Patients: Prime Drivers of Value and Access

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Patients: Driving Value and Access

- **Changing Role of Patients in Health Care Ecosystem**
- Regulatory Impact: Patients Assessing Benefit/Risk
- HTA Assessment: Patient Preferred Outcomes and Values
- Patients as Payers: Impacting HC System Budgets, Incentives and Direct Buyers of Products and Services
- Patients Determining Value: Behavior, Adherence and Outcomes
Patients: Central Actors in Complex Network Of Health Care
Key trends increasing patient impact on access

- Scientific opportunities for product innovation expanding rapidly
- Regulatory science and policies are allowing for early product approvals and increased patient participation
- Patient advocacy groups expanding their role in product development, approval and health care delivery systems
- Patients are paying for an increasing amount of health care products and services through premiums and out of pocket payments
Key trends increasing patient impact on access

- Patients and their caregivers have improved access to information about their conditions and potential treatments through the internet, peers
- Patients playing more active role in shared decision-making with providers and treatment selection
- Increasing importance of chronic vs. acute conditions mean patient behavior and choices are key determinants of treatment outcome (and therefore value)
Sanofi: Vision and Three Pillars for Patient Centricity

**INPUT & UNDERSTANDING**
Inform organizational strategies and decision making based on patients’ unique underlying needs

**OUTCOMES & SOLUTIONS**
Engage and support patients and other stakeholders to ensure solutions fit into people’s lives

**PLANNING & CULTURE**
Integrate patient-centered strategies into all business activities to drive 360° differentiation

People take control and improve their healthcare outcomes to **positively impact their health and lives**
Patient-Centered Focus And Engagement Will Drive Tangible Differences

**GAIN**
Deep understanding of patients’ needs

**SUPPORT**
Commercialization and Value Demonstration

**EMPOWER PATIENTS**
With solutions to improve outcomes and give people control

- Holistic needs
- Behaviors & choices
- Solution ideas & early buy-in

- Differentiated offerings & support
- Recognition of value
- Diagnoses, Disease Awareness

- Enrollment
- Adherence
- Long-term relationships
- Evidence generation

Outcomes

*Insights shape solution differentiation and value demonstration*
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Patient Engagement: FDA Benefit/Risk Framework

![Figure 1: FDA Benefit-Risk Framework](source)

<table>
<thead>
<tr>
<th>Decision Factor</th>
<th>Evidence and Uncertainties</th>
<th>Conclusions and Reasons</th>
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<tr>
<td>Analysis of Condition</td>
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<td>Current Treatment Options</td>
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<td>Benefit</td>
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<td>Risk</td>
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<td>Risk Management</td>
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**Benefit-Risk Summary Assessment**

Source: FDA.gov
Influencing FDA Approval: PPMD

- PPMD Goal – develop scientific evidence for caregiver preference for benefits and risks for emerging therapies
- Collaboration between patient groups, academic and industry researchers
- Cross-sectional study of caregiver preference for treatment benefit/risk – recruiting through patient advocate group
- Methodologically sound approach to benefit/risk preference – Best Worst Scaling 2
- Enrolled wide range of disease stage, severity, relatively well educated convenience sample
- Results published in Clinical Therapeutics
- Plans for additional studies with older patients, other targets

http://www.fastercures.org/assets/Uploads/Benefit-Risk-Boot-Camp/Session-6-DMD-Case-Study.pdf
Influencing FDA Approval Process: PPMD

Strength Happens Together: PPMD Submits FDA Draft Guidance on Duchenne
PPMD and a broad coalition of stakeholders has submitted the first-ever patient advocacy-initiated draft guidance for a rare disease to the FDA to help accelerate development and review of potential therapies for Duchenne.

Read more: http://www.fastercures.org/assets/Uploads/Benefit-Risk-Boot-Camp/Session-6-DMD-Case-Study.pdf
Influencing FDA Approval Process: PPMD

● Parent Project Muscular Dystrophy (PPMD)- patient advocacy group for Duchenne’s muscular dystrophy (DMD)

● DMD rare genetic disease, occurs in males, diagnosis usually by age 5, progressive muscular weakness, loss of ambulation at 10 years, death at 20 years

● Standard of care off label corticosteroids to stabilize muscle strength, delay loss of ambulation

● No FDA approved therapy

● Some experimental therapeutics in development

http://www.fastercures.org/assets/Uploads/Benefit-Risk-Boot-Camp/Session-6-DMD-Case-Study.pdf
Influencing FDA Approval Process: PPMD

Putting Patients First
Recommendations to speed responsible access to new therapies for Duchenne muscular dystrophy and other rare, serious and life-threatening neurologic disorders

Benefit-Risk Assessments in Rare Disorders
THE CASE FOR THERAPEUTIC DEVELOPMENT IN DUCHENNE MUSCULAR DYSTROPHY AS THE PROTOTYPE FOR NEW APPROACHES

http://www.fastercures.org/assets/Uploads/Benefit-Risk-Boot-Camp/Session-6-DMD-Case-Study.pdf
Influencing FDA Approval: PPMD

