Entrada Therapeutics Announces Positive Topline Results from Cohort 1 of Participants with Duchenne Muscular Dystrophy Treated with ENTR-601-44 in Phase 1/2 ELEVATE-44-201 Study

May 7, 2026

-- Achieved the primary objective with favorable safety and tolerability, no discontinuations and no serious adverse events --

-- Markers of kidney function via eGFR, Cystatin C and magnesium were all within normal ranges and comparable to placebo --

-- Observed lower plasma exposure in Cohort 1 participants who are all between six and 17 years of age when compared with healthy adult volunteers; A similar trend was seen between recently received juvenile and adult NHP PK data --

-- Consequently, Cohort 1 demonstrated an increase of 2.36% in dystrophin over a baseline of 4.00% and an increase of 2.31% in exon skipping over a baseline of 2.66% in treated participants --

-- Statistically significant and potentially differentiated improvement in treated participants versus placebo in Time to Rise velocity, a clinically validated functional measurement --

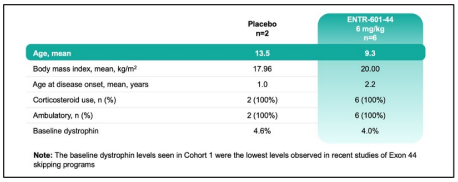
-- Company's updated PK modeling predicts Cohort 2, building upon Cohort 1 data and combined with the recently received juvenile NHP data, will result in a significant increase of plasma AUC and substantially higher dystrophin levels with continued benefit in muscle function --

-- Company has initiated dosing of ELEVATE-44-201 Cohort 2 at the increased dose of 12 mg/kg and is on track to report data by year-end 2026 --

-- Entrada to host investor webcast and conference call today, Thursday, May 7, at 8:30 a.m. ET --

BOSTON, May 07, 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-44-201 clinical study. ELEVATE-44-201 is a clinical study of ENTR-601-44 in ambulatory participants ages four to 20 with a confirmed mutation in the *DMD* gene amenable to exon 44 skipping. Study participants in Cohort 1 were randomized 3:1 to receive three doses of 6 mg/kg of ENTR-601-44, the lead investigational product in Entrada's Duchenne muscular dystrophy (DMD) franchise, or placebo. Muscle biopsies were performed at the time of screening and six weeks after the last dose.

Table 1

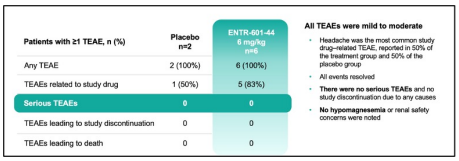


	Placebo n=2	ENTR-601-44 6 mg/kg n=6
Age, mean	13.5	9.3
Body mass index, mean, kg/m²	17.96	20.00
Age at disease onset, mean, years	1.0	2.2
Corticosteroid use, n (%)	2 (100%)	6 (100%)
Ambulatory, n (%)	2 (100%)	6 (100%)
Baseline dystrophin	4.6%	4.0%

Note: The baseline dystrophin levels seen in Cohort 1 were the lowest levels observed in recent studies of Exon 44 skipping programs

Demographics and baseline characteristics

Table 2



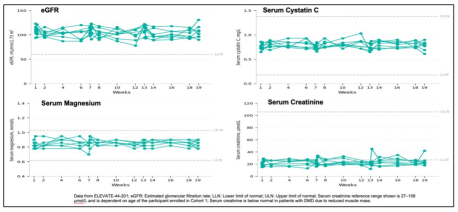
Patients with ≥1 TEAE, n (%)	Placebo n=2	ENTR-601-44 6 mg/kg n=6
Any TEAE	2 (100%)	6 (100%)
TEAEs related to study drug	1 (50%)	5 (83%)
Serious TEAEs	0	0
TEAEs leading to study discontinuation	0	0
TEAEs leading to death	0	0

All TEAEs were mild to moderate

- Headache was the most common study drug–related TEAE, reported in 50% of the treatment group and 50% of the placebo group
- All events resolved
- **There were no serious TEAEs** and no study discontinuation due to any causes
- **No hypomagnesemia** or renal safety concerns were noted

Adverse events: All TEAEs were mild to moderate

Table 3



Renal markers were within normal range and comparable to placebo (Participant-level data)

The average age of treated participants in the Cohort 1 study was 9.3 years old with a mean age of disease onset of 2.2 years. Per protocol, all participants were ambulatory and all were on a stable dose of steroids. Baseline dystrophin in both the placebo and treatment population was also lower than that reported in competitive exon 44 skipping clinical studies. This is also notable, as treatment response generally correlates with higher baseline dystrophin levels.

