CRISPR Therapeutics Announces Oral Presentation of New Data on CTX001, a CRISPR Gene-Edited Therapy for β-Thalassemia and Sickle Cell Disease, at the ASH Annual Meeting

December 10, 2017

CRISPR gene-editing in hematopoietic stem cells demonstrates potential to treat β-thalassemia and sickle cell disease through upregulation of fetal hemoglobin

ZUG, Switzerland and CAMBRIDGE, Mass., Dec. 10, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP) today announced the presentation of new data on CTX001, an investigational CRISPR gene-edited therapy for patients suffering from β-thalassemia and sickle cell disease. The data were discussed in an oral presentation at the American Society of Hematology (ASH) Annual Meeting on Sunday, December 10th, 2017.

"With CTX001, we are able to efficiently edit hematopoietic stem cells to achieve high levels of fetal hemoglobin, a protein that is known to significantly reduce or even eliminate the transfusion dependence of β-thalassemia patients and the frequency of painful and debilitating sickle crises in sickle cell patients. These data strongly support the advancement of CTX001 into clinical trials in patients," commented Dr. Tony Ho, MD, Head of R&D of CRISPR Therapeutics.

CRISPR Therapeutics has filed a Clinical Trial Application for CTX001, and is planning to start a Phase 1/2 trial in β-thalassemia in Europe in 2018.

Key Data from ASH Session 112

Data presented at ASH demonstrate that CRISPR Therapeutics' proprietary CRISPR gene-editing approach results in high editing efficiency, with >90% of the hematopoietic stem cells edited at the target site. A vast majority of these cells are edited on both copies of the gene, which leads to expression levels of fetal hemoglobin of 40%, well above the level believed to be sufficient to ameliorate symptoms in patients with β-thalassemia and sickle cell disease.

CRISPR conducted extensive genome-wide off-target assessment including detailed analyses at over 6,000 sites, which showed no off-target editing. Full toxicology analysis also demonstrated that the CRISPR gene-editing had no adverse impact on engraftment of the hematopoietic stem cells and no other safety signals.

These results were presented in an oral session by Dr. Bill Lundberg, Chief Scientific Officer of CRISPR in a session entitled *CRISPR/Cas9 Genome Editing to Treat Sickle Cell Disease and β-Thalassemia: Re-Creating Genetic Variants to Upregulate Fetal Hemoglobin Appear Well-Tolerated, Effective and Durable*.

Slides from the ASH presentation are available under the Investors and Media tab on the CRISPR Therapeutics website.

About CTX001

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.

About the CRISPR-Vertex Collaboration

CTX001 is the first CRISPR/Cas9-based treatment to advance from a research program jointly conducted by CRISPR Therapeutics and Vertex Pharmaceuticals under the companies' collaboration aimed at the discovery and development of new gene editing treatments that use the CRISPR/Cas9 technology. Under the agreement, Vertex has exclusive rights to license up to six new CRISPR/Cas9-based treatments that emerge from the collaboration.

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.

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/Cas-9 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.

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