



# 2012 Strategic Plan

**Presentation to ICOC**

**May 24, 2012**

**Agenda Item # 12**

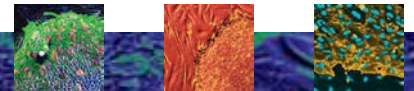
# Guiding CIRM's work

## Strategic Plan is a living document

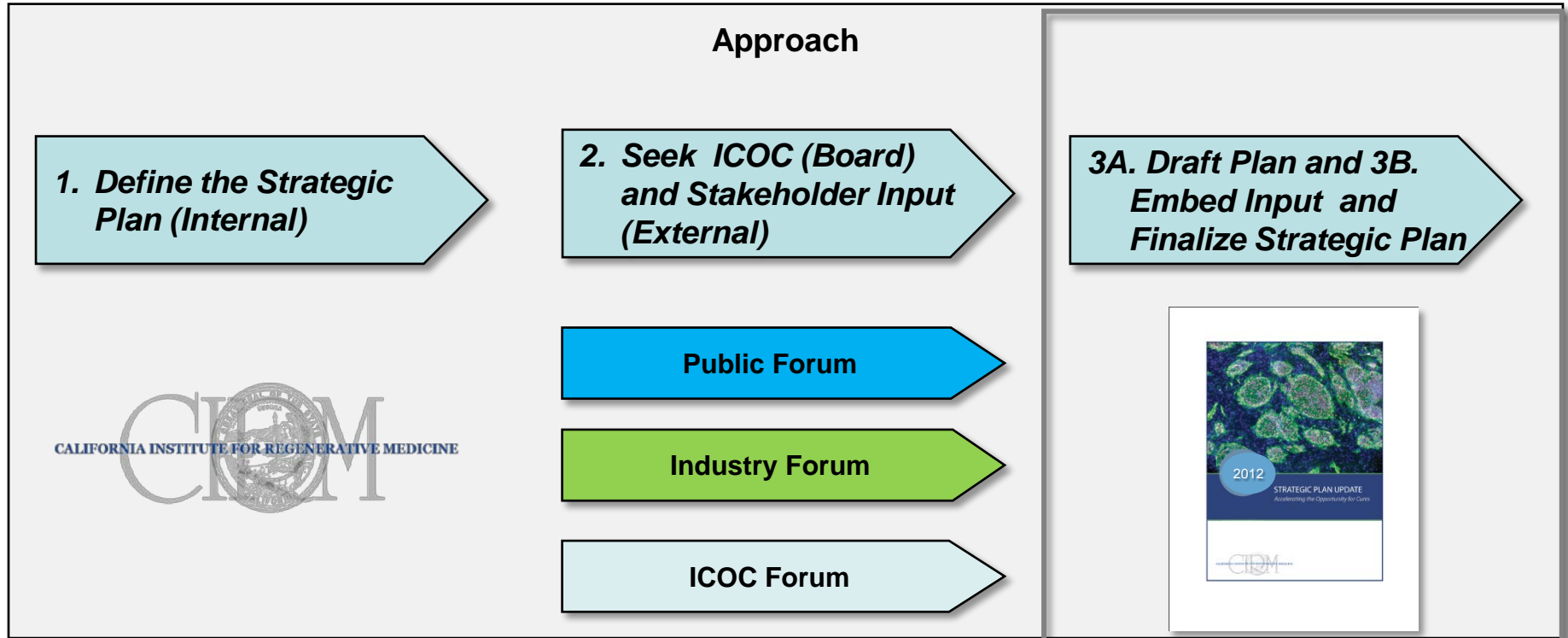


- Strategic Plan initial 2006, updated 2009/2010, in preparation for External Review Panel (ERP)
- 2012 update reflects ERP recommendations, other shifts in the field, ICOC and stakeholder input:
  - CIRM sr. staff retreat Aug 2011; Science Team Aug 2011
  - Updates at each ICOC meeting from Aug 2011 through Mar 2012
  - Stem Cell Research Leaders Sept 2011
  - Industry, Patient Advocates, Collaborative Funding Partners, Clinical Development Advisors, Professional Societies, Alliance for Regenerative Medicine Sept through Dec 2011

