

# Real Life™

**Gil Sambrano, PhD**

Vice President, Portfolio Development and Review

Grants Working Group Recommendations DISC2

January 27, 2022

**CIRM**  
CALIFORNIA'S STEM CELL AGENCY

# Core Funding Opportunities for Therapy Development



**DISCOVERY**



**TRANSLATION**



**CLINICAL**

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



2/Year



2/Year



12/Year

## 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 directly reprogrammed 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 for a two-year project**

Single candidate identified

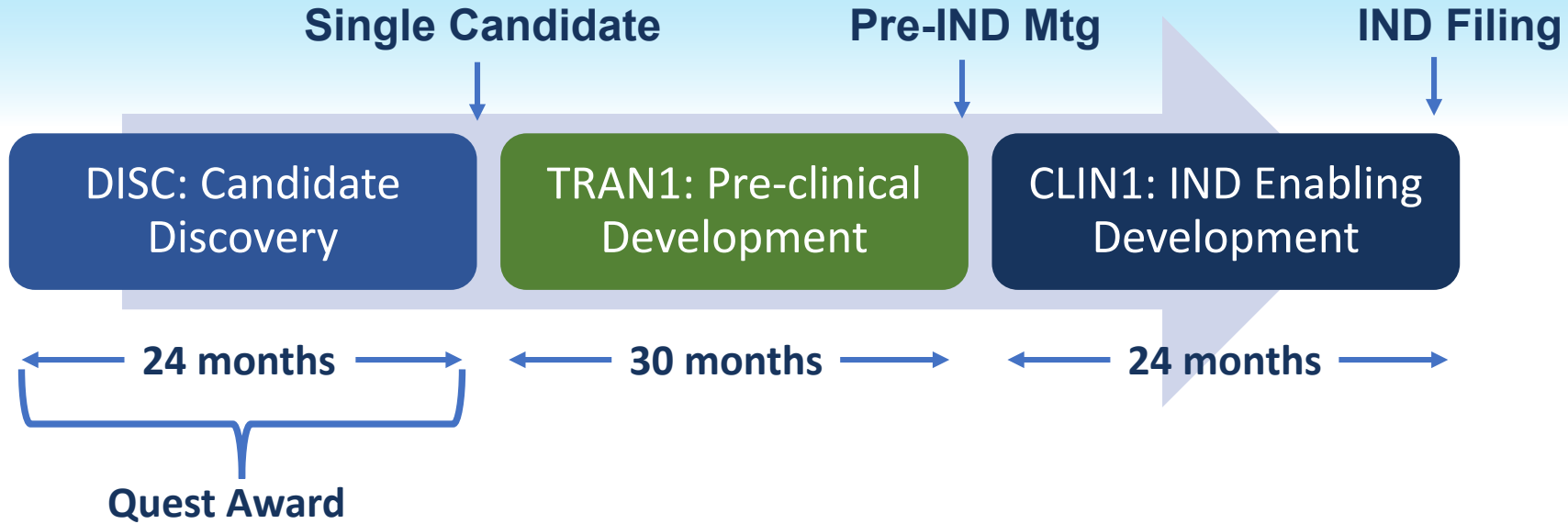
Candidate prototype identified

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.

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 the underserved?

# 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 79 eligible applications were reviewed and 50 were selected to advance 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*

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

The median of all individual GWG scores determines final score.



	Number of Apps	Total Applicant Request	Funds Available
<b>Recommended for funding</b> Score 85-100	16	\$20,754,879	\$40,000,000
<b>Not recommended for funding</b> 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.

- 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.

Score	Num $\geq$ 85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
84	6	9	82-86	\$1,354,928

**TITLE:** iPSC Extracellular Vesicles for Diabetes Therapy

**DISEASE INDICATION:** Type I Diabetes

**PRODUCT TYPE:** Biologic and scaffold combination

**APPROACH:** Extracellular vesicles secreted by human induced pluripotent stem cells to suppress T cell activation and treat Type I diabetes.