CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

*The state stem cell agency*

# President's Report

***Alan O. Trounson***

***ICOC Meeting – May 2011***

***Los Angeles, CA***

# Self–organizing Optic-cup Mophogenesis in Three-Dimensional Culture. Eiraku et al., RIKEN Center Dev Biol, Kobe Japan. *Nature*, 7 April, 2011

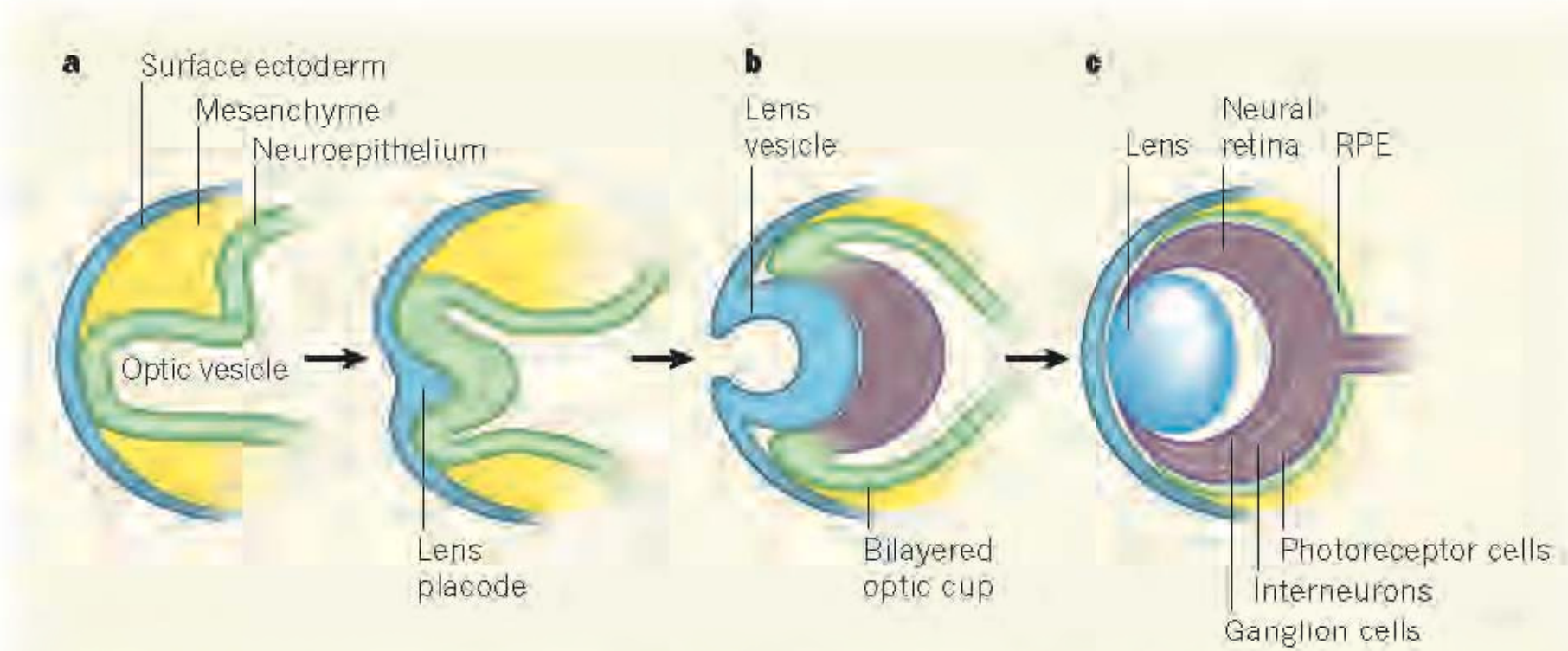
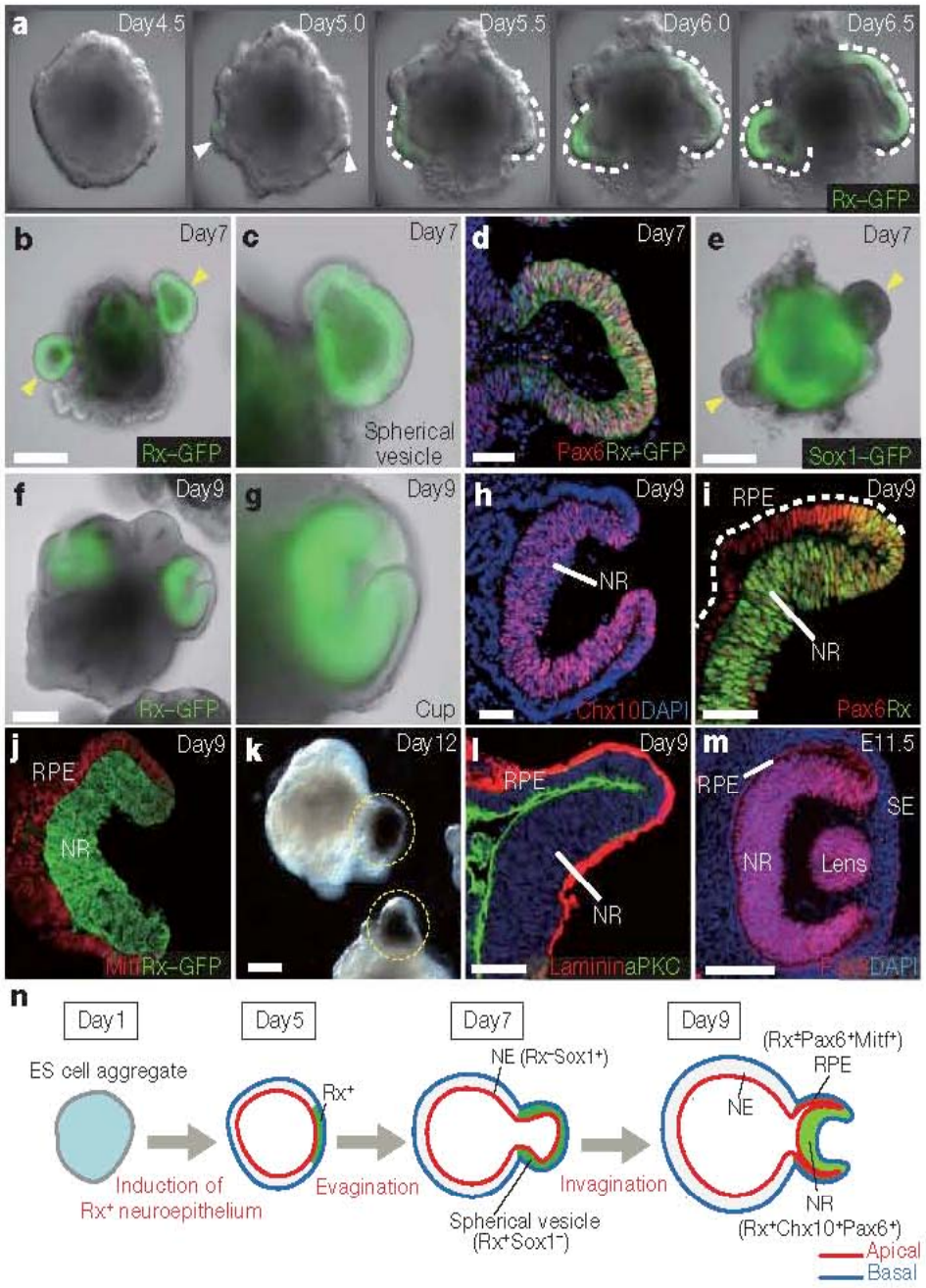



