CIRM
CALIFORNIA'S STEM CELL AGENCY

TRANSFORMING *medicine lives futures*

ICOC Meeting
October 18, 2018

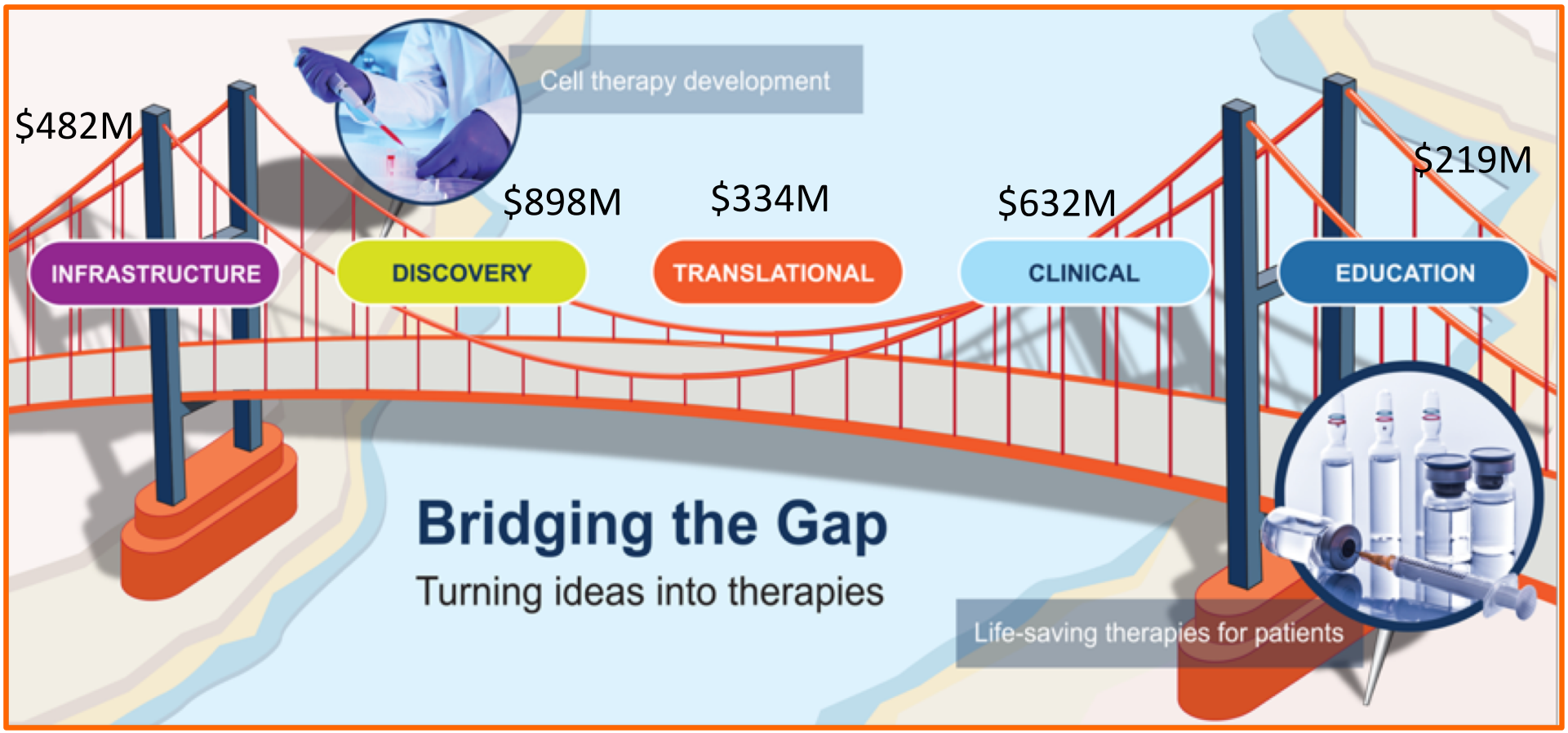
Maria T. Millan, M.D.
President & CEO
California Institute for Regenerative Medicine

CIRM's Mission

To accelerate stem cell treatments to patients with unmet medical needs

Executing on Strategic Plan 2016-2020

CIRM Programs: Investment to Date



Operating on the November 2017 Transition Plan

- Execute on 5-year Strategic Plan approved by the ICOC on December 2015
- Critical personnel level required to execute on the Strategic Plan while maintaining operational excellence
- Essential to preserve CIRM's value proposition to increase the probability of and the speed by which stem cell treatments can reach patients

