Sarepta - MOMENTUM

A Phase 2, Two-Part, Multiple-Ascending-Dose Study of SRP-5051 for Dose Determination, Then Dose Expansion, in Patients With Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment

Hub Summary

This phase 2 study is designed to determine the maximum dose for Sarepta Therapeutics Exon 51 skipping therapy, as well as its safety and tolerability.

There will be two arms to the study - in Part A, patients will receive 1 of 5 doses of SRP-5051 monthly by intravenous infusion. Once the maximum dose has be has been determined, all patients will then roll over into Part B and will receive the maximum dose by intravenous infusion for 24 weeks. In Part B, an additional 15 patients will also be enrolled at the beginning of the study.

Part A recruitment has now been completed and Part B will be beginning soon, involving the original patients from Part A as well as some additional patients.

The UK sites have not yet been finalised, we will provide an update once we have these details.

Study Number: NCT04004065

Description by Sarepta Therapeutics

This study will be comprised of 2 parts: Part A (Multiple Ascending Dose [MAD]) which will be conducted to evaluate the safety and tolerability of SRP-5051 at multiple ascending dose levels to determine the maximum tolerated dose (MTD); Part B (Dose Expansion) will be conducted to evaluate SRP-5051 administered at the MTD, both in patients who will complete Part A and in an expansion cohort of approximately 15 patients who will be enrolled in the study at the beginning of Part B.

Experimental: Part A: SRP-5051

Patients will be sequentially assigned to receive 1 of the 5 escalating dose levels of SRP-5051, monthly, via intravenous (IV) infusion for at least 12 weeks during Part A. Once the maximum tolerated dose (MTD) has been determined in Part A, all patients who have completed Part A will transition to Part B.

Experimental: Part B: SRP-5051

Patients will receive SRP-5051 at the MTD determined in Part A, monthly, via IV infusion, for 24 weeks. This includes the patients who roll over from Part A, as well as the expansion cohort of approximately 15 patients who will enroll in the study at the beginning of Part B.

Primary Outcome Measures

Primary Outcome Measures :

1. Part A: Incidence of Adverse Events (AEs) [Time Frame: Part A: approximately 68 weeks]. Incidence of adverse events includes clinically significant laboratory abnormalities.
2. Part B: Change From Baseline in Dystrophin Protein Level [Time Frame: Baseline, Part B Week 24]

Secondary Outcome Measures

1. Part A: Pharmacokinetic (PK) Plasma Concentration of SRP-5051

Trial Status
Trial terminated

UK Locations
London - GOSH, Trial complete/terminated, Alder Hey, Trial complete /terminated, Glasgow, Trial complete/terminated, Oxford, Trial complete /terminated

Trial Sponsor
Sarepta Therapeutics

Phase
2

Recruitment Target
45

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Exon Skipping

Age
7-21 years old

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

Muscle Biopsy
Muscle Biopsy Required

MRI
No

dmdhub.org

[Time Frame: Predose and at multiple timepoints (up to 24 hours) after end of infusion].

2. Part B: Change From Baseline in Exon-Skipping Levels [Time Frame: Baseline, Part B Week 24.
3. Part B: Incidence of Adverse Events (AEs) [Time Frame: Part B: approximately 44 weeks]
4. Incidence of adverse events includes clinically significant laboratory abnormalities.
5. Part B: Change From Baseline in Forced Vital Capacity (FVC) (percent predicted) [Time Frame: Baseline, Part B Week 24.
6. Part B: Change From Baseline in the North Star Ambulatory Assessment (NSAA) [Time Frame: Baseline, Part B Week 24]
7. Part B: Change From Baseline in the Performance of Upper Limb (PUL) Scores [Time Frame: Baseline, Part B Week 24]
8. Part B: Change From Baseline in the Brooke Upper Extremity Scale score (Brooke score) [Time Frame: Baseline, Part B Week 24]
9. Part B: PK Plasma Concentration of SRP-5051 [Time Frame: Part B predose and at multiple timepoints (up to 24 hours) after end of infusion]

Can I take part?

Inclusion Criteria

- ✔ Has a genetic diagnosis of DMD and an out-of-frame deletion mutation of the DMD gene amenable to exon 51-skipping treatment.
- ✔ Has been on a stable dose of oral corticosteroids for at least 12 weeks prior to study drug administration, or has not received corticosteroids for at least 12 weeks prior to study drug administration.

Exclusion Criteria

- ✖ Has a left ventricular ejection fraction (LVEF) less than (<) 40.0 percent (%) based on an echocardiogram (ECHO) performed within 12 weeks prior to Screening or at the Screening visit.
- ✖ Has a FVC < 40.0% of predicted value within 12 weeks prior to Screening or at Screening.
- ✖ Initiation or change of dosing (except for modifications to accommodate changes in weight) within 12 weeks prior to Screening for any of the following: angiotensin-converting enzyme inhibitors, angiotensin receptor-blocking agents, -blockers, or potassium.
- ✖ Initiation or change of dosing within 12 weeks prior to Screening for over-the-counter preparations, such as herbal/nonherbal supplements, vitamins, minerals, and homeopathic preparations.
- ✖ Treatment with any exon 51-skipping therapy within 12 weeks prior to Screening, or with any experimental gene therapy for the treatment of DMD at any time.

Other inclusion/exclusion criteria apply

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

Duchenne UK

PDF created on 24/11/2026.