

Real Life™

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Grants Working Group Recommendations CLIN

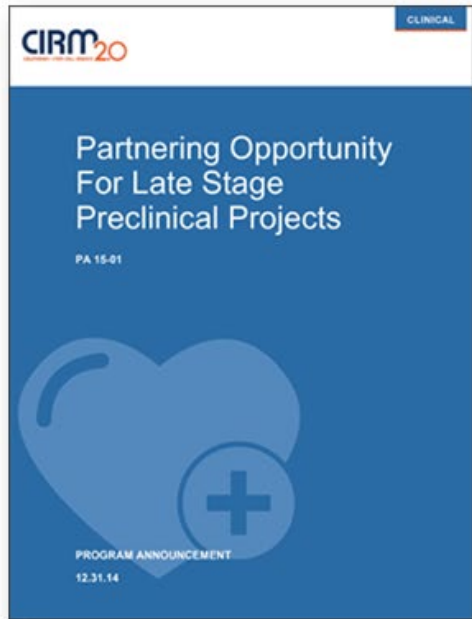
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CIRM
CALIFORNIA'S STEM CELL AGENCY

OUR MISSION

Accelerating world class science
to deliver transformative
regenerative medicine treatments
in an equitable manner to a
diverse California and world





CLIN 1



CLIN 2

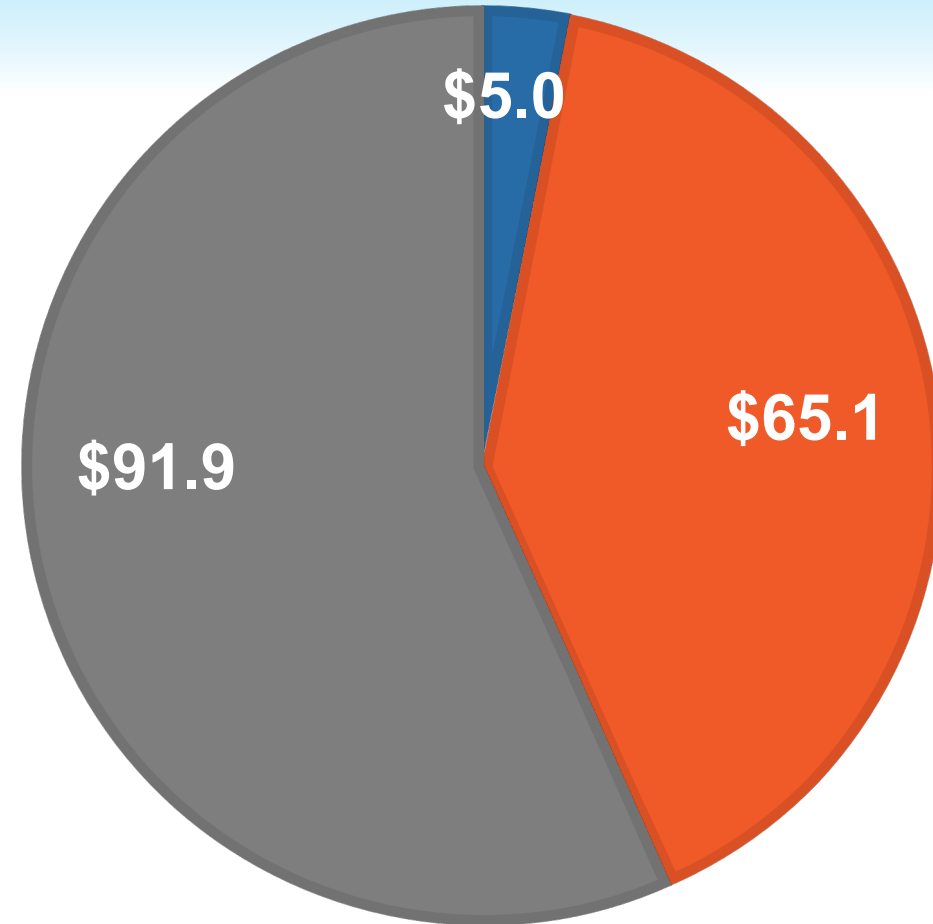


CLIN 3

Annual Allocation: \$162 million

- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



- **Score of “1”**

Exceptional merit and warrants funding.

May have minor recommendations and adjustments that do not require further review by the GWG

- **Score of “2”**

Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.

GWG should provide recommendations that are achievable (i.e., “fixable changes”) or request clarification/information on key concerns.

- **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.

1. Does the project hold the necessary significance and potential for impact? (i.e., what value does it offer; is it worth doing?)
2. Is the rationale sound? (i.e., does it make sense?)
3. Is the project well planned and designed?
4. Is the project feasible? (i.e., can they do it?)
5. Does the project address the needs of underserved communities?

Scientific GWG
Member



Scientific evaluation (disease area expert,
regulatory, CMC, product development)
Provides scientific score on all applications

Patient Advocate
or Nurse GWG
Member



DEI evaluation, patient perspective on significance
and potential impact, oversight on process
Provides DEI score on all applications
Provides a suggested scientific score

Scientific
Specialist
(non-voting)



Scientific evaluation (specialized expertise as
needed)
Provides initial but not final scientific score

Title	Skin regeneration and wound healing with a topical BRAF inhibitor
Therapy	Small molecule BRAF inhibitor
Indication	Venus leg ulcers
Goal	Completion of IND-enabling studies and IND submission
Funds Requested	\$5,005,126 (co-funding: \$0)

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

Clinical Background: Venous leg ulcers are sores on the leg that develop from chronic suboptimal blood flow and are very slow to heal, lasting weeks to years.

Value Proposition of Proposed Therapy: The current standard of care options are compression bandage or stocking and wound management with the goal of improving local blood circulation and healing. There are no topical or systemic drug treatments available for this condition. If successful, the proposed therapy would provide an easy to administer topical drug treatment that would accelerate the healing process.

Why a stem cell or gene therapy project: The therapeutic candidate is a small molecule that acts on epidermal stem cells.

There are currently no active CIRM awards targeting this disease indication.

Previous CIRM Funding to Applicant Team

Project Stage	Indication	Project Outcome	Project Duration	Award Amount	Milestones/Aims
Preclinical and clinical	Metastatic melanoma	Complete IND-enabling activities and initiate phase clinical 1 trial	Apr 2014 – Nov 2020	\$19,999,563	<p>M1-M6: Manufacture of product, studies of safety and efficacy (Achieved on time)</p> <p>M7: Submit IND to the FDA (Achieved on time)</p> <p>M8: Enroll first patient (Achieved on time)</p>
Preclinical	Melanoma	Develop a T cell therapeutic candidate	Jan 2009 – Dec 2013	\$3,072,000	<p>M1: Develop candidate for adoptive Tc ell therapy (Achieved on time)</p> <p>M2: Develop stem cell candidate for immune reconstitution (Completed with delay)</p>

GWG Recommendation: Exceptional merit and warrants funding

Scientific Score	GWG Votes
1	12
2	2
3	0

DEI Score: 7 (scale 1-10)

CIRM Team Recommendation: Fund (concur with GWG recommendation)

CIRM Award Amount: \$ 5,005,126*

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