



The logo for Cyclotherapeutics features the word "cyclo" in large, teal, lowercase letters. Each letter contains a photograph of a diverse individual: a woman in a white dress with arms raised, a young boy jumping, a woman in a white top and red pants in a yoga pose, two children with colorful handprints, an elderly couple embracing, and a woman in a pink shirt jumping. The letters "l" and "o" are solid teal. Below "cyclo" is the word "therapeutics" in a blue, lowercase, sans-serif font.

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Investment Opportunity

Deep expertise with Cyclodextrins with over 10 years of patient exposure

Lead program, Trappsol® Cyclo™ demonstrated to be safe and effective in multiple clinical studies in NPC

Significant market opportunity in high value indications

Management team with proven expertise

Manufacturing at commercial scale inclusive of 60-month stability and 96hr In-use stability

Currently Targeting 2 Serious Diseases with Unmet Medical Need

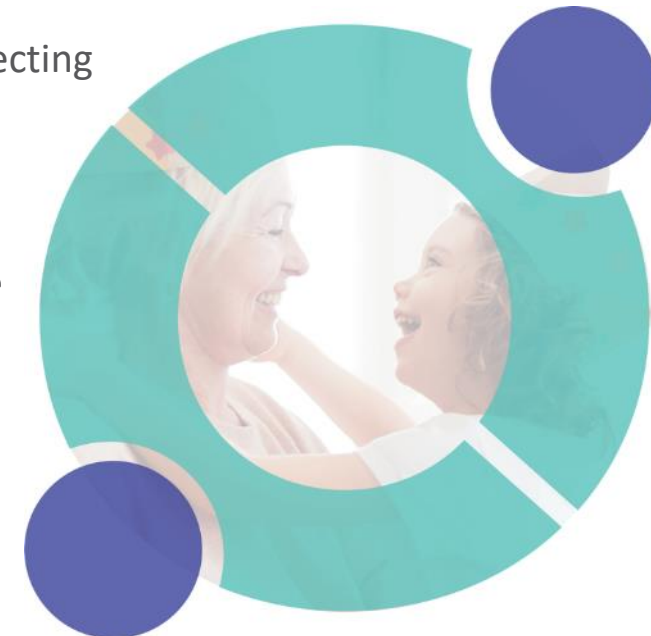
Niemann Pick Disease Type C

Fatal and progressive genetic disorder
Orphan indication affecting 3,000 people¹

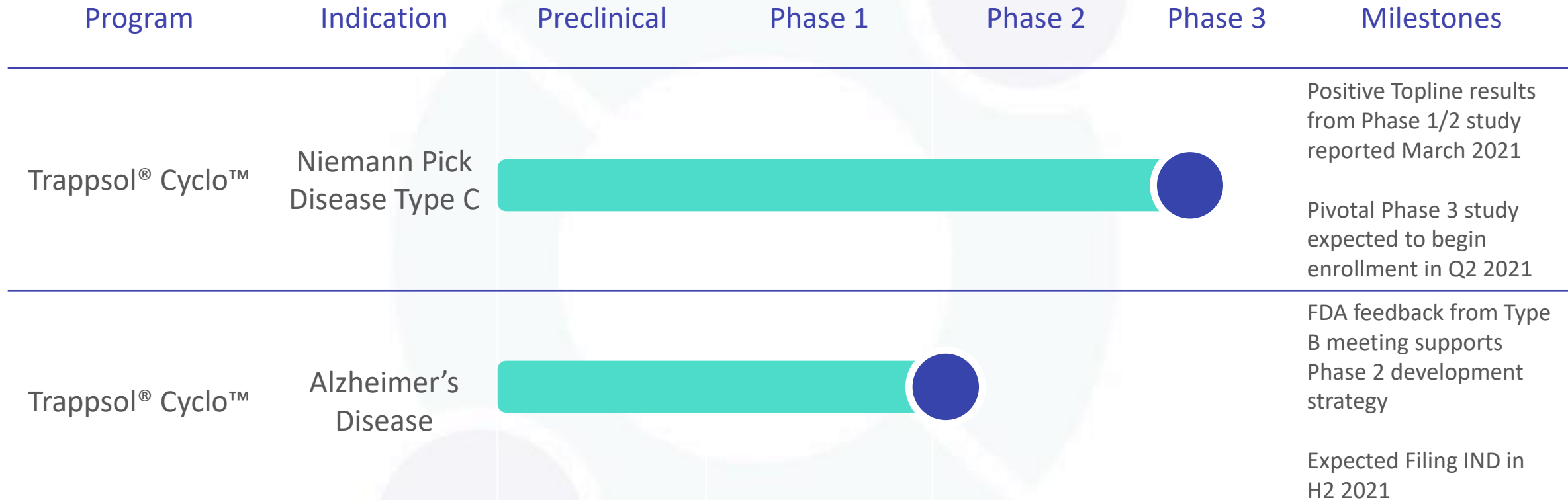
Alzheimer's Disease

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

Platform technology has potential to fuel pipeline expansion opportunities



Pipeline



Management Team with Proven Experience



N. Scott Fine
Chief Executive Officer & Director



Michael Lisjak
Chief Regulatory Officer & SVP for Business Development



Sharon H. Hrynkow, Ph.D.
Chief Scientific Officer & SVP Medical Affairs



Russ Belden
Acting Chief Commercial Officer
Genentech



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



Joshua M. Fine
Chief Financial Officer



Gerald F. Cox, M.D., Ph.D.
Acting Chief Medical Officer



Lori McKenna Gorski
Global Head of Patient Advocacy



Niemann Pick Disease Type C

Enrollment in pivotal Phase 3 study
expected to commence Q2 2021



