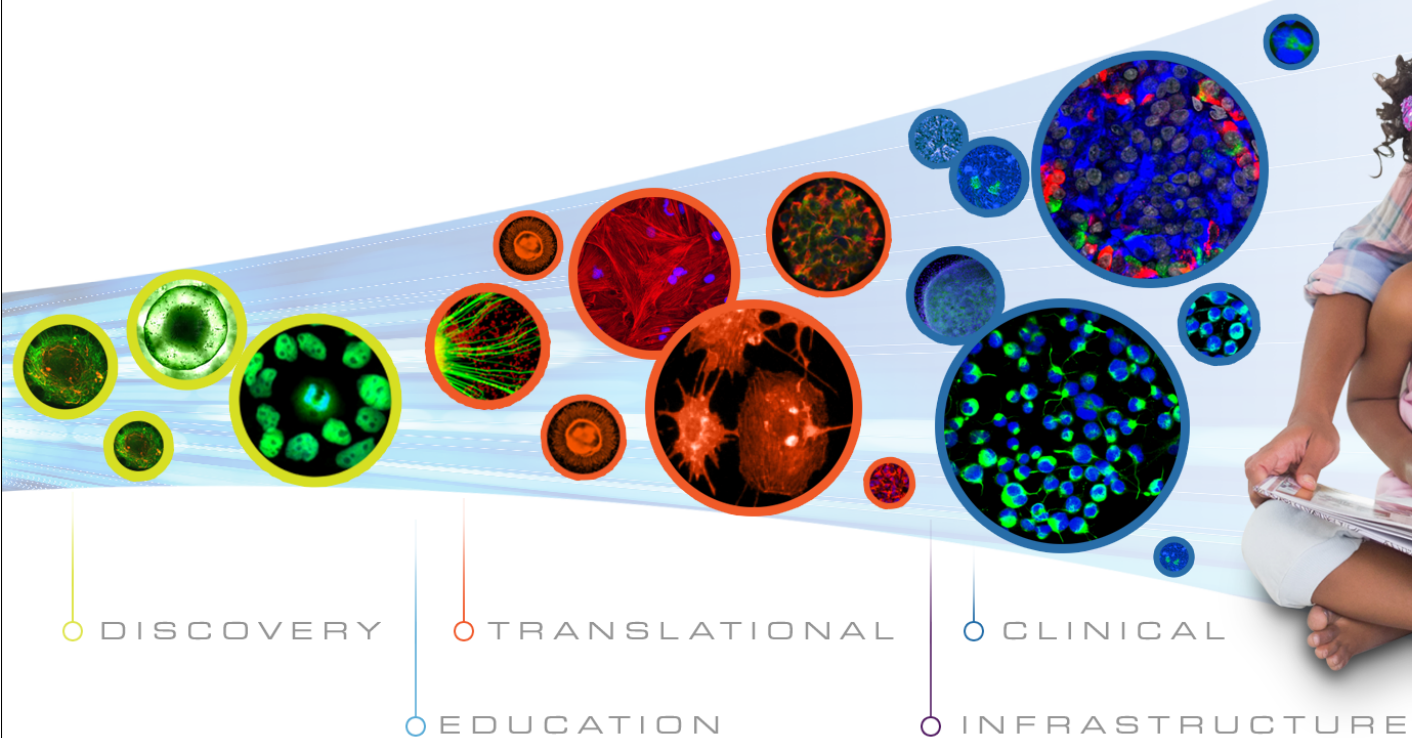


March 16, 2016

# CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY



## Translation Research Program: GWG Recommendations

**Rahul G. Thakar**

Senior Science Officer for Development & Review  
California Institute for Regenerative Medicine

