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

August 7, 2018

-Obtained Approvals of CTAs in Multiple Countries for CTX001 in β-thalassemia and SCD-
-Initiation of Clinical Trials for CTX001 in β-thalassemia and SCD on Track for 2018-
-Preclinical Studies for CTX110 Targeting CD19+ Malignancies Have Begun-
-Significantly Expanded Patent Portfolio with New Allowances-
-$319.7 million in cash as of June 30, 2018-

ZUG, Switzerland and CAMBRIDGE, Mass., Aug. 07, 2018 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ: CRSP), a biopharmaceutical company focused on developing transformative gene-based medicines for serious diseases, today reported financial results for the second quarter 2018 and commented on recent accomplishments and clinical development plans.

"In the first half of 2018, we continued to drive rapid and meaningful progress across our hemoglobinopathies program and our immuno-oncology platform," commented Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "We obtained approval of Clinical Trial Applications (CTA) in multiple countries for both β-thalassemia and sickle cell disease (SCD) and we continue to work closely with various clinical sites to initiate these trials. In parallel, we are working diligently with the FDA and have a clear path to resolve the current clinical hold of the Investigational New Drug application (IND) in the U.S. for SCD. In immuno-oncology, a key area of focus for us, preclinical studies have begun, and we look forward to initiating clinical trials early next year."

Dr. Kulkarni continued, "As we move to the second half of the year, our focus remains on the execution of our development programs to bring us closer to our goal of providing transformational medicines to patients with significant unmet medical needs. We look forward to continuing our progress across key areas of our pipeline throughout the remainder of 2018."

Recent Highlights and Outlook

- **Obtained approvals of CTAs in multiple countries for CTX001 in β-thalassemia and SCD.** CTX001 is an investigational autologous gene-edited hematopoietic stem cell therapy for patients suffering from severe hemoglobinopathies. CRISPR, together with its partner, Vertex, has obtained approvals of CTAs in multiple countries for both β-thalassemia and SCD and remain on track to initiate a Phase 1/2 trial to assess the safety and efficacy of CTX001 in patients with transfusion dependent β-thalassemia later this year.

- **Rapidly progressing CRISPR's wholly-owned, allogeneic chimeric antigen receptor T cell (CAR-T) platform.** The Company believes CRISPR/Cas9 has the potential to create the next-generation of CAR-T cell therapies that may benefit broader patient populations. Process development and manufacturing activities have been initiated for CTX110, an allogeneic CAR-T product in development targeted towards CD19+ malignancies, and preclinical studies have begun. The Company remains on track to file the IND for CTX110 by the end of this year and will provide an update upon approval of the IND by the FDA. CRISPR is also advancing two additional allogeneic CAR-T candidates, targeting BCMA for multiple myeloma and CD70 for both hematologic malignancies and solid tumors. In recently presented data the Company observed high editing rates, consistent expression, and selective and potent cell killing. These data confirm and expand upon CRISPR's existing body of data on CTX110.

- **Multiple abstracts highlighting CRISPR/Cas9 preclinical research selected for presentation at the Annual American Society of Gene & Cell Therapy (ASGCT) meeting.** CRISPR announced multiple data presentations at ASGCT highlighting the potential of CRISPR/Cas9 gene-editing in multiple diseases. The data demonstrated several potential advantages of CRISPR's approach to achieve efficient and specific multiplexed editing, as well as robust process development and manufacturing. The Company also presented supportive preclinical data for its hemoglobinopathies program.

- **Recruitment of top talent across all functions.** The new hires expand core capabilities in critical areas such as clinical, regulatory, and manufacturing as the Company advances its multiple scientific programs. In July, the Company appointed Richard Schwartz, Ph.D., to head Technical Operations.

