



Agenda Item #6
October 26, 2011 Board Meeting

October 20, 2011

From: Ellen G. Feigal, M.D., Senior Vice President, Research and Development

To: ICOC/Board

Re: Pre-Read for 2012 Strategic Plan Update Meetings—ICOC/Board meeting in Irvine, CA

The attached document serves as a pre-read for the upcoming Independent Citizens Oversight Committee (ICOC) Board Meeting in Irvine, CA, on October 26, 2011. Our objective for this meeting is to have a collaborative, consultative and inclusive discussion on your views, early in the process, of revising the strategic plan. This will be one of several discussions that we will have with you as the strategic plan evolves, gathering inputs from various stakeholders including patients, patient advocacy organizations, researchers, members of industry and other members of the public. We look forward to an interactive and productive discussion, hearing the range of viewpoints and approaches. At the ICOC meeting, we plan to provide a concise recap of the information provided in this document, identifying where we are in the timetable of revising the strategic plan, and conveying inputs from stakeholders received to date, as an introduction to facilitate discussion with and gather perspectives from the ICOC on the revising of the strategic plan re: strategic objectives and strategies to achieve them, so that we can meet a shared vision and mission.

California Institute for Regenerative Medicine

Pre-Read for 2012 Strategic Plan Update Meetings—ICOC Board

Irvine, CA / October 26, 2011

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Time Table

- CIRM maintains a Strategic Plan, initially adopted by the ICOC in 2006 and updated in 2009/2010.
- A review by the External Review Panel in October 2010 generated key recommendations for consideration for this update.
- The Strategic Plan needs to be updated to reflect these ERP recommendations, other shifts in the field, and our agreement to readdress the Strategic Plan approximately every 3 years. The process of updating the Strategic Plan will continue through ICOC consideration in March of 2012.
- Discussions to date include those from the CIRM senior staff retreat on August 18th ; the Science Team discussion on August 23rd ; the process update to the ICOC on August 25th, and the discussion with California stem cell leaders on September 13th .
- As in prior years, CIRM is seeking stakeholder input from patients, patient advocacy organizations, members of the public, researchers, and members of industry and looks forward to listening to their perspectives on how they think CIRM is doing in achieving its goals and what they think CIRM could do differently. Public meetings with stakeholders on October 25th in Los Angeles and October 31st in San Francisco regarding the Strategic plan have been posted on our website and information has been e-mailed to stakeholder audiences.
- Engage in collaborative, consultative and inclusive discussion with the ICOC, early in the strategic plan revision process, at the ICOC board meeting on October 26th
- The Strategic Plan will be shared with the Institute of Medicine (IOM) Review Committee.

ICOC Discussion Goals

At the ICOC Board Meeting on October 26, 2011, we will seek perspectives on key strategic items, such as our mission and objectives, and the rationale for any change.

1. Our Mission: What does success look like?

- CIRM is focused on achieving its mission, as this drives organizational planning and focus.
- Alignment on our mission is required to define the Strategic Plan, specifically in terms of
 - Strategic objectives
 - Staffing
 - Resource allocation
 - Request for Applications (RFA) Focus

2. Our Strategic Objectives: What are the priorities for the program to deliver over the next six years?

- Strategic objectives provide direction to achieve CIRM's mission.
- The objectives were originally defined in 2006 and updated in 2009/2010 for ERP and are now being revised in 2012 to reflect ERP advice and shifts in the field.

3. Our mission and strategy drive our processes: What changes in our processes will be needed?

- Our strategic objectives form the basis of plans for operational implementation. These include the following:
 - Operations Plan
 - Communication Plan
 - RFA content and prioritization
 - Grant review criteria
- As objectives and processes change, so will metrics used to measure progress. We will solicit your input and feedback on such proposed changes.

What Would Success Look Like?

CIRM's mission is to advance science towards stem cell-based therapies and our strategies focus on success across several dimensions that all need to be effectively communicated to the public; these dimensions include scientific advancement and treatments in clinical trials, with the expectation of a derivative economic benefit to California.

We would look for success in each of the three areas outlined below:

1. Science: Research is the foundation of therapy.

- Advancing the most promising scientific projects that have been thoroughly vetted through robust competitions, and encourage international collaborations that strengthen the project

2. Medicine: Stem cell-based therapies are the core of CIRM's mission.

- Getting treatments in clinical trials that evolved from stem cell research; we plan to do this in the following ways:
 - Show great strides getting game-changing, innovative therapies into clinical trials that would not have happened without CIRM
 - Transform public thought about science and medicine so the public better understands the dynamic connection between basic research and clinical development

3. Stem-Cell Hub: Economic benefits arise from creating a stem cell hub in California

- Achieving recognition of California as the “Stem Cell State,” which is viewed as a world-wide center of excellence in stem cell science and stem cell-based therapy.
 - Provide economic benefits as a leader in the field
 - Build a sustainable program that has capacity to compete and contribute
 - Stimulate California's biotech industry

Draft CIRM Target Outcomes

CIRM’s mission and desired success imply specific outcomes that fall into four categories: Scientific, Medical, Economic, and Social.

