

A Study to Evaluate the Safety and Efficacy of Oral Nizubaglustat (AZ-3102) in Late-infantile and Juvenile Forms of GM1 Gangliosidosis or GM2 Gangliosidosis

NCT07082543

Status	RECRUITING
Phase	Phase 3
Sponsor	Azafaros A.G.
Enrollment	75 participants

Key Eligibility Criteria

Inclusion (6)

- Confirmed GM1 gangliosidosis or Tay-Sachs, Sandhoff, or GM2AB variant
 - Male and female participants aged 4 years and older at the time of informed consent
 - Onset of neurological symptoms from 1 to 10 years
 - Disability level at Baseline: Ataxic disturbances with a total SARA score of e3 and d30 at Baseline
 - Females of childbearing potential who are sexually active willing to follow the contraceptive guidance
- ... and 1 more (see full listing online)

Exclusion (9)

- A history of medical conditions other than GM1 or GM2 gangliosidosis that, in the opinion of the Principal Investigator, would confound scientific rigor or the interpretation of results
 - Body weight of <10 kg
 - The presence of another neurologic disease
 - The presence of moderate or severe hepatic impairment
 - The presence of moderate or severe renal impairment
- ... and 4 more (see full listing online)

Locations (36 total)

UCSF Children's Hospital and Research Center at Oakland, Oakland, California, United States
University of Minnesota Medical School, Minneapolis, Minnesota, United States
Mayo Clinic Children's Center - PIN, Rochester, Minnesota, United States
... and 33 more locations