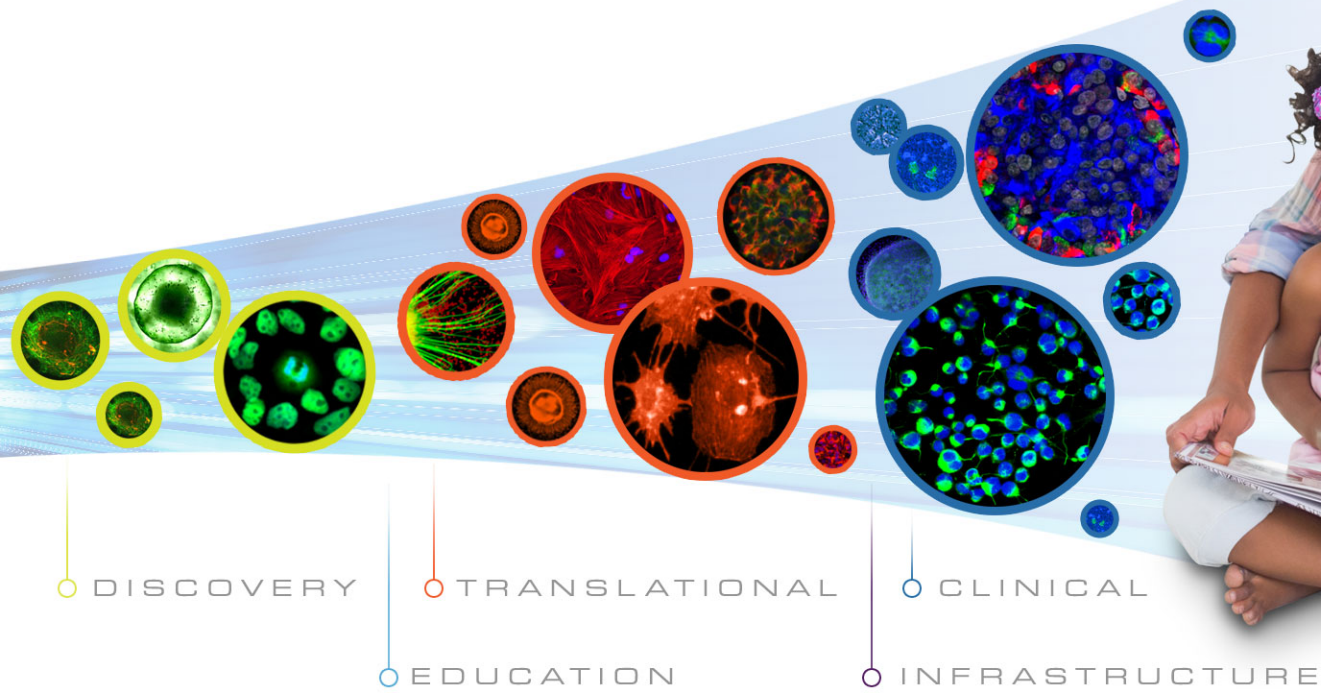


CIRM

CALIFORNIA'S STEM CELL AGENCY



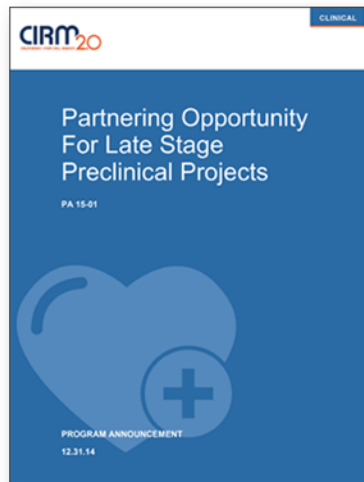
GWG Review of Clinical Program Applications

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.

CLIN1-08671: Preclinical Development of a Cell Therapy and Device for Diabetes

Therapy: Human embryonic stem cell (hESC)-derived pancreatic progenitor cells delivered in a device that allows direct vascularization

Indication: High-risk type 1 diabetes including "brittle" diabetes and hypoglycemia unawareness

Goal: Complete preclinical research activities needed to submit an IND and support a future clinical trial.

Major Proposed Activities:

- Manufacture and quality control assessment of the cells and devices for preclinical and clinical studies
- Execute GLP preclinical safety study
- Prepare and submit IND application to FDA to allow clinical testing

Funds Requested: \$3,984,164 (\$994,343 Co-funding)

CLIN1-08671: Preclinical Development of a Cell Therapy and Device for Diabetes

Budget Review: Pass

GWG Score: 1* Exceptional merit, warrants funding

- Votes for score of 1: **6**
- Votes for score of 2: **4**
- Votes for score of 3: **3**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$3,984,164

*Revised application score – originally scored 2 (0-9-2)

CLIN2-08938: Phase 3 Clinical Trial of an Acellular Graft for Hemodialysis

Therapy: Implanted human acellular vessel (HAV)

Indication: End-stage renal disease patients requiring vascular access for hemodialysis

Goal: Complete a Phase 3 clinical trial to attain FDA approval for clinical use of the product

Major Proposed Activities:

- Manufacturing and distribution of the HAV for clinical testing
- Enrollment in Phase 3 clinical trial and implantation of HAV into patients requiring vascular access for hemodialysis
- Longitudinal patient follow-up, data collection and analysis, and regulatory approval of HAV for widespread clinical use

Funds Requested: \$9,999,528 (\$9,999,528 Co-funding)

CLIN2-08938: Phase 3 Clinical Trial of an Acellular Graft for Hemodialysis

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: **3**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$9,999,528

*Revised application score – originally scored 2 (5-6-0), (4-8-0), and (1-11-0)