Conditional attribute importance

http://www.fastercures.org/assets/Uploads/Benefit-Risk-Boot-Camp/Session-6-DMD-Case-Study.pdf
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Developing Measures: EXACT PRO Tool

- Exacerbations of Chronic Pulmonary Disease Tool – first FDA qualified clinical outcome assessment for drug development

- COPD (emphysema and chronic bronchitis) affects more than 24 million people in US and 64 million worldwide

- Patients experience acute episodes of worsening symptoms and day to day variations—need to assess frequency, severity and duration

- 14 item tool was developed by industry supported, multi-stakeholder consortium, research team at Evidera

- Instrument designed with and for patients with COPD

- Purpose—expedite drug development with valid, reliable measure of impact (e.g. for new antimicrobials) on severity of exacerbations of symptoms of COPD

http://www.exactproinitiative.com/
## Countries engaging with patients during reimbursement decisions for technology assessment/payer decision making

<table>
<thead>
<tr>
<th>Country</th>
<th>Agency</th>
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<tbody>
<tr>
<td>Australia</td>
<td>Pharmaceutical Benefits Advisory Committee</td>
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<tr>
<td>Canada</td>
<td>Canadian Agency for Drugs and Technologies in Health</td>
</tr>
<tr>
<td>England and Wales</td>
<td>National Institute for Health and Care Excellence</td>
</tr>
<tr>
<td>France</td>
<td>French Authority for Health</td>
</tr>
<tr>
<td>Germany</td>
<td>Institute for Quality and Efficiency in Healthcare as well as Joint Federal Committee</td>
</tr>
<tr>
<td>New Zealand</td>
<td>Pharmaceutical Management Agency</td>
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<tr>
<td>Scotland</td>
<td>Scottish Medicines Consortium</td>
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<tr>
<td>Sweden</td>
<td>Dental and Pharmaceutical Benefits Agency</td>
</tr>
<tr>
<td>The Netherlands</td>
<td>National Health Care Institute (formerly College voor zorgverzekeringen, Health Care Insurance Board</td>
</tr>
<tr>
<td>United States</td>
<td>Patient-Centered Outcomes Research Institute</td>
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Source: [http://dij.sagepub.com/content/49/6/929.full.pdf+html](http://dij.sagepub.com/content/49/6/929.full.pdf+html)
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JDRF- Patient Advocacy for Artificial Pancreas

- Basic and clinical research
- Regulatory pathway creation
- **Extensive reimbursement/insurance coverage advocacy (Continuous Glucose Monitoring for Medicare example)**
- Evidence generation for potential cost-saving
Influencing Coverage & Benefit Design: CMS Payment and Value Demonstrations

- CMS goals are to have 30% of fee for service Medicare to quality or value through accountable care organizations or bundled payments by the end of 2016 and 50% by end of 2018.

- CMS is encouraging other payers, providers, consumers, employers, etc. to join the Health Care Payment Learning and Action Network to expand models into private sector.

- Oncology Payment Model Example – will bundle episodes of care from diagnosis on in 6 month episodes, measure and reward efficiency and quality.

- Patient groups are actively demanding a voice at the table- recent letter from the Partnership to Improve Patient Care demands same level of patient participation as in FDA’s PFDD and want patient centeredness as explicit goal.
US: Per capita out-of-pocket expenditures have grown since 1970

Per capita out-of-pocket expenditures, 1970-2013

- **Out of pocket**
- **In Constant 2013 Dollars**

1970: $119 Per capita
1976: $200 Per capita
1982: $400 Per capita
1988: $600 Per capita
1995: $800 Per capita
2001: $1,000 Per capita
2007: $1,200 Per capita
2013: $1,076 Per capita

Source: Kaiser Family Foundation analysis of National Health Expenditure (NHE) data from Centers for Medicare and Medicaid Services, Office of the Actuary, National Health Statistics Group
Average health expenditures: higher percentage spent on insurance premiums, then out of pocket

Average household expenditures on health (nonelderly families with no Medicare beneficiaries), 2002 and 2012

<table>
<thead>
<tr>
<th></th>
<th>2002</th>
<th>2012</th>
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<tbody>
<tr>
<td>Health insurance</td>
<td>51%</td>
<td>59%</td>
</tr>
<tr>
<td>Medical services</td>
<td>32%</td>
<td>28%</td>
</tr>
<tr>
<td>Prescription drugs</td>
<td>14%</td>
<td>10%</td>
</tr>
<tr>
<td>Med Sup.</td>
<td>3%</td>
<td>3%</td>
</tr>
</tbody>
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2002: $1,827

2012: $2,754

Source: Kaiser Family Foundation analysis of Consumer Expenditure Survey
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Patient Adherence: A Key Value Driver

- Chronic diseases are highly prevalent and difficult to control.
- Patient behavior is prime driver of health outcomes.
- Adherence levels to therapy remain low.
- Traditional approaches to improve adherence have failed.
- Proving product and service value in real world settings requires patient empowerment and self management.
Guiding Principles to Get You Started
Provides background on what you will need to know and do in advance of reaching out to individual patients.

