



## **CRISPR Therapeutics Provides Business Update and Reports Fourth Quarter and Full Year 2019 Financial Results**

*-Enrollment ongoing in clinical trials of CTX001™ for patients with severe hemoglobinopathies-*

*-Enrollment ongoing in clinical trial of CTX110™, targeting CD19+ malignancies-*

*-Enrollment has begun in clinical trial of CTX120™, targeting B-cell maturation antigen (BCMA)-*

ZUG, Switzerland and CAMBRIDGE, Mass., Feb. 12, 2020 (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, 2019.

"In 2019, CRISPR Therapeutics achieved important milestones and momentum across key programs. We announced positive interim safety and efficacy data from the first two patients in our ongoing CTX001 clinical trials, one patient with beta thalassemia and one patient with sickle cell disease. These preliminary data support our belief in the potential of CTX001 to have meaningful benefit for patients following a one-time intervention," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "In addition, we advanced our first allogeneic CAR-T cell therapy, CTX110, targeting CD19+ malignancies and, building on this progress, today announced that we have begun enrolling patients in a clinical trial for our second allogeneic CAR-T therapy, CTX120, targeting BCMA for the treatment of relapsed or refractory multiple myeloma."

Dr. Kulkarni added: "2020 has the potential to be a pivotal year in our company's growth. We expect to conduct clinical trials in five indications, and we anticipate new data from our immuno-oncology and hemoglobinopathies programs. Our continued progress brings us closer to potentially providing transformative therapies to patients with serious diseases."

### **2019 Highlights and Outlook**

#### **• Beta Thalassemia and Sickle Cell Disease**

- In November 2019, CRISPR Therapeutics and its partner Vertex announced positive, interim data from the first two patients with severe hemoglobinopathies - one patient with transfusion-dependent beta thalassemia (TDT) and one patient with severe sickle cell disease (SCD) - treated with the investigational CRISPR/Cas9 gene-editing therapy CTX001 in the ongoing Phase 1/2 CLIMB clinical trials. Enrollment is ongoing in both trials and the companies expect to provide additional data for these programs in 2020.
- CTX001 has been granted orphan drug designation (ODD) by the European Commission for the treatment of SCD. The European Commission previously granted CTX001 ODD for the treatment of TDT.

#### **• Immuno-Oncology**

- Patient enrollment continues in a clinical trial to assess the safety and efficacy of CTX110, its wholly-owned allogeneic CAR-T cell therapy targeting refractory CD19+ B-cell malignancies. The multi-center, open label clinical trial is designed to enroll up to 95 patients and investigate several dose levels of CTX110. If successful, CTX110 could enable off-the-shelf use of cell therapies and greatly expand their applicability and accessibility in immuno-oncology.
- CRISPR Therapeutics has begun enrolling patients in a clinical trial to assess the safety and efficacy of CTX120, its wholly-owned allogeneic CAR-T cell therapy targeting BCMA for the treatment of relapsed or refractory multiple myeloma. The multi-center, open label trial is designed to enroll up to 80 patients and investigate several dose levels of CTX120.
- CRISPR Therapeutics continues to advance additional allogeneic CAR-T candidates toward clinical development, including CTX130™, its wholly-owned allogeneic CAR-T cell therapy targeting CD70 for the treatment of both solid tumors, such as renal cell carcinoma, and T-cell and B-cell hematologic malignancies. The Company continues to scale its capabilities to enable rapid advancement of these programs into and through the clinic.