Niemann Pick Disease Type C (NPC)

Rare, fatal and progressive genetic disorder characterized by a defect in the NPC1 protein causing cholesterol and lipids to accumulate in cells of major organs leading to cell and tissue dysfunction.

Average Life Expectancy:

Before age 5 if symptoms appear in infancy

Age 20 in juvenile onset





Increasing diagnosis in later onset disease

Symptomology Inclusive of Systemic and Neurological Manifestations

- Enlarged liver and spleen (hepatosplenomegaly)
- Severe liver disease and dysfunction
- Respiratory infections and lung disease
- Loss of cognitive skills
- Difficulty with speech
- Seizures
- Difficulty with swallowing and feeding
- Difficulty coordinating movement (ataxia)
- Abnormal eye movements (vertical supranuclear gaze palsy)
- Poor muscle tone (hypotonia)

No U.S. Approved NPC Therapies
Only 1 E.U. Approved Therapy

Significant Competitive Advantages

Company	Product / Route	Descriptor	Potential Indication
	Trappsol® Cyclo™ (Intravenous every 2 weeks, Home infusions)	Met all primary endpoints of the Phase 1 and Phase 1/2 studies showing favorable safety and efficacy. Phase 3 ready asset, first patient in planned Q2 2021.	Systemic and Neurological
	Zavesca* (Oral 3 times daily)	EU and other international countries approved. Off-label in the US.	Neurological
	Arimoclomol (Oral 3 times daily)	PDUFA date for FDA feedback is June 2021. EMA submission completed November 2020, expected feedback H2 2021. Phase 2/3 data met primary endpoints in two-sub-groups, but was not statistically significant overall, co-primary failed.	Neurological
	IB1001 (Oral 3 times daily)	Phase 2 Study Active, patients 6 years of age and older.	Neurological

NPC Prevalence

Incidences

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

Existing Cases

>9,000 in 80 countries
(~400 in U.S. / 320 EU5)

Of Diagnosed Patients

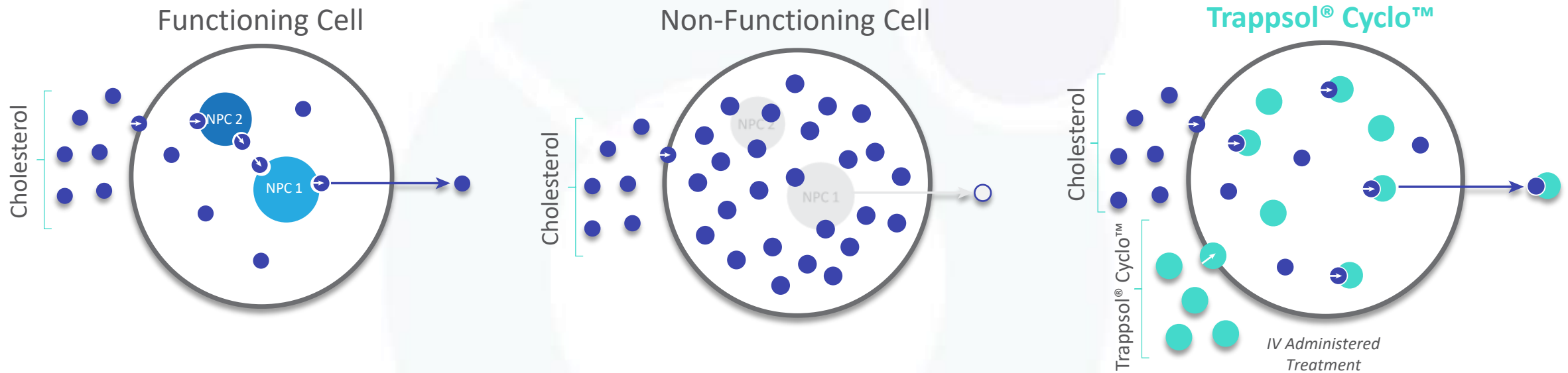
~ 3% are age 3 and below
~ 97% are age 3 and above
~ 60% age 16 and above

