
Type 1 Diabetes Therapy Funded by Stem Cell Agency Submits Application to Start Clinical Trial

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San Francisco, CA – A promising treatment for type 1 diabetes that has received almost \$40 million in funding from the stem cell agency, the California Institute for Regenerative Medicine (CIRM), has taken a major step towards being tested in people.

ViaCyte Inc., the company behind the therapy, has submitted an Investigational New Drug application (IND) with the Food and Drug Administration (FDA) seeking permission to start a phase 1/2 clinical trial in patients with type 1 diabetes (T1D). The trial is designed to see if their implantable product, the VC-01™ product candidate, is safe and shows evidence of being efficacious for patients with the disease.

Viacyte's approach uses a thin plastic pouch, containing an immature form of pancreatic cells that, when implanted under the skin, are designed to mature and become insulin-producing and other cells needed to regulate blood glucose levels. These cells are able to sense when blood glucose is high, and then secrete insulin to restore it to a healthy level. In effect this is designed to mimic the glucose regulating functions of the pancreas, which, in people with T1D, no longer works. This approach was shown to be effective in extensive preclinical testing in models of the disease.

"This is good news for ViaCyte and is a clear sign of the progress they are making," says C. Randal Mills, Ph.D., the President and CEO of the stem cell agency. "Filing for an IND is a crucial step along the path to bringing a stem cell treatment to patients. CIRM will be working with them and supporting them every step of the way to try and make this happen as quickly, and as safely, as possible."

"The filing of this IND represents the culmination of many years of research and development by a dedicated team that is focused on transforming the way patients with type 1 diabetes manage this tremendously difficult disease," said Paul Laikind, Ph.D., president and CEO of ViaCyte. "The talented ViaCyte team has been assisted and supported by the California Institute for Regenerative Medicine (CIRM) a leading organization focused on advancing the field of stem cell-based technologies and JDRF, the leading research and advocacy organization focused on type 1 diabetes."

In the proposed clinical trial ViaCyte will implant the product candidate in people with T1D who have little or no insulin-production of their own. The first goal is to show that this approach is safe, and the second goal is to demonstrate that it can replace the glucose regulating role of the pancreas.

"We have been strong supporters of Viacyte for many years and it's great to see that they are well on the way to starting a First-in-Human trial, hopefully in the next few months," says Jonathan Thomas, J.D., Ph.D., the Chair of the agency's governing Board. "This therapy's growth from an idea to a potential treatment highlights CIRM's commitment to following promising science at all stages of development."

This work was also supported by funding from the JDRF.

About CIRM: CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided \$3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research. www.cirm.ca.gov

About ViaCyte: ViaCyte is a private regenerative medicine company currently focused on development of a novel cell therapy for the treatment of diabetes. The Company's lead product candidate, VC-01, is based on the production of pancreatic progenitors derived from human pluripotent stem cells. These cells are implanted in a durable and retrievable encapsulation device called the Encaptra® drug delivery system. Once implanted and matured, these cells are designed to secrete insulin and other regulatory factors in response to blood glucose levels. The Company is funded in part by the California Institute for Regenerative Medicine (CIRM) and JDRF. For more information please visit www.viacyte.com.

About JDRF: JDRF is the leading global organization focused on type 1 diabetes (T1D) research. JDRF's goal is to progressively remove the impact of T1D from people's lives until we achieve a world without T1D. JDRF collaborates with a wide spectrum of partners and is the only organization with the scientific resources, regulatory influence and a working plan to bring life-changing therapies from the lab to the community. As the largest charitable supporter of T1D research, JDRF is currently sponsoring \$568 million in charitable research in 17 countries. For more information, please visit www.jdrf.org.

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