



# Funding CIIRM Translation: Public/Private Partnership Recommendations

December 11<sup>th</sup>, 2013  
Agenda Item #6D

James Gollub Associates



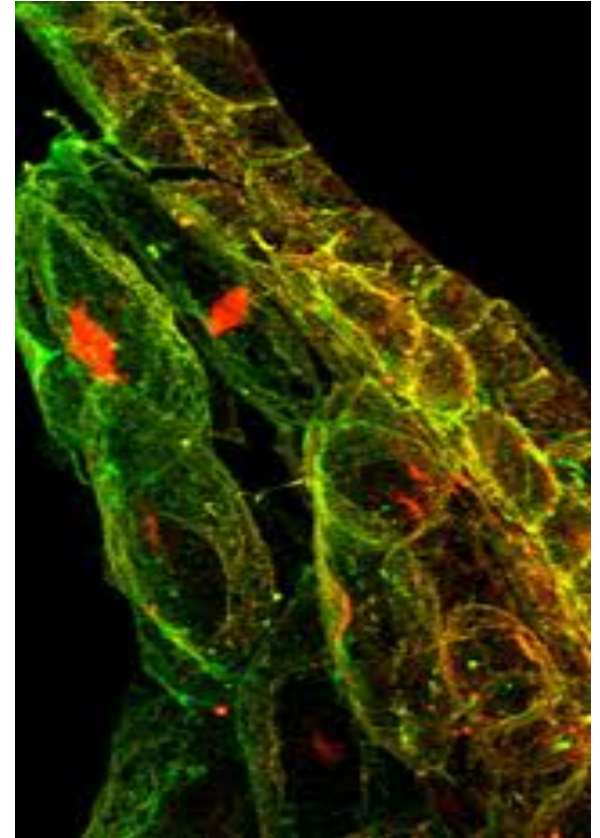
# President's Goal 2 for 2012-14

## October ICOC Meeting

Agenda Item #6D  
ICOC Board Meeting  
December 11-12, 2013

### Ensure CIRM Project Portfolio Progress

“Explore a preferred mechanism to cost effectively assist early stage stem cell research projects to mature enough so that they are investment ready”



# **Core CIRM Objective: Secure Funding to Carry Forward ‘Worthy’ Translational Development Projects from Portfolio**

**90 Projects  
\$661.4M Investment**



Blood Diseases	6 projects: \$35.7M	Neurological Diseases	26 projects: \$161.0M
Bone Diseases	4 projects: \$32.4M	Heart Disease	10 projects: \$73.6M
Cancer	10 projects: \$117.5M	Liver Disease	4 projects: \$10.3M
Cartilage Disorders	3 projects: \$11.6M	Skin Disease	1 project: \$11.7M
Diabetes	3 projects: \$34.5M	Immune Diseases	4 projects: \$33.0M
Eye Diseases	7 projects: \$54.5M	Genetic Disease	1 project: \$5.5M
HIV/AIDS	4 projects: \$43.0M	Vascular Disease/Multi	2 projects: \$20.0M
Muscle Diseases	5 projects: \$17.1M		

# Fundamental CIRM Challenges

## Fund and Develop Projects to Investment Readiness

### Early Translational Projects

- Fund Current & New Pre-Clinical Trials Projects
- Manage Projects to Milestones
- Ensure Replicable Lab Research
- Screen Candidates Against pre-IND Criteria for Next Stage

### Disease Team Projects

- Screen & Prioritize Strongest Projects
- Fund Priority IND to Clinical Trials Projects
- Manage to Product Development Milestones
- Introduce to Biopharmaceutical Partners
- Achieve License or Launch Start-up

**From the CIRM Innovation Pipeline Portfolio**

# Background Models

## See CIRM Website:

Agenda Item #6D  
ICOC Board Meeting  
December 11-12, 2013

**<http://www.cirm.ca.gov/about-cirm/cirm-publications>**

- “Investing in Translational Research to Produce Clinical, Commercial and Financial Outcomes: Current and Future mechanisms”
- **Prepared for CIRM by:**
  - Steve Dickman and CBT Advisors
  - Cambridge, MA, March 29, 2013



