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CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
The state stem cell agency

Ethics and Regulatory recommendation

IOM Recommended CIRM fund research and training on ethical and regulatory issues. IOM recommended CIRM should sponsor training programs and workshops and offer new grant opportunities aimed specifically at identifying and addressing ethical and regulatory issues surrounding stem cell-based clinical trials research. CIRM should use the information resulting from these initiatives, together with current knowledge, to strengthen its ethical standards for CIRM-funded human subjects research based on sound empirical and theoretical grounds**.**

Legal Assessment: The proposal for funding (e.g., RFA or a supplement to leverage existing initiatives) would be treated as any other funding initiative requesting additional funds. Workshops may occur without board approval if within budget.

Response:
Management would establish initiatives on ethical and regulatory issues that relate to human subjects research.

CIRM gathered perspectives from the Standards Working Group members, CIRM funded researchers and other experts to the IOM recommendations concerning ethics and public policy programs, and considered their input in proposing the following:

I. Basic Research / iPS Research & Biological Repositories:

A. Legal and ethical rights of somatic cell donors and material sharing in iPS research;

This is a pressing topic as patient donors have greater expectations in the research enterprise. For example, patients desire to be informed of discoveries made with their lines. Patients and patient groups want to ensure that discoveries benefit the community, and that access barriers, such as patents and restrictive licenses, are not erected. Further, advocacy groups have interest in benefit sharing that can support patients.

Approach: Workshop and Extramural Research to:

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- Define the needs and expectations of different donor populations (patients, controls, disease groups)
- Provide policy analysis to consider existing constraints faced by researchers and research institutions
- Identify model approaches for donor involvement that are consistent with the established regulatory framework for donor protection and privacy (Common Rule / HIPAA)
- Identify policy needs or approaches to enable a more robust donor involvement
- Identify tools for enabling greater flexibility (e.g. Portable Legal Consent for Common Genomics Research)
- Consider steps to be taken in the context of cell repositories and genomics research to reduce the likelihood of donor identification

Among additional areas that may merit further ethical and regulatory considerations:

Embryo donation and stem cell research

The need for a bank of hES cells for compatible transplants

II. Priorities for the ethical conduct of human clinical trials research and identify strategic focus areas;

The issue of clinical trials and alpha clinics is nuanced and complicated. Consider a process similar to the Disease Team Program where a workshop was used to inform the development of an RFA for extramural research. The workshop could address the following questions:

- What do experts perceive to be the issues with clinical trials – this can be done with some general guidance in what CIRM thinks needs addressing
- How can ethics considerations be addressed prospectively, for example, in the context of GWG review (do we need to do more up front such as pre review)
- What are patients looking for and what are their expectations with regard to therapies (can we develop systems that provide alternatives to "rogue clinics" or become the standard of care to be adopted broadly)
- What is the best mode of communication, especially in cases where the situation is not as acute as an emergent injury or sudden onset of disease
- How can patient advocates and provider networks interact to meet patient needs and what are effective models for bilateral communication and stakeholder engagement

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- How can advisory boards, with representation from the community, support clinical research and patients

Funding should support the identification of model systems that have a proven track record in addressing these issues and questions. Look at previous clinical trials or clinical interventions especially those employing community-based approaches.

Other areas to consider:
Treatment of athletes and young people with sports injuries with stem cell therapies – adipose and bm cells;
The role of California clinicians in stem cell tourism
Patient advocacy role in regulatory approval
Harmonization of international regulations
Consideration of accelerated approval for some types of cell therapies

DRAFT: Options for Addressing IOM Recommendations Bioethics Key Questions