Scientific	Medical	Economic	Social
<ul style="list-style-type: none"> ▪ Build stem cell network/community/hub ▪ Conduct research ▪ Construct sustainable infrastructure/research model ▪ Enable innovation ▪ Positively impact industry ▪ Establish reputation ▪ Make CIRM-supported scientists more competitive for other grants 	<ul style="list-style-type: none"> ▪ Move patient treatments to clinical trials ▪ Improve medical outcomes ▪ Reduce healthcare costs ▪ Find pediatrics therapies (inherent long-term benefit) ▪ Develop new diagnostics and tests ▪ Establish a regulatory pathway ▪ Improve quality of life 	<ul style="list-style-type: none"> ▪ Design "better" medical practice model ▪ Positively impact industry ▪ Reduce healthcare costs ▪ Create financial returns to CIRM ▪ Improve California’s tax revenues ▪ Retain, create, or move jobs and talent to California ▪ Leverage momentum ▪ Increase career opportunities ▪ Create companies ▪ Return patients to work ▪ Provide relief for caregivers (e.g., family members) 	<ul style="list-style-type: none"> ▪ Improve awareness of stem cell science ▪ Build reputation ▪ Create career opportunities ▪ Improve medical practice model ▪ Provide science education ▪ Pave path for innovative emerging technologies to reach market ▪ Create platform for discovery ▪ Provide hope for patients who didn't have it ▪ Improve quality of life ▪ Return patients to work/play ▪ Improve health economics ▪ Enable ethical policy changes

Strengths, Weaknesses, Opportunities, and Challenges Analysis

Strengths: CIRM’s motivated and knowledgeable staff has created a strong set of processes to support the conduct of high-quality research in unique shared lab space.

- Nimble and Adaptive Organization: Embracing changes to stem cell field; designing programs to capitalize on opportunities

- People: Combining an engaged, expert board with a dedicated staff for a team that is knowledgeable of, aligned with, and committed to the mission
- Process: Bringing members of the academic community together through disease teams, outreach, and other activities coordinated by CIRM
- Shared Laboratory Space: Building laboratory space that receives accolades from the research community
- Research Results: Constructing very productive RFAs that have resulted in a wide range of successful grants

Weaknesses:

As stem cell research moves from basic research to therapies that could benefit patients, CIRM needs to prioritize activities, make appropriate changes to process, and communicate progress to the public.

- Prioritized Activities: Reacting to resource constraints; focusing on specific activities to accomplish goals; being cautious about the “let a thousand flowers bloom” approach
- Building Industry Relationships: Improving weak connection to business/industry that will be required to get therapies through late-stage clinical trials and make them accessible to patients in the marketplace
- Alignment of External Reviewers: Helping external reviewers, each of whom is knowledgeable about distinct aspects of CIRM’s program, to be aligned on the big picture; educating groups (e.g., ICOC, GWG) on the broader portfolio
- Revisions to Process: Implementing prioritized portfolio approach in program review; bringing commercial review to quality of current regulatory review; deciding how to help with licensing, patents, and other moves to commercialization; staffing appropriately
- Communication: Communicating broadly achievements and progress to public; including message that CIRM works across all types of stem cells, not just embryonic

Opportunities: Opportunities identified revolve around a transition in focus from basic research to moving therapies to commercialization.

- Specific Initiatives: Creating alpha clinics (centers of excellence in California with the staff and competencies to deliver stem cell-based therapies); issuing reverse translational medicine RFA (issues identified in clinic that need a basic research answer); making focused effort in tissue bioengineering and devices
- Leveraging Collaborations: Recognizing other states, institutions, countries "bolting-on" to CIRM projects; creating mechanism to bolt on CIRM to projects of other institutions, states, and countries
- Pull-In Model: Pulling in top projects and companies from outside California; building a business-development type of function that would require staff to scour the world and a

match-making service for collaborative funders; pairing non-Calif. researchers with Calif.-based researchers

- Commercialization Support/Tech Transfer: Bringing on regulatory, finance, and business development staff to provide support to grantees; being active in creating academic partnerships with Pharma and VC, but having BD experts on staff to help guide early commercialization
- Communication: Telling the CIRM story better and more broadly: being a good steward of taxpayer dollars; taking steps other than clinical therapy that are important; broadening public understanding of science and how the CIRM's major contributions to date are often basic but relevant; finding a poster child of an innovative project that gets through clinic

Challenges: Multiple issues create challenges for the successful transition from basic research to an approved therapy.

