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A Phase II, open-label long-term extension study in participants with Duchenne muscular dystrophy amenable to exon skipping to assess the long-term safety, tolerability, pharmacokinetics, and efficacy of endosomal escape vehicle phosphorodiamidate morpholino oligomer platform products (ELEVATE-LTE)

Submission date 03/03/2026	**Recruitment status** Recruiting	[X] Prospectively registered [_] Protocol [_] Statistical analysis plan [_] Results [_] Individual participant data [X] Record updated in last year
Registration date 01/07/2026	**Overall study status** Ongoing	
Last Edited 12/08/2026	**Condition category** Genetic Diseases	

Plain English summary of protocol

Background and study aims

This long-term extension (LTE) is an open-label study following the initial ENTR-601-44-201 (ELEVATE-44; https://www.isrctn.com/ISRCTN15088415) and ENTR-601-45-201 (ELEVATE-45; https://www.isrctn.com/ISRCTN18511908) studies. There is no control or placebo (a dummy comparison drug). All participants will receive the active study drug at the same dose they were receiving upon completion of the initial study. This long-term extension study looks at the investigational medicines ENTR-601-44 and ENTR-60145 in participants with a rare genetic condition, Duchenne muscular dystrophy (DMD).

Changes in the genes, called variants, can alter the production of certain proteins. As a result of one or more changes to the DMD gene, the body produces little or a non-working dystrophin, a protein needed to help muscle function correctly. ENTR-601-44 and ENTR-601-45 are exon skipping therapies that aim to help the body ‘skip over’ the relevant variant so that the body can produce a shorter but working form of dystrophin.

The researchers will test over a period of time how safe ENTR-601-44 and ENTR-601-45 are, learn about any side effects, and look at the potential positive effects of ENTR-601-44 and ENTR-601-45.

Who can participate?

Males who have completed their participation in clinical study ENTR-601-44- 201 or ENTR-601-45-201.

What does the study involve?
In this summary, ENTR-601-44 and ENTR-601-45 are both called study medications. The study medication will be given as an intravenous infusion (slow injection) into a vein. Participants will receive a fixed number of doses of the study drug.

What are the possible benefits and risks of participating?
Participants may or may not benefit from this study. Participation could help increase knowledge about DMD and the study medication. The possible benefit of receiving ENTR-601-44 or ENTR-601-45 for participants is that dystrophin production may increase and improve muscle function, but there is no guarantee this will happen. This is an early study of ENTR-601-44 and ENTR-601-45 in individuals with DMD, and therefore, information on its potential positive effects in people is limited.

There is a risk that participants may have side effects while participating in the study. Participants might have side effects related to the study medication or study procedures while taking part in the study that could make them feel unwell, uncomfortable, or cause harm. The study staff will take measures to reduce the potential risks. Everyone taking part in the study will be watched for any side effects; however, the study staff does not know all the effects that the study medication may have on the participants. These effects may be mild or serious. In some cases, these effects might be long-lasting or permanent and may even be life-threatening. The study staff may give participants medicines to help reduce any side effects. Participants are advised to report anything to their study doctor that is bothersome or concerning for them.

Since this is an early study with the medication in humans, there is limited information on side effects. There have been studies done in laboratories on animals, there has been one other study on healthy men, and there have been studies with similar medicines. From this research, possible side effects might include issues with kidney function, blood clotting, blood cell count, and liver enzymes. There could also be side effects from the medication administration and muscle biopsies. However, researchers do not know all the side effects that could happen.

Where is the study run from?
Entrada Therapeutics, Inc, USA.

When is the study starting and how long is it expected to run for?
August 2026 to March 2032.

Who is funding the study?
Entrada Therapeutics, Inc, USA.

Who is the main contact?
clinicaltrials@entradatx.com

Contact information

Type(s)
Public, Scientific

Contact name
None . Entrada Therapeutics Clinical Trials

Contact details

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Type(s)
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Additional identifiers

Integrated Research Application System (IRAS)
1013685

Central Portfolio Management System (CPMS)
73038

Sponsor's protocol code number
ENTR-601-DMD-202

Study information

Scientific Title

A Phase II, open-label long-term extension study in participants with Duchenne muscular dystrophy amenable to exon skipping to assess the long-term safety, tolerability, pharmacokinetics, and efficacy of endosomal escape vehicle phosphorodiamidate morpholino oligomer platform products (ELEVATE-LTE)

Acronym
ELEVATE-LTE

Study objectives
Primary objective:
To evaluate the long-term safety and tolerability of study drug in participants with DMD

Secondary objectives:
1. To characterize the pharmacokinetics (PK) of study drug in participants with DMD after long-term dosing
2. To evaluate the impact of study drug on measures of function in participants with DMD after long-term dosing
3. To evaluate the immune response to study drug in participants with DMD after long-term dosing

Ethics approval required
Ethics approval required

Ethics approval(s)
Approved 27/04/2026, South Central - Berkshire Research Ethics Committee (2 Redman Place, Stratford, London, E20 1JQ, United Kingdom; -; berkshire.rec@hra.nhs.uk), ref: 26/SC/0088

Primary study design
Interventional

Allocation
N/A: single arm study

Masking
Open (masking not used)

Control
Uncontrolled

Assignment
Single

Purpose
Treatment, Safety

Study type(s)
Efficacy, Safety, Treatment, Other

Health condition(s) or problem(s) studied
Duchenne muscular dystrophy

Interventions
Participants with DMD who completed either clinical study ENTR-601-44-201 or ENTR-601-45-201 will receive study medication administered intravenously (IV) every 6 weeks for 24 months (ie, ENTR-601-44 for participants continuing from Study ENTR-601-44-201 and ENTR-601-45 for participants continuing from Study ENTR-601-45-201).

