Dedicated to developing life-changing medicines for patients and families living with challenging diseases

Cyclo Therapeutics Announces Positive Outcome from Type C Meeting with the U.S. FDA Discussing Trappsol® Cyclo™ Clinical Program for the Treatment of Niemann-Pick Disease Type C1

Dec 18, 2023

Ongoing pivotal Phase 3 study ("TransportNPC™") evaluating Trappsol® Cyclo™ for the treatment of Niemann-Pick Disease Type C1 (NPC1)

FDA feedback provides direction for the comparative interim analysis

With aligned strategy, interim analysis data readout is anticipated for Q1 2025

FDA and Cyclo Therapeutics to meet in the first half of 2024 for pre-NDA meeting

GAINESVILLE, Fla. – Cyclo Therapeutics, Inc. (Nasdaq: CYTH) ("Cyclo Therapeutics" or the "Company"), a clinical stage biotechnology company focused on developing transformative therapies for rare and neurological diseases with limited treatment options, today announced a positive outcome from its recent Type C meeting with the U.S. Food and Drug Administration (FDA), held on Thursday, December 14, 2023 to discuss a full data review of the Company's Trappsol® Cyclo™ clinical development program to date and proposed strategies for the statistical analysis for the ongoing pivotal Phase 3 study, TransportNPC™, in order to enable a patient population lacking available therapies to have earlier access to a potentially efficacious product with an acceptable safety profile.

N. Scott Fine, Chief Executive Officer of Cyclo Therapeutics, added, "We view our Type C meeting with FDA as a catalytic and transformational milestone for our ongoing pivotal Phase 3 NPC program. By working closely and in alignment with FDA, I am confident our leadership team has positioned Cyclo Therapeutics for rapid continued advancement and has established the appropriate path to generate the data required to successfully drive our TransportNPC study across the finish line."

In summary, the Company's clinical data (NCT02939547, NCT03893071 and NCT02912793) provided suggestive evidence, based on individual patient data, of potential therapeutic benefit which may be observed at or before 48 weeks of treatment. Given the significant unmet need within this Orphan Disease and the lack of available effective pharmacotherapy, and that aggregate clinical and nonclinical data argue for a potentially clinically relevant therapeutic effect, the FDA aligned with Cyclo Therapeutics' proposal to continue with the agreed upon Phase 3 96-week study, but change the timing of the planned comparative interim analysis to execute data readout in Q1 2025.

"The FDA feedback we received allows us to proceed as planned to meet our full enrollment goals as well as our regulatory and corporate objectives to bring a potential treatment option to this underserved patient community as quickly as possible. We look forward to implementing these changes and to our future pre-NDA meeting with the FDA as we continue to execute across all aspects of our drug development program," stated Michael Lisjak, Chief Regulatory Officer and Senior Vice President of Business Development for Cyclo Therapeutics.

The Company's ongoing TransportNPC™ study is a randomized, double-blind, placebo-controlled, parallel group, multicenter study designed to evaluate the safety, tolerability, and efficacy of 2,000 mg/kg doses of Trappsol® Cyclo™ administered intravenously and standard of care (SOC), compared to placebo administered intravenously and SOC alone, in patients with NPC1, a rare, genetic disease causing cholesterol accumulation in cells, leading to dysfunction of the liver, lung, spleen and brain and premature death. The Phase 3 study intends to enroll at least 93 pediatric (age 3 years and older) and adult patients with NPC1 and is now active in 12 countries.

For more information about the Company's TransportNPC™ pivotal Phase 3 study, visit www.ClinicalTrials.gov and reference identifier NCT04860960.

Cyclo Therapeutics received Orphan Drug Designation for Trappsol® Cyclo™ to treat NPC1 in both the U.S. and EU and Fast Track and Rare Pediatric Disease Designations in the U.S. The Rare Pediatric Disease Designation is one of the chief requirements for sponsors to receive a Priority Review Voucher in the U.S. upon marketing authorization.

About Cyclo Therapeutics

Cyclo Therapeutics, Inc. is a clinical stage biotechnology company focused on developing transformative therapies for rare and neurological diseases with limited treatment options. The Company's Trappsol® Cyclo™, an orphan drug designated product in the United States and Europe, is the subject of four formal clinical trials for Niemann-Pick Disease Type C, a rare and fatal genetic disease, (www.ClinicalTrials.gov NCT02939547, NCT02912793, NCT03893071 and NCT04860960). The Company is conducting a Phase 2b clinical trial using Trappsol® Cyclo™ intravenously in early Alzheimer's disease (NCT05607615) based on encouraging data from an Expanded Access program for 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, 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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