Mission: Proposals Made During June 1 ICOC Discussion

➢PREAMBLE: In accordance with the mandate of the citizens of California, as specified in the California Stem Cell Research and Cures Act, the mission of the California Institute for Regenerative Medicine is to...

➤To support and promote stem cell research and related technologies with the aim of developing diagnostics, treatments and therapies (for chronic disease and injury) (to reduce human suffering and improve human health)

➤To mobilize and coordinate the very best scientists to discover and develop stem cell diagnostics and therapeutics for these diseases (as mentioned in Prop 71)

>...to develop cures and therapies using stem cell technologies to reduce human suffering and improve human health

Mission: Proposals Made During June 1 ICOC Discussion (cont'd)

- > ... stem cell therapies to advance regenerative medicine
- ...stem cell technologies and other vital research opportunities to advance regenerative medicine
- ...development of stem cell and related technologies for the treatment of serious human diseases or disorders

Proposed Mission Statements Derived from June 1 ICOC Meeting

- PREAMBLE: In accordance with the mandate of the citizens of California, as specified in the California Stem Cell Research and Cures Act, the mission of the California Institute for Regenerative Medicine is ...
- To support and promote (advance) stem cell research and regenerative medicine for the discovery and development of diagnostics, therapies, and cures to improve human health.
- To mobilize the very best scientists to discover and develop stem cell diagnostics and therapeutics for the prevention, treatment and cure of disease.
- To support and promote (advance) stem cell research and related technologies (under the highest standards) with the aim of developing treatments and the therapies for chronic disease and injury.

Mission: Written Comments Received after June 1 ICOC Discussion

- ➤To support and promote stem cell research and (other vital related) medical technologies for the development of lifesaving regenerative medical treatments and cures in order to reduce human suffering.
- …cures for intractable medical diseases
- ...advance regenerative medicine to reduce human suffering and advance human health
- ...reduce burden on health care system by providing cures for currently intractable diseases

CLINICAL: Proposed Long-Term Objectives*

- To have, in early stage clinical trials, new therapies based on stem cell research for several diseases.
- 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.
- To use stem cell and related research to develop new therapies and cures that are effective, safe and affordable

*Draft: For Discussion Purposes Only

Colored text are written comments subsequently received from ICOC members

TRANSLATIONAL: Proposed Long-Term Objectives*

- 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.

> Other...

BASIC SCIENCE: Proposed Long-Term Objectives*

- To have a thorough understanding of the factors regulating stem cell selfrenewal 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.
- ➤ Other...

INFRASTRUCTURE : Proposed Long-Term Objectives*

- 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.
- To create .. collaborative research, clinical and interdisciplinary networks and infrastructures
- To contribute to the public understanding of and education in stem cell science and its application
- To contribute to the international dialogue pertaining to medical and ethical standards for stem cell research
- To foster/facilitate international collaboration to further stem cell research and understanding

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Colored text are written comments subsequently received from ICOC members

<u>Goals / Long-term Objectives: Written comments</u> <u>received after June 1 ICOC discussion</u>

Advance the field of stem cell research

Catalyze participation/efforts of private and public organizations in the development of new therapies and cures for currently incurable diseases

Set the highest standards for scientific integrity, openness, collaboration and technology transfer

Improve the California health care system and reduce the long term burden of chronic disease

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<u>Themes for Objectives Derived from</u> <u>June 1 ICOC Meeting</u>

- Set aspirational / stretch goals.
- Reflect a sense of urgency in the objectives.
- > Employ measurable outcomes in the objectives.
- Consider how to address education and expectation management related to CIRM's objectives.
- Provide periodic reports on outcomes and progress against objectives.
- Construct objectives that permit CIRM to leverage resources as appropriate.