CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

President's Report

Alan O. Trounson

ICOC Meeting – March 2011

San Francisco, CA

Agenda Item # 7

Personnel

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Ellen Feigal, M.D.
Vice President, R&D
(formerly with Amgen)

Personnel

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Kevin Whittlesey, Ph.D.
Science Officer
(formerly with FDA)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

2011-12 Budget Process
March 2011

John Robson, PhD
VP Operations

Budget Process - Timetable

December 15, 2010	Kick-off
January 19, 2011	Budgets due
February 16, 2011	Discussion with President and Chair
March 1, 2011	Discussion with Chair, co-Chair, FSC
March 10, 2011	Initial overview to ICOC
March 23, 2011	Discussion with President and Chair
Week of April 11 or 18	Presentation to FSC
May 3-4, 2011	Presentation to ICOC

Budget Process – Drivers

Meeting CIRM's Mission

- External Review Panel
- SB1064

On-going Activities

Activity	FY 2009-10	FY 2010-11
Progress Reports	467	563
Grant and Loan Payments	663	975
Grants with co-funding partners	4	15

Budget Process - Drivers

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

New Initiatives

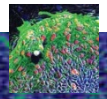
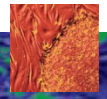
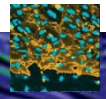
- Monitoring clinical programs
- Creating pathways to the clinic

Audits

- Performance Audit
- Institute of Medicine Audit

Exercising Restraint

- Hiring only for core mission and fiduciary responsibilities
- Remaining under CIRM's 6% limit

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

Management Response To External Review Report

Recurring Message

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Move Away From Traditional Funding Agency Model

↓

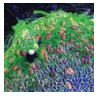
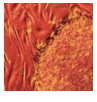
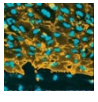
Aggressive and Proactive Approach to Funding

↓

Limit Portfolio

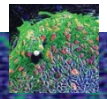
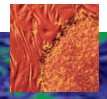
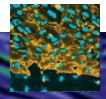
↓

Seek out Promising Projects

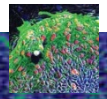
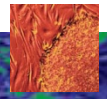
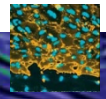
External Review Recommendations

- *Maintain focus on meaningful, targeted scientific excellence*
- *Sustain fundamental discovery*
- *Pave a path from fundamental to translational research, translational medicine, product development and healthcare delivery*
- *Conduct a critical assessment and prioritization of the current portfolio with input from CIRM's diverse stakeholders*

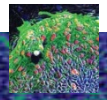
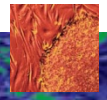
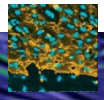
External Review Recommendations

- *Develop an open innovation-focused, porous pipeline strategy*
- *Assume a leadership role in the critical social, ethical, regulatory and health care delivery issues*
- *Develop strategies to improve/expand engagement with industry*
- *Broaden international partnerships to leverage expertise and resources*

External Review Recommendations

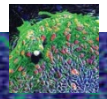
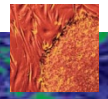
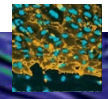
- *Expand breadth of outreach and education to ensure state-wide visibility and awareness*
- *Clarify the roles and responsibilities of the Governing Board Chair and the President as it pertains to CIRM's strategic directions*

Keep CIRM on the leading edge of Stem Cell Science: To fund the best research proposals and continue to fund basic research (Recommendations 1, 2)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

- Strengthen & Continue to:
  - Develop innovative prescreening methods, such as Pre-application review, to consider large numbers of proposals
  - Recruit world-class scientists from academia and industry to GWG
  - Ensure funding driven by scientific merit and compelling, mission-critical programmatic and portfolio considerations
  - Maintain regular funding opportunities for basic research on stem cells

Optimize CIRM's portfolio…To Achieve Clinical Therapies (Recommendation 4)

Prioritize portfolio & Make difficult funding decisions

CURRENT PRACTICES: DISEASE TEAM STRUCTURE

- Clinical Advisory Panel -- consult with the VP, R&D on project strategy, progress against milestones, and success at go-no-go decision points, and advise the President about the merit of continued support.

