RFA 13-06 CIRM Alpha Stem Cell Clinics Network- Alpha Clinic Award
Grants Working Group
September 16 & 17. 2014

**Summary of the additional discussions during the Grant's Working Group Review**
*(Prepared at the request of a Patient Advocate member of the GWG)*

- During the review and discussions related to the Alpha Clinics applications, there were discussions by the reviewers that were unrelated to individual applications that at least one Patient advocate member thought were important to capture. Some of the discussions are summarized below.
    - Though the GWG felt that many of the applicants demonstrated strong institutional support and valuable assets, infrastructure and experience to bring to a network, a major focusing question was whether the additional resources would incrementally accelerate stem cell therapeutic development at their site. The reviewers discussed whether the proposed plans and resources would add value to what is already available at their site.
    - How to determine the incremental "value add" of a funded Alpha Clinic to resources already in existence at their site. In some cases, the applicant institution already had active clinical trial programs in (stem) cell therapies.
    - There was a difference in opinion as to whether shared activities and personnel between existing programs and the proposed alpha clinic should be considered a weakness or strength.

- Some reviewers had questions regarding the wording of the RFA and the potential for duplication of costs. Specifically, there was confusion over the portion of the RFA that listed "Clinical Trial Costs" as specifically being outside the scope of the RFA, while simultaneously permitting a funded Alpha Clinic to "defray some costs, such as clinical operations."

- The reviewers discussed the advantages and disadvantages of centers focused on 1 or 2 disease area(s) versus centers offering a broader repertoire of diseases and injuries. There were different opinions and reviewers were not asked to come to a consensus on this. It is noteworthy that one of the highest ranked applications had lead clinical trials focused on patients with cancer ---the first in patients with a CNS solid tumor and the other in AIDS patients with hematologic malignancy.

- In general, the reviewers thought it was a strength to have lead trials ready to go, as required by the RFA. Identification of lead trials enabled a tangible way to address the judgment of the applicants in the type and quality of

clinical trials they thought could be brought to the clinic. The GWG discussed "acceleration" and "value" with respect to the lead trials and/or acceleration of, and value to, future pipeline trials. In general, the panel felt that there would be a limited ability to accelerate the Lead Clinical Trials themselves; however, the reviewers thought that setting up these clinics and putting the proposed systems in place could accelerate, and make more efficient, future activities and pipeline trials.

- Some members of the GWG stated that many activities (i.e. pre-award activities, aspects of logistical and clinical operations) are not typically covered by clinical trial budgets and this is especially true of Investigator-initiated and small company trials.

- To prevent duplication, GWG recommends additional budget review to tease out the in-scope costs versus those already covered by Sponsors or existing institutional or other network resources, such as previous CIRM funding. Some reviewers raised questions about the overall appropriateness of the costs. Is the amount allowed too much? OK? Too little.

- In a discussion held after the review, some GWG members felt that it was necessary for the Network to implement the Coordination and Data Management Center (planned RFA) in order to realize the potential for the Alpha Clinics Network. Reviewers were not asked to come to a consensus on this. For instance, one of the reviewers with extensive experience in clinical networks felt that centralized data management was critical to the success of the network. Other reviewers questioned the role of the Coordination and Data Management Center if the Alpha Clinics are all to be located in one region of California.

In that same discussion, some members questioned the rationale for clinical trial center that offered their patients "stem cell only" or "stem cell directed" treatment options. The question was raised whether a technology focused, versus a disease focused network, would be the best way to accelerate stem cell therapy development. The GWG chair noted that this would be addressed by how one creates the "patient portal." Of note, the applicants (and prominently one of the Tier 1 applicants) do propose to leverage pre-existing disease networks to direct the specialized, stem cell focused activities within these disease networks, into the Alpha Clinics.