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Scientific Presentation
December 2023

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# Niemann-Pick Disease Type C

2 Completed Studies (101 and 201)

Ongoing Long-Term Extension Study (102)

Ongoing Pivotal Phase 3 Study (Transport NPC™, 301)

# Trappsol® Cyclo™ as a potential therapy for NPC
## Suggestive evidence of safety and systemic and neurological benefit

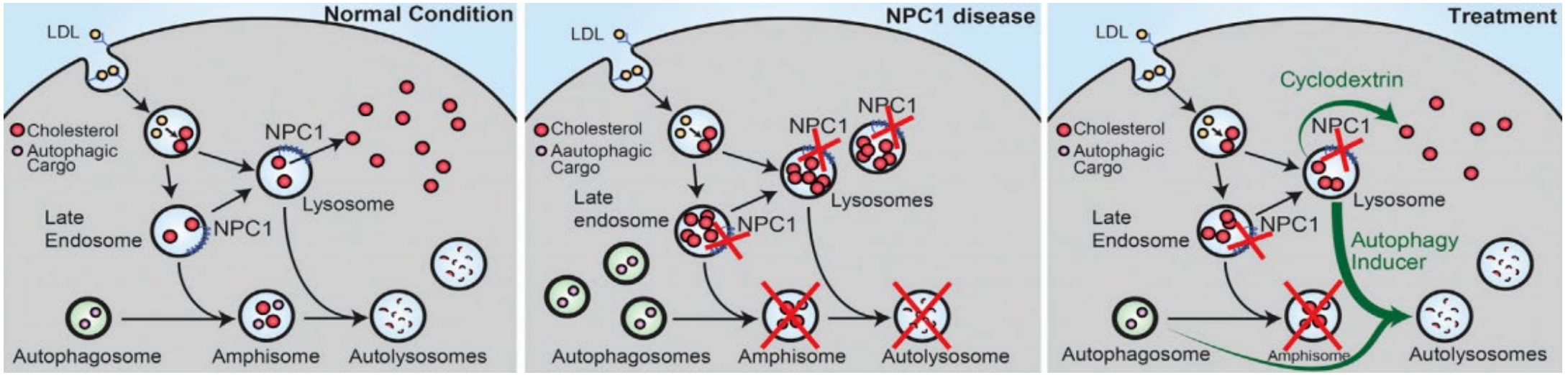
- **Mechanism of Action:** Trappsol® Cyclo™, with its cyclic structure, facilitates the transport of cholesterol between cells and subcellular structures thus circumventing blockades and enabling the normal function of cells and tissues.
- **Pre-clinical data**: Demonstrates the amount of HPβCD getting into the brain is at a safe and efficient concentration in order to enhance transfer of cholesterol between (intra)cellular membranes and therefore supportive of Trappsol® Cyclo™ having a potential safe and effective benefit for the neurological symptoms of NPC.
- **Phase 1 Study (CTD-TCNPC-101)**: Data suggests that Trappsol® Cyclo™ has an acceptable well-tolerated safety profile and demonstrates clearance of trapped liver cholesterol, indicates that cholesterol turnover is normalized and cholesterol synthesis rate adjusted for the whole body, shows reduced changes in metabolite level is indicative of reduced cholesterol storage and indicates normalization of cholesterol storage in brain neurons. These data are suggestive of a potential benefit for both the systemic and neurological symptoms of NPC.
- **Phase 2 Study (CTD-TCNPC-201)**: Data demonstrates that Trappsol® Cyclo™ has an acceptable well-tolerated safety profile and that a potential therapeutic benefit may be observed at or before 48 weeks of treatment compared to disease progression. Although studied in a small patient sample size, provides compelling evidence of disease stabilization or improvement for the 5D-NPC-SS, CGI-I and SARA scales.
- **Ongoing Extension Study (CTD-TCNPC-102)**: Provides long-term safety data demonstrating the product is well-tolerated along with data suggestive of durability of effect.

**Conclusion**

**The data obtained from preclinical studies and Cyclo Therapeutics completed and ongoing clinical studies suggest Trappsol® Cyclo™ is well-tolerated and has the potential to treat both the systemic and neurological symptoms of NPC. The data from our early phase studies and OLE are currently being confirmed in our ongoing Phase 3 Study (CTD-TCNPC-301).**

# Trappsol® Cyclo™
## Mechanism of Action: Mobilizing lysosomal cholesterol (NPC)

Under normal conditions, NPC1, located on the late endosomal/lysosomal (LE/L) compartments, regulates cholesterol efflux (Maetzel et al, 2014) (see left panel below). In NPC, mutations in the *NPC1* gene on both alleles lead to accumulation of cholesterol in the LE/L compartments by inhibiting its efflux, and to a block in autophagic flux, which causes accumulation of autophagosomes and autophagy substrate due to impaired formation of amphisomes (see middle panel below). Trappsol® Cyclo™ mediated cholesterol release independent of the function of both NPC1 and NPC2 proteins, circumventing blockades in cholesterol transport, and therefore has the potential to bring significant benefit to patients with NPC (see right panel below).



# Current status of product development for Trappsol® Cyclo™
## Pre-clinical published data and 4 clinical studies

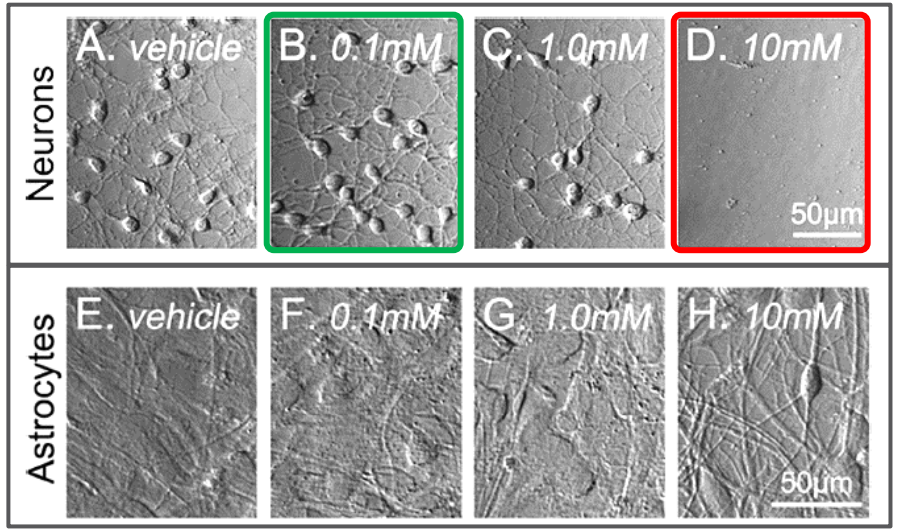
- **Study CTD-TCNPC-101 (NCT02939547):** A Phase I study to evaluate the single and multiple-dose pharmacokinetics of intravenous Trappsol Cyclo (HPβCD) in patients with Niemann-Pick disease Type C (NPC1) and the effects of dosing upon biomarkers of NPC disease (1 site in the US) has completed. Final Clinical Study Report submitted September 24, 2020
- **Study CTD-TCNPC-201 (NCT02912793)**: A Phase 1/2 study to evaluate the safety and pharmacokinetics of intravenous Trappsol Cyclo (HPβCD) in patients with NPC1 and the pharmacodynamic effects of treatment upon markers of cholesterol metabolism and clinical outcomes (United Kingdom and Israel). Final Clinical Study Report submitted November 9, 2021.
- **Study CTD-TCNPC-102 (NCT03893071):** An open-label extension study of the long-term safety and efficacy of intravenous Trappsol Cyclo (HPβCD) in patients with NPC1. Ongoing.
- **Study CTD-TCNPC-301 (NCT04860960)**: A multicenter, double-blind, randomized, placebo-controlled pivotal trial to evaluate the safety, tolerability, and efficacy of 2000 mg/kg of Trappsol Cyclo (administration as a slow infusion over 6.5 hours every 2 weeks) and standard of care (SOC) compared to placebo and SOC in patients with NPC. Ongoing.
    - **Sub-study under CTD-TCNPC-301 (EMEA-002839-PIP01-20)**: A multicenter sub-study in patients newborn to <3 years of age to evaluate the safety of 2,000mg/kg of Trappsol Cyclo (administration as a slow infusion over 6.5 hours every 2 weeks) and to obtain descriptive data regarding global severity and improvement in response to Trappsol Cyclo from both Investigator and patient perspectives (Clinical Global Impression – Severity [CGI-S], Clinical Global Impression of Change [CGI-C], Caregiver's Global Impression Scales [CaGI-S], Caregiver Global Impression of Change [CaGI-C], and Caregiver Global Impression of Change 24 hours after infusion [CaGI-C24]). Ongoing.

