Vertex Announces Positive Results From Ongoing Phase 1/2 Study of VX-880 for the Treatment of Type 1 Diabetes Presented at the American Diabetes Association 84th Scientific Sessions

June 21, 2024

– All 12 patients who received the full dose of VX-880 as a single infusion demonstrated islet cell engraftment and glucose-responsive insulin production by Day 90 –

– All patients achieved ADA-recommended target HbA1c levels <7.0% and >70% time-in-range (70-180 mg/dL), and 11 of 12 patients reduced or eliminated use of exogenous insulin –

- 3 patients with at least 12 months of follow-up, and therefore evaluable, met the primary endpoint of elimination of severe hypoglycemic events (SHEs) with HbA1c <7.0%, and the secondary endpoint of insulin independence –

- Trial expanded to enroll ~37 participants as company progresses toward pivotal development –

BOSTON--(BUSINESS WIRE)--Jun. 21, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today presented new data from 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). These updated data on 12 patients who received the full dose as a single infusion in Parts B and C of the trial are consistent with previously reported positive results in the VX-880 trial and reinforce the transformative potential of this therapy.

At baseline, all patients in the study had undetectable fasting C-peptide (a marker of endogenous insulin secretion), a history of recurrent SHEs in the year prior to screening, and required an average of 39.3 (min, max; 19.8, 52.0) units of insulin per day. Following a single infusion of VX-880 at the full dose, all 12 patients demonstrated islet cell engraftment and glucose-responsive insulin production by Day 90. At the latest visit, all patients had improved glycemic control and achieved ADA-recommended targets for both HbA1c below 7.0% and time-in-range above 70% on continuous glucose monitoring. Nearly all participants (11 of 12) had a reduction or elimination of exogenous insulin use at their last visit. All patients had elimination of SHEs during the evaluation period (from Day 90 onward). Finally, all three patients who had at least one year of follow-up, and are therefore evaluable for the primary endpoint, met the primary endpoint of elimination of SHEs (from Day 90 after infusion) with HbA1c <7.0% and the secondary endpoint of insulin independence.

VX-880 has been generally well tolerated. The majority of adverse events (AEs) were mild or moderate, and there were no serious AEs related to VX-880 treatment. As previously reported, two patient deaths occurred, both unrelated to treatment with VX-880. The safety profile is generally consistent with the immunosuppressive regimen used in the study, the infusion procedure, and complications from long-standing diabetes.

“These remarkable data add to the growing body of evidence for VX-880 as a potentially curative therapy for T1D,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. “As we plan toward pivotal development, we are pleased to have secured regulatory approval to expand study enrollment and look forward to advancing this program for patients who have long awaited a transformative therapy.”

“The data we’ve seen to-date in this study are extremely exciting. Stem cell-derived islets regulate blood glucose control as well as natural human islets,” said Piotr Witkowski, M.D., Ph.D., Professor of Surgery and Director, Pancreatic, and Islet Transplant Program, University of Chicago Medicine, one of the investigators on the study, and a member of Vertex’s VX-880 Steering Committee. “The marked improvements seen across several key glycemic measures, the elimination of severe hypoglycemic episodes, and the reduction or total elimination of reliance on exogenous insulin have the potential to fundamentally change the treatment landscape for T1D and alleviate the significant burden this disease carries for patients.”

These data were presented during the American Diabetes Association 84th Scientific Sessions Conference on June 21, 2024, in Orlando, Florida at an invited talk, “Update on Clinical Trials Using Stem Cell Replacement,” as part of the joint ADA/IPITA symposium from 3:45 – 5:15 p.m. ET.

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, and Europe.

VX-880 has been granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations by the U.S. FDA, and PRIME designation by the European Medicines Agency.

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.

The original 17-patient study is fully enrolled. Fourteen patients have been dosed in Parts A, B, and C of the study, and the remaining patients will be dosed soon. Twelve patients received the full dose as a single infusion in Parts B and C.

At baseline, all 14 patients dosed in the study had undetectable fasting C-peptide, a history of recurrent SHEs in the year prior to screening, and required an average of 39.3 (min, max; 19.8, 52.0) units of insulin per day. Following an infusion of VX-880, all patients demonstrated islet cell engraftment and glucose-responsive insulin production by Day 90.

Based on the positive data shown to date, the study has been expanded to approximately 37 patients.

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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease, and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including acute and neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes, myotonic dystrophy type 1, and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America, and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 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 LinkedIn, Facebook, Instagram, YouTube, and Twitter/X.

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 Piotr Witkowski, M.D., Ph.D., in this press release, (ii) our plans, expectations for, and the potential benefits of VX-880, and (iii) our plans for expanding the 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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