



TRANSFORMING *medicine
lives
futures*

Science Subcommittee
October 2018

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President & CEO
California Institute for Regenerative Medicine

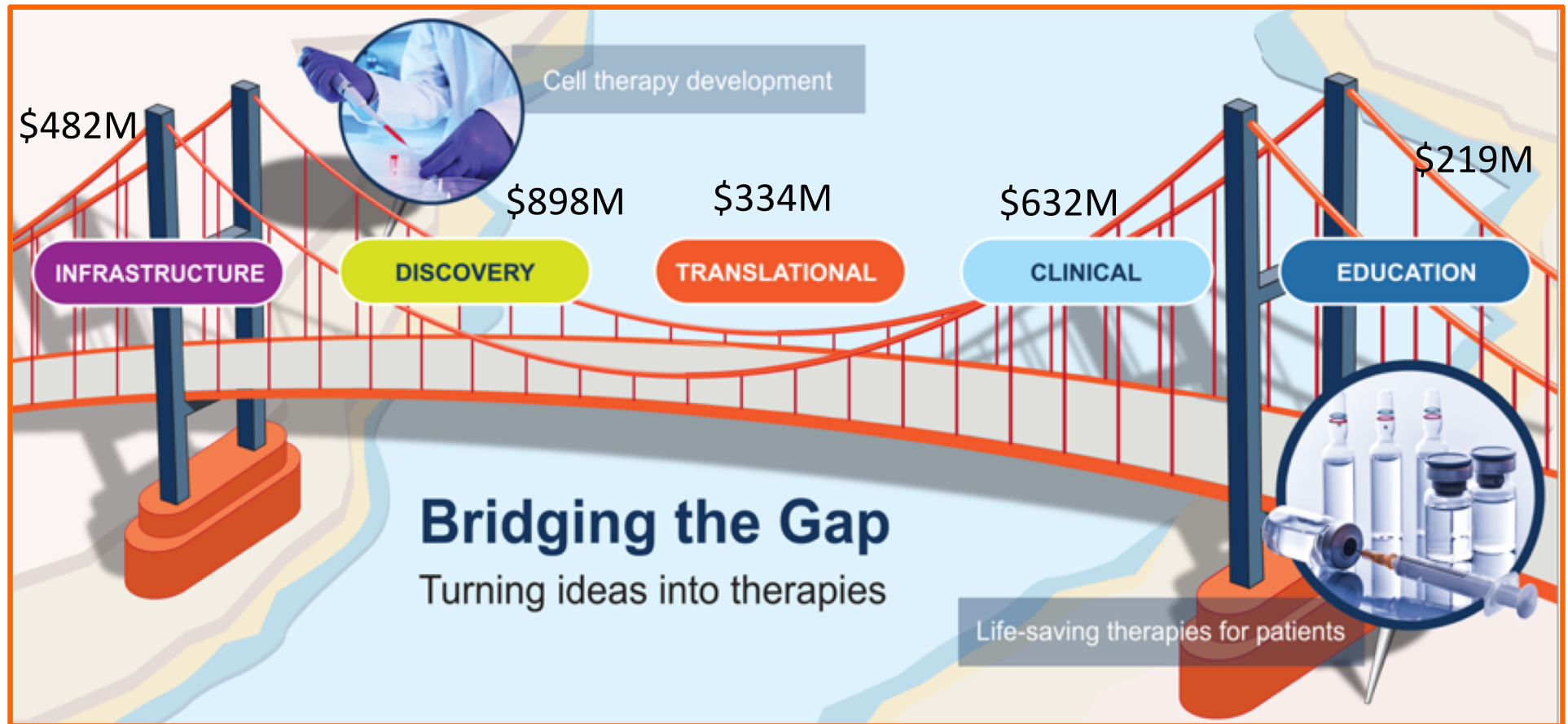
CIRM's Mission

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

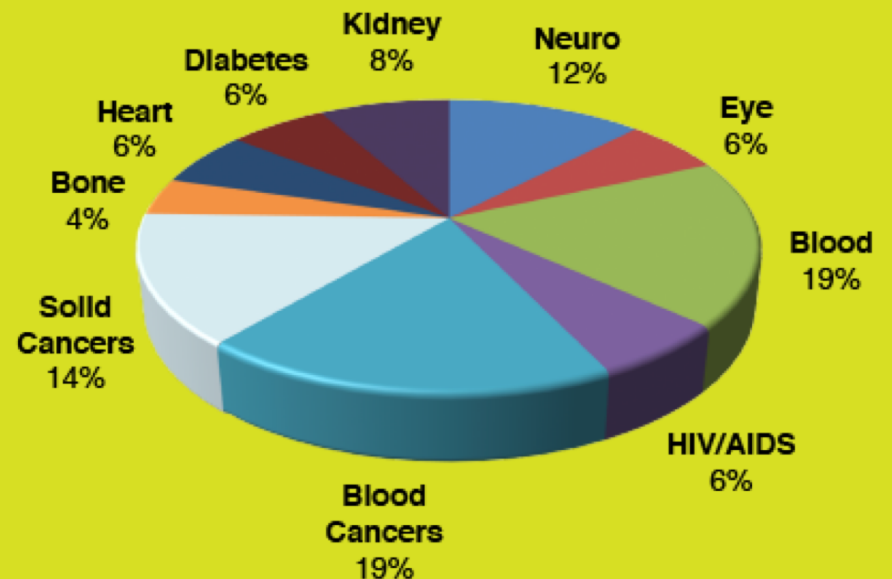
Building the CIRM Clinical Portfolio

Broad Disease Indications – Unmet Medical Needs
Diverse Therapeutic Approaches

CIRM-FUNDED CLINICAL TRIALS:



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	\$10M	7 projects
Education	\$750K	\$150K	
Total	\$171M	\$148M	

2018 Year End Budget Update

As of January 1, 2018

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

2018 Estimated Activity

- 30 Awards Added \$148.05M
- 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 (2019) ~\$30M

CIRM Budget Update

January 1st, 2019



Research

+







Administration

2019 Budget Planning

