CALIFORNIA'S STEM CELL AGENCY

**MEMORANDUM**

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| **TO:** | ICOC |
| **FROM:** | Gil Sambrano, Director of Portfolio Development and Review |
| **SUBJECT:** | Proposed Updates to CIRM 2.0 Concept Plans |
| **DATE:** | December 7, 2015 |

## Background

On December 31, 2014, we launched CIRM 2.0 with three new program announcements to offer funding opportunities for stem cell projects in the late preclinical to clinical trial stages. Since then, the Board has adopted concept proposals for CIRM's discovery and translation programs. Despite our best effort to put forth flawless concepts, we understood that in practice there would be much to learn and that adjustments would be necessary to ensure our programs are effective and efficient. The clinical program has been in place for close to one year and overall implementation has been a success. However, there are adjustments to the concept that we believe would improve the clinical program as well as the other CIRM 2.0 programs. This memorandum summarizes four key changes for which we seek your approval.

## Proposed Changes

**1. Make available to the Grants Working Group (GWG) objective information from previous CIRM awards for consideration in the evaluation of a new and related application.** CIRM began issuing research awards in 2006 and has awarded grants to hundreds of unique investigators. As a result, CIRM has significant experience with many applicants, some of whom may apply for a new award or a continuation of a program that has received previous funding from CIRM. Frequently, members of the GWG request information about an applicant's previous CIRM awards such as: achievement of specific milestones/aims, data produced under a related award, or historical information about the proposed product or technology. Such information is often useful for reviewers as it may affect the reviewers' evaluation of the application based on the review criteria, particularly the assessment of feasibility and rationale. In order to ensure that we have a uniform process in place to provide such information in an objective and consistent manner, we propose to include "past CIRM award information, if applicable" as a formal component utilized by the GWG to evaluate the new proposal as follows:

**Consideration of Past CIRM Award Information (If Applicable):**

The GWG may consider information from a previously funded and related CIRM award as part of its review. CIRM will provide the GWG with objective information regarding a related award that CIRM, in its sole discretion, deems relevant, including but not limited to achievement of specific milestones, data, and outcomes for a related CIRM award or awards.

A "related CIRM award" includes: (1) an award for which the applicant PI served as the PI, a co-PI, a co-investigator, or otherwise substantially participated in the conduct of the award; (2) an award involving the same research project or product; or (3) an award that includes overlapping team members.

**2. Include Accuracy and Completeness of Application in Eligibility Criteria.** Applicants for CIRM funding are currently required to attest to the accuracy and completeness of the information submitted in an application. In order to ensure that CIRM has a mechanism in place to address concerns regarding the accuracy or completeness of an application, we propose to include the accuracy and completeness of an application as an eligibility criterion for all CIRM 2.0 programs.

Pursuant to this change, if CIRM were to determine, in its sole discretion, that the application includes a material misstatement or omission, CIRM would discontinue its review of the application and communicate its determination to the applicant, who would have the opportunity to resubmit its application or explain why it has decided not to address CIRM's concerns. If the applicant were either to resubmit an application that satisfactorily addressed CIRM's concerns or satisfactorily explain why CIRM's determination was erroneous, review of the application or revised application would proceed. If the applicant were to elect not to resubmit the application or CIRM is not satisfied with the explanation or revised submission, the applicant would have the ability to appeal CIRM's eligibility determination to the GWG.

**3. Require the intended or current IND sponsor to be the applicant organization (if an organization-sponsored IND) or the PI (if an investigator-sponsored IND) for applications in the Clinical Stage Program (CLIN 1, CLIN 2, and CLIN 3)**. CIRM partners with grantees to conduct work that will result in a successful IND submission or that is subject to the specifications of an existing IND. To maintain proper oversight of clinical stage projects, a direct relationship with the IND sponsor is critical to ensure access to information that impacts the conduct of the work.

**4. Allow individuals under contract to act on behalf of the applicant organization to qualify as a Principal Investigator (PI)**. To be eligible to serve as PI on a CIRM award, an individual must 1) be an employee of the applicant organization, 2) commit minimum listed effort on the project, and 3) be authorized by the applicant organization to conduct the research and assume the responsibilities of the PI. The requirement for a PI to be an employee of the applicant organization was established to ensure accountability of the PI to the applicant organization, which is ultimately responsible for the conduct of the proposed project to CIRM. However, we've encountered circumstances where the most appropriate person to lead a project may not be an employee of the applicant organization, particularly if the majority of the work is to be conducted off-site at a collaborating institution. Therefore, we propose to extend

eligibility as a PI to individuals who are accountable to the applicant organization, either by employment or by a contractual agreement to act as an agent of the organization.

**Requested Action:**

Recommend Board approval of modifications to concept plans for CIRM 2.0 programs.