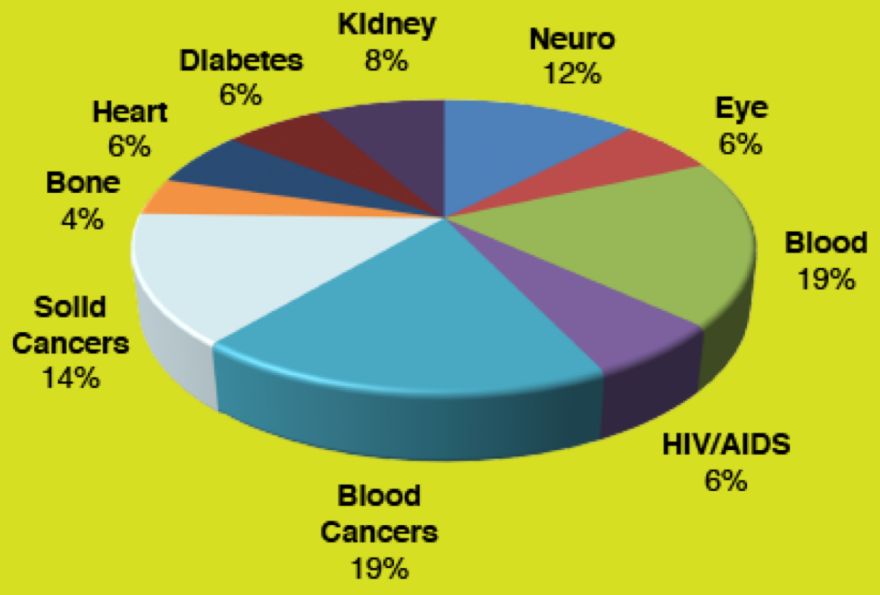
Building the CIRM Clinical Portfolio

Broad Disease Indications – Unmet Medical Needs
Diverse Therapeutic Approaches

CIRM-FUNDED CLINICAL TRIALS:

49

DISEASE AREAS:



2018 CIRM Research Awards

Program	2018 Allocation	Est 2018 Year End	Awards, Estimated
Clinical	$130M	$111M	7 CLIN2s 6 CLIN1s
Translation	$30M	$28M	7 candidates
Discovery	$10M	$18M*	13 projects
Education	$750K	$150K	
Total	**$171M**	**$156M**	

* $8 M will be advanced from the 2019 CLIN budget

2018 Year End Budget Update

As of January 1, 2018

- Committed / Uncommitted Balance $2.48B / $269M

2018 Estimated Activity

- 30 Awards Added $155.66M
- Recovery of unexpended funds $23.50M

As of December 31, 2018

- Committed / Uncommitted $2.60B / $144M

Available Big Bucket research funds January 1st, 2019 $144M

Future Recovery Estimated Unallocated (2019) ~$30M*

** Plan for $8 M to replenish the planned 2019 CLIN funding*

CIRM Budget Update

January 1st, 2019

Research **Administration**

2019 Budget Planning

- $144M Research Budget includes $8 M from recuperated funds projected for 2019

- Research Programs:
  - No DISC awards budgeted for 2019
  - TRAN and CLIN1 awards will change in scope; previously funded “pipeline” and Sickle Cell projects will be unaffected
  - Strategic plan target of 50 new clinical trials (total of 67). Remaining budget will support 43-45 new clinical trials (bringing CIRM total to approximately 60).
  - $30M budget for CIRM-NHLBI for Sickle Cell Cures initiative.

- Administrative Budget will provide sufficient staffing to manage all CIRM awards regardless of 2020 bond outcome

Proposed Research Budget Allocation

	2019
CLIN1&2 CLIN1&2 SCD	$93M* $30M
TRAN	$20M
DISC	$0M
EDUC	$600K

* Includes $8M from projected returned/recovered funds
Can achieve 43-45 new clinical trials & ~43 new candidates

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Comments or questions regarding the proposed recommendations for the 2019 scientific research budget?