**Figure 1 | Eye development.** **a**, At early stages of eye development, the surface ectoderm thickens and invaginates together with the underlying neuroepithelium of the optic vesicle. **b**, The inner layer of the bilayered optic cup gives rise to neural retina and the outer layer gives rise to the retinal pigmented epithelium (RPE) (**c**). The mature neural retina (**c**) comprises three cellular layers: photoreceptors, interneurons (horizontal, amacrine and bipolar cells), and retinal ganglion cells. Eiraku *et al.*[1] generated optical cups *in vitro* from embryonic stem cells.

Ali & Snowden, Nature 2011



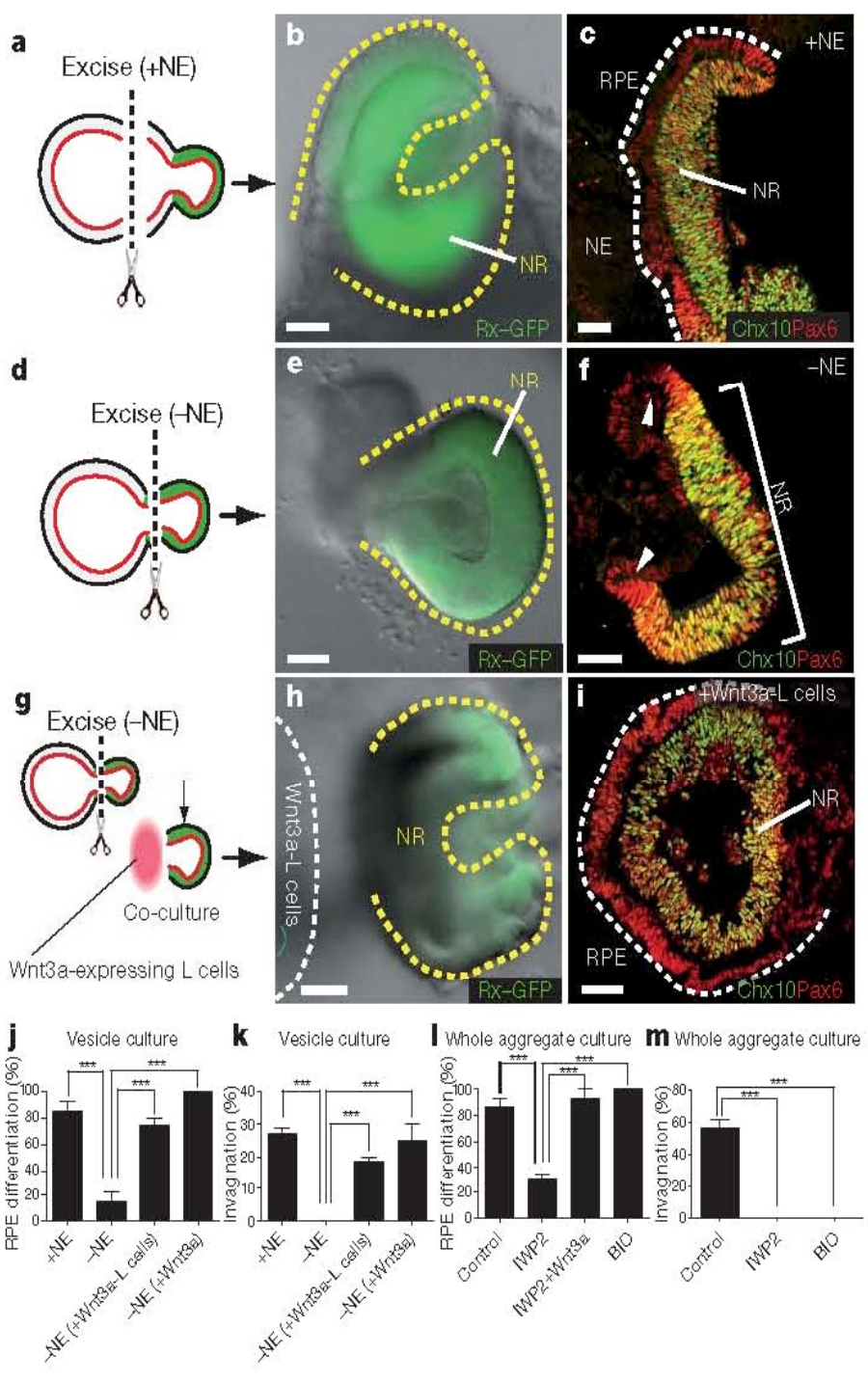




**Figure 1 | Self-formation of an optic-cup-like structure in 3D culture of ES cell aggregates.** **a–e**, SFEBq/matrigel culture. **a–e**, Self-formation of vesicles

# Modelling Schizophrenia Using Human Induced Pluripotent Stem Cells. Brennand et al., Gage's Lab Salk Institute, *Nature*, April 2011

SCZD showed diminished connectivity, decreased neurite number, PSD95-protein levels and glutamate receptor expression.

