

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 (NPC)

NCT07082725

Status	RECRUITING
Phase	Phase 3
Sponsor	Azafaros A.G.
Enrollment	72 participants

Key Eligibility Criteria

Inclusion (8)

- Signed informed consent
 - Confirmed diagnosis of NPC disease
 - Patient is unable or unwilling to take miglustat, or is, in the opinion of the investigator, unsatisfactorily treated with miglustat
 - Male and female participants aged 4 years and older at the time of informed consent
 - Onset of neurological symptoms from 2 to 15 years
- ... and 3 more (see full listing online)

Exclusion (10)

- A history of medical conditions other than NPC disease 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 5 more (see full listing online)

Locations (33 total)

UCSF Children's Hospital and Research Center at Oakland, Oakland, California, United States
University of Minnesota Medical School, Minneapolis, Minnesota, United States
Children's Medical Center Dallas, Dallas, Texas, United States
... and 30 more locations