**CRISPR Therapeutics Provides Business Update and Reports Third Quarter 2019 Financial Results**

*-Provides update from ongoing Phase 1/2 clinical trials of CTX001® for patients with severe hemoglobinopathies-*

*-Began treating patients in Phase 1/2 clinical trial of CTX110 ™, targeting CD19+ malignancies-*

ZUG, Switzerland and CAMBRIDGE, Mass., Oct. 28, 2019 (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 third quarter ended September 30, 2019.

"In 2019, we've made significant progress across several development programs, including ongoing enrollment of our CTX001 trials in beta thalassemia and severe sickle cell disease, with preliminary data expected from these programs later this year. We also began treating patients in our clinical trial for CTX110, our allogeneic CAR-T therapy, and are advancing additional CAR-T candidates toward clinical development," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "Looking forward, we expect a robust 2020, with continued focus on execution as we anticipate conducting five clinical trials in parallel."

**Recent Highlights and Outlook**

- **Beta Thalassemia and Sickle Cell Disease**
    - Enrollment is ongoing at six clinical trial sites in the U.S., Canada and Europe for the Phase 1/2 study of CTX001 in patients with transfusion-dependent beta thalassemia (TDT) and at ten clinical trial sites in the U.S., Canada and Europe for the study in patients with severe sickle cell disease (SCD).
    - The European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion for orphan drug designation (ODD) of CTX001 for the treatment of TDT. In addition, we are expanding the TDT patient population for CTX001 to include beta zero/beta zero subtypes.
    - The Company expects to release preliminary safety and efficacy data from the ongoing Phase 1/2 clinical trials in late 2019.
- **Immuno-Oncology**
    - The Company has begun treating patients in a Phase 1/2 trial to assess the safety and efficacy of CTX110, its wholly-owned allogeneic CAR-T cell therapy targeting CD19+ malignancies. The multi-center, open label trial is designed to enroll up to 95 patients and investigate several dose levels of CTX110. The study is currently enrolling at five clinical trial sites in the U.S. and Australia. In addition, the Company obtained approval from Health Canada for its Clinical Trial Application (CTA). The Company believes its CRISPR-based allogeneic CAR-Ts may have a superior product profile compared to current autologous therapies and allow accessibility to broader patient populations.
    - The Company expects to initiate a Phase 1/2 clinical trial of CTX120™, targeting B-cell maturation antigen (BCMA) for the treatment of multiple myeloma, in the first half of 2020. CRISPR Therapeutics continues to advance additional allogeneic CAR-T candidates toward clinical development including CTX130™, targeting CD70 for the treatment of solid tumors and hematologic malignancies. The Company continues to scale its capabilities to enable rapid advancement of these programs into and through the clinic.
    - The Company recently announced it entered into a license agreement with KSQ Therapeutics whereby CRISPR Therapeutics gained access to KSQ intellectual property (IP) for editing certain novel gene targets in its allogeneic oncology cell therapy programs, and KSQ gained access to CRISPR Therapeutics' IP for editing novel gene targets identified by KSQ as part of its current and future eTIL$^{TM}$ (engineered tumor infiltrating lymphocyte) cell programs. The agreement further strengthens the Company's proprietary allogeneic CAR-T platform.
    - The Company will present a poster at the Society for Immunotherapy of Cancer (SITC) Conference on November

9, 2019 related to single-cell RNA sequencing and functional assessment of healthy donor and cancer patient-derived T and CAR-T cells (#P187).

- **Regenerative Medicine**
  - On September 17, 2019, CRISPR Therapeutics, in collaboration with ViaCyte, presented positive *in vitro* data for a potential immune-evasive cell replacement therapy for diabetes at the 55th Annual Meeting of the European Association for the Study of Diabetes (EASD) in Barcelona, Spain. The oral presentation included new data that demonstrate the successful differentiation of CRISPR-edited human pluripotent stem cells to pancreatic precursor cells.

- **Other Corporate Matters**
  - Vertex has exercised the options granted under the collaboration it established with CRISPR Therapeutics in 2015 to in-license three additional targets for the development of treatments using CRISPR-based gene editing. The targets include the cystic fibrosis transmembrane conductance regulator (CFTR) gene and two undisclosed targets. Under the terms of the agreement, CRISPR Therapeutics will receive an upfront payment of $30 million in connection with the option exercise and has the potential to receive up to $410 million in development, regulatory and commercial milestones and royalties on net product sales for each of the three targets, and Vertex will receive exclusive rights to develop and commercialize products related to these targets globally. The research term of the Company's 2015 collaboration with Vertex has now expired, and Vertex no longer holds rights to in-license additional targets under that agreement.
  - The Company recently announced proposed plans that Casebia Therapeutics, previously a joint venture between CRISPR Therapeutics and Bayer, would operate under the direct management of CRISPR Therapeutics. Upon closing of the transaction, Casebia Therapeutics would focus on the development of its lead programs in hemophilia, ophthalmology and autoimmune diseases, with Bayer having opt-in rights for two products at IND submission. The transaction is subject to negotiation and execution of definitive agreements as well as certain customary conditions. The Company and Bayer are negotiating the definitive agreements and, subject to the finalization of the definitive agreements and satisfaction of closing conditions, anticipate to close the transaction in the fourth quarter of 2019.

**Third Quarter 2019 Financial Results**

- **Cash Position:** Cash as of September 30, 2019, was $629.7 million, compared to $427.9 million as of June 30, 2019, an increase of $201.8 million as increased cash operating expenses were offset by $68.6 million net proceeds from financing activities and $175 million upfront payments received from Vertex related to the 2019 collaboration agreement announced in June.

- **Revenues:** Total collaboration revenues were $211.9 million for the third quarter of 2019 compared to $0.6 million for third quarter of 2018 with the increase primarily driven by the collaboration agreement with Vertex.

- **R&D Expenses:** R&D expenses were $57.2 million for the third quarter of 2019 compared to $39.8 million for the third quarter of 2018. The increase was driven by increased headcount and services expense supporting the advancement of the hemoglobinopathies program, the broadening of the Company's wholly-owned immuno-oncology portfolio, as well as increased investment in the Company's CRISPR/Cas9 platform research and some non-cash expense related to the Company's collaboration with Vertex.

- **G&A Expenses:** General and administrative expenses were $15.5 million for the third quarter of 2019 compared to $10.2 million for the third quarter of 2018. The increase was driven by increased headcount-related expense and external professional and consulting service expense.

- **Net Income (Loss):** Net Income was $138.4 million for the third quarter of 2019 compared to a loss of $50.7 million for the third quarter of 2018, driven predominantly by increased revenue recognized in connection with the Company's collaboration with Vertex.

**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 collaborations with leading companies including Bayer AG, 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 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) the safety, efficacy and clinical progress of CRISPR Therapeutics' various clinical programs including CTX001, CTX110, CTX 120 and CTX 130; (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 proposed transaction involving 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 KSQ 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 (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' and/or Casebia 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; uncertainties inherent in the proposed transaction involving Casebia Therapeutics, including the expected timing for completion of such transaction and the possibility such transaction is not consummated; 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. 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 September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Collaboration revenue | $ 211,928 | $ 563 | $ 212,574 | $ 3,009 |
| Operating expenses: | | | | |
| Research and development | 57,246 | 39,820 | 130,601 | 84,972 |

| | | | | |
|---|---|---|---|---|
| General and administrative | 15,519 | 10,175 | 46,216 | 31,752 |
| Total operating expenses | 72,765 | 49,995 | 176,817 | 116,724 |
| Income (loss) from operations | 139,163 | (49,432) | 35,757 | (113,715) |
| Total other income (expense), net | (466) | (1,142) | 1,003 | (3,357) |
| Net income (loss) before income taxes | 138,697 | (50,574) | 36,760 | (117,072) |
| Provision for income taxes | (274) | (137) | (444) | (319) |
| Net income (loss) | 138,423 | (50,711) | 36,316 | (117,391) |
| Foreign currency translation adjustment | (12) | (6) | (14) | (15) |
| Comprehensive income (loss) | $ 138,411 | $ (50,717) | $ 36,302 | $ (117,406) |
| Reconciliation of net income (loss) to net income (loss) attributable to common shareholders: | | | | |
| Net income (loss) | $ 138,423 | $ (50,711) | $ 36,316 | $ (117,391) |
| Net income (loss) per share attributable to common shareholders - basic | $ 2.52 | $ (1.07) | $ 0.68 | $ (2.51) |
| Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - basic | 54,829,057 | 47,391,988 | 53,380,123 | 46,709,388 |
| Net income (loss) per share attributable to common shareholders - diluted | $ 2.40 | $ (1.07) | $ 0.65 | $ (2.51) |
| Weighted-average common shares outstanding used in calculating net loss per share attributable to common shareholders - diluted | 57,598,901 | 47,391,988 | 55,821,420 | 46,709,388 |

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

| | As of | |
|---|---|---|
| | 30-Sep-19 | 31-Dec-18 |
| Cash | $ 629,717 | $ 456,649 |
| Working capital | 584,286 | 438,649 |
| Total assets | 720,590 | 489,016 |
| Total shareholders' equity | 591,878 | 392,195 |

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Source: CRISPR Therapeutics AG