

# Grants Working Group Recommendations DISC2 (Quest) Review

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Review

September 26, 2024



# Our Mission

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

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# Discovery

## DISC2 (Quest), Objective

To promote the discovery of promising new stem cell-based and genetic therapeutic candidates that can be translated for clinical use, OR to promote the discovery of promising new biomarker candidates that can be translated for clinical use, particularly in drug development

## DISC2 (Quest), Scope:

Projects funded through the Quest Awards must propose a therapeutic or biomarker candidate that is uniquely enabled by human stem/progenitor cells or directly reprogrammed cells, or uniquely enabling for the advancement of stem cell-based therapies or genetic therapies

# Discovery

## DISC2 (Quest), What's New?

- Maximum direct project costs for a therapeutic candidate have increased to \$1,750,000, and the optional budget supplement removed.
- CIRM has added a **biomarker candidate track** with maximum direct project costs of \$1,500,000.
- DISC2 awards no longer fund *stand-alone* medical devices, tools or platform technologies.
- Review criteria and application components have been revised, with additions for the biomarker candidate track.

# Discovery

## DISC2 (Quest), Therapeutic Track:

\$1,750,000 direct costs, projects up to 3 years

### Expected outcomes by conclusion of award

- Single candidate ready for translation (TRAN1 readiness or equivalent)
- A draft Target Product Profile
- Reproducible demonstration of disease modifying activity with candidate

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## DISC2 (Quest), Biomarker Track:

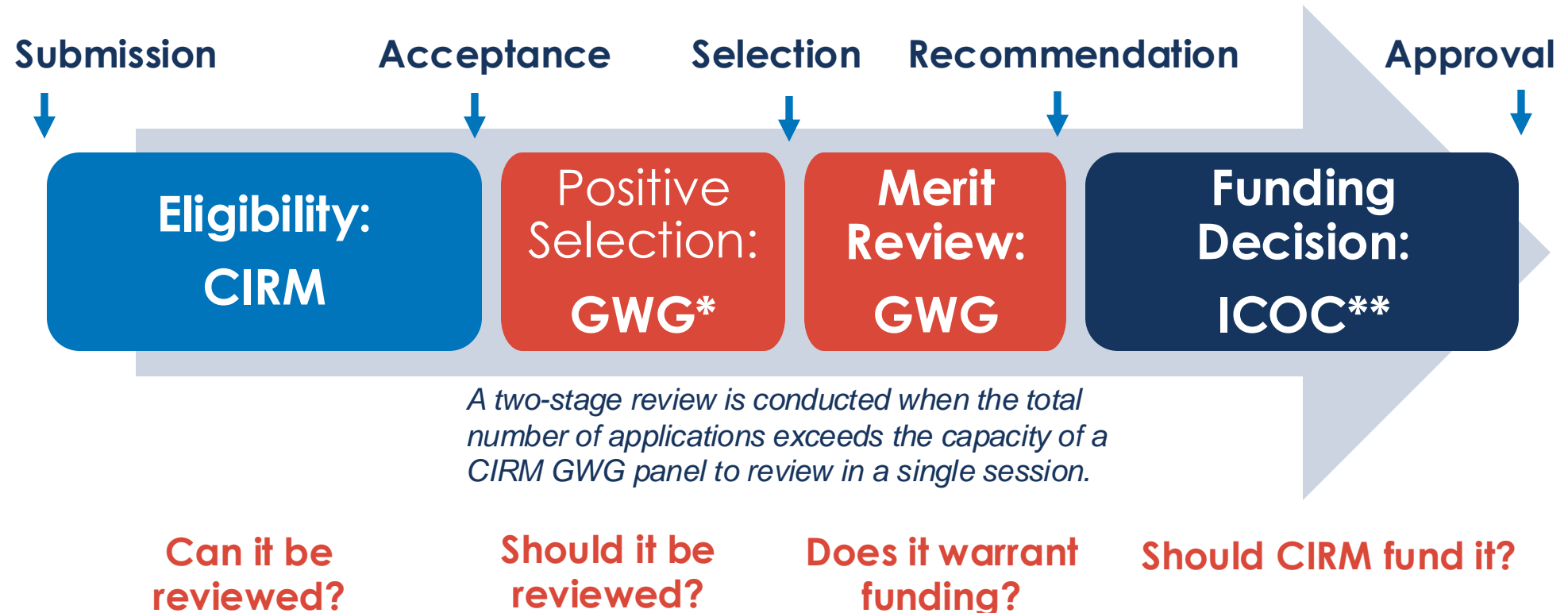
\$1,500,000 direct costs, projects up to 3 years

