RaNA Therapeutics Relaunches as Translate Bio to Advance RNA Therapeutics

-- Company Expands Executive Leadership Team with Key Appointments in Early 2017 --

CAMBRIDGE, Mass. – June 27, 2017 – RaNA Therapeutics today announced the company has relaunched as Translate Bio (www.translate.bio). The new name stems from the company’s focus on pioneering the translation of RNA science into therapeutics promoting healthy gene expression for people living with debilitating genetic diseases. In addition to its new name, the company also announced the recent leadership appointments of Michael Heartlein, Ph.D., as Chief Technical Officer and Brian Fenton as Chief Business Officer.

In January, Translate Bio announced the company acquired the MRT platform – a messenger RNA (mRNA) therapy platform developed by subsidiaries of Shire plc – significantly expanding the company’s ability to potentially correct a wide range of disease genotypes regardless of mutation and access new targets not currently addressable by existing modalities. Resulting from the acquisition, the company also welcomed Shire’s former MRT employees and gained two near-clinic programs in cystic fibrosis and ornithine transcarbamylase (OTC) deficiency, a urea cycle disorder.

“Translate Bio is the combination of two passionate teams of scientists coming together under one shared mission: to translate RNA science into revolutionary medicines,” said Ron Renaud, Chief Executive Officer, Translate Bio. “Representing a decade of RNA expertise and know-how, we are poised to rapidly advance treatments in cystic fibrosis and OTC – two historically hard-to-treat diseases where there is dire need.”

Leadership Appointments

In conjunction with the MRT platform acquisition, Translate Bio appointed Michael Heartlein, Ph.D., as Chief Technical Officer in January 2017. Dr. Heartlein’s extensive career in research and development has focused on the discovery and development of platform technologies, product discovery and product development. At Shire, he discovered and led early development of several of Shire’s enzyme replacement therapies and, most recently, discovered and developed Shire’s Messenger RNA Therapy (MRT) technology acquired by RaNA. Prior to joining Shire, Dr. Heartlein held a hospital appointment in genetics at Children’s Hospital Boston and a faculty appointment in pediatrics at Harvard Medical School. He received his Ph.D. in genetics from Oak Ridge National Laboratory – University of Tennessee.

Translate Bio has also recently appointed Brian Fenton as Chief Business Officer, who previously served as Vice President, Corporate Development for RaNA since late 2015. Prior to joining RaNA, Mr. Fenton served as Head of Business Development, Neuroscience at Shire, and was also responsible for managing Shire’s portfolio investments. In addition, Mr. Fenton spent several years in corporate development at Shire within the company’s rare disease group,
where he successfully identified, led and executed a number of strategic transactions for the company. Prior to Shire, Mr. Fenton spent over 11 years in various roles in business development and alliance management at Idenix Pharmaceuticals, Codexis and Abbott Laboratories. Mr. Fenton has an undergraduate degree in biochemistry from the University of Massachusetts/Amherst, an M.S. in chemical engineering from the University of Virginia and an MBA from the Worcester Polytechnic Institute.

**Lead Programs in Cystic Fibrosis and Ornithine Transcarbamylase (OTC) Deficiency, a Urea Cycle Disorder**

Translate Bio is committed to building the leading portfolio of RNA medicines with two near-clinic, lead programs in cystic fibrosis and ornithine transcarbamylase (OTC) deficiency, a urea cycle disorder.

Cystic fibrosis causes severe damage to the lungs, digestive system and other organs. This inherited disease is caused by a defect in the CFTR gene, which normally makes a protein that controls the movement of salt and water in and out of the body's cells. According to the Cystic Fibrosis Foundation Patient Registry, more than 30,000 people are living with CF in the U.S., and more than 70,000 worldwide. Approximately 1,000 people are newly diagnosed with CF each year. Translate Bio has developed an mRNA therapy that targets the CFTR pathway. By providing a new set of instructions to replace those of the defective CFTR gene, healthy protein production can be restored for all patients, regardless of mutation type.

Ornithine transcarbamylase (OTC) deficiency is a rare inborn error of metabolism and the most common of the urea-cycle disorders. Occurring in one out of every 40,000 births, OTC deficiency is a devastating disease. Typically, newborns with this condition slip into a coma within 72 hours of birth. Most suffer severe brain damage, half die in the first month and half of the survivors die by age five. Translate Bio is rapidly advancing a treatment utilizing its mRNA technology to restore healthy levels of OTC protein production in the liver.

**Additional Programs**

Translate Bio’s platform for powering endogenous therapeutic protein production is applicable to a broad range of diseases caused by insufficient protein production. In addition to cystic fibrosis and OTC deficiency, the company is committed to building a diverse pipeline of development candidates and is currently exploring rare diseases of the liver, lung and central nervous system (CNS), as well as other therapeutic areas where its MRT technology may have an impact.

**Upcoming Milestones**

Translate Bio’s team of scientists has developed novel therapeutic mRNA, designed to enable the *in vivo* production of both intracellular, membrane-associated and secreted proteins. Clinical programs in cystic fibrosis and OTC are expected to begin Phase 1 clinical testing in early 2018 and mid-2018, respectively.
In addition, Translate Bio has plans to centralize its Cambridge, Mass. and Lexington, Mass. locations to a standalone location in Lexington by the first quarter of 2018.

“It’s an incredibly exciting time for Translate Bio and the field of RNA therapeutics,” continued Renaud. “Equipped with the most comprehensive RNA-based platform in the industry, we are committed to making an impact for people suffering from serious, life-threatening diseases. That starts with filing our first IND by the end of this year, getting us one step closer to reaching patients.”

About Translate Bio
Translate Bio is a leading biotechnology company focused on pioneering the translation of RNA science into therapeutics promoting healthy gene expression in people living with debilitating genetic diseases. The company’s RNA platforms for powering endogenous therapeutic protein production is applicable to a broad range of diseases caused by insufficient protein production, including rare diseases of the liver, lung and central nervous system (CNS). For more information about the company, please visit www.translate.bio.

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