



Clinical Program GWG Recommendations

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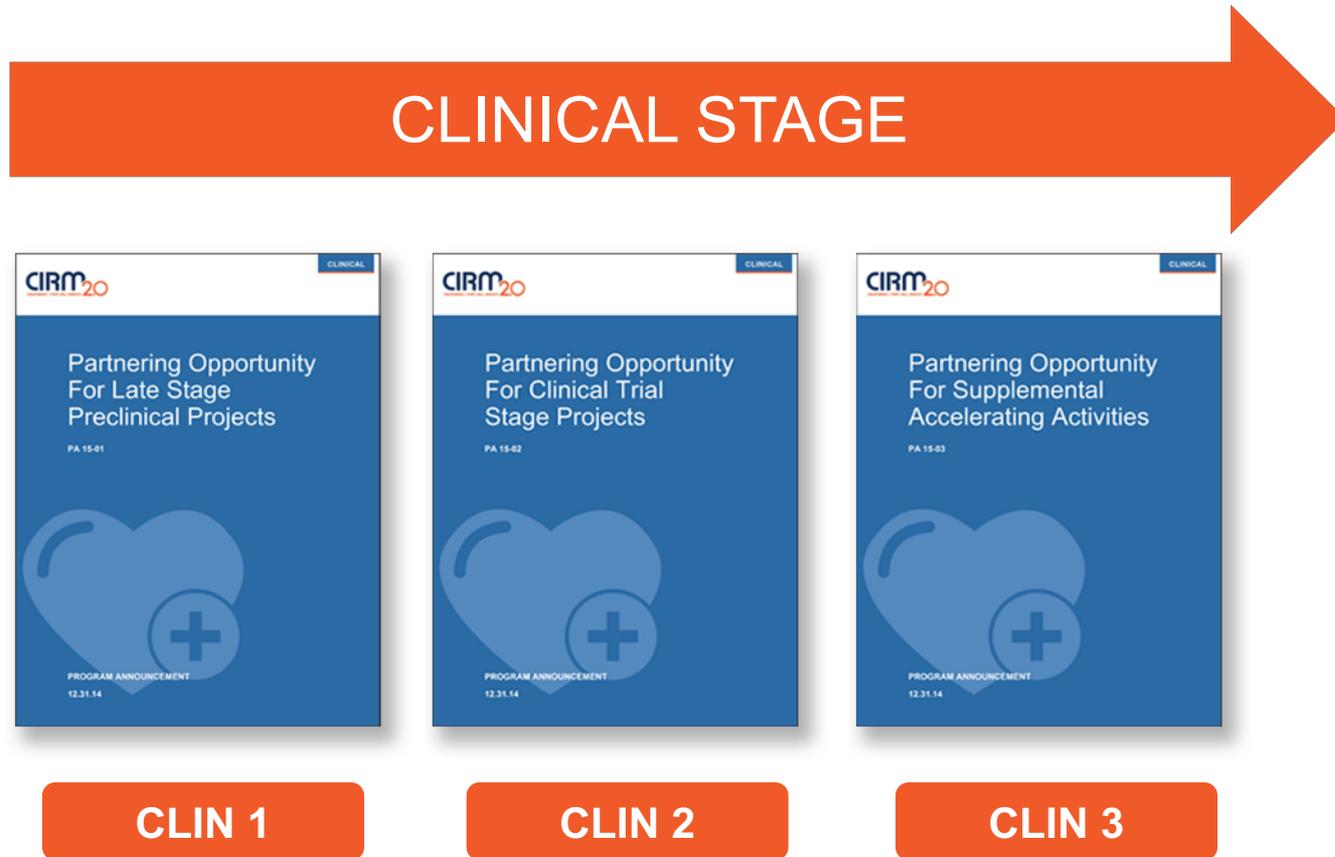
TRANSFORMING

*medicine
lives
futures*

December 13, 2018



Clinical Stage Programs



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.