Table 1: Demographics and baseline characteristics

	Placebo n=2	ENTR-601-44 6 mg/kg n=6
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Baseline dystrophin	4.6%	4.0%

Note: The baseline dystrophin levels seen in Cohort 1 were the lowest levels observed in recent studies of Exon 44 skipping programs

The results demonstrated a favorable safety and tolerability profile with no reported serious adverse events (SAEs) and no adverse events (AEs) leading to discontinuation from the study. Markers of kidney function were normal.

“The first dosing cohort readout from ELEVATE-44-201 is a major step forward, showing that ENTR-601-44 has a strong safety profile and is driving important, clinically meaningful and potentially differentiated early functional benefits. We were very encouraged to see that the Cohort 1 data delivered statistically significant improvement in Time to Rise velocity across participants treated with ENTR-601-44. TTR velocity is an approvable clinical endpoint in Phase 3 studies and importantly, ENTR-601-44’s TTR velocity data are compelling and we believe differentiated,” said Dipal Doshi, Chief Executive Officer at Entrada Therapeutics. “We were initially surprised to see a significant difference in the pharmacokinetics between juveniles and adults; however the consistency seen between our recently received juvenile nonhuman primate (NHP) data and the Cohort 1 participant data explains these differences. This clear explanation gives us confidence that we will see higher plasma exposure, which we expect will drive continued functional responses in Cohort 2. We are incredibly grateful to those living with Duchenne, their care partners and the study investigators and personnel who are taking part in our clinical study.”

All study participants in Cohort 1 have now progressed to the open-label, Phase 2 portion of the study, where they will receive six additional doses of 6 mg/kg of ENTR-601-44. Additional study participants are now being dosed in Cohort 2, in which they will receive three doses of 12 mg/kg of ENTR-601-44 or placebo. The Company expects to report results from the Cohort 1 open-label study and Cohort 2 MAD study by year-end 2026, with data from Cohort 3 (up to 18 mg/kg) to follow.

Natarajan Sethuraman, PhD, President of R&D at Entrada Therapeutics, said, “We believe we have a highly differentiated delivery mechanism, including the ability to access quiescent satellite cells. Access to satellite cells enables the repair of existing muscle fibers and importantly, the formation of new healthy fibers. These drivers are emerging as a potentially significant competitive differentiator and may explain why the dystrophin levels in Cohort 1 were sufficient to improve TTR velocity.”

Dr. Sethuraman further commented, “Safety and functional benefit are at the forefront of consideration for patients. Our Cohort 1 data show that ENTR-601-44 is safe at the 6 mg/kg dose and is promoting early functional benefit which represent an important point of differentiation versus other approved and investigational approaches. We look forward to seeing the results from our Cohort 1 open-label and Cohort 2 MAD study later this year.”

Highlights from the topline results of Cohort 1 ELEVATE-44-201 include:

Safety and tolerability

- Favorable safety and tolerability with ENTR-601-44 at the 6 mg/kg dose.
- All treatment emergent adverse events (TEAEs) were mild to moderate.
- No reported SAEs and no AEs leading to discontinuation from the study.
- The most common AE was headache.
- Markers of kidney function including eGFR, Cystatin C and magnesium were within normal ranges and comparable to placebo.
- There were no discontinuations and all eight Cohort 1 participants have transitioned to the open-label portion of the study.

