

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

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Development and Review

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Our Mission

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

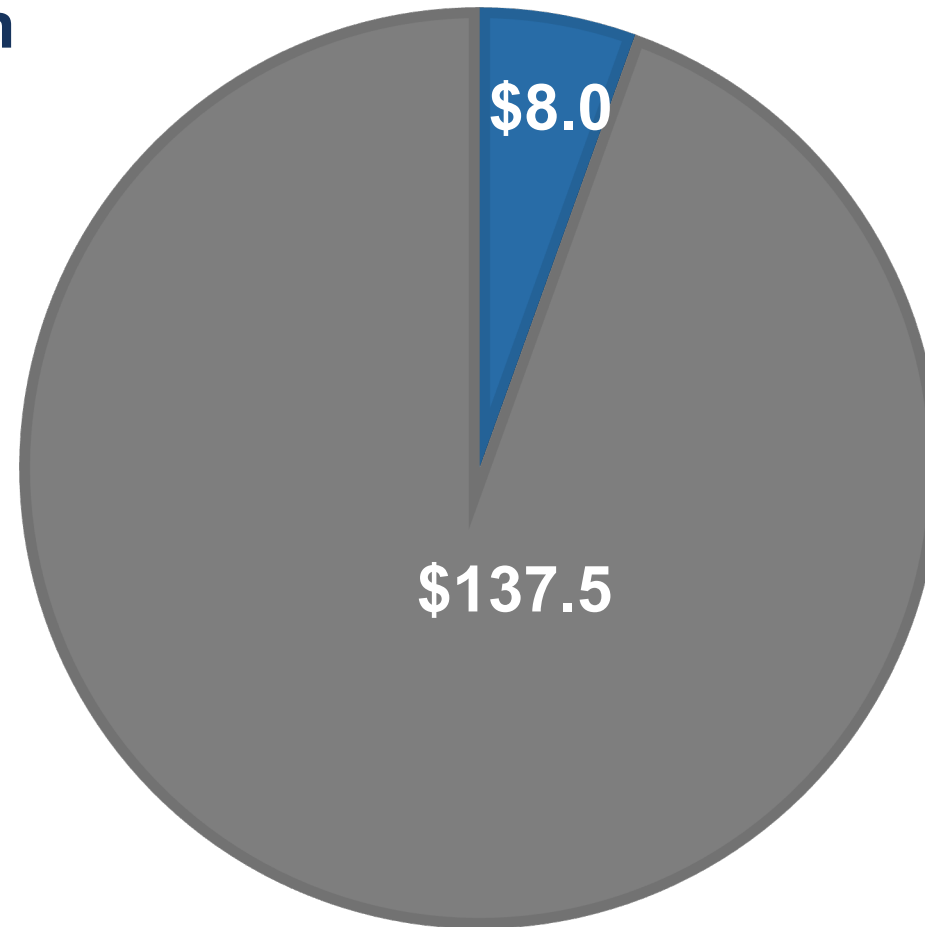


2024/25 Clinical Budget Status

**Annual Allocation: \$ 145.5 million
(July to December 2024)**

- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



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.

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?*)