- **Strengthened global patent portfolio with the issuance of U.S. Patent No. 10,000,772 ("the '772 patent") and the allowance of U.S. Patent Application No. 15/138,604 ("the "604 application") 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 '772 patent in June 2018. The patent broadly encompasses widely adopted methods of using optimized dual- and single-guide RNA compositions of CRISPR/Cas9 in various environments, including eukaryotic cells and human therapeutics. Additionally, the USPTO has allowed the '604 application in July 2018. The application broadly encompasses optimized dual- and single-guide RNA compositions that will be particularly useful in developing human therapeutics. The issuance of this patent and allowance of this application further strengthen UC's intellectual property estate as well as CRISPR's discovery and development programs. Both the '772 patent and the '604 application claim priority to a U.S. provisional application, filed by UC on May 25, 2012. The issuance of the '772 patent and allowance of the '604 application fall outside of the ongoing legal proceedings related to the patent interference between UC and the Broad Institute.

Second Quarter 2018 Financial Results

- **Cash Position:** Cash as of June 30, 2018 was $319.7 million, compared to $341.8 million as of March 31, 2018, a decrease of $22.1 million. The decrease in cash was primarily driven by research and development expenditures.

- **Revenues:** Total collaboration revenues were $1.1 million for the second quarter of 2018 compared to $3.6 million for the second 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 $25.6 million for the second quarter of 2018 compared to $17.1 million for the second quarter of 2017. The increase in expense was driven by the advancement of the hemoglobinopathies program, the broadening of the wholly owned immuno-oncology portfolio, as well as increased investment in CRISPR/Cas9 platform research.

- **G&A Expenses:** General and administrative expenses were $12.7 million for the second quarter of 2018 compared to $7.8 million for the second quarter of 2017. The increase in expense was primarily driven by legal services and headcount related expense.

- **Net Loss:** Net loss was $38.4 million for the second quarter of 2018 compared to a loss of $22.3 million for the second quarter of 2017, 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. The Company has established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology and rare diseases. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic collaborations with leading companies including Bayer AG and Vertex Pharmaceuticals. 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

Certain statements set forth in this press release constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: the timing of filing of clinical trial applications and INDs, any approvals thereof and timing of commencement of clinical trials, the intellectual property coverage and positions of the Company, its licensors and third parties, the sufficiency of the Company's cash resources and the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies. You are cautioned that forward-looking statements are inherently uncertain. Although the Company believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, the 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: uncertainties regarding the intellectual property protection for our technology and intellectual property belonging to third parties; uncertainties inherent in the initiation and completion of preclinical studies for the Company's 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; expectations for regulatory approvals to conduct trials or to market products; and those risks and uncertainties described under the heading "Risk Factors" in the Company's most recent annual report on Form 10-K, and in any other subsequent filings made by the Company with the U.S. Securities and Exchange Commission (SEC), 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. The Company 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017

Collaboration revenue	$ 1,088	$ 3,582	$ 2,446	$ 6,285
Operating expenses:				
Research and development	25,633	17,120	45,152	31,925
General and administrative	12,741	7,768	21,577	16,410
Total operating expenses	38,374	24,888	66,729	48,335
Loss from operations	(37,286)	(21,306)	(64,283)	(42,050)
Total other (expense) income, net	(998)	(666)	(2,215)	(1,118)
Net loss before income taxes	(38,284)	(21,972)	(66,498)	(43,168)
Provision for income taxes	(96)	(343)	(182)	(622)
Net loss	(38,380)	(22,315)	(66,680)	(43,790)
Foreign currency translation adjustment	(21)	6	(9)	30
Comprehensive Loss	$ (38,401)	$ (22,309)	$ (66,689)	$ (43,760)
Reconciliation of net loss to net loss attributable to common shareholders:				
Net loss	$ (38,380)	$ (22,315)	$ (66,680)	$ (43,790)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.82)	$ (0.56)	$ (1.44)	$ (1.10)
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted	46,842,316	39,895,938	46,362,538	39,811,412

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

	As of June 30, 2018	December 31, 2017
Cash	$ 319,737	$ 239,758
Working capital	312,068	233,874
Total assets	353,130	271,346
Total shareholders' equity	265,154	187,832

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