Sarepta - MIS51ON

A Randomized, Double-Blind, Dose Finding and Comparison Study of the Safety and Efficacy of a High Dose of Eteplirsen, Preceded by an Open-Label Dose Escalation, in Patients WITH DUCHENNE MUSCULAR DYSTROPHY With Deletion Mutations Amenable to Exon 51 Skip

Hub Summary

This phase 3 study is designed to evaluate the safety and tolerability of two doses of eteplirsen. Part 1 (now closed for recruitment) will investigate two doses with Part 2 comparing the most effective dose from Part 1 with a 30mg/kg dose of eteplirsen.

Study Number: NCT03992430

Description by Sarepta Therapeutics

This study will be comprised of 2 parts: Part 1 (now closed) will be conducted to evaluate the safety and tolerability of two doses (high dose level 1 and high dose level 2) of eteplirsen in approximately 4-6 patients; Part 2 will be conducted for the selection of a high dose (high dose level 1 vs high dose level 2) (dose finding phase), and its comparison with the 30 mg/kg dose of eteplirsen (dose comparison phase), in approximately 149 DMD patients with genetically confirmed deletion mutations amenable to treatment by skipping exon 51.

Primary Outcome Measures

1. Part 1: Incidence of Adverse Events (AEs) [Time Frame: Up to Week 148]
2. Part 2: Change From Baseline in the NSAA Total Score at Week 144 [Time Frame: Baseline, Week 144]

Secondary Outcome Measures

1. Part 2: Change From Baseline in Time to Rise From the Floor, Time to Complete 10-Meter Walk/Run, and the Timed Stair Ascend Test [Time Frame: Baseline, Week 144]
2. Part 2: Change From Baseline in the Total Distance Walked During 6-Minute Walk Test (6MWT) [Time Frame: Baseline, Week 144]
3. Part 2: Change from Baseline in Forced Vital Capacity Percent Predicted (FVC%p) at Week 144 [Time Frame: Baseline, Week 144]
4. Part 2: Time to Loss of Ambulation (LOA) [Time Frame: Baseline up to Week 144]
5. Part 2: Change From Baseline in Skeletal Muscle Dystrophin Expression [Time Frame: Baseline, Postdose (at Week 24, Week 48, or Week 144)]

Trial Status
Fully recruited

UK Locations
London - GOSH, Fully recruited, Birmingham, Fully recruited, Leeds, Fully recruited

Trial Sponsor
Sarepta Therapeutics

Phase
3

Length Of Participation
144 weeks

Recruitment Target
149

Ambulatory
Ambulant

Therapeutic Category
Exon Skipping

Age
4-13

Mutation Specific
Mutation specific therapies, must be amenable to exon 51 skipping

Muscle Biopsy
Muscle Biopsy Required

MRI
No

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6. Part 2: Incidence of Adverse Events (AEs) [Time Frame: Baseline up to Week 148]
7. Part 2: Pharmacokinetic (PK) Plasma Concentration of Eteplirsen [Time Frame: 0 (predose) to 2 hours postdose up to Week 144]

Can I take part?

Inclusion Criteria

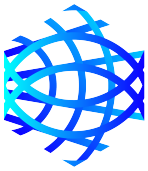
- ✔ Be a male with an established clinical diagnosis of DMD and an out-of-frame deletion mutation of the DMD gene amenable to exon 51 skipping.
- ✔ Ambulatory participant, able to perform TTRISE in 10 seconds or less at the time of screening visit.
- ✔ Able to walk independently without assistive devices.
- ✔ Have intact right and left biceps muscles or an alternative upper arm muscle group.
- ✔ Have been on a stable dose or dose equivalent of oral corticosteroids for at least 12 weeks prior to randomization and the dose is expected to remain constant (except for modifications to accommodate changes in weight and stress-related needs as per the recently published guidelines throughout the study.
- ✔ For ages 7 years and older, has stable pulmonary function (forced vital capacity 50 percent (%) of predicted and no requirement for nocturnal ventilation). For ages 4 to 6 years, does not require support from ventilator or non-invasive ventilation at time of screening.

Exclusion Criteria

- ✖ Use of any pharmacologic treatment (other than corticosteroids) that may have an effect on muscle strength or function within 12 weeks prior to randomization.
- ✖ Current or previous treatment with any other experimental pharmacologic treatment for DMD or any prior exposure to antisense oligonucleotide, gene therapy or gene editing; except the following: Ezutromid in the last 12 weeks prior to first dose; Drisapersen in the last 36 weeks prior to first dose; Suvodirsen in the last 12 weeks prior to first dose; Vamorolone in the last 12 weeks prior to first dose; and Eteplirsen (previous or current use).
- ✖ Major surgery within 3 months prior to randomization and planned surgeries during the study.
- ✖ Presence of any other significant neuromuscular or genetic disease other than DMD.
- ✖ Presence of any known impairment of renal function and/or other clinically significant illness.
- ✖ Has evidence of cardiomyopathy, as defined by left ventricular ejection fraction less than <50% on the screening echocardiogram or Fridericia's correction formula (QTcF) 450 millisecond based on the screening electrocardiograms (ECGs).

Other inclusion/exclusion criteria apply.

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



Duchenne UK

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