



## CRISPR Therapeutics Announces Second Quarter 2017 Financial Results and Provides Business Update

August 10, 2017

*On track to file for clinical trial application (CTA) for lead program in beta-thalassemia in 2017  
Rapid progress in immuno-oncology including a lead program in allogeneic CAR-T cell therapy  
Expanded foundational and therapeutic intellectual property position  
Strong financial position to support development of pipeline and fund operations*

ZUG, Switzerland and CAMBRIDGE, Mass., Aug. 10, 2017 (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 second quarter ended June 30, 2017 and provided a business update.

"CRISPR Therapeutics has had a very productive first half of the year across all aspects of our business and we remain on track to achieve the goals we set at the beginning of the year," said Dr. Rodger Novak, CEO of CRISPR Therapeutics. "Our lead program in hemoglobinopathies remains on track for a CTA submission in late 2017, with clinical trials beginning in 2018. We are advancing our immuno-oncology portfolio and other *in vivo* applications supported by the signing of key collaborations, and we remain focused on building an organization with top notch talent."

### Recent Highlights and Outlook:

**Lead hemoglobinopathies program remains on track to file CTA by year-end 2017, and begin clinical trials in 2018.** CRISPR Therapeutics highlighted the most recent progress of its hemoglobinopathies program during the Presidential Symposium at the 22<sup>nd</sup> European Hematology Association Annual Congress in June. The data presented provided further support for CRISPR's approach of re-creating the natural condition of hereditary persistence of fetal hemoglobin (HPFH) that is protective in sickle cell disease and in beta-thalassemia. The presentation described the ability to re-create specific HPFH gene variants in the intended target tissue, human CD34+ stem cells, and demonstrated that these gene variants increase the expression of protective fetal hemoglobin. The data presented continues to support the development of the lead product candidate for beta-thalassemia and sickle cell disease and the Company remains on track to file a clinical trial application (CTA) in Europe by year-end 2017 for beta-thalassemia.

**Advancing immuno-oncology program through lead allogeneic CAR-T program and collaborations.** CRISPR Therapeutics is rapidly advancing its lead immuno-oncology program through pre-IND studies and process development for manufacturing. The lead program, CTX101, is an allogeneic CAR-T cell therapy being developed for the treatment of CD19-positive malignancies. CRISPR has demonstrated the use of its proprietary gene editing technology to make targeted modifications in T cells, thereby creating an allogeneic or "off-the-shelf" product that is designed for a broader patient population and addresses several challenges of the current generation of autologous therapies. In June, the Company announced an agreement with MaSTherCell SA, a full-service contract development and manufacturing organization, to develop and manufacture CTX101 under cGMP conditions for use in future clinical trials. Beyond its lead program, CRISPR announced a research collaboration with Neon Therapeutics (Neon) to explore the combination of its CRISPR gene editing platform with Neon's neo-antigen platform to develop novel T cell therapies.

**Advancing *in vivo* applications through in-licensing and collaborations.** In May, CRISPR Therapeutics announced the signing of an exclusive license to a family of proprietary lipid nanoparticle (LNP) technologies from the Massachusetts Institute of Technology. Utilizing this technology, the Company demonstrated high-efficiency elimination of a target protein produced in the liver. These data were presented at the Cold Spring Harbor Laboratory Genome Engineering conference in July. Additionally, CRISPR and its collaborators at the University of Florida were awarded a two-year grant from Target ALS Foundation, a non-profit organization dedicated to accelerating new treatments for ALS, to support discovery and validation of CRISPR/Cas9-based therapeutic approaches for ALS and frontotemporal dementia (FTD). In April, CRISPR announced a collaboration with StrideBio, a leading developer of adeno-associated virus (AAV) based technologies, to co-develop new vectors for the *in vivo* delivery of CRISPR/Cas9-based therapeutics to various organ systems.

