

Cyclo Therapeutics Inc. Announces Positive Safety Profile of its Drug Trappsol® Cyclo™ in the Treatment of Niemann Pick Disease Type C

GAINESVILLE, FL – (Businesswire) – March 23, 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 (NPC) and Alzheimer’s Disease, today announced that the Safety Review Committee for its Phase I trial in NPC has determined that Trappsol® Cyclo™ has an acceptable safety profile in patients with Niemann-Pick Disease Type C1. The Committee evaluated all safety data in a blinded fashion from the Phase I trial, “A Phase I Study to Evaluate the Single and Multiple-dose Pharmacokinetics of Intravenous Trappsol® Cyclo™ (HP-β-CD) in Patients With Niemann-Pick Disease Type C (NPC-1) and the Effects of Dosing Upon Biomarkers of NPC Disease,” in making its determination. The last patient in the trial received the last dose in February 2020. Patients in this trial are age 18 years and older.

“Today’s announcement represents another major milestone for our company and all stakeholders,” said Company Chairman and CEO, N. Scott Fine. “The Safety Review Committee findings are consistent with our experience gained over many years from our named patient use of Trappsol® Cyclo™. With our first formal clinical trial coming to its conclusion, we are pleased to learn of the Safety Review Committee findings, and we look forward to sharing this information with the U.S. Food and Drug Administration (FDA) and other regulatory bodies in due course.”

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.

“The review of individual and cumulative blinded safety data shows that Trappsol® Cyclo™ is well tolerated with no serious safety signals observed,” said Professor Alan Boyd, MD, Chairman of the Safety Review Committee. “Overall, the intravenous route of administration of the study drug resulted in only minor and expected adverse events.”

The Company is now in the process of unblinding the data from the Phase I trial. Since all patients participating in the Phase I trial received study drug, the findings of the Safety Review Committee are critical to release at this time. Cyclo Therapeutics, Inc. expects to release Top Line results from the Phase I trial, inclusive of unblinded evaluations, in May 2020.

Data from the current study combined with those of the companion Phase I/II companion study underway in Europe and Israel (ClinicalTrials.gov [NCT02912793](#)) will be used to inform the design of the Company's Phase III global pivotal trial. Cyclo Therapeutics, Inc. met with FDA in the February 2020 to discuss the design of the Phase III global pivotal trial and expects to meet with EMA for the same purpose in the second quarter of 2020. The Company previously issued a press release on the positive nature of the meeting with FDA with respect to the Phase III study design, see [HERE](#). "We gratefully acknowledge the many patients and families who participated in our Phase I trial, and we thank our Co-Principal Investigators, Dr. Caroline Hastings and Dr. Benny Liu, and all of our supporting partners, for the hard work that allowed us to reach this point," said Sharon Hrynkow, PhD, Cyclo Therapeutics' Chief Scientific Officer and Senior Vice President for Medical Affairs. "Without the concerted effort on the part of many, we would not be able to share such positive news with the community."

About the Clinical Trial:

Complete enrollment in the study required 12 patients aged 18 and above who had NPC Type C1 disease. The study was a randomized, double-blind study using Trappsol® Cyclo™ intravenously. Study subjects received 7 doses of the drug at either 1500 mg/kg or 2500 mg/kg and were assessed for adverse events, markers for cholesterol metabolism following drug administration, and symptomatic changes using an NPC severity scoring tool, among other tests. (See ClinicalTrials.gov [NCT02939547](#) for additional details.)

The company has previously reported initial data from this study suggesting that the use of Trappsol® Cyclo™ administered intravenously has a favorable safety profile; that there is a temporal link between administration of the drug and clearance of cholesterol from cells; that demonstrate the presence of the drug in the cerebrospinal fluid following IV administration; and that show a reduction in a neuron-specific biomarker, tau, associated with neuronal degeneration in NPC patients. On unblinding of the data, full interpretation will be possible.

One site participated in this trial: UCSF Benioff Children's Hospital Oakland under the direction of Co-Principal Investigators Caroline Hastings MD and Benny Liu MD.

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

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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