Median Survival

Early Infantile (2m-2): 4.6y
Late Infantile (3-6): 9.4y
Juvenile (7-15): 15.4y
Adolescent/Adult (16+): 12.2y

Trappsol® Cyclo™

A Potential Treatment for the Systemic and Neurological Symptoms of NPC



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

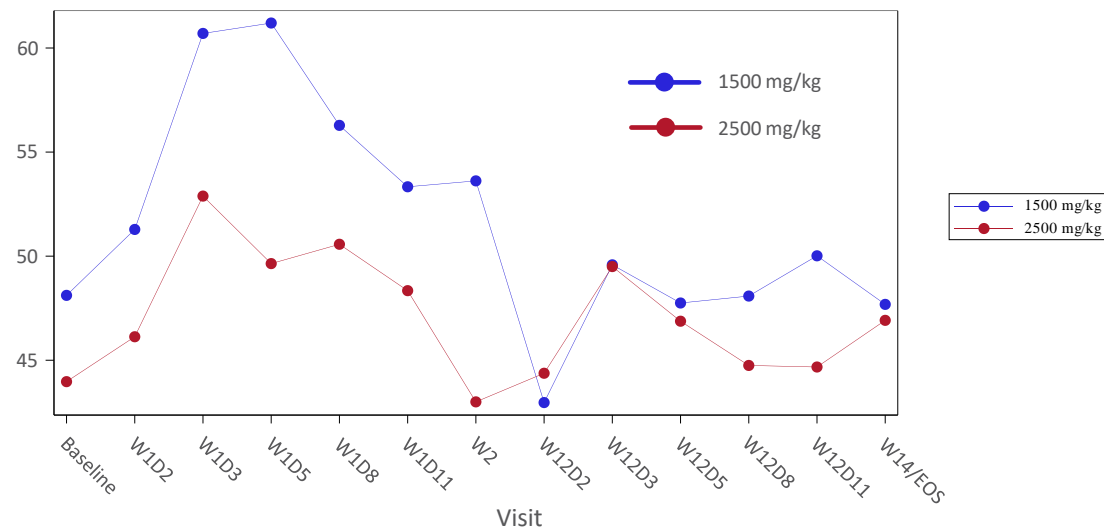
Biomarkers - Central Nervous System (CNS)

24S-hydroxycholesterol, a cholesterol metabolite from the CNS transported across the BBB, increases in serum following IV administration of HP β CD. Shown here are data after 1st dosing and 7th dosing. 24S-hydroxycholesterol increases in serum following IV infusion of HP β CD, signaling removal of excess cholesterol from the brain.

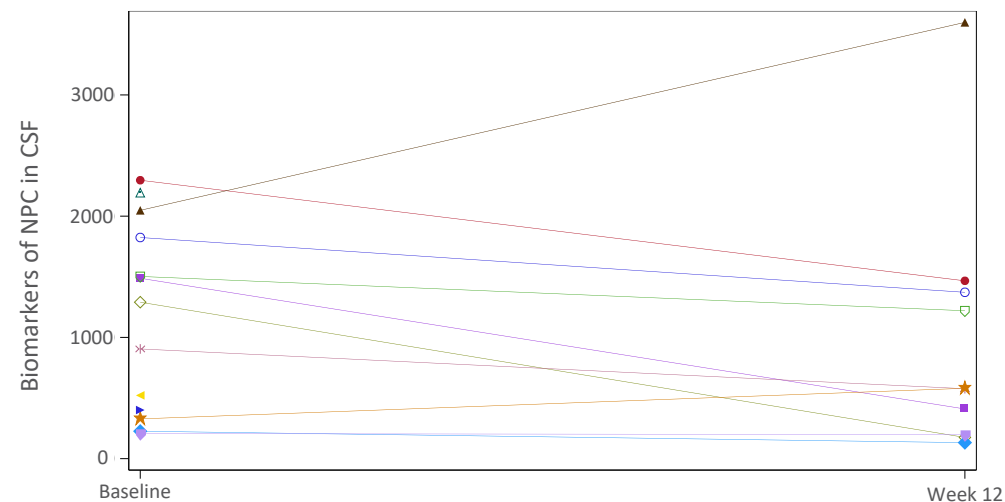
Tau levels as measured in the CSF are shown here for 10 NPC patients who had lumbar punctures prior to treatment with HP β CD and after seven doses

