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Cyclo Therapeutics Signs Agreement with Worldwide Clinical Trials to Conduct Pivotal Trial in Niemann-Pick Disease Type C

Pivotal trial will study the intravenous administration of the Company's Trappsol® Cyclo™ drug and will focus on systemic and neurologic outcomes

GAINESVILLE, Fla.--(BUSINESS WIRE)-- 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 it has signed an agreement with Worldwide Clinical Trials (Worldwide), a leading Contract Research Organization (CRO), to conduct a pivotal trial that will evaluate the Company's proprietary hydroxypropyl beta cyclodextrin, Trappsol® Cyclo™, administered by intravenous infusion to treat NPC. The Company currently supports a Phase I trial in the United States, which recently completed enrollment (ClinicalTrials.gov NCT02939547); an Extension Protocol for the US study, which includes home-based infusions (NCT03893071); and a Phase I/II trial in Europe and Israel, which is nearing completion of enrollment (NCT02912793). The Company plans to share its design of the pivotal trial in scientific advice meetings with regulators in the US and Europe in first and second quarters of 2020, respectively. Enrollment of the trial is expected to commence shortly thereafter.

Niemann-Pick Disease Type C is a rare and fatal genetic disease affecting 1 in 100,000 live births globally. NPC affects every cell in the body due to the defect in the NPC protein which is responsible for cholesterol processing in the cell. Because of the NPC protein defect, cholesterol accumulates abnormally in every cell in the body, causing symptoms in the brain, liver, spleen, lung and other organs. There are no approved drug therapies for NPC in the United States, and only one, Miglustat/Zavesca, in Europe.

"We are very excited to work with Worldwide, an industry leader in the rare disease space, on this critical clinical program," said N. Scott Fine, Company Chairman and CEO. "Our early phase trials are showing promising results, as we have reported publicly during the past year. Because of this, we are working quickly to present our pivotal trial design to regulators and hope to commence our pivotal trial in the early part of 2020. Worldwide will play a vital role as we build and execute the pivotal study."

Worldwide Clinical Trials' Chief Medical and Scientific Officer, Michael F. Murphy, M.D., Ph.D., said, "The scientific, medical and operational experts at Worldwide are privileged to

be associated with Cyclo Therapeutics' innovative clinical development program seeking to address the significant unmet clinical need represented by NPC. The acumen demonstrated by Cyclo Therapeutics during the process of program and protocol design has been exceptional – greatly facilitating the collaborative research and development effort demanded by orphan diseases with complex and variable phenotypes. Orphan disease research requires a tenacious and uncompromising commitment by all contributors, and commitment and follow through have long been woven into the business fabric at Worldwide."

A manuscript, published on October 21, 2019 in the Orphanet Journal of Rare Diseases, a scientific, peer-reviewed publication (see https://www.ncbi.nlm.nih.gov/pubmed/31639011), documented the most extensive set of case studies to-date on expanded access use of hydroxypropyl beta cyclodextrin to treat patients with NPC. Eighty percent of the patient use data presented in the manuscript derive from Cyclo Therapeutics' Trappsol® Cyclo™ product.

Cyclo Therapeutics' Chief Scientific Officer and Senior Vice President for Medical Affairs, Sharon Hrynkow PhD, the senior author on the manuscript said, "In our review of the data on expanded access use in 12 NPC patients, we found that hydroxypropyl beta cyclodextrin was not only safe when administered intravenously, but also that individual patients showed improvements in disease symptoms, including reduction in the size of the liver, clearance of interstitial lung disease, and neurologic improvements in terms of gait, balance, and ability to focus on tasks. This manuscript's findings, coupled with initial data from our ongoing NPC clinical trials, are promising indications that Trappsol® Cyclo™ when administered intravenously has benefit as a treatment in NPC."

About Cyclo Therapeutics:

Cyclo Therapeutics, Inc. is a clinical-stage biotechnology company that develops cyclodextrin-based products for the treatment of disease. The company's Trappsol® Cyclo™, an orphan drug designated product in the United States and Europe, is in three ongoing formal clinical trials for Niemann-Pick Disease Type C, a rare and fatal genetic disease (Clinical Trials.gov NCT02939547, NCT02912793 and NCT03893071), and in 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.

About Worldwide Clinical Trials:

Worldwide Clinical Trials employs more than 1,700 professionals around the world, with offices in North and South America, Eastern and Western Europe, Russia, and Asia. Founded by physicians committed to advancing medical science, Worldwide is out to change how the world experiences CROs—in the best possible way. From early phase and bioanalytical sciences through late phase, post-approval and real-world evidence, we provide world-class, full-service drug development services.

With infrastructure and talent spanning 60 countries, we execute predictable, successful studies with operational excellence across a range of therapeutic areas, including central nervous system, cardiovascular, metabolic, general medicine, oncology and rare diseases. We never compromise on science or safety. We're never satisfied with the status quo. We're

the Cure for the Common CRO.

For more information, visit http://www.worldwide.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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