# Pre-clinical data

## Safe and efficient HPβCD target concentration within the brain

HPβCD effects in cell culture system:

- **0.1 mM HPβCD** released cholesterol trapped in the lysosomes
- **1 mM HPβCD** primarily extracted cholesterol from the cell membrane
- **10 mM HPβCD** was profoundly toxic to neurons




Effect of HPßCD (24 h) on cell cultures of neurons and astrocytes (mouse). At 10 mM HPßCD kills 100% of neurons (D), but not astrocytes (H) [1]

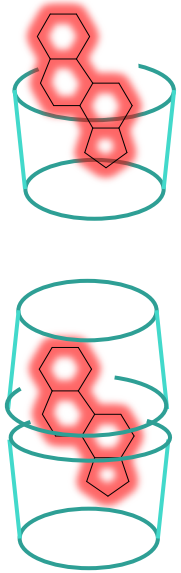


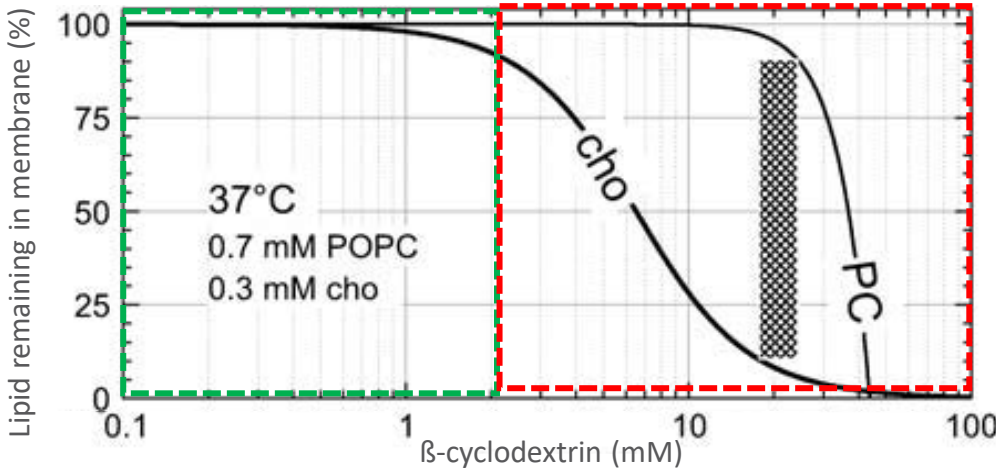
Figure courtesy of David Begley, Kings College, London

Below 1mM HPβCD can partially move cholesterol out from cell membranes (one HPβCD interacts with one cholesterol molecule).

This significantly enhances transfer of cholesterol between (intra)cellular membranes and provides an exit from lysosomes.

Abbove 1mM an increasing amount of cholesterol will be **extracted** from membranes (by two cyclodextrins per cholesterol) – this will damage sensitive cells.

At low concentrations effects of HPβCD and sulfobutyl-ß-CD in precinical models are similar. The latter compound cannot form dimers[3]




Effect of increasing concentrations of ß-cyclodextrin on lipid membranes. Above 1 mM ß-cyclodextrin an increasing fraction of cholesterol is extracted from membranes (image modified) [2]

[1] Peake KB, Vance JE; J Biological Chemistry (2012)
[2] Tsamaloukas A, et al; Biophys J (2005)
[3] Taylor AM, etal; JLR: (2012)

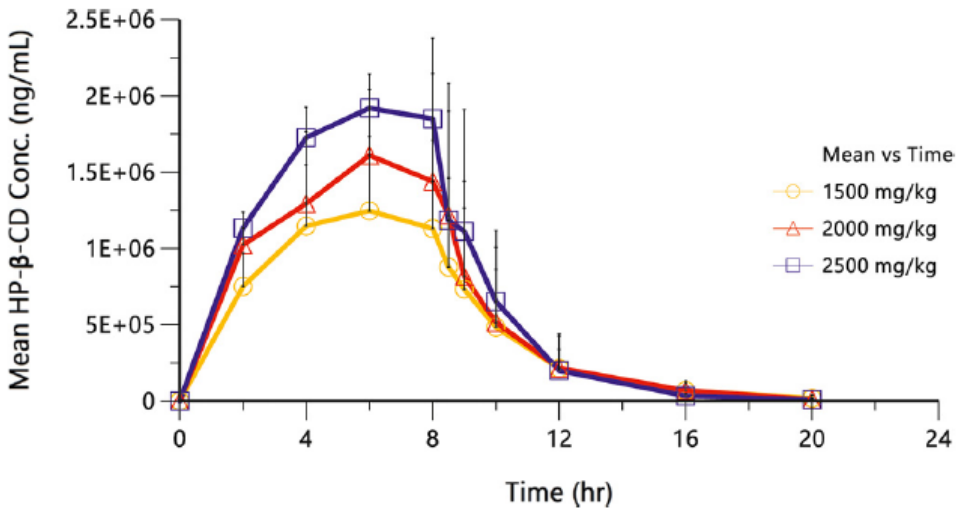
# Pre-clinical intravenous application and Study CTD-TCNPC-201 data
# Blood brain barrier transfer of saccharide compounds and 201 PK

- Under the extended infusion used in clinical trials for Trappsol® Cyclo™, a stable concentration of about 1 mM is reached in plasma
- $T_{1/2}$ Plasma app 2h; $T_{1/2}$ CNS app 6h (human and mouse data)
- Most mono-, oligo-, and polysaccharides show moderate transfer over the BBB. Transfer happens by micropinocytosis (transport in small vesicles, largely independent from molecular weight [2, 3]).
- Four saccharide compounds investigated for BBB transport [2, 3] are shown below. Uptake over the BBB was observed at 0.1 µl/g/min to 0.2 µl/g/min. Trappsol®Cyclo™ fits into the compound class and into the molecular weight range.
- Under the conditions used in the Trappsol® Cyclo™ NPC Phase 1 trial a peak concentration of 40 µM to 80 µM Trappsol® is expected in the CNS.
- The concentrations found in lumbar CSF of patients at the end of treatment of about 30 µM [1, 4] are in line with this value.
  A ratio of $C_{max\ (cranial)}$ to $C_{max\ (lumbar)}$ of 2 – 4 is assumed in literature [5].

