CONCEPT PLAN FOR ATP3

Objective

The mission of the California Institute for Regenerative Medicine ("CIRM") is to accelerate the development of stem cell treatments to patients with unmet medical needs.

To meet this mission, CIRM has invested approximately $2B in developing a portfolio of over 300 technologies and is aggressively expanding its portfolio under CIRM 2.0. Under the current five year strategic plan, CIRM has positioned itself to most efficiently and proactively "push" these programs through the preclinical stage and into clinical development and to patients. However, there is currently insufficient industry "pull" to take these technologies to the marketplace, where groundbreaking science can benefit the greatest number of patients.

This RFA will fund a public private partnership to advance high quality CIRM-funded stem cell technologies toward commercialization and to patients. In keeping with its mission and as the public partner, CIRM will provide continued funding for an aggregated group of existing CIRM projects that are selected by the Industry partner for in-licensing and continued development to commercialization. CIRM, by leveraging its review and administrative infrastructure, will provide valuable insight and rigor to the process by which the Industry partner chooses its aggregated portfolio. The Industry partner will be required to invest a significant sum of capital to execute on the business plan and to achieve the goals of this initiative.

Though CIRM funds will not be used directly to form the private entity (industry partner), it is envisioned that CIRM's funding commitment for continued research and development of the chosen CIRM projects will attract and incentivize a world class management team to take on this portfolio of "high risk" but high reward projects. In this way, CIRM can bring in the necessary expertise resident within the Industry sector to take its most promising cell therapeutics to the late stages of development and increase the chance of commercialization and of reaching patients.

Upholding its commitment to the public and to the researchers, CIRM will require that the pricing and access provisions common to other CIRM awards will be in full force, as will CIRM's "March-In Rights," which enable CIRM to retake control of any program that is exclusively licensed and subsequently abandoned.

The aggregation of a basket of otherwise unpartnered CIRM projects offers the successful applicant "multiple shots on goal." This increases the probability of successfully developing and commercializing a stem cell treatment, and makes significant industry investment in stem cell technology more attractive. The program also benefits other important stakeholders:

- For Researchers – continued funding for the advancement of their CIRM project.
- For Universities - demand creation for the out-licensing of CIRM-funded technologies with a greater opportunity to achieve a financial return due to the aggregation of risk.

- For Citizens of California – the creation of an industrial stem cell therapeutic powerhouse that expands the tax base, adds high quality jobs, and increases the likelihood of the commercialization of stem cell therapies for patients with unmet needs.

Successful applicants will demonstrate their commitment to the continued research, development, and commercialization of CIRM projects by:

- creating an exceptional business plan that describes the synergies they intend to realize through their technology aggregation strategy,

- assembling a top-tier leadership team with the skill set necessary to successfully execute the business plan, and

- securing the significant investment capital necessary for long-term success.

Consistent with CIRM 2.0 principles, the Institute will bring to bear both its internal resources and vast external team of world-class subject matter experts to actively advance the projects being funded. The result of a successful application will be the formation of a true public-private partnership that accelerates the various projects selected and gives them the greatest opportunity to truly benefit patients in need.

Award Information

What is the CIRM funding allocation and project term?

Under this program, a single applicant will be funded over a period of five years to continue the development of CIRM-funded projects. CIRM will allocate a total of up to $75 million to this program, contingent upon at least a 1 to 1 capital investment from the applicant.

What activities will CIRM fund?

CIRM resources may only be used to support therapeutically focused activities involving CIRM-funded programs chosen by the awardee for in-licensing and development, referred to as CIRM's most promising technologies ("MPTs"). CIRM will support the following activities under this opportunity.

- ✓ Activities that are eligible for funding under any of the CIRM 2.0 Discovery, Translational and Clinical Program award mechanisms
- ✓ Translational, preclinical, and IND-enabling studies
- ✓ Phase 1, Phase 2, or Phase 3 clinical trials
- ✓ Process Development and Manufacturing
- ✓ Utilization of CIRM infrastructure to accelerate the above activities (i.e., Translating Center, Accelerating Center, Alpha Clinics Network)
- ✓ Research and development costs for projects that have been approved by CIRM

What activities will CIRM not fund?

