Vertex and CRISPR Therapeutics to Present New Clinical Data on Investigational CRISPR/Cas9 Gene-Editing Therapy CTX001™ For Severe Hemoglobinopathies at the Annual European Hematology Association Virtual Congress

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CAMBRIDGE, Mass. and ZUG, Switzerland and BOSTON, May 12, 2021 (GLOBE NEWSWIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and CRISPR Therapeutics (Nasdaq: CRSP) today announced two abstracts detailing updated data from the ongoing CTX001 clinical trials have been accepted for presentation during the European Hematology Association (EHA) 2021 Virtual Congress.

Abstract #EP736 entitled "*CTX001 for Sickle Cell Disease: Safety and Efficacy Results from the Ongoing CLIMB SCD-121 Study of Autologous Crispr-Cas9-Modified CD34+ Hematopoietic Stem and Progenitor Cells*," will be made available on the virtual platform as an e-poster Friday, June 11 at 9:00 CEST. The abstract posted online today includes data on patients with severe sickle cell disease with more than 3 months of follow-up, as of the interim data cut on January 28, 2021. Data will be updated and information on additional patients will be included for the congress.

Abstract #EP733 entitled "*CTX001 for Transfusion-Dependent B-Thalassemia: Safety and Efficacy Results from the Ongoing CLIMB Thal-111 Study of Autologous Crispr-Cas9-Modified CD34+ Hematopoietic Stem and Progenitor Cells,*" will be made available on the virtual platform as an e-poster Friday, June 11 at 9:00 CEST. The abstract posted online today includes data on patients with transfusion-dependent beta thalassemia (TDT) with more than 3 months of follow-up, including patients with the most severe genotypes, as of the interim data cut on January 21, 2021. Data will be updated and information on additional patients will be included for the congress.

The accepted abstracts are now available online on the EHA website https://library.ehaweb.org/eha/#!*menu=6*browseby=8*sortby=2*media=3*ce_id=2035*label=21989*ot_id=25562*marker=1286.

CTX001 is being investigated in two ongoing clinical trials as a potential one-time curative therapy for patients suffering from TDT and severe SCD.

About CTX001
CTX001 is an investigational, autologous, *ex vivo* CRISPR/Cas9 gene-edited therapy that is being evaluated for patients suffering from TDT or severe SCD, in which a patient's hematopoietic stem cells are edited 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, which then switches to the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion requirements for patients with TDT and reduce painful and debilitating sickle crises for patients with SCD. Earlier results from these ongoing trials were published as a Brief Report in *The New England Journal of Medicine* in January of 2021.

Based on progress in this program to date, CTX001 has been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug, and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) for both TDT and SCD. CTX001 has also been granted Orphan Drug Designation from the European Commission, as well as Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), for both TDT and SCD.

Among gene-editing approaches being investigated/evaluated for TDT and SCD, CTX001 is the furthest advanced in clinical development.

About CLIMB-111
The ongoing Phase 1/2 open-label trial, CLIMB-Thal-111, is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with TDT. The trial will enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up trial.

About CLIMB-121
The ongoing Phase 1/2 open-label trial, CLIMB-SCD-121, is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with severe SCD. The trial will enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up trial.

About CLIMB-131

This is a long-term, open-label trial to evaluate the safety and efficacy of CTX001 in patients who received CTX001 in CLIMB-111 or CLIMB-121. The trial is designed to follow participants for up to 15 years after CTX001 infusion.

About the Gene-Editing Process in These Trials
Patients who enroll in these trials will have their own hematopoietic stem and progenitor cells collected from peripheral blood. The patient's cells will be edited using the CRISPR/Cas9 technology. The edited cells, CTX001, will then be infused back into the patient as part of a stem cell transplant, a process which involves, among other things, a patient being treated with myeloablative busulfan conditioning. Patients undergoing stem cell transplants may also encounter side effects (ranging from mild to severe) that are unrelated to the administration of CTX001. Patients will initially be monitored to determine when the edited cells begin to produce mature blood cells, a process known as engraftment. After engraftment, patients will continue to be monitored to track the impact of CTX001 on multiple measures of disease and for safety.