CLIN DEI Scoring

CIRM CLIN Program DEI Rubric				
CRITERIA	Score of 0 to 2	Score of 3 to 5	Score of 6 to 8	Score of 9 to 10
	Not Responsive	Not Fully Responsive	Responsive	Outstanding Response
1. Commitment to DEI	Fails to address how success of this project would lead to a therapy that positively impacts underserved or disproportionately affected communities.	Inadequately addresses how success of this project would lead to a therapy that positively impacts underserved or disproportionately affected communities.	Adequately describes how success of this project would likely lead to a therapy that positively impacts underserved or disproportionately affected communities.	Convincingly and clearly describes how success of this project would lead to a therapy that positively impacts underserved or disproportionately affected communities.
	Does not set goals for diverse trial population enrollment and provides no justification for the target enrollment.	May set trial population enrollment goals that are inappropriate or infeasible relative to the population affected or at risk for the indication.	Sets adequate goals for trial population enrollment relative to the population affected or at risk for the indication.	Trial population goals are based on a deep understanding of health disparities and disease burden.
	Inadequate personnel/expertise or budget to implement DEI-oriented activities.	May have inadequate personnel/expertise or budget to implement DEI-oriented activities.	Adequate personnel/expertise or budget to implement DEI-oriented activities.	Strong personnel/expertise and appropriate budget to implement DEI-oriented activities.
2. Project Plans	Planned activities do not reflect a good faith effort and are unlikely to be effective in outreach and engagement.	Planned activities are incomplete or inadequate and may not reflect a good faith effort for outreach and engagement.	Planned activities reflect a good faith effort and have the potential to be effective in outreach and engagement.	Planned activities reflect an outstanding and comprehensive effort for outreach and engagement.
	Does not demonstrate an understanding of the potential barriers to participation in the clinical trial.	Does not fully demonstrate an understanding of the potential barriers to participation in the clinical trial.	Demonstrates an understanding of the potential barriers to participation in the clinical trial.	Demonstrates a clear understanding of the potential barriers to participation in the clinical trial.
	Inadequate plan to address potential barriers to participation.	May not have an adequate plan to address potential barriers to participation.	Has an adequate plan to address potential barriers to participation.	Has a strong plan to address potential barriers to participation.
	Unlikely to achieve the recruitment of trial participants from underserved or disproportionately affected populations.	May not be able to achieve the recruitment of trial participants from underserved or disproportionately affected populations.	Likely to achieve the recruitment of trial participants from underserved or disproportionately affected populations.	Very likely to achieve the recruitment of trial participants from underserved or disproportionately affected populations.
3. Cultural Sensitivity	Does not include activities to increase cultural sensitivity on the team or at partner institutions, or activities proposed are not appropriate.	Proposed activities may not be effective or sufficient to increase cultural sensitivity on the team or at partner institutions. Activities may not match the needs of the project.	Has appropriate plans to increase cultural sensitivity on the team or at partner institutions. Activities match the needs of the project.	Outstanding plans to increase cultural sensitivity on the team or at partner institutions. Activities are well matched to the needs of the project.

DEI Scores

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

- DEI Score of 9-10

Outstanding Response

- DEI Score of 6-8

Responsive

- DEI Score of 3-5

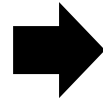
Not Fully Responsive

- DEI Score of 0-2

Not Responsive

GWG Composition and Roles

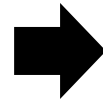
Scientific GWG
Member



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

Provides scientific score on all applications

GWG Board
Member
(Patient
Advocate/Nurse)

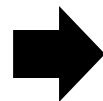


DEI evaluation, patient perspective on significance
and potential impact, oversight on process

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

CLIN2-15901

Title	Autologous BCMA CAR-T Cells for the Treatment of Relapsed Refractory Light Chain Amyloidosis
Therapy	Autologous BCMA CAR-T Cells
Indication	Amyloid light-chain amyloidosis
Goal	Phase 1 trial completed
Funds Requested	\$7,999,467 Co-funding: \$5,332,978 (40% required) California organization

Maximum funds allowable for this category: \$15,000,000

CLIN2-15901: Background Information

Clinical Background: Amyloid light-chain amyloidosis (AL amyloidosis) is a rare plasma cell disorder where an immune system protein is overproduced and accumulates in various organs of the body. Eventually, this can lead to multi-organ failure and death. In the United States, close to 4,000 people are diagnosed each year, and African Americans have a two-fold higher rate of occurrence as compared to whites.

Value Proposition of Proposed Therapy: No cures for AL amyloidosis exist. The current standard of care usually results in eventual progression of the disease. The proposed therapy targets and destroys the dysfunctional plasma cells and is intended to be a one-time treatment.

Why a stem cell or gene therapy project: The therapy comprises genetically modified autologous immune cells.

CLIN2-15901: Similar CIRM Portfolio Projects

CIRM does not currently have any active TRAN or CLIN awards addressing amyloid light-chain amyloidosis.

CLIN2-15901: Previous CIRM Funding to Applicant Team

Applicant has not previously received a CIRM award.

CLIN2-15901: GWG Review

GWG Recommendation: Exceptional merit and warrants funding

Scientific Score	GWG Votes
1	14
2	0
3	0

DEI Score: 7 (scale 1-10)

CIRM Team Recommendation: Fund (concur with GWG recommendation)

CIRM Award Amount: \$ 7,999,467*

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