Vertex and CRISPR Therapeutics Establish Collaboration to Use CRISPR-Cas9 Gene Editing Technology to Discover and Develop New Treatments for Genetic Diseases

October 26, 2015

–Gene editing technology to be used to discover treatments to address the mutations and genes known to cause and contribute to cystic fibrosis-

-Vertex and CRISPR to utilize gene editing approach to discover treatments for genetic diseases, including sickle cell disease-

-Companies establish four-year research collaboration; CRISPR to receive $105 million up-front payment, of which $30 million is an equity investment and $75 million is cash, with potential for additional milestones and royalty payments–

BOSTON AND CAMBRIDGE, MASS – October 26, 2015 – Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and CRISPR Therapeutics today announced that the two companies have entered into a strategic research collaboration focused on the use of CRISPR's gene editing technology, known as CRISPR-Cas9, to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. The collaboration will evaluate the use of CRISPR-Cas9 across multiple diseases where targets have been validated through human genetics. Vertex and CRISPR will focus their initial gene editing research on discovering treatments to address the mutations and genes known to cause and contribute to cystic fibrosis and sickle cell disease. Vertex and CRISPR will also evaluate a specified number of other genetic targets as part of the collaboration. Vertex will have exclusive rights to license up to six new CRISPR-Cas9-based treatments that emerge from the collaboration. As part of the collaboration, Vertex made an up-front commitment of $105 million to CRISPR, including $75 million in cash and a $30 million equity investment. CRISPR is also eligible to receive future development, regulatory and sales milestones and royalty payments on future sales.

"CRISPR-Cas9 is an important scientific and technological breakthrough that holds significant promise for the future discovery of potentially transformative treatments for many genetic diseases," said David Altshuler, M.D., Ph.D., Vertex's Executive Vice President, Global Research and Chief Scientific Officer. "As a company founded on innovative science, we're excited to begin this collaboration with CRISPR, as it puts us at the forefront of what we believe may be a fundamental change in the future treatment of disease -- using gene editing technologies to address the underlying genetic causes of many diseases."

"Vertex has a track record of developing innovative medicines for cystic fibrosis and other serious diseases, making them a great partner to accelerate the therapeutic promise of gene editing," said Rodger Novak, M.D., Chief Executive Officer of CRISPR Therapeutics. "For CRISPR, this collaboration validates the potential for gene editing in human therapeutics and provides important financial support for continued investment in our platform and proprietary pipeline of programs."

About the Collaboration
Under the terms of the collaboration, Vertex and CRISPR will jointly use the CRISPR-Cas9 technology to discover and develop potential new treatments that correct defects in specific gene targets known to cause or contribute to particular diseases. The initial focus of the collaboration will be on the use of CRISPR-Cas9 to potentially correct the mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene known to result in the defective protein that causes CF and to edit other genes that contribute to the disease. Additionally, the companies will seek to discover and develop gene-based treatments for hemoglobinopathies, including sickle cell disease. Additional discovery efforts focused on a specified number of other genetic targets will also be conducted under the collaboration. Discovery activities will be conducted primarily by CRISPR, and the related expenses will be fully funded by Vertex. Vertex has the option to an exclusive license for up to six gene-based treatments that emerge from the four-year research collaboration. Vertex will fund 100 percent of the development expenses of licensed treatments. For each of the up to six treatments in-licensed for development, Vertex will pay future development, regulatory and sales milestones of up to $420 million as well as royalty payments on future sales.

Vertex and CRISPR will collaborate on the research, development and commercialization of treatments for hemoglobinopathies that emerge from the collaboration. Specifically for hemoglobinopathies, including treatments for sickle cell disease, Vertex and CRISPR will equally share all research and development costs and sales, with CRISPR Therapeutics leading commercialization efforts in the U.S. For all other diseases, Vertex will lead all development and global commercialization activities.

Vertex will pay CRISPR $75 million in cash as part of its up-front commitment. Vertex will also provide a $30 million investment in CRISPR, which is a private company. The investment will provide Vertex with an ownership stake in CRISPR. The collaboration also provides Vertex with an observer seat on the CRISPR Board of Directors, which will be filled by Dr. Altshuler.

About Gene Editing with CRISPR-Cas9
"CRISPR" refers to Clustered Regularly Interspaced Short Palindromic Repeats that occur in the genome of certain bacteria, from which the system was discovered. Cas9 is a CRISPR-associated endonuclease (an enzyme) known to act as the "molecular scissors" that cut and edit, or correct, disease-associated DNA in a cell. A guide RNA directs the Cas9 molecular scissors to the exact site of the disease-associated mutation. Once the molecular scissors make a cut in the DNA, additional cellular mechanisms and exogenously added DNA will use the cell's own machinery and other elements to specifically 'repair' the DNA. This technology may offer the ability to directly modify or correct the underlying disease-associated changes in the human genome for the potential treatment of a large number of both rare and common diseases.

Emmanuelle Charpentier, Ph.D., one of CRISPR Therapeutics' scientific founders , co-invented the CRISPR-Cas9 technology and is the recipient of multiple prestigious awards in recognition of the potential contribution that the CRISPR-Cas9 technology may have on global health. The other scientific co-founders of CRISPR are Craig Mello, Ph.D., Chad Cowan, Ph.D., Matthew Porteus, M.D., Ph.D., and Daniel Anderson, Ph.D.

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 five 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 CRISPR Therapeutics
The mission of CRISPR Therapeutics is to develop transformative gene-based medicines for patients with serious diseases. Our therapeutic approach aims to cure diseases at the molecular level using the breakthrough gene editing technology called CRISPR-Cas9. With our multi-disciplinary team of world-renowned academics, drug developers and clinicians, we are uniquely positioned to translate CRISPR-Cas9 technology into human therapeutics. We have licensed the foundational CRISPR-Cas9 patent estate for human therapeutic use from our scientific founder, Dr. Emmanuelle Charpentier. We are headquartered in Basel, Switzerland, our R&D operations are in Cambridge, Massachusetts and we have corporate offices in London, United Kingdom. www.crisprtx.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, Dr. Novak's statements in the third paragraph of the press release and the information provided regarding the future development of treatments for genetic diseases using the CRISPR-Cas9 technology. 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 gene-based treatments 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.

(VRTX-GEN)

Vertex Pharmaceuticals Incorporated

Investors:
Michael Partridge, 617-341-6108
or
Kelly Lewis, 617-961-7530
or
Eric Rojas, 617-961-7205

or

Media: mediainfo@vrtx.com
Zach Barber: 617-341-6992

CRISPR MEDIA CONTACTS:
MacDougall Biomedical Communications
Kari Watson in US – kwatson@macbiocom.com +1 (781) 235-3060
Anca Alexandru in Europe – aalexandru@macbiocom.com +49 (89) 2424-3494