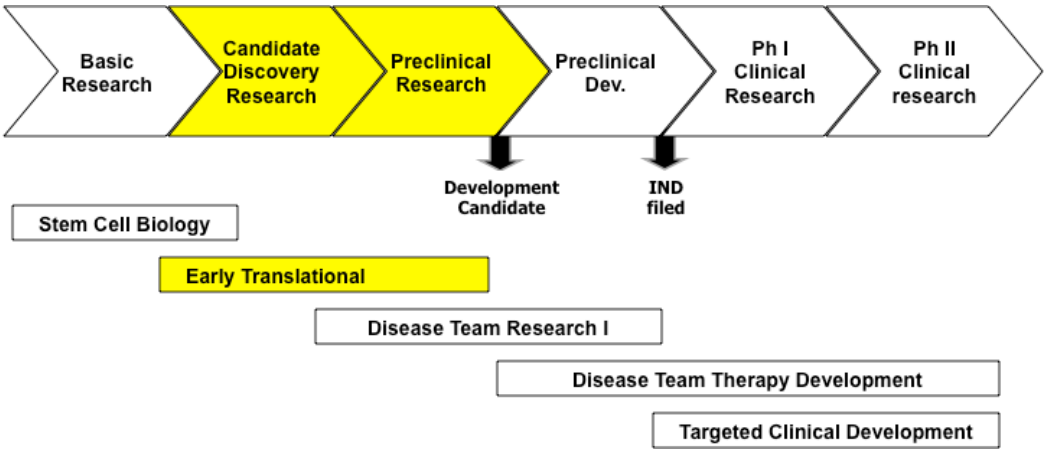
RFA 11-02 CONCEPT PROPOSAL
CIRM EARLY TRANSLATIONAL III RESEARCH AWARDS

The objective of the CIRM Early Translational Research Awards is to fund and advance innovative translational stem cell science toward clinical development. The Governing Board (ICOC) has funded two cycles of Early Translational Research Awards, initially in 2009 (19 awards) and most recently in October of 2010 (21 awards). These programs cover diverse therapeutic areas such as neurodegenerative disease, neurologic disorders, bone/cartilage disorders, and eye disease. The stage of research encompassed by CIRM's Early Translational Program in the context of other CIRM RFAs is highlighted below.

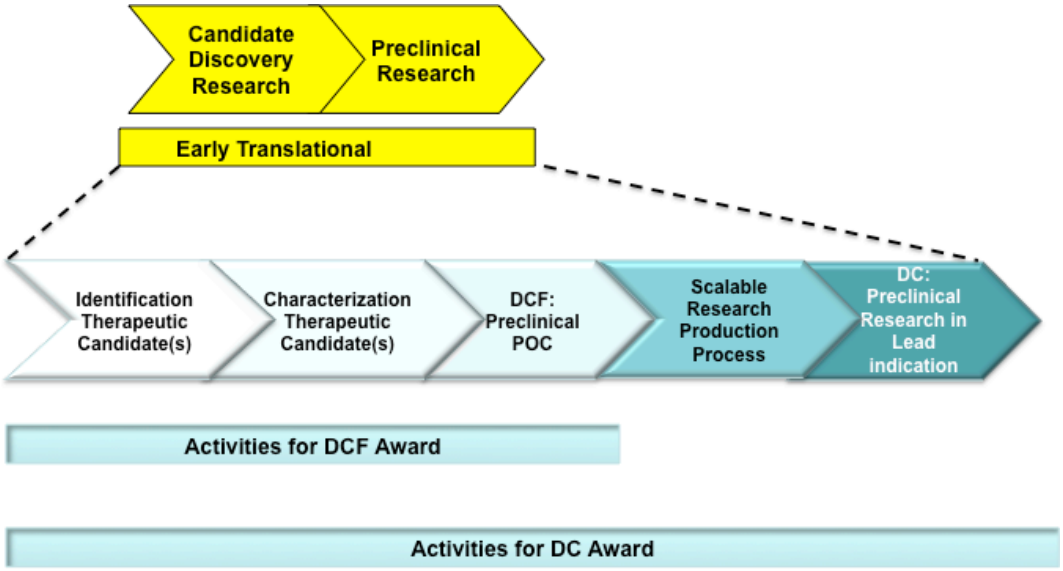


The Early Translational III Research Awards will support two categories of stem cell translational research projects:

1) Research that results in a stem cell-derived development candidate (DC) to treat an unmet medical need where all necessary activities to move into IND-enabling preclinical development have been completed.
   OR
2) Research conducted to identify and/or establish the feasibility of a potential stem cell-derived development candidate. These development candidate feasibility (DCF) awards are designed for projects that, by the end of the award will complete all necessary activities including a preclinical proof of concept study.

