Generation and characterization of high-quality, footprint-free human induced pluripotent stem cell lines from 3,000 donors to investigate multigenic diseases

Grant Award Details

Generation and characterization of high-quality, footprint-free human induced pluripotent stem cell lines from 3,000 donors to investigate multigenic diseases

Grant Type: hiPSC Derivation

Grant Number: ID1-06557

Project Objective: The objective of this project is to
- receive blood, skin or fibroblast samples form 3000 donors,
- reprogram them into hiPSC lines (p5 master banks) and characterize them,
- transfer master banks to Coriell for banking.

Investigator:

Name:	Thomas Novak
Institution:	Cellular Dynamics International
Type:	PI

Disease Focus: Alzheimer's Disease, Autism, Developmental Disorders, Genetic Disorder, Heart Disease, Infectious Disease, Liver Disease, Metabolic Disorders, Neurological Disorders, Respiratory Disorders, Vision Loss

Cell Line Generation: iPS Cell

Award Value: $16,000,000

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Reporting Period: Year 3

View Report

Reporting Period: NCE Year 4

View Report

Grant Application Details

Application Title: Generation and characterization of high-quality, footprint-free human induced pluripotent stem cell lines from 3,000 donors to investigate multigenic diseases

Public Abstract: Induced pluripotent stem cells (iPSCs) have the potential to differentiate to nearly any cells of the body, thereby providing a new paradigm for studying normal and aberrant biological networks in nearly all stages of development. Donor-specific iPSCs and differentiated cells made from them can be used for basic and applied research, for developing better disease models, and for regenerative medicine involving novel cell therapies and tissue engineering platforms. When iPSCs are derived from a disease-carrying donor; the iPSC-derived differentiated cells may show the same disease phenotype as the donor, producing a very valuable cell type as a disease model. To facilitate wider access to large numbers of iPSCs in order to develop cures for polygenic diseases, we will use a an episomal reprogramming system to produce 3 well-characterized iPSC lines from each of 3,000 selected donors. These donors may express traits related to Alzheimer's disease, autism spectrum disorders, autoimmune diseases, cardiovascular diseases, cerebral palsy, diabetes, or respiratory diseases. The footprint-free iPSCs will be derived from donor peripheral blood or skin biopsies. iPSCs made by this method have been thoroughly tested, routinely grown at large scale, and differentiated to produce cardiomyocytes, neurons, hepatocytes, and endothelial cells. The 9,000 iPSC lines developed in this proposal will be made widely available to stem cell researchers studying these often intractable diseases.

Statement of Benefit to California: Induced pluripotent stem cells (iPSCs) offer great promise to the large number of Californians suffering from often intractable polygenic diseases such as Alzheimer's disease, autism spectrum disorders, autoimmune and cardiovascular diseases, diabetes, and respiratory disease. iPSCs can be generated from numerous adult tissues, including blood or skin, in 4–5 weeks and then differentiated to almost any desired terminal cell type. When iPSCs are derived from a disease-carrying donor, the iPSC-derived differentiated cells may show the same disease phenotype as the donor. In these cases, the cells will be useful for understanding disease biology and for screening drug candidates, and California researchers will benefit from access to a large, genetically diverse iPSC bank. The goal of this project is to reprogram 3,000 tissue samples from patients who have been diagnosed with various complex diseases and from healthy controls. These tissue samples will be used to generate fully characterized, high-quality iPSC lines that will be banked and made readily available to researchers for basic and clinical research. These efforts will ultimately lead to better medicines and/or cellular therapies to treat afflicted Californians. As iPSC research progresses to commercial development and clinical applications, more and more California patients will benefit and a substantial number of new jobs will be created in the state.