



Vertex and Entrada Therapeutics Establish Collaboration to Discover and Develop Endosomal Escape Vehicle (EEV) Therapeutics for Myotonic Dystrophy Type 1 (DM1)

December 8, 2022

- Entrada to receive \$224 million upfront payment and \$26 million equity investment, as well as potential milestone payments and royalties -
- Global collaboration includes ENTR-701, Entrada's EEV-investigational candidate for the treatment of DM1 -

BOSTON--(BUSINESS WIRE)--Dec. 8, 2022-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) and [Entrada Therapeutics, Inc.](#) (Nasdaq: TRDA) today announced a global collaboration focused on discovering and developing intracellular Endosomal Escape Vehicle (EEV™) therapeutics for myotonic dystrophy type 1 (DM1). The collaboration includes Entrada's program for DM1, ENTR-701, which is in late-stage preclinical development.

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"Vertex's strategy is to discover and develop transformative medicines for people with serious diseases, and DM1 has therefore been a disease area of interest to Vertex for some time," said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research, and Chief Scientific Officer of Vertex. "Entrada's innovative EEV approach, the significant progress in their DM1 program, and the potential for it to reach the clinic in the near-term hold exciting potential for patients. Working together, we believe we have the opportunity to develop a transformative treatment for this devastating disease."

"Our collaboration with Vertex represents an important step for Entrada as we work to make intracellular therapeutics a reality through our novel EEV approach," said Dipal Doshi, President and Chief Executive Officer of Entrada Therapeutics. "DM1 is a progressive disease with no treatment options available. Working with Vertex will enable us to expeditiously move this program forward, while focusing the majority of our internal resources on advancing new therapeutic options for patients living with Duchenne and expanding our commitment to non-neuromuscular disease programs."

Transaction Terms

Under the terms of the agreement, Entrada will receive an upfront payment of \$224 million, as well as an equity investment of \$26 million. Entrada is eligible to receive up to \$485 million for the successful achievement of certain research, development, regulatory and commercial milestones, and tiered royalties on future net sales for any products that may result from this collaboration agreement.

The agreement includes a four-year global research collaboration whereby Entrada will continue to advance and receive payments for certain research activities related to ENTR-701, as well as additional DM1-related research activities. Vertex will be responsible for global development, manufacturing and commercialization of ENTR-701 and any additional programs stemming from Entrada's DM1 research efforts.

The collaboration is subject to review under the Hart-Scott-Rodino Antitrust Improvements Act.

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 of the disease-associated trinucleotide repeats in dystrophin myotonia protein kinase transcripts. 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.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust clinical pipeline of investigational small molecule, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 13 consecutive years on Science magazine's Top Employers list and one of Fortune's Best Workplaces in Biotechnology and Pharmaceuticals and Best Workplaces for Women. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

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, resulting in 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](#).

Vertex Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements by Dr. David Altshuler and Dipal Doshi in this press release, statements about the terms of and expectations for Vertex's collaboration with Entrada, the expected advancement of ENTR-701 and potential benefits and results that may be achieved through the collaboration, statements regarding the future activities of the parties pursuant to the collaboration, and statements regarding upfront and milestone payments, potential royalties on future sales and anticipated equity investment. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the collaboration is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, that the anticipated benefits and potential of Vertex's collaboration with Entrada may not be achieved on the anticipated timeline, or at all, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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Entrada Special Note Regarding 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 related to the potential benefits and results that may be achieved through Entrada's collaboration with Vertex, the ability of Entrada and Vertex to complete the proposed collaboration, including the parties' ability to satisfy the conditions to the consummation of the offer contemplated thereby and the other conditions set forth in the collaboration agreement, the expected timetable for completing the transaction, the anticipated advancement of Entrada's DM1 program, Entrada's strategy, future operations, prospects and plans, objectives of management, the potential therapeutic benefits of its EEV candidates, and expectations regarding the Company's therapeutic candidates, including ENTR-701, its related potential for the continued development and advancement for the treatment of DM1, ENTR-601-44 targeting Duchenne muscular dystrophy (DMD), ENTR-701 targeting myotonic dystrophy type 1 (DM1) and non-neuromuscular programs 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 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; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; uncertainties associated with the impact of the ongoing COVID-19 pandemic on Entrada's business and operations; risks related to obtaining the requisite consents to the transaction, including, without limitation, the timing, including possible delays, and receipt of clearance under the Hart-Scott-Rodino Antitrust Improvements Act; 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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