



Clinical Program GWG Recommendations

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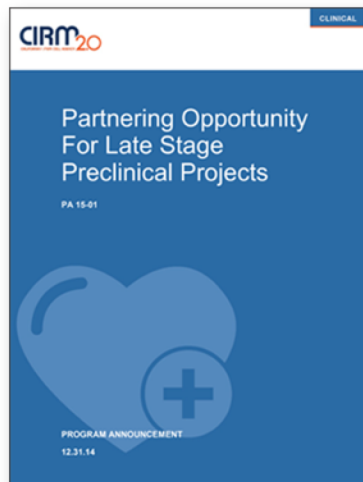
TRANSFORMING

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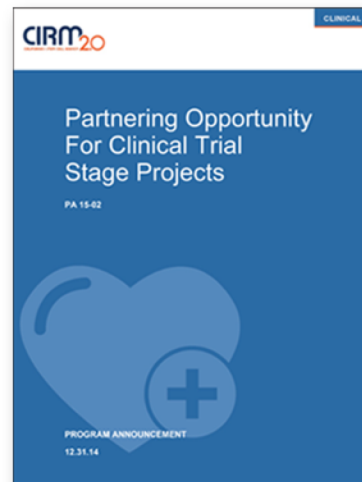
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Clinical Stage Programs



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.

CLIN2-10830: Phase 1 Clinical Trial of Therapy for Artemis-SCID

Project Summary

Therapy	Gene-corrected blood stem cell transplant
Indication	Children with severe combined immunodeficiency (SCID) due to Artemis gene defect
Goal	Complete a phase 1 clinical trial to assess safety and feasibility of the therapy
Funds Requested	\$12,000,000 (\$0 Co-funding)

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

CLIN2-10830: Phase 1 Clinical Trial of Therapy for Artemis-SCID

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	15
2	0
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$12,000,000*

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

CLIN2-10846: Phase 1 Clinical Trial of a (CAR) T Cell Therapy for B Cell Cancers

Project Summary

Therapy	T cells genetically engineered to target CD19 and/or CD22 on cancer B cells
Indication	Patients with relapsed and refractory B cell cancers
Goal	Complete phase 1 trial to assess safety and feasibility of the therapy
Funds Requested	\$11,976,906 (\$2,283,796 Co-funding)

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

CLIN2-10846: Phase 1 Clinical Trial of a (CAR) T Cell Therapy for B Cell Cancers

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	13
2	1
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$11,976,906*

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

CLIN2-11031: Phase 1/2 Clinical Trial of a Therapy for Beta-Thalassemia

Project Summary

Therapy	Gene-corrected, autologous blood stem cell transplant
Indication	Patients with transfusion-dependent beta-thalassemia
Goal	Complete phase 1/2 trial to assess safety, activity, and efficacy of the therapy
Funds Requested	\$8,000,000 (\$14,993,120 Co-funding)

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

CLIN2-11031: Phase 1/2 Clinical Trial of a Therapy for Beta-Thalassemia

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: \$8,000,000*

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