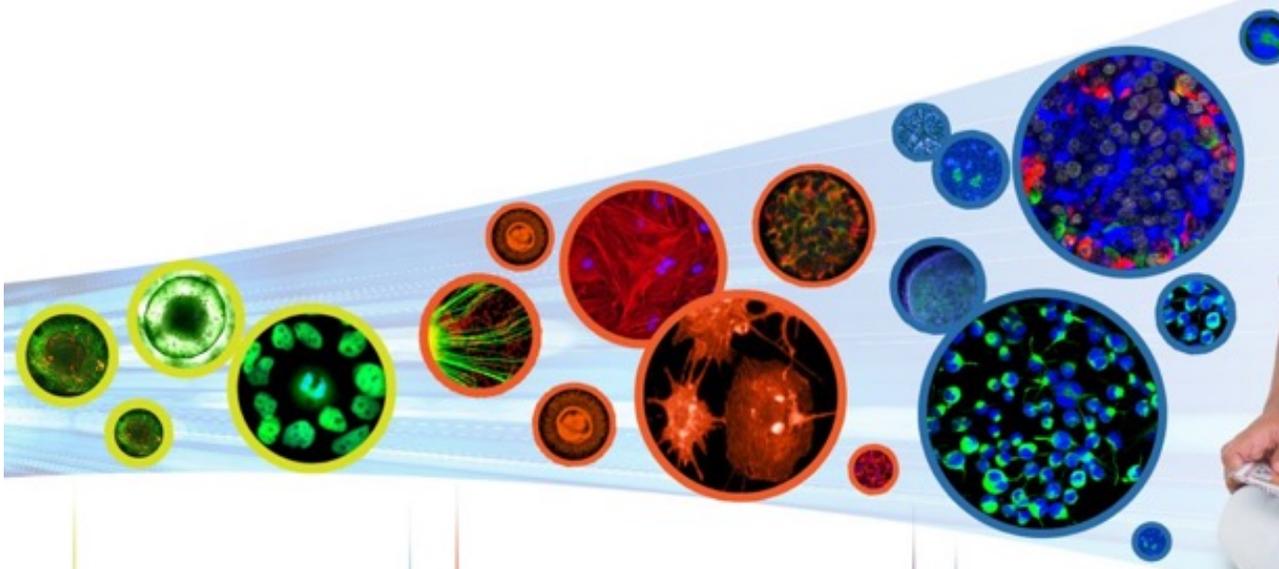




# CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY



**Joint Transition and Science Subcommittee  
Presentation of Budget Scenarios  
November 27, 2017**

**Maria T. Millan, M.D.**

President and CEO  
The California Institute for Regenerative Medicine

# Agenda

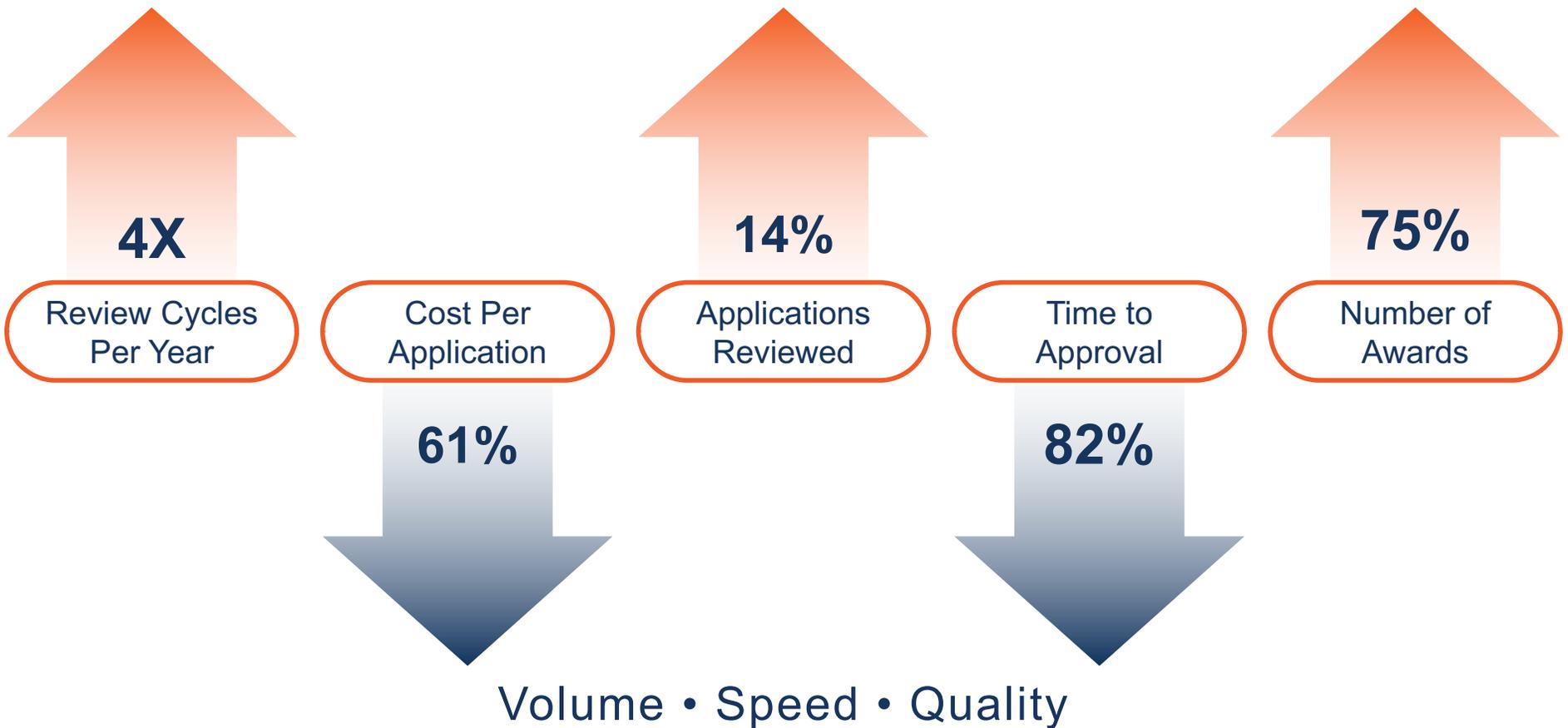
- Operating Assumptions
- Big Bucket for Research
- Small Bucket for Administration

