

PHASE 3 MASTER PROTOCOL TO ASSESS EFFICACY AND SAFETY OF NIZUBAGLUSTAT IN LATE-INFANTILE AND JUVENILE FORMS OF **GMx** GANGLIOSIDOSES AND NIEMANN-PICK TYPE C

NPC, GM1 and GM2 diseases are neurodegenerative lysosomal diseases. Nizubaglustat is an oral, brain-penetrant, selective inhibitor of glucosylceramide synthase (GCS) and non-lysosomal neutral glucosylceramidase (NLGase). The competitive inhibition of those enzymes, has shown clinical improvement in animal models and encouraging results in a phase 2 study.

Study design

Two subprotocols, evaluated independently but conducted operationally in an identical manner.



NPC: 72 participants (not using miglustat)

GMx: 75 participants

Study countries

Currently, 15 countries and > 30 sites



Other countries are being explored

Key inclusion criteria

- Aged 4 years and older
- Weight \geq 10 Kg
- Confirmed diagnosis (genetic/enzymatic)
- Late infantile or juvenile onset of diseases:
 - **NPC** neurological onset: 2-15 years old.
 - **GMx** neurological onset: 1-10 years old.
- Total SARA score \geq 3 and \leq 30 points

Miglustat is not allowed during the study.

Other approved or alternative (non-investigational) drugs are allowed. Doses must be stable before baseline.