



Translate Bio Resumes Enrollment and Dosing in Phase 1/2 Clinical Trial of MRT5005 in Cystic Fibrosis

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LEXINGTON, Mass., Sept. 08, 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 diseases caused by protein or gene dysfunction, today announced that enrollment and dosing in its Phase 1/2 clinical trial for MRT5005 in cystic fibrosis (CF) has resumed, with multiple clinical sites being open for enrollment. In March 2020, the Company had announced a pause to enrollment and dosing in the clinical trial in response to the COVID-19 pandemic. The Company plans to provide updated timing on the expected interim data readout of the additional single-ascending dose (SAD) group and the multiple-ascending dose (MAD) portion of the clinical trial at a later date.

"We are pleased that enrollment and dosing in the Phase 1/2 clinical trial of MRT5005 has resumed. Our top priority remains the safety of the patients and the health care providers involved with the study, and we continue to work together with the clinical trial sites as we navigate these unprecedented circumstances," said Dr. Ann Barbier, Chief Medical Officer of Translate Bio.

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 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 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. 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 mRNA therapeutic program is being developed as a treatment for cystic fibrosis (CF) and is in a Phase 1/2 clinical trial. The Company is also pursuing the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur. The Company also believes its technology is applicable to a broad range of diseases, including diseases that affect the liver. Additionally, the Company believes the platform has the potential to be applied to various classes of treatments, such as therapeutic antibodies for infectious disease and other diseases. 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 and benefit patients; Translate Bio's plans to update its timing to report data from the additional SAD dose group and MAD portion of the Phase 1/2 clinical trial of MRT5005; 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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