### Expected outcomes by conclusion of award

- Selection of a biomarker candidate and specification of context of use (COU)
- A draft Biomarker Candidate Profile
- Selection of an analytical method for biomarker assessment with basic measures of performance and reproducibility
- Demonstration that the biomarker can be measured at biologically relevant levels
- Initial proof-of-concept studies using relevant clinical data or clinical samples supporting relevance of the biomarker

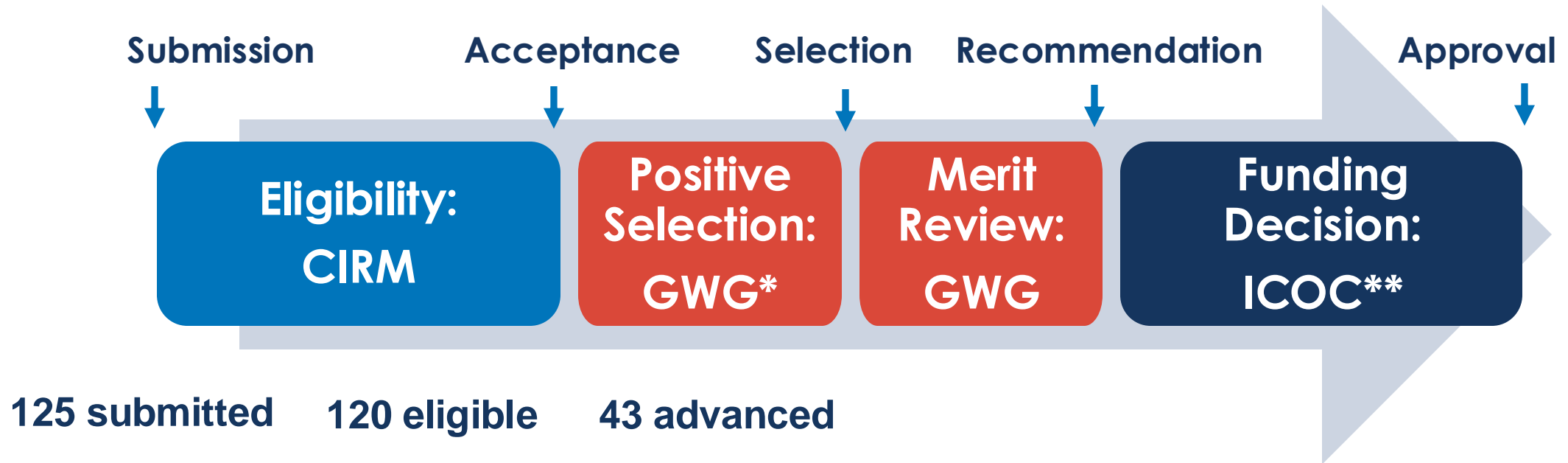
# Discovery

## DISC2 (Quest), Application Review Process



# Discovery

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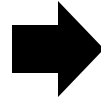
\*Grants Working Group \*\*Independent Citizens Oversight Committee



# Discovery



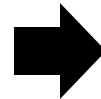
**Scientific GWG  
Members**



Scientific evaluation based on broad subject area or  
methods expertise

Enter final scores for **every** application

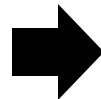
**GWG Board  
Members**



Patient perspective on significance and potential  
impact, oversight on process

Do not enter final scores; may recommend scores

**Specialists**



Scientific evaluation based on specialized expertise

Provide recommended scores during discussion

## Review Panel Roles

# Discovery

## DISC2 (Quest), 1 to 100 Scoring

- Score of **85 to 100**: Application has exceptional merit and warrants funding.
- Score of **1 to 84**: Application is not recommended for funding.
- Score of **80 to 84**: Application has exceptional merit and requires only minor revisions. If resubmitted in a future DISC2 competition, this application can *bypass* positive selection and automatically undergo full scientific review.

# Discovery

## DISC2 (Quest), Scoring Criteria

1. Does the project hold the necessary significance and potential for impact? (i.e., 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 the applicant do it?)
5. Does the project uphold principles of diversity, equity and inclusion?

## GWG Recommendations

Score	Number of Apps	Total Applicant Request	Funds Available
<b>Recommended for funding</b> Score of 85-100	9	\$22,455,687	\$28,000,000
<b>Not recommended for funding</b> Score of 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.

## ■ 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.

## ■ Applications with Minority Report

App Number	Title	Funds Requested	Score
<b>DISC2-16686</b>	Development of iPSC-derived neural progenitors secreting GDNF for the treatment of ALS	<b>\$2,677,445</b>	81

The CIRM Team supports the majority position to not fund application DISC2-16686.