



CRISPR Therapeutics Provides Business Update and Reports Second Quarter 2020 Financial Results

-Dosing re-initiated in clinical trials of CTX001™ for patients with severe hemoglobinopathies-

- CTX001 has received orphan drug designation from the U.S. FDA for sickle cell disease (SCD)-

-Expands regenerative medicine portfolio through a collaboration with University Health Network (UHN), gaining access to hepatocytes-

ZUG, Switzerland and CAMBRIDGE, Mass., July 27, 2020 -- CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today reported financial results for the second quarter ended June 30, 2020.

“We continue to make substantial progress driving our multiple, ongoing clinical development programs. Enrollment in our immuno-oncology trials is ongoing, and we’ve re-initiated dosing in our CTX001 trials,” said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. “Further, we expect to report data from our CTX001 program targeting hemoglobinopathies and our CTX110 program later this year. Despite the challenges posed by COVID-19, we continue to execute on our programs and remain focused on our commitment to patients and their families.”

Recent Highlights and Outlook

- **Beta Thalassemia and Sickle Cell Disease**

- CRISPR Therapeutics and its partner Vertex provided new clinical data at the European Hematology Association (EHA) Congress from the two ongoing Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 in patients with transfusion-dependent beta thalassemia (TDT) and in patients with severe sickle cell disease (SCD). Data from two TDT patients demonstrated clinical proof-of-concept for CTX001 in this disease, and longer duration data from one SCD patient showed durable effects on fetal hemoglobin (HbF) levels and the patient was free of vaso-occlusive crises. Screening, enrollment and mobilization in these studies are ongoing; conditioning and dosing have been resumed following temporary COVID-19-related pauses in both studies. CRISPR Therapeutics and Vertex expect to report data from additional patients in the second half of 2020.
- In May, CRISPR Therapeutics and its partner Vertex announced that the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to CTX001, an investigational, autologous, gene-edited hematopoietic stem cell therapy, for the treatment of TDT and SCD. In addition to RMAT designation, CTX001 has received Orphan Drug Designation from the U.S. FDA for TDT and SCD and from the European Commission for TDT and SCD. CTX001 also has Fast Track Designation from the U.S. FDA for both TDT and SCD.

- **Immuno-Oncology**

- Patient dosing continues in a clinical trial to assess the safety and efficacy of CTX110™, CRISPR Therapeutics’ wholly-owned allogeneic CAR-T cell therapy targeting relapsed or refractory

CD19+ B-cell malignancies. The Company expects to report top-line data for CTX110 at the end of 2020.

- Patient dosing continues in a clinical trial to assess the safety and efficacy of CTX120™, CRISPR Therapeutics' wholly-owned allogeneic CAR-T cell therapy targeting BCMA for the treatment of relapsed or refractory multiple myeloma.
- Two independent clinical trials assessing the safety and efficacy of CTX130™, CRISPR Therapeutics' wholly-owned allogeneic CAR-T cell therapy targeting CD70 for the treatment of both solid tumors and certain hematologic malignancies, are open for enrollment.