SCZD hiPSC neurones altered expression of cyclic AMP and WNT signaling pathways.

Antipsychotic loxapine ameliorated the SCZD phenotype

SCZD is a complex genetic psychiatric disorder. The iPSC model will be informative for therapeutics going forward.

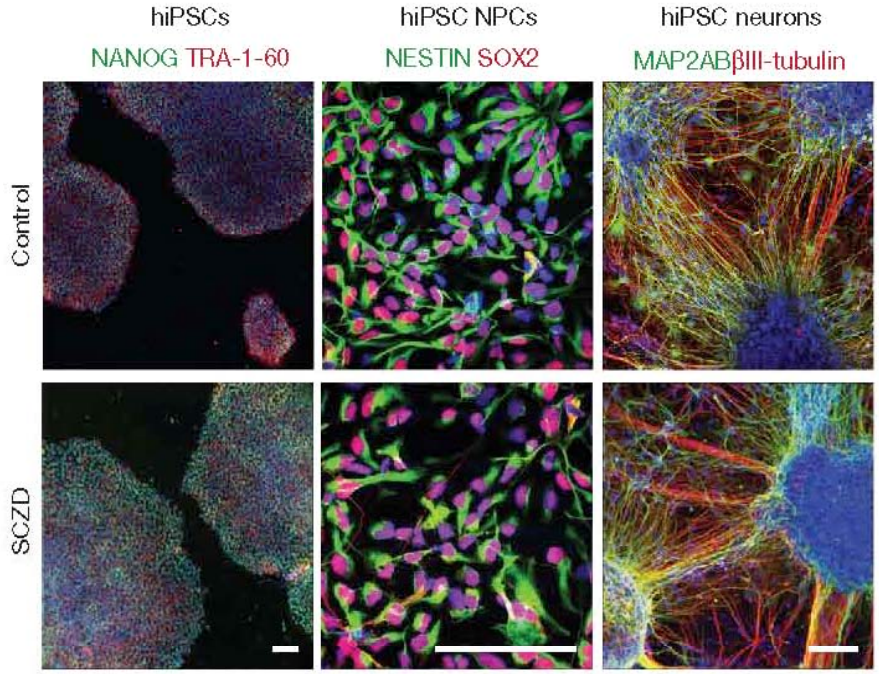


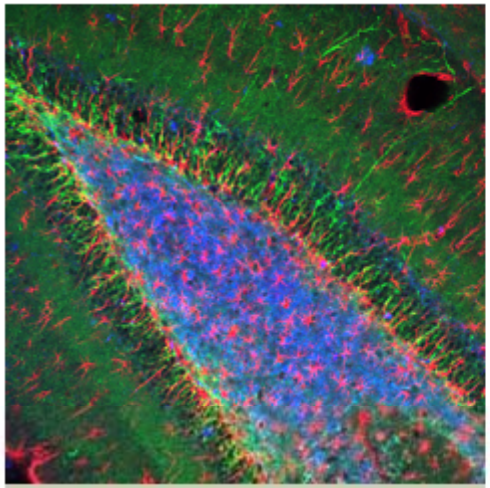

**Figure 1 | Patient-specific hiPSCs, NPCs and neurons.** Left, hiPSCs express

# In 2010 CIRM was ready for a new phase of Patient Advocate outreach



- Working on diseases patients could relate to
  - Disease teams were up and running
  - CIRM website added disease-specific information
  - All CIRM grants became searchable by disease
- Issued RFA for early clinical trials with pluripotent stem cell therapy
  - Access to and information on innovative therapy
- RFP for patient advocate outreach coordinators
  - Contract to StiehlWorks, Lorraine & Chris Stiehl

# CIRM website features diseases we are working on…

A slice through a rat brain showing cells of the hippocampus, a region affected by Alzheimer's Disease (Frank LaFerla at the University of California, Irvine)

CIRM funds many projects seeking to better understand the origins of Alzheimer's disease and to translate those discoveries into new therapies. One of these projects received an Early Translational Award to move basic laboratory discoveries toward new therapies.

