

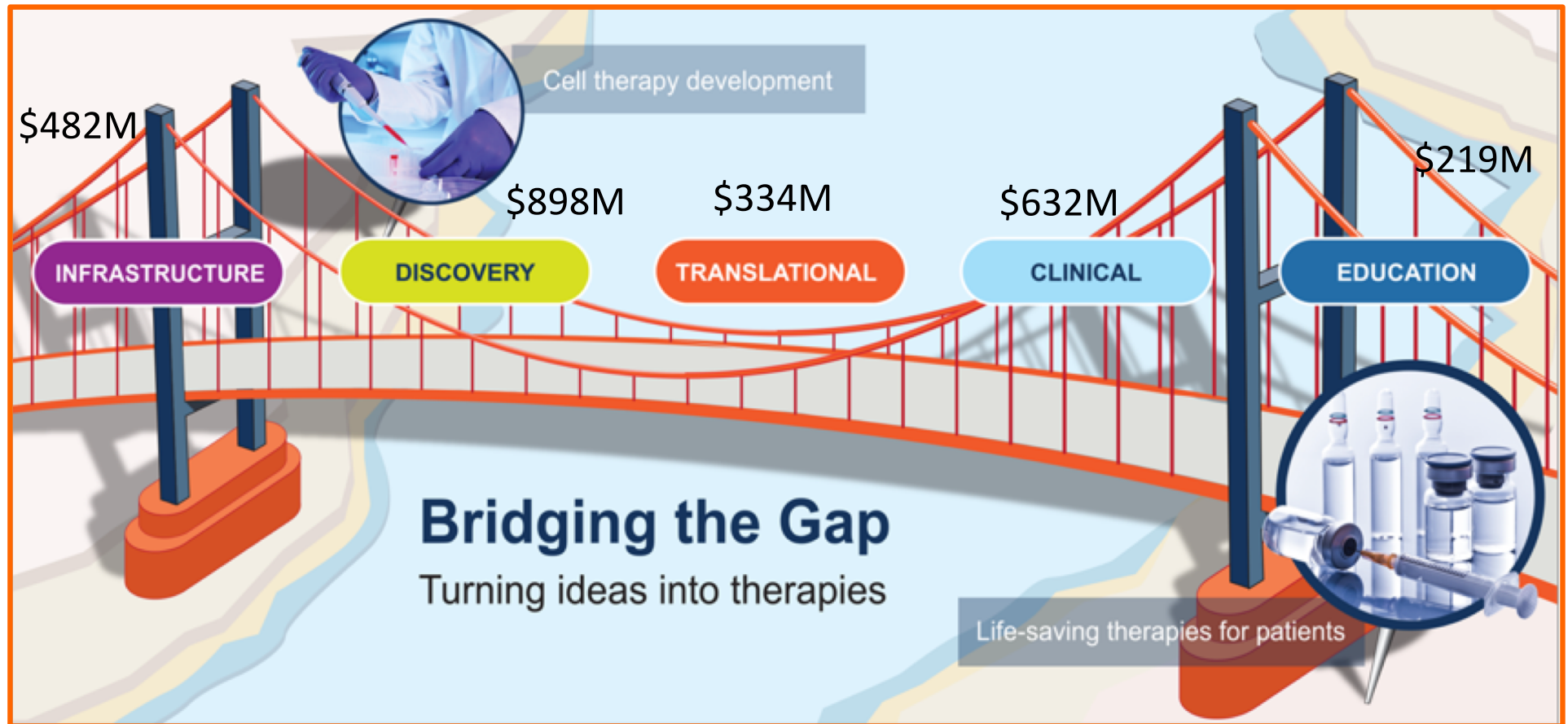


**TRANSFORMING** *medicine  
lives  
futures*

Science Subcommittee  
October 2018

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

# 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



# Executing on Strategic Plan 2016-2020





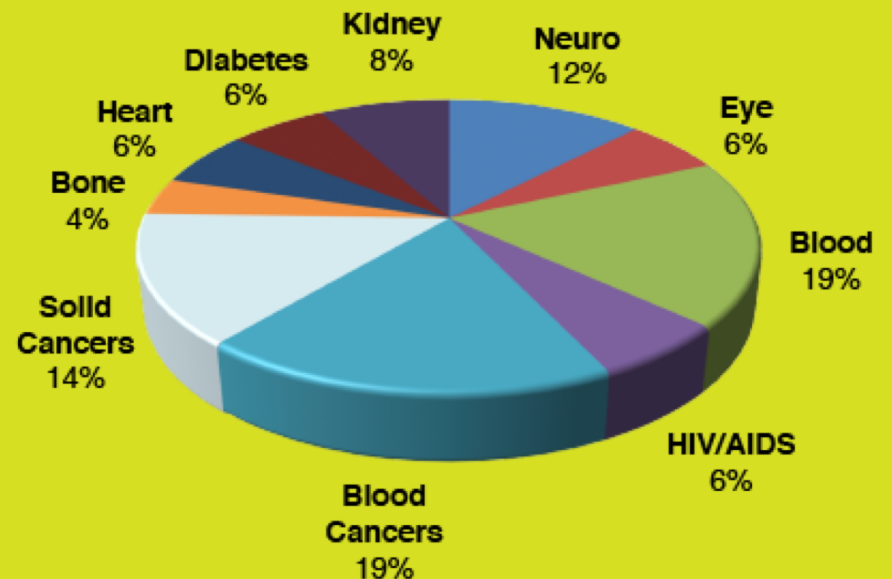
# Building the CIRM Clinical Portfolio

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	Notes
Clinical	\$130M	\$111M	~7 CLIN2s ~6 CLIN1s
Translation	\$30M	\$28M	7 candidates
Discovery	\$10M	\$10M	7 projects
Education	\$750K	\$150K	
<b>Total</b>	<b>\$171M</b>	<b>\$148M</b>	

# CIRM Budget Update

January 1<sup>st</sup>, 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:
  - Currently insufficient funds for DISC awards in 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 60).
  - \$30M budget NHLBI-CIRM program for CLIN awards for the Sickle Cell Cures initiative would enable us to fund 3 more CLIN programs than currently possible with remaining budget.
- Administrative Budget will provide sufficient staffing to manage all CIRM awards regardless of 2020 bond outcome

# 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 1<sup>st</sup>, 2019 \$144M**

Future Recovery Estimated (2019) ~\$30M

