



## CRISPR Therapeutics Reports Financial Results for the Three Months Ended March 31, 2017 and Provides Business Update

May 11, 2017

*On track to file for clinical trial authorization (CTA) for lead program in beta-thalassemia in 2017  
Expanded partnerships enhancing both ex vivo and in vivo delivery of CRISPR/Cas9 technology  
Expanded foundational and therapeutic intellectual property position  
Strong financial position to support development of pipeline and fund operations*

BASEL, Switzerland and CAMBRIDGE, Mass., May 11, 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 three months ended March 31, 2017, and provided a business update.

"CRISPR Therapeutics continued its strong progress and we remain on track to achieve the goals that we first discussed when we completed our initial public offering in October 2016," said Dr. Rodger Novak, CEO of CRISPR Therapeutics. "Our lead program in hemoglobinopathies remains on track with our CTA submission expected late in 2017. We have also advanced our efforts in other disease areas such as immuno-oncology and *in vivo* liver applications, successfully broadened our intellectual property position, and entered into new partnerships to access technologies."

### Recent Highlights and Outlook:

**Advanced the lead hemoglobinopathies program toward the clinic, and progressed efforts in immuno-oncology and *in vivo* gene editing.** CRISPR Therapeutics remains on track to file for its clinical trial authorization (CTA) in Europe by year-end 2017 for its lead therapeutic product to treat beta-thalassemia. The preclinical program, including the manufacturing process, has been vetted and agreed by the Paul-Ehrlich Institute in Germany and by the United Kingdom's health regulatory authority (MHRA). CRISPR's gene editing approach is designed to re-create genetic variants that are associated with hereditary persistence of fetal hemoglobin (HPFH), which has been shown to significantly reduce morbidity in patients with beta-thalassemia or sickle cell disease. In two presentations at the 58th American Society of Hematology (ASH) Annual Meeting in December 2016, CRISPR Therapeutics demonstrated that CRISPR/Cas9 gene editing can re-create the genetics of naturally occurring HPFH with high efficiency and no detectable off-target editing in human hematopoietic stem cells, leading to high expression levels of protective fetal hemoglobin. In addition to progress with the lead program, CRISPR advanced its pre-clinical efforts in immuno-oncology, showing the ability to both insert and delete multiple genes in human T-cells, and with *in vivo* editing, demonstrating high efficiency gene disruption in mouse liver using a single dose of CRISPR/Cas9 delivered via lipid nanoparticle (LNP).

**Partnered with MaxCyte and StrideBio to access delivery technologies.** In March, CRISPR Therapeutics obtained commercial rights to MaxCyte's cell engineering platform to develop *ex vivo* CRISPR/Cas9-based therapies. CRISPR and Casebia's lead programs, hemoglobinopathies and severe combined immunodeficiency (SCID) respectively, use *ex vivo* gene editing where the CRISPR/Cas9 components are delivered to hematopoietic stem cells using MaxCyte technology. The agreement with MaxCyte is intended to position both CRISPR and Casebia for future developments by securing access to leading preferred *ex vivo* delivery solution for both clinical and commercial use. In April, CRISPR Therapeutics and Casebia also signed an exclusive agreement to use StrideBio's proprietary platform to develop novel AAV vectors. StrideBio's platform is directed to the engineering of next-generation AAV vectors with improved properties such as increased tissue specificity and reduced susceptibility to host immune responses. The agreement with StrideBio is designed to position CRISPR and Casebia at the forefront of viral delivery technology for their *in vivo* programs, and further demonstrates the potential of CRISPR and Casebia to partner in accessing technologies that can strengthen their shared gene editing platform.

**Expanded the foundational and therapeutic CRISPR/Cas9 intellectual property position.** A number of key developments in the patent landscape surrounding the CRISPR/Cas9 gene-editing technology emerged in the first quarter, which serve to expand our global intellectual property position:

- In March, CRISPR Therapeutics announced that the European Patent Office (EPO) indicated its intention to grant a broad patent covering CRISPR's in-licensed gene editing technology. The patent was the subject of numerous challenges that the EPO ultimately determined did not impact the patentability of the inventions disclosed therein. The claims being granted include the use of CRISPR/Cas9 in cellular and non-cellular settings, including in human cells.
- In early April, CRISPR Therapeutics, along with the co-owners of the foundational intellectual property relating to the CRISPR/Cas9 technology, announced they will appeal to the U.S. Court of Appeals for the Federal Circuit the Patent Trial and Appeal Board's (PTAB) February decision terminating the interference between certain CRISPR/Cas9 patent claims licensed by CRISPR and others owned by the Broad Institute, Harvard University, and the Massachusetts Institute of Technology. The PTAB's February decision terminated the interference without determining which inventors actually invented the use of CRISPR/Cas9 in eukaryotic cells.
- In late April, CRISPR Therapeutics announced that the United States Patent and Trademark Office (USPTO) is expected to issue a CRISPR/Cas9 genome editing patent to Vilnius University. The patent, to which CRISPR holds a sublicense for most human therapeutic uses, is directed to the use of CRISPR/Cas9 in ribonucleoprotein (RNP) format, which is complementary to our foundational IP estate.

**Continued organization build.** 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 May, CRISPR appointed Dr. Samarth Kulkarni to the position of President of CRISPR Therapeutics Inc., expanding his role beyond Chief Business Officer to oversee U.S. operations. In immuno-oncology, CRISPR has established a separate business unit with its own dedicated scientific leadership. To head this group, CRISPR in February appointed Dr. Jon Terrett as Head of Immuno-Oncology Research and Translation. As CRISPR moves its lead programs into the clinic, the company will continue to expand its senior management team with leaders having deep expertise in later-stage clinical development and registration of breakthrough therapies.

#### **Financial Results for Three Months Ended March 31, 2017 (U.S. GAAP)**

As of March 31, 2017, CRISPR Therapeutics had \$288.9 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.

CRISPR Therapeutics reported a net loss of \$21.5 million for the three months ended March 31, 2017 as compared to a net loss of \$8.4 million for the three months ended March 31, 2016. The increase in net loss of \$13.0 million resulted primarily from an increase in loss from operations of \$9.1 million and a decrease in other income, net of \$3.7 million.

Collaboration revenue for the three months ended March 31, 2017 was \$2.7 million, compared to \$0.5 million for the three months ended March 31, 2016. The increase of \$2.2 million was primarily due to an increase of research and development service revenue under our collaboration agreement with Vertex of \$1.0 million and research and development service revenue of \$1.2 million under our collaboration agreement with Casebia.

Research and development expenses for the three months ended March 31, 2017 was \$14.8 million, compared to \$6.0 million for the three months ended March 31, 2016. The increase of \$8.8 million was primarily attributable to increases of \$1.6 million of facilities costs including rent and utilities at our enlarged research facility, \$3.3 million of variable process and platform development costs, \$2.6 million of employee-related costs, \$1.1 million of employee stock based compensation costs and \$0.2 million in license fees.

General and administrative expenses were \$8.6 million for the three months ended March 31, 2017, compared to \$6.1 million for the three months ended March 31, 2016. The increase of \$2.5 million was primarily attributable to increases of \$1.2 million for employee-related costs to support our overall growth, \$1.2 million for employee stock based compensation, \$0.7 million of facilities costs including rent and utilities at our enlarged research facility, and \$0.4 million of professional and consulting expenses. These increases were partially offset by a \$1.0 million decrease in legal and intellectual property costs.

#### **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 Basel, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge, Massachusetts. 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 March 31,	
	2017	2016
Collaboration revenue	\$ 2,703	\$ 476
Operating expenses:		
Research and development	14,805	6,012

General and administrative	8,642	6,116
Total operating expenses	23,447	12,128
Loss from operations	(20,744 )	(11,652 )
Other (expense) income, net	(452 )	3,286
Net loss before income taxes	(21,196 )	(8,366 )
Provision for income taxes	(279 )	(76 )
Net loss	(21,475 )	(8,442 )
Foreign currency translation adjustment	24	(4 )
Comprehensive Loss	\$ (21,451 )	\$ (8,446 )
Loss attributable to noncontrolling interest	-	3
Net loss attributable to common shareholders	\$ (21,475 )	\$ (8,439 )
Net loss per share attributable to common shareholders - basic and diluted:	\$ (0.54 )	\$ (1.53 )
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic and diluted:	39,725,947	5,528,079

## CRISPR Therapeutics AG

### Condensed Consolidated Balance Sheets Data

(Unaudited, in thousands)

	As of	
	March 31, 2017	December 31, 2016
Cash	\$ 288,872	\$ 315,520
Working capital	279,547	298,190
Total assets	322,346	344,962
Total shareholders' equity	215,175	232,846

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