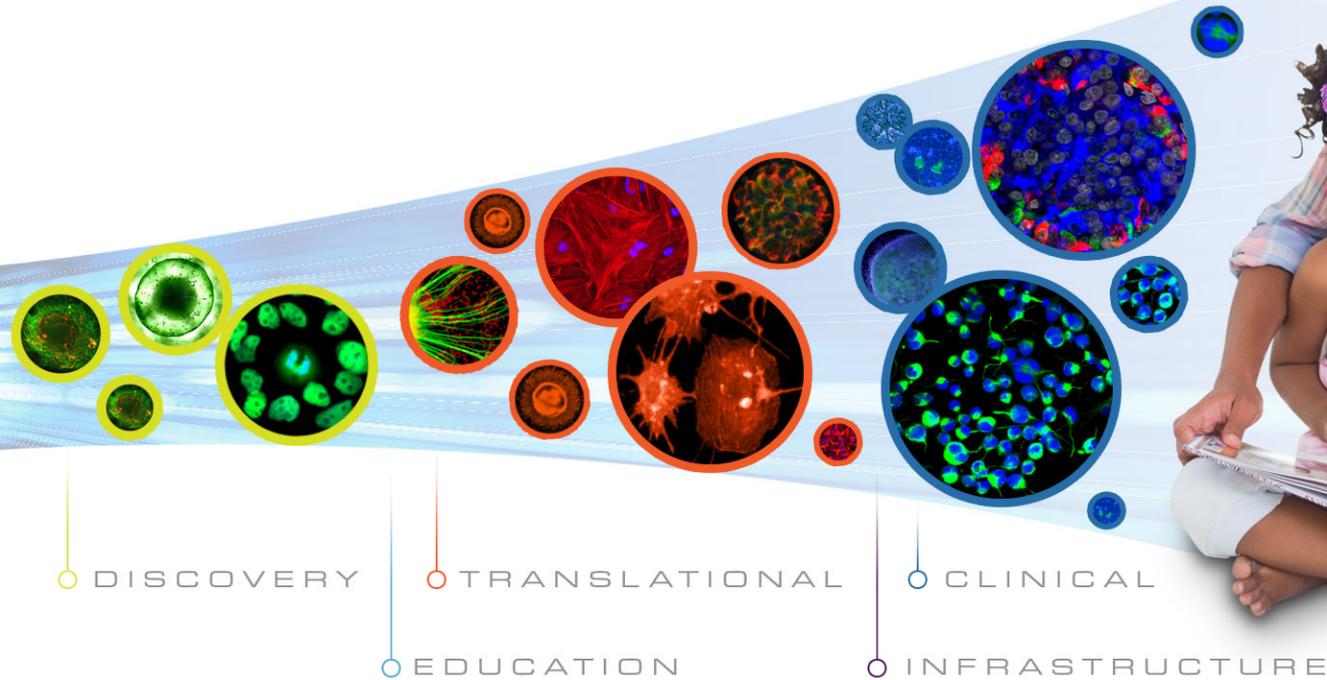


May 21, 2015

CIRM

CALIFORNIA'S STEM CELL AGENCY



President's Report

C. Randal Mills, Ph.D.

President and Chief Executive Officer
California Institute for Regenerative Medicine

