



Entrada Therapeutics Announces Positive Results from the First Cohort of Participants with Duchenne Muscular Dystrophy (DMD) in the Phase 1/2 ELEVATE-45-201 Study of ENTR-601-45

October 14, 2026

-- Cohort 1 met the primary objectives of favorable safety and tolerability at the initial dose of 5 mg/kg --

-- ENTR-601-45 plasma exposures were consistent with those predicted based on juvenile non-human primate exposure data --

-- In line with expectations, the secondary objectives demonstrated a 0.10% mean increase in dystrophin production versus baseline in treated participants at the initial dose of 5 mg/kg --

-- Company expects a significant increase in dystrophin production and functional benefit in future cohorts while maintaining a convenient Q6W dosing schedule --

-- ELEVATE-45-201 Cohort 2 enrollment is complete and the Company is on track to report data at the increased dose of 10 mg/kg in the first half of 2027 --

BOSTON, Oct. 14, 2026 (GLOBE NEWSWIRE) -- Entrada Therapeutics, Inc. (Nasdaq: TRDA) today announced positive topline data from Cohort 1 of the double-blind, placebo-controlled, multiple ascending dose (MAD) portion of the Phase 1/2 ELEVATE-45-201 clinical study of ENTR-601-45 in ambulatory participants with a confirmed mutation in the DMD gene amenable to exon 45 skipping.

Study participants in Cohort 1 received three doses of 5 mg/kg of ENTR-601-45 or placebo. Topline results met the primary objectives of favorable safety and tolerability. The secondary objectives also met Company expectations and demonstrated a 0.10% mean increase in dystrophin and a 0.15% mean increase in exon skipping compared with baseline levels in treated participants. All study participants in Cohort 1 have now progressed to the open-label, Phase 2 portion of the study, where they will receive six doses of 5 mg/kg of ENTR-601-45.

"Establishing a favorable safety profile in Cohort 1 was our main objective and we are pleased with the outcome. These positive safety results, combined with an increase in dystrophin across treated participants at the initial low dose of 5 mg/kg, are promising. Importantly, ENTR-601-45 is designed to be dosed once every six weeks. For families currently managing a weekly infusion schedule, ENTR-601-45's current dosing schedule translates to a potential therapy built around approximately nine administrations per year instead of more than fifty," said Natarajan Sethuraman, PhD, President of Research and Development at Entrada Therapeutics. "Additionally, an Independent Data Monitoring Committee's recent recommendation to proceed to the highest dose of 18 mg/kg in Cohort 3 of the ELEVATE-44-201 clinical study supports our confidence in the opportunity to escalate the dose up to 15 mg/kg in Cohort 3 of ELEVATE-45-201, where we expect to see meaningfully higher exposure, dystrophin production and functional benefit."

"People with a confirmed mutation in the DMD gene who are amenable to exon 45 skipping represent a significant portion of the DMD patient population. As one of the most advanced next-generation exon skipping conjugate PMOs in clinical trials, ENTR-601-45 has the potential to offer a well-tolerated, low-frequency of dosing option for a large Duchenne subpopulation with a clear and increasing unmet need," said Professor Giovanni Baranello, MD, PhD, Professor of Paediatric Neuromuscular Disorders at UCL and Honorary Consultant in Paediatric Neuromuscular Diseases at the Great Ormond Street Hospital, London, and principal investigator in the ELEVATE-45-201 clinical study. "Importantly, establishing a favorable safety profile at this initial dose in a three dose MAD study is the essential foundation for escalating to higher doses, and seeing any dystrophin produced in participants this young, with such low baseline levels, is an encouraging early signal."

