

# 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) (ENVOL)

## Hub Summary

This open-label, single-arm study will evaluate the safety and expression of delandistrogene moxeparvovec in participants with DMD. Participants (Aged up to 3 years of age) will be in the study for approximately 264 weeks.

**Study Number:** NCT06128564

## Description by Hoffman - La Roche

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.

This study consists of four different cohorts of patients. Cohort A: Aged 3 years of age, Cohort B: Aged 2 years of age, Cohort C: Aged from over 6 months to less than 2 years of age and finally Cohort D: Aged less than or equal to 6 months of age.

## Primary Outcome Measures

Percentage of Participants with a Treatment-emergent Adverse Event (TEAE), Serious Adverse Event (SAE), and Adverse Event of Special Interest (AESI)

## Secondary Outcome Measures

Change in Quantity of Delandistrogene Moxeparvovec Dystrophin as Measured by Western Blot

## Can I take part?

### Inclusion Criteria

- ✓ Cohort A:  $\geq 3$  years of age to  $< 4$  years of age
- ✓ Cohort B:  $\geq 2$  years of age to  $< 3$  years of age
- ✓ Cohort C:  $> 6$  months to  $< 2$  years of age
- ✓ Cohort D:  $\leq 6$  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

For contact details and to find out more, please refer to [dmdhub.org](https://dmdhub.org).

## Trial Status

### On hold

**UK Locations**  
London - GOSH, On hold,  
Oxford, On hold

**Trial Sponsor**  
Hoffman - La Roche

**Phase**  
Phase 2

**Length Of Participation**  
264 weeks

**Recruitment Target**  
21

**Ambulatory**  
Ambulant and non-ambulant

**Therapeutic Category**  
Gene Therapy

**Age**  
up to 3 Years

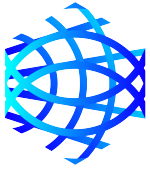
**Mutation Specific**  
Mutation specific therapies, A pathogenic frameshift mutation or premature stop codon contained between exons 18 and 79 (inclusive)

**Muscle Biopsy**  
Muscle Biopsy Required

**MRI**  
No

[dmdhub.org](https://dmdhub.org)


  
DMD HUB



Duchenne  
**UK**

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