# Selection of a Model

James Gollub Associates

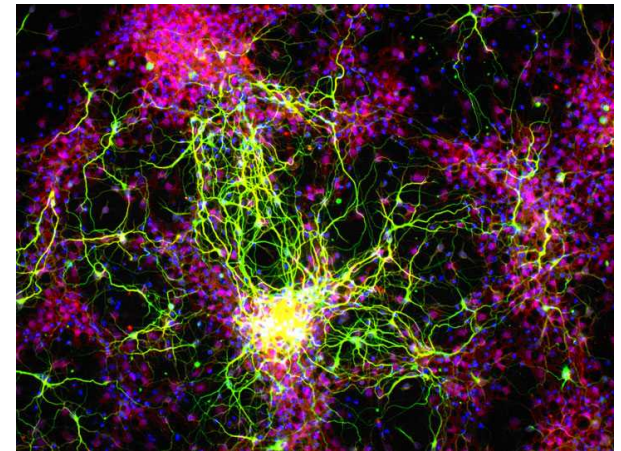


- **RFP for Consultant**
- **Selected James Gollub Associates**
- **Worked Closely with CRIM Steering Committee**
- **Completed Four Steps**
  - SWOT Review: Appraisal of Needs
  - Model Analysis: Alternative Approaches
  - Stakeholder Review: Decision-maker view
  - Three Recommendations

# Strategic Roadmap for Innovation Mission

Agenda Item #6D  
COC Board Meeting  
December 12, 2013

- **Focus: Vetted Options**
  - Provide President of CIRM with recommendations for attracting private sector investment to and further facilitate development of CIRM's translational research portfolio
- **Target: Models for Advancing Innovation to Market**
  - Consider CIRM status
  - Range of options
  - Most feasible & attractive to partners
  - Lay out staged implementation



# **Recommended Model**

## Three Strategic Actions Proposed to Expand CIRM Research and Enhance Pre-Commercial Readiness

1. Public-Private Pre-Clinical & Clinical Trials Fund
2. CIRM Accelerator
3. Pre-Competitive R&D Program

# **Public-Private Pre-Clinical & Clinical Trials Fund**

- **Challenge: Fund**
- Secure Funding to Advance Projects from Pre-IND Thru Ph1 & 2
- **Action: Partnership**
- Build New Collaborative Public-Private Fund to Fill Pipeline
- **Outcome: Progress**
- Projects Advance to 2a,b License or Start-up

# Public/Private Pre- & Clinical Trials Fund

## ■ Step 1. Plan

- Reach out to disease foundations, family foundations, major pharmaceuticals to collaboratively plan a public-private RM-focused pre-clinical and clinical trials fund with CIRM offering knowledge, experience, projects and co-funding leverage

