

Concept Proposal: RFA 10-02: Tools and Technologies for Translational Bottlenecks (Tools and Technologies II)

Development of novel stem cell therapies and medical treatments depends on the translation of basic discoveries in stem cell biology. Rapid progress along this translational pathway will require overcoming technical obstacles and translational bottlenecks. Significant advances will be facilitated by the development of new tools and technologies that surmount these bottlenecks, support innovative translational research, and drive the development of novel therapeutic approaches using stem cells. This award program will support projects to develop such tools and techniques that address technical bottlenecks and enable novel translational approaches.

RFA 10-02 will support the inception, early stage development and evaluation for stem cell research applications of innovative tools and technologies that will overcome current roadblocks in translational stem cell research. CIRM encourages the submission of proposals that are focused on both the creation and design of novel tools and technologies and the optimization, improvement, standardization or scale up of an existing tool or technology for addressing a translational bottleneck. These awards will also support the development of stem cell based disease models (“disease-in-a-dish”) that will be valuable tools for assay development, drug screening, and therapeutic analysis. Possible goals for projects under this RFA include but are not limited to:

- Discovery of novel biomarkers (including monoclonal antibodies) for identification, selection, purification, tracking, functional analyses and clinical responsiveness of stem cells and their derivatives
- Development of safer and more effective viral and non-viral vectors for gene transduction in human stem cells
- Development and validation of stem cell scale-up technologies including novel cell expansion methods, bioreactor, and cryopreservation technologies for both pluripotent cells and differentiated cell types
- Development and optimization of new cell separation and purification technologies to effectively remove undifferentiated or unwanted cells from differentiated progeny
- Development of technologies for the robust and efficient derivation of functional cell types from pluripotent stem cells
- Development of sensitive imaging and molecular techniques for tracking transplanted stem cell derivatives *in vivo*
- Development and preclinical testing of devices for clinical delivery of stem cell-derived therapies

- Development of complex tissue scaffold structures for cell and tissue models in vitro
- Development of non-genetic methods for identification and purification of specific differentiated cell types from cellular mixtures
- Development of disease models using hESC and/or hiPSC
- Development and validation of screens using disease-in-a-dish models
- Development of diagnostic tools based on stem cell models
- Development of new animal models for testing cellular therapies for specific disease conditions
- Development of models to predict oncogenic potential of stem cell therapies

The CIRM Tools and Technologies for Translational Bottlenecks Awards program will be open to Principal Investigators (PI) with a Ph.D., M.D. or other equivalent qualifications, at non-profit or for-profit institutions. The PI must be 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. PIs must devote a minimum of 20 percent effort exclusively to research proposed in their application, and higher levels of commitment are encouraged. Each principal investigator may submit only a single application.

Submission of an application for RFA 10-02 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. There are no institutional limits on the number of PreApps.

Applicants may request research support for up to three years with direct project costs up to \$400,000 per year. Funds will be awarded as grants. CIRM proposes to fund up to 20 awards for a total program cost of up to \$40 million.

Provisional timetable:

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| • Release of RFA 10-02 | March 2010 |
| • Pre-Applications due | May 2010 |
| • Applications due | September 2010 |
| • Review | November 2010 |
| • ICOC approval | January 2011 |