



Grants Working Group Public Review Summary

CIRM Alpha Stem Cell Clinic

Application Number: INFR4-10361

Review Date: July 25, 2017

Partnering Opportunity to Create a CIRM Alpha Stem Cell Clinic (INFR4)



REVIEW SUMMARY

08.23.17

INFRASTRUCTURE



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CIRM Alpha Stem Cell Clinic

APPLICATION NUMBER: INFR4-10361

REVIEW DATE: July 25, 2017

REQUEST FOR APPLICATIONS: INFR4 Partnering Opportunity to Create a CIRM Alpha Stem Cell Clinic

Application Summary

We propose a cellular trial operation that will focus on childhood hereditary hematological and immunologic disorders and in anti-inflammatory lung cellular therapies. This alpha clinic operation will expand participation by California citizens in these novel clinical themes.

The objective of this proposal is to expand the opportunity for California citizens to receive cellular therapies that have curative potential, especially in under-represented populations and diseases. We will accomplish this by giving therapy that can cure hereditary blood disorders, highlighted by an ongoing gene therapy trial in sickle cell disease, and in treating disorders of the lung caused by inflammation that accompany trauma and infection.

Funds Requested

\$7,999,999

Recommendation

Score: 1

Votes for Score 1 = 9 GWG members

Votes for Score 2 = 6 GWG members

Votes for Score 3 = 0 GWG members

- A score of "1" means that the application has exceptional merit and warrants funding;
- A score of "2" means that the application needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement if the Application Review Subcommittee has not approved an application for funding following the Grants Working Group's Review;
- A score of "3" means that the application is sufficiently flawed that it does not warrant funding.

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Review Overview

The proposed clinic would add value to the Network by extending the geographic reach to northern California, providing expertise in hematological/immunological disease indications and anti-inflammatory therapies and by fostering collaborations with other institutions in the state. Reviewers noted that the proposed clinic would expand access for minority patient populations in the state. Reviewers thought that the detailed clinical operations plan and leveraging of existing resources at the institution were a strength of the proposal. They had minor concerns about the fellowship program, collaboration between the two sites, the large number of unfilled positions and potential redundancy from multiple GMP manufacturing sites. However, they were satisfied with the applicant's responses to these concerns. Reviewers recommended the application for funding.

Review Summary

Will the proposed Alpha Clinic accelerate completion of stem cell therapy clinical trials, enhance the value of the Alpha Stem Cell Clinic Network, and be positioned to be sustainable?

- a) **Would the proposed clinic accelerate and/or expand patient access to stem cell clinical trials?**
- Addition of this site in northern California near several major academic centers could have a significant impact on expanding patient access to clinical trials.
 - The minority population outreach plans would expand patient access to stem cell clinical trials.
- b) **Would the proposed clinic expand the value of the Network, for example, broadening the Network's geographic reach, providing expertise in new disease areas, providing new/unique technical capability, or other elements that accelerate/support stem cell clinical trials. Does the proposed center offer a sufficient, impactful, and practical value proposition for patients, trial sponsors and/or health care providers?**
- The proposed clinic will expand the value of the Network by extending geographical reach to northern California.
 - The proposed clinic will add specific expertise in hematological/immunological disease areas and anti-inflammatory therapies to the Network.
 - The proposed clinic will enhance minority and pediatric patient participation in stem cell clinical trials.
 - The proposal had a strong focus on collaboration, not only between the applicant's two primary sites but also with other institutions in the state.

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c) Is the proposed clinic positioned to be sustainable beyond four years?

- The proposed clinic appears to have good institutional support and potential access to philanthropic funding mechanisms.
- The alpha clinic's reliance on the existing Clinical Translational and Science Center's resources could be a concern for long-term sustainability.
- The proposal outlined a business plan for sustainability but it was not detailed.

Has the applicant developed a plan designed to successfully establish and operationalize the Alpha Clinic?

a) Does the project plan and timeline for establishing the center, conducting clinical trials, and implementing the pipeline development plan demonstrate an urgency that is commensurate with CIRM's mission?

- The project plan and timeline demonstrate an urgency that is commensurate with CIRM's mission.

b) Does the application propose all required core activities (including the Fellows Program) and are they appropriately designed to meaningfully enhance the value of the Network?

- All core activities were proposed.
- The proposal lacked detail with respect to the design of the fellowship program. Reviewers thought that the applicant's response to their request for more information, which indicated that the fellowship program would build on existing programs and provide comprehensive training in stem cell therapy development, was satisfactory.
- Reviewers were unclear on the need for multiple GMP facilities and expressed concern about redundancy. Reviewers thought that the applicant's response to their request for more information, which indicated that the multiple facilities would be matched to disease expertise and that CIRM funding would be used to improve collaboration and regulatory expertise, was satisfactory.

c) Is the operation of the clinic appropriately planned and designed to provide meaningful, accelerating, and impactful resources dedicated to stem cell clinical trials?

- Detailed plans were provided for stem cell therapy clinical trial operations across the two sites.
- The fact that the bulk of the clinic activity won't be performed at the Program Director's home site was a concern for some reviewers.

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Is the proposal feasible?

a) Is the proposed plan, including the fellows program and pipeline development plan, feasible and likely to be implemented within the proposed timeline?

- The proposed clinic will leverage existing Clinical and Translational Science Center resources at the applicant institution.
- Some reviewers thought that tying together existing infrastructure at the two sites could be challenging.

b) Is the proposed team appropriately qualified and staffed and have access to all the necessary resources to establish, operate, and maintain the clinic?

- The Center Director has a strong reputation and the identified members of the team are well qualified.
- Reviewers initially expressed concern about the number of unfilled positions on the team but were reassured when the applicant indicated that candidates had been identified for these open positions.

c) Will the clinic have the capability and resources to provide the required core services to support stem cell clinical trials?

- The proposed clinic will have the capability and resources to provide the required core services.

d) Does the team have a viable contingency plan to manage risks and delays?

- A viable contingency plan was provided but it mostly focused on common systemic risks and lacked specific details.