Real Life™

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Grants Working Group Recommendations TRAN

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CIRM

CALIFORNIA'S STEM CELL AGENCY

Mission Statement

OUR MISSION

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

Recurring Programs

DISCOVERY

TRANSLATION

CLINICAL

New Idea → 2/Year → Single Product Candidate → 2/Year → Pre-IND or Equivalent → 12/Year → Approved Therapy

Translation Program

TRAN1
Therapeutic

30 months
$4M
(Cell/Biologic)
$2M (Drug)

TRAN2
Diagnostic

24 months
$1.2M

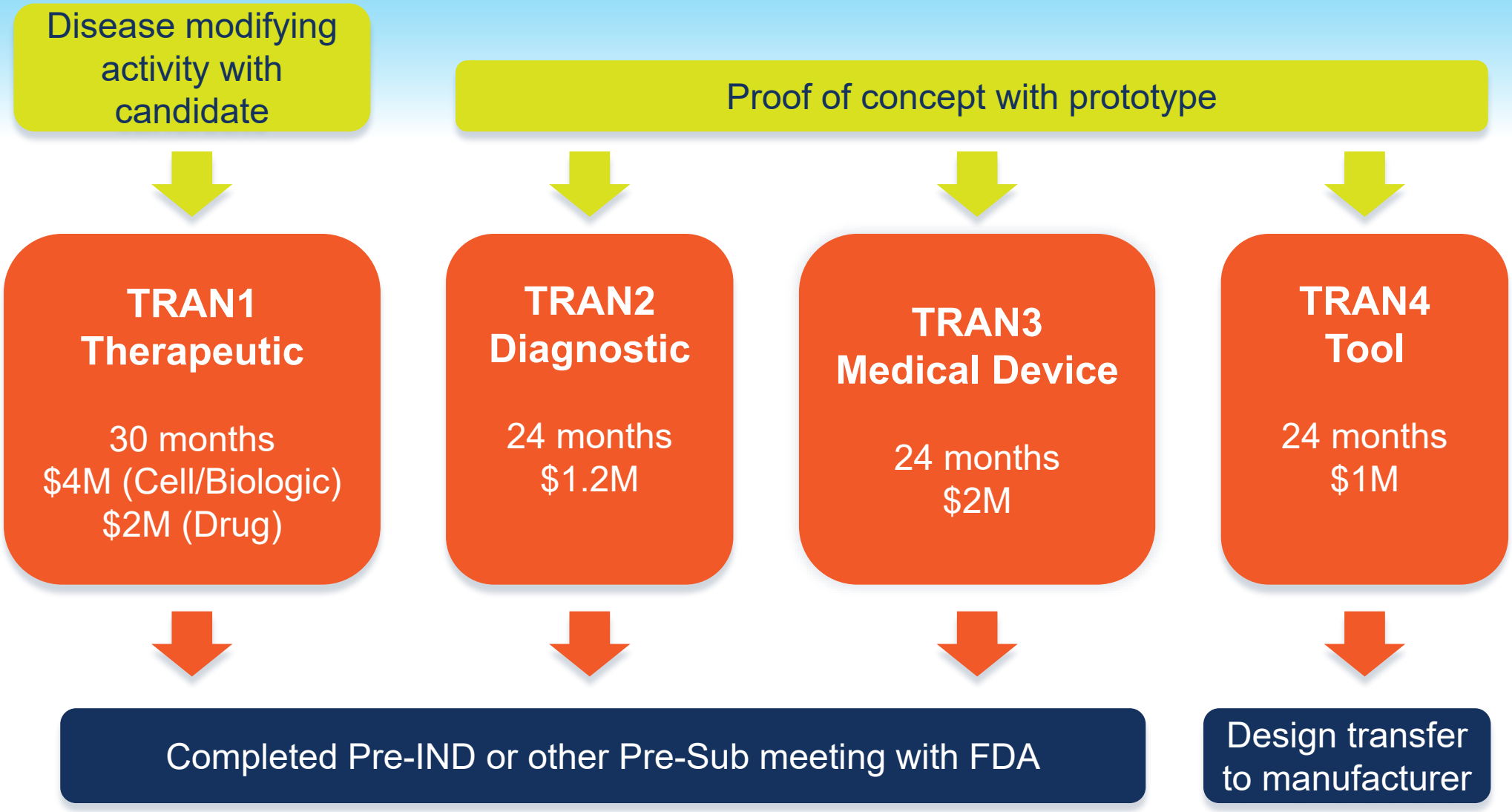
TRAN3
Medical Device

24 months
$2M

TRAN4
Tool

24 months
$1M

Translation Program



Scientific Review Criteria

1. Does the project hold the necessary significance and potential for impact? (i.e., what value does it offer; is it worth doing?)
2. Is the rationale sound? (i.e., does it make sense?)
3. Is the project well planned and designed?
4. Is the project feasible? (i.e., can they do it?)
5. Does the project uphold principles of Diversity, Equity, and Inclusion (DEI)?

Scientific 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.

