



CRISPR Therapeutics Announces Third Quarter 2017 Financial Results and Provides Business Update

November 8, 2017

- On track to begin first company-sponsored clinical trial of a CRISPR-based therapeutic -
- Wholly-owned CRISPR-based CAR-T programs advancing rapidly -
- New collaborations enhance work in immuno-oncology and in vivo indications -
- Company Presentation and Investor Event at upcoming Society for Immunotherapy in Cancer Annual Meeting -

ZUG, Switzerland and CAMBRIDGE, Mass., Nov. 08, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a genome editing company focused on creating transformative medicines for serious diseases, today announced financial results for the third quarter ended September 30, 2017 and provided a business update.

"CRISPR Therapeutics substantially completed the preclinical activities in the third quarter for CTX001, our lead program targeting two hemoglobinopathies, Sickle Cell Disease and β -Thalassemia. Based on these accomplishments, we are on track to file a Clinical Trial Application in β -Thalassemia by year-end, positioning us to begin the first company-sponsored clinical trial of a CRISPR-based therapeutic in 2018. Further, we are planning to submit an IND application for CTX001 in Sickle Cell Disease in the first half of 2018," commented Samarth Kulkarni, President of CRISPR Therapeutics. "We also continue to make rapid progress in immuno-oncology with the development of our proprietary CRISPR-based allogeneic CAR-T candidates. We look forward to reporting new preclinical data on our lead CAR-T candidate, CTX101 for CD19+ malignancies, at the upcoming Society for Immunotherapy in Cancer Annual Meeting."

"We also continue to strengthen our management team and capabilities as we advance our CRISPR/Cas9 technology into the clinic. A significant highlight of the third quarter was the appointment of Dr. Tony Ho as EVP and Head of R&D. Tony is an accomplished R&D leader, having led several drugs through clinical development, approval and commercialization. Importantly, Tony brings a successful immuno-oncology drug development track record and I believe he joined us at an ideal time as our programs advance toward the clinic and our immuno-oncology efforts accelerate," said Kulkarni.

CRISPR Therapeutics Recent Highlights and Outlook:

On track to file CTA for CTX001 by year-end in β -Thalassemia, with clinical trials to begin in 2018. Substantially all preclinical work, including process development for GMP manufacturing is now complete for CTX001, the lead program targeting Sickle Cell Disease and β -Thalassemia. The CTA submission package in β -Thalassemia remains on track to be filed by year-end 2017. Clinical trials are anticipated to begin in 2018 with the initial study designed to assess safety and efficacy in transfusion-dependent patients with β -Thalassemia. The study is intended to measure fetal hemoglobin (HbF) levels and the change in the number of transfusions required. The Company plans to present data on the efficacy and safety of CTX001 in pre-clinical models for β -Thalassemia and Sickle Cell programs at the American Society for Hematology Annual meeting in December 2017.

Advancing CRISPR-based allogeneic CAR-T programs, with lead program in CD19+ malignancies. CRISPR is continuing to accelerate its wholly-owned immuno-oncology portfolio with development programs in allogeneic CAR-T cell therapy. The lead program in this area, CTX101, is an allogeneic CD19 CAR-T product that has several potential advantages over other approaches in the clinic due to the unique capabilities of the CRISPR/Cas9 system to achieve efficient and specific multiplexed editing. These allogeneic CAR-T products are engineered from healthy donor cells using CRISPR to be "off-the-shelf", durable and available to many different patients from the same batch. The Company has demonstrated high levels of editing, and has shown efficacy of the CAR-T cells in eliminating tumor cells in pre-clinical models. The Company has also optimized and successfully transferred the process to MaSTherCell, a GMP-capable contract manufacturing organization.

To supplement its internal programs, the Company is continuing to establish collaborations in immuno-oncology, and recently announced a two-year research collaboration and license option agreement with Massachusetts General Hospital (MGH) to develop novel T cell therapies for cancer and a partnership with Neon Therapeutics to create neo-antigen based T-cell constructs. Marcela V. Maus, M.D., Ph.D., Director of the Cellular Immunotherapy Program at MGHCC and Assistant Professor of Medicine at Harvard Medical, will lead the scientific work at MGH.

Continuing to advance *in vivo* applications through external collaborations. CRISPR is making investments in both lipid nanoparticle (LNP) and viral delivery vehicles to address diseases with *in vivo* approaches. Previously, CRISPR disclosed an exclusive license from MIT for LNPs and has demonstrated *in vivo* mouse data demonstrating ~90% knock-down of proteins in the liver at therapeutically relevant doses.