Cure Sickle Cell Co-Funding Initiative

Gabriel Thompson
Vice President
Grants and Operations

Cure Sickle Cell

CIRM NHLBI Co-Funding Initiative

Purpose: To *accelerate* the implementation of accessible cures for sickle cell disease

Highlights:
Use CIRM's CLIN Funding process (monthly submission and review)

- NIH will rely on CIRM's application, scientific peer review, contracting and post-award management
- Funding decisions in as little as 85 days
- Leveraged funding for sponsors
- Data Sharing

Changes to our CLIN Program & RFA

in support of the Cure Sickle Cell Initiative

- Application materials will be shared with NHLBI representatives
- Awardees are required to comply with NHLBI DS&M and Data Sharing policies
- Allows Non-CA Orgs to apply who are requesting their CIRM Unallowable Costs to be covered with NHLBI funds
- All sickle cell applications will submit to this revised program

What is the process for review?

Process Time: 80 to 110 days

CIRM Award Management

- NHLBI funds come to CIRM via ”Other Transaction Authority”
- CIRM issues Notice of Award for CIRM + NHLBI funds
- Progress & Financial Reports shared with NHLBI via Grants Management System
- NHLBI representative to be appointed to CAP
- CIRM retains ability to suspend or terminate award

Comments or questions regarding the proposed recommendations for the Cure Sickle Cell Co-Funding Initiative?