# 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. CIRM's Award Caps and Co-funding requirements will apply to the combined CIRM + NHLBI portion of the project
5. All sickle cell applications will submit to this revised program

# What is the process for review?



**Process Time: 80 to 110 days**

# You Don't Have to be in CA to Apply for CIRM Funding

**BUT**

1. Applicant must be the IND-holding organization
2. Different Allowable Project Costs rules apply for CA versus Non-CA applicant:
  - **California applicant organization** > 50% CA employees
  - **Non-California Organizations**  $\leq$  50% CA employees



# CIRM Award Caps

now applied to the CIRM+NHLBI share

Applicant Type	Pre-Clinical	Phase 1	Phase 2	Phase 3	CLIN3
Non-Profit	\$6M	\$12M	\$15M	\$10M	same as parent
For-Profit	\$4M	\$8M	\$15M	\$10M	same as parent

# Co-Funding Requirements

now applied to the Total Project Costs

Applicant Type	Pre-Clinical	Phase 1	Phase 2	Phase 3	CLIN3
Non-Profit	0%	0%	40%	50%	50%
For-Profit	20%	30%	40%	50%	50%

# CIRM Award Management

1. CIRM issues Notice of Award for CIRM + NHLBI funds
2. Progress & Financial Reports shared with NHLBI via Grants Management System
3. NHLBI representative to be appointed to Clinical Advisory Panel
4. 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**