Topics



DISCOVERY
TRANSLATIONAL
CLINICAL
EDUCATION
INFRASTRUCTURE

- 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



Award Budget

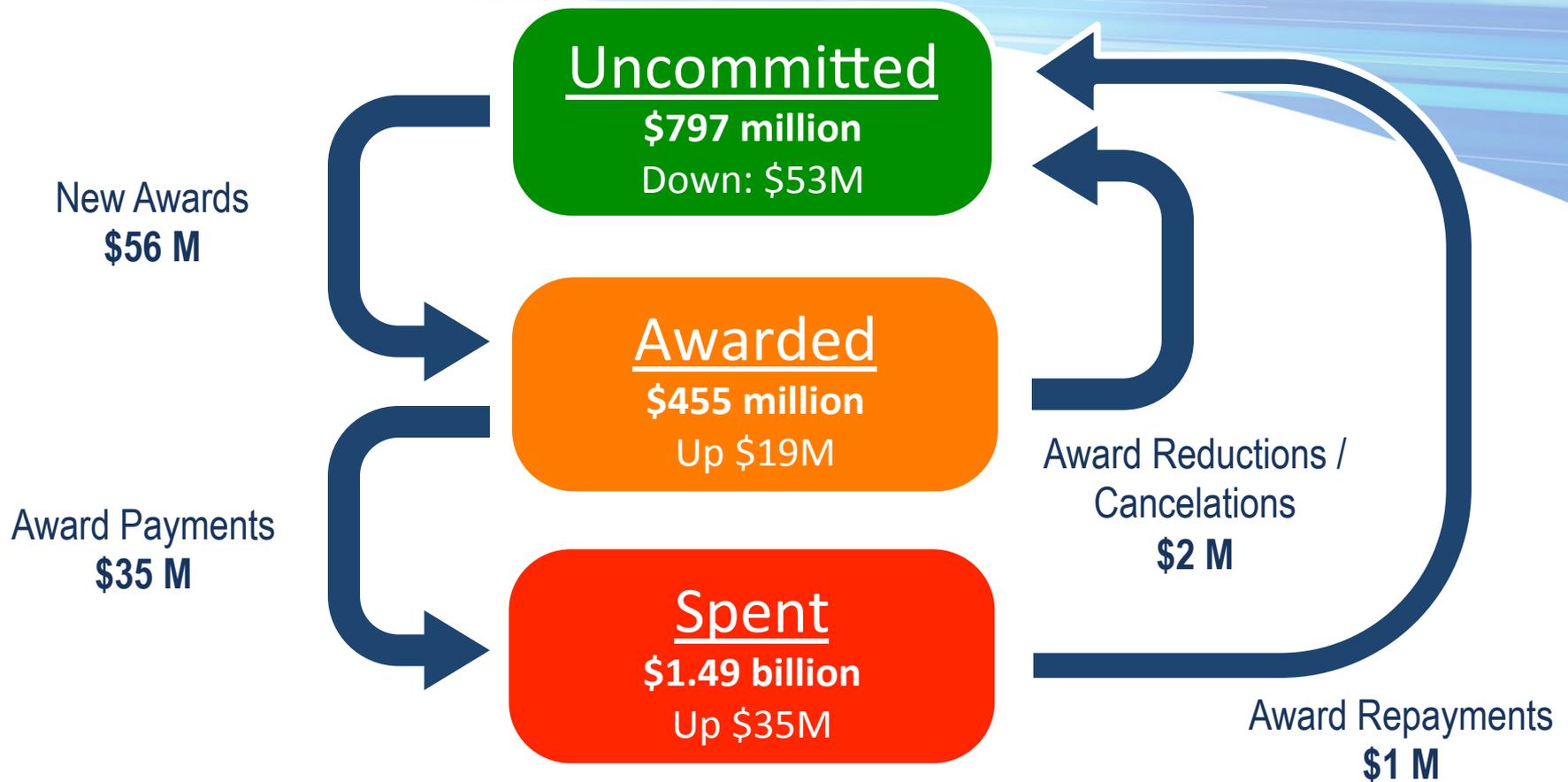
\$2.75 Billion

Sufficient to last into FY 2020