About the Vertex-CRISPR Collaboration
Vertex and CRISPR Therapeutics entered into a strategic research collaboration in 2015 focused on the use of CRISPR/Cas9 to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. CTX001 represents the first potential treatment to emerge from the joint research program. Under a recently amended collaboration agreement, Vertex will lead global development, manufacturing and commercialization of CTX001 and split program costs and profits worldwide 60/40 with CRISPR Therapeutics. This amendment is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.

About Vertex
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 cell and genetic 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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Vertex 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, our plans and expectations to present clinical data from the ongoing CTX001 clinical trials during the EHA Virtual Congress, expectations regarding the abstracts that will be made available on the virtual platform, the expectation that data will be updated for the conference, the potential benefits of CTX001, our plans and expectations for our clinical trials and pipeline products, the status of our clinical trials of our product candidates under development by us and our collaborators, including activities at the clinical trial sites and patient enrollment, and our expectations regarding the transaction contemplated by the amended collaboration agreement with CRISPR, including satisfaction of closing conditions and antitrust clearances, and the future activities of the parties pursuant to the amended collaboration agreement. 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 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 from a limited number of patients may not be indicative of final clinical trial results, that data from the company's development programs, including its programs with its collaborators, may not support registration or further development of its compounds due to safety and/or efficacy, or other reasons, that the COVID-19 pandemic may impact the status or progress of our clinical trials and clinical trial sites and the clinical trials and clinical trial sites of our collaborators, including patient enrollment, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. You should not place undue reliance on these statements or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

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 platform. CRISPR/Cas9 is a revolutionary gene editing technology that allows for precise, directed changes to genomic DNA. CRISPR Therapeutics has established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine and rare diseases. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic collaborations with leading companies including Bayer, Vertex Pharmaceuticals and ViaCyte, Inc. 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, and business offices in

San Francisco, California and London, United Kingdom. For more information, please visit www.crisprtx.com.

CRISPR Therapeutics Forward-Looking Statement

This press release may contain a number of "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, as well as statements regarding CRISPR Therapeutics' expectations about any or all of the following: (i) the safety, efficacy and clinical progress of CRISPR Therapeutics' various clinical programs, including CTX001, including expectations regarding the abstracts that will be made available on the virtual platform and the clinical data that are being presented from the ongoing CTX001 clinical trials during the EHA Virtual Congress; (ii) the timing of the potential closing of the transaction contemplated by the amended collaboration agreement, future activities of the parties pursuant to the collaboration and the potential benefits of CRISPR Therapeutics' collaboration with Vertex; and (iii) the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies. Without limiting the foregoing, the words "believes," "anticipates," "plans," "expects" and similar expressions are intended to identify forward-looking statements. You are cautioned that forward-looking statements are inherently uncertain. Although CRISPR Therapeutics believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, existing and prospective investors are cautioned that forward-looking statements are inherently uncertain, are neither promises nor guarantees and not to place undue reliance on such statements, which speak only as of the date they are made. 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: the potential for initial and preliminary data from any clinical trial and initial data from a limited number of patients (as is the case with CTX001 at this time) not to be indicative of final or future trial results; the potential that CTX001 clinical trial results may not be favorable or may not support registration or further development; that future competitive or other market factors may adversely affect the commercial potential for CTX001; the transaction contemplated by the amended collaboration agreement is subject to certain closing conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act; CRISPR Therapeutics may not realize the potential benefits of the collaboration with Vertex; potential impacts due to the coronavirus pandemic, such as to the timing and progress of clinical trials; the potential that future competitive or other market factors may adversely affect the commercial potential for CTX001; uncertainties regarding the intellectual property protection for CRISPR Therapeutics' technology and intellectual property belonging to third parties; and those risks and uncertainties described under the heading "Risk Factors" in CRISPR Therapeutics' most recent annual report on Form 10-K, quarterly report on Form 10-Q, and in any other subsequent filings made by CRISPR Therapeutics with the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov. CRISPR Therapeutics disclaims any obligation or undertaking to update or revise any forward-looking statements contained in this press release, other than to the extent required by law.

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