



Forward-Looking Statements

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Company Snapshot

Who: In 1990, the company was formed as Specialty Fine Chemical business specializing in cyclodextrins. In 2014, the business was expanded into a biotechnology company dedicated to developing life-changing medicines through science and innovation for patients and families living with challenging diseases.

What: Trappsol® Cyclo™ is a proprietary formulation of hydroxypropyl beta cyclodextrin and in multiple clinical studies has shown encouraging results to effectively manage the transportation of cholesterol.

Why: Because cholesterol is so important to the normal function of our cells, its synthesis and degradation is tightly controlled by an array of cellular processes. When there is an imbalance in cholesterol synthesis or metabolism, cells and organs may not function properly, leading to disease or death.

How: Trappsol® Cyclo™, with its cyclic structure, facilitates the transport of accumulated cholesterol out of cellular lysosomes so it can be further processed and excreted out of cells.

Currently Targeting 2 Serious Diseases with Unmet Medical Need

Niemann-Pick Disease Type C

Fatal and progressive genetic disorder
Orphan indication affecting >9,000 in
80 countries (~400 in U.S. / 320 EU5) ¹

Alzheimer's Disease

6th leading cause of death affecting
5 million people in the U.S. ²

Platform technology has
potential to fuel pipeline
expansion opportunities

1. April 2021, Tessellon Inc. (former Kantar Health experts with 25+ years of epidemiology and forecasting experience), (www.Tessellon.com); Exhaustive literature search with a broad range of MESH terms in United States + 79 other countries.

2. <https://www.alz.org/alzheimers-dementia/facts-figures>

Platform Technology Pipeline:

Trappsol® Cyclo™ allows for a multiple shots on goal model

Program	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Milestones
Trappsol® Cyclo™	Niemann-Pick Disease Type C					TransportNPC™ (Pivotal Phase 3 Study) Site activation ongoing, enrolling and dosing patients
Trappsol® Cyclo™	Alzheimer's Disease					Launched Phase 2b study, first patient dosed expected before year end 2022

Ongoing Collaboration with University of the Witwatersrand, Johannesburg to Advance Trappsol® Cyclo™ Platform and Explore Pipeline Expansion Opportunities

Orphan Drug Designation in U.S. | Fast Track Status in U.S. | Potential for Priority Review Voucher (PRV) in U.S

Orphan Designation in EU | EMA Pediatric Investigational Plan Adopted

Leadership Team with Proven Experience



N. Scott Fine
Chief Executive Officer & Director



Joshua M. Fine
Chief Financial Officer



Lise Lund Kjems, MD, PhD
Chief Medical Officer



Michael Lisjak
Chief Regulatory Officer



Jeffrey L. Tate, Ph.D.
Chief Operating Officer, Chief Quality Officer & Director



Lori McKenna Gorski
Global Head of Patient Advocacy



Scientific Advisory Board



Rita Colwell, Ph.D.

Co-Chair

Internationally recognized scientist, microbiologist and founder of CosmosID, a privately held bioinformatics firm. Distinguished University Professor at U. Maryland and Johns Hopkins University. Former Director, National Science Foundation (1998 - 2006). National Medal of Science awardee. Member, US National Academy of Sciences.



Benny Liu, M.D.

Key Opinion Leader in Niemann-Pick Disease Type C

Gastroenterologist at Alameda Healthy System, CA and Highland Hospital. Globally recognized expert in lipid metabolism. First to discover that cyclodextrins release cholesterol from cells using an animal model. Assistant Clinical Professor, UCSF.



Gerald F. Cox, M.D., Ph.D.

Internationally Renowned for Clinical Drug Development

Seasoned biotechnology executive with 20-year successful track record of drug development for rare genetic diseases and extensive worldwide regulatory experience



Sharon H. Hrynkow, Ph.D.

Co-Chair

Neuroscientist with more than 25 years' experience in global health arena, public and private sectors. Senior executive at NIH. First president of non-profit Global Virus Network. Former Member of President's Council of Advisors on Science and Technology. 5 years at Cyclo Therapeutics leading clinical and scientific programs.



Caroline Hastings, M.D.

Key Opinion Leader in Niemann-Pick Disease Type C

Pediatric hematologist oncologist, Director of NeuroOncology, and Professor of Pediatrics, UCSF Benioff Children's Hospital Oakland. First physician in US to use cyclodextrins for treatment in NPC, compassionate use. Advisor to US and Australian NPC Advocacy organizations and to physicians globally on NPC.



