

Phase I/II/III Gene Transfer Clinical Trial of scAAV9.U1a.hSGSH

NCT02716246

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	Ultragenyx Pharmaceutical Inc
Enrollment	36 participants

Key Eligibility Criteria

Inclusion (7)

- Diagnosis of MPS IIIA confirmed by the following methods:
 - No detectable or significantly reduced SGSH enzyme activity by leukocyte assay, and
 - Genomic DNA analysis demonstrating homozygous or compound heterozygous mutations in the SGSH gene (based upon review of documented results from a qualified laboratory, and with confirmation with Medical Monitor)
 - Age:
 - For Cohort 1-3: From birth (participating sites in USA and Australia) OR 6 months (participating sites in Spain) to 2 years of age with no BSITD-III Cognitive Development Quotient (DQ) requirement, or older than 2 years with a BSITD-III Cognitive DQ of 60 or above (participating sites globally).
- ... and 2 more (see full listing online)

Exclusion (32)

- Inability to participate in the clinical evaluation as determined by PI
 - Cohorts 1 to 3 only: Identification of two nonsense or null variants on genetic testing of the SGSH gene (based upon review of documented results from a qualified laboratory, and with confirmation with Medical Monitor)
 - At least one S298P mutation in the SGSH gene (based upon review of documented results from a qualified laboratory, and with confirmation with Medical Monitor)
 - Has evidence of an attenuated phenotype of MPS IIIA, in the judgement of the PI
 - Presence of a concomitant medical condition that precludes lumbar puncture or use of anesthetics
- ... and 27 more (see full listing online)

Locations (5 total)

Nationwide Children's Hospital, Columbus, Ohio, United States
Children's Hospital of Pittsburgh, Pittsburgh, Pennsylvania, United States
Women's and Children's Hospital, North Adelaide, South Australia, Australia
... and 2 more locations

<https://clinicaltrials.gov/study/NCT02716246>

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