# CIRM Value Proposition: 5 Pillars of investment accelerate science to treatments



# CIRM Value Proposition:

## Acceleration through 2.0 Operational Excellence



# 5-Year Strategic Plan

Launched January 2016

Beyond  
**CIRM**<sub>CALIFORNIA'S STEM CELL AGENCY</sub> 2.0  
*now it's personal*



NEW  
CANDIDATES  
INTO DEVELOPMENT



CLINICAL  
PROGRAMS  
WITH COMMERCIAL  
PARTNERS



INCREASE  
PROGRESSION  
EVENTS

BIG 6 2020 VISION



ENACT  
NEW REGULATORY  
PARADIGM



REDUCE  
TRANSLATION TIME



NEW  
CLINICAL  
TRIALS

# Operating Assumptions

- 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

# 5-Year Strategic Plan

## Research Budget Projections

- \$890M Budget to fund 5 Pillars through mid 2020
- \$440M projected for CLIN awards (50 CLIN2s at \$5-10M/award)
- Administrative & Research budgets to be fully expended by mid 2020

# Strategic Plan Research Budget

## Year 2 Update

- \$890M Budget to fund 5 Pillars through mid 2020
  - Success of the Clinical Program has led to faster than expected expenditure of the \$890M budget by Q4 2019
- \$440M planned for CLIN awards (50 CLIN2s at \$5-10M/award)
  - \$300M already expended on 26 trials + 9 CLIN1s
  - Average CLIN trial award has increased from \$10.9 in 2015/2016 to \$12.1M in 2017
- Administrative & Research budgets to be fully expended by mid 2020
  - Final Research awards will be made in 2019; Administrative Budget can fund into early 2021

# 2017 Big Bucket Budget

The projected dollar value of 2017 awards by category

| Program        | 2017 Allocation | 2017 Through Oct | Est 2017 Total * |
|----------------|-----------------|------------------|------------------|
| Education      | \$1M            | \$0.3M           | <b>\$1M</b>      |
| Discovery      | \$52M           | \$21M            | <b>\$46M</b>     |
| Translation    | \$45M           | \$10M            | <b>\$24M</b>     |
| Clinical       | Up to \$215M    | \$213M           | <b>\$213M</b>    |
| Infrastructure | \$16M           | \$16M            | <b>\$16M</b>     |
| <b>Total</b>   | <b>\$329M</b>   | <b>\$260.3M</b>  | <b>\$300M</b>    |

9 \* Est Remaining Approvals: 3 TRAN1s for \$13.6M; 13 DISC1s for \$2.8M; 11 DISC2s for \$21.1M

