CRISPR Therapeutics Provides Business Update and Reports Fourth Quarter and Full Year 2017 Financial Results

March 8, 2018

- On track to begin first company-sponsored clinical trials of a CRISPR-based therapy -
- Wholly-owned CRISPR-based allogeneic CAR-T programs advancing rapidly -

ZUG, Switzerland and CAMBRIDGE, Mass., March 08, 2018 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a biopharmaceutical company focused on developing transformative gene-based medicines for serious diseases, today provided a business update and announced financial results for the fourth quarter and full year ended December 31, 2017.

"2017 was a momentous year for CRISPR Therapeutics as we reached our goal of filing the first clinical trial application for a CRISPR-based therapy," said Samarth Kulkarni, PhD, Chief Executive Officer of CRISPR Therapeutics. "In addition to the accomplishments in our hemoglobinopathies program, we made tremendous progress with our versatile immuno-oncology platform and bolstered our team with senior leaders across a variety of key functions."

"In 2018 we expect to build on our success by beginning a Phase 1/2 study of CTX001 in β-thalassemia, the first company-sponsored clinical trial for a CRISPR-based therapeutic. We also plan to begin clinical trials of CTX001 for sickle cell disease in the United States this year. Further, in immuno-oncology, an important area of focus for us, we expect to make substantial progress by filing an IND by the end of the year for CTX101, an allogeneic CRISPR-based CAR-T cell therapy targeting CD19+ malignancies."

Recent Highlights and Outlook:

- **Initiating Phase 1/2 trial of CTX001 in β-thalassemia in 2018.** CRISPR, together with Vertex Pharmaceuticals, has filed clinical trial applications (CTAs) in various European countries to conduct a Phase 1/2 trial of CTX001, an autologous gene-edited hematopoietic stem cell therapy for patients suffering from severe hemoglobinopathies. CRISPR has received approval for the first of these CTAs and expects to initiate clinical trials in Europe in 2018. The Phase 1/2 trial of CTX001 is designed to assess its safety and efficacy in adult transfusion-dependent β-thalassemia patients. Additionally, the Company plans to file an Investigational New Drug (IND) Application for CTX001 in SCD in the US in the first-half of 2018.
- **Updated preclinical data for CTX001.** CRISPR presented new data at the American Society of Hematology (ASH) Annual Meeting in December 2017 demonstrating that its CRISPR gene-editing approach results in high editing efficiency, with >90% of the hematopoietic stem cells edited at the target site. The expression levels of fetal hemoglobin in these cells were above the level believed to be sufficient to ameliorate symptoms in patients with β-thalassemia and sickle cell disease.
- **Initiated manufacturing and other IND-enabling activities for CTX101, an allogeneic CAR-T cell therapy targeting CD19+ malignancies.** The Company presented *in vitro* and *in vivo* data demonstrating potent anti-tumor activity during a poster session at the Society for Immunotherapy in Cancer (SITC) 32nd Annual Meeting. The Company believes its allogeneic CAR-T cell therapies may have distinct advantages over the current generation of autologous cell therapies including greater access due to their "off-the-shelf" nature, and greater efficacy due to the homogeneity and consistency of the product. CRISPR plans to file an IND for CTX101 in the US by year-end 2018.
- **Added two new candidates to the CRISPR-based allogeneic CAR-T portfolio.** During a company-sponsored reception at the SITC 32nd Annual Meeting, CRISPR announced it will be advancing the next two candidates targeting B-cell maturation antigen (BCMA) and CD70. The Company believes it has the necessary capabilities in place to rapidly advance these product candidates to the clinic.
- **Continued advancement of the pipeline through collaborations with CureVac and StrideBio for *in vivo* programs.** In November 2017, CRISPR entered into a collaboration with CureVac to develop novel Cas9 mRNA constructs with improved properties for gene editing applications that could include increased potency, decreased duration of expression and reduced potential for immunogenicity. The Company obtained an exclusive license to the improved constructs for use in three of their *in vivo* gene-editing programs in liver diseases. Additionally, CRISPR Therapeutics entered into a collaboration with StrideBio to develop AAV vectors with greater tissue specificity, and reduced immunogenicity.
- **Continued organizational growth and senior leadership changes.** CRISPR continues to recruit top talent across all functions as it evolves into a clinical-stage company. Notably, in 2017, the Company appointed Dr. Samarth Kulkarni as Chief Executive Officer and added key executives in positions including Head of Research and Development, Chief Financial Officer and General Counsel, each of whom bring an extensive track-record of success and leadership in biopharma.
- **Completed an offering of 5,750,000 common shares**. The offering, including full exercise of the overallotment at $22.75

per share, resulted in net proceeds to the Company of greater than $120 million.