Strategic Plan updates shared with IOM Review Committee



# CIRM used a three-step approach to revise the Strategic Plan for 2012



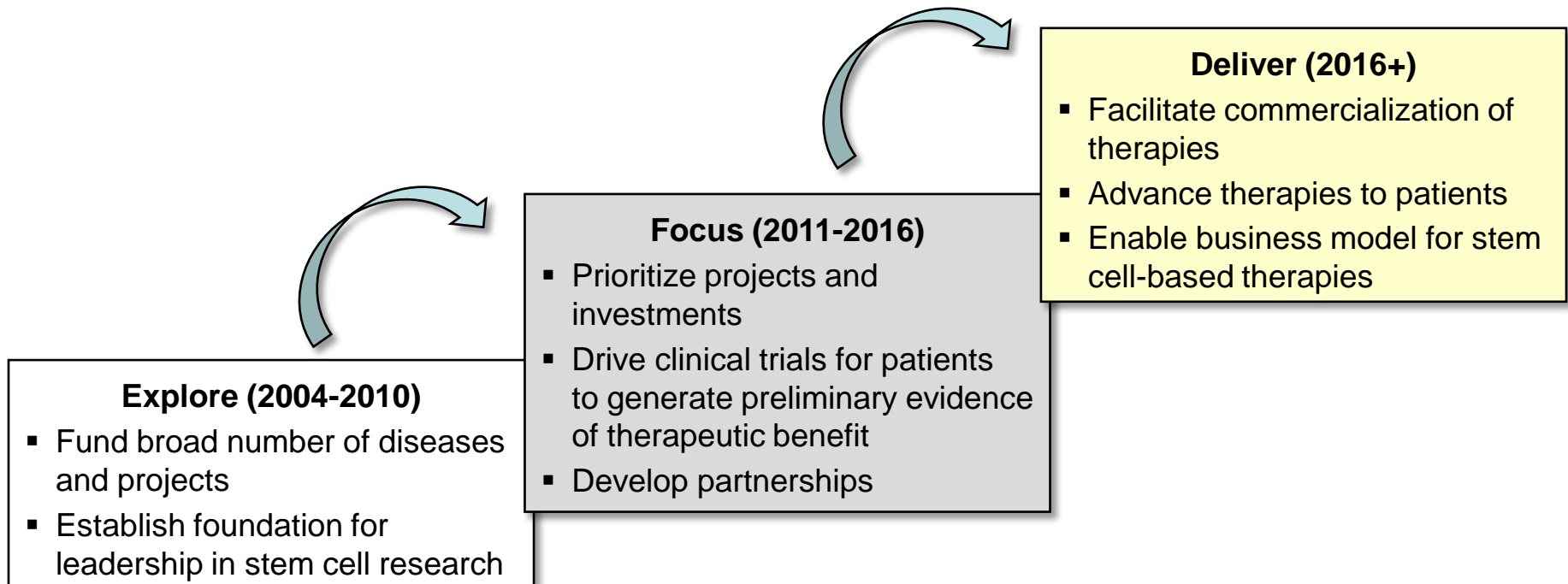
***This document focuses on step 3B and provides the strategic plan for consideration by ICOC (Board)***

# 2012 Strategic Plan

## CIRM Mission and Vision

### Mission

“To support and advance stem cell research and regenerative medicine under the highest ethical and medical standards for the discovery and development of cures, therapies, diagnostics, and research technologies to relieve human suffering from chronic disease and injury”



# Objectives and Single Most Important Key Outcomes over next 5 years- Aligns with Mission

## Strategic Objectives for Next Five Years

Scientific	Clinical	Economic	Community
Accelerate understanding of stem cell science and its applications towards human diseases and injuries	Advance stem cell science into clinical trials to achieve evidence of therapeutic benefit to patients	Drive economic development for California from stem cell science and therapies	Maintain California as the world stem cell leader

## Single Most Important Key Outcomes

Scientific	Clinical	Economic	Community
Achieve transformative research discoveries	Achieve clinical proof-of -concept for stem cell therapies	Leverage CIRM's investment in California	California globally recognized as the "Stem Cell State"

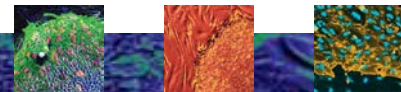
# 2012 Strategies to achieve objectives and goals

