Beyond CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

now it's personal

QUEST Program

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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	11	$21,057,289	$25,832,311
Not recommended for funding Score 1-84	32		

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

DISC2-10591

TITLE: Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes

INDICATION: Type 1 diabetes

PRODUCT TYPE: Cell therapy

APPROACH: Universal donor cell line

DISC2-10524

TITLE: Genome Editing of Sinusoidal Endothelial Stem Cells for Permanent Correction of Hemophilia A

INDICATION: Hemophilia A

PRODUCT TYPE: Gene therapy

APPROACH: In vivo correction of factor VIII gene

DISC2-10679

TITLE: Towards hepatocyte cell replacement therapy: developing a renewable source of human hepatocytes from pluripotent stem cells

INDICATION: Liver failure

PRODUCT TYPE: Cell therapy

APPROACH: Renewable hepatocytes from hPSCs

DISC2-10748

TITLE: Engineering Lifelong Cellular Immunity to HIV

INDICATION: HIV infection

PRODUCT TYPE: Gene-modified cell therapy

APPROACH: Modify blood stem cells to target HIV

DISC2-10714

TITLE: iPS Glial Therapy for White Matter Stroke and Vascular Dementia

INDICATION: Vascular dementia

PRODUCT TYPE: Cell therapy

APPROACH: Glial enriched progenitors from iPSCs

DISC2-10604

TITLE: Stimulating endogenous muscle stem cells to counter muscle atrophy

INDICATION: Muscle atrophy

PRODUCT TYPE: Small molecule

APPROACH: Drug to stimulate muscle stem cells

DISC2-10753

TITLE: Generation and in vitro profiling of neural stem cell lines to predict in vivo efficacy for chronic cervical spinal cord injury

INDICATION: Spinal cord injury

PRODUCT TYPE: Cell therapy

APPROACH: Human neural stem cell lines

DISC2-10751

TITLE: Silicon Nanopore Membrane encapsulated enriched-Beta Clusters for Type 1 Diabetes treatment

INDICATION: Type 1 diabetes

PRODUCT TYPE: Cell encapsulation device

APPROACH: Transplantation of beta cells in device

DISC2-10695

TITLE: Identification and Generation of Long Term Repopulating Human Muscle Stem Cells from Human Pluripotent Stem Cells

INDICATION: Muscle disorders

PRODUCT TYPE: Cell therapy

APPROACH: Muscle stem cells from hPSCs

DISC2-10747

TITLE: Targeting Cancer Stem Cells in Hematologic Malignancies

INDICATION: Acute myeloid leukemia

PRODUCT TYPE: Monoclonal antibody

APPROACH: Antibody targets cancer stem cells

DISC2-10559

TITLE: Development of immune invisible beta cells as a cell therapy for type 1 diabetes through genetic modification of hESCs

INDICATION: Type 1 diabetes

PRODUCT TYPE: Cell therapy

APPROACH: Non-immunogenic beta cell progenitors