

**SOMETHING
BETTER
THAN HOPE**

**GWG Recommendations for Applications
Submitted to the CLIN Program**

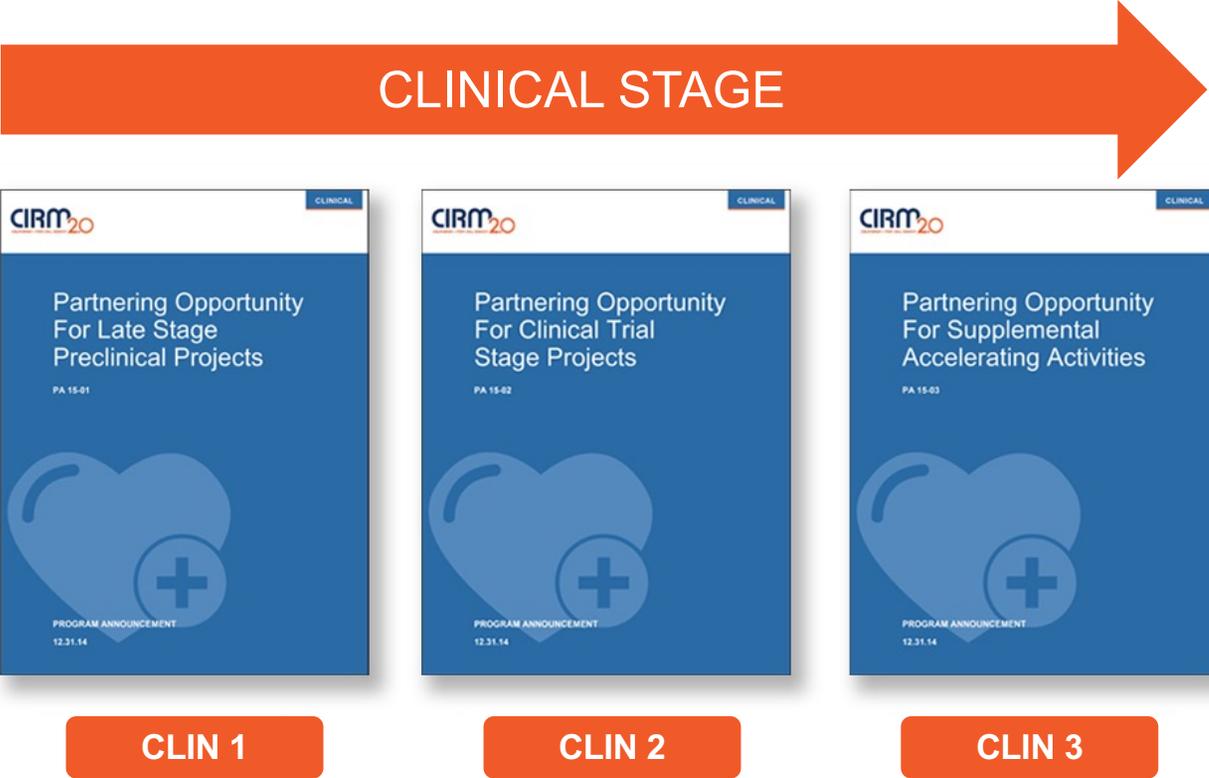
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Vice President
Portfolio Development and Review

