**CRISPR Therapeutics Provides Business Update and Reports First Quarter 2020 Financial Results**

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

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

*-Began treating patients in clinical trial of CTX120™, targeting B-cell maturation antigen (BCMA)-*

*-IND and CTA approved for CTX130™, wholly-owned allogeneic CAR-T cell therapy targeting CD70-*

*-Received $25 million milestone payment from DMD/DM1 collaboration with Vertex in April 2020-*

ZUG, Switzerland and CAMBRIDGE, Mass., April 28, 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 first quarter ended March 31, 2020.

"We made substantial progress in the last quarter despite the challenges posed by COVID-19. We are now progressing five cell therapy clinical trials in parallel targeting hemoglobinopathies and various cancers," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "Our CTX001 and CTX110 programs continue to enroll patients, and we expect to report data for these programs this year. We are pleased to have begun treating patients in our CTX120 trial for the treatment of relapsed or refractory multiple myeloma and we expect to begin treating patients in our CTX130 trial in renal cell carcinoma and T-cell and B-cell hematologic malignancies in the second half of this year. Despite these unprecedented times, we continue to execute on our programs and we remain focused on our commitment to patients and their families."

**Recent Highlights and Outlook**

- **Beta Thalassemia and Sickle Cell Disease**
    - CRISPR Therapeutics and its partner Vertex remain on track to provide additional data from the two ongoing Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 in patients with transfusion-dependent beta thalassemia and in patients with severe sickle cell disease in 2020. New data expected in 2020 include initial data from additional patients dosed in each of the Phase 1/2 studies and longer duration follow-up data for the first patients dosed in each study. Screening, enrollment and mobilization in these studies is ongoing, however no additional patients are scheduled to initiate conditioning or dosing at this time due to COVID-19.
- **Immuno-Oncology**
    - Patient enrollment continues in a clinical trial to assess the safety and efficacy of CTX110, CRISPR Therapeutics' 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 treating patients with these hematologic malignancies. The Company expects to report top-line data for CTX110 at the end of 2020.
    - CRISPR Therapeutics has begun treating 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.
    - Earlier this year, the U.S. Food and Drug Administration (FDA) accepted CRISPR Therapeutics' Investigational New Drug (IND) application for 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. Additionally, CRISPR Therapeutics has obtained approval from Health Canada for its Clinical Trial Application (CTA). The Company expects to begin treating patients with CTX130 in the second half of this year.

- **Other Corporate Matters**

    - Under the June 2019 collaboration agreement with Vertex to discover and develop gene editing therapies for the treatment of Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1), CRISPR Therapeutics received a payment of $25 million from Vertex related to the achievement of a research milestone in the DM1 program. CRISPR Therapeutics is eligible to receive additional milestone payments from Vertex of up to $800 million for these two programs.

    - CRISPR Therapeutics is scheduled to present two posters during the virtual American Society of Gene & Cell Therapy 2020 Annual Meeting, to be held from May 12 to 15, 2020, via https://annualmeeting.asgct.org/am20/, as follows:

      **Title:** Dual Guide CRISPR/Cas9 Editing of the CCR5 Gene Provides Complete Protection Against HIV in Humanized Mouse Models (abstract #1046)
      **Session Title**: Gene Targeting and Gene Correction
      **Date and Time**: Thursday, May 14, 2020; 5:30 PM - 6:30 PM

      **Title**: Multiplexing of Up to 10 Gene Edits Using CRISPR/Cas9 to Generate CAR-T Cells with Improved Function (abstract #1151)
      **Session Title**: Cancer - Immunotherapy, Cancer Vaccines
      **Date and Time**: Thursday, May 14, 2020; 5:30 PM - 6:30 PM

    - In February, CRISPR Therapeutics announced its proposal to elect Doug Treco, Ph.D. to its Board of Directors at the Company's upcoming annual general meeting to be held later this year.

