Translate Bio Announces Pipeline Program Update

September 9, 2019

- Prioritizing and expanding pulmonary disease programs based on early positive cystic fibrosis (CF) data for MRT5005, the first inhaled mRNA therapeutic –

- Discontinuing development of MRT5201, a liver-targeted treatment for ornithine transcarbamylase (OTC) deficiency –

LEXINGTON, Mass. , Sept. 09, 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 that it is prioritizing the development of pulmonary disease programs including the ongoing development of MRT5005, its clinical candidate for the treatment of CF, as well as the evaluation of targets in additional pulmonary diseases. Additionally, the Company has decided to discontinue the development of MRT5201, a liver-targeted treatment for OTC deficiency.

The Company's prioritization of pulmonary diseases is supported by the previously reported positive single-ascending dose Phase 1/2 data from the CF program, which utilizes its proprietary lung delivery platform. MRT5005 is a first-in-class mRNA therapeutic 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 multiple-ascending dose portion of the clinical trial is ongoing with data expected in 2020. Preclinical research efforts are focused on additional pulmonary diseases, including primary ciliary dyskinesia (PCD), pulmonary arterial hypertension (PAH) and idiopathic pulmonary fibrosis (IPF).

The Company's decision to discontinue the development of MRT5201 for OTC deficiency is based on data from recently completed preclinical studies which do not support the desired pharmacokinetic and safety profile for advancement of the program. These data are related to the first-generation lipid nanoparticle (LNP) designed to be delivered to the liver via intravenous administration for the OTC deficiency program. As such, this LNP is different than that used in the CF and other pulmonary development programs which are designed to deliver the LNP-encapsulated mRNA through nebulization. Additionally, ongoing discovery efforts have generated promising, novel next-generation LNPs supporting the further development of liver disease mRNA therapeutics with potentially favorable product profiles.

"We believe that the success to date in our cystic fibrosis program positions us well to build on our lung delivery platform and maximize the potential of our mRNA technology in additional pulmonary diseases with unmet medical need," said Ronald Renaud, chief executive officer, Translate Bio. "With respect to our liver disease program, our goal is an optimal product profile with patient safety as our top priority. We look forward to further data from our next-generation delivery program to support that effort."

Renaud continued, "We are always evaluating various methods for the delivery of mRNA and we continue to believe that LNPs are currently the most promising technology with advantages over other approaches. We design each LNP with distinct chemical structures and formulations specific to the target organ and route of administration in order to optimize safety and potency. We are excited about the robust mRNA delivery effort underway at Translate Bio and we'll continue to apply our expertise in the development of novel lipids."

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 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 plans to prioritize the development of lung disease programs and evaluate targets in additional lung diseases; Translate Bio's expectations regarding its positioning to advance its programs and achieve its drug development goals; Translate Bio's expectations for data from the multiple ascending dose portion of its Phase 1/2 clinical trial of MRT5005 in 2020; its expectation regarding the potential benefits and advantages of its technology; 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: 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 U.S. Food and Drug Administration, other regulatory authorities and investigational review boards at clinical trial sites, including 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, 2019 and in any other subsequent filings made by Translate Bio with the Securities and Exchange Commission. 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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