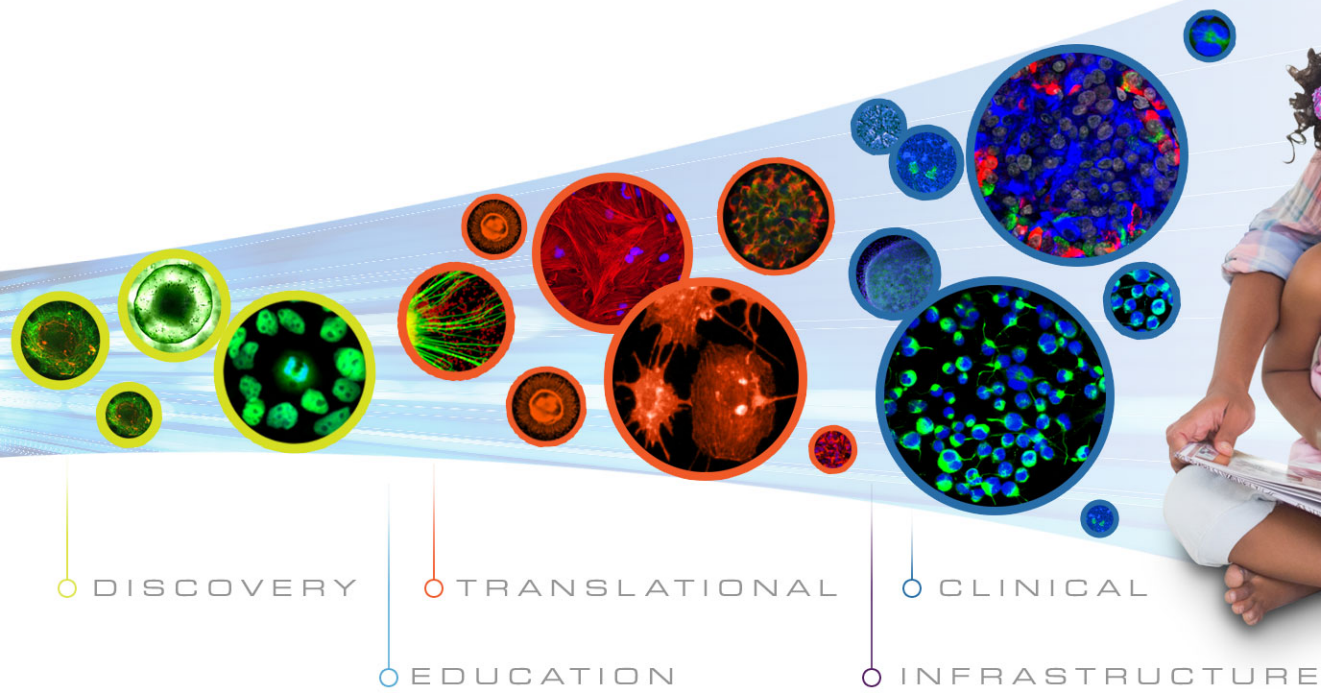


# CIRM

CALIFORNIA'S STEM CELL AGENCY



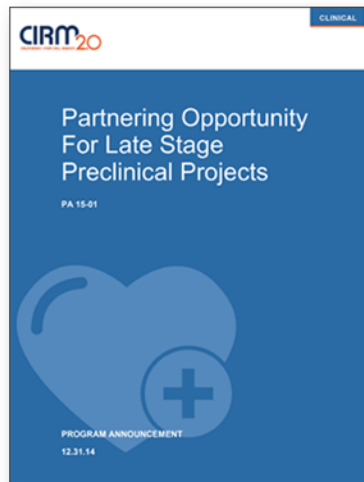
## GWG Review of Clinical Program Applications

**Gil Sambrano**

Director of Portfolio Development and Review

# Clinical Stage Programs

## CLINICAL STAGE



PA15-01



PA15-02



PA15-03

# New Scoring System for 2.0 Applications

- Score of “1”  
*Exceptional merit and warrants funding.*
- Score of “2”  
*Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.*
- Score of “3”  
*Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted.*

Applications are scored by all scientific members of the GWG with no conflict.

# LSP1-08309: Preclinical Development of a Small Molecule for Osteoarthritis

**Therapy:** Small molecule drug that promotes cartilage resident mesenchymal stem cell differentiation into chondrocytes.

**Indication:** Osteoarthritis and cartilage injury

**Goal:** Complete IND-enabling studies supporting the safety and efficacy of the candidate for clinical study and file an IND application with FDA.

## **Major Proposed Activities:**

- GLP toxicology and preclinical safety studies.
- Non-GLP studies to determine maximum tolerated dose.
- Prepare IND document and file.

**Funds Requested:** \$1,667,832

# LSP1-08309: Preclinical Development of a Small Molecule for Osteoarthritis

**Budget Review:** Pass

**GWG Score:** 1 exceptional merit and warrants funding

- Votes for score of 1: **12**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

**CIRM Team Recommendation:** Fund (concur with GWG recommendation)

**Award Amount:** \$1,667,832

# LSP1-08292: Preclinical Development of Adipocyte Stem Cells for Liver Regeneration

**Therapy:** Adipocyte stem cell-derived hepatocyte-like Cells

**Indication:** Acute alcohol-induced liver failure without pre-existing cirrhosis

**Goal:** Complete IND-enabling studies supporting the safety and efficacy of the candidate for clinical study and file an IND application with FDA.

## Major Proposed Activities:

- Develop a method for monitoring the function of transplanted hepatocytes in patients.
- Demonstrate the efficacy and safety of induced hepatocytes for liver regeneration in preclinical model.
- Develop method for production of adipose-derived hepatocyte-like cells.

**Funds Requested:** \$2,990,293

# LSP1-08292: Preclinical Development of Adipocyte Stem Cells for Liver Regeneration

**Budget Review:** Pass

**GWG Score: 3** sufficiently flawed that it does not warrant funding and the same project may not be resubmitted

- Votes for score of 1: **0**
- Votes for score of 2: **0**
- Votes for score of 3: **13**

**CIRM Team Recommendation:** Do not fund and do not allow resubmission (concur with GWG recommendation)