CRISPR Therapeutics Provides Business Update and Reports First Quarter 2019 Financial Results

-Enrollment ongoing in Phase 1/2 clinical trials of CTX001™ for patients with severe hemoglobinopathies-

-IND and CTA approved for CTX110™, wholly-owned allogeneic CAR-T cell therapy targeting CD19+ malignancies-

-On track to initiate Phase 1/2 clinical trial for CTX110 in 1H 2019-

-$437.5 million in cash as of March 31, 2019-

ZUG, Switzerland and CAMBRIDGE, Mass., April 29, 2019 (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 first quarter ended March 31, 2019.

"This past quarter, we began an important new period for CRISPR Therapeutics with the treatment of the first patient in our clinical trial for CTX001 in hemoglobinopathies," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "This is a significant landmark for the Company and we continue to enroll patients in our trials for both beta thalassemia and sickle cell disease. With the acceptance of our IND and CTA for CTX110, we look forward to the initiation of our clinical trials for our allogeneic CAR-T programs in the near-term and hope to bring other CAR-T programs to the clinic in the next six to twelve months."

Recent Highlights and Outlook

- **Hemoglobinopathies**
  - **Beta thalassemia**
    In April 2019, CRISPR Therapeutics and its partner, Vertex, announced that the U.S. Food and Drug Administration (FDA) had granted Fast Track Designation for CTX001, an investigational, autologous, gene-edited hematopoietic stem cell therapy, for the treatment of transfusion-dependent beta thalassemia (TDT). In February 2019, CRISPR and Vertex announced that the first patient had been treated with CTX001 in a Phase 1/2 clinical study of patients with TDT, marking the first company-sponsored use of a CRISPR/Cas9 therapy in a clinical trial. Enrollment in the Phase 1/2 study in patients with TDT is ongoing.
  - **Sickle Cell Disease**
    The companies are also evaluating CTX001 for the treatment of sickle cell disease (SCD) and received Fast Track Designation for CTX001 from the FDA in January 2019 for SCD. The companies announced in February 2019 that the first patient had been enrolled in a Phase 1/2 clinical study of CTX001 in severe SCD in the U.S. and is expected to be infused with CTX001 in mid-2019. Enrollment in the Phase 1/2 study in patients with SCD is ongoing.
- **Immuno-Oncology**
  - **CTX110**
    Earlier this year, the FDA approved CRISPR Therapeutics' Investigational New Drug (IND) application for CTX110, its wholly-owned allogeneic CAR-T cell therapy targeting CD19+ malignancies. Additionally, the Company has obtained approval from Health Canada for its Clinical Trial Application (CTA). CRISPR Therapeutics remains on track to initiate a Phase 1/2 trial to assess the safety and efficacy of CTX110 in the first half of 2019 and continues to work closely with various clinical sites to begin the trial. The Company's proprietary CRISPR-based allogeneic CAR-Ts have the potential to create the next-generation of cell therapies that may have a superior product profile compared to current autologous therapies and allow accessibility to broader patient populations.
  - CRISPR Therapeutics continues to advance additional allogeneic CAR-T candidates including: CTX120™, targeting B-cell maturation antigen (BCMA) for the treatment of multiple myeloma; and CTX130™, targeting CD70 for the treatment of solid tumors and hematologic malignancies. Earlier this month, the Company presented preclinical data targeting multiple solid tumor types with anti-CD70 allogeneic CAR-T cells, further demonstrating consistent

expression, durability, selectivity and potent cell killing. The Company also presented data targeting CD33 that showed potent preclinical activity against acute myeloid leukemia (AML) cells. These data were presented during poster sessions at the 2019 American Association for Cancer Research (AACR) Annual Meeting.

- CRISPR Therapeutics will present additional data at the upcoming 2019 American Society of Gene and Cell Therapy (ASGCT) Annual Meeting demonstrating the potential of CRISPR/Cas9 allogeneic CAR-T cell candidates for multiple oncology targets. CRISPR/Cas9 allogeneic CAR-T cells show consistently high percent CAR expression, while maintaining durable potency, low exhaustion, and lack of alloreactivity (#841). The Company will also present data evaluating homology-independent CRISPR/Cas9 off-target assessments methods (#134).

