

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

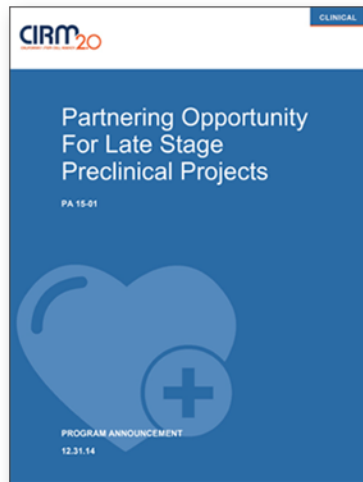


Gil Sambrano, Ph.D.

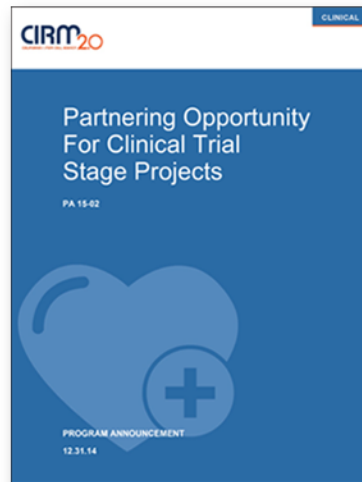
Vice President Portfolio Development
and Review

HOPE

Clinical Stage Programs



CLIN 1



CLIN 2



CLIN 3

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.

CLIN2-09894: Phase 3 Clinical Trial for Amyotrophic Lateral Sclerosis (ALS)



Therapy: Modified mesenchymal stem cells

Indication: Amyotrophic lateral sclerosis (ALS)

Goal: Complete a phase 3 clinical trial to establish efficacy of a cell therapy in patients with ALS

Major Proposed Activities:

- Manufacture of cell therapy product
- Enrollment of 200 patients for the study
- Conduct of clinical trial

Funds Requested: \$15,912,390 (\$15,912,390 Co-funding)

CLIN2-09894: Phase 3 Clinical Trial for Amyotrophic Lateral Sclerosis (ALS)



Budget Review: Pass

GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **9**
- Votes for score of 2: **3**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$ \$15,912,390*

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