Niemann-Pick Disease Type C

Ongoing Pivotal **TransportNPC**[™]
Phase 3 Study



NPC: A Debilitating Disease with Fatal Outcomes

- Rare, fatal and progressive genetic disorder affecting the brain, liver, spleen and lungs
- Characterized by a defect in the NPC1 protein
- Cholesterol and lipids accumulate in cells of major organs and tissues
- Leading to cell and tissue dysfunction

0 U.S. Approved NPC Therapies

1 EU Approved Therapy with No Systemic Effects

Market Opportunity¹

United States: \$300 Million | Worldwide: \$600 Million

Incidences

1/100,000 (~35 per year in U.S.)

Of Diagnosis

~ 3% are age 3 and below

~ 97% are age 3 and above

~ 60% age 16 and above

Median Survival

Early Infantile (2m-2): 4.6y

Late Infantile (3-6): 9.4y

Juvenile (7-15): 15.4y

Adolescent/Adult (16+): 12.2y

Neurologists & Geneticists Crucial Specialties in the Diagnosis of NPC (Return on Focus Patient Journey Interviews, January 2022)

Specialties of Diagnosing Physicians
n=10

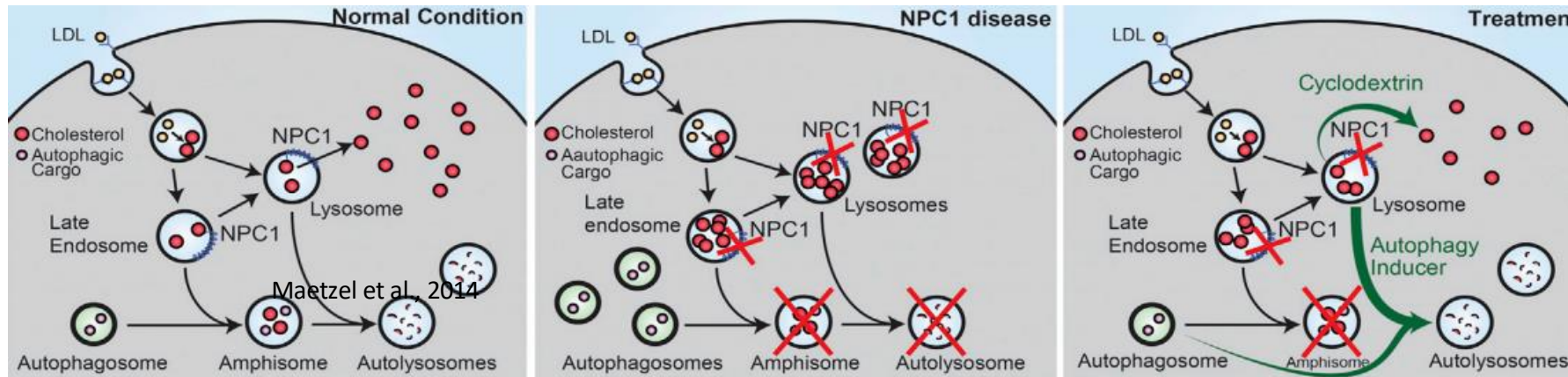


Multidisciplinary Team Needed in Support of Patient Care

Discipline	Features of NPC for Which this Discipline May Be of Assistance
Primary Care Physician	Assist with general medical care; coordinate specialists; provide support for family
Metabolic Diseases Specialist	Diagnosis of NPC and exclusion of other disorders in the differential diagnosis; ongoing patient assessment for disease progression and response to therapy
Neurologist	Cataplexy, movement disorders, dystonia, seizures
Psychiatrist	Psychosis, behavioral disturbances; depression
Neuro-ophthalmologist	Diagnosis (vertical gaze palsy) and assess response to therapy (changes in saccadic eye movement velocity)
Anesthesiologist	Assess for anesthetic risk as needed
Neuropsychologist	Assess for cognitive involvement at baseline and in response to therapy
Speech and Language Therapist	Assess for dysphagia and aspiration risk; speech therapy for children
Occupational and Physical Therapists / Rehabilitation Physician	Assess development and develop aids and home adjustments as needed for patients with communication and physical challenges
Orthopaedic Surgeon	Assess the need for surgical correction of severe scoliosis, osteo-articular retractions, spasticity treatments and hip problems
Nutritionist / Gastroenterologists	Assess nutritional status in patients who may be losing weight due to dysphagia or side effects of therapy; Gastrostomy tube insertion when swallowing is unsafe
Social Worker	Support of patients and families living with disabilities who require enhanced resources in the community
Genetic Counselor	Provide counselling for families as to recurrence risk and options for prenatal diagnosis if desired

Trappsol® Cyclo™

Enables the Effective Transport of Cholesterol Out of Cells



Cholesterol as measured by Filipin staining at Baseline and after 7 doses over 14 weeks



The lack of light blue represents the clearing of cholesterol from cells

Trappsol® Cyclo™ Summary of Completed Clinical Studies in NPC

Study 101

Phase 1 study in NPC patients age 18 years and older showed Trappsol® Cyclo™ was well-tolerated with an acceptable safety and tolerability profile

