Translate Bio Advances Programs in Cystic Fibrosis (CF) and Ornithine Transcarbamylase (OTC) Deficiency

January 3, 2019

-- Received approval to begin multiple-ascending dose in Phase 1/2 clinical trial of MRT5005, an mRNA therapeutic for CF --

-- Submitted IND for Phase 1/2 clinical trial of MRT5201, an mRNA therapeutic for OTC deficiency --

LEXINGTON, Mass., Jan. 03, 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 several updates relating to its two lead mRNA product candidates in development for the treatment of cystic fibrosis (CF) and ornithine transcarbamylase (OTC) deficiency.

Cystic Fibrosis and MRT5005: targeted delivery to the lung
A Phase 1/2 clinical trial of MRT5005, the Company's lead mRNA product candidate, is currently ongoing. MRT5005 is designed to treat patients with CF by addressing the underlying cause of the disease regardless of the genetic mutation. The clinical trial consists of both a single-ascending dose (SAD) and multiple-ascending dose (MAD) part. The updates from this program are as follows:

- The Company received approval from the Protocol Review Safety Committee to begin dosing in the MAD portion of the trial. This approval followed a review of the initial safety and tolerability data from the first dose level of the SAD portion of the trial;
- The Company continues to dose patients in the SAD portion of the trial and anticipates first patient dosing in MAD to begin in early 2019; and
- The Company anticipates reporting interim data from this trial in the second half of 2019.

OTC Deficiency and MRT5201: targeted delivery to the liver
The Company has advanced MRT5201, its second mRNA product candidate, towards the clinic. MRT5201 is designed to treat patients with OTC deficiency, the most common urea cycle disorder. The updates from this program are as follows:

- The Company submitted an Investigational New Drug (IND) Application to the U.S. Food and Drug Administration (FDA) to support the initiation of a Phase 1/2 clinical trial of MRT5201 in patients with OTC deficiency; and
- The Company anticipates initiating screening of patients with OTC deficiency for the Phase 1/2 clinical trial of MRT5201 in the first half of 2019.

"We are pleased to share that MRT5005, the first clinical-stage mRNA therapeutic with targeted delivery to the lung, is continuing to advance in our Phase 1/2 clinical trial and we look forward to the availability of interim data from this study this year," said Ronald Renaud, chief executive officer, Translate Bio. "Additionally, the IND submission for MRT5201 represents the first step towards advancing our second mRNA therapeutic into the clinic."

Renaud continued, "Our progress in both programs demonstrates the potential of mRNA as a therapeutic. In the treatment of genetic diseases, mRNA therapy represents a new approach - by giving the body instructions to produce its own functional protein, we are seeking to treat the underlying cause of the disease."

About MRT5005 and the Ongoing Phase 1/2 Clinical Trial
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. In 2015, the FDA granted orphan drug designation to MRT5005 for the treatment of CF.

The randomized, double-blind, placebo-controlled Phase 1/2 clinical trial of MRT5005 is designed to enroll at least 32 adult patients with CF who have at least one Class I or Class II mutation. The primary endpoint of the trial will be the safety and tolerability of single and multiple escalating doses of MRT5005 administered by nebulization. Forced expiratory volume in one second (FEV1), 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.

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 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 MRT5201
MRT5201 is designed to treat patients with OTC deficiency by intravenous delivery of mRNA encoding fully functional OTC enzyme to the liver to enable the hepatocytes, the predominant type of liver cell, to produce the normal OTC enzyme. MRT5201 has been granted orphan drug designation for the treatment of OTC deficiency in the U.S. and EU.

About OTC Deficiency
OTC deficiency is a metabolic liver enzyme disorder that results from a mutation in the OTC gene, and is the most common urea cycle disorder. The OTC enzyme is necessary for preventing the accumulation of ammonia, a normal byproduct of protein breakdown. When the enzyme is defective or absent, high levels of ammonia accumulate in the blood, which can cause serious and irreversible neurological damage. Based on published research, the incidence of OTC deficiency is estimated to be 1 in 56,500 live births in the United States. OTC deficiency is an X-chromosome-linked disease, and females are typically less severely affected than males.

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 and its MRT delivery systems are 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 and MRT delivery systems 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 potential for MRT5005 to address the underlying cause of CF, Translate Bio's plans to continue to dose patients in its SAD portion of its Phase 1/2 clinical trial of MRT5005 and to initiate the MAD dose part of the study in early 2019; the anticipated availability of the clinical data regarding MRT5005; the Company's plans to initiate screening of patients for the Phase 1/2 clinical trial of MRT5201; 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; Translate Bio's ability to enroll patients in its ongoing clinical trial and screen patients for the anticipated clinical trial for MRT5201; 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 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.

Contact for Translate Bio

Teri Dahlman
tdahlman@translate.bio
857-242-7792

Translate BIO

Source: Translate Bio, Inc.