Diabetes Research Institute Launches Trial of Novel Investigational Treatment for Type 1 Diabetes

Researchers with the Diabetes Research Institute (DRI) at the University of Miami Miller School of Medicine and Vertex Pharmaceuticals Incorporated have initiated clinical trials for VX-880, a novel investigational cell therapy with the potential to restore normal glucose control in people suffering from type 1 diabetes with severe hypoglycemia and impaired hypoglycemic awareness. The University of Miami Health System was the first clinical site activated for this trial.

Dr. Camillo Ricordi

The program was granted Fast Track Designation by the U.S. Food and Drug Administration, which offers an expedited review process to accelerate treatments for urgent and unmet medical needs. It will evaluate the safety, tolerability and efficacy of VX-880 infusion in certain type 1 diabetes (T1D) patients. To be considered as a candidate for the trial, participants must have a clinical history of type 1 diabetes of at least five years, be 18-65 years of age, and have multiple episodes of severe hypoglycemia and impaired hypoglycemic awareness 12 months prior to enrollment.

“It’s a remarkable time for T1D research efforts worldwide as
this investigational treatment enters the clinic,” said Camillo Ricordi, M.D., professor of surgery, director of the Diabetes Research Institute and the Cell Transplant Center at the Miller School of Medicine, steering committee chair for the VX-880 clinical trial, and principal investigator at the UHealth clinical site. “The field’s experience with the limited cadaveric islet transplants available, where some patients have experienced prolonged insulin independence for years, provides important proof-of-concept for the potential of cell therapy to be transformative for patients living with T1D.”

The program’s groundbreaking work began in the lab of Doug Melton, Ph.D., who developed this line of insulin-producing pancreatic cells derived from stem cells. Vertex acquired Semma Therapeutics, the biotechnology company founded by Dr. Melton, in 2019.

Type 1 diabetes is an autoimmune disorder that occurs when the body’s immune system attacks its own insulin-producing islet cells. Once all the islets are destroyed, patients must take daily injections of insulin or use an insulin pump to replace the hormone. As one of the largest and most comprehensive research centers dedicated to curing diabetes, the Diabetes Research Institute is aggressively working to develop a biological cure by restoring natural insulin production and normalizing blood sugar levels without imposing other risks.

“I am grateful to the DRI team that will help with this unprecedented, first-in-human clinical trial,” said Dr. Ricordi. “Representing the DRI heart of our entire translational and clinical efforts: the cGMP Advanced Cell and Biologic Product Manufacturing Facility (Dr. Elina Linetsky,
Xiaojing Wang, Clarissa Lenero); the CCTP Clinical Cell Transplant Program (Dr. Rodolfo Alejandro, Dr. David Baidal, Dr. Raffaella Poggioli, Ana Alvarez); the regulatory team (Dr. Kemraj Hirani, Luis Roque, Burlett Masters); and Dr. Prasoon P. Mohan, the UHealth interventional radiologist who will infuse the cells.”

The University of Miami Health System was the first clinical site activated for this trial, which now also includes the University of Pennsylvania and Massachusetts General Hospital. The three clinical sites are open for enrollment, and additional sites will be activated this year. To learn more, visit clinicaltrials.gov. Patients can contact the DRI about the clinical trial by calling 305-243-5321, or emailing Ana Alvarez at axa383@med.miami.edu, Dr. Poggioli at rpgggioli@med.miami.edu, or Dr. Alejandro at ralejand@med.miami.edu.