# Questions and Answers from Webinar April 25, 2012
# Strategic Partnership I Initiative
# RFA 12-05

| Questions | Answers |
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| 1. Are mesenchymal stem cells excluded from the RFA? | The RFA states the cell types that fall outside the scope of this RFA, and they include the following:<br><br>• minimally manipulated bone marrow cells<br>• umbilical cord blood stem cells<br>• adipose-derived stem cells<br>• unmodified hematopoietic stem cells<br><br>The allowable cell types are described on page 2 of the RFA. As stated there, adipose derived or cord blood cells, including MSCs from those sources, are excluded. MSCs isolated from other sources, such as bone marrow, could be eligible if they meet the other criteria stated in the RFA. |
| 2. We are doing adult stem cell research. Can we apply for this grant? | It depends on the type and source of the adult stem cell. The allowable cell types are described on page 2 of the RFA. |
| 3. Must there be transplantation of a stem cell in order to qualify?<br><br>Related question: Will CIRM fund clinical trials of conventional drug development with small molecules or biologics? | Stem cell transplantation is not required. See page 2 of the RFA:<br><br>A small molecule or biologic candidate that i) mobilizes endogenous stem cells to promote tissue repair/regeneration and for which there is convincing evidence of such activity; or ii) was identified and characterized using patient-derived induced pluripotent stem cells or their derivatives; or iii) is specifically targeted to destroy cancer stem cells and for which there is compelling evidence for such activity based on serial clonal transplantation assays in an in vivo model. |
| 4. We are developing immunosuppressants for the treatment of organ transplant. The scope of our project would be to test if immunosuppressive drugs block the immune response to bone marrow transplant. Is this appropriate for this RFA, or for a subsequent RFA? | If the immunosuppressant is not one of the therapeutic candidates described on page 2 of the RFA, it would not be eligible for this RFA. A drug intended to block the immune response, does not appear to be eligible for this RFA. The questioner should call CIRM to identify whether another RFA or initiative might be appropriate. |

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| 5. We are developing a new radiopharmaceutical for labeling stem cells and will conduct a trial to support the IDE. If we have the appropriate investments, would this be considered eligible? | That study would not, on its own, be eligible for funding under this RFA. CIRM is not funding research solely directed at biomarkers or diagnostics in this RFA. If, however, the labeling is a necessary part of a trial of an investigational therapeutic intervention that meets the requirements on page 2 of the RFA, it may be within scope as an activity that could be funded under this RFA as part of an award supporting development of the therapeutic. |
| 6. Can the stem cells be transferred from a location outside of California to a California lab? | Yes. |
| 7. Is there a priority for more advanced clinical trials? Should we apply for our program in Phase 2, or our program on clinical hold? | Priority will be given to eligible proposals aimed at furthering the development of CIRM-funded projects, or having a strong probability of achieving clinical proof of concept. Programs on clinical hold at the time of submission will be evaluated for eligibility on a case-by-case basis. If all other criteria are equal regarding scientific rationale, feasibility, etc for a clinical trial on hold vs a clinical trial ready to proceed to phase 2, it is likely the reviewers would view the clinical trial ready to proceed as a higher priority for funding. |
| 8. Is a trial that has already been initiated (Phase I) eligible for funding for Phase II? | Yes, if the Phase II trial can be completed within the 4 year time frame. |
| 9. Please clarify "may be required" in the following statement in the RFA (VIII.A. Letter of Intent):<br><br>"Documentation in support of commercial validation may be required as part of the LOI submission." | The RFA outlines two different ways to establish commercial validation, which can be either through 'financial strength and historical investment', or via a 'collaborative development arrangement'. Depending on which of those you choose, the LOI asks for different things. If you choose 'financial strength and historical investment' you do need to submit supporting documentation with your LOI, by the LOI deadline, May 16th. If you select 'collaborative development arrangement' there are no documents required at the time of the LOI, but you must acknowledge that you will be able to meet the requirement of being able to submit a copy of an executed contract or term sheet by the application deadline, June 26th. |
| 10. We are a company. Do we need to have an academic partner? | No. |

