Vertex Announces FDA Clearance of Investigational New Drug Application for VX-264, a Novel Encapsulated Cell Therapy for the Treatment of Type 1 Diabetes

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– Vertex to initiate a Phase 1/2 clinical trial in the coming months –

– VX-264 is the second investigational program in Vertex's pipeline containing stem cell-derived, fully differentiated pancreatic islet cells for the treatment of type 1 diabetes –

– VX-264 program does not require immunosuppression –

BOSTON--(BUSINESS WIRE)--Mar. 9, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug Application (IND) for VX-264, a stem cell-derived, fully differentiated pancreatic islet cell therapy encapsulated into a Vertex-developed, immunoprotective device with the potential to treat type 1 diabetes (T1D). The VX-264 program does not require the use of immunosuppression, which may broaden the population of people with T1D that this investigational therapy could reach.

Vertex plans to initiate a Phase 1/2 clinical trial in the first half of 2023 to study the safety, tolerability and efficacy of VX-264 in patients with T1D. The company previously received approval from Health Canada on the Clinical Trial Application (CTA) for VX-264, and the Phase 1/2 trial is ongoing in Canada.

"VX-264 uses the same stem cell-derived pancreatic islet cells as our VX-880 program where we've already demonstrated proof-of-concept, with the addition of a proprietary immunoprotective device that allows us to eliminate the need for immunosuppression," said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "We are excited to see our second program in T1D advancing into the clinic and look forward to bringing transformative, if not curative, therapies to T1D patients who are waiting."

Vertex is pursuing multiple investigational approaches using stem cell-derived islets with the aim of replacing the insulin-producing islet cells that are destroyed in people with T1D. Vertex's first clinical investigational program in T1D, VX-880, is a stem cell-derived, fully differentiated, insulin-producing islet cell replacement therapy used in combination with immunosuppression. Vertex has demonstrated clinical proof-of-concept in the VX-880 program, and the Phase 1/2 clinical study is ongoing in the U.S. and Canada.

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.

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, 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.

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 in this press release, and statements regarding the expectations for the clinical trial and potential benefits of VX-264, including potential eligible patient population, the plans to initiate a Phase 1/2 clinical trial of VX-264, including trial design, enrollment plans and timing of initiation, and expectations and plans regarding our T1D programs generally. 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 results, that the trials may not be initiated or completed in the expected timeframe, or at all, that data from the company's development programs may not support registration or further development of its products due to safety, efficacy, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report filed with the Securities and Exchange Commission (SEC) and available through the company'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.

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