How California Institute for Regenerative Medicine Research Programs Support Science and Regulatory Policy

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INTRODUCTION

Effective science policy has been central to the development of stem cell research. The California Institute for Regenerative Medicine (CIRM) is a case in point. The institute exists because more than 7 million California voters endorsed Proposition 71 in 2004. In parallel, related and complementary national and international policy initiatives have been developed, contributing to accelerated growth in the field. As an example of this growth, between 2004 and 2010, the annual rate of “stem cell” publications increased more than 30-fold [1].

CIRM is cognizant of the critical roles that science and regulatory policy play in advancing stem cell research. The institute sustains a programmatic focus on state, national, and international regulatory policy issues impacting basic research and translational medicine. CIRM deploys a variety of mechanisms including conducting policy research, convening advisory committees, and establishing collaborative partnerships. This focus is vital for advancing the institute’s mission to support the development of safe and effective therapies, with the long-term goal of improving health care.

RESOURCE AND INFORMATION SHARING SYSTEMS ARE VITAL

Success in advancing this mission depends, in part, on the development of systems to enable researchers to effectively and efficiently share resources, data, and knowledge. CIRM funds 12 Centers of Regenerative Medicine and is creating new capacity in targeted areas, including:

- an induced pluripotent stem cell bank for disease modeling and drug discovery,
- a center of excellence in stem cell genomics and bioinformatics, and
- a clinical trials network for evaluating stem cell–based investigational therapies.

Each of these initiatives is specifically designed to catalyze innovation by creating resource and information hubs for the broader research community. We believe this approach can serve to (a) empower patients and the public to make informed decisions, (b) accelerate rigorous testing and development of safe and effective therapies, and (c) engage the broad community through coordinated progress toward medical innovation and clinical research.

CIRM INITIATIVES ARE CONCORDANT WITH LARGER POLICY EFFORTS

There is an emerging consensus on the value of information sharing, especially in clinical research. Public, private, and nonprofit research organizations are increasingly open to clinical trial data sharing [2–3