

# 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](http://dmdhub.org).



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### Trial Status

Enrolling by invitation

**UK Locations**  
London - GOSH,  
Enrolling by invitation,  
Newcastle, Enrolling by  
invitation

**Trial Sponsor**  
Sarepta Therapeutics

**Age**  
7-23

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

**Muscle Biopsy**  
No Muscle Biopsy  
Required

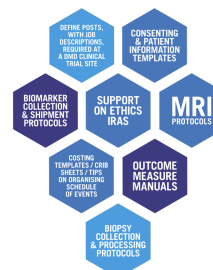
**MRI**  
No

**Phase**  
III

**Length Of Participation**  
144 weeks

**Recruitment Target**  
260

**Ambulatory**  
Ambulant and non-  
ambulant



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