- **Corporate Development**

  - The U.S. Patent Office issued four patents to Dr. Emmanuelle Charpentier, together with The Regents of the University of California and University of Vienna (collectively, CVC). Additionally, CVC was recently granted its third patent in Europe. These patents expand CVC's wide-ranging CRISPR-Cas9 gene-editing patent portfolio.

  - Earlier this year, CRISPR Therapeutics announced strategic collaborations with StrideBio, Inc. and ProBioGen. The Company continues to examine new technologies and capabilities in support of existing programs.

  - In February, CRISPR Therapeutics proposed to elect John T. Greene and Katherine A. High, M.D. to its Board of Directors at the Company's upcoming annual general meeting to be held later this year. Together, they will bring significant strategic and operational experience to CRISPR Therapeutics.

First Quarter 2019 Financial Results

- **Cash Position:** Cash as of March 31, 2019, was $437.5 million, compared to $456.6 million as of December 31, 2018, a decrease of $19.1 million. Cash used in operations drove the decrease, offset by $25.7 million in net cash provided by financing activities during the quarter.

- **Revenues:** Total collaboration revenues were $0.3 million for the first quarter of 2019 compared to $1.4 million for first quarter of 2018. CRISPR's collaboration revenue is attributable to its research partnerships with Casebia and Vertex. Cost sharing on the Vertex co-development and co-promotion agreement related to hemoglobinopathies is not included in revenue, but instead as an offset to expense in R&D.

- **R&D Expenses:** R&D expenses were $33.8 million for the first quarter of 2019 compared to $19.5 million for the first quarter of 2018. The increase was driven by headcount and services expense supporting the advancement of the hemoglobinopathies program, the broadening of the wholly-owned immuno-oncology portfolio, as well as increased investment in the Company's CRISPR/Cas9 platform research.

- **G&A Expenses:** General and administrative expenses were $14.9 million for the first quarter of 2019 compared to $8.8 million for the first quarter of 2018. The increase was driven by headcount-related expense and external professional and consulting service expense.

- **Net Loss:** Net loss was $48.4 million for the first quarter of 2019 compared to a loss of $28.3 million for the first quarter of 2018, driven predominantly by increased R&D expense in the quarter.

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 AG, 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 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 regarding CRISPR Therapeutics' expectations about any or all of the following: (i) clinical trials (including, without limitation, the timing of filing of clinical trial applications and INDs, any approvals thereof and the timing of commencement of clinical trials), development timelines and discussions with regulatory authorities

related to product candidates under development by CRISPR Therapeutics and its collaborators; (ii) 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, and the ability to use that data for the design and initiation of further clinical trials; (iii) the scope and timing of ongoing and potential future clinical trials; (iv) the intellectual property coverage and positions of CRISPR Therapeutics, its licensors and third parties; (v) the sufficiency of CRISPR Therapeutics' cash resources; and (vi) 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 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 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.

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

	Three Months Ended March 31,	
	2019	2018
Collaboration revenue	$ 328	$ 1,358
Operating expenses:		
Research and development	33,822	19,519
General and administrative	14,929	8,836
Total operating expenses	48,751	28,355
Loss from operations	(48,423)	(26,997)
Total other (expense) income, net	100	(1,217)
Net loss before income taxes	(48,323)	(28,214)
Provision for income taxes	(85)	(86)
Net loss	(48,408)	(28,300)
Foreign currency translation adjustment	8	12
Comprehensive Loss	$ (48,400)	$ (28,288)
Reconciliation of net loss to net loss attributable to common shareholders:		
Net loss	$ (48,408)	$ (28,300)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.93)	$ (0.62)
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted	52,093,208	45,877,428

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

	As of

	March 31, 2019	December 31, 2018
Cash	$ 437,549	$ 456,649
Working capital	421,398	438,649
Total assets	497,873	489,016
Total shareholders' equity	379,810	392,195

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