SOMETHING BETTER THAN HOPE

Grants Working Group Recommendations: DISC2 Review
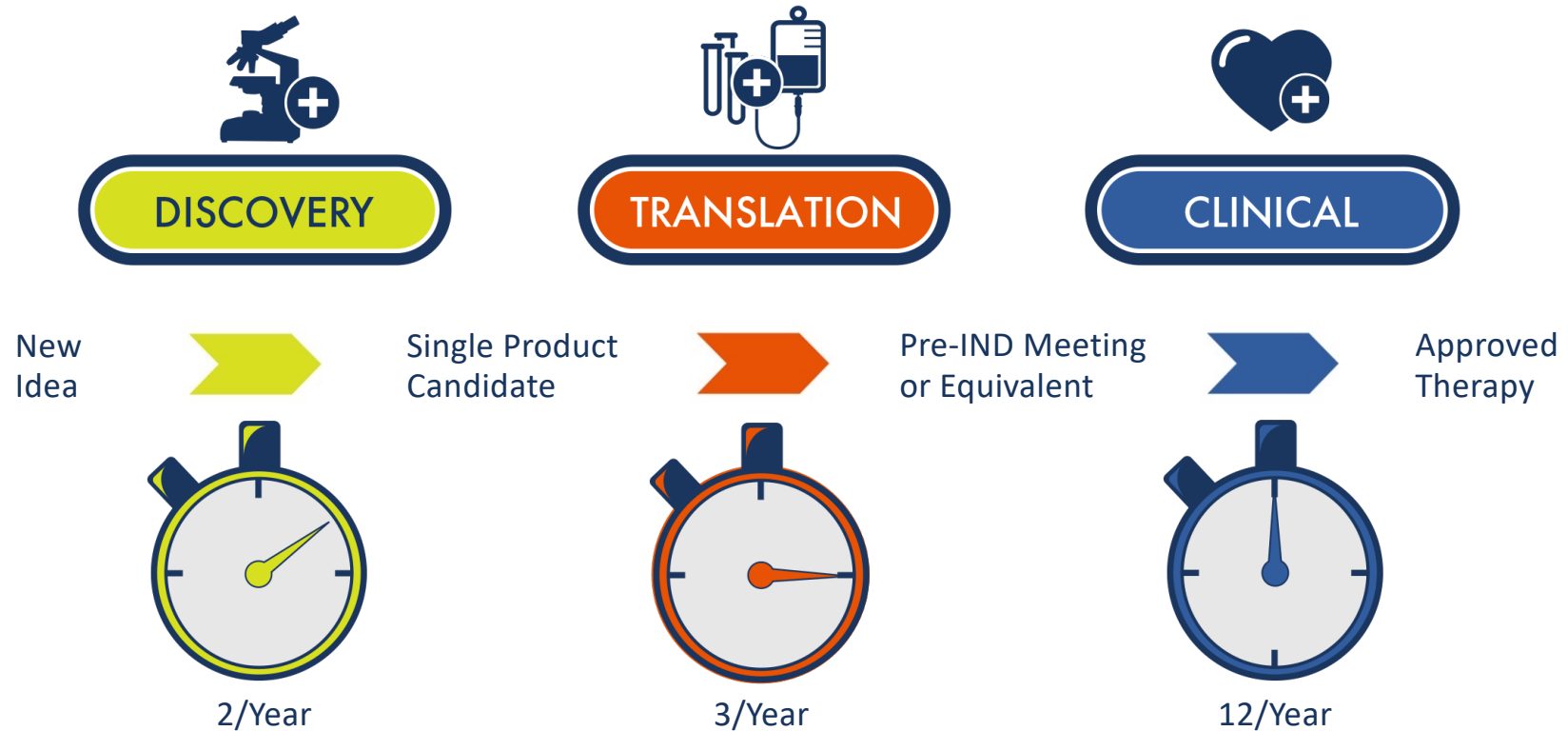
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CIRM Has Relaunched Recurring Core Funding Opportunities for Therapy Development

DISCOVERY

TRANSLATION

CLINICAL

New Idea → Single Product Candidate

Pre-IND Meeting or Equivalent → Approved Therapy

2/Year

3/Year

12/Year

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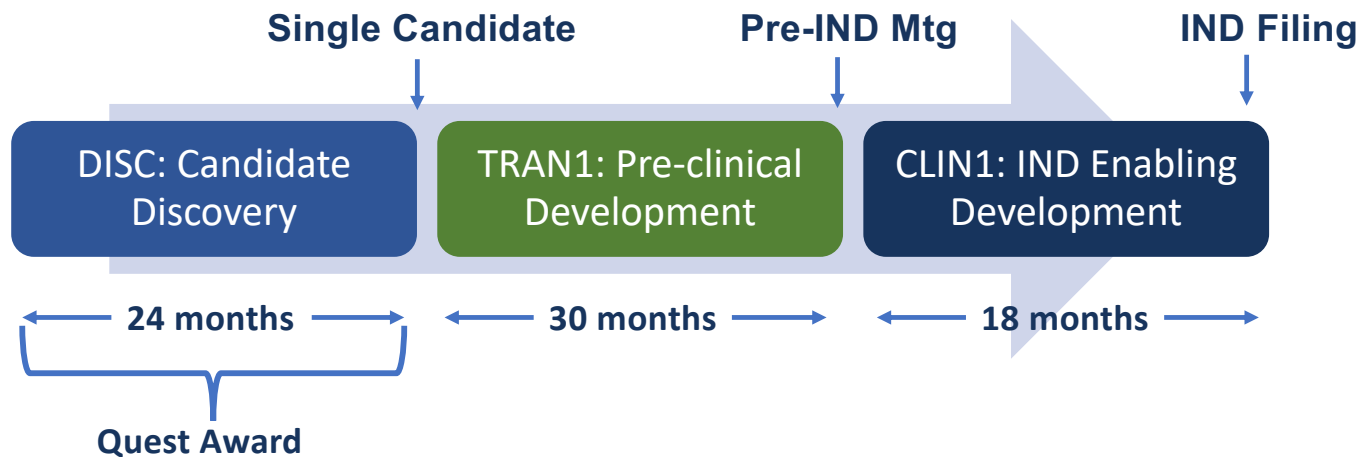
CIRM Quest Program

Objective

The Quest Program will promote the discovery of promising new stem cell-based technologies that could be <u>translated to enable broad use</u> 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 or gene therapies.

What are we looking for?

- We are looking for projects with the capability to develop a novel candidate product (i.e., therapeutic, diagnostic, medical device, or tool) that will be ready for translational studies within two years.

- The product type (i.e., therapeutic, diagnostic, medical device, or tool) determines the specific outcomes expected and readiness for translational studies.
  - Single therapeutic candidate with disease-modifying activity
  - Prototype with proof of concept

- If successfully realized (long-term), the candidate offers the potential to improve patient care **or** to facilitate the discovery, development or use of stem cell-based or gene therapies.

Accelerating Therapeutic Development

Expected **outcomes** of DISC2 applications:

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

Review Criteria

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?

Scoring System

- **Score of "85-100"**

  Recommended for funding, if funds are available

- **Score of "1-84"**

  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.

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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.

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 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 103 eligible applications were reviewed and 57 were selected to advance to the full discussion stage by GWG.

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GWG Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	10	$12,038,037	$22,000,000
Not recommended for funding Score 1-84	47		

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.