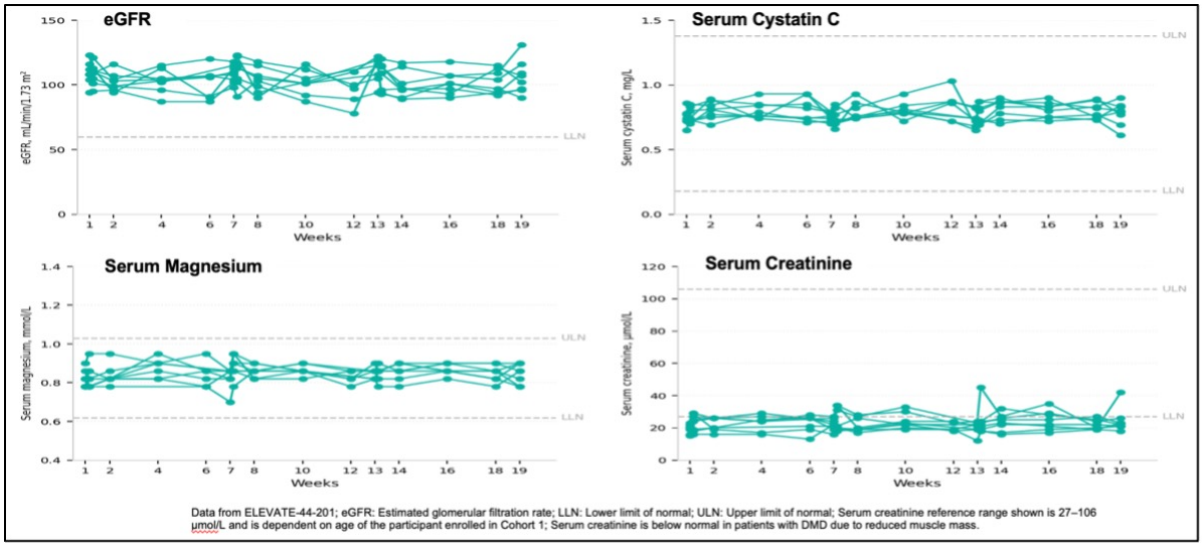
Table 2: Adverse events: All TEAEs were mild to moderate

Patients with ≥1 TEAE, n (%)	Placebo n=2	ENTR-601-44 6 mg/kg n=6
Any TEAE	2 (100%)	6 (100%)
TEAEs related to study drug	1 (50%)	5 (83%)
Serious TEAEs	**0**	**0**
TEAEs leading to study discontinuation	0	0
TEAEs leading to death	0	0

All TEAEs were mild to moderate

- Headache was the most common study drug–related TEAE, reported in 50% of the treatment group and 50% of the placebo group
- All events resolved
- **There were no serious TEAEs** and no study discontinuation due to any causes
- **No hypomagnesemia** or renal safety concerns were noted

Table 3: Renal markers were within normal range and comparable to placebo (Participant-level data)



Pharmacokinetics
Consistent with the recently received data in juvenile NHPs, the Company observed a lower-than-expected plasma C_{max} and AUC (area under the curve) in pediatric DMD participants when compared with that seen in healthy adult volunteers and the adult NHPs. The levels observed in Cohort 1 were in line with the exposures observed in juvenile NHPs, thus providing confidence on future modeling of exposure. Updated modeling, following review of the juvenile NHP data, suggest that the AUC will significantly increase in Cohort 2, resulting in higher muscle concentration, exon skipping and dystrophin production. The DMD community at large continues to learn about the biology of Duchenne and the relationship between dystrophin and functional benefit. Despite the drug plasma concentration and dystrophin levels, the Company obtained earlier-than-expected functional responses that were both statistically significant and clinically meaningful.

Efficacy and functional improvement
Mean change in TTR velocity is a robust, low variability measure which carries the largest absolute and proportional annual signal and is used as an early prognostic factor for disease progression and loss of ambulation. Cohort 1 results demonstrated a statistically significant improvement in mean TTR velocity in treated versus placebo participants ($p<.05$, *post hoc* analysis) which was 3.5 times higher than the minimal clinically important difference (MCID) threshold of 0.023, suggesting ENTR-601-44 is potentially changing the trajectory of the disease.

Positive change in TTR velocity was seen across the majority of participants, irrespective of their severity of disease or age, which likely supports that Cohort 1's functional benefit represents a true drug-related effect.