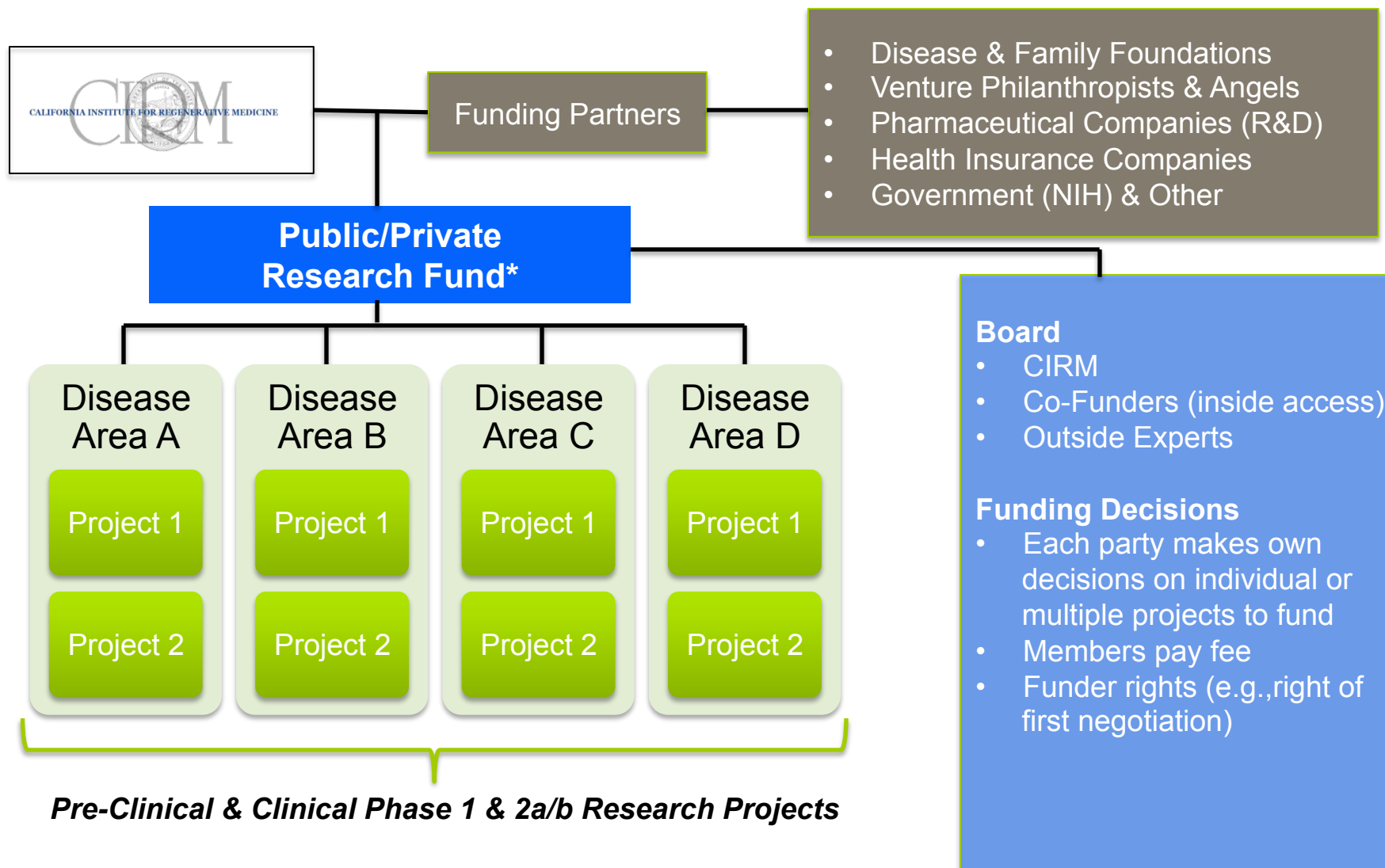
## ■ Step 2. Pilot

- CIRM organizes committed public-private partners to fund projects presented by CIRM by disease theme with no formal intermediary (analogous to Strategic Partnerships)

## ■ Step 3. Scale-up

- Foundation (501c3) holds and manage disease-theme funding commitments with funders

# 1. Public/Private Pre- & Clinical Trials Fund: Organization



\*Administration of fund(s) to be determined. See next slide.

