Vertex and CRISPR Therapeutics Announce Licensing Agreement to Accelerate Development of Vertex's Hypoimmune Cell Therapies for the Treatment of Type 1 Diabetes

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-Vertex to receive non-exclusive rights to CRISPR Therapeutics' CRISPR/Cas9 to accelerate development of potentially curative cell therapies for T1D-

-CRISPR Therapeutics to receive $100M upfront payment plus milestone and royalty payments on potential future gene-edited hypoimmune T1D products-

BOSTON & ZUG, Switzerland--(BUSINESS WIRE)--Mar. 27, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and CRISPR Therapeutics (Nasdaq: CRSP) today announced that they have entered into a new non-exclusive licensing agreement for the use of CRISPR Therapeutics' gene editing technology, known as CRISPR/Cas9, to accelerate the development of Vertex's hypoimmune cell therapies for type 1 diabetes (T1D).

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20230324005339/en/

"We have multiple programs in our T1D portfolio including VX-880 and VX-264, which are in the clinic, as well as our hypoimmune program, in preclinical development," said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "Having successfully demonstrated clinical proof of concept in T1D in our VX-880 program, we are excited to deepen our relationship with CRISPR Therapeutics with this agreement, which will allow us to further accelerate our goal of generating fully differentiated, insulin-producing hypoimmune islet cells for T1D."

"We are pleased to expand our long and successful relationship with Vertex with this collaboration which fully leverages our gene editing platform to develop hypoimmune cell therapies for T1D," said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. "In parallel, we continue to expand our capabilities in regenerative medicine and advance our existing allogeneic gene-edited cell therapy programs."

Under this agreement, Vertex will pay CRISPR Therapeutics $100 million up-front for non-exclusive rights to CRISPR Therapeutics' technology for the development of hypoimmune gene-edited cell therapies for T1D. CRISPR Therapeutics will be eligible for up to an additional $230 million in research and development milestones and receive royalties on any future products resulting from this agreement.

CRISPR and ViaCyte, Inc., which was acquired by Vertex in 2022, will continue to collaborate on their existing gene-edited allogeneic stem cell therapies, using ViaCyte cells, for the treatment of diabetes under the terms of their collaboration. A Phase 1/2 study of VCTX211, an allogeneic, gene-edited, stem cell-derived product candidate for T1D, which originated under the CRISPR Therapeutics and ViaCyte collaboration, has been initiated and is ongoing. CRISPR Therapeutics will not obtain any interest in Vertex's pre-existing pipeline of T1D products, including VX-880 and VX-264.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF,Vertex has a robust clinical pipeline of investigational small molecule, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, and alpha-1 antitrypsin deficiency.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 13 consecutive years on Science magazine's Top Employers list and one of Fortune's Best Workplaces in Biotechnology and Pharmaceuticals and Best Workplaces for Women. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

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Vertex Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements by Bastiano Sanna, Ph.D., and Samarth Kulkarni, Ph.D., in this press release, statements about the terms of and expectations for Vertex's collaboration with CRISPR, potential benefits and results that may be achieved through the collaboration, including acceleration of the development of Vertex's hypoimmune cell therapies for T1D, statements regarding the future activities of the parties pursuant to the collaboration, statements regarding upfront and milestone payments, and potential royalties on future products, and statements regarding the collaboration between CRISPR and ViaCyte and that CRISPR will not obtain any interest in Vertex's pre-existing pipeline of T1D products. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the anticipated benefits and potential of Vertex's collaboration with CRISPR may not be achieved on the anticipated timeline, or at all, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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, 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 in Boston, Massachusetts and San Francisco, California, and business offices in London, United Kingdom. For more information, please visit www.crisprtx.com.

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CRISPR Therapeutics 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 without limitation, statements made by Samarth Kulkarni, Ph.D. and Bastiano Sanna, Ph.D. in this press release, as well as statements regarding CRISPR Therapeutics' expectations about any or all of the following: (i) the future activities of the parties pursuant to the ViaCyte collaboration and the expected benefits of such collaboration, including expectations regarding VCTX211; (ii) the safety, efficacy and progress of its clinical programs; (iii) upfront and milestone payments, and potential royalties on future products, under the non-exclusive license; and (vii) 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: that it may not realize the potential benefits of its collaborations on the anticipated timeline, or at all; the potential that clinical trial results may not be favorable; that one or more of product candidate programs will not proceed as planned for technical, scientific or commercial reasons; and those risks and uncertainties described under the heading "Risk Factors" in CRISPR Therapeutics' most recent annual report on Form 10-K, quarterly report on Form 10-Q 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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