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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

Leadership 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

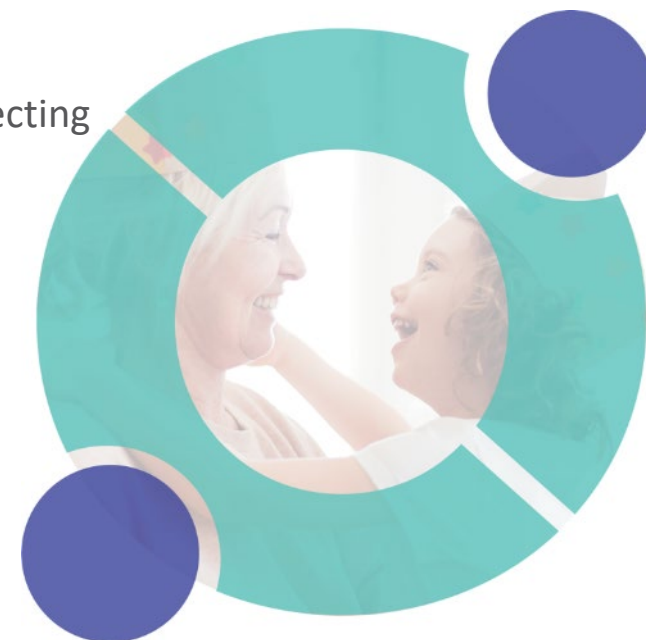
Niemann Pick Disease Type C

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

Alzheimer's Disease

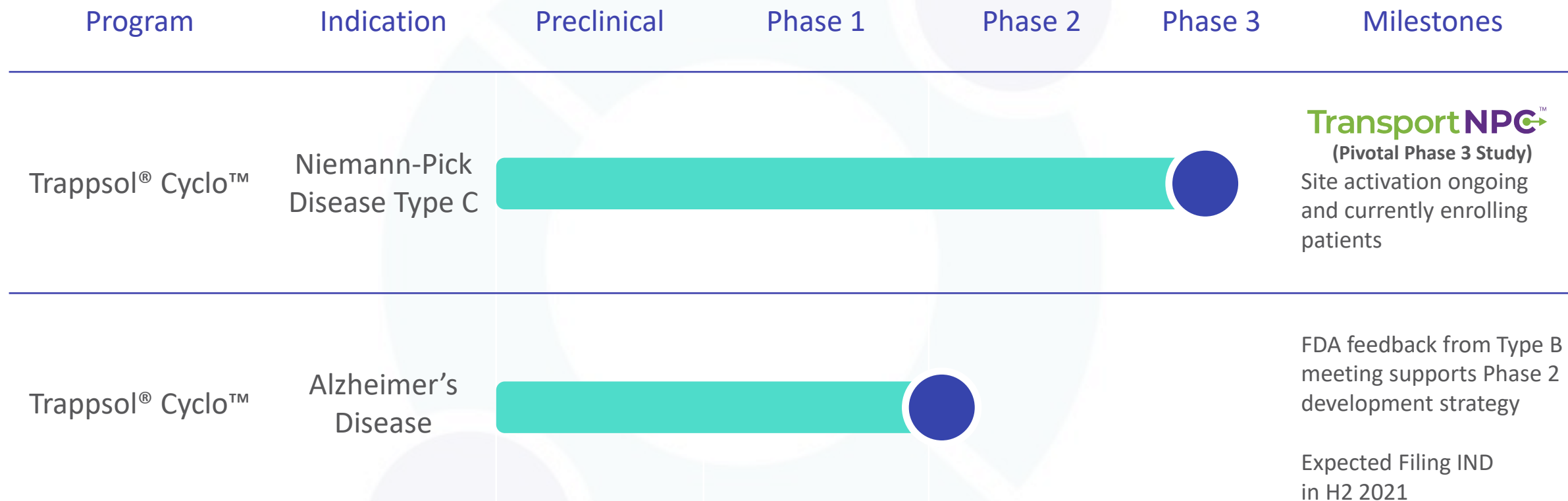
6th leading cause of death affecting 5 million people in the U.S.²

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>

Pipeline



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



Sharon H. Hrynkow, Ph.D.
Chief Scientific Officer



Russ Belden
Acting Chief Commercial Officer



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



Lori McKenna Gorski
Global Head of Patient Advocacy



Niemann-Pick Disease Type C

Site activation ongoing and currently enrolling patients in the Pivotal Phase 3 study, TransportNPC™

The TransportNPC™ trial is currently the most advanced clinical research program underway to identify a treatment for NPC.