Six of 10 patients showed a reduction in Tau levels, two remained stable, and two increased, no dose-response relationship.

24S-hydroxycholesterol (ug/L)



Tau (ng/L)



Trappsol[®] Cyclo[™]

Development Path Towards Potential Approval

Phase 1

Completed May 2020

- ✓ Positive results
- ✓ Demonstrated favorable safety and tolerability profile in patients

Phase 1/2

Positive Topline Results Reported March 2021

- ✓ Positive interim analysis
- ✓ Encouraging signals in efficacy

Pivotal Phase 3

Enrollment Expected to Commence Q2 2021

Global clinical protocol agreed with U.S. FDA and EMA

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

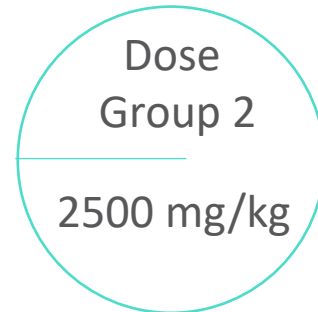
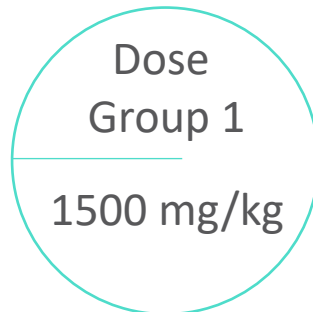
Orphan Designation in EU | EMA Pediatric Investigational Plan Adopted

Phase 1 Study

Demonstrated Favorable Safety and Tolerability Profile and Target Engagement

All 8 patients eligible for the Extension Study participated

Bi-weekly, 8-hour, IV treatment for a period of 14 weeks
RANDOMIZE (N=12) Ages 18 years and above



Primary Endpoint	<ul style="list-style-type: none"> Plasma levels of Trappsol® Cyclo™
Secondary Endpoint	<ul style="list-style-type: none"> Markers of Cholesterol metabolism/ synthesis CSF Levels of Trappsol® Cyclo™ Hepatic and splenic morphology Global impression of disease
Exploratory Endpoint	<ul style="list-style-type: none"> CSF biomarkers of NPC Disease

Summary of Results

- Confirmed a favorable safety profile
- Removed trapped cholesterol from cells and impacts cholesterol homeostasis
- Tau decreased: suggesting neuroprotective benefit
- Increase in 24S biomarker demonstrates removal of excess cholesterol from the brain
- Neurological improvements, higher energy, and greater focus exhibited by the patient
- Drug crosses the blood brain barrier



Phase 1/2 Study

Positive Results Demonstrated Promising Safety and Efficacy

Trial data suggest Trappsol® Cyclo™ overcomes the NPC1 defect by removing trapped cholesterol from cells both systemically and in the central nervous system (CNS)

Bi-weekly, 8-hour, IV treatment for a period of 48 weeks
RANDOMIZE (N=12) Ages 2 years and above



Primary Endpoint

- Plasma levels of Trappsol® Cyclo™

Secondary Endpoint

- Markers of cholesterol metabolism/ synthesis
- CSF levels of Trappsol® Cyclo™
- Clinical outcomes (motor skills, cognition, eye movements, liver morphology, et al)
- Global impression of disease

Exploratory Endpoint

- CSF biomarkers of NPC Disease

Summary of Results

- 89% of patients met the efficacy outcome measure of improvement in at least 2 domains of the 17-domain NPC Severity Scale
- 100% of patients who completed the trial improved or remained stable per their treating physicians
- Demonstrated improvements in all dose groups
- Favorable safety profile across all dose groups
- Tau decreased: suggesting neuroprotective benefit
- Confirmed Trappsol® Cyclo™ crosses the blood brain barrier
- Improves neurological features of disease, including ataxia, swallow, walking, and quality of life

