

# Cyclo Therapeutics Announces Completion of Enrollment in its Phase I/II Trial to Evaluate Trappsol® Cyclo™ for the Treatment of Niemann-Pick Disease Type C

GAINESVILLE, FL – (Businesswire) – February 20, 2020 – Cyclo Therapeutics, Inc. (OTCQB: CTDH), a clinical-stage biotechnology company that develops cyclodextrin-based products for the treatment of Niemann-Pick Disease Type C and Alzheimer’s Disease, today announced that it has completed patient enrollment in its Phase I/II trial to evaluate the safety, tolerability, and efficacy of Trappsol® Cyclo™ administered intravenously to Niemann-Pick Disease Type C1 (NPC1) patients.

“Today’s ‘Last-Patient-In’ announcement is a major milestone for our company and the NPC community,” said Company Chairman and CEO, N. Scott Fine. “It completes another important step in our development and registration strategies for Trappsol® Cyclo™ to treat NPC, a disease which causes so much suffering for the patients and their families. We are delighted to share this news with our many supporters and all of our stakeholders.”

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 NPC1 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.

“We are deeply grateful to the physicians in UK, Sweden and Israel who have worked diligently to enroll and treat patients in this study, and to our EU Coordinating Investigator, Dr. Reena Sharma, Salford, UK. We continue to be thankful to the participating patients and their caregivers, knowing the sacrifice of time and energy it takes to participate in the study,” said Sharon Hrynkow PhD, Chief Scientific Officer and Senior Vice President for Medical Affairs.

Data from the current study combined with those of the companion Phase I study (ClinicalTrials.gov [NCT02939547](https://clinicaltrials.gov/ct2/show/study/NCT02939547)) 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 the Phase III pivotal trial.

## ***About the Clinical Trial:***

Complete enrollment in the study required 12 patients aged 2 and above. The study is a randomized, double-blind study using Trappsol® Cyclo™ intravenously. Study subjects receive 24 doses of the drug at either 1500 mg/kg, 2000 mg/kg, or 2500 mg/kg and are assessed for adverse events, markers for cholesterol metabolism following drug administration, and symptomatic changes using an NPC severity scoring tool, among other tests. (See Clinical Trials.gov [NCT02912793](https://clinicaltrials.gov/ct2/show/study/NCT02912793) for additional details.)

The company has reported initial data from this study that suggest a favorable safety profile; a temporal link between administration of the drug and clearance of cholesterol from cells; presence of the drug in the cerebrospinal fluid following IV administration; and reduction in a

neuron-specific biomarker, tau, that is associated with neuronal degeneration in NPC patients. The company has also reported encouraging data from the first 2 patients to complete this study suggesting that the drug improves neurologic symptoms of the disease. On unblinding of the data, full interpretation will be possible

Sites involved in this trial are Salford Royal Foundation Trust Hospital, UK, under the direction of Dr. Reena Sharma; Birmingham Women's and Children's Hospital, UK, under the direction of Dr. Julian Raiman; Karolinska Institute, Sweden, Dr. Martin Paucar-Arce; Soroka Medical Center, Israel, Dr. Orna Staretz-Chacham; and, HaEmek Medical Center, Israel, Dr. Ronen Spiegel.

***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](#), [NCT02912793](#) and [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](#)). Additional indications for the active ingredient in Trappsol® Cyclo™ 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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