



Alpha Stem Cell Clinics Network Expansion Award

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CONCEPT PROPOSAL

01.26.17



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Objective

The mission of the California Institute for Regenerative Medicine (CIRM) is to accelerate stem cell treatments to patients with unmet medical needs. To support this mission, CIRM created the Alpha Stem Cell Clinic Network to conduct high-quality stem cell clinical trials. The Network currently includes sites at University of California San Diego, City of Hope Duarte, University of California Los Angeles, and University of California Irvine. Twenty-nine clinical trials are being conducted at these sites and hundreds of patients have been enrolled. These trials emanate from CIRM's funding pipeline as well as non-CIRM funded investigator and industry sponsored projects.

The objective of the Network Expansion Award is to support additional Alpha Clinic sites that will (1) deliver core services necessary to conduct high-quality stem cell clinical trials, (2) support the career development of physicians seeking to perform clinical trial and (3) enhance the value of the network, as a whole. Proposed sites could enhance the value by, 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.

The Award provides infrastructure funding to California-based medical centers to operate an Alpha Stem Cell Clinic. The clinics will provide a platform (personnel, facilities and operations) specifically dedicated to supporting the unique needs of clinical trials for investigational cell based therapies. Funds from this award shall be used to leverage existing assets within the medical center to support the investigation of cell based products. The award does not directly fund the clinical trials or the construction of new facilities.

Organizations funded under the CIRM Infrastructure Programs must participate in a coordinated effort to develop systems and capacities to accelerate the efficient delivery of treatments to patients. This effort will be facilitated by the Alpha Stem Cell Clinics Network Steering Committee. The Committee comprises the Program Directors from each Alpha Clinic, a CIRM representative and a designee from the CIRM Stem Cell Center, which provides translational and clinical services to CIRM awardees.

The Alpha Stem Cell Clinics Network is one of multiple coordinated Infrastructure Programs designed to overcome obstacles and accelerate the progression of stem cell treatments through translational, preclinical, and clinical research to demonstrate clinical proof of concept.¹ Other Infrastructure Programs include the CIRM Stem Cell Center. Together, these CIRM Infrastructure Programs will support CIRM-funded

¹ See Beyond CIRM 2.0: 2016 Strategic Plan p. 26 -30.
https://www.cirm.ca.gov/sites/default/files/files/agenda/151217_Agenda_7_CIRM_StratPlan_final_120815.pdf

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translational, preclinical, and clinical projects to develop stem cell treatments for patients with unmet medical needs.

Award Information

What is the CIRM funding allocation and project term?

Under this RFA, up to two organizations will be supported to develop and operate the Alpha Stem Cell Clinics within California. Each award will provide up to \$8.0 million in total funding over a four-year period.

What activities must be performed under the CIRM award?

The Alpha Stem Cell Clinic will contract with sponsors to conduct CIRM-funded and non CIRM-funded clinical trials. The clinic must provide the following Core Services for cell-based clinical trials and contribute to the development of the Network:

- ✓ **Patient access, recruitment, education and consent.** Enable patients in California to access the Alpha Stem Cell Clinics Network. Maintain systems designed to identify and recruit patients. Provide screening and education services to support robust informed consent. These systems should include the capacity to effectively recruit and educate among California's diverse population.
- ✓ **Clinical trial operations and management services.** Work with sponsors to facilitate clinical trial agreements/contracts and required assurances. Provide coordinated care for patients enrolled in a clinical trial. Develop operations and management systems to address the unique technical needs of cell-based clinical trials – including but not limited to specialized platforms for cell processing, manufacturing and delivery – with the aim of achieving optimal outcomes.
- ✓ **Physician training and career development.** Develop a CIRM Alpha Clinic Fellows Program to support the career development of physicians seeking to perform stem cell clinical trials. This program should orient physicians to clinical and regulatory considerations unique to cell-based therapies. Training may include (1) regulatory strategy, (2) protocol design and (3) data collection, management and reporting methods to support successful trials and product commercialization.
- ✓ **Network participation and development.** Contribute to the development of a Network designed to expand and accelerate stem cell treatments to patients with unmet medical needs. Adopt established procedures and policies developed by the Network Steering Committee. Provide a proportional contribution to the development of Network wide assets deemed to be a priority by the Alpha Stem Cell Clinics Network Steering Committee.

What activities will CIRM not fund?

CIRM funds cannot be used to support the following activities under this opportunity:

- ✗ Activities already paid for by CIRM under a prior or existing award, i.e., double billing
- ✗ Construction or renovation of physical facilities
- ✗ Subsidizing direct costs of core services provided to non-CIRM funded projects

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What are the expected outcomes of the CIRM Alpha Clinics Network?

