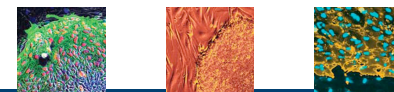


President's Report

Alan Trounson

August 2008

Agenda Item 6



New Developments in Stem Cells



iPS Cells Generated from Patients with ALS Can Be Pushed to Become Motor Neurons

Dimos et al, Science Express July 2008- Kevin Eggan's Lab, Harvard Stem Cell Centre

Generated iPS cells by retroviral transduction of four genes using skin cells of an 82 year-old woman diagnosed with a familial form of amyotrophic lateral sclerosis (ALS). The iPS cells were capable of differentiating into motor neurons, the cell type destroyed in ALS.

These cells may provide insight into the cause of ALS and the cellular and molecular mechanisms responsible for the phenotype. This could also lead to discovery of small molecule drugs that may treat the disease.



New Developments in Stem Cells



Highly Efficient, Functional Engraftment of Skeletal Muscle

**Cerletti et al, Cell 134, 37–47, July 11, 2008 – Amy Wagers lab,
Harvard Stem Cell Institute**

They used marker expression on mouse muscle cells to purify a distinct population of skeletal muscle precursors (SMPs) from among the satellite cells that function as muscle stem cells. When engrafted into muscle of dystrophin-deficient mice, purified SMPs contributed to up to 94% of myofibers, restoring dystrophin expression and significantly improving muscle histology and contractile function.

These data support the use of progenitor muscle cells for cell therapy in muscular dystrophy.



New Developments in Stem Cells



Embryonic Stem Cells and Umbilical Cord Blood-derived Progenitor Cells Differ in Ability to Reconstitute Immune Cells

**Martin et al, Blood July 2008 from D. S. Kaufman's lab,
University of Minnesota**

They found that hES cells form Natural Killer cells easily but are unable to form functional T cells, nor did they produce B cells. Similar umbilical cord blood cells routinely produced T and B cells in vitro. This demonstrates a fundamental difference that may have implications for immune reconstitution in disease states



New Developments in Stem Cells



Generation of Functional Murine Cardiac Myocytes From iPS Cells

Mauritz et al, Circulation July 2008 - Hannover, Germany

Stem Cell Differentiation of Cardiovascular Cells From Mouse iPS Cells

Narazaki et al, Circulation July 2008 – Yamanaka Lab, Kyoto

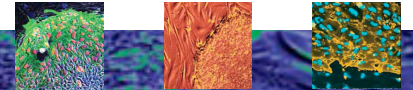
Back to back papers showing that iPS cells will form beating heart muscle cells of ventricular, atrial and pacemaker phenotypes when using methods developed for ES cells and have action potentials similar to that seen with ES cells



President's Priorities



- **CIRM 2008 Strategic Plan**
- **Translational and Disease Teams Research**
- **Discussions with For-Profit Biotech and Pharmaceutical Companies**
- **International Connections**
- **Development of CIRM Productivity Measures**
- **Full range of discussions with research institutes and key scientists**



President's Priorities



- **Update on International & National Linkages**

Signed MOUs

**Canadian Cancer Stem Cell Consortium
Victoria (Australia)**

Under Negotiation

**UK/Medical Research Council (MRC)
Juvenile Diabetes Research Foundation (JDRF)
Foundation for Fighting Blindness**

Initiated Talks

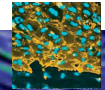
**Alliance for Gene Therapy
Germany
Japan**



President's Priorities

• Report on Predictive Toxicology Workshop: Potential Priorities for CIRM

- Chemical reference library needed: to provide a reference set for validating cell lines.
- Olympiad cell lines: Need additional human ES lines that are robust, can be reproducibly differentiated with high efficiency and show reproducible behaviors for use in screening assays. Should include iPS-derived lines for diversity and/or disease specificity.
- The most desired cell types for toxicity testing are hepatocytes, cardiomyocytes and neurons.
- Current differentiation protocols for do not yield the mature fully functional phenotype desired for toxicity testing.
 - 3D environment, other cell types required?
 - Other cell sources - amniocytes , adult stem cells, as a starting point?



President's Priorities



- **Report on Predictive Toxicology Workshop:
Potential Priorities for CIRM**

Pharmaceutical industry already using mouse ES cells – will likely gear up to hESCs if they have a suitable mature phenotype – area for CIRM support?

Environmental toxicity testing has some major toxicology assays available and public agencies are very keen to work with CIRM to develop effective cell model systems, reduce costs and reduce the use of large numbers of animals in screening systems



Grant Reviews



- **COMPLETED GRANT REVIEWS**

- **New Faculty II**

- ICOC Review August 12 & 13

- **UPCOMING GRANT REVIEWS**

- **Tools & Technologies (Double Review)**

- Sept 8, 9 & 10, 2008



RFAs



- **Training Grants II (CIRM Scholars)**
 - **20 LOIs**

- **Bridges to Stem Cell Research**
 - **23 LOIs**
 - 17 California State Universities
 - 4 Community Colleges
 - 2 Private Colleges



Upcoming RFAs



- **Translational Research I**
 - RFA Released – August 08
 - GWG Review – February 09

- **Disease Team Research Awards**
 - RFA Released - November 08
 - GWG Review - April 09



Proposed Workshops



- **Cancer Stem Cells – Aug 26, 08**
 - Address issues related to California/Canada Collaborations in Cancer Stem Cell
 - Caltech, Stanford, USC, UCSF, UCLA, UCSD, COH
- **IP Regs & Grant Writing Public Sessions**
 - Sept 11 – San Francisco & Sept 12 - San Diego
- **Cell Production Facilities – Nov 3, 08**
- **UK/MRC/CIRM – Jan 09** (tentative)
- **Immunology Tools – Feb 09** (tentative)



Science Meetings



- **CIRM 2008 Grantee Conference**

Sept 17-19, 08 San Francisco

- Bring together investigators and trainees that CIRM is funding,
- Highlight their research, and
- Encourage scientific discussion and collaborations.
- **The first Grantee Meeting will center around four topics:**
 - Challenges faced in achieving the functional integration of new cells into existing tissue
 - The relationship between stem cells, the immune system and cancer
 - Translating stem cell research into advances in human health
 - Pluripotency and cellular differentiation



Stem Cell Awareness Day



September 25, 2008

