# CRISPR Therapeutics Submits First Clinical Trial Application for a CRISPR Gene-Edited Therapy, CTX001 in β-thalassemia

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**-Phase 1/2 trial in β-thalassemia expected to begin in 2018-**

ZUG, Switzerland and CAMBRIDGE, Mass., Dec. 07, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP) today announced the submission of a Clinical Trial Application (CTA) for CTX001 in β-thalassemia. CTX001 is an investigational CRISPR gene-edited autologous hematopoietic stem cell therapy for patients suffering from β-thalassemia and sickle cell disease.

"CRISPR Therapeutics is pioneering a new class of medicines with the CTA submission for CTX001 to conduct the first company-sponsored clinical trial of a CRISPR gene-edited therapy," commented Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "We are committed to translating the groundbreaking science of the CRISPR platform into therapies that can fundamentally change the lives of patients suffering from serious diseases such as β-thalassemia and sickle cell disease."

The Phase 1/2 trial of CTX001 is designed to assess its safety and efficacy in adult transfusion dependent β-thalassemia patients and is expected to begin in Europe in 2018. CRISPR also plans to file an Investigational New Drug Application for CTX001 to treat sickle cell disease with the United States Food and Drug Administration in 2018.

"β-thalassemia is a devastating disease that requires serious and chronic medical intervention," said Tony Ho, M.D., Head of Research and Development at CRISPR. "The efficient and precise editing in a patient's own blood cells using CRISPR provides the possibility of a one-time treatment for those suffering from β-thalassemia and sickle cell disease."

**About CTX001**

CTX001 is an investigational 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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