Sarepta Extension Study for Casimersen or Golodirsen

An Extension Study to Evaluate Casimersen or Golodirsen in Patients With Duchenne Muscular Dystrophy

Hub Summary

This is an open-label, non-randomized extension trial for patients who have already taken part in the trials testing the drugs, casimersen or golodirsen, to evaluate the effects of their long term use.

This trial focuses on patients with Exon 45 and Exon 53 mutations, and will involve weekly intravenous infusions of 30mg/kg for up to 2 years.

Study Number: NCT03532542

Description by Sarepta Therapeutics

The main objective of this study is to evaluate the safety and tolerability of long-term treatment with casimersen or golodirsen in patients with Duchenne muscular dystrophy (DMD).

Experimental: Casimersen

Patients amenable to exon 45 skipping who have completed a clinical trial evaluating casimersen will receive open-label casimersen intravenous (IV) infusions, weekly, at 30 mg/kg for up to 144 Weeks.

Experimental: Golodirsen

Patients amenable to exon 53 skipping who have completed a clinical trial evaluating golodirsen will receive open-label golodirsen intravenous (IV) infusions, weekly, at 30 mg/kg for up to 144 Weeks.

Primary Outcome Measures

- Number of Patients With Serious Adverse Events (SAEs) [Time Frame: Up to 30 days after the last infusion of study drug (assessed up to 148 weeks)]]

Can I take part?

Inclusion Criteria

- ✔ Completed a clinical trial evaluating casimersen or golodirsen, per protocol.
- ✔ Aged between 7 and 23 years, inclusive, at enrollment.

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

Duchenne UK

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Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated, Newcastle, Trial complete /terminated

Trial Sponsor
Sarepta Therapeutics

Phase
III

Length Of Participation
144 weeks

Recruitment Target
260

Ambulatory
Ambulant and non-ambulant

Age
7-23

Mutation Specific
Mutation specific therapies, Exon 45 (Casimersen) and Exon 53 (Golodirsen)

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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