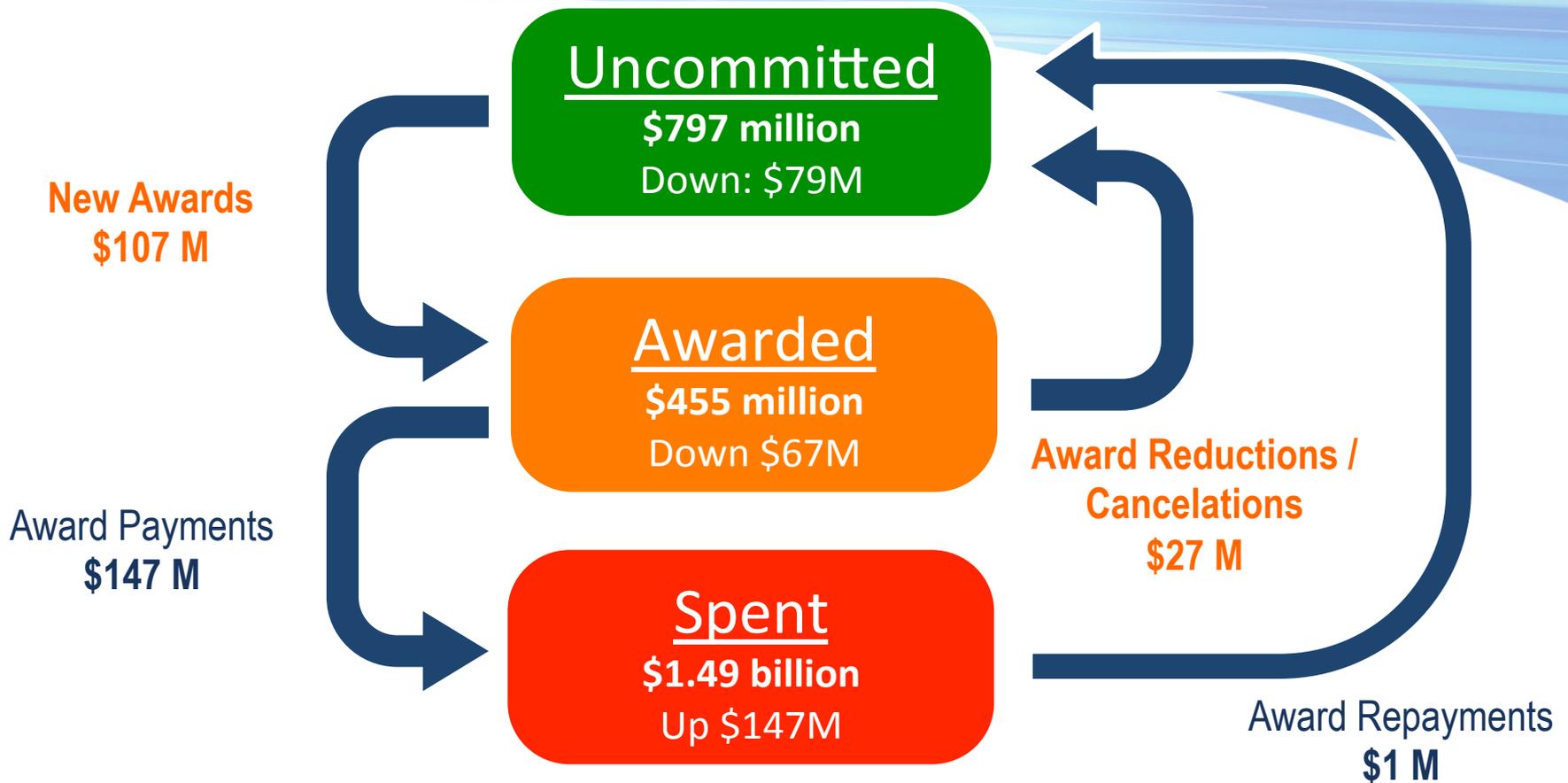
- \$1.95 billion awarded or spent
- \$800 million uncommitted
- Planned NET commitment rate of \$170 million per year
 - \$190 million in new awards
 - \$20 million in reductions

