

Cyclo Therapeutics Announces Its Publication of the Most Extensive Set of Case Study Reports To-Date on Expanded Access Use of Hydroxypropyl Beta Cyclodextrin in Niemann-Pick Disease Type C

Publication describes favorable safety profile and efficacy in neurologic and other disease features in individual NPC patients following intravenous administration of drug

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 and Alzheimer's Disease, today announced its publication of the most extensive set of case studies to-date on expanded access use of hydroxypropyl beta cyclodextrin to treat patients with Niemann-Pick Disease Type C (NPC). The manuscript was 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>). Eighty percent of the patient use data presented in the manuscript derive from Cyclo Therapeutics' Trappsol® Cyclo™ product.

"This manuscript represents years of work and the efforts of many. We are proud that our company's Founder, C.E. Rick Strattan, worked closely with the Hempel family, Caroline Hastings, MD, and other experts, to develop the expanded access protocol authorized by the FDA in 2009, allowing cyclodextrin use in NPC patients," said N. Scott Fine, Company Chairman and CEO. "We were equally proud to have supplied our cyclodextrin product to US and Brazilian NPC families as other physicians adapted the expanded access Hempel-Hastings protocol for their own patients. Looking back, we are indebted to these pioneers and to the teams of scientists, especially Dr. Benny Liu, whose seminal work in animal models of NPC formed the foundation for expanded use programs with cyclodextrins in NPC patients. This manuscript is a milestone in NPC."

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.

"In our extensive 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," said Dr. Caroline Hastings, lead author on the manuscript. "This included 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. As well, we did not see that adding another route of administration by lumbar puncture, or "intrathecal", added clinical benefit following intravenous administration alone."

Other highlights of the manuscript are that physicians noted that their patients receiving intravenous cyclodextrin showed increased alertness, improved ability to communicate, and enhanced overall well-being.

Dr. Hastings, a pediatric hematologist oncologist at UCSF Benioff Children's Hospital in Oakland, CA, is also Co-Principal Investigator of Cyclo Therapeutics' clinical trial site using Trappsol® Cyclo™ via intravenous administration for NPC. Co-authors on the manuscript, entitled "Expanded Access with Intravenous hydroxypropyl-beta-cyclodextrin to treat children and young adults with Niemann-Pick disease type C1: A case report analysis" are Camilo Vieira, MD (Brazil); Benny Liu, MD (US); Cyrus Bascon, MD (US); Claire Gao, BA (US); Raymond Wang, MD (US); Alicia Casey, MD (US); and Sharon Hrynkow, PhD, Cyclo Therapeutics' Chief Scientific Officer and Senior Vice President for Medical Affairs.

Dr. Vieira was the first physician in Brazil to use cyclodextrins (also Cyclo Therapeutics' Trappsol® Cyclo™ product) in NPC patients, via an expanded access program. Dr. Liu made the seminal discovery in the mouse model of NPC that cyclodextrins could clear cholesterol from the liver and brain, delay onset of symptoms, and prolong life. Dr. Liu is Co-Principal Investigator along with Dr. Hastings at the UCSF Benioff clinical site of Cyclo Therapeutics.

Cyclo Therapeutics' Dr. Sharon Hrynkow said, "The work described in this manuscript formed the basis of Cyclo Therapeutics' initial discussions with regulators in the United States, Europe and Israel on the design of our formal clinical trials, now underway. We are deeply grateful to Dr. Hastings and all of the physicians as well as the patients and families who shared their data as we developed these case studies. We look forward to continuing to work in collaboration with families and physicians as Cyclo Therapeutics advances Trappsol® Cyclo™ on the pathway to market approval for the benefit of NPC patients globally."

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](#).

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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