



Proposal for an Opportunity Fund Program

Objectives:

- Accelerate development of stem cell therapies, (as set forth in Proposition 71)
- Implement recommendation of the External Review Panel to “Pave a path from fundamental to translational research, translational medicine, product development and healthcare delivery” by a “Transition to a much more proactive strategy of funding that aligns CIRM’s peer review and other processes with its mandate of delivering new treatments to the clinic as articulated in CIRM’s mission and strategic goals”.
- Implement the recommendation of the External Review Panel to adopt a “porous opportunity model rather than an internal pipeline model... [to] keep California as the hub of clinical proof of concept in regenerative medicine for the world rather than just for CIRM funded pipeline projects.” (Recommendation 5)
- Attract industry participation through a funding mechanism that is more aligned with industry’s financing practices as recommended by the External Review Panel. (Recommendation 7)

Description of Potential Opportunity Fund Programs

To meet the described objectives, we propose that the Governing Board (ICOC) authorize creation of an opportunity fund. We propose 3 components that would access such an opportunity fund: 1) Bridge Funding Program; 2) External Innovation Funding Program and an 3) Industry Strategic Partnership Program. We propose an initial \$30M opportunity fund for all 3 components, subject to replenishment by the ICOC. Up to \$5M of the \$30M shall be allocated for payment of costs and services associated with technology transfer and patenting expense incurred by the grantee for outside services in these areas. Each of these components is briefly described below.

- **Bridge Funding Program** provides supplemental funding for selected existing CIRM funded projects to enable uninterrupted funding of development activities (but any new patient enrollment would be limited to projects for which an FDA clinical trial was within the scope of the prior grant award) to occur until the next relevant RFA/review is offered. Projects that are proposed for opportunity funding under this program must be mission critical, competitive and in ‘good standing’ (meeting mutually agreed upon milestones and on track for planned outcome). Those projects that receive

bridge funding would be required to submit an application to the next applicable RFA/GWG review but would not be required to participate in a pre-application/planning award process.

- Only active CIRM funded projects, previously reviewed by the Grants Working Group and approved by the ICOC, would be eligible for Bridge Funding. Specifically, only the following translational and development projects would be eligible for funding from a Bridge Opportunity Fund:
 - Tools & Technology if high value significance to CIRM's mission
 - Early Translational projects targeting a Development Candidate
 - Disease Team projects
 - Targeted Clinical Development Projects
- CIRM to report to the ICOC on Bridge Funding disbursements when requesting replenishment or at least annually
- Implementation
 - Maximum opportunity fund supplemental funding is \$5 MM. President has authority to approve (Note: a DT award amortized equally over 4 years would be \$5 MM/yr. A year is expected to be the maximum bridge period)
 - Internal staff recommendation is required to initiate process for supplemental Bridge funding. Recommendation based on significance to mission, competitiveness, project performance, timing (relative to next RFA), and other defined criteria.
 - Following internal recommendation, and receipt from the grantee of, minimally, a description of the proposed work, a detailed budget and justification, and appropriate milestones, the project will be considered by the next Clinical Development Advisory Panel (CDAP) meeting for review against set criteria (for example, see DT RFAs). Based on CDAP advice, the VP, R&D and staff will advise the President who will make supplemental funding decision. (To reduce the burden on the CDAP, for those recommended projects that want to "bridge" from Early Translational Development Candidate into IND-enabling preclinical development, consider using an internal staff review, in association with external expert consultants.)
 - As noted above, projects that receive Bridge funding would be required to submit an application to the next applicable RFA/GWG review (but would not be required to undergo pre-application/planning award review)

- **External Innovation Funding Program** would catalyze the collaboration, through the funding of a research fellowship in California, of researchers with innovative, cutting-edge stem cell research outside of California to work with CIRM-funded investigators offering complementary expertise and/or unique scientific/clinical environment. The program would provide 12 month supplemental funding to an existing CIRM-funded research award to support the high impact external research opportunity. The external research opportunity would have been identified through an inventory of the research landscape e.g., conferences, publications, networking, and would be collaboratively partnered with a CIRM-funded researcher through a supplement mechanism. The team is assembled and research is available for immediate implementation. Foundations could participate, through a collaborative funding partnership, in the funding of any research component of the

collaboration outside of California.

