



## Entrada Therapeutics Announces FDA Removal of Clinical Hold on ENTR-601-44

February 24, 2025

- Receives FDA authorization to initiate ELEVATE-44-102, a Phase 1b multiple ascending dose clinical study of ENTR-601-44 in adults living with Duchenne muscular dystrophy –
- Follows recently received Medicines and Healthcare Products Regulatory Agency authorization in the United Kingdom for ELEVATE-44-201 –
- Expands global ELEVATE-44 clinical program to include pediatric, adult, ambulatory and non-ambulatory patients with Duchenne muscular dystrophy –

BOSTON, Feb. 24, 2025 (GLOBE NEWSWIRE) -- Entrada Therapeutics, Inc. (Nasdaq: TRDA) today announced that the United States Food and Drug Administration (FDA) has lifted the clinical hold on ENTR-601-44 and provided authorization to initiate ELEVATE-44-102, a Phase 1b multiple ascending dose (MAD) clinical study of ENTR-601-44 for the potential treatment of Duchenne muscular dystrophy (DMD) in adult patients with a confirmed mutation in the *DMD* gene amenable to exon 44 skipping. The global ELEVATE-44 program, which includes the ELEVATE-44-102 study in the U.S. and ELEVATE-44-201 outside of the U.S., will cover a broad population of patients with DMD and support evaluation of ENTR-601-44 in patients with both early and advanced disease.

“Given the strength of our safety and target engagement data from our Phase 1 clinical study and the profound unmet need in adults living with Duchenne, we are pleased to have obtained FDA clearance for the ELEVATE-44-102 study,” said Dipal Doshi, CEO of Entrada Therapeutics. “The study will help assess the potential of ENTR-601-44 in both non-ambulatory and ambulatory adult patients who are unfortunately often left out of clinical studies due to the advanced stage of their disease. Nearly half of those living with Duchenne who are amenable to exon 44 skipping are adults. ELEVATE-44-102 will provide clinical experience from this important population for our growing data package in support of what we believe will be a best-in-class therapy.”

ELEVATE-44-102 is a randomized, double-blind placebo-controlled Phase 1b study evaluating the safety and tolerability of ENTR-601-44 in approximately 32 non-ambulatory and ambulatory adult patients with DMD who are exon 44 skipping amenable. The MAD study is also designed to evaluate target engagement as measured by exon skipping and dystrophin production, and pharmacokinetics. Dosing will be administered every six weeks, with the planned doses across four cohorts anticipated to range from 0.16 mg/kg up to 1.28 mg/kg. Study participants may be eligible to enter an open label extension study (OLE), in which the safety, efficacy and tolerability of ENTR-601-44 will be evaluated over a longer period of time. The Company plans to initiate study enrollment in the first half of 2026, which would enable a seamless transition into an OLE.

### About ENTR-601-44

ENTR-601-44, a proprietary Endosomal Escape Vehicle (EEV™)-conjugated phosphorodiamidate morpholino oligomer (PMO), is the lead product candidate within Entrada’s Duchenne muscular dystrophy franchise from its growing pipeline of EEV-therapeutics. Each EEV-PMO therapeutic candidate has an oligonucleotide sequence designed and optimized for the specific subpopulation of interest. ENTR-601-44 is designed to address the underlying cause of Duchenne due to mutated or missing exons in the *DMD* gene. ENTR-601-44, an investigational therapy for the potential treatment of people living with Duchenne who are exon 44 skipping amenable, is 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.

### About Duchenne Muscular Dystrophy (DMD)

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 have Duchenne.

### About Entrada Therapeutics

Entrada Therapeutics is a clinical-stage biopharmaceutical company aiming to transform the lives of patients by establishing a new class of medicines that engage intracellular targets that have long been considered inaccessible. The Company’s Endosomal Escape Vehicle (EEV™)-therapeutics are designed to enable the efficient intracellular delivery of a wide range of therapeutics into a variety of organs and tissues, resulting in an improved therapeutic index. Through this proprietary, versatile and modular approach, Entrada is advancing a robust development portfolio of RNA-, antibody- and enzyme-based programs for the potential treatment of neuromuscular, ocular, metabolic and immunological diseases, among others. The Company’s lead oligonucleotide programs are in development for the potential treatment of people living with Duchenne 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](http://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 planned ELEVATE-44-102 randomized, double-blind placebo-controlled Phase 1b study, including its initiation in the U.S. in the first half of 2026, expectations about the planned dosing levels of the planned ELEVATE-44-102 study, the ability to recruit for, enroll and complete the ELEVATE-44-102 study, the anticipated number of patients to be enrolled in the ELEVATE-44-102 study, the potential of Entrada’s EEV product candidates, including the potential for ENTR-601-44 to be a transformative treatment option and best-in-class therapy, and the continued development and advancement of ENTR-601-44 for the potential treatment of DMD, 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 trials; 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 trials; whether results from preclinical studies or clinical trials will be predictive of the results of later preclinical studies and clinical trials; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2024 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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