BBB: Blood Brain Barrier; CSF: Cerebrospinal Fluid

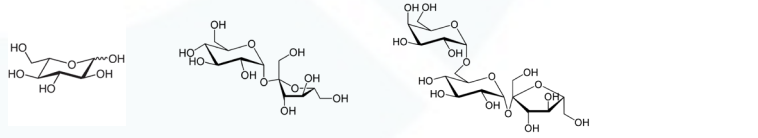

[1] Hastings C, et al; Molecular Genetics and Metabolism (2022)
[2] Amtorp O; Acta Physiol Scand (1980);
[3] Lucchesi KJ, Gosselin RE; Am J Physiol (1990);
[4] Hastings C, et al; Molecular Genetics and Metabolism Reports (2023)
[5] Gaohua L, et al; Drug Metab Pharmacokin (2016)


**Plasma Pharmacokinetic Data of Trappsol Cyclo from CTD-TCNPC-201 Study**

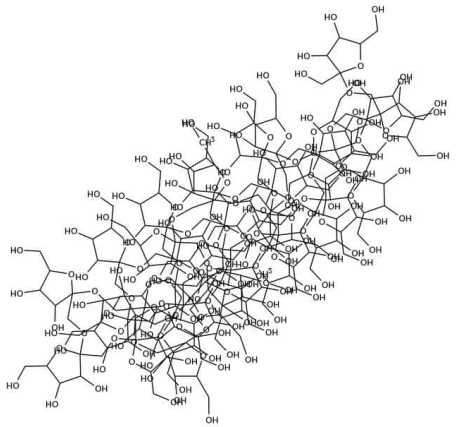



Mean (SD) Plasma Concentrations (ng/mL) of HPβCD versus Time in Patients Following an Initial IV Infusion of Trappsol® over 8 hours at 1500, 2000, or 2500 mg/kg[1]. The 2'000 mg/kg dose was selected for the phase 3 trial.

HPβCD (Trappsol® Cyclo™)



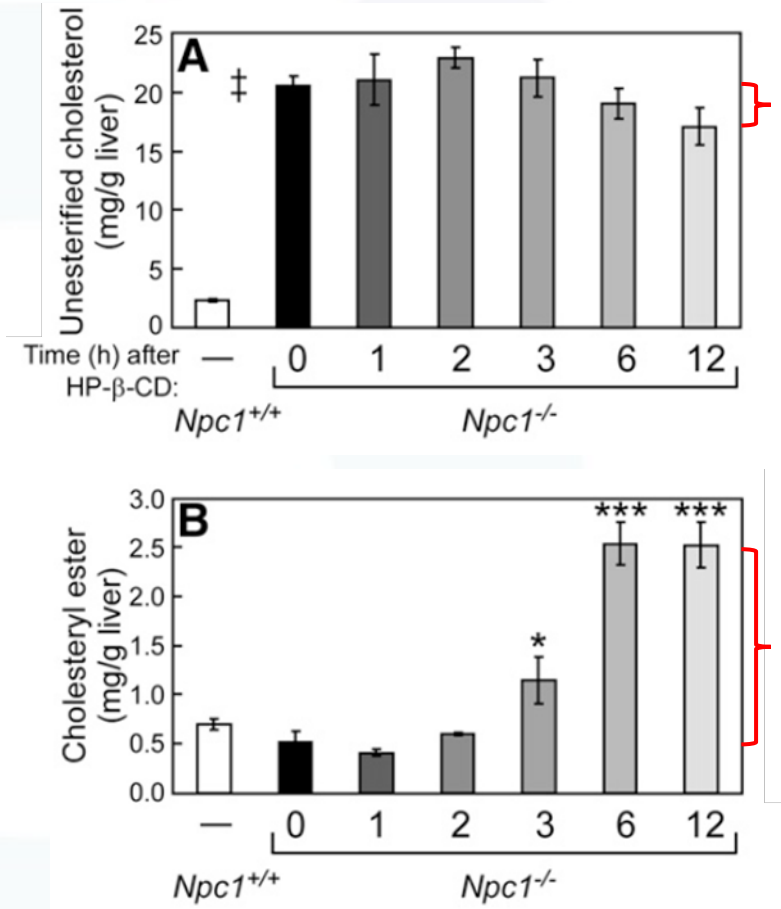
L-Glucose Sucrose Raffinose Inulin →



# Pre-clinical data from mice over 0hr-12hr

## Immediate effect: mobilization of cholesterol from lysosomes in liver

- Rate of uptake of Trappsol® into liver cells was found to be similar to transfer over the BBB (10 µl/h/g – 20 µl/h/g)
- **Export of unesterified cholesterol from lysosomes until 12h from start of single HPßCD injection is about 3 mg/g of liver tissue**
- Exported cholesterol transiently stored as cholesterol ester in lipid droplets in the cytosol
- **Increase in cholesterol ester concentration in cytosol over 12h reflects decrease in lysosomal concentration**
- Note: Slow infusion of Trappsol® in humans makes an export of 3 mg – 6 mg of cholesterol / g of liver tissue per treatment cycle likely




About 3 mg/g of unesterified cholesterol removed from liver lysosomes

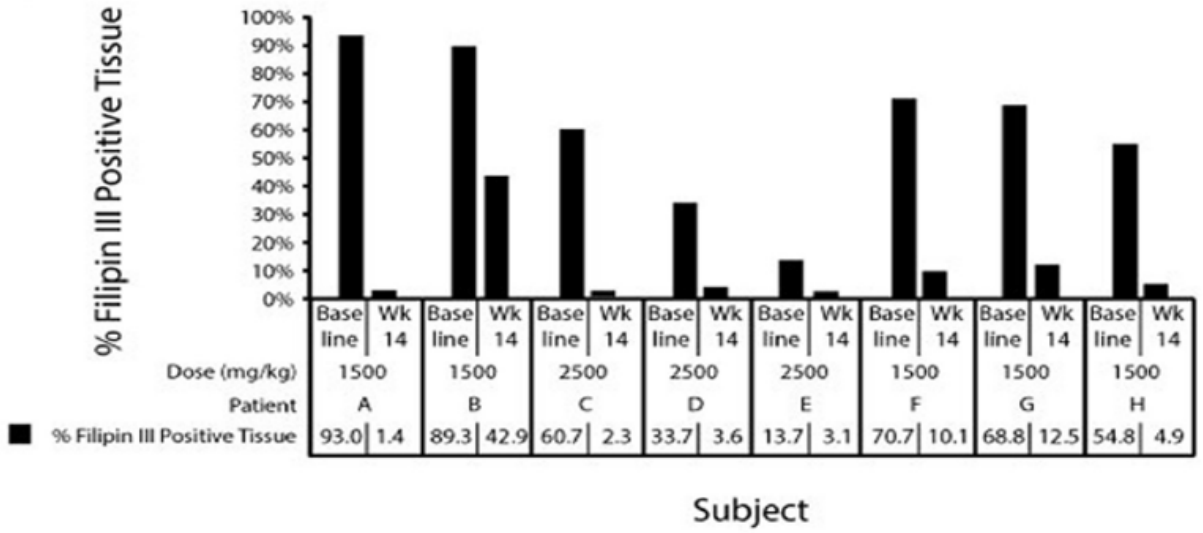
@12h about 2 mg/g of esterified cholesterol transiently stored in cytosol of hepatocytes