DEI Scoring

CIRM TRAN Program DEI Rubric				
CRITERIA	Score of 0 to 2 Not Responsive	Score of 3 to 5 Not Fully Responsive	Score of 6 to 8 Responsive	Score of 9 to 10 Outstanding Response
1. Product Development	Inadequate discussion on what is currently known about demographic disparities in the population that will benefit from the proposed product.	May have inadequate discussion on what is currently known about demographic disparities in the population that will benefit from the proposed product.	Adequate discussion on what is currently known about demographic disparities in the population that will benefit from the proposed product.	Strong discussion on what is currently known about demographic disparities in the population that will benefit from the proposed product.
	Planned/completed activities do not demonstrate an understanding of the potential influence of patient diversity on the safety, efficacy, and overall utility of the proposed product.	Planned/completed activities are incomplete or inadequate and may not demonstrate an understanding of the potential influence of patient diversity on the safety, efficacy, and overall utility of the proposed product.	Planned/completed activities reflect a good faith effort and have the potential to be effective in understanding the potential influence of patient diversity on the safety, efficacy, and overall utility of the proposed product.	Planned/completed activities reflect an outstanding and comprehensive effort and are likely to be effective in understanding the potential influence of patient diversity on the safety, efficacy, and overall utility of the proposed product.
	Does not address limitations, advantages and/or challenges of this project in developing a product that addresses the unmet medical needs of the diverse California population, including underserved racial/ethnic communities.	May not fully address limitations, advantages and/or challenges of this project in developing a product that addresses the unmet medical needs of the diverse California population, including underserved racial/ethnic communities.	Addresses limitations, advantages and/or challenges of this project in developing a product that addresses the unmet medical needs of the diverse California population, including underserved racial/ethnic communities.	Comprehensive discussion on limitations, advantages and/or challenges of this project in developing a product that addresses the unmet medical needs of the diverse California population, including underserved racial/ethnic communities.
2. DEI Enhancement Strategies	Has not or will not include diverse and inclusive perspectives and experience from the population that will benefit from the product, or activities proposed are not appropriate.	May not have or may not effectively or sufficiently bring diverse and inclusive perspectives and experience from the population that will benefit from the product. Activities may not match the needs of the project.	Has or will appropriately bring diverse and inclusive perspectives from the population that will benefit from the proposed product. Activities match the needs of the project.	Has or will have outstanding incorporation of diverse and inclusive perspectives from the population that will benefit from the proposed product. Activities are well matched to the needs of the project.

DEI Scores

Applications are scored for adherence to principles of DEI by all GWG Board Members with no conflict.

- DEI Score of 9-10

  Outstanding Response

- DEI Score of 6-8

  Responsive

- DEI Score of 3-5

  Not Fully Responsive

- DEI Score of 0-2

  Not Responsive

GWG Composition and Roles

Scientific GWG Member	Scientific evaluation (disease area expert, regulatory, CMC, product development) Provides scientific score on all applications
GWG Board Member (Patient Advocate/Nurse)	Patient perspective on significance and potential impact, oversight on process Provides a DEI score on all applications
Scientific Specialist (non-voting)	Scientific evaluation (specialized expertise as needed) Provides initial but not final scientific score

GWG Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	9	$45,505,384	$49,998,740
Not recommended for funding 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.

Minority Reports

- Under Prop 14, any application that is not recommended for funding by the GWG, but which had 35% or more members score to fund the application must include a minority report.

- The minority report is included in the review summary and provides a brief synopsis of the opinion of reviewers that scored the application 85 or above.

Applications with a Minority Report

App Number	Title	Funds Requested	Score
TRAN1-14710	AAV Gene Therapy for Treating Congenital Hereditary Endothelial Dystrophy (CHED) associated with Biallelic SLC4A11 Mutations	**$4,338,166**	80
TRAN4-14726	Development of a low-cost, clinical-grade iPS maintenance medium for enabling stem cell therapy manufacturing	**$999,848**	80

The CIRM Team supports the minority position for application **TRAN1-14710** and recommends funding of this application.

Minority Report for TRAN1-14710

Score	Num ≥85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
80	5	8	70-90	$4,338,166

TITLE: AAV Gene Therapy for Treating Congenital Hereditary Endothelial Dystrophy (CHED) associated with Biallelic SLC4A11 Mutations

AREA OF IMPACT: CHED is a pediatric hereditary blinding disease. Corneal transplantation is the current standard of care and is technically difficult with significant care burden, and often needs to be repeated. The proposed gene therapy could offer an alternative treatment for this rare disease, and potentially pave the way for similar treatments in other inherited corneal diseases.

Minority Report for TRAN4-14726

Score	Num ≥85 (Fund)	Num <85 (Do not fund)	Range	Funds Requested
80	5	9	55-90	$999,848

TITLE: Development of a low-cost, clinical-grade iPS maintenance medium for enabling stem cell therapy manufacturing

AREA OF IMPACT: This medium for manufacturing of iPS cell-based therapies could offer a lower-cost and lower-risk alternative for stem cell therapy developers.

Board members with Conflicts of Interest

Board members with Conflicts of Interest for TRAN applications	
Maria Bonneville	Elena Flowers
Le Ondra Clark Harvey	Christine Miaskowski
Mark Fischer-Colbrie	Joe Panetta
Ysabel Duron	Karol Watson