



New Data for Investigational CRISPR/Cas9 Gene-Editing Therapy CTX001™ for Severe Hemoglobinopathies Accepted for Oral Presentation at the 25th European Hematology Association (EHA) Congress

May 14, 2020

ZUG, Switzerland and CAMBRIDGE, Mass. and BOSTON, May 14, 2020 (GLOBE NEWSWIRE) -- [CRISPR Therapeutics](#) (Nasdaq: CRSP) and [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced that new data from two ongoing Phase 1/2 clinical trials of the CRISPR/Cas9 gene-editing therapy CTX001 in severe hemoglobinopathies have been accepted for an oral presentation at the EHA Congress, which will take place virtually from June 11-14, 2020.

An abstract posted online today includes 12 months of follow-up data for the first patient treated in the ongoing Phase 1/2 CLIMB-111 trial in transfusion-dependent beta thalassemia (TDT) and 6 months of follow-up data for the first patient treated in the ongoing Phase 1/2 CLIMB-121 trial in severe sickle cell disease (SCD). Updated data will be presented at EHA, including longer duration follow-up data for the first two patients treated in these trials and initial data for the second patient treated in the CLIMB-111 trial.

The accepted abstract is now available on the EHA conference website: <https://ehaweb.org/congress/eha25/key-information-2/>.

Abstract Title: Initial Safety and Efficacy Results With a Single Dose of Autologous CRISPR-Cas9 Modified CD34+ Hematopoietic Stem and Progenitor Cells in Transfusion-Dependent β -Thalassemia and Sickle Cell Disease

Session Title: Immunotherapy - Clinical

Abstract Code: S280

About the Phase 1/2 Study in Transfusion-Dependent Beta Thalassemia

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 18 to 35 with TDT. The study 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 study.

About the Phase 1/2 Study in Sickle Cell Disease

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 18 to 35 with severe SCD. The study 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 study.

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) related to product candidates under development by CRISPR Therapeutics and its collaborators, including expectations regarding the data that is expected to be presented at the European Hematology Association's upcoming congress; (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'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. 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, information regarding the data that is expected to be presented at the European Hematology Association (EHA)'s upcoming Congress.

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.

(VRTX-GEN)

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