a patient preference study, which identifies gaps in current involvement activities by pharmaceutical companies and recommends that bio-pharmaceutical companies close the gaps by focussing on “genuine patient involvement across the product/treatment lifecycle” (2023 p. 31), proactively disseminating information on early-access opportunities, and demonstrating genuine interest in the expertise of the patient community on their needs and experiences (ibid.).

A study of a patient population in Sweden showed that as many as 50% of patients struggle with taking their medicine as prescribed which leads to unnecessary individual and societal costs (Fuller et al. 2021). When asked for their lived experience and attitudes towards medicine-taking, participants described medicines as “life-saving and indispensable”, “normal and a daily routine”, but also as “confusing and concerning”, “unsuitable without adjustment”, and “intrusive and unwelcome” (ibid. p. 5). Alleviating the risk for the latter three sentiments early by addressing the needs and routines of users better throughout development of the product (e.g. better formulation and dosing, information material, improved support functions), could help avoid much damage and waste, and thus, improve health system effectiveness and patient outcome.

31.3 Final Reflections

Can the Pharmaceutical Industry afford to ignore ‘the patient voice’ or the evidence reflecting patient lived experience and perspectives in the value proposition or in any other element of the medicine life cycle? External expectations have accelerated in the past 4 years with the FDA, European Medicines Agency (EMA) and National Institute for Health and Care Excellence (NICE) guidance as well as WHO and the updated Helsinki Convention calling for robust patient involvement throughout the life cycle of medicine development. Companies may risk failing to demonstrate patient-value when facing HTA due to a lack of data demonstrating what is relevant to the health or care of patients in their day-to-day lives. Not addressing the patient perspective and their lived experience during the clinical development may have severe consequences for the approvability and marketability of new medicines.

Change is happening! In this chapter, we have explained some of the elements and success factors for such change. However, there are still many barriers to overcome for pharmaceutical companies to fully embrace the benefits of early and systematic patient involvement throughout the life cycle. Among these barriers are structural perceptions and awareness gaps, competing inter-divisional interests in terms of budget and stakeholder hierarchies, as well as distrust in patient expertise or qualitative research.

External barriers such as recognition of the robustness of patient-based evidence and its use by regulatory or HTA agencies need to be addressed. Pre-competitive collaboration such as the European Innovative Health Initiative (IHI), that fosters multi-stakeholder public-private partnerships, may help to drive further

development and broader recognition. Examples for such pre-competitive collaborations are the Project Hercules (Chap. 6) or many IHI projects including PREFER, PARADIGM, or PaLaDIn (IHI 2025).

Change will be accelerated by sharing accounts of impact and success from companies that are the leaders in developing medicines *for patients with patients* by systematically integrating patients' perspectives on value.

Acknowledgements We would like to thank David L. Grainger, for comprehensively outlining in the first edition of the book the imperatives for moving to patient involvement in medicine development and assessment and thus, laying the foundation for the changes that have happened since.

All content as presented reflect the authors' personal experiences and insights and are not representative for any specific organisation, institution or for their employers. None of the authors received funding for writing this manuscript. MD is employed by Novartis AG in Switzerland, AM is based in France and employed by UCB, EHD is employed by Aparito Ltd., a wholly owned subsidiary of Eli Lilly and Company in the UK, APH is employed by Health Outcomes Strategies GmbH, a company in Switzerland consulting health technology developers. The content of this chapter is not intended to establish any legally enforceable rights, obligations, or commitments on the authors or their employers.

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Chapter 32

Patient Involvement in the Life Cycle of Medical Technology Development



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32.1 Introduction

The term medical technologies (medtech) describes health technologies such as medical devices, diagnostics, medical imaging, health informatics, digital health and communication technologies. Traditionally, health technology assessment (HTA) has not been required as routinely for medtech as for pharmaceuticals. Some suggest this is because they have short product life cycles and represent a lower budgetary impact on the healthcare system relative to drugs (Ming et al. 2022). However, it is more likely the large number of new medical technologies available in a year, and their varied impact on the health system, that challenges HTA resources. Hence in many jurisdictions, HTA is limited to high risk or high impact devices.

Medtech differs from pharmaceuticals because their implementation or use (e.g. a surgical implant) is in many cases more dependent on clinicians' involvement and expertise, and because device life cycles are often iterative; modifications or improvements to the debut technology are made after the initial launch. Iterative development pathways challenge HTA efforts, and frequently require separate

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processes and methods (Ming et al. 2022). However, there are some cases where patients self-administer devices (such as inhalers, closed loop insulin systems, virtual reality systems, digital technologies, etc.). In other cases, patients may need to prepare for use of the technology in a manner that is intrusive (e.g. bowel cleansing before colon capsule colonoscopy). In both use cases, issues of user acceptability and ability to use the device shape the evaluation of value.

The differences between medtech and pharmaceuticals also influence the domains considered in HTA. When medtech is used for treatment rather than diagnosis, the device-user interaction (clinician or patient) can influence the clinical outcomes achieved. There is a recognised 'learning curve' where users develop skills for optimal use of the device and achieve best outcomes. Consequently, medtech HTA often addresses organisational issues such as how the technology will be introduced into the health system (delivery and service) and aspects of quality such as minimum volumes of surgical procedures performed to ensure good outcomes or the training and education that is needed for patients.

A substantial difference between HTA for medtech and medicines is that medicine regulators require evidence of quality, safety and efficacy, whereas a risk-proportionate model is used for medtech. Although high-risk medtech requires evidence of quality, safety and performance, for many lower-risk devices, trials in humans are usually not required. Hence the level of evidence available to HTA is often less for most medtech.

It is important to consider patients as users of some medtech. Even when evidence is available for an HTA, it often focuses on how accurate a medical technology is or its short-term uptake and effects. Integrating patient aspects into medtech HTA bolsters the determination of the overarching value of the technology and its fitness to support effective and sustained entry into the health system. Some HTA bodies have recognised this (Chaps. 17, 18, 24, and 27) and built involvement methods that are bespoke for medtech HTA or adapted their medicine HTA processes. These processes have often extended the familiar participation approaches used for medicines HTA to include both primary and secondary research into patient aspects.

We present the need for medtech developers to leverage multidisciplinary expertise to plan and undertake robust research into patient aspects (Chap. 3) over the medtech life cycle that can inform medtech development, regulatory, post-marketing assessment and HTA processes. This may involve patient participation in clinical research (e.g. to determine outcomes of importance, the potential for patient-reported outcomes, etc.) or specific research into patient aspects such as patient preferences, ethnic differences in priorities for preferred outcomes, patient satisfaction, qualitative research with focus groups or interviews, etc. This research is sometimes called patient-based evidence or patient experience data (Bertelsen et al. 2025). This chapter outlines the value of patient involvement for medtech development and assessment and how it can be operationalised over the life cycle of development. It then explores two specific research approaches that are underused in HTA and identifies gaps and opportunities where additional effort is needed.

32.2 Patient Involvement in Medtech Development

Medtech developers are often driven by commercial goals and see clinicians as primary users, sole decision-makers and their customers. Many undervalue the clinician-patient collaboration that underpins shared decision-making, personalised care, and delivers insight into service principles that highlight what is important when making decisions about how to design medtech and deploy it within a service (Murray et al. 2024). If challenged, some developers assert that clinicians' views are adequate proxies for patients' perspectives and indirectly 'patient-centric'. However, such inattention to patients' experiences and preferences as a strategic asset may explain why some medtech developments have failed in the market, because patients did not use them due to lack of privacy, fear of stigmatisation, or the impracticality of using the device (Tase et al. 2022).

Involving patients and carers in the development and evaluation of medtech has many benefits, including:

- Improved satisfaction, uptake or adherence (Bombard et al. 2018; Krist et al. 2017; Vat et al. 2021)
- Improved health outcomes (Bombard et al. 2018)
- Potential cost savings by averting costly design changes and product recalls (Krist et al. 2017; Vat et al. 2021).

This augments the more general benefits of patient involvement in HTA that were covered in Chaps. 3 and 4.

