

**California Institute for Regenerative Medicine  
Meeting of the Independent Citizens Oversight Committee**

**DRAFT Long-Term Objectives for Discussion  
with the ICOC on August 1 and 2, 2006**

Presented below for discussion is a draft of the long-term objectives for the CIRM strategic plan, which were developed based on discussions with the ICOC held on June 1-2, 2006:

**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.
- Catalyze participation/efforts of private and public organizations in the development of new therapies and cures for currently incurable diseases
- Improve the California health care system and reduce the long term burden of chronic disease

**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.

**BASIC SCIENCE: Proposed Long-Term Objectives**

- 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: 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.