# TRAN: Translation Research Awards Presentation Overview

1. Introduction of Translation (TRAN) Research Program
2. Recommendations of the GWG for TRAN Review, Feb 2016



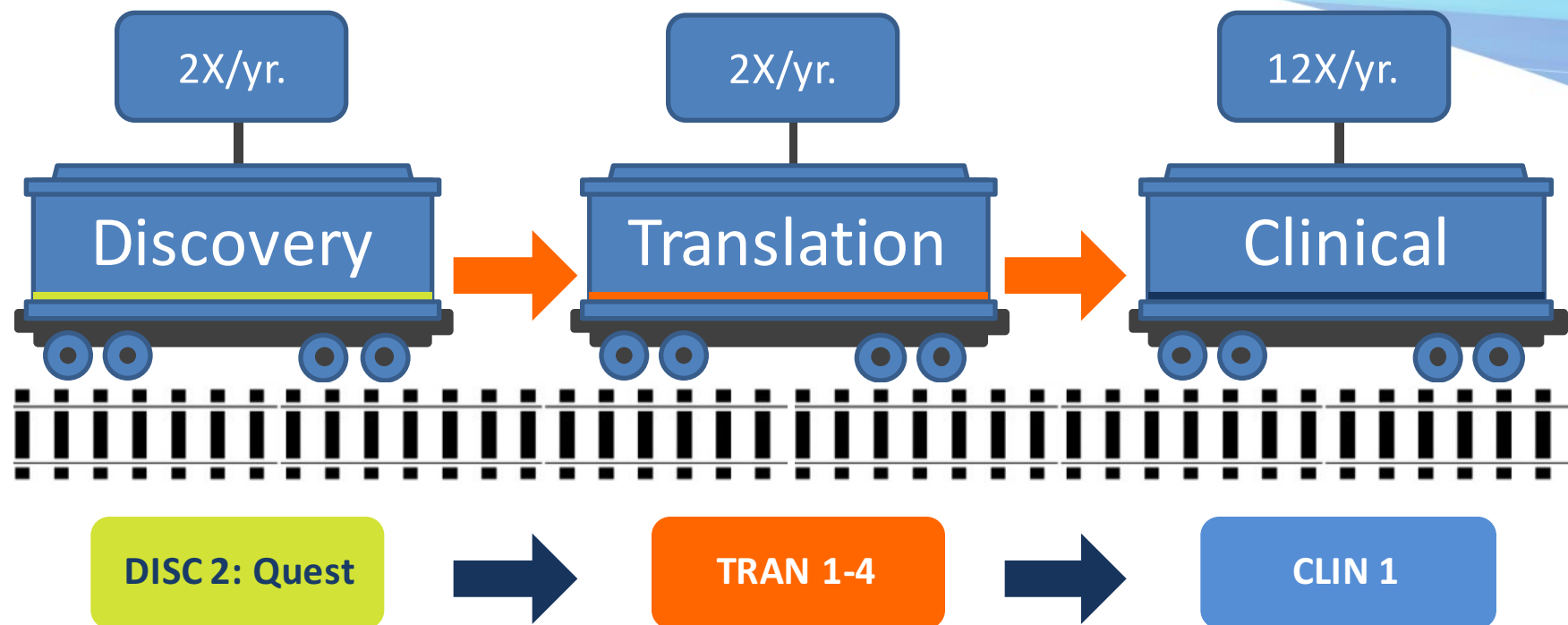
**PROGRAM OVERVIEW:**

**TRANSLATION RESEARCH  
PROJECTS**

# CIRM 2.0 Translation Research Program

- **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.

# Where the Translation Program Fits



# CIRM 2.0: Translation Program

Proof of concept with a candidate product

**Therapeutic  
(TRAN 1)**

**Diagnostic  
(TRAN 2)**

**Device  
(TRAN 3)**

**Tool  
(TRAN 4)**



Pre-IND or Pre-Submission meeting with FDA

Ready to move to  
manufacturing for  
broad use or  
commercialization



# CIRM 2.0: Translation Program

Proof of concept with a candidate product

**Therapeutic  
(TRAN 1)**

30 months

\$5M/\$2.5M

**Diagnostic  
(TRAN 2)**

24 months

\$2M

**Device  
(TRAN 3)**

24 months

\$1.2M

**Tool  
(TRAN 4)**

24 months

\$1M

# Key Points for the Translation Review

- All product types were assessed together.
- The review criteria were the same for all 4 product types.
- The goal was to identify the projects that were most likely to achieve the objective of this program.



# Review Criteria

1. Did the project hold the necessary significance and potential for impact?
2. Was the rationale sound?
3. Was the project well planned and designed?
4. Was the project feasible?



# **RECOMMENDATIONS OF THE GWG FOR THE TRANSLATION 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.

# TRAN: GWG Recommendations

	TRAN Apps	Funds
<b>Tier 1:</b> Score 85-100 Exceptional merit and warrant funding, if funds available	8	\$39,707,765
<b>Tier 2:</b> Score 1-84 Not recommended for funding	22	\$59,152,553

# 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 (20-0)

Patient Advocate GWG members voted unanimously in favor of 2 (6-0)

# TRAN: CIRM Team Recommendations

	TRAN Apps	Funds
<b>Tier 1:</b> Score 85-100 Exceptional merit and warrant funding, if funds available	7*	\$36,777,876
<b>Tier 2:</b> Score 1-84 Not recommended for funding	22	\$59,152,553

\*The CIRM Team Recommendation for application TRAN1-08522 in Tier 1 is deferred for further review.