CIRM's \$2.75 Billion Award Budget

3Q15 Activity



CIRM's \$2.75 Billion Award Budget Year-to-Date Activity

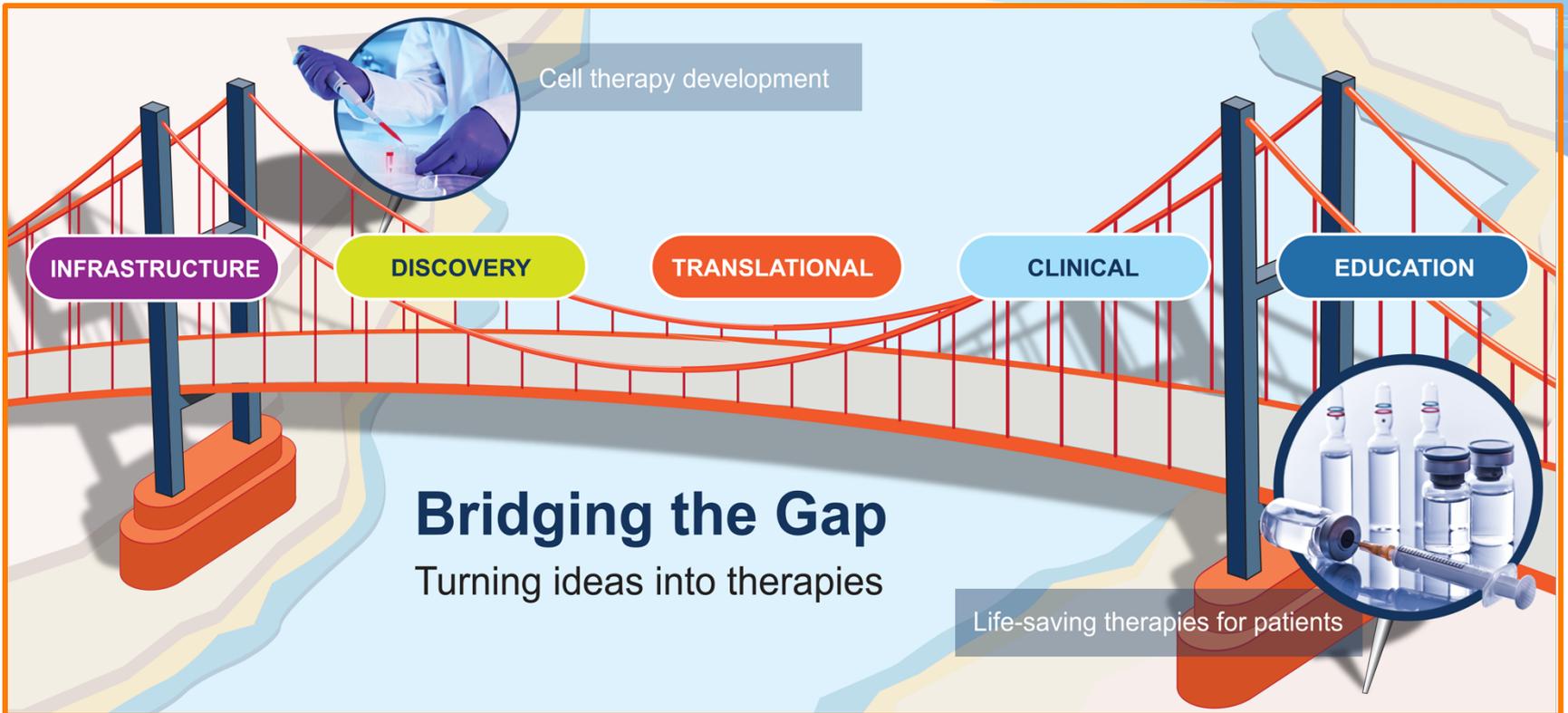


Net Commitments of \$79M YTD



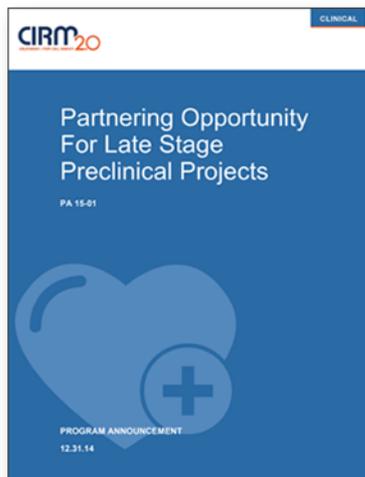
CIRM 2.0 CLINICAL REVIEW

The CIRM Spectrum



Clinical Stage Has Three Programs

CLINICAL STAGE



PA15-01



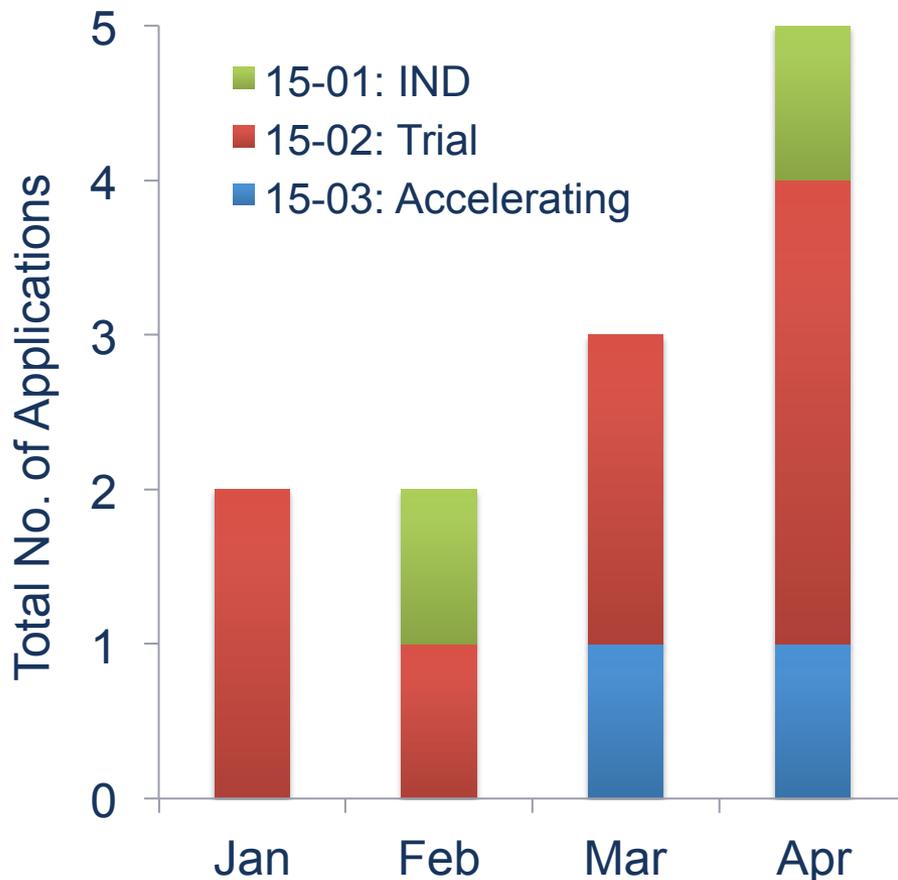
PA15-02



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

Month	Submission	Screening	Budget Review	Sent to GWG	GWG Score	CIRM Rec	ICOC Decision	Contract Signed
	Day 0	Day 7	Day 21	Day 30	Day 60	Day 70	Day 81	Day 120
January	2 15-02	1 Passed 1 Failed	1 Passed	1 Sent	Delayed 1 Cycle	Done	Today	Pending
February	1 15-01 1 15-02	15-01 P 15-02 F	1 Passed	1 Sent	Done	Done	Today	Pending
March	2 15-02 1 15-03	2 15-02 P 1 15-03 F	2 Passed	2 Sent	Pending	Pending	Pending	Pending
April	1 15-01 3 15-02 1 15-03	1 15-01 F 2 15-02 P 1 15-02 FA 1 15-03 P	Pending	Pending	Pending	Pending	Pending	Pending
Within Target	12	12/12	4/4	4/4	1/2	2/2	Pending	Pending

