

January 5, 2021



Cyclo Therapeutics Announces Positive Efficacy Data from Extension Protocol with Trappsol® Cyclo™ in Patients with Niemann-Pick Disease Type C

Home-based intravenous infusions of Trappsol® Cyclo™ for up to one year show improvement in disease features or disease stabilization

GAINESVILLE, Fla.--(BUSINESS WIRE)-- Cyclo Therapeutics, Inc. (NasdaqCM:CYTH) (NasdaqCM:CYTHW), a clinical stage biotechnology company developing a cyclodextrin platform for the treatment of neurodegenerative diseases, including its lead candidate Trappsol® Cyclo™ for the treatment of Niemann-Pick Disease Type C (NPC), today announced initial data from its “Open-Label Study of Long-Term Safety and Efficacy of Intravenous Trappsol® Cyclo™ (HPBCD) in Niemann-Pick Disease Type C,” [NCT03893071](#). Eight patients living in the US who completed the Phase I trial ([NCT02939547](#)) were eligible for the extension program. All eight enrolled.

The first patient was dosed in the Open-Label extension study in May 2019 and the last patient enrolled was in February 2020. The initial efficacy results presented below have a data cut-off of September 2020.

All patients included in this analysis demonstrated disease stability or improvement as measured by the 17-domain NPC Severity Score scale (NPC-SS, Yanjinin et al, 2010). The NPC-SS measures primarily neurologic features of the disease, including ambulation, fine motor skills, ability to speak and swallow, and cognition. Without intervention, over the course of one year, NPC patients would be expected to worsen by one to two points using this scale.

During this Open-Label Study, patients receive intravenous infusions of Trappsol® Cyclo™ in their homes under the care of healthcare professionals every two weeks, with regular assessments for NPC-SS at UCSF Benioff Children’s Hospital Oakland under the care of Dr. Caroline Hastings and Dr. Benny Liu, Co-Principal Investigators.

Of the patients who received Trappsol® Cyclo™ at the 1500 mg/kg body weight dose level, four patients remained stable in terms of their NPC-SS and two patients improved:

Patient 1 had a baseline NPC-SS of 24; and the same score 10 months later.

Patient 2 had a baseline NPC-SS of 18, with the same score 4 months later and again 6

months after the second assessment.

Patient 3 had a baseline NPC-SS of 11 and the same score 7 months later.

Patient 4 had a baseline NPC-SS of 12 and the same score 6 months later.

Patient 5 had a baseline NPC-SS of 16, a score of 15 five months later (improvement of 1 point), and a score of 13 eight months later (representing improvement by total 3 points).

Patient 6 had a baseline NPC-SS of 12 and a score of 10 eight months later (an improvement of 2 points).

Of the patients in the 2500 mg/kg dose group, both improved overall:

Patient 7 had a baseline NPC-SS of 18, followed by a score of 20 four months later (a worsening of 2 points) and a score of 15 six months later (a total improvement of 3 points compared to baseline during the 10-month assessment period).

And, Patient 8 had a baseline NPC-SS of 12 and a score of 8 five months later (an improvement of 4 points).

“These preliminary data provide additional signals of efficacy for our drug as a treatment for NPC, a progressive and debilitating disease often leading to premature death. The fact that patients either stabilized or improved during periods up to one year is consistent with data emerging from our Phase I/II in Europe and Israel,” said Cyclo Therapeutics CEO and Director, N. Scott Fine. “These extension protocol data provide additional compelling support for the efficacy of our drug in this patient population, and as we launch our pivotal Phase 3 trial in early 2021.”

The safety profile was favorable with 16 Adverse Events (AEs) in total between May 2019 and September 2020, all mild (Grade 1 and 2) and none related to the study drug, per the treating physician.

“NPC is a progressive, devastating disorder without approved therapies in the United States. The overall safety profile of Trappsol[®] Cyclo[™] is reassuring in that the drug is well tolerated, with adverse events in the mild and moderate range of severity. In addition, the clinical trajectory of the participants suggests stabilization while on therapy. These results offer the exciting promise of an effect on the progression of symptoms in NPC, a notable breakthrough for this condition,” said Loren Pena, MD, PhD, Associate Professor, Department of Pediatrics, University of Cincinnati College of Medicine and Attending Physician, Division of Human Genetics, Cincinnati Children’s Hospital, who is not associated with the study.

Cyclo Therapeutics has authorization from FDA to begin enrollment in its phase 3 trial and plans to enroll the first patient early in 2021.

About Cyclo Therapeutics:

Cyclo Therapeutics, Inc. is a clinical-stage biotechnology company that develops cyclodextrin-based products for the treatment of Niemann-Pick Disease Type C and Alzheimer’s Disease. The company’s Trappsol[®] Cyclo[™], an orphan drug designated

product in the United States and Europe, is the subject of three ongoing formal clinical trials for Niemann-Pick Disease Type C, a rare and fatal genetic disease, (ClinicalTrials.gov [NCT02939547](https://clinicaltrials.gov/ct2/show/study/NCT02939547), [NCT02912793](https://clinicaltrials.gov/ct2/show/study/NCT02912793) and [NCT03893071](https://clinicaltrials.gov/ct2/show/study/NCT03893071)). The company is planning an early phase clinical trial using Trappsol[®] Cyclo[™] intravenously in Alzheimer's Disease based on encouraging data from an Expanded Access program for late-onset Alzheimer's Disease ([NCT03624842](https://clinicaltrials.gov/ct2/show/study/NCT03624842)). Additional indications for the active ingredient in Trappsol[®] Cyclo[™] are in development. For additional information, visit the company's website: www.cyclotherapeutics.com

Safe Harbor Statement:

This press release contains "forward-looking statements" about the company's current expectations about future results, performance, prospects and opportunities, including, without limitation, statements regarding the satisfaction of closing conditions relating to the offering and the anticipated use of proceeds from the offering. Statements that are not historical facts, such as "anticipates," "believes" and "expects" or similar expressions, are forward-looking statements. These statements are subject to a number of risks, uncertainties and other factors that could cause actual results in future periods to differ materially from what is expressed in, or implied by, these statements. The factors which may influence the company's future performance include the company's ability to obtain additional capital to expand operations as planned, success in achieving regulatory approval for clinical protocols, enrollment of adequate numbers of patients in clinical trials, unforeseen difficulties in showing efficacy of the company's biopharmaceutical products, success in attracting additional customers and profitable contracts, and regulatory risks associated with producing pharmaceutical grade and food products. These and other risk factors are described from time to time in the company's filings with the Securities and Exchange Commission, including, but not limited to, the company's reports on Forms 10-K and 10-Q. Unless required by law, the company assumes no obligation to update or revise any forward-looking statements as a result of new information or future events.

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Source: Cyclo Therapeutics, Inc.