Translate Bio Presents Preclinical Results in Primary Ciliary Dyskinesia (PCD) at American Thoracic Society (ATS) 2021 International Conference

May 18, 2021

– Positive results suggest potential for an mRNA-based therapeutic to correct ciliary function in patients with PCD due to DNAI1 mutations –

– Company expects to advance a lead PCD candidate into IND-enabling studies in 2H 2021 –

LEXINGTON, Mass., May 18, 2021 (GLOBE NEWSWIRE) -- Translate Bio (Nasdaq: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company, today presented positive results from a preclinical study of a novel mRNA-based therapeutic designed to treat the pulmonary component of primary ciliary dyskinesia (PCD), a rare genetic disease. Mutations in the genes that cause PCD result in ineffective mucociliary clearance which can lead to lung disease. Study results suggest that delivery of an mRNA-based therapeutic to the lungs can lead to the expression of DNAI1, which could potentially restore mucociliary clearance, the cellular mechanism of the disease. The data are being presented at the American Thoracic Society (ATS) 2021 International Conference and can also be viewed on the Company's website.

PCD is an autosomal recessive disease caused by a genetic mutation that leads to dysfunction of the cilia, which prevents proper mucociliary clearance from the lungs. PCD causes chronic inflammation and pulmonary infection risks which can lead to irreversible lung damage (bronchiectasis) in adulthood including severe impact on quality of life, potential for respiratory failure and, in some cases, the need for lung transplantation. There is no cure for PCD. Disease management is focused on relieving symptoms and slowing the progression of lung damage. While PCD can result from a mutation in one or more of 30+ genes involved in ciliary function, DNAI1 is one of the more frequently mutated genes, accounting for approximately 5–10 percent of diagnosed PCD cases.

Translate Bio designed multiple mRNA sequences to produce functional human DNAI1 using a codon optimization algorithm. The mRNA sequences were screened for DNAI1 protein expression and those sequences producing the highest DNAI1 levels were packaged into proprietary lipid nanoparticles (LNPs) designed for delivery to the lung via nebulization. Data presented include preclinical assessments of the level and distribution of protein expression as well as ciliary activity. Key findings from the presentation titled "A messenger RNA (mRNA)-based therapeutic designed to treat primary ciliary dyskinesia,"were as follows:

- Multiple iterations of codon optimization resulted in the selection of an mRNA sequence that had further improved protein expression by 30%;
- DNAI1 mRNA packaged in proprietary LNPs and delivered by an intratracheal or nebulized route of administration resulted in DNAI1 expression in multiciliated airway epithelial cells *in vivo*;
- *In vivo*, DNAI1 expression colocalized with a known ciliary protein along the length of the cilia, as required for restoration of function; and
- Repeat administration showed an increased number of DNAI1-expresssing cells and suggested a steady state of cilia restoration can be achieved.

"With no currently approved therapeutics to treat the underlying cause of PCD, our goal is to provide inhaled delivery of a potent mRNA-based treatment that will restore mucociliary clearance and prevent progressive lung pathology," said Richard Wooster, PhD, Chief Scientific Officer at Translate Bio. "This research suggests the potential for an mRNA therapeutic to correct ciliary function in people with PCD due to a mutation in the DNA1 gene. We look forward to advancing this program toward IND-enabling studies later this year."

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 also believes its technology is applicable to a broad 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 vaccines in areas such as infectious disease and oncology. For more information about the Company, please visit www.translate.bio or on Twitter at @TranslateBio.

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 an mRNA-based therapeutic to restore mucociliary clearance and correct cilia function in patients; the anticipated advancement of a lead PCD candidate into IND-enabling studies in 2H 2021; the goal to provide inhaled delivery of a potent mRNA-based treatment to restore mucociliary clearance and prevent progressive lung pathology; Translate Bio's beliefs regarding the broad applicability of its MRT platform; and Translate Bio's plans, strategies and prospects for its business. 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; 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 Quarterly Report on Form 10-Q for the three months ended March 31, 2021 filed with the Securities and Exchange Commission on May 6, 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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Source: Translate Bio, Inc.