Topics

- Mission
- FY 3Q15 Financial Update
- CIRM 2.0 Clinical Stage Program Review
- Preview: CIRM 2.0 Discovery and Translation
- Preview: CIRM 2.0 Educational Initiatives
- Coming Attractions
Our Mission

To accelerate stem cell treatments to patients with unmet medical needs.
FINANCIAL UPDATE FOR FY-3Q15
Two Separate Funding “Buckets”

Award Budget
$2.75 Billion

Administrative Budget
$180 Million
The Administrative Bucket

Administrative Budget

$180 Million

Five more years of funding available at this rate

- $98 million spent
- $82 million remaining
- Current spend rate of $15-16 million per year
The Award Bucket

Sufficient to last into FY 2020

- $1.95 billion awarded or spent
- $800 million uncommitted
- Planned NET commitment rate of $170 million per year
  - $190 million in new awards
  - $20 million in reductions

Award Budget
$2.75 Billion
CIRM’s $2.75 Billion Award Budget
3Q15 Activity

Uncommitted
$797 million
Down: $53M

Awarded
$455 million
Up: $19M

Spent
$1.49 billion
Up: $35M

New Awards
$56 M

Award Payments
$35 M

Award Repayments
$1 M

Award Reductions / Cancelations
$2 M
CIRM’s $2.75 Billion Award Budget

Year-to-Date Activity

Uncommitted
$797 million
Down: $79M

Awarded
$455 million
Down $67M

Spent
$1.49 billion
Up $147M

New Awards
$107 M

Award Payments
$147 M

Award Repayments
$1 M

Award Reductions / Cancelations
$27 M

Net Commitments of $79M YTD
CIRM 2.0 CLINICAL REVIEW
The CIRM Spectrum

Bridging the Gap
Turning ideas into therapies

Cell therapy development
Life-saving therapies for patients
Clinical Stage Has Three Programs

**CLINICAL STAGE**

- Partnering Opportunity For Late Stage Preclinical Projects (PA15-01)
- Partnering Opportunity For Clinical Trial Stage Projects (PA15-02)
- Partnering Opportunity For Supplemental Accelerating Activities (PA15-03)
Application Summary
CIRM 2.0 Clinical Programs

Key Takeaways

- 12 applications requesting a total of $111 million
- Demand is growing (for now)
- 8 (66%) applications have been for clinical trials
  - 2 - Phase I
  - 1 - Phase II
  - 5 - Phase III
- Starting to see requests for accelerating activities of existing programs
# Evaluating the Review Process

## Clinical Program

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<th>Screening</th>
<th>Budget Review</th>
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CIRM 2.0 Clinical is Thus Far…

- Being well utilized
- Manageable from an operational standpoint
- Significantly reducing evaluation times
- Attracting later stage programs than currently exist in the CIRM portfolio
- Not without challenges
- Only the first part of the CIRM development continuum to be introduced
CIRM 2.0 DISCOVERY AND TRANSLATIONAL UPDATE
Objective: To promote the discovery of promising new stem cell technologies and drive their rapid translation towards improving patient care.

Key characteristics:

1. Accelerate
2. Increase probability of success
3. Address unmet medical needs
4. Efficient
1. We Need a Continuous, Predictable Path from Discovery to Clinical
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2. Progression Needs to be Possible, Expected, and Incentivized

Funded project “A” spontaneously progresses to “B” only on very rare occasions (5%).
2. Progression Needs to be Possible, Expected, and Incentivized

When CIRM issues an RFA with an “intent to progress,” projects advance to the next development stage (B to C) approximately **30% of the time!**

“If you want a bike, don’t ask for a toy.”

Dr. Lila Collins
CIRM Sr. Science Officer
3. Opportunities Must be Responsive to New Developments and Downstream Needs
4. Development Pathways Are Needed for All Product Types
4. Development Pathways Are Needed for All Product Types
Summary: CIRM 2.0 will Improve Discovery & Translation in Four Key Areas

- Continuous, Predictable Pathway
- Prescribed Progression
- Responsive
- Multiple Development Pathways
CIRM 2.0 FOR CREATIVITY AND BRIDGES
Objective: To inspire, educate and motivate high school students to become involved in CIRM’s mission of accelerating stem cell treatments to patients with unmet medical need.

What We Are Keeping

- Supervised “hands on” research activities in laboratories focused on human disease and regenerative medicine
- Poster Day presentation on individual research projects at the conclusion of each summer program
- Broadening enrollment and participation from under-represented populations
Objective: To prepare California’s undergraduate and Master’s level students for a highly productive career in stem cell research & development.

What We Are Keeping

- Hands-on research internship
- Stem cell techniques training course
- Educational enhancement related to stem cells
- Broadening participation from under-represented populations
SPARK and Bridges

A first class research experience
SPARK and Bridges
Reimagined to Add More Context

Dynamic Career Counseling*
Patient Interaction
Regulatory Training
Community Outreach Activities

*Bridges Students
THINGS TO COME
What’s Next

July
- Creativity and Bridges Concept Plans
- CIRM 2.0 Discovery and Translational Stage Program Concept Plans
- Strategic Plan
- Performance Audit Action Plan

Remainder of 2015
- Initiate CIRM 2.0 Core
- Relocate CIRM’s offices
- Submit Alpha Clinics “Accelerating Center” Concept Plan
- Operationalize the New Discovery, Translation, Creativity, and Bridges Programs
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