

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

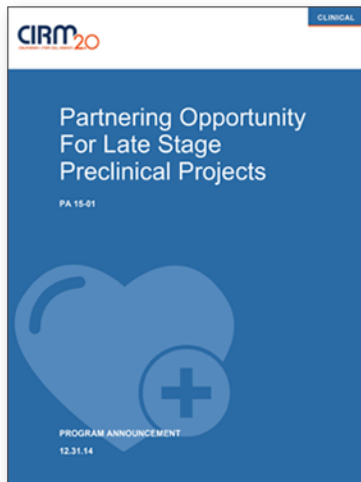


Gil Sambrano, Ph.D.

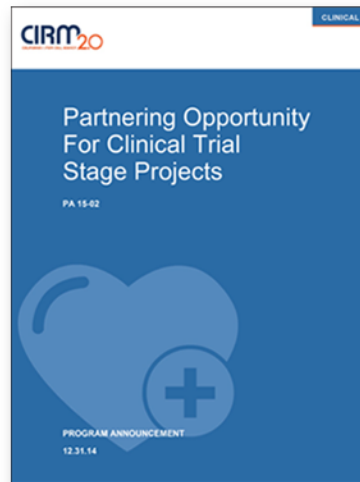
Vice President Portfolio Development and Review
Agenda Item # 7
Clinical Presentation

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.

CLIN2-09574: Phase 2 Clinical Trial of Therapy for Neutropenia in AML Patients



Therapy: Umbilical cord blood stem cells

Indication: Neutropenia arising from high-dose chemotherapy for Acute Myeloid Leukemia

Goal: Complete a phase 2 clinical trial to assess safety and efficacy of the therapy in AML patients

Major Proposed Activities:

- Preparation for scale-up of product manufacturing
- GMP manufacture of cell therapy
- Conduct of phase 2 clinical trial

Funds Requested: \$6,922,109 (\$16,852,313 Co-funding)

CLIN2-09574: Phase 2 Clinical Trial of Therapy for Neutropenia in AML Patients



GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$6,922,109*

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

CLIN2-09672: Phase 1 Trial of a Cell Therapy for High-Risk Type 1 Diabetes



Therapy: Human ESC-derived pancreatic progenitors in a delivery device

Indication: High-risk type 1 diabetes

Goal: Complete a phase 1/2 clinical trial to assess safety and efficacy of the cell therapy in patients

Major Proposed Activities:

- Manufacture and quality control of the cell/device product
- Launch and conduct of clinical trial
- Assay development

Funds Requested: \$20,000,000 (\$8,571,429 Co-funding)

CLIN2-09672: Phase 1 Trial of a Cell Therapy for High-Risk Type 1 Diabetes



GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **15**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$20,000,000*

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

CLIN2-09688: Phase 3 Clinical Trial of Human Acellular Vessel for Hemodialysis



Therapy: Human Acellular Vessel (HAV)

Indication: Conduit for Vascular Access for Hemodialysis

Goal: Complete a phase 3 clinical trial comparing HAV to gold standard AV fistula

Major Proposed Activities:

- Manufacture and distribution of HAV for clinical testing
- Enrollment of patients requiring vascular access for hemodialysis
- Longitudinal test subject follow-up, data collection and analysis

Funds Requested: \$14,082,865 (\$26,425,033 Co-funding)

CLIN2-09688: Phase 3 Clinical Trial of Human Acellular Vessel for Hemodialysis



GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **8**
- Votes for score of 2: **2**
- Votes for score of 3: **5**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$14,082,865*

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

CLIN1-10084: IND-Enabling Studies of a Therapy for Sickle Cell Disease



Therapy: Genetically modified autologous blood stem cells

Indication: Severe sickle cell disease

Goal: Complete a IND-enabling activities and file an IND to test the therapy in a future clinical trial

Major Proposed Activities:

- Generate viral vector for gene correction
- Establish reproducibility of manufacturing process
- Prepare and file IND to begin a phase 1/2 trial

Funds Requested: \$5,194,431 (\$0 Co-funding)

CLIN1-10084: IND-Enabling Studies of a Therapy for Sickle Cell Disease



GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$5,194,431*

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

CLIN2-10144: Phase 1b Clinical Trial of an Antibody Therapy for AML



Therapy: Monoclonal antibody targeting cancer stem cells

Indication: AML

Goal: Complete a phase 1b clinical trial to assess safety and efficacy of the therapy

Major Proposed Activities:

- Assess safety and tolerance of antibody alone or with azacitidine
- Assess efficacy of antibody alone or with azacitidine
- Assess optimal dosing regimen

Funds Requested: \$5,000,000 (\$7,390,868 Co-funding)

CLIN2-10144: Phase 1b Clinical Trial of an Antibody Therapy for AML



GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **14**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$5,000,000*

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

CLIN2-10248: Phase 1 Clinical Trial of CAR T-Cell Therapy for Malignant Glioma



Therapy: T cells engineered to target cancer stem cells

Indication: Malignant glioma

Goal: Complete a phase 1 clinical trial to assess safety and preliminary efficacy of the therapy

Major Proposed Activities:

- Manufacture product and evaluate route of administration
- Evaluate safety and preliminary safety of product
- Develop and establish reagents and methods for phase 2

Funds Requested: \$12,753,854 (\$0 Co-funding)

CLIN2-10248: Phase 1 Clinical Trial of CAR T-Cell Therapy for Malignant Glioma



GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **15**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$12,753,854*

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