- **Other Corporate Matters**

- In June, CRISPR Therapeutics announced the pricing of an underwritten public offering of 6,428,572 common shares at a public offering price of \$70.00 per share, plus the exercise in full of the underwriters' option to purchase 964,285 additional common shares. Gross proceeds from the offering (including the exercise of the underwriters' option), before deducting underwriting discounts and commissions and other offering expenses, were \$517.5 million. The common stock offering and the option to purchase additional shares closed in July 2020.
- CRISPR Therapeutics today announced a research agreement with UHN, Canada's largest research hospital, affiliated with the University of Toronto, and a member of the Toronto Academic Health Science Network. Through UHN's McEwen Stem Cell Institute, the aim of the collaboration is to combine CRISPR Therapeutics' gene editing technology with UHN's methods for differentiating stem cells into hepatocytes at high yield and purity, with the goal of developing regenerative medicine cell therapies for a number of different diseases. The agreement provides CRISPR Therapeutics an option to commercialize the technology.
- In June, CRISPR Therapeutics presented four posters at the American Association for Cancer Research (AACR) Virtual Annual Meeting II. The posters addressed the potential of CRISPR-modified CAR-T cells as follows: an assessment of CRISPR-modified CAR-T cells in patients with non-small cell lung cancer; functionality *in vivo* and *in vitro* of allogeneic CAR-T cell products containing multiple CRISPR/Cas9 gene edits; assessment of allogeneic anti-PTK7 CAR-T cells for the treatment of solid tumors; and the potential of CRISPR/Cas9-generated anti-CD70 allogeneic CAR-T cells to target T cell lymphomas.
- CRISPR Therapeutics today announced it entered into a lease agreement with Breakthrough Properties for a new location in Boston, Massachusetts. The new facility will consolidate CRISPR's various office and laboratory locations in the greater Boston area into a single location and support the Company's anticipated future growth for five to seven years from the date of occupancy, which is expected in 2022.
- In June, CRISPR Therapeutics announced that it is building a new cell therapy manufacturing facility in Framingham, Massachusetts, for clinical and commercial production of the Company's investigational cell therapy product candidates. The facility is being designed to provide GMP manufacturing according to FDA and European Medicines Agency (EMA)

regulations and guidelines to support clinical supply and commercial product upon potential regulatory approval.

Second Quarter 2020 Financial Results

- **Cash Position:** Cash and cash equivalents as of June 30, 2020 were \$945.1 million compared to \$889.7 million as of March 31, 2020, an increase of \$55.4 million. The increase in cash was primarily driven by financing activities during the quarter of \$89.5 million and the \$25.0 million milestone received from Vertex in April. The increase was offset by cash used in operating activities during the quarter of \$54.3 million (exclusive of the \$25.0 million milestone received by Vertex in April) to support spending on the Company's clinical and pre-clinical programs, as well as payroll and payroll-related expenses to support growth. After including the \$484.8 million in net proceeds from our underwritten public offering completed in July, pro forma cash exceeds \$1.4 billion.
- **Revenue:** Total collaboration revenue was less than \$0.1 million for the second quarter of 2020 compared to \$0.3 million for second quarter of 2019. Collaboration revenue primarily consisted of charges to partners for research activities.
- **R&D Expenses:** R&D expenses were \$59.4 million for the second quarter of 2020 compared to \$39.5 million for the second quarter of 2019. The increase in expenses was driven by increased headcount and development activities supporting the advancement of the hemoglobinopathies program and wholly-owned immuno-oncology programs.
- **G&A Expenses:** General and administrative expenses were \$21.4 million for the second quarter of 2020 compared to \$15.8 million for the second quarter of 2019. The increase in general and administrative expenses for the year was driven by headcount-related expense and higher facilities cost.
- **Net Loss:** Net loss was \$79.7 million for the second quarter of 2020 compared to net loss of \$53.7 million for the second quarter of 2019.

About CTX001™

CTX001 is an investigational *ex vivo* CRISPR gene-edited therapy that is being evaluated for patients suffering from TDT or severe SCD 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 TDT patients and painful and debilitating sickle crises for SCD patients.

CTX001 is being developed under a co-development and co-commercialization agreement between CRISPR Therapeutics and Vertex.

About CTX110™

CTX110 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting cluster differentiation 19, or CD19, for the treatment of CD19+ malignancies. A wholly-owned asset of CRISPR Therapeutics,

CTX110 is being investigated in a clinical trial designed to assess the safety and efficacy of CTX110 for the treatment of relapsed or refractory B-cell malignancies. The multi-center, open-label clinical trial is designed to enroll up to 131 patients and investigate several dose levels of CTX110.

About CTX120™

CTX120 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting B-cell maturation antigen, or BCMA. A wholly-owned asset of CRISPR Therapeutics, CTX120 is being investigated in a clinical trial designed to assess the safety and efficacy of CTX120 for the treatment of relapsed or refractory multiple myeloma. The multi-center, open-label clinical trial is designed to enroll up to 88 patients and investigate several dose levels of CTX120.

