CRISPR Therapeutics Provides Business Update and Reports Third Quarter 2018 Financial Results

November 7, 2018

-Initiated Phase 1/2 Clinical Trial of CTX001 in β-thalassemia-
-Targeting Initiation of Clinical Trial for CTX110, Targeting CD19+ Malignancies, in 1H 2019-
-$487.3 Million in Cash as of September 30, 2018-

ZUG, Switzerland and CAMBRIDGE, Mass., Nov. 07, 2018 (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, 2018 and commented on recent accomplishments and clinical development plans.

"As we enter the fourth quarter, we are actively moving our programs forward and expanding our clinical development activities in sickle cell disease, β-thalassemia and immuno-oncology," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "Importantly, enrollment for a Phase 1/2 trial of CTX001 for β-thalassemia is currently open at multiple clinical trial sites in Europe, and the first patient has been enrolled in the trial. We remain focused on our strategy to deliver on the potential of transformative gene editing therapies to change the lives of patients across a broad array of disease areas."

Recent Highlights and Outlook

- **Continued clinical development of CTX001 for the treatment of β-thalassemia and sickle cell disease (SCD).**

  - **β-thalassemia:** Enrollment for a Phase 1/2 trial in people with β-thalassemia is currently open at multiple clinical trial sites in Europe, and the first patient has now enrolled in this trial. The Phase 1/2 trial is designed to assess safety and efficacy in adult transfusion-dependent non-beta zero/beta zero β-thalassemia patients. The first two patients in the trial will be dosed sequentially and, pending data from these initial two patients, the trial will be open for broader concurrent enrollment. This trial is designed to enroll up to 45 patients.

  - **Sickle Cell Disease:** In the U.S., CRISPR Therapeutics, together with its partner, Vertex, recently announced that the FDA lifted the clinical hold on the CTX001 Investigational New Drug application (IND) for the treatment of SCD that was submitted earlier this year. The companies previously received regulatory approval to conduct a Phase 1/2 SCD in multiple countries in Europe and Canada. Similar to the trial in β-thalassemia, the first two patients in the trial will be dosed sequentially and, pending data from these initial two patients, the trial will be open for broader concurrent enrollment. This trial is designed to enroll up to 45 patients.

- **Advancement of wholly-owned allogeneic CRISPR-based CAR-T cell therapies.** CRISPR Therapeutics believes CRISPR/Cas9 has the potential to create the next-generation of CAR-T cell therapies that may have a superior product profile compared to current autologous therapies and allow broader accessibility for patient populations. The Company is targeting the initiation of a clinical trial for CTX110, its lead allogeneic CAR-T cell therapy targeted toward CD19+ malignancies, in the first half of 2019. CRISPR Therapeutics is also advancing two additional allogeneic CAR-T candidates, targeting BCMA for multiple myeloma and CD70 for both hematologic malignancies and solid tumors. CRISPR Therapeutics will present a poster at the Society for Immunotherapy of Cancer (SITC) Conference, November 7 – 11, related to multiplex editing and production of allogeneic CAR-T therapies (#P279). Additionally, the Company will present a poster at the upcoming American Society of Hematology (ASH) 2018 Annual Meeting on December 1, highlighting further development and preclinical data for CTX120, an allogeneic "off the shelf" CAR-T cell, targeting BCMA in patients with multiple myeloma. The study showed consistent and high percent CAR expression while retaining cytotoxic capacity over multiple *in vitro* re-challenges, demonstrating durable potency and lack of exhaustion (#1921).

- **Entered strategic collaboration with ViaCyte.** CRISPR Therapeutics and ViaCyte, Inc. entered into a collaboration focused on the discovery, development, and commercialization of gene-edited allogeneic stem cell derived islet cell progenitors which may offer curative benefit to patients with insulin-requiring diabetes. CRISPR gene editing offers the potential to protect the transplanted cells from the patient's immune system by *ex vivo* editing immune-modulatory genes within the stem cell line used to produce the pancreatic-lineage cells. The combination of ViaCyte's stem cell capabilities and CRISPR Therapeutics' gene editing capabilities has the potential to enable a beta-cell replacement product that may deliver durable benefit to patients without the need for immune suppression.

