



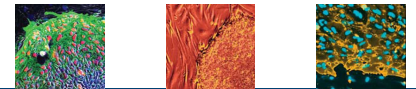
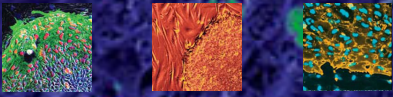
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

# Proposed Concept Plan

Accelerating the Development of Stem Cell Treatments  
**Clinical Stage Programs**

**“CIRM 2.0”**

December 11, 2014



# CIRM 2.0: Purpose

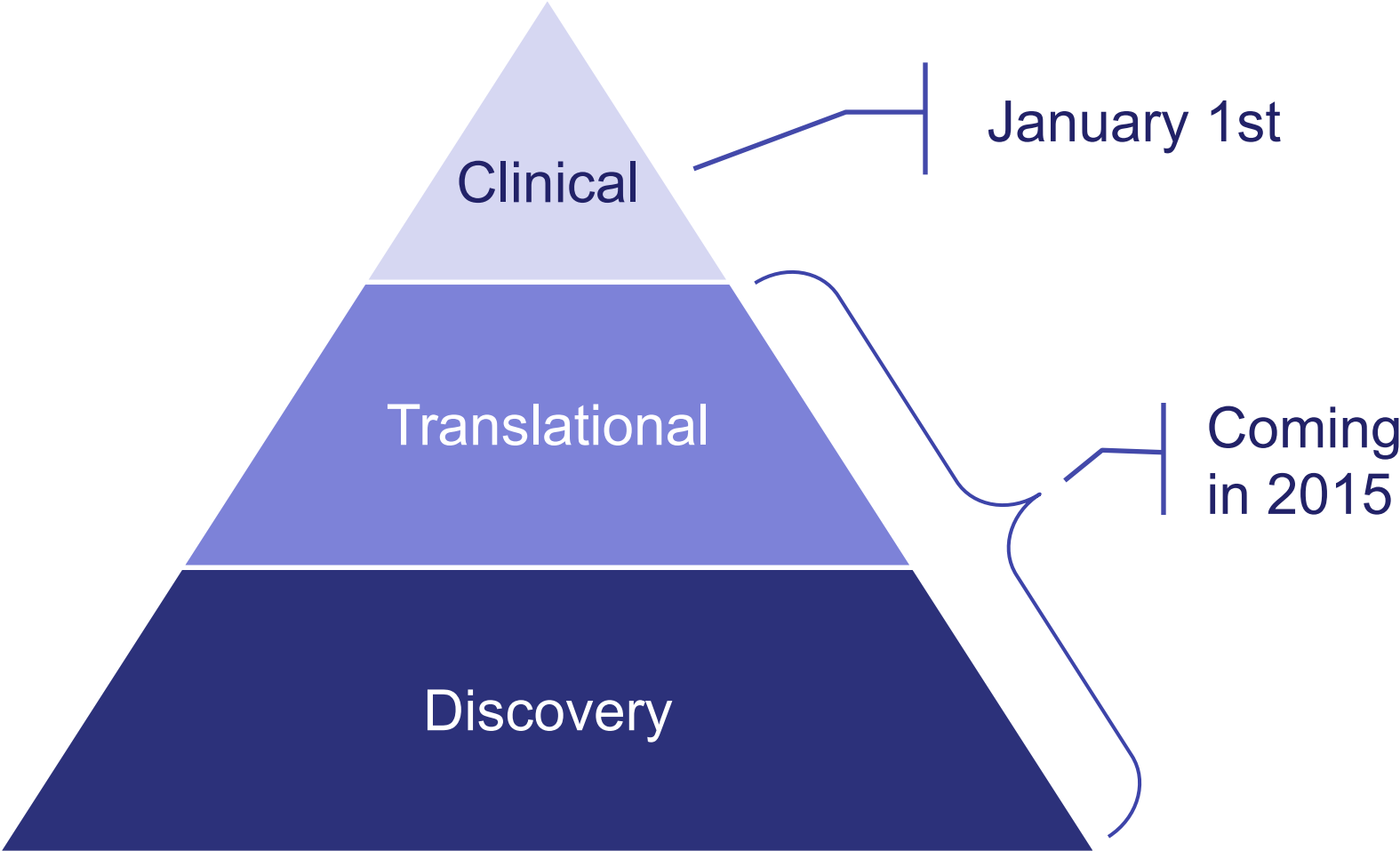
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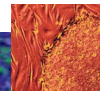
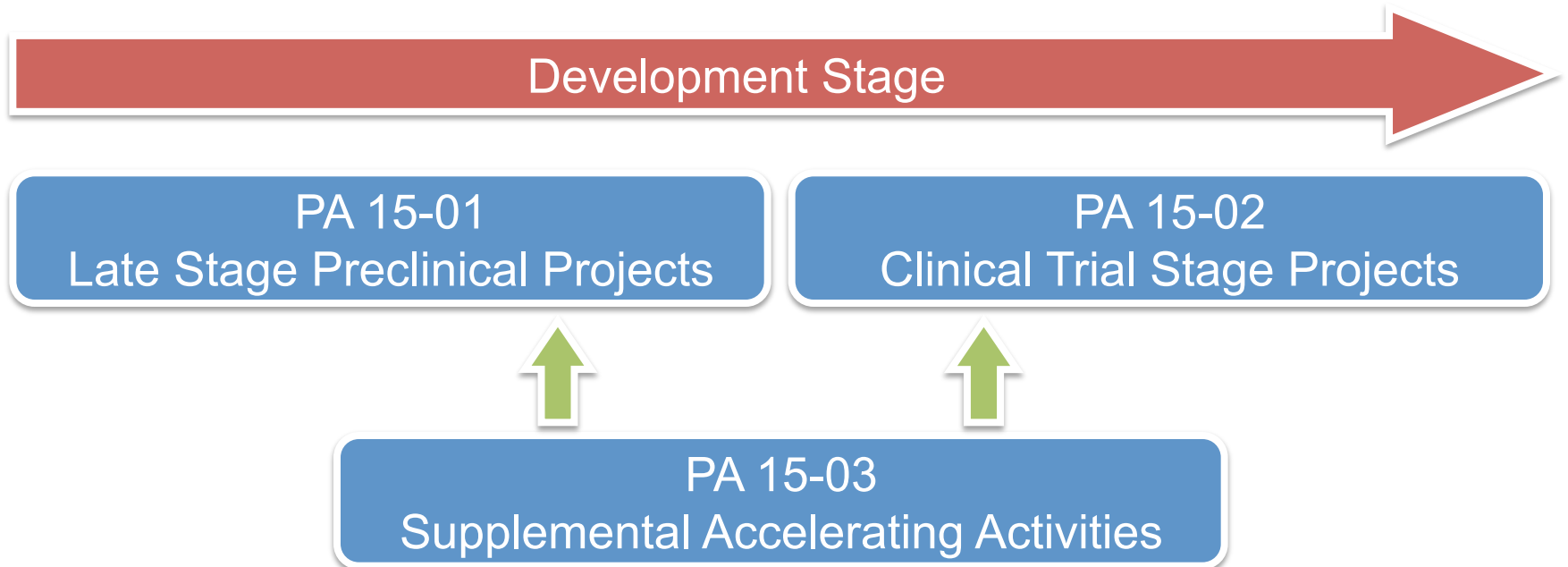
*To create a process at CIRM for attracting,  
awarding and administering projects that better  
serves our mission*