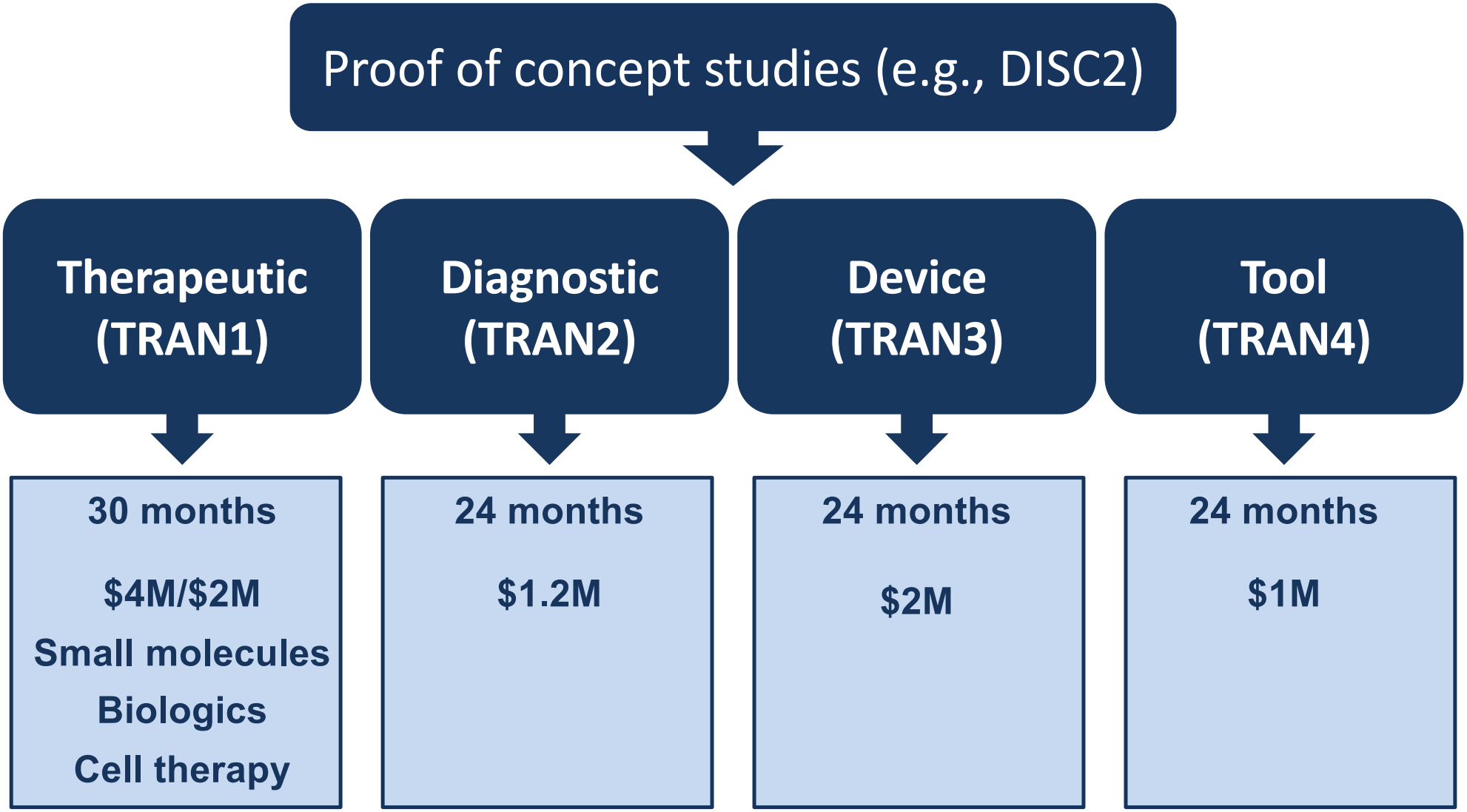
Funding Opportunity Concept Changes

Gil Sambrano, Ph.D.
Vice President
Portfolio Development and Review

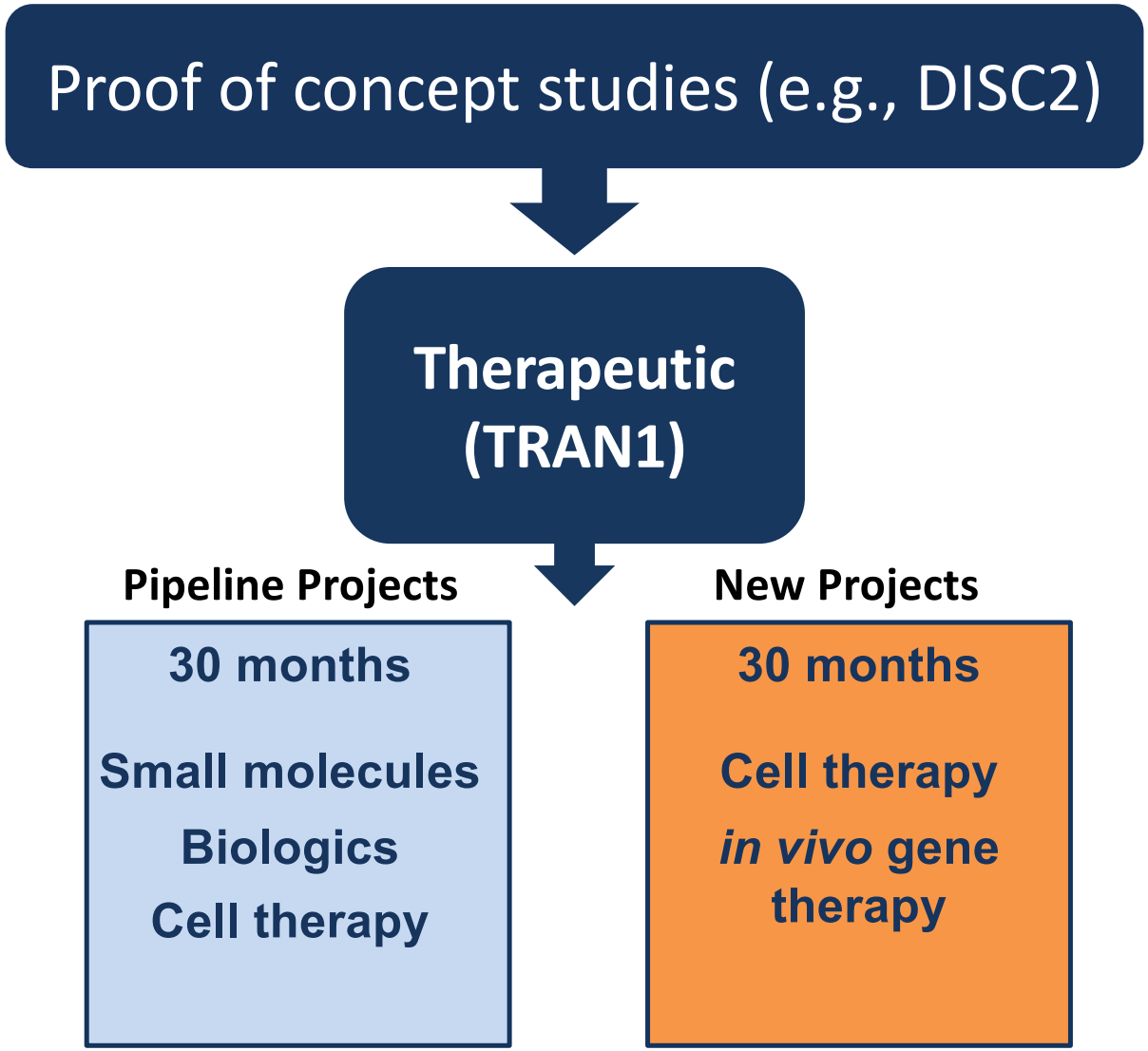
Funding Opportunity Concept Changes

- Affects Translational and Clinical Programs
- Removes small molecules and biologics from eligibility for TRAN & CLIN1 except for previously funded “pipeline” & Sickle Cell projects.
- Adds *in vivo* gene therapy to all programs
- Adds requirements for NHLBI/CIRM Cure Sickle Cell Disease Joint Initiative