In October, CRISPR Therapeutics announced a grant from Friedrich's Ataxia Research Alliance to collaborate with University of Alabama at Birmingham on gene-editing treatments for Friedrich's Ataxia (FA) which will utilize viral delivery approaches. CRISPR will research *in vivo* CRISPR/Cas9-based gene-editing approaches to treat FA, which will be performed in collaboration with Dr. Marek Napierala at the University of Alabama at Birmingham. Studies will include humanized animal models of FA to demonstrate preclinical proof-of-concept. Upon conclusion of these studies, CRISPR Therapeutics will have the option to advance the programs into clinical trials.

Organizational growth and senior leadership promotions. CRISPR recently announced that Samarth Kulkarni, Ph.D. will be assuming the role of Chief Executive Officer on December 1st, with Rodger Novak, M.D., co-founder and current Chief Executive Officer continuing to serve as a member of the Company's board of directors. In August, CRISPR appointed Tony Ho, M.D., as Executive Vice President and Head of Research & Development. Dr. Ho is a highly accomplished R&D leader with substantial experience across all phases of R&D, from discovery through

commercialization including direct oversight of four successful drug approvals. The Company continues to recruit top talent across all functions, including clinical operations, manufacturing and development, as it transitions to a clinical stage organization.

Financial Results for Three and Nine Months Ended September 30, 2017 (U.S. GAAP).

As of September 30, 2017, CRISPR Therapeutics had \$253.5 million in cash as compared to \$315.5 million in cash as of December 31, 2016. Based on its current operating plan, CRISPR expects its existing cash resources will be sufficient to fund operating expenses and capital expenditure requirements for at least the next two years.

Three Months Ended September 30, 2017

CRISPR Therapeutics reported a net loss of \$24.7 million for the three months ended September 30, 2017 as compared to a net loss of \$14.7 million for the three months ended September 30, 2016. The increase in net loss of \$10.0 million resulted primarily from an increase in loss from operations of \$8.9 million, an increase in the provision for income taxes of \$0.7 million and an increase in other expense of \$0.4 million.

Collaboration revenue for the three months ended September 30, 2017 was \$2.4 million, compared to \$1.5 million for the three months ended September 30, 2016. The increase of \$0.9 million was primarily due to an increase in research and development service revenue under our collaboration agreements with Vertex and Casebia.

Research and development expenses were \$17.8 million for the three months ended September 30, 2017, compared to \$12.1 million for the three months ended September 30, 2016. The increase of \$5.7 million was primarily attributable to the following increases: \$2.3 million of variable research and development costs including manufacturing, \$1.7 million of employee-related costs, \$1.1 million of employee stock based compensation costs and the remainder consisting primarily of rent, utilities and research supplies.

General and administrative expenses were \$8.1 million for the three months ended September 30, 2017, compared to \$4.1 million for the three months ended September 30, 2016. The increase of \$4.0 million was primarily attributable to the following increases: \$1.5 million of employee stock based compensation costs, \$1.3 million of employee-related costs to support our overall growth, \$1.0 million of professional and consulting expenses and \$1.0 million in facilities costs including rent and utilities at our new facility. These increases were partially offset by a decrease of \$0.8 million in legal and intellectual property fees.

Nine Months Ended September 30, 2017

CRISPR Therapeutics reported a net loss of \$68.5 million for the nine months ended September 30, 2017, compared to a net loss of \$40.3 million for the nine months ended September 30, 2016. The increase in net loss of \$28.2 million resulted primarily from an increase of \$22.8 million in loss from operations, an increase of \$1.2 million in the provision for income taxes, a decrease of \$11.5 million on the gain on extinguishment of the convertible loan with Vertex, an increase of \$0.6 million in the loss from equity method investment, an increase of \$0.1 million in other expense, offset by a decrease in interest expense of \$8.0 million from the convertible loan with Bayer.

Collaboration revenue for the nine months ended September 30, 2017 was \$8.7 million, compared to \$2.8 million for the nine months ended September 30, 2016. The increase of \$5.9 million was due to an increase in research and development service revenue from our collaboration agreements with Vertex and Casebia.

Research and development expenses were \$49.8 million for the nine months ended September 30, 2017, compared to \$26.7 million for the nine months ended September 30, 2016. The increase of \$23.1 million was primarily attributable to the following increases: \$5.5 million consisting primarily of rent, utilities and research supplies, \$8.3 million of variable research and development program costs, \$6.4 million of employee-related costs and \$3.3 million of employee stock based compensation costs. These increases were primarily offset by a decrease in professional services costs.