# Recommendation 1: Structure Public/Private Pre- & Clinical Trials Fund

<i>Objectives</i>	<ul style="list-style-type: none"> <li>• <b>New Funding:</b> Raise non-taxpayer funds from diverse sources to leverage and expand CIRM project development from pre-clinical through clinical trials.</li> <li>• <b>Near-term:</b> Increase number of IND projects by disease theme that reach phase 2a/b.</li> <li>• <b>Longer-term:</b> Potential to extend CIRM's lifetime and increase prospects for CIRM sponsored research to become therapies through new venture or biopharma relationships</li> </ul>
<i>Develop</i>	<ul style="list-style-type: none"> <li>• <b>Plan:</b> Develop an operational business plan to engage partners.</li> <li>• <b>Pilot Phase:</b> CIRM selects 5-10 projects with CIRM contribution of <b>\$50M+ to attract \$50M+ of external funds</b> (&lt; 12 months).</li> </ul>
<i>Scale-up</i>	<ul style="list-style-type: none"> <li>• <b>Organize:</b> CIRM convenes pharmaceutical firms and foundations around targets for co-funding to test public-private model.</li> <li>• <b>Formalize:</b> Possible creation of a 501c3 Foundation by third parties</li> </ul>
<i>Operating Funding</i>	<ul style="list-style-type: none"> <li>• <b>Open:</b> CIRM manages development of new public-private RM research pool until pool administration can self fund (minimal), with CIRM managing award of grants to projects.</li> </ul>

# Public/Private Pre- & Clinical Trials Fund: Stakeholder Quotes

## ■ Importance

- “This is an important direction for CIRM to take and to expand...” – Industry
- “A multi-pharmaceutical fund with disease-focused foundations focusing on CIRM projects within specific disease themes makes sense.” – Industry

## ■ Readiness to Participate

- “We are eager to explore how we can participate in co-funding projects” – Industry
- “Most foundations will be interested in how CIRM funds can leverage foundation dollars.” - Foundation
- “We believe in collaborative funding of research” – Foundation

## ■ Terms

- “We only ask for repayment of grants from financial windfalls. There is, however, a trend in foundations seeking some return.” – Foundation
- “Primarily interested in accessing innovation, possibly with right of first negotiation” – Industry

# Critical Resources for Building This Fund

- **Key Need: Champion**
  - Needs champion with experience, scientific knowledge and high level business contacts and acumen to attract potential funding partners – major Biotech and Pharmaceuticals, Foundations, and Angel Investors.
- **Harness Internal Support**
  - CIRM Business Development Unit – Elona Baum and team
  - CIRM Science Office – Ellen Feigal, Pat Olson and team
  - Patient Advocates – Key stakeholders
- **New President**
  - Recruitment need to enhance ability to reach potential co-funding partners
- **Chair and Members of the Board**
  - Crucial role in building partnership links
- **Business Community Advisors and Network Associates**
  - Leaders who are willing to co-champion CIRM mission

# Public-Private R&D Partnership Examples

- **Snycona (£200M+)**
  - Welcome Trust
- **Israel Life Sciences Fund (\$222M)**
  - Government takes LP with venture funds having favored position
- **Massachusetts Life Science Center (\$467M)**
  - Leveraged \$1.2B from corporations, foundations, institutes, NIH, and universities matching funds
- **Roche Collaborative Start-ups (NA)**
  - Roche, QB3, Mission Bay Capital with seed stage focus
- **Atlas/Novartis/Amgen (\$265M)**
  - Private syndicated funding of translational research-based start-ups at key university centers (not true public-private)

# CIRM Accelerator

## ■ Step 1. Plan

- CIRM provides integrated set of centralized services and teams of selected external experts to guide each CIRM project step-by-step to achieve commercial readiness

## ■ Step 2. Pilot

- CIRM organizes and internally funds first demonstration of the CIRM Accelerator focusing on the six-to-eight SAB recommended priority projects

## ■ Step 3. Scale-up

- CIRM formalizes Accelerator Program to encompass multiple teams, possibly financed by the public-private partnership, or through private accelerator firms and early-stage venture investors, including pharmaceutical corporate venture funds, who will bring projects to readiness for licenses or new ventures

# Pre-Commercial Readiness Accelerator

- **Challenge: Fund**

- Secure Funding to Advance Projects from Pre-IND  
Thru Ph1 & 2

- **Action: Guidance**

- Provide Direct Services to Every Project Thru  
Each Milestone

- **Outcome: Product Development**

- Attract Further Funding, Licensing & Investment

# How to Achieve Acceleration Goal?

## Leverage Resources in Three Ways

### 1. Internally

- Prepare and de-risk candidate therapies for commercial adoption

- Deliver centralized CIRM capacities, expand CDAP model, and utilize future Alpha Clinics

