

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

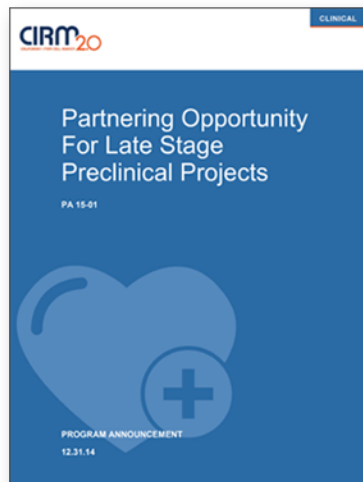


Gil Sambrano, Ph.D.

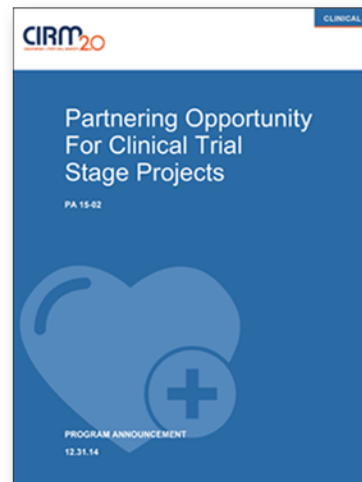
Vice President Portfolio Development
and Review

HOPE

Clinical Stage Programs



CLIN 1



CLIN 2



CLIN 3

GWG Vote on Review Process

1. *All members*: “The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG.”
2. *Patient advocate members*: “The review was carried out in a fair manner and was free from undue bias.”

Vote was unanimously in favor on both statements for all applications reviewed in this cycle.

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.

CLIN1-09759: Pre-Clinical Development of a Cell Therapy for Parkinson's Disease



Therapy: hESC-derive neural progenitors expressing MEF2C

Indication: Moderate to severe Parkinson's disease

Goal: Complete preclinical activities to file an IND for testing the cell therapy product in a clinical trial

Major Proposed Activities:

- Manufacturing of cGMP qualified cell bank for GLP studies
- Preclinical dose-response efficacy and toxicity/safety studies
- Prepare and file IND

Funds Requested: \$4,817,184 (\$0 Co-funding)

CLIN1-09759: Pre-Clinical Development of a Cell Therapy for Parkinson's Disease



Budget Review: Pass

GWG Score: 3 Does **not** warrant funding and should not be resubmitted for at least 6 months

- Votes for score of 1: **0**
- Votes for score of 2: **0**
- Votes for score of 3: **13**

CIRM Team Recommendation: Do not fund (concur with GWG recommendation)