# CIRM 2.0 – Starting with Clinical



# Clinical Stage has Three Programs

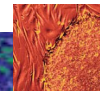


# General Eligibility Requirements

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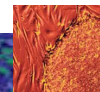


- Must be ready to initiate work within 45 days of approval
- Must propose studies with a single stem cell-based therapeutic candidate\*
- Must demonstrate appropriate stage of readiness
- Must include a project manager
- Co-funding requirements\*
- For-profit organizations must demonstrate solvency
- For PA 15-03: Must supplement an active, clinical stage, CIRM-funded project (PA 15-01, PA 15-02, or equivalent)



# Single Stem Cell-based Therapeutic Candidate Requirement

- A cell therapy where stem or progenitor cells either comprise the therapy or are used to manufacture the therapy.
- Cord blood or unmodified hematopoietic stem cells
  - only if being developed as a novel method of addressing a rare or unmet need
  - unlikely to receive funding from other sources.
- A small molecule or biologic
  - 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

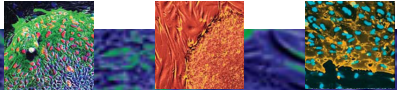


# Co-Funding Requirements



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



# Key California Requirements

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## California Research Organizations

- California organizations may use CIRM funds for all eligible project costs
- To qualify as a California organization
  - organization must have >50% of its employees located within the state, and
  - conduct the award activities from the California location

## Non-California Research Organizations

- Non-California organizations may also apply
- CIRM funding can be used only for allowable expenditures incurred within California
- Allowable expenditures are subject to the co-funding requirements
- If there is a clinical trial, at least one site must be in California





# California Requirement Example

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## Request for Phase 3 Clinical Trial Funding

10 sites total (\$10M in total trial cost)

5 sites in California (\$5 M in cost)

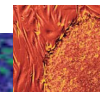
5 sites outside of California (\$5M in cost)

### California Organization

- 50% of the total program cost
- 50% x \$10M
- **Funding Opportunity = \$5M**

### Non-California Organization

- 50% of cost within California
- 50% x \$5M
- **Funding Opportunity = \$2.5M**

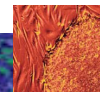


# Requested Funding Allocation

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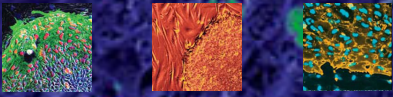


- \$50 million allocated for the remainder of FY2015
- Indirect cost rate set at 10%
- No preset cap on funding individual projects (except for Phase 3 programs)



**QUESTIONS?**





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**Thank you!**

**C. Randal Mills, Ph.D.**

President and Chief Executive Officer  
California Institute for Regenerative Medicine