CRISPR Therapeutics Provides Business Update and Reports First Quarter 2021 Financial Results

- Granted Priority Medicines designation by the European Medicines Agency for CTX001™ for transfusion-dependent beta thalassemia (TDT)-

-More than 30 patients have been dosed with CTX001 across CLIMB-Thal-111 and CLIMB-SCD-121 to date; completion of enrollment in both trials is expected in 2021-

-Enrollment ongoing in CTX110™, CTX120™ and CTX130™ clinical trials-

ZUG, Switzerland and CAMBRIDGE, Mass., April 27, 2021 – CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today reported financial results for the first quarter ended March 31, 2021.

"We continue to make progress across our hemoglobinopathies and immuno-oncology programs on the clinical front, and expect to disclose data on multiple programs in 2021," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "Our revised agreement with Vertex for CTX001 streamlines operations and allows us to further invest in innovation to build upon the remarkable results we have seen with the program thus far. In addition, we are continuing to execute on our earlier stage pipeline and look forward to bringing our regenerative medicine and *in vivo* programs to the clinic."

Recent Highlights and Outlook

- **Beta Thalassemia and Sickle Cell Disease**
    - The European Medicines Agency granted Priority Medicines (PRIME) designation to CTX001™, an investigational, autologous, *ex vivo* CRISPR/Cas9 gene-edited therapy for the treatment of transfusion-dependent beta thalassemia (TDT). CTX001 was previously granted PRIME designation for the treatment of sickle cell disease (SCD) in 2020.
    - Enrollment and dosing are ongoing in the clinical trials for CTX001. More than 30 patients have been dosed with CTX001 across both trials to date. Completion of enrollment in both trials is expected in 2021.
- **Immuno-Oncology**
    - The Company expects to report additional data in 2021 from its ongoing Phase 1 CARBON trial assessing the safety and efficacy of several dose levels of CTX110, its wholly-owned allogeneic chimeric antigen receptor T cell (CAR-T) investigational therapy targeting CD19, for the treatment of relapsed or refractory B-cell malignancies.
    - CRISPR Therapeutics' Phase 1 clinical trial assessing the safety and efficacy of several dose levels of CTX120, its wholly-owned allogeneic CAR-T investigational therapy targeting B-

cell maturation antigen for the treatment of relapsed or refractory multiple myeloma, is ongoing. The Company expects to report top-line data from this trial in 2021.

- CRISPR Therapeutics' two independent Phase 1 clinical trials assessing the safety and efficacy of several dose levels of CTX130, its wholly-owned allogeneic CAR-T investigational therapy targeting CD70, for the treatment of both solid tumors and certain hematologic malignancies, are ongoing. The Company expects to report top-line data from these trials in 2021. Earlier this month, the Company announced preclinical data from its CAR-T program at the American Association for Cancer Research (AACR) Annual Meeting 2021. The data, presented in an e-poster session entitled, "*CD70 knockout: A novel approach to augment CAR-T cell function, found that the generation of CAR-T cells including knockout of the CD70 show improved properties, including potency and persistence over CAR-T cells where the CD70 gene remains intact*."

- **Regenerative Medicine**

  - CRISPR Therapeutics and its partner ViaCyte remain on track to initiate a Phase 1/2 trial of their allogeneic stem cell-derived therapy for the treatment of Type 1 diabetes in 2021. The combination of ViaCyte's stem cell capabilities and CRISPR's gene editing capabilities has the potential to enable a beta-cell replacement product that may deliver durable benefit to patients without requiring immune suppression.

- **Other Corporate Matters**

  - Earlier this month, CRISPR Therapeutics and its partner Vertex announced that the companies have amended their collaboration agreement to develop, manufacture and commercialize CTX001. Under the terms of the amended agreement, CRISPR Therapeutics will be responsible for 40% of the program costs and will receive 40% of the profits from future sales of CTX001 worldwide. With this revised agreement, Vertex will deploy the breadth of its established global capabilities and proven experience in development, manufacturing, and commercialization to maximize the potential for CTX001 to transform the lives of tens of thousands of patients in the U.S., Europe and other countries. CRISPR Therapeutics will continue to support the development of CTX001 and invest in further innovation to maximize its potential. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.

First Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1,806.2 million as of March 31, 2021, compared to $1,690.3 million as of December 31, 2020. The increase in cash of $115.9

million was primarily driven by cash from financing activities of $226.0 million, which consists primarily of proceeds received from sales under our "at-the-market" offering in January and early February of 2021 offset by continuing operating expenses.

- **Revenue:** Total collaboration revenue was $0.2 million for the first quarter of 2021 compared to $0.2 million for first quarter of 2020. Collaboration revenue primarily consisted of charges to partners for research activities.

- **R&D Expenses:** R&D expenses were $90.6 million for the first quarter of 2021 compared to $54.2 million for the first quarter of 2020. The increase in expense was driven by development activities supporting the advancement of the hemoglobinopathies program and wholly-owned immuno-oncology programs, as well as increased headcount and supporting facilities related expenses.

- **G&A Expenses:** General and administrative expenses were $24.5 million for the first quarter of 2021 compared to $19.6 million for the first quarter of 2020. The increase in general and administrative expenses for the year was primarily driven by headcount-related expense.

- **Net Income/Loss:** Net loss was $113.2 million for the first quarter of 2021 compared to a net loss of $69.7 million for the first quarter of 2020.

About CTX001

CTX001 is an investigational, autologous, *ex vivo* CRISPR/Cas9 gene-edited therapy that is being evaluated for patients suffering from TDT or severe SCD, in which a patient's hematopoietic stem cells are edited 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, which then switches to the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion requirements for patients with TDT and reduce painful and debilitating sickle crises for patients with SCD.

