ForPatients
by Roche

Duchenne Muscular Dystrophy (DMD)

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

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Trial Status	Trial Runs In	Trial Identifier
Completed	1 Country	NCT03375164 IRB17-00512 2021-000077-83 SRP-9001-101

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

Systemic Gene Delivery Phase I/IIa Clinical Trial for Duchenne Muscular Dystrophy Using rAAVrh74.MHCK7.Micro-dystrophin (microDys-IV-001)

Trial Summary:

This study was an open-label single-dose gene transfer therapy study evaluating the safety of delandistrogene moxeparvovec intravenous (IV) administration in boys with DMD. This study was originally designed to consist of 12 patients across 2 Cohorts. Cohort A would have included participants ages 3 months to 3 years, and Cohort B included participants ages 4 to 7 years old. No participants were enrolled in Cohort A.

Sarepta Therapeutics, Inc.	Phase 1/Phase 2
Sponsor	Phase

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Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Male	#3 Months & # 7 Years	No

Inclusion Criteria:

- Cohort A participants: 3 months to 3 years of age, inclusive
- Cohort B participants: 4 to 7 years of age, inclusive
- Definitive diagnosis of DMD based on documented clinical findings and prior genetic testing.
- Ability to cooperate with motor assessment testing.
- Cohort A participants: No previous treatment with corticosteroids.
- Cohort B participants: Stable dose equivalent of oral corticosteroids for at least 12 weeks prior to screening and the dose is expected to remain constant (except for potential modifications to accommodate changes in weight) throughout the first year of the study.
- Cohorts A & B: A frameshift mutation contained between exons 18 and 58 (inclusive).

Exclusion Criteria:

- 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.
- Presence of any other clinically significant illness, medical condition, or requirement for chronic drug treatment that in the opinion of the Investigator creates unnecessary risk for gene transfer.

Other inclusion or exclusion criteria could apply.