Study to Evaluate the Safety, PK, PD, and Efficacy of PRX-102 in Japanese Patients With Fabry Disease

NCT05710692

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	Chiesi Farmaceutici S.p.A.
Enrollment	16 participants

Key Eligibility Criteria

Inclusion (28)

- Must have been born in Japan and have their biological parents and all 4 grandparents of Japanese descent
- A documented diagnosis of Fabry disease, as determined by the following:
- Males: Plasma and/or leukocyte alpha-galactosidase-A activity (by activity assay) that is d 5% of mean normal laboratory levels or, if the enzymatic activity is above the 5% limit but still under the normal level, a confirmed disease-causing mutation of the GLA gene
- Females: Historical genetic test results consistent with Fabry mutations or, in the case of novel mutations, a first-degree male relative with Fabry disease
- All subjects: At least one of the following characteristic features of Fabry disease: neuropathic pain, cornea verticillata, and/or clustered angiokeratoma

... and 23 more (see full listing online)

Exclusion (15)

- Administration of ERT for Fabry disease within 14 days before baseline, substrate reduction therapy for Fabry disease within 3 days before baseline, or chaperone therapy for Fabry disease within 3 days before baseline
- History of type I hypersensitivity reactions (anaphylactic or anaphylactoid life-threatening reaction) to other ERT treatment for Fabry disease or to any component of the study drug
- Cohort A only: eGFR value of \>90 to d120 mL/min/1.73 m2 at screening and a historical eGFR value \>120 mL/min/1.73 m2 in the 9 to 24 months before screening, indicating absence of renal impairment. eGFR to be calculated using the JPN-CKD-EPI creatinine equation (2009).
- 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
- Initiation of treatment, or a change in dose to ongoing treatment, with an angiotensin-converting-enzyme inhibitor (ACEI) or angiotensin II receptor blocker (ARB) in the 4 weeks prior to screening.

... and 10 more (see full listing online)

Locations (10 total)

Fukuoka University Chikushi Hospital, Chikushino-shi, Fukuoka, Japan
Tohoku University Hospital, Sendai, Miyagi, Japan
University of the Ryukyu Hospital, Nishihara, Okinawa, Japan
... and 7 more locations