Based on progress in this program to date, CTX001 has been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug, and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) for both TDT and SCD. CTX001 has also been granted Orphan Drug Designation from the European Commission for both TDT and SCD, as well as Priority Medicines (PRIME) designation from the European Medicines Agency (EMA) for SCD and TDT.

About the CRISPR-Vertex Collaboration

Vertex and CRISPR Therapeutics 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. CTX001 represents the first potential treatment to emerge from the joint research program. Under a recently amended collaboration agreement, Vertex will lead global development and commercialization of CTX001 and split program costs and profits worldwide 60/40 with CRISPR Therapeutics. This amendment is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.

About CLIMB-Thal-111
The ongoing Phase 1/2 open-label trial, CLIMB-Thal-111, is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with TDT. The trial will enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up trial.

About CLIMB-SCD-121
The ongoing Phase 1/2 open-label trial, CLIMB-SCD-121, is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with severe SCD. The trial will enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up trial.

About CTX110
CTX110, a wholly owned program of CRISPR Therapeutics, is a healthy donor-derived gene-edited allogeneic CAR-T investigational therapy targeting cluster of differentiation 19, or CD19. CTX110 is being investigated in the ongoing CARBON trial.

About CARBON
The ongoing Phase 1 single-arm, multi-center, open label clinical trial, CARBON, is designed to assess the safety and efficacy of several dose levels of CTX110 for the treatment of relapsed or refractory B-cell malignancies.

About CTX120
CTX120, a wholly-owned program of CRISPR Therapeutics, is a healthy donor-derived gene-edited allogeneic CAR-T investigational therapy targeting B-cell maturation antigen, or BCMA. CTX120 is being investigated in an ongoing Phase 1 single-arm, multi-center, open-label clinical trial designed to assess the safety and efficacy of several dose levels of CTX120 for the treatment of relapsed or refractory multiple myeloma. CTX120 has been granted Orphan Drug designation from the FDA.

About CTX130
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 developed for the treatment of both solid tumors, such as renal cell carcinoma, and T-cell and B-cell 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 for the treatment of relapsed or refractory renal cell carcinoma and various subtypes of lymphoma, respectively.

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

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 Dr. Kulkarni in this press release, as well as statements regarding CRISPR Therapeutics' expectations about any or all of the following: (i) the safety, efficacy and clinical progress of CRISPR Therapeutics' various clinical programs, including CTX001, CTX110, CTX120 and CTX130; (ii) the status of clinical trials and preclinical studies (including, without limitation, the expected timing of data releases and development, as well as completion of clinical trials) and development timelines for CRISPR Therapeutics' product candidates; (iii) the data that will be generated by ongoing and planned clinical trials, and the ability to use that data for the design and initiation of further clinical trials, including expectations regarding the CTX001 and CTX110 data and CAR-T CD70 preclinical data; (iv) the actual or potential benefits of regulatory designations; (v) the timing of the potential closing of the transaction contemplated by the amended collaboration agreement, future activities of the parties pursuant to the collaboration and the potential benefits of CRISPR Therapeutics' collaboration with Vertex, as well as other partners; (vi) the intellectual property coverage and positions of CRISPR Therapeutics, its licensors and third parties as well as the status and potential outcome of proceedings involving any such intellectual property; (vii) the sufficiency of CRISPR Therapeutics' cash resources; and (viii) 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; that one or more of CRISPR Therapeutics' internal or external product candidate programs will not proceed as planned for technical, scientific or commercial reasons; that future competitive or other market factors may adversely affect the commercial potential for CRISPR Therapeutics' product candidates; uncertainties inherent in the initiation and completion of preclinical studies for CRISPR Therapeutics' product candidates (including, without limitation, availability and timing of results and whether such results will be predictive of future results of the future trials); uncertainties about regulatory approvals to conduct trials or to market products; the potential impacts due to the coronavirus pandemic such as (x) delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy; (y) the timing and progress of clinical trials, preclinical studies and other research and development activities; and (z) the overall impact of the coronavirus pandemic on its business, financial condition and results of operations; 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 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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Investor Contact:
Susan Kim
+1-617-307-7503
susan.kim@crisprtx.com

Media Contact:
Jennifer Paganelli
Real Chemistry on behalf of CRISPR
+1-347-658-8290
jpaganelli@realchemistry.com

CRISPR Therapeutics AG
Condensed Consolidated Statements of Operations
(Unaudited, In thousands except share data and per share data)

	Three Months Ended March 31,	
	2021	2020
Revenue:		
Collaboration revenue	$ 202	$ 157
Grant revenue	337	—
Total revenue	$ 539	$ 157
Operating expenses:		
Research and development	90,565	54,193
General and administrative	24,517	19,550
Total operating expenses	115,082	73,743
Loss from operations	(114,543)	(73,586)
Total other income (expense), net	1,955	4,232
Net loss before income taxes	(112,588)	(69,354)
Provision for income taxes	(575)	(377)
Net loss	(113,163)	(69,731)
Foreign currency translation adjustment	5	(25)
Unrealized loss on marketable securities	(383)	—
Comprehensive loss	$ (113,541)	$ (69,756)
Net loss per common share — basic	$ (1.51)	$ (1.15)
Basic weighted-average common shares outstanding	75,005,187	60,847,683
Net loss per common share — diluted	$ (1.51)	$ (1.15)
Diluted weighted-average common shares outstanding	75,005,187	60,847,683

CRISPR Therapeutics AG
Condensed Consolidated Balance Sheets Data
(Unaudited, in thousands)

	As of	
	March 31, 2021	December 31, 2020
Cash	$ 1,126,059	$ 1,168,620
Marketable securities	680,144	521,713
Working capital	1,749,269	1,622,361
Total assets	1,957,910	1,827,966
Total shareholders' equity	1,805,498	1,664,234