### 2. Locally

- Link CIRM funded projects to accessible business, technical and laboratory services

- Collaborate with non-profit accelerators across universities in California

### 3. Nationally

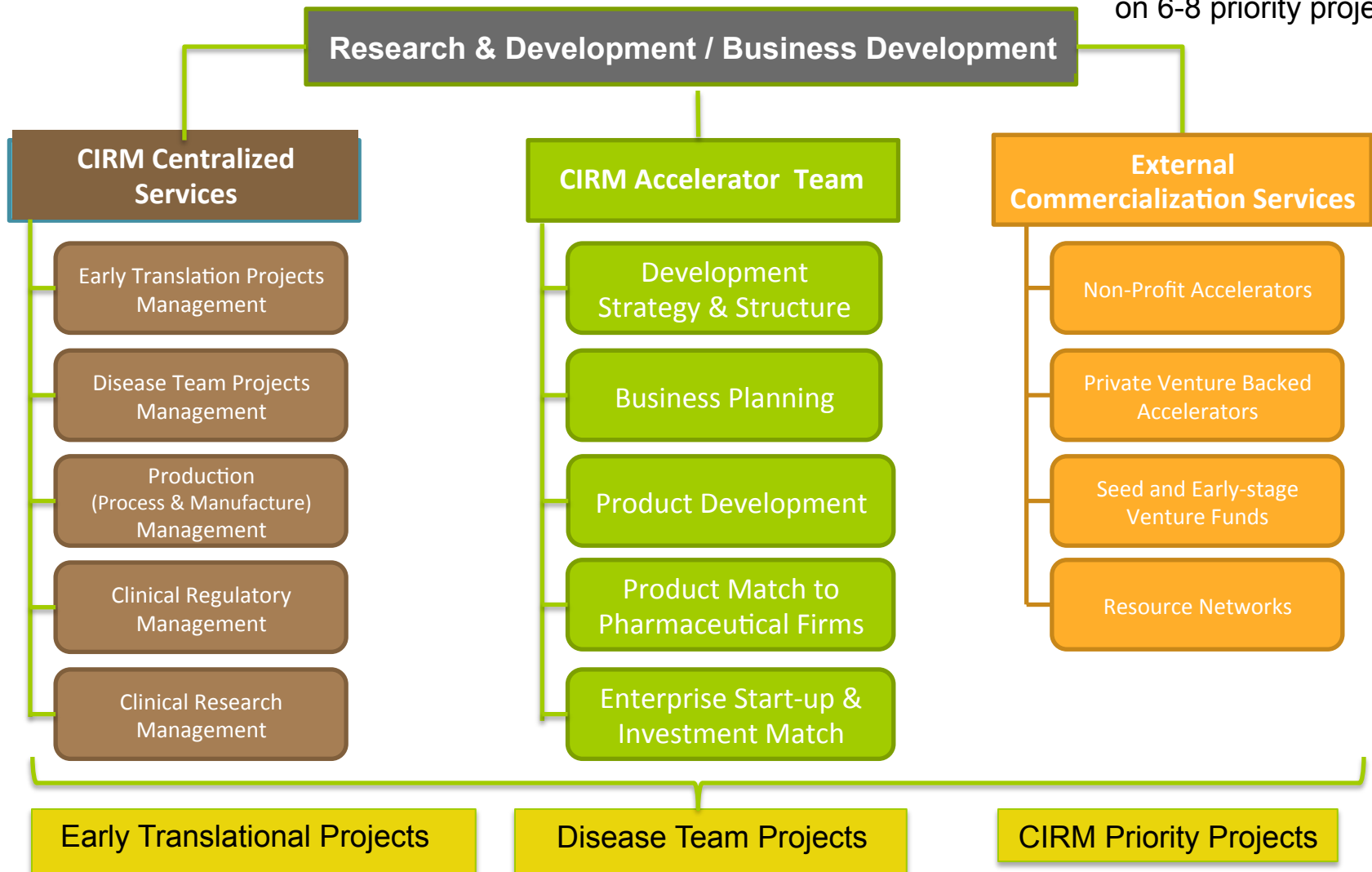
- Engage expertise & services to build project readiness, arrange license and collaboration agreement with a biopharmaceutical partner

