Entrada Therapeutics Receives Authorization in the United Kingdom to Initiate ELEVATE-44-201, a Phase 1/2 Multiple Ascending Dose Clinical Study of ENTR-601-44 in Patients Living with Duchenne Muscular Dystrophy

February 3, 2025

– Company on track to initiate ELEVATE-44-201 in Q2 2025 –

– ENTR-601-44 regulatory filings submitted in additional geographies including the U.S. and EU, with regulatory discussions ongoing –

BOSTON, Feb. 03, 2025 (GLOBE NEWSWIRE) -- Entrada Therapeutics, Inc. (Nasdaq: TRDA) today announced it had received authorization from the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA) and Research Ethics Committee for its Clinical Trial of an Investigational Medicinal Product to initiate ELEVATE-44-201, a Phase 1/2 multiple ascending dose (MAD) clinical study of ENTR-601-44 for the potential treatment of Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation in the *DMD* gene amenable to exon 44 skipping.

"The clearance by the MHRA marks a new phase in Entrada's growth and, most importantly, moves us closer to realizing our commitment to families living with Duchenne muscular dystrophy," said Dipal Doshi, Chief Executive Officer of Entrada Therapeutics. "As the first authorization for our global MAD clinical study of ENTR-601-44 in patients, we are pleased to be initiating the study at what we believe to be an effective therapeutic dose. This is even more important since families living with Duchenne do not have time on their side as the progressive decline in function profoundly impacts the quality of life for patients and their care partners. It is this urgency that drives our work each day."

"The MHRA authorization of ELEVATE-44-201 is an exciting development in the clinical progress of ENTR-601-44, a new and very encouraging treatment option for boys and young men living with Duchenne muscular dystrophy who are exon 44 skipping amenable," said Francesco Muntoni, MD, Professor of Paediatric Neurology. "There is a significant unmet medical need in this population, with limited therapeutic options available. The unique properties of Entrada's EEV-therapeutic candidates offer the potential to provide tangible benefits for people with this life-shortening disease."

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 patients with DMD who are exon 44 skipping amenable. Part A is a multiple ascending dose study designed to evaluate the safety, pharmacokinetics, and pharmacodynamics, including exon skipping and dystrophin production in approximately 24 patients. Dosing will be administered every six weeks, with the planned doses across three cohorts anticipated to range from 6 mg/kg up to 18 mg/kg. Part B of the study is designed to further evaluate the optimal dose established in Part A for safety and efficacy, including patient reported outcomes and quality of life measures. 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 is on track to initiate ELEVATE-44-201 in Q2 2025.

The MHRA authorization follows the completion of a Phase 1 clinical study to evaluate the safety and tolerability of a single dose of ENTR-601-44. This study demonstrated ENTR-601-44 was generally well-tolerated in healthy volunteers with no serious adverse events, no drug-related adverse events and no clinically significant changes or trends noted in vital signs, electrocardiograms, physical exams or laboratory assessments. The study also demonstrated significant plasma concentration, muscle concentration and exon skipping.

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 and 50 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 planned Phase 1/2 multiple ascending dose clinical study ENTR-601-44, including its initiation in the United Kingdom in Q2 2025, the expectations about the planned dosing levels of the planned Phase 1/2 trial for ENTR-601-44 and their efficacy, the ability to recruit for and complete a global Phase 1/2 trial for ENTR-601-44 and the anticipated number of patients to be enrolled, the potential of Entrada's EEV product candidates, including the potential for ENTR-601-44 to be a transformative treatment option, 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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