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. Site activation ongoing and currently enrolling patients in pivotal Phase 3 study, TransportNPC.	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)	FDA provided a Complete Response Letter 17-Jun-2021 citing additional qualitative and quantitative evidence was needed to show the drug's effectiveness. EMA submission completed November 2020, expected feedback H2 2021, with potential approval Q1 2022.	Neurological
	IB1001 (Oral 3 times daily)	Met with FDA, EMA and UK to discuss Phase 2 results. 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.	Neurological
	Adrabetadex	Received Court Approval to buy from MNK 29-Jun-2021. MNK Ph 2/3 failed and program was concluded in January 2021. The Expanded Access Program (EAP) was transferred in the deal. The FDA lifted the Clinical Hold for the EAP and can now add new patients who are not eligible for a formal clinical trial.	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

*Scope: United States + 79 other countries; *Commissioned 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.

Cyclo Therapeutics Scientific Rationale for NPC

Mechanistic attributes of Trappsol® Cyclo™

- Trappsol® Cyclo™ is a hydroxypropyl-beta-cyclodextrins (HPBCDs) and has an affinity for cholesterol. What distinguishes the clinical program is the Intravenous Route of Administration allowing the drug to reach major peripheral organs and shown in studies to clear cholesterol from cells peripherally...

Figure 1: Mechanism of Action

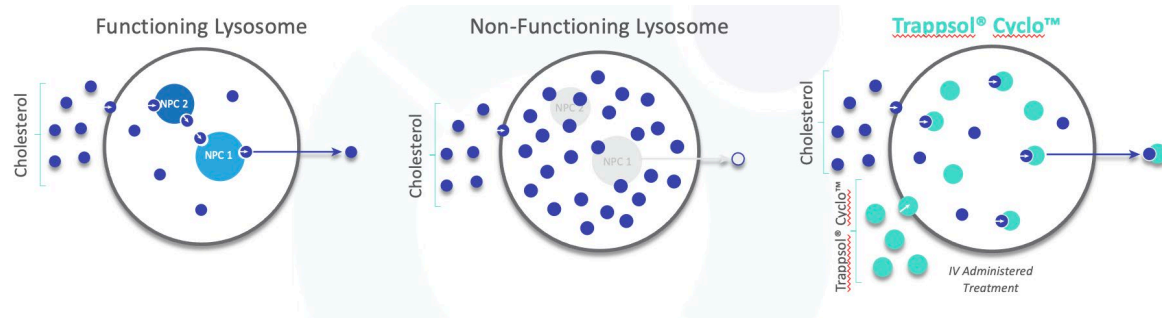
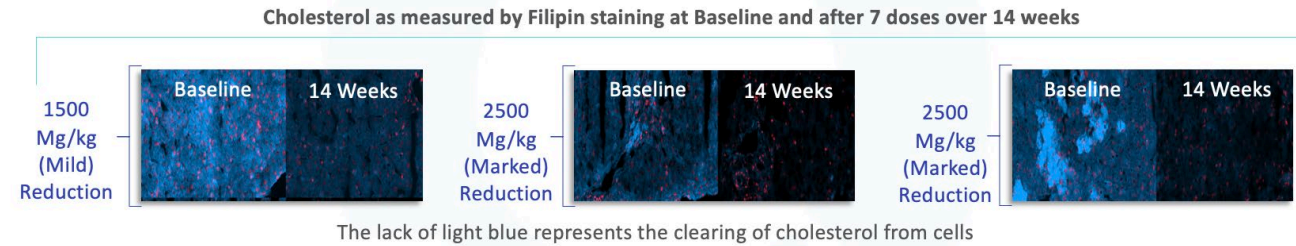


Figure 2: Cholesterol Clearance from Liver cells



- and centrally, affects CNS biomarkers and underpin neurologic outcomes as demonstrated in our current data from our completed and ongoing studies.

