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**Development of a new therapeutic for directing target specific stem cell migration and treatment**

**Grant Award Details**

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Development of a new therapeutic for directing target specific stem cell migration and treatment

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-12666

**Project Objective:** Characterize CXCR4 agonist SDV1a and test the combination of HFB2050 hNSCs and SDV1a in a mouse model of ALS for therapeutic efficacy.

**Investigator:**

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|---------------------|-------------------------------------|
| <b>Name:</b>        | Ziwei Huang                         |
| <b>Institution:</b> | University of California, San Diego |
| <b>Type:</b>        | PI                                  |

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**Disease Focus:** Amyotrophic Lateral Sclerosis, Neurological Disorders

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$1,129,512

**Status:** Active

**Grant Application Details**

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**Application Title:** Development of a new therapeutic for directing target specific stem cell migration and treatment

**Public Abstract:****Research Objective**

A drug-stem cell combination therapy wherein the drug will direct and promote the delivery and distribution of stem cells to the disease site for the optimal therapeutic effect of the stem cells

**Impact**

Amyotrophic lateral sclerosis (ALS) and the way to deliver and enhance stem cell-based treatment of ALS

**Major Proposed Activities**

- Complete the additional in vitro studies and initiate the in vivo studies in SOD1 mouse model
- Determine whether the combined effect of hNSCs intraparenchymally augmented/guided by SDV1a has a synergistic effect on improving disease onset/progression & symptom-free survival in the SOD1 mouse
- Establish the preliminary toxicity and pharmacokinetics profiles of SDV1a in mouse model
- Elucidation of structure and other characteristics; development and validation of analytical procedures
- Process development and characterization in lab scale, stability study

**Statement of Benefit to California:**

This new therapeutic will address a significant unmet medical need in the treatment of amyotrophic lateral sclerosis (ALS) and have important benefits to the patients with ALS and impact on the healthcare and bio industry in California.

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