FUTURE PRACTICES:

Consult with internationally recognized experts, including scientists, clinicians, regulatory experts, industry representatives, venture capitalists, and disease advocacy organizations to:

- Create an "Opportunity Fund" (~$25 Million*) to accelerate rapidly advancing and successful projects towards clinical trials and patient treatments
- Develop criteria that define projects most likely to succeed
- Incorporate Relevance** as a consideration in evaluating projects
- Identify key gaps where they exist in CIRM's portfolio
- Determine which diseases are most amenable to therapies for patients

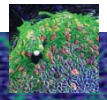
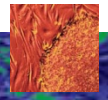
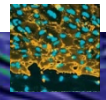
*up to $3Million could be awarded at President's discretion; and >$3M following presentation and vote of the Board

** Relevance: Competitive as an alternative treatment and viable development plan

Develop a proactive strategy that enables porosity of access and targets for the most promising research (Recommendations 3, 5, 7)

Be aggressive & proactive & seek out the best research:

- *Bring promising projects into the development pipeline at all stages*
- *Efficiently push forward the most promising projects*
- *Provide direction to academia and industry on critical needs in specific areas based on portfolio analysis, internal assessment and external advice*
- *Better engage with industry*
  - *Capture industry's special capabilities (e.g. toxicity testing, manufacturing and scale-up)*
  - *Better meet industry timelines*
  - *Ensure development projects can attract outside investment*

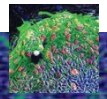
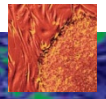
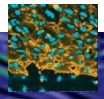
Porosity of access and targets for the most promising research (Recommendations 3, 5, 7)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

FUTURE PRACTICES for programs already funded by CIRM and approaching the clinical stage of the development pipeline:

Create an "Opportunity Fund" for highly successful projects to accelerate existing promising and competitive CIRM projects and reduce the amount of time spent writing proposals and in review

Repeat core RFAs (Basic Biology, Early Translation, Disease Teams, Therapy Development) on a regular basis so that new projects can enter the pipeline at the appropriate stage and those projects within the pipeline can plan for progression in the context of a competitive renewal, if they do not receive Opportunity Funds.

Porosity of access and targets for the most promising research (Recommendations 3, 5, 7)

FUTURE PRACTICES for Programs singled out by the priority review process but not currently funded by CIRM:

Within California

- Contact research groups & make them aware of up-coming competitions that could fund their research.
- Encourage collaborations among researchers with complementary expertise and invite them to apply.

Outside California

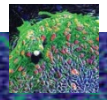
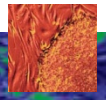
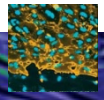
- Invite research groups to networking meetings/ workshops with California researchers &/or companies that have overlapping interests.
- The goals would be to establish collaborations and encourage their development in California.

Porosity of access and targets for the most promising research & accommodate the private sector (Recommendations 3, 5, 7)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

FUTURE PRACTICES:

- Fund grant writing
- Translational arena--
  - require partnerships between academia and industry
- Explore strategies that would target industry expertise and accommodate the need for shorter timelines

Engage with Industry to encourage and enable commercialization of the most promising stem cell research (Recommendations 3, 7)

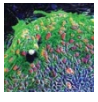
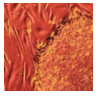
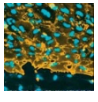
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Encourage and foster stem cell industrial expansion in California to produce economic benefits and health-related benefits to its citizens.

FUTURE PRACTICES:

Promote California-based stem cell related companies to the broader stem cell research community

- Invite California-based research support companies to the CIRM Grantee Meeting to exhibit their stem cell related products and services
- Feature companies in a searchable resource portal on the CIRM website

Engage with Industry to encourage and enable commercialization of the most promising stem cell research (Recommendations 3, 7)

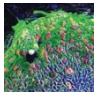
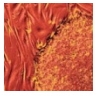
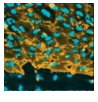
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Encourage and foster stem cell industrial expansion in California to produce economic benefits and health-related benefits to its citizens.