- \$144M Research Budget does not include projected recuperated/returned funds. Any residuals will be applied to a 2020 Research Budget.
- Research Programs:
 - No DISC awards planned for 2019
 - TRAN and CLIN1 awards will be more limited in scope; previously funded programs will be "grandfathered"
 - Strategic plan target of 50 new clinical trials (bringing CIRM total to 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

- Can achieve 43-45 new clinical trials & ~43 new candidates

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



CLINICAL

Pre-IND
Meeting or
Equivalent



Approved
Therapy

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

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

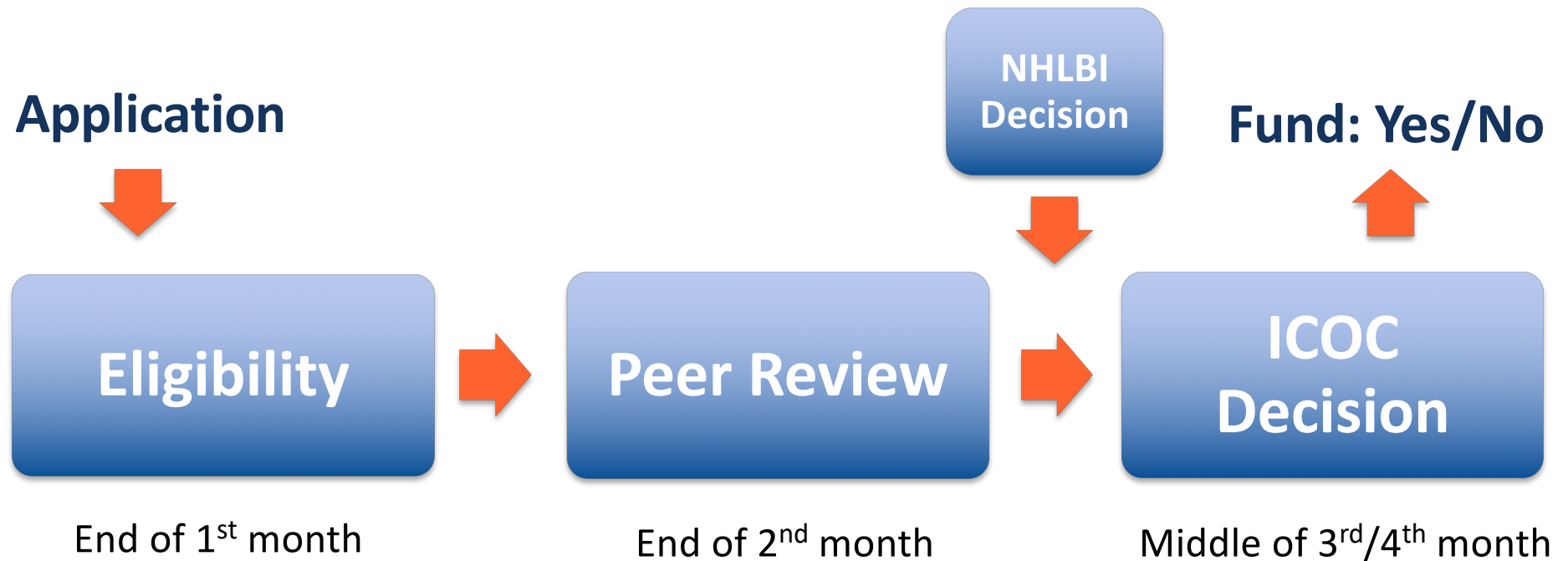
- NIH will rely on CIRM's application, scientific peer review, contracting and post-award management
- Funding decisions in as little as 85 days
- Leveraged funding for sponsors
- Data Sharing

