

# CURE

Beyond  
**CIRM** 2.0  
CALIFORNIA'S STEM CELL AGENCY

*now it's personal*

## TRAN PROGRAM

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# HOPE

# Funding Opportunities



**DISCOVERY**



**TRANSLATION**



**CLINICAL**

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



1-2/Year



3/Year



12/Year

Program Offerings Per Year

# CIRM Translation Research Program (TRAN)



## Objective

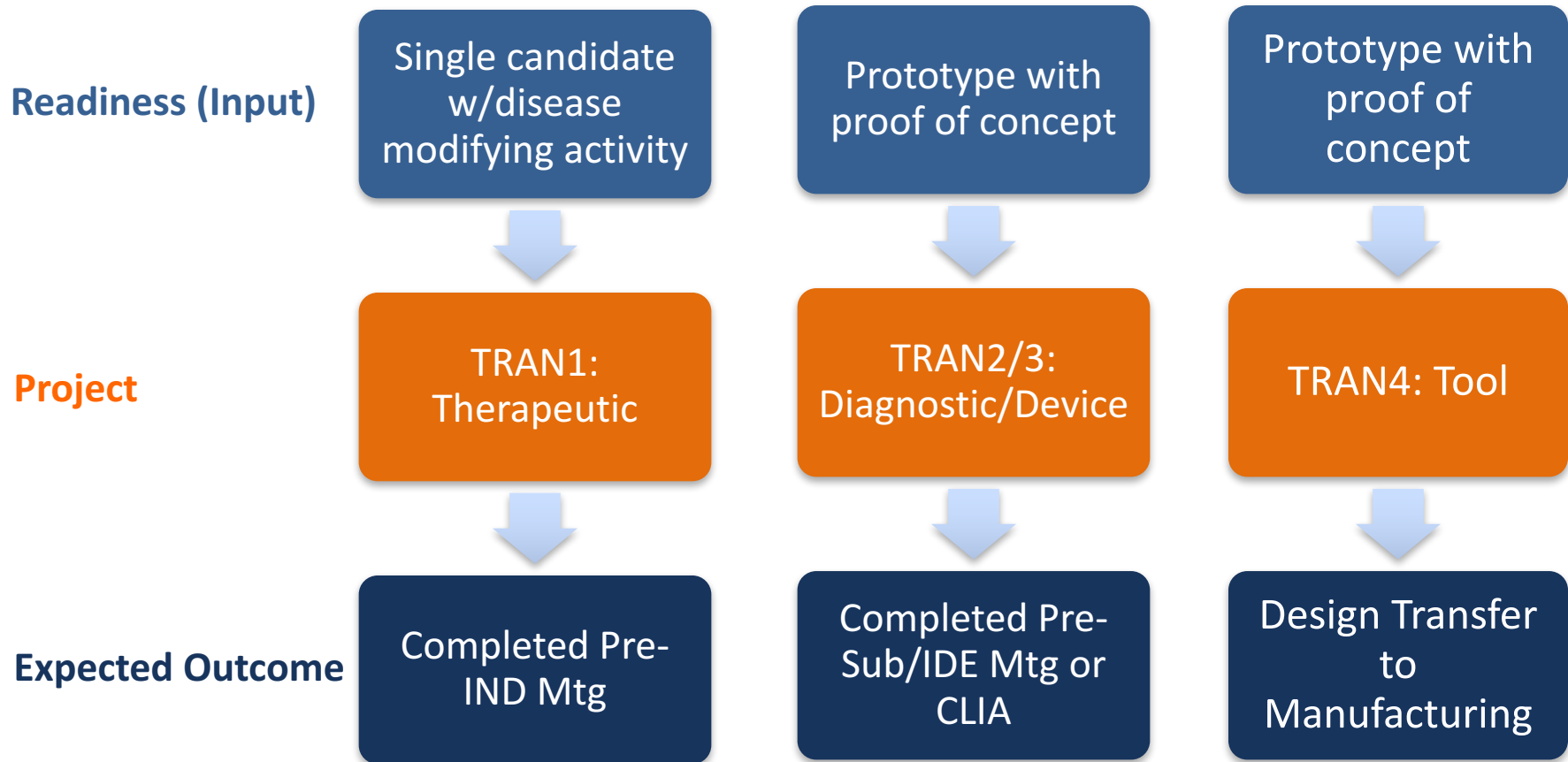
To support promising stem cell-based projects that accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use.

# What qualifies for TRAN?

Projects that propose a candidate:

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

# CIRM 2.0: 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
<b>Recommended for funding</b> Score 85-100	3	\$13,415,719	~\$35,000,000
<b>Not recommended for funding</b> Score 1-84	11		

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

# TRAN1-10416

**TITLE:** DEBCT: Genetically Corrected, Induced Pluripotent Cell-Derived Epithelial Sheets for Definitive Treatment of Dystrophic Epidermolysis Bullosa

**DISEASE INDICATION:** Epidermolysis bullosa

**PRODUCT TYPE:** Gene-modified cell therapy

**APPROACH:** Autologous, collagen gene-corrected, iPSCs-derived keratinocytes

# TRAN1-10587

**TITLE:** Human Embryonic Stem Cell-Derived Natural Killer Cells for Cancer Treatment

**DISEASE INDICATION:** Acute myeloid leukemia

**PRODUCT TYPE:** Cell therapy

**APPROACH:** hESC-derived (allogeneic) NK cells targeting cancer cells

# TRAN1-10540

**TITLE:** A Splicing Modulator Targeting Cancer Stem Cells in Acute Myeloid Leukemia

**DISEASE INDICATION:** Acute myeloid leukemia

**PRODUCT TYPE:** Small molecule therapeutic

**APPROACH:** RNA splice-modulator inhibitor acting on CSC survival genes