In addition to the external innovation funding program's accelerating the research opportunity and catalyzing California collaboration, the external researchers with innovative programs and successful collaborations might subsequently increase the pool of candidates to be considered for CIRM's research leadership award.

- Projects proposed for supplemental funding under this program could include:
 - Early and applied research on cutting edge technologies
 - Transfer of unique methods/ techniques that a CIRM grantee needs --if they can identify a scientist/researcher that is willing to conduct the research in California
 - Preclinical/clinical development stage programs that could provide the needed preliminary data to support the submission of a more comprehensive program in an emerging field critical to advancing stem cell science
 - Strengths/complementary expertise/unique scientific/medical environment of the collaboration
- The application would need to include a plan for addressing intellectual property issues proactively
- External Investigators considered for stipend, salary and research support would include criteria consistent with the CIRM research leadership award:
 - MD, PhD or equivalent degree
 - Primarily employed outside of California
 - Have been an independent investigator for at least 3 years
- Applicant institution eligibility and principal investigator
 - The parent CIRM-funded program which would receive the supplement, would have at least one year remaining of active research funding
- Review criteria
 - Applications would be evaluated in 3 areas: research vision and plans, external investigator experience and potential, and the institutional commitment and environment
- Funding – approx \$500,000 up to \$750,000 per award
 - Supplemental funding would provide a stipend for travel, partial salary support, accommodations (consistent with CIRM's Visiting Faculty awards) and research expenses of the external researcher working in California. Foundation funding, through a collaborative funding partnership could be used to fund any component of the collaborative research outside of California; Research anticipated to require more than a 12 month commitment would require the applicant, at the time of submission, to provide evidence of other potential funding sources satisfactory to CIRM, in its sole discretion, to complete the project after the 12 month funding from CIRM

- Program announcement with a rolling submission and review by GWG two times per year. Consideration could be given for a review by the Clinical Development Advisory Panel for research focused on IND enabling or clinical studies.
- **Strategic Partnership Program:** Is intended to help foster industry, venture capital and other funding support of significant magnitude to support CIRM-funded programs. The support will be provided using a streamlined process comprised of a Program Announcement with rolling submissions and Grants Working Group reviews approximately two times a year.
 - A Program Announcement would seek:
 - Projects having a California-based principal investigator partnered with a non-profit, a biotech company or a biopharmaceutical company that is willing to commit to in-kind support (which could include access to proprietary assays, regulatory and manufacturing process development expertise etc) as well as funding or co-funding of product development activities and preclinical research and/or
 - Projects having a California-based principal investigator who is funded primarily by venture capital , or has significant funding through foundation support or other sources
 - Objectives of the Program are:
 - To accelerate development of potential therapies by encouraging the involvement of industry which could have the following benefits :
 - Bringing to bear industry expertise in regulatory processes, manufacturing and process development for the potential therapy, and clinical trial design and implementation
 - Leveraging CIRM's funding through all translational phases of development
 - Providing a possible source for Phase III funding (follow-on financing)
 - To further leverage CIRM's funding
 - Core elements of the program include the following:
 - A submission process that is rolling so that the Governing Board (ICOC) can be as agile as possible in responding to select opportunities
 - A process that allows the Governing Board (ICOC) to commit to future funding today in the event that certain future milestones and conditions are met so that the strategic partner has the certainty it needs to invest early on (rather than take a wait and see approach)
 - Incentives for grantees and partners that include: accelerated review and future funding commitments (if conditions are satisfied)
 - Intellectual property terms and key terms of a collaboration/co-development agreement between CIRM and partners to be approved by the Intellectual Property Sub-Committee
 - The funded Program will be up to 4 years, provided that milestones set forth as part of a co-development and collaboration agreement are satisfied. The Intellectual Property Sub-Committee may approve a term longer than four years.

- A maximum per project amount (not including technology transfer and patent funding assistance) is \$15M of state bond funds, unless the Finance Sub-Committee recommends a greater amount