

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



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Director, Portfolio Development and
Review

ARS Meeting

May 29, 2025

Our Mission

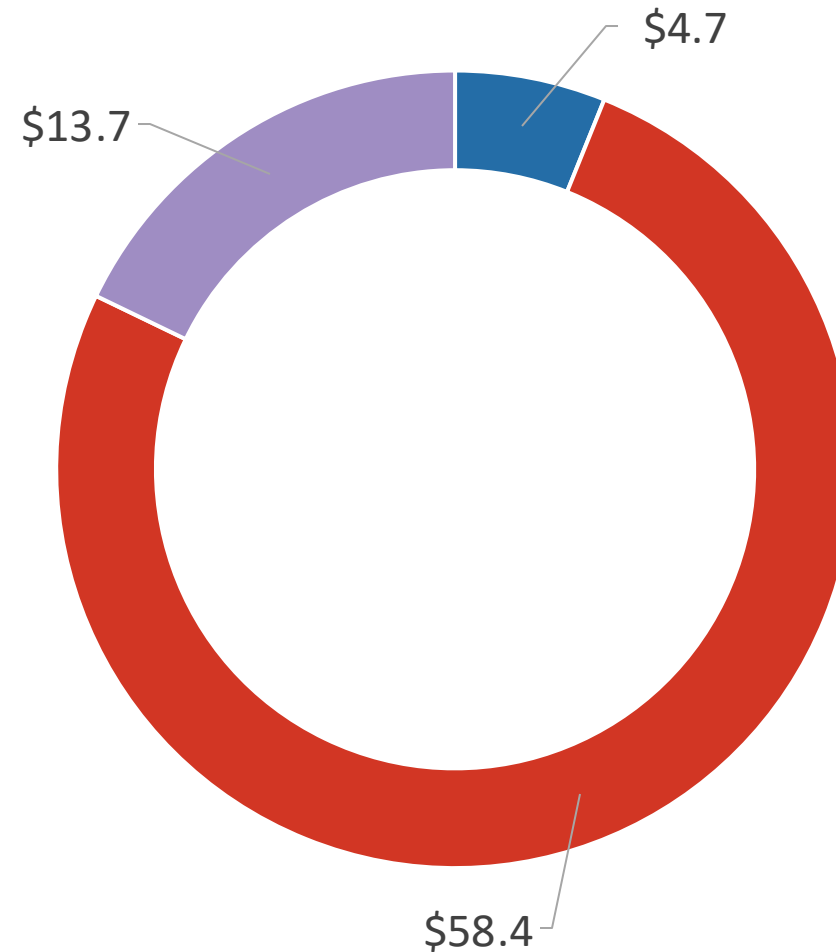
Accelerating world class science
to deliver transformative
regenerative medicine treatments
in an equitable manner to a
diverse California and world.



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.

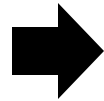
Clinical

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?*)

Clinical

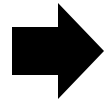
Scientific GWG
Members



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

Provides scientific score on all applications

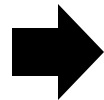
GWG Board
Member
(Patient Advocate/Nurse)



Patient perspective on significance and potential impact, oversight on process

Provides a suggested scientific score

Scientific
Specialist
(non-voting)



Scientific evaluation (specialized expertise as needed)

Provides initial but not final scientific score

CLIN1-17090

Development of a novel intravitreal AAV gene therapy for the rare disease blue cone monochromacy

FUNDS REQUESTED

\$4,691,936

Co-funding: \$0 (none required)

California organization



THERAPY

Retinal gene therapy



INDICATION

Blue cone monochromacy



GOAL

IND approval

CLIN1-17090 Background Information

Clinical background

Blue cone monochromacy (BCM) is a rare inherited disease where gene mutations impair the function of red and green photoreceptors in the retina. BCM is usually diagnosed in infancy. BCM impacts the quality of life of people with the condition, causing low vision, photosensitivity, impaired color discrimination, and uncontrolled eye movement.

Value proposition of proposed therapy

Current treatments manage symptoms only. The proposed therapy is designed as a one-time injection into the eye. The gene therapy aims to express a functional copy of L-opsin gene in the cone photoreceptor cells to restore visual function.

Why a stem cell or gene therapy project

The therapy is a gene therapy product.

CLIN1-17090 Similar CIRM Portfolio Projects

CIRM does not currently have any active TRAN or CLIN awards addressing blue cone monochromacy.

CLIN1-17090 Previous CIRM Funding to Applicant Team

Applicant has not previously received a CIRM award

CLIN1-17090 GWG Review

Development of a novel intravitreal AAV gene therapy for the rare disease blue cone monochromacy

CIRM Award Amount:
\$4,691,936*

*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

CIRM TEAM RECOMMENDATION

Fund (concur with GWG recommendation)



Thank You



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