# 2017 Year End



## Estimated award activity for the calendar year 2017

As of January 1, 2017

▪ Committed / Uncommitted Balance \$2.22B / \$528M

2017 Estimated Activity

▪ 88 Awards Added \$299.9M

▪ Award Reductions \$41M

As of December 31, 2017

▪ Committed / Uncommitted \$2.48B / \$269M

Future Recovery (2018-2019) \$66M

**Available Big Bucket research funds (total) \$335M**

# CIRM Budget

January 1<sup>st</sup>, 2018



**Research**

+



**Administration**

# Budget Scenario

- \$335M left in Research and \$48M in Administration
- Research Budget - last awards planned for Q4 2019
- Budget for Administration for Q1 2018 - Q4 2023
  - Provide for sufficient staffing to manage all CIRM awards
  - Leave enough for wind-down funds in the event the 2020 bond is unsuccessful
- Adjustment of CLIN award caps
  - to allow predictably & planning for strategic plan goals
  - funding of full complement of research programs

# Research Budget Containment:

- Without reduction in CLIN award caps, we would only be able to fund the CLIN programs (assuming a target of 50 trials)
- Reduction in CLIN award caps would generate ~\$68M in savings
- \$68M savings would allow us to fund all 5 Pillars

# Current and Proposed Award Caps



| CLIN Program                                   | CLIN1        | Phase 1<br>Phase 1/2 | Phase 2        | Phase 3        |
|--|--------------|----------------------|----------------|----------------|
| Current CLIN Award Caps*                       | \$20M        | \$20M                | \$20M          | \$20M          |
| 2017 Average Award \$                          | \$4.9M       | \$10.3M              | \$15.3M        | \$16.7M        |
| Proposed Award Cap<br>Non-Profit<br>For-Profit | \$6M<br>\$4M | \$12M<br>\$8 M       | \$15M<br>\$15M | \$10M<br>\$10M |

\* \$20M CLIN Award caps are a holdover from Disease Team Awards which funded a combination of activities currently funded separately under CIRM2.0 TRAN, CLIN1 & CLIN2 programs.

# Research Budget Allocation Proposal with new award caps

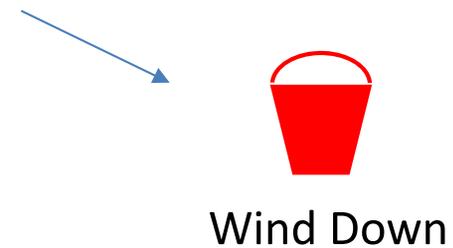
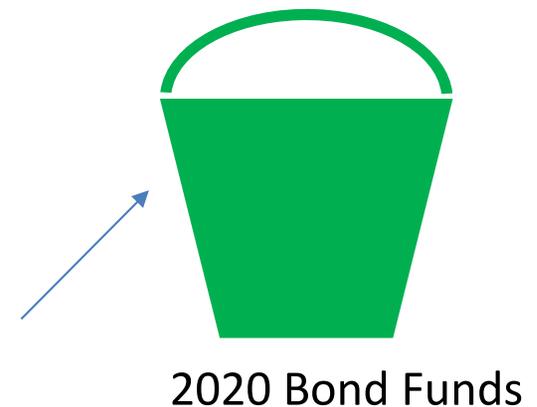


|             |   | 2018  | 2019  |
|-------------|---|---|---|
| <b>CLIN</b> |    | <b>\$130M</b><br>(12 trials + 4 Candidates) | <b>\$130M</b><br>(12 trials + 4 Candidates) |
| <b>TRAN</b> |    | <b>\$30M</b><br>(6 Candidates)              | <b>\$25M</b><br>(5 Candidates)              |
| <b>DISC</b> |   | <b>\$10M</b><br>(10 Projects)               | <b>\$9.25M</b><br>(9 Projects)              |
| <b>EDUC</b> |  | <b>\$750K</b>                               | <b>\$0</b>                                  |

- Can achieve 50 new clinical trials & 40 new candidates
- DISC and EDU with limited budget

# Administration Budget

| FY     | 17/18   | 18/19   | 19/20   | 20/21 - beyond |
|--------|---------|---------|---------|----------------|
| Budget | \$14.4M | \$14.1M | \$12.6M | \$14M          |



# Administration Budget

## Wind-Down Scenario

 *Trigger wind down only if no 2020 bond*

| FY                            | 17/18   | 18/19   | 19/20   | 20/21  | 21/22  | 22/23  | Q3-Q4<br>2023 |
|-------------------------------|---------|---------|---------|--------|--------|--------|---------------|
| Budget with additional \$8.5M | \$14.4M | \$14.1M | \$13.8M | \$9.5M | \$5M   | \$4M   | \$2.7M        |
| Budget with no funds raised   | \$14.4M | \$14.1M | \$12.6M | \$5.7M | \$3.0M | \$3.0M | \$2.3M        |

|  |         |
|--|---------|
| Wind Down Costs beyond June 2020*                          | \$22.4M |
| Residual Funds \$6.9M (Prop 71)<br>+ \$7.0M raised to date | \$13.9M |
| Fundraising Needs  | \$8.5M  |