General and administrative expenses were \$24.5 million for the nine months ended September 30, 2017, compared to \$19.0 million for the nine months ended September 30, 2016. The increase of \$5.5 million was primarily due to the following increases: \$3.1 million of employee-related costs to support our overall growth, \$2.9 million in facilities costs including rent and utilities at our new research facility, \$1.8 million of employee stock based compensation costs and \$0.5 million in professional services costs. The increases were offset by a reduction of our 2016 Passive Foreign Investment Company tax obligation and franchise taxes on the convertible preferred stock financings.

CRISPR Therapeutics Investor Reception

CRISPR will hold an Investor Reception during the upcoming Society for Immunotherapy in Cancer Annual Meeting

Location: Gaylord Hotel at the Gaylord National Resort & Convention Center

Date: Friday November 10, 2017

Time: 6:00 PM – 8:00 PM ET, Presentation to begin at 6:30 PM ET

Guest Speaker: Stephan A. Grupp, MD, PhD, Director of the Cancer Immunotherapy Program, director of Translational Research for the Center for Childhood Cancer Research at Children's Hospital of Philadelphia and medical director of the Stem Cell Laboratory, Chief of the Section of Cellular Therapy and Transplant in the Hospital's Division of Oncology

A live webcast of the presentation can be accessed beginning at 6:30pm ET under "Events & Presentations" in the Investors & Media section of the Company's website at www.crisprtx.com.

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 gene editing platform. CRISPR/Cas9 is a revolutionary technology that allows for precise, directed changes to genomic DNA. The Company's multi-disciplinary team of world-class researchers and drug developers is working to translate this technology into breakthrough human therapeutics in a number of serious diseases. Additionally, CRISPR Therapeutics has established strategic collaborations with Bayer AG and Vertex Pharmaceuticals to develop CRISPR-based therapeutics in diseases with high unmet need. The foundational CRISPR/Cas9 patent estate for human therapeutic use was licensed from the Company's scientific founder Emmanuelle Charpentier, Ph.D. 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 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/Cas-9 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 September 30, | | Nine Months Ended September 30, | |
|---|----------------------------------|--------------|---------------------------------|--------------|
| | 2017 | 2016 | 2017 | 2016 |
| Collaboration revenue | \$ 2,387 | \$ 1,549 | \$ 8,672 | \$ 2,820 |
| Operating expenses: | | | | |
| Research and development | 17,845 | 12,052 | 49,770 | 26,666 |
| General and administrative | 8,112 | 4,107 | 24,522 | 18,974 |
| Total operating expenses | 25,957 | 16,159 | 74,292 | 45,640 |
| Loss from operations | (23,570) | (14,610) | (65,620) | (42,820) |
| Total other (expense) income, net | (430) | (76) | (1,548) | 2,604 |
| Net loss before income taxes | (24,000) | (14,686) | (67,168) | (40,216) |
| Provision for income taxes | (707) | (8) | (1,330) | (84) |
| Net loss | (24,707) | (14,694) | (68,498) | (40,300) |
| Foreign currency translation adjustment | 8 | (1) | 38 | (18) |
| Comprehensive Loss | \$ (24,699) | \$ (14,695) | \$ (68,460) | \$ (40,318) |
| Reconciliation of net loss to net loss attributable to common shareholders: | | | | |
| Net loss | \$ (24,707) | \$ (14,694) | \$ (68,498) | \$ (40,300) |
| Loss attributable to noncontrolling interest | - | 14 | - | 24 |
| Net loss attributable to common shareholders | \$ (24,707) | \$ (14,680) | \$ (68,498) | \$ (40,276) |
| Net loss per share attributable to common shareholders - basic and diluted | \$ (0.62) | \$ (2.77) | \$ (1.72) | \$ (7.43) |
| Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted | 40,088,718 | 5,292,348 | 39,904,863 | 5,422,617 |

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Condensed Consolidated Balance Sheets Data

(Unaudited, in thousands)

| | As of | |
|------|--------------------|-------------------|
| | September 30, 2017 | December 31, 2016 |
| Cash | \$ 253,519 | \$ 315,520 |

| | | |
|----------------------------|---------|---------|
| Working capital | 246,469 | 298,190 |
| Total assets | 282,319 | 344,962 |
| Total shareholders' equity | 178,958 | 232,846 |

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