



Clinical Program GWG Recommendations

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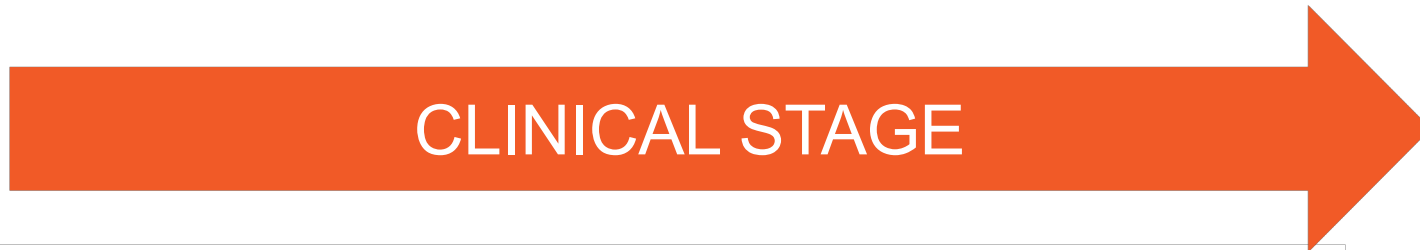
TRANSFORMING

*medicine
lives
futures*

October 18, 2018



Clinical Stage Programs



Every Moment Counts | Don't Stop Now

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.

