



Entrada Therapeutics Announces Collaboration with the Myotonic Dystrophy Clinical Research Network to Study the Natural History of Myotonic Dystrophy Type 1

August 4, 2022

The natural history study will generate data to further the ongoing efforts of the myotonic dystrophy research community to understand disease progression and identify potential clinical outcome measures and endpoints for clinical trials

Entrada recently announced new preclinical data that supports further development of the Company's second clinical candidate, ENTR-701, for the potential treatment of myotonic dystrophy type 1

BOSTON, Aug. 04, 2022 (GLOBE NEWSWIRE) -- Entrada Therapeutics, Inc. (Nasdaq: TRDA), a biopharmaceutical company aiming to transform the lives of patients by establishing intracellular Endosomal Escape Vehicle (EEV™) therapeutics as a new class of medicines, today announced a new collaboration with the Myotonic Dystrophy Clinical Research Network (DMCRN) supporting END-DM1 (Establishing Biomarkers and Clinical Endpoints in Myotonic Dystrophy Type 1). END-DM1 is a natural history study to advance the understanding of disease progression in patients with myotonic dystrophy type 1 (DM1). This is a non-interventional study designed and conducted by the DMCRN, a network of medical centers with expertise in DM1 clinical care and research that is collecting data to support future clinical trials of potential therapies for DM1.

"Entrada is developing new therapeutic options that will potentially transform the treatment of devastating diseases and make a meaningful difference in the lives of patients living with myotonic dystrophy type 1, a disease where there are currently no approved therapies," said Nerissa Kreher, MD, Chief Medical Officer of Entrada Therapeutics. "Partnering with the DM1 community through our support of the END-DM1 study will provide important data regarding biomarkers and clinical endpoints in DM1, informing the design of our future clinical trials and the ongoing development of potential DM1 treatments."

Entrada's Endosomal Escape Vehicle (EEV™)-conjugated phosphorodiamidate morpholino oligomer (PMO) is designed to enable the efficient intracellular delivery of a wide range of therapeutics, previously considered inaccessible and undruggable, into a variety of organs and tissues. Entrada's EEV-PMO uses an allele-specific approach that sterically blocks the triplet repeats in the mRNA that sequesters these critical proteins in order to restore muscle function. At the [TIDES USA 2022: Oligonucleotide & Peptide Therapeutics Conference](#), Entrada's clinical candidate ENTR-701 for DM1 was supported by new preclinical data indicating prolonged splicing correction in the tibialis anterior, triceps and quadriceps, and amelioration of myotonia in a DM1 mouse model following a single dose.

"We are excited to have Entrada join the DMCRN collaboration and participate in our shared mission to transform the lives of people affected by neuromuscular diseases through the study of DM1," said [Nicholas E. Johnson](#), M.D., MSCI, FAAN, an associate professor, division chief of neuromuscular medicine and vice chair of research in the [Department of Neurology](#) at [Virginia Commonwealth University's School of Medicine](#), who serves as co-chair of the END-DM1 Study. "The data collected through the natural history study, END-DM1, and associated pilot studies, will provide critical knowledge for DM1 researchers and Entrada to successfully design DM1 clinical trials and potentially provide much needed treatment options for patients and families living with this serious condition."

About Myotonic Dystrophy Type 1 (DM1)

Myotonic dystrophy type 1 is a genetic, multi-systemic neuromuscular disease that causes progressive muscle loss and weakness, leading to physical impairment, activity limitations and decreased participation in social activities and work. DM1 is an autosomal dominant disease caused by a mutation to the *dystrophia myotonica protein kinase* gene, which leads to toxic mRNA that sequesters and reduces function of the muscle blind-like proteins. This causes mis-regulation of multiple RNA splicing events that are correlated with DM1 symptoms, which include muscle weakness, myotonia, cardiac conduction abnormalities, respiratory muscle impairment, gastrointestinal complications and fatigue. DM1 can occur at any age, however, classic disease onset typically occurs in the 20s and 30s and life expectancy can range from 45 to 60 years, with 70% of early mortality caused by cardiac or respiratory complications. There are currently no approved therapies for people living with DM1. Instead, treatment is focused largely on symptom management.

About ENTR-701

ENTR-701, a proprietary Endosomal Escape Vehicle (EEV™)-conjugated phosphorodiamidate morpholino oligomer, is the second novel clinical candidate from Entrada's growing pipeline of EEV therapeutics. ENTR-701 is designed to address the underlying cause of myotonic dystrophy type 1 through allele-specific targeting and blocking of the excess repeat-containing transcripts in dystrophia myotonica protein kinase mRNA. In doing so, ENTR-701 has the potential to restore the function of muscle blind-like proteins, correct the mis-splicing and aberrant expression of downstream transcripts and restore normal muscle function. Data from preclinical studies of ENTR-701 suggest correction of disease relevant biomarkers in various muscle groups. Entrada expects to file an IND application with the U.S. FDA for ENTR-701 in 2023.

About Entrada Therapeutics

Entrada Therapeutics is a biopharmaceutical company aiming to transform the lives of patients by establishing a new class of medicines, Endosomal Escape Vehicle (EEV™) therapeutics, to engage intracellular targets that have long been considered inaccessible and undruggable. The Company's EEV therapeutics are designed to enable the efficient intracellular delivery of a wide range of therapeutics into a variety of organs and tissues with an improved therapeutic index. Through its proprietary, highly versatile and modular EEV platform, Entrada is building a robust development portfolio of oligonucleotide-, antibody- and enzyme-based programs for the potential treatment of neuromuscular diseases, immunology, oncology and diseases of the central nervous system. The Company's lead oligonucleotide programs include ENTR-601-44 targeting Duchenne muscular dystrophy (DMD) and ENTR-701 targeting myotonic dystrophy type 1 (DM1).

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

Forward-Looking Statements

This press release contains 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, prospects and plans, the continued development of ENTR-701, the timing of Entrada's planned regulatory filings regarding ENTR-701, expectations regarding the preclinical data of ENTR-701 and the related potential for development, the continued development and advancement of ENTR-701 for the treatment of DM1, and the potential therapeutic benefits of its EEV candidates 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 studies; the timing of and Entrada's ability to submit and obtain regulatory clearance for IND applications and initiate clinical trials; whether results from preclinical studies 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; uncertainties associated with the impact of the ongoing COVID-19 pandemic on Entrada's business and operations; 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.

Entrada Investor/Media Contact

Karla MacDonald

Chief Corporate Affairs Officer

kmacdonald@entradatx.com