A Gene Transfer Therapy Study to Evaluate the Safety of and Expression From Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD)

NCT04626674

Status	RECRUITING
Phase	Phase 1
Sponsor	Sarepta Therapeutics, Inc.
Enrollment	83 participants

Key Eligibility Criteria

Inclusion (14)

- For Cohorts 1-8: Has a definitive diagnosis of DMD based on documented clinical findings and prior genetic testing.
- Cohort 1: Is ambulatory, and e4 to \<8 years of age at the time of Screening.
- Cohort 2: Is ambulatory, and e8 to \<18 years of age at the time of Screening.
- Cohort 3: Non-ambulatory per protocol specified criteria at the time of Screening.
- Cohort 4: Is ambulatory and e3 to \<4 years of age at the time of Screening.

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

Exclusion (4)

- Has a concomitant illness, autoimmune disease, chronic drug treatment, and/or cognitive delay/impairment that in the opinion of the Investigator creates unnecessary risks for gene transfer.
- Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression within protocol-specified time limits.
- Abnormality in protocol-specified diagnostic evaluations or laboratory tests.
- Cohort 8: Any confounding factors that would prevent the use of oral sirolimus including a known hypersensitivity to sirolimus or any of its excipients.

Locations (5 total)

Stanford University, Palo Alto, California, United States
University of California, Davis, Sacramento, California, United States
Washington University in St. Louis, St Louis, Missouri, United States
... and 2 more locations