



CRISPR Therapeutics Announces FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to CTX130™ for the Treatment of Cutaneous T-Cell Lymphomas (CTCL)

ZUG, Switzerland and BOSTON, Sept. 28, 2022 (GLOBE NEWSWIRE) -- [CRISPR Therapeutics](#) (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today announced that the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to CTX130™, the Company's wholly-owned allogeneic CAR T cell therapy targeting CD70, for the treatment of Mycosis Fungoides and Sézary Syndrome (MF/SS).

"The RMAT designation is an important milestone for the CTX130 program that recognizes the transformative potential of our cell therapy in patients with T-cell lymphomas based upon encouraging clinical data to date," said Phuong Khanh (P.K.) Morrow, M.D., FACP, Chief Medical Officer of CRISPR Therapeutics. "We continue to work with a sense of urgency to bring our broad portfolio of allogeneic cell therapies to patients in need."

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.

About CTX130 and COBALT™ Trials

CTX130, a wholly-owned program of CRISPR Therapeutics, is a healthy donor-derived gene-edited allogeneic CAR T investigational therapy targeting Cluster of Differentiation 70, or CD70, an antigen expressed on various solid tumors and hematologic malignancies. CTX130 is being investigated in two ongoing independent Phase 1 single-arm, multi-center, open-label clinical trials that are designed to assess the safety and efficacy of several dose levels of CTX130 in adult patients. The COBALT-LYM trial is evaluating the safety and efficacy of CTX130 for the treatment of relapsed or refractory T or B cell malignancies. The COBALT-RCC trial is evaluating the safety and efficacy of CTX130 for the treatment of relapsed or refractory renal cell carcinoma. CTX130 has received Orphan Drug and Regenerative Medicine Advanced Therapy designations from the FDA.

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 Boston, 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 made by Dr. Morrow in this press release, as well as regarding CRISPR Therapeutics' expectations about any or all of the following: (i) the status of clinical trials and discussions with regulatory authorities related to product candidates under development by CRISPR Therapeutics including, without limitation, expectations regarding the benefits of RMAT designation; and (ii) 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 for initial and preliminary data from any clinical trial and initial data from a limited number of patients not to be indicative of final trial results; the potential that clinical trial results may not be favorable; potential impacts due to the coronavirus pandemic, such as the timing and progress of clinical trials; that future competitive or other market factors may adversely affect the commercial potential for CRISPR Therapeutics' product candidates; 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, 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. 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.

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