

# Sarepta 51 (Young)



## Study of Eteplirsen in Young Patients with DMD Amenable to Exon 51 Skipping

### Hub Summary

This phase 2 open-label study is designed to determine the safety and efficacy of eteplirsen in young patients with DMD. Eteplirsen (EXONDYS 51®) is an exon-skipping drug designed to treat patients with DMD amenable to exon 51 skipping. This study will enroll young males between the ages of 6 months and 4 years.

**Study Number: NCT03218995**

### Description by Sarepta Therapeutics, Inc.

This is a multicenter, open-label, dose-escalation study to evaluate the safety, tolerability, PK, and efficacy of once-weekly IV infusions of eteplirsen in approximately 12 male patients, ages 6 months to 48 months (inclusive), who have genotypically confirmed DMD with a deletion mutation amenable to exon 51 skipping.

### Primary Outcome Measures

- Incidence of adverse events [ Time Frame: Up to 96 Weeks ]
- Abnormal changes from baseline or clinically significant worsening of clinical safety laboratory abnormalities (hematology, chemistry, coagulation, and urinalysis) [ Time Frame: Change from Baseline ]
- Abnormal changes from baseline or worsening of vital signs [ Time Frame: Change from Baseline ]
- Abnormal changes from baseline or worsening of physical examination findings [ Time Frame: Change from Baseline ]
- Abnormal changes from baseline or clinically significant worsening of electrocardiogram (ECG) and echocardiogram (ECHO) [ Time Frame: Change from Baseline ]

### Secondary Outcome Measures

- Maximum plasma concentration [ Time Frame: 24 Weeks ]
- Time of Cmax (Tmax) [ Time Frame: 24 Weeks ]
- Area under the concentration-time curve (AUC) [ Time Frame: 24 Weeks ]
- Apparent volume of distribution at steady state (Vss) [ Time Frame: 24 Weeks ]
- Clearance (CL) [ Time Frame: 24 Weeks ]
- Elimination half-life (t½) [ Time Frame: 24 Weeks ]
- Amount of drug eliminated in urine (Ae%) [ Time Frame: 24 Weeks ]

### Can I take part?

#### Inclusion Criteria

- ✓ Male between 6 months to 48 months of age (inclusive):
  - ✓ Cohort 1: Age 24-48 months (enrolment closed)
  - ✓ Cohort 2: Age 6-24 months (currently enrolling)
- ✓ Diagnosis of DMD with a deletion mutation amenable to exon 51 skipping
- ✓ Parent(s) or legal guardian(s) who is willing to provide written informed consent

#### Exclusion Criteria

- ✗ Received treatment that might have an effect on muscle strength or function within 12 weeks prior to dosing
- ✗ Received previous or current treatment with any experimental treatment
- ✗ Clinically significant illness other than DMD
- ✗ Clinically significant laboratory abnormality
- ✗ Any other condition that could interfere with the patient's participation

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

## Trial Status Recruiting

**UK Locations**  
London - GOSH, Recruiting

**Trial Sponsor**  
Sarepta Therapeutics, Inc.

**Phase**  
2

**Length Of Participation**  
12 weeks

**Therapeutic Category**  
Exon-skipping

**Age**  
6 months to 4 years

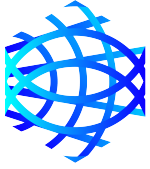
**Mutation Specific**  
Mutation specific therapies,  
Must be amenable to exon  
51 skipping

**Muscle Biopsy**  
No Muscle Biopsy Required

**MRI**  
No

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





Duchenne  
**UK**

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