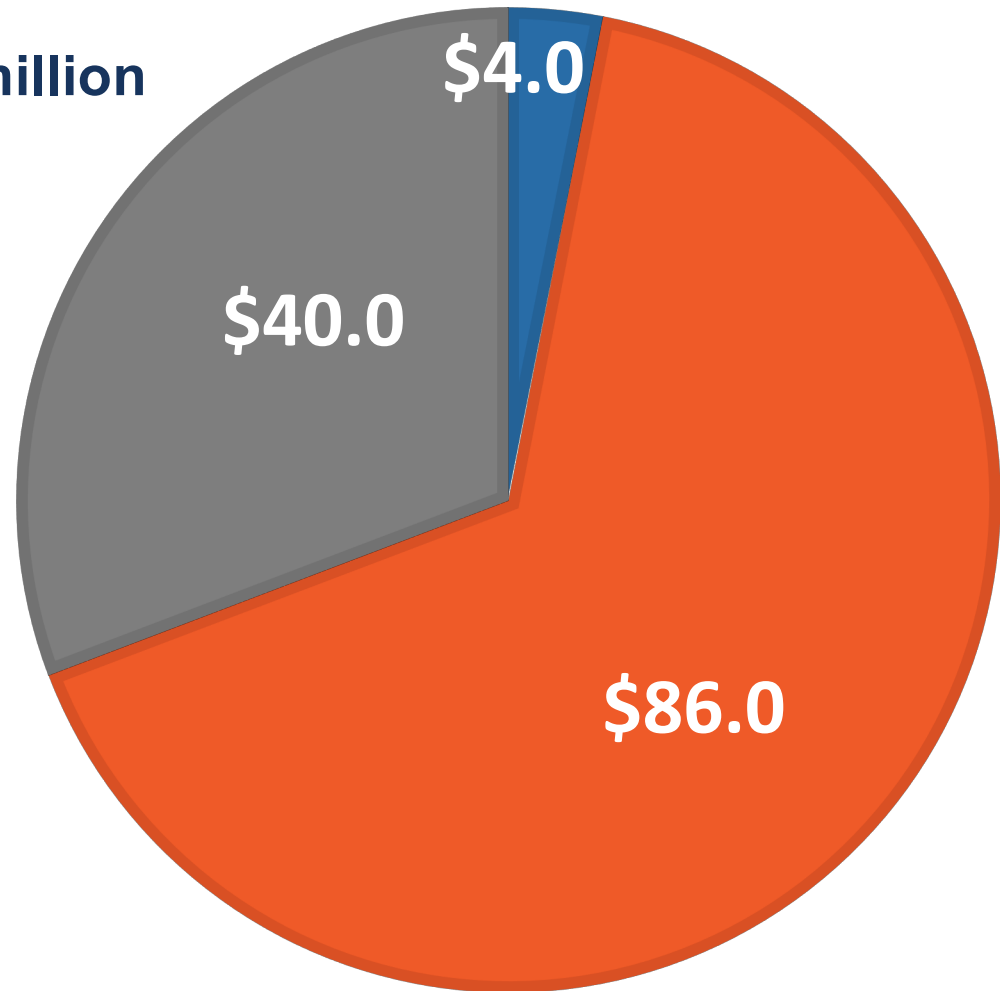
2018 Clinical Budget Status

End of September

Annual Allocation: \$130 million

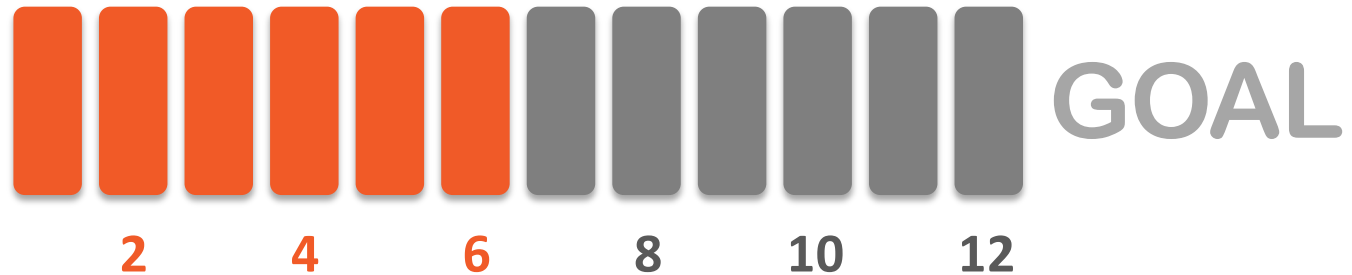
- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



2018 Clinical Award Targets

CLIN2 Clinical Trials



CLIN1 Late Stage Preclinical



 Approved Award

 Awaiting Today's Approval

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CLIN1-11223: Late-Stage Preclinical Studies of Therapy for HIV/AIDS

Project Summary

Therapy	Genetically-engineered CAR-T cells
Indication	Patients with HIV/AIDS
Goal	Product manufacturing, conduct preclinical safety and efficacy studies, prepare and submit IND
Funds Requested	\$3,812,797 (\$0 Co-funding)

Maximum funds allowable for this category: \$6,000,000

CLIN1-11223: Late-Stage Preclinical Studies of Therapy for HIV/AIDS

Potential impact: There are more than 1.1 million people in the US living with HIV and there are approximately 40,000 newly diagnosed patients each year (HHS). Approximately 16,000 HIV patients die in the US each year.

Value Proposition: The current standard-of-care is antiretroviral therapy (ART). While ART is effective at controlling HIV it requires daily administration and is associated with various morbidities including cardiovascular disease. The proposed CAR-T therapy has potential to achieve complete or functional cure of HIV infection without the need for ART.

Why a stem cell project: This is a cell therapy composed of central memory and 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	HIV/AIDS	CAR-T cells	CAR-T mediated elimination of HIV infected cells
SP3A-07536	Phase 1	01/31/20	HIV/AIDS	Gene modified HSC	CCR5 gene-modified HSC for HIV infection resistance
CLIN2-08289	Phase 1/2	08/31/19	AIDS Lymphoma	Gene modified HSC	HSC modified with triple combination of HIV- resistance genes.

Previous CIRM Funding

{CIRM funds have not been previously awarded to earlier stages of this project.}

CLIN1-11223: Late-Stage Preclinical Studies of Therapy for HIV/AIDS

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: \$3,812,797*

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