Patients bring unique value to the product development and assessment process. They can critique usability and functionality by sharing and building on their experience of certain diseases or interventions. Medical devices, unlike medicines, can be modified frequently and relatively quickly. For example, if a manufacturer learns that a new coating could reduce the potential for infection when a device is inserted into the patients' body, the regulatory process to make that change is relatively simple. In addition, patients highlight the under-recruitment of key demographics such as women in medtech studies (Epstein et al. 2024). This iterative nature of medtech product development and regulatory approval offers more opportunities to incorporate patient input in the device development process than in pharmaceuticals. Figure 32.1 illustrates with the feedback loop that patient input into medtech may permeate and shape product modification after its initial regulatory approval. This could impact product adoption, uptake and effectiveness. Alternative risk-based regulatory pathways allow these modifications to be implemented without burdensome clinical trial requirements. However, this can result in a paucity of evidence available for HTA and so patient-based evidence and patient input can hold more weight than in the pharmaceutical setting. This potential evolution of a technology after regulatory approval can mean that patient involvement in HTA could add evidence about the safety and effectiveness of the latest version of the technology. In future, the potential for using real world data to collect this information could be more common.

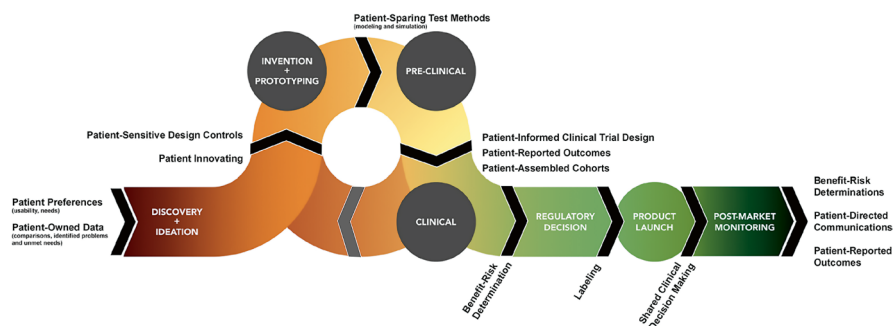


Fig. 32.1 Patient involvement preference information and the medtech product development life cycle. (Modified from non-binding recommendations in draft US Food and Drug Administration guidance (2024))

32.3 Incorporating Patient Aspects in Medtech Development to Inform HTA

32.3.1 From Product Development to Regulatory Approval

Medtech developers can involve patients throughout product development. As outlined in the first edition of this book, Table 32.1 provides examples of the aims and ways to involve patients at each stage (Wurcel et al. 2017). To align with the evaluation of other evidence in the HTA process, these methods should be assessed for their potential to introduce bias and/or lack of generalisability.

32.3.2 Opportunities for Patient Involvement in Medtech Clinical Trials

Medtech clinical trials present unique characteristics that create distinct patient involvement opportunities compared to pharmaceutical studies (Faris and Shuren 2017). Device trials often involve invasive procedures for implantation, present challenges with irreversibility as implanted devices may become permanent and require specialised approaches to control groups through sham procedures, all of which significantly impact patients' willingness to participate and their ongoing engagement throughout the study (Neugebauer et al. 2017). Unlike pharmaceutical trials where patients can discontinue medication, device studies may result in permanent changes to the patient's anatomy, creating unique psychological and practical barriers to recruitment. Additionally, once a device is implanted, patients may feel less connected to the ongoing study activities compared to drug trials where regular medication administration maintains the patient-study relationship, potentially leading to challenges in collecting follow-up data on side effects, symptoms, and long-term effectiveness (Gliklich et al. 2014). These

Table 32.1 Examples of different forms of patient involvement in medtech development (Wurcel et al. 2017)

Stage	Aim	Techniques
Stage I—Initial concept	When a new project is defined: • At country level—User needs and values are established, and scoping exercises are carried out (with users) • Globally—The product requirement document is developed	• Focus group • Individual contextual enquiry (interview with patients in their context, performing the activities of daily living, to gather raw data for later analysis) • Task analysis and usability tests (cognitive walk-through to see whether a new user can easily carry out a specific task related to a new medtech product) • Questionnaires • Interviews • Social media
Stage II—Validation and refinement	Validate and refine the concept	• Focus group • Individual contextual enquiry • Task analysis and usability tests • Questionnaires • Interviews • Delphi techniques
Stage III—Design	Iterative device design based on the needs and preferences of users	• Defined by regulatory requirement documents, and design for inclusive, user-centred devices and service delivery
Stage IV—Evaluation	• Pre-launch and post-launch ongoing continuous improvement process • Suggestions and questions about products gathered in countries and then reported through regional channels	• Usability tests • Heuristic evaluation (more holistic usability inspection) • Focus groups • Questionnaires • Interviews

device-specific considerations require patient engagement strategies that address concerns about invasiveness, irreversibility, and the complex benefit-risk evaluations that patients must undertake, necessitating more sophisticated approaches to informed consent, patient education, and maintaining patient connection throughout the study life cycle to ensure robust data collection and meaningful patient participation in device clinical research (US FDA 2022).

As stated throughout this book, defining the objectives for patient involvement are key to determining the most appropriate form of involvement. For medtech clinical trials, specific objectives can be defined considering the invasiveness of the technology, selection of outcomes, or population for study and trial design (MDIC 2021). Table 32.2 provides illustrative examples of the kinds of objectives that might be considered for each purpose.

When medtech developers progress towards engaging with regulatory agencies, they can meet an agency’s evidence requirements by using rigorous research methods to assess patient preferences and benefit-risk trade-offs, as outlined in the

Table 32.2 Device trial characteristics and their relationship to patient involvement objectives (MDIC 2021)

Category	Illustrative patient involvement objectives
Elements of level of invasiveness: • Implantable device • External device/impact on daily life • Reversibility • Impact relative to disease state (early disease vs later-stage disease)	Understanding how device trial patient participants evaluate the benefits and risks of undergoing an invasive procedure associated with use of a device (as compared with other potential therapy options) Understanding how patients might evaluate various procedures related to implantation and/or use of the device, in order to (1) best explain these elements to patients and (2) consider ways to mitigate concern regarding invasiveness/mode of administration of the device (where possible)
Elements of outcomes assessment: • Selection of primary and secondary endpoints • Selection of performance goals • Required follow-up • Reporting of side effects/symptoms by patients	Understanding what outcomes are most relevant to the patient population and incorporate those as endpoints (where possible) Understanding patients' views on risk-benefit priorities Identifying measurement and data capture approaches that are most acceptable to device trial patient participants to maximise successful adherence to trial protocol elements Leveraging patient input to reduce/eliminate burdens on trial participants (including appropriate stratification of patients and effective use of mobile technology, e.g. elderly, rural populations) and other means for tracking progress on trial (e.g. measurement of symptoms, side effects, patient-reported outcomes, overall outcomes) while allowing patients to remain connected to the study and the sponsor over time
Elements of trial enrolment: • Study population (including participation by under-represented populations) • Size of enrolment • Patient screening/inclusion and exclusion criteria • Education needs of patients (for successful participation in the trial)	Understanding patient desires for enrolling in the trial, seeking opportunities to adjust inclusion/exclusion criteria, and making it easier to accrue patients (where possible) to enhance opportunities for broader access among the target study population Understanding what information is most relevant for device trial patient participants in deciding to enrol in, remain on, and be adherent participants within a trial Evaluating best approaches for most successfully communicating this information to patients in order to develop and convey the most relevant messaging and materials Determining patient interest in receiving real-time (where possible) and follow-up communication/information about their own data and the overall success/failure of the trial (data sharing/feedback loop)

(continued)

Table 32.2 (continued)

Category	Illustrative patient involvement objectives
Elements of trial design: • Placebo/sham procedure • Crossover option • Single-arm studies • Unblinded studies • Adaptive design • Device modifications during the trial • Use of real world evidence (RWE)	Understanding how device trial patient participants feel about a variety of trial design aspects (including use of placebo/sham, lack of control group, unmasked study arms, adaptive design, and potential to modify the device during the trial) to (1) adjust the design if possible/where necessary to facilitate success and/or (2) position patient-facing messaging/materials to speak to/address patients questions/ concerns. Understanding patient perspectives on best ways to reduce/eliminate burdensome study protocol requirements including leveraging RWE and other innovations in clinical trial conduct

Medical Device Innovation Consortium (MDIC), the Patient Focused Medicines Development (PFMD) and IMI PREFER frameworks (Feldman et al. 2021; IMI PREFER Consortium 2022; MDIC 2015). MDIC also offers a framework that provides insights into how patient preference information can be incorporated into various aspects of clinical trial design, including: informing and prioritising endpoint selection (including development of weighted composite endpoints); and informing endpoint performance goals, the uncertainty (alpha error) and sample size (Rincon-Gonzalez et al. 2023). An example is shown in Box 32.1.

