
CIRM
CALIFORNIA'S STEM CELL AGENCY
Clinical Program
GWG Recommendations
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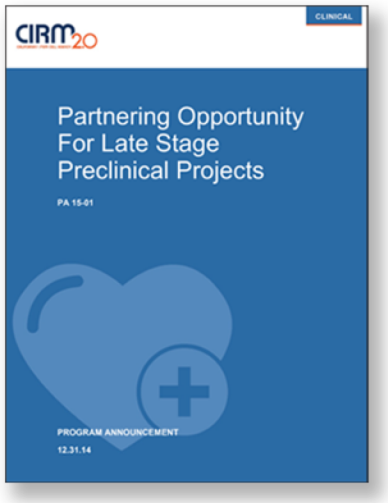
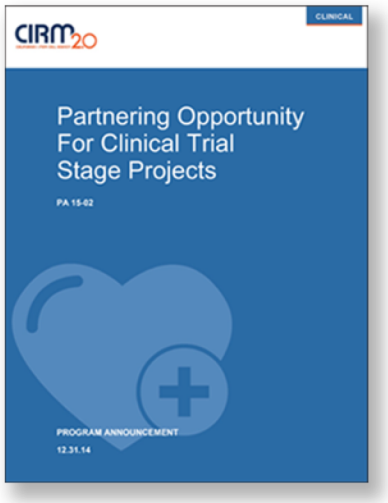
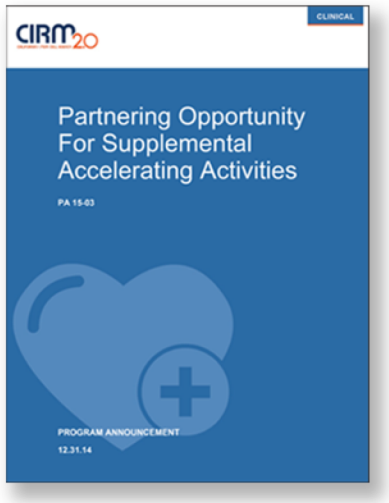
California Institute for Regenerative Medicine
TRANSFORMING
medicine
lives
futures
July 19, 2018

Clinical Stage Programs

CLINICAL STAGE







CLIN 1 **CLIN 2** **CLIN 3**

Scoring System for Clinical 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"**

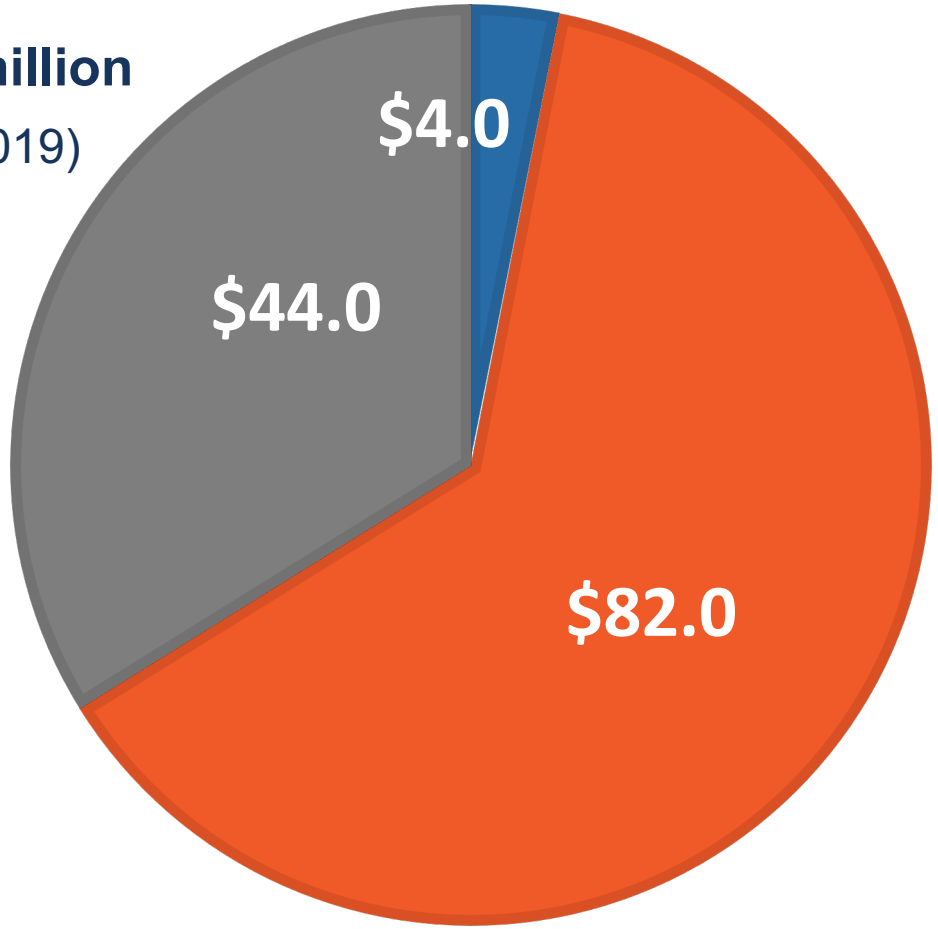
  Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted ***for at least 6 months****.*