Fourth Quarter and Full Year 2017 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2017 were $239.8 million, compared to $315.5 million as of December 31, 2016, a decrease of $75.7 million, which was primarily driven by expenditures on research and development. Additionally, on January 5, 2018, CRISPR announced a follow-on offering that raised $122.6 million of net proceeds that are not reflected in year-end cash. Combined with the 2017 year-end cash balance, CRISPR Therapeutics enters 2018 with more than $360 million available to advance its portfolio.
- **Revenue:** Total collaboration revenue was $32.3 million for the fourth quarter of 2017 compared to $2.3 million for fourth quarter of 2016, and $41.0 million for the year ended December 31, 2017, compared to $5.2 million for the year ended December 31, 2016. The increase in annual revenue is primarily attributable to deferred revenue recognized in conjunction with the execution of the Company's collaboration agreement with Vertex.
- **R&D Expenses:** R&D expenses were $20.0 million for the fourth quarter of 2017 compared to $15.6 million for the fourth quarter of 2016, and $69.8 million for the year ended December 31, 2017 compared to $42.2 million for the year ended December 31, 2016. The increase in expense was driven by greater investment in CRISPR's lead hemoglobinopathies program partnered with Vertex, in addition to accelerating the Company's wholly owned immuno-oncology and *in vivo* programs.
- **G&A Expenses:** General and administrative expenses were $11.3 million for the fourth quarter of 2017 compared to $12.1 million for the fourth quarter of 2016, and $35.8 million for the year ended December 31, 2017 compared to $31.1 million for the year ended December 31, 2016. The increase in general and administrative expenses for the year was driven by increases in employee-related costs associated with our growing organization.
- **Net Income/Loss:** Net income was $0.1 million for the fourth quarter of 2017 compared to $17.1 million for the fourth quarter of 2016, and net loss was $68.3 million for the year ended December 31, 2017 compared to $23.2 million for the year ended December 31, 2016.

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.

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,	
	2017	2016	2017	2016
Collaboration revenue	$ 32,325	$ 2,344	$ 40,997	$ 5,164
Operating expenses:				
Research and development	20,030	15,572	69,800	42,238
General and administrative	11,323	12,082	35,845	31,056

Total operating expenses	31,353	27,654	105,645	73,294
Income (Loss) from operations	972	(25,310)	(64,648)	(68,130)
Total other (expense) income, net	(413)	42,808	(1,960)	45,412
Net income(loss) before income taxes	559	17,498	(66,608)	(22,718)
Provision for income taxes	(419)	(400)	(1,749)	(484)
Net income (Loss)	140	17,098	(68,357)	(23,202)
Foreign currency translation adjustment			40	(18)
Comprehensive Income (Loss)	$ 140	$ 17,098	$ (68,317)	$ (23,220)
Reconciliation of net loss to net loss attributable to common shareholders:				
Net income (Loss)	$ 140	$ 17,098	$ (68,357)	$ (23,202)
Loss attributable to noncontrolling interest	-	1	-	25
Dividends attributable to Redeemable Convertible Preferred Stock	-	(763)	-	-
Net income allocated to participating securities	-	(2,299)	-	-
Net Income (loss) attributable to common shareholders	$ 140	$ 14,037	$ (68,357)	$ (23,177)
Net income (loss) per share attributable to common shareholders - basic	$ -	$ 0.43	$ (1.71)	$ (1.89)
Net income (loss) loss per share attributable to common shareholders - diluted	$ -	$ 0.40	$ (1.71)	$ (1.89)
Weighted-average common shares outstanding used in calculating net income (loss) per share attributable to common shareholders – basic	40,509,897	32,987,335	40,057,365	12,257,483
Weighted-average common shares outstanding used in calculating net income (loss) per share attributable to common shareholders – diluted	41,635,843	34,989,218	40,057,365	12,257,483

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

	As of December 31, 2017	December 31, 2016
Cash	$ 239,758	$ 315,520
Total assets	271,346	344,962
Total liabilities	83,514	112,116
Total shareholders' equity	187,832	232,846

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