Vertex Presents Positive, Updated VX-880 Results From Ongoing Phase 1/2 Study in Type 1 Diabetes at the European Association for the Study of Diabetes 59th Annual Meeting

October 3, 2023

- All patients treated with VX-880 in Parts A and B have follow-up data beyond Day 90 and have demonstrated islet cell engraftment and glucose-responsive insulin production -

- All patients showed improvement across all measures of glucose control, including decreases in HbA1c, increases in blood glucose time-in-range, and reduction or elimination of insulin use -

- The two patients with at least 1 year of follow-up met the criteria for the primary endpoint of elimination of severe hypoglycemic events (SHEs) and HbA1c <7.0% -

- VX-880 was generally well tolerated -

- Part C concurrent dosing well underway -

BOSTON--(BUSINESS WIRE)--Oct. 3, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today presented longer-term data on patients dosed in Parts A and B of its Phase 1/2 clinical trial of VX-880, an investigational stem cell-derived, fully differentiated islet cell therapy in people with type 1 diabetes (T1D) with impaired hypoglycemic awareness and severe hypoglycemic events (SHEs). Prior to VX-880 treatment, all six patients enrolled had long-standing T1D with no endogenous insulin secretion, required an average of 34.0 units of insulin per day, and had a history of recurrent severe hypoglycemic events (SHEs) in the year prior to screening.

All patients in Part A and B now have more than 90 days of follow-up and have demonstrated islet cell engraftment and endogenous glucose-responsive insulin production on the Day 90 mixed-meal tolerance test (MMTT). All patients demonstrated improved glycemic control across all measures, including decreases in HbA1c, improved time-in-range on continuous insulin monitoring, and reduction or elimination of exogenous insulin use.

The two patients with at least 12 months of follow-up after VX-880 infusion, who were therefore evaluable for the study's primary efficacy endpoint, met the criteria for the primary endpoint of elimination of SHEs between Day 90 and Month 12 with an HbA1c <7.0%. The first patient achieved insulin independence at Day 270 through Month 24. This is a patient who has had T1D for nearly 42 years and prior to trial enrollment was on 34 units of daily exogenous insulin. The second patient achieved insulin independence at Day 180 through Month 12. This is a patient who has had T1D for 19 years and prior to trial enrollment was on 45.1 units of daily exogenous insulin. Starting at Month 15, this patient was started on four units of basal insulin daily, at the investigator's discretion. After the data cut-off date, a third patient achieved insulin independence at Day 180.

VX-880 has been generally well tolerated in all patients dosed to date. The majority of adverse events (AEs) were mild or moderate, and there were no serious AEs related to VX-880 treatment. As previously reported, one subject had SHEs in the perioperative period. There have been no other SHEs in the study.

"We continue to marvel at the impressive data from the VX-880 program as evidenced by the improvements in all treated patients across all glycemic measures," said Trevor Reichman, M.D., Department of Surgery, University of Toronto. "This represents an incredibly promising investigational therapy, one with far-reaching potential."

"These data are particularly meaningful in the context of our overall investigational T1D program, as these same VX-880 cells are the foundation for our VX-264 cells-plus-device program, and our hypoimmune islet cell program," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "We are moving with urgency to bring these potentially transformative therapies to patients who are waiting."

These data were presented during the European Association for the Study of Diabetes 59th Annual Meeting on October 3, 2023, in Hamburg, Germany as an oral presentation, "Glucose-Dependent Insulin Production and Insulin-Independence in Patients with Type 1 Diabetes Infused with Stem Cell-Derived, Fully Differentiated Islet Cells (VX-880)" (abstract/publication #449).

About Vertex T1D Programs in Clinical Development

About VX-880

VX-880 is an investigational allogeneic stem cell-derived, fully differentiated, insulin-producing islet cell therapy manufactured using proprietary technology. VX-880 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 glucose responsive insulin production. VX-880 is delivered by an infusion into the hepatic portal vein and requires chronic immunosuppressive therapy to protect the islet cells from immune rejection. The VX-880 trial has expanded to additional sites that are currently active and enrolling in the U.S., Canada, U.K., Germany, Norway, Switzerland, Italy, Netherlands, and France.

VX-880 was recently granted PRIME designation by the European Medicines Agency in March 2023, in addition to Fast Track Designation by the U.S. FDA in March 2021. PRIME designation is granted to innovative new therapies that have demonstrated the potential to significantly address an unmet medical need.

About the VX-880 Phase 1/2 Clinical Trial
The clinical trial is a Phase 1/2, multi-center, single-arm, open-label study in patients who have T1D with impaired hypoglycemic awareness and severe hypoglycemia. This study is designed as a sequential, multi-part clinical trial to evaluate the safety and efficacy of VX-880. Approximately 17 patients will be enrolled in the clinical trial. Enrollment in Part C of the study is ongoing and multiple patients have been dosed.

About VX-264
VX-264 is an investigational cell therapy in which allogeneic human stem cell-derived islets are encapsulated in a channel array device designed to shield the cells from the body's immune system. VX-264 is designed to be surgically implanted and is currently being evaluated for patients with T1D.

About the VX-264 Phase 1/2 Clinical Trial
The clinical trial is a Phase 1/2, single-arm, open-label study in patients who have T1D. This will be a sequential, multi-part clinical trial to evaluate the safety, tolerability, and efficacy of VX-264. Approximately 17 patients will be enrolled in the global clinical trial. Enrollment is ongoing in this study.

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 people with T1D. Current standards of care do not address the underlying causes of the disease, and there are limited treatment options beyond insulin for the management of T1D; there is currently no cure for diabetes.

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, mRNA, cell and genetic therapies (including gene editing) in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic 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 100 Best Companies to Work For. 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, as amended, including, without limitation, (i) statements by Carmen Bozic, M.D., and Trevor Reichman, M.D., in this press release, (ii) our plans, expectations for, and the potential benefits of VX-880 and VX-264, and (iii) our plans for dosing and enrollment of patients. 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 data from a limited number of patients may not be indicative of final clinical trial results, that data from the company's research and development programs may not support registration or further development of its compounds due to safety, efficacy, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report and subsequent quarterly reports 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, or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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