If you want to learn more about CIRM funding decisions or make a comment directly to our board, join us at a public meeting. You can find agendas for upcoming public meetings on our meetings page.

**Learn more about stem cell research:**
Stem Cell Basics Primer | Stem Cell Videos | What We Fund

**Find clinical trials:**
CIRM does not currently fund or track stem cell clinical trials. If you or a family member is interested in participating in a clinical trial, please see the national trial database to find a trial near you: clinicaltrials.gov

## Description

- •18 Disease-specific web pages
- •Basic information about the disease
- •Links to CIRM funded research projects
- •Links to related stories, articles
- •Links to related videos on CIRM's YouTube channel CIRMTV

# First Phase of CIRM's patient advocate outreach was a Listening Tour



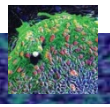
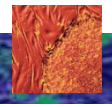
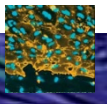
What do you know of CIRM/What can we do to help you? Here's what we heard

- Keep me current on research
  - Keep me informed on research; how CIRM research relates to the clinic, and help me access new treatments
- Help me be a better advocate
  - Become a partner with CIRM; a better advocate for my agency, and understand the benefit of CIRM
- Educate me and my constituents
  - About CIRM, about ways we can stay informed, and provide materials for learning

# How have we responded to the patient advocates?



- 16 meetings involving over 2,000 advocates
- Two major events this month
  - May 7th Focus on neurodegenerative diseases
    UC Irvine with Leeza Gibbons
  - May 21st Focus on Inflammation, Cardiovascular, Autoimmunity
    Stockton
- Nearly 600 advocates signed up for Monthly Digest
- Foster sharing of CIRM educational materials on their web
- Created disease specific handouts for them
- Drafted newsletter stories for them (3 so far)

# Patient Advocacy Day and Stem Cell Update on Neurodegenerative Diseases

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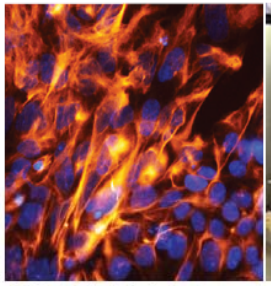
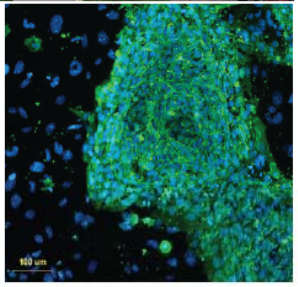




## SYNAPSES FIRING: CONNECTIONS MADE

### CIRM Patient Advocacy Day & Stem Cell Update on Neurodegenerative Diseases

**Saturday**
May 7th, 2011

**Sue and Bill Gross Stem Cell Research Center**
Biomedical Research Park
UC-Irvine (UCI)

**9:00 AM** **Registration and Continental Breakfast; Patient Advocate Display Tables**

**10:00 AM** **"An Overview of UC-Irvine's Stem Cell Program and Stem Cell Basics"**
Peter J. Donovan, Ph.D., Director, Sue and Bill Gross Stem Cell Research Center, UC-Irvine

**Welcome and Keynote Presentation, "CIRM's Investment in Neurodegenerative Diseases"**
Leeza Gibbons, CIRM ICOC Governing Board, Alzheimer's Disease Patient Advocate; the Leeza Gibbons Memory Foundation

**"The Faces of Neurodegenerative Disease"**
Neal S. Hermanowicz, M.D., Neurologist, Movement Disorder Program, Department of Neurology, UC-Irvine

