CIRM’s Mission

Accelerate stem cell treatments to patients with unmet medical needs
Agenda

- 2017 Highlights & Update on Strategic Plan
- Budget
- Proposal and Requested Action
  - CLIN award CAP Reduction
  - 2018 Budget approval
2017 Highlights

Cell therapy is coming of age

- Marketing approvals for Novartis and Kite/Gilead’s CAR-T products
- FDA Regulatory Reform under the 21st Century Cures Act
- NIH – CIRM Partnership Opportunities
2017 Highlights

RMAT Expedited Regulatory Pathway

- 21st Century Cures Act of Dec 2016 created the expedited FDA pathway RMAT launched in Q1 2017
  - Stem cell, gene therapy, regenerative medicine, tissue engineered
  - Nimble process
  - Combines breakthrough designation and accelerated pathway

- CIRM grantees among the first in the U.S. to obtain RMAT designation
  - Humacyte- engineered vascular graft for kidney failure
  - jCyte- cell therapy for blinding eye disease
  - Asterias- cell therapy for spinal cord injury
2017 Highlights

CIRM-NIH Partnership

- NIH Cures Act-funded Regenerative Medicine Innovation Program
  - Dec. 6-7 NIH-FDA Regenerative Medicine Innovation Workshop
  - NIH $30M/5yrs for adult stem cells

- CIRM-NIH joint site visit for NHLBI Sickle Cell Cures Initiative
  - $1.7M 2-yr. award to COH for GMP-compatible gene editing

- CIRM involvement in NIDCR multi-center award
  - Harmonization of project objectives so successful researchers would be eligible and competitive for CIRM’s TRAN program
5 Pillars of investment to Accelerate Science to Cures

Infrastructure
$16M

Education
$1M

Discovery
$45M

Translation
$24M

Clinical
$213M
Acceleration by Design
2017 Performance

57% LOWER cost per application
82% LESS time to approval
33% More applications reviewed
75% More high quality programs funded

2.5 fold Expansion Clinical Portfolio
700 CA Patients enrolled in CIRM trials to date
75% on time clinical milestones

Every Moment Counts | Don’t Stop Now
2017 Update on Strategic Plan: 
Clear and on Target

- **Ahead**
  - 24 of 50 New Candidates

- **Exceed**
  - 2-fold increase in Progression

- **Lead**
  - Expedited Regulatory Path
  - 25% RMATs

- **Gaining**
  - 3 INDs in <18 Months

- **Ahead**
  - 26 of 50 New trials

- **Increase**
  - 6 Industry Investments in 2017

**Private Industry Investment**

- **$41.3M**
  - 2015

- **$125M**
  - 2016

- **$306.7M**
  - 2017

Wheels up and powered to Break Barriers
Leveraging CIRM Investment to Date

$911M*<sup>a</sup>
Co-funding by applicant

$473M<sup>a</sup>
Resulting Partnership Funding

> $395M<sup>a</sup>
Additional funds due to CIRM award

* Including $50M from non CA
<sup>a</sup> Approximate amounts

Every Moment Counts | Don’t Stop Now
Agenda Item #5

CIRM Budget Update
January 1st, 2018

Research
$335M

Administration
$48M
5-year Strategic Plan launched in 2016 with a $890M Budget

- Success of the Clinical Program has led to faster than expected expenditure
- Estimate last awards by Q4 2019 vs. original Q2 2020 projection

Strategic Plan projected $440M for 50 clinical trials

- $300M already expended on 26 trials + 9 CLIN1s
- Average CLIN trial award increased from $10.9 in 2015/2016 to $12.1M in 2017

Administrative & Research budgets to be fully expended mid 2020

- Final Research awards will be made in 2019; Administrative Budget available beyond 2020
# 2017 CIRM Research Awards

<table>
<thead>
<tr>
<th>Program</th>
<th>2017 Allocation</th>
<th>Est 2017 Total *</th>
</tr>
</thead>
<tbody>
<tr>
<td>Education</td>
<td>$1M</td>
<td>$1M</td>
</tr>
<tr>
<td>Discovery</td>
<td>$52M</td>
<td>$46M</td>
</tr>
<tr>
<td>Translation</td>
<td>$45M</td>
<td>$24M</td>
</tr>
<tr>
<td>Clinical</td>
<td>Up to $215M</td>
<td>$213M</td>
</tr>
<tr>
<td>Infrastructure</td>
<td>$16M</td>
<td>$16M</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>$329M</strong></td>
<td><strong>$300M</strong></td>
</tr>
</tbody>
</table>

