

**RFA 09-01 Concept Proposal  
CIRM Disease Team Research Awards**

Stem cells will play an increasing role in the treatment of acute and chronic disease and serious injury. The application of stem cell research to clinical problems is in its infancy, and though few stem cell-based therapies have progressed into clinical testing and practice to date, preclinical data suggest that many stem cell therapies are ready for translation. The purpose of the California Institute of Regenerative Medicine (CIRM) Disease Team Research Awards is to accelerate potential therapies based on stem cell research toward clinical testing. To facilitate this goal, CIRM intends to support actively managed multidisciplinary teams engaged in milestone-driven research. Because translation is an emerging area of stem cell-based research, the first round of the CIRM Disease Team Research Awards focuses on activities leading up to initiation of clinical studies. CIRM anticipates that future rounds of the Disease Team Research Awards may include early clinical studies.

The key objective of the Disease Team Research Awards is to produce approvable Investigational New Drug Applications (INDs) within 4 years of the start of the award. Projects of suitable scientific maturity for this award will have demonstrated, at a minimum, reproducible evidence of disease- (or injury-) modifying activity; however, projects further along in preclinical research or preclinical development are also suitable for this award. CIRM intends to support projects targeting a broad range of diseases and injuries. CIRM seeks potential therapeutics for which there is an unmet medical need, and for which the use of stem cells can offer a significant advantage over current therapies and / or therapies in development. CIRM will support novel research across the full spectrum of pluripotent (particularly embryonic stem cells), multipotent and progenitor stem cell types and experimental approaches, and will prioritize research that is unlikely to be funded by the federal government. This initiative will consider multiple roles for human stem cells in the development of therapies, including but not limited to: cell transplantation and integration, mobilization of endogenous cells, modification of the immune system, stem cells as delivery vehicles, and stem cell-based disease models for drug screening and development (in cases in which no clinically relevant screening model exists), but not diagnostics.

Eligibility requirements for the CIRM Disease Team Research Awards have been developed to support multi-disciplinary team-based research. The award will be open to Principal Investigators (PIs) and up to two additional Co-Principal Investigators (Co-PIs) with Ph.D., M.D. or equivalent degrees, who are authorized by the applicant institution to conduct the proposed research in California. Mindful of the urgency of CIRM's mission and the scope of these awards, CIRM will require PIs and Co-PIs to each commit a minimum 30% effort to this project. An investigator may submit one application as either a PI or a Co-PI; and, an investigator may submit up to one additional application as a Co-PI. An investigator who applied as a PI on CIRM RFA 08-05 "Early Translational Research Award" is not eligible to apply as a PI on this award.

Non-profit and for-profit institutions are eligible to apply for this award. Allowable numbers of proposals per institution will be discussed at the Independent Citizen's Oversight Committee (ICOC) meeting.

CIRM proposes to fund up to 12 awards for up to four years of support. Awards may include justifiable total project costs of up to \$20 million, for a total program cost of up to

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\$210 million. The basic award instrument will be a CIRM grant. Under CIRM's Loan Program, for-profit institutions may also apply for a loan. Additional funding may be available to teams via CIRM's Collaborative Funding Partner Program with the State of Victoria, Australia; the Cancer Stem Cell Consortium of Canada; the Juvenile Diabetes Research Foundation; and potentially other organizations with which CIRM has executed a memorandum of understanding for collaborative research funding prior to release of the RFA.

Provisional Time Table:

|                           |                |
|---------------------------|----------------|
| RFA Release               | February 2009  |
| Applications due          | June 2009      |
| Review of Applications    | September 2009 |
| ICOC Approval for Funding | Fall 2009      |