*Patient Advocate Spotlight: Serge Morales and Susan Franklin*
**"Using Stem Cells to Understand and Treat Neurodegeneration, including Alzheimer's Disease and Huntington's Disease"**
Mathew Mark Blurton-Jones, Ph.D., Neurology and Behavior, School of Biological Sciences, Institute for Memory Impairments and Neurological Disorders (UCI-MIND), UC-Irvine

*Patient Advocate Spotlight: Evan Henry*
**"Stem Cells and Parkinson's Disease"**
Birgitt Schuele, M.D., Clinical Molecular Geneticist, the Parkinson's Institute and Clinical Center, Sunnyvale

**Complimentary Lunch**

*Patient Advocate Spotlight: Bill Johnston*
**"Using Stem Cells to Model Neurodegenerative Diseases in Huntington's Disease and Spinal Muscular Atrophy"**
Virginia B. Mattis, Ph. D., Regenerative Medicine Institute at Cedars-Sinai Medical Center, Los Angeles

**Patient Advocate Sharing Sessions**

**2:00 PM** **Program Concludes**

Please contact Lorraine Stiehl, CIRM Patient Advocate Coordinator • lorstiehl@stiehlworks.com
**RSVP** 619-516-2864

# CIRM moving to strengthen/expand relationships with patient advocates



- Continue in-person chapter/local meetings
- Expand our outreach through the emailing lists
- Expand Stem Cell Awareness Day Oct. 5
  - Last year's Stem Cell Awareness Day grew to 20 events with 1,500 attendees from 5 countries and 6 states
- Continue listening—follow-up in June with the patient advocates in the fall tour to assess our progress on areas of mutual interest – research, advocacy, education
- Strengthen and expand relationships in clinical research and clinical trials

# Upcoming Workshops



## Japan Science and Technology Agency (JST)/CIRM

-- Kobe, Japan – May 16 – 17, 2011

-- Focus on Early Translation and Basic Biology

-- 9 California Scientists/18 Japanese Scientists

## Newborn Neurological Disorders Leading to Cerebral Palsy

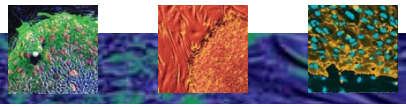
– San Francisco – June 7 – 8, 2011

– 22 invited US and international speakers

## CIRM/Agence Nationale de la Recherche (ANR)

– Paris, France – July 12-13, 2011

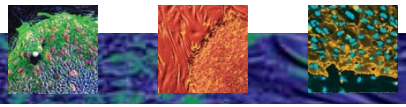
--Focus on basic biology – 8 Californians/12 French Basic Scientists



# CIRM 2011 Bridges to Stem Cells Annual Trainee Meeting - San Francisco, July 7-8, 2011



- 16 Bridges programs will participate
- Up to 160 trainees, mentors, and program directors
- Speakers, poster presentations, career panel
- The meeting will bring together trainees to present their research, meet peers and interact with scientists in the stem cell community.



# CIRM 2011 Grantee meeting - San Francisco September 14-16, 2011

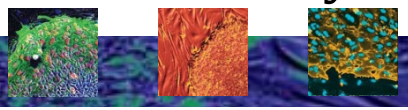


Purpose:

- Bring together investigators and trainees funded by CIRM and CIRM's collaborative funding partners
- Highlight grantee work from basic through translational/clinical research
- Encourage scientific exchange and collaboration

Meeting Agenda:

- Plenary talks by CIRM Principal Investigators and other leaders in the field
- Posters and short talks by trainees
- Networking opportunities
- Training opportunity in public communication and advocacy



# Workshop Report: Australia/CIRM Workshop – April 11 & 12, 2011



Walter & Eliza Hall Institute of Medical Research (WEHI) – Melbourne

NH & MRC, WEHI, National Trauma Institute, Monash University, Monash Institute of Medical Research, Royal Melbourne Hospital, University of Queensland and Mater Hospital & Health Services,

Stanford, UCLA, UCSF, UCSC, UCI

-- Talks by 10 California Scientists/14 Australian Scientists

-- Open to scientists and the public

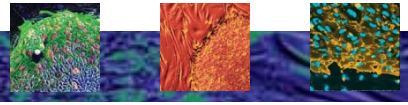
Topics covered:

