RFA 10-03 CONCEPT PROPOSAL
CIRM TARGETED CLINICAL DEVELOPMENT AWARDS

Purpose: Facilitate the development of novel cell therapies derived from pluripotent stem cells for the benefit of persons with disease or serious injury.

Goal: Completion of early stage clinical trials within three years that: 1) demonstrate preliminary safety in humans and 2) provide compelling data for proof of mechanistic concept and/or early testing for efficacy that could lead to more definitive efficacy studies.

Eligibility Requirement: IND filed by application deadline on the novel cell therapy derived from human pluripotent stem cells proposed for CIRM funding.

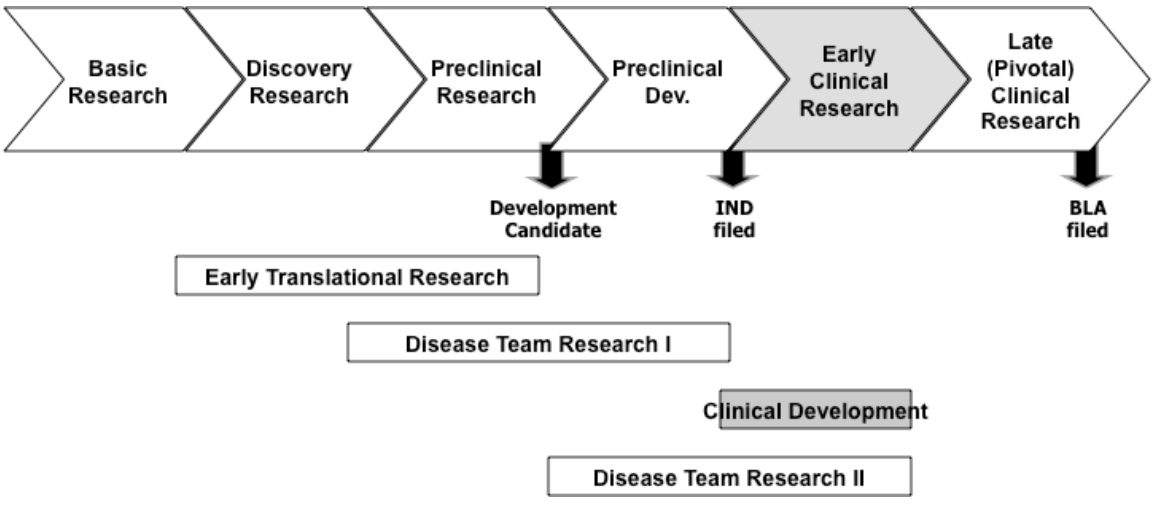
Scope: This award will support activities commensurate with the program goal including:

- The conduct of early clinical trials (including Phase 1 and Phase 2a) that will be completed within three years, resulting in demonstration of safety and generation of compelling data for proof of mechanistic concept and/or early testing for efficacy. CIRM expects that clinical trials that it funds will include women and members of minority groups where feasible.
- Supporting activities that provide important additional information for understanding the clinical question.
- Supporting activities such as optimization of cGMP production of the candidate therapeutic and/or cGMP production of candidate therapeutic for the proposed trial(s).

Research activities that fall outside of the scope of this RFA include the following examples:

- Pivotal efficacy studies
- Scale-up or production for pivotal efficacy studies.

The diagram below illustrates the pipeline stage covered under this RFA.



Award Information:
- CIRM proposes to fund one or two programs. CIRM will fund the lesser of $25 million or 50% of the total costs of a proposed program. Funding will be made on based on a negotiated activity-based budget.
- CIRM will require matching funding from applicants. CIRM and applicant funding contributions to be reasonably distributed over award period.
- CIRM proposes to commit up to $50 million under this RFA.

Award Requirement: Award recipient must have an active IND (able to enroll patients) on the novel cell therapy proposed for CIRM funding before issuance of Notice of Award. Awards must be started within 6 months of ICOC approval unless the President of CIRM determines that an extension is justified. Successful applicant(s) will be required to provide evidence, as part of pre-funding administrative review, that IND is active.

Award Mechanism
- Loan, if for-profit applicant organization. If a for-profit organization holds the IND, that organization must be the applicant organization and the Principal Investigator (PI) must be an employee of that organization. The loan holder will be responsible for the entire award from CIRM, even if a Co-PI is from a non-profit organization. Loan terms are described in the Interim CIRM Loan Administration Policy, available at: http://www.cirm.ca.gov/reg/default.asp.
- Grant if PI is from a non-profit that holds the IND.

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

Investigator Eligibility: As a multidisciplinary team often most effectively conducts translational research, this award will be open to:
- A Principal Investigator (PI) and up to 1 Co-Principal Investigator (Co-PI) with 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 programs supported under this RFA.

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

Provisional Time Table:

• Release of RFA 10-02	Apr/May	2010
• LOI due	early June	2010
• Applications due	mid July	2010
• Grants Working Group Review of Applications	Oct	2010
• ICOC Approval	Dec	2010