Drug Discovery for Dilated Cardiomyopathy using Patient-Derived Human iPSCs

Grant Award Details

Drug Discovery for Dilated Cardiomyopathy using Patient-Derived Human iPSCs

Grant Type: Foundation - Discovery Stage Research Projects

Grant Number: DISCo-13901

Project Objective: To develop and characterize an iPSC-based model of LMNA mutation-based dilated cardiomyopathy (LMNA-DCM), identify secreted factors involved in intercellular crosstalk (potential targets), computationally identify small molecules that modulate targets and validate hits for safety and efficacy in in vitro LMNA-DCM model.

Investigator:

<table>
<thead>
<tr>
<th>Name</th>
<th>Syed Ahmed</th>
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<tr>
<td>Institution</td>
<td>Greenstone Biosciences</td>
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<td>Type</td>
<td>PI</td>
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Disease Focus: Heart Disease

Human Stem Cell Use: iPS Cell

Award Value: $1,350,000

Status: Pre-Active

Grant Application Details

Application Title: Drug Discovery for Dilated Cardiomyopathy using Patient-Derived Human iPSCs
Public Abstract: Research Objective

Greenstone Biosciences will use patient-derived stem cells to discover safe and effective drugs for cardiovascular disease.

Impact

The use of patient-derived stem cells to discover novel drug targets for cardiovascular disease, identify drug candidates, and establish clinical trial in a dish to evaluate drug safety and efficacy.

Major Proposed Activities

- Use patient-derived induced pluripotent stem cells (iPSCs) to generate different cardiac cell types.
- Identify drug targets that cause cardiovascular disease using iPSC-derived cardiac cell types.
- Identify drug candidates from doing a virtual screening of chemicals.
- Evaluate and modify drug candidates to improve safety and/or efficacy for humans.
- Validate drug candidate for safety and efficacy in patient-derived human iPSC cell models.
- Validate drug candidate for safety and efficacy in structurally complex 3D cardiac organoids.

Statement of Benefit to California:

Dilated cardiomyopathy affects ~1 in 2,500 people and often leads to heart failure. The discovery of novel drugs that are safe and effective will be life-saving for many worldwide including California citizens. Greenstone Biosciences is also based in California (Stanford Research Park, Palo Alto, CA) and have actively recruited patients from California to generate induced pluripotent stem cells. We also collaborate with Stanford University and California-based companies (e.g., Eclipsebio).