

Melpida: Recombinant Adeno-associated Virus (serotype 9) Encoding a Codon Optimized Human AP4M1 Transgene (hAP4M1opt)

NCT05518188

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Elpida Therapeutics SPC
Enrollment	4 participants

Key Eligibility Criteria

Inclusion (9)

- Age 4 months-10 years old
- Confirmed diagnosis of SPG50 disease by:
 - Genomic DNA mutation analysis demonstrating homozygous or compound heterozygous, confirmed pathogenic variants in the AP4M1 gene
 - Clinical history or examination features consistent with SPG50 and that include neurologic dysfunction
 - Parent/legal guardian willing to provide written informed consent for their child prior to participation in the study
- ... and 4 more (see full listing online)

Exclusion (14)

- Inability to participate in study procedures (as determined by the site investigator)
- Presence of a concomitant medical condition that precludes lumbar puncture (LP) or use of anesthetics
- History of bleeding disorder or any other medical condition or circumstance in which lumbar puncture is contraindicated according to local institutional policy
- Inability to be safely sedated in the opinion of the clinical anesthesiologist
- Active infection, at the time of dosing, based on clinical observations
- ... and 9 more (see full listing online)

Locations (1 total)

Children's Medical Center Dallas, Dallas, Texas, United States