



NAVIGATE STUDY for NPC Information

A double-blind placebo-controlled study, with a 2:1 randomization which means 2 of 3 patients will receive the Investigational product and 1 patient will receive placebo.

This study is aimed to investigate the safety and efficacy of oral nizubaglustat, an investigational product designed for the treatment of late-infantile/juvenile forms of GM1 and GM2 (Tay Sachs and Sandhoff disease) and Niemann-Pick type C disease

The aim is to recruit about 72 NPC participants

Participants will be asked to remain in the study for 78 weeks (including preparation time) and agree to visit the clinic site for assessments on approximately 8 occasions. Following the scheduled time in the study, participants may be offered the option to continue in the study as part of an open-label treatment period, meaning they will be offered the opportunity to take the investigational product (nizubaglustat) without any placebo use and continue attending regular visits.

To be eligible to participate in the study patients need to:

- Be 4 years old or older.
 - Children under the age of 18 need a parent or guardian to sign the ICF and agree to take part in the study.
- Have an NPC diagnosis (genetically confirmed).
- Have a late-infantile or juvenile form of the disease:
 - This means that the first neurological signs of the disease appeared between 2 and 15 years of age.

There are other criteria, more related to the clinical progression of the disease, that must be evaluated by the clinician.

Compound

Nizubaglustat is a brain-penetrant small molecule with a unique dual mode of action that will be administered once a day via an oral dispersible tablet. Due to its mode of action, it cannot be combined with compounds with similar therapeutic approach like miglustat. If you currently use miglustat or other (co) medication, and are interested in participating in this study, please contact your physician to discuss the best option for you.

For more information regarding study, timing and study centers, please reach out to your health care team or visit clinicaltrials.gov/study/NCT07082725 or navigate.azafaros.com