CRISPR Therapeutics and ViaCyte Present Positive In Vitro Data Towards a Potential Immune-Evasive Cell Replacement Therapy for Diabetes at EASD 2019

-New data demonstrate successful differentiation of CRISPR-edited human pluripotent stem cells to pancreatic precursor cells-

ZUG, Switzerland, CAMBRIDGE, Mass., and SAN DIEGO, Sept. 17, 2019 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP), and ViaCyte, Inc., a privately-held cell therapy company, today presented data from the Companies' regenerative medicine program targeted towards type 1 diabetes (T1D) in an oral presentation at the 55th Annual Meeting of the European Association for the Study of Diabetes (EASD) in Barcelona, Spain. The data demonstrate that the CyT49 pluripotent stem cell line, which has been shown to be amenable to efficient scaling and differentiation, can be successfully edited with CRISPR. The CyT49 pluripotent stem cell line is currently being used to generate islet progenitors for clinical trials.

"These data provide further evidence that 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," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "We look forward to advancing our T1D program in partnership with ViaCyte."

"We are pleased with the data presented at EASD, which bring us potentially one step closer to a transformational therapy for patients with insulin-requiring diabetes through the development of an immune-evasive gene-edited version of our technology," said Paul Laikind, Ph.D., Chief Executive Officer and President of ViaCyte. "ViaCyte has led the field over the past decade, being the first group to demonstrate a number of essential milestones on the path to a broadly applicable cell replacement therapy for diabetes. Now, in partnership with CRISPR Therapeutics, we aim to achieve yet another first, the development of an immune-evasive cell replacement therapy as a potential cure for T1D. The work being presented at EASD is an important step along that path."

To protect pancreatic islet cells from immune rejection, researchers utilized CRISPR/Cas9 gene editing to generate CyT49 clones that lack the β2-microglobulin (B2M) gene, a required component of the major histocompatibility complex class I (MHC-I), and express a transgene encoding programmed death-ligand 1 (PD-L1) to further protect from T-cell attack. Edited clonal cells maintained karyotypic stability and showed *in vitro* protection against T-cell mediated cell lysis.

About the CRISPR-ViaCyte Collaboration
CRISPR Therapeutics and ViaCyte entered into a strategic collaboration in 2018 focused on the discovery, development, and commercialization of novel regenerative medicines including gene-edited allogeneic stem cell-derived therapies for the treatment of diabetes. The Companies are currently evaluating a preclinical-stage therapeutic candidate for insulin-requiring diabetes including type 1 diabetes, for which the Companies will jointly assume responsibility for development and commercialization worldwide.

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.

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 islet progenitor cells from pluripotent stem cells, which are then implanted in durable and retrievable cell delivery devices. Over a decade ago, ViaCyte scientists were the first to report on the production of pancreatic cells from a stem cell starting point and the first to demonstrate in an animal model of diabetes that, once implanted and matured, these cells 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

islet 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 islet 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 developing immune-evasive stem cell lines, from its proprietary CyT49 cell line, which have the potential 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

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 our various clinical programs including CTX001 and CTX110; (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 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; (v) the sufficiency of CRISPR Therapeutics' cash resources; and (vi) 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 and CTX110) 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.

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