

A First-in-Human, Open-Label, Dose-Escalation Study to Evaluate the Safety and Tolerability of Gene Therapy With TTX-381 for the Ocular Manifestations Associated With Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease

NCT05791864

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Tern Therapeutics, LLC
Enrollment	16 participants

Key Eligibility Criteria

Inclusion (16)

- A participant is eligible to be included in the study only if all of the following criteria apply:
- Has biallelic CLN2 mutations.
- Has decreased leukocyte TPP1 activity.
- Has clinical signs or symptoms consistent with CLN2 disease (eg, developmental delay, developmental decline, seizure, vision loss, or other signs/symptoms) OR an older sibling with confirmed CLN2 diagnosis.
- Is currently receiving biweekly ICV ERT treatment with cerliponase alfa.

... and 11 more (see full listing online)

Exclusion (21)

- Participants are excluded from the study if any of the following criteria apply:
- Any ocular or systemic condition that, in the opinion of the investigator, would prevent administration and evaluation of the investigational product or interpretation of participant safety or study results (eg, significant lens or corneal opacities, glaucoma, amblyopia, gross retinal anatomical abnormality, etc).
- Difference in screening CRT measurement between the right and left eye $>10\mu\text{m}$.
- Prior Grade 3 or 4 hypersensitivity reaction, eg, bronchospasm and hypotension requiring intravenous treatment, cardiac dysfunction, anaphylaxis to ICV cerliponase alfa infusion.
- Any other contraindication to the administration of ICV cerliponase alfa, including ventriculo-peritoneal shunt, acute intracerebroventricular access device leakage, device failure, or device-related infection that would impact ability to receive ICV cerliponase alfa.

... and 16 more (see full listing online)

Locations (2 total)

University Medical Center Hamburg-Eppendorf (UKE)- Childrens Hospital, Hamburg, Germany
Greater Ormond Street Hospital, London, United Kingdom