Further Clinical Evaluation of Stem Cell-Derived Islet Replacement Therapy (VC-02) Demonstrates Production of C-Peptide with Improvements in Glycemic Control Parameters in Multiple Patients with Type 1 Diabetes and Hypoglycemia Unawareness

Manasi Jaiman. *ViaCyte*

Manasi Jaiman[1], David Thompson[2], Melena Bellin[3], Bart Keymeulen[4], Evert Kroon[1], Mark Daniels[1], Timothy Kieffer[1], Daniel Pipeleer[4], Richard Wang[1], Howard Foyt[1]

[1]ViaCyte, San Diego, Calif., [2]The University of British Columbia, Vancouver, British Columbia, [3]University of Minnesota, Minneapolis, Minn., [4]Vrije Universiteit Brussel, Brussels, Belgium

VC-02 combination product (PEC‑Direct) is a clinical-stage regenerative medicine therapy being developed for patients with type 1 diabetes and hypoglycemia unawareness intended to restore homeostatic glycemic control. It is comprised of stem cell-derived pancreatic progenitors (PEC‑01) loaded into a macroencapsulation device that facilitates the in-growth of blood vessels into the device lumen, supporting the maturation and differentiation of the graft into hormone-positive islet cells comprising glucose‑responsive beta cells. Initial data from one patient (2021, Keymeulen) demonstrated clinically-relevant levels of stimulated C-peptide and corresponding improvements in glycemic control within six months after subcutaneous implantation. These improvements now include a time-in-range consistently >90% and a persistent HbA1C <7%. Enrollment of additional patients with negligible C‑peptide levels in the ongoing VC02‑101 study implanted with the same device configuration has now resulted in multiple patients demonstrating C-peptide levels >0.3 ng/mL. All patients are challenged with a mixed-meal tolerance test at baseline and post‑implantation to detect and monitor increases in plasma C‑peptide levels. In some cases, the increased C‑peptide levels are observed to occur as soon as two months post‑implant with subsequent increases resulting as the patients progress further into the treatment period as high as 0.4 ng/mL by five months with additional time points at longer periods of exposure pending. This progressive rise in beta cell function is consistent with preclinical findings showing time-dependent differentiation of the pancreatic progenitors into beta cells and subsequent increases in beta cell mass. The increases in plasma C‑peptide levels also correlate with trends toward improved glycemic control parameters. Relative to baseline levels, improvements are observed in time‑in‑range data captured from continuous glucose monitoring, as well as substantial decreases in HbA1C by as much as 1.5%. These positive trends also correlate with decreases in the amount of exogenous insulin required by as much as 70%. Importantly, these data and observations have been obtained at separate clinical sites involving different surgeons, indicating that the achievement of functional engraftment of PEC‑Direct is reproducible with proper product handling, surgical training, and patient compliance with an adjunctive immunosuppression regimen. These data provide further proof‑of‑concept that continued optimization of PEC‑Direct can result in a functional cure for T1D.

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