

Grants Working Group Public Review Summary

An Accelerating Center designed to expedite the development and delivery of high quality stem cell therapies to patients

Application Number: INFR1-09166

Review Date: May 23-24, 2016

Partnering Opportunity to Create a CIRM Accelerating Center (INFR1)



REVIEW SUMMARY

06.03.16



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An Accelerating Center designed to expedite the development and delivery of high quality stem cell therapies to patients

APPLICATION NUMBER: INFR1-09166

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REQUEST FOR APPLICATIONS: INFR1 Partnering Opportunity to Create a CIRM Accelerating Center

Project Objective

A successful partnership, resulting in the creation of an Accelerating Center recognized as the leader in providing end-to-end clinical development services (regulatory, clinical, data management) to accelerate the availability of stem cell treatments to patients with unmet medical needs.

Statement of Benefit to California

The proposed collaborative research will benefit the lives of patients in CA with serious unmet medical needs by accelerating the development and delivery of high quality stem cell treatments and establishing CA's position as a leader in advancing stem cell research. As the leading center of regenerative medicine, CA will attract patient advocates, drive science and academic innovation and reduce health care costs through curative treatments and stimulating economic growth.

Funds Requested

\$15,000,942

Recommendation

Score: 89

Mean = 89

Median = 90

Standard Deviation = 4

Recommended for Funding = score 85 and above, representing applications that have exceptional merit and that warrant funding, if funds are available; or

Not Recommended for Funding = score below 85, representing applications that are not recommended for funding



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Review Overview

Reviewers thought the applicant to be highly capable of delivering the required core services and establishing and sustaining the proposed CIRM Accelerating Center. More importantly, reviewers thought the applicant had given great thought about how best to partner with CIRM to accelerate delivery of stem cell treatments to patients with unmet medical needs by leveraging CIRM Infrastructure Programs and offering innovative and impactful preclinical and clinical development resources to both CIRM and non-CIRM funded investigators.

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 projects from the late preclinical to clinical stage and the conduct of safe, efficient, and effective stem cell clinical trials.**
 - The applicant carefully considered the existing CIRM Infrastructure Programs and how to best integrate into this ecosystem to accelerate delivery of stem cell treatments to patients and increase throughput to commercialization.
 - The proposed CIRM Accelerating Center should be able to accommodate the needs of rare disease and surgically intensive treatments to accelerate stem cell treatments to these patients.
 - While there is a lack of stem cell experience in the team, there is a clear commitment and plan to aggregate stem cell expertise and leverage knowledge gained over time to build a center focused on accelerating progression of stem cell projects toward commercialization.
- b) **Consider whether the proposed center is positioned to be sustainable beyond five years.**
 - The track record and clear commitment of the parent company suggest that the center is positioned to be sustainable.
- c) **Consider whether the proposed center offers a sufficient, impactful, and practical value proposition for trial sponsors, patients and/or health care providers.**
 - The applicant offers an impactful value proposition, particularly through its innovative ideas to reduce time for clinical development, recruit patients, decrease the number of patients needed, and accelerate progression through the regulatory pathway.

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

- a) **Consider whether the operation of the center is appropriately planned and designed to provide meaningful, accelerating, and impactful resources (including the required core services) to stem cell clinical trials.**
 - The operation of the center is planned to aggregate stem cell-specific expertise and provide resources to accelerate development of stem cell treatments to patients and improve the overall quality of the clinical development plans and the treatments developed.
 - The applicant is innovative in its approach to patient recruitment and could



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accelerate trial enrollment by applying the proposed approaches to recruitment in stem cell therapy clinical trials.

b) Consider whether the project plan and timeline for establishing the center demonstrate an urgency that is commensurate with CIRM's mission.

- The timelines demonstrate an urgency commensurate with CIRM's mission.

c) Consider whether there is an effective plan proposed to provide optimal access and support for CIRM-funded projects while the Center builds its business with non-CIRM funded projects?

- The applicant has articulated a plan to create a semi-autonomous center focused on developing key capabilities in the stem cell space while utilizing the parent company to provide key services for CIRM-funded projects.

d) Consider whether the proposed fee plan is competitive for CIRM-funded projects.

- The proposed fee plan is competitive as a preferred customer discount will be provided in addition to the CIRM-funded discounts.

Is the project feasible?

a) Consider whether the proposed center is likely to be established within the proposed timeline.

- The center is likely to be established and capable of providing end-to-end services within the proposed timelines.

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

- The team is highly experienced in late stage preclinical and clinical development, clinical operations, and data management.
- The applicant lacks expertise in stem cell clinical and product development.
- Reviewers were not convinced that the proposed Center Director is sufficiently experienced to direct the CIRM Accelerating Center. However, there is great confidence in the parent organization, which alleviated some of the concerns.
- The proposed advisory boards are an added strength.
- There are well thought out job roles for each of the lead team members.

c) Consider whether the team has access to all the necessary resources to establish, equip, operate, and maintain the center.

- The team has access to all necessary resources to establish, equip, operate, and maintain the center.
- The proposed flex resourcing model should allow the center to rapidly meet demand.

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

- The applicant is highly capable of providing the core services and has access to all necessary resources.
- The applicant clearly understands the complexity of data management and appears committed to developing a resource to support broad learnings that will be made available to the community.

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



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and delays.

- The resources of the parent company ensure that a viable contingency plan is in place, and it appears the company is committed to the CIRM Accelerating Center even if it is cash neutral.



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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).