

# Real Life™

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Vice President, Portfolio Development and Review

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



2/Year

Single Product  
Candidate



2/Year

Pre-IND or  
Equivalent



12/Year

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.

## Candidate Type

Therapeutic

Diagnostic/Device/Tool

## Expected Outcomes for a two-year or three-year project

Single candidate identified

Candidate prototype identified

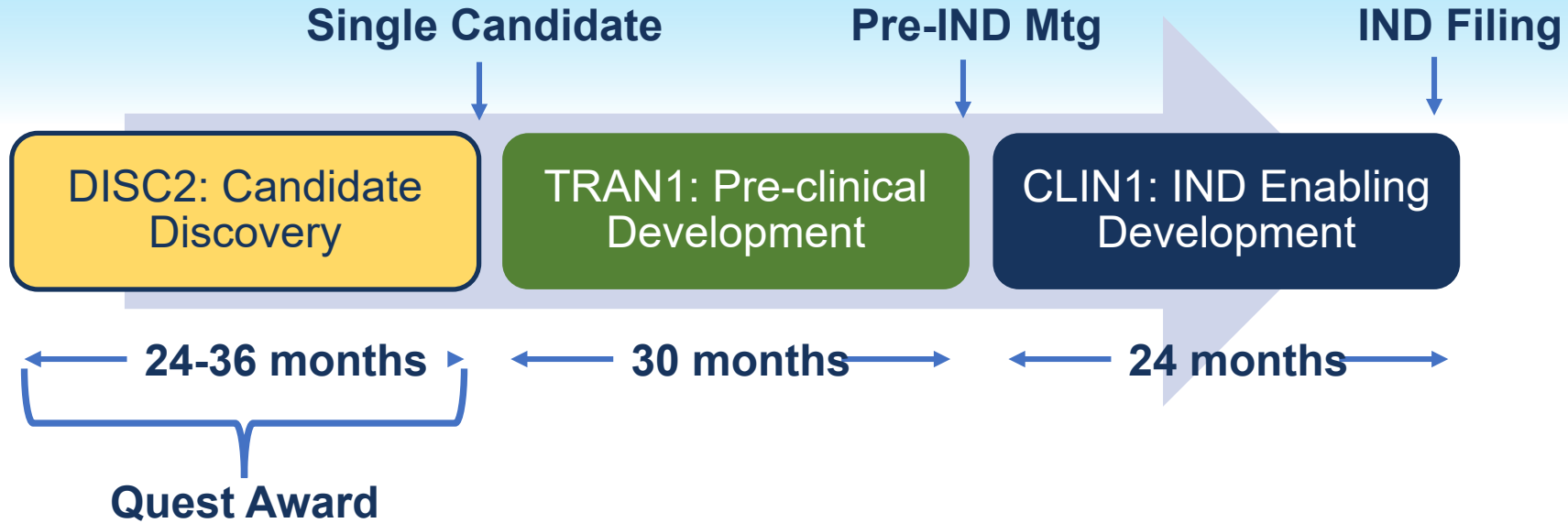
Target Product Profile drafted

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.



Board members with Conflicts of Interest for DISC applications	
Haifaa Abdulhaq	Larry Goldstein
Kim Barrett	James Kovach
Michael Botchan	Pat Levitt
Linda Boxer	Linda Malkas
George Blumenthal	Shlomo Melmed
Ysabel Duron	Christine Miaskowski
Elena Flowers	Michael Stamos
Mark Fischer-Colbrie	Karol Watson
Judy Gasson	

# 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 54 eligible applications were submitted, 8 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 “80-84”**

*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
<b>Recommended for funding</b> Score 85-100	14	\$28,658,837	\$106,000,000
<b>Not recommended for funding</b> Score 1-84	32		

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.

App Number	Title	Funds Requested	Score
DISC2-14047	A Novel Therapy for Sanfilippo B	\$2,297,884	84
DISC2-14097	In Utero Treatment of Duchenne Muscular Dystrophy with Non-viral Gene Editing	\$2,035,544	83
DISC2-14089	Chemically engineered photoreceptors for vision restoration in retinal degeneration associated blindness	\$1,845,093	83

Score	Num $\geq$ 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
84	6	8	80-90	\$2,297,884

**TITLE:** A Novel Therapy for Sanfilippo B

**DISEASE INDICATION:** Sanfilippo B

**PRODUCT TYPE:** Cell and gene therapy

**APPROACH:** Embryonic stem cell-derived neural progenitor cells engineered to overexpress the missing enzyme and correct the neuropathology associated with this disease.



Score	Num $\geq$ 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
83	6	8	80-87	\$2,035,544

**TITLE:** In Utero Treatment of Duchenne Muscular Dystrophy with Non-viral Gene Editing

**DISEASE INDICATION:** Duchenne muscular dystrophy

**PRODUCT TYPE:** Genetic therapy

**APPROACH:** Develop a lipid nanoparticle/mRNA complex that can safely and efficiently edit muscle stem cells in utero

Score	Num $\geq$ 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
83	7	7	65-90	\$1,845,093

**TITLE:** Chemically engineered photoreceptors for vision restoration in retinal degeneration associated blindness

**DISEASE INDICATION:** Retinal degeneration associated blindness

**PRODUCT TYPE:** Cell therapy

**APPROACH:** Develop a chemically-induced photoreceptor cell created by reprogramming skin fibroblasts for transplant in the retina