- Partner with venture backed accelerators and early-stage venture capital firms

# CIRM Accelerator: Organization



**Note:** Pilot could focus new Accelerator team on 6-8 priority projects



# CIRM Accelerator: Stakeholder Quotes

## ■ Importance

- “This is a fundamental need.” – Industry
- “There is a need to jump over the valley of death.” – Industry
- “CIRM needs to pick and focus on successes to sustain operations.” – Foundation

## ■ Feasibility

- “Creating a pool of funds for de-risked RM projects will *then* make sense to investors – Industry
- “Our foundation does this now, providing readiness services, there are steps” – Foundation

## ■ Readiness to Participate

- “Company and corporate venture funds may consider participating.” – Industry
- “Ready to explore this with CIRM.” – Foundation
- “We do this now and are ready to explore this with CIRM.” – Foundation

## ■ Terms

- “If our corporate venture fund participates would expect conventional terms.” – Industry
- “Open to many agreements.” – Foundation
- “Access to non-dilutive funds for projects can attract participation.”

# **CIRM Pre-Competitive R&D Program**

## ■ **Plan**

- CIRM builds on its history of previous work with universities and industry to define and fund R&D on challenges that are barriers to delivery of benefits for patients—from tools (biomarkers) to finance (reimbursement models)

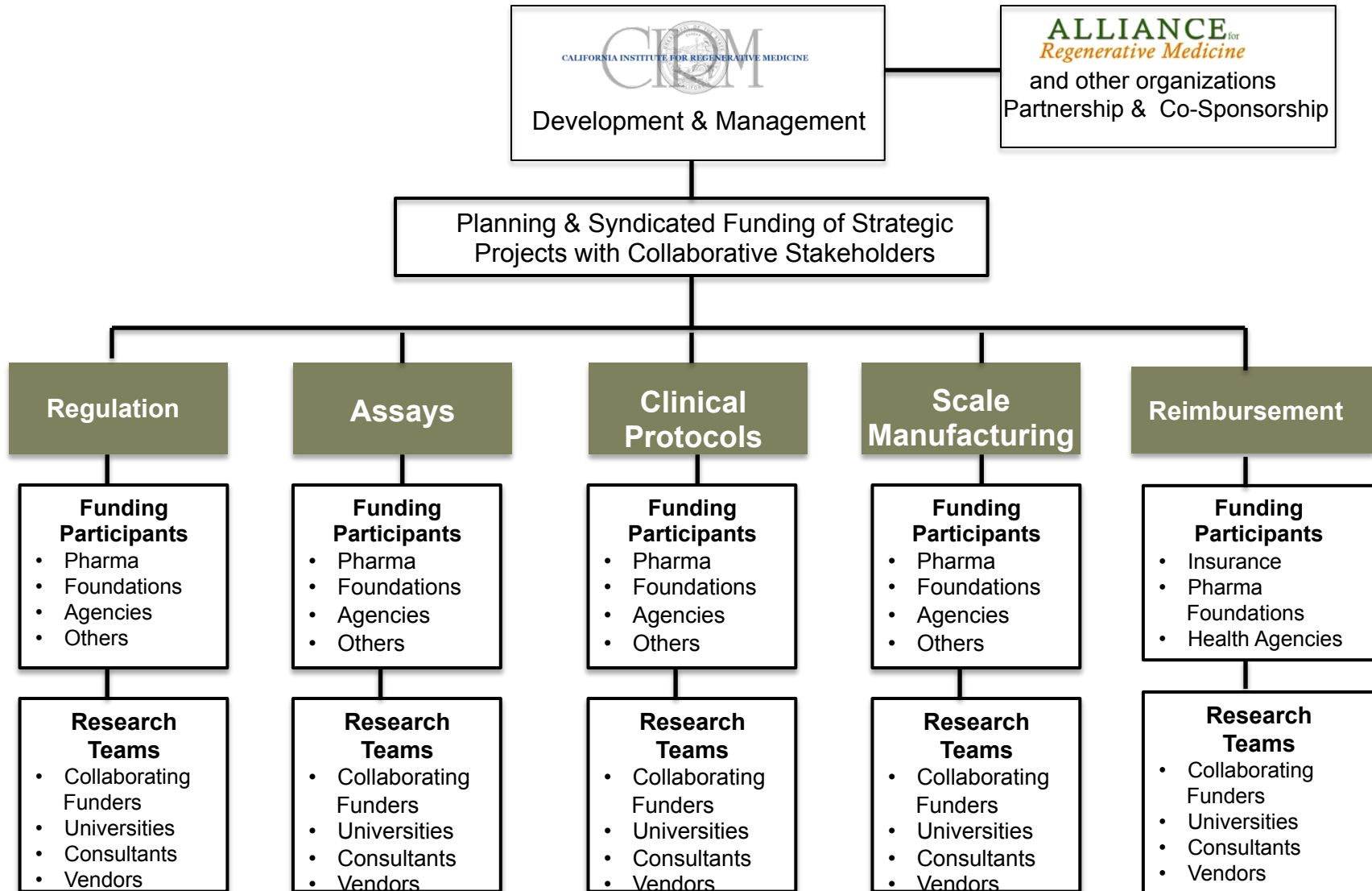
## ■ **Pilot**

- CIRM organizes, develops, matches funds (<25%) and manages one or more nationally syndicated collaborative R&D projects with pharmaceutical, health insurance, and government, with RFA to university and private providers to deliver analysis and share results

## ■ **Scale-up**

- CIRM continues and expands self-financed pre-competitive R&D program, building strong relations with public and private stakeholders, growing CIRM visibility and advancing diffusion of solutions

# Pre-Competitive R&D Program: Organization



# Pre-Competitive R&D Program Stakeholder Quotes

## ■ Importance

- “Very important. Very concerned first with improving management of research and clinical trials so that the right data is ready for the pharmaceutical industry early on.” – Industry.
