

A First-in-Human Clinical Trial to Evaluate the Safety, Tolerability, and Efficacy of a Novel CRISPR RNA-editing Therapy in Patients with Mecp2 Duplication Syndrome, a Rare Orphan Disease (HERO)

NCT06615206

Status	RECRUITING
Phase	Not Applicable
Sponsor	HuidaGene Therapeutics Co., Ltd.
Enrollment	6 participants

Key Eligibility Criteria

Inclusion (5)

- Males e 2 and d18 years at the time of signing informed consent;
- Genetic test and clinical confirmed diagnosis of MDS;
- Stable pattern of seizures, or has had no seizures while currently receiving medical treatment (including antiepileptics) and physical therapy are stable for at least 2 months before screening;
- Willing to adhere to protocol, including biological samples collection and hospitalization for intracerebroventricular injection surgery;
- Acceptable hematology, clinical chemistry, and urine laboratory parameters.

Exclusion (8)

- MECP2 gene triplication;
- Concurrent genetic syndromes other than MDS;
- Significant brain or cerebellar atrophy, or other significant degenerative changes as shown in cranial MRI at screening;
- Prior or current hypertension, cardiomyopathy, myocardial ischemia or atrial fibrillation and other cardiovascular diseases;
- Prior central nervous system surgery within 6 months before enrolment;
- ... and 3 more (see full listing online)

Locations (1 total)

Peking University First Hospital, Beijing, Beijing Municipality, China