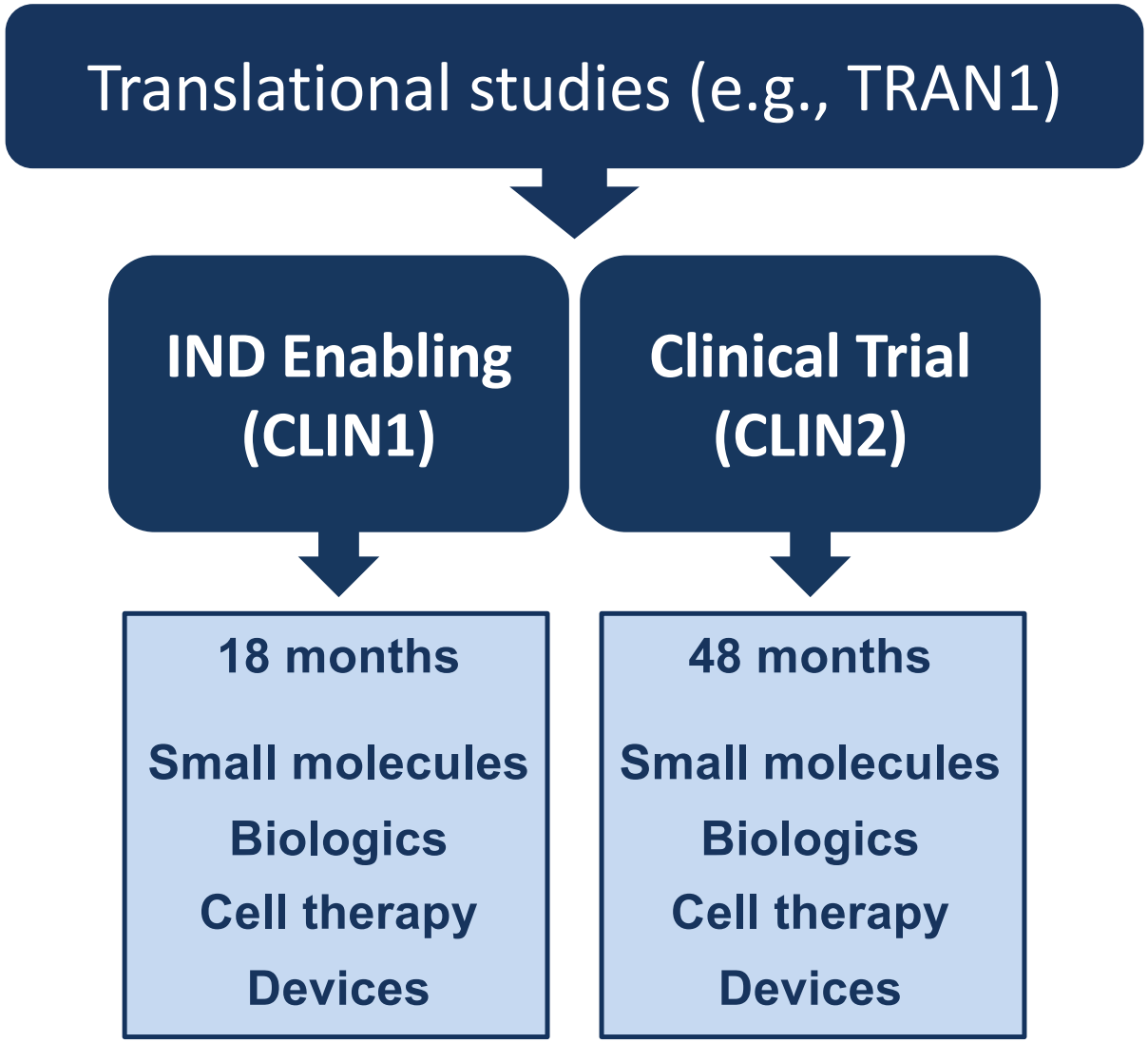
Current CIRM Translation Program



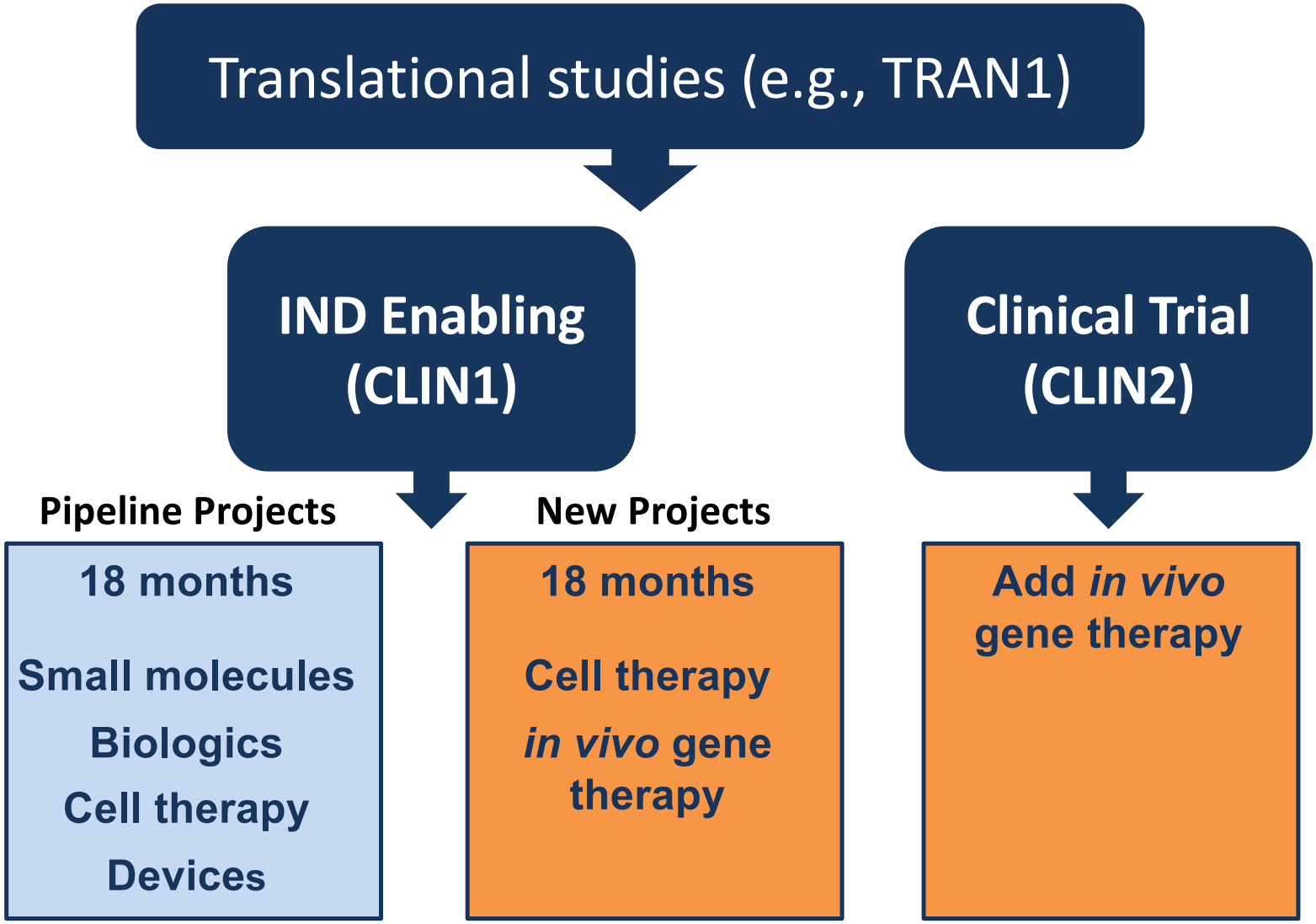
Proposed CIRM Translation Program



Current CIRM Clinical Program



Proposed CIRM Clinical Program



Addition to All Programs

- *in vivo* gene therapy projects will be eligible for CIRM funding

- Requires GWG 2/3 majority vote deeming any *in vivo* gene therapy project a “vital research opportunity”

Changes to Support CIRM/NHLBI Sickle Cell Disease Joint Initiative

- All sickle cell disease applications must be considered for joint funding
- Sickle cell projects will be exempt from CLIN1 therapeutic candidate restrictions.
- Application materials will be shared with NHLBI
- Non-CA applicants may apply for NHLBI funds to cover unallowable activities outside of CA
- Co-funded projects must adhere to NHLBI policies for Data and Safety Monitoring and Data Sharing (including Sickle Cell Data Coordinating Center)

Comments or questions regarding the concept plan changes?