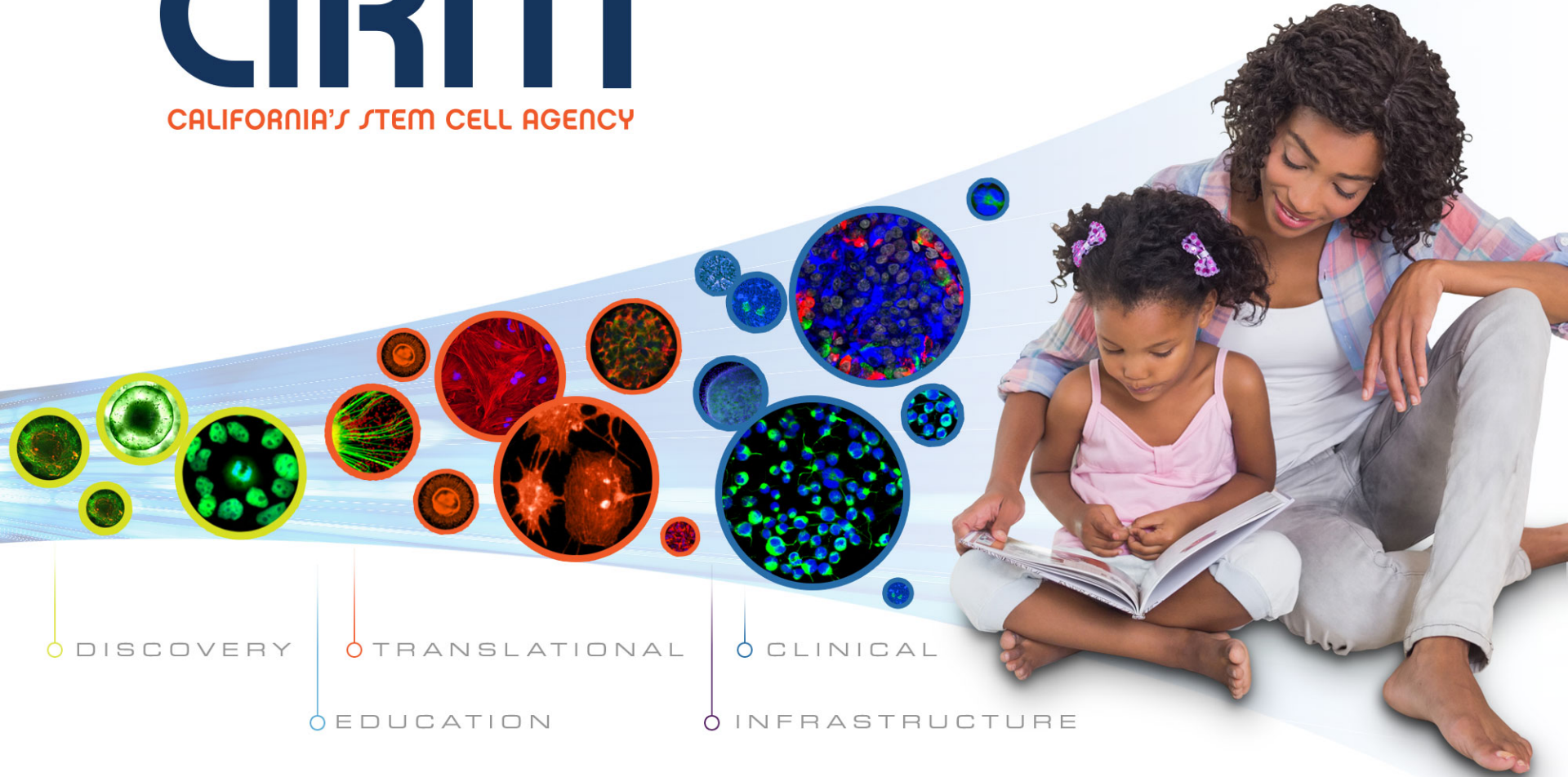


CIRM

CALIFORNIA'S STEM CELL AGENCY



○ DISCOVERY

○ TRANSLATIONAL

○ CLINICAL

○ EDUCATION

○ INFRASTRUCTURE

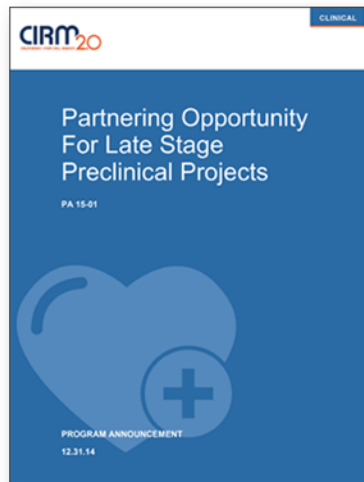
GWG Review of Clinical Program Applications

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.

CLIN2-09698: Clinical Trial of Cell Therapy for Retinitis Pigmentosa

Therapy: Allogeneic human retinal progenitor cells

Indication: Retinitis pigmentosa

Goal: Complete a phase 2 clinical trial to test efficacy of the cell therapy treatment in patients with retinitis pigmentosa

Major Proposed Activities:

- Enroll patients in phase 2b trial
- Conduct patient follow up
- Collect all clinical outcome measures

Funds Requested: \$8,295,750 (\$5,530,501 Co-funding)

CLIN2-09698: Clinical Trial of Cell Therapy for Retinitis Pigmentosa

Budget Review: Pass

GWG Score: 1 Exceptional merit, warrants funding

- Votes for score of 1: **11**
- Votes for score of 2: **4**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$8,295,750*

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

CLIN2-09439: Clinical Trial to Prevent Immune Rejection of Kidney Transplants

Therapy: Hematopoietic stem cells and T cells from organ transplant donors

Indication: Kidney transplant

Goal: Complete a phase 1 clinical trial to assess safety and preliminary efficacy of the treatment in kidney transplant patients

Major Proposed Activities:

- Manufacture donor cell product
- Assess the clinical safety of the donor cell injection
- Assess the ability to withdraw immunosuppressive drugs

Funds Requested: \$6,653,266 (\$0 Co-funding)

CLIN2-09439: Clinical Trial to Prevent Immune Rejection of Kidney Transplants

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: \$6,653,266*

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