# Entrada - ELEVATE-44

## A 2-Part, Randomized, Double-Blind, Placebo-Controlled Study in Participants With Duchenne Muscular Dystrophy Amenable to Exon 44 Skipping to Evaluate the Safety and Efficacy of ENTR-601-44 ( ELEVATE-44 )

### Hub Summary


Entrada Therapeutics is developing an exon 44 skipping therapy (called ENTR-601-44) for people living with Duchenne. Its goal is to help the body make a shorter, but still potentially functional dystrophin protein. Dystrophin is important because it helps keep muscles strong and stable.

The ENTR-601-44-201 study (also called ELEVATE-44) is a global, two-part, randomized, double-blind placebo-controlled, Phase 1/2b study evaluating the safety, tolerability and effectiveness of ENTR-601-44 in people living with Duchenne who are amenable to exon 44 skipping.


### Study Number: ENTR-601-44-201

### Description by Entrada Therapeutics Inc.

This is a study of the investigational medicine ENTR-601-44 in participants who have Duchenne muscular dystrophy (DMD), a rare genetic condition.
The researchers want to: Test how safe ENTR-601-44 is, learn about any side effects, and look at the potential positive effects of ENTR-601-44, compared to placebo. Placebo looks like the investigational medicine but does not contain
any active ingredient. In this summary ENTR-601-44 and placebo are both called study treatments.

The study has 2 parts: Part A: to evaluate if ENTR-601-44 is safe and to determine the best dose of ENTR-601-44 for Part B. Part B: to further evaluate the effect and safety of ENTR-601-44 at the dose determined in Part A.

Part A consists of a Double-Blind (DB) Period and an Open Label (PL Period.
- DB period: Participants will receive 3 doses of either ENTR-601-44 or placebo.
- OL period: All participants (even those who initially received placebo) will receive 6 additional doses of ENTR-601-44.

During Part A, participants will:
- Receive study treatment in the form of an intravenous (IV) infusion (slow injection) into a vein for several weeks
- Visit the clinic regularly for checkups and tests such as: blood and urine tests, physical examinations working, questionnaires, muscle biopsies and exercise tests

Additional details on Part B will become available as we approach its expected start.

Participants are allowed to continue receiving their standard of care therapy for DMD during the study, as long as their health remains stable.

Participants may be eligible to enter an long term extension (LTE) study. An LTE study allows participants to continue receiving the study drug, which helps researchers better understand the safety, tolerability and efficacy of ENTR-601-44 over a longer period of time. All participants in the LTE will receive ENTR-601-44.

## Primary Outcome Measures

- To see how safe ENTR-601-44 is compared to placebo

## Secondary Outcome Measures

- To evaluate ENTR-601-44 compared to placebo on improvements of body function
- To look at what ENTR-601-44 does to the body (pharmacodynamics)
- To look at how the body interacts with ENTR-601-44 (pharmacokinetics)
- Determine the optimal dose for further study in Part B

### Can I take part?

## Inclusion Criteria

## Trial Status
Recruiting

**UK Locations**
London - GOSH, Recruiting, Alder Hey, Recruiting, Leeds, Recruiting, Manchester, Not yet recruiting, Newcastle, Recruiting, Oxford, Recruiting

**Trial Sponsor**
Entrada Therapeutics Inc.

**Phase**
Phase 1/2b

**Length Of Participation**
62 Weeks

**Recruitment Target**
Part A: 24 participants

**Ambulatory**
Ambulant

**Therapeutic Category**
Exon Skipping

**Age**
4-20 years

**Mutation Specific**
Mutation specific therapies, amenable to exon 44 skipping

**Muscle Biopsy**
Muscle Biopsy Required

**MRI**
No

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- Genetic diagnosis of DMD and confirmed pathologic variant in the dystrophin gene amenable to exon 44 skipping as reviewed by a central genetic counsellor
- Assigned male at birth with clinical signs compatible with Duchenne muscular dystrophy as determined by the investigator.
- Part A: 4-20 years of age, inclusive.
- Ambulatory Status Part A: ambulatory with a Performance of the Upper Limb v2.0 (PUL 2.0) Entry as per protocol at Screening
- Adequate muscle for obtaining tissue biopsy as assessed by the investigator.
- Must be on a stable dose of Glucocorticoid (GC) therapy for at least 6 months prior to screening and remain on stable dose for the duration of the study. (GC therapy includes, but is not limited to, prednisone, deflazacort, or vamorolone)
- Other protocol-defined criteria apply.

## Exclusion Criteria

- ✖ Any significant concomitant medical condition that might interfere with the ability to comply with protocol requirements.
- ✖ Has an acute illness within 4 weeks prior to the first dose of study drug which may interfere with study measurements or jeopardize participant's safety.
- ✖ Use of the following medications:
  - ✖ Prior treatment with any exon skipping therapy at any time
  - ✖ Prior treatment with any gene therapy, at any time
  - ✖ Use of anti-coagulants, anti-thrombotics, or anti-platelet agents from at least 30 days prior to the start of the screening period until the end of the study
  - ✖ Use of immunosuppressants (including systemic or oral corticosteroids for chronic non-DMD conditions) from at least 30 days prior to the start of the screening period until the end of the study
  - ✖ Has taken or is currently taking a histone deacetylase (HDAC) inhibitor, including (but not limited to) givinostat from at least 30 days prior to the start of the screening period until the end of the study
- ✖ Laboratory abnormalities.
- ✖ Daytime ventilator dependence or any use of invasive mechanical ventilation via tracheostomy.
- ✖ Has an abnormal electrocardiogram (ECG) reading assessed as clinically significant by the investigator, and/or a QT interval with Fridericia correction method (QTcF) >450 msec at Screening or prior to the first dose of study drug on Day 1.
- ✖ Received any experimental or investigational drug, etc. within 3 months prior to first dose or within 5 half-lives (whichever is longer).

Other protocol-defined criteria apply.

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

Duchenne UK

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