Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes

Grant Award Details

Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-10591

Project Objective: Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes.

Investigator:

Name:	Alan Agulnick
Institution:	ViaCyte, Inc.
Type:	PI

Disease Focus: Diabetes, Metabolic Disorders, Type 1 diabetes

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $1,470,987

Status: Closed

Progress Reports

Reporting Period: Year 2

View Report

Grant Application Details

Application Title: Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes

Public Abstract:

Research Objective

We will produce a universal donor cell (UDC) line by gene editing an embryonic stem cell line. Cell therapies produced from the UDC line will not be rejected by a patient's immune system.

Impact

The UDC line will address the bottleneck of patient immunity that is currently slowing development of many potential cell therapies. It will first be tested in a type 1 diabetes cell therapy.

Major Proposed Activities

- Produce banks of UDC that are of suitable quality for use in manufacturing therapeutic cells for clinical trials.
- Demonstrate that pancreatic cells produced from UDC and implanted into rodents can secrete insulin in response to glucose.
- Demonstrate that pancreatic cells produced from UDC evade immunity, i.e. are destroyed much less efficiently than the unmodified cells in immunological tests.
- Demonstrate function of a gene added into the UDC as a "safety switch". This safety gene causes implanted cells to die when a specific drug is taken and is a precautionary part of product development.

Statement of Benefit to California:

The universal donor stem cell line would firstly be used to help the thousands of Californians with insulin-requiring diabetes, but soon thereafter would be applied to other substantially unmet medical needs. Cell therapies have the potential to restore a relatively normal life to patients and their families, extend patients' lives, and dramatically reduce the state's health care burden. This would represent a tremendous achievement and asset for California, its taxpayers, and CIRM.

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