



Grants Working Group Public Review Summary

A Translating Center Designed to Expedite the Development and
Delivery of High Quality Stem Cell Therapies to Patients

Application Number: INFR2-09298

Review Date: October 4, 2016

Partnering Opportunity to Create a CIRM Translating Center (INFR2)



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A Translating Center Designed to Expedite the Development and Delivery of High Quality Stem Cell Therapies to Patients

APPLICATION NUMBER: INFR2-09298

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REQUEST FOR APPLICATIONS: INFR2 Partnering Opportunity to Create a CIRM Translating Center

Summary

The Translating Center (TC) proposed by the applicant is focused on reshaping the preclinical research paradigm and supporting CIRM's vision to dramatically reduce the cycle time between candidate nomination and first-in-human (FIH) studies. The TC will be a CA-based organization providing preclinical support services for cell based therapies to CIRM clinical grantees and other academic / industry entities worldwide.

While the delivery of its core services is vital, the TC's true differentiator is its vision and seamlessly transitioning the treatments into patient focused clinical programs in concert with Quintiles' Accelerating Center. Our ultimate goal is to empower the TC and other elements CIRM infrastructure to become the premier enabler of cell based therapies to patients in CA and worldwide.

Funds Requested

\$15,000,000

Recommendation

Score: 89

Mean = 89

Median = 90

Standard Deviation = 5

Recommended = 85-100

Not Recommended = 1-84

For programs for which only one application will be funded, the application receiving the highest average scientific score of 85 or above will be deemed to be the GWG's recommendation for funding. If no application receives a score of 85 or above, all applicants shall have the opportunity to submit an amended application, based on the summary of the GWG review, to address reviewer concerns. The GWG shall conduct a supplemental review of the amended applications and re-score the applications using the same range (1-100). The application that receives the highest average score of 85 or above shall be deemed to be the application recommended for funding by the GWG.

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Review Overview

The applicant organization is a large and successful organization that appears to be fully dedicated to establishing a CIRM Translating Center capable of accelerating progression of late stage preclinical products to IND filing and into clinical testing. Given the wealth of resources that will be dedicated to the center by the parent organization, the key collaborations built by the applicant organization to offer the core services, and the commitment of the applicant organization to executing the sound plan to operationalize and sustain the CIRM Translating Center as envisioned in the request for applications (RFA), reviewers enthusiastically recommended this application for funding.

Review Summary

Does the proposed center hold the necessary significance and potential for impact?

- a) **Consider whether the proposed center is likely to accelerate the progression of stem cell projects from the late preclinical stage to IND filing and conduct of clinical trials.**
 - The proposed center is likely to accelerate progression of stem cell projects from the late preclinical stage to IND filing and conduct of clinical trials if the center is established and operationalized as described and envisioned by the applicant.
- b) **Consider whether the proposed center offers a sufficient, impactful, and practical value proposition for preclinical researchers, patients, and/or healthcare providers by increasing the speed and quality of preclinical stage stem cell projects.**
 - There is a strong value proposition offered to researchers, patients, and healthcare providers in aggregating expertise and experience as proposed in this application. The applicant recognizes the value of and optimizes opportunities for learning across products and projects and across its collaborating organizations.
 - The applicant organization understands and articulates the critical components of the value proposition to researchers, patients, and healthcare providers of the CIRM Translating Center and the proposed plan maximizes these components. As such, clientele will retain individual intellectual property related to the product, and the CIRM Translating Center will aggregate stem cell preclinical development know-how to accelerate the speed and improve the quality of preclinical stage stem cell projects.

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

- a) **Consider whether the center is likely to seamlessly integrate with the CIRM Accelerating Center to accelerate CIRM-funded stem cell projects.**
 - The applicant organization has a sound plan to integrate with the CIRM Accelerating Center to accelerate preclinical development of CIRM-funded stem cell projects.
 - The applicant organization is in a unique position to effectively collaborate with

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the CIRM Accelerating Center.

- b) **Consider whether the operation of the center is appropriately planned and designed to provide meaningful, accelerating, and impactful resources (including the required core services) for the conduct of stem cell preclinical research.**
- There is a well thought-out and comprehensive plan to provide meaningful, accelerating, and impactful resources in all the core services for the conduct of stem cell preclinical research.
 - Reviewers noted some capacity concerns with manufacturing core services. While the proposed manufacturing services are of the highest quality, current capacity is limited. There is a reasonable plan to prioritize CIRM projects and to improve capacity should this become an issue as the center experiences growth, but this is an area that will need monitoring as the center is operationalized.
 - There was concern that despite the applicant's size and breadth of expertise, specialized animal models (for example, surgical models) might be better done outside the center, and the applicant would need to be flexible and work with outside groups that have specialized expertise. The applicant assured reviewers that a la carte services would be available and that outsourcing would be done as necessary.
- c) **Consider whether the project plan and timeline for establishing the center demonstrate an urgency that is commensurate with CIRM's mission?**
- The project plan is aligned with and commensurate with CIRM's mission to accelerate stem cell treatments to patients with unmet medical needs.
 - The timeline demonstrates an urgency commensurate with CIRM's mission.
- d) **Consider whether there is an effective plan proposed to provide specialized services that are unique to diverse stem cell and gene modified cell treatments.**
- The applicant organization has effectively partnered with leading manufacturing and preclinical research organizations and has an effective plan in place to provide specialized services for diverse stem cell and gene modified cell treatments.
- e) **Consider whether the business and sustainability plan is appropriate to serve CIRM projects with competitive pricing while ensuring sustainability of the center beyond five years.**
- There is an appropriate marketing plan in place to support sustainability, and it is clear that the applicant organization will put its extensive resources into establishing a sustainable CIRM Translating Center.
 - The discounting plan is sufficient to provide competitive pricing. The plan offered by the applicant institution is excellent. The plan offered by the collaborating organizations is acceptable.

Is the project feasible?

- a) **Consider whether the proposed center is likely to be established within the proposed timeline.**
- It is likely that the proposed center will be established within the proposed timeline.

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b) Consider whether the proposed team appropriately qualified and staffed.

- The applicant organization is extremely successful, experienced, and qualified.
- The proposed Center Director (CD) does not have the level of experience desired by reviewers. However, it is apparent that the parent organization is fully supportive of the CD and will do everything necessary to ensure his/her success in the role.
- Amongst the collaborating organizations there are extensive qualifications and expertise in all of the required core service areas and the great synergy between the collaborating organizations only improves upon those qualifications.

c) Consider whether the team has access to all the necessary resources, including necessary collaborations and partnerships, and whether their track record support feasibility to establish, equip, operate, and maintain the center.

- The applicant organization has formed most of the necessary collaborations and partnerships to establish, equip, operate, and maintain the proposed center.

d) Consider whether the center will have the capability and resources to provide the required core services.

- The center has the capacity and resources to provide all required core services.
- While it is not clear that there is sufficient manufacturing capacity to serve all potential clientele, capacity is likely sufficient for initial operations and there are plans in place to acquire additional manufacturing capacity should it be necessary.

e) Consider whether the team has a viable contingency plan to manage risks and delays.

- There is a viable contingency plan to manage risks and delays, particularly as it pertains to expanding the cell therapy market.

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CIRM Recommendation to Application Review Subcommittee

The CIRM recommendation to the Application Review Subcommittee is considered after the GWG review and did not affect the GWG outcome or summary. This section will be posted publicly.

RECOMMENDATION: Fund (CIRM concurs with the GWG recommendation).