

Real Life™

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Grants Working Group DISC2 Review

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CIRM
CALIFORNIA'S STEM CELL AGENCY

OUR MISSION

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





DISCOVERY



TRANSLATION



CLINICAL

New
Idea



Single Product
Candidate



Pre-IND or
Equivalent



Approved
Therapy

Objective

The Quest Program will promote the discovery of promising new stem cell-based and gene therapy technologies that could be translated to enable broad use and ultimately, improve patient care.

Projects funded through the Quest Awards should propose technology that is uniquely enabled by human stem/progenitor cells or uniquely enabling for the advancement of stem cell-based therapies or aimed at developing a gene therapy approach.

What are we looking for?

Candidate Type

Therapeutic

Diagnostic/Device/Tool

Expected Outcomes

Single candidate identified

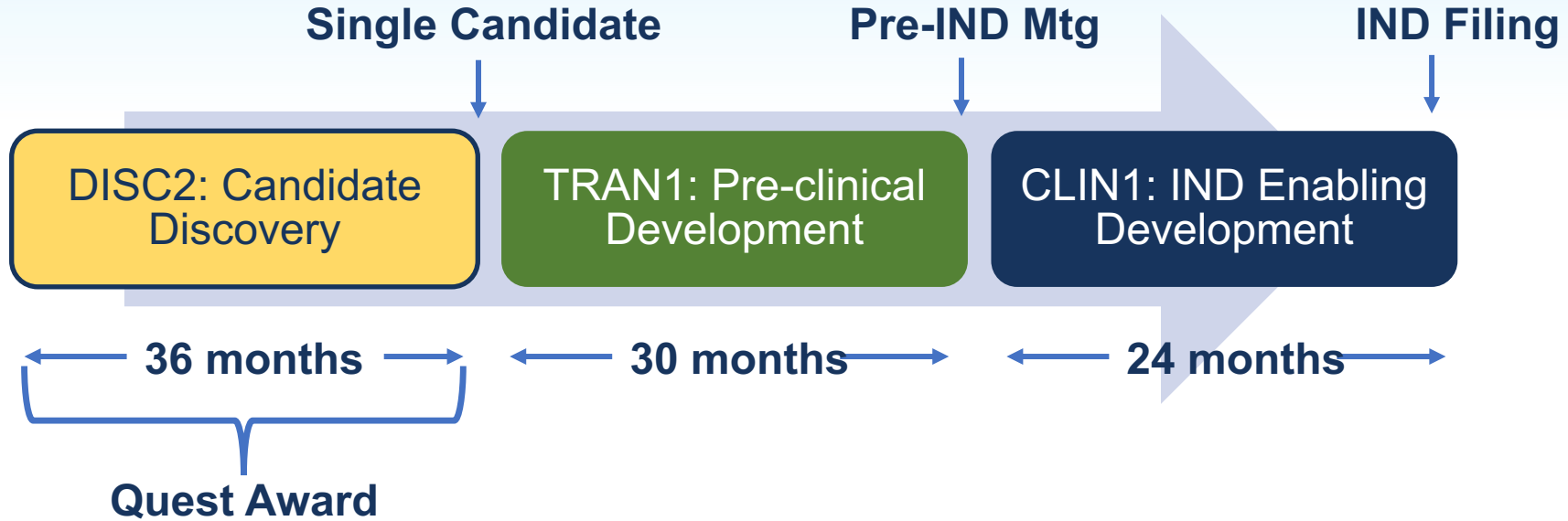
Candidate prototype identified

Target Product Profile drafted

Target Product Profile drafted

Disease modifying activity demonstrated

Scientific proof-of-concept demonstrated



Expected **outcomes** of DISC2 applications:

- A single candidate ready for translational studies identified.
- A Target Product Profile developed.
- Demonstration of disease-modifying activity or proof of concept.

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 89 eligible applications were submitted, 4 bypassed the process with a previous 80-84 score, and a total of 46 advanced to the full discussion stage by GWG.

- **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.

1. 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 address the needs of underserved communities?

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	10	\$17,621,520	\$47,279,576
Not recommended for funding Score 1-84	36		

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.

- 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.

CIRM Team concurs with the GWG recommendations for funding.

In addition, the CIRM Team supports the minority position for applications DISC2-14900 and DISC2-15114 and recommend funding of these applications.

App Number	Title	Funds Requested	Score
DISC2-14900	Developing a breast cancer stem cell drug	\$2,293,051	84
DISC2-15114	Development of a VAV2 antisense oligonucleotide (ASO) treatment for ALS	\$2,296,376	84

Score	Num \geq 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
84	6	8	78-90	\$2,293,051

TITLE: Developing a breast cancer stem cell drug

DISEASE INDICATION: Triple negative breast cancer

PRODUCT TYPE: Small molecule

APPROACH: Develop a drug that specifically targets breast cancer stem cells.

Score	Num \geq 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
84	7	8	70-90	\$2,296,376

TITLE: Development of a VAV2 antisense oligonucleotide (ASO) treatment for ALS

DISEASE INDICATION: Amyotrophic lateral sclerosis (ALS)

PRODUCT TYPE: Gene therapy

APPROACH: Develop a gene therapy that will improve survival of neurons in ALS.