Figure 3: Reduction in Brain Cholesterol

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

24S-hydroxycholesterol (ug/L)

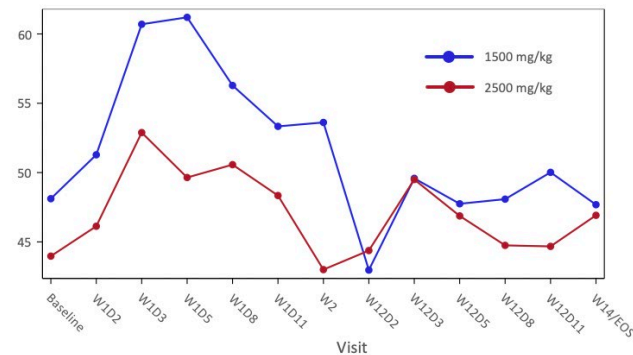
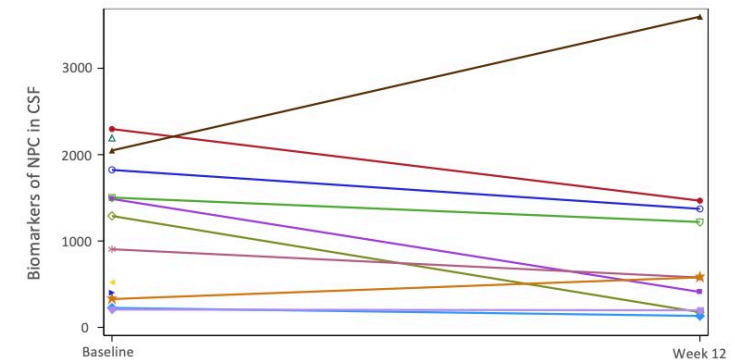


Figure 4: Reduction in Tau

Tau levels as measured in the CSF are shown here for 10 NPC patients who had lumbar punctures prior to treatment with HPβCD and after seven doses. **Six of 10 patients** showed a reduction in Tau levels, two remained stable, and two increased, no dose-response relationship.

Tau (ng/L)



Trappsol® Cyclo™

Development Path Towards Potential Approval

Phase 1

Completed May 2020

- ✓ Favorable safety and tolerability profile
- ✓ Removal of trapped cholesterol
 - ✓ Drug present in CSF
- ✓ Increase in 24S biomarker
 - ✓ Decrease in Tau

Phase 1/2

Encouraging Topline Results
Reported March 2021

- ✓ Favorable safety and tolerability profile
- ✓ 100% of patients who completed the trial improved or remained stable per their treating physicians
- ✓ Demonstrated improvements in ataxia, swallow, walking and Quality of Life

TransportNPC[®] (Pivotal Phase 3)

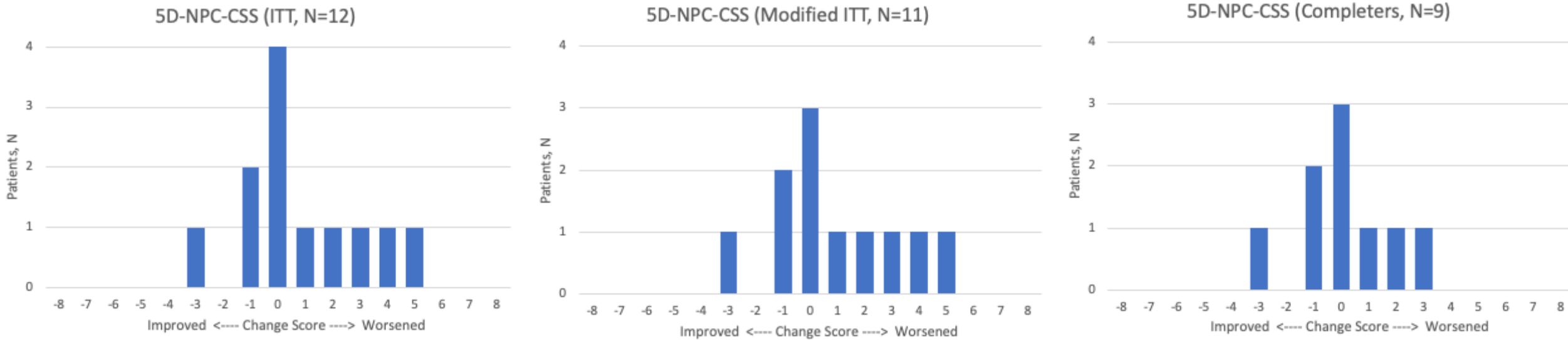
Site activation ongoing and currently enrolling patients

