CRISPR Therapeutics Announces U.S. Food and Drug Administration (FDA) Approval of CASGEVY™ (exagamglogene autotemcel) for the Treatment of Transfusion-Dependent Beta Thalassemia

- Approximately 1,000 patients in the U.S. 12 years of age and older are now eligible for this one-time treatment-

ZUG, Switzerland and BOSTON, Mass., January 16, 2024 – 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) has approved CASGEVY™ (exagamglogene autotemcel [exa-cel]), a CRISPR/Cas9 gene-edited cell therapy, for the treatment of transfusion-dependent beta thalassemia (TDT) in patients 12 years and older.

"We are pleased with the approval of CASGEVY in TDT well ahead of the PDUFA date," said Samarth Kulkarni, Ph.D., Chairman and Chief Executive Officer of CRISPR Therapeutics. "The approval is a reflection of the power and versatility of the CRISPR platform to bring a potentially curative treatment option to patients suffering from this devastating disease."

The administration of CASGEVY requires experience in stem cell transplantation; therefore, our partner Vertex Pharmaceuticals Incorporated, is engaging with experienced hospitals to establish a network of independently operated, authorized treatment centers (ATCs) throughout the U.S. to offer CASGEVY to patients. All nine ATCs activated in the U.S. are able to offer CASGEVY to eligible patients with TDT and sickle cell disease (SCD). Additional ATCs will be activated in the coming weeks and a complete list of ATCs can be accessed at CASGEVY.com.

About Transfusion-Dependent Beta Thalassemia (TDT)
TDT is a serious, life-threatening genetic disease. TDT patients report health-related quality of life scores below the general population and the lifetime health care costs in the U.S. of managing TDT are estimated between $5 and $5.7 million. TDT requires frequent blood transfusions and iron chelation therapy throughout a person's life. Due to anemia, patients living with TDT may experience fatigue and shortness of breath, and infants may develop failure to thrive, jaundice and feeding problems. Complications of TDT can also include an enlarged spleen, liver and/or heart, misshapen bones and delayed puberty. TDT requires lifelong treatment and significant use of health care resources, and ultimately results in reduced life expectancy, decreased quality of life and reduced lifetime earnings and productivity. In the U.S., the median age of death for patients living with TDT is 37 years. Stem cell transplant from a matched donor is a curative option but is only available to a small fraction of people living with TDT because of the lack of available donors.

About CASGEVY™ (exagamglogene autotemcel [exa-cel])
CASGEVY™ is a non-viral, *ex vivo* CRISPR/Cas9 gene-edited cell therapy for eligible patients with SCD or TDT, in which a patient's own hematopoietic stem and progenitor cells are edited at the erythroid specific enhancer region of the *BCL11A* gene through a precise double-strand break. This edit results in the production of high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is the form of the oxygen-carrying hemoglobin that is naturally present during fetal development, which then switches

to the adult form of hemoglobin after birth. CASGEVY has been shown to reduce or eliminate VOCs for patients with SCD and transfusion requirements for patients with TDT.

CASGEVY is approved for certain indications in multiple jurisdictions for eligible patients.

About the CRISPR Collaboration and Vertex
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. CASGEVY™ (exagamglogene autotemcel [exa-cel]) represents the first treatment to emerge from the joint research program. Under an amended collaboration agreement, Vertex now leads global development, regulatory, manufacturing and commercialization of exa-cel and splits program costs and profits worldwide 60/40 with CRISPR Therapeutics.

About CRISPR Therapeutics
Since its inception over a decade ago, CRISPR Therapeutics has transformed from a research-stage company advancing programs in the field of gene editing, to a company with a diverse portfolio of product candidates across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine, cardiovascular and rare diseases. The Nobel Prize-winning CRISPR science has revolutionized biomedical research and represents a powerful, clinically validated approach with the potential to create a new class of potentially transformative medicines. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic partnerships with leading companies including Bayer and Vertex Pharmaceuticals. 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 San Francisco, California, and business offices in London, United Kingdom. To learn more, 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 made by Samarth Kulkarni, Ph.D. in this press release, as well as statements regarding CRISPR Therapeutics': (i) plans and expectations for the commercialization of, and anticipated benefits of, CASGEVY, including the anticipated patient population eligible for CASGEVY in the United States and plans for patient access to CASGEVY; (ii) timelines for and expectations regarding additional regulatory agency decisions; and (iii) expectations regarding 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 clinical data from ongoing clinical trials of exa-cel will not continue or be repeated in ongoing or planned clinical trials or may not support regulatory approval or renewal of conditional authorization; regulatory approval in other jurisdictions may not occur on anticipated timelines 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 CASGEVY; CRISPR Therapeutics may not realize the potential benefits of its collaboration with Vertex; there are 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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