Imminent Initiation of Pivotal Phase 3 Study

Patient enrollment expected to commence Q2 2021

Double-blind, Randomized, Placebo-controlled, Parallel-group study

Number of Subjects	93
Sites	23 across 9 countries United 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



Patient Progress: A Case Study

61-year-old patient with NPC: Improvements with intravenous (IV) Trappsol® Cyclo™ over 15 months

Diagnosed at age 59 years: slurred speech, ataxia, vertical gaze palsy, mild dysmetria/dystonia, mild hearing loss, mild short term memory loss with intact cognition, cough with eating

Completed Phase 1 trial and received 7 infusions IV Trappsol® Cyclo™; no toxicities

Currently enrolled on extension protocol and receives IV Trappsol® Cyclo™, 1500 mg/kg every 2 weeks at home

Patient and spouse see notable improvements in speech and swallow, seen within hours of the infusion and maintained for 5-10 days

- Increased speech fluency and word finding, more comfortable to communicate, more interactive and happy, positive impact on quality of life
- Takes solids and un-thickened liquids without cough; rare cough on saliva every few weeks
- Clinical severity score improved by 1 point due to change in cough; scale for speech does not include changes in speech fluency/word finding
- Cognition remains stable



Alzheimer's Disease

Filing IND in H2 2021 for
potential Phase 2 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

Trappsol® Cyclo™ for the Treatment of Alzheimer's Disease

Targeting Reduction of Amyloid Beta and Tau

FDA feedback from Type B meeting supports Phase 2 development strategy for Alzheimer's disease asset; IND filing on track for H2 2021

Positive Results in Alzheimer Patient Under Compassionate Use Program

FDA authorized use of Trappsol® Cyclo™ in geriatric patient

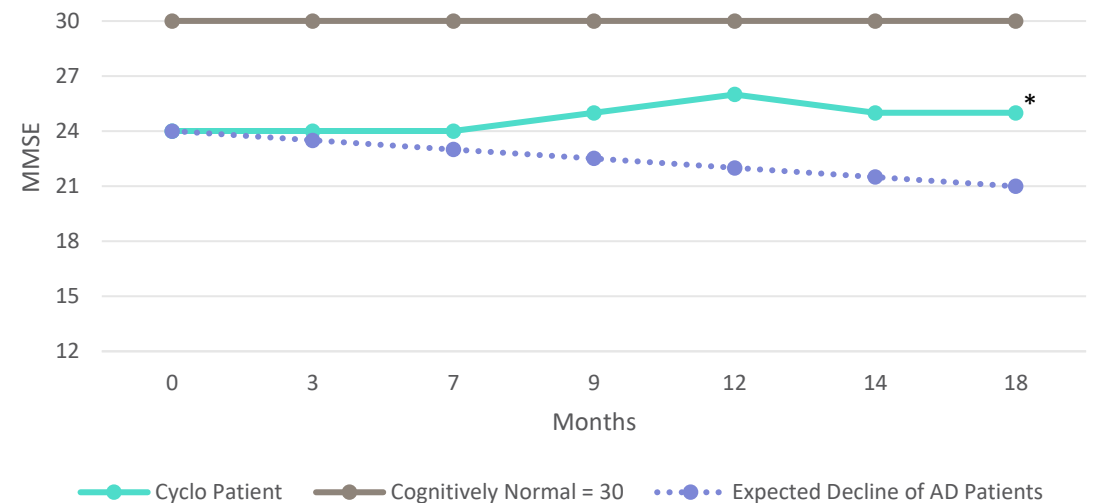
18 months of monthly IV infusion

Disease did not progress

Family reported less volatility and greater word-finding ability

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

Alzheimer's Mini-Mental State Evaluation Performance¹



"The patient has shown cognitive and neurologic stability in serial examinations during this study that indicates possible benefit as there would be an expected measurable cognitive and functional decline over a 18-month period in persons with Alzheimer's disease dementia"

Treating Physician

*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.

