

Clinical trial to Evaluate the Efficacy, Safety and Tolerability of RO7239361 in Ambulatory Boys with Duchenne muscular dystrophy

Hub Summary

Please note the development of this drug was stopped after this trial failed to reach its objectives.

This placebo-controlled study is designed to assess the efficacy, safety and tolerability of two different doses of RO7239361 in ambulatory males with DMD. RO7239361 is a subcutaneously delivered treatment which inhibits myostatin.

Myostatin is a protein in the body which inhibits muscle growth and it is required to stop muscles from growing too large. Myostatin production increases naturally with age. It is thought that inhibiting myostatin function could lead to increased muscle size and strength in patients with DMD.

Study Number: NCT03039686

Description by Hoffmann-La Roche

This is a multi-center, randomized, double-blind, placebo-controlled study to assess the efficacy, safety and tolerability of two different weekly doses of RO7239361 in ambulatory boys with Duchenne Muscular Dystrophy (DMD).

Primary Outcome Measures

- Change from baseline in the North Star Ambulatory Assessment (NSAA) total score in RO7239361 treated participants
- Change from baseline in the North Star Ambulatory Assessment (NSAA) total score in placebo treated participants.

Can I take part?

Inclusion Criteria

- ✓ Diagnosed with DMD by a blood test
- ✓ Able to walk without assistance
- ✓ Minimum North Star Ambulatory Assessment (NSAA) score of 15 at screening
- ✓ Able to walk up 4 stairs in 8 seconds or less
- ✓ Weigh at least 15 kg (33 lbs)
- ✓ Taking corticosteroids for DMD

Exclusion Criteria

- ✗ Any behaviour or mental issue that will affect the ability to complete the required study procedures
- ✗ Previously or currently taking medications like androgens or human growth hormone
- ✗ Use of a ventilator during the day
- ✗ Unable to have blood samples collected or receive an injection under the skin

Other protocol defined Inclusion/Exclusion Criteria could apply.

For contact details and to find out more, please refer to dmdhub.org.



**Duchenne
UK**

Trial Status

Trial terminated

UK Locations
London - GOSH, Trial complete/terminated, Alder Hey, Trial complete /terminated

Trial Sponsor
Hoffmann-La Roche

Phase
2/3

Length Of Participation
48 weeks

Recruitment Target
159

Ambulatory
Ambulant

Therapeutic Category
Myostatin inhibition

Age
6-11

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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