Agenda Item # 11 6/2/06 Full-Day ICOC Meeting

California Institute for Regenerative Medicine

Background for Consideration Regarding the Mission Statement and Long-Term Objectives for the CIRM

ICOC Strategic Planning Meeting June 1, 2006

Mission Statement

To help guide our discussion, a number of sample mission statements are presented below for review. Also presented below is language taken primarily from Proposition 71 related to the mission of CIRM. This information is not intended to be definitive or restrictive in any way, but is designed to help frame our discussion.

Sample Mission Statements from Disease Focused Organizations

- Juvenile Diabetes Research Foundation: "To find a cure for diabetes."
- High Q Foundation (Huntington's disease): "To bring together academia, industry, governmental agencies, and other funding organizations in the search for Huntington disease treatments."
- Cure Autism Now: "Cure Autism Now believes that, with enough determination, money and manpower, science can be hurried so that answers are found sooner rather than later."

Sample Mission Statements from Broadly Focused Organizations

- Canadian Stem Cell Network: "To be a catalyst for realizing the full potential of stem cell research for Canadians."
- National Institute of Standards and Technology: "To accelerate development of innovative technologies for broad national benefit through partnerships with the private sector."
- Wellcome Trust: "Our mission is to foster and promote research with the aim of improving human and animal health."

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CIRM Mission Language

- From Proposition 71, Section 2, Findings and Declarations: "...to support stem cell research, emphasizing pluripotent stem cell and progenitor cell research and other vital medical technologies, for the development of life-saving regenerative medical treatments and cures."
- From Proposition 71, Section 3, Purpose and Intent: "[To m]aximize the use of research funds by giving priority to stem cell research that has the greatest potential for therapies and cures, specifically focused on pluripotent stem cell and progenitor cell research among other vital research opportunities that cannot, or are unlikely to, receive timely or sufficient federal funding, unencumbered by limitations that would impede the research.
- From Proposition 71, Section 4 (Article XXXV of the California Constitution, Section 2): "The institute shall have the following purposes:
 - To make grants and loans for stem cell research, for research facilities, and for other vital research opportunities to realize therapies, protocols, and/or medical procedures that will result in, as speedily as possible, the cure for, and/or substantial mitigation of, major diseases, injuries, and orphan diseases.
 - To support all stages of the process of developing cures, from laboratory research through successful clinical trials.
 - To establish the appropriate regulatory standards and oversight bodies for research and facilities development."
- "To support stem cell research and related technologies under the highest standards with the aim of developing treatments and the therapies for chronic disease and injury."

Long-term Objectives

The long-term objectives of CIRM will define our scientific and clinical goals for the ten years of the strategic plan. Where do we want to be in ten years and how will we judge out success? Possible long-term objectives that have been suggested so far, taken from the interviews we have conducted to date, the scientific conference, and informal conversations with scientists, clinicians, and ICOC members are presented below. These are presented as samples to spur discussion and not intended to be definitive or restrictive in any way.

<u>Clinical</u>

To have, in early stage clinical trials, new therapies based on stem cell research for several diseases.

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- > To establish "proof-of-principle" for stem cell therapy in humans for several diseases.
- To demonstrate a level of success for stem cell therapy that will attract the large investment from others that will be necessary to bring stem cell therapies to patients.
- > To develop approaches that will address immune rejection of transplanted tissue.

Translational

- To establish "proof of principle" for stem cell therapy in preclinical models in a variety of diseases.
- > To develop the use of stem cells for toxicity testing and drug discovery.
- To demonstrate the usefulness of disease-specific stem cells in target identification and discovery of therapeutics.
- To develop procedures for large-scale production of stem cells and their derivatives that will ensure their safety and efficacy.

<u>Basic</u>

- > To have a thorough understanding of the factors regulating stem cell self-renewal and differentiation.
- > To understand the early steps of stem cell differentiation *in vivo* and in *vitro*
- To develop methods for creating and maintaining stem cell lines with a variety of genotypes in an efficient and reproducible manner.
- > To produce disease-specific stem cell lines to understand the mechanisms of disease.

Infrastructure

- > To generate a skilled workforce for stem cell research in California through training programs and recruitment.
- To create facilities, technology cores, networks, and infrastructure that will support basic and clinical stem cell research in California.