Translate Bio Announces Timing of Interim Results from mRNA Therapeutic Clinical Trial in Patients with Cystic Fibrosis; Data Expected in Early Q2 2021

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-- MRT5005 is the first clinical-stage mRNA therapeutic with nebulized delivery to the lung and first to be administered in multiple doses for the treatment of a chronic genetic disease --

-- Completed enrollment and dosing for upcoming second interim data analysis; includes 8, 12 and 16 mg multiple-ascending dose groups and 20 mg single-ascending dose group --

LEXINGTON, Mass., Jan. 07, 2021 (GLOBE NEWSWIRE) -- Translate Bio (NASDAQ: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company, announced today that it has completed enrollment and dosing in the dose cohorts comprising the second interim data analysis from its Phase 1/2 clinical trial for MRT5005 in cystic fibrosis (CF). These include multiple-ascending dose (MAD) groups (8, 12 and 16 mg), and a 20 mg single-ascending dose (SAD) group. The Company anticipates reporting interim clinical data from these cohorts early in the second quarter of 2021.

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. Currently approved CFTR modulator treatments are specific to a patient's genetic mutation; therefore, there remains a significant unmet medical need for patients with CF who have genetic mutations non-amenable to these treatments or for patients who derive little therapeutic benefit from or are unable to tolerate these treatments.

Translate Bio previously reported the first interim analysis data from the Phase 1/2 clinical trial which included 8, 16 and 24 mg single-ascending dose groups.

"We are pleased to share the progress we have continued to make in this first-of-its-kind trial of an mRNA therapeutic, MRT5005, in people with cystic fibrosis—especially after having paused enrollment and dosing in response to the pandemic," said Dr.Ann Barbier, chief medical officer, Translate Bio. "Similar to the advances in mRNA vaccine development, we believe that mRNA-based therapeutics hold great potential to transform the treatment of many diseases caused by protein or gene dysfunction."

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 United Stated 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 8, 16 and 24 mg SAD groups of the Phase 1/2 clinical trial. A second interim analysis is anticipated early in the second quarter of 2021 and will include 8, 12 and 16 mg MAD groups and a 20 mg SAD group. The clinical trial continues to enroll and dose in the remaining dose groups, including a 20 mg MAD group and a daily dosing cohort. 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 32.4 years in 2019. 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.

Translate Bio 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 CF

patients; Translate Bio's plans to report interim data from its Phase 1/2 clinical trial for MRT5005 in CF in the second quarter of 2021; Translate Bio's plans to continue to enroll and dose patients in its Phase 12/clinical trial of MRT5005; the potential for mRNA-based therapeutics to apply to the treatment of many diseases caused by protein or gene dysfunction; 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 and continued development of mRNA vaccines for the treatment of infectious diseases. 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: the current and potential future impacts of the COVID-19 pandemic on Translate Bio's business, financial condition, operations and liquidity; Translate Bio's ability to advance the development of its platform and programs, including without limitation, 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 fiscal quarter ended September 30, 2020 filed with the Securities and Exchange Commission on November 5, 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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