

RFA 10-05 CONCEPT PROPOSAL**CIRM DISEASE TEAM RESEARCH AWARDS II**

This RFA will support both a Planning Award and a Research Award, each of which will undergo an application process, GWG review, and ICOC approval. Receipt of a Planning Award will be a prerequisite for submission of an application for a Research Award.

Purpose: Facilitate development of novel therapies based on stem cells or direct reprogramming for the benefit of persons with disease or serious injury

Objective: The goal of the program is to support teams in moving stem-cell based therapies toward the clinic through: 1) filing an approvable IND; and/or 2) completing a Phase I, Ph I/II study to demonstrate preliminary safety and preliminary activity in humans; and/or 3) completing a controlled Phase II clinical study conducted to evaluate efficacy of the therapeutic in a particular indication.

Eligibility Requirement: At the time of the Planning Award application deadline, applicants must at a minimum have a therapeutic development candidate. A development candidate is a candidate therapeutic entity, suitable for use in humans that has completed all the research necessary to decide whether to initiate the preclinical development activities required for regulatory approval for testing in humans. Projects that are further along in the development pipeline are also eligible.

Scope – Activities:

This award will support the following activities:

- Preclinical Development activities for a single therapeutic entity including: develop GMP master and working cell banks; develop formulation and scaled production methods under cGMP; analytical method development, production of GLP and GMP lots; stability studies, IND-supporting safety/toxicology and pharmacology studies, development of clinical protocol, preparation of IND package
- The conduct of early clinical trials (Phase I, Phase I/II, and Phase II) for a single therapeutic entity
- Supporting studies performed in the context of the proposed trial that will provide critical additional data to better inform decision on continued clinical testing
- Supporting activities such as optimization of cGMP production of the candidate therapeutic and/or cGMP production of candidate therapeutic for the proposed trial(s).

This award excludes Early Translational activities leading up to selection of a therapeutic development candidate, pivotal efficacy studies (Phase III), and scale-up or production for pivotal efficacy studies.

Scope – Use of Stem Cells and Cell Type:

CIRM intends to support projects targeting a broad range of diseases and injuries. CIRM seeks novel candidate therapeutics for which there is an unmet medical need, and for which the use of

stem cells or directly reprogrammed cells can offer a significant advantage over current therapies and therapies in pivotal or late-stage development. CIRM will support meritorious product development research that is uniquely defined by its mission and that is unlikely to be funded by other agencies.

Specifically, this award will support therapeutic candidates utilizing or derived from the following cell types:

- pluripotent-derived cells
- genetically-modified stem cells (e.g. for the purpose of correction of a disease phenotype)
- neural stem cells or neural progenitor cells
- stem cell-engineered functional tissues for implantation in vivo
- novel regenerative therapies directed at endogenous stem cells in vivo
- directly reprogrammed cells (either ex vivo or in vivo)

Award information and mechanism – Planning Award:

- Any eligible applicant from an eligible institution with an eligible project may submit a Letter of Intent for a Planning Award
- CIRM is targeting up to 20 highly innovative project concepts from prospective Disease Teams
- CIRM will fund grants of up to \$100,000 (plus 10% indirect costs with total costs not to exceed \$110,000) per prospective Disease Team to support up to six months of project planning and Disease Team Research Award II application preparation
- As preclinical and clinical development projects are multidisciplinary efforts, establishing collaborations with scientists in participating Collaborative Funding Partner countries, as well as between academia and industry, is encouraged
- Planning Award funds cannot be used for wet lab, preclinical or clinical research activities
- CIRM proposes to commit up to \$2.2M under this RFA for Planning Awards

Award information – Research Award:

- CIRM is targeting 6 highly innovative, well-designed programs
- CIRM will fund up to \$20M total costs per project for programs to develop the candidate therapeutic, for up to four years to meet one or more of the objectives of the RFA
- Matching funds will not be required for this program. If additional resources are required to complete the project, applicants must detail in the proposal the source of additional funds
- CIRM proposes to commit up to \$120M under this RFA for Research Awards

Award mechanism – Research Award:

CIRM expects to fund approved proposals from non-profit and for-profit institutions (separately or in collaborations), through grants or loans.

- Loan, if a for-profit organization holds (sponsors) the IND and is the applicant organization. The loan holder will be responsible for the entire award from CIRM, even if a Co-PI is from a non-profit organization.
- Grant, if PI holds the IND and is from a non-profit organization

For an **investigator-sponsored IND**, the investigator-sponsor must be the PI on the CIRM application.

For an **organization-sponsored IND**, the organization sponsor must be the applicant organization on the CIRM application, and the PI must be an employee of that organization.

Institutional Eligibility:

- All CIRM-supported research must be conducted in the state of California
- Open to all academic, non-profit and for profit research institutions in the state of California

Investigator Eligibility:

- A Principal Investigator (PI) and up to 1 Co-Principal Investigator (Co-PI) with a Ph.D., M.D. or equivalent degrees who are authorized by the applicant institution and Co-PI sponsoring institution to conduct the proposed research in California.
- PIs and Co-PIs must commit a minimum of 30% and 20% effort respectively towards Research Award programs supported under this RFA
- PIs or Co-PIs who are PI or Co-PI on existing Disease Team Awards are not eligible to apply
- PIs or Co-PIs may be PI or Co-PI on no more than 3 active CIRM awards at the DT application deadline to be eligible for this award

Provisional Time Table:

Post RFA 10-05 and Planning Award Application Forms	Nov	2010
Planning Award LOIs due	Dec	2010
Planning Award Applications due	Feb	2011
GWG Review of Planning Award Applications	May	2011
ICOC Approval of Planning Awards	Aug	2011
Planning Award Start Date	Sept 1st	2011
Research Award Applications due	late Jan	2012
GWG Review of Research Award Applications	late March/April	2012
ICOC Approval of Research Awards	June	2012