California Institute for Regenerative Medicine (CIRM)

- 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
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:

- Efficiency
- Speed
- Medical Needs
- Success

Is it efficient?
What is CIRM 2.0

- 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
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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

- Greater Number of High Quality Applications
- Faster Progression of Funded Projects
- Applications
- Cycle Time
- Progression
- Reduced Cycle Time
- Clarity
CIRM 2.0 – Starting with Clinical

Clinical
Launched January 1st, 2015

2.0

Discovery
Coming in 2015

Translation
Coming in 2015
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
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

<table>
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<tr>
<th>Applicant Type</th>
<th>Pre-Clinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3*</th>
<th>Supplement</th>
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<tr>
<td>Non-Profit</td>
<td>None</td>
<td>None</td>
<td>40%</td>
<td>50%</td>
<td>40%</td>
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<tr>
<td>For-Profit</td>
<td>20%</td>
<td>30%</td>
<td>40%</td>
<td>50%</td>
<td>40%</td>
</tr>
</tbody>
</table>

*CIRM contribution not to exceed $20 million
Contingency Plan
California In-State Requirements

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 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**.
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

APPLICATION
9-15 months
OPEN

REVIEW
5-7 months
81 days

CONTRACTING
5-7 months
45 days

ADMINISTRATION
4 years
ASAP

2.0
Reduces Cycle Time
22 Months > 120 Days
For more information contact Dr. Gil Sambrano at GSambrano@cirm.ca.gov