CRISPR Therapeutics Provides Business Update and Reports Fourth Quarter and Full Year 2018 Financial Results

-First patient infused with CTX001 in a Phase 1/2 clinical trial for patients with beta thalassemia-

-First patient enrolled in a Phase 1/2 clinical trial of CTX001 for patients with sickle cell disease-

-On track to initiate clinical trial for CTX110, targeting CD19+ malignancies, in 1H 2019-

-$456.6 million in cash as of December 31, 2018-

ZUG, Switzerland and CAMBRIDGE, Mass., Feb. 25, 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 fourth quarter and full year ended December 31, 2018.

"This past year was truly transformational for CRISPR Therapeutics as we achieved milestones across our key programs in β-thalassemia, sickle cell disease and immuno-oncology. We're pleased with the progress we've made in 2018, especially in clinical execution and the expansion of our development pipeline. We also strengthened our team with key new hires, positioning us well as we advance to the next stage of development. This progress brings us closer to realizing our mission of bringing transformative therapies to patients with serious diseases," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics.

Dr. Kulkarni added: "Over the next two years, we expect to generate data from clinical trials across multiple indications as we bring CRISPR technology to patients. In addition, we are making deliberate steps to scale the Company as we advance programs across a number of therapeutic areas while continuing to bolster our proprietary CRISPR platform."

Recent Highlights and Outlook

- **Hemoglobinopathies**

  ° β-thalassemia
  CRISPR Therapeutics, together with its partner, Vertex, announced that the first patient has been treated with CTX001 in a Phase 1/2 clinical study of patients with transfusion-dependent beta thalassemia (TDT), marking the first company-sponsored use of a CRISPR/Cas9 therapy in a clinical trial. The Phase 1/2 open-label trial is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 18 to 35 with TDT, non-beta zero/beta zero subtypes. The first two patients in the trial will be treated sequentially and, pending data from these initial two patients, the trial will open for broader concurrent enrollment. The companies plan to target presentations of data at scientific conferences once there is sufficient data on multiple patients.

  ° Sickle Cell Disease
  CRISPR Therapeutics, together with its partner, Vertex, are also investigating CTX001 for the treatment of severe sickle cell disease (SCD) and announced that the first patient has 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. The Phase 1/2 open-label trial is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 18 to 35 with severe SCD. Similar to the trial in beta thalassemia, the first two patients in the trial will be treated sequentially prior to broader concurrent enrollment. The companies plan to target presentations of data at scientific conferences once there is sufficient data on multiple patients. CTX001 was recently granted Fast Track Designation by the U.S. Food and Drug Administration for the treatment of SCD.

- **Immuno-Oncology**

  ° CRISPR Therapeutics is on track to initiate a clinical trial for CTX110, its wholly-owned allogeneic CAR-T cell therapy targeting CD19+ malignancies, in the first half of 2019. 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 accessibility to broader patient populations. The Company continues to advance two additional allogeneic CAR-T candidates; CTX120, targeting B-cell maturation antigen (BCMA) for the treatment of multiple myeloma; and CTX130,

targeted towards CD70 for the treatment of both solid tumors and hematologic malignancies. In November, the Company presented a poster at the Society for Immunotherapy in Cancer (SITC) 33rd Annual Meeting related to multiplex editing and production of allogeneic CAR-T therapies. Additionally, the Company presented a poster at the American Society of Hematology (ASH) 2018 Annual Meeting in December, highlighting further development and preclinical data for CTX120. The study showed maintained cytotoxic capacity over multiple *in vitro* re-challenges, demonstrating durable potency and reduced susceptibility to exhaustion.

- **Other Programs**

  ° In September, CRISPR Therapeutics and ViaCyte, Inc. announced 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. 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.

  ° Earlier this year, CRISPR Therapeutics announced strategic collaborations with StrideBio, Inc. and ProBioGen. The Company's collaboration with StrideBio expands upon an existing agreement to generate engineered AAV capsids with improved properties for *in vivo* gene editing programs and now includes additional undisclosed applications. CRISPR Therapeutics and ProBioGen announced a collaboration focused on the development of novel *in vivo* delivery modalities for CRISPR/Cas9 leveraging ProBioGen's existing technology and expertise.

