CRISPR Therapeutics and Vertex Pharmaceuticals Announce FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to CTX001™ for the Treatment of Severe Hemoglobinopathies

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CTX001 has received Orphan Drug Designation from the U.S. Food and Drug Administration for transfusion-dependent beta thalassemia and from the European Medicines Agency for sickle cell disease and transfusion-dependent beta thalassemia

ZUG, Switzerland and CAMBRIDGE, Mass. and BOSTON, May 11, 2020 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP) and Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to CTX001, an investigational, autologous, gene-edited hematopoietic stem cell therapy, for the treatment of severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

“RMAT designation is another important regulatory milestone for CTX001 and underscores the transformative potential of a CRISPR-based therapy for patients with severe hemoglobinopathies,” said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. “We expect to share additional clinical data on CTX001 in medical and scientific forums this year as we continue to work closely with global regulatory agencies to expedite the clinical development of CTX001.”

“The first clinical data announced for CTX001 late last year represented a key advancement in our efforts to bring CRISPR-based therapies to people with beta thalassemia and sickle cell disease and demonstrate the curative potential of this therapy,” said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. “We are encouraged by these recent regulatory designations from the FDA and EMA, which speak to the potential impact this therapy could have for patients.”

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising pipeline products, including genetic therapies. A regenerative medicine therapy is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or therapy has the potential to address unmet medical needs for such disease or condition. Similar to Breakthrough Therapy designation, RMAT designation provides the benefits of intensive FDA guidance on efficient drug development, including the ability for early interactions with FDA to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the biologics license application (BLA) and other opportunities to expedite development and review.

In addition to RMAT designation, CTX001 has received Orphan Drug Designation from the U.S. FDA for TDT and from the European Commission for TDT and SCD. CTX001 also has Fast Track Designation from the U.S. FDA for both TDT and SCD.

About CTX001

CTX001 is an investigational *ex vivo* CRISPR gene-edited therapy that is being evaluated for patients suffering from TDT or severe SCD 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 TDT patients and painful and debilitating sickle crises for SCD patients. CTX001 is the most advanced gene-editing approach in development for beta thalassemia and SCD.

CTX001 is being developed under a co-development and co-commercialization agreement between CRISPR Therapeutics and Vertex.

About the CRISPR-Vertex Collaboration

CRISPR Therapeutics and Vertex 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 treatment to emerge from the joint research program. CRISPR Therapeutics and Vertex will jointly develop and commercialize CTX001 and 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 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 partnerships 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 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, including statements regarding CRISPR Therapeutics' expectations about any or all of the following: (i) the status of clinical trials (including, without limitation, the expected timing of data releases) and discussions with regulatory authorities related to product candidates under development by CRISPR Therapeutics and its collaborators, including expectations regarding the benefits of RMAT designation; (ii) the expected benefits of CRISPR Therapeutics' collaborations; 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, 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: the potential impacts due to the coronavirus pandemic, such as the timing and progress of clinical trials; 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 trial results; the potential that CTX001 clinical trial results may not be favorable; 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 the outcome of proceedings (such as an interference, an opposition or a similar proceeding) involving all or any portion of such intellectual property; and those risks and uncertainties described under the heading "Risk Factors" in CRISPR Therapeutics' most recent annual report on Form 10-K, 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. 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. 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.

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 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'sInnovation 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. 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, the information provided regarding the status of, and expectations with respect to, the CTX001 clinical development program and related global regulatory approvals, and expectations regarding the RMAT designation. 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 the development of CTX001 may not proceed or support registration 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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