CRISPR Therapeutics Announces Completion of FDA Advisory Committee Meeting for Exagamglogene Autotemcel (exa-cel) for Severe Sickle Cell Disease

-Exa-cel PDUFA target action date is December 8, 2023 for severe sickle cell disease (SCD)-

ZUG, Switzerland and BOSTON, Oct. 31, 2023 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP) today announced the completion of the U.S. Food and Drug Administration's (FDA) Cellular, Tissue, and Gene Therapies Advisory Committee meeting for exagamglogene autotemcel (exa-cel) for the treatment of SCD in people ages 12 and older with recurrent vaso-occlusive crises (VOCs). Exa-cel is the first potential therapy to emerge from a strategic partnership between CRISPR Therapeutics and Vertex Pharmaceuticals.

If approved, exa-cel could be the first genetic therapy available to approximately twenty thousand people with severe SCD in the U.S. The FDA granted priority review for exa-cel in the treatment of people with SCD and assigned a Prescription Drug User Fee Act (PDUFA) action date of December 8, 2023. Exa-cel's Biologics License Application (BLA) for transfusion-dependent beta-thalassemia (TDT) was assigned a PDUFA date of March 30, 2024.

About the CRISPR Therapeutics and 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. Exa-cel represents the first potential treatment to emerge from the joint research program. Under an amended collaboration agreement, Vertex now leads global development, manufacturing and commercialization of exa-cel and splits program costs and profits worldwide 60/40 with CRISPR Therapeutics.

(CRSP-GEN)

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 collaborations 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 in Boston, Massachusetts and San Francisco, California, and business offices in London, United Kingdom. For more information, please visit www.crisprtx.com.

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CRISPR Therapeutics 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) exa-cel's potential for approval in TDT and SCD, including accelerated approval for SCD in the U.S., as well as the timing of any such approval by the FDA and that exa-cel could be the first genetic therapy available to eligible patients with SCD in the U.S. if approved; (ii) potential benefits of exa-cel for patients; (iii) timelines for and expectations regarding additional regulatory agency decisions; (iv) the benefits of its collaboration with Vertex; and (v) 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, existing and prospective investors are cautioned that forward-looking statements are inherently uncertain, are neither promises nor guarantees and not to place undue reliance on such statements, which speak only as of the date they are made. 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, that: the efficacy and safety results from ongoing clinical trials of exa-cel will not continue or be repeated in ongoing or planned clinical trials or may not support regulatory submissions; the FDA or other regulatory authorities may not approve exa-cel on a timely basis or at all; adequate pricing or reimbursement may not be secured to support continued development or commercialization of exa-cel following regulatory approval; future competitive or other market factors may adversely affect the commercial potential for exa-cel; CRISPR Therapeutics may not realize the potential benefits of its collaboration with Vertex; uncertainties regarding the intellectual property protection for CRISPR Therapeutics' technology and intellectual property belonging to third parties; 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. 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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