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

-Reported positive top-line results from the Company's ongoing Phase 1 CARBON trial evaluating the safety and efficacy of CTX110™, targeting CD19+ B-cell malignancies-

- Received Rare Pediatric Disease designation from the U.S. Food and Drug Administration (FDA) for CTX001™ for sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT)-

-Received Orphan Drug Designation (ODD) for Phase 1 clinical trial of CTX120™, targeting B-cell maturation antigen (BCMA)-

-Began treating patients in two Phase 1 clinical trials of CTX130™, targeting CD70 for the treatment of both solid tumors and certain hematologic malignancies-

ZUG, Switzerland and CAMBRIDGE, Mass., Oct. 28, 2020 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today reported financial results for the third quarter ended September 30, 2020.

"The Nobel Prize in Chemistry provides a timely recognition of the groundbreaking potential of CRISPR/Cas9 as our team at CRISPR Therapeutics continues to make important progress in our immuno-oncology and hemoglobinopathies clinical trials. Earlier this month, we were pleased to report positive top-line data from our Phase 1 CARBON trial of CTX110 in patients with relapsed or refractory B-cell malignancies, which demonstrate the potential of CRISPR gene editing for the treatment of cancers. Enrollment in our CTX120 and CTX130 trials continue and we expect to report initial data for these programs next year," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "In parallel, our clinical trials in hemoglobinopathies continue to advance rapidly and we expect to report additional data from our CTX001 program later this year."

Recent Highlights and Outlook

- **Beta Thalassemia and Sickle Cell Disease**
    - CRISPR Therapeutics and Vertex previously announced that, as of June 2020, seven patients had been dosed across its two Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 and presented data at the European Hematology Association Congress from two TDT patients and one SCD patient. Additional patients have been enrolled and dosed in both TDT and SCD studies and the Company expects to report clinical data from more patients treated with CTX001 in addition to data from patients with longer follow-up in the fourth quarter.
    - The European Medicines Agency granted Priority Medicines (PRIME) designation to CTX001 for the treatment of severe SCD. CTX001 has also been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug, and Rare Pediatric Disease designations from the FDA and ODD from the European Commission for both TDT and SCD.
- **Immuno-Oncology**
    - On October 21, 2020, CRISPR Therapeutics announced positive top-line results from its ongoing Phase 1 CARBON trial evaluating the safety and efficacy of CTX110, its wholly-owned allogeneic CAR-T investigative therapy targeting CD19+ B-cell malignancies. The data showed early evidence of a dose dependent response to CTX110 and, at DL3, demonstrated a 50% (2/4) complete response (CR) rate and an acceptable safety profile at DL3 or below. Both responders at DL3 remained in CR at the 3-month assessment. The Company expects to report additional data from this trial in 2021.
    - CRISPR Therapeutics received ODD from the FDA for CTX120, the Company's wholly-owned allogeneic CAR-T investigative therapy targeting BCMA for the treatment of relapsed or refractory multiple myeloma. CRISPR Therapeutics continues to enroll and dose patients in a Phase 1 clinical trial assessing the safety and efficacy of CTX120. The Company expects to report top-line data from this trial in 2021.

- CRISPR Therapeutics continues to enroll and dose patients in two independent Phase 1 clinical trials assessing the safety and efficacy of CTX130, the Company's wholly-owned allogeneic CAR-T investigative therapy targeting CD70 for the treatment of both solid tumors and certain hematologic malignancies. The Company expects to report top-line data from this trial in 2021.

- **Regenerative Medicine**

  - CRISPR Therapeutics, together with its partner ViaCyte, are planning to initiate a Phase 1/2 trial of its allogeneic stem cell-derived therapy for the treatment of Type 1 diabetes in 2021. CRISPR Therapeutics and ViaCyte entered into a strategic collaboration focused on the development and commercialization of novel regenerative medicines including gene-edited allogeneic stem cell-derived therapies for the treatment of diabetes in 2018. 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**

  - In October, Professor Emmanuelle Charpentier, CRISPR Therapeutics' co-founder, was awarded the 2020 Nobel Prize in Chemistry for her groundbreaking work on the CRISPR/Cas9 system. She is Founding, Scientific and Managing Director of the Max Planck Unit for the Science of Pathogens and Honorary Professor at Humboldt University, Berlin, Germany.

