Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs

Grant Award Details

Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-11070

Project Objective: To develop a candidate drug treatment.

Investigator:

Name:	Stuart Lipton
Institution:	Scripps Research Institute
Type:	PI

Disease Focus: Autism, Neurological Disorders

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: $1,827,576

Status: Active

Grant Application Details

Application Title: Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs

Public Abstract:

Research Objective

We will use human patient induced pluripotent stem cell (hiPSC)-based models to screen for a drug that activates a transcription factor critical to the treatment of Autism Spectrum Disorder (ASD).

Impact

Our goal is to develop a small molecule to treat Autism Spectrum Disorder (ASD), which currently affects 1/68 children born in the USA. Currently, there is no effective treatment.

Major Proposed Activities

- Assay Development for Drug Screening: Generate and characterize "disease-in-a-dish" models using hiPSCs generated from MEF2C Haploinsufficiency Syndrome patients, a form of ASD (month 1 - month 6).
- High-throughput Screening: Screen for hit-to-lead compounds that upregulate MEF2 activity by reporter-gene assay (month 3 - month 9).
- Efficacy Evaluation of Hits: Evaluate candidate therapeutics using ASD patient hiPSC-derived neurons (month 10 - month 18)
- Drug Optimization - 1) Perform additional SAR and optimization, and 2) Perform additional CNS permeability studies and initial PK (month 18 - month 24).
- Develop a Target Product Profile (month 21 - month 24). 1) Using the standard CIRM form, a TPP will be formulated for treatment of the MEF2C Haploinsufficiency Syndrome (MCHS) type of ASD.
- NA

Statement of Benefit to California:

Recent studies show that MEF2C activity not only affects MCHS but also other forms of ASD because MEF2C drives the activity of other ASD-related genes. Thus, while we are developing a treatment for the MCHS form of ASD, in fact MEF2 activator drugs may prove effective for a much large group of ASD patients. ASD is now reported to occur in 1 in every 68 births in both CA and the USA, so the benefit to the ASD community is potentially immense.

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