- ✓ Global clinical protocol agreed with U.S. FDA and EMA
- ✓ EMA PDCO agreed to Sub-study in patients 0-3 years of age in EU only
- ✓ EMA PDCO feedback stated it has potential as a preventative

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

Rare Pediatric Disease Designation | Orphan Designation in EU | EMA Pediatric Investigational Plan Adopted

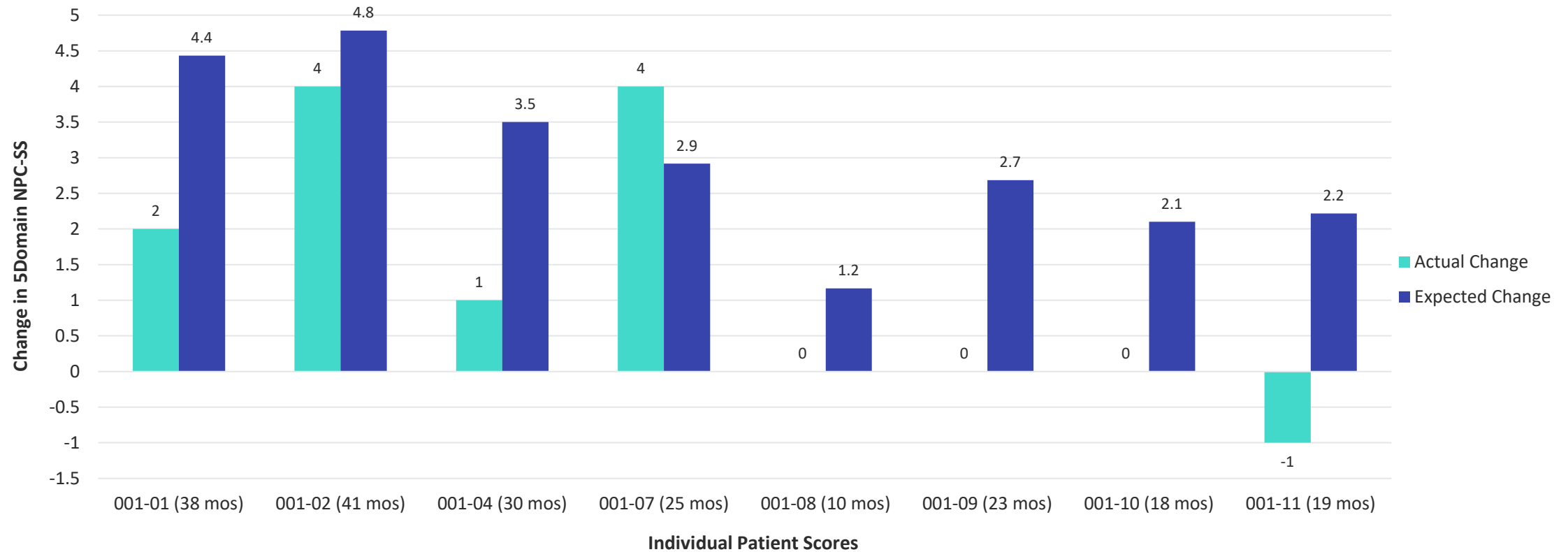
Efficacy of Trappsol® Cyclo™ in the CTD-TCNPC-201 study assessed using the 5-Domain NPC Clinical Severity Scale



ITT, intention to treat

For all 3 study population analyses, the median change in the 5D-NPC-CSS was 0, and 3 subjects showed improvements of 1 to 3 points. The mean changes in each population were 0.8 (ITT), 0.9 (modified ITT), and 0.1 (Completers). In the completer population, which arguably has the best potential to demonstrate a treatment effect, 67% (6/9) of subjects were either improved (33%, 3/9) or stable (33%, 3/9), while 33% worsened (3/9). Although a 1-point decrease in the 5D-NPC-CSS score clearly represents a clinically meaningful improvement, stability (change score of 0) or slowing of disease progression (change score <1.4 points/year) also reflect clinically meaningful treatment goals for a neurodegenerative disease like NPC that causes progressive disability and premature death.

