

Rocket Study: A Study to Characterize Biomarkers and Disease Progression in Participants With Pelizaeus-Merzbacher Disease

NCT05659901

Status RECRUITING
Sponsor Ionis Pharmaceuticals, Inc.
Enrollment 32 participants

Key Eligibility Criteria

Inclusion (4)

- Participant has a parent or caregiver capable of providing informed consent (signed and dated) and able to attend all scheduled study visits and provide feedback regarding the participant's symptoms and performance as described in the protocol and be able to comply with all study requirements
- Participant has a diagnosis of Pelizaeus-Merzbacher Disease with genetic confirmation of PLP1 duplication
- Male, 6 months-17 years old, inclusive, at the time of informed consent and phenotype consistent with classic PMD
- No contraindications for lumbar punctures (LPs), blood draws, neuroimaging, sedation (if necessary) or other study procedures

Exclusion (3)

- Clinically significant abnormalities in medical history or physical examination
- > 2 copies of the PLP1 gene
- Have any other conditions, which, in the opinion of the investigator would make the participant unsuitable for inclusion, or could interfere with the participant taking part in or completing the study

Locations (8 total)

Ionis Investigative Site, Atlanta, Georgia, United States
Ionis Investigative Site, Philadelphia, Pennsylvania, United States
Ionis Investigative Site, Clermont-Ferrand, France
... and 5 more locations