Entrada Therapeutics Announces ENTR-501 for the Treatment of Mitochondrial Neurogastrointestinal Encephalomyopathy (MNGIE)

January 8, 2020

BOSTON, Mass., January. 8, 2020 – Entrada Therapeutics, Inc. a biotechnology company dedicated to transforming the treatment of devastating diseases through the intracellular delivery of biologics, today announced that it is developing ENTR-501, a novel thymidine phosphorylase enzyme replacement therapy, for the treatment of mitochondrial neurogastrointestinal encephalomyopathy (MNGIE).

“MNGIE is a rare, autosomal recessive disorder that results in a clinically distinct combination of severe gastrointestinal and neurological manifestations caused by the systemic accumulation of toxic metabolites in the affected tissues,” said Michio Hirano, M.D., Chief of the Division of Neuromuscular Medicine in the Department of Neurology at Columbia University Vagelos College of Physicians and Surgeons. “There is a critical need for a therapeutic option that addresses the underlying thymidine phosphorylase deficiency in MNGIE patients.”

ENTR-501 is a recombinant form of the thymidine phosphorylase enzyme engineered using Entrada’s proprietary Endosomal Escape Vehicle (EEV) technology and is designed to replace deficient thymidine phosphorylase in MNGIE patients. Entrada’s EEV technology enables the functional intracellular delivery of biologics like thymidine phosphorylase by facilitating improved cellular uptake and efficient endosomal escape. Animal studies with ENTR-501 have demonstrated sustained normalization of toxic metabolites.

Dipal Doshi, President and Chief Executive Officer of Entrada, stated, “The need for a therapy that will address the underlying cause of MNGIE is clear to anyone who has seen the debilitating effects of this disease. We are proud to work with Dr. Hirano and the MNGIE patient community. We believe that success with ENTR-501, our most advanced program in a rapidly expanding portfolio, will provide support for the clinical potential of our overall intracellular biologics platform. We expect that our EEV technology will allow us to develop intracellular biologics for a wide range of patients who currently have limited or no treatment options.”

To support the development of ENTR-501 and to better understand this fatal disease, Entrada is planning an international, multi-center MNGIE natural history study to be led by Dr. Hirano. Dr. Hirano presented preclinical data from murine model studies that demonstrate the potential of ENTR-501 to treat the underlying cause of MNGIE at Mitochondrial Medicine 2019, which took place December 11-13 in Cambridge, U.K. Entrada provides financial support to and sponsors the preclinical research conducted in Dr. Hirano’s lab at Columbia.

About MNGIE

MNGIE is a rare, multisystem mitochondrial disease caused by a genetic deficiency of the thymidine phosphorylase enzyme. MNGIE primarily affects the digestive and nervous systems and is characterized by severe gastrointestinal dysmotility, pronounced weight and muscle loss, and neurological symptoms including ptosis and peripheral neuropathy. MNGIE is often misdiagnosed for years as the disease can be mistaken for other illnesses such as neuropathy, anorexia, Crohn’s disease, or irritable bowel syndrome. In MNGIE patients, the symptoms are relentless, leading to significant morbidity and early mortality. There are currently no approved therapies for MNGIE.

About Entrada Therapeutics

Entrada Therapeutics’ mission is to treat devastating diseases through the intracellular delivery of biologics. Entrada’s EEV technology enables the efficient intracellular delivery of proteins, peptides, and nucleic acids, thus allowing for the development of programs across several intracellular target classes. The Company’s novel approach addresses current challenges associated with both large and small molecule therapeutics and represents a fundamental advancement in the delivery of molecules into the cell. For more information, please visit www.entradatx.com.

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