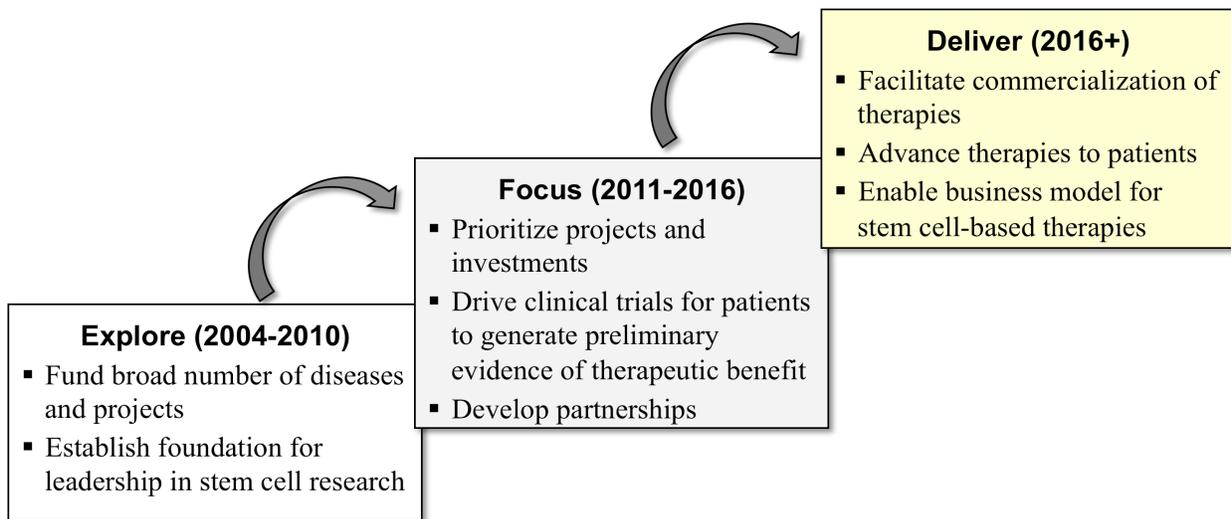
- Funding: Receiving no further funding past current \$3 billion for reasons such as other organizations that claim to have better uses for California funds
- Stem Cell Business Model: Lacking a viable model that is immediately apparent to industry and a clear reimbursement mechanism
- Delivery Model/Infrastructure: Lacking organized clinical community (e.g., physicians, nurses, technicians); missing clarity regarding whether administration will be by therapeutic area or all by stem cell specialists
- Public Awareness: Recognizing potential for a large negative impact occurring if there adverse events in a clinical trial and public expectations ahead of technological and economic impact

This analysis illustrates the need to focus the organization on specific strategies so that it will be able to best fulfill its mission with finite resources.

2009/2010 Strategic Cascade

CIRM'S Strategic Cascade 2009/2010					
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"					
Strategic Objective	Acceleration of Therapeutic Discoveries	Operational Excellence	Regulatory Certainty	Public Education	Economic Benefit to California
Initiatives	<ul style="list-style-type: none"> Academic-biotech partnerships Utilize scientific discoveries Manage portfolio for outcomes Share common data sets International/state collaboration Core RFAs New staff appointments in R&D 	<ul style="list-style-type: none"> Agency reorganization Outstanding recruitments Grants management systems Alliance management Robust grant reporting Improved financial planning and reporting 	<ul style="list-style-type: none"> Identify regulatory hurdles and difficulties Support registries of approved cells/tissues FDA collaboration Reimbursement study Leadership in policy development 	<ul style="list-style-type: none"> HS curriculum Face-to-face meetings Print World Wide Web Video (YouTube) Community involvement Inform legislators 	<ul style="list-style-type: none"> Economic impact studies Major facilities Leveraged funding Recruiting to California Collaborations Reduce healthcare costs

CIRM Vision



Strategic Cascade: Update Strategic Objectives for 2012

Can we refine our strategic objectives to align with the four target outcome areas (as previously defined)?

Refining 2009/2010 Strategic Objectives . . .

	Acceleration of Therapeutic Discoveries	Operational Excellence	Regulatory Certainty	Public Education	Economic Benefit to California
Rationale for Change	Morphed into science and medical benefit	Underpins all objectives	Became a strategy	Morphed into social benefit	Remains

. . . to Focus on 2012 Targets

Scientific Objective	Medical Objective	Economic Objective	Social Objective
Accelerate understanding of stem cell science and its applications towards human diseases and injuries	Advance science into clinical trials to achieve preliminary evidence of therapeutic benefit to patients	Drive economic development for California from stem cell science	Increase awareness of California as the leader in stem cell research and therapies

As a consequence of refining the strategic objectives, we could refine the underlying strategies for each objective as outlined below.

Scientific Strategies	Medical Strategies	Economic Strategies	Social Strategies
<ul style="list-style-type: none"> ▪ Enhance footprint (intellectual, IP, and physical infrastructure) ▪ Build partnerships with industry ▪ Increase scientific research collaborations ▪ Leverage expertise ▪ Revise prioritization and decision-making framework 	<ul style="list-style-type: none"> ▪ Prioritize/be more selective at all stages of the process ▪ Ensure that there is a pathway forward (regulatory, commercial) ▪ Build partnerships with industry, medical community, and global organizations ▪ Engage patients and advocates early 	<ul style="list-style-type: none"> ▪ Leverage investment through partnership ▪ Partner with California government ▪ Empower California Economic Development agency ▪ Draw companies to California 	<ul style="list-style-type: none"> ▪ Communicate value proposition ▪ Broaden communication, education, and messaging ▪ Partner with patient advocacy groups and the state of California ▪ Increase global outreach efforts