



GWG TRAN Recommendations

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

TRANSFORMING *medicine*
lives
futures

October 31, 2019

Funding Opportunities



DISCOVERY



TRANSLATION



CLINICAL

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



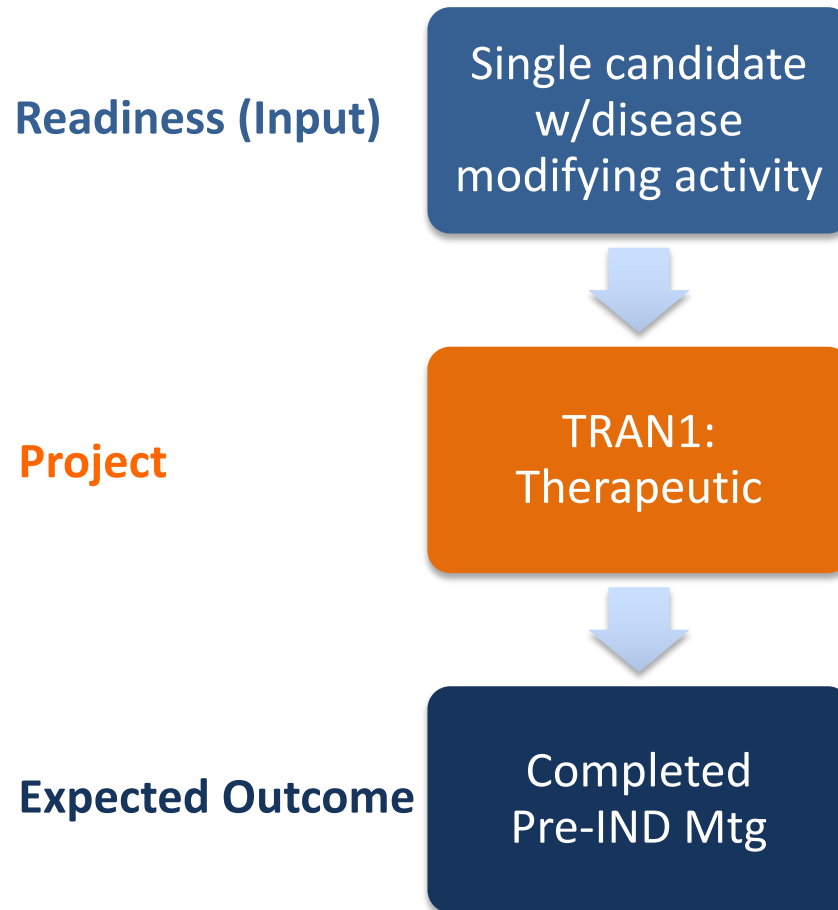
Every Moment Counts | Don't Stop Now

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.

CIRM Translation Program



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.

Today's Applications

	Number of Apps	Total Applicant Request
Recommended for funding Score 85-100	3	\$10,946,695
Not recommended for funding Score 1-84	1	\$5,246,287

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

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

TRAN1-11555

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

Overview of Application Not Recommended

TRAN1-11611

TITLE: Development of a human stem cell-derived inhibitory neuron therapeutic for the treatment of chronic focal epilepsy

DISEASE INDICATION: Epilepsy

PRODUCT TYPE: Cell therapy

APPROACH: Allogeneic hESC-derived inhibitory neural cells for transplant into seizure focal area of brain