

Safety and Dystrophin Expression of SPOT-mRNA03 in Duchenne Muscular Dystrophy (DMD) Patients

NCT07188012

Status	RECRUITING
Phase	Early Phase 1
Sponsor	Shanghai Siponuoyin Biotechnology Co Ltd
Enrollment	6 participants

Key Eligibility Criteria

Inclusion (8)

- According to the requirements of the region/country and/or IRB/IEC, the patient and/or legal guardian have signed a written informed consent form and are aware of all relevant study content.
- Ambulatory boys aged between 2 to 6 years of age, inclusive who can walk without assistance for at least 10 meters.
- The medical history includes clinical diagnosis of DMD and confirmed Duchenne mutations using validated genetic testing (MLPA and whole genome sequencing).
- Able to tolerate muscle biopsy under anesthesia and have no contraindications to biopsy.
- Heart, liver, lung, and kidney functions are sufficient:
... and 3 more (see full listing online)

Exclusion (21)

- Complications other than DMD that may cause muscle weakness and/or motor dysfunction.
- There are severe intellectual disabilities (such as severe autism, severe cognitive impairment, and severe behavioral disorders) that, according to the investigator's judgment, can affect the study.
- Hospitalization for respiratory failure within 8 weeks prior to screening.
- Asthma or underlying lung diseases that are poorly controlled, such as bronchitis, bronchiectasis, emphysema, or recurrent infectious pneumonia that investigator believes may affect respiratory function.
- Severe uncontrolled heart failure (NYHA III-IV), including any of the following conditions
... and 16 more (see full listing online)

Locations (1 total)

Shanghai Children's Medical Center, Shanghai, Shanghai Municipality, China