Scientific	Clinical	Economic	Community
<ul style="list-style-type: none"> <li>▪ Foster an engine of discovery and transformative research</li> <li>▪ Create a collaborative research community that enhances California's leadership and competitiveness</li> <li>▪ Realign funding programs, review and decision making with current strategic objectives</li> </ul>	<ul style="list-style-type: none"> <li>▪ Foster disease-specific research toward clinical proofs-of-concept</li> <li>▪ Expand multidisciplinary collaborative efforts to enhance clinical outcome</li> <li>▪ Foster developing a regulatory path for stem cell therapies</li> <li>▪ Boost the biotechnology sector in California</li> <li>▪ Focus, prioritize and evaluate projects to move the most promising forward</li> <li>▪ Enhance interactions with patients and advocates</li> </ul>	<ul style="list-style-type: none"> <li>▪ Attract co-funding and follow-on financing of CIRM projects</li> <li>▪ Foster the growth of California's stem cell industry and the creation of stem cell clusters that accelerate investment</li> <li>▪ Set the stage for cost savings in health care for the state and private payers</li> <li>▪ Establish a platform to enable grantees, disease foundations, venture capitalists and others to pursue CIRM's mission upon the expiration of CIRM's bond funding</li> </ul>	<ul style="list-style-type: none"> <li>▪ Communicate value proposition of CIRM and the stem cell field</li> <li>▪ Engage with stakeholders on why stem cell science matters to them</li> <li>▪ Create an awareness among stakeholders of CIRM's role in making California the leader in the field</li> </ul>

# CIRM set one year goals to achieve by July 2013



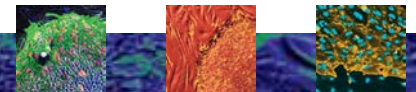
- At least 2 programs within CIRM's portfolio have an approved Investigational New Drug (IND) filed with the US Food and Drug Administration (FDA) to enter human clinical trials
- Achieved \$50 million in new, outside financial commitment (ie., collaborative funding partners, industry, venture capitalists, matching funds from institutions)
- Prioritized solicitations to ensure potentially high-impact projects that could result in transformative research are funded
- Educated and engaged the California community in CIRM's mission and achievements, in part by increasing the number of monthly online engagements from the current 70,000 to 100,000.
- Optimized CIRM's workforce staffing and processes to meet changing priorities within the 6% ceiling



# CIRM set five year goals to achieve by July 2017



- Factors regulating the self-renewal and tumor-causing potential of stem cells and their derivatives will be identified and characterized.
- Thorough description of the steps of differentiation leading to the production of critical cells of the body desired for transplantation will be achieved.
- New approaches for ensuring successful allogeneic cell transplantation are in clinical development.
- Established proof-of-principle in preclinical animal models for treatment of >10 diseases.
- Created disease-specific cell lines for 20-30 diseases and used them to gain new information about their underlying pathogenesis, and to identify new drug targets for discovery of new therapeutics.





# CIRM set five year goals to achieve by July 2017



- Developed new procedures for the production of a variety of stem and / or progenitor cells that meet requirements for clinical application.
- 20 CIRM funded programs with outside capital commitments for funding development work.
- 10 therapies in phase 1 or 2 clinical trials, in at least 5 different therapeutic areas, based on stem cell research.
- Clinical proof-of-concept that transplanted cells derived from pluripotent or progenitor cells can be used to restore function in disease or injury.
- Educated and engaged the California community in CIRM's mission and achievements, in part by having increased the number of monthly online engagements to 250,000.



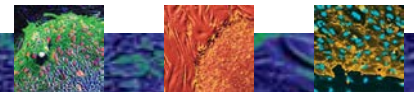
# Research Funding Strategy must align with Strategic Plan



Strategic Plan will guide:

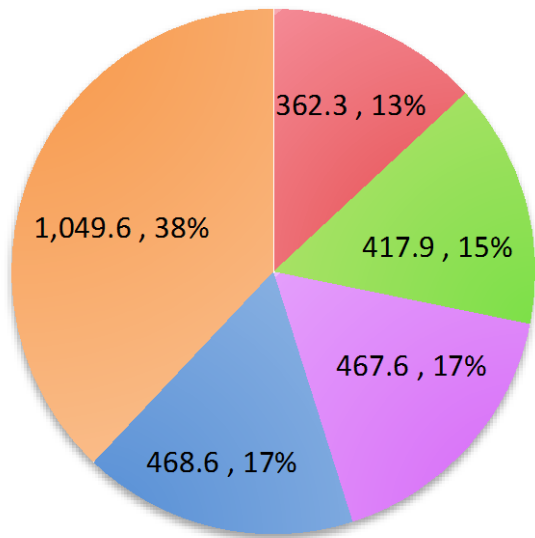
- Development of concepts, solicitations
- Inform GWG recommendations
- Inform ICOC decisions
- Resource prioritization and allocations