FUTURE PRACTICES:

CREATE A POOL OF INDUSTRY AND FOUNDATION EXPERTS WHO WOULD HELP CIRM:

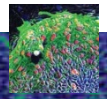
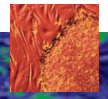
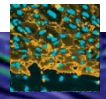
- Make its programs attractive to industry
- Identify research areas most appropriate for industry
- Identify CIRM-funded inventions that should be patented;
- Create opportunities for follow-on funding for CIRM-funded research programs especially those approaching clinical trail;
- Identify and advance business models for regenerative medicine;
- Identify and assist CIRM in fostering industry-academic partnering opportunities.

Engage with Industry to encourage and enable commercialization of the most promising stem cell research (Recommendations 3, 7)

FUTURE PRACTICES:

- Feature companies in a searchable resource portal on the CIRM website
- Identify research areas most appropriate for industry
- Provide a process to fund the costs of patent filing for the most promising stem cell technologies
- Help broker research collaborations between academic institutions in California and relevant companies
- Create a forum for researchers to present their findings to industry and venture representatives

Take a leadership role in developing national and international standards related to regulatory issues, policy and ethics: (Recommendation 6)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

CURRENT PRACTICES:

- CIRM representatives Participate in key national panels and committees developing standards
- CIRM regulatory webinars and roundtables with FDA participation.
- CIRM conceptually approved the sponsoring of a Regenerative Medicine Translational Journal



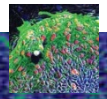
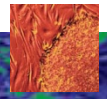
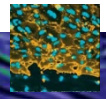
Take a leadership role in developing national and international standards related to regulatory issues, policy and ethics: (Recommendation 6)

CURRENT PRACTICES:

- CIRM is and will remain vigilant and responsive to legislative and judicial efforts to restrict stem cell research.

FUTURE PRACTICES:

- Monitor and, when possible, help resolve issues that present regulatory and ethical challenges
- Partner with grantees to understand these issues

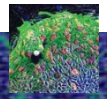
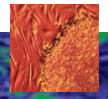
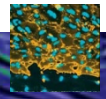




Take a leadership role in developing national and international standards related to regulatory issues, policy and ethics: (Recommendation 6)

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

FUTURE PRACTICES:

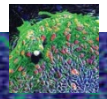
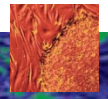
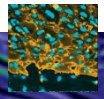
- Donor consent
  - Create an educational module about consent issues for donors to banks
  - explore ethical issues that will arise as research with banked cells reveals health risks to the donors
- Overseas research
  - Address issues on a case by case basis including developing core guiding principles
  - Develop tools on its website to educate the public on the ethics of stem cell research.

Expand CIRM's international partnerships and collaborations (Recommendation 8)

FUTURE PRACTICES:

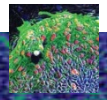
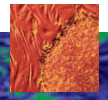
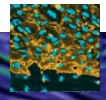
- Identify research needs of the California stem cell research community
- Identify and approach additional participants for the CFP program based upon:
  - The identified areas of California need; and
  - The strength of California's existing work and emerging programs

Expand CIRM's international partnerships and collaborations (Recommendation 8)

FUTURE PRACTICES:

- Adopt an "Opportunity Fund" program to fast-track clinical and advanced translational projects
- Develop regular communications to disease foundations to partner with CIRM in moving late stage projects into clinical development
- Include disease foundations in efforts to develop and support regulatory pathways

Expand CIRM's international partnerships and collaborations (Recommendation 8)

FUTURE PRACTICES:

- CIRM should encourage selective use of supplemental funding ("bolt-on")
- CFPs would be invited to fund supplemental work by foreign scientists
- The CFPs would have more autonomy within their research component