

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

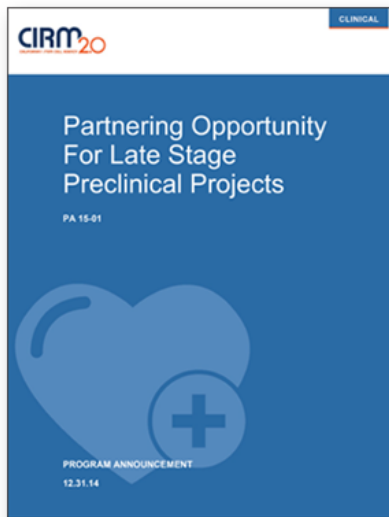
Clinical Program

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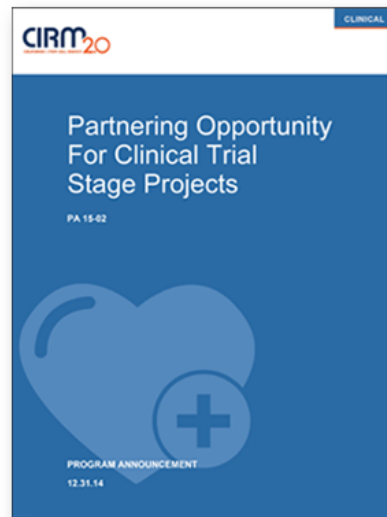


HOPE

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.

CLIN2-10388: Phase 1 Clinical Trial of a Small Molecule Therapy for Osteoarthritis



Therapy: Small molecule drug

Indication: Osteoarthritis

Goal: Complete a phase 1 clinical trial to assess safety of the therapy in patients with knee osteoarthritis

Major Proposed Activities:

- Site selection, start-up activities, and execution of clinical study
- Data analysis and reporting
- Evaluation of potential biomarkers for clinical development

Funds Requested: \$8,447,523 (\$0 Co-funding)

CLIN2-10388: Phase 1 Clinical Trial of a Small Molecule Therapy for Osteoarthritis



GWG Score: 1 Exceptional merit and warrants funding

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

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$8,447,523*

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

CLIN2-10392: Phase 1/2 Trial of a Cell Therapy for Viral Infection



Therapy: Partially HLA-matched virus specific T cells

Indication: Persistent viral infections that fail conventional treatment

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

Major Proposed Activities:

- Assess safety of administering partially HLA-matched T cells to treat viral infection
- Assess efficacy of product and overall survival at 6 and 12 months

Funds Requested: \$4,825,587 (\$0 Co-funding)

CLIN2-10392: Phase 1/2 Trial of a Cell Therapy for Viral Infection



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: \$4,825,587*

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

CLIN2-10395: Phase 1 Clinical Trial of a Cell Therapy for Multiple Myeloma



Therapy: Stem cell memory CAR-T cells

Indication: Multiple myeloma

Goal: Complete a phase 1 clinical trial to assess safety of the CAR-T cell therapy

Major Proposed Activities:

- Manufacture of product for clinical testing
- Enrollment, treatment, and follow-up of patients
- Complete nonclinical safety studies

Funds Requested: \$19,997,927 (\$8,571,294 Co-funding)

CLIN2-10395: Phase 1 Clinical Trial of a Cell Therapy for Multiple Myeloma



GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **10**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$19,813,407* (adjusted by GMO)

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