September 23, 2021

CIRM
CALIFORNIA / STEM CELL AGENCY

Clinical Stage Programs

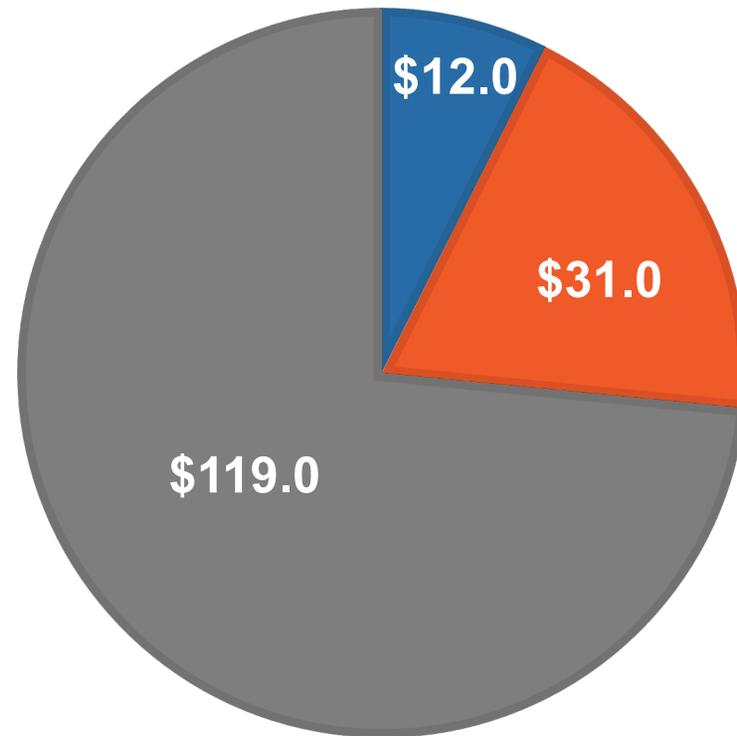


2021/22 Clinical Budget Status

Annual Allocation: \$162 million

- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



Review Criteria

1. Does the project hold the necessary significance and potential for impact? (i.e., what value does it offer; is it worth doing?)
2. Is the rationale sound? (i.e., does it make sense?)
3. Is the project well planned and designed?
4. Is the project feasible? (i.e., can they do it?)
5. Does the project address the needs of underserved communities?

Scientific Scoring System for Clinical Applications

- **Score of “1”**

Exceptional merit and warrants funding.

May have minor recommendations and adjustments that do not require further review by the GWG

- **Score of “2”**

Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.

GWG should provide recommendations that are achievable (i.e., “fixable changes”) or request clarification/information on key concerns.

- **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.

Elements in CIRM Application Review

- **Addressing the Needs of Underserved Communities**
 - This section describes the applicant's plan for outreach and enrollment of a diverse patient cohort that accounts for racial, ethnic and gender diversity
 - The section is evaluated as part of the overall project and incorporated into the scientific merit score (1,2 or 3)
- **Diversity, Equity and Inclusion**
 - This section describes how the applicant team incorporates diverse perspectives and experiences to improve the project through the composition of the team and/or any other approaches
 - This section is evaluated and scored by patient advocate/nurse members of the Board (appointed to the GWG) and shown in the DEI score (0-10)

CLIN2-12563: A T-Cell Immunotherapy for Patients with Hematologic Malignancies

Therapy	Allogeneic T-cell product enriched for regulatory T-cells
Indication	Hematologic malignancies requiring HSCT transplant
Goal	Complete a phase 1 trial to select phase 2 dose, assess safety and initial efficacy
Funds Requested	\$11,996,634 (co-funding: \$0)

Maximum funds allowable for this category: \$12,000,000

CLIN2-12563: Background Information

Clinical Background: Hematologic malignancies (such as acute leukemias and lymphomas) are the most common types of cancer in children and young adults.

Value Proposition of Proposed Therapy: The current standard of care for high-risk or refractory cancers is chemotherapy and allogeneic hematopoietic stem cells transplant. However, there is often a lack of matched donors as well as a high risk of rejection or GVHD for these patients. The proposed therapy offers the opportunity for greatly improving outcomes for patients undergoing HSCT.

Why a stem cell project: The therapeutic candidate is manufactured from CD4+ T-cell progenitor cells and is combined with a hematopoietic stem cell transplant.

CLIN2-12563: Similar CIRM Portfolio Projects

No other currently active projects in the CIRM portfolio for stroke.

CLIN2-12563: Previous CIRM Funding to Applicant

Applicant has not previously had other CIRM grant awards.

CLIN2-12563: GWG Review

GWG Recommendation: Exceptional merit and warrants funding

Scientific Score	GWG Votes
1	15
2	0
3	0

DEI Score: 9

CIRM Team Recommendation: Fund (concur with GWG recommendation)

CIRM Award Amount: \$ 11,996,634*

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