Long-Term Treatment with Trappsol® Cyclo™ in the CTD-TCNPC-102 assessed using 5-Domain NPC Severity Scale



8 patients who completed Phase 1 trial were eligible for the Extension protocol, all joined. Green bars are actual change in 5D NPC-SS from Baseline (at start of Phase 1 trial) through last data point available in Extension protocol. Purple bars are expected changes without intervention using 1.4 point change per year after Cortina-Borja 2018. * = no change observed. Pt 001-09 added Miglustat after 1 year with no change to 5 D score or overall disease progression. Mean change in this group overall is 0.4.

Applying Development Insights to the Ph3 Program (**TransportNPC**[™])

Combined Results of 5D-NPC-CSS across the 2 long-term studies (102 and 201)

The 5D-NPC-CSS suggest a beneficial effect of Trappsol[®] Cyclo[™]. The mean annual change score for subjects receiving at least 48 weeks of treatment in the 2 studies was a mean increase of 0.24 (n=17). Most subjects had a better than expected change score (88%, 15/17) compared to the average rate of decline reported in the literature (increase of 1.4 points per year). Among these 17 patients, 24% (n=4) showed improvement, 35% (n=6) were stable, and 29% (n=5) showed slowing of disease progression.

Key features of the Ph3 Study via scientific advice with FDA and EMA:

- Primary Endpoint is the mean change from baseline to 48wks or 96wks as measured by improvement in the NPC-SS of Trappsol[®] Cyclo[™] over Placebo
 - 4D-NPC-SS (AM, FM, SP and SW) for United States and 5D-NPC-SS (AM, FM, SP, SW and CO) for Non-US.
- Increased number of patients for increased power of study
- Included ASIS scoring within inclusion criteria to bring homogeneity to patient cohort, allow for a more predictable view of patient trajectory
- Increased Study duration to 2-years with a 1-year Interim Analysis to capture disease progression

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 countries <div>United States, United Kingdom, Italy, Germany, Spain, France, Poland, Israel, and Australia</div>
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 Potential 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

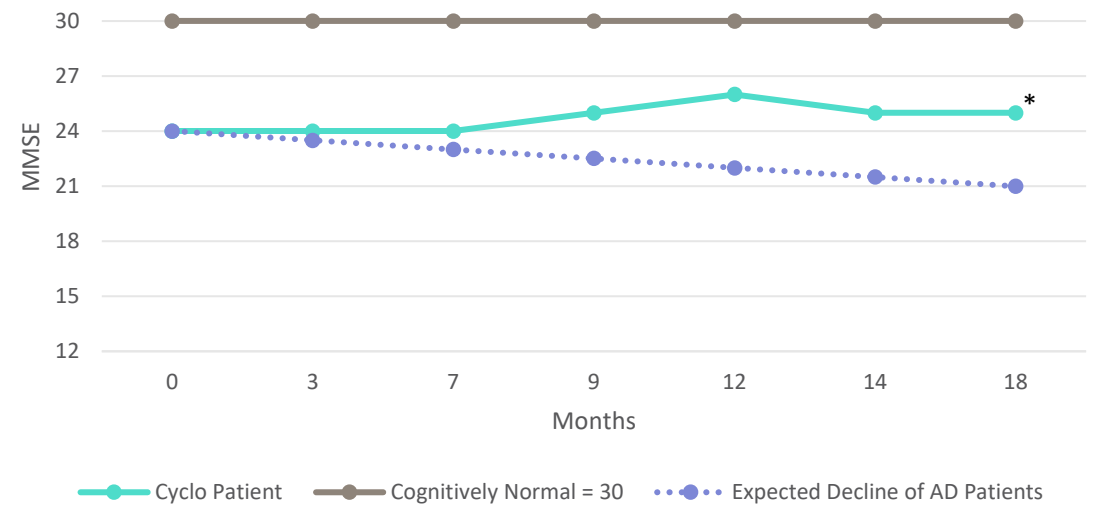
18 months of monthly IV infusion

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 an 18-month period in persons with Alzheimer's disease dementia"

