

JAG201 Gene Therapy Study in Children & Adults With SHANK3 Haploinsufficiency

NCT06662188

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Jaguar Gene Therapy, LLC
Enrollment	6 participants

Key Eligibility Criteria

Inclusion (8)

- Is male or female, and 2 to 9 years of age at the time of JAG201 administration
- Has a molecular confirmation of a loss of function mutation in SHANK3 or a 22q13.3 deletion classified as a Class I deletion
- Has evidence of developmental/cognitive delay of at least 2 standard deviations (SD) below the mean (i.e., d 70) via either Intelligence Quotient (IQ) OR Developmental Quotient (DQ) assessment (as applicable)
- Has an overall Phelan-McDermid Syndrome (PMS) Assessment of Severity (PMSA-S) Score of 3 or greater at Screening
- Willing to initiate structured therapies and continue for the duration of the study as determined by the specific therapist (structured therapies may include, at a minimum, physical therapy, occupational therapy, speech therapy, and applied behavior analysis)
- ... and 3 more (see full listing online)

Exclusion (11)

- A pediatric participant who meets any of the following criteria will be excluded from this study:
- Has history of developmental regression defined in this study as a prolonged loss of previously acquired skills (defined as skills maintained for at least 3 months) with loss of skills persisting for at least 3 months
- Has known or suspected prion disease (e.g., Creutzfeldt-Jakob Disease)
- Has poorly-controlled epilepsy (defined as an increase in the dose or addition of new anti-epileptic medications within the past 3 months) or any history of status epilepticus or seizure-induced hospitalizations within the last 12 months
- Has history of acute cerebrovascular episodes
- ... and 6 more (see full listing online)

Locations (3 total)

Rush University, Chicago, Illinois, United States
Boston Children's Hospital, Boston, Massachusetts, United States
Seaver Autism Center at Mount Sinai, New York, New York, United States

<https://clinicaltrials.gov/study/NCT06662188>

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