Taylor AM, etal; JLR: (2012); Cyclodextrin mediates rapid changes in lipid balance in Npc1-/- mice without carrying cholesterol through the bloodstream;

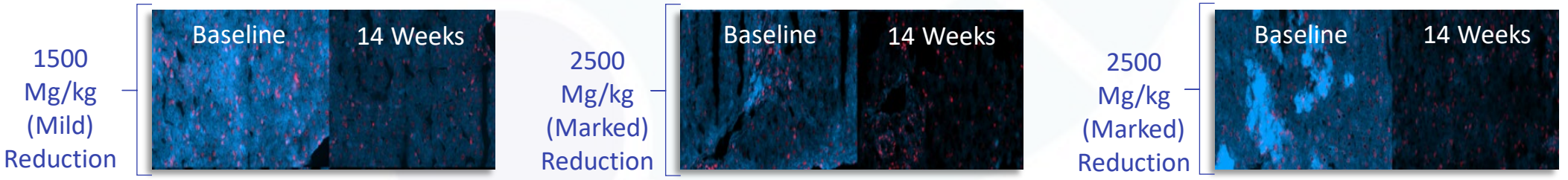
# Phase 1 Study (CTD-TCNPC-101)

## Clearance of trapped liver cholesterol following 14 weeks of treatment

The percentage of filipin III (filipin) - stained positive tissue area in liver tissue samples from 8 NPC1 subjects at Baseline and 2 weeks after the seventh HPβCD infusion




Representative images of filipin staining of liver tissue of unesterified lysosomal cholesterol at Baseline and 14weeks post-treatment with low (1500 mg/kg) and high (2500 mg/kg) doses

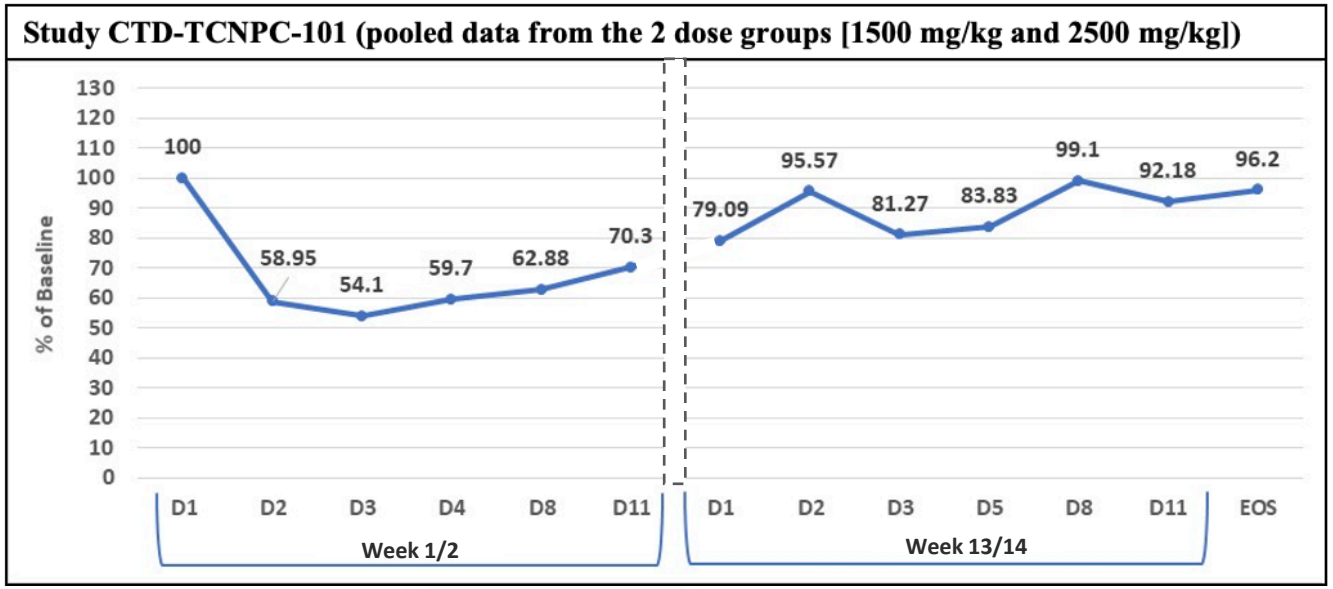



The lack of light blue represents the clearing of cholesterol from cells

# Phase 1 Study (CTD-TCNPC-101): Lathosterol biomarker
## Single infusion reduces cholesterol biosynthesis

- Lathosterol is a precursor of whole-body cholesterol
- Observed pattern of serum lathosterol levels in week 1 – 2 consistent with reduction in cholesterol synthesis following release of trapped cholesterol
- New equilibrium following repeated infusions (week 13 – 14)
- Indicates that cholesterol turnover normalized and cholesterol synthesis rate adjusted

**Mean Serum Levels of Lathosterol as a Percentage of Baseline Mean Value (Studies in Patients with NPC)**

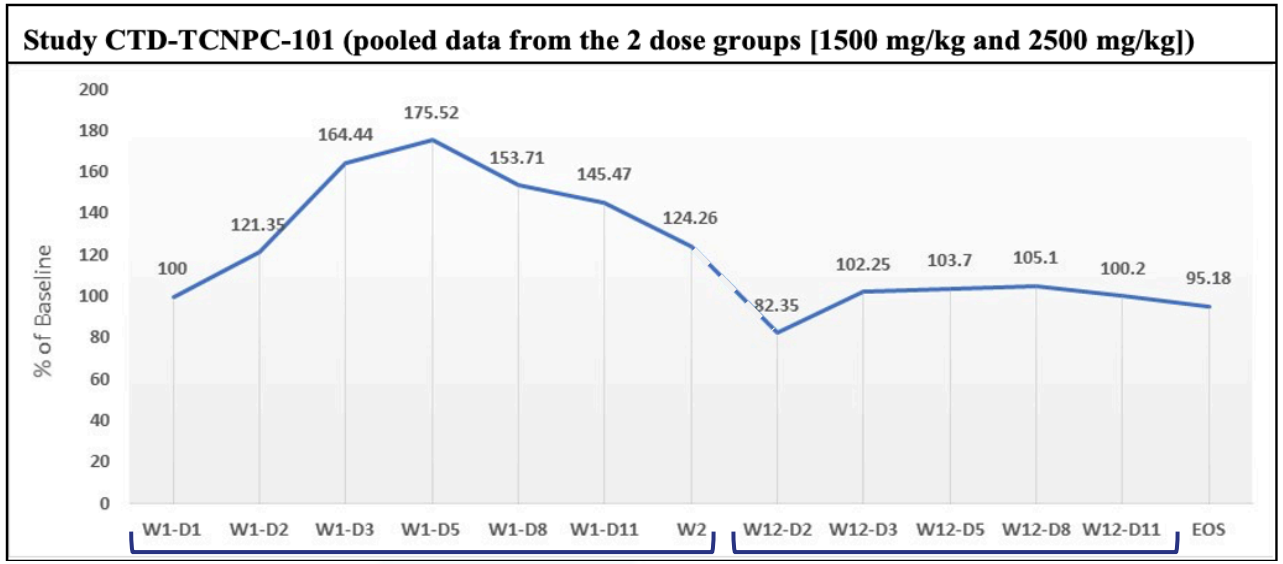



Abbreviations: D = Day 1, 2, 3, etc.; EOS = end of study;

