

SOMETHING BETTER THAN HOPE

Right now.

Shyam Patel, Ph.D.

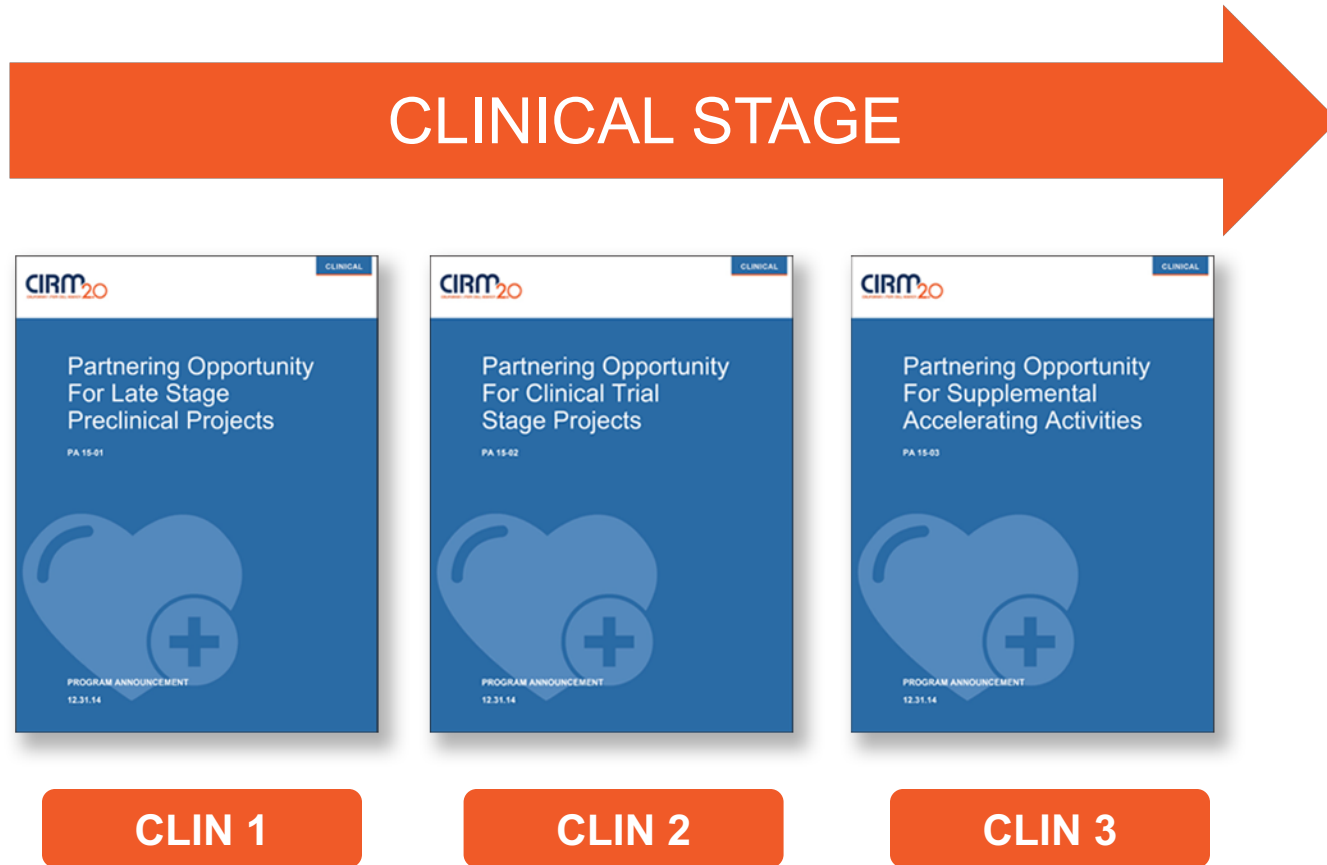
Associate Director, Portfolio
Development & Review
California Institute for Regenerative
Medicine

