



## **Cyclo Therapeutics Receives Positive Opinion from the Paediatric Committee of the European Medicines Agency on the Agreement of a Paediatric Investigation Plan for Trappsol<sup>®</sup> Cyclo<sup>™</sup>**

**Gainesville, FL – (Business wire) – March 9, 2021 -- [Cyclo Therapeutics, Inc.](#)** (Nasdaq: CYTH) (“Cyclo Therapeutics” or the “Company”), a clinical stage biotechnology company dedicated to developing life-changing medicines through science and innovation for patients and families suffering from disease, today announced a positive opinion has been adopted from the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) on its agreement with the proposed Paediatric Investigation Plan (PIP) for Trappsol<sup>®</sup> Cyclo<sup>™</sup>, a proprietary formulation of hydroxypropyl beta cyclodextrin, used intravenously (IV), currently in development for the treatment of Niemann-Pick Type C (NPC1).

As part of the regulatory process for the registration of new medicines in Europe, pharmaceutical companies are required to provide a PIP outlining their strategy for investigation of the new medicinal product in the paediatric population. The PIP opinion from PDCO has endorsed the clinical program to evaluate the safety, tolerability and efficacy of Trappsol<sup>®</sup> Cyclo<sup>™</sup> in patients from 3 years to less than 18 years of age with NPC Type C1 as well as the inclusion of a single-arm sub-study with patients from birth to less than 3 years of age with NPC Type C1 to evaluate safety and obtain descriptive data on global severity and improvement in response to Trappsol<sup>®</sup> Cyclo<sup>™</sup>. This adoption of the PIP paves the way for the potential submission of a MAA in Europe for Trappsol<sup>®</sup> Cyclo<sup>™</sup> in the treatment of NPC1 following the completion of the pivotal Phase 3 study which is expected to commence in the second quarter of 2021.

“We are incredibly pleased to receive positive notice from the PDCO and accomplish this important regulatory milestone as we continue to advance Trappsol<sup>®</sup> Cyclo<sup>™</sup> for the treatment of NPC. This approval of the PIP bolsters our confidence in the potential of Trappsol<sup>®</sup> Cyclo<sup>™</sup> to address this area of significant unmet need and provides us with a pathway towards approval for the pediatric population in Europe in parallel with our U.S regulatory strategy. Moving forward, we look to continuing our work with the regulatory authorities to bring Trappsol<sup>®</sup> Cyclo<sup>™</sup> to the European market as expeditiously as possible,” commented Michael Lisjak, Chief Regulatory Officer of Cyclo Therapeutics.

With successful completion of the agreed PIP, Cyclo Therapeutics would be eligible for up to an additional two years of marketing exclusivity in the EU, on top of the ten-year EU market exclusivity after market approval.

Cyclo Therapeutics has received Fast Track Designation for NPC from the U.S. Food and Drug Administration (FDA), along with Orphan Drug Designation for Trappsol® Cyclo™ in both the U.S. and EU, and Rare Pediatric Disease Designation from the FDA. Additionally, Trappsol® Cyclo has the potential for Priority Review Voucher (PRV) in U.S.

NPC 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. As cholesterol accumulates in cells, 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.

### **About the Paediatric Committee (PDCO)**

The Paediatric Committee (PDCO) is the European Medicines Agency's (EMA) scientific committee responsible for activities on medicines for children and to support the development of such medicines in the European Union by providing scientific expertise and defining paediatric needs.

### **About the Paediatric Investigation Plan (PIP)**

A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, to support the authorisation of a medicine for children. All applications for marketing authorisation for new medicines have to include the results of studies as described in an agreed PIP, unless the medicine is exempt because of a deferral or waiver. This requirement also applies when a marketing-authorisation holder wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorised and covered by intellectual property rights.

### **About Cyclo Therapeutics**

Cyclo Therapeutics, Inc. is a clinical-stage biotechnology company dedicated to developing life-changing medicines through science and innovation for patients and families suffering from 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, 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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