

GWG Recommendations: Clinical Program (CLIN1, CLIN2, CLIN4)

Hayley Lam, PhD

Director, Portfolio Development and
Review

ARS Meeting

April 24, 2025



Our Mission

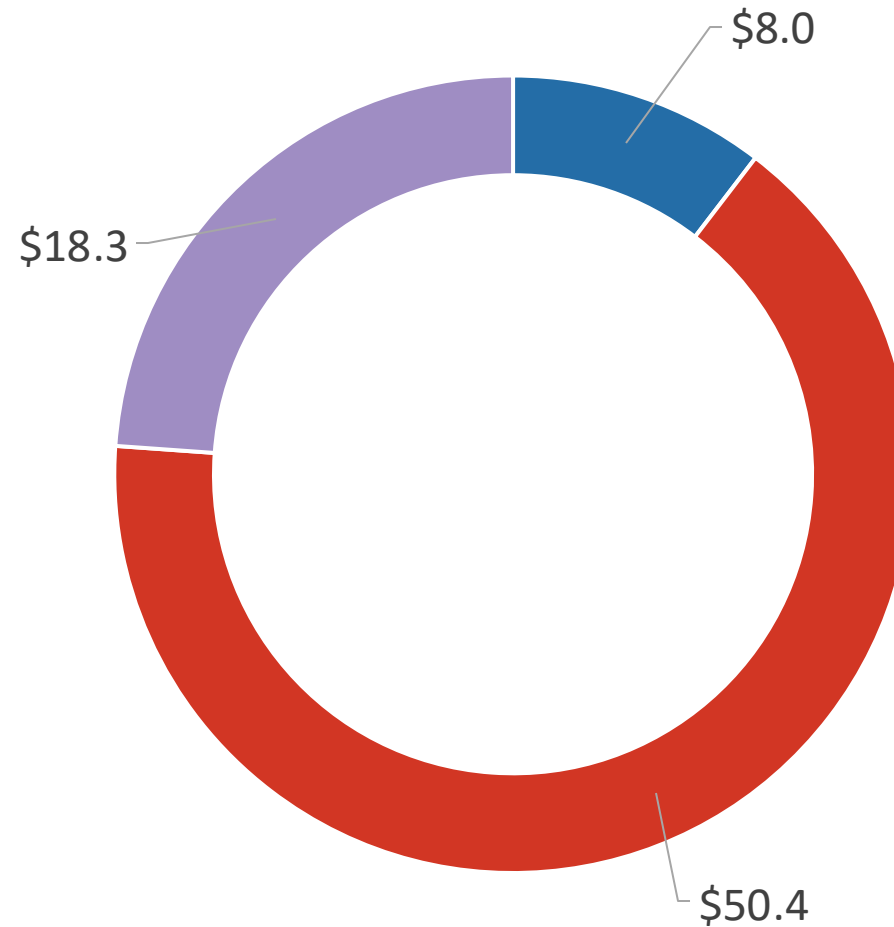
Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world.



Clinical Budget Status

Budget Allocation: \$76.7 million
(Jan to June 2025)

- Amount Requested Today
- Approved Awards
- Unused Balance



Clinical

Scientific Scoring System

- **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.

Clinical



Scientific Review Criteria

1. Does the project hold the necessary significance and potential for impact? (*what value does it offer; is it worth doing?*)
2. Is the rationale sound? (*does it make sense?*)
3. Is the project well planned and designed?
4. Is the project feasible? (*can they do it?*)
5. Does the project uphold principles of diversity, equity, and inclusion (DEI)? (*e.g., does it consider patient diversity?*)

Clinical

Diversity, Equity and Inclusion Scoring System

- DEI Score of 9-10: *Outstanding Response*
- DEI Score of 6-8: *Responsive*
- DEI Score of 3-5: *Not Fully Responsive*
- DEI Score of 0-2: *Not Responsive*

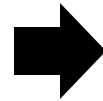
Applications are scored for adherence to principles of DEI by all GWG Board Members with no conflict.

The criteria used to measure adherence fall under overarching categories of: Commitment to DEI, Project Plans and Cultural Sensitivity.

Clinical



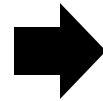
Scientific GWG
Members



Scientific evaluation (disease area expert, regulatory, CMC, product development)

Provides scientific score on all applications

GWG Board
Member
(Patient Advocate/Nurse)

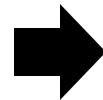


DEI evaluation, patient perspective on significance and potential impact, oversight on process

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

CLIN2-17086

Optogenetic Gene Therapy for
Treatment of Retinitis Pigmentosa

FUNDS REQUESTED

\$7,975,224

Co-funding: \$3,417,953 (30%
required)

California organization



THERAPY

Optogenetic gene therapy



INDICATION

Retinitis Pigmentosa



GOAL

Complete Phase 1 trial

CLIN2-17086 Background Information

Clinical background

Retinitis Pigmentosa (RP) is a rare inherited genetic disease where there is progressive loss of light sensitive cells in the retina, resulting in vision loss and blindness. RP impacts daily activities, such as the person's ability to work and drive. There are no current treatment options for advanced RP except for a small subset (~1%) of people with a specific gene mutation.

Value proposition of proposed therapy

The product could improve the visual function of people with RP by delivering a light sensitive gene to retinal cells, allowing the surviving cells to detect light and transmit vision signals to the brain. This approach is independent of the underlying genetic mutation and therefore could target a broad patient population.

Why a stem cell or gene therapy project

The therapy is a gene therapy product.

CLIN2-17086 Similar CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
CLIN2 \$10,444,063	Phase 1/2a Clinical Trial	Q3 2026	Retinitis Pigmentosa	Allogeneic human neural progenitor cells injected subretinally	The injected cells clear the area of debris, release factors to inhibit photoreceptor cell death and modulate inflammation.

CLIN2-17086 Previous CIRM Funding to Applicant Team

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
TRAN1 \$3,999,553	Preclinical	Aug 2023	Retinitis Pigmentosa	Optogenetic gene therapy	Light sensing genes are delivered to the retinal cells, enabling them to detect light and transmit vision signals to the brain.
TRAN1 \$3,998,930	Preclinical	April 2025	Geographic Atrophy	Optogenetic gene therapy	Light sensing genes are delivered to the retinal cells, enabling them to detect light and transmit vision signals to the brain.

CLIN2-17086

GWG Review

Optogenetic Gene Therapy for
Treatment of Retinitis Pigmentosa

CIRM Award Amount:
\$7,975,224*

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

GWG RECOMMENDATION

Exceptional merit and warrants funding

Scientific Score	GWG Votes
1	15
2	0
3	0

DEI SCORE

7 (scale 1-10)

CIRM TEAM RECOMMENDATION

Fund (concur with GWG recommendation)

C I R M

CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Thank You



CONTACT US

(510) 340-9101

INFO@CIRM.CA.GOV

601 GATEWAY BLVD, SUITE 400
SOUTH SAN FRANCISCO, CA 94080

CIRM.CA.GOV