

A Study to Evaluate the Safety and Efficacy of Nizubaglustat (AZ-3102) in Patients With GM2 Gangliosidosis or Niemann-Pick Type C Disease

NCT07399704

| | |
|-------------------|-----------------|
| Status | RECRUITING |
| Phase | Phase 2 |
| Sponsor | Azafaros A.G. |
| Enrollment | 21 participants |

Key Eligibility Criteria

Inclusion (10)

- Cohort 1 (NPC and GM2 patients):
- Have been randomized into Phase 2 Study AZA-001-5A2-01.
- OR
- Cohort 2 (NPC patients):
- Be male or female aged e12 years
- ... and 5 more (see full listing online)

Exclusion (4)

- A positive serum pregnancy test (only tested for women of childbearing potential).
- Female planning to breastfeed during the study.
- Any medical event/condition that prevents participation in the study based on the judgment of the Principal Investigator.
- Participation in another interventional or non-interventional study or early access program.

Locations (3 total)

Associação Hospitalar de Prot à Infância Dr. Raul Carneiro, Água Verde, Curitiba, Brazil
Hospital de Clínicas de Porto Alegre, Porto Alegre, Rio Grande do Sul, Brazil
Instituto Nacional de Saúde da Mulher, da Criança e do Adolescente Fernandes Figueira, Rio de Janeiro, Brazil