Box 32.1: Case Study: Patient Preferences Used to Design a Medtech Clinical Trial

People diagnosed with severe mitral valve regurgitation have a 34% likelihood of death or heart failure readmission within 1 year (Messika-Zeitoun et al. 2020). Few treatment options beyond open-heart surgical intervention exist, so medtech developers identified less invasive approaches. One approach explored repairing a valve through a small incision on a beating heart, but its efficacy rate was unknown and potentially less certain than the conventional surgical standard of care.

Using techniques outlined in the MDIC and IMI PREFER frameworks, a medtech sponsor worked with patients and the Food and Drug Administration (FDA) to assess the target patient population’s tolerance for risk and uncertainty about whether the technology would work, in exchange for the trade-off of a less invasive approach. A patient preference study concluded that patients were willing to tolerate significant risks and uncertainties in exchange for the combined net benefits associated with a ‘beating heart’ treatment. On average, patients were willing to accept a maximum acceptable risk of 34.6% points for increased risk of symptom reappearance or 22.6% points for increased risk of reintervention (Janssen et al. 2022). Based on these patient preference data, the sponsor obtained FDA approval to proceed with a clinical trial that would approve the device even if it was only 80% effective (versus the surgical standard of approximately 98%).

32.3.3 *During HTA*

For medtech HTA it is common for HTA bodies to undertake literature searches to gather the evidence required rather than take submissions from health technology developers. When this is the case, they may identify patient aspects reported in clinical or economic studies or conduct specific literature searches to determine patient aspects. There is use of qualitative research for different purposes in HTAs (Germeni and Szabo 2023). For example, the Swedish Agency for HTA and Assessment of Social Science frequently employs Qualitative Evidence Synthesis (Chap. 11). Qualitative Evidence Synthesis draws on traditional skills found in HTA of systematic literature searching and can identify and synthesise a range of international studies about patient needs, experiences and preferences but has a range of nuances that are different to searching for clinical effectiveness including the publication databases search. There is increased use of primary qualitative research including ethnographic research (Chap. 10), particularly interviews and focus groups. Both approaches are used in Wales (Chap. 27) and Spain (Chap. 24), and Belgium uses a co-development approach to its research into patient aspects (Chap. 15).

Patient preference research may be used to gain understanding of patients' trade-off assessments, estimating treatment uptake and adherence, estimating quality-adjusted life years (QALYs), gauging indication-specific treatment attributes to formulate overall benefit scores, assessing the payer's/patients' willingness to pay, and assigning weight to criteria pertinent to HTA such as health gain, cost, and disease severity (Marsh et al. 2021). Germany (Chap. 19) piloted two patient preference research studies (Chap. 9) and although their use has not been systematised by the HTA body, such research is considered as part of the evaluation of scientific literature for medicines. Box 32.2 outlines an example of the use of patient preference information alongside other participatory input in Ontario for a medical device, but there are few other such examples of use of patient preference research in HTA. Hence Sect. 32.4.1 outlines the potential for further use of this research.

Box 32.2: Case Study: Patient Involvement in Canadian HTA

Transcatheter aortic valve replacement (TAVR) is a minimally invasive procedure used to treat aortic valve stenosis in patients deemed at low surgical risk. The Ontario HTA Committee (OHTAC) reviewed TAVR to determine its efficacy, safety, and cost-effectiveness.

OHTAC's decision-making process involved evaluation of clinical evidence, economic analysis, and input from various stakeholders, including healthcare professionals, patients, and industry experts. The committee scrutinised data from clinical trials, real world studies, and health economic assessments to weigh the benefits and risks associated with TAVR compared to conventional surgical aortic valve replacement (SAVR).

OHTAC conducted a systematic literature review and identified quantitative and qualitative patient preference data played a key role in the decision-making process. Patient preference data revealed that patients, especially a key target population of older patients, accepted the risks associated with TAVR such as the potential need for a pacemaker and the knowledge that TAVR is a new procedure because they preferred the less invasive procedure.

OHTAC obtained patient input from patients who had undergone TAVR. Sharing their lived experience through testimonials (Health Quality Ontario 2020), they highlighted the procedure's positive impact on their lives, emphasising how much they valued the ability to resume normal activities sooner and reported improved overall well-being compared to conventional surgery. According to Health Quality Ontario, this was a factor in their decision to recommend expanded coverage of the technology.

Not all HTA bodies have the capacity or remit to undertake primary or secondary research to understand patient aspects. Some rely on participation approaches, with structured inputs from patient organisations and patient contributions to scoping and/or appraisal deliberations, such as in Australia (Chap. 14), England (Chap. 17), and Singapore (Chap. 22).

For medtech HTA where submissions from health technology developers are required, submission templates may only allow structured presentation of clinical, organisational and economic evidence. Unlike the FDA, HTA submission templates do not all have a separate section to submit research into patient aspects. Consequently, the developer needs to find a way to articulate integration of patient involvement over the life cycle of technology development. This can, for example, be weaved into the explanation of the design, conduct, choice of outcomes, analysis, and reporting of studies.

32.3.4 *Post-market Assessments*

As described earlier, the iterative development process unique to medtech products can support improvements in user experiences and satisfaction, clinical outcomes, treatment efficacy, and/or cost-effectiveness through patient involvement. Post-market assessment is a critical component of medtech HTA, providing invaluable insights into the real world adoption, utility, and performance of medtech.

Importantly, post-market assessment provides opportunities to include the behavioural aspects related to the use of the technology, the lived experiences and user preferences of the people using these devices. For example, it enables collection of patient-reported outcomes, patient-centred outcomes, and patient preferences information. Methods to gather post-market patient perspective and patient experience data include the following:

- User surveys of healthcare professionals, patients, and other users that can provide valuable insights into device performance and user satisfaction (Chap. 3)
- Patient registries created for medtech that allow for systematic data collection (Wicks et al. 2023)
- Post-market clinical follow-up studies that augment clinical trials with real world evidence, as they involve monitoring a larger patient population to assess long-term safety, effectiveness and durability
- Focus groups with stakeholders (e.g. clinicians, patients, and carers) can stimulate in-depth discussions that can uncover user experiences, challenges, and unmet needs related to medical devices (Chaps. 3 and 10)
- Analysis of social media content across platforms that can provide real-time insights into device performance (Chap. 7).

The importance of post-market assessment is an early warning system for detecting usability and design issues that may not have been evident during pre-market testing. Timely identification of such issues and any revealed patient preferences enables manufacturers to be responsive and take swift action, including modifying the device's user interface, labelling, or usage instructions. The evidence generated in this stage can also be valuable for regulators and HTA, recognising patients' key role as stewards of the safety, sustainability, value, and effectiveness of the technology.

32.4 Specific Forms of Research into Patient Aspects That Could Be Valuable for Medtech HTA

32.4.1 Patient Preference Research

To facilitate the incorporation of patient involvement in medtech product development and decision-making throughout the product life cycle, patient preference research can be particularly valuable. This is because a new medtech may be radically different in terms of its delivery (keyhole vs open heart surgery), operation (hospital visit vs home application) and outcomes (hospital length of stay, repeated assessment) than the current practice with which it will be compared in HTA. Given the complex stakeholder landscape in medical technology development and decision making, incorporating robust patient perspectives alongside clinical evidence has become essential. This need is particularly acute for medical devices, which historically reach market with less evidence than pharmaceuticals. Patient preference research can provide additional evidence needed to demonstrate that new medical technologies genuinely address patient priorities and values across all aspects of its delivery, operation and outcomes.

