Cell Therapy for Diabetes

Grant Award Details

Cell Therapy for Diabetes

Grant Type: Disease Team Research I

Grant Number: DR1-01423

Project Objective: File an allowable IND on a combination pancreatic progenitor cell/device therapy for a first-in–human (FIH) study in type 1 diabetics

Investigator:

Name:	Allan Robins
Institution:	ViaCyte, Inc.
Type:	PI

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Name:	Peter Stock
Institution:	University of California, San Francisco
Type:	Co-PI

Disease Focus: Diabetes, Metabolic Disorders

Collaborative Funder: JDRF

Human Stem Cell Use: Embryonic Stem Cell

Cell Line Generation: Embryonic Stem Cell

Award Value: $22,999,933

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Reporting Period: Year 3

View Report

Reporting Period: Year 4 + NCE

View Report

Grant Application Details

Application Title: Cell Therapy for Diabetes

Public Abstract: Diabetes exacts a tremendous toll on patients, their families, and society in general. Autoimmune Type 1 diabetes, often called juvenile-onset diabetes, is caused by a person's own immune system mistakenly destroying their insulin-producing cells in the pancreas, known as beta cells. When those beta cells are lost, the ability to produce insulin in response to food intake is lost, and blood sugar can increase to toxic levels. Although not due to autoimmunity, Type 2 diabetics often lose their ability to produce insulin as well. While pharmaceutical insulin is commonly used to control both types of diabetes, it does not sufficiently replace beta cells, and the adverse short- and long-term effects of diabetes remain, including dangerous episodes of low blood sugar, nerve damage, blindness, kidney damage, foot ulcers leading to amputations, and cardiovascular disease. Ideally, one would like to replace lost beta cells, and attempts to do so have included the use of pancreatic transplants, beta cell (islet) transplants, and transplants of animal cells or tissues. Unfortunately, those approaches are hindered by 1) the limited amount of donor tissue available, and 2) issues regarding immunological complications between donors and recipients. To solve the first problem, the Diabetes Disease Team applying for this CIRM award has developed methods to make replacement beta cells from human embryonic stem cells (hESC), which can be reliably grown in large-scale batches. The hESC-derived beta cells have been shown to cure experimental diabetes in mice and rats. Regarding the issue of donor-recipient compatibility, the Team has had initial success with several strategies, including administering the cells inside a simple device, implantable under the skin, as well as next-generation pharmaceuticals that enable transplantation between unmatched individuals without major side effects.

With the critical proof-of-concept milestones behind us, the Team now needs to perform all of the manufacturing and laboratory testing required to assure reliable production of a safe and effective product, thereby generating the data needed to seek FDA approval to test the product in humans. The project engages over 30 scientists and physicians, as well as numerous associates and technicians, whose expertise covers all of the critical areas from process development and manufacturing to clinical testing of novel biomedical products. The proposal includes active project management, and regulatory and ethical oversight. The Team has well defined time lines and milestones to advance the candidate product to an FDA submission. If successful, testing in diabetic patients could begin as early as 3 years from the project initiation.

Statement of Benefit to California:	Diabetes mellitus currently afflicts more than 250 million people worldwide, with projections of 380 million by the year 2030 (source: International Diabetes Federation). In 2007, there were an estimated 2.7 million Californians with diabetes (source: California Diabetes Program, California Department of Public Health). Further, the disease disproportionately affects certain minority groups and the elderly. Despite the use of insulin and advances in its delivery, the human cost of diabetes is underscored by the financial costs to society: tens of billions of dollars each year in California alone. The primary cause of Type 1 diabetes, and contributing significantly to Type 2 diabetes as well, is the loss of insulin-producing pancreatic beta cells. The proposed Disease Team will develop a beta cell replacement therapy for diabetes. If successful, the therapy will go beyond insulin function, and will perform the full array of normal beta cell functions, including responding in a more physiological manner than manual or mechanized insulin administration. Because they will be more physiological, the replacement cells should also reduce the long-term effects of diabetes. Moreover, the cell therapy will alleviate patients of the constant monitoring of blood glucose and painful insulin injections. For these reasons, it is possible that the product could transform the diabetes treatment landscape and replace pharmaceutical insulin in the market. This product will be available in California first, through clinical trials, and if approved by the FDA for commercial production, could eventually help hundreds of thousands of diabetic Californians. The product will substantially increase quality of life for diabetics and significantly reduce the health care burden in the state. The Team will employ various Californian physicians and scientists, and success of the Team will generate positive recognition for the state. Lastly, once commercially marketed, the product will yield additional jobs in manufacturing, sales, and related industries, and generate revenue for California. Given the market need and the clear feasibility, the product could become the most significant stem cell-based medical treatment of the coming decade, and that would be a great achievement for California, its taxpayers, and CIRM.