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

CIRM 2.0 For Discovery and Translation

Where We Started

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

Discovery



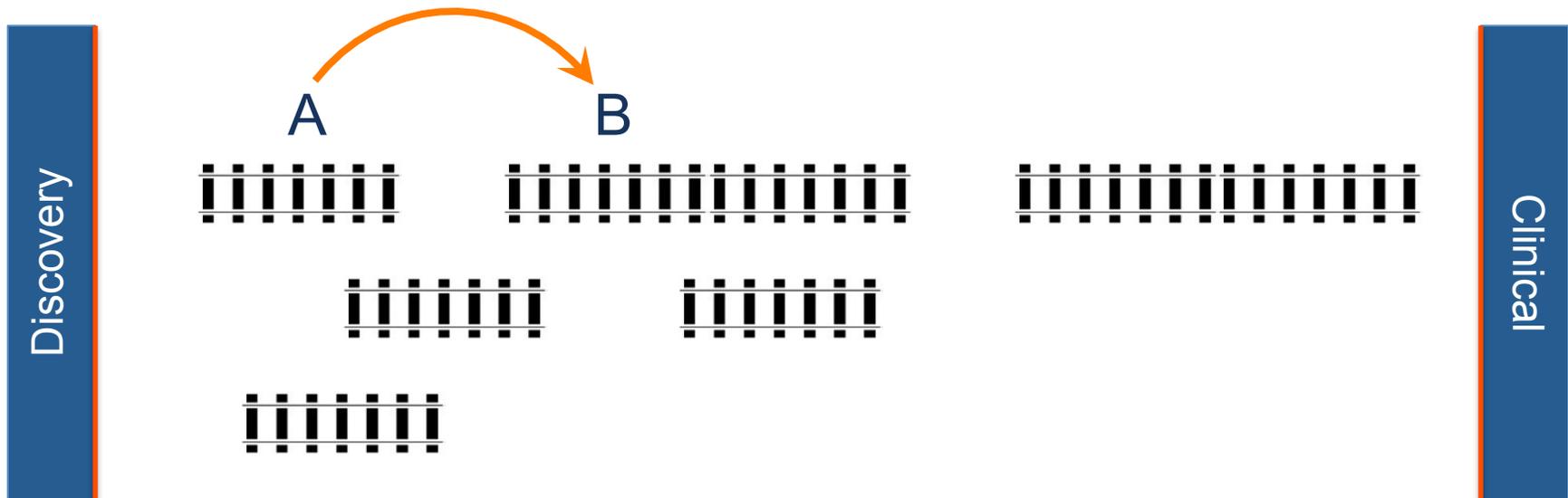
Clinical

1. We Need a Continuous, Predictable Path from Discovery to Clinical



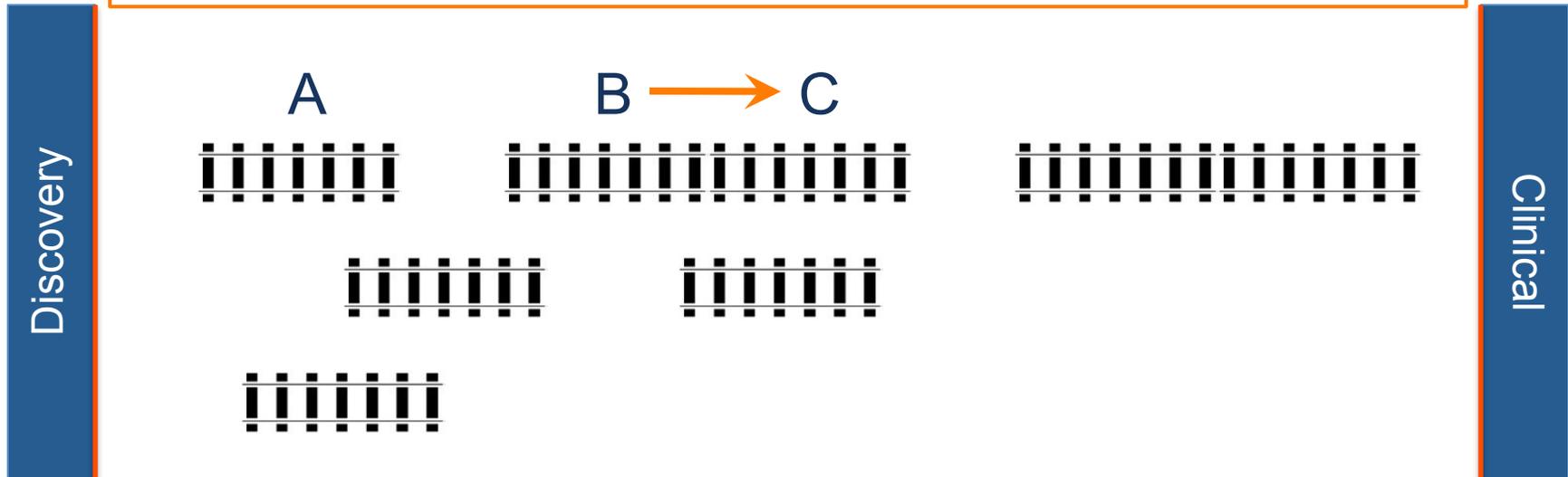