  ° In November, CRISPR Therapeutics and MaxCyte announced the expansion of an existing collaboration by entering into a non-exclusive commercial license agreement allowing CRISPR Therapeutics to deploy MaxCyte's Flow Electroporation® Technology to develop CRISPR/Cas9-based therapies in immuno-oncology. The collaboration builds on an existing agreement which allowed for the development of commercial therapeutics for hemoglobin-related diseases.

- **Company Building**

  ° CRISPR Therapeutics continued to expand core capabilities in critical areas with the addition of key new talent across several functions.

  ° 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.

Fourth Quarter and Full Year 2018 Financial Results

- **Cash Position:** Cash and cash equivalents as of December 31, 2018 were $456.6 million, compared to $239.8 million as of December 31, 2017, an increase of $216.8 million, which was primarily driven by the net proceeds of $307.1 million from the sale of shares in follow-on financing rounds executed in January and September of 2018, offset by the Company's use of $96.2 million for operating activities.

- **Revenue:** Total collaboration revenue was $0.1 million for the fourth quarter of 2018 compared to $32.3 million for fourth quarter of 2017, and $3.1 million for the year ended December 31, 2018, compared to $41.0 million for the year ended December 31, 2017. The decrease in annual revenue is primarily attributable to deferred revenue recognized in 2017 in conjunction with the execution of the Company's collaboration agreement with Vertex. During 2018 and going forward, Vertex funding of hemoglobinopathies programs are categorized as a contra-expense as opposed to revenue.

- **R&D Expenses:** R&D expenses were $28.8 million for the fourth quarter of 2018 compared to $20.0 million for the fourth quarter of 2017, and $113.8 million for the year ended December 31, 2018 compared to $69.8 million for the year ended December 31, 2017. The increase in expense for the year was driven by greater investment in CRISPR's lead hemoglobinopathies program partnered with Vertex, one-time expense associated with beginning our ViaCyte collaboration and expenses from the Company's wholly owned immuno-oncology and *in vivo* programs.

- **G&A Expenses:** General and administrative expenses were $16.5 million for the fourth quarter of 2018 compared to $11.3 million for the fourth quarter of 2017, and $48.3 million for the year ended December 31, 2018 compared to $35.8 million for the year ended December 31, 2017. The increase in general and administrative expenses for the year was driven by increased professional services and employee-related costs associated with our growing organization.

- **Net Income/Loss:** Net loss was $47.6 million for the fourth quarter of 2018 compared to income of $0.1 million for the fourth quarter of 2017, and net loss was $165.0 million for the year ended December 31, 2018 compared to a loss of $68.4

million for the year ended December 31, 2017.

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 Therapeutics 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 December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Collaboration revenue	$ 115	$ 32,325	$ 3,124	$ 40,997
Operating expenses:			-	
Research and development	28,801	20,030	113,773	69,800
General and administrative	16,542	11,323	48,294	35,845
Total operating expenses	45,343	31,353	162,067	105,645
(Loss) income from operations	(45,228)	972	(158,943)	(64,648)

Total other (expense) income, net	(2,128)	(413)	(5,485)	(1,960)
Net (loss) income before income taxes	(47,356)	559	(164,428)	(66,608)
Provision for income taxes	(234)	(419)	(553)	(1,749)
Net (loss) income	(47,590)	140	(164,981)	(68,357)
Foreign currency translation adjustment	(7)	-	(22)	40
Comprehensive (loss) income	$ (47,597)	$ 140	$ (165,003)	$ (68,317)
Reconciliation of net loss to net loss attributable to common shareholders:			$ -	
Net (loss) income	$ (47,590)	$ 140	$ (164,981)	$ (68,357)
Net (loss) income per share attributable to common shareholders - basic	$ (0.92)	$ -	$ (3.44)	$ (1.71)
Net (loss) income per share attributable to common shareholders - diluted	$ (0.92)	$ -	$ (3.44)	$ (1.71)
Weighted-average common shares outstanding used in calculating net (loss) income per share attributable to common shareholders - basic	51,688,383	40,509,897	47,964,368	40,057,365
Weighted-average common shares outstanding used in calculating net (loss) income per share attributable to common shareholders - diluted	51,688,383	41,635,843	47,964,368	40,057,365

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

	As of	
	December 31, 2018	December 31, 2017
Cash	$ 456,649	$ 239,758
Working Capital	438,649	233,874
Total assets	489,016	271,346
Total shareholders' equity	392,195	187,832

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

CRISPR Media Contact:
Jennifer Paganelli
WCG on behalf of CRISPR
+1-347-658-8290
jpaganelli@wcgworld.com

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