Preference research can be used to identify attributes of a medical technology that are important from patients' perspectives and an analytical structure to estimate the value of the attributes in the context of trade-offs. Furthermore, the use of patient preference research in HTA has advanced in recent years (Chap. 9) with relatively novel methods and frameworks to formalise both qualitative and quantitative patient preference information that can help support the medtech product development and decision-making processes. As outlined by researchers (Ho et al. 2016) and endorsed by the FDA (US FDA 2016) to assist developers and regulators in the collection and assessment of patient preferences, these methods involve direct interaction with patients, and the collection of feedback from patients, using structured interviews (qualitative), surveys or tests (quantitative) designed to elicit their preferences.

Since then, FDA has published several guidance documents that reference the use of patient preferences in product development and clinical trial design. The IMI PREFER project expanded and refined MDIC's original 14 methods of eliciting patient preferences to generate quantitative data (IMI PREFER Consortium 2022) and explored the use of such research in HTA. The European Network for Health Technology Assessment (EUnetHTA) was heavily involved in the development of IMI PREFER's framework which was subsequently recognised in Qualifications of Novel Methodologies Opinion for the European Medicines Agency and later that year (Committee for Medicinal Products for Human Use 2022). In addition to these useful tools, an International Society for Pharmacoeconomics and Outcomes Research (ISPOR) task force has published a roadmap for ensuring patient preferences are most useful to healthcare decision-makers—from regulators to HTAs (Bridges et al. 2023).

These methods have the potential to provide robust research that simultaneously optimises the representativeness and reliability of evidence about important patient aspects. The research can be undertaken at various points during the medtech life cycle to inform its development, regulatory evaluation and HTA (Chap. 9). Medtech developers and assessors need to demonstrate/confirm that the processes and methods assure the data are from an appropriately diverse target patient population, and that risk of bias is minimised. This need was highlighted by the example in which the limitations of pulse oximetry for all skin tones during COVID-19 were exposed (Sjoding et al. 2020).

32.4.2 Generation of Real World Evidence

Real world evidence (RWE) facilitates the ongoing evaluation of medtech throughout its continual iterative product development and could be particularly valuable in post-market assessments. The FDA has worked to incorporate RWE into its decision-making effort, having defined RWE as “information on health care that is

derived from multiple sources outside typical clinical research settings, including electronic health records (EHRs), claims and billing data, product and disease registries, and data gathered through personal devices and health applications” (Sherman et al. 2016, p. 2293). In addition to FDA’s efforts, the European Medicines Agency (EMA) has established a framework for use of RWE to support regulatory decision-making (EMA 2023).

RWE generated from data collected during routine clinical practice may help close the evidence gap between clinical research and practice and to understand patient’s health experiences and preferences in cases where outcomes that matter to patients are not or cannot be collected in a clinical trial. For example, novel outcomes may be collected in the real world and simple patient experience questions or patient preference information may indicate that the method of delivery of a medical technology could be important to a patient’s treatment decision or that there is a challenge in its use (and therefore impact uptake/utilisation/effectiveness considerations in HTA). As the demand for evidence in real world settings increases (Dang 2023), it is critical to incorporate RWE at every stage of the device development journey and this could fill important evidence gaps in HTA.

RWE can and should represent patients’ voices and experiences, in part through patient experience data, defined in U.S. law as data “collected by any persons (including patients, family members and carers of patients, patient advocacy organisations, disease research foundations, researchers, and drug manufacturers)” and “intended to provide information about patients’ experiences with a disease or condition” (US Congress 2016, 21 USC 360bbb–8c(c)). Incorporating patient input regarding values and preferences is already done in some randomised controlled trials through patient-reported outcomes but RWE can validate the values and choices in real-life usage. It may be generated from several real world data sources, including the following three ways.

32.4.2.1 EHRs

The volume and complexity of real world data available from electronic health records which can be analysed to generate robust RWE medical product evaluations has grown exponentially. Observational research can profit from the actuality of EHR data linked with longitudinal information that is routinely collected over the lifetime or length of treatment of the patient. For HTA, this information may be of specific value in reassessments throughout the life cycle of technologies.

32.4.2.2 Clinical Registries

Clinical registries are heterogeneous and numerous. Professional societies set up some of the largest registries. For example, within cardiology, the American College of Cardiology's National Cardiovascular Data Registry includes 14 unique registries encompassing both outpatient and inpatient care, including specific procedures such as implantable cardioverter-defibrillator placement.

32.4.2.3 Digital Health

Recent, rapid technological innovation has driven the development of thousands of health-related mobile applications, wearables such as fitness trackers, and technologies that can be synchronised such as digital weight scales and portable electrocardiographic sensors. These technologies generally involve personal data and offer the prospect of monitoring patients virtually. Advantages of digital health include the ability to capture detailed and longitudinal information, including patient-generated data and patient-reported outcomes. Because nearly all of people's activities occur outside of health care settings, these technologies can provide rich data for a more complete understanding of patients' experiences in the real world that could be beneficial in HTA.

32.5 Barriers to Patient Involvement and Possible Enablers

Table 4.3 in Chap. 4, outlines some barriers to patient participation in HTA. For medtech, patient involvement poses specific challenges for both medtech developers and in HTA. For developers, these include a lack of perceived confidence in the patient community's ability to successfully navigate and impact the HTA process, and concerns that collaborating with patients in the process could raise compliance considerations (Aillet 2021). Medtech developers argue that, in comparison to medicine developers, they have fewer resources and shorter product development timelines, both of which work against long-term engagement with patient organisations. Furthermore, there is a perception that patients are often not aware of medical devices or involved in the decisions to use them so they may not have an informed opinion.

To overcome these barriers, more examples and case studies of how patient involvement can facilitate HTA of medtech would be helpful. For HTA, the specific barriers are outlined in Table 32.3 along with potential solutions.

Table 32.3 Barriers and potential enablers to increase patient involvement in medtech HTA

Barrier	Potential enablers
Scarcity of HTA processes that have systematic approach to patient involvement in medtech HTA	• Patient involvement early in the medtech development should be recognised as key evidence for the assessment of medical technologies, e.g. patient preferences can inform choice of outcome measures and endpoints in randomised controlled trials and other clinical research • HTA should acknowledge that how patients interact with devices directly affects uptake, adherence and effectiveness of many medical technologies • All entities assessing medtech should require active patient involvement in product development • Patient representatives should be on each committee that plays a role in assessing medical technologies • Patient opinions should be sought routinely throughout HTA evaluations, and multiple methods from one-on-one interviews to focus groups and online surveys should be considered to gather quantitative and qualitative data
Organisational change needed to facilitate patient involvement	• HTA bodies must recognise the need for patient involvement to comprehensively assess medical technologies, and steps should be taken and resources made available to meet patients where they are—to reach out to them and listen to their stories, rather than expecting them to struggle to make themselves heard • HTA processes must adapt to attract and enable participation by patients who represent the target audience—this may include mandating the provision of accessible and plain language summaries of publications and medtech documents • Patient input must be made as easy as possible, by taking active steps to enable participation (e.g. reimbursement for travel and childcare, travelling to patients' homes, and meeting at their convenience) • It is important to follow-up with patient participants to tell them how their input was used and reiterate its value. To ensure this support is provided consistently, a dedicated expert member of staff should have responsibility for patient involvement activities
How to ensure individual/disease-specific patient representation	• As a standard practice, all HTAs on medical technologies, should actively seek patients willing to provide input • Individual patients may be approached through online forums, social media, patient organisations (including umbrella organisations) and/or relevant charities • Individual patients may be asked to participate in online consensus building processes with clinicians and other stakeholders

(continued)

Table 32.3 (continued)