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

2018 Clinical Budget Status
End of June

Annual Allocation: $130 million

(Additional $130 planned for 2019)

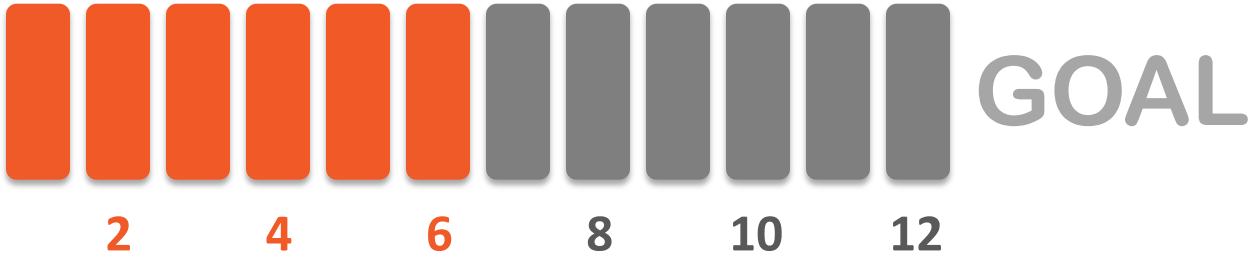


- **Amount Requested Today**
- **Approved Awards**
- **Unused Balance**

Amounts are shown in millions

2018 Clinical Award Targets

CLIN2
Clinical Trials



CLIN1
Late Stage Preclinical

Approved Award

Awaiting Today's Approval

CLIN1-10999: Late-Stage Preclinical Studies of Therapy for Prostate Cancer

Project Summary

Therapy	Genetically-engineered CAR-T memory stem cells
Indication	Patients with metastatic castrate-resistant prostate cancer (mCRPC)
Goal	Product manufacturing, conduct preclinical safety and efficacy studies, prepare and submit IND
Funds Requested	$3,992,090 ($998,023 Co-funding)

Maximum funds allowable for this category: $4,000,000

CLIN1-10999: Late-Stage Preclinical Studies of Therapy for Prostate Cancer

Potential impact: There will be 164,690 new cases of prostate cancer and an estimated 29,430 people will die from this disease in 2018 (NIH). While mCRPC patients represent a small percentage of the prostate cancer population they account for most of the prostate cancer deaths. If successful, the proposed therapy would impact mCRPC patients.

Value Proposition: Currently available therapies include abiraterone plus prednisone, enzalutamide, docetaxel and sipuleucel-T, all of which have shown modest survival benefit in clinical trials. The proposed therapy has the potential to be a safer and more effective option for improving patient survival.

Why a stem cell project: This is a cell therapy composed of memory stem T cells.

Related CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	IND	N/A	mCRPC	CAR-T memory stem cells	CAR-T mediated elimination of cancer cells
CLIN1-10893	IND	06/30/19	Solid Tumors	iPSC derived Natural Killer Cells	NK cell mediated elimination of cancer cells
CLIN2-09577	Phase 1b/2	12/31/21	Advanced Solid Tumors and Colorectal Cancer	Anti Cd-47 and cetuximab antibodies	Phagocytosis of cancer stem cells
DR3-07067	Phase 1	08/31/18	Advanced Solid Tumors	Small molecule	Inhibits cancer stem cell growth

Previous CIRM Funding

Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Phase 1	12/31/21	Multiple Myeloma	CAR-T memory stem cells	CAR-T mediated elimination of cancer cells

CLIN1-10999: Late-Stage Preclinical Studies of Therapy for Prostate Cancer

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	12
2	0
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: $3,992,090*

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