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

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 2 years to 17 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- 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.
- Clinical condition that, in the investigator's opinion, requires ERT treatment.

Exclusion Criteria:

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.
- Currently taking another investigational drug for any condition.
- History of acute kidney injury in the 12 months before screening, including specific kidney diseases (e.g., acute interstitial nephritis, acute glomerular and vasculitic renal diseases); non-specific conditions (e.g., ischaemia, toxic injury); or extrarenal pathology (e.g., prerenal azotaemia, acute postrenal obstructive nephropathy).
- History of renal dialysis or kidney transplantation.
- History of or current malignancy requiring treatment.
- Severe cardiomyopathy or significant unstable cardiac disease within six months before screening.
- A positive test for Severe Acute Respiratory Syndrome-Coronavirus 2 (SARS-CoV-2) within three months before screening.
- Presence of any medical, emotional, behavioural, or psychological condition that, in the Investigator's judgement, could interfere with the subject's compliance with the requirements of the study.

Additional Exclusion Criteria for Subjects Enrolled in Stage I:

- Female
- Non-classic form of Fabry disease
- Receipt of treatment for Fabry disease within six months before screening
- Positive for anti-PRX-102 antibodies at screening

Additional Exclusion Criteria for Subjects in Stage II (i.e., non-treatment naïve males or females):

- Unwilling to discontinue current ERT treatment for Fabry disease before baseline.
- Females: Pregnant or lactating, or of childbearing potential with a fertile male partner and unwilling to use a highly reliable method of contraception from the informed consent signature until 30 days after the last infusion.

Conditions & Interventions

Interventions:
DRUG: PRX-102 1 mg/kg every two weeks
Conditions:
Fabry Disease

More Information

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