

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

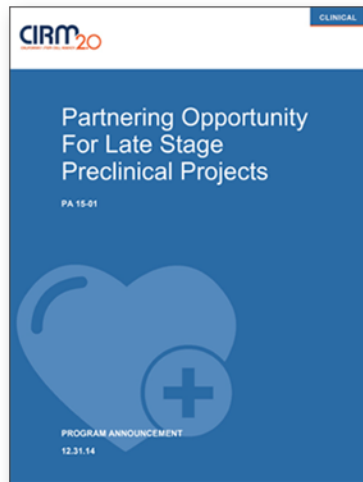
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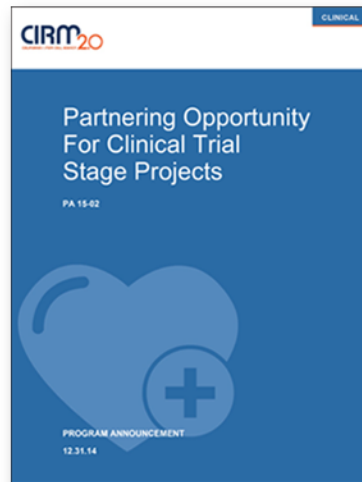
Gil Sambrano, Ph.D.
Vice President Portfolio Development
and Review

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.

CLIN2-09183: Clinical Trial for Fetal Alpha Thalassemia Major



Therapy: Maternal bone marrow-derived hematopoietic stem cells

Indication: Fetal alpha thalassemia major

Goal: Complete clinical trial and establish safety of in utero transplantation of maternal HSCs for thalassemia

Major Proposed Activities:

- Manufacture maternal bone marrow stem cells
- Establish safety of in utero transplantation in fetuses
- Establish feasibility of harvest/transplant procedures

Funds Requested: \$12,131,817 (\$0 Co-funding)

CLIN2-09183: Clinical Trial for Fetal Alpha Thalassemia Major



Budget Review: Pass

GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **12**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$12,131,817*

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

CLIN1-09433: Pre-Clinical Development of a Cell Therapy for Stroke



Therapy: Human neural stem cells (hESC-derived)

Indication: Chronic ischemic stroke motor deficits

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

Major Proposed Activities:

- Perform IND enabling in vitro studies
- GLP toxicology and biodistribution studies
- Submit IND

Funds Requested: \$5,300,000 (\$0 Co-funding)

CLIN1-09433: Pre-Clinical Development of a Cell Therapy for Stroke



Budget Review: Pass

GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$5,300,000*

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

CLIN1-09776: Pre-Clinical Development of a Therapy for Acute Myeloid Leukemia



Therapy: Antibody-drug conjugate targeting leukemic stem cells

Indication: Acute myeloid leukemia (AML)

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

Major Proposed Activities:

- Complete non-clinical and IND-enabling GLP studies
- Manufacture product to support IND-enabling studies and phase 1 trial
- Generate clinical protocol and file IND

Funds Requested: \$6,863,755 (\$1,715,939 Co-funding)

CLIN1-09776: Pre-Clinical Development of a Therapy for Acute Myeloid Leukemia



Budget Review: Pass

GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$6,863,755*

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

CLIN2-10344: Phase 2b Clinical Trial of a Cell Therapy for Stroke



Therapy: Modified mesenchymal stem cells

Indication: Chronic ischemic stroke motor deficits

Goal: Complete phase 2b trial to establish safety and efficacy of modified MSC in patients with chronic disability secondary to ischemic stroke

Major Proposed Activities:

- Complete clinical trial enrollment and treatment
- Manufacture modified MSC for trial
- Investigate MOA and identify measures of potency

Funds Requested: \$19,998,580 (\$22,465,474 Co-funding)

CLIN2-10344: Phase 2b Clinical Trial of a Cell Therapy for Stroke



Budget Review: Pass

GWG Score: 1 Exceptional merit and 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: \$19,998,580*

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