A Study to Learn About the Safety and Effects of the Study Drug PRX-102 in Children and Adolescents With Fabry Disease

NCT06328608

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	Chiesi Farmaceutici S.p.A.
Enrollment	22 participants

Key Eligibility Criteria

Inclusion (6)

- Participants with the provision of informed consent from their legal guardians
- Boys and girls aged 2 to 7 years (Cohort A), 8 to 12 years (Cohort B), or 13 to \<18 years (Cohort C).
- Confirmed diagnosis of Fabry disease
- Presence of at least one of the following characteristic features of Fabry disease: neuropathic pain, cornea verticillata, and/or clustered angiokeratoma.
- History of Fabry pain: Fabry crises OR chronic pain.

... and 1 more (see full listing online)

Exclusion (18)

- All Subjects:
- Estimated glomerular filtration rate (eGFR) at screening \< 80 mL/min/1.73 m2.
- History of type I hypersensitivity reactions (anaphylactic or anaphylactoid life-threatening reaction) to other ERT treatment for Fabry disease or any component of the study drug.
- Initiation of treatment with an angiotensin-converting enzyme inhibitor (ACEi) or angiotensin II receptor blocker (ARB) or a dose change in ongoing treatment in the four weeks before screening.
- Urine protein to creatinine ratio (UPCR) \> 0.5 g/g (0.5 mg/mg or 500 mg/g) if not treated with an ACE inhibitor or ARB.

... and 13 more (see full listing online)

Locations (12 total)

Phoenix Children's, Phoenix, Arizona, United States
Emory Genetics Clinical Trials Center, Atlanta, Georgia, United States
University of Iowa, Iowa City, Iowa, United States
... and 9 more locations