Highlights from the topline results of Cohort 1 of ELEVATE-45-201 include:

Baseline Characteristics	Placebo n=2	ENTR-601-45 5 mg/kg n=6
Age, mean, years	9.0	6.3
Body mass index, mean, kg/m ²	19.75	16.62
Age at diagnosis, mean, years	4.0	1.9
Corticosteroid use, n (%)	2 (100%)	6 (100%)
Ambulatory, n (%)	2 (100%)	6 (100%)
Baseline dystrophin, mean, %	0.34%	0.49%

Dystrophin measured by western blot and normalized to MHC; Dystrophin values are actual observed values, including values <LLOQ; Two participants in the ENTR-601-45 group had baseline dystrophin levels <LLOQ and >LOD; LLOQ for dystrophin was 0.25%; LOD for dystrophin was 0.1%; Biopsy performed at baseline; LLOQ: Lower limit of quantification; LOD: Limit of detection; MHC: Myosin heavy chain.

Safety, Tolerability and Pharmacokinetics

- Favorable safety and tolerability with ENTR-601-45 at the 5 mg/kg dose
 - Headache (5 events in 2 participants) was the most common treatment-emergent adverse event (TEAE)
 - One participant experienced 2 serious TEAEs (vomiting and pyrexia) which subsequently resolved
 - There were no study discontinuations due to any cause, and all participants enrolled in the open-label extension
- No hypomagnesemia or renal safety concerns were noted
 - Renal markers, including eGFR and cystatin C, were within normal ranges in treated participants
 - Magnesium levels were also within the normal range
- Plasma ENTR-601-45 exposures were consistent with those predicted by juvenile non-human primate (NHP) data
 - Modeled plasma exposures provide confidence in a significant increase in dystrophin expression at higher doses in Cohort 2 (10 mg/kg) and Cohort 3 (up to 15 mg/kg)

Dystrophin Production, Exon Skipping and Functional Benefit

- A mean increase of 0.10% (n = 6, nominal $p = 0.03$) from baseline in dystrophin production was observed in study participants who received ENTR-601-45 versus a 0.02% mean decrease from baseline in study participants who received placebo
- A mean increase of 0.15% (n = 5, no p -value calculated) from baseline in exon skipping was observed in study participants who received ENTR-601-45 versus a 0.04% mean decrease from baseline in study participants who received placebo
- Based on the age of Cohort 1 participants and baseline levels of function, no observable functional benefit was expected or observed after three doses of ENTR-601-45
- Functional impact will be assessed with higher doses and over longer dosing periods via future cohorts and the open-label studies

About the ELEVATE-45-201 Phase 1/2 Study

ELEVATE-45-201 is a global, two-part, randomized, double-blind, placebo-controlled Phase 1/2 study evaluating the safety, tolerability and effectiveness of ENTR-601-45 in ambulatory participants aged four to 20 with Duchenne who are amenable to exon 45 skipping. The multiple ascending dose (MAD) Part A portion of the study is evaluating the safety, pharmacokinetics, pharmacodynamics and functional parameters following intravenous administration of ENTR-601-45 to study participants in three cohorts at sites in the U.K. and EU. Cohort 1 of the MAD portion of the study enrolled eight participants with Duchenne. They were randomized 3:1 to receive ENTR-601-45 at a dose of 5 mg/kg or placebo, administered intravenously. During this double-blind period, doses were administered on days one, 43 and 85, and muscle biopsies were performed at the time of screening and at six weeks after the last dose. Following the initial three doses administered in Part A, all participants continued into the Phase 2, open-label portion in which the safety and efficacy of ENTR-601-45 are evaluated over a longer period of time. Cohort 2 enrollment is complete and dosing at 10 mg/kg is ongoing. The Company is on track to report data from Cohort 2 (10 mg/kg) in the first half of 2027, with data from Cohort 3 (up to 15 mg/kg) to follow.

About ENTR-601-45

ENTR-601-45 is a proprietary Endosomal Escape Vehicle (EEV™)-conjugated oligonucleotide that has a sequence designed and optimized for people with a confirmed mutation in the *DMD* gene who are amenable to exon 45 skipping. This population comprises approximately nine percent of the Duchenne patient population globally. These patients face an aggressive disease course, with a median age at loss of ambulation of between 11 and 14 years among those receiving standard-of-care treatment. ENTR-601-45 is an investigational therapy being evaluated for its potential to restore the mRNA reading frame and allow for the translation of dystrophin protein that is slightly shortened but still functional.