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| 11. If the LOIs are submitted before the due date – will we be told sooner if we can proceed forward? | Yes. CIRM will only contact you if your LOI contains information that makes your proposal ineligible. If you do not hear from CIRM within 2 weeks from the date you submitted your LOI, you should assume you are eligible. May 16th is the final date in which LOIs can be submitted to CIRM; however, you may submit your LOI earlier. |
| 12. Who, specifically, is on the Intellectual Property and Industry Sub-Committee? | The composition of the Intellectual Property and Industry subcommittee is listed in the RFA, along with the membership link at http://www.cirm.ca.gov/our-board-meetings/governing-board/icoc/governing-boardicoc-cirm. The subcommittee is comprised of seven members; four of whom were appointed to the Board based on their affiliation with life science commercial entities; one of whom was appointed to the Board based on her position as an executive officer of a UC campus with a medical school; one of whom was appointed to the Board based on his affiliation with a patient advocacy organization; and the Chair of the Governing Board, who is a patient advocate elected by the Board from among candidates nominated by the Governor, the Lieutenant Governor, the Treasurer and the Controller. |
| 13. How do you assess commercial validation? | Please see section V,D and Section VIII, 21 for a description of documentation needed to establish commercial validation. The validation will be established on the basis of "Financial Strength and Historical Investment" and/or "Collaborative Development Arrangements" and will be assessed by the IP and Industry subcommittee, as applicable on measures including:<br><br>• The amount of historical equity and/or programmatic investment in the applicant organization, with larger amounts being rated more highly<br>• The number of years of balance sheet cash at the applicant organization as of 3/31/2012 and pro forma for any concurrent investment<br>• The amount of co-funding and future financing committed by the applicant, and if applicable, its partner, for the proposed project and for future development required to achieve FDA approval to market the proposed therapeutic<br>• The strength of the commitments by the applicant and, if applicable, any of its partners to fund a) the project and b) future development |

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| 14. Can you please discuss the treatment of any IP generated during the grant period? | CIRM has IP regulations that apply. The regulations are explained here: http://www.cirm.ca.gov/for-researchers/intellectual-property-faq. Feel free to contact CIRM if you have questions about the IP regulations. |
| 15. The RFA says "For-profit applicants may choose to accept the award in the form of a grant or a loan." Why the choice? Are there any other criteria if an organization wants to opt for a grant instead of a loan? | The revenue sharing provisions differ between a grant and a loan and therefore CIRM gives awardees the option to choose the form of their award. There are no additional criteria for selecting a grant. With respect to loans, there is a level of financial due diligence that is undertaken and fees for such costs are paid out of the loan. |
| 16. How should "in kind" matching funds be presented in the application when applicant is providing an IND to support Phase I studies and when applicant is providing drug to support clinical trial? | The amount, nature and value of in-kind services, including but not limited to FTEs, that an industry partner or applicant will provide without charge, such as regulatory expertise, process development and clinical development expertise should be included in the application. If the drug provided by the industry partner or applicant has already been manufactured, there will be no separate value attributed to such drug as a basis for satisfying the matching requirement. The definition of value of in-kind services should capture only expenses directly incurred in pursuit of the program e.g., salary, benefits and consumables, but not any allocation for facilities or G and A. Please contact CIRM if you have more specific questions regarding this |
| 17. Is the requirement for "Co-Funding" an absolute requirement? If so, if we were able to obtain additional industry partner(s) after submitting the LOI is it okay to make those changes in the grant submission?<br><br>Related question: Do the matching funds have to be available at the time of the application? | Co-funding is absolutely required. Ability to co-fund is demonstrated through commercial validation, which the applicant can be shown in either of two ways, as described on page 8 of the RFA. If shown through 'financial strength and historical investment', the funds to meet that requirement must be available by the time of the LOI. If co-funding is shown though a 'collaborative development arrangement,' it is not necessary to have the funds available at the time of the application, but the applicant must show either an executed funding agreement with a large biotechnology or pharmaceutical company, or a commitment to enter into such an agreement., at the time the LOI is due. |

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| 18. Can we claim money spent in 2012 before award in January? | Matching funds can only include budgeted costs incurred during the project period, or during the 90 days before the start of the project period. If the applicant would want the prior expenses covered with CIRM funds, the same 90 limit applies, as provided in CIRM's Grants Administration Policy:<br><br>Pre-award Costs: After the ICOC approves an Application for funding, a Grantee may, at its own risk and without Prior Approval, incur obligations and expenditures to cover costs up to 90 days prior to the effective date of Award if such costs are necessary to conduct the project and would be allowable if the Application were funded. |
| 19. What do I have to submit from my Pharma/biotech partner regarding due diligence analysis? | If the proposed project has undergone a due diligence analysis by a pharmaceutical/biotechnology partner resulting in an executed development agreement, or term sheet, or letter of intent to enter into a development agreement, the application should include a summary of the scope and extent of the due diligence investigation, including a list of specific assessments, the number and expertise of personnel involved in conducting the investigation, and a summary of the outcomes. |