Barrier	Potential enablers
Difficulty locating and engaging representative users	• Medtech developers and HTA assessors should ensure that patients and carers are given opportunities to provide input from early stages of projects without fear of critical questioning or judgment • It is crucial to ensure that patient participants represent current or prospective users and a diversity of experience (Staley et al. 2021) • Research should attempt to determine whether there are important differences between participants and those who opt out—similarly, it is as important to talk with patients who stopped using a medical device as it is to speak with those who continued • Patients should be informed about how their data will be used and when appropriate should be assured that their responses and opinions will be deidentified and will not be shared with their providers or payers • Patient participants can be empowered to serve as ambassadors, recruiting other patients or prospective users to provide input and feedback • Where necessary, patient participants can be recognised as intermediaries for other patients who are content to engage with them but do not wish for further active involvement (Staley et al. 2021)
Acceptance of carers' or family members' input as proxy for patient data	• Clear principles for working with children and/or people with disabilities should be established • Clear, culturally sensitive, age-appropriate information about the device and all survey instruments should be available in multiple languages • Responses requiring translation should be performed by validated interpreters or native speakers to prevent misinterpretation
Legal concerns	• A clear legal framework, including informed consent and when indicated, any regulatory approvals, should be in place for interactions between patients, manufacturers and/or HTA bodies

Adapted from Wurcel et al. (2017), Table 34.2

32.6 Conclusion

Involving patients in the development and assessment of medtech represents a pivotal shift towards more patient-centred healthcare. There are substantial challenges posed by the unique nature of medtech products, such as their wide variety, different form of use (implantable vs patient use), lack of patient knowledge about devices in development and use in their healthcare, and shorter life cycles of development. But some health technology developers proactively engage in early dialogue with regulators and HTA bodies for guidance on involving patients to incorporate their

perspectives and quantitative preference evidence earlier in development and throughout various stages of product development and assessment.

A wide range of research methods could be used to generate valuable research into patient aspects to contribute to HTAs of medtech as outlined in Part II. However, the country chapters in this book show that their use has been limited. This chapter has given specific examples showing the use of patient preference research given the recent work of IMI PREFER and ISPOR to explore its use in HTA.

We have explored how patients can inform a range of decisions throughout the medtech life cycle from product development, regulatory approval and HTA to post-market assessment, ultimately leading to improved outcomes, increased satisfaction, and better alignment with patient needs and preferences. However, significant barriers still exist. Few HTA processes undertake patient involvement for medtech HTA and when they do, involvement approaches used for medicines may not fit medtech HTA where identifying representative recipients of the technology can be much more challenging. Unlike regulatory submissions, HTA submissions do not have an explicit section where the health technology developers can report their patient involvement. Alongside this, few HTA bodies have the expertise to evaluate research into patient aspects. Addressing these barriers requires concerted efforts from stakeholders across the healthcare ecosystem, including medtech developers, regulatory agencies, HTA bodies, clinical teams and patient organisations.

If we are to create and deliver high value medtech that addresses patients' needs, it is imperative that patient involvement is embedded within medtech development and assessment processes. Until the inclusion of patients as stakeholders, medtech development too often felt like an unauthorised biography, detailing everyone's perspective except for the people at the heart of it. By actively seeking and incorporating patients' perspectives, experiences, preferences and needs early in the medtech life cycle, we can ensure that medtech not only meet clinical standards but also delivers meaningful benefits to those who rely on them most—the patients.

Acknowledgements The authors would like to acknowledge the authors of this chapter (previously Chap. 34) from the 2017 edition of this book for their framework and creative vision, including Victoria Wurcel, Sophie Cros, Sebastian Gaiser, Lisse-Lotte Hermansson, Katherine Jeays-Ward, Laura Norburn and Markus A. Ott. We thank Barbara Wexler for helping to prepare this updated chapter.

Liden and Kotlarz, have each spent more than 20 years working in the medtech industry and represent the perspectives of medical devices and/or in vitro diagnostic manufacturers. Liden serves as a paid consultant to medical technology developers. At the time of the writing of this chapter, Kotlarz was a paid employee of the Medical Device Innovation Consortium, an association supported with dues from the medical device industry.

Marshall is a professor and Svare Chair in Health Economics at the University of Calgary and did not receive any funding for work on this chapter.

O'Brien received honoraria as a patient representative for the MHRA pilot of the Innovative Devices Access Pathway. She has received honoraria as a member of a NICE medicines HTA committee. She has received honoraria as a public representative on funding award panels for NIHR's (National Institute for Health and Care Research) i4i (invention for innovation) and other research calls which may involve medtech; for UKRI (UK Research and Innovation) and the MRC (Medical Research Council).

Facey is an independent consultant who undertakes paid and unpaid work for HTA bodies and patient organisations and receives expenses to participate in meetings. She also undertakes paid consultancy work for health technology developers. She is an unpaid member of the Scottish Health Technologies Group Council, which appraises medtech in Scotland.

All views and opinions expressed in this chapter are those of the authors and are not necessarily shared by their employer organisations or manuscript reviewers.

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Chapter 33

Patient Involvement in HTA: The Journey Continues



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33.1 Introduction

Health technology assessment (HTA) is both a scientific and deliberative process, shaped not only by clinical and economic data but also by the broader context in which assessments take place—the setting, the domains considered, and crucially, the perspectives of those involved. These contextual factors influence how evidence

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is interpreted, what questions are prioritised and ultimately, the outcomes of the assessment. In this context, patient involvement is essential—not just as a matter of principle, but to ensure that HTAs are grounded in what truly matters to patients and communities. It is underpinned by foundational principles of relevance (ensuring assessments reflect what matters to patients), fairness and equity (ensuring diverse patient voices are heard), legitimacy (enhancing public trust and acceptance), and capacity building (enabling meaningful participation) (HTAi 2014, cited in Table 1.2). Patient involvement also serves multiple purposes: it strengthens the scientific relevance of evidence, improves the practical application of decisions, promotes democratic inclusion, and supports the development of more responsive systems and communities (OHTAC Public Engagement Subcommittee 2015, cited in Table 1.2). These combined aims highlight the importance of embedding patient involvement throughout HTA.

In the past 32 chapters, this book has explored the key concepts, methods and approaches for patient involvement in HTA, drawing on real-world examples from HTA bodies in over 20 jurisdictions, and highlighting the perspectives of patient organisations, developers of medicines and medical devices and academia. Throughout we have used the term patient in an inclusive sense—encompassing individuals living with or who have lived with a condition, those who care for them, organisations or groups whose primary aim is to advocate for or support patients, and people who have undertaken specialised training to represent patients. Patient involvement has been defined in two distinct, but complementary ways adapted from Facey et al. (2010):

- Research into patient aspects (patients' experiences, preferences, perspectives, needs)
- Patient participation in HTA (individual HTAs, methods, processes, governance, and policy).

Participation can elicit patient knowledge gained in the 'system', the 'self' and the patient's 'community' (Dumez and L'Espérance 2024). This knowledge can help interpret clinical and economic evidence in ways that align with the local context to inform individual HTAs, and shape HTA methods, processes and policy to

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better reflect patients' needs and priorities. As a form of social participation in public policy, it supports the legitimacy and transparency of HTA, helping to build public trust and strengthen the role of people and communities in health systems (World Health Assembly 2024).

Meanwhile, secondary research of published literature can provide robust evidence to address general questions, and primary research may address specific gaps that are relevant to, and only available from, specific patient communities. Participation and research into patient aspects each bring unique strengths and limitations. Combining these approaches in HTA allows us to triangulate evidence and provide a richer, more comprehensive understanding of patient perspectives. This requires a supportive structure that enables patient knowledge to be elicited, integrated, and applied consistently across HTAs. In this final chapter of *Patient Involvement in Health Technology Assessment*, we briefly summarise key developments since the first edition of the book in 2017, before reflecting on current practice and offering recommendations to strengthen patient involvement to more fully realise its value in healthcare decision making.

33.2 Developments in Patient Involvement in HTA

Since this book's first edition, there has been an increase in the adoption of HTA globally, bringing with it a rise in patient involvement. While some jurisdictions are in the early stages of developing their processes, others are now refining and embedding involvement more systematically across their organisations. Notably, this edition includes contributions from new jurisdictions, including some that not only had no formal patient involvement in place in 2017, but had not established HTA systems at that point. As with HTA itself, involvement is shaped by its context. Emerging experiences, particularly from low- and middle-income countries, offer new insights that challenge and expand existing understandings of how patient involvement can be conceptualised and implemented. Recognising the different types of patient knowledge (Dumez and L'Espérance 2024) opens new possibilities for addressing persisting gaps in traditional scientific evidence.

