

Expanded FORWARD Trial Demonstrates Continued Potential for Stem Cell-Derived Islet Cell Therapy to Eliminate Need for Insulin for People with T1D

American Diabetes Association Symposium Shows Patients with Type 1 Diabetes Experienced Insulin Independence and Elimination of Severe Hypoglycemic Events with Novel Therapy

ORLANDO, FL. (JUNE 21, 2024) – Today, new data from the Phase 1/2 FORWARD clinical study of the VX-880 islet cell therapy was presented at a symposium at the American Diabetes Association® (ADA) 84th Scientific Sessions in Orlando, FL. The results demonstrated that VX-880 reduced or eliminated the need for insulin use in patients with type 1 diabetes (T1D), indicating that VX-880 stem cell-derived islets can restore physiological islet function and glycemic control.

Despite use of advanced diabetes technologies, a recent survey of individuals with type 1 diabetes revealed that [approximately 6% of people experience recurrent severe hypoglycemic events](#) and have impaired awareness of hypoglycemia. Hypoglycemia, known as low blood glucose, is common in individuals with type 1 diabetes. People with type 1 diabetes may develop impaired awareness of hypoglycemia over time, meaning they may not feel symptoms despite blood glucose readings falling below a level that may provoke symptoms. If left untreated, this can lead to severe hypoglycemic events (SHEs) which can present as confusion, coma, seizures, cardiovascular events, and even death. Currently, there are limited treatment options beyond exogenous insulin for the management of the disease, creating a substantial unmet medical need for those living with type 1 diabetes.

The phase 1/2, open-label three-part study enrolled adults with type 1 diabetes, impaired hypoglycemic awareness, and at least two SHEs in the year before screening. The study evaluated the use of VX-880, an investigational, allogeneic, stem cell-derived, fully differentiated insulin-producing islet cell therapy. Participants had an average age of approximately 44 years, mean HbA1c 7.8%, total daily insulin use of approximately 40 units per day, and had experienced between two and four SHEs in the year prior to screening, and all participants had undetectable C peptide at baseline - a sign that your body is producing insulin.

Participants who received a full dose of VX-880 as a single infusion demonstrated engraftment of islet cells and endogenous insulin (C-peptide) production, eliminated severe hypoglycemic events, and significantly improved glycemic control while simultaneously reducing or eliminating insulin use. All 12 participants achieved a reduction in HbA1c to <7.0% and a time in target range of >70%.

Of 10 participants who completed the Day 180 visit: seven are no longer using exogenous insulin and two had approximately 70% reduction in their daily insulin use. Notably, 100% of patients with greater than one year of follow-up met the criteria for the primary endpoint of eliminating SHEs with HbA1c <7.0% at month 12 and achieved the secondary endpoint of insulin independence. These data indicate that VX-880 stem cell-derived islets function like bona fide islets and have the potential to provide profound benefit to patients.

“This positive data adds to the growing body of evidence for VX-880’s potential to revolutionize the treatment of type 1 diabetes that would give patients an alternative solution other than exogenously administered insulin,” said Piotr Witkowski, MD, PhD, Professor of Surgery, Director, Pancreatic and Islet Transplant Program, University of Chicago, Chicago, IL, and one of the investigators conducting the study. “These findings will also support further evaluation of VX-880, and we hope to see this treatment become a pivotal development in type 1 diabetes care.”

The trial has been further expanded to enroll a total of approximately 37 participants in order to generate clinical data that will support the goal of making VX-880 available for people living with type 1 diabetes in the future.

Research presentation details:

Professor Witkowski will present the findings at the following symposium:

- Symposium - Joint ADA/IPITA Symposium
Clinical Beta-Cell Replacement Therapy Today
- Presented on Friday, June 21, 2024 at 3:45 PM EDT

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About the ADA’s Scientific Sessions

The ADA's 84th Scientific Sessions, the world's largest scientific meeting focused on diabetes research, prevention, and care, will be held in Orlando, FL on June 21-24.



84TH SCIENTIFIC SESSIONS
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More than 11,000 leading physicians, scientists, and health care professionals from around the world are expected to convene both in person and virtually to unveil cutting-edge research, treatment recommendations, and advances toward a cure for diabetes. Attendees will receive exclusive access to thousands of original research presentations and take part in provocative and engaging exchanges with leading diabetes experts. Join the Scientific Sessions conversation on social media using #ADAScientificSessions.

About the American Diabetes Association

The American Diabetes Association (ADA) is the nation's leading voluntary health organization fighting to bend the curve on the diabetes epidemic and help people living with diabetes thrive. For 83 years, the ADA has driven discovery and research to treat, manage, and prevent diabetes while working relentlessly for a cure. Through advocacy, program development, and education we aim to improve the quality of life for the over 136 million Americans living with diabetes or prediabetes. Diabetes has brought us together. What we do next will make us Connected for Life®. To learn more or to get involved, visit us at diabetes.org or call 1-800-DIABETES (1-800-342-2383). Join the fight with us on Facebook ([American Diabetes Association](https://www.facebook.com/AmericanDiabetesAssociation)), Spanish Facebook ([Asociación Americana de la Diabetes](https://www.facebook.com/AsociaciónAmericanaDeLaDiabetes)), LinkedIn ([American Diabetes Association](https://www.linkedin.com/company/american-diabetes-association)), Twitter ([@AmDiabetesAssn](https://twitter.com/AmDiabetesAssn)), and Instagram ([@AmDiabetesAssn](https://www.instagram.com/AmDiabetesAssn)).