The CIRM Alpha Clinics Network must demonstrate substantial progress toward improving the efficiency and effectiveness of stem-cell clinical trials as measured by performance metrics. These performance metrics, compiled by CIRM through Network progress reporting, must demonstrate continuous improvements in trial startup time, protocol optimization, and pipeline expansion.

Integration with CIRM Infrastructure Programs. The Expansion Sites will coordinate with the existing Alpha Clinics Network to bring new stem cell clinical trials to the network and leverage, improve upon, and scale up the services and unique resources provided by the Alpha Clinics Network, so called AVARs (Accelerating and Value Added Resources). Expansion sites should propose new AVARs and/or facilitate the dissemination of AVARs to support stem cell clinical trials throughout the state. The Alpha Clinics Network Steering Committee, which will include representatives from the Expansion Sites, will coordinate these activities.

Sustainability Plan. The Expansion Sites are expected to develop a sustainability plan designed to leverage these assets in the context of CIRM's Infrastructure Programs. The aim of this leveraging strategy is to create a sustainable platform for ongoing delivery of stem cell treatments to patients.

The Sustainability plan must be designed to (i) maximize broad access to sponsors who are developing stem cell treatments, (ii) increase the probability of creating a sustainable platform and (iii) minimize competing interests to support development.

How will funds be awarded?

CIRM will disburse funds pursuant to a Notice of Award (NOA) and based on operational milestones. **Costs resulting from a delay or failure to meet an operational milestone will be the sole responsibility of the recipient.** Successful applicants will have thoughtfully accounted for foreseeable project risks and developed contingency plans that **do not** require additional funding from CIRM.

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Eligibility

What are the eligibility requirements for partnering with CIRM?

To be eligible, the proposed project must satisfy the following requirements:

(1) Must be ready to initiate work on the funded project within 45 days of approval

Given the urgency of CIRM's mission, all approved awardees must initiate work on the funded project within 45 days of approval and authorization for funding by the Application Review Subcommittee of CIRM's governing board, the Independent Citizens' Oversight Committee ("ICOC").

(2) Must have a California operating location

Both non-profit and for-profit organizations are eligible to apply. At the time of the application deadline, the applicant organization must be located in California (that is, the organization must have employees who are conducting business or operations at a leased or owned location in California). At the time of application, the applicant organization must have the appropriate hospital/clinics and facilities accreditations and operational medical facilities in California. If these requirements are not met, CIRM may terminate all further action on the application.

(3) Must have demonstrated ability to perform stem cell clinical trials

Applicants must currently be conducting at least one clinical trial that meets the eligibility criteria for a CIRM [Clinical Trial Stage Projects](#). Applicants must identify two lead clinical trials to be initiated no later than 90 days after the Notice of Award. The lead clinical trials must conform to the eligibility criteria for CIRM [Clinical Trial Stage Projects](#). Applicants must also provide a Pipeline Development Plan for attracting additional eligible clinical trials.

(4) Must reside in an existing academic medical center with demonstrated capacity and potential for impact

Applicants must be located within an academic medical center², licensed by the California Department of Public Health, that is currently performing regulated clinical trials registered on ClinicalTrials.gov. The medical center should consist of the necessary facilities and infrastructure to successfully implement stem cell clinical trials that meet the eligibility criteria for a CIRM [Clinical Trial Stage Projects](#).

(5) For-Profit applicants must demonstrate solvency

For-profit applicants must provide documentation indicating the entity is a going concern with a minimum of 180-days cash on-hand from the date of application submission to fund operations and the financial ability to meet the contingency requirements for the term of the project.

² Defined as a medical center that has an accredited medical school and/or accredited medical residency or fellowship programs and that conducts regulated clinical trials.

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For non-profit organizations that have been in existence for less than 5 years, the organization must demonstrate accounting systems capable of tracking the utilization of CIRM funds.

(6) Must not currently have an onsite CIRM Alpha Clinic program.

Applicants must not currently have an active Alpha Clinic on site.

(7) Application must be accurate and complete

All required components of the application must be completed and may not contain false or inaccurate information.

Who can serve as the Center Director (CD)?

To be eligible, the CD must satisfy the following requirements:

Must be authorized by the Institution's Authorized Organizational Officer to apply for this RFA.

- Must have an active California medical license and hospital privileges at the applicant institution's medical center.
- The CD must commit a minimum 30% effort at the initiation of the award (grant start date), with the expectation that this commitment would increase to 100% if needed.

Schedule and Deadlines

Applications Due	2:00 PM PT, May 15, 2017
Grants Working Group (GWG) Review	June or July 2017
ICOC Review and Approval	August 2017
Award Start	Must start within 45 days of award approval