# WEBINAR – 5 November 2013
# RFA 13-06: Alpha Stem Cell Clinics
# RFA 13-07: Coordinating and Information Management Center

## Questions and Answers

| Questions | Answers |
|---|---|
| 1. What role could industry, with extensive experience planning and conducting clinical trials, have in this initiative, if any? | Industry could have a role in multiple aspects. RFA 13-07 is open to for-profit and non-profit applicant organizations. RFA 13-06 is also open to for-profit and non-profit academic medical Institutions as long as they meet the institutional eligibility criteria. In addition, companies could sponsor the Lead Clinical Trials and/or future trials that enter the Alpha Clinics. |
| 2. Is CIRM encouraging the establishment of collaborative relationships between Alpha Clinics and community-based hospitals that have access to diversified patient populations for clinical trial enrollment and which are often times highly qualified to conduct educational outreach? | CIRM recognizes the importance of demographically diversified patient populations for clinical trials enrollment and encourages the Alpha Clinics applicants to form partnerships that will advance the goal of improved access to clinical trials and educational outreach across all demographic groups. |
| 3. Do you take account of the fact that academic medical centers may have several sites of operation, such as several teaching hospitals, and that the Alpha Stem Cell Clinic activities may therefore be best carried out by using existing resources in several sites of the center? | Yes. We do take that into account. In the Instructions for RFA 13-06, the applicant is asked to describe existing institutional assets and infrastructure and how these would be leveraged into the implementation and operational plan for the Alpha Clinic. These assets could be at different locations within the academic medical center. |

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| 4. What do we do if we have already established a network of institutions to work together on clinical trials? Can this network submit as a group? | CIRM will create the Alpha Stem Cell Clinics Network by funding up to 5 alpha clinics under RFA 13-06 (each at a single institution and each with a distinct Program Director). If you participate in a current multi-institutional clinical trial network, each eligible institution in that network may submit an application. However, each application will be evaluated independently and must be "standalone" in terms of meeting the eligibility/scope of the award. |
| Questions Regarding Lead Clinical Trials:<br><br>5. If autologous stem cells are reintroduced into a patient after purification or expansion in numbers, would that be considered minimally manipulated and therefore out of scope?<br>6. Will CD34+ cells being used in a current phase III trial which may lead to approval qualify? | Lead Clinical Trials must meet the Eligibility Criteria as stated in RFA 13-06.<br>Eligibility criteria for the Lead Clinical Trial states that the candidate stem/progenitor cell product is tested in the FDA regulatory space (i.e. requiring an IND). Please refer to your own regulatory input or FDA interactions to determine whether, based on CFR 21 Part 1271, your candidate product is minimally manipulated (i.e. falls under section 361 of the PHS Act). |

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| 7. We are proposing 2 Lead Clinical Trials with a high degree of novelty (i.e. using pluripotent cell derivatives). However our institution also has a number of clinical trials using MSCs, which are experimental, but are "minimally manipulated" and not regulated by the FDA. Will it be advantageous for us to list these in the application as potential trials that can be carried out at the clinical site we are proposing? | The Lead Clinical Trials must meet the scope and eligibility criteria defined in RFA 13-06. The Alpha Stem Cell Clinic is also expected to support the conduct of a robust pipeline of clinical trials and activities in the long-term, as the field matures and more products are approved. The applicant may propose future clinical activities with stem cell therapies in the "Sustainability Plan and Pipeline" section of the application. The reviewers will evaluate "the feasibility and strength of the proposed pipeline of future clinical activities" |
| 8. How will future clinical trials entering the Alpha Stem Cell Clinics be selected? | Alpha Clinics Program Directors will determine future trials entering the network and they will receive guidance and advice from the Steering Committee, in which they also serve. |
| 9. What if the foremost lead trial will be actually started by the time of grant submission?" Is that acceptable? | If the Clinical Trial falls within the scope and eligibility of RFA 13-06, it would be allowable as a Lead Clinical Trial even if it has already started by the time of grant submission or award start date. |
| 10. Will the CASC award support GMP operations such as personnel, monitoring, and preventive maintenance? | Neither RFA 13-06 nor RFA 13-07 fund manufacturing activities or GMP operations, but the CIMC could provide regulatory support and other shared knowledge resources that could be useful to the GMP operations of projects/trials within the CASC network. |

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| 11. May we get clarification on what you mean by "minor modification of current cell therapy"? | Minor modifications would mean a variation of current therapy. Some examples are trials to study a new cell delivery device and to test a new imaging technique to evaluate cord blood or CD34+ cells already used in medical practice. An example of a “major modification” is gene modification of CD34+ cells or other stem/progenitor cells. |
| 12. What are the salary caps for this RFA? | Salary caps are found on the following page and apply to all RFAs. http://www.cirm.ca.gov/our-funding/grants-management#salary |
| 13. What rights will the Clinical Trial Sponsor have with respect to its inventions? | The inventions are owned by the Clinical Trial Sponsor. |
| 14. Is it required that the staff for all components of the CIMC be located at the same site within California? | To be eligible, the applicant organization must have a location in California from which it will engage in activities critical to the project. The facility shall be the primary place of business from which key personnel operate. The CIMC, its staff and management, must be configured in such a way as to achieve the mission of creating a critical mass of experienced staff, and critical resources readily accessible to the CASC Network participants. |