

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

Clinical Program

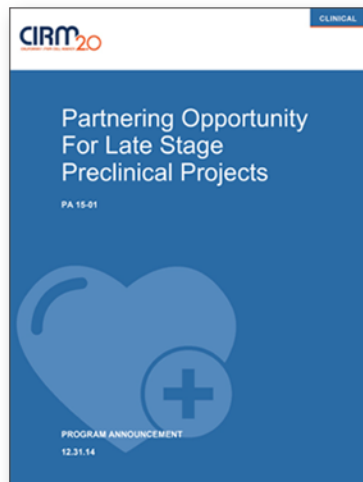
Gil Sambrano, Ph.D.

Vice President Portfolio Development
and Review

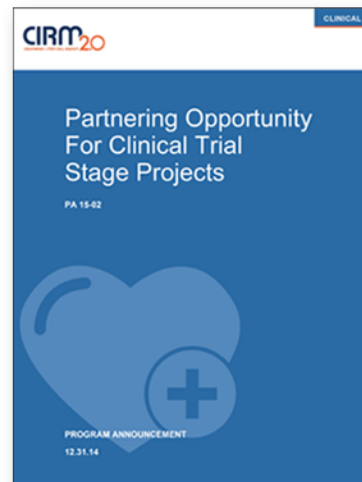


HOPE

Clinical Stage Programs



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.

CLIN2-10411: Phase 3 Clinical Trial of Therapy to Induce Immune Tolerance



Therapy: Blood stem cell and T cell combination

Indication: Kidney transplant patients

Goal: Complete a phase 3 clinical trial and BLA submission for approval

Major Proposed Activities:

- GMP manufacture of cell therapy product
- Demonstrate predictive value of donor mixed chimerism testing
- Demonstrate ability to achieve durable immune tolerance

Funds Requested: \$18,763,585 (\$18,763,585 Co-funding)

CLIN2-10411: Phase 3 Clinical Trial of Therapy to Induce Immune Tolerance



GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$18,763,585*

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