ForPatients
*by Roche*

Duchenne Muscular Dystrophy (DMD)

# A Gene Delivery Study to Evaluate the Safety and Expression of Delandistrogene Moxeparvovec in Participants Under the Age of Four With Duchenne Muscular Dystrophy (DMD)

| Trial Status | Trial Runs In | Trial Identifier |
|---|---|---|
| Recruiting | 5 Countries | NCT06128564 2022-000691-19 BN43881 |

*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:*

A Two-Part, Open-Label Systemic Gene Delivery Study to Evaluate the Safety and Expression of RO7494222 (SRP-9001) in Subjects Under the Age of Four With Duchenne Muscular Dystrophy

## *Trial Summary:*

This open-label, single-arm study will evaluate the safety and expression of delandistrogene moxeparvovec in participants with DMD. Participants will be in the study for approximately 264 weeks.

**Hoffmann-La Roche**
Sponsor

**Phase 2**
Phase

**NCT06128564 2022-000691-19 BN43881**
Trial Identifiers

## *Eligibility Criteria:*

| Gender | Age | Healthy Volunteers |
|---|---|---|
| Male | # 3 Years | No |

## *Inclusion Criteria:*

- Cohort A: >=3 years of age to <4 years of age
- Cohort B: >=2 years of age to <3 years of age
- Cohort C: >6 months to <2 years of age
- Cohort D: <=6 months of age

- Has a definitive diagnosis of DMD prior to screening based on documentation of clinical findings and prior confirmatory genetic testing using a clinical diagnostic genetic test
- Able to cooperate with age-appropriate motor assessment testing
- A pathogenic frameshift mutation or premature stop codon contained between exons 18 and 79 (inclusive)

## *Exclusion Criteria:*

- Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression, within protocol-specified time limits
- Recombinant Adeno-Associated Virus Serotype rh74 (rAArh74) antibody titers are elevated, as per protocol-specified criteria
- Receiving regular oral corticosteroids as a treatment for DMD or planning to receive oral corticosteroids as a treatment for DMD within 1 year of baseline
- 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
- Medical condition or extenuating circumstance that, in the opinion of the investigator, might compromise the participant's ability to comply with the protocol required testing or procedures, or compromise the participant's well-being or safety, or clinical interpretability

Other inclusion or exclusion criteria could apply