

Evaluation of Safety and Efficacy of Gene Therapy Drug in the Treatment of Spinal Muscular Atrophy (SMA) Type 3 Patients

NCT06421831

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	GeneCradle Inc
Enrollment	21 participants

Key Eligibility Criteria

Inclusion (5)

- e2years of age on the day of signing the informed consent form;
- Genetic and clinical diagnosis of type 3 SMA with bi-allelic deletion of SMN1 of 5qSMA;
- Hammersmith Functional Motor Scale - Expanded (HFMSE) score is between 10 and 54 at screening;
- Female patients of childbearing age who are pregnant or lactating, as well as all enrolled patients (both male and female), should take effective contraceptive measures within 6 months after the treatment;
- Patients or patient's legal guardian(s) must be able to understand the purpose and risks of the study and voluntarily provide signed and dated informed consent prior to any study-related procedures being performed.

Exclusion (13)

- Patient who has participated in any previous gene therapy research trials;
 - Patient who has AAV9 neutralizing antibody titer e1:200;
 - Patient who has received Nusinersen within 120 days and Risdiplam within 15 days before treatment;
 - Patient who requires invasive or non-invasive ventilatory support averaging 16 hours/day at screening;
 - SMN2 copy numbers >4
- ... and 8 more (see full listing online)

Locations (1 total)

Beijing Tiantan Hospital, Capital Medical University, Beijing, Beijing Municipality, China