2. Progression Needs to be Possible, Expected, and Incentivized

Funded project “A” spontaneously progresses to “B” only on very rare occasions (5%).



2. Progression Needs to be Possible, Expected, and Incentivized

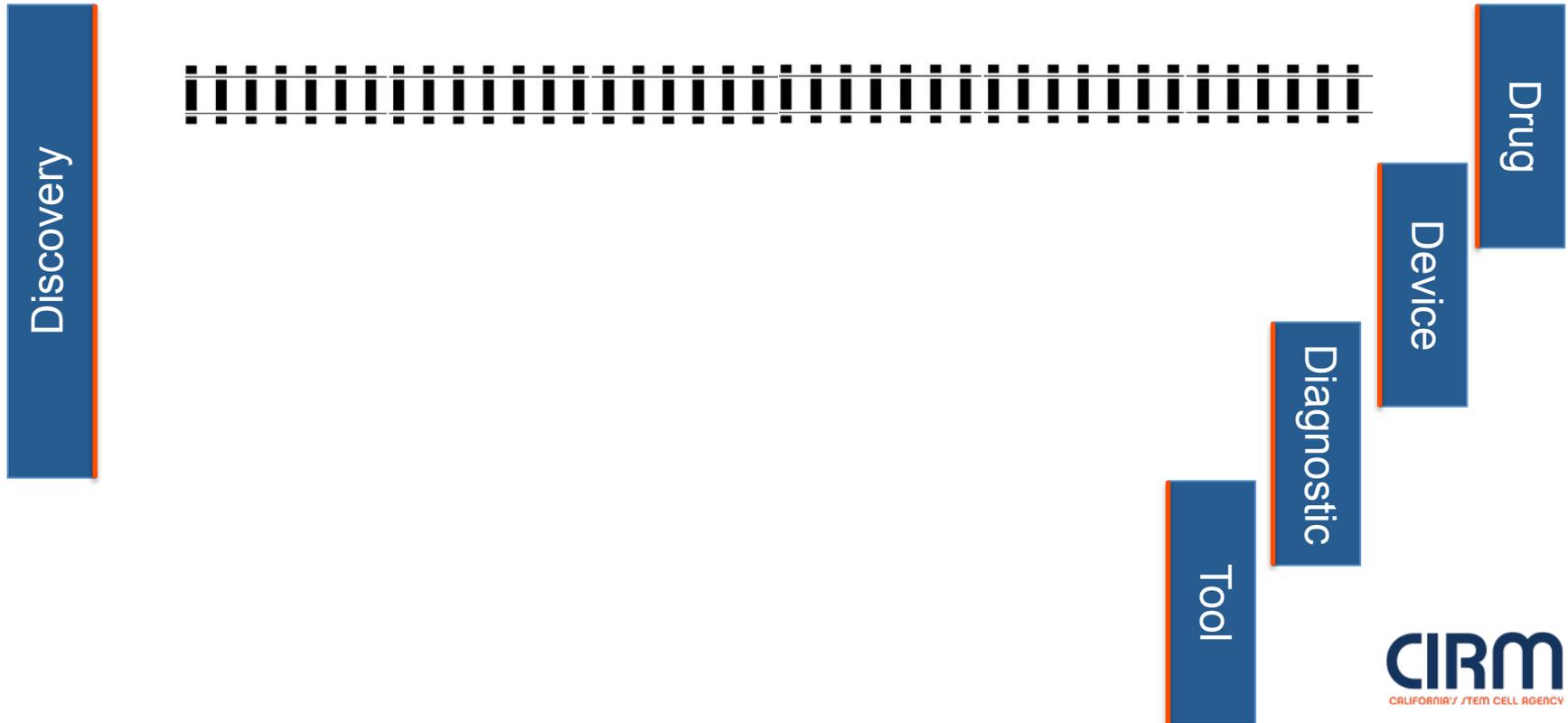
When CIRM issues an RFA with an “intent to progress,” projects advance to the next development stage (B to C) approximately **30% of the time!**