For all proposals, the use of human stem cells must be necessary or significantly advantageous to the proposed research compared to other approaches. Proposals that convincingly target endogenous stem cells are also eligible.

These two categories of projects and key research activities are represented below:



Research that is outside of the scope of these awards includes:

- Basic research and research with a focus on drug target discovery.
- IND-enabling preclinical development activities (e.g. GMP production, GLP toxicology and tumorigenicity studies)
- Clinical studies. Analysis of human subject samples, if directly related to the proposed research, can be funded.

CIRM will prioritize projects:

- That propose cell therapies, especially cell therapies that are differentiated derivatives of pluripotent stem cells (PSC);
- Based on pluripotent stem cells (PSCs). For example, differentiated derivatives of PSCs may be used for therapeutic candidate discovery;
- Ineligible for or unlikely to receive timely or sufficient federal funding.

Given that CIRM now has a significant translational portfolio, for disease areas already well represented the portfolio (e.g. eye, bone and cartilage, cancer), a project targeting these disease areas must propose a compelling and novel approach.

CIRM Award Information

CIRM proposes to commit up to $95 million* to this Early Translational Research III Awards program for:

- Development Candidate (DC) awards, each with justifiable total direct project costs of up to $3.5 million over the three-year project period.
- Development Candidate Feasibility awards, each with justifiable total project costs of up to $1.2 million over the three-year project period.

* CIRM intends to commit up to $75 million towards Development Candidate Awards to support about fifteen projects and up to $20 million towards Development Candidate Feasibility (DCF) Awards to support about ten projects.

For-profit applicant organizations will be permitted to apply for either grants or loans. Loan terms will be applicable Board-approved terms. Non-profit applicant organizations will be funded through grants.

CIRM Institutional Eligibility

- All CIRM supported research must be conducted in the state of California
- Open to all academic, non-profit and for-profit institutions in the state of California
- CIRM proposes to use its pre-application process to identify the most promising, competitive and responsive pre-application proposals, so no limits will be applied on the number of pre-applications an eligible institution can submit.

CIRM Investigator Eligibility

- A Principal Investigator (PI) with Ph.D., M.D or equivalent degree who is authorized by the applicant institution to conduct the proposed research in California.
- As a multidisciplinary team often most effectively conducts translational research, applicants for DC awards only may include up to 1 Co-Principal Investigator (Co-PI) with a Ph.D., M.D or equivalent degree who is authorized by the Co-PI institution to conduct the proposed research in California:
- PIs and Co-PIs must commit 20% and 15% effort respectively towards programs supported under this RFA.
- CIRM will ask reviewers to give added consideration to proposals where the PI is a physician-scientist who is new faculty, within six years of the start of her/his first position as an independent investigator.

CIRM encourages collaborative endeavors between non-profit and for-profit institutions.

Collaborative Funding Partner Participation

CIRM has established a program with several other government agencies that fund stem cell and regenerative medicine research. Through this Collaborative Funding Partner program, California-based PIs can collaborate with a Funding Partner PI from a Funding Partner applicant institution eligible for funding from one of CIRM's collaborative funding partners to bring important additional resources to proposed projects. If a collaborative funding proposal is approved CIRM will fund all project work done within the State of California and its Funding Partner will fund all project work within its jurisdiction. Collaborative Funding Partners who currently are expected to participate in this Early Translational III RFA include the National Health and Medical Research Council (NHMRC) in Australia, the Japan Science and Technology Agency (JST) and the German Federal Ministry for Education and Research (BMBF). Additional Collaborative Funding Partners may also participate.

Provisional Time Table*:

• Release of RFA 11-02	June	2011
• Pre-Applications due	August	2011
• Applications due	January	2012
• Grants Working Group Review of Applications	March	2012
• Earliest ICOC Approval	May	2012

* Assumes a Pre-Application process