SOMETHING BETTER THAN HOPE

Right now.

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California Institute for Regenerative Medicine

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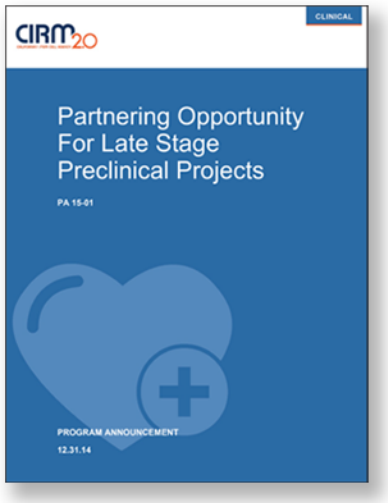
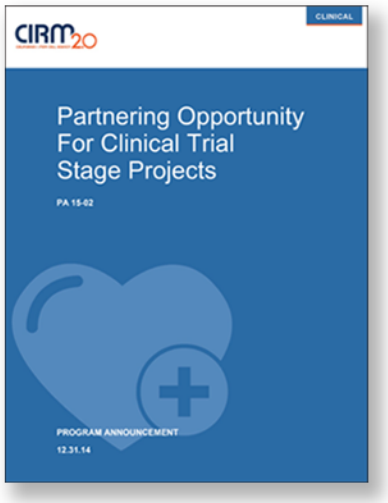
CIRM
CALIFORNIA'S STEM CELL AGENCY

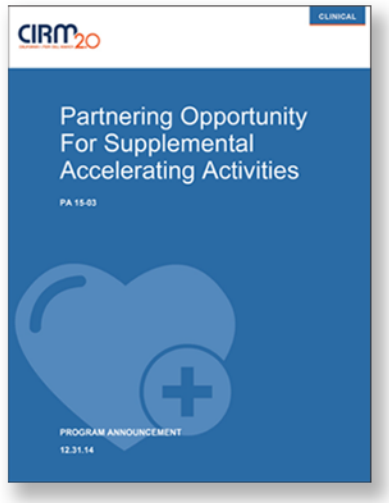
Every Moment Counts. **Don't Stop** Now.

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.

