

Orbit Study: A Study to Evaluate the Safety, Pharmacokinetics, and Pharmacodynamics of Intrathecally Administered ION356 in Participants With Pelizaeus Merzbacher Disease (PMD)

NCT06150716

Status	RECRUITING
Phase	Phase 1
Sponsor	Ionis Pharmaceuticals, Inc.
Enrollment	24 participants

Key Eligibility Criteria

Inclusion (5)

- Participant's parent or legally accepted representative can provide informed consent, attend all scheduled study visits, provide feedback regarding the participant's symptoms, and can comply with all study requirements.
- Diagnosis of PMD with genetic confirmation of PLP1 gene duplication.
- Clinical phenotype and brain imaging consistent with a diagnosis of PMD.
- Male between the ages of 2 and 17 years, inclusive, at the time of informed consent.
- Able and willing to meet all study requirements (in the opinion of the Investigator), including travel to Study Center, procedures, measurements, and visits.

Exclusion (10)

- Clinically significant abnormalities in medical history, laboratory tests or physical examination.
- Unwillingness to comply with study procedures, including follow-up, as specified by this protocol, or unwillingness to cooperate fully with the Investigator.
- Any contraindication or unwillingness to undergo magnetic resonance imaging (MRI).
- Treatment with another investigational drug, biological agent, or device within 1 month of Screening, or 5 half-lives of the investigational agent, whichever is longer.
- Previous treatment with an oligonucleotide (including small interfering ribonucleic acid) within 4 months of Screening if a single dose was received, or within 12 months of Screening if multiple doses were received. This exclusion does not apply to vaccines (both messenger ribonucleic acid [mRNA] and viral vector vaccines).

... and 5 more (see full listing online)

Locations (7 total)

Ionis Investigative Site, Atlanta, Georgia, United States
Ionis Investigative Site, Philadelphia, Pennsylvania, United States
Ionis Investigative Site, Salt Lake City, Utah, United States
... and 4 more locations

<https://clinicaltrials.gov/study/NCT06150716>

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