Planning Your Patient Interactions
Describes how individual patients can be identified and recruited, who we define as individual patients in the scope of this guidance, and six (6) recommended steps for successful patient interactions.

Kinds of Patient Interactions to Consider
Identifies existing promising practices for engaging individual patients across the value chain (excluding current clinical trial patients): from leadership development to post-market patient support programs.

This Guide also provides helpful links to:
- Global Policy on Patient Interactions;
- Select country-specific Standard Operating Procedures (SOPs);
- Read and Understand Module (Compliance);
- FAQs (e.g. compensation; recruitment strategies; protocols;);
- Rationale Form for engaging individual patients;
- Checklist for engaging individual patients;
- Complete list of country-specific PAG leaders (globally);
- Rationale Form;
- A template Contract;
- Visual of Standard Process Flows for Engagement (by business line);
- Promising practice case studies;
- Country-specific “pockets of innovation” with individual patients.

Listening. Learning. Acting... Leading
Uncover **unmet needs** from people living with multiple, chronic conditions, and the nurses who interact with them.

Develop viable and differentiated **ideas** to enable patient empowerment and better self-management.

**Equip Sanofi** with the necessary skills to use Design Thinking methods in the future.

**EVENT OBJECTIVES**
PAM (Patient Activation Measurement) a tool for targeting and assessing services

Level 1
STARTING TO TAKE A ROLE

Level 2
BUILDING KNOWLEDGE AND CONFIDENCE

Level 3
TAKING ACTION

Level 4
MAINTAINING BEHAVIORS

Increasing Level of Activation

Source: J. Hibbard, University of Oregon
Diabetes Control With Reciprocal Peer Support Versus Nurse Care Management
A Randomized Trial

Michele Helsler, MD, MPA; Sandeep Vijan, MD, MS; Fatima Makki, MPH; and John D. Plette, PhD

Background: Resource barriers complicate diabetes care management. Support from peers may help patients manage their diabetes.

Objective: To compare a reciprocal peer-support (RPS) program with nurse care management (NCM).

Design: Randomized, controlled trial. (ClinicalTrials.gov registration number: NCT00320112)

Setting: 2 U.S. Department of Veterans Affairs health care facilities.

Patients: 244 men with hemoglobin A$_{1c}$ (HbA$_{1c}$) levels greater than 7.5% during the previous 6 months.

Measurements: The primary outcome was 6-month change in HbA$_{1c}$ level. Secondary outcomes were changes in insulin therapy; blood pressure; and patient reports of medication adherence, diabetes-related support, and emotional distress.

Intervention: Patients in the RPS group attended an initial group session to set diabetes-related behavioral goals, receive peer communication skills training, and be paired with another age-matched peer patient. Peers were encouraged to talk weekly using a telephone platform that recorded call occurrence and provided reminders to promote peer contact. These patients could also participate in optional group sessions at 1, 3, and 6 months. Patients in the NCM group attended a 1.5-hour educational session and were assigned to a nurse care manager.

Results: Of the 244 patients enrolled, 216 (89%) completed the HbA$_{1c}$ assessments and 231 (95%) completed the survey assessments at 6 months. Mean HbA$_{1c}$ level decreased from 8.02% to 7.73% (change, −0.29%) in the RPS group and increased from 8.13% to 8.22% (change, 0.10%) in the NCM group. The difference in HbA$_{1c}$ change between groups was 0.58% (P = 0.004). Among patients with a baseline HbA$_{1c}$ level greater than 8.0%, those in the RPS group had a mean decrease of 0.88%, compared with a 0.07% decrease among those in the NCM group (between-group difference, 0.81%; P < 0.001). Eight patients in the RPS group started insulin therapy, compared with 1 patient in the NCM group (P = 0.020). Groups did not differ in blood pressure, self-reported medication adherence, or diabetes-specific distress, but the RPS group reported improvement in diabetes social support.

Limitation: The study included only male veterans and lasted only 6 months.

Conclusion: Reciprocal peer support holds promise as a method for diabetes care management.

Primary Funding Source: U.S. Department of Veterans Affairs Health Services Research and Development Service.

For author affiliations, see end of text.
Adherence: Patients Like Me and UCB - Epilepsy

- Patients Like Me - online community partners with UCB a company with epilepsy products to understand patient experience.

- Participants in online community reported increased knowledge about disease and adherence with medications.

- Veteran’s Affairs partnering with both UCB and Patients Like Me to enhance communication with veteran’s with epilepsy, increase provider interaction, analyze patterns of care and needs.

http://www.neurology.org/content/early/2015/06/17/WNL.000000000001728.full.pdf+html
Patient Partnerships are Key to Access

- Identify patient needs and incorporate into priorities for product and service development
- Leverage the power of patient networks and organizations to develop meaningful measures of benefit/risk and valued attributes – including patient reported outcomes
- Partner with patients to co-create services that support patient self-management adherence and outcomes in real world settings