Patients and Their Care Partners

Patients and their care partners are a critical part of our community, and we are committed to keeping them informed and connected. To receive community updates in real time and read today's update, please visit [Community Updates](#) on our corporate website.

About Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is a rare disease caused by mutations in the *DMD* gene, which encodes the dystrophin protein. These mutations lead to inadequate dystrophin production. Dystrophin is essential to maintaining the structural integrity and function of muscle cells. Lack of functional dystrophin leads to progressive loss of muscle strength, impacting mobility and causing heart or respiratory complications that contribute to high mortality rates. An estimated 41,000 people in the U.S. and Europe are living with DMD. Of those, approximately 11,500 are amenable to skipping of exon 44, 45, 50, or 51.

About Entrada Therapeutics

Entrada Therapeutics is a clinical-stage biopharmaceutical company aiming to transform the lives of patients by establishing a new class of genetic medicines that engage intracellular targets that have long been considered inaccessible. Through proprietary, versatile and modular approaches, Entrada is advancing a robust development portfolio of genetic medicines for the potential treatment of neuromuscular and inherited retinal diseases, among others. The Company's lead oligonucleotide programs are in development for the potential treatment of people living with Duchenne muscular dystrophy who are exon 44, 45, 50 and 51 skipping amenable. Entrada has partnered to develop a clinical-stage program, VX-670, for myotonic dystrophy type 1.

For more information about Entrada, please visit our website, www.entradatx.com, and follow us on [LinkedIn](#).

Forward-Looking Statements

This press release contains express and implied forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release, including statements regarding Entrada's strategy, future operations, prospects and plans, objectives of management, the validation and differentiation of Entrada's approach and EEV platform and its ability to provide a potential treatment for patients, expectations regarding Entrada's Phase 1/2 MAD clinical study of ENTR-601-45, including the timing of data from Cohort 2 in the first half of 2027 with data from Cohort 3 to follow, a significant increase in dystrophin production and functional benefit in future cohorts, the potential for dosing up to 15 mg/kg in Cohort 3 to lead to meaningfully higher exposure, dystrophin production and functional benefit and the ability of the MAD Part A portion of the study to evaluate the safety, pharmacokinetics, pharmacodynamics and functional parameters of ENTR-601-45, expectations regarding ENTR-601-45, including the potential to restore the mRNA reading frame and allow for the translation of dystrophin protein that is slightly shortened but still functional and the potential for a dosing schedule built around approximately nine administrations a year, the potential therapeutic benefits of Entrada's EEV product candidates, including the potential for ENTR-601-45 to be a transformative treatment option, the continued development and advancement of ENTR-601-44, ENTR-601-45, ENTR-601-50, and ENTR-601-51 for the treatment of DMD and the partnered product candidate VX-670 for the potential treatment of DM1, the ability to continue to expand and develop additional therapeutic programs and modalities, including the potential treatment of neuromuscular and inherited retinal diseases, constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Entrada may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the conduct of research activities and the initiation and completion of preclinical studies and clinical studies; uncertainties as to the availability and timing of results from preclinical and clinical studies; the timing of and Entrada's ability to submit and obtain regulatory clearance and initiate clinical studies; whether results from preclinical studies or clinical studies will be predictive of the results of later preclinical studies and clinical studies; whether Entrada's cash resources will be sufficient to fund the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; as well as the risks and uncertainties identified in Entrada's filings with the Securities and Exchange Commission (SEC), including the Company's most recent Form 10-K and in subsequent filings Entrada may make with the SEC. In addition, the forward-looking statements included in this press release represent Entrada's views as of the date of this press release. Entrada anticipates that subsequent events and developments will cause its views to change. However, while Entrada may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Entrada's views as of any date subsequent to the date of this press release.

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