



M E M O R A N D U M

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To: Independent Citizens Oversight Committee (ICOC)

Subject: Concept proposal for RFA 13-03: Strategic Partnership III

This concept proposal addresses the continuation of CIRM's Strategic Partnership Initiative which was created to attract industry engagement and investment in CIRM-funded stem cell research and enhance the likelihood that CIRM-funded projects will obtain follow-on funding for Phase 3 clinical trials. In summary, we propose to add an alternative funding track to the next Strategic Partnership solicitation, RFA 13-03: Strategic Partnership III, under which awardees would receive payment only upon successful achievement of a Major Development Milestone(s).

Background:

The Strategic Partnership concept was approved by the ICOC on October 26, 2011 and amended on September 5, 2012 and on October 25, 2012. The current concept directs CIRM to implement the program using a standard Request for Application (RFA) process that has a defined submission period. Each RFA will define the specific scope of research to be funded, within the currently approved concept scope which encompasses a broad range of research from basic research to Phase 2 clinical studies.

Specific features of the Strategic Partnership concept include (i) a requirement that the applicant show evidence of either having the financial capacity to move the project through development, or of being able to attract the capital to do so (Commercial Validation) and ii) a requirement to provide co-funding for the proposed project.

Under the current concept, CIRM will fund up to \$10M justifiable total project costs per project, with the possibility to increase the award up to \$15M per project, subject to ICOC approval and only under exceptional circumstances.

The Strategic Partnership initiative is aligned with CIRM's 5 year strategic goal to attract industry engagement and investment in CIRM funded stem cell research, and with CIRM's 5 year strategic clinical objective to advance stem cell science into clinical trials to achieve therapeutic benefit for patients.

Concept Proposal for RFA 13-03: Strategic Partnership III

For RFA 13-03, the third RFA under the Strategic Partnership initiative, the addition of an alternative funding track is proposed. The existing track, as implemented for Strategic Partnership I and II and now referred to as Track A, will provide funding for a single approved project over a project period of up to 4-years. Track A applications, which must be focused on a single development project, will be reviewed by the Grants Working Group (GWG) and must receive ICOC approval in order to receive CIRM funding. Project milestones and success criteria will be mutually agreed upon between CIRM and the applicant and project progress will be assessed by the Clinical Development Advisory Panel (CDAP), a group of external experts, at various points during the project period.

To complement Track A, CIRM proposes to add Track B, also referred to as the Milestone Payment Pathway. Track B permits a for-profit applicant to apply for funding for up to 5 different projects. As with Track A applications, the GWG will review the submission for scientific merit and funding is contingent on ICOC approval. However, under a Track B award, payment (or loan disbursement) will take place only upon successful achievement of a Major Development Milestone arranged in advance between CIRM and the applicant. There will be no upfront payment. The Major Development Milestone and success criteria will be mutually agreed upon between CIRM and the applicant at the beginning of the project(s) and at a minimum will require completion of a clinical trial that shows some level of biological activity/clinical efficacy and safety. The advantage to CIRM of this Track B pathway is that CIRM funds will only be applied to projects that are successful. The intent is that such milestone payment provided by CIRM would enable the project to proceed to the next stage of research with the ultimate goal of gaining regulatory approval to bring a novel therapy to patients. As for Track A, Track B applicants will be required to provide evidence of Commercial Validation as well as co-funding for the proposed project(s).

Details of the Concept for Strategic Partnership III

Strategic Partnership III will have two tracks, Track A and Track B, as outlined below.

Track A: Similar to Strategic Partnership I and II in all aspects.

Award: CIRM will fund up to \$10M/award over up to 4-years for 1 project (\$15M in extraordinary circumstances) .

Objective: Completion of a Phase 1 and/or Phase 2 clinical trial within the 4-year time frame.

Eligibility: Requires Commercial Validation.

Co-funding: Requires co-funding from the applicant

Track B: Milestone Payment Pathway

Award: CIRM will fund project costs up to \$10M/project, on a shared basis with the grantee, for up to 5 projects per applicant, if and only if, the Major Development Milestone is successfully achieved within the 4-year project period.

Eligibility: Requires Commercial Validation.

Award information:

Total allocation for SPIII - \$80M

These funds are proposed to come from funds allocated for this program under the Board-approved Strategic RFA Funding Plan (\$40M) together with unallocated funding that was planned for Strategic Partnership I and II (\$40 M).

Award mechanism:

Grant, if PI holds the IND and is from a not-for-profit organization

Choice of Grant or Loan, if a for-profit organization holds (sponsors) the IND and is the applicant

organization.

Provisional Time Table:

Post RFA 13-03	July	2013
LOI applications due	Aug	2013
Full Applications due	Oct	2013
Review of Applications by Grants Working Group (GWG)	Feb	2014
Review and Approval by ICOC	Mar	2014
Earliest Funding of Awards	3 Q	2014