About CTX130™

CTX130 is a healthy donor-derived gene-edited allogeneic CAR-T 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. A wholly-owned asset of CRISPR Therapeutics, CTX130 is being investigated in two independent clinical trials that are designed to assess the safety and efficacy of CTX130 for the treatment of relapsed or refractory renal cell carcinoma and various subtypes of lymphoma, respectively. The multi-center, open-label clinical trial investigating CTX130 for the treatment of relapsed or refractory renal cell carcinoma is designed to enroll approximately 95 patients and investigate several dose levels of CTX130. The multi-center, open-label clinical trial investigating CTX130 for the treatment of various lymphomas is designed to enroll approximately 46 patients and investigate several dose levels of CTX130.

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 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 (including, without limitation, the expected timing of data releases and activities at clinical trial sites), development timelines and discussions with regulatory authorities related to product candidates under development by CRISPR Therapeutics and its collaborators; (iii) the number of patients that will be evaluated, the anticipated date by which enrollment will be completed and the data that will be generated by ongoing and planned clinical

trials, including expectations regarding the data that was presented at the European Hematology Association's virtual congress, and the ability to use that data for the design and initiation of further clinical trials; (iv) potential impacts due to the coronavirus pandemic; (v) the actual or potential benefits of FDA designations, such as orphan drug, fast track and regenerative medicine advanced therapy, and European equivalents; (vi) CRISPR Therapeutics' ability to build out new facilities in anticipated timeframes and need for infrastructure expansion; (vii) 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; (viii) the sufficiency of CRISPR Therapeutics' cash resources; (ix) the expected benefits of CRISPR Therapeutics' collaborations; and (x) 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 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; the potential for initial and preliminary data from any clinical trial (including CTX001, CTX110, CTX120 and CTX130) not to be indicative of final trial results; the risk that the initial data from a limited number of patients (as is the case with CTX001 at this time) may not be indicative of results from the full planned study population; the outcomes for each CRISPR Therapeutics' planned clinical trials and studies 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; availability and timing of results from preclinical studies; whether results from a preclinical trial will be predictive of future results of the future trials; uncertainties about regulatory approvals to conduct trials or to market products; 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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CRISPR Therapeutics AG
Condensed Consolidated Statements of Operations
(Unaudited, In thousands except share data and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Collaboration revenue	\$ 44	\$ 318	\$ 201	\$ 646
Operating expenses:				
Research and development	59,380	39,533	113,573	73,355
General and administrative	21,353	15,768	40,903	30,697
Total operating expenses	<u>80,733</u>	<u>55,301</u>	<u>154,476</u>	<u>104,052</u>
Loss from operations	(80,689)	(54,983)	(154,275)	(103,406)
Total other income, net	1,412	1,369	5,644	1,469
Net loss before income taxes	(79,277)	(53,614)	(148,631)	(101,937)
Provision for income taxes	(379)	(85)	(756)	(170)
Net loss	(79,656)	(53,699)	(149,387)	(102,107)
Foreign currency translation adjustment	(3)	(10)	(28)	(2)
Comprehensive loss	<u>\$ (79,659)</u>	<u>\$ (53,709)</u>	<u>\$ (149,415)</u>	<u>\$ (102,109)</u>
Reconciliation of net loss to net loss attributable to common shareholders:				
Net loss	\$ (79,656)	\$ (53,699)	\$ (149,387)	\$ (102,107)
Net loss per share attributable to common shareholders - basic	<u>\$ (1.30)</u>	<u>\$ (1.01)</u>	<u>\$ (2.44)</u>	<u>\$ (1.94)</u>
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic	<u>61,420,746</u>	<u>53,188,041</u>	<u>61,134,214</u>	<u>52,643,649</u>

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

	As of	
	June 30, 2020	December 31, 2019
Cash	\$ 945,068	\$ 943,771
Working capital	891,113	930,441
Total assets	1,046,532	1,066,752
Total shareholders' equity	910,670	939,425

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