- **Other Corporate Matters**

- In November 2019, CRISPR Therapeutics announced the pricing of an underwritten public offering of 4,250,000 common shares at a public offering price of \$64.50 per share, plus the exercise in full of the underwriters' option to purchase 637,500 additional common shares. Gross proceeds from the offering (including the exercise of the underwriters' option), before deducting underwriting discounts and commissions and other offering expenses, were \$315.2 million. The initial offering closed in November 2019 and the closing of the option to purchase additional shares occurred in December 2019.
- In 2019, CRISPR Therapeutics broadened its business development efforts through additional transactions, including an agreement with Bayer to place Casebia Therapeutics, previously a joint venture between Bayer and CRISPR Therapeutics, under CRISPR Therapeutics' direct management; a license agreement with KSQ Therapeutics whereby CRISPR Therapeutics gains access to KSQ intellectual property (IP) for editing certain novel gene targets in its allogeneic oncology cell therapy programs, and KSQ gains access to CRISPR Therapeutics' IP for editing novel gene targets for certain applications; a collaboration and license agreement with ProBioGen focused on the development of novel *in vivo* delivery modalities for CRISPR/Cas9 leveraging ProBioGen's existing technology and expertise; and a collaboration with StrideBio, which expands upon an existing agreement to generate engineered adeno-associated viruses (AAV) capsids with improved properties for *in vivo* gene editing programs and includes additional undisclosed applications.
- In June 2019, CRISPR Therapeutics and Vertex expanded their collaboration and entered into an exclusive licensing agreement to discover and develop gene editing therapies for the treatment of Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1). In connection with this agreement, CRISPR Therapeutics received a \$175.0 million up-front payment from Vertex, and is eligible to receive milestone payments from Vertex of up to \$825.0 million in the aggregate. CRISPR Therapeutics continues to make advancements with programs utilizing an *in vivo* approach, which remains a key area of focus.
- In June 2019, CRISPR Therapeutics received notification that the United States Patent and Trademark Office (USPTO) has initiated an interference proceeding at the Patent Trial and Appeal Board between certain pending U.S. patent applications co-owned by the University of California, the University of Vienna and Dr. Emmanuelle Charpentier (collectively, the "CVC Group") and certain patents and a patent application currently owned by the Broad Institute, Harvard University and the Massachusetts Institute of Technology, all of which are related to the single guide format of CRISPR/Cas9 genome editing technology in eukaryotic cells. As of December 2019, the USPTO has granted twenty-one patents to the CVC group. None of these issued patents are involved in the interference.

#### **Fourth Quarter 2019 Financial Results**

- **Cash Position:** Cash and cash equivalents as of December 31, 2019, were \$943.8 million, compared to \$456.6 million as of December 31, 2018, an increase of \$487.2 million. The increase in cash was primarily driven by cash from financing activities of \$430.9 million from our at-the market offering, completed in July 2019; from our November public offering; and from stock options exercised during 2019. In addition, the Company generated cash from operations in the current year of \$56.7 million, driven by cash received from Vertex for milestone and option payments of \$208.0 million as well as cash obtained from the Bayer transaction of \$31.8 million, offset by operating expenses.
- **Revenue:** Total collaboration revenue was \$77.0 million for the fourth quarter of 2019 compared to \$0.1 million for fourth quarter of 2018, and \$289.6 million for the year ended December 31, 2019, compared to \$3.1 million for the year ended December 31, 2018. The increase in revenue was primarily attributable to revenue recognized in connection with the Company's collaboration agreements with Vertex.
- **R&D Expenses:** R&D expenses were \$48.8 million for the fourth quarter of 2019 compared to \$28.8 million for the fourth quarter of 2018, and \$179.4 million for the year ended December 31, 2019 compared to \$113.8 million for the year ended December 31, 2018. The increase in expense for the year was driven by increased headcount and development activities supporting the advancement of the hemoglobinopathies program, the broadening of the Company's wholly-owned immunoncology portfolio and \$10.0 million of non-cash expense related to the Company's collaboration with Vertex.
- **G&A Expenses:** General and administrative expenses were \$17.3 million for the fourth quarter of 2019 compared to \$16.5 million for the fourth quarter of 2018, and \$63.5 million for the year ended December 31, 2019, compared to \$48.3 million

for the year ended December 31, 2018. The increase in general and administrative expenses for the year was driven by headcount-related expense and external professional and consulting service expense.

- **Net Income/Loss:** Net income was \$30.5 million for the fourth quarter of 2019 compared to net loss of \$47.6 million for the fourth quarter of 2018, and net income was \$66.9 million for the year ended December 31, 2019, compared to a loss of \$165.0 million for the year ended December 31, 2018.

#### **About CTX001™**

CTX001 is an investigational *ex vivo* CRISPR gene-edited therapy that is being evaluated for patients suffering from TDT or severe SCD in which a patient's hematopoietic stem cells are engineered to produce high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is a form of the oxygen-carrying hemoglobin that is naturally present at birth and is then replaced by the adult form of hemoglobin. The elevation of HbF by CTX001 has the potential to alleviate transfusion requirements for TDT patients and painful and debilitating sickle crises for SCD patients.

CTX001 is being developed under a co-development and co-commercialization agreement between CRISPR Therapeutics and Vertex.