In the examples of how different jurisdictions apply patient involvement in Part III, there is evidence of a growing investment in training and resource development to equip patient communities to better understand HTA and contribute meaningfully to it. We also observe the expanding role of patients, particularly through the increased implementation of co-design and co-production processes. At the same time, methodologies for capturing patient aspects—such as patient-reported outcome measures (PROMs), patient reported experience measures (PREMs), and patient preference studies—have continued to evolve and strengthen. This growth is even more remarkable when we consider that it occurred during a time when the COVID-19 pandemic placed immense pressure on health systems, resources, and patient communities globally. The pandemic also revealed deeper societal fractures, where mistrust in institutions—whether scientific, governmental or

healthcare-related—has led to some populations not accepting and therefore, not accessing available quality healthcare. In response to this challenge, the 77th World Health Assembly (WHA) adopted a resolution supporting social participation in universal health coverage (2024). This signals the potential for a wider culture of participation and a renewed focus on the democratic goals of HTA and research. While the case studies presented in Part III of this book point to benefits of these broad developments, they also show that some aspects of patient involvement remain overlooked or under-resourced. The remainder of this chapter outlines key areas for action to strengthen and sustain meaningful patient involvement in HTA.

33.3 Strengthening Patient Involvement in HTA: Seven Areas for Action

Introducing and developing patient involvement in HTA is not easy. As detailed throughout the book (Chaps. 4, 28, 31, and 32), the barriers are well documented, and the progress described by the authors has often been hard won and still may remain fragile—vulnerable to resource constraints, competing priorities, staff turnover, the beliefs of leaders and broader policy trends. Considering these challenges, there are seven key areas where many HTA systems could benefit from greater focus and investment. These are discussed in more detail in the following sections.

33.3.1 Implementing Patient Involvement Beyond Individual HTAs

Patients should be involved across the entire life cycle of health technologies, and long before HTA and coverage decisions are needed. Emerging examples show that the patient community can not only contribute to or co-develop research, but can also initiate, lead, and shape research to reflect their experiences and priorities (Project Hercules, Chap. 6). Additionally, this book presents examples of patient involvement throughout the innovation process—from early-stage discovery and research to the planning and implementation of clinical trial protocols, and even through to performance testing or improvement once the technologies are in use (Chap. 31, Fig. 31.1). While many health technology developers claim to be patient-focused and to incorporate patient perspectives in clinical development, little is formally reported on the nature or impact of this engagement (Vanneste et al. 2025). As a result, it remains unclear what mechanisms are being used and how they affect research and development. Improved transparency and reporting of patient involvement, and the impact it had on research/development decisions, could enhance the evidence submitted to HTA bodies and reduce the burden on patients to duplicate their inputs for both developers and decision-makers.

Additionally, although there has been growth in the generation of patient-based evidence by health technology developers, this has not been matched by its use in HTA submissions or subsequent appraisals. This stands in contrast to the regulatory environment in the United States (US), where ‘patient experience data’ has gained formal recognition. There, methodological guidance exists for collecting such data, and developers are expected to submit it alongside clinical evidence. In HTA, however, patient-based evidence often lacks equivalent standing, and many submission templates do not include space to report it or to indicate how patient aspects have shaped clinical trial designs.

Call to Action 1 *We call on HTA bodies to require documentation of patient involvement and its outcomes in the developer’s submission to inform the HTA, reduce duplication, avoid unnecessary resource use, and minimise the burden placed on patients.*

Further, we observe that participation is often narrowly conceptualised as something that occurs only during the short, pressured timeline of an individual HTA. This limited view not only strains the relationship building needed for meaningful sense-making and partnership with patient communities, but may also contribute to underinvestment in research into patient aspects (see Sect. 33.3.3). Expanding participation to include policy and organisational levels creates space for more sustained engagement and deeper collaboration. Broader forms of involvement such as co-developing legislation (as seen in Czechia, Chap. 16), serving on HTA body boards or committees, co-developing and reviewing HTA methods and processes, and co-designing participation processes, enable HTA systems to more effectively incorporate patient needs, evidence and knowledge.

Call to Action 2 *We call for greater attention to participation before and after individual HTAs and at the organisational and policy levels as essential elements of good practice.*

33.3.2 Investing in and Applying Research into Patients’ Needs, Preferences, Experiences and Perspectives

The definition of HTA emphasises its multidisciplinary nature and its reliance on state-of-the art methods to assess the best available evidence to determine the value of a health technology, including the implications for patients, their families and carers (O’Rourke et al. 2020). This definition also acknowledges that the concept of value may vary depending on the perspective taken, the stakeholders involved, and the decision-making context. Taken together, these elements underscore the importance of clearly defining the objectives of patient involvement. Is the goal to elicit perspectives and experiences to address specific research questions related to the use of a health technology? Or is it to bring insights from patients, families and carers into different stages of the HTA process—shaping its scope, methods, and

interpretation of results? Too often, the former—patient-informed research to address specific questions—is overlooked, despite its potential to strengthen the evidence base and make HTAs more relevant to real-world needs.

Part II highlights specific examples of research-based approaches to understanding patient insights. In recent years, there has been significant progress by international organisations in publishing methodologies and guidance, particularly in areas such as qualitative evidence synthesis (Chap. 11) and patient preference research (Chap. 9). Despite these advancements, there is still little recognition and uptake of this new work by HTA bodies. Instead, we continue to observe the use of more informal approaches to gathering and evaluating qualitative evidence, often based on interviews and focus groups, with limited methodological rigour. Still, only a few HTA bodies have actively invested in research into patients' needs, preferences, experiences and perspectives—whether through systematic reviews or primary research. This remains a significant gap, particularly compared to the level of investment in methods development in other areas of HTA. It was identified in the first edition of this book, and regrettably, has yet to be adequately addressed. Compounding this issue, few HTA bodies employ researchers with the expertise to critically appraise such patient-based evidence when it is submitted. One promising example of progress is RedETS (Chap. 24), which demonstrates cross-jurisdictional collaboration to build qualitative research skills and capacity.

Another shortcoming is the lack of emphasis on patient-reported outcomes, and as Chap. 3 suggests, the underlying reasons for this need to be explored. Challenges remain—for instance, while patients understand what it is like to live with a condition, they may find it difficult to imagine life without some, or all the condition-related effects, or to articulate what constitutes a meaningful improvement. This is particularly true for individuals who have lived with a condition since early in life and may lack a reference point for what a 'good outcome' looks like. These challenges, however, are not insurmountable. By expanding the evidence base and refining methodologies, we can elicit patient knowledge to inform a richer data set to better probe these challenging questions. Part II of this edition highlights promising approaches, including qualitative and quantitative research, social media analysis, the use of artificial intelligence (Chap. 7) and patient preference research (Chap. 9). Importantly, such research should be co-developed not only with patients, but also in collaboration with HTA bodies to ensure relevance and usability (Chap. 6).

This ongoing underinvestment in research has led to participation often being used as a proxy for research, blurring the distinction between the two and ultimately diminishing the value of both. The consequences extend beyond methodological confusion. Critically, it means we are failing to build a lasting, systematic evidence base of patients' needs, preferences, experiences and perspectives—evidence that could inform not only future HTAs but also broader research and policy development. For example, there is still no global database of the patient submissions made to HTA bodies over the past 20 years. This gap has significant implications for the patient community, contributing to unnecessary burden and repeated demands for

input (see Sect. 33.3.4). Without a stronger evidence base, we risk inefficiency, duplication, and missed opportunities for more person-centred decision making.

