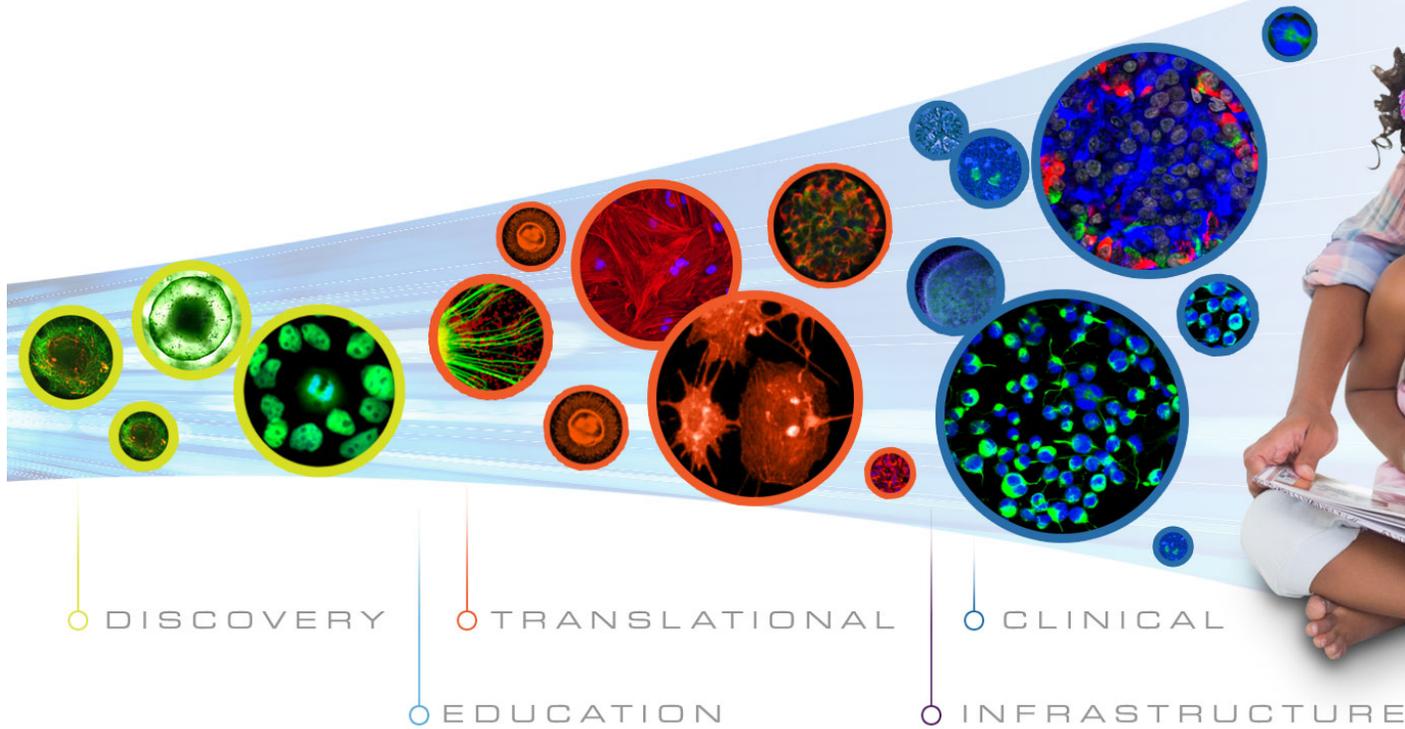


February 18, 2016

CIRM

CALIFORNIA'S STEM CELL AGENCY



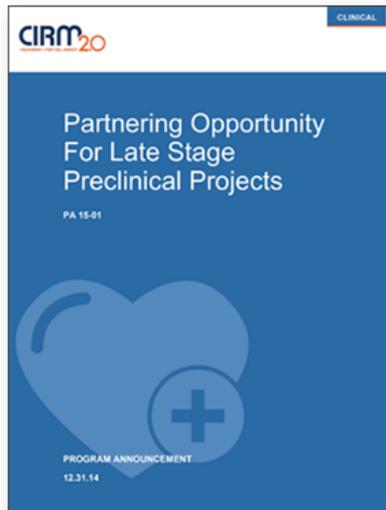
GWG Review of Clinical Program Applications

Gil Sambrano

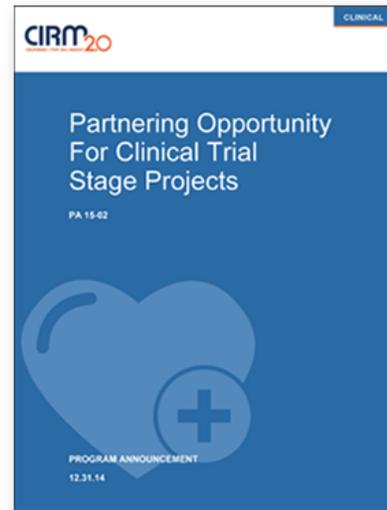
Director of Portfolio Development and Review

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.

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

CLIN1-08342: Preclinical Development of a Product to Improve Cord Blood Transplant Outcomes

Therapy: AB-110: CD34+ cord blood-derived hematopoietic stem and progenitor cells co-cultured and co-infused with genetically modified endothelial cells

Indication: Patients with life-threatening high-risk hematologic malignancies including leukemia and lymphoma

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

Major Proposed Activities:

- Develop/validate the GMP process for manufacturing the product in a closed system bioreactor
- Complete safety assessment of the E-CEL HUVEC component of AB-110
- Prepare & file IND application with FDA to advance the product into clinical studies

Funds Requested: \$3,797,117 (\$844,638 Co-funding)

CLIN1-08342: Preclinical Development of a Product to Improve Cord Blood Transplant Outcomes

Budget Review: Pass

GWG Score: 1* Exceptional merit, warrants funding

- Votes for score of 1: 8
- Votes for score of 2: 4
- Votes for score of 3: 0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$3,797,117

*Revised application score - originally scored 2 (0-12-2)