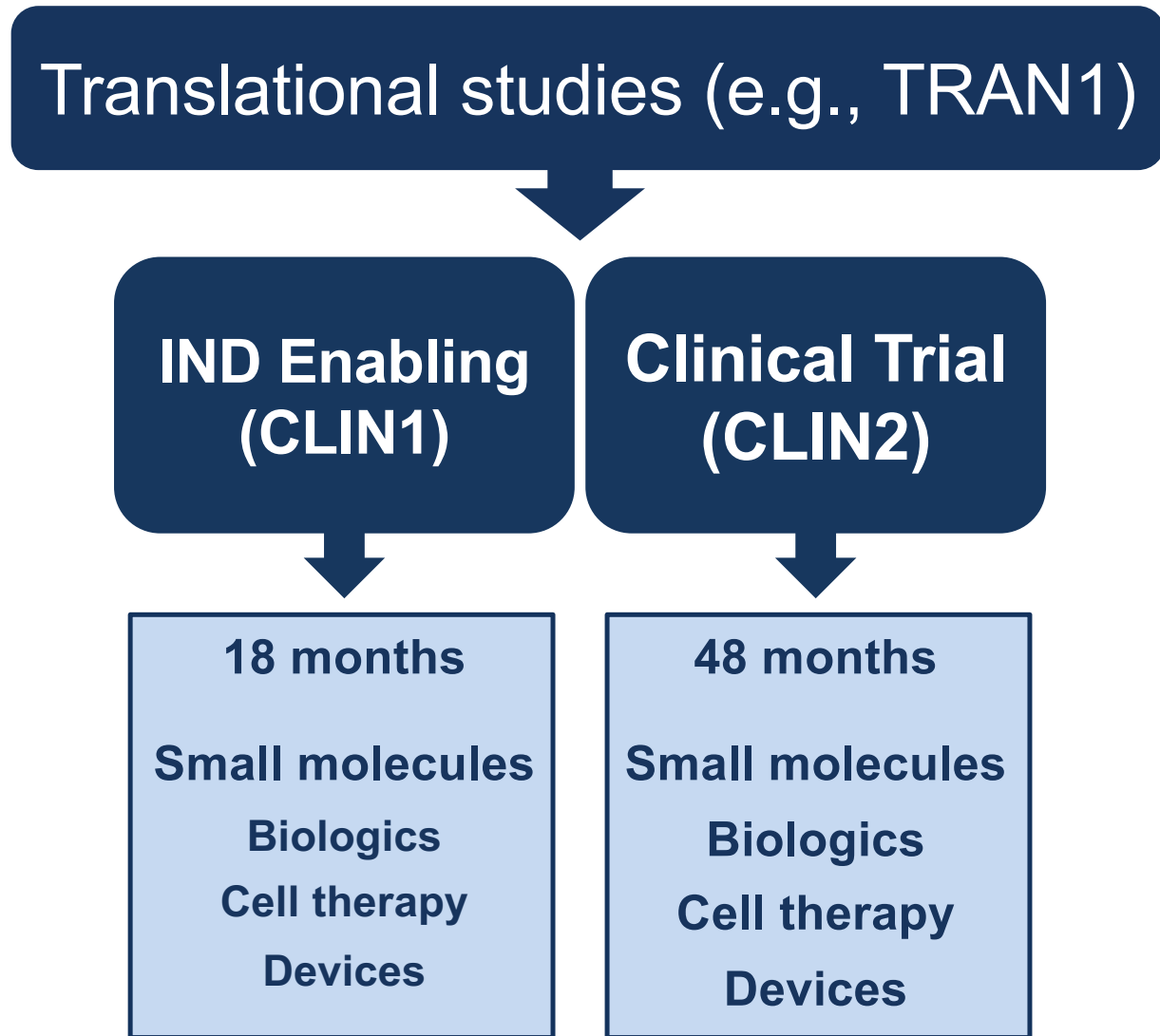
**Cell therapy**

**New Projects**

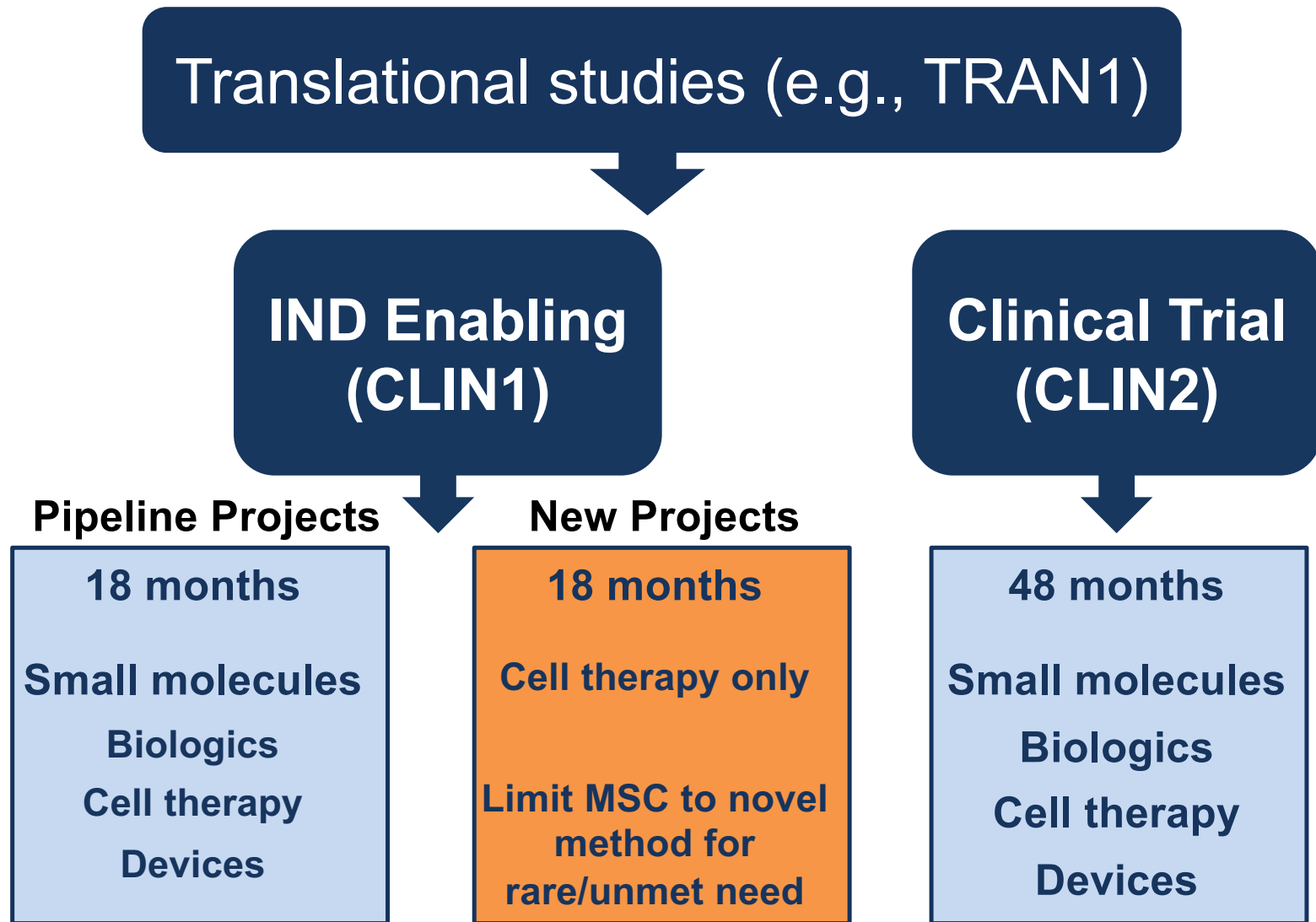
**30 months**

**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
- CIRM/NHLBI funding is subject to current maximums
- 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?