Translate Bio Announces Publication of Preclinical Results of COVID-19 mRNA Vaccine Candidate MRT5500 in npj Vaccines

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-- Multiple antigen constructs, including the construct used in MRT5500, induced potent neutralizing antibodies against SARS-CoV-2 in preclinical studies in multiple species --

-- Efficacy demonstrated in Syrian Golden Hamster challenge model under single and two-dose vaccination regimens of MRT5500 --

-- MRT5500 developed under a collaboration agreement with Sanofi Pasteur; Phase 1/2 clinical trial underway with clinical data anticipated in the third quarter of 2021 --

LEXINGTON, Mass., April 19, 2021 (GLOBE NEWSWIRE) -- Translate Bio (Nasdaq: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company, today announced the peer-reviewed publication of preclinical results of its COVID-19 vaccine candidate, MRT5500, in the journal *npj Vaccines*. Preclinical evaluation of MRT5500 demonstrated a favorable immune response profile against SARS-CoV-2 and conferred protective efficacy against the disease in an infection challenge model. The full journal article is available here. MRT5500 is being developed under a collaboration agreement between Sanofi Pasteur and Translate Bio. The Phase 1/2 clinical trial of MRT5500 began in March 2021 and clinical data is anticipated in the third quarter of this year.

Frank DeRosa, PhD, chief technology officer at Translate Bio and an author of the publication said, “These preclinical results demonstrated the ability of MRT5500 to elicit a robust immune response and protection against COVID-19 in multiple species through a highly rigorous set of studies. This growing body of preclinical data for MRT5500, as well as the new variant mRNA constructs that we are testing, support the potential for our mRNA vaccine candidates to play a role in protecting people against COVID-19. We look forward to seeing the results from the ongoing first-in-human trial.”

The publication outlines the main findings of the preclinical studies as follows:

Intracellular trafficking of mRNA-encoded target antigens demonstrated mutation dependence within the spike glycoprotein.

- Various constructs were evaluated across a number of studies to select a lead candidate including evaluation of expression and intracellular trafficking *in vitro*, as well as immunogenicity in mice and non-human primates (NHPs). The data demonstrated intracellular trafficking is construct dependent with unique trafficking observed when the expressed antigen contains furin-cleavage site mutations. These mutations can help define immunogenic responses as determined in both mouse and non-human primate studies measuring neutralizing antibody titers against SARS-CoV-2.
- In mice, MRT5500 (0.2, 1, 5 and 10 µg) induced dose-dependent binding antibodies and neutralizing antibodies specific to the SARS-CoV-2 spike (S) glycoprotein; neutralizing antibody titers were detected after one dose of MRT5500 in higher dose groups (5 µg, 10 µg), and were enhanced after a second dose at day 21.
- In NHPs, MRT5500 (15, 45 and 135 µg) induced antibodies reactive to recombinant S [protein] in nearly all NHPs; neutralizing antibody titers were detected after one dose of MRT5500 and were enhanced after a second dose at day 35. In NHPs, neutralizing antibody titers reached levels higher than those from human convalescent sera.

MRT5500 demonstrated protection against viral infection and disease progression.

- Syrian golden hamsters were immunized with MRT5500 (0.15, 1.5, 4.5 and 13.5 µg dose levels) with either a single immunization, or two administrations 21 days apart. MRT5500 demonstrated the ability to induce both humoral and cell-mediated antiviral responses and confer protection against a virus challenge in hamsters with all dose regimens, except the single 0.15 µg dose. Vaccination further resulted in protection from lung pathology and clearance of virus from the lungs as determined through viral subgenomic RNA measurements, thus supporting the further development of MRT5500 as a clinical candidate.

Data from MRT5500 indicated a low risk of vaccine-associated enhanced respiratory disease.

- Immunization with MRT5500 induced T_H1-biased responses in both mice and NHPs.

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 with a lead pulmonary candidate 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 also believes its technology may apply broadly to a wide range of diseases, including diseases that affect the liver. Additionally, the platform may be applied to various classes of treatments, such as therapeutic antibodies or protein degradation. Translate Bio is also pursuing the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur. 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 plans to report interim results from the Phase 1/2 clinical trial of MRT5500 in the third quarter of 2021; the potential ability of MRT5500 to elicit a robust immune response; the potential for MRT5500 to be a promising COVID-19 vaccine candidate and play a role in protecting people against COVID-19; the expected benefits of Translate Bio's collaboration with Sanofi; Translate Bio's beliefs regarding the broad applicability of its technology; 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 its vaccine development program generally and MRT5500 specifically, 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 COVID-19; 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 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2020 filed with the Securities and Exchange Commission on March 1, 2021 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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