Third Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities of $1.4 billion as of September 30, 2020, compared to $945.1 million as of June 30, 2020, an increase of $420.1 million. The increase in cash was primarily driven by our July public offering, which resulted in net proceeds of approximately $484.8 million, offset by cash used for operations to support spending on the Company's clinical and pre-clinical programs, as well as payroll and payroll-related expenses to support growth.

- **Revenue:** Total collaboration revenue was $0.1 million for the third quarter of 2020 compared to $211.9 million for third quarter of 2019, which resulted from the collaboration agreements with Vertex effective in the third quarter of 2019.

- **R&D Expenses:** R&D expenses were $71.0 million for the third quarter of 2020 compared to $57.2 million for the third quarter of 2019. The increase in expenses was driven by increased headcount and supporting facilities related expenses, as well as development activities supporting the advancement of the hemoglobinopathies program and wholly-owned immuno-oncology programs.

- **G&A Expenses:** General and administrative expenses were $21.5 million for the third quarter of 2020 compared to $15.5 million for the third quarter of 2019. The increase in general and administrative expenses for the year was driven by headcount-related expense.

- **Net Loss:** Net loss was $92.4 million for the third quarter of 2020 compared to net income of $138.4 million for the third quarter of 2019.

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 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, which then switches to the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion requirements for TDT patients and reduce painful and debilitating sickle crises for SCD patients.

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). 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.

CTX001 is being developed under a co-development and co-commercialization agreement between CRISPR Therapeutics and Vertex. Among gene-editing approaches being investigated/evaluated for TDT and SCD, CTX001 is the furthest advanced in clinical development.

About CLIMB-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-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 investigative therapy targeting cluster of differentiation 19, or CD19. CTX110 is being investigated in the 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 asset of CRISPR Therapeutics, is a healthy donor-derived gene-edited allogeneic CAR-T investigative 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.

Based on progress to date in this program targeting B-cell maturation antigen (BCMA), CTX120 has been granted Orphan Drug designation from the FDA.

About CTX130™
CTX130, a wholly-owned asset of CRISPR Therapeutics, is a healthy donor-derived gene-edited allogeneic CAR-T investigative 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 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; (ii) the status of clinical trials (including, without limitation, expectations regarding the data that is being presented, the expected timing of data releases and development); (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 that was recently presented; (iv) the actual or potential benefits of regulatory designations; (v) 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; (vi) the sufficiency of CRISPR Therapeutics' cash resources; (vii) the expected benefits of CRISPR Therapeutics' collaborations; 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, 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Collaboration revenue	$ 148	$ 211,928	$ 349	$ 212,574
Operating expenses:				
Research and development	71,008	57,246	184,581	130,601
General and administrative	21,539	15,519	62,442	46,216
Total operating expenses	92,547	72,765	247,023	176,817
Income (Loss) from operations	(92,399)	139,163	(246,674)	35,757
Total other income (expense), net	160	(466)	5,804	1,003
Net income (loss) before income taxes	(92,239)	138,697	(240,870)	36,760
Provision for income taxes	(200)	(274)	(956)	(444)
Net income (loss)	(92,439)	138,423	(241,826)	36,316
Foreign currency translation adjustment	31	(12)	3	(14)
Unrealized loss on marketable securities	(144)	—	(144)	—
Comprehensive income (loss)	$ (92,552)	$ 138,411	$ (241,967)	$ 36,302
Net income (loss) per common share - basic	$ (1.32)	$ 2.52	$ (3.77)	$ 0.68
Basic weighted-average common shares outstanding	70,143,481	54,829,057	64,159,224	53,380,123
Net income (loss) per common share - diluted	$ (1.32)	$ 2.40	$ (3.77)	$ 0.65
Diluted weighted-average common shares outstanding	70,143,481	57,598,901	64,159,224	55,821,420

Condensed Consolidated Balance Sheets Data

(Unaudited, in thousands)

	As of	
	September 30, 2020	December 31, 2019
Cash	$ 1,041,417	$ 943,771
Marketable securities	324,573	—
Working capital	1,306,790	930,441
Total assets	1,485,018	1,066,752
Total shareholders' equity	1,343,301	939,425

Investor Contact:
Susan Kim
+1-617-307-7503
susan.kim@crisprtx.com

Media Contact:
Rachel Eides
WCG on behalf of CRISPR
+1-617-337-4167
reides@wcgworld.com

Source: CRISPR Therapeutics AG