

Cyclo Therapeutics Announces Formation of Global Steering Committee Comprised of Leading Experts to Advise on the Global Phase 3 Clinical Development Program for Trappsol® Cyclo™ in Niemann-Pick Disease Type C

Feb 3, 2022

- Company's pivotal Phase 3 study, TransportNPC™, ongoing with global site activation and patient enrollment underway*
- Data seen to-date provide support for the capacity of Trappsol® Cyclo™ to stabilize disease progression with home-based intravenous infusions in NPC*
- Trappsol® Cyclo™ demonstrates acceptable safety profile, administered intravenously, for more than 2 years*

GAINESVILLE, Fla. –

[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 living with diseases, today announced the formation of a Global Steering Committee (GSC) to guide the pivotal Phase 3 global clinical development program of Trappsol® Cyclo™ for the treatment of Niemann-Pick Disease Type C (NPC). As the Global Principal Investigator for the TransportNPC™ study, Caroline Hastings, MD serves as the senior scientific and clinical expert for the trial and will also chair the GSC.

"Dr. Caroline Hastings, global principal investigator for TransportNPC™ and chair of the GSC, has been instrumental in assembling this high caliber Global Steering Committee with representation of renowned Key Opinion Leaders and clinical experts in NPC. It is another testimony of our commitment to serve the NPC community and deliver on the unmet medical needs. I feel humbled and privileged to be working with this outstanding group of professionals who are committed to advance science and clinical trials that can bring hope and treatment benefits to so many patients and their families," commented Lise Kjems, MD, PhD, Chief Medical Officer of Cyclo Therapeutics.

The Company's ongoing pivotal Phase 3 study, TransportNPC™, 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. The Phase 3 study intends to enroll at least 93 pediatric (age 3 years and older) and adult patients with NPC1 in at least 23 study centers in 9 countries. Eligible patients will be randomized 2:1 to receive either

Trappsol® Cyclo™ or a placebo. Randomization will not be constrained based on patient age, nor will patient enrollment be gated by patient age. The study duration is 96 weeks and includes an interim analysis at 48 weeks.

Dr. Hastings, Global Principal Investigator for the TransportNPC™ trial and member of Cyclo Therapeutics' Scientific Advisory Board added, "I am very grateful by the overwhelmingly positive responses as I reached out to fellow scientists and physicians to invite them to join the Global Steering Committee. I am honored to be working alongside these wonderful colleagues with outstanding knowledge and expertise and who represent the excellent investigators taking part in the TransportNPC™ trial. Together, we have a very unique opportunity to further refine the scientific strategy for Trappsol® Cyclo™ and help drive this important program toward potential approval."

"NPC is a devastating neurodegenerative disease that needs more effective therapies. Given the clinical course and progressive nature of this disease, novel therapeutic strategies with the potential for disease modifying effects are necessary. The TransportNPC™ trial is unique as it is designed to demonstrate the long-term clinical benefits and potential for disease modification," commented Professor Roberto Giugliani, MD, PhD.

"I have been caring for patients with NPC for more than 25 years. These patients urgently need better treatment options that will better halt the cruel, neurodegenerative course that this disease takes. In this study with cyclodextrin intravenously, I see an opportunity to improve the therapeutic offer," added Dr. Eugen Mengel.

The members of the TransportNPC™ Global Steering Committee are:

- **Professor Caroline Hastings, MD** is the Chair of the Phase 3 Trappsol® Cyclo™ Program Steering Committee, and the Global Principal Investigator for the Company's ongoing TransportNPC™ study evaluating Trappsol® Cyclo™ for the treatment of NPC. Dr. Hastings currently serves as the Pediatric hematologist oncologist, Director of Neuro-oncology, and Professor of Pediatrics at UCSF Benioff Children's Hospital Oakland and is an advisor to U.S. and Australian NPC Advocacy organizations and to physicians globally on NPC. She has been practicing in the field of Pediatric Hematology Oncology since 1992 and has served as the director of the fellowship program at the Children's Hospital & Research Center Oakland since 1996. She has devoted herself to her patients and to fostering education in this specialty. Her academic interests include tumors of the brain and spinal cord, relapsed acute lymphoblastic leukemia, and lysosomal storage diseases including Niemann Pick Type C disease.
- **Professor Mark Walterfang MBBS Hons, PhD, FRANZCP** is a consultant psychiatrist at the Adult Mental Health Rehabilitation Unit at Sunshine Hospital and a consultant neuropsychiatrist at the Royal Melbourne Hospital Neuropsychiatry Unit. Dr. Walterfang has been involved in the development in a number of clinical tools for use in psychiatric patients in the areas of cognition and behavioral observation, and their validation in a variety of medical, neurological and psychiatric settings since 2000. His continuing research involves the neuroimaging and neuropsychiatric investigation of neurometabolic disorders including phenylketonuria and NPC, and in shape analysis of cortical and

subcortical regions in neurodegenerative disorders. He has published over 170 Medline-indexed scientific papers, and recently contributed a new chapter on the Neuropsychiatry of Neurometabolic and Neuroendocrine Disorders to the world's most respected reference text in psychiatry, Kaplan and Sadock's *"Comprehensive Textbook of Psychiatry"*.

