

# CURE

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

*now it's personal*

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Vice President Portfolio Development  
and Review



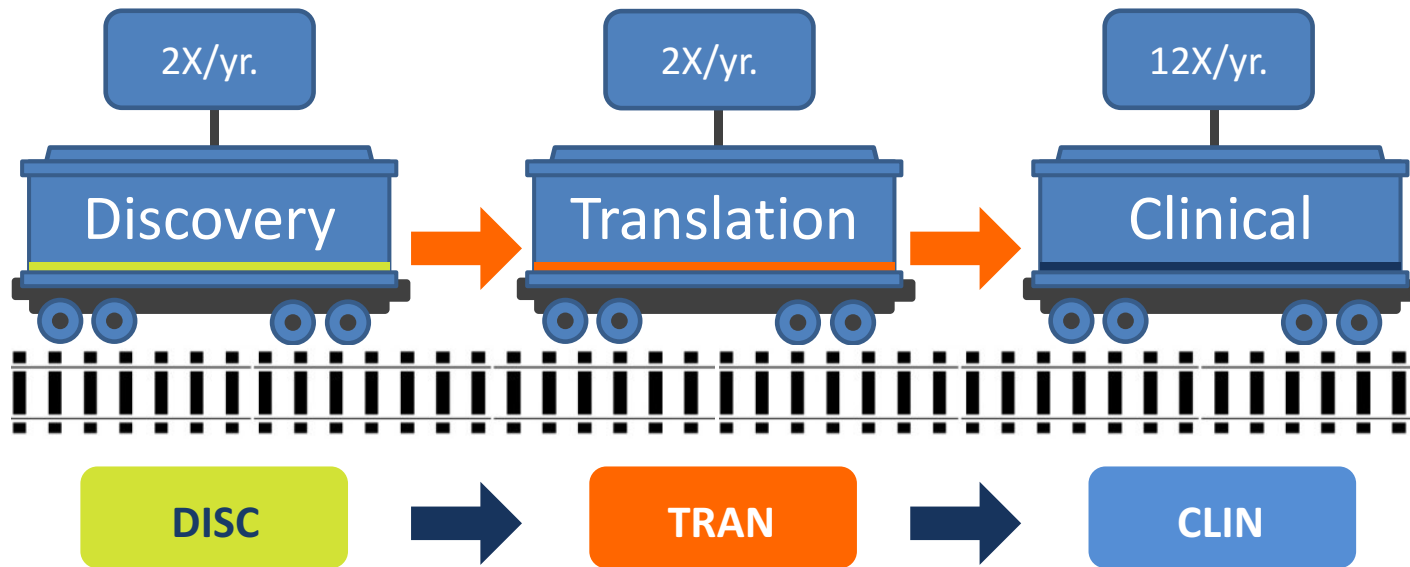
# HOPE



# **PROGRAM OVERVIEW:**

# **INCEPTION RESEARCH PROJECTS**

# Track with Continuous and Recurring Funding Opportunities



# CIRM 2.0 Inception Program



## Objective

Provide **seed funding** for **great ideas** that may impact the field of human stem cell research but need modest support to test and compete for larger funding opportunities.



# Emphasis of GWG Review

- **“Great new ideas”** with potential to ultimately result in a translatable human stem/progenitor cell-based product or technology.
- Ideas with a sound scientific rationale is important, but **preliminary data are not required or expected**. This is a high risk, high reward program.
- We will provide **\$150K to test the idea** and generate data to compete for larger (more substantial) funding opportunities through CIRM or other sources.



# Positive Selection

- Started with total **72** proposals.
- Positive selection process narrowed to **38** proposals.
- Selection by any one member of the GWG was sufficient to move an application forward to full review.



# Scoring System

- **Tier 1: Score of “85-100”**

*Exceptional merit and warrants funding, if funds are available.*

- **Tier 2: Score of “1-84”**

*Not recommended for funding.*

Applications are scored by all scientific members of the GWG with no conflict.

# DISC 1: GWG Recommendations



|   | DISC1 Apps | Funds       |
|---|------------|-------------|
| <b>Tier 1:</b> Score 85-100<br>Exceptional merit and warrants funding, if funds available | 6          | \$1,367,078 |
| <b>Tier 2:</b> Score 1-84<br>Not recommended for funding                                  | 32         |             |





## 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 and 2



# Overview of Recommended Applications



# DISC1-10074

**TITLE:** Reprogramming Human Stem Cells for Blood Cell Generation

**OBJECTIVE:** To create a universal donor blood cell line that can be used to produce human red blood cells for transplantation.



# DISC1-10036

**TITLE:** Prodrug Innovation to Target Muscle Stem Cells and Enhance Muscle Regeneration

**OBJECTIVE:** To develop an approach to target therapeutics to muscle stem cells, the building blocks of skeletal muscle.



# DISC1-09912

**TITLE:** A Novel Tissue Engineering Technique to Repair Degenerated Retina

**OBJECTIVE:** To develop a therapeutic product from hESCs to treat advanced human retinal degenerative diseases.



# DISC1-10079

**TITLE:** An Exosome-Based Translational Strategy to Mitigate Alzheimer's Disease Neuropathology

**OBJECTIVE:** To develop a therapeutic product from stem cell-derived exosomes to treat Alzheimer's disease symptoms.



## DISC1-09984

**TITLE:** Hypo-Immunogenic Cardiac Patches for Myocardial Regeneration

**OBJECTIVE:** To engineer an allogeneic cardiac patch that restores function after a heart attack but does not elicit an immune response.



## DISC1-09999

**TITLE:** Generation of Expandable, Self-Renewing Muscle Stem Cells for Duchenne Muscular Dystrophy

**APPLICATION:** To generate expandable, self-renewing human muscle stem cells from hiPSC for Duchenne Muscular Dystrophy disease modeling and therapeutics.