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Novel Stem Cell-Derived Islet Cell Therapy Continues to Show Promise for Achieving Insulin Independence for Individuals with Type 1 Diabetes

Stem Cell-Derived Fully Differentiated Islet Cells Shown to Restore Endogenous Insulin Secretion and Improved Glycemic Control in People Living with Type 1 Diabetes

SAN DIEGO, Ca. (June 23, 2023) – Today, findings from the ongoing clinical trial of VX-880, a phase 1/2, multi-center, single-arm, open-label study in patients with type 1 diabetes (T1D) were presented, demonstrating the potential of stem cell-derived islet cell therapy as a future treatment option for patients with T1D. All patients treated with VX-880 had undetectable insulin secretion and a history of recurrent severe hypoglycemic events (SHE) in the year prior to treatment; following treatment, all six patients demonstrated restored insulin secretion, improved glycemic control, improved time-in-range, reduction or elimination of exogenous insulin usage and complete absence of SHEs in the post-Day 90 evaluation period. The results were presented at the 83rd Scientific Sessions of the American Diabetes Association® (ADA) in San Diego, CA.

The study is focused on adult T1D patients with impaired hypoglycemic awareness and severe hypoglycemia. Hypoglycemia, known as low blood glucose, is common in individuals with T1D and often occurs because of imbalances between insulin administered relative to an individual's requirements at a particular time that can be affected by a multitude of factors e.g., diet, sleep, stress, activity levels.¹ Over time, people with T1D may lose awareness of hypoglycemia, meaning they may not feel symptoms despite blood glucose readings falling below a level which normally may provoke symptoms, usually below 70 mg/dL. If left untreated, this can lead to severe hypoglycemic events which can present with loss of consciousness, coma, seizures or serious injury as such, these events can potentially be fatal. Current standards of care do not address the underlying causes of the disease, and there are limited treatment options beyond exogenous insulin for the management of T1D.

Two patients treated with VX-880 (one in Part A of the study who, per protocol, received half the target dose, and one in Part B who received the full target dose) were treated for at least 12 months of follow-up and were evaluable for the study's primary efficacy endpoint of elimination of SHEs between Day 90 and Month 12 with HbA1c of <7%.

Both patients treated for more than 12 months are insulin independent; Patient A1 had HbA1c of 5.3% at Month 21 (compared to 8.6% at baseline) Patient B1 had HbA1c of 6.0% at Month 12 (compared to 7.6% at baseline). This level of glucose control is highly unusual in T1D patients treated with exogenous insulin, with recent data indicating that only approximately 25% of

¹ <https://diabetes.org/healthy-living/medication-treatments/blood-glucose-testing-and-control/hypoglycemia>

people with T1D meet the recommended HbA1c target of 7.0%. In fact, both patients displayed HbA1c levels that are below the diagnostic threshold for diabetes (6.5%). Both patients also showed over 95% time-in-range, well above the ADA recommended target of 70% and substantial improvement over baseline.

The three additional patients in Part B, each administered the full target dose of VX-880 given as a single infusion, have had up to 90 days of follow-up and also have shown insulin production, reduction in HbA1c, improvements in time-in-range and reductions in daily insulin usage. Their trajectory is consistent with that observed in the two patients with more than one year of follow up at equivalent periods of follow-up after VX-880 infusion.

VX-880 has been generally safe and well tolerated in all patients dosed to date, with a safety profile consistent with immunosuppressant therapy and cadaveric islet cell transplantation. The majority of adverse events (AEs) were mild or moderate and there were no SAEs related to VX-880 treatment.

“These new findings demonstrate the potential of stem cell-derived islets as a future treatment for patients with type 1 diabetes, signaling a new era that could potentially remove the need for exogenously administered insulin to achieve glycemic control,” said Trevor W. Reichman, MD, PhD, Surgical Director of Pancreas and Islet Cell Transplantation, Ajmera Transplant Center at the University of Toronto. “We are hopeful that this first-of-its-kind research could be a gamechanger for the treatment of type 1 diabetes.”

As a result of these positive safety and efficacy data in Parts A and B, an independent data review committee has recommended moving to Part C of the trial, which allows for concurrent dosing of patients at the full target dose of VX-880.

The VX-880 trial has expanded to additional sites that are active and enrolling in Norway, Switzerland and the Netherlands.

Research presentation details:

Dr. Reichman presented the findings at the following sessions:

- Glucose-Dependent Insulin Production and Insulin-Independence in Type 1 Diabetes from Stem Cell-Derived, Fully Differentiated Islet Cells—Updated Data from the VX-880 Clinical Trial
- Presented on Friday, June 23, 2023 in San Diego, California at 3:50pm PT as an oral presentation. These data will also be presented as a poster presentation on Sunday, June 25 at 11:30am PT (poster #836-P)

About the ADA's Scientific Sessions

The ADA's 83rd Scientific Sessions, the world's largest scientific meeting focused on diabetes research, prevention, and care, will be held in San Diego, CA on June 23–26. More than 12,000

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 #ADA2023.

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 82 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 133 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)).

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