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Vertex and CRISPR Therapeutics to Co-Develop and Co-Commercialize CTX001 as CRISPR/Cas9 Gene Edited Treatment for Sickle Cell Disease and β-Thalassemia

-Vertex selects CTX001 as first gene edited treatment to be developed as part of collaboration with CRISPR Therapeutics-

-Clinical Trial Application for CTX001 submitted in Europe to support initiation of Phase 1/2 clinical study in β-thalassemia in 2018-

-Preclinical data for CTX001 presented this week at the American Society for Hematology Annual Meeting-

BOSTON, CAMBRIDGE, Mass. & ZUG, Switzerland--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) and CRISPR Therapeutics AG (NASDAQ: CRSP) today announced that the companies will co-develop and co-commercialize CTX001, an investigational gene editing treatment, as part of the companies' previously announced collaboration aimed at the discovery and development of new gene editing treatments that use the CRISPR/Cas9 technology. CTX001 represents the first gene-based treatment that Vertex exclusively licensed from CRISPR Therapeutics as part of the collaboration. For CTX001, CRISPR and Vertex will equally share all research and development costs and profits worldwide. A Clinical Trial Application was submitted earlier this month for CTX001 to support the initiation of a Phase 1/2 trial in β-thalassemia in 2018 in Europe, and an Investigational New Drug (IND) Application is planned for submission in 2018 to support the initiation of a Phase 1/2 trial in sickle cell disease in the U.S. Preclinical data presented for CTX001 at the American Society for Hematology on December 10, 2017 showed clinically relevant increases in fetal hemoglobin and a high editing rate that support the advancement of CTX001 into the planned trials in β-thalassemia and sickle cell disease in 2018.

This press release features multimedia. View the full release here:
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"Over the past two years, we've made significant progress with CRISPR Therapeutics on the discovery and preclinical development of multiple CRISPR/Cas9-based treatments, and we're pleased to select CTX001 as the first of these treatments to move into clinical development as part of our collaboration," said David Altshuler, M.D., Ph.D., Vertex's Executive Vice President, Global Research and Chief Scientific Officer. "The addition of CTX001 to our clinical development pipeline provides us with a near-term opportunity to generate the first proof-of-concept clinical data for a CRISPR/Cas9-based medicine in two genetic diseases that are highly aligned with our research strategy."

"The submission of a Clinical Trial Application for CTX001 in Europe, supported by the robust data presented at the recent ASH Annual Meeting, reflect the advances we have achieved in translating the potential of CRISPR/Cas9 science into transformative therapies. We now look forward to working closely with Vertex as we initiate clinical trials next year," commented Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "The study of CTX001 in β-thalassemia will be the first company-sponsored clinical trial of a CRISPR-based therapy and is a major step forward for both the treatment of certain inherited blood diseases and for our collaboration with Vertex."

Clinical Development Plans for CTX001
CRISPR Therapeutics and Vertex will co-develop and co-commercialize CTX001 for the treatment of hemoglobinopathies, including β-thalassemia and sickle cell disease. A Phase 1/2 trial of CTX001 is expected to begin in 2018 in Europe and will be designed to assess the safety and efficacy of CTX001 in adult transfusion dependent β-thalassemia patients. The companies also plan to file an IND Application for CTX001 with the United States Food and Drug Administration to support the initiation of a Phase 1/2 trial in sickle cell disease in 2018 in the U.S. Additional details on the trial designs will be provided upon study initiation.

About CTX001 and Recent Data Presented at the American Society for Hematology (ASH) Annual Meeting
CTX001 is an investigational *ex vivo* CRISPR gene-edited therapy for patients suffering from β-thalassemia and sickle cell disease in which a patient's hematopoietic stem cells are engineered to produce high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is a form of the oxygen carrying hemoglobin that is naturally present at birth, and is then replaced by the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion-requirements for β-thalassemia patients and painful and debilitating sickle crises for sickle cell patients.

On December 10, 2017, CRISPR Therapeutics presented preclinical data at the 2017 ASH Annual Meeting that showed greater than 90% editing of hematopoietic stem cells at the target site, leading to clinically relevant increases in fetal hemoglobin. These data support the advancement of CTX001 into the planned trials in β-thalassemia and sickle cell disease in 2018.

About the CRISPR-Vertex Collaboration
CRISPR and Vertex entered into a strategic research collaboration in 2015 aimed at the discovery and development of gene editing treatments using the CRISPR/Cas9 technology to correct defects in specific gene targets known to cause or contribute to particular diseases. Vertex has exclusive rights to license up to six new CRISPR/Cas9-based treatments that emerge from the collaboration, and CTX001 represents the first treatment to emerge from the joint research program. For CTX001, CRISPR and Vertex will equally share all research and development costs and profits worldwide.

About CRISPR Therapeutics
CRISPR Therapeutics is a leading gene-editing company focused on developing transformative gene-based medicines for serious diseases using its proprietary CRISPR/Cas9 gene-editing platform. CRISPR/Cas9 is a revolutionary technology that allows for precise, directed changes to genomic DNA. The company's multi-disciplinary team of world-class researchers and drug developers is working to translate this technology into breakthrough human therapeutics in a number of serious diseases. Additionally, CRISPR Therapeutics has established strategic collaborations with Bayer AG and Vertex Pharmaceuticals to develop CRISPR-based therapeutics in diseases with high unmet need. The foundational CRISPR/Cas9 patent estate for human therapeutic use was licensed from the company's scientific founder Emmanuelle Charpentier, Ph.D. CRISPR Therapeutics AG is headquartered in Zug, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge, Massachusetts. For more information, please visit http://www.crisprtx.com.

About Vertex
Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for seven years in a row. For additional information and the latest updates from the company, please visit www.vrtx.com.

CRISPR Forward-Looking Statement
Certain statements set forth in this press release constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: the timing of filing of clinical trial applications and INDs and timing of commencement of clinical trials, the intellectual property coverage and positions of the Company, its licensors and third parties, the sufficiency of the Company's cash resources and the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies. You are cautioned that forward-looking statements are inherently uncertain. Although the Company believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, the forward-looking statements are neither promises nor guarantees and they are necessarily subject to a high degree of uncertainty and risk. Actual performance and results may differ materially from those projected or suggested in the forward-looking statements due to various risks and uncertainties. These risks and uncertainties include, among others: uncertainties regarding the intellectual property protection for our technology and intellectual property belonging to third parties; uncertainties inherent in the initiation and completion of preclinical studies for the Company's product candidates; availability and timing of results from preclinical studies; whether results from a preclinical trial will be predictive of future results of the future trials; expectations for regulatory approvals to conduct trials or to market products; and those risks and uncertainties described under the heading "Risk Factors" in the Company's most recent annual report on Form 10-K, and in any other subsequent filings made by the Company with the U.S. Securities and Exchange Commission (SEC), which are available on the SEC's website at www.sec.gov. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date they are made.

Vertex's 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, the statements in the second and third paragraphs and statements regarding the planned clinical development timeline, including submission of an IND to the FDA. 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 from the company's development programs may not support registration or further development of its compounds 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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