WEBINAR – May 13, 2014
RFA 14-02: CIRM Preclinical Development I Awards
Questions and Answers

Questions	Answers
1. What if the applicant can check some, but not all, of the readiness criteria in the LOI? Is it possible to still be eligible?	You can submit an LOI; CIRM will determine eligibility based on specific information provided. It is expected that eligible development candidates will have conducted some research addressing all relevant readiness criteria.
2. What if I already had a pre-IND meeting, but I haven't completed all the activities needed to initiate pivotal IND-enabling studies?	If you have already had a pre-IND meeting but have not carried out the activities that are in scope of this award, you may still be eligible to apply for this award and can submit an LOI for eligibility evaluation. CIRM will determine eligibility based on specific information provided.
3. Can I submit a proposal for a small molecule targeting cancer stem cells, as long as I have matching co-funding?	If your Development Candidate targeting cancer stem cells was identified with prior CIRM funding, you may apply. If it was not, then your project is not eligible, whether or not you have co-funding.
4. Can an institution submit more than one LOI?	Yes, but a single PI can only submit a single application.
5. Is a compound that has already been in the clinic for another indication eligible for this award?	No, the intent of this RFA was to move new therapeutic candidates toward the clinic.
6. We do not have previous CIRM funding but would like to apply? Are we eligible?	Yes, however you must have a one-to-one match of co-funding. The co-funding may come from any source, however applications with co-funding provided by an industry partner are designated as a CIRM priority.
7. Does previous CIRM funding need to be on exactly the project proposed? What about translational projects that are on a related disease but did not have exactly the same plans?	The previous CIRM funding needs to have been used to identify the Development Candidate for a disease that is related to the one proposed for the Preclinical Development Award, to the extent that the mechanism of action is expected to be the same. For example, a candidate that is intended for bone regeneration might be applied for different types of bone diseases or injuries.

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8. If the DC came from a CIRM grant for regeneration, but is more valuable for cancer stem cells, is that ok?	No, you must focus the candidate on a disease related to that for which it was funded by CIRM.
9. We had an Early Translational project that had two components: one is a small molecule to stimulate stem cell proliferation and differentiation, and another is a stem cell. Can we make a proposal on the small molecule alone for the same indication?	A project that focuses on a small molecule Development Candidate must have convincing evidence that it is targeting an endogenous stem cell.
10. If we are on an IDE/PMA regulatory pathway, are we eligible for these grants?	The intent of this award is fund a stem-cell-based therapy. A device without stem cells would not be eligible.
11. I have a DC that was funded during an earlier CIRM Disease Team award, but which is now modified so that we will need to work out new manufacturing conditions. Am I eligible?	Modifications of therapeutic candidates that were already funded by CIRM Disease Team or Strategic Partnership Awards are not eligible (without Presidential exception).
12. When, after submitting the LOI, will notice be given if eligible to submit a full application?	You will be notified only if you are NOT eligible for the full application. This notification will be made in early June, no later than June 17.
13. Our Development Candidate was not developed with prior CIRM funding. If our total budget is $6M, do we have to raise $6M in matching funds, or only $3M?	In the event that co-funding is required, you need to determine your total budget first. Then, CIRM will fund half and the co-funder(s) will fund the other half.
14. What if I get $1M (matching funding) for a total of $2M? This is less than $4-5M mentioned in the RFA. Would the total amount affect the review?	The budget should reflect the actual costs of the work proposed, and the work proposed should meet the objectives of the RFA. If you can achieve the objectives of the RFA for less than $4-5M, then the lower budget is appropriate.
15. If milestones are met early and for less money than expected, can some of the grant money be used for clinical expenses?	This award is for preclinical work only. CIRM has other funding mechanisms for clinical trials.

