

RFA 13-06 CONCEPT PROPOSAL

CIRM Tools and Technologies for Translational Bottlenecks (Tools and Technologies III)

CIRM's mission is to support stem cell research towards the development of cures to benefit patients. As the stem cell field moves towards and into clinical trials, multiple technical hurdles remain to be addressed. CIRM's Tools and Technology initiative intends to support research that can address regenerative medicine's unique translational bottlenecks. These challenges include limitations in the areas of cell engraftment, preclinical evaluation, cell tracking, and cost efficient production of stem cell therapies. Resolving these bottlenecks will help to realize the promise of regenerative medicine.

CIRM encourages the submission of proposals that are focused on the creation, design and testing of broadly applicable novel tools and technologies and the optimization, improvement, standardization or scale up of existing tools or technologies for addressing translational bottlenecks to stem cell therapies. In particular, CIRM will prioritize programs addressing the following:

- For indications where large animal modeling is critical, development and testing of
 - technologies or animal strains that enable long-term xenograft retention of human stem cells in large animal models
 - high purity, therapeutically relevant cell types from pluripotent stem cells of large animal model species to enable same species modeling of efficacy and/or safety of PSC-derived therapies
- Development and testing of clinically compatible technologies to achieve cell survival, engraftment and integration of human stem cells
- Methods or reagents/probes to enable the use of clinically compatible imaging modalities for in vivo imaging and tracking of human stem cells at high sensitivity
- Development of xenobiotic free reagents to improve reproducibility and reduce cost of human stem cell manufacturing processes (ex: extracellular matrices and chemical replacements for growth factors)
- Methods to expand isolated human HSC and/or generate HSC from human pluripotent stem cells resulting in long term, multilineage engraftment
- Application of clinically compatible nanotechnologies for human stem cell delivery to target tissues, targeted in vivo cell clearance, for in vivo monitoring of cell function, biodistribution and/or cell fate

The CIRM Tools and Technologies for Translational Bottlenecks Awards program will be open to:

- All academic, non-profit and for-profit institutions in the state of California
- Principal Investigators (PI) with a Ph.D., M.D. or other appropriate degree authorized by the applicant institution to conduct the proposed research at the applicant institution in California. By the application deadline, the PI must be an independent

investigator at a non-profit applicant institution, or have an equivalent position and be an employee of a for-profit applicant institution.

- The PI must devote a minimum of 20 percent effort exclusively to research proposed in his/her application, and higher levels of commitment are encouraged.
- Each principal investigator may submit only a single application.

The testing of novel tools and technologies for the translation of potential stem cell therapies will require a multidisciplinary effort. CIRM therefore requires all applicant teams to include an investigator(s) with expertise in human stem cell translation as well as in development of the proposed technology.

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.

Submission of an application for RFA 13-06 will involve a two-step process in which an applicant first submits a Preliminary Application (PreApp) and subsequently submits a full Application only if invited to do so by CIRM.

Award Information

- Applicants may request research support for up to three years with justifiable direct total project costs up to \$900,000.
- Those applicants developing relevant large animal models may request research support for up to three years with justifiable direct costs of up to \$1,200,000.
- Funds will be awarded as grants.

CIRM proposes to commit up to \$35 million to the Tools and Technologies III program to fund approximately 20 awards.

Provisional timetable:

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| • Release of RFA 13-06 | September 2013 |
| • Pre-Applications due | October 2013 |
| • Applications due | March 2014 |
| • Review | June 2014 |
| • ICOC approval | Summer 2014 |