Funding CIRM Translation: Public/Private Partnership Recommendations

December 11th, 2013
Agenda Item #6D
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

<table>
<thead>
<tr>
<th>Disease Type</th>
<th>Projects</th>
<th>Investment</th>
</tr>
</thead>
<tbody>
<tr>
<td>Blood Diseases</td>
<td>6</td>
<td>$35.7M</td>
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<tr>
<td>Bone Diseases</td>
<td>4</td>
<td>$32.4M</td>
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<tr>
<td>Cancer</td>
<td>10</td>
<td>$117.5M</td>
</tr>
<tr>
<td>Cartilage Disorders</td>
<td>3</td>
<td>$11.6M</td>
</tr>
<tr>
<td>Diabetes</td>
<td>3</td>
<td>$34.5M</td>
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<tr>
<td>Eye Diseases</td>
<td>7</td>
<td>$54.5M</td>
</tr>
<tr>
<td>HIV/AIDS</td>
<td>4</td>
<td>$43.0M</td>
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<tr>
<td>Muscle Diseases</td>
<td>5</td>
<td>$17.1M</td>
</tr>
<tr>
<td>Neurological Diseases</td>
<td>26</td>
<td>$161.0M</td>
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<tr>
<td>Heart Disease</td>
<td>10</td>
<td>$73.6M</td>
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<tr>
<td>Liver Disease</td>
<td>4</td>
<td>$10.3M</td>
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<tr>
<td>Skin Disease</td>
<td>1</td>
<td>$11.7M</td>
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<tr>
<td>Immune Diseases</td>
<td>4</td>
<td>$33.0M</td>
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<tr>
<td>Genetic Disease</td>
<td>1</td>
<td>$5.5M</td>
</tr>
<tr>
<td>Vascular Disease/Multi</td>
<td>2</td>
<td>$20.0M</td>
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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:
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

- **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

Public/Private Research Fund*

- Disease & Family Foundations
- Venture Philanthropists & Angels
- Pharmaceutical Companies (R&D)
- Health Insurance Companies
- Government (NIH) & Other

Board
- CIRM
- Co-Funders (inside access)
- Outside Experts

Funding Decisions
- Each party makes own decisions on individual or multiple projects to fund
- Members pay fee
- Funder rights (e.g., right of first negotiation)

Pre-Clinical & Clinical Phase 1 & 2a/b Research Projects

*Administration of fund(s) to be determined. See next slide.
## Recommendation 1: Structure Public/Private Pre- & Clinical Trials Fund

### Objectives

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<tr>
<th>Action</th>
<th>Description</th>
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</thead>
<tbody>
<tr>
<td><strong>New Funding</strong>:</td>
<td>Raise non-taxpayer funds from diverse sources to leverage and expand CIRM project development from pre-clinical through clinical trials.</td>
</tr>
<tr>
<td><strong>Near-term</strong>:</td>
<td>Increase number of IND projects by disease theme that reach phase 2a/b.</td>
</tr>
<tr>
<td><strong>Longer-term</strong>:</td>
<td>Potential to extend CIRM’s lifetime and increase prospects for CIRM sponsored research to become therapies through new venture or biopharma relationships</td>
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### Develop

<table>
<thead>
<tr>
<th>Action</th>
<th>Description</th>
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<tbody>
<tr>
<td><strong>Plan</strong>:</td>
<td>Develop an operational business plan to engage partners.</td>
</tr>
<tr>
<td><strong>Pilot Phase</strong>:</td>
<td>CIRM selects 5-10 projects with CIRM contribution of $50M+ to attract $50M+ of external funds (&lt; 12 months).</td>
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### Scale-up

<table>
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<tbody>
<tr>
<td><strong>Organize</strong>:</td>
<td>CIRM convenes pharmaceutical firms and foundations around targets for co-funding to test public-private model.</td>
</tr>
<tr>
<td><strong>Formalize</strong>:</td>
<td>Possible creation of a 501c3 Foundation by third parties</td>
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### Operating Funding

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<tr>
<td><strong>Open</strong>:</td>
<td>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.</td>
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</table>
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

Interviews were conducted by James Gollub Associates in September, October and November, 2013.
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

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

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

- Deliver centralized CIRM capacities, expand CDAP model, and utilize future Alpha Clinics
- Collaborate with non-profit accelerators across universities in California
- Partner with venture backed accelerators and early-stage venture capital firms
CIRM Accelerator: Organization

Research & Development / Business Development

CIRM Centralized Services
- Early Translation Projects Management
- Disease Team Projects Management
- Production (Process & Manufacture) Management
- Clinical Regulatory Management
- Clinical Research Management

CIRM Accelerator Team
- Development Strategy & Structure
- Business Planning
- Product Development
- Product Match to Pharmaceutical Firms
- Enterprise Start-up & Investment Match

External Commercialization Services
- Non-Profit Accelerators
- Private Venture Backed Accelerators
- Seed and Early-stage Venture Funds
- Resource Networks

Early Translational Projects
Disease Team Projects
CIRM Priority Projects

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.”

Interviews were conducted by James Gollub Associates in September, October and November, 2013.
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

Development & Management

Planning & Syndicated Funding of Strategic Projects with Collaborative Stakeholders

ALLIANCE for Regenerative Medicine
and other organizations
Partnership & Co-Sponsorship

Regulation
- Funding Participants
  - Pharma
  - Foundations
  - Agencies
  - Others
- Research Teams
  - Collaborating Funders
  - Universities
  - Consultants
  - Vendors

Assays
- Funding Participants
  - Pharma
  - Foundations
  - Agencies
  - Others
- Research Teams
  - Collaborating Funders
  - Universities
  - Consultants
  - Vendors

Clinical Protocols
- Funding Participants
  - Pharma
  - Foundations
  - Agencies
  - Others
- Research Teams
  - Collaborating Funders
  - Universities
  - Consultants
  - Vendors

Scale Manufacturing
- Funding Participants
  - Pharma
  - Foundations
  - Agencies
  - Others
- Research Teams
  - Collaborating Funders
  - Universities
  - Consultants
  - Vendors

Reimbursement
- Funding Participants
  - Insurance
  - Pharma Foundations
  - Health Agencies
- Research Teams
  - Collaborating Funders
  - Universities
  - Consultants
  - Vendors

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

Interviews were conducted by James Gollub Associates in September, October and November, 2013.
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