- **Completed successful public offering.** In September, CRISPR Therapeutics announced the completion of a public offering of 4,210,526 common shares at a public offering price of $47.50 per share, resulting in net proceeds of approximately $185.7 million after deducting underwriting discounts and commissions, original issuance taxes and offering expenses.

- **Strengthened global patent portfolio with the issuance of a second patent, U.S. Patent No. 10,113,167 (“the ‘167 patent") by the U.S. Patent and Trademark Office (USPTO).** The Regents of the University of California, the University of Vienna and Emmanuelle Charpentier, Ph.D. (collectively, "UC") were granted the ‘167 patent in October 2018. This patent, together with U.S. Patent Number 10,000,772 (“the ‘772 patent”) granted to UC in June 2018, cover optimized dual- and single-guide RNA compositions of CRISPR/Cas9 and uses thereof in various environments, including eukaryotic cells and human therapeutics compositions. Both the ‘167 and the ’772 patents claim priority to a U.S. provisional application, filed by UC on May 25, 2012.

Third Quarter 2018 Financial Results

- **Cash Position:** Cash as of September 30, 2018 was $487.3 million, compared to $319.7 million as of June 30, 2018, an increase of $167.6 million. The increase in cash was primarily driven by the September follow-on offering.

- **Revenues:** Total collaboration revenues were $0.6 million for the third quarter of 2018 compared to $2.4 million for the third quarter of 2017. CRISPR's collaboration revenue is primarily attributable to revenue recognized for work outside the hemoglobinopathies programs. 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 $39.8 million for the third quarter of 2018 compared to $17.8 million for the third quarter of 2017. The increase of $22.0 million in expense was primarily due to CRISPR Therapeutics’ $15 million investment in the ViaCyte collaboration as well as increasing expenditures on hemoglobinopathy and immuno-oncology programs.

- **G&A Expenses:** General and administrative expenses were $10.2 million for the third quarter of 2018 compared to $8.1 million for the third quarter of 2017. The increase of $2.1 million in expense was primarily driven by legal services expense and headcount related expense.

- **Net Loss:** Net loss was $50.7 million for the third quarter of 2018 compared to a loss of $24.7 million for the third quarter of 2017, driven by the increase in R&D and G&A expense described above.

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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Collaboration revenue	$ 563	$ 2,387	$ 3,009	$ 8,672
Operating expenses:				
Research and development	39,820	17,845	84,972	49,770
General and administrative	10,175	8,112	31,752	24,522
Total operating expenses	49,995	25,957	116,724	74,292
Loss from operations	(49,432)	(23,570)	(113,715)	(65,620)
Total other (expense) income, net	(1,142)	(430)	(3,357)	(1,548)
Net loss before income taxes	(50,574)	(24,000)	(117,072)	(67,168)
Provision for income taxes	(137)	(707)	(319)	(1,330)
Net loss	(50,711)	(24,707)	(117,391)	(68,498)
Foreign currency translation adjustment	(6)	8	(15)	38
Comprehensive Loss	$ (50,717)	$ (24,699)	$ (117,406)	$ (68,460)
Reconciliation of net loss to net loss attributable to common shareholders:				
Net loss	$ (50,711)	$ (24,707)	$ (117,391)	$ (68,498)
Net loss per share attributable to common shareholders - basic and diluted	$ (1.07)	$ (0.62)	$ (2.51)	$ (1.72)
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted	47,391,988	40,088,718	46,709,388	39,904,863

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

	As of September 30, 2018	December 31, 2017
Cash	$ 487,295	$ 239,758
Working capital	464,410	233,874
Total assets	518,903	271,346
Total shareholders' equity	417,314	187,832

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