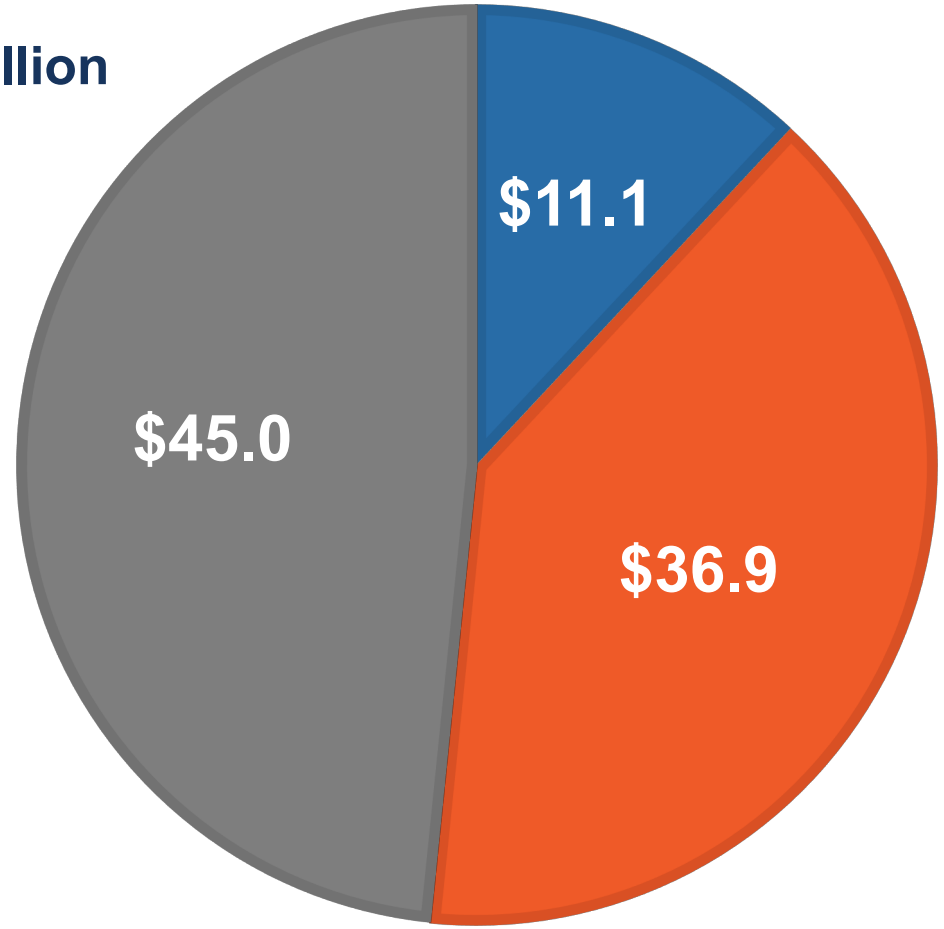
- **Score of "3"**

  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.

2019 Clinical Budget Status

Annual Allocation: $93 million

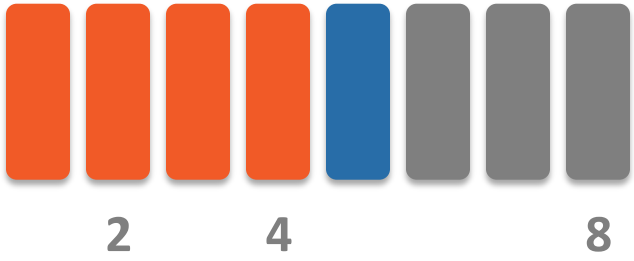


- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions

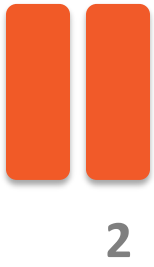
2019 Clinical Award Targets

CLIN2
Clinical Trials



GOAL

CLIN1
Late Stage Preclinical



GOAL

Approved Award

Awaiting Today's Approval

CLIN2-11437: Project Summary

Therapy	Allogeneic pancreatic islets and parathyroid gland (PTG) combination graft
Indication	Type 1 diabetes (T1D)
Goal	Phase 1/2a trial completion
Funds Requested	$11,083,012 ($0 Co-funding)

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

CLIN2-11437: Background Information

Clinical Background: T1D is a chronic disease affecting approximately 1.25M Americans, and 40,000 are newly diagnosed each year. Autoimmune destruction of pancreatic beta cells results in lack of insulin hormone production and blood sugar control in T1D patients. T1D causes disabling and life-threatening complications such as retinopathy, neuropathy, nephropathy and cardiovascular disease.

Value Proposition of Proposed Therapy: There is no cure for T1D, the disease is chronically managed with blood sugar monitoring and insulin therapy. Allogeneic islet transplantation into the liver portal vein can achieve insulin independence but has a high failure rate and is currently an experimental therapy in the US. The proposed therapy aims to address graft failure by implanting islets intramuscularly along with PTG tissue to improve engraftment and survival.

Why a stem cell project: The therapy includes CD34+ progenitor cells and also induces angiogenesis.

Sources: JDRF, NIDDK, CDC

CLIN2-11437: Related CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	Phase 1/2a	N/A	T1D	Allogeneic islets and parathyroid glands	PTG co-transplant improves engraftment and survival of insulin secreting islets
CLIN2	Phase 2	07/2020	T1D	Expanded autologous regulatory T cells	Tregs dampen autoimmune attack on patient's beta cells
CLIN2	Phase 1/2	01/2021	T1D	Allogeneic ESC-derived pancreatic progenitors in encapsulation device	Encapsulated cells mature and secrete insulin

CLIN2-11437: Previous CIRM Funding

Applicant has not received previous funding from CIRM for development of the proposed therapy.

CLIN2-11437: GWG Review

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	13
2	2
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: $11,083,012*

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