* Est Remaining Approvals: 11 DISC2s for $21.1M
2017 Year End Budget Update

As of January 1, 2017
• Committed / Uncommitted Balance $2.22B / $528M

2017 Estimated Activity
• 88 Awards Added $299.9M
• Recovery from unexpended funds $41M

As of December 31, 2017
• Committed / Uncommitted $2.48B / $269M

Future Recovery (2018-2019) $66M
Available Big Bucket research funds (total) $335M
Budget Planning

Operating Principles

- 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
Research Budget Background:

- $335M left in Research with last awards in Q4 2019

- With the current CLIN award budgets and a goal of 50 new clinical trials, we would only have enough research funds for CLIN programs (no additional DISC or TRAN awards)

- Reduction in CLIN award caps would generate ~$68M in savings to fund additional DISC and TRAN programs
## CLIN Award Caps:
### Current Status and Proposed Caps

<table>
<thead>
<tr>
<th>CLIN Program</th>
<th>CLIN1</th>
<th>Phase 1 (Phase 1/2)</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Current CLIN Award Caps*</td>
<td>$20M</td>
<td>$20M</td>
<td>$20M</td>
<td>$20M</td>
</tr>
<tr>
<td>2017 Average Award $</td>
<td>$4.9M</td>
<td>$10.3M</td>
<td>$15.3M</td>
<td>$16.7M</td>
</tr>
<tr>
<td>Proposed Award Cap Non-Profit</td>
<td>$6M</td>
<td>$12M</td>
<td>$15M</td>
<td>$10M</td>
</tr>
<tr>
<td>Proposed Award Cap For-Profit</td>
<td>$4M</td>
<td>$8M</td>
<td>$15M</td>
<td>$10M</td>
</tr>
</tbody>
</table>

* $20M CLIN Award caps are a holdover from Disease Team Awards which funded a combination of activities currently funded separately under TRAN, CLIN1 & CLIN2 programs.
CLIN Award Sizes (2015-2017)

Averages and Medians

- **Max**
- **Avg**
- **Median**
- **Min**
### Proposed Research Budget Allocation with CLIN Award Cap Reduction

<table>
<thead>
<tr>
<th></th>
<th>2018</th>
<th>2019</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>CLIN</strong></td>
<td><strong>$130M</strong> (12 trials + 4 Candidates)</td>
<td><strong>$130M</strong> (12 trials + 4 Candidates)</td>
</tr>
<tr>
<td><strong>TRAN</strong></td>
<td><strong>$30M</strong> (6 Candidates)</td>
<td><strong>$25M</strong> (5 Candidates)</td>
</tr>
<tr>
<td><strong>DISC</strong></td>
<td><strong>$10M</strong> (8 Projects)</td>
<td><strong>$9.25M</strong> (7 Projects)</td>
</tr>
<tr>
<td><strong>EDUC</strong></td>
<td><strong>$750K</strong></td>
<td><strong>$0</strong></td>
</tr>
</tbody>
</table>

- Can achieve 50 new clinical trials & 40 new candidates
- DISC and EDU with decreased offerings
CIRM’s Research Budget Scenarios:

- Consistent with 5-year strategic goals
- Preserves the full complement of research programs
- Preserves CIRM’s “acceleration by design” operations
- Administration Budget* can support the personnel required for this Research Budget Plan.

*Administration Budget scenario presented to the Joint Research and Transition subcommittees in November. Formal Budget request for 18/19 will be brought to the ICOC in March 2018
Requested Action

The CIRM team requests that the ICOC approve the proposed reduction in the maximum funding level for CLIN awards according to the below:

<table>
<thead>
<tr>
<th>CLIN Program</th>
<th>CLIN1</th>
<th>Phase 1 (Phase 1/2)</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Non-Profit</td>
<td>$6M</td>
<td>$12M</td>
<td>$15M</td>
<td>$10M</td>
</tr>
<tr>
<td>For-Profit</td>
<td>$4M</td>
<td>$8M</td>
<td>$15M</td>
<td>$10M</td>
</tr>
</tbody>
</table>
Requested Action

The CIRM team requests that the ICOC approve the 2018 proposed research budget allocation of:

- $130M for CLIN
- $30M for TRAN
- $10M for DISC
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Accelerate stem cell treatments to patients with unmet medical needs