Phase 3 Efficacy Study With Concurrent Control of IT MELPIDA in SPG50.Concurrent Controls.

NCT06692712

Status	RECRUITING
Phase	Phase 3
Sponsor	Elpida Therapeutics SPC
Enrollment	24 participants

Key Eligibility Criteria

Inclusion (15)

- To be eligible to participate in this study candidates must meet the following eligibility criteria at Screening:
- For the treatment group
- Male and females between the ages of 4 months to 72 months at the time of screening.
- Molecularly-confirmed diagnosis of SPG50 (confirmed by a CLIA certified, CE-marked, or equivalent lab): Genomic DNA mutation analysis demonstrating bi-allelic pathogenic variants in the AP4M1 gene.
- Subjects must have features of neurologic dysfunction by clinical history and physical examination.

... and 10 more (see full listing online)

Exclusion (23)

- For the treatment group
- Inability to participate in the clinical evaluation as determined by the principal investigators.
- Clinically significant abnormal laboratory values (hemoglobin \< 6 or \> 20 g/dL; white blood cell \> 20,000 per cmm, platelets count \< 100,000 per cmm; INR \> ULN; GGT, ALT, and AST or total bilirubin \> 1.5 × ULN, creatinine e 1.5 mg/dL) prior to gene replacement therapy.
- Presence of a concomitant medical condition (eg, scoliosis or bleeding disorder) that precludes a lumbar puncture or use of anesthetics for sedated procedures.
- Documented cardiomyopathy or significant congenital heart abnormalities.

... and 18 more (see full listing online)

Locations (2 total)

University of Texas Southwestern Medical Center, Dallas, Texas, United States
Sant Joan de Deu, Barcelona, Spain