Translate Bio Announces FDA Clinical Hold on Investigational New Drug Application (IND) for MRT5201 for the Treatment of Ornithine Transcarbamylase (OTC) Deficiency

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LEXINGTON, Mass., Jan. 22, 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 the Company has received verbal notification from the U.S. Food and Drug Administration (FDA) that the Agency has completed its review of the Company's Investigational New Drug Application (IND) submission for MRT5201 for the treatment of ornithine transcarbamylase (OTC) deficiency and has additional clinical and nonclinical questions. The Company submitted the IND in December 2018 to support the initiation of a Phase 1/2 clinical trial in patients with OTC deficiency. The FDA is placing the IND on clinical hold until these questions are resolved. The Company expects to receive formal written communication with additional information from the FDA in the near future and plans to work with the FDA in an effort to resolve its questions as promptly as possible.

About MRT5201
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, the predominant type of liver cell, 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.

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 and its MRT delivery systems are 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, central nervous system and lymphatic system. The Company also believes its MRT platform and MRT delivery systems 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 expectations regarding the timing in which it will receive additional communications from the FDA regarding the clinical hold on MRT5201 and the scope of any questions and concerns regarding its IND, product candidate, platform and delivery system; Translate Bio's plans to work with FDA in an effort to resolve its questions and concerns as promptly as possible; Translate Bio's beliefs regarding the broad applicability of its MRT platform and its MRT delivery systems.; 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 risk that Translate Bio may not resolve this or any other clinical hold in the near term or at all, or that the FDA could make decisions that adversely impact the ability of Translate Bio to advance its programs in development; 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 decisions that may arise as a result of the clinical hold on MRT5201; 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 September 30, 2018 filed with the Securities and Exchange Commission on November 8, 2018 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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