



Moderna and Vertex Establish New Collaboration to Treat Cystic Fibrosis Using Gene Editing

September 16, 2020

Second collaboration between Moderna and Vertex based on Moderna's proprietary mRNA and LNP technology

Collaboration will leverage Vertex's investments and capabilities in genetic technologies for CF

Moderna to receive \$75 million upfront, with potential for additional development, regulatory and commercial milestones and royalty payments

CAMBRIDGE & BOSTON, Mass.--(BUSINESS WIRE)--Sep. 16, 2020-- Moderna, Inc. (Nasdaq:MRNA) and Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX) announced today a new strategic research collaboration and licensing agreement aimed at the discovery and development of lipid nanoparticles (LNPs) and mRNAs for the delivery of gene-editing therapies for the treatment of cystic fibrosis (CF). The three-year research collaboration initially will focus on the discovery and optimization of novel LNPs and mRNAs that can deliver gene-editing therapies to cells in the lungs, enabling functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to be produced.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20200916005915/en/>

"We are pleased to enter into this second collaboration with Vertex aimed at delivering potentially novel treatments for patients with cystic fibrosis using gene editing," said Stéphane Bancel, Chief Executive Officer of Moderna. "Our first collaboration with Vertex to deliver mRNA coding for cystic fibrosis protein in lung cells is advancing well and this second collaboration aims at using Moderna's technologies to explore the use of gene editing in lung cells."

"Vertex's CFTR modulator therapies have the potential to treat the vast majority of CF patients and address the underlying cause of their disease. However, approximately 10 percent of patients do not produce any CFTR protein and so are unlikely to benefit from our existing medicines. Over the past 5 years, we have made important progress in our research efforts aimed at the creation of genetic therapies for CF, with the delivery of such therapies remaining the most significant technological and scientific challenge," said David Altshuler, M.D., Ph.D., Vertex's Executive Vice President, Global Research and Chief Scientific Officer. "The combination of Moderna's unique expertise in the discovery and manufacturing of novel LNP delivery systems and mRNA technologies, combined with Vertex's scientific, clinical and regulatory capabilities in CF, will accelerate the development of groundbreaking genetic therapies for people with CF and supports our commitment to developing therapies for all people living with CF."

Under the terms of the agreement, Moderna will conduct research activities to discover and optimize novel LNPs for the delivery of gene-editing therapies to lung cells for the treatment of CF. Moderna will receive \$75 million upfront and will be eligible to receive up to \$380 million in development, regulatory and commercial milestones, plus tiered royalties on any products that result from the collaboration. Moderna will be responsible for the discovery and manufacturing of LNPs and mRNA constructs encoding gene-editing endonucleases. Vertex will be responsible for providing other components of the gene-editing therapies to be formulated into LNPs, as well as subsequent preclinical and clinical development and potential commercialization efforts. This new collaboration adds to the existing relationship between Moderna and Vertex in their recently extended collaboration aimed at the discovery and development of mRNA therapeutics for the treatment of CF.

About Moderna

Moderna is advancing messenger RNA (mRNA) science to create a new class of transformative medicines for patients. mRNA medicines are designed to direct the body's cells to produce intracellular, membrane or secreted proteins that can have a therapeutic or preventive benefit and have the potential to address a broad spectrum of diseases. Moderna's platform builds on continuous advances in basic and applied mRNA science, delivery technology and manufacturing, providing the Company the capability to pursue in parallel a robust pipeline of new development candidates. Moderna is developing therapeutics and vaccines for infectious diseases, immuno-oncology, rare diseases, cardiovascular diseases, and autoimmune and inflammatory diseases, independently and with strategic collaborators.

Headquartered in Cambridge, Mass., Moderna currently has strategic alliances for development programs with AstraZeneca PLC and Merck & Co., Inc., as well as the Defense Advanced Research Projects Agency (DARPA), an agency of the U.S. Department of Defense; the Biomedical Advanced Research and Development Authority (BARDA), a division of the Office of the Assistant Secretary for Preparedness and Response (ASPR) within the U.S. Department of Health and Human Services (HHS) and the

Coalition for Epidemic Preparedness Innovations (CEPI). Moderna has been named a top biopharmaceutical employer by *Science* for the past five years.

About Vertex Pharmaceuticals

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 10 consecutive years on *Science* magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by *Forbes*.

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, Mr. Bancel's statements in the second paragraph of the press release, Dr. Altshuler's statements in the third paragraph of the press release, and the information provided regarding the future development of therapies to treat the underlying cause of CF, and the potential commercialization opportunities from those therapies. While Vertex and Moderna each believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the companies' beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov, as well as those risks listed under the heading "Risk Factors" in Moderna's most recent quarterly report on Form 10-Q filed with the SEC and in subsequent filings made by Moderna with the SEC, which are available on Moderna's website at www.modernatx.com and on the SEC's website at www.sec.gov. Except as required by law, Vertex and Moderna each disclaim any intention or responsibility for updating or revising any forward-looking statements contained in this press release in the event of new information, future developments or otherwise.

(VRTX – GEN)



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