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16. Can co-funding supplied by an external source be spent outside of California?	Yes.
17. For projects that received prior CIRM funding, how much priority will be given to projects that have at least 25% co-funding?	The question of priority comes into play when reviewers are considering grants that have similar qualifications. Reviewers are instructed to give extra consideration when scoring the grant that has the 25% co-funding.
18. Can 25% co-funding be from NIH?	Yes. Regardless of whether the matching requirement is 100% or 25%, the matching funds may come from any source. Projects in which one to one co-funding is provided by an industry partner are designated as a CIRM priority.
19. Does the 25% co-funding requirement matter for the LOI, or only at the full application stage? We are looking for co-funding but may not find it for a few months.	Please see RFA Section IX.A for the requirements at the LOI stage, and Section IX.B.14 for the requirements at the full application stage.
20. If a potential co-funder provides a letter during the LOI submission, but later the negotiation did not go through and an agreement cannot be reached, will this affect the application and final decision making of CIRM?	If the application concerns a development candidate that was not identified with CIRM funding, the application process will be terminated.
21. If the 25% agreement is reached, and the grant is approved for funding by CIRM, but later the co-funder could not provide that 25% funding, will CIRM revoke the funding?	CIRM requires a signed co-funding agreement prior to funding decisions. If the agreement is broken after the start of the project, CIRM has the option to revoke the remaining funding.

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22. When is the latest date to be able to demonstrate co-funding?	Please see the RFA, Section IX.A and IX.B.14. A term sheet or letter of intent outlining terms of the co-funder's support and signed by the co-funder must be submitted by September 29, 2014. The last possible date to provide a fully executed agreement will be two weeks prior to the date of the ICOC/Application Review Subcommittee meeting in Q1, 2015 (exact date to be determined). If such an agreement is not provided, the application will not be considered for funding.
23. Would co-funding from an industry partner in the form of in kind services amounting to the 25% be acceptable?	Yes.
24. After development of a cGMP manufacturing process protocol, will this award fund pilot manufacturing of the cGMP products?	This award will fund cGMP process runs to address process consistency as well as any tech transfer runs (i.e. moving the process to the actual manufacturing site). It will not fund manufacture of clinical lots.
25. Will CIRM fund GLP production of product for IND-enabling studies?	This award will fund manufacture of product for the pivotal IND-enabling safety studies using the process intended for clinical use, but will not fund the studies themselves.
26. What is the nature of the pilot safety studies in scope for funding under this mechanism as compared to the pivotal safety studies to be performed after the pre-IND meeting?	Pilot safety studies might be similar in nature to the pivotal safety studies, and should be done with product made using the process still being developed for clinical use. These studies should be designed to refine estimates of tolerable dose range, and to examine overt toxicity, immunogenicity and tumorigenicity. The plans for pivotal safety studies need to have been reviewed with the FDA at the pre-IND meeting and are done with GMP product or a GMP-compatible GLP lot that meets predefined specifications.

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27. Do you have to know the exact route dose and regimen for the proposed TPP?	No. The TPP is a statement of the overall intent of the development program for a given therapeutic, and is used as a tool to define a variety of optimal and minimally acceptable parameters for the therapeutic. This document should be updated as necessary as you obtain more knowledge about the drug during development. For more information, see the FDA Guidance at file://localhost/(http/::www.fda.gov:downloads:Drugs:GuidanceComplianceRegulatoryInformatio n:Guidances:ucm080593.pdf.
28. Does CIRM have plans to fund any clinical work outside of alpha clinics?	Yes, we have a Strategic Partnership RFA that is currently accepting Letters of Intent (due June 19). In addition, we expect to post additional RFAs for clinical trials.
29. Does the preclinical work need to be conducted in California?	The research needs to be conducted in California. See Section IX.B.5 Budget: Consultants/Subcontracts). However, certain activities are not considered "research". Please contact CIRM to determine whether your work is considered "research".

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30. In RFA, the award will fund "Preparation of a draft Development Plan through End of Phase 2". What does "Phase 2" mean? Is it a Phase 2 clinical trial, Phase 2 of your CIRM Phase 2 funding?	End of Phase 2 refers to the end of the Phase 2 clinical trial program prior to initiating pivotal studies.
31. Is the LOI reviewed?	The LOI is assessed internally and is used to ensure that eligibility criteria are met before the applicant can submit a full application.
32. Will CIRM assist in identifying co-funding partners?	That is the responsibility of the applicant.