Beyond CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

now it's personal

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Funding Opportunities

Beyond CIRM 2.0 now it's personal

New Idea → Single Product Candidate → Pre-IND Meeting or Equivalent → Approved Therapy

1-2/Year | 3/Year | 12/Year

Program Offerings Per Year

CIRM Quest Discovery Program

Objective

The Quest Program promotes the discovery of promising new stem cell-based technologies that will be ready for translational studies within two years to ultimately, improve patient care.

What qualifies for Quest?

Projects that propose a candidate:

- Therapeutic
- Diagnostic
- Medical device
- Tool

What qualifies for Quest?

- Stem/progenitor cell therapy
- Reprogrammed cell therapy
- Small molecule or biologic that stimulates, recruits or targets human endogenous stem cells or cancer stem cells
- Device, diagnostic or tool that:
  - Uses stem/progenitor cells
  - Addresses a critical bottleneck in the stem cell therapy field

Review Criteria

- ✓ Does the project hold the necessary significance and potential for impact?
- ✓ Is the rationale sound?
- ✓ Is the project well planned and designed?
- ✓ Is the project feasible?

Scoring System

- **Score of "85-100"**

  Recommended for funding, if funds are available

- **Score of "1-84"**

  Not recommended for funding

Applications are scored by all scientific members of the GWG with no conflict.

The **median** of all individual GWG scores determines final score.

GWG Vote on Review Process

1. *All members*: "The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG."

2. *ICOC patient advocate members*: "The review was carried out in a fair manner and was free from undue bias."

All members voted unanimously in favor of 1

Patient Advocate GWG members voted unanimously in favor of 2

GWG Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	12	$18,977,751	~$40,000,000
Not recommended for funding Score 1-84	27		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.

CIRM Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	13	$20,039,827	~$40,000,000
Not recommended for funding Score 1-84	26		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.

Overview of Recommended Applications

DISC2-10088

TITLE: Preclinical development of AAV vector-mediated in vivo hepatic reprogramming of myofibroblasts as a therapy for liver fibrosis

INDICATION: Liver fibrosis/cirrhosis

PRODUCT TYPE: Gene therapy

DISC2-10110

TITLE: Multipotent Cardiovascular Progenitor Regeneration of the Myocardium after MI

INDICATION: Heart failure

PRODUCT TYPE: Cell therapy

DISC2-10090

TITLE: Human Cardiac Chip for Assessment of Proarrhythmic Risk

INDICATION: Drug cardiotoxicity screening

PRODUCT TYPE: Drug discovery tool

DISC2-10124

TITLE: Targeted Gene Editing in the Treatment of X-Linked Hyper-IgM Syndrome

INDICATION: X-linked hyper-IgM syndrome

PRODUCT TYPE: Gene-modified cell therapy

DISC2-10061

TITLE: Lgr5-mediated self-renewal in B cell selection and leukemia-initiation

INDICATION: B cell tumors

PRODUCT TYPE: Biologic therapy

DISC2-10120

TITLE: Microenvironment for hiPSC-derived pacemaking cardiomyocytes

INDICATION: Cardiac arrhythmia

PRODUCT TYPE: Cell therapy

DISC2-10195

TITLE: Identification and characterization of the optimal human neural stem cell line (hNSC) for the treatment of traumatic brain injury (TBI) 2.0

INDICATION: Traumatic brain injury

PRODUCT TYPE: Cell therapy

DISC2-10182

TITLE: Discovery of therapeutics for Huntington's Disease

INDICATION: Huntington's disease

PRODUCT TYPE: Drug discovery tool

DISC2-10067

TITLE: A tool for rapid development of clinical-grade protocols for dopaminergic neuronal differentiation of Parkinson's Disease patient-derived iPSCs

INDICATION: Parkinson's disease

PRODUCT TYPE: Cell production tool

DISC2-10129

TITLE: Non-Toxic, Highly-Effective Bioinspired Cryoprotectants for On-Demand Stem Cell Therapies

INDICATION: Cell cryopreservation

PRODUCT TYPE: Cell cryopreservation medium

DISC2-10188

TITLE: Immunization strategies to prevent Zika viral congenital eye and brain disease

INDICATION: Zika virus infection

PRODUCT TYPE: Vaccine discovery tool

DISC2-10107

TITLE: A Novel Approach to Eradicate Cancer Stem Cells

INDICATION: Colorectal cancer

PRODUCT TYPE: Small molecule

DISC2-10134

TITLE: Platform Technology for Pluripotent Stem Cell-Derived T cell Immunotherapy

INDICATION: Cancer

PRODUCT TYPE: Cell immunotherapy

DISC2-10134

- Promising novel technology with potential for great impact to patients.
- GWG reviewers felt the application has scientific merit (84 score) with compelling preliminary data.
- GWG concerns related to achieving outcome within 2-year award period and possible future product development and manufacturing issues beyond the current proposal.
- No clear remedy to improve proposal for resubmission despite voiced support from reviewers.