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Vice President, Portfolio Development and Review Grants Working Group DISC0 Recommendations September 20, 2022





Mission Statement



OUR MISSION

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





Recurring Programs









CIRM's DISC0 Purpose & Objectives

- Broadly re-initiate funding of basic stem/progenitor ("stem") cell science and genetic research.
- Support rigorous studies addressing critical basic knowledge gaps or bottlenecks in regenerative medicine research.
- Advance the development of stem cell-based tools for innovation.





DISCO Proposal Objective

Projects should culminate in a discovery or technology that would advance (1 or more):

- Understanding of biology of stem cells that is relevant to human biology and disease,
- Genetic research relevant to human biology or disease that pertains to stem cells or regenerative medicine,
- The development of human stem cells as tools for biomedical innovation, and/or
- Greater applicability of regenerative medicine discoveries to communities representing the full spectrum of diversity.





DISC0 funds can support the following **EXAMPLE** activities

- Basic research into stem cell or genetic research mechanisms as they relate to human biology.
- Basic research to address bottlenecks such as tissue targeting, immunogenicity or toxicity in the development of stem cell-based therapies.
- Studies to better understand healthy and/or diseased human cells and tissues such as omics profiling or human cell / tissue atlases.
- Mechanistic studies of disease to enable rational design of stem cell-based treatments.
- Investigation of stem cells or their derivatives as tools for therapeutic or other innovation, e.g., for modeling disease.
- Generation of omics data that will extend or validate the applicability of regenerative medicine discoveries to underserved racial or ethnic groups.





- Applications must address a knowledge gap or a bottleneck in regenerative medicine research.
- Awards are for 3 years and up to \$1 million in direct project costs
- Opportunity planned to occur every 6 months



Positive Selection (Two-Stage) Review Process



- Performed when the total number of applications exceeds the capacity of the GWG to review in a single session.
- In the first stage, GWG members including patient advocates and nurse Board Members conduct a pre-review of applications and select which ones to advance to a full review.
- The CIRM President and CIRM will examine non-selected applications to determine if any merit a full review. The remainder are not considered further.
- A total of 75 eligible applications were submitted and a total of <u>45</u> advanced to the full discussion stage by GWG.



GWG Composition and Roles



Scientific GWG Members



Positive selection

Scientific evaluation based on broad subject area or methods expertise

Enter final scores for every application

GWG Board Members



Positive selection

Patient perspective on significance and potential impact, oversight on process

Visiting Specialists



Scientific evaluation based on specialized expertise

Provide recommended scores in the discussion



Scoring System for DISC0 Applications



Score of "85-100"

Recommended for funding, if funds are available

Score of "1-84"

Not recommended for funding

Applications receiving a score of **80-84** in this review cycle were deemed by the GWG to have sufficient merit to bypass the positive selection process and advance to full scientific review if resubmitted in the next review cycle

Score of "1-79"

Not recommended for funding

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

The **median** of all individual GWG scores determines final score.





- 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 uphold the principles of diversity, equity and inclusion (DEI)?



GWG Recommendations Summary



	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	11	\$15,595,795	\$33,923,570
Not recommended for funding Score 1-84	34		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.

CIRM Minority Reports



- Under Prop 14, any application that is not recommended for funding by the GWG, but which had 35% or more members score to fund the application must include a minority report.
- The minority report is included in the review summary and provides a brief synopsis of the opinion of reviewers that scored the application 85 or above.

No applications qualified for a Minority Report in this cycle.



Board members with Conflicts of Interest



Board members with Conflicts of Interest for DISC 0 applications		
Haifaa Abdulhaq	Jim Kovach	
Loren Alving	Linda Malkas	
Kim Barrett	Shlomo Melmed	
George Blumenthal	*Christine Miaskowski	
Michael Botchan	Suzanne Sandmeyer	
Linda Boxer	Barry Selick	
Carol Christ	Michael Stamos	
*Ysabel Duron	*Art Torres	
Leon Fine	Kristiina Vuori	
*Elena Flowers	*Karol Watson	
Judy Gasson	Keith Yamamoto	
Larry Goldstein		

^{*} Application Review Subcommittee members