Translate Bio Highlights mRNA Platform Potential for the Treatment of Cystic Fibrosis (CF) at the 34th Annual North American Cystic Fibrosis Conference

October 7, 2020

-- Lead candidate MRT5005 leverages Translate Bio's proprietary LNP and mRNA platform and is the only clinical-stage mRNA therapeutic in development for the treatment of CF --

-- Second CF program, next-generation CFTR mRNA, applies advances in mRNA science, LNP chemistry and CFTR biology --

-- mRNA capabilities continue to expand to address unmet needs in CF and other pulmonary diseases --

LEXINGTON, Mass., Oct. 07, 2020 (GLOBE NEWSWIRE) -- Translate Bio (Nasdaq: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company developing a new class of potentially transformative medicines to treat or prevent debilitating or life-threatening diseases, today announced upcoming oral and poster presentations at the 34th Annual North American Cystic Fibrosis Conference (NACFC) taking place virtually from October 21 to 23, 2020. Beginning today, presentation recordings and posters are available on the NACFC platform to registered attendees.

In an oral presentation entitled, "Delivering CFTR mRNA: An Inhaled, Mutation-agnostic Approach to Treat CF," the Company discusses the potential of nucleic acid therapies to treat the underlying cause of cystic fibrosis (CF), particularly through the promise of messenger RNA (mRNA) therapeutics. The presentation underscores the remaining unmet need in treating CF and provides an overview of the development of its lead clinical candidate, MRT5005, currently being evaluated in a Phase 1/2 clinical trial in people with CF.

In addition, the oral presentation and a poster presentation entitled, "Design of Next Generation CFTR mRNA Therapeutics (MRT) for the Treatment of Patients with Cystic Fibrosis," summarize preclinical research related to the design of the Company's next-generation CF program. Both presentations describe advancements in the Company's mRNA technology as applied to the treatment of CF, including ideal target profile and preclinical data demonstrating that the design of novel mRNA sequences encoding rationally engineered CFTR protein produced highly active ion channels and enhanced protein expression when compared to a reference sequence encoding the native protein *in vitro*. The presentations also highlight the potential benefits of a next-generation cystic fibrosis transmembrane conductance regulator (CFTR) mRNA which may include lower dosing requirements and/or reduced administration time. Preclinical studies are ongoing to select the optimal mRNA sequence and lipid nanoparticle (LNP) for Translate Bio's next generation CFTR mRNA product.

"We are steadfast in our commitment to the CF community, especially those without any efficient, corrective treatment, and we believe that mRNA therapeutics have the potential to benefit all people with CF, regardless of underlying genetic mutation," said Richard Wooster, chief scientific officer at Translate Bio. "Based on our technological leadership in mRNA research and a strong understanding of CFTR biology, we are excited to advance both our lead clinical candidate, MRT5005, and our next-generation CFTR program."

The NACFC presentations highlight multiple innovations across the Company's mRNA therapeutic platform (MRT TM) involved in advancing a next-generation CFTR program including:

- Optimization of mRNA sequences and improvements in the amount of protein produced per unit of mRNA delivered;
- Deep evaluation of rationally engineered CFTR proteins with potential to increase the activity of the CFTR ion channel; and
- Incorporation of novel proprietary lipids for inhaled delivery.

Oral Presentation

Title:	S02.4: Delivering CFTR mRNA: An Inhaled, Mutation-agnostic Approach to Treat CF
Session:	S02--PTC-APP&D-NT: Novel Nucleic Acid Strategies to Treat the Fundamental CF Defect
Date and time:	October 21: 10:00 am to 11:20 am ET Livestream Symposium; 2:15 p.m. ET Q&A session

Poster Presentation

Title:	Design of Next Generation CFTR mRNA Therapeutics (MRT) for the Treatment of Patients with Cystic Fibrosis
Poster number:	515
Date:	October 7

The NACFC portal can be accessed here. Links to the poster and oral presentation will be available on Translate Bio's investor website on October 21.

About MRT5005
MRT5005 is the first clinical-stage mRNA product candidate designed to address the underlying cause of CF by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to the lung epithelial cells through nebulization. MRT5005 is being developed to treat all patients with CF, regardless of the underlying genetic mutation, including those with limited or no CFTR protein. The U.S. Food and Drug Administration (FDA) has granted MRT5005 Orphan Drug, Fast Track and Rare Pediatric Disease designation.

About the MRT5005 Phase 1/2 Clinical Trial
The randomized, double-blind, placebo-controlled Phase 1/2 clinical trial of MRT5005 is designed to enroll at least 40 adult patients with CF who have two Class I and/or Class II mutations. The primary endpoint of the trial will be the safety and tolerability of single and multiple escalating doses of

MRT5005 administered by nebulization. Percent predicted forced expiratory volume in one second (ppFEV1), which is a well-defined and accepted endpoint measuring lung function, will also be measured at pre-defined timepoints throughout the trial. The Phase 1/2 clinical trial of MRT5005 for the treatment of CF is being conducted in collaboration with the Cystic Fibrosis Foundation Therapeutics Development Network and the Emily's Entourage Patient Registry. In July 2019, the Company announced interim results from the single-ascending dose (SAD) portion of its Phase 1/2 clinical trial of MRT5005 in patients with CF. For more information about the Phase 1/2 clinical trial, visit https://clinicaltrials.gov/ct2/show/NCT03375047.

About Cystic Fibrosis

Cystic fibrosis 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 30.8 years in 2018. There is no cure for CF. Currently marketed CFTR modulators 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, or to prevent infectious diseases by generating protective immunity.Translate Bio is primarily focused on applying its technology to treat pulmonary diseases caused by insufficient protein production or where the reduction of proteins can modify disease. Translate Bio's lead pulmonary candidate is being evaluated as an inhaled treatment for cystic fibrosis (CF) in a Phase 1/2 clinical trial. Additional pulmonary diseases are being evaluated in discovery-stage research programs that utilize a proprietary lung delivery platform. Translate Bio believes that mRNA can be delivered to target tissues via multiple routes of administration and, consequently, its technology may apply broadly to a wide range of diseases, including diseases that affect the liver. Translate Bio is also pursuing the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur.

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 and benefit all patients, including the potential benefits of nucleic acid therapies and of a next-generation CFTR mRNA therapeutic; Translate Bio's plans to advance MRT5005 and its next-generation CFTR program; 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: the current and potential future impacts of the COVID-19 pandemic on the Company's business, financial condition, operations and liquidity; 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 successful advancement of the collaboration agreement between Translate Bio and Sanofi; uncertainties relating to the discovery and development of vaccine candidates based on mRNA, and specifically as it relates to the novel coronavirus, COVID-19; the content and timing of decisions made by the FDA, other regulatory authorities and investigational review boards at clinical trial sites, including decisions 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 June 30, 2020 filed with the Securities and Exchange Commission on August 6, 2020 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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