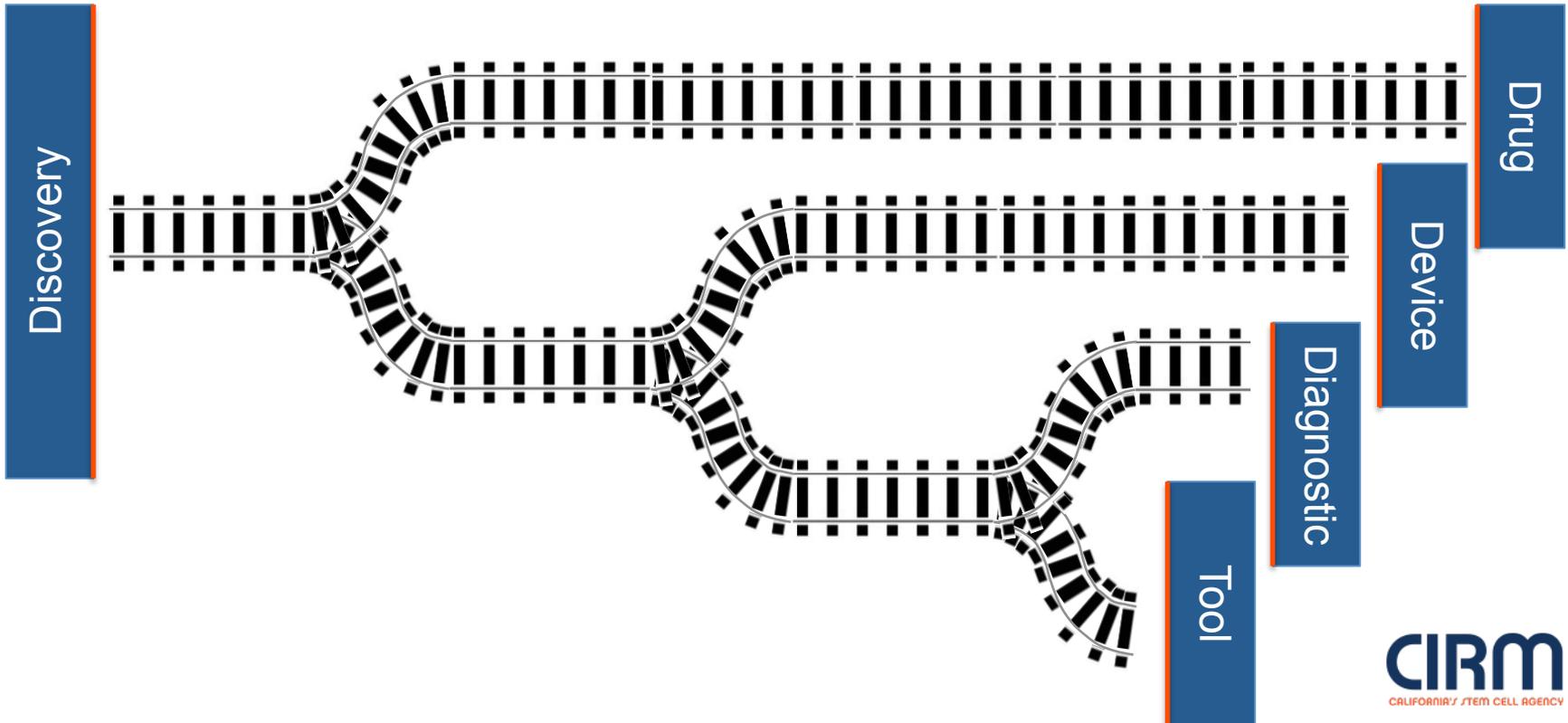
“If you want a bike, don’t ask for a toy.”

