Translate Bio Receives FDA Fast Track Designation for MRT5005 for the Treatment of Cystic Fibrosis

February 26, 2020

-- First inhaled mRNA therapeutic remains on track to report results from its ongoing multiple-ascending dose (MAD) portion of Phase 1/2 clinical trial in the third quarter of 2020 --

LEXINGTON, Mass., Feb. 26, 2020 (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 that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for MRT5005 for the treatment of cystic fibrosis (CF). MRT5005, the first mRNA therapeutic with delivery to the lung, is designed to address the underlying cause of CF, regardless of genetic mutation, by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to cells in the lung through nebulization. The Phase 1/2 clinical trial of MRT5005 is currently ongoing.

"While there have been significant advances in the treatment of CF, substantial unmet need remains. Roughly 10 percent of the CF population is not anticipated to benefit from currently approved CFTR modulators," said Dr. Ann Barbier, chief medical officer, Translate Bio. "MRT5005 has the potential to treat all people with CF, including those with mutations that result in limited to no CFTR protein production. The Fast Track designation will help Translate Bio to expedite the clinical development of this potentially transformative therapeutic."

The FDA's Fast Track program facilitates the expedited development and review of drugs that are intended to treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs. Clinical development programs granted Fast Track designation may be eligible for several benefits, including more frequent meetings and communications with the FDA and, if relevant criteria are met, the potential for Accelerated Approval, Priority Review and Rolling Review of a Biologics License Application (BLA).

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 FDA has granted MRT5005 both Orphan Drug and Fast Track 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 ($ppFEV_1$), 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. In July 2019, the Company announced interim results from the single-ascending dose (SAD) portion of the 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. 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 is primarily focused on applying its MRT platform to treat pulmonary diseases caused by insufficient protein production or where production of proteins can modify disease. The Company also believes its technology is applicable to a broad range of diseases, including diseases that affect the liver, eye and central nervous system. Additionally, the 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 lead program is being developed as a treatment for cystic fibrosis (CF) and is in an ongoing Phase 1/2 clinical trial. 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: Translate Bio's expectations regarding the potential benefits of Fast Track designation by the FDA for MRT5005; the potential for MRT5005 to address the underlying cause of CF and benefit patients; and Translate Bio's plans to report data from its Phase 1/2 clinical trial of MRT5005 in the third quarter of 2020. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "forward", "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 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 September 30, 2019 filed with the Securities and Exchange Commission on November 6, 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.

Contacts for Translate Bio

Investors	**Media**
Teri Dahlman	Maura Gavaghan
tdahlman@translate.bio	mgavaghan@translate.bio
857-242-7792	857-242-7789

Source: Translate Bio, Inc.