# Phase 1 Study (CTD-TCNPC-101): 4β-hydroxycholesterol biomarker
## Single infusion increases cholesterol catabolism

- Rapid onset of effects of Trappsol Cyclo with increase in cholesterol catabolism within days and lasting for more than 1 week post infusion
- Peak in cholesterol metabolite supports extensive release of trapped cholesterol
- Following 12 weeks of treatment, reduced changes in metabolite level indicative of reduced cholesterol storage

**Mean Serum 4β-Hydroxycholesterol as a Percentage of Baseline Values**

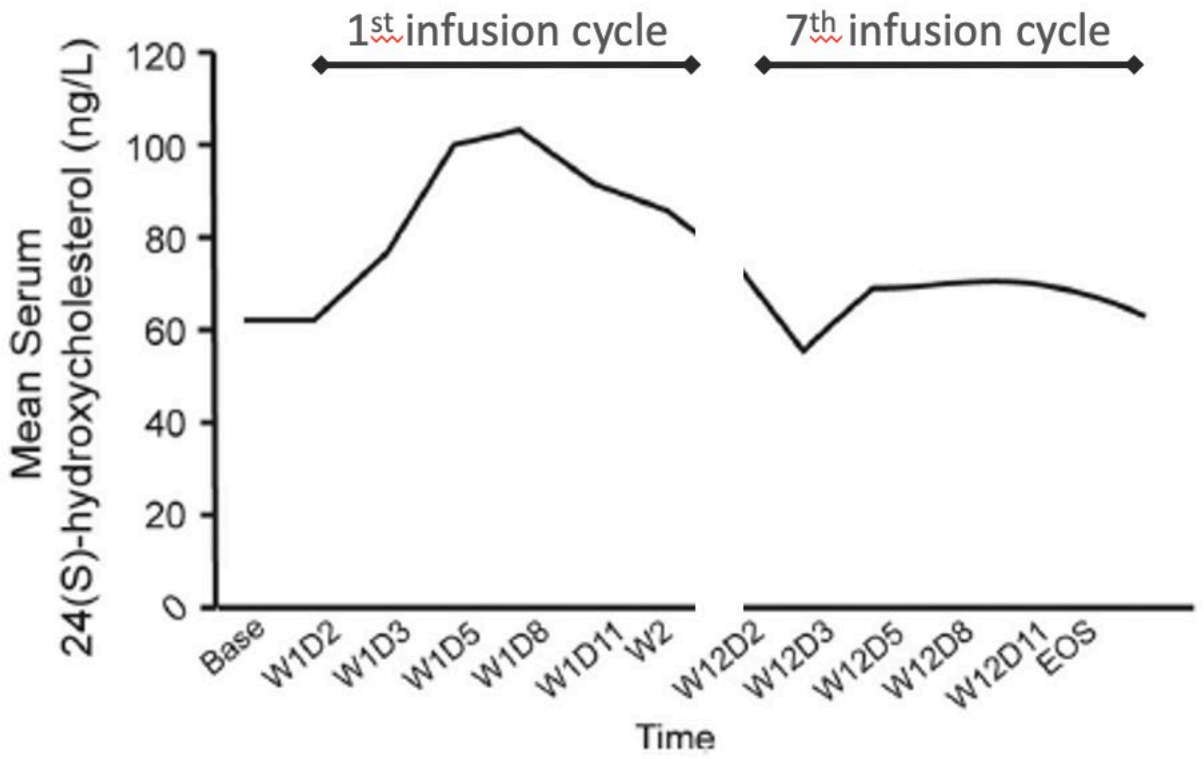



Abbreviations: W= Week 1, 2, 12; D = Day 1, Day 2, Day 3, etc.; EOS = end of study;

Note: In mice, there is a clear transient increase of 7-α-hydroxycholesterol, prime cholesterol degradation product; not measured in humans due to significant diurnal variation.

# Phase 1 Study (CTD-TCNPC-101): 24S-Hydroxycholesterol, biomarker
## Increased cholesterol catabolism in brain neurons

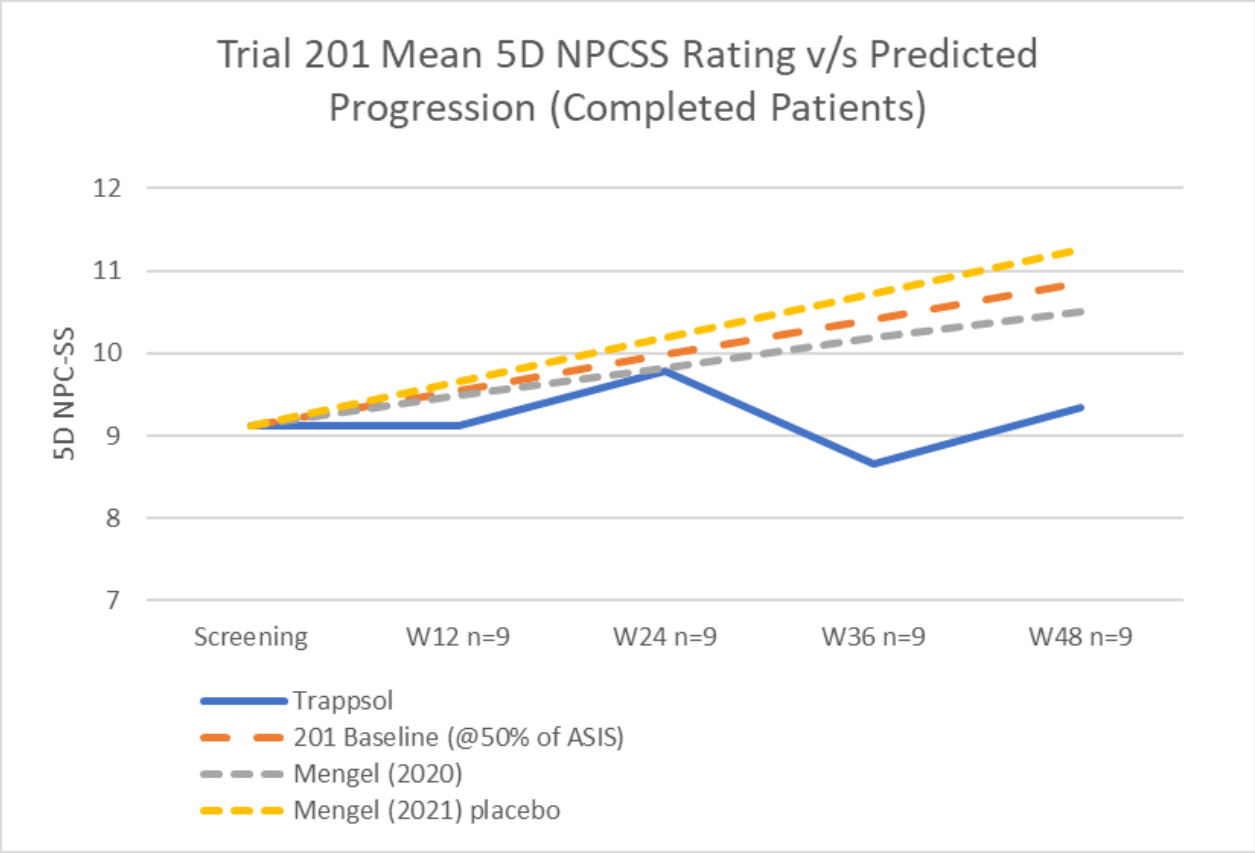
- **24S-hydroxycholesterol**, a cholesterol metabolite from neurons in the CNS. Crosses the BBB and measured in serum.
- Increases in serum **24S-hydroxycholesterol** following initial administration of HPβCD
- Reduction of effect over 14 weeks, indicates normalization of cholesterol storage in brain neurons




