Translate Bio Announces Publication of Preclinical Data Demonstrating Efficacy of Systemic mRNA Delivery in Fabry Disease Model in the Journal Molecular Therapy

March 18, 2019

LEXINGTON, Mass., March 18, 2019 (GLOBE NEWSWIRE) -- Translate Bio (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 announced the publication of preclinical results demonstrating efficacy in a mouse model of Fabry disease using a lipid nanoparticle (LNP) formulation for systemic delivery of mRNA derived from the Company's mRNA therapeutic (MRT) platform. Fabry disease is a rare inherited disorder caused by a deficiency of α-galactosidase A. The disease is progressive, destructive and potentially life-threatening.

The paper, published in the journal *Molecular Therapy* and titled *Improved Efficacy in a Fabry Disease Model Using a Systemic mRNA Liver Depot System as Compared to Enzyme Replacement Therapy*, reported the sustained expression of human α-galactosidase (GLA) protein via LNP-formulated mRNA in mice and non-human primates. The results also demonstrate the efficacy of this approach through reduction of a clinically relevant biomarker in a mouse model of Fabry disease. The article was published online and will appear in the April 10, 2019 issue of the journal.

"This research demonstrates the ability of our mRNA, delivered via LNP, to target the liver directly, resulting in the potential for an improved therapeutic profile compared with conventional treatment for this disease," said Michael Heartlein, chief scientific officer, Translate Bio. "This publication is representative of our proof-of-concept, foundational research that has since enabled our ongoing next-generation mRNA design and delivery efforts for our liver-targeted programs."

Heartlein continued, "The sustained expression of the GLA enzyme that we observed further supports our belief that the liver is a factory for the production of protein and the prolonged expression of that protein."

In the study, multi-component lipid nanoparticles were formulated for delivery of mRNA encoding human GLA protein. Upon delivery of human GLA mRNA to mice, serum GLA protein levels reached as high as ~1,330-fold over normal physiological values. Additionally, treatment with mRNA therapy was compared with conventional enzyme replacement therapy (ERT) in the mouse model of Fabry disease. Results demonstrated increased protein levels in the key organs affected by the disease with mRNA therapy compared to ERT. These higher protein levels translated into a greater reduction in two clinically relevant biomarkers using mRNA therapy.

About Fabry Disease
Fabry disease is a rare inherited lysosomal storage disorder caused by deficiency of α-galactosidase A, an enzyme needed to break down a fatty substance in the body called globotriaosylceramide. When this substance accumulates in the body's cells, the resulting cell damage can cause a range of mild to severe symptoms including life-threatening complications such as kidney failure, heart attacks and strokes, often at a young age. Fabry disease occurs in all racial and ethnic populations, though males are typically more severely affected than females.

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 ability of Translate Bio's mRNA to target the liver directly; the potential benefits of mRNA therapy, including in the treatment of Fabry disease; Translate Bio's beliefs regarding the liver's ability to produce the GLA protein; 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; 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2018 filed with the Securities and Exchange Commission on November 8, 2018 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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Source: Translate Bio, Inc.