#### **About CTX110™**

CTX110 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting cluster of differentiation 19, or CD19, for the treatment of CD19+ malignancies. A wholly-owned asset of CRISPR Therapeutics, CTX110 is in a clinical trial designed to assess the safety and efficacy of CTX110 in relapsed or refractory B-cell malignancies. The multi-center, open-label clinical trial is designed to enroll up to 95 patients and investigate several dose levels of CTX110.

#### **About CTX120™**

CTX120 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting B-cell maturation antigen, or BCMA. A wholly-owned asset of CRISPR Therapeutics, CTX120 is in a clinical trial designed to assess the safety and efficacy of CTX120 in relapsed or refractory multiple myeloma. The multi-center, open-label clinical trial is designed to enroll up to 80 patients and investigate several dose levels of CTX120.

#### **About CTX130™**

CTX130 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting CD70, an antigen expressed on hematologic cancers. A wholly-owned asset of CRISPR Therapeutics, CTX130 is in development for the treatment of both solid tumors, such as renal cell carcinoma, and T-cell and B-cell hematologic malignancies.

#### **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 partnerships with leading companies including Bayer, 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 San Francisco, California and London, United Kingdom. For more information, please visit [www.crisprtx.com](http://www.crisprtx.com).

#### **CRISPR 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) the safety, efficacy and clinical progress of CRISPR Therapeutics' various clinical programs including CTX001, CTX110, CTX120 and CTX130; (ii) the status of 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; (iii) 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; (iv) the integration of Casebia Therapeutics; (v) the intellectual property coverage and positions of CRISPR Therapeutics, its licensors and third parties as well as the status and potential outcome of proceedings involving any such intellectual property; (vi) the sufficiency of CRISPR Therapeutics' cash resources; ; (vii) the expected benefits of CRISPR Therapeutics' collaborations, including those with Bayer, KSQ, ProBioGen, StrideBio and Vertex; and (viii) 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 potential for initial and preliminary data from any clinical trial (including CTX001, CTX110, CTX120 and CTX130) not to be indicative of final trial results; the risk that the initial data from a limited number of patients may not be indicative of results from the full planned study population; 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'; 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 the outcome of proceedings (such as an interference, an opposition or a similar proceeding) involving all or any portion of such intellectual property; the risk that the CRISPR Therapeutics' business and Casebia Therapeutics' business will not be integrated successfully; 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](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 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		Year Ended December 31,	
	December 31,			
	2019	2018	2019	2018
Collaboration revenue	\$ 77,016	\$ 115	\$ 289,590	\$ 3,124
Operating expenses:				
Research and development	48,762	28,801	179,362	113,773
General and administrative	17,271	16,542	63,488	48,294
Total operating expenses	<u>66,033</u>	<u>45,343</u>	<u>242,850</u>	<u>162,067</u>
Income (loss) from operations	10,983	(45,228)	46,740	(158,943)
Total other income (expense), net	<u>19,563</u>	<u>(2,128)</u>	<u>20,566</u>	<u>(5,485)</u>
Net income (loss) before income taxes	30,546	(47,356)	67,306	(164,428)
Provision for income taxes	<u>(4)</u>	<u>(234)</u>	<u>(448)</u>	<u>(553)</u>
Net income (loss)	30,542	(47,590)	66,858	(164,981)
Foreign currency translation adjustment	29	(7)	15	(22)
Comprehensive income (loss)	<u>\$ 30,571</u>	<u>\$ (47,597)</u>	<u>\$ 66,873</u>	<u>\$ (165,003)</u>
Reconciliation of net income (loss) to net income (loss) attributable to common shareholders:				
Net income (loss)	\$ 30,542	\$ (47,590)	\$ 66,858	\$ (164,981)
Net income (loss) per share attributable to common shareholders - basic	<u>\$ 0.53</u>	<u>\$ (0.92)</u>	<u>\$ 1.23</u>	<u>\$ (3.44)</u>
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic	<u>57,395,839</u>	<u>51,688,383</u>	<u>54,392,304</u>	<u>47,964,368</u>
Net income (loss) per share attributable to common shareholders - diluted	<u>\$ 0.51</u>	<u>\$ (0.92)</u>	<u>\$ 1.17</u>	<u>\$ (3.44)</u>
Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - diluted	<u>60,233,927</u>	<u>51,688,383</u>	<u>56,932,798</u>	<u>47,964,368</u>

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

	As of	
	December 31, 2019	December 31, 2018
Cash	\$ 943,771	\$ 456,649
Working capital	930,441	438,649
Total assets	1,066,752	489,016
Total shareholders' equity	939,425	392,195

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Source: CRISPR Therapeutics AG