Call to Action 3 *We call on HTA bodies, technology developers, and stakeholders committed to improving health outcomes to invest in, publish, and actively use robust research into patients' needs, preferences, experiences and perspectives.*

33.3.3 *Broadening Participation in Research, HTA and Implementation*

To date, participatory approaches in HTA have shown limited effort to engage underserved populations or to critically examine who is represented by patient communities involved in the process. This is despite widespread awareness that those who often carry the greatest burden of illness are frequently unable to contribute to HTAs (Chaps. 2 and 4). Since the first edition of this book, several jurisdictions have introduced health policies that seek to protect and support the health of Indigenous Peoples. As a result, the role of HTA in either reinforcing or reducing inequities between Indigenous Peoples and the broader population has gained increasing attention. However, the picture remains mixed. For instance, HTA bodies in Canada have introduced specific policies in this area (see Chap. 4 example), whereas the disestablishment of the Māori Health Authority in 2025 has led to concern and uncertainty in Aotearoa New Zealand. Research into patient aspects (Chap. 3) holds promise for expanding who HTA can learn from—but, as discussed earlier, the lack of investment and/or skills to apply this research in HTA remains a significant barrier. The authors acknowledge the fundamental inequalities that exist between those who live with ill health or have caring responsibilities, and those who do not. As highlighted in Chap. 4, equity-centred engagement (Ul Haq et al. 2023) is essential for meaningfully involving underserved communities and has the potential to improve engagement with all patients. This type of participation requires specific training for HTA bodies, including in trauma-informed approaches (Sect. 33.3.4), and agreement on clear goals (Sect. 33.3.5). Fortunately, there is a growing body of practical resources and guidance to support broader, more inclusive participation in HTA and research (Chap. 4).

Needs assessments with patient communities, such as those conducted in Singapore (Chap. 23) play a valuable role in supporting and broadening participation. They help identify both the strengths patient organisations can build upon and the areas where further support is needed to enable effective input into HTA processes.

Such assessments might reveal needs such as a clearer understanding of the HTA process, equitable and two-way communication, and strategies for preserving patient knowledge (e.g. in data repositories). Importantly, they acknowledge that participation is a continuous learning process for all stakeholders. Collaborative approaches are essential to clarify both immediate priorities and those that can be

addressed later. This practice can also be adapted for individual HTAs. For example, Wales (Chap. 27) has adopted the Scottish model of creating bespoke involvement mechanisms for each HTA.

In contexts where health systems are hierarchical or where large, underserved populations exist, patients may feel they don't have the 'right' to contribute their perspectives. In such settings, structural dynamics may privilege the voices of other stakeholders, particularly healthcare professionals, over those of patients. These barriers highlight the need for further research to better understand and address cultural and systemic obstacles to inclusive participation. The 77th WHA resolution on social participation offers an important opportunity to support greater and more equitable involvement in decision making (World Health Assembly 2024). Patient participation plays a vital role in enhancing the relevance and legitimacy of HTA, but only if we are genuinely reaching the communities that experience lower levels of trust in the health systems. Lessons can be drawn from other marginalised or alienated communities that are connecting with their health systems, providing models for inclusive, respectful and trust-building engagement.

While most chapters in Part III relate to higher income countries, HTA is increasingly being adopted across the world to support decision making in lower resource settings (Chaps. 20, 21, and 28). As in higher-income contexts, patient or community involvement in these settings is still an emerging concept. There is significant benefit in HTA bodies networking and collaborating to ensure that context-appropriate and effective strategies for patient involvement are developed and implemented more widely, regardless of resource levels. As jurisdictions work to build needs- and evidence-based coverage systems, an intimate knowledge of the population-specific needs and socio-cultural context becomes essential. This understanding not only strengthens decision making but also reinforces the importance of meaningful participation. In practice, lower resource settings differ not only in how HTA is applied but also in the extent and way patients or communities are, or can be, involved in healthcare decisions (Holtorf et al. 2021).

Call to Action 4 We call on HTA bodies and technology developers to co-create and implement patient involvement frameworks that take into account the issues faced by all patients or carers, including those from underserved communities.

33.3.4 Building Capacity and Knowledge of Good Practice and Methods

While training for patients has continued to evolve—both to support their input into HTA and broader policy engagement—there has been far less focus on the patient involvement skills and knowledge of HTA bodies, evaluators and committee members. Most do not receive training on the nature and value of patient knowledge, principles of good practice in participation, how to conduct or appraise research into patient aspects, or the interpersonal skills needed to work effectively with patients.

As a result, even when formal processes for patient involvement are in place, there may be limits to what is heard, understood, and acted upon. The gap may stem from an underlying perception of patients primarily as stakeholders with a vested interest in accessing a particular health technology, and an assumption that patient communities lack the relevant knowledge because theirs differs from that of health professionals or policymakers. The emphasis on managing conflicts of interest and training patients—rather than shared learning—may also potentially stem from this view.

As a result, better use of patient knowledge remains a critical need in HTA. Dumez and L'Espérance (2024) offer a valuable contribution to the mindset shift required for genuinely working with and learning from patients. Their classification reframes the conversation, moving away from assumptions of interests and biases, toward one of “learning, dialogue and knowledge exchange” (ibid. p. 183). This shift is already reflected in some HTA systems, for example, in Germany (Chap. 19), patients are defined as ‘knowledgeable persons’. Addressing persisting epistemic injustice (Chap. 2) requires further work to ensure that HTA systems can fully recognise and draw from the different types of knowledge patients hold. This includes identifying what and when specific aspects of their medical, navigational, embodied, monitoring, relational and cultural knowledge are required to delineate the value of the health technology (Dumez and L'Espérance 2024).

Additionally, there seems to be limited recognition that patients and carers participating in HTA may have experienced trauma, or that staff from patient organisations may face emotional and psychological strain as they support their communities’ needs and expectations throughout the process. These challenges are not unique to HTA—they are relevant to patient involvement across all areas of the health system.

The Health Technology Assessment international (HTAi) Patient and Citizen Involvement in HTA Interest Group’s (PCIG) ‘Europe 360 study’ (Holtorf and Bertelsen 2023; Holtorf et al. 2024) offers several recommendations for good practice that can help address these issues. These include two-way communication, clarity on the purpose of patient involvement and what constitutes useful input/information, ensuring the relevance of questions to patient communities, active outreach, and creating a welcoming and respectful environment. Together, these practices can help encourage a wider part of society to participate in HTA but also minimise the risk of harm to those involved (see Chap. 4).

The authors acknowledge the importance of providing good quality information and fit-for-purpose training for patients who wish to be involved in HTA. However, situation-related frequent turnover among patient organisation staff and patient populations participating in HTAs result in knowledge being lost, sometimes within a short time period (Holtorf and Bertelsen 2023). While formal training for staff in HTA bodies, evaluation teams, and committee members is essential for supporting high-quality patient involvement, much can also be gained through cross-jurisdictional learning. Adapting processes and lessons from other systems to fit local contexts, such as the RedETS’ experience (Chap. 24), can strengthen local practices. Maintaining these networks for shared learning and collaboration is vital to the continued evolution of patient involvement in HTA. We, therefore, urge HTA bodies, patient communities, and health technology developers to continue working together through scientific societies such as

Table 33.1 Risks related to restrictive CoI procedures

Risk type	Background information
Funding	If patient organisations are excluded from participation due to receiving support from technology developers, alternative funding must be made available to ensure their continued functioning.
Participation along the technology life cycle	The progress in patient involvement across the life cycle of technology development (Chaps. 31 and 32) is welcomed, but those involved face a greater risk of being excluded from later HTA processes due to a CoI. To address this, it is important to establish practical, proportionate approaches that allow patient insights to meaningfully inform both the development and the assessment of health technologies, without penalising those who contributed to technology development in good faith.
Fairness	HTA involves a range of stakeholders, including clinicians, technical experts, and researchers. To maintain trust and fairness, CoI rules must be applied consistently and transparently across all groups—not only to patients and their representatives.

COI Conflict of interest

HTAi, particularly through its PCIG, as well as through the International Network of Agencies for Health Technology Assessment (INAHTA) and regional networks to share experiences and continually improve processes.

Call to Action 5 We call on HTA bodies to invest in appropriate training for HTA researchers, evaluators, and committee members to build the skills and understanding needed for meaningful patient involvement, and to better recognise the value of patient knowledge.