Hematopoiesis

Blood Cells and Cancer

Epithelial Stem Cells in Disease

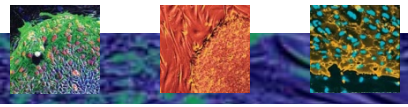
Neurogenesis



# Upcoming RFAs



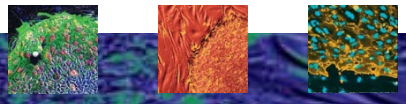
- Early Translational III
  - Concept Presentation – May ICOC
- Targeted Clinical Development
  - ICOC – May 2011
- Basic Biology III
  - ICOC – May 2011
- Disease Team Therapy Development
  - RFA November 2010
    - Review of Planning Applications – May 2011
    - ICOC – August 2011
  - Research Applications – January 2012
    - Review of Research Applications – March/April 2012
    - ICOC – June 2012



# Measuring CIRM Productivity



- **What should be measured?**
  - Moving Toward the Clinic/Defining a Clinical Pathway
  - Stimulating Growth in Stem Cell Research
  - Scientific Accomplishments
  - Economic Impact
  - Engaging the Private Sector
  - Educational and Communications Activities



# What should be measured to determine CIRM's Success?

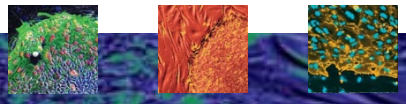


- **Moving Toward the Clinic/Defining a Regulatory Pathway**
  - Number of IND Filings/Clinical Trials based on CIRM-funded research
  - Number of comments submitted with respect to regulatory draft guidance
  - Number of meetings with FDA and other regulatory agencies
  - Number of papers submitted by CIRM advancing a more defined regulatory pathway
  - Number of stakeholder meetings focused on developing standards and best practices

# What should be measured to determine CIRM's Success?



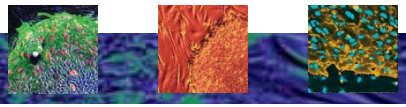
- **Stimulating Growth in Stem Cell Research**
  - Number of new stem cell scientists who have moved to California annually
  - Number of companies that moved to CA
  - Number of companies created in CA
  - Number of companies shifted focus to stem cells
  - Funded Grant applications based on collaborations
    - Number of grants per Country or State
    - Value of co-funders investment



# What should be measured to determine CIRM's Success?



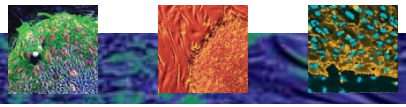
- **Scientific Accomplishments**
  - Ranking of CA stem cell scientists
  - Number of publications from CIRM-funded research
  - Number of patent applications from CIRM-supported research
  - Expanded usage of specimens and biological materials generated by CIRM funded research



# What should be measured to determine CIRM's Success?



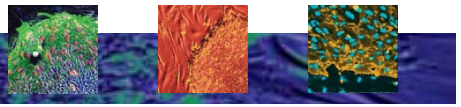
- **Economic Impact**
  - Leveraged Funds - New monies brought to CA
    - Grants obtained from public and private foundations based on preliminary results obtained with CIRM funding or due to access to CIRM Shared Labs or Major Facilities
    - Philanthropic donations to CIRM major facilities or CIRM-funded investigators
    - Co-Funding of CIRM grants/loans



# What should be measured to determine CIRM's Success?



- **Engaging the Private Sector**
  - CIRM support of companies through subcontracts on grants
  - Capital that is attracted to CIRM funded projects
  - Number of grants and loans to Academic and Industry Partnerships



# What should be measured to determine CIRM's Success?



- **Educational and Communications Activities**
  - Education
    - Use of curriculum developed with CIRM Funds
    - Stem Cell Awareness Day Activities
    - School Visits by CIRM-funded scientists.
  - Communications
    - Press articles about CIRM and CIRM Grantees
    - Hits to CIRM Website, and CIRM videos on YouTube