

RB001 Gene Therapy Study in Children With SHANK3-related Phelan McDermid Syndrome (PMS)

NCT07014020

Status	RECRUITING
Phase	Not Applicable
Sponsor	Peking University First Hospital
Enrollment	8 participants

Key Eligibility Criteria

Inclusion (7)

- Age ≥3 years and <18 years (at the time of signing informed consent), any gender
- Genetic test and clinical confirmed diagnosis of SHANK3-related PMS
- Meets diagnostic criteria for moderate or more severe Autism Spectrum Disorder (ASD)
- Intelligence Quotient (IQ) score <70 or Developmental Quotient (DQ) (excluding gross motor) average score <70
- Willing to provide biological samples required for the study (e.g., blood, urine)

... and 2 more (see full listing online)

Exclusion (12)

- A pediatric participant who meets any of the following criteria will be excluded from this study:
- Previous or current participation in other PMS drug clinical trials or other AAV gene therapy clinical studies
- Has known allergic constitution, including allergy or hypersensitivity to prednisone acetate, other glucocorticosteroids, their excipients, or local anesthetics
- Subjects with status epilepticus within 3 months prior to enrollment
- Subjects requiring invasive or non-invasive ventilatory support

... and 7 more (see full listing online)

Locations (1 total)

Peking University First Hospital, Beijing, Beijing Municipality, China