Dr. Lila Collins
CIRM Sr. Science Officer

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

SPARK (Creativity) 2.0: A Reimagined Initiative Better Serving CIRM's Mission

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

Bridges 2.0: A Reimagined Initiative Better Serving CIRM's Mission

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**



**A first class
research
experience**

**Regulatory
Training**



**Community
Outreach Activities**



CIRM
CALIFORNIA'S STEM CELL AGENCY

*Bridges Students



THINGS TO COME

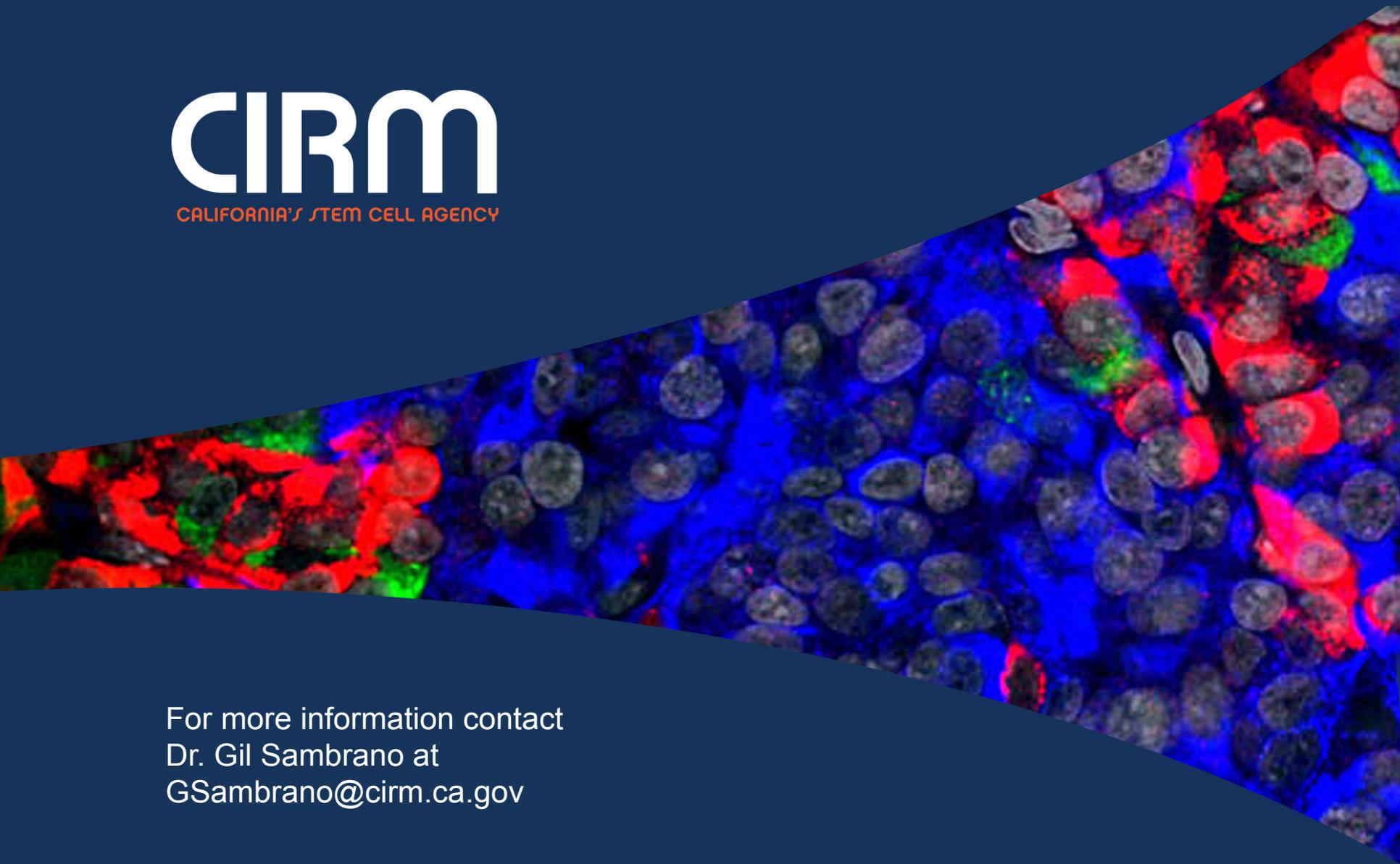
What's Next

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



For more information contact
Dr. Gil Sambrano at
GSambrano@cirm.ca.gov