

# CURE

Beyond  
**CIRM** 2.0  
CALIFORNIA'S STEM CELL AGENCY

*now it's personal*

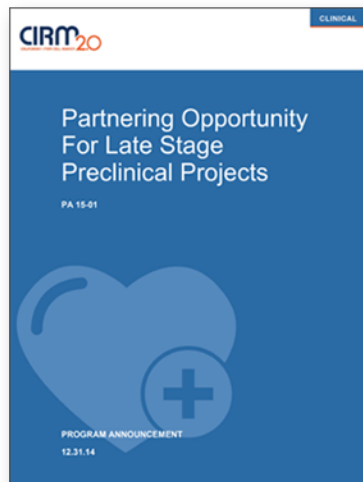


**Gil Sambrano, Ph.D.**

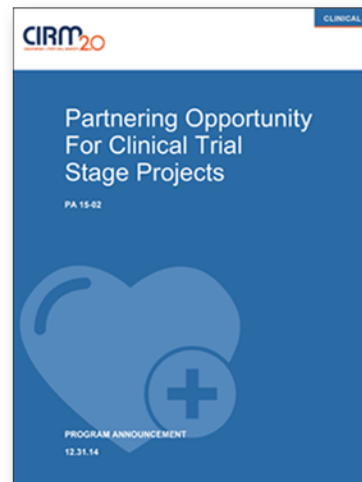
Vice President Portfolio Development  
and Review

# HOPE

# Clinical Stage Programs



CLIN 1



CLIN 2



CLIN 3

# GWG Vote on Review Process

1. *All members*: “The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG.”
2. *Patient advocate members*: “The review was carried out in a fair manner and was free from undue bias.”

Vote was unanimously in favor on both statements for all applications reviewed in this cycle.

# 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 **for at least 6 months.***

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

# CLIN2-09284: Clinical Trial of a Cell Therapy for ALS



**Therapy:** Neural progenitor cells secreting GDNF

**Indication:** Amyotrophic Lateral Sclerosis (ALS)

**Goal:** Complete a phase 1/2a clinical trial to test safety in patients with ALS

## **Major Proposed Activities:**

- Enroll patients into clinical trial
- Assess safety of the therapeutic product

**Funds Requested:** \$6,154,067(\$0 Co-funding)

# CLIN2-09284: Clinical Trial of a Cell Therapy for ALS



**Budget Review:** Pass

**GWG Score:** 1 Exceptional merit, warrants funding

- Votes for score of 1: **7**
- Votes for score of 2: **3**
- Votes for score of 3: **0**

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

**Award Amount:** \$6,154,067\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's assessment of allowable costs and activities.

# CLIN1-09472: Pre-Clinical Development of a Cell Therapy for Knee Osteoarthritis



**Therapy:** Adipose-derived mesenchymal progenitor cells

**Indication:** Knee osteoarthritis

**Goal:** Complete preclinical activities to file an IND for testing the product in a clinical trial

## **Major Proposed Activities:**

- Manufacture product to supply the proposed trial
- Complete non-clinical safety study requested by FDA
- Prepare and file IND

**Funds Requested:** \$2,291,976 (\$572,994 Co-funding)

# CLIN1-09472: Pre-Clinical Development of a Cell Therapy for Knee Osteoarthritis



**Budget Review:** Pass

**GWG Score:** 1 Exceptional merit, warrants funding

- Votes for score of 1: **10**
- Votes for score of 2: **1**
- Votes for score of 3: **1**

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

**Award Amount:** \$2,291,976\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's assessment of allowable costs and activities.



# CLIN2-09504: Clinical Trial of a Cell Therapy for X-linked Severe Combined Immunodeficiency



**Therapy:** Gene-corrected autologous bone marrow stem cells

**Indication:** X-linked severe combined immunodeficiency (XSCID)

**Goal:** Complete a phase 1/2a clinical trial to test safety and initial efficacy in patients with XSCID

## **Major Proposed Activities:**

- Open trial in California partner institution
- Enroll patients
- Analyze immune reconstitution and safety in XSCID patients

**Funds Requested:** \$11,924,780(\$0 Co-funding)

# CLIN2-09504: Clinical Trial of a Cell Therapy for X-linked Severe Combined Immunodeficiency



**Budget Review:** Pass

**GWG Score:** 1 Exceptional merit, warrants funding

- Votes for score of 1: **10**
- Votes for score of 2: **2**
- Votes for score of 3: **0**

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

**Award Amount:** \$11,924,780\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's assessment of allowable costs and activities.

# CLIN2-09730: Clinical Trial of a Cell Therapy for Type 1 Diabetes Mellitus



**Therapy:** Autologous Ex Vivo Expanded Regulatory T-Cells

**Indication:** Early onset Type 1 diabetes mellitus (T1D)

**Goal:** Complete a phase 2 clinical trial to test safety and efficacy of product in adolescents with T1D

## **Major Proposed Activities:**

- Enroll and treat 92 subjects in phase 2 trial
- Manufacture product for the 92 subjects

**Funds Requested:** \$12,211,255 (\$8,140,837 Co-funding)

# CLIN2-09730: Clinical Trial of a Cell Therapy for Type 1 Diabetes Mellitus



**Budget Review:** Pass

**GWG Score:** 1 Exceptional merit, warrants funding

- Votes for score of 1: **11**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

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

**Award Amount:** \$12,211,255\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's assessment of allowable costs and activities.

# CLIN1-09759: Pre-Clinical Development of a Cell Therapy for Parkinson's Disease



**Therapy:** hESC-derive neural progenitors expressing MEF2C

**Indication:** Moderate to severe Parkinson's disease

**Goal:** Complete preclinical activities to file an IND for testing the cell therapy product in a clinical trial

## **Major Proposed Activities:**

- Manufacturing of cGMP qualified cell bank for GLP studies
- Preclinical dose-response efficacy and toxicity/safety studies
- Prepare and file IND

**Funds Requested:** \$4,817,184 (\$0 Co-funding)

# CLIN1-09759: Pre-Clinical Development of a Cell Therapy for Parkinson's Disease



**Budget Review: Pass**

**GWG Score: 3** Does **not** warrant funding and should not be resubmitted for at least 6 months

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

**CIRM Team Recommendation:** Do not fund (concur with GWG recommendation)