The mean serum 24(S)-hydroxycholesterol (ng/L) level increased in subjects from both dose groups after the first (Week 1 Day 2, W1D2) and seventh (Week 12 Day 3, W12D3) infusions with HPβCD and then returned to baseline levels. The peak following the seventh infusion was reduced compared to that of the first infusion. Hastings et al. 2022

# Phase 1/2 Study (CTD-TCNPC-201)

## Potential benefit may be observed at or before 48 weeks




Data represented in above graph is from different studies. Solid line is from Cyclo Therapeutics CTD-TCNPC-201 Study. The dashed lines are disease progression rates identified from the following 3 sources:

- In the most recent studies on disease progression in 2 reference patient cohorts, both with and without baseline treatment by miglustat, an average annual progression of 2.94 points (21 patients: Ory, 2017) and 2.92 points (36 patients: Mengel, 2020) on the 17D scale was found, with the 5D-NPC-CSS scale showing a progress of 1.5 units per year (Mengel, 2020).
- The placebo group in the Orphazyme Phase 2/3 study (16 patients: Mengel, 2021) showed an annual progression on the 5D-scale of 2.15 points and on the 17D-scale (excluding hearing domains) of 2.8 points. The 17D-NPC-CSS parameter is in good agreement with the 2 natural history cohorts mentioned above, while the 5D-NPC-CSS parameter is higher than in the 2 earlier studies (Mengel, 2021).
- The predicted disease progression of Cyclo Therapeutics Study CTD-TCNPC-201 cohort (completers) without treatment, based on 50% of baseline ASIS.

### Data Included

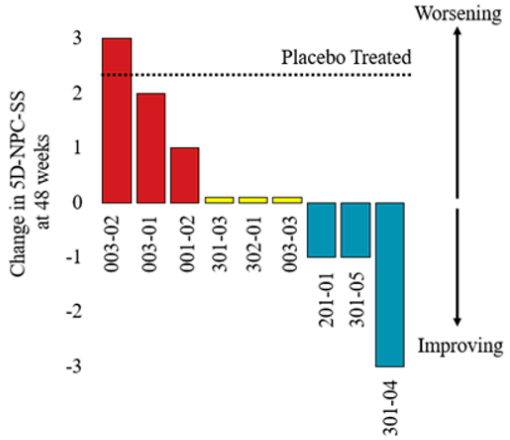
- 9 completers (2 patients in the 1500 mg/kg group; 4 patients in the 2000 mg/kg group; 3 patients in the 2500 mg/kg group).
    - Three patients were excluded from this analysis due to 1 only having baseline data and 2 patients discontinued prior to Week 48 for non-safety reasons.

### Key Outcomes

- Potential therapeutic benefit which may be observed at or before 48 weeks of treatment compared to disease progression.
- The small cohort size (9 patients) limits extensive analysis of the changes within the study.
- Importantly, transient variations cannot be separated from disease progression, but are expected to be reduced in a larger cohort (the ongoing Phase 3 CTD-TCNPC-301).

# Phase 1/2 Study (CTD-TCNPC-201): Continued
## Individual data suggestive of potential benefit on 5D-NPC-SS and CGI-I

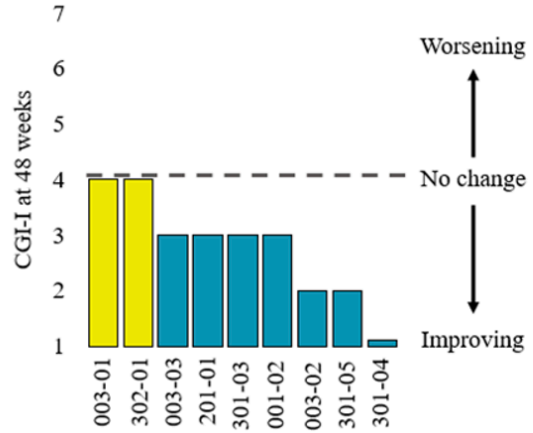
**201 Individual Completer Patient data: change in 5D-NPC-CSS score at 48 weeks**




- Scores ranked by benefit: least (left) to most (right)
- Individual patient numbers shown
- Majority of patients (6 of 9) exhibit stabilized or improved 5D-NPC-CSS scores at 48 weeks

Reference cohorts from the literature 2.15 points per annum (Mengel, 2021)

**201 Individual Completer Patient data: CGI-I score at 48 weeks**




- All patients (9 of 9) exhibit stabilized or improved CGI scores at 48 weeks
- Scores ranked by benefit: least (left) to most (right)
- Individual patient numbers shown

### Data Included

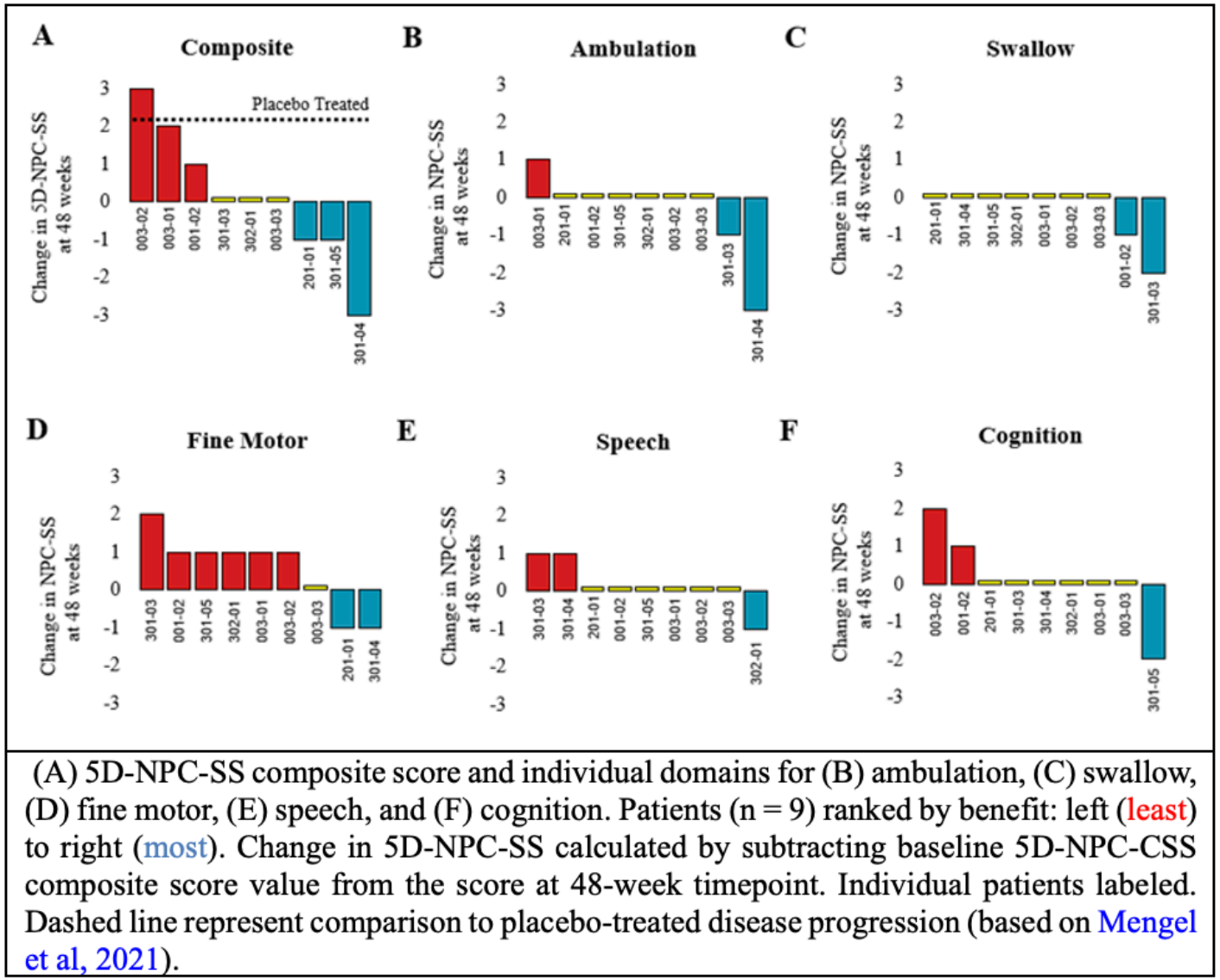
- 9 completers (2 patients in the 1500 mg/kg group; 4 patients in the 2000 mg/kg group; 3 patients in the 2500 mg/kg group).
  - Three patients were excluded from this analysis due to 1 only having baseline data and 2 patients discontinued prior to Week 48 for non-safety reasons.

