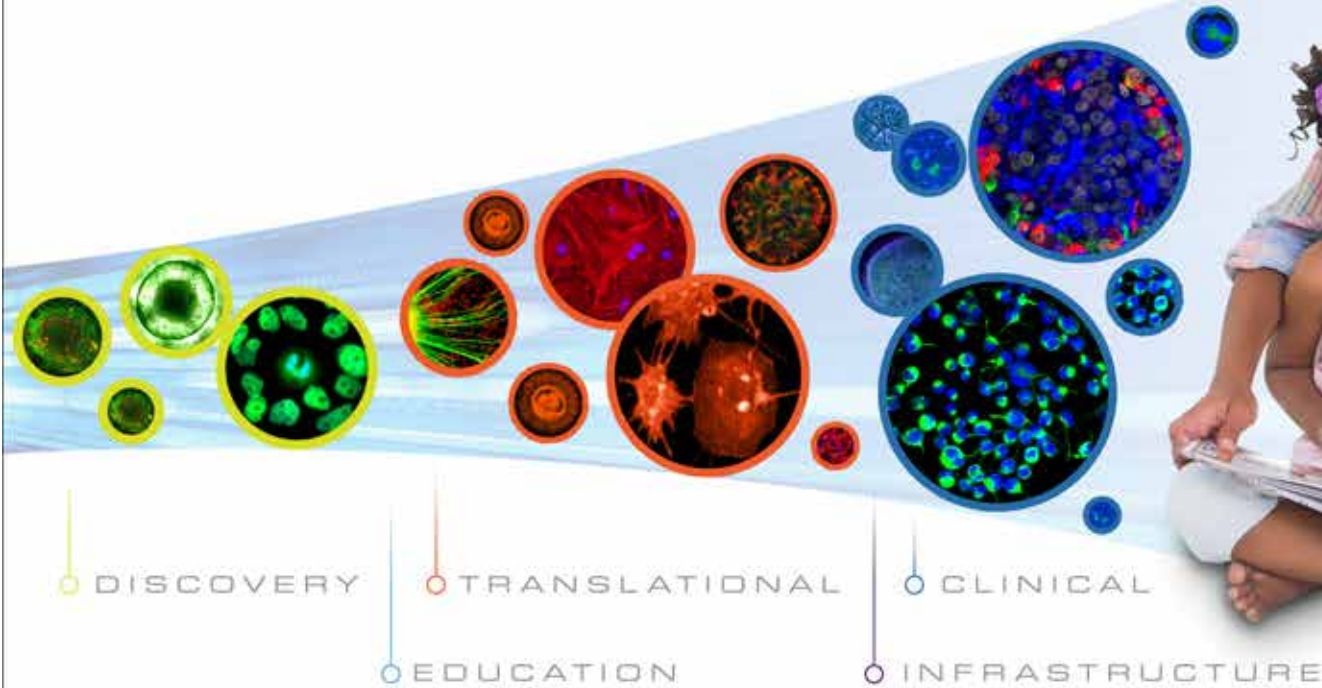


January 21, 2015

CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY



C. Randal Mills, Ph.D.

President and Chief Executive Officer
California Institute for Regenerative Medicine

California Institute for Regenerative Medicine (CIRM)



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EDUCATION
INFRASTRUCTURE

- Created by the people of California to accelerate stem cell treatments to patients with unmet medical needs
- We act with a sense of urgency commensurate with that mission
- Our team of professionals actively partners with academia and industry to fast-track the development of today's most promising technologies
- With \$3 billion in funding and 300 active programs, CIRM is the world's largest institution dedicated to helping people by developing cell therapies

MISSION

Our Mission

Accelerating stem cell treatments to patients with unmet medical needs.

Mission Validation

When taking action, we consider what impact our efforts will have in these areas:



Mission Validation

When taking action, we consider what impact our efforts will have in these areas:

