



Translate Bio Announces Oral Presentation of mRNA Therapeutics in Models of Metabolic Disorders at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

April 29, 2019

-- Preclinical results demonstrate successful delivery of lipid-nanoparticle encapsulated mRNA in relevant metabolic disease models --

LEXINGTON, Mass., April 29, 2019 (GLOBE NEWSWIRE) -- Translate Bio, Inc. (Nasdaq: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction, today presented preclinical results that demonstrate the potential of mRNA therapeutics as treatments for urea cycle disorders (UCDs) and organic acidemias (OAs). The oral presentation, entitled *Treatment of Metabolic Disorders Using Lipid Nanoparticle (LNP)-Encapsulated Messenger RNA Therapeutics (MRT)*, was given by Frank DeRosa, Translate Bio's senior vice president of research and process development, at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting on April 29, 2019 in Washington, D.C.

"We have engineered our mRNA therapeutic (MRT) platform to deliver mRNA that codes for the missing enzymes in several metabolic disorders," said Dr. DeRosa. "These data demonstrate the potential broad applicability of the MRT platform to treat various genetic disorders using our proprietary liver delivery technology."

The presentation summarizes findings from experiments in three mouse models of metabolic disorders including ornithine transcarbamylase (OTC) deficiency, argininosuccinate synthetase (ASS1) deficiency (citrullinemia) and methylmalonic acidemia (MMA), which are each caused by a deficiency of a single enzyme specific for each disease. Messenger RNA encoding human OTC, human ASS1 or human methylmalonyl-CoA mutase enzymes were synthesized, incorporated in lipid nanoparticles (LNPs) and formulated for intravenous (IV) administration. Data from these studies demonstrated that the mRNA encapsulated in an LNP can be successfully delivered to the liver by IV administration, resulting in the production of the desired enzyme. In each disease model, the enzyme was functional as seen by the reduction, prevention or normalization of the metabolic abnormalities characteristic of these three disorders.

Inherited metabolic disorders are a class of rare diseases with high unmet medical need. Three such diseases were addressed in the ASGCT presentation. OTC deficiency and citrullinemia are inherited UCDs that cause toxic levels of ammonia to accumulate in the blood. MMA is an inherited OA disorder that disrupts normal amino acid metabolism and causes buildup of toxic levels of methylmalonic acid in the blood. High blood levels of these substances can lead to neurological conditions.

The full abstract #26 can be found on the ASGCT conference website at https://www.asgct.org/global/documents/asgct19_abstracts_final.

About Translate Bio

Translate Bio is a clinical-stage mRNA therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction. The Company's MRT platform is designed to develop product candidates that deliver mRNA carrying instructions to produce intracellular, transmembrane and secreted proteins for therapeutic benefit. Translate Bio believes that its MRT platform is applicable to a broad range of diseases caused by insufficient protein production or where production of proteins can modify disease, including diseases that affect the lung, liver, eye, central nervous system and lymphatic system. The Company also believes its MRT platform may be applied to various classes of treatments, such as therapeutic antibodies or vaccines in areas such as infectious disease and oncology. Translate Bio's two lead programs are being developed as treatments for cystic fibrosis (CF) and ornithine transcarbamylase (OTC) deficiency. For more information about the Company, please visit www.translate.bio or on Twitter at @TranslateBio.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include, but are not limited to, those regarding: the potential for mRNA therapeutics to treat metabolic disorders; the potential benefits of mRNA therapy; Translate Bio's beliefs regarding the broad applicability of its MRT platform; and Translate Bio's plans, strategies and prospects for its business, including its lead development programs. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs, including but not limited to: Translate Bio's ability to advance the development of its platform and programs under the timelines it projects, demonstrate the requisite safety and efficacy of its product candidates and replicate in clinical trials any positive findings from preclinical studies; the content and timing of decisions made by the U.S. Food and Drug Administration, other regulatory authorities and investigational review boards at clinical trial sites, including as it relates to ongoing and planned clinical trials; Translate Bio's ability to obtain, maintain and enforce necessary patent and other intellectual property protection (for example, two of our patents issued in Europe are under opposition); the availability of significant cash required to fund operations; competitive factors; general economic and market conditions and other important risk factors set forth under the caption "Risk Factors" in Translate Bio's Annual Report on Form 10-K for the year ended December 31, 2018 filed with the Securities and Exchange Commission on March 21, 2019, and in any other subsequent filings made by Translate Bio. Any forward-looking statements contained in this press release speak only as of the date hereof, and Translate Bio specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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