A Study to Investigate the Safety and Biodistribution of a Single Intrathecal (IT) Injection of INS1201 in Ambulatory Males With Duchenne Muscular Dystrophy (DMD)

NCT06817382

Status	RECRUITING
Phase	Phase 1
Sponsor	Insmed Gene Therapy LLC
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (6)

- Participant must be male at birth, 3 to \<5 years of age, inclusive (Part 1) and 2 to \<3 years of age (Part 2), at the time of legally authorized representative (LAR) signing and dating the informed consent form.
- Ambulatory -as defined as the ability to walk at least 10 meters unassisted (ie, without personal assistance or use of any assistive devices) Note: children who have not yet developed the ability to walk by the time of screening (for whatever reason) will not be eligible for the study.
- Has a definitive diagnosis of DMD prior to Screening or as part of Screening based on genetic testing. Note that participants who rescreen do not have to repeat genetic testing for the diagnosis of DMD if one is already on file. Genetic reports must describe a frameshift deletion, frameshift duplication, premature stop ("nonsense"), canonical splice site mutation, or other pathogenic variant in the DMD gene fully contained between exons 18 to 58 (inclusive) that is expected to lead to absence of a functional dystrophin protein (mutations in exons 1-17 or 59-71 are therefore not permitted).
- Able to cooperate with motor assessment testing.
- Has received vaccinations recommended for the participant's age and DMD disease according to Centers for Disease Control and Prevention (CDC) Child and Adolescent Immunization Schedule by Age, World Health Organization, or local recommendation incorporating the Advisory Committee on Immunization Practices (ACIP) Vaccine Recommendations and Guidelines for Patients with Altered Immunocompetence.

... and 1 more (see full listing online)

Exclusion (14)

- Prior treatment with gene or cell-based therapy at any time.
- Oligonucleotide-based exon skipping or small molecule stop codon readthrough-promoting therapies for at least 6 months prior to enrolment.
- Has left ventricular ejection fraction \< 50% on the screening echocardiogram (ECHO) or clinical signs and/or symptoms of cardiomyopathy.
- Has cardiac arrhythmia or significant electrocardiogram (ECG) interval abnormalities.
- Major surgery within 3 months prior to Day 1 or planned surgery or procedures that would interfere with the conduct of the study at any time during this study.

... and 9 more (see full listing online)

Locations (9 total)

USA012, Little Rock, Arkansas, United States
USA010, Davis, California, United States
USA002, Palo Alto, California, United States
... and 6 more locations