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Vertex and Moderna Establish Exclusive Collaboration to Discover and Develop mRNA Therapeutics™ for Cystic Fibrosis

-Collaboration to explore use of mRNA Therapeutics to treat the underlying cause of CF by enabling cells to produce functional CFTR proteins in the lungs-

-Moderna to receive $40 million upfront, made up of a $20 million cash payment and a $20 million convertible note investment, with potential for up to additional $275 million in milestones plus royalty payments-

BOSTON & CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Moderna Therapeutics today announced that the two companies have entered into an exclusive research collaboration and licensing agreement aimed at the discovery and development of messenger Ribonucleic Acid (mRNA) Therapeutics™ for the treatment of cystic fibrosis (CF). The three-year collaboration will focus on the use of mRNA therapies to treat the underlying cause of CF by enabling cells in the lungs to produce functional copies of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which is known to be defective in people with CF. Through the collaboration, the companies will explore the potential utilization of pulmonary mRNA delivery.

"We are excited to begin this collaboration with Moderna to further broaden our pipeline and support our goal of treating the underlying cause of CF for all people with this disease. The collaboration with Moderna underscores our commitment to invest in and pioneer multiple innovative approaches to expand and enhance the future treatment of CF," said David Altshuler, M.D., Ph.D., Vertex's Executive Vice President, Global Research and Chief Scientific Officer. "Moderna's messenger RNA Therapeutics represent a promising new approach that could provide functional CFTR proteins to the lungs of people with CF, which would complement our existing approach of using CFTR modulators to treat CF."

"Vertex's deep scientific expertise and commitment to creating the best treatment regimens for cystic fibrosis patients make them an ideal partner to advance mRNA Therapeutics for this disease," said Stéphane Bancel, Chief Executive Officer of Moderna. "This collaboration is illustrative of our consistent strategy to partner with world class therapeutic experts in order to maximize the potential of leveraging mRNA as a novel therapeutic approach across a spectrum of serious diseases. We're particularly excited to explore the potential of pulmonary delivery as a new modality to bring transformative mRNA medicines to patients and address unmet needs."

Under the terms of the collaboration, Vertex and Moderna will conduct exclusive research, development and commercialization activities to advance mRNA Therapeutics that aim to enable cells of people with CF to produce functional CFTR proteins in the lungs. Moderna will lead discovery efforts, leveraging its leading mRNA platform technology and mRNA delivery expertise along with Vertex's scientific experience in CF biology and the functional understanding of CFTR, as well as the company's proprietary assay platform that utilizes human bronchial epithelial (HBE) cells of multiple different CF gene mutations from people with CF. Vertex will lead all preclinical, development and commercialization activities associated with the advancement of mRNA Therapeutics that result from this collaboration and will fund all expenses related to the collaboration.

Vertex will pay Moderna $20 million in cash as part of its upfront commitment to the collaboration. Vertex will also make a $20 million investment in Moderna in the form of a convertible note that will convert to equity. The investment will provide Vertex with an ownership stake in Moderna. Vertex will also pay Moderna future development and regulatory milestones of up to $275 million, including $220 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales.

About Moderna's mRNA Therapeutics™

mRNA is responsible for carrying genetic instructions transcribed from DNA, which cells then translate to produce proteins that, when defective or missing, can underlie certain diseases, including CF. Moderna's pioneering mRNA Therapeutics™ are designed to trigger the cellular machinery to utilize their natural processes to produce specific functional proteins. Through this collaboration, mRNA Therapeutics may be developed to enable the delivery of correct genetic instructions into cells in the lungs, which may trigger the cells to produce functional CTFR protein. This mRNA-based approach could be applicable to any person with CF regardless of a person's specific CFTR mutations. Moderna currently has two Phase 1 clinical studies underway of mRNA vaccines for the prevention of infectious diseases. The company has over 90 discovery

programs advancing across its ecosystem of therapeutically focused ventures and external partners that span rare diseases, infectious diseases, cancer and cardiovascular disease, among others.

Vertex's CF Research and Development Pipeline

Vertex's strategy in CF is to increase the number of people eligible for treatment with its two currently approved CF medicines and to develop new medicines that have the potential to treat all people with the disease in the future. Vertex's two approved CF medicines, known as CFTR modulators, are designed to treat the defective CFTR protein in people with specific mutations in the CF gene. Beyond its two approved medicines, Vertex is also advancing multiple other potential medicines for the treatment of CF, including additional CFTR modulators as well as other collaborative approaches, including sodium channel inhibition, gene editing and, as announced today, mRNA-based therapies. The most advanced approach is the development of an additional two-drug combination of CFTR modulators, which is currently being evaluated in a broad Phase 3 development program. The company plans to advance a three-drug combination of CFTR modulators into Phase 2 development in people with CF in the second half of 2016.

About Cystic Fibrosis

Cystic fibrosis is a rare, life-shortening genetic disease affecting approximately 75,000 people in North America, Europe and Australia. Today, the median predicted age of survival for a person with CF is between 34 and 47 years, but the median age of death remains in the mid-20s.

CF is caused by a defective or missing CFTR protein resulting from mutations in the CFTR gene. Children must inherit two defective CFTR genes — one from each parent — to have CF. There are more than 1,900 known mutations in the CFTR gene. Some of these mutations, which can be determined by a genetic, or genotyping test, lead to CF by creating non-working or too few CFTR protein at the cell surface. The defective function or absence of CFTR proteins in people with CF results in poor flow of salt and water into and out of the cell in a number of organs, including the lungs. This leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For six years in a row, Science magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

About Moderna Therapeutics

Moderna is a clinical stage pioneer of messenger RNA (mRNA) Therapeutics™, an entirely new *in vivo* drug technology that produces human proteins, antibodies and entirely novel protein constructs inside patient cells, which are in turn secreted or active intracellularly. This breakthrough platform addresses currently undruggable targets and offers a potentially superior alternative to existing drug modalities for a wide range of diseases and conditions. Moderna is developing and plans to commercialize its innovative mRNA drugs through its own ventures and its strategic relationships with established pharmaceutical and biotech companies. Its current ventures are: Onkaido, focused on oncology, Valera, focused on infectious diseases, Elpidera, focused on rare diseases, and Caperna, focused on personalized cancer vaccines. Cambridge-based Moderna is privately held and currently has strategic agreements with AstraZeneca, Alexion Pharmaceuticals, Merck and Vertex Pharmaceuticals. To learn more, visit www.modernatx.com.

Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Altshuler's statements in the second paragraph of the press release, Mr. Bancel's statements in the third paragraph of the press release and the information provided regarding the future development of mRNA therapies to treat the underlying cause of CF. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that data may not support further development of the mRNA therapies subject to the collaboration due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to

update the information contained in this press release as new information becomes available.

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