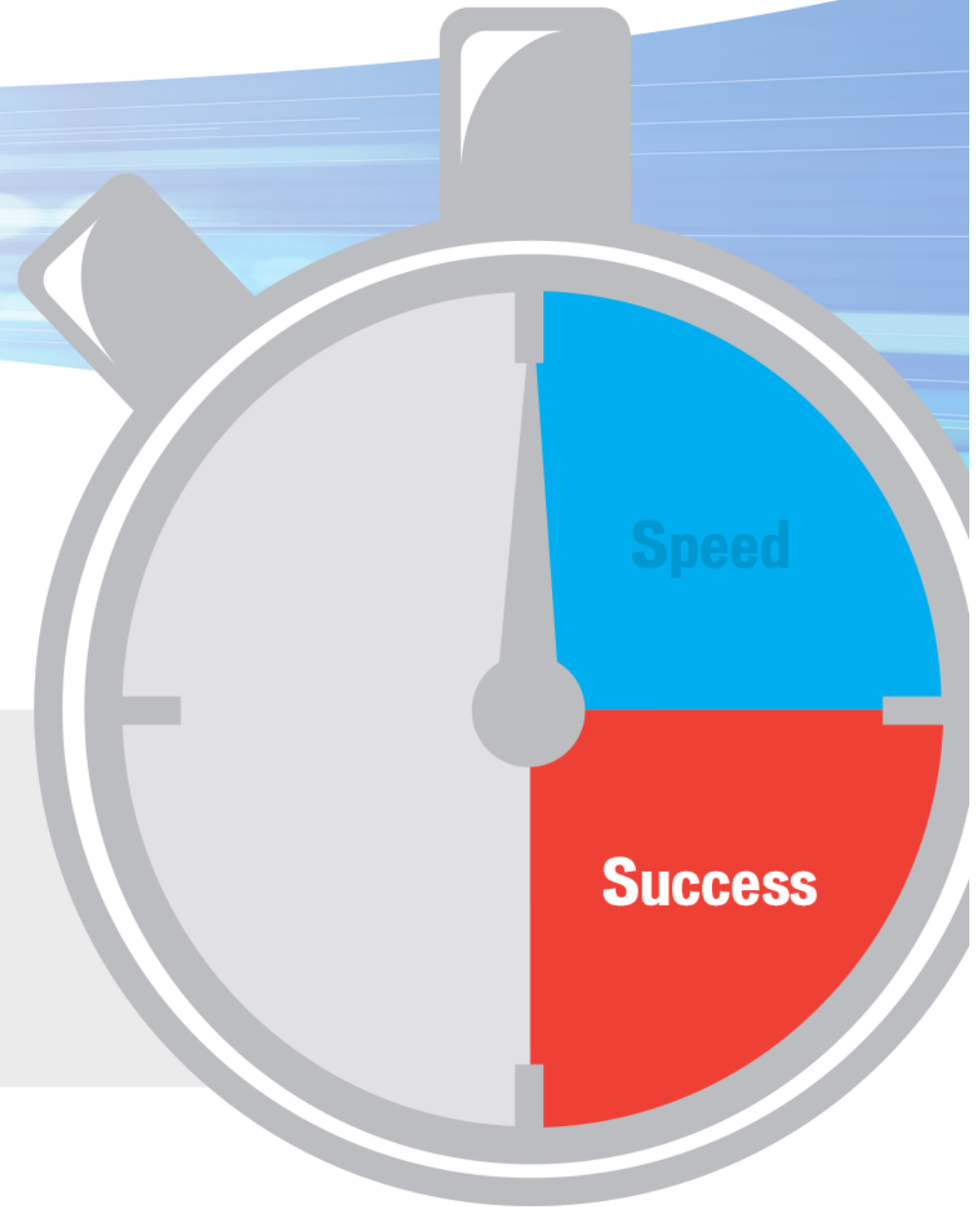
Will it speed up the development of a stem cell treatment?



Mission Validation

When taking action, we consider what impact our efforts will have in these areas:

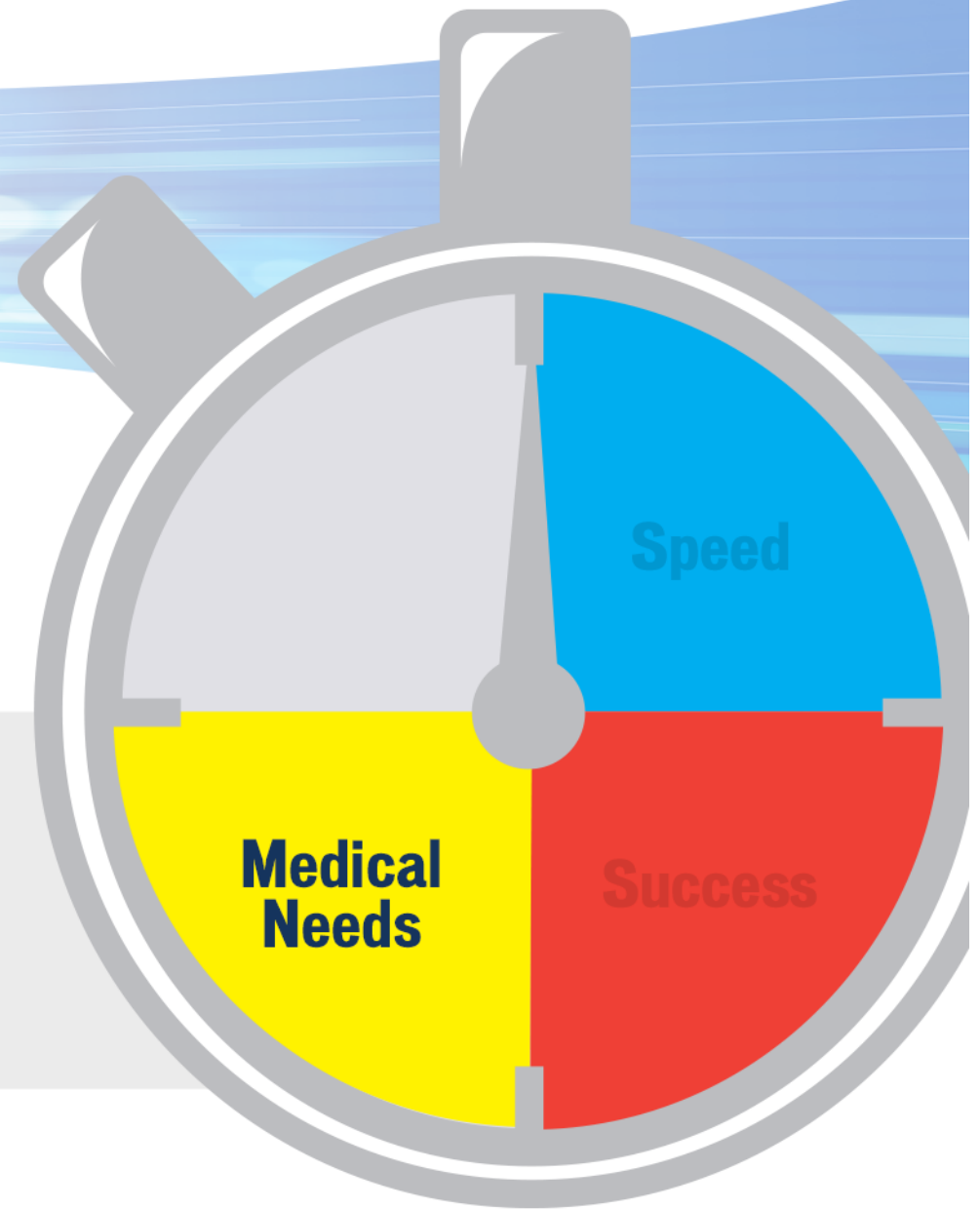
Will it increase the likelihood of success?



Mission Validation

When taking action, we consider what impact our efforts will have in these areas:

Will it fill an unmet medical need?



Mission Validation

When taking action, we consider what impact our efforts will have in these areas:

Is it efficient?



NEW

What is CIRM 2.0



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- A radical overhaul of the way the Institute does business
- Introduces faster, more efficient systems
- Places added emphasis on:
 - ✓ Speed
 - ✓ Partnerships
 - ✓ Patients

