ACTIVE Clinical Trials Niemann-Pick Disease Type C

Sponsor	azafaros	cyclo therapeutics
Study Title	A Study to Evaluate the Safety and Efficacy of Oral Nizubaglustat (AZ-3102) in Late-infantile and Juvenile Forms of Niemann-Pick Type C Disease (NAVIGATE)	Phase 3 Study to Evaluate Intravenous Trappsol® Cyclo™ in Pediatric and Adult Patients with Niemann-Pick Disease Type C1 (TransportNPC)
Condition Studied	Niemann-Pick Type C Disease (NPC)	Niemann-Pick Disease Type C1 (NPC)
Patient Population	NPC patients with late infantile and juvenile onset disease ages 4 years and older	NPC patients ages 3 years and older with a sub-study enrolling patients under the age of 3
Currently Enrolling Patients?	Actively recruiting globally. The study is open in Oakland, CA with other sites expected to open for recruitment in the coming months	Active, but no longer recruiting participants
U.S. Trial Sites	5 sites: Open: Oakland, CA. Pending: Fairfax, VA, Rochester, MN, Dallas TX, Minneapolis MN	7 sites: Oakland, CA; Jacksonville, FL; Atlanta, GA; Cincinnati, OH; Pittsburgh, PA; Salt Lake City, UT; Fairfax, VA
Estimated Length of Trial	18 months with a planned option for an open label extension	96 weeks followed by an option for an open-label extension of up to another 96 weeks
Placebo-Controlled?	Yes (randomized, double-blind, placebo-controlled, 2:1 ratio). This means, on average, 2 out of every 3 patients who enroll will receive the study drug while 1 out of every 3 will receive placebo.	Yes (randomized, double-blind, placebo-controlled, 2:1 ratio) This means, on average, 2 out of every 3 patients who enroll will receive the study drug while 1 out of every 3 will receive placebo. This means, on average, 2 out of every 3 patients who enroll will receive the study drug while 1 out of every 3 will receive placebo.
Route of Administration	Oral (by mouth) dispersible tablet	Intravenous (slow infusion every two weeks)
Allowed Other Medications	Certain stable, approved medications may be allowed; requires review by study team. Patients are not allowed to take concurrent/background miglustat. Patients who have been satisfactorily treated with miglustat for over 12 months are not eligible to participate. Patients who have not been satisfactorily treated with miglustat (defined by a less than effective dose or inability to tolerate it) are eligible to participate but they have to discontinue miglustat at least 1 month prior to starting the study.	Patients might be able to take concurrent/background miglustat (if stable for 3 months); investigational drugs or leucine not allowed within 3 months prior to study. Treatment with any form of leucine, whether as an investigational drug or other formulation is not allowed.
Contact for More Information	Azafaros contacts: info@azafaros.com www.navigate.azafaros.com See clinicaltrials.gov study number **NCT07082725** at https://clinicaltrials.gov/study/NCT07082725	Cyclo Therapeutics contacts: Study Director: Karen Mullen, MD (Cyclo Therapeutics, Inc.): info@cyclotherapeutics.com See study number **NCT04860960** on clinicaltrials.gov at https://clinicaltrials.gov./study/NCT04860960