

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

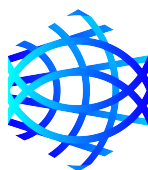
- Number of Patients With Serious Adverse Events (SAEs) [Time Frame: Up to 30 days after the last infusion of study drug (assessed up to 148 weeks)]]

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

dmdhub.org