Managing Conflicts of Interest We recognise that fair participation and valuing patient knowledge in HTA processes depend on transparency and the responsible management of interests by all participants. No individual, whether patient, clinician, policymaker, or technology developer, should exert or be subject to undue influence by others. Any potential conflict of interest (CoI) should be declared through a formal *Declaration of Interests* procedure and managed using clearly defined, risk-appropriate processes. ‘Risk-appropriate’ herein refers to evaluating interests based on their potential to introduce bias. For example, recognising that the risk is generally higher for voting members of appraisal or decision-making committees than for individuals contributing knowledge as patients or carers (Chap. 5). While this may seem straight forward in principle, there are still unresolved issues regarding how patient interests are perceived and managed. Some of these are described in Table 33.1.

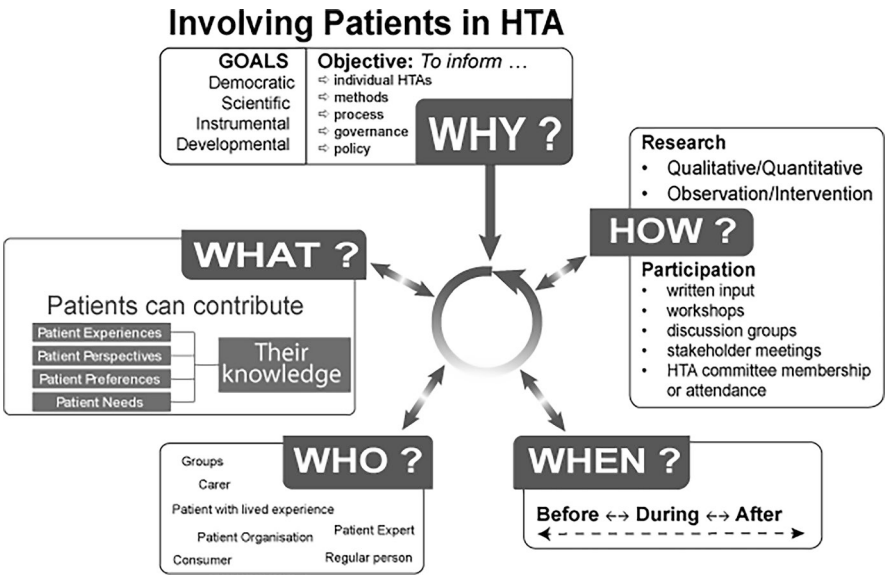


Fig. 33.1 Goals, objectives and methods to guide planning of obtaining patient knowledge

33.3.5 *Agreeing on Goals and Measuring and Communicating Impact Transparently*

Patient involvement is fundamentally about people—about learning from those eligible to use a technology and those who care for them. In our view, by beginning with the questions “who are the patients?” and “what can they bring to HTA?” we can create an opportunity for shared learning and a shift in mindset. This shift moves ethical concerns toward inclusivity and preventing harm, rather than primarily focusing on training patients or mitigating perceived risks of bias. As patient involvement can place an additional burden on patients, it should only be undertaken with clear and transparent communication about its goals, limitations and potential impact. Without these in place, it risks damaging trust and becoming ineffective, resource intensive, and ultimately frustrating and tokenistic for both patients and researchers (Chap. 4). We strongly advocate for careful planning and preparation of patient involvement in HTA, beginning with clear goals and objectives. This includes agreeing on the specific objectives of involving patients and then considering how specific types of patient knowledge are appropriately elicited and integrated (Fig. 33.1).

With explicit goals and objectives established, the tasks of providing feedback and measuring impact become clearer too. In terms of feedback to those who provide input or evidence, we can identify what did and did not help to achieve the objective. Additionally, we can articulate how this input was used with other input



Fig. 33.2 Evaluating process, quality and impact to refine processes for patient involvement in HTA

and evidence, what it was used for, and what mattered most (see Chap. 5 regarding epistemic justice). By being attentive to and documenting impact at the individual activity level, HTA bodies potentially not only build capacity and trusting relationships among patient communities but also lay the foundations for effective evaluation of patient involvement, including increased understanding of practices that best achieve goals, objectives, and outcomes, and how the process was experienced by participants (Fig. 33.2). Evaluating both process quality and impact was also a key recommendation of the Europe 360 study (Holtorf and Bertelsen 2023). Similarly, Silva et al. (2022) encourage HTA organisations to adopt a systematic and planned approach with regular evaluation when implementing or strengthening patient involvement practices.

Previous analyses have shown that evaluation of patient involvement is not yet done systematically across HTA bodies (Weeks et al. 2017, Chap. 12). This gap persists despite broad recognition that “patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA” (Value of Relevance in HTAi (2014), Chap. 1, Table 1.2). For participation to be truly meaningful, evaluation must reflect not only the perspectives of HTA bodies but also those of the patient communities who agreed to the goals and objectives of involvement (see three-domain framework by Lopez Gousset et al. 2024, Chap. 12).

Call to Action 6 We call on the HTA bodies to agree on their goals of patient involvement and to instil a culture of evaluation that includes two-way feedback, to monitor, strengthen, and continuously improve the impact and patient experience of patient involvement practices.

In addition, opportunities for *reflexive learning* may support a richer conceptualisation of patient involvement in HTA, generating insights to enhance both its practice and its impact (Gunn et al. 2021, Chap. 2).

Call to Action 7 We call on the HTA bodies to redouble their efforts to provide timely and specific feedback on how patient knowledge was considered alongside other forms of evidence and input, and to publicly document the impact.

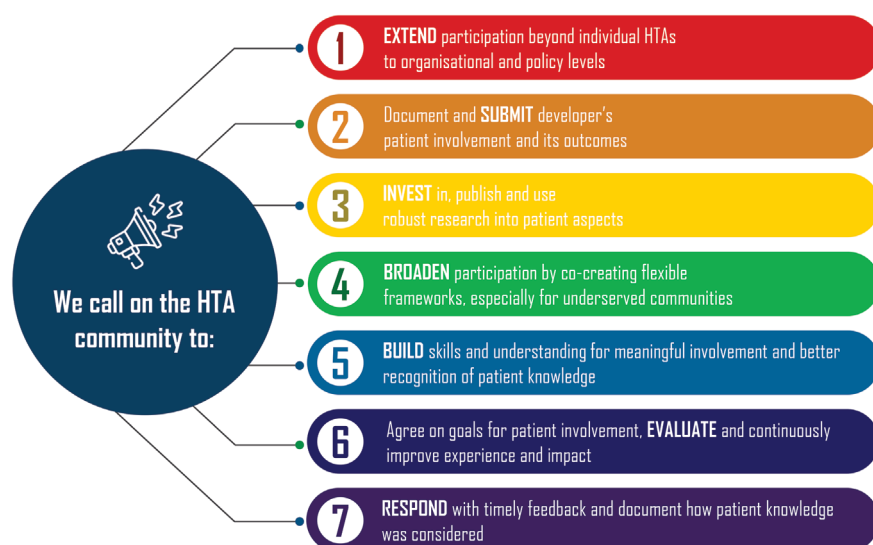


Fig. 33.3 Call to continue improving patient involvement in HTA

33.4 Conclusion

The practice of patient involvement in HTA has evolved significantly over the past decade. Today, we are better equipped than ever to define, describe and categorise the why, what, who, where, when and how of involvement. The experiences have reshaped the perspectives of HTA practitioners, patient communities, HTA bodies, and indeed, the authors of this chapter. Yet, the slow pace of adoption and the inconsistent level of involvement remain a source of frustration for many. To move forward, we must overcome the persistent framing of patient involvement as a threat to robust assessment, a view that continues to limit meaningful progress in this space. The improved understanding of patient knowledge along with the growing confidence in co-design, and the urgent call for social participation in healthcare decision making give us reason to be optimistic and to end our book with a call to action with attention to the specific areas for improvement as detailed in Fig. 33.3.

By following these actions, we can enrich and guide the continuing improvement of patient involvement, ensuring it becomes a lasting and valued part of HTA, and most importantly supports better quality care and outcomes.

Acknowledgements This book lives from the contribution of all the current authors and those who contributed to the first edition and the patients they learnt with. Specifically, we would like to thank Janet Wale, who not only co-authored the final reflection chapter in the first edition but also provided much leadership in the field through her academic work and her leadership in the Patient and Citizen Involvement in HTA Interest Group (PCIG) at HTAi.

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