Vertex Announces FDA Fast Track Designation and Initiation of a Phase 1/2 Clinical Trial for VX-880, a Novel Investigational Cell Therapy for the Treatment of Type 1 Diabetes

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– VX-880 is the first investigational stem cell-derived therapy utilizing fully differentiated, insulin-producing pancreatic islet cells for the treatment of type 1 diabetes –

– VX-880 is the first and only pancreatic islet replacement therapy known to receive Fast Track Designation –

BOSTON--(BUSINESS WIRE)--Mar. 10, 2021--

Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for VX-880 and that the company has initiated a clinical trial for VX-880 in patients who have type 1 diabetes (T1D) with severe hypoglycemia and impaired hypoglycemic awareness.

"This program has its roots in the groundbreaking work that began in Dr. Doug Melton's lab, progressed at Semma Therapeutics, and has been accelerated and brought to the clinic by the team at Vertex," said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "Ours is the only approach that produces fully differentiated and fully functional insulin-secreting pancreatic islets. We are very pleased to have received FDA's Fast Track Designation, which facilitates the development and expedites the review of drugs that treat serious conditions and fill an unmet medical need. We continue to work with urgency to bring this innovative therapy to patients."

"It's a remarkable time for T1D research efforts worldwide, as this investigational treatment enters the clinic," said Camillo Ricordi, M.D., Professor of Surgery, Director of the Diabetes Research Institute (DRI) and the Cell Transplant Center at the University of Miami Miller School of Medicine, and Steering Committee Chair for the VX-880 clinical trial. "The field's experience with the limited cadaveric islet transplants available, where some patients have experienced prolonged insulin independence for years, provides important proof-of-concept for the potential of cell therapy to be transformative for patients living with T1D."

The first clinical trial sites at the University of Miami Health System, the University of Pennsylvania and Massachusetts General Hospital are open for enrollment, and additional sites will be activated this year. To learn more visit clinicaltrials.gov.

About VX-880

VX-880, formerly known as STx-02, is an investigational allogeneic human stem cell-derived islet cell therapy that is being evaluated for patients who have T1D with impaired hypoglycemic awareness and severe hypoglycemia. VX-880 has the potential to restore the body's ability to regulate glucose levels by restoring pancreatic islet cell function, including insulin production.

The VX-880 clinical trial will involve an infusion of fully differentiated, functional islet cells, as well as the chronic administration of concomitant immunosuppressive therapy, to protect the islet cells from immune rejection.

About the Phase 1/2 Clinical Trial

The clinical trial is a Phase 1/2, single-arm, open-label study in patients who have T1D with impaired hypoglycemic awareness and severe hypoglycemia. This will be a sequential, multi-part clinical trial to evaluate the safety and efficacy of different doses of VX-880. Approximately 17 patients will be enrolled in the clinical trial.

About Type 1 Diabetes

T1D results from the autoimmune destruction of insulin-producing islet cells in the pancreas, leading to loss of insulin production and impairment of blood glucose control. The absence of insulin leads to abnormalities in how the body processes nutrients, leading to high blood glucose levels. High blood glucose can lead to diabetic ketoacidosis and over time, to complications such as kidney disease/failure, eye disease (including vision loss), heart disease, stroke, nerve damage and even death.

Due to the limitations and complexities of insulin delivery systems, it can be difficult to achieve and maintain balance in glucose control in patients with T1D. Hypoglycemia often results because of the difficulty in balancing the different factors that impact glucose levels, including insulin, diet and exercise. Hypoglycemia remains a critical limiting factor in glycemic management, and severe hypoglycemia can cause loss of consciousness, coma, seizures, injury, and can be fatal. Over time, patients with T1D can

develop impaired awareness of hypoglycemia, meaning they are no longer able to perceive the early signs of a hypoglycemic event, which can be dangerous and result in life threatening events.

There are currently limited treatment options beyond insulin for the management of T1D.

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 pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of cell and genetic therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, statements made by Dr. Bastiano Sanna and Dr. Camillo Ricordi in this press release, statements regarding the development, plans and expectations for our T1D pipeline program, including our Phase 1/2 clinical trial in people with T1D, statements regarding patient enrollment, dosing and activation of new sites, statements regarding potential clinical trial results and anticipated benefits of VX-880, and our plans to provide further updates on our T1D pipeline program. 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 FDA may not approve our IND, that data from a limited number of patients may not be indicative of final clinical trial results, that data from the company's development programs may not support registration or further development due to safety, efficacy or other reasons, that the COVID-19 pandemic may impact the status or progress of our clinical trial of VX-880, and other risks listed under the heading “Risk Factors” in Vertex's most recent annual report filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. 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.

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