CIRM 2.0 Purpose

Applications

Cycle Time

Progression

Clarity

CIRM 2.0 Purpose

Greater Number
of High Quality
Applications

Applications

Cycle Time

Progression

Clarity

CIRM 2.0 Purpose

Greater Number
of High Quality
Applications

Applications

Cycle Time

Progression

Clarity

Reduced
Cycle Time

CIRM 2.0 Purpose

Greater Number
of High Quality
Applications

Faster
Progression of
Funded Projects

Applications

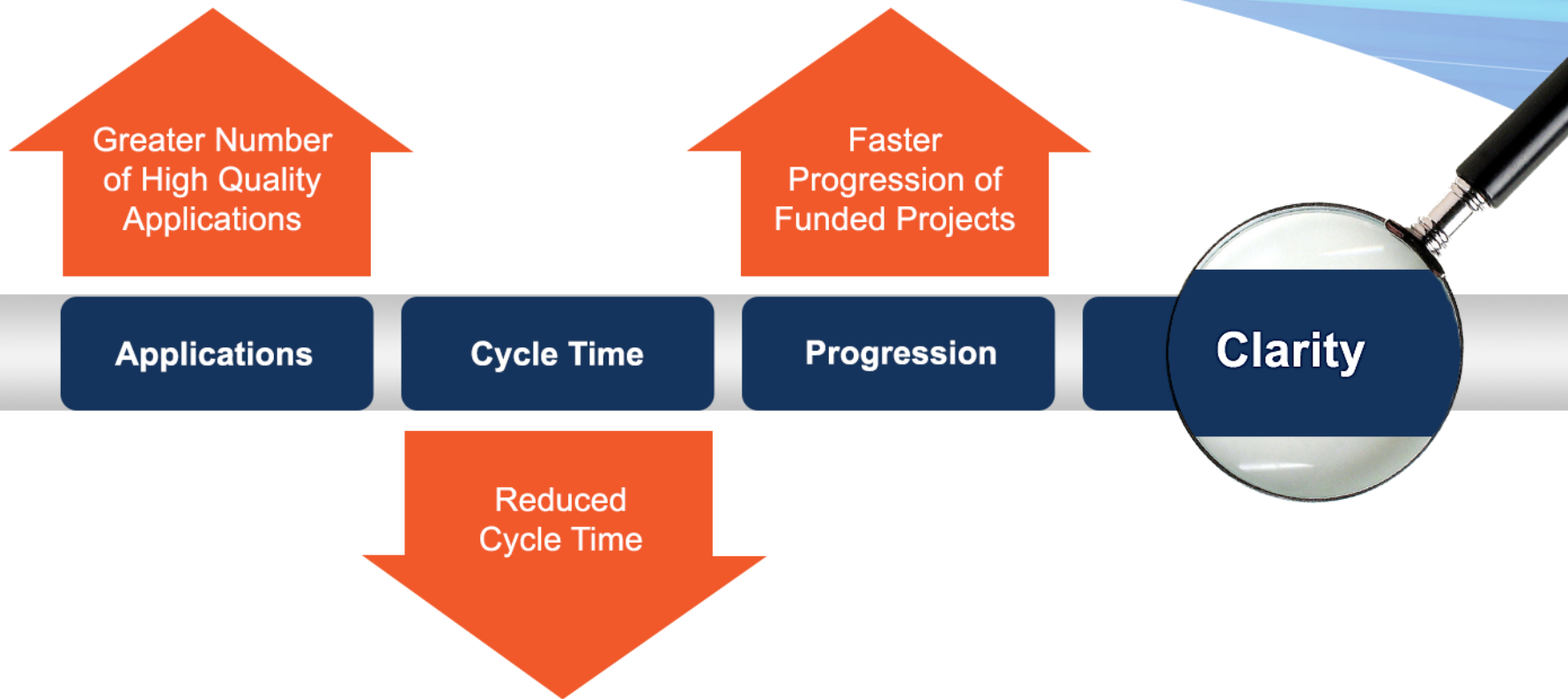
Cycle Time

Progression

Clarity

Reduced
Cycle Time

CIRM 2.0 Purpose



CIRM 2.0 – Starting with Clinical

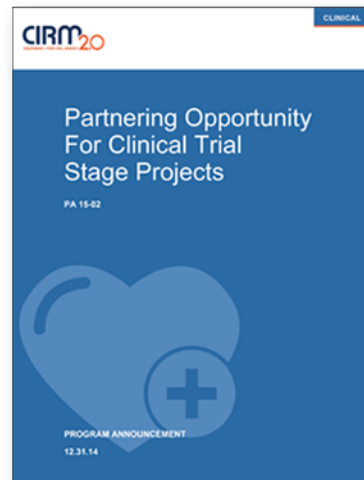


Clinical Stage has Three Programs

CLINICAL STAGE



PA15-01



PA15-02



PA15-03

Therapeutic Candidate Requirements

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Stem Cell Therapies

- ✓ Stem or progenitor cells either compose the therapy or are used in manufacturing

Cord Blood or Unmodified Hematopoietic Stem Cells

- ✓ Being developed as a novel method of addressing a rare or unmet need
- ✓ Unlikely to receive funding from other sources

Small molecules or biologics

- ✓ Acts on endogenous or cancer stem cells as the primary MOA, and
- ✓ Is being developed for a rare or unmet need unlikely to receive funding from other sources

Co-Funding Requirements

Applicant Type	Pre-Clinical	Phase 1	Phase 2	Phase 3*	Supplement
Non-Profit	None	None	40%	50%	40%
For-Profit	20%	30%	40%	50%	40%

**CIRM contribution not to exceed \$20 million*

Contingency Plan



California In-State Requirements

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California Based Organizations

- May use CIRM funds for all eligible project costs
 - ✓ organization must have >50% of its employees located within the state, and
 - ✓ manage the award activities from the California location

Non-California Based Organizations

- CIRM funding can be used only for expenditures incurred within California
- Expenditures subject to the co-funding requirements
- If there is a clinical trial, at least one site must be in California

The Application Process



Under CIRM 2.0 we will accept applications continuously. This removes 9+ months of delay and allows the applicant **to apply when ready.**



The Review Process



The open application cycle in 2.0 leads to smaller numbers of applications per review. As a result, funding decisions can be made in **81 days vs. 6 months.**



The Review Process



Applications undergo a strict **budget review** prior to scientific scoring.