- After IV infusion, the drug is detectable in the cerebrospinal fluid within hours after the start of infusion
- Cholesterol synthesis and metabolism affected, and cholesterol cleared from cells, mimicking effects from nonclinical studies (*in vitro* and *in vivo*) in NPC models

Study 201

Consistent pharmacodynamic effects and safety profile observed in a 48-week Phase 1/2 study in NPC patients aged 2 years and older

- 100% of patients assessed by treating physicians to be either stable or improved
- 88% (8 of 9 patients who completed the study), experienced clinically meaningful improvements in one or more efficacy endpoints, assessed by the 17 Domain NPC Severity Scale
- Based on totality of data from the Phase 1 and Phase 2 studies, the 2000 mg/kg dose was selected for the Phase 3 study

Double-blind, randomized, placebo-controlled, parallel-group study and is currently the most advanced clinical research program underway to identify a treatment for NPC

Number of Subjects	93
Current Sites	23 across 9 countriesUnited States, United Kingdom, Italy, Germany, Spain, France, Poland, Israel, and Australia
Duration	96-week trial, with interim analysis at 48 weeks
Dose	2000 mg/kg via IV infusion
Primary Endpoint	NPC Composite Severity Score
Secondary Endpoints	SCAFI, Swallow, Vineland-2
Exploratory Endpoints	Inclusive of speech, liver and lung function

We Have the Only Active Late-Stage Clinical Program in NPC

Company	Indication	Preclinical	Phase 1	Phase 2	Phase 3	*Potential Indication (Systemic and Neurological Effects)	*Safety Profile	Summary
	Trappsol® Cyclo™ (Intravenous every 2 weeks, home infusions)							Met all primary endpoints of the Ph1 and Ph1/2 showing favorable safety and efficacy. Ph3 currently enrolling and additional site activation ongoing.
	Zavesca* (Oral 3 times daily)							FDA: Data did not support benefit risk. Off-label in US. EMA: Approved January 2009.
	IB1001 (Oral 3 times daily)							Met with FDA, EMA and UK, Phase 3 study required, 6-month placebo-controlled crossover trial with approximately 50 patients (4 years of age and above). Study enrollment anticipated H1 2022.
	Arimoclomol (Oral 3 times daily)							KenPharm has reached agreement to acquire Arimoclomol from Orphazyme with completion planned by June 1, 2022. FDA: CRL received June 17, 2021, noting additional data required to support benefit risk. Resubmission planned 1Q 2023. EMA: To be determined
	Adrabetadex (VTS-270) (Intrathecal every 2 weeks)							MNK concluded program 20-Jan-2021 noting the benefit risk is negative. Mandos received Court Approval to buy from MNK 29-Jun-2021. FDA has not found drug to be safe and effective for use to treat NPC1 or for any other use.
	ESB1609 (Oral, brain-penetrant)					TBD	TBD	

Alzheimer's Disease

Ongoing Phase 2b Study



Alzheimer's Disease

The Most Common Form of Dementia

An irreversible, progressive neurologic disorder that slowly degrades memory, thinking and social skills that affects a person's ability to function independently.

Similarities with NPC

Cognitive decline

Elevated levels of tau

Amyloid plaques

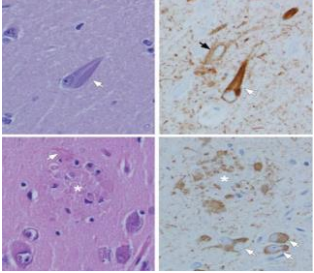


- Affects more than 5 million people in the U.S.¹
- 6th leading cause of death in the U.S.¹
- 500,000 new cases every year²
- 13.8 million cases projected by 2050¹

Commonality Across Target Neurodegenerative Diseases

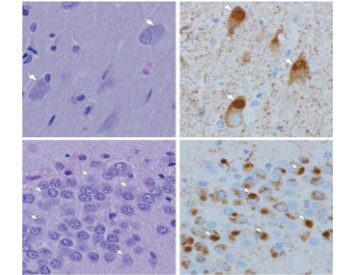
Alzheimer's Disease

Secondary Tauopathy



Niemann-Pick Disease Type C

Primary Tauopathy



Biologic Similarities

Cholesterol Accumulation in Regions of Brain

Elevated Levels of Tau in CSF

Amyloid Plaques in the Brain

Disease Manifestation

Cognitive decline / dementia

Premature death

Clumsiness

Progressive motor symptoms

Ataxia, dystonia, dysarthria, dysphasia

Psychiatric signs: psychosis, depression

Weight loss

Disease Manifestation

Progressive cognitive decline / early dementia

Premature death

Clumsiness, gait disturbance

Delayed motor milestones

Progressive: ataxia, dystonia

Seizures

Weight loss

Trappsol® Cyclo™ for the Potential Treatment of Early Alzheimer's Disease Targeting Reduction of Amyloid Beta and Tau

