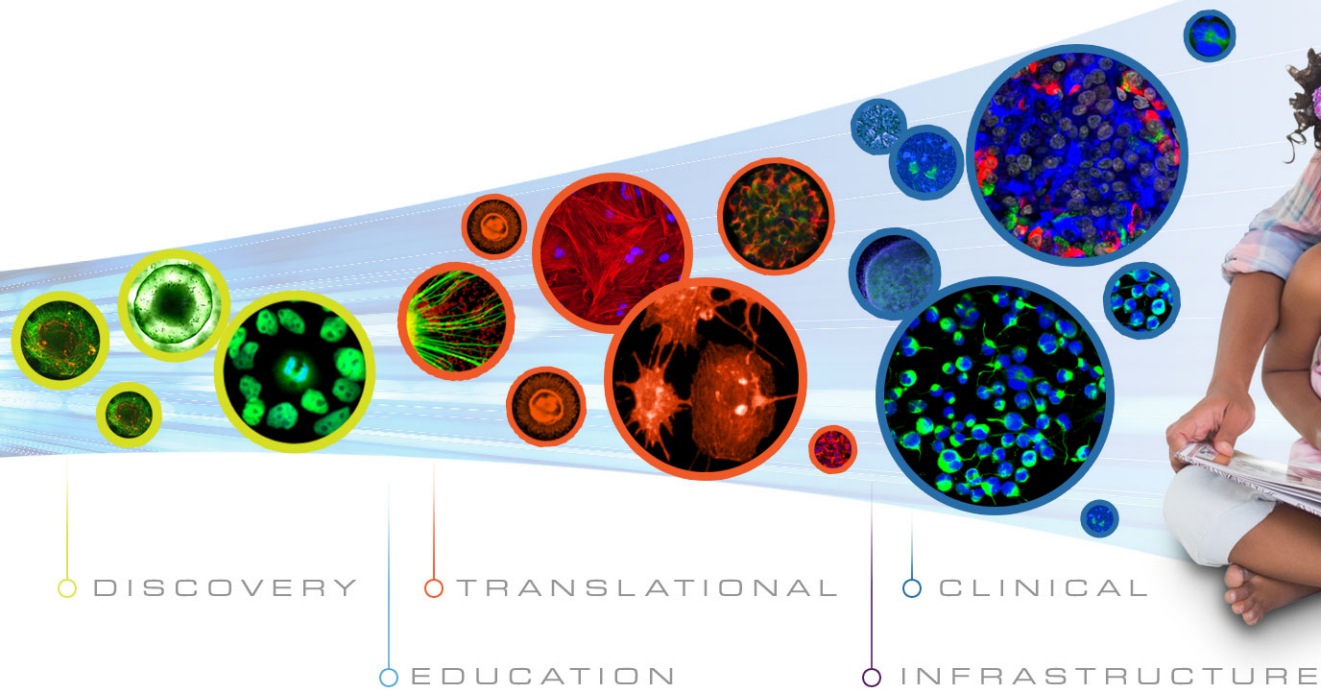


CIRM

CALIFORNIA'S STEM CELL AGENCY



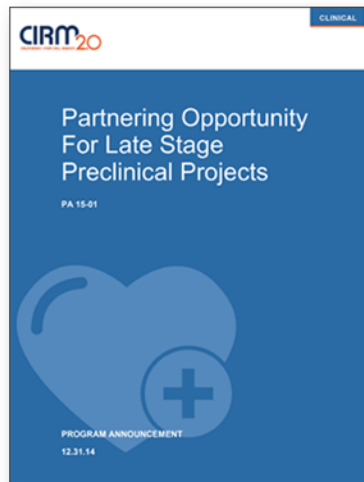
GWG Review of Clinical Program Applications

Rebecca Jorgenson

Senior Science Officer, Portfolio Development and Review

Clinical Stage Programs

CLINICAL STAGE



PA15-01



PA15-02



PA15-03

New 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.

Applications are scored by all scientific members of the GWG with no conflict.

CTS1-08289: Phase 1 Clinical Trial for AIDS Lymphoma Patients

Therapy: Gene-modified hematopoietic stem cells (HSCs)

Goal: Conduct a Ph1 trial of the safety, feasibility, & efficacy of gene-modified HSCs to generate HIV resistant immune cells & improve immune function of patients with AIDS-related lymphoma.

Major Proposed Activities:

- Produce clinical-grade anti-HIV vector and HIV-resistant HSCs.
- Conduct Ph1 clinical trial in AIDS lymphoma patients.
- Evaluate and monitor function of transplanted cells and their effects on HIV.

Funds Requested: \$8,521,441

CTS1-08289: Phase 1 Clinical Trial for AIDS Lymphoma Patients

Budget Review: Pass

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: concur with GWG recommendation

Award Amount: \$8,521,441

CTS1-08231: Phase 1/2 Clinical Trial for Chronic Granulomatous Disease

Therapy: Gene-modified hematopoietic stem cells (HSCs)

Goal: Conduct a clinical trial to test the safety and efficacy of gene-modified HSCs to replace the patient's cells with gene-corrected cells in order to improve immune function.

Major Proposed Activities:

- Produce patient-specific gene-corrected HSCs.
- Transplant patients with severe XCGD lacking matched donors with autologous cell product.
- Perform two-year follow-up to assess trial end-points.

Funds Requested: \$7,402,549 (plus \$4,633,848 co-funding)

CTS1-08231: Phase 1 Clinical Trial for Chronic Granulomatous Disease

Budget Review: Pass

GWG Score: 1 *exceptional merit and warrants funding*

- Votes for score of 1: **5**
- Votes for score of 2: **1**
- Votes for score of 3: **4**

CIRM Team Recommendation: concur with GWG recommendation

Award Amount: \$7,402,549

SAA1-08273: Supplement to Phase 1 Clinical Trial in Chronic Lymphocytic Leukemia (CLL)

Therapy: A monoclonal antibody therapy

Parent Award: A Ph1 clinical trial to test the safety, feasibility, and efficacy of the candidate therapeutic (which is proposed to eradicate cancer stem cells) in CLL patients.

Goal: Conduct an extension study to administer repeat doses of the candidate therapeutic to patients enrolled in the ongoing Ph1 trial.

Major Proposed Activities:

- Produce sufficient antibody for completion of the proposed extension trial.
- Conduct the proposed extension.

Funds Requested: \$2,895,587

SAA1-08273: Supplement to Phase 1 Clinical Trial in Chronic Lymphocytic Leukemia (CLL)

Budget Review: Pass

GWG Score: *2 needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement*

- Votes for score of 1: **2**
- Votes for score of 2: **6**
- Votes for score of 3: **3**

CIRM Team Recommendation: Do not fund; concur with GWG recommendation