### Key Outcomes

- In a disease characterized by linear disease progression, treatment with Trappsol Cyclo resulted in the stabilization or improvement in 6 of 9 (67%) completer patients on the 5D-NPC-SS
- 8 of 9 (89%) completer patients showed Improvements over disease progression (2.15 points per annum) in the 5D-NPC-SS
- 9 of 9 (100%) completer patients exhibited stabilized or improved in CGI-I ratings, a clear indicator of efficacy in NPC-1 patients

# Phase 1/2 Study (CTD-TCNPC-201): Continued

## Data suggestive of the utility of the 5D-NPC-SS alongside secondary endpoints




(A) 5D-NPC-SS composite score and individual domains for (B) ambulation, (C) swallow, (D) fine motor, (E) speech, and (F) cognition. Patients (n = 9) ranked by benefit: left (least) to right (most). Change in 5D-NPC-SS calculated by subtracting baseline 5D-NPC-CSS composite score value from the score at 48-week timepoint. Individual patients labeled. Dashed line represent comparison to placebo-treated disease progression (based on Mengel et al, 2021).

## Data Included

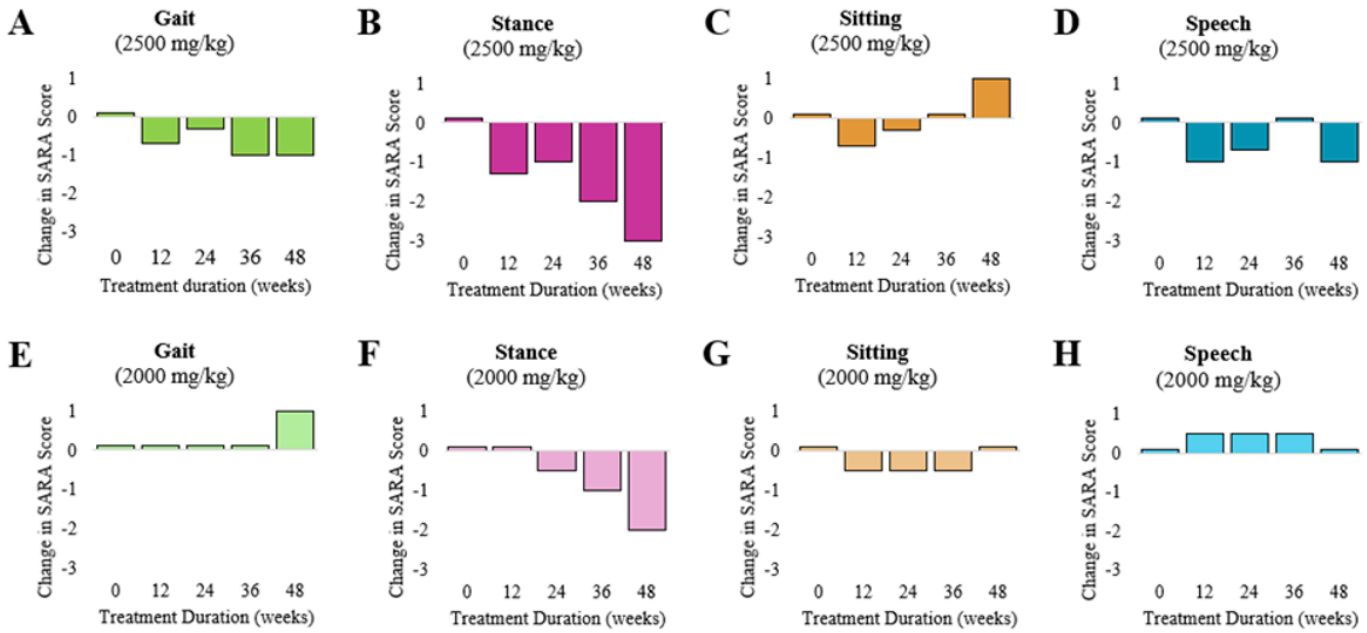
- 9 completers (2 patients in the 1500 mg/kg group; 4 patients in the 2000 mg/kg group; 3 patients in the 2500 mg/kg group).
  - Three patients were excluded from this analysis due to 1 only having baseline data and 2 patients discontinued prior to Week 48 for non-safety reasons.

## Key Outcomes

- On a patient-specific level, no consistent treatment patterns were observed across 5D-NPC-SS domains at 48 weeks.
- Although some domains were universally stabilized (i.e., swallowing), each patient stabilized or improved on a unique combination of assessments that contributed to their composite score.
- Cyclo Therapeutics believes this can be attributed to the diverse ages of enrolled patients (2-39 years of age) and reflects the heterogeneity of disease stage and progression, yielding differential sensitivity to drug effect based upon patient phenotype.
- Data suggest the utility of the 5D-NPC-SS composite score alongside planned secondary outcome measures (i.e., Spinocerebellar Ataxia Functional Index [SCAFI], Vineland-2, PAS) to holistically observe disease progression across diverse patient population, consistent with those randomized and approaching a 48-week endpoint in the ongoing Phase 3 (CTD-TCNPC-301)

# Phase 1/2 Study (CTD-TCNPC-201): Continued

## Dosage data suggestive of potential benefit on SARA, an exploratory measure




Scale for Assessment and Rating of Ataxia (SARA) scores for select domains: (A, E) gait, (B, F) stance, (C, G) sitting, and (D, H) speech. Patients receiving (upper panel) 2500 mg/kg dose (n = 3) or (lower panel) 2000 mg/kg dose (n = 4) for 48 weeks. Change in scores for individual SARA domains was calculated by subtracting respective value at baseline from the value at 12-, 24-, 36-, or 48-week timepoint. A negative change in score for individual SARA domain suggests improvement, whereas a positive change suggests worsening of the disease progression.

