Translate Bio to Present Preclinical Data Supporting MRT5005 at the 32nd Annual North American Cystic Fibrosis Conference

October 18, 2018

-- Findings support ongoing Phase 1/2 study of once-weekly nebulized MRT5005 in adults with cystic fibrosis --

LEXINGTON, Mass., Oct. 18, 2018 (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 announced the presentation of preclinical data for MRT5005, a first-in-class mRNA therapeutic designed to treat all patients with cystic fibrosis regardless of mutation. The presentation includes data demonstrating that MRT5005 was efficiently delivered to the lungs of rodents and non-human primates by nebulization, resulting in functional cystic fibrosis transmembrane conductance regulator (CFTR) protein expression. These data will be featured in a poster presentation at the 32nd Annual North American Cystic Fibrosis Conference (NACFC) taking place in Denver, Colorado, from October 18-20, 2018.

“MRT5005 is engineered to deliver mRNA encoding fully functional CFTR protein to the lung epithelial cells through nebulization. These data demonstrate MRT5005’s ability to reach the lungs in animal studies, including in a disease model with increased mucus accumulation, resulting in functional protein expression,” said Dr. Ann Barbier, Chief Medical Officer of Translate Bio. “These studies support our ongoing Phase 1/2 clinical trial and demonstrate the potential to bring clinically meaningful benefit to all patients with cystic fibrosis, including in patients with little or no CFTR expression.”

Details of the thematic poster session are as follows:
Poster Title*: In Vitro* and *In Vivo* Evaluation of an mRNA Therapeutic (MRT) for the Treatment of Patients with Cystic Fibrosis (CF)
Thematic Poster Session:*TPS02--CFTR: Novel Therapeutic Approaches to Treat CFTR Defects*
Presenting Author: Ann Barbier, MD, PhD, Chief Medical Officer
Date and Time: October 18, 2018, 2:00 PM - 3:25 PM MST
Location: 702
Abstract number: 281
Dr. Barbier will also present the poster during the NACFC poster session on October 18 from 11:15 AM – 1:30 PM MST in the NACFC Exchange.

Preclinical study data

- Findings will be presented from functional experiments demonstrating the ability of MRT5005 to restore chloride ion channel activity in cells lacking CFTR as well as in a CFTR knockout animal model when compared to control conditions. Importantly, the increase in nasal potential difference observed in the knockout rat model indicate MRT5005 can cross the mucus layer and produce functional human CFTR (hCFTR) protein in nasal epithelia *in vivo*.

- Additional experiments show robust dose-dependent delivery of codon-optimized hCFTR mRNA with increased levels compared to endogenous CFTR mRNA in both rats (up to 1000-fold) and non-human primates (up to 1500-fold) after a single dose. Subsequent hCFTR protein production was observed by immunohistochemical staining with dose-dependent staining intensity that generally reflected the mRNA levels that were measured. Widespread CFTR protein expression was observed throughout the upper and lower airways after a single dose in both species. Importantly, at the highest doses, the observed levels of mRNA and expressed hCFTR were higher than the normal (endogenous) levels through 28 days after administration by nebulization.

- Successful repeat dosing experiments with MRT5005 was also conducted in rats and non-human primates. After five weekly nebulized treatments with MRT5005, the presence of hCFTR protein was observed 24 hours after the final dose as well as after the 28-day recovery period in both species. MRT5005 was well tolerated with no adverse effects seen in all doses tested.

The full abstract #281 can be found at
https://onlinelibrary.wiley.com/doi/epdf/10.1002/ppul.24152.

About MRT5005
MRT5005 is the first clinical-stage mRNA product candidate designed to address the underlying cause of cystic fibrosis (CF) by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to the lung epithelial cells through nebulization. Once the inhaled MRT5005 has entered the epithelial cells lining the patient’s lungs, the therapeutic mRNA uses the cells’ own machinery for translation and expression of fully functional CFTR protein. This treatment aims to restore the essential ion channel that is defective or absent in patients with CF, addressing the pathology of CF directly regardless of genetic mutation. In 2015, the U.S. Food and Drug Administration granted orphan drug designation to MRT5005 for the treatment of CF.

About Cystic Fibrosis
Cystic fibrosis (CF) is the most common fatal inherited disease in the United States, affecting more than 30,000 patients in the U.S. and more than 70,000 patients worldwide. CF is caused by genetic mutations that result in dysfunctional or absent CFTR protein. This defect causes mucus buildup in the lungs, pancreas and other organs. Mortality is primarily driven by a progressive decline in lung function. According to the Cystic Fibrosis Foundation, the median age at death for patients with CF was 29.6 years in 2016. There is no cure for CF. CFTR modulators that are currently

marketed or in clinical development are effective only in patients with specific mutations, and patients still experience pulmonary exacerbations and a progressive decline in lung function, which represents a significant unmet need.

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, lymphatic system and circulatory system. The Company also believes its platform may be applied to produce therapeutic antibodies and 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 MRT5005 to address the underlying cause of CF, the ability of MRT5005 to deliver mRNA encoding fully functional CFTR protein to the lung and to treat all patients with CF, regardless of the underlying genetic mutation, and the success of Translate Bio's ongoing Phase 1/2 clinical trial of MRT5005. 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; Translate Bio's ability to enroll patients in its ongoing clinical trial; 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; 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 June 30, 2018 filed with the Securities and Exchange Commission on August 9, 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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