

February 11, 2020



## Last Patient Last Visit in Cyclo Therapeutics' Phase I Trial in Niemann-Pick Type C

Data cleaning underway, top line results expected the first quarter of 2020

GAINESVILLE, Fla.--(BUSINESS WIRE)-- Cyclo Therapeutics, Inc. (OTCQB: CTDH), a biotechnology company that develops cyclodextrin-based products for the treatment of Niemann-Pick Disease Type C (NPC) and Alzheimer's Disease, today announced that the last patient has undergone the final assessment in its Phase I trial to evaluate the safety and tolerability of Trappsol<sup>®</sup> Cyclo<sup>™</sup> administered intravenously to NPC patients.

Cyclo Therapeutics Chairman and CEO, N. Scott Fine, said, "We are very grateful to the investigators, the patients, and their families whose coordinated efforts made this study possible. Our team has now started the process to clean the data, with a view toward top-line reports in the March/April timeframe. Our announcement today represents another significant milestone for the company and for all of our stakeholders."

The Phase I study (ClinicalTrials.gov [NCT02939547](https://clinicaltrials.gov/ct2/show/study/NCT02939547)) was conducted at the UCSF Benioff Children's Hospital Oakland with Co-Principal Investigators, Caroline Hastings MD and Benny Liu MD. A total of 12 patients participated in the study, which was double blinded and randomized. Patients received bimonthly intravenous infusions of Trappsol<sup>®</sup> Cyclo<sup>™</sup>, the company's proprietary formulation of hydroxypropyl beta cyclodextrin, at either 1500 mg/kg or 2500 mg/kg by slow infusion.

Niemann-Pick Disease Type C is a rare genetic disease affecting 1 in 100,000 live births globally. NPC affects every cell in the body due to a defect in the NPC protein which is responsible for cholesterol processing in the cell. NPC causes symptoms in the brain, liver, spleen, lung and other organs and often leads to premature death. There are no approved drug therapies for NPC in the United States and only one approved therapy in Europe.

Cyclo Therapeutics Chief Scientific Officer and Senior Vice President for Medical Affairs, Sharon Hrynkow PhD, said, "We look forward to our presentation at WORLDSymposium<sup>™</sup> in Orlando on Feb. 12 with new blinded data from this trial showing effects of our drug in clearing cholesterol from liver tissue ([see LINK](#)). Unblinding of the data in the near term will provide key insights into dose and effect, and this will be important as we select our final dose for the pivotal trial, now in the final design stage."

Data from the current study combined with those of the companion Phase I/II study (ClinicalTrials.gov [NCT02912793](https://clinicaltrials.gov/ct2/show/study/NCT02912793)) will be used to inform the design of the Phase III global

pivotal trial. Cyclo Therapeutics, Inc. will meet with FDA in the first quarter of 2020 and expects to meet with EMA in the second quarter of 2020 to discuss the global pivotal development plan and timelines to initiate a Phase III pivotal trial.

***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<sup>®</sup> Cyclo<sup>™</sup>, 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)) and is planning an early phase trial in Alzheimer's Disease based in part on an expanded access program in late-onset Alzheimer's Disease ([NCT03624842](https://clinicaltrials.gov/ct2/show/study/NCT03624842)). Additional indications for the active ingredient in Trappsol<sup>®</sup> Cyclo<sup>™</sup> are in development. For additional information, visit the company's website: [www.cyclotherapeutics.com](http://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. 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.