An Open-Label Study of Oral NNZ-2591 in Phelan-McDermid Syndrome

Study Purpose

The purpose of the study is to look at the safety, tolerability, and efficacy of NNZ-2591 in the treatment of children with Phelan-McDermid syndrome.

This study investigates an oral medication called “NNZ-2591”, which aims to improve the impaired connections and signaling between brain cells that are involved in Phelan-McDermid syndrome. The study medication is experimental.

Participation in the study will be required for approximately 19 weeks, with a combination of in-clinic, remote, and telehealth consultations. An in-home nurse will also visit your house to check on the participant and administer a number of assessments.

Participants in the Phelan-McDermid study will need to:

- Attend clinic appointments
- Take study medication
- Have blood, urine, and stool samples collected for laboratory testing
- Have Phelan-McDermid syndrome symptoms monitored
- Have heart activity monitored
- Caregivers will also need to complete a number of assessments capturing their view of the participant’s progress.

To ease the burden of travel on families, an in-home nurse will be available to assist with telehealth/remote visits. At a time convenient to you, the in-home nurse will visit your home and conduct a number of the required study assessments. This has been included in the study design to make it easier for your family to participate in the study and promote inclusion, without compromising on the data that needs to be collected at each visit.

There is no cost to participate in this clinical trial. Reimbursement is available for reasonable travel and incidental costs incurred attending your clinic visits. More details can be found here.

Recruitment Criteria

Study Type: Interventional
Potential participants must:

- Have a diagnosis of Phelan-McDermid syndrome.
- Be aged between 3-12 years old.
- Be able to swallow a liquid medication.

A full list of inclusion and exclusion criteria can be found at ClinicalTrials.gov. The clinical team managing the study at one of the participating sites will determine if your child is eligible to enroll.

Parents with children in the 3-5 years age range are especially encouraged to consider enrolling.

For more information, please speak with one of the participating site teams.

Trial Details

ClinicalTrials.Gov Id: NCT05025241
Phase: 2
Duration: 19 weeks with a combination of in-clinic, remote, and telehealth consultations
Lead Sponsor: Neuren Pharmaceuticals Limited
Countries: United States

Trial Sites

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Questions

More information on the study can be found here and a list of FAQs can be found here. For additional recruitment criteria, visit clinicaltrials.gov. To discuss enrollment and questions about recruitment, please reach out to the site coordinators listed above. If you are interested in receiving travel support towards participation in the study or would like further information, please contact the PMSF directly via travelprogram@pmsf.org.

Additional Links:

- Eligibility Criteria
- Travel Assistance Program
- Clinical Trial Recruitment Announcement
- Understanding Clinical Research
- Clinical Trial Informed Decision Tool
- Clinical Trials in PMS 2022 Conference Session