

# **Cyclo Therapeutics Announces Positive Feedback from European Medicines Agency on its Pivotal Trial Design for Trappsol® Cyclo™ for Niemann-Pick Disease Type C1**

Company reported in March 2020 that FDA had also provided positive feedback on the pivotal trial design

GAINESVILLE, FL – (Businesswire) – May 18, 2020 – Cyclo Therapeutics, Inc. (OTCQB: CTDH), a clinical-stage biotechnology company that develops cyclodextrin-based products for the treatment of Niemann-Pick Disease Type C1 (NPC1) and Alzheimer’s Disease, today announced that it received positive feedback on its request for Scientific Advice from the European Medicines Agency (EMA). The request focused on the design of the Company’s global pivotal trial for intravenous administration of Trappsol® Cyclo™ in NPC1. Trappsol® Cyclo™ is the company’s proprietary formulation of hydroxypropyl beta cyclodextrin. Cyclo Therapeutics, Inc. was informed on April 17, 2020 that EMA’s Scientific Advice Working Party concluded that a face-to-face meeting would not be required as part of the advice process, and that advice would be provided based only on the briefing package submitted. The Final Advice Letter was received on May 15, 2020. Additionally, within the letter, the EMA’s Committee for Orphan Medicinal Products concluded that the currently proposed clinical development is sufficient to demonstrate significant benefit.

“We were very pleased with the productive and supportive comments made by the EMA on our Phase III pivotal trial design,” said Company Chairman and CEO, N. Scott Fine. “Considering the EMA advice in conjunction with advice received from FDA, we look forward to executing the regulatory processes required to start our global pivotal trial as quickly as possible.”

The design of the pivotal trial is based on data and experience from the Company’s completed Phase I trial in the US and on the ongoing Phase I/II trial in Europe and Israel for which an unblinded Interim Analysis was recently completed.

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.

Cyclo Therapeutics has obtained several designations from regulatory authorities to enhance opportunities for agency discussion and to provide opportunities for exclusivity

in marketing once Trappsol® Cyclo™ is approved. The company has Fast Track Designation for NPC from the FDA, along with Orphan Drug Designation for Trappsol® Cyclo™ in both the US and EU, and Rare Pediatric Disease Designation from the FDA.

***About Fast Track Designation:***

The FDA Fast Track program facilitates the expedited development and review of new drugs or biologics that are intended to: 1) treat serious or life-threatening conditions and 2) demonstrate the potential to address unmet medical needs.

***About Orphan Drug Designations:***

Orphan Drug designation is a special status granted by a regulatory body to a drug to treat a rare disease condition upon request of a Sponsor. For a drug to qualify both the drug and the disease must meet certain criteria. Orphan drug status qualifies the sponsor for certain drug development incentives, and on market approval, provides for market exclusivity of 7 years in the US and up to 12 years in the EU.

***About Rare Pediatric Disease Designation:***

Under this FDA program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

***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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