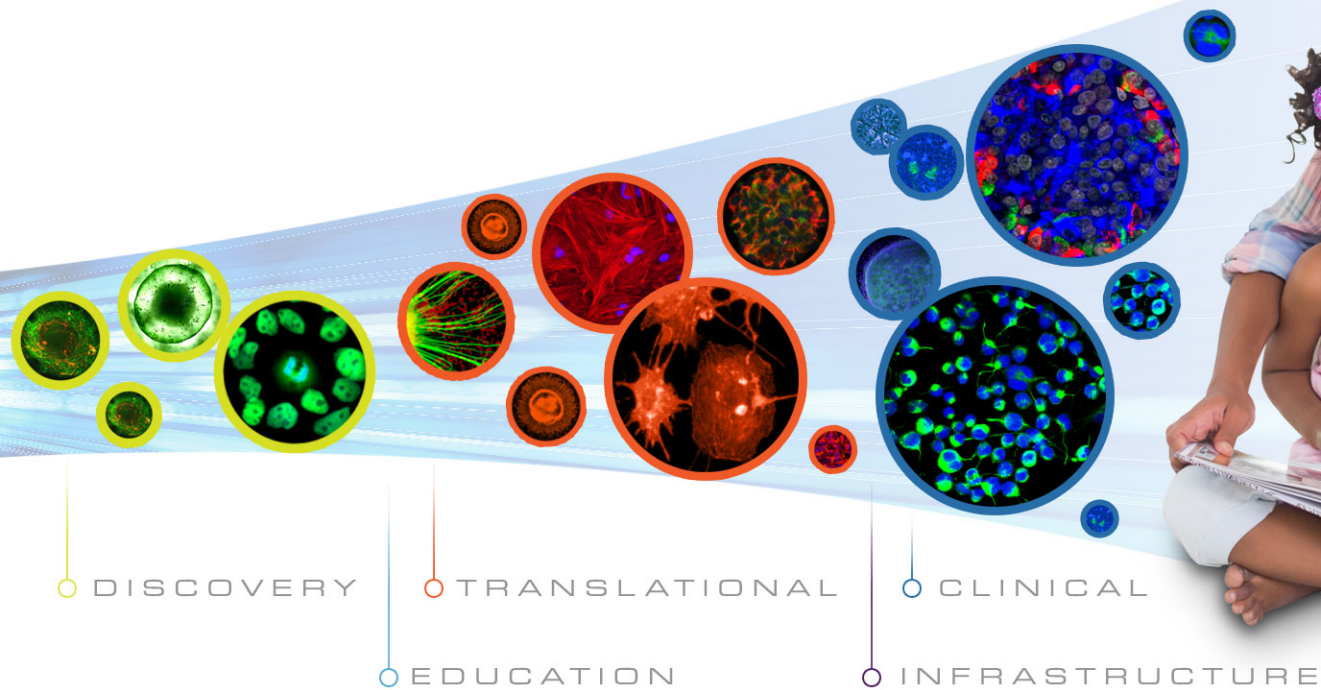


May 21, 2015

# CIRM

CALIFORNIA'S STEM CELL AGENCY



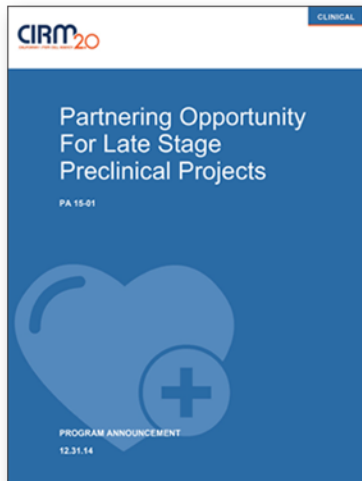
## GWG Review of Clinical Program Applications

**Gil Sambrano**

Director of Portfolio Development and Review

# Clinical Stage Programs

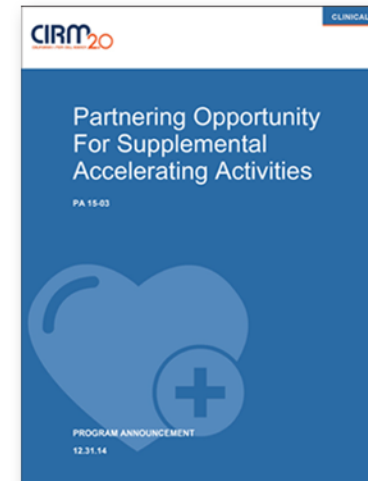
## CLINICAL STAGE



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.

# LSP1-08235: Preclinical Study for Retinitis Pigmentosa

**Therapy:** Human neural progenitor cells

**Goal:** To stabilize disease progression and maintain ocular integrity and vision for retinitis pigmentosa (RP) patients

## **Major Proposed Activities:**

- Produce sufficient NPCs for all preclinical studies and a Phase 1/2a clinical trial.
- Complete IND-enabling preclinical studies to understand dosing, scale-up, toxicity, and tumorigenicity.
- Obtain FDA regulatory approval to commence clinical trial with NPC in RP subjects.

**Funds Requested:** \$4,954,514

# LSP1-08235: Retinitis Pigmentosa

**Budget Review:** Pass

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

- Votes for score of 1: **15**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

**CIRM Team Recommendation:** concur with GWG recommendation

**Award Amount:** \$4,954,514

# CTS1-08239: Phase 3 Clinical Trial for Metastatic Melanoma

**Therapy:** Tumor stem cell-targeted immunotherapy

**Goal:** To demonstrate efficacy of a dendritic cell immunotherapy for patients metastatic melanoma

## **Major Proposed Activities:**

- Completion of a Phase 3 pivotal trial conducted under SPA agreement with FDA.
- Manufacturing of tumor stem cell-targeted immunotherapy.
- Assessment of clinical safety and efficacy of the therapeutic candidate.

**Funds Requested:** \$17,731,554



# CTS1-08239: Metastatic Melanoma

**Budget Review:** Pass

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

- Votes for score of 1: **6**
- Votes for score of 2: **3**
- Votes for score of 3: **5**

**CIRM Team Recommendation:** concur with GWG recommendation

**Award Amount:** \$17,731,554