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.

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

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