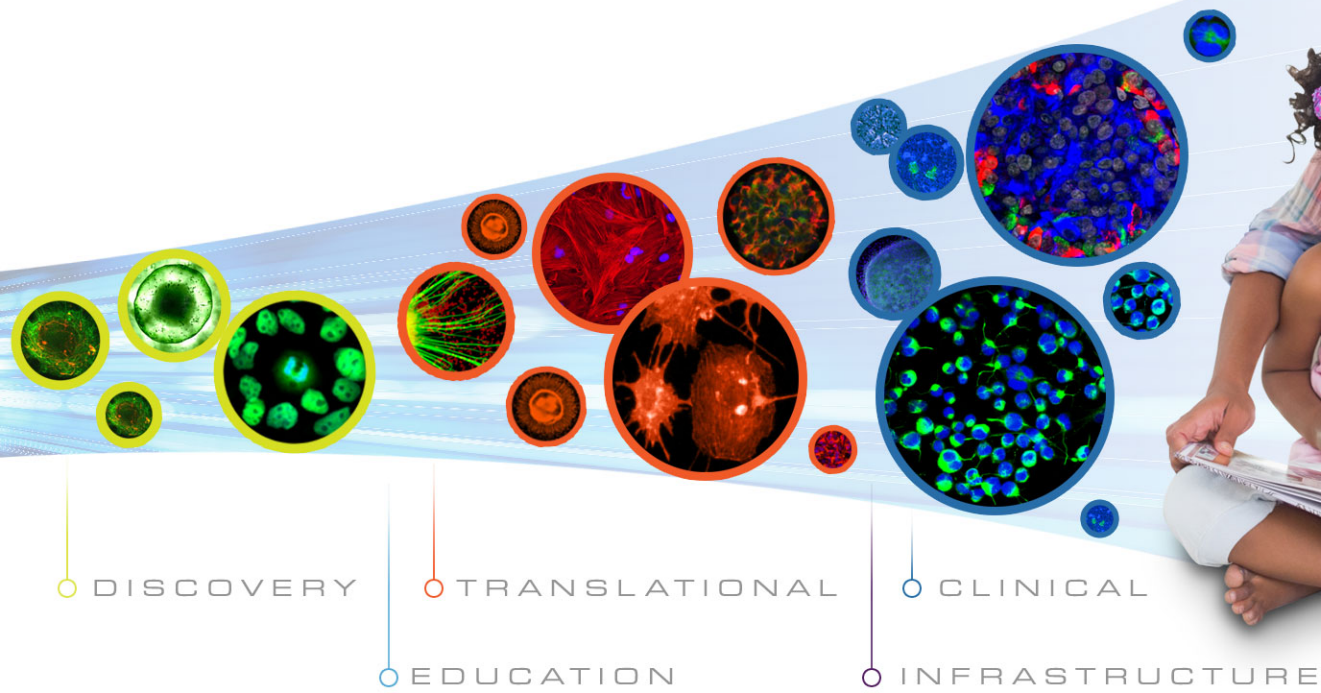


# CIRM

CALIFORNIA'S STEM CELL AGENCY



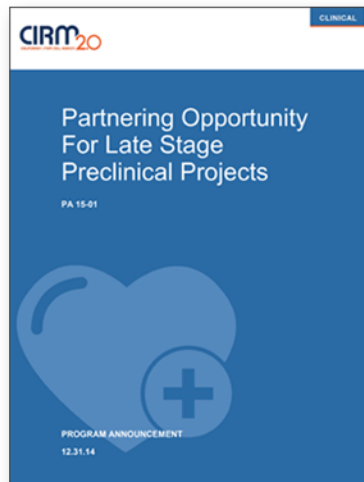
## GWG Review of Clinical Program Applications

**Gil Sambrano**

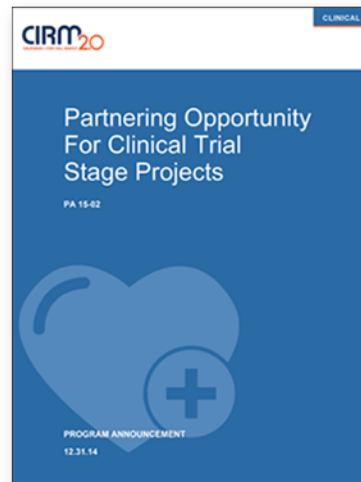
Director of Portfolio Development and  
Review

# Clinical Stage Programs

## CLINICAL STAGE



CLIN 1



CLIN 2



CLIN 3

# 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.

# CLIN2-09339: Clinical Trial to Treat a Severe Combined Immunodeficiency

**Therapy:** Autologous CD34+ hematopoietic stem cells (HSCs) with vector encoding ADA gene

**Indication:** Adenosine Deaminase – Severe Combined Immunodeficiency (ADA-SCID)

**Goal:** Complete a phase 1/2 clinical trial and seek FDA approval for treatment

## **Major Proposed Activities:**

- GMP manufacture of 10 patient specific lots of HSC and transplant 10 subjects with ADA-SCID
- Submit a biologics license application (BLA) to FDA
- Complete two-year follow up in treated patients

**Funds Requested:** \$20,000,000 (\$18,151,530 Co-funding)

# CLIN2-09339: Clinical Trial to Treat a Severe Combined Immunodeficiency

**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: **0**

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

**Award Amount:** \$20,000,000\*

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

# CLIN2-09444: Clinical Trial to Treat Pulmonary Arterial Hypertension

**Therapy:** Allogeneic cardiosphere-derived stem cells

**Indication:** Pulmonary Arterial Hypertension (PAH)

**Goal:** Complete a phase 1a/2b clinical trial to assess safety and preliminary efficacy of the treatment in PAH patients

## **Major Proposed Activities:**

- Assess maximum tolerable dose and safety in patients with PAH
- Assess long term safety and preliminary efficacy in patients with PAH

**Funds Requested:** \$7,354,772 (\$0 Co-funding)



# CLIN2-09444: Clinical Trial to Treat Pulmonary Arterial Hypertension

**Budget Review:** Pass

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

- Votes for score of 1: **7**
- Votes for score of 2: **5**
- Votes for score of 3: **0**

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

**Award Amount:** \$7,354,772\*

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

# CLIN2-09577: Clinical Trial to Treat Solid Tumor and Colorectal Cancer

**Therapy:** Anti-CD47 monoclonal antibody combined with cetuximab to target cancer stem cells

**Indication:** Patients with advanced colon cancer

**Goal:** Complete phase 1b/2 clinical trial to support continued development of the combination product

## **Major Proposed Activities:**

- Determine safety and tolerability of treatment
- Determine optimal dose regimen
- Determine therapeutic efficacy in colon cancer patients

**Funds Requested:** \$10,234,048 (\$6,822,698 Co-funding)



# CLIN2-09577: Clinical Trial to Treat Solid Tumor and Colorectal Cancer

**Budget Review:** Pass

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

- Votes for score of 1: **14**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

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

**Award Amount:** \$10,234,048\*

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