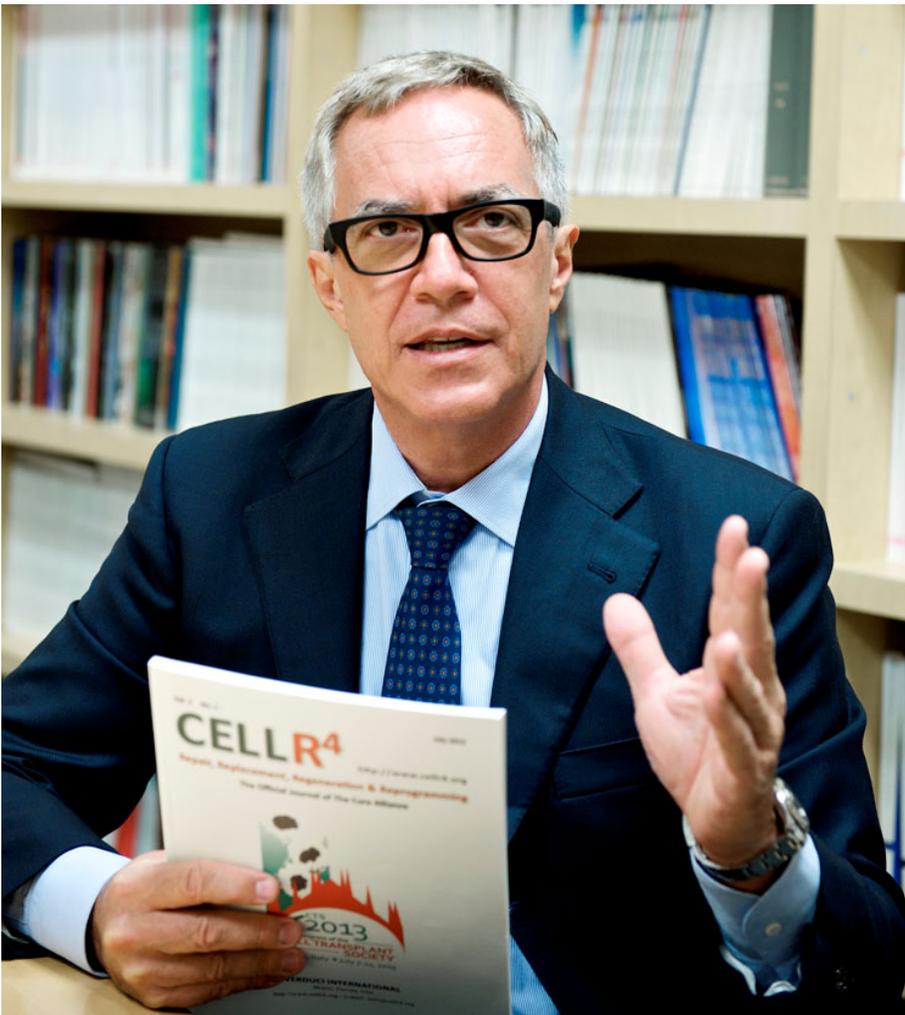


FDA Gives Orphan Status for Potential Type 1 'Brittle' Diabetes Therapy

While insulin does not cure type 1 diabetes, it helps many people maintain healthy blood sugar levels. However, for many people suffering from type 1 *brittle* diabetes, insulin just isn't enough. Fortunately, a new treatment may soon be available to help them: islet cell transplantation.



Camillo Ricordi, M.D.

Recently, the U.S. Food & Drug Administration (FDA) has given Orphan Drug Designation for islet cell transplants to the Diabetes Research Institute at the University of Miami Miller School of Medicine. This coveted designation could streamline the approval process and bring this advanced treatment to patients faster.

“It’s quite important because it allows us to proceed towards a

biological license application in a more rapid way,” said Camillo Ricordi, M.D., who directs the Diabetes Research Institute and the Cell Transplant Program. “This may allow the FDA to consider some kind of accelerated path for approval.”

People develop type 1 diabetes when their immune systems wrongly attack insulin-producing pancreatic islet cells. When those cells ultimately die off, patients must manage their blood sugar with injected insulin. However, people with brittle diabetes face even more severe consequences.

“Type 1 brittle diabetes is characterized by severe hypoglycemic (low blood sugar) episodes and unawareness,” said Dr. Ricordi, who is Stacy Joy Goodman Professor of Surgery, Distinguished Professor of Medicine, and professor of biomedical engineering, microbiology and immunology. “Patients cannot sense a hypoglycemic episode coming on. They don’t have the symptoms to warn them they are having low blood sugar and can lose consciousness, putting themselves and others at risk.”

Around 70,000 people in the U.S. with brittle diabetes have failed all treatments and are candidates for islet cell transplants. In some ways, this is an ideal treatment because it restores the 24/7 blood sugar monitoring and insulin delivery that only islet cells can provide.

However, transplantees face two separate immune system risks. First, the preexisting autoimmune response that caused their disease will continue to target islet cells. In addition, because these transplanted cells come from donors, the immune

system can perceive them as foreign attackers and reject them entirely. Transplant recipients must take powerful drugs to suppress their immune systems and guard against these risks.

However, Dr. Ricordi and team are testing better ways to control the immune response without fully suppressing it. This advance could eventually open up islet cell transplantation for more people with type 1 diabetes and other conditions.

“There is a new agent, a new monoclonal antibody, we are testing that blocks the activation of the cells that selectively want to attack the islets,” said Dr. Ricordi. “This could be a game changer for the whole field of transplantation.”