- “Yes, pre-competitive R&D is important and our firm believes in collaborative process AND in thinking about the broader process from start to patient and its implications.” – Industry.

## ■ Feasibility

- “Our company believes in and actively promotes collaborative projects.” – Industry
- “The foundation believes that there are pre-competitive developments that could be done around very core issues, such as quality assurance, standards and manufacturing.” – Foundation

## ■ Readiness to Participate

- “We are ready to explore specific collaborative projects on pre-competitive issues.” – Industry
- “This foundation has been active and participates in a collaborative projects with 14 partners, primarily pharmaceutical companies.” – Foundation
- “This foundation just had a three-day consortium meeting on spinal cord issues.” – Foundation

## ■ Terms

- “There is a need for “stewardship” that perhaps CIRM can formally play.” – Industry
- “No specific terms, other than first right of negotiation on IP arising from pre-competitive projects.” – Industry

# Summary

## ■ Choices

- Each of the three key recommended actions for achieving continued CIRM operation and innovation has value

## ■ Approach

- Actions proposed leverage existing CIRM capabilities, expertise, relationships networks, projects and funding and can easily be integrated

## ■ Implement

- Proposed pilots will deliver 'proof of concept' and scalable results

## ■ Outlook

- CIRM continues to serve as a catalytic engine for the emerging field of RM
- Levers public-private resources to grow critical mass of RM development
- Moves innovation to clinics, patients and enterprise in California and globally

# Next Steps

- Preparation of Operational Plan – 3-4 months
  - Prepare an operational plan and concepts for any funding requests which will be presented to the ICOC
  - Prepare a brief memorandum to present to potential funders
- Execution of Operational Plan – 12 months
  - Convene group and individual meetings with biopharmas, foundations, VCs, donors and other potential funders