Changes to our CLIN Program & RFA

in support of the Cure Sickle Cell Initiative

1. Application materials will be shared with NHLBI representatives
2. Awardees are required to comply with NHLBI DS&M and Data Sharing policies
3. Allows Non-CA Orgs to apply who are requesting their CIRM Unallowable Costs to be covered with NHLBI funds
4. 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

1. NHLBI funds come to CIRM via "Other Transactional Authority"
2. CIRM issues Notice of Award for CIRM + NHLBI funds
3. Progress & Financial Reports shared with NHLBI via Grants Management System
4. NHLBI representative to be appointed to CAP
5. 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 new (non-pipeline) projects
- Adds requirements for NHLBI/CIRM Cure Sickle Cell Disease Joint Initiative

Current CIRM Translation Program

Proof of concept studies (e.g., DISC2)

**Therapeutic
(TRAN1)**

**Diagnostic
(TRAN2)**

**Device
(TRAN3)**

**Tool
(TRAN4)**

30 months

\$4M/\$2M

Small molecules

Biologics

Cell therapy

24 months

\$1.2M

24 months

\$2M

24 months

\$1M

Proposed CIRM Translation Program

Proof of concept studies (e.g., DISC2)

**Therapeutic
(TRAN1)**

Pipeline Projects

30 months

Small molecules

Biologics

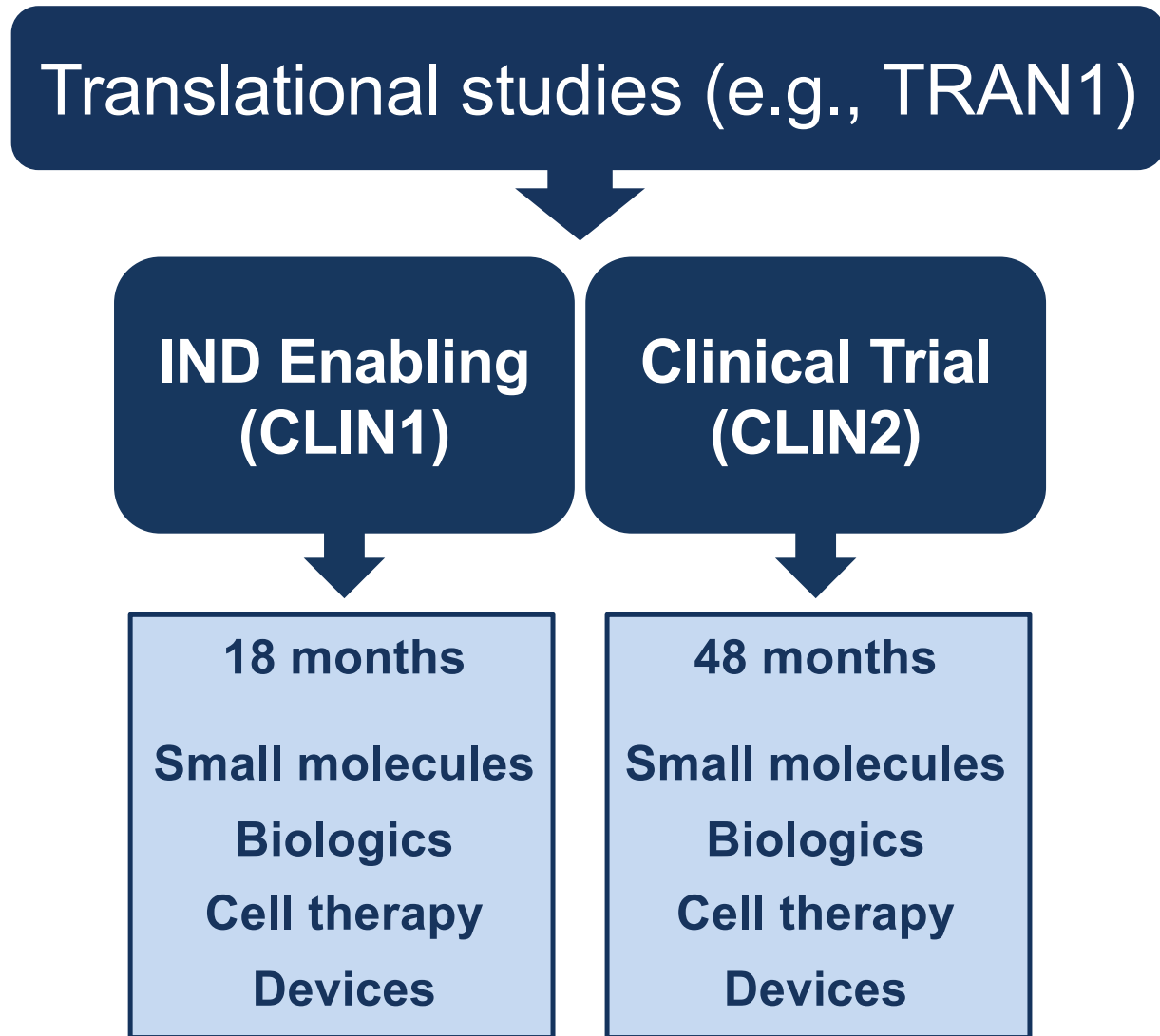
Cell therapy

New Projects

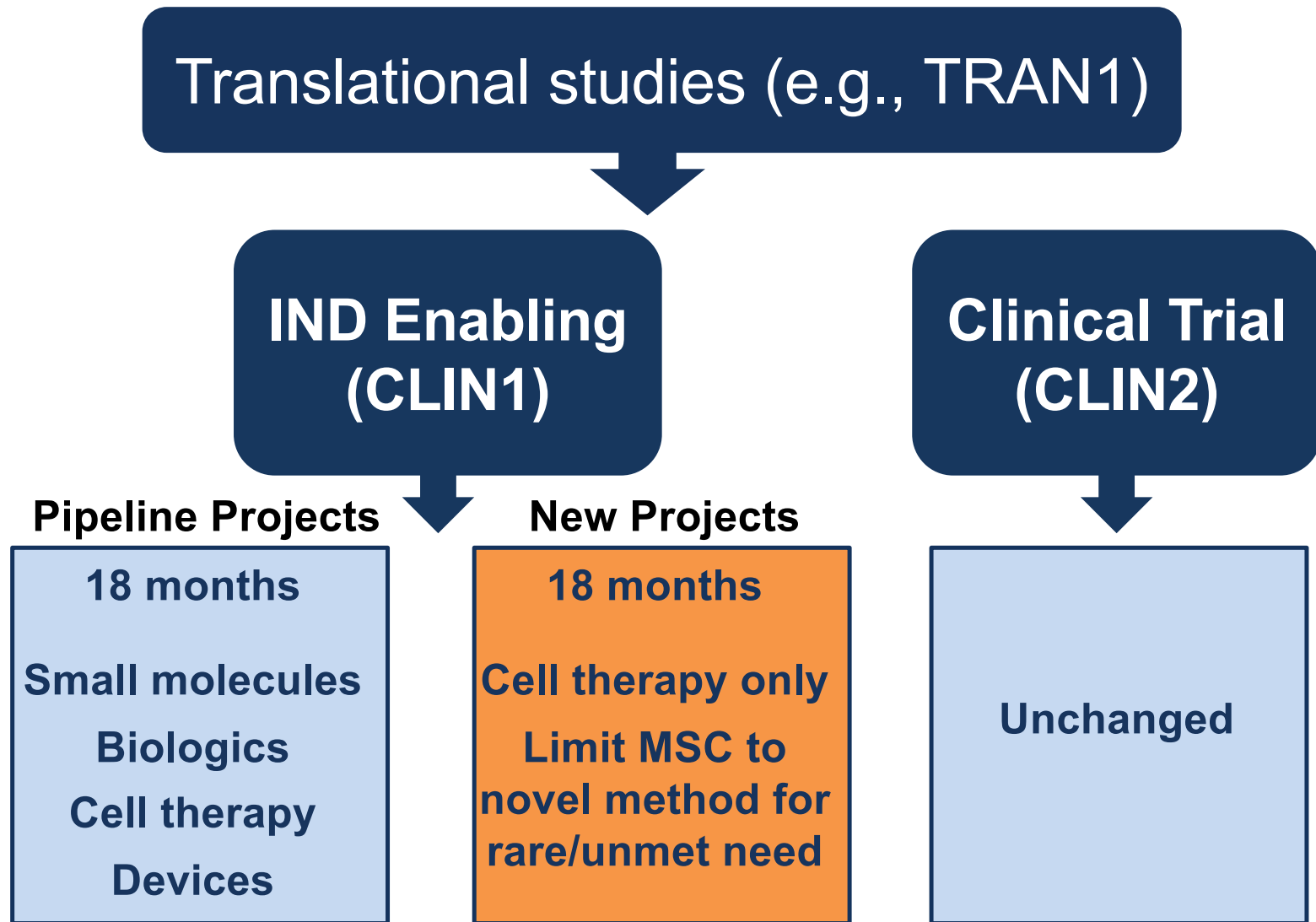
30 months

Cell therapy only

Current CIRM Clinical Program



Proposed CIRM Clinical Program



Changes to Support CIRM/NHLBI Sickle Cell Disease Joint Initiative

- All sickle cell disease applications must be considered for joint funding
- 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?