
Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

Grant Award Details

Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-11131

Project Objective: Identify a therapeutic candidate for Danon Disease comprising autologous, HSPCs transduced with lentivirus containing the human LAMP2 cDNA, and to demonstrate disease-modifying activity in LAMP2 KO mice with analogous murine product

Investigator:

Name:	Eric Adler
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Danon Disease, Heart Disease, Metabolic Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: \$1,393,200

Status: Closed

Progress Reports

Reporting Period: Year 3-NCE

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Grant Application Details

Application Title: Genetically Modified Hematopoietic Stem Cells for the Treatment of Danon Disease

Public Abstract:**Research Objective**

We propose to discover a novel, genetically modified hematopoietic stem cell based treatment for Danon disease, a rare lysosomal storage disease

that affects the heart.

Impact

As the only existing treatment for Danon disease is cardiac transplant, this therapy would significantly meet an unmet need. It also may help many other similar diseases.

Major Proposed Activities

- Generation of ex vivo Genetically Modified Human HSPC Product (Month 1-6)
- Functional Characterization of ex vivo Genetically Modified Human HSPC Product
- Generation of Analogous Murine Product
- In vivo Efficacy Evaluation of Analogous Murine Product in the Mouse Model of Danon Disease
- Elucidate Purported Mechanism of Action

Statement of Benefit to California:

Danon disease is a fatal disease without cure, therefore the cellular treatment we plan to develop could directly benefit the citizens of California. Our findings may assist in the development of new treatments for other cardiac diseases. Thus the work also has the potential to help Californians who suffer from similar cardiac conditions. This project utilizes CA scientists and laboratories. With success, it will generate additional research and employment opportunities for CA citizens.

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