Program Strategy Supported by Neuroscientist Researcher,
Cynthia A. Lemere, PhD, as Senior Advisor for Advancement of Our Alzheimer's Disease Asset

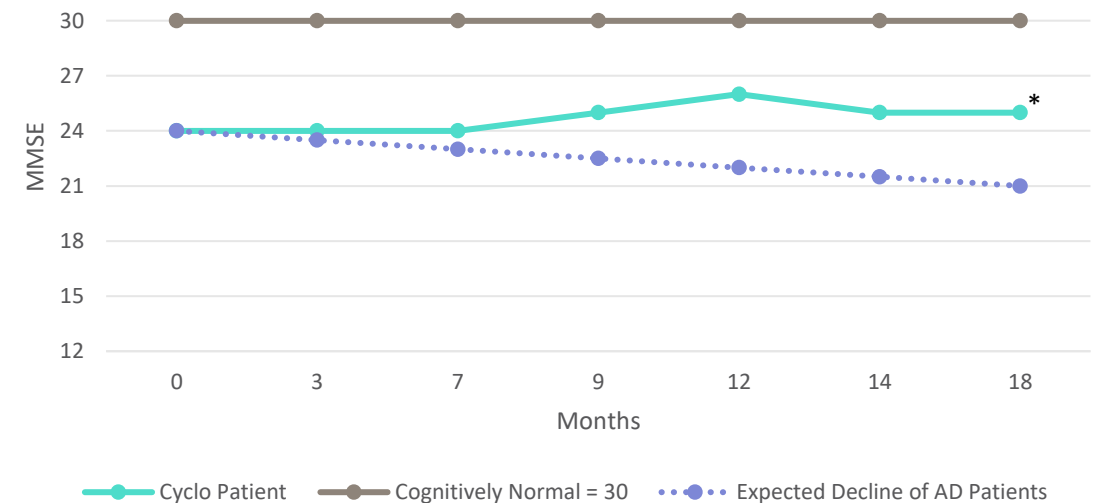
Positive Results in Alzheimer Patient Under Compassionate Use Program

FDA authorized use of Trappsol® Cyclo™
in geriatric patient (data on file from Study CTDH-AD-
EA-001)

- 18 months of monthly IV infusion
- Disease did not progress based on Memory assessment with the Digital Clock Drawing Test (DCT) showing stabilization and results correlating with the stability of the MMSE score.

18 months of data has led to development
of Phase 2 protocol

Alzheimer's Mini-Mental State Evaluation Performance¹



*Treating physician reported the 18-month score as a range between 24-26

1: Rate of MMSE decline in AD patients: Eldholm, RS et al, J. Alz. Disease, 61: 1221, 2018. Suh, GH et al., Intl. J. Geriatric Psychiatry, 19(9): 817, 2004.

Ongoing Phase 2b Study in Early Alzheimer's Disease

Patient Dosing Expected Before Year End 2022

U.S. Multicenter, Randomized, Placebo-Controlled,
Double-Blind, Parallel Group, 6-Month Study

Number of Subjects	~120
Current Sites	United States, Multi-Centered
Duration	6 Months
Dose	Randomized across three study arms: 500 mg/kg or 1000 mg/kg of Trappsol® Cyclo™ and Placebo
Study Endpoints	Safety, Tolerability and Potential Efficacy

Corporate Overview



Financial Snapshot - Nasdaq: CYTH

Cash
Balance¹

\$7.5M

Market
Cap³

~\$16M

Shares
Outstanding²

8.5M

Investment Summary

Leveraging over 3 decades of experience with cyclodextrins to advance clinically de-risked programs towards approval in diseases with unmet medical need

TransportNPC™

Enrolling and dosing patients in Pivotal Phase 3 study in Niemann-Pick Disease Type C

Significant market opportunity with no approved therapy to treat both systemic and neurological manifestations of NPC

FDA: Orphan Drug Designation (ODD), Fast-Track, Rare Pediatric Disease Designation, potential Priority Review Voucher; EMA: ODD and adopted Paediatric Investigation Plan

Pipeline expansion into Alzheimer's Disease (AD), patent filed globally and is currently being prosecuted

Launched Phase 2b Study
First Patient Dosed Expected
Before Year End 2022

Platform technology has demonstrated to be safe and potentially effective with over 10 years of patient exposure

Platform technology with opportunity to expand into multiple indications
Leadership team with proven track-record



NASDAQ: CYTH
cyclotherapeutics.com



Thank you!