Questions and Answers
RFA 13-01: CIRM Disease Team Therapy Development III Award Webinar

Questions	Answers
Are autologous cell therapy products eligible for this RFA?	Some autologous cell therapy approaches are eligible for funding under this RFA. For example, autologous induced pluripotent stem cell-derived approaches as well as autologous genetically- or pharmacologically-modified hematopoietic stem cells (HSCs) are eligible. Furthermore, the RFA allows for tissue engineered functional tissues, which might utilize autologous cells. The excluded autologous cell types include those already substantively represented in the CIRM portfolio. There are other funding opportunities outside of CIRM for funding autologous approaches.
If we are proposing to use autologous cells derived from induced pluripotent stem cells, what method should we use to reprogram the cells to avoid licensing/intellectual property issues?	CIRM does not advise on which methodologies to use. Since the commercial viability of therapeutic approaches is important to CIRM's mission, intellectual property and freedom to operate will be considered as part review of applications for this RFA. If you have concerns about intellectual property related to your therapeutic candidate you should consult with your institution's technology transfer office and/or general counsel.
Which vectors can be used for reprogramming in order to be able to transplant the cells in patients?	CIRM does not advise on which methodologies to use. Methods selected should be appropriately justified and supported with data demonstrating use of the intended therapeutic candidate, in this case using the same vectors for reprogramming as those that will be used for the clinical product.

I developed a small molecule or biologic that targets endogenous stem cells under a CIRM Early Translational award. Am I eligible?	The ET Allowance Pathway in this RFA allows biologics that target endogenous stem cells to apply for a Disease Team III award. Such an approach would be eligible assuming that it is the same development candidate and the same target indication as was funded by the CIRM Early Translational award.
I am developing adoptive therapy using engineered T cells against targets with evidence of expression on cancer stem cells / tumor initiating cells. Is this approach applicable to apply for funding a Phase 1/2 clinical trial?	Adult T cells are not eligible for funding under this RFA. The list of eligible cell types can be found on pages 7-8 in the RFA.
Do the criteria mean that therapeutic antibodies targeting cancer stem cells in hematological malignancies are excluded?	If the antibody therapeutic targeting a cancer stem cell was developed under a Disease Team I or Early Translational award and is applying to Disease Team III for the same therapeutic candidate and the same disease indication, the approach would be eligible for funding.
Can we submit a proposal with a small molecule candidate targeting cancer stem cells?	If the small molecule targeting a cancer stem cell was developed under a Disease Team I or Early Translational award and is applying to Disease Team III for the same therapeutic candidate and the same disease indication, the approach would be eligible for funding. A small molecular targeting cancer stem cells which has not been previously funded by CIRM is not eligible for funding under this RFA.
Will there be an opportunity to send in additional supplemental data/progress after submission of the grant?	Yes. Section IX.C of the RFA addresses the ability to submit supplemental information.

The requirement in the RFA is to request a pre-IND by March 13. How rigid is this timeline?	Regulatory interaction and the resulting feedback from the FDA is an important indicator of readiness of the therapeutic candidate. As written in the RFA, a pre-IND meeting should be scheduled by the LOI submission due date (March 13, 2013) and the meeting should be completed by the full application submission date of May 15, 2013.
The RFA notes that applicants with projects beginning with a clinical trial must have filed a complete IND package with the FDA by the LOI due date (3/13/13). As a Disease Team I awardee, do I need to have a complete IND package filed with the FDA by the LOI submission date?	Disease Team I grant holders are expected to complete their IND filing under their existing award. Disease Team I awardees should contact their CIRM Science Officer to discuss the current timeline for IND filing to assess eligibility if you are considering applying to Disease Team III to propose a clinical trial with the same therapeutic candidate as was developed under the Disease Team I award.
Does a compound already in the clinic for another indication require a pre-IND meeting for CIRM Early Translational Allowance?	The Early Translational Allowance Pathway does not require a pre-IND meeting at the time of application. Awards through this pathway will have the objective of completing an IND filing within the award period and, therefore, may complete a pre-IND meeting during the award period.
Does a compound already in the clinic for another indication but NOT a CIRM ET project require a pre-IND meeting?	Regulatory interaction and feedback from the FDA is an important indicator of readiness of the therapeutic candidate. Since not all small molecules development programs utilize a pre-IND meeting, please contact CIRM to discuss expectations at the time of submission.

Can collaborators be located outside of California?	Collaborators can be located outside of California. The use of CIRM funding out side of California, however, is subject to some limitations. CIRM funds can be used applied to contractors (e.g. CROs), consultants, and clinical sites outside of California. Please see the RFA and CIRM's Grants Administration Policy (Section V – Payment and Use of Funds) for information on allowable use of CIRM funds. Additionally, this RFA includes participation from four Collaborative Funding Partners (MRC, NIH, MOST, IATA) to fund work in the participating country. See Appendices B-E for additional information on each CFP's terms.
Does the CMO used for manufacturing of non-clinical and clinical cells used in the proposed study be located in CA?	Contract manufacturers and other service providers can be located outside of California. Please see the RFA and CIRM's Grants Administration Policy (Section V – Payment and Use of Funds) for information on allowable use of CIRM funds.
Could funding from a Collaborative Funding Partner (CFP) be applied towards the required 25% matching funds for a proposed clinical trial using a small molecule or biologic (e.g antibody, protein)?	Funding provided by a CFP agency, supporting the work of a Partner PI, cannot be used to meet the matching funds requirement in the CIRM RFA. Similarly, the CIRM matching funds requirement only applies to the CIRM-funded part of the project — CFP funds do not require matching.
I'm developing a pluripotent stem cell-based therapy. Do I need to have matching funds for a proposed clinical trial?	The 25% matching requirement for a clinical trial only applies to small molecules and biologics (e.g. antibodies, proteins).