**First Quarter 2020 Financial Results**

- **Cash Position:** Cash and cash equivalents as of March 31, 2020, were $889.7 million, compared to $943.8 million as of December 31, 2019, a decrease of $54.1 million. The decrease in cash was primarily driven by cash used in operating activities of $52.2 million to support spending on the Company's clinical and pre-clinical programs, as well as payroll and payroll-related expenses to support growth. In April 2020, the Company received a milestone payment of $25 million from Vertex under the collaboration agreement for DMD and DM1, resulting in pro forma cash exceeding $900 million.

- **Revenue:** Total collaboration revenue was $0.2 million for the first quarter of 2020 compared to $0.3 million for first quarter of 2019. Collaboration revenue primarily consisted of charges to partners for research activities.

- **R&D Expenses:** R&D expenses were $54.2 million for the first quarter of 2020 compared to $33.8 million for the first quarter of 2019. The increase in expenses was driven by increased headcount and development activities supporting the advancement of the hemoglobinopathies program and wholly-owned immuno-oncology programs.

- **G&A Expenses:** General and administrative expenses were $19.6 million for the first quarter of 2020 compared to $14.9 million for the first quarter of 2019. The increase in general and administrative expenses for the year was driven by headcount-related expense and higher facilities cost.

- **Net Loss:** Net loss was $69.7 million for the first quarter of 2020 compared to net loss of $48.4 million for the first quarter of 2019.

**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 being investigated in a clinical trial designed to assess the safety and efficacy of CTX110 for the treatment of 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 being investigated in a clinical trial designed to assess the safety and efficacy of CTX120 for the treatment of 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 cluster of differentiation 70, or CD70, an antigen expressed on various solid tumors and hematologic malignancies. A wholly-owned asset of CRISPR Therapeutics, CTX130 is being developed 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.

**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) potential impacts due to the coronavirus pandemic; (ii) the safety, efficacy and clinical progress of CRISPR Therapeutics' various clinical programs including CTX001, CTX110, CTX120 and CTX130; (iii) the status of clinical trials (including, without limitation, the timing of filing of clinical trial applications and INDs, any approvals thereof, the timing of commencement of clinical trials and the expected timing of data releases), development timelines and discussions with regulatory authorities related to product candidates under development by CRISPR Therapeutics and its collaborators; (iv) 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; (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; 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 impacts due to the coronavirus pandemic such as (x) delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy; (y) the timing and progress of clinical trials, preclinical studies and other research and development activities; and (z) the overall impact of the coronavirus pandemic on its business, financial condition and results of operations; 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 (as is the case with CTX001 at this time) 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' 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; 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; 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. 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 March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Collaboration revenue | $ 157 | $ 328 |
| Operating expenses: | | |
| Research and development | 54,193 | 33,822 |
| General and administrative | 19,550 | 14,929 |
| Total operating expenses | 73,743 | 48,751 |
| Loss from operations | (73,586) | (48,423) |
| Total other income, net | 4,232 | 100 |
| Net loss before income taxes | (69,354) | (48,323) |
| Provision for income taxes | (377) | (85) |
| Net loss | (69,731) | (48,408) |
| Foreign currency translation adjustment | (25) | 8 |
| Comprehensive loss | $ (69,756) | $ (48,400) |
| Reconciliation of net loss to net loss attributable to common shareholders: | | |
| Net loss | $ (69,731) | $ (48,408) |
| Net loss per share attributable to common shareholders - basic | $ (1.15) | $ (0.93) |
| Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic | 60,847,683 | 52,093,208 |

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

| | As of | |
|---|---|---|
| | March 31, 2020 | December 31, 2019 |
| Cash | $ 889,712 | $ 943,771 |
| Working capital | 874,196 | 930,441 |
| Total assets | 1,007,300 | 1,066,752 |
| Total shareholders' equity | 886,097 | 939,425 |

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Source: CRISPR Therapeutics AG