Corporate Overview



Financial Snapshot

Cash Balance¹

\$15.5M

Market Cap²

~\$54M

Shares
Outstanding³

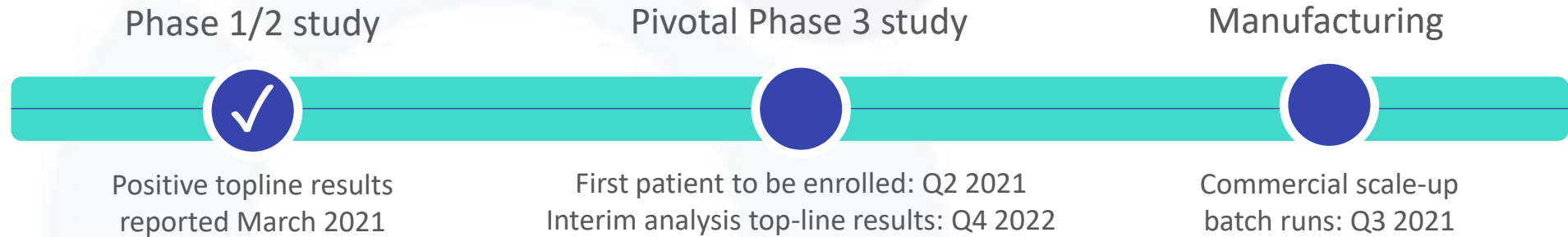
~6.4M

Average Volume²

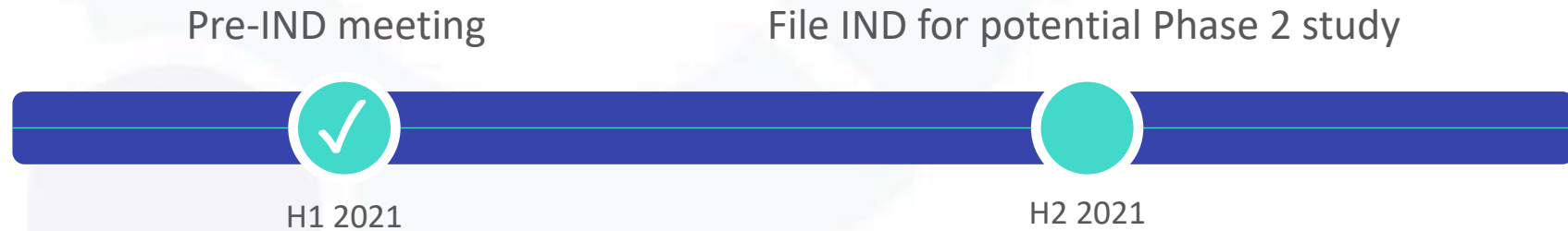
~480K

Target Upcoming Milestones with Potential to Drive Value

Niemann Pick Disease Type C



Alzheimer's Disease



Investment Summary

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

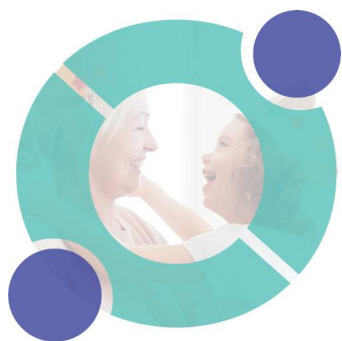
Lead asset demonstrated to be safe and effective with over 10 years of patient exposure

Entering 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

Pipeline expansion into Alzheimer's Disease

IND filing on track for H2 2021



Multiple value-driving milestones expected throughout 2021

Platform technology with opportunity to expand into multiple indications

Leadership team with proven track-record in execution and value creation

Board of Directors



Markus W. Sieger

Chairman

President & CEO of Polpharma Group
Independent Director



F. Patrick Ostronic

Vice Chairman

Officer of US Pharmacia International & CFO of The USP Group
Independent Director



N. Scott Fine

Chief Executive Officer & Executive Director



Jeffrey L. Tate, Ph.D.

Chief Operating Officer, Chief Quality Officer & Executive Director



C.E. "Rick" Strattan

Founder, Former Director of Marketing & Business Development of Pharmatec
Independent Director



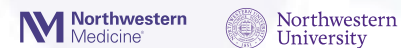
William S. Shanahan

Former President & COO of Colgate-Palmolive
Independent Director



Randall M. Toig, M.D.

Associate Professor at Northwestern University, Northwestern Memorial Hospital
OBGYN Surgeon & Serial Entrepreneur
Independent Director



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.



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.



M. Flint Beal, M.D.

Key Opinion Leader in Neurodegenerative Diseases

Internationally recognized authority on neurodegenerative diseases. University Professor, Weill Cornell Medical College. Leads a highly productive research laboratory focused on Alzheimer's Disease, Huntington's Disease, and ALS and published seminal manuscript on use of cyclodextrins to improve memory in an animal model of Alzheimer's Disease. Member, National Academy of Medicine.



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.



Benny Liu, M.D.

Key Opinion Leader in Niemann Pick Disease Type C

Gastroenterologist at Alameda Health 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.



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Thank you!