CIRM
CALIFORNIA'S STEM CELL AGENCY

GWG TRAN Recommendations

Gil R Sambrano
Vice President of Portfolio Development and Review
California Institute for Regenerative Medicine

TRANSFORMING medicine lives futures

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Funding Opportunities

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

CIRM Translation Research Program (TRAN)

Objective

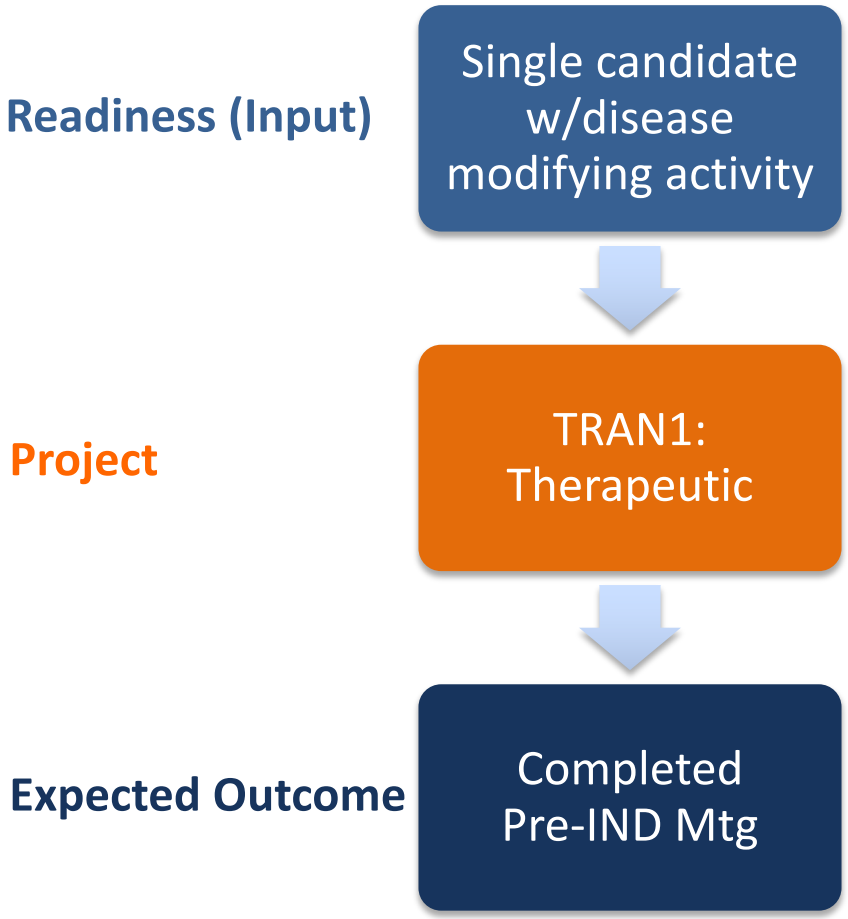
To support promising stem cell-based projects that accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use.

What qualifies for TRAN?

Projects that propose a candidate:

- Therapeutic (TRAN 1) 2019 Cycle
- ~~Diagnostic (TRAN 2)~~
- ~~Medical device (TRAN 3)~~
- ~~Tool (TRAN4)~~

CIRM Translation Program



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	7	$30,713,103	$20,000,000
Not recommended for funding Score 1-84	12		

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 Team Recommendations

The CIRM Team recommends that the Application Review Subcommittee approve funding of 4 recommended applications, which will use the available $20M and leave consideration for the remaining 3 applications open for possible allocation of funds in September.

Application	Score (Median)	Disease Category	Applicant Request	CIRM Recommends
TRAN1-11536	92	Blood/immunity	$4,896,628	Approve
TRAN1-11532	88	Vision loss	$3,733,556	Approve
TRAN1-11579	85	Neurological	$6,235,897	Select 2 of 3
TRAN1-11548	85	Neurological	$4,833,271	Select 2 of 3
TRAN1-11628	85	Neurological	$4,963,684	Select 2 of 3
TRAN1-11555	85	Cancer (blood)	$3,176,805	Hold open
TRAN1-11544	85	Cancer (solid)	$2,873,262	Hold open

Overview of Recommended Applications

TRAN1-11536

TITLE: Ex Vivo Gene Editing of Human Hematopoietic Stem Cells for the Treatment of X-Linked Hyper-IgM Syndrome

DISEASE INDICATION: X-linked hyper-IgM syndrome

PRODUCT TYPE: Cell and gene therapy

APPROACH: Ex vivo gene corrected autologous hematopoietic stem cells for transplant

TRAN1-11532

TITLE: PRPE-SF, polarized hESC-derived RPE Soluble Factors, as a Therapy for Early Stage Dry Age-related Macular Degeneration

DISEASE INDICATION: Dry age-related macular degeneration (AMD)

PRODUCT TYPE: Biologic

APPROACH: Soluble factors from hESC-derived retinal progenitor cells for intravitreal delivery

TRAN1-11579

TITLE: Human Embryonic Stem Cell-Derived Neural Stem Cells for Severe Spinal Cord Injury (SCI)

DISEASE INDICATION: Spinal cord injury

PRODUCT TYPE: Cell therapy

APPROACH: hESC-derived neural stem cells in an optimized graft for transplant

TRAN1-11548

TITLE: An optimized human neural stem cell line (hNSC) for the treatment of traumatic brain injury (TBI)

DISEASE INDICATION: Traumatic brain injury

PRODUCT TYPE: Cell therapy

APPROACH: hESC-derived neural stem cells for transplant

TRAN1-11628

TITLE: Human neural stem cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling studies

DISEASE INDICATION: Perinatal hypoxic-ischemic brain injury

PRODUCT TYPE: Cell therapy

APPROACH: Fetal-derived neural stem cells for neonatal transplant

TRAN1-11555

TITLE: BCMA/CS1 Bispecific CAR-T Cell Therapy to Prevent Antigen Escape in Multiple Myeloma

DISEASE INDICATION: Multiple myeloma

PRODUCT TYPE: Cell and gene therapy

APPROACH: Bispecific CAR-T cells targeting BCMA and CS1 in multiple myeloma cells

TRAN1-11544

TITLE: Neural Stem Cell-mediated oncolytic immunotherapy for ovarian cancer

DISEASE INDICATION: Ovarian cancer

PRODUCT TYPE: Cell therapy

APPROACH: Allogeneic neural stem cells to target ovarian cancer and deliver oncolytic virus