Vertex Presents Positive Data for Zimislecel in Type 1 Diabetes at the American Diabetes Association 85th Scientific Sessions

June 20, 2025

– Results from the study continue to demonstrate the transformative potential of zimislecel with consistent and durable patient benefit –

– All 12 patients with at least one year of follow-up who received a full dose of zimislecel as a single infusion achieved ADA-recommended target HbA1c levels <7% and >70% time-in-range (70-180 mg/dL), and 10/12 patients were insulin free –

– Data presented at ADA simultaneously published in the New England Journal of Medicine –

– Vertex to host investor webcast tonight, June 20, 2025, at 7:15 p.m. CT / 8:15 p.m. ET –

BOSTON--(BUSINESS WIRE)--Jun. 20, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced simultaneous presentation and publication of updated data from the Phase 1/2 portion of the Phase 1/2/3 FORWARD-101 clinical trial of zimislecel (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). The data were featured in an oral presentation at the American Diabetes Association (ADA) annual conference in Chicago as part of the symposium, “Innovation and Progress in Stem Cell-Derived Islet-Cell Replacement Therapy,” from 6:15-6:30 p.m. CT (abstract 2025-A-1921) and published online by the *New England Journal of Medicine*.

The data are from 12 patients who received the full dose of zimislecel as a single infusion and were followed for at least one year, as of October 2024. Results from the study to date continue to demonstrate the transformative potential of zimislecel with consistent and durable patient benefit with longer follow-up. All 12 participants:

- Demonstrated engraftment with glucose-responsive endogenous C-peptide production, which was durable through one year of follow-up.
- Achieved the ADA targets of HbA1c <7% and time in range of >70%.
- Were free of SHEs from day 90 onwards.
- Had a reduction in exogenous insulin use (mean reduction in daily insulin dose: 92%).
    - 10/12 (83%) no longer required exogenous insulin at Month 12.
- Achieved the Phase 1/2 primary endpoint of elimination of SHEs with HbA1c <7%.

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

“These data on the first fully differentiated, stem cell-derived, off-the-shelf islet cell therapy continue to be unprecedented. The magnitude, consistency and durability of the results from all 12 patients with more than one year of follow-up reinforce the transformative potential of zimislecel for people living with T1D complicated by severe hypoglycemia,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. “We are excited to complete enrollment and dosing in the Phase 1/2/3 Program and look forward to potential regulatory submissions next year.”

“It’s remarkable to see 12 out of 12 patients with baseline HbA1c above 7% and multiple severe hypoglycemic events reach consensus targets for glycemic control by both HbA1c and time in range as well as elimination of severe hypoglycemic events,” said Michael R. Rickels, M.D., M.S., Medical Director, Pancreatic Islet Cell Transplant Program, Willard and Rhoda Ware Professor in Diabetes and Metabolic Diseases in the Perelman School of Medicine at the University of Pennsylvania, and Presenting Author and Steering Committee Co-Chair for the zimislecel clinical program, and author on the *New England Journal of Medicine* paper. “As I think about my patients and the unmet need in the type 1 diabetes community, the results we’ve seen so far for restoring endogenous insulin secretion with a stem cell-derived islet therapy bring me hope and confidence for a transformative treatment option for individuals with type 1 diabetes in the not-so-distant future.”

About Type 1 Diabetes
T1D results from the autoimmune destruction of insulin-producing beta cells in pancreatic islets. Insulin deficiency results in hyperglycemia and can lead to acute life-threatening complications such as diabetic ketoacidosis.

People with T1D are reliant on lifelong treatment with exogenous insulin that requires careful monitoring of blood glucose levels. Even with the availability of advanced exogenous insulin delivery and glucose monitoring systems, people with T1D can have periods of very low and very high blood sugar levels. Exogenous insulin has a narrow therapeutic range and carries an inherent risk of causing low blood sugar levels or hypoglycemic events, which can potentially result in arrhythmias, seizures, coma and even death. Due to the limitations and complexities of exogenous insulin treatment, it can be difficult for people with T1D to achieve and maintain good glucose control. Exposure to prolonged periods of high blood glucose levels, or hyperglycemia, can lead to long-term complications such as nerve damage, kidney disease/failure, eye disease (including vision loss), cardiovascular disease, stroke and even death.

HbA1c is a measure of average blood glucose over the most recent ~2-3 months, and the consensus guidance is to maintain an HbA1c of <7% to reduce the risk of long-term complications; only ~1 in 4 people with T1D globally meet this clinical target. Current standards of care do not address the underlying cause of the disease and leave people with T1D susceptible to both hypo- and hyperglycemia and their associated morbidity and mortality. There is no cure for T1D.

About Zimislecel
Zimislecel (VX-880) is an investigational allogeneic stem cell-derived, fully differentiated, insulin-producing islet cell therapy manufactured using proprietary technology. Zimislecel is being evaluated for patients who have T1D with impaired hypoglycemic awareness and severe hypoglycemia. Zimislecel has the potential to restore the body's ability to regulate glucose levels by restoring pancreatic islet cell function, including glucose-responsive insulin production. Zimislecel is delivered by an infusion into the hepatic portal vein and requires chronic immunosuppressive therapy to protect the islet cells from immune rejection. The zimislecel trial has expanded to additional sites that are currently active and enrolling in the U.S., Canada and Europe.

Zimislecel has been granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. Food and Drug Administration, Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and has secured an Innovation Passport under the Innovative Licensing and Access Pathway (ILAP) from the UK Medicines and Healthcare products Regulatory Agency (MHRA). Zimislecel is investigational and has not been approved by health authorities globally.

About Vertex
Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. 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 neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1.

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 15 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 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 Michael R. Rickels, M.D., M.S., in this press release, (ii) plans, expectations for, and the potential benefits of zimislecel, (iii) expectations for the Phase 1/2/3 clinical trial for zimislecel, including expectations for the trial to complete enrollment and dosing, and (iv) plans for potential regulatory submissions next year. 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 potential medicines in a timely manner, or at all, due to safety, efficacy, that timelines for regulatory submissions may be longer than anticipated, 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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Investor Event and Webcast

Vertex will host an investor event on Friday, June 20, 2025, at 7:15 p.m. CT/8:15 p.m. ET, in Chicago, to discuss the positive zimislecel data in type 1 diabetes. A live webcast of the presentation and Q&A portions can be accessed through the Investor Relations section of Vertex's website at https://investors.vrtx.com/. An archived webcast will be available on the company's website.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250620485709/en/

Vertex Pharmaceuticals Incorporated

Investors:
InvestorInfo@vrtx.com

Media:
mediainfo@vrtx.com
or
International: +44 20 3204 5275

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