

**FOR IMMEDIATE RELEASE****Translate Bio Announces FDA Clearance to Proceed with Phase 1/2 Clinical Trial in Patients with Cystic Fibrosis (CF)**

*-- mRNA therapeutic product candidate MRT5005 designed to treat all patients with CF regardless of underlying genetic mutation --*

**LEXINGTON, Mass. – April 12, 2018** – Translate Bio, a leading 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 U.S. Food and Drug Administration (FDA) has cleared the Company to begin a first-in-human clinical trial of MRT5005 in patients with cystic fibrosis. The Company is developing MRT5005, an mRNA therapeutic product candidate that is designed to treat patients with CF by addressing the underlying cause of the disease. The Company expects to begin dosing patients in a Phase 1/2 clinical trial in mid-2018.

“As our first IND submission and the first clinical trial to evaluate a product candidate derived from our mRNA technology (MRT) platform, this is a significant milestone for the Company,” said Ronald Renaud, chief executive officer, Translate Bio. “It also represents a pivotal time in the mRNA field as it will be the first mRNA therapeutic to enter the clinic with targeted delivery to the lung.”

Mr. Renaud continued, “We are excited to get this study underway because it represents a new approach to treating CF. It is designed to treat the underlying cause of the disease regardless of an individual patient’s mutation by giving the body instructions to produce its own functional protein.”

In December 2017, the Company submitted an Investigational New Drug (IND) application to initiate the Phase 1/2 clinical trial of MRT5005. At the end of the 30-day review period, the FDA requested additional chemistry, manufacturing and controls (CMC) information related to certain materials, processes and testing used during the manufacturing of MRT5005. The information requested did not require additional work or studies. While the Company gathered the requested information and submitted a complete response to the FDA, the IND was placed on clinical hold. The Company has now received notification that the FDA has removed the clinical hold, allowing the Phase 1/2 clinical trial to begin.

**Phase 1/2 Clinical Trial Design**

The randomized, double-blind, placebo-controlled Phase 1/2 clinical trial of MRT5005 will enroll at least 32 adult patients with CF who have at least one Class I or Class II mutation. The primary endpoint of the trial will be the safety and tolerability of single and multiple escalating doses of MRT5005 administered by nebulization. Forced expiratory volume in one second (FEV1), 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.

### **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. In 2015, the FDA granted orphan drug designation to MRT5005 for the treatment of CF.

### **About Cystic Fibrosis**

Cystic fibrosis is the most common fatal inherited disease in the United States, affecting more than 30,000 patients in the U.S. and more than 70,000 patients worldwide. CF is caused by genetic mutations that result in dysfunctional or absent CFTR protein. This defect causes mucus buildup in the lungs, pancreas and other organs. Mortality is primarily driven by a progressive decline in lung function. According to the Cystic Fibrosis Foundation, the median age at death for patients with CF was 29.6 years in 2016. There is no cure for CF. CFTR modulators that are currently marketed or in clinical development are effective only in patients with specific mutations, and patients still experience pulmonary exacerbations and a progressive decline in lung function, which represents a significant unmet need.

### **About Translate Bio**

Translate Bio is a leading 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. The Company 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, central nervous system, lymphatic system and circulatory system. The Company's two lead programs are being developed as treatments for CF and ornithine transcarbamylase (OTC) deficiency. For more information about the Company, please visit [www.translate.bio](http://www.translate.bio) or on Twitter at @TranslateBio.

###

### **Contacts for Translate Bio**

Teri Dahlman  
[tdahlman@translate.bio](mailto:tdahlman@translate.bio)  
857-242-7792

Maura Gavaghan  
[mgavaghan@translate.bio](mailto:mgavaghan@translate.bio)  
857-242-7789