CRISPR Therapeutics and ViaCyte Announce Strategic Collaboration to Develop Gene-Edited Stem Cell-Derived Therapy for Diabetes

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- Aims to develop an immune-evasive stem cell therapy as a potentially curative treatment for diabetes -

- Parties will collaborate through commercialization and share costs and profits worldwide -

ZUG, Switzerland and CAMBRIDGE, Mass. and SAN DIEGO, Sept. 17, 2018 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ: CRSP), a biopharmaceutical company focused on developing transformative gene-based medicines for serious diseases, and ViaCyte, Inc., a privately held regenerative medicine company, today announced a collaboration focused on the discovery, development, and commercialization of gene-edited allogeneic stem cell therapies for the treatment of diabetes.

Decades of clinical data with islet transplants indicate that beta-cell replacement approaches may offer curative benefit to patients with insulin-requiring diabetes. ViaCyte has pioneered the approach of generating pancreatic-lineage cells from stem cells and delivering them safely and efficiently to patients. PEC-Direct, ViaCyte's lead product candidate currently being evaluated in the clinic, uses a non-immunoprotective delivery device that permits direct vascularization of the cell therapy. This approach has the potential to deliver durable benefit; however, because the patient's immune system will identify these cells as foreign, PEC-Direct will require long-term immunosuppression to avoid rejection. As a result, PEC-Direct is being developed as a therapy for the subset of patients with type 1 diabetes at high risk for acute complications.

CRISPR gene editing offers the potential to protect the transplanted cells from the patient's immune system by ex vivo editing immune-modulatory genes within the stem cell line used to produce the pancreatic-lineage cells. The speed, specificity, and multiplexing efficiency of the CRISPR system make it ideally suited to this task. CRISPR Therapeutics is pursuing a similar approach for its allogeneic CAR-T programs and has established significant expertise in immune-evasive gene editing. The combination of ViaCyte's stem cell capabilities and CRISPR's gene editing capabilities has the potential to enable a beta-cell replacement product that may deliver durable benefit to patients without triggering an immune reaction.

"We believe the combination of regenerative medicine and gene editing has the potential to offer durable, curative therapies to patients in many different diseases, including common chronic disorders like insulin-requiring diabetes. ViaCyte is a pioneer in the regenerative medicine field, and has built a compelling clinical program, robust manufacturing capabilities, and assembled a strong intellectual property position. Partnering with ViaCyte will allow us to accelerate our efforts in regenerative medicine, an area that we believe will provide a variety of longer-term opportunities for our company," commented Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics.

Under the terms of the agreement, CRISPR and ViaCyte will jointly seek to develop an immune-evasive stem cell line as a first step on the path to an allogeneic stem-cell derived product. Upon successful completion of these studies and identification of a product candidate, the parties will jointly assume responsibility for further development and commercialization worldwide. Upon execution of the agreement ViaCyte will receive $15 million from CRISPR, which at CRISPR's election may be paid in either cash or CRISPR stock. ViaCyte also has the option, under certain circumstances, to receive an additional $10 million from CRISPR in the form of a convertible promissory note.

"Creating an immune-evasive gene-edited version of our technology would enable us to address a larger patient population than we could with a product requiring immunosuppression. CRISPR Therapeutics is the ideal partner for this program given their leading gene editing technology and expertise and focus on immune-evasive editing. We are thrilled to have the opportunity to partner with CRISPR Therapeutics on what we believe could be a transformational therapy for patients with insulin-requiring diabetes," commented Paul Laikind, Ph.D., Chief Executive Officer and President of ViaCyte. "We also believe that this approach may have many other applications which we and CRISPR may explore in the future."

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 and rare diseases. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic collaborations with leading companies including Bayer AG and Vertex Pharmaceuticals. 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.

About ViaCyte
ViaCyte is a privately-held regenerative medicine company developing novel cell replacement therapies as potential long-term diabetes treatments to achieve glucose control targets and reduce the risk of hypoglycemia and diabetes-related complications. ViaCyte's product candidates are based on the derivation of pancreatic progenitor cells from stem cells, which are then implanted in durable and retrievable cell delivery devices. Once implanted and matured, these cells are designed to secrete insulin and other pancreatic hormones in response to blood glucose levels. ViaCyte has two product candidates in clinical-stage development. The PEC-Direct™ product candidate delivers the pancreatic progenitor cells in a non-immunoprotective device and is being developed for type 1 diabetes patients who have hypoglycemia unawareness, extreme glycemic lability, and/or recurrent severe hypoglycemic episodes. The PEC-Encap™ (also known as VC-01) product candidate delivers the same pancreatic progenitor cells in an immunoprotective device and is being developed for all patients with diabetes, type 1 and type 2, who use insulin. ViaCyte is also seeking to develop immune-evasive 'universal donor' stem cell lines, from its proprietary CyT49 cell line, which are expected to further broaden the availability of cell therapy for diabetes and other potential indications. ViaCyte is headquartered in San Diego, California. ViaCyte is funded in part by the California

Institute for Regenerative Medicine (CIRM) and JDRF. For more information, please visit www.viacyte.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, any approvals thereof and timing of commencement of clinical trials, the intellectual property coverage and positions of CRISPR Therapeutics, its licensors and third parties, the sufficiency of CRISPR Therapeutics' cash resources 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 CRISPR Therapeutics 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 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; expectations for regulatory approvals to conduct trials or to market products; 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 (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. 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 Media Contact:
Jennifer Paganelli
WCG on behalf of CRISPR
347-658-8290
jpaganelli@wcgworld.com

CRISPR Investor Contact:
Susan Kim
617-307-7503
susan.kim@crisprtx.com

ViaCyte Media Contact:
Jessica Yingling, Ph.D.
Little Dog Communications, Inc.
858-344-8091
jessica@litldog.com

ViaCyte Investor Contact:
Eugene Brandon, Ph.D.
858-455-3708
ebrandon@viacyte.com

ViaCyte, Inc. Logo

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