



First U.S. Study Site Open for Azafaros' Phase 3 Clinical Trial NAVIGATE

The National Niemann-Pick Disease Foundation (NNPDF) is hereby informing the community on the opening of the first U.S. study site for the Phase 3 NAVIGATE clinical trial, sponsored by Azafaros.

NAVIGATE is a double-blind placebo-controlled study, investigating the safety and efficacy of oral nizubaglustat, an investigational product designed for the treatment of late-infantile/juvenile forms of Niemann-Pick type C disease and GM1 and GM2 (Tay Sachs and Sandhoff disease).

The opening of the first study site in the U.S. marks another significant milestone in advancing treatment research for individuals and families affected by Niemann-Pick disease.

The inaugural U.S. site is located at Children's Hospital and Research Center at **Oakland, California**, under the leadership of Dr. Hastings, who will serve as the site's Principal Investigator.

Additional U.S. sites for the Navigate trial are expected to open in the upcoming months:

Fairfax, Virginia

Lysosomal Rare Disorders Research and Treatment Center, Dr. Goker-Alpan

Dallas, Texas

Children's Medical Center of Dallas, Dr. Ramirez

Rochester, Minnesota

Mayo Clinic, Dr. Dhamija

Minneapolis, Minnesota

University of Minnesota Medical School, Dr. Jarnes

If you are interested to participate you can reach out to your care team and ask for a referral to one of the study sites.

For more information about the study visit navigate.azafaros.com

For more information on the study and on-site participation, please visit clinicaltrials.gov/study/NCT07082725