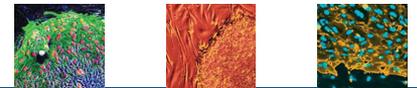




President's Report

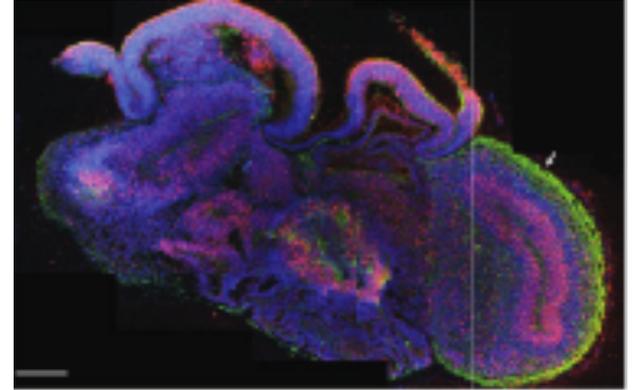
Ellen Feigal, M.D.
ICOC Meeting — October 2013
Burlingame, CA



iPS Cells form organoids with multiple types of brain cells

J. Knoblich et al. *Austrian Academy, Nature*, Sept. 19

- Most complex neural tissue to date
- Assembled spontaneously in lab
- Key to assembly: gel that mimicked natural connective tissue
- Organization not like normal brain
- Immediate impact, researching neurologic disease
- i.e. iPS cells created from patient with microcephaly resulted in smaller clumps of cells
 - neural stem cells seemed to mature too quickly



Down Syndrome Cognitive Deficits Linked to Stem Cell Regulation

Michael Clark, Stanford, *Nature*, Sept. 11



- Gene found studying cancer is on Chromosome 21
 - Usp16 determines rate stem cells are depleted
 - Down patients' third copy results in stem cell loss as in aging
 - Lowering expression of Usp16 causes cells to behave more normally
 - This gene and its protein
are now therapeutic targets
- Unlikely only culprit gene*



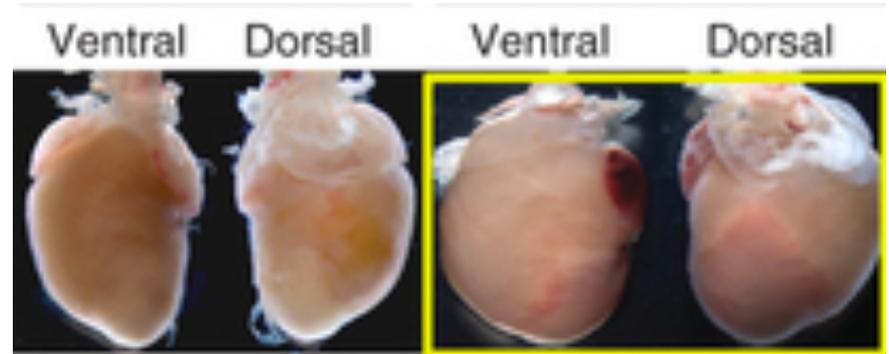
Genetic Manipulation Gets Mouse Hearts to Repair Themselves

Kenneth Chien et al. Harvard, *Nature Biotech*, Sept. 8



- VEGF a logical gene, it promotes new blood vessels
 - Prior studies of direct injection of raw DNA not good results
- Synthetic mRNA provided brief pulsed expression
- Reduced infarct size, improved survival
- Seems to mobilize native progenitor stem cells

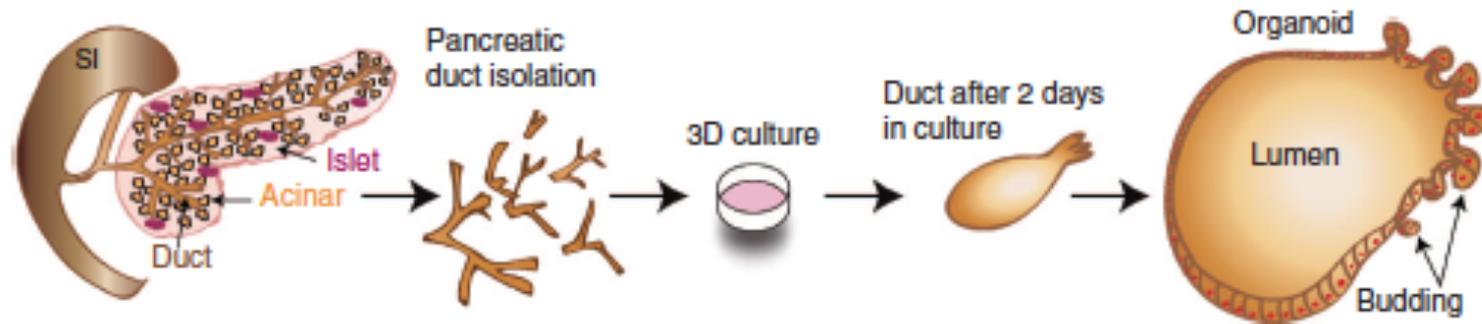
• Parallel study in *Cell Research* Sept. 10 showed similar effect in human cells