**Strengthened international intellectual property around the foundational and therapeutic CRISPR/Cas9 gene editing technology.** Following recent patent grants in the United Kingdom and Europe that broadly cover its in-licensed gene editing technology, CRISPR Therapeutics announced it has received a similarly broad patent from China's State Intellectual Property Office (SIPO), and it has recently received additional grants or notices of allowance from Australia, New Zealand and Singapore. The claims being granted in these jurisdictions are directed to CRISPR/Cas9 single-guide gene editing methods for modifying target DNA in both non-cellular and cellular settings, including cells from vertebrate animals such as human or mammalian cells – as well as composition of matter and system claims for use in any setting, including claims for the use in producing medicine for treating disease. The growing international recognition of the broad applicability of CRISPR's patent applications for use in all settings, including in human and other eukaryotic cells, continues to reinforce the Company's position as a leader in the rapidly evolving gene editing industry.

In the U.S., CRISPR announced, jointly with its licensors and other sub-licensees, that it had filed the opening brief to the U.S. Court of Appeals for the Federal Circuit (the Federal Circuit) seeking reversal of a decision by the U.S. Patent and Trademark Office's Patent Trial and Appeal Board (PTAB) in an interference proceeding relating to CRISPR/Cas9 gene editing technology. In the appeal, University of California (UC) requests reversal of the PTAB's decision terminating the interference between certain CRISPR/Cas9 patent claims owned by UC and claims of the Broad Institute, Harvard University and the Massachusetts Institute of Technology (collectively, "Broad"). In parallel with the appeal, CRISPR is pursuing other patent applications in the U.S. to pursue patents claiming the CRISPR/Cas9 technology and its use in non-cellular and cellular settings, including eukaryotic cells.

**Organizational growth and senior leadership additions.** CRISPR Therapeutics continues to enhance its team by attracting and hiring experts across all critical functions including research and development, manufacturing, clinical operations and other areas. In June, CRISPR appointed James R. Kasinger as General Counsel. Mr. Kasinger will oversee the company's corporate legal and governance matters. Prior to joining CRISPR, Mr. Kasinger was General Counsel and Secretary at Moderna Therapeutics. Recently, in August, CRISPR announced the appointment of Dr. Tony Ho as the new Head of Research and Development for the company. Tony brings over 20 years of experience in the industry across both research and development in multiple therapeutic areas. Most recently, Tony was SVP and Head of Oncology Integration and Innovation at AstraZeneca. Currently CRISPR Therapeutics employs 114 people across its three locations. In July, the Company's global headquarters was moved from Basel to Zug, Switzerland, as approved by shareholders at the Company's recent annual meeting.

#### **Financial Results for Three and Six Months Ended June 30, 2017 (U.S. GAAP):**

As of June 30, 2017, CRISPR Therapeutics had \$272.3 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 June 30, 2017*

CRISPR Therapeutics reported a net loss of \$22.3 million for the three months ended June 30, 2017 as compared to a net loss of \$17.2 million for the three months ended June 30, 2016. The increase in net loss of \$5.1 million resulted primarily from an increase in loss from operations of \$4.7 million, an increase in the provision for income taxes of \$0.3 million and an increase in other expense of \$0.1 million.

Collaboration revenue for the three months ended June 30, 2017 was \$3.6 million, compared to \$0.8 million for the three months ended June 30, 2016. The increase of \$2.8 million was primarily due to an increase in research and development service revenue under our collaboration agreements with Casebia and Vertex of \$1.5 million and \$1.3 million, respectively.

Research and development expenses for the three months ended June 30, 2017 were \$17.1 million, compared to \$8.6 million for the three months ended June 30, 2016. The increase of \$8.5 million was primarily attributable to increases of \$3.2 million of variable process and platform development costs, \$2.1 million of facilities costs including rent and utilities at our new research facility, \$2.1 million of employee-related costs and \$1.1 million of employee stock based compensation costs.

General and administrative expenses were \$7.8 million for the three months ended June 30, 2017, compared to \$8.8 million for the three months ended June 30, 2016. The decrease of \$1.0 million was primarily due to decreases of \$2.0 million in costs associated with a 2016 passive foreign investment company (PFIC) tax liability and \$0.9 million in employee stock based compensation costs. The decreases were offset by increases of \$1.0 million in employee-related costs to support our overall growth, \$0.6 million in professional and consulting expenses, and \$0.3 million in facilities costs including rent and utilities at our new facility.

