Translate Bio Announces FDA Clearance to Proceed with a Single-ascending Dose (SAD) Phase 1/2 Clinical Trial for Ornithine Transcarbamylase (OTC) Deficiency

June 26, 2019

LEXINGTON, Mass., June 26, 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 has received clearance from the U.S. Food and Drug Administration (FDA) to proceed with a single-ascending dose (SAD) Phase 1/2 clinical trial of MRT5201 in adult patients with ornithine transcarbamylase (OTC) deficiency. MRT5201 is a first-in-class treatment designed to directly address the underlying cause of OTC deficiency by providing mRNA encoding the fully functional OTC enzyme in patients with the disease.

"We are very pleased to be able to move forward with this first-in-human clinical trial of MRT5201 in patients with OTC deficiency," said Ronald Renaud, chief executive officer, Translate Bio. "This represents a significant milestone for our team as we advance our second mRNA therapeutic for the treatment of a genetic disease into clinical development. Progressing this program underscores the potential application of our MRT platform in multiple disease areas."

As previously announced, in December 2018, the Company submitted an investigational new drug application (IND) for a SAD and multiple-ascending dose (MAD) Phase 1/2 study of MRT5201, which the FDA placed on clinical hold, pending additional preclinical toxicology data. After discussions with the FDA and after the Company amended the protocol, the FDA has removed the clinical hold and will allow the Company to move forward with a SAD clinical trial. Additional preclinical studies will be required to support future clinical development of MRT5201, including a MAD clinical trial. These additional preclinical studies are currently underway, and the Company plans to submit data from these studies to the FDA in the fourth quarter of 2019.

About MRT5201 and OTC Deficiency
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 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. 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. Based on published research, the incidence of OTC deficiency is estimated to be 1 in 56,500 live births in the United States.

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 is 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 and central nervous system. The Company also believes its 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 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: Translate Bio's plans to conduct additional preclinical studies for MRT5201 and its plan to submit data from these studies to FDA in the fourth quarter of 2019; 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; Translate Bio's ability to obtain additional preclinical data to support further clinical development of MRT5201; the content and timing of decisions made by the FDA, 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 March 31, 2019 filed with the Securities and Exchange Commission on May 9, 2019 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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