## Data Included

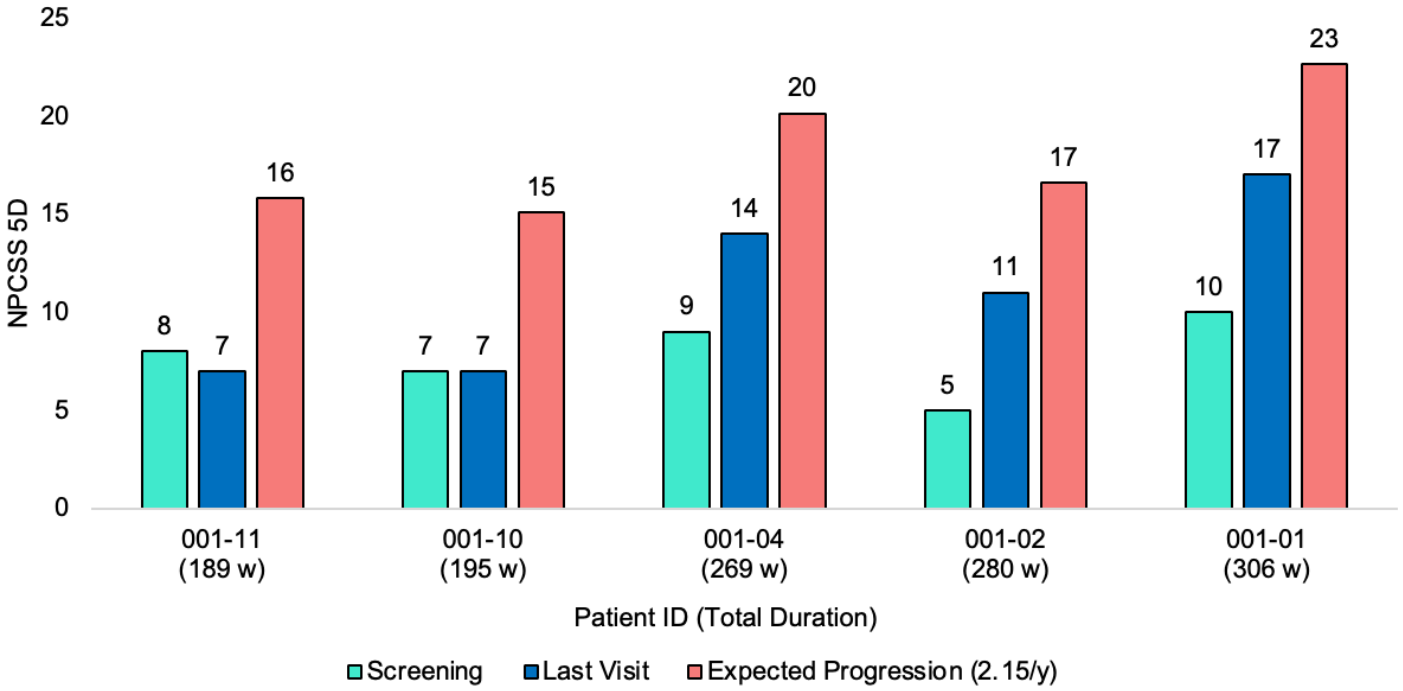
- Scores for individual SARA domains on a group level for multiple doses: 2500 mg/kg, 2000 mg/kg, and 1500 mg/kg. Descriptive presentation of patient-level data focused on the following SARA domains: gait, stance, sitting, and speech. Patients receiving a 1,500 mg/kg dose were not included in subsequent analyses due to incomplete or missing data at 48 weeks.

## Key Outcomes

- Multiple domains are reflected in these average scores: gait, stance, sitting, speech, finger chase (L/R), nose-finger (L/R), fast alt hands (L/R), and heel shin (L/R).
- Patients who received (2500 mg/kg or 2000 mg/kg), exhibit stabilized or improved scores for the SARA domains considered (gait, stance, sitting, and speech) compared to baseline.
- Higher dose (2500 mg/kg) suggestively improved gait, stance, and speech at 48 weeks (Figure A-D).
- Lower dose (2000 mg/kg) suggestively improved stance and stabilized sitting and speech at 48 weeks (Figure E-H).
- Potential benefits, at least descriptively, are dose-ordered and higher exposures may produce a more consistently favorable effect, acknowledging small sample size and heterogeneity of patient presentation.

# Ongoing Long-Term Extension (OLE) Study (CTD-TCNPC-102)
## Suggestive evidence of durability of effect

### Efficacy data from Ongoing Long-Term Extension (OLE) Study




Expected disease progression has been identified from the following source:

- The placebo group in the Orphazyme Phase 2/3 study (16 patients: Mengel, 2021) showed an annual progression on the 5D-scale of 2.15 points and on the 17D-scale (excluding hearing domains) of 2.8 points.

**Data Included is from an August 2023 data cut**

- 5 patients remain on active treatment, all receiving 1,500 mg/kg every 2-weeks via in-home infusions.
- Some patients had breaks during their treatment; 001-01 (break of 65wks and 55wks), 001-02 (break of 54wks) and 001-04 (break of 38 wks)

**Key Outcomes from the OLE**

- All 5 patients exhibited slowing of disease progression, with some patients (2 of 5 who did not have any breaks in treatment) showing stabilization or improvement.
- Suggestive evidence of Durability of effect for Trappsol® Cyclo™ as a chronic medication
- The Safety and tolerability remains consistent with completed and ongoing trials and has been well tolerated.

# Safety from completed and ongoing long-term treatment
## Suggestive of a benefit risk that is safe and well-tolerated

- The observed safety and tolerability profile consistent across studies and treatment duration, irrespectively of age spectrum and disease severity

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- Treatment-Emergent Adverse Events majority mild to moderate in severity, manageable and monitorable and most considered unrelated to Trappsol® Cyclo™
- No evidence of any untoward effects of Trappsol® Cyclo™ on core organ systems (cardiovascular, respiratory, renal, hepatic, gastrointestinal systems or CNS)

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- Hearing loss and infusion reactions (most localized) are adverse events of interest
- Events of hearing loss resolved in most patients, with hearing returning to baseline levels or improved and stabilized while patients continued on study drug
- A degree of hearing impairment remained at the last available auditory assessment in a limited number of patients
- The effect on hearing will continue to be monitored closely in the ongoing studies

# Trappsol® Cyclo™ Summary of completed studies and OLE

## 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
- Publication to the Molecular Genetics and Metabolism Report: Intravenous 2-hydroxypropyl-β-cyclodextrin (Trappsol® Cyclo™) demonstrates biological activity and impacts cholesterol metabolism in the central nervous system and peripheral tissues in adult subjects with Niemann-Pick Disease Type C1: Results of a phase 1 trial (2022)

## 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
- 8 of 9 (89%) completer patients showed Improvements over disease progression (2.15 points per annum) in the 5D-NPC-SS
- 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
- Publication to the Molecular Genetics and Metabolism Report: The Long-term administration of intravenous Trappsol® Cyclo™ (HPβCD) results in clinical benefits and stabilization or slowing of disease progression in patients with Niemann-Pick disease type C1: Results of an international 48-week Phase I/II trial (2023)

## Study 102

Has the potential to treat both the systemic and neurological manifestations of NPC and is well-tolerated with an acceptable safety and tolerability profile

- The available data from the OLE study indicate that the study medication has an acceptable tolerability profile
- Although the number of patients is small, the data for the 5-Domain clinical severity scores (5-D-NPC-CSS) does show a potential for stabilization and slower disease progression following administration of Trappsol® Cyclo™ than may be otherwise expected based on published literature on the natural course of the disease over the longer term, therefore providing evidence of durability of effect.

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*Thank you!*