An important feature of CIRM 2.0 is the added option the Board has to recommend an application be **amended** instead of only accepted/rejected.



Contracting Process Improvement



This reduces contracting time by 5 months. In CIRM 2.0, contracting is more standardized and focused on operational milestone delivery.

Applicants must be ready to start within 45 days of approval.



Partnership Process Improvement



Under CIRM 2.0 we will provide more meaningful help to our investigators in real-time and incentivize them to complete their projects in a timely manner.



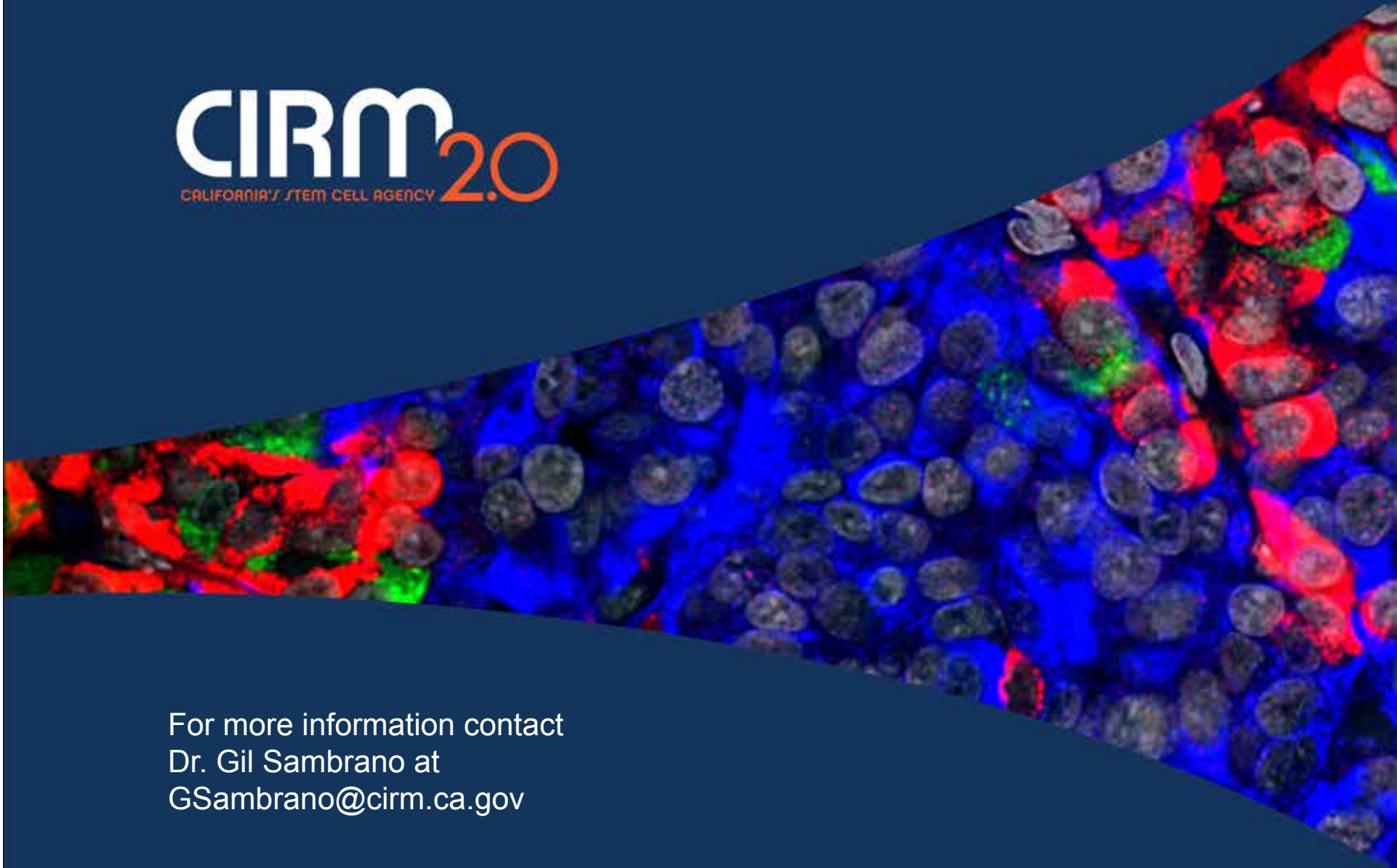
The Result is a Better, Faster Product





Buckle Up!

CIRM2.0
CALIFORNIA / STEM CELL AGENCY



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