SAFARI44

A Phase 3 Study to Evaluate the Safety and Efficacy of AOC 1044 (Also Referred to as Delpacibart Zotadirsen) in Participants With DMD With Gene Mutations Amenable to Exon 44 Skipping (SAFARI44)

Hub Summary

This Phase 3 trial is testing whether AOC 1044, an experimental drug designed to help the body make more dystrophin, can safely improve muscle function in boys with Duchenne muscular dystrophy (DMD) who have gene mutations that can be treated with exon 44 skipping.

Study Number: NCT07587242

Description by Avidity Biosciences, Inc.

A Randomized, Double-blind, Placebo-controlled, Phase 3 Study to Evaluate the Efficacy and Safety of Intravenous AOC 1044 for the treatment of Duchenne Muscular Dystrophy (DMD) with Gene Mutations Amenable to Exon 44 Skipping.

The study consists of a Screening Period of up to 44 Days, a Randomized 54 Week Treatment Period, a 54 Week Open Label Extension (OLE), and a Follow-Up Period of 6 weeks. Participants will be randomized (to receive an intravenous infusion of either delpacibart-zotadirsen or placebo (a 50-50 chance (or 1 to 1 chance)) of receiving the investigational drug or the placebo in the first part of the study) at the clinical study site every 6 weeks for a total of 9 doses. After completion of the randomized treatment period, all participants may enter the OLE portion of the study consisting of 9 doses of AOC 1044 regardless of group assignment in the randomized period. The final dose will occur at Week 102, followed by a final assessment at Week 108 and a safety follow-up visit at Week 114. An Independent Data Monitoring Committee (IDMC) comprising members independent and external to the Sponsor will review safety, tolerability, and efficacy (as needed) data of this study at regular intervals.

Primary Outcome Measures

Change from Baseline in Time to Rise (TTR) Velocity at Week 54

Secondary Outcome Measures

Change from Baseline in Creatine Kinase (CK) at Week 54
Change from Baseline in 4-Stair Climb (4SC) Velocity at Week 54
Change from Baseline in 10-Meter Walk/Run Test (10MWRT) Velocity at Week 54
Change from Baseline in Stride Velocity 95th Centile (SV95C) at Week 54
Change from Baseline in North Star Ambulatory Assessment (NSAA) Total Score at Week 54
Change from Baseline in DMD Quality of Life (DMD-QoL) Score at Week 54
Change from Baseline in Patient Global Impression of Severity (PGI-S) at Week 54
Change from Baseline in Caregiver Global Impression of Severity (CaGI-S) at Week 54
Change from Baseline in Quantitative Muscle Testing (QMT) at Week 54
Patient Global Impression of Change (PGI-C) at Week 54
Caregiver Global Impression of Change (CaGI-C) at Week 54

Can I take part?

Inclusion Criteria

- ✔ Ambulatory males with clinical and genetic diagnosis of DMD.
- ✔ Acceptable genetic test confirming dystrophin gene mutation amenable to exon 44 skipping.
- ✔ 7 to 16 years of age at time of consent.
- ✔ TTR and NSAA assessment completed within the protocol specified parameters at Screening.
- ✔ On a stable regimen of corticosteroids (including Vamolorone) for at least 6 months prior to Day 1. Steroid regimen must be anticipated to remain stable (appropriate changes to account for body weight and composition or acute illness are allowed).

Exclusion Criteria

- ✖ Previous treatment cell or gene therapy.

Trial Status
Not yet recruiting

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

Trial Sponsor
Avidity Biosciences, Inc.

Phase
Phase 3

Length Of Participation
108 weeks

Recruitment Target
70

Ambulatory
Ambulant

Therapeutic Category
Exon Skipping

Age
7-16 years

Mutation Specific
Mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

dmdhub.org

- Treatment with another oligonucleotide within 6 months of informed consent (not including COVID-19 RNA vaccines).
- Lab values outside of the protocol specified range at Screening
- If on any of the following treatments (growth hormone, testosterone or givinostat), participants must be on a stable regimen and must plan to maintain it for the duration of the study. Participants will be excluded if regimen stability prior to informed consent is as follows:-
  - Less than 1 month, for growth hormone and/or testosterone
  - Less than 6 months for givinostat

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

Duchenne UK

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