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

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.***

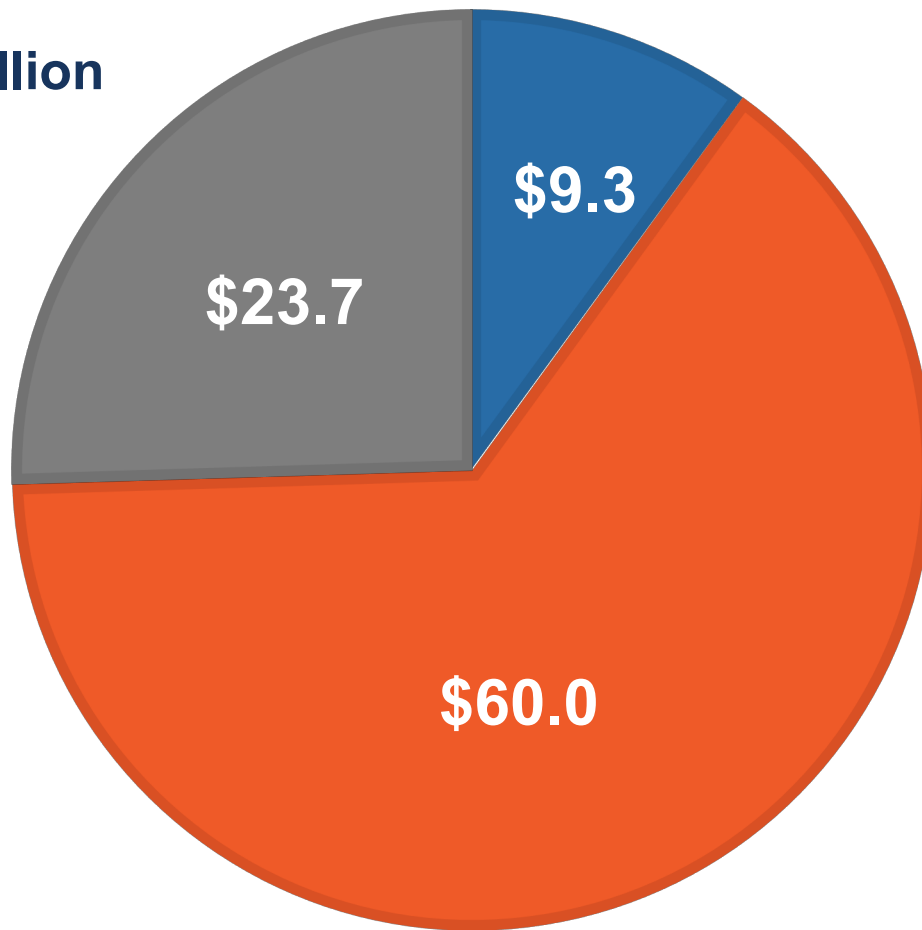
Applications are scored by all scientific members of the GWG with no conflict.

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



CLIN1
Late Stage
Preclinical



 Approved Award  Awaiting Today's Approval

CLIN2-11574: Project Summary

Therapy	Autologous HER2 CAR-engineered naïve-stem/memory T cells
Indication	HER2+ breast cancer central nervous system (CNS) metastasis
Goal	Phase 1 trial completion
Funds Requested	\$9,288,375 (\$0 Co-funding)

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

CLIN2-11574: Background Information

Clinical Background: Breast cancer is the most common cancer in women; 1 in 8 women will develop it during her lifetime. HER2+ cancer represents 20-25% of breast cancer cases and is highly metastatic with up to 50% of these patients developing CNS tumors. While CNS metastases are treated with a combination of surgery, radiation, chemotherapy and immunotherapy the prognosis and quality of life for these patients remain very poor.

Value Proposition of Proposed Therapy: HER2-targeted immunotherapy is only effective for extra-cranial metastases. By delivering HER2 CAR engineered naïve-stem/memory T cells into the CNS, the proposed therapy is designed to have a localized and sustained anti-tumor immunotherapy response. It has the potential to greatly improve survival and quality of life.

Why a stem cell project: The therapy includes gene-modified naïve and stem memory T cells.

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CLIN2-11574: Related CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	Phase 1	N/A	HER2+ CNS metastases	HER2 CAR- engineered Tn/mem cells	T cell-mediated elimination of HER2+ tumor cells
CLIN2	Phase 1	11/2021	Malignant Glioma	IL13R α 2 CAR- engineered Tcm or Tn/mem cells	T cell-mediated elimination of IL13R α 2+ tumor cells

CLIN2-11574: Previous CIRM Funding

Project Stage	Project Outcome	Project Duration	Award Amount	Milestones*
Discovery	Closed	03/01/13 - 08/31/16	\$5.22M (\$5.22M issued to date)	<p>OM1: IL13Ra2 CAR T cell generation (Achieved on time)</p> <p>OM2: In vivo activity of IL13 CAR-T cells (Achieved on time)</p> <p>OM3: Assess IL13Ra2, HER2, EGFR antigens in glioblastoma (Achieved on time)</p> <p>OM4: In vivo efficacy of CAR-T cell combinations (Achieved with minor delays)</p>

CLIN2-11574: GWG Review

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	11
2	4
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$9,288,375*

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