

RFA 13-02 CONCEPT PROPOSAL CIRM BASIC BIOLOGY AWARDS V

Recent and rapid progress in the field of stem cell science is yielding a new era of regenerative medicine. Nonetheless, there remain a number of key gaps in our understanding of fundamental human stem cell behaviors that hinder the pace of discovery, and ultimately prevent the potential of this research from being fully realized. Motivated by these challenges, CIRM developed the Basic Biology Awards to foster cutting-edge research tackling significant, unresolved issues in human stem cell biology, with emphasis on unraveling the key molecular and cellular mechanisms that dictate cell fate. CIRM has previously sponsored four rounds of Basic Biology Awards, each targeting a defined set of priorities that has evolved to keep pace with advances in the field.

As with previous iterations of this program, the CIRM Basic Biology Awards V will support rigorous investigations into the molecular and cellular basis for key stem cell behaviors and proposals that are most likely to have significant impact. Additionally, CIRM seeks to encourage and support projects with a potential to dramatically and rapidly advance our understanding or capabilities in the stem cell field. While such transformative findings are difficult to predict, they can be nurtured in an environment that fosters high-risk, exploratory pursuits. Given the urgency of its mission, CIRM seeks to broaden the potential impact of the Basic Biology Awards V by supporting a limited number such highly exploratory projects.

The Basic Biology Awards V will support two possible tracks through which an applicable proposal may be submitted:

- 1) The **Fundamental Mechanisms track (FM)** will target studies elucidating basic molecular and cellular mechanisms underlying key human stem cell properties and behaviors such as self-renewal, differentiation and maturation into functional tissue types. These awards will also support studies utilizing human stem cell-based in vitro models to gain novel insights about disease mechanisms and other medically relevant processes.

OR

- 2) The **Exploratory Concepts track (EC)** will target studies testing highly novel hypotheses, that, if proven, would challenge dogma and result in a transformative discovery for the stem cell field.

Project Eligibility

- Studies submitted through the Fundamental Mechanism track must utilize human stem cells or their derivatives; studies submitted through the Exploratory Concept track may utilize human and/or (with compelling justification) vertebrate animal model systems.
- Research that is outside the scope of the Basic Biology Awards V includes:
 - New approaches for generating induced pluripotent stem cells (iPSC) or making iPSC more efficiently
 - Deriving new iPSC lines from patient cells and performing descriptive studies (e.g. differentiation assays, profiling experiments) to identify a phenotype
 - Screening chemical or biological libraries for the purpose of identifying therapeutic compounds

- Translational research, i.e. determining feasibility of cell populations, drugs or biologics for therapeutic efficacy; studying animal models of disease to identify therapeutics
- Exploratory Concept track studies that are not directly related to stem cell biology, direct reprogramming or determination of cell fate and identity

Institutional Eligibility

- Open to all academic, non-profit and for-profit institutions in the state of California
- All CIRM supported research must be conducted in the state of California
- CIRM proposes to use its pre-application process to identify the most promising, competitive and responsive pre-application proposals; thus, no limits will be applied on the number of Pre-Applications that may be submitted by an eligible institution.

Principal Investigator Eligibility

- Any Principal Investigator (PI) with a Ph.D., M.D or equivalent degree who is authorized by the applicant institution to conduct the proposed research in California.
- An eligible PI may submit a single Preliminary Application for the Basic Biology Awards V, either through the FM track or the EC track, but not both.
- The PI must have documented authority from the applicant institution to staff the proposed project and to have access to space and shared resources sufficient to carry out the proposed research.
- The PI must devote at least 20 percent effort exclusively to the proposed program; higher levels of commitment are encouraged.

This RFA is open to participating Collaborative Funding Partners.

CIRM Award Information

CIRM proposes to commit up to \$40M to the Basic Biology Awards V Program for:

- Fundamental Mechanisms Awards (FM), each with justifiable direct project costs of up to \$250,000/year for three years
- Exploratory Concept Awards (EC), each with justifiable total direct project costs of up to \$200,000/year for two years

CIRM intends to support up to 30 Basic Biology V Awards, with approximately 2/3 funded through the Fundamental Mechanism Track, and 1/3 through the Exploratory Concept track.

Provisional timetable*

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| • Release of RFA | January | 2013 |
| • Pre-Applications due | March | 2013 |
| • Applications due | June | 2013 |
| • Grants Working Group Review | September | 2013 |
| • Earliest ICOC approval | December | 2013 |

*Assumes a Pre-Application process