Development of a Cell Replacement Therapy Product for Insulin Dependent Diabetes

Grant Award Details

Development of a Cell Replacement Therapy Product for Insulin Dependent Diabetes

Grant Type: Disease Team Planning

Grant Number: DT1-00672

Investigator:

Name:	Emmanuel Baetge
Institution:	ViaCyte, Inc.
Type:	PI

Award Value: $48,950

Status: Closed

Grant Application Details

Application Title: Development of a Cell Replacement Therapy Product for Insulin Dependent Diabetes

Public Abstract:

With a current prevalence of greater than 170 million individuals world-wide, diabetes has attained epidemic proportions. The widespread secondary complications this disease extract a relentless and costly toll on the patients and health care establishment required for their treatment. To date, cellular replacement therapies for the treatment of diabetes has been performed either by transplantation of whole pancreas, or via infusion of isolated primary pancreatic islets into the portal vein . While effective, the availability of such procedures is severely limited for the treatment of the general diabetes population since it relies upon the extremely limited supply of organs from deceased donors.

One approach to overcoming the problem of insufficient organ and islet supply is to generate glucose responsive human insulin secreting pancreatic islet cells from stem cell populations. Only human embryonic stem cells (hES cells) demonstrate sufficient cell expansion capacity to achieve production levels needed to treat patients with diabetes. Furthermore, hES cells are capable of efficiently and rapidly progressing through a series of defined steps to generate most cell types found in the human body, but more importantly, pancreatic beta cells that release insulin in response to changing blood glucose levels. In a recent breakthrough, stem cell derived beta cells have been produced for the first time, providing confirmation that hES cells can serve as a realistic and renewable source of functional glucose responsive islet cells.

To achieve the goal of producing a safe product for clinical trials in patients with diabetes, the disease team must meet the following objectives: 1.) Identification and characterization of the clinical cell product; 2.) Development of methods to sufficiently purify the clinical cell product; 3.) Development of scale-up methodologies sufficient for achieving clinical trial product supplies; 4.) Design and completion of safety studies; 5.) Identification of implantation site for human clinical trials; 6.) Design of clinical trial; 7.) Development of product shipping and storage procedures; 8.) Development of FDA approved manufacturing process; 9.) FDA approved product facility construction and validation; 10) Submission of product and process documentation to the FDA sufficient to begin human clinical trials.

The disease team members critical to efficiently realizing these objectives include a consortium of scientists and managers who are currently fully focused on the realization of these goals. The team includes experienced stem cell researchers who have already developed glucose sensing pancreatic islet cells from hES cells. Together with these hES cell experts the team will include experts in the development, scale-up, manufacture and commercialization of cell-based therapies and on the safety and toxicology studies required for clinical trials with human cell therapy products.

Statement of Benefit to California:	With a current prevalence of greater than 170 million individuals world-wide, diabetes has attained epidemic proportions. The widespread secondary complications of kidney failure, cardiovascular disease, peripheral nerve disease, and severe retinopathies, this disease extracts a relentless and costly toll on the patients and the health care establishments required for their treatment. Current estimates are that California spends minimally $12 billion on diabetes not including lost wages. There are more than 300,000 diabetes related hospitalizations costing $3.4 billion annually. To date, cellular replacement has been performed either by transplantation of whole pancreas organs, or via infusion of isolated primary pancreatic islets into the portal vein . While effective, the availability of such procedures is severely limited for the treatment of the general diabetes population since it relies upon the extremely limited supply of pancreas organs from deceased donors and usually requires life-long administration of immuno-suppressive drugs. The Disease team to be assembled to develop a cellular therapy for diabetes, has the best stem cell research and product development experience available in California. The team is internationally recognized for their ground-breaking work in stem cell derived production of glucose responsive islet cells and in technologies for the development, scale-up, delivery, manufacture and commercialization of cell-based therapies. In addition the team includes experts in the design and implementation of the safety studies required for production of FDA approvable human cell therapy products. The work carried out by this diabetes disease team may ultimately lead to commercial manufacturing of these products in California and may spawn related support industries. The hope is that these novel stem cell based cellular therapies and technologies will lead to a reduction of the the massive health care burden this disease inflicts on the patients and their families in California.