- **Professor Maurizio Scarpa, MD, PhD, Paediatrician** is the Founder and President of the Brains for Brain Foundation, a Pan-European task force on brain and neurodegenerative diseases. He is Professor of Paediatrics at the Department of Women's and Children's Health, University of Padova, Italy and the Director, Coordinating Center for Rare Diseases, Udine University Hospital, Udine, Italy. Professor Scarpa has extensive expertise as a basic scientist in genetics and biotechnology, and as a clinician in the diagnosis and treatment of paediatric rare disorders, neurometabolic diseases in particular. He is especially interested in developing innovative health approaches for the diagnosis and the treatment of metabolic inherited diseases. Professor Scarpa has published about 140 international peer reviewed clinical and scientific papers, book chapters and reviews. Professor Scarpa is the Coordinator of the European Reference Network for Inherited Metabolic Diseases (MetabERN).
- **Dr. Eugen Mengel** is the principal investigator, and the founder and CEO of the SphinCS GmbH & SphinCS Lyso non-profit UG. Dr. Mengel has dedicated himself to research in the field of lysosomal diseases and has already significantly contributed to the advancement of this field of study. He is the co-author of 129 peer-reviewed publications with focuses on Gaucher disease, NPC and Pompe disease and is an active member of the Society for the Study of Inborn Errors of Metabolism (SSIEM), European Study Group on Lysosomal Diseases (ESGLD) and European Working Group on Gaucher Disease (EWGGD).
- **Orna Staretz-Chacham, MD** is a neonatologist, metabolic specialist and senior lecturer at the Faculty of Health Sciences, Ben-Gurion University of the Negev, Israel. For years Dr. Staretz-Chacham has worked in the field of inherited metabolic disorders with a focus on neonate and leads the Center for Rare Disease in Soroka Medical Center in since 2021. Among the many critical contributions stemming from her research is characterization of the pulmonary pathologies in Niemann-Pick C1 and most recently in lysosomal patients with COVID-19. Dr. Staretz-Chacham is regarded as one of most prominent clinical experts in the clinical pulmonary presentations in patients with NPC. Dr. Staretz-Chacham has been involved in clinical trials with Trappsol® Cyclo™ from early on and has significant experience in treating patients with NPC. Her commitment to clinical research is evidenced by the more than 40 published articles in peer-reviewed journals in the field of inherited metabolic diseases.
- **Loren DM Pena, MD, PhD** is a Clinical Geneticist, Division of Human Genetics, and Associate Professor, Department of Pediatrics at Cincinnati Children's Hospital Medical Center. As a clinical geneticist, Dr. Pena works with children who have rare and orphan diseases, lysosomal storage disorders and metabolic disorders, and also works in neurogenetics and gene discovery. Dr. Pena has made noteworthy contributions to the field through her research as a lead faculty member for clinical trials in genetics and leading several clinical trials for rare conditions.

- **Professor Roberto Giugliani, MD, PhD** is a medical geneticist with specialization in inherited metabolic diseases. He is Full Professor at Department of Genetics of the Federal University of Rio Grande do Sul and active member of the Medical Genetics Service of HCPA (University hospital), Brazil. He is also Coordinator of the Brazilian Institute of Population Medical Genetics, Editor-in-Chief of the Journal of Inborn Errors of Metabolism and Screening, Chairman of the Latin American School of Human and Medical Genetics, Member of Brazilian Academy of Sciences, Head of Rare Diseases of DASA/GeneOne, and co-founder of House of Rares. He is the past President of the Latin American Society of Inborn Errors of Metabolism and Newborn Screening, the Latin American Network of Human Genetics (RELAGH), and the Brazilian Society of Medical Genetics. Professor Giugliani's main interests are concentrated in screening, diagnosis, and treatment of IEM, having supervised the training of over 100 MSc/PhDs, and authoring of over 500 scientific papers.
- **Professor Dr. Fatih Ezgü** is a Professor of Pediatrics and Head of the Department of Pediatric Genetics at Gazi University, Faculty of Medicine, Department of Pediatrics in Ankara, Turkey. He is also on faculty at the Department of Pediatric Metabolic Disorders at the same institution. Professor Ezgü has worked in the field of inborn metabolic and genetic diseases for 21 years and has published over 100 articles in peer-reviewed journals. His primary interests are lysosomal disorders, mitochondrial disorders, clinical dysmorphism and genetic and metabolic bone disease and novel treatments for genetic disorders. Dr. Ezgü is the vice chairman of the Phase 1 Clinical Trials Center for Genetic Diseases at Gazi University.

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 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 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 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.

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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