

A Study of SGT-003 Gene Therapy in Duchenne Muscular Dystrophy (SGT-003-301)



A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Investigate the Efficacy of a Single Intravenous Dose of SGT-003 in Ambulant Males With Duchenne Muscular Dystrophy (IMPACT)

Hub Summary

This is a Phase 3, double-blind, placebo-controlled study with the primary objective of evaluating the efficacy of a single IV infusion of SGT-003 in pediatric ambulant male participants with DMD. The secondary objectives include the evaluation of additional efficacy and safety outcomes. The study will be divided into 2 parts. Approximately 80 participants will be enrolled in the study. Participants will be randomized 1:1 to either SGT-003 in Part 1 followed by placebo in Part 2 or to placebo in Part 1 followed by SGT-003 in Part 2. Participants will continue to be monitored in long term follow up (LTFU) for at least 5 years from their SGT-003 dosing date.

Study Number:

Description by Solid Biosciences Inc.

This is a Phase 3, double-blind, placebo-controlled study with the primary objective of evaluating the efficacy of a single IV infusion of SGT-003 in pediatric ambulant male participants with DMD. The secondary objectives include the evaluation of additional efficacy and safety outcomes. The study will be divided into 2 parts. Approximately 80 participants will be enrolled in the study. Participants will be randomized 1:1 to either SGT-003 in Part 1 followed by placebo in Part 2 or to placebo in Part 1 followed by SGT-003 in Part 2. Participants will continue to be monitored in long term follow up (LTFU) for at least 5 years from their SGT-003 dosing date.

Functional efficacy assessments will be performed at Screening/Baseline, Day 90, Day 180, Day 360, and Day 540. These assessments will include evaluations of motor function (timed function tests [time to rise from supine, 10-meter walk/run, 4-stair climb], North Star Ambulatory Assessment [NSAA], video assessment, and activity monitoring by wearable device (stride velocity 95th centile [SV95C]), pulmonary function (% predicted forced vital capacity [FVC], peak expiratory flow [PEF], forced expiratory volume in 1 second [FEV1]), and patient-reported outcome (PRO) measures (Pediatric Outcomes Data Collection Instrument [PODCI]). Individual outcome assessments will be evaluated at each timepoint, and change from baseline will be calculated for interpretation of differences over time.

In addition, the first 20 participants enrolled in the study at investigative sites capable of performing muscle biopsies will have muscle biopsies performed at the Screening Visit and at the Part 1 Day 90 Visit. Investigative sites able to conduct biopsies will continue enrolling to ensure that approximately 20 participants will have biopsies performed.

Can I take part?

Inclusion Criteria

1. Participant 7 to <12 years of age.
2. Participant is ambulatory. Ambulatory is defined as "being able to walk without the use of an assistive device."
3. Established clinical diagnosis of DMD and documented dystrophin gene mutation predictive of DMD phenotype.
4. Negative for antibodies against AAV9.
5. A stable daily oral regimen of at least 0.5 mg/kg/day prednisone or 0.75 mg/kg/day deflazacort for at least 6 months prior to entering the study, allowing for weight-based dose modifications in accordance with clinical practice.
6. 10-meter walk/run time <10 seconds.
7. Time to rise from supine <7 seconds and not faster than 3 seconds or faster than the 90th percentile for age.
8. Participant has bodyweight 50 kg.
9. Participant is genetically male.
10. Participant is able to understand and comply with all study procedures as appropriate by age and has a parent(s) or legal guardian(s) (i.e., legally authorized representative [LAR]) who is (are) able to understand and comply with the study procedure requirements. Be willing to provide informed assent and have an LAR(s) who is (are) willing to provide written informed consent for the participant to participate in the study.
11. If participant is of reproductive potential, participant and partner of childbearing potential are willing to use 2 highly effective forms of contraception for 12 months following study drug administration.

Trial Status Not yet recruiting

UK Locations
London - Evelina, Not yet recruiting, London - GOSH, Not yet recruiting, Newcastle, Not yet recruiting, Oxford, Not yet recruiting

Trial Sponsor
Solid Biosciences Inc.

Phase
3

Length Of Participation
6.5 years

Recruitment Target
80

Ambulatory
Ambulant

Therapeutic Category
Gene Therapy

Age
7-11 years

Mutation Specific
Mutation specific therapies

Muscle Biopsy
Muscle Biopsy Required

MRI
Yes

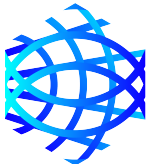
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Exclusion Criteria

1. Prior or ongoing medical condition, medical history, or physical finding that in the Investigator's opinion could adversely affect the safety of the participant, make it unlikely that the course of treatment or follow-up would be completed, or could impair the assessment of study results.
2. Known liver disease or abnormal liver function as measured by gamma-glutamyl transferase [GGT] $>1.5 \times$ upper limit of normal [ULN] or total bilirubin $>ULN$.
3. Abnormal renal function (cystatin C $>1.2 \times$ ULN).
4. Clinically significant abnormalities of coagulation including international normalized ratio (INR) $>1.2 \times$ ULN, activated partial thromboplastin time (aPTT) $>1.2 \times$ ULN, or platelets.
5. Impaired cardiovascular function as follows:
 - a. Clinical History in the opinion of the primary investigator
 - b. Ejection fraction [EF]
 - c. EF $<50-55\%$ and the presence of late gadolinium enhancement by cardiac MRI that is determined to be clinically significant or includes 3 or more segments
6. Pulmonary function predictive of or requiring the use of daytime ventilatory support outside of acute illnesses.
7. History of severe hypersensitivity reactions, including anaphylaxis, to the product or its components.
8. Current or prior treatment with an approved or investigational gene transfer drug or gene editing therapy.
9. Exposure to vamorolone, givinostat, approved or investigational dystrophin- or disease modifying drugs (such as eteplirsen, golodirsen, casimersen, viltolarsen, and ataluren), or another investigational drug for any indication within 6 months or 5 half-lives, whichever is longer, prior to enrollment.
10. Major surgery within 3 months prior to recruitment or planned surgery any time during this study that would have the potential to interfere with the ability or performance on outcome measures.
11. Established clinical diagnosis of DMD that is associated with any deletion mutation in exons 1 to 11 or exons 42 to 45, inclusive, of the DMD gene as documented by a genetic report.
12. Sponsor employees and their family members are ineligible to participate in this study.

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



**Duchenne
UK**