\*includes close-out costs

# Budget Scenario

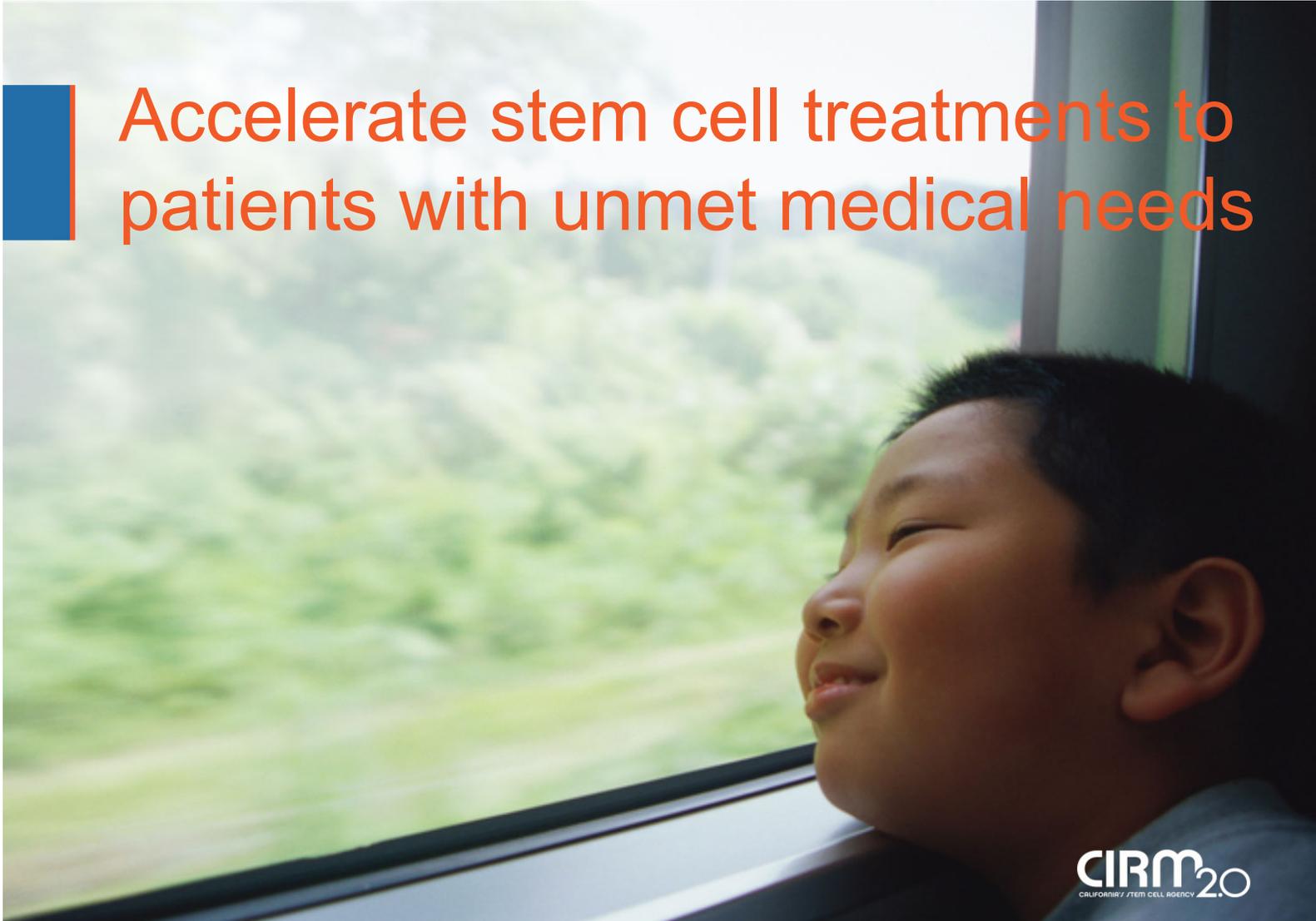


- Consistent with 5-year strategic goals
- Preserves the 5 pillars
- Ensures that Awards from 2019 (including those launched in early 2020) will be managed until completion
- Keeps CIRM value proposition and operations as intact as possible for a planned 2020 bond measure & reserves enough funds if wind down necessary

# CIRM's Mission

Beyond  
**CIRM**<sub>CALIFORNIA / STEM CELL AGENCY</sub> 2.0  
*now it's personal*

Accelerate stem cell treatments to patients with unmet medical needs



**CIRM**<sub>CALIFORNIA / STEM CELL AGENCY</sub> 2.0

All In All Out | Every Moment Counts