Participants enrolling in ENTR-601-DMD-202 will begin the LTE at the dose level they received upon completion of the open-label portion of the parent study. Dose escalation in the LTE study may be permitted based on emerging safety and efficacy data from the parent studies. After review of the cohort's benefit-risk profile, participants may escalate to the higher dose that is deemed favourable compared with the preceding dose.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
ENTR-601-44, ENTR-601-45

Primary outcome(s)
To evaluate the long-term safety and tolerability of study drug in participants with DMD assessed by monitoring adverse events, physical examination, electrocardiogram (ECG) parameters, vital signs and clinical laboratory tests. From parent study baseline through End of Study (up to 2 years).

Key secondary outcome(s)
1. To characterise the pharmacokinetics (PK) of study medicine in participants with DMD after long-term dosing by measuring the plasma concentration of study drug compounds and their final metabolite from parent study baseline through End of Study (up to 2 years)
2. To evaluate the impact of study medicine on measures of function in participants with DMD after long-term dosing by measuring:
2.1. Change from parent study Part A and OL (open-label) Period baselines to LTE EOS (long term extension end of study) in 10-Meter Walk/Run (10MWR) from parent study baseline through End of Study (up to 2 years)
2.2. Change from parent study Part A and OL Period baselines to LTE EOS in timed rise from floor (TRF) from parent study baseline through End of Study (up to 2 years)
2.3. Change from parent study Part A and OL Period baselines to LTE EOS in Timed 4-Stair Climb (4SC) from parent study baseline through End of Study (up to 2 years)
2.4. Change from parent study Part A and OL Period baselines to LTE EOS in stride velocity 95th centile (SV95C) from parent study baseline through End of Study (up to 2 years)
2.5. Change from parent study Part A and OL Period baselines to LTE EOS in North Star Ambulatory Assessment (NSAA) from parent study baseline through End of Study (up to 2 years)
2.6. Change from parent study Part A and OL Period baselines to LTE EOS in Performance of the Upper Limb v2.0 (PUL 2.0) from parent study baseline through End of Study (up to 2 years)
3. To evaluate the immune response to study medicine in participants with DMD after long-term dosing by measuring anti-drug antibody (ADA) and anti-dystrophin antibody in serum from parent study baseline through End of Study (up to 2 years)

Completion date
31/03/2032

Eligibility

Key inclusion criteria
1. Willing and able to provide consent (if at the age of majority) or assent (if a minor), as required by local regulations or the institutional review board (IRB)/independent ethics committee (IEC) after the nature of the study has been explained and prior to the performance of any study-related procedures
2. For participants under the legal age of consent, parent(s) or guardian(s) must be willing and able to provide written, signed informed consent after the nature of the study has been explained and prior to the performance of any study-related procedures. Adult participants must be willing and able to provide written, signed informed consent after the
nature of the study has been explained and prior to the performance of any study-related procedures. Participants who reach the age of majority in their country while the study is ongoing will be asked to provide their own written consent again upon reaching the legal age of majority.
3. Participant completed clinical study ENTR-601-44- 201 or ENTR-601-45-201
4. Males who are sexually active with a female partner of childbearing potential must agree to use condoms during sexual intercourse.

Healthy volunteers allowed
No

Age group
All

Lower age limit
0 Years

Upper age limit
120 Years

Sex
Male

Total final enrolment
0

Key exclusion criteria
1. Any change from the applicable parent study eligibility criteria, including safety events during the parent study, that in the opinion of the investigator in consultation with the medical monitor and/or sponsor designee precludes safe use of study drug.
2. Participant has a condition or circumstance that in the view of the investigator places the subject at high risk of poor treatment compliance or for not completing the study.

Date of first enrolment
28/10/2026

Date of final enrolment
18/08/2029

Locations

Countries of recruitment
United Kingdom

England

Belgium

Italy

Netherlands

Spain

Study participating centre
John Radcliffe Hospital
Headley Way
Headington
Oxford
England
OX3 9DU

Study participating centre
Great Ormond Street Hospital for Children
Great Ormond Street
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WC1N 3JH

Study participating centre
Royal Victoria Infirmary
Queen Victoria Road
Newcastle upon Tyne
England
NE1 4LP

Study participating centre
Alder Hey Children's Hospital
Eaton Road

West Derby
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L12 2AP

Study participating centre
Leeds General Infirmary
Great George Street
Leeds
England
LS1 3EX

Sponsor information

Organisation
Entrada Therapeutics, Inc

Funder(s)

Funder type

Funder Name
Entrada Therapeutics

Alternative Name(s)
Entrada Therapeutics, Inc.

Funding Body Type
Government organisation

Funding Body Subtype
For-profit companies (industry)

Location
United States of America

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not expected to be made available