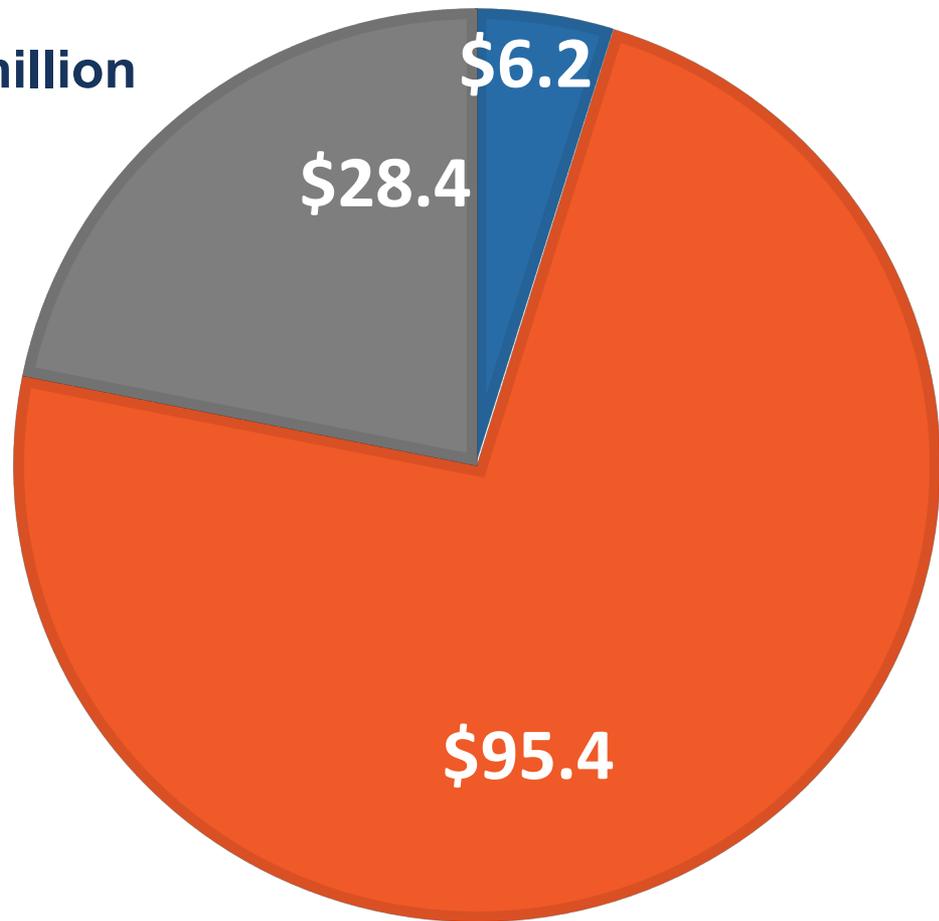
2018 Clinical Budget Status

End of November

Annual Allocation: \$130 million

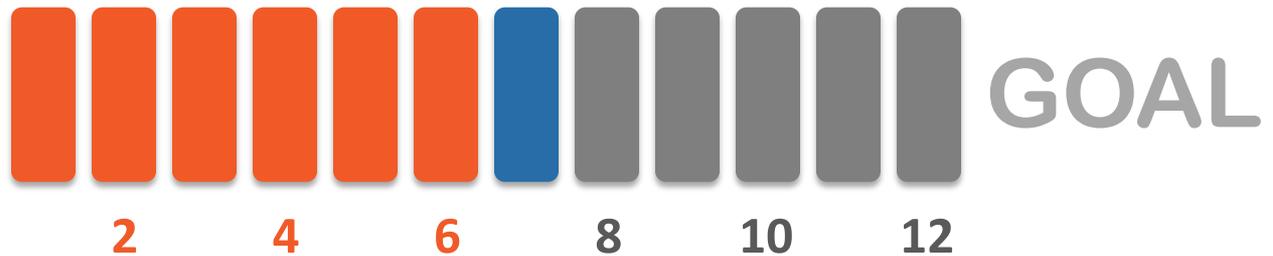
- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



2018 Clinical Award Targets

CLIN2 Clinical Trials



CLIN1 Late Stage Preclinical



 Approved Award  Awaiting Today's Approval

CLIN2-11371: Clinical Study of Therapy for Chemotherapy-Induced Toxicities

Project Summary

Therapy	Genetically engineered CD31+ cells derived from human umbilical veins
Indication	Lymphoma treated with high-dose chemotherapy followed by autologous stem cell transplant (HDT-ASCT)
Goal	Product manufacturing, conduct phase 1 trial
Funds Requested	\$6,192,579 (\$2,653,963 Co-funding)

Maximum funds allowable for this category: \$8,000,000

CLIN2-11371: Clinical Study of Therapy for Chemotherapy-Induced Toxicities

Potential impact: An estimated 83,000 new US cases of lymphoma will be diagnosed in 2018. Lymphoma is highly treatable and some types are curable. HDT-ASCT is standard therapy for relapsed or refractory lymphoma and is associated with various morbidities including mucositis, bone marrow toxicity, infections and pneumonitis.

Value Proposition: There are various organ-specific supportive treatments for chemotherapy-induced toxicity. These include agents that mobilize blood cells, prevent mucositis or treat gastrointestinal distress. The proposed cell therapy could activate the stem cell niche and induce recovery in multiple organ systems.

Why a stem cell project: This is a cell therapy that acts on endogenous stem cells for its therapeutic effect.

Related CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	Phase 1	N/A	Relapsed or Refractory Lymphoma	Engineered HUVEC	Organ recovery after HDT-ASCT via stem cell niche activation
CLIN2	Phase 1	12/31/2021	Leukemia	Cord blood stem cells and engineered HUVEC	Curative cord blood stem cell transplant

Previous CIRM Funding

Applicant has received previous funding from CIRM for a related candidate and indication.

Project Stage	Project Outcome	Project End Date
IND-Enabling	IND Filed	08/31/2017
Phase 1 Trial	N/A	Ongoing

CLIN2-11371: Clinical Study of Therapy for Chemotherapy-Induced Toxicities

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	8
2	4
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$6,192,579*

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