##### *Six Months Ended June 30, 2017*

CRISPR Therapeutics reported a net loss of \$43.8 million for the six months ended June 30, 2017, compared to a net loss of \$25.6 million for the six months ended June 30, 2016. The increase in net loss of \$18.2 million resulted primarily from an increase of \$13.8 million in loss from operations, an increase of \$0.5 million in the provision for income taxes, an increase of \$0.3 million in the loss from equity method investment, an increase of \$0.1 million in other expense and a decrease of \$11.5 million on the gain on extinguishment of the convertible loan with Vertex offset by a decrease in interest expense of \$8.0 million from the convertible loan with Bayer.

Collaboration revenue for the six months ended June 30, 2017 was \$6.3 million, compared to \$1.3 million for the six months ended June 30, 2016. The increase of \$5.0 million was primarily due to an increase in research and development service revenue under our collaboration agreements with Casebia and Vertex of \$2.6 million and \$2.4 million, respectively.

Research and development expenses for the six months ended June 30, 2017 were \$31.9 million, compared to \$14.6 million for the six months ended June 30, 2016. The increase of \$17.3 million was primarily attributable to increases of \$5.9 million in variable process and platform development costs, \$4.7 million in facilities costs including rent and utilities at our new research facility, \$4.5 million in employee-related costs, and \$2.2 million in employee stock based compensation costs.

General and administrative expenses were \$16.4 million for the six months ended June 30, 2017, compared to \$14.9 million for the six months ended June 30, 2016. The increase of \$1.5 million was primarily due to the increases of \$2.1 million in employee-related costs to support our overall growth, \$1.5 million in facilities costs including rent and utilities at our new research facility, and \$0.3 million in employee stock based compensation costs. The increases were offset by a reduction of \$2.4 million in our 2016 PFIC tax obligation and franchise taxes on the convertible preferred stock financings.

#### **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](http://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 intellectual property coverage and positions of the company, its licensors and third parties, 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](http://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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue	\$ 3,582	\$ 795	\$ 6,285	\$ 1,271
Operating expenses:				
Research and development	17,120	8,602	31,925	14,614
General and administrative	7,768	8,751	16,410	14,867
Total operating expenses	24,888	17,353	48,335	29,481
Loss from operations	(21,306 )	(16,558 )	(42,050 )	(28,210 )
Total other (expense) income, net	(666 )	(606 )	(1,118 )	2,680
Net loss before income taxes	(21,972 )	(17,164 )	(43,168 )	(25,530 )
Provision for income taxes	(343 )	-	(622 )	(76 )
Net loss	(22,315 )	(17,164 )	(43,790 )	(25,606 )
Foreign currency translation adjustment	6	(13 )	30	(17 )
Comprehensive Loss	\$ (22,309 )	\$ (17,177 )	\$ (43,760 )	\$ (25,623 )
Reconciliation of net loss to net loss attributable to common shareholders:				
Net loss	\$ (22,315 )	\$ (17,164 )	\$ (43,790 )	\$ (25,606 )
Loss attributable to noncontrolling interest	-	7	-	10
Net loss attributable to common shareholders	\$ (22,315 )	\$ (17,157 )	\$ (43,790 )	\$ (25,596 )
Net loss per share attributable to common shareholders - basic and diluted	\$ (0.56 )	\$ (3.15 )	\$ (1.10 )	\$ (4.66 )
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted	39,895,938	5,448,855	39,811,412	5,488,467

## CRISPR Therapeutics AG

### Condensed Consolidated Balance Sheets Data

(Unaudited, in thousands)

	As of	
	June 30, 2017	December 31, 2016
Cash	\$ 272,264	\$ 315,520
Working capital	265,471	298,190
Total assets	304,975	344,962
Total shareholders' equity	197,803	232,846

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