Vertex Announces FDA Clearance of Investigational New Drug (IND) Application for VX-880, a Novel Cell Therapy for the Treatment of Type 1 Diabetes (T1D)

January 28, 2021

- Vertex will initiate a Phase 1/2 clinical trial in first half of 2021 -

- VX-880 is the first stem cell-derived therapy evaluating fully differentiated pancreatic islet cells for the treatment of T1D -

BOSTON--(BUSINESS WIRE)--Jan. 28, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has cleared the IND, enabling the company to proceed with initiating a clinical trial for VX-880, an investigational stem cell-derived, fully differentiated pancreatic islet cell therapy to treat T1D. Vertex plans to initiate a Phase 1/2 clinical trial in the first half of 2021 in patients who have T1D with impaired hypoglycemic awareness and severe hypoglycemia.

"As we celebrate the 100th anniversary of the discovery of insulin this year, we are excited to bring a first-in-class cell therapy to the clinic with the potential to meaningfully impact people living with T1D," said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "We look forward to getting our clinical program underway and testing our unique approach of replacing pancreatic islet cells, which are destroyed in people with type 1 diabetes, with our stem cell-derived fully differentiated insulin-producing pancreatic islet cells."

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 subjects 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 remains a critical limiting factor in glycemic management, and severe hypoglycemia can cause loss of consciousness, coma, seizures, injury, and can be fatal.

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 Bastiano Sanna, Ph.D., in this press release, statements regarding the development, plans and expectations for our T1D pipeline program, including our plans to initiate a Phase 1/2 clinical trial in people with T1D and expected timeline of our clinical trials, statements regarding patient enrollment and dosing, 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 trials, 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. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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