
Process development for establishing an iPSC-based therapeutic candidate for Canavan disease

Grant Award Details

Process development for establishing an iPSC-based therapeutic candidate for Canavan disease

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-08525

Project Objective: Pre-IND meeting and readiness for manufacturing.

Investigator:

Name:	Yanhong Shi
Institution:	City of Hope, Beckman Research Institute
Type:	PI

Disease Focus: Canavan Disease, Neurological Disorders

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$7,377,384

Status: Active

Grant Application Details

Application Title: Process development for establishing an iPSC-based therapeutic candidate for Canavan disease

Public Abstract:**Translational Candidate**

Genetically-corrected patient iPSC-derived neural progenitor cells, which have demonstrated efficacy to correct disease phenotype in a CD mouse model.

Area of Impact

This candidate has the potential to develop into a therapy for Canavan disease, a fatal neurological disease that has no cure or standard treatment.

Mechanism of Action

The proposed candidate is intended to correct disease phenotype through a cell replacement approach. Moreover, the derivative of the genetically-corrected iPSCs will provide ASPA enzymatic activity, which is deficient in Canavan disease patients. The ASPA enzyme will be able to reduce NAA level, which accumulates to a toxic level in patient brains to cause sponge degeneration.

Unmet Medical Need

There is neither cure nor a standard course of treatment for Canavan disease. The therapeutic candidate we propose to develop in this study has the potential to lead toward the development of a cell replacement therapy for this disease.

Project Objective

Pre-IND meeting and readiness for manufacturing.

Major Proposed Activities

- Establishing a cGMP-compatible process in order to transfer the therapeutic candidate to manufacturing.
- Determining the in vivo efficacy and safety of the therapeutic candidate prepared using the cGMP-compatible process in CD mice.
- Preparing and conducting a pre-IND meeting with the FDA.

Statement of Benefit to California:

California is estimated to have ~12% of all cases of Canavan disease in the U.S. Besides the emotional and physical pain this disease inflicts on families, it produces a medical and fiscal burden in California that is larger than any other states. The proposed therapeutic candidate will represent great potential for both California patients and industry. It would also help to maintain California's leading position in clinical developments by creating safe and effective cell replacement therapy.

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