

SOMETHING BETTER THAN HOPE

Amendments to DISC, TRAN and CLIN Concept Proposals

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Background

- CIRM's Governing Board, the ICOC, approved changes to the DISC, TRAN and CLIN concepts to effectively relaunch these core programs starting January 2021 under Proposition 14.
- At this time, we are bringing some additional changes to ensure some consistency among these concepts and to remove some unnecessary barriers to applicants.
- Later this year, we expect to bring additional and more comprehensive changes that will align the concepts with a new strategic plan.

Value Proposition of CIRM Funding Opportunities

Prop 71 and Prop 14 requirement:

“In order to ensure that institute funding does not duplicate or supplant existing funding, a high priority shall be placed on funding pluripotent stem cell and progenitor cell research that cannot, or is unlikely to, receive timely or sufficient federal funding, unencumbered by limitations that would impede the research. In this regard, other research categories funded by the National Institutes of Health shall not be funded by the institute, unless such research funding is not timely or sufficient.”

Value Proposition of CIRM Funding Opportunities

Eligibility requirement in RFA/PA:

“...and is being developed for a rare or unmet need unlikely to receive funding from other sources.”

Difficult for CIRM or GWG to assess this objectively or meaningfully on an application-by-application basis.

Value Proposition of CIRM Funding Opportunities

Concept Plan Determination:

Although NIH provides funding in many areas in which CIRM is active, such funding is not timely or sufficient until there is a widely available treatment or cure. CIRM's value proposition is to accelerate the delivery of treatments and cures through targeted funding and active award management, including providing ongoing expertise and resources to awardees.

Therefore, we propose that the ICOC determine (via concept approval) that the proposed concept plan satisfies this requirement by providing funding and other resources that help promote a speedier or more efficacious result in the absence of CIRM funding.

Concept Text for CLIN1 Program

Through the CLIN1 program, CIRM continues to create funding opportunities for the types and stages of clinical research that otherwise do not exist or are of limited scope and focus to advance the field of regenerative medicine. Existing federal funding opportunities for late stage preclinical or pivotal IND/IDE-enabling activities are primarily driven by the internal priorities and interests of the administering body and, therefore, are unpredictable and limited in both scope and focus. The CLIN1 program is a part of CIRM's core product development programs that unlike other funding sources, provide reliable and predictable funding throughout the award period, and brings expert CIRM staff and advice to support accelerated outcomes and advancement of projects along key stages of the product development pathway. CIRM therefore provides this unique opportunity to California scientists to support stages in the development of clinical research projects that are unlikely to receive timely or sufficient funding from other sources.

Global Changes to DISC, TRAN and CLIN Concepts

- Addition of a statement that addresses how these programs provide a unique opportunity to support stages of research that are unlikely to receive timely or sufficient funding from other sources.
- Correspondingly, remove statements in eligibility section that require applicants to demonstrate that they are unlikely to receive timely or sufficient funding from other sources.

Global Changes to DISC, TRAN and CLIN Concepts

- Broadly include gene therapy projects as in-scope for CIRM funding for diagnostic, device, and tool projects.
- Remove eligibility requirement that small molecule/biologics proposals must involve a therapeutic candidate previously funded by CIRM.
- Minor clarifications and adding “regenerative medicine” to broadly describe scope of CIRM funding.

Proposed Changes to DISC2 Program

Discovery awards to identify a candidate therapeutic, medical device, diagnostic, or tool

- Minor clarifications in the description of eligible gene therapy candidates

Proposed Changes to TRAN Program

(TRAN1, TRAN2, TRAN3, TRAN4)

Translational awards to advance a candidate therapeutic, medical device, diagnostic, or tool to the clinic or commercialization.

- Change Project Manager percent effort requirements to 50% (prev. 35%)
- Addition of gene therapy to scope of diagnostic (TRAN2), medical device (TRAN3), and tool (TRAN4) projects

Proposed Changes to CLIN Program

(CLIN1, CLIN2, CLIN3)

Awards to support IND-enabling activities and clinical trials

- Change time to IND filing in CLIN1 from 18 to 24 months
- Change percent effort requirement for project manager to 50% for CLIN1 (prev. 75%)
- Remove preference for rare or pediatric indications for phase 3 trials

Requested Action

CIRM requests the Science Subcommittee recommend Board consideration of the proposed amendments to the DISC, TRAN, and CLIN concept plans.