Further, the end of Cohort 1 dystrophin levels correlated with the end of Cohort 1 TTR velocity improvement, suggesting that dystrophin production in both damaged muscle fibers and activated satellite cells may have crossed a critical threshold for functional improvement.

Importantly, a majority of participants on treatment achieved functional benefit.

- In ELEVATE-44-201 Cohort 1, a statistically significant change from baseline in TTR velocity was observed:
  - Mean change in TTR velocity versus placebo of 0.115.
  - Mean change in TTR velocity for the treatment group of 0.08.
- Demonstrated 2.36% increase in dystrophin over 4.00% baseline in treated participants.
- Demonstrated 2.31% increase in exon skipping over 2.66% baseline in treated participants.

"The topline results from Cohort 1 of the ELEVATE-44-201 study are promising. Individuals with Duchenne are in urgent need of new treatments that can provide functional improvements while also offering a more targeted treatment option," said Dr. Laurent Servais, Professor of Paediatric Neuromuscular Diseases at the University of Oxford and principal investigator in the ELEVATE-44-201 clinical study. "For those living with Duchenne, a therapy that could lead to both near- and long-term improvements in functional outcomes would be an important breakthrough. I am excited to be a part of this clinical study and look forward to the data from Cohort 2."

Investor Webcast and Conference Call Information
Entrada Therapeutics will host an investor webcast and conference call today, Thursday, May 7, 2026, at 8:30 a.m. ET to discuss the topline results from Cohort 1 of the Phase 1/2 ELEVATE-44-201 study. The webcast can be accessed by visiting the Investor Relations section of the Company's website at www.entradatx.com. Analysts planning to participate during the Q&A portion of the live call can join the conference call at the audio-conferencing link here. The webcast will be archived and available for replay on the Entrada Therapeutics website for 90 days following the call.

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 the ELEVATE-44-201 Phase 1/2 Study
ELEVATE-44-201 is a global, two-part, randomized, double-blind, placebo-controlled Phase 1/2 study evaluating the safety, tolerability and effectiveness of ENTR-601-44 in ambulatory participants ages four to 20 with Duchenne who are exon 44 skipping amenable. 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-601-44 to study participants in three cohorts at sites in the U.K. and EU. The Cohort 1 MAD portion of the study enrolled eight participants ages six to 17 with Duchenne. They were randomized 3:1 to receive ENTR-601-44 at a dose of 6 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-601-44 are evaluated over a longer period of time.

About ENTR-601-44
ENTR-601-44 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 that is amenable to exon 44 skipping, which comprises approximately eight percent of the Duchenne patient population globally. ENTR-601-44 is designed to address the underlying cause of Duchenne, facilitating production of functional dystrophin from the endogenous (naturally occurring) *DMD* gene.

In December 2025, the U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease Designation to ENTR-601-44.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (DMD) is a rare disease caused by mutations in the *DMD* gene, which encodes for 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 Duchenne. Of those, 14,000 are amenable to exon 44, 45, 50 and 51 skipping.

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, the timing of data from Entrada's Phase 1/2 MAD clinical study of ENTR-601-44, including the Cohort 1 open-label study and Cohort 2 MAD by year-end 2026 with data from Cohort 3 to follow, the ability to recruit for and complete the global Phase 2 clinical study for ENTR-601-44, the potential of TTR velocity data observed in Cohort 1 to predict clinically meaningful and potentially differentiated early functional benefits, expectations regarding the Cohort 1 open-label portion of ENTR-601-44, expectations regarding Cohort 2 of ENTR-601-44, including the potential for higher plasma concentrations with a significant increase in plasma exposure, higher muscle concentrations, exon skipping, dystrophin production and substantially higher dystrophin levels, a deepening of functional responses, the potential for further enhanced muscle function, and continued functional benefit, expectations regarding planned Cohort 3 of Entrada's ELEVATE-44-201 study, the potential therapeutic benefits of Entrada's EEV product candidates, including the potential for ENTR-601-44 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 further exon skipping programs and 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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Photos accompanying this announcement are available at:

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