

Evaluation of the Safety, Tolerability, and Efficacy of LE051 in Patients With Duchenne Muscular Dystrophy

NCT06900049

Status	RECRUITING
Phase	Early Phase 1
Sponsor	Shanghai Jiao Tong University School of Medicine
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (3)

- Male 4-8 years old at the beginning of screening (including boundary values);
- DMD subjects with a clinical diagnosis of DMD referred to the Duchenne Clinical Practice Guidelines for Progressive Muscular Dystrophy (2020 edition) and whose genetic test results were confirmed to be applicable to exon skipping at No.51.
- The subjects and/or his guardian voluntary participate in this trial and can comprehend and sign ICF.

Exclusion (3)

- Clinical signs of heart failure: left ventricular ejection fraction (LVEF) $\lt 40\%$
- The average FVC percentage of the predicted value is less than 40%
- lead ECG QT interval (QTc) $\gt 0.45$ seconds.

Locations (1 total)

Shanghai Children's Medical Center, Shanghai Jiao Tong University School of Medicine, Shanghai, Shanghai Municipality, China