



Grants Working Group Public Review Summary

*The Alpha Clinic: A Partner in the Advancement of Cell Therapy
Research*

Application Number: INFR4-10313

Review Date: July 25, 2017

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



REVIEW SUMMARY

08.23.17

INFRASTRUCTURE



CIRM
2.0

CALIFORNIA'S
STEM CELL
AGENCY

The Alpha Clinic: A Partner in the Advancement of Cell Therapy Research

APPLICATION NUMBER: INFR4-10316

REVIEW DATE: July 25, 2017

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

Application Summary

The Alpha Clinic plans to provide the necessary framework for expanding patient access to well-designed and scientifically sound stem cell trials and to support researchers in their quest to contribute to advancing the science and bringing FDA approved stem cell therapy to the clinic! With funding support from CIRM, we will have the necessary means to create a stem cell Alpha Clinic.

One of our main objectives is to provide access for the citizens of California who may benefit from stem cell therapy while providing the best care possible. Hopefully one day, our alpha clinic will not only offer investigational stem cell therapy but also clinically approved ones as part of standard of care!

Funds Requested

\$7,999,999

Recommendation

Score: 1

Votes for Score 1 = 7 GWG members

Votes for Score 2 = 4 GWG members

Votes for Score 3 = 2 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.

INFRASTRUCTURE



CIRM
2.0

CALIFORNIA'S
STEM CELL
AGENCY

Review Overview

The proposed Alpha Clinic leverages the applicant's expertise in operating cell therapy clinical trials in cardiovascular, neuromuscular and neurodegenerative disease indications. Reviewers thought that the proposed Alpha Clinic would expand the geographic reach of the network by utilizing its satellite sites and expanding its telemedicine capabilities. Reviewers expressed minor concerns about the stem cell technology platform, fellowship program and satellite sites. Reviewers noted that the team is appropriately qualified and the operational plan for the proposed clinic is feasible. They 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?**
 - The proposed alpha clinic would expand patient access by allowing better integration of, and access to, existing trials in cardiovascular, neuromuscular and neurodegenerative disease indications.
- 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 expertise in stem cell-based treatment approaches for cardiovascular, neuromuscular and neurodegenerative disease indications.
 - The proposed clinic has the potential to expand geographic reach into northern California by engaging its satellite sites. However, reviewers questioned the feasibility of conducting stem cell trials at the proposed satellite sites.
- c) **Is the proposed clinic positioned to be sustainable beyond four years?**
 - Reviewers found that the sustainability plan, which would rely on various sources of funding including grants, philanthropy and revenue from testing services, to be reasonable and feasible.
 - Some reviewers were concerned that sustainability of the alpha clinic would be partly reliant on successful clinical translation of the applicant's stem cell technology platform.

INFRASTRUCTURE



CIRM
2.0

CALIFORNIA'S
STEM CELL
AGENCY

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 timeline demonstrates an urgency 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?

- In general, the proposed application contains all the required core activities to enhance the value of the Network.
- It was unclear how the fellowship training program would be tailored to build specific expertise in cell therapies.
- It was unclear why the fellowship training program couldn't support more than 2 fellows per year.
- The plan for scaling up the GMP manufacturing facility to support greater volume and diversity of cell-based therapies was unclear.
- It was unclear whether the applicant would support clinical trials for potential competitors of its own stem cell platform technology.
- The applicant's plan to utilize telemedicine at satellite sites has the potential to improve patient access and add value to the network.

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

- The applicant is already successfully performing cell therapy trials.
- The proposed plan to operationalize the clinic is appropriately designed.

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 applicant has expertise in cell therapy clinical trials and has presented a reasonable rollout plan for the alpha clinic.

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 proposed team is appropriately qualified and has already demonstrated the ability to lead fellowship programs, obtain funding, manage GMP manufacturing and operate stem cell trials.

INFRASTRUCTURE



CIRM
2.0

CALIFORNIA'S
STEM CELL
AGENCY

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

- The applicant has the capability and resources to support stem cell trials.

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

- The applicant has a viable contingency plan and appropriate funding to manage risks and delays.