Treating Physician

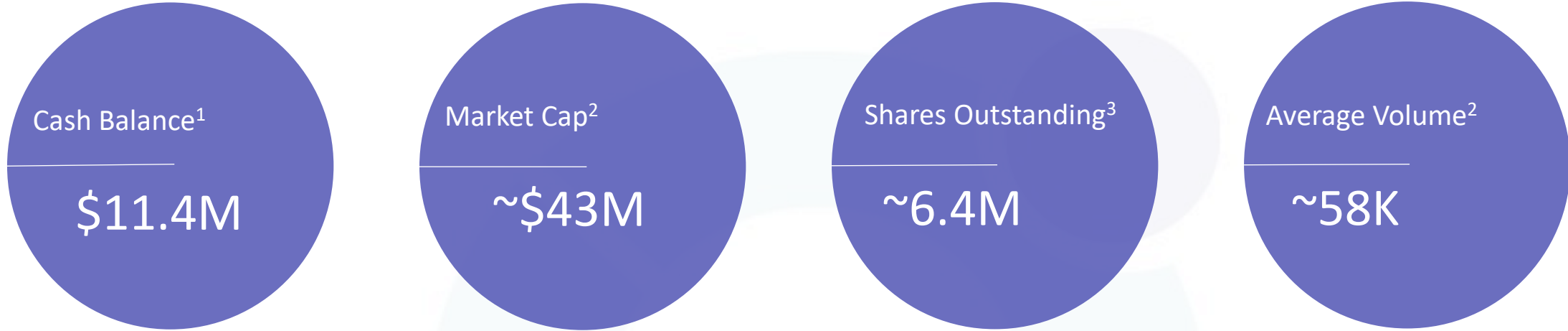
*Treating physician reported the 18-month score as a range between 24-26

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

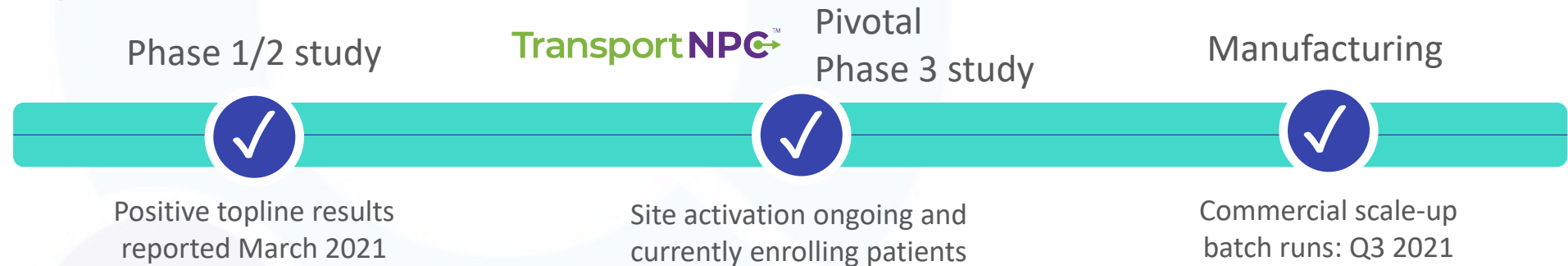


Financial and Capital Markets Snapshot

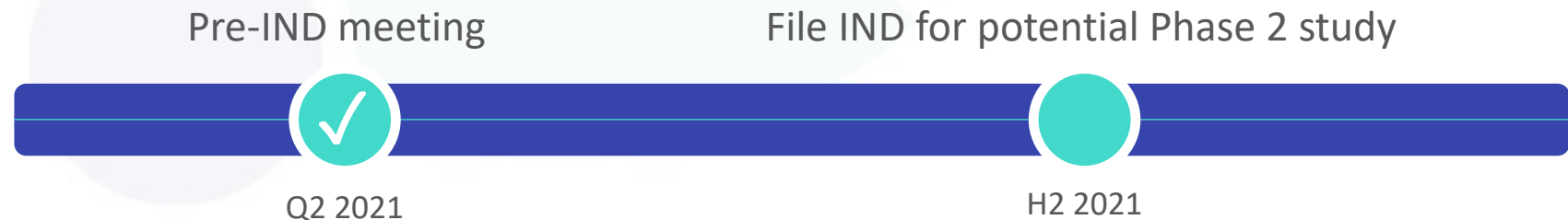


Target Upcoming Milestones with Potential to Drive Value

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

TransportNPC™

Site activation ongoing and currently enrolling 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

Planning and executing pre-approval commercialization imperatives

Pipeline expansion into Alzheimer's Disease (AD), patent pending

AD 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.



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.