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

- ✗ Activities that are already funded by CIRM under a prior award
- ✗ Early research and translation for candidate discovery/selection
- ✗ Construction or renovation of physical infrastructure
- ✗ Formation, operation and management of the applicant entity
- ✗ Development activities not involving CIRM MPTs; the successful entity may utilize its own funds to develop non-CIRM projects but it may not utilize CIRM funds to do so.

How will funds be awarded?

This award will fund research activities to advance CIRM MPTs along the development path to commercialization. This award will be funded as a grant and/or loan, with the terms to be defined after careful consideration of the options by the CIRM team and subject to the consideration and approval of such terms by the Science and Intellectual Property and Industry Subcommittees at a joint meeting.

Successful applicants will have thoughtfully accounted for foreseeable project risks and developed contingency plans that **do not** involve additional funding from CIRM.

Project Requirements

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

(1) Capital Investment requirements

Successful applicants will be awarded up to a total of $75 million. CIRM will require the applicant to commit $75M upfront and this may come from any funding source arranged by the applicant, but may not include “in-kind” or similar types of support. CIRM will provide funding on a reimbursement basis upon the applicant’s demonstration that it has expended CIRM funds on in scope activities as describe above and that the applicant entity has invested an equivalent amount of money to advance the company’s cellular therapy enterprise, including research costs, licensing, and operations.

(2) CIRM Review and Approval of In-Licensed MPTs

Successful applicants must propose and obtain CIRM’s approval (GWG and Application Review Subcommittee) to in-license CIRM MPTs. Applicants must also agree to provide licensors of CIRM projects with a right of first refusal for the return of the CIRM-funded technology back if the applicant decides not to develop it.

(3) Operations must be located in California

Successful applicants must be California-based organizations. To qualify as a California organization, an applicant must have >50% of its employees located in, and paid in, the state of California, and the award activities must be managed from the California location. Successful applicants may use CIRM funds for eligible project costs incurred both in California and outside California.

For clinical stage projects, applicants are expected to utilize clinical trial sites and conduct other research activities in California to the extent possible, and should provide justification for activities outside the State.

(4) Must propose an experienced management team and board of directors on par with the magnitude and scope of the proposal

Applicants must propose a core team, including a chief executive officer, chief scientific and/or medical officer, financial executive, project manager, and intellectual property and licensing expert. The project manager must have experience in managing clinical development programs and must be able to devote 100 percent effort to the project.

(5) Must provide a business plan

Applicants will be responsible for:

- Identifying and proposing an area(s) of focus based on current CIRM portfolio and indicate which candidates they would initially pursue as MPTs. Successful applicants will be responsible for identifying the MPTs (with input from CIRM through CIRM’s peer review process), building the portfolio, and executing on the proposed business plan.
- Proposing a development and commercialization plan for the portfolio of MPTs.
- Outlining plans for value creation, additional fundraising, value inflection points, liquidity event(s) that will provide return to the stakeholders within the five-year award period.

Who can apply?

This opportunity is open exclusively to organizations proposed to be based within California that are able to clearly demonstrate the track record and expertise necessary to assemble a world class management team, and board of directors, to advance the development and commercialization of stem cell therapies.

Who can serve as the Program Director (PD)

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

- Must be an officer of the applicant company.
- Must commit 100 percent effort to working on the project (note: "project" includes both the CIRM-funded and applicant co-funded components). Any effort for which salary from CIRM is claimed must be expended in California
- Must not currently have another application pending review or approval under this partnering opportunity

Schedule and Deadlines[1]

Letters of Intent Due	1H 2016
Applications Due	2H2016
Grants Working Group (GWG) Review	2H2016
ICOC Review and Approval	2H2016
Award Start	Must start within 60 days of award approval (i.e., approximately 145 days post submission)

[1] This schedule is subject to change by CIRM's President depending upon CIRM's resources and priorities.