A Study of NNZ-2591 in Pediatric Participants With Phelan-McDermid Syndrome

NCT07281079

Status	RECRUITING
Phase	Phase 3
Sponsor	Neuren Pharmaceuticals Limited
Enrollment	160 participants

Key Eligibility Criteria

Inclusion (5)

- Male or female pediatric participants with Phelan-McDermid syndrome ages 3 to 12 years (inclusive) at the time of signing the informed consent.
- Clinical diagnosis of Phelan-McDermid syndrome with a documented disease-causing genetic abnormality of SHANK3.
- Body weight e 10 kg at Screening.
- Participants with a PMSA-S overall score e 3 at the Screening and Baseline visits.
- Not actively undergoing regression or loss of skills.

Exclusion (7)

- Use of exclusionary medication or unstable treatment regimens of acceptable concomitant medications as required by the protocol.
- Current treatment with more than 3 allowable psychotropic medications.
- Participants with seizures must be controlled on no more than 2 anticonvulsant medications (not counting rescue medications).
- Psychotropic medications or any other medication used for a chronic illness (not including antibiotics, pain relievers, anti-diarrheals, and laxatives) with doses and dosing regimen that have not been stable for at least 4 weeks before Screening. If the treatment was discontinued, the discontinuation must have occurred no fewer than 2 weeks before the start of Screening.
- Any intercurrent seizures in the past 6 months and /or more than 1 seizure in the past 12 months. •A single febrile seizure in the 6 months prior to screening is allowable if no rescue medication was required.

... and 2 more (see full listing online)

Locations (2 total)

Neuren PMS-301 Site#111, San Rafael, California, United States
Neuren PMS-301 Site#109, Chevy Chase, Maryland, United States