Pancreatic stem cells isolated in mice and shown able to produce two key tissues

Hans Clevers, Hubrecht Inst, Netherlands, *EMBO*, Sept. 17

- Activated progenitor cells by turning on key genes
- 3-D culture system allowed organoids to form
- Organoids could expand many fold
- Organoids could create Beta cells and duct cells



RFA Program

- Disease Team III
 - ICOC Funding Decision – December 2013
- Basic Biology V
 - ICOC Funding Decision – January 2013
- Genomics
 - GWG Review of Applications – November 2013
- Strategic Partnership III
 - GWG Review of Applications – February 2014
- Research Leadership Extension
 - GWG Review of Applications – March 2014



RFA Program

- Tools and Technologies III
 - RFA Posting – October 2013
- Alpha Clinics
 - RFA Posting – October 2013



Public Outreach and Engagement

- October 2nd Stem Cell Awareness Day
 - 20 events in 4 countries and 4 U.S. states
 - reached more than 4,500 high school students in CA
- Patient advocate day in LA
 - 25 joined in a roundtable discussion
- Town Hall: HIV Cure Research
 - @ 150 attended in SF



CIRM mini symposium: Breaking the Bottleneck: Deriving Definitive HSC from hPSC Aug 29, 2013



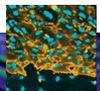
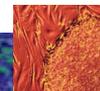
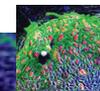
- Goal to define and discuss key scientific and technical bottlenecks preventing successful derivation of fully functional HSC from hPSC, and how CIRM might act to address these challenges
- Overcoming bottleneck would have significant impact not only on basic and developmental biology, but also on translation of stem cell science from bench to bedside for many hematological and non-hematological diseases, including inborn errors of metabolism and genetic diseases
- Presentations from 6 external thought leaders, including 4 CIRM investigators and panel discussion with CIRM scientific staff
- White paper to be produced by end of year: examples of recommendations include: consider as priorities in upcoming RFAs; allowing co-PIs in basic research grants and promoting collaborations with investigators external to California



CIRM works with FDA on regulatory pathway for cell therapy



- Regulatory Pathways: International Workshop on Cell Therapies, September 17, 2013 Bethesda, MD
 - CIRM-led international regulatory workshop, focus on N. American, European, and Japanese regulatory frameworks for developing cell-based therapies



CIRM works with FDA on regulatory pathway for cell therapy



- CIRM webinar on Moving cell based therapies to the clinic for Parkinson's Disease speakers from FDA, academia, industry
November 14, 2013 10 am to 12 noon Pacific
<http://www.cirm.ca.gov/our-funding/regenerative-medicine-consortium>

Speakers

- Wilson Bryan, M.D., Director, Division of Clinical Evaluation and Pharmacology/Toxicology, OCTGT, CBER, FDA
- Jeffrey Kordower, Ph.D., Professor of Neurological Sciences and Neurology, Rush University Medical Center
- Karl Johe, Ph.D., Chief Scientific Officer, Neuralstem
- White paper from CIRM sponsored PD workshop as reference



Business Development Update

- Stem Cell Meeting on the Mesa:
 - October 14 – 16th
 - Partnering Forum: Representatives from regenerative medicine companies; pharma and investment community and CIRM Funded programs
 - CIRM Funded Team participated in “pitch practice” with two VC’s providing input
 - Roundtable Meeting (Oct. 16th): follow up to the June Workshop on technology hurdle. Topics to include:
 - Building a Stem Cell Tool Kit
 - Suspension Culture for increasing titer





Finance Report

October 9, 2013

Financial Highlights for 13/14 FY

- Current Year OpEx 13/14 FY: \$2.0M
 - Prior FY OpEx 12/13 FY: \$1.8M

- Grant disbursements 13/14 FY: \$59.3M
 - Prior period 12/13 FY: \$39.1mm



Operating Expense Detail

Dollars in 000

	Jul 2013- Aug 2013	Jul 2012 - Aug 2012
Employee Expenses	1,857	1,622
External Services	42	139
Reviews, Meetings, Workshops	86	28
Memberships/Training	3	2
Travel	30	14
Equip/Supplies/Telecom/Software	17	8
TOTAL	2,034	1,813

Major drivers of OpEx variance vs. prior period:

- Employees: Increase from 53 to 56 FTEs



Audit/Cash Update

- 2012/13 Annual Financial Audit
 - Completed
 - Report by MGO
- Available cash as of Sept 30, 2013
 - \$61.4M



Donations

- \$1,000
 - Amalgamated Transit Union Local 1277
Los Angeles

