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 Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	14	$25,507,913	$21,355,094
Not recommended for funding Score 1-84	21		

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.

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 (20-0)

Patient Advocate GWG members voted unanimously in favor of 2 (6-0)

Overview of Recommended Applications

DISC2-09526

TITLE: Gene Editing for FOXP3 in Human HSC

INDICATION: IPE (immunodysregulation polyendocrinopathy enteropathy) X-linked syndrome

PRODUCT TYPE: Gene-modified cell therapy

DISC2-09649

TITLE: A treatment for Zika virus infection and neuroprotection efficacy

INDICATION: Zika

PRODUCT TYPE: Small Molecule

DISC2-09565

TITLE: Preclinical development of human hepatocyte progenitor cells for cell therapy

INDICATION: Liver diseases

PRODUCT TYPE: Cell therapy

DISC2-09615

TITLE: Targeted off-the-shelf immunotherapy to treat refractory cancers

INDICATION: Cancer (solid & hematologic)

PRODUCT TYPE: Gene-modified cell/immuno therapy

DISC2-09569

TITLE: hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington’s disease

INDICATION: Huntington's disease

PRODUCT TYPE: Gene-modified cell and biologic therapy

DISC2-09624

TITLE: Protein tyrosine phosphatase - sigma inhibitors for hematopoietic regeneration

INDICATION: Regenerating blood/immune system after myelo-ablation/suppression

PRODUCT TYPE: Small molecule

DISC2-09596

TITLE: Direct Cardiac Reprogramming for Regenerative Medicine

INDICATION: Heart failure

PRODUCT TYPE: Gene therapy

DISC2-09635

TITLE: Designing a cellular niche for transplantation of human embryonic stem cell-derived beta cells

INDICATION: Type 1 diabetes

PRODUCT TYPE: Cell therapy

DISC2-09559

TITLE: Thin Film Encapsulation Devices for Human Stem Cell derived Insulin Producing Cells

INDICATION: Type 1 diabetes

PRODUCT TYPE: Cell therapy

DISC2-09610

TITLE: CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson’s disease

INDICATION: Parkinson's disease

PRODUCT TYPE: Gene therapy

DISC2-09631

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

INDICATION: Traumatic brain injury

PRODUCT TYPE: Cell therapy

DISC2-09542

TITLE: Multipotent Cardiovascular Progenitor Regeneration of the Myocardium after MI

INDICATION: Heart failure

PRODUCT TYPE: Cell therapy

DISC2-09637

TITLE: Genome Editing to Correct Cystic Fibrosis Mutations in Airway Stem Cells

INDICATION: Cystic fibrosis

PRODUCT TYPE: Gene-modified cell therapy

DISC2-09460

TITLE: Microenvironment for hiPSC-derived pacemaking cardiomyocytes

INDICATION: Cardiac arrhythmia

PRODUCT TYPE: Cell therapy