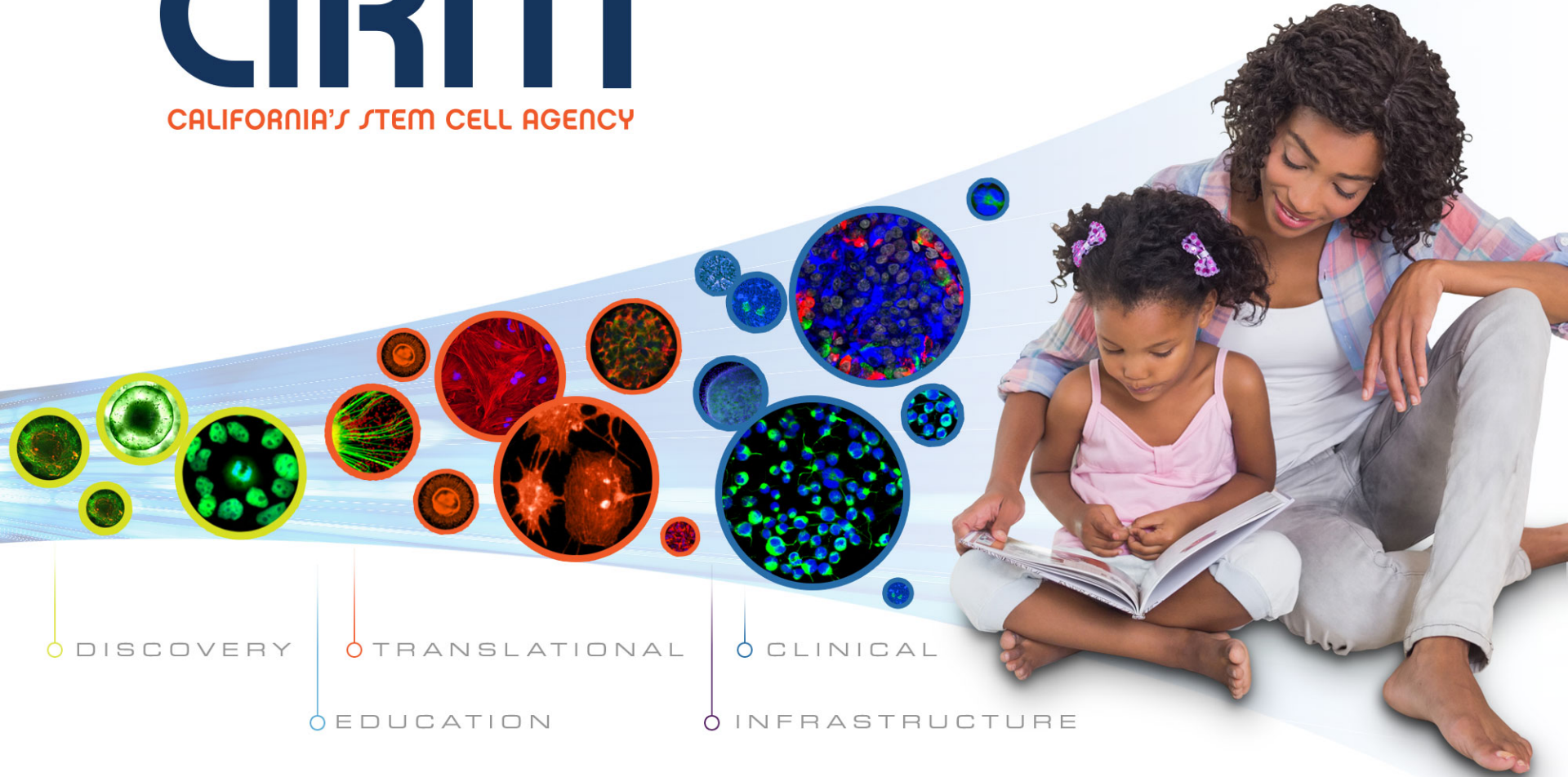


CIRM

CALIFORNIA'S STEM CELL AGENCY



○ DISCOVERY

○ TRANSLATIONAL

○ CLINICAL

○ EDUCATION

○ INFRASTRUCTURE

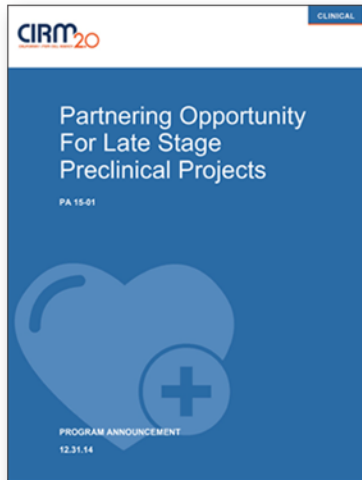
GWG Review of Clinical Program Applications

Gil Sambrano

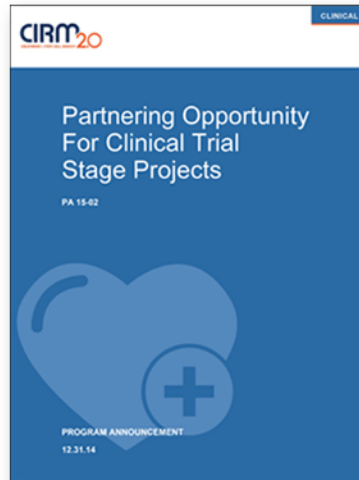
Director of Portfolio Development and Review

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.

CLIN1-09230: Preclinical Development of a Gene Therapy Approach for Cystinosis

Therapy: Genetically-modified hematopoietic stem cells (HSCs) from peripheral blood of patients with cystinosis

Indication: Cystinosis

Goal: Complete IND-enabling activities to support the filing of an IND and the conduct of a future clinical trial.

Major Proposed Activities:

- Perform pharmacology/toxicology studies
- Develop large-scale manufacturing (GMP) methods
- Prepare and submit IND

Funds Requested: \$5,273,189 (\$0 Co-funding)

CLIN1-09230: Preclinical Development of a Gene Therapy Approach for Cystinosis

Budget Review: Pass

GWG Score: 1 Exceptional merit, warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$5,273,189