



CRISPR Therapeutics Highlights New Additions to Portfolio of Allogeneic CRISPR-based CAR-T Therapies at SITC Annual Meeting

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- CRISPR-based CAR-T cell therapy candidates targeting CD19+, BCMA and CD70 demonstrate high editing rates, and potent anti-tumor activity in preclinical models -
- Expanding applicability of CRISPR-based CAR-T therapy to solid tumors -

ZUG, Switzerland and CAMBRIDGE, Mass., Nov. 10, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a genome editing company focused on creating transformative medicines for serious diseases, today described advances in its portfolio of wholly owned allogeneic CAR-T therapeutic candidates created with CRISPR/Cas9 gene editing. These presentations took place during a poster session and reception at the Society for Immunotherapy 32nd Annual Meeting.

"We are making rapid progress on our portfolio of CRISPR-based allogeneic CAR-T programs. Today at the SITC Annual Meeting, scientists from CRISPR presented encouraging data from our CD19 Allogeneic CAR-T development program including *in vitro* and *in vivo* data demonstrating potent anti-tumor activity. Based on these data, we have initiated manufacturing and other pre-clinical activities and expect to file an IND for this program in late 2018," said Sam Kulkarni, President of CRISPR Therapeutics.

"We believe that the precision and efficiency of multiplexed editing with CRISPR/Cas9 will allow us to access the full potential of immune cell therapy in solid tumors and off-the-shelf products," commented Tony Ho, MD, Head of R&D at CRISPR Therapeutics. "We have now built the capabilities necessary to rapidly generate new CAR-T product candidates with high consistency and potency, and are advancing the next two candidates in our allogeneic CAR-T portfolio targeting BCMA and CD70. Our CAR-T portfolio aims to demonstrate the power of our allogeneic platform using validated hematologic targets, and expand into solid tumors with our CD70 program."

CRISPR believes that its CAR-T cells therapies may have distinct advantages over the current generation of autologous cell therapies including better access due to its "off-the-shelf" nature, and greater efficacy and safety due to the homogeneity and consistency of the product.

These data were presented during the Society for Immunotherapy in Cancer 32nd Annual Meeting during the Cellular Therapy Approaches Track poster session and a company-sponsored reception highlighting allogeneic CRISPR-based CAR-T cell therapy. The poster, *Production of site-specific Allogeneic CD19 CAR-T Cells by CRISPR-Cas9 for B-Cell Malignancies* and an archive of the reception discussion can be found on the [event calendar page](#) of CRISPR Therapeutics' website.

About CRISPR-based allogeneic CAR-T programs

The lead program in CRISPR's immuno-oncology portfolio, CTX101, is an allogeneic CD19 CAR-T product that has several potential advantages over other approaches in the clinic due to the unique capabilities of the CRISPR/Cas9 system to achieve efficient and specific multiplexed editing. First, the CD19 chimeric antigen receptor, or CAR, is inserted into a specifically chosen locus rather than the random insertion common in current-generation products. Second, the T cell receptor (TCR) is eliminated to enable off-the-shelf use of a single batch of product in many different patients. Finally, the class 1 major histocompatibility complex (MHC I) is eliminated to improve durability of the CAR-T cells in the off-the-shelf setting. CTX101 is based on healthy donor cells that are edited *ex vivo* using CRISPR/Cas9, a process that the company has optimized and successfully transitioned to a GMP-capable CMO. CRISPR anticipates filing and IND with the US FDA in late 2018 with clinical trials beginning for CTX101 in early 2019.

To supplement its internal programs, the company is continuing to develop collaborations in immuno-oncology, and recently announced a two-year research collaboration and license option agreement with Massachusetts General Hospital (MGH) to develop novel T cell therapies for cancer and a partnership with Neon Therapeutics to create neo-antigen based T-cell constructs. Marcela V. Maus, MD, PhD, Director of the Cellular Immunotherapy Program at MGHCC and Assistant Professor of Medicine at Harvard Medical, will lead the scientific work at MGH.

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. For more information, please visit <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 timing of filing of clinical trial applications and INDs and timing of commencement of clinical trials, the intellectual property coverage and positions of the Company, its licensors and third parties, the sufficiency of the Company's cash resources and the therapeutic value, development, and commercial potential of CRISPR/Cas-9 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. 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.

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