Progress Updates, Outcomes, Changes in Field will inform CIRM's actions; ICOC and public will be informed at regular intervals

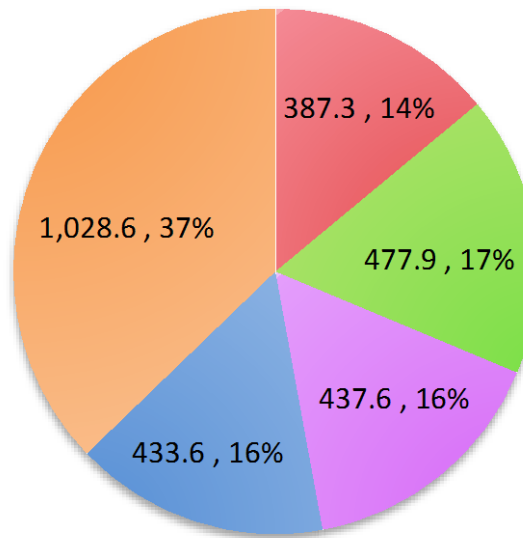


# Percentage distribution by category of total research funding for current, approved, and each of 2 potential future scenarios

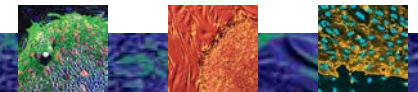
## Scenario 1



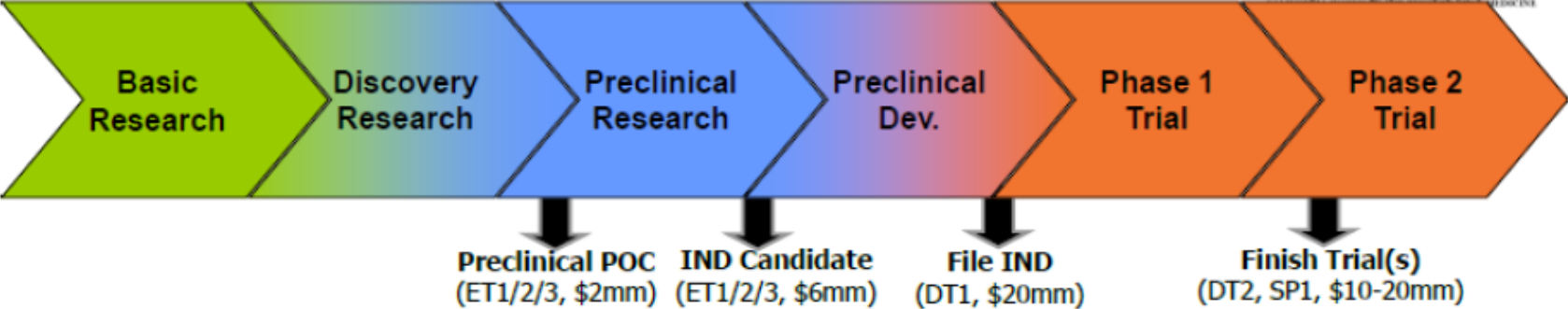
## Scenario 2



- Facilities/Core Resources
- Training/Career Dev.
- Basic Res
- Translational Res
- Development Res



# Translational/Development programs 9% by number, and 31% by \$, of \$1.28 B allocated to funded programs to date



Early Translational I,II (ET1, ET2)

Disease Team I (DT1)

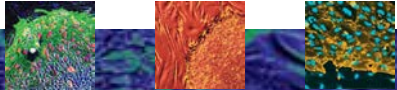
} 42 current translational programs

Early Translational III (ET3)

Disease Team II (DT2)

Strategic Partner I (SP1)

}

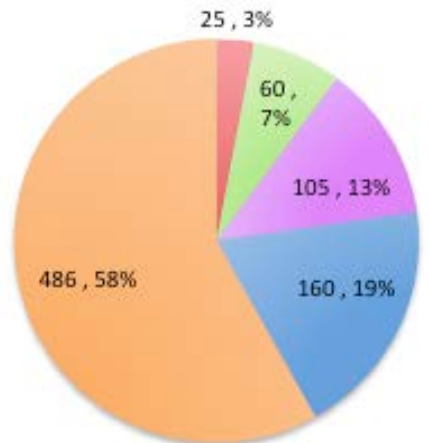
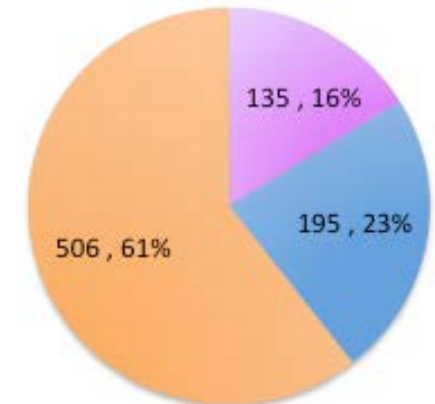
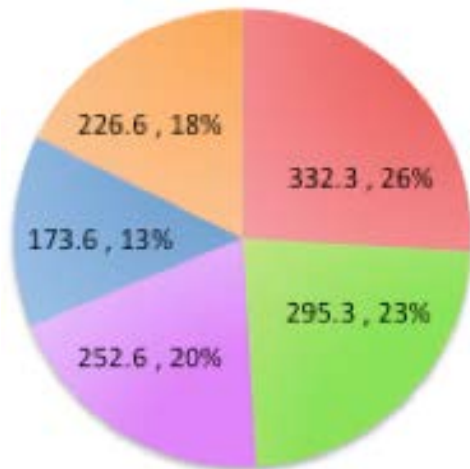


# Percentage Allocation of Funding – Funding for Translational/Development Increasing

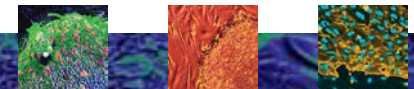
**Funded, \$1.28 B**

**Concept Approved, \$649 MM**

**Future – Scenarios, \$836 MM**



- Facilities/Core Resources
- Training/Career Development
- Basic Research
- Translational Research
- Development Research

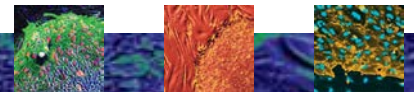


# Strategic Funding to Achieve Key Outcomes



1.5 Billion of current bond allocation remains to achieve key strategic outcomes

- Achieve transformative research discoveries
- Achieve clinical proof-of-concept for stem cell therapies
- Leverage CIRM's investment in California
- California globally recognized as the "Stem Cell State"



# Next Steps

## ICOC consideration of final version today

**2011**

### **September 19–December 5**

- ✓ Collect stakeholder feedback through public and invitational meetings and conference calls

### ✓ **December 8 ICOC/Board Update**

- ✓ **December 20**
  - First revision to senior management

✓ Completed activity

**2012**

### **January 3**

- Second revision to broader staff

### **January 17**

- Third revision to ICOC/Board for input-want to see goals and funding priorities and strategy at March ICOC and, final at May ICOC

### **March 21**

- ICOC/Board for input

### **May 24**

- ICOC/Board for consideration

# Back-up

---



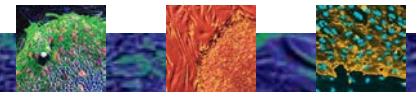


# Table of Contents for Strategic Plan

---



- Introduction and Context
- Executive Summary
- Vision
- Strategic Objectives and Key Outcomes
  - Accelerate Stem Cell Science
  - Advance Science into Clinical Trials
  - Drive Economic Development in California
  - Maintain Support for California's Leadership in the Field
- Goals with Strategies and Tactics Toward Success
- Res
- Appendices
  - A. Summary of External Advisory Panel Report and Leadership Response
  - B. The Process for, and Summary of, Stakeholder Inputs
  - C. Progress to the 2006 Five-year and Ten-year Goals
  - D. Supporting Data for Research Funding Projections
  - E. Drug Development Statistics



# ICOC (Board) Discussion and Stakeholder Input

- Public Meeting
- ICOC Meeting
- Industry Meeting

## Meeting Overview

In order to obtain ICOC (Board) and external stakeholder perspectives regarding the draft 2012 Strategic Objectives, the CIRM leadership team met with distinct stakeholder groups in five cities across California.



**USC:** Tuesday, October 25  
(Public Meeting)



**UCI:** Wednesday, October 26  
(ICOC Board Meeting)



**Sanford Burnham Cons. SD:**  
Thursday, October 27  
(Industry Meeting)



**ELAN:** Friday, October 28  
(Industry Meeting)



**UCSF:** Monday, October 31  
(Public Meeting)

*At each meeting, CIRM leadership provided an overview of progress to date and the proposed changes to the 2012 Strategic Objectives before seeking stakeholder input through a facilitated discussion.*

# Stakeholder Input

## Key Themes

Across all meetings, stakeholders communicated five key themes for CIRM leadership to consider during the 2012 Strategic Plan update.

1

**Established  
Momentum**

CIRM has made great initial progress in establishing an extensive program in support of stem cell research and the advancement of science.

2

**Sustainability**

CIRM needs to be more aggressive in finding alternate funding resources and to implement greater creativity in identifying the types of organizations that may be able to contribute to the sustainability of CIRM's work.

3

**Communication/  
Public  
Awareness**

Robust public affairs tactics are necessary, and CIRM needs to better communicate the organizational initiatives, as well as educate the public more broadly.

4

**Global  
Networking**

CIRM needs to provide greater opportunities for networking and breed collaborative projects that unite academia and industry, as well as researchers across geographic regions.

5

**Process  
Optimization**

Greater transparency in the funding process is needed, and there is a great need for the process to be less bureaucratic and easier to navigate.

# ICOC (Board) and Stakeholder Input

## Overarching Recommendations

- Public Meeting
- ICOC Meeting
- Industry Meeting

ICOC (Board) and Stakeholder input relative to each strategic objective, rolled this information up to provide a summary of considerations for the CIRM leadership team.

		Strategic Objectives			
		Scientific	Medical	Economic	Social
ICOC (Board) and Stakeholder Type	Public Input	<ul style="list-style-type: none"> <li>▪ Focus on imaging sciences</li> <li>▪ Create disease “hubs”</li> <li>▪ Share “lessons learned”</li> </ul>	<ul style="list-style-type: none"> <li>▪ Facilitate partnership opportunities</li> <li>▪ Obtain a cure or pass a major milestone</li> <li>▪ Consider surgery and somatic cell nuclear transfer</li> </ul>	<ul style="list-style-type: none"> <li>▪ Create funding opportunities</li> <li>▪ Find funding partners</li> <li>▪ Create three-way agreements</li> <li>▪ Allow project flexibility</li> </ul>	<ul style="list-style-type: none"> <li>▪ Ensure public awareness</li> <li>▪ Educate high school teachers/students</li> <li>▪ Simplify presentations</li> <li>▪ Create apps</li> <li>▪ Create CIRM image/logo</li> </ul>
	ICOC Input	<ul style="list-style-type: none"> <li>▪ Consider sustainability</li> </ul>	<ul style="list-style-type: none"> <li>▪ Require bi-directional communication</li> <li>▪ Categorize promising areas of research</li> <li>▪ Reduce threshold towards translational research</li> <li>▪ Define success</li> <li>▪ Learn from others</li> </ul>	<ul style="list-style-type: none"> <li>▪ Create matrix to funding platform</li> <li>▪ Engage and partner with HMOs</li> <li>▪ Include sustainability objective to strategic objectives</li> </ul>	<ul style="list-style-type: none"> <li>▪ Promote CIRM's work</li> <li>▪ Involve national stakeholder groups</li> <li>▪ Create list of statistics on disease and cost</li> <li>▪ Tailor strategies for various audiences</li> <li>▪ Create apparatus for “stars” (i.e., “star” patients, researchers) to shine</li> </ul>
	Industry Input	<ul style="list-style-type: none"> <li>▪ Share “lessons learned”</li> </ul>	<ul style="list-style-type: none"> <li>▪ Create communication bridges</li> <li>▪ Split review process (academic vs. industry)</li> <li>▪ Teach core processes</li> </ul>	<ul style="list-style-type: none"> <li>▪ Better communicate the details of the funding process</li> <li>▪ Create a shorter approval process</li> <li>▪ Create CIRM “champion” role(s)</li> </ul>	<ul style="list-style-type: none"> <li>▪ Identify and promote CIRM's expertise</li> <li>▪ Educate patients on stem cell facilities as well as stem cell medicine</li> </ul>

# Stakeholder input

---

- Industry advisors - November 10<sup>th</sup>
- Collaborative Funding Partners - Nov 15<sup>th</sup> and 18<sup>th</sup>
- Patient advocate organizations - December 2<sup>nd</sup> and 5<sup>th</sup>
- Clinical development advisors – November 28<sup>th</sup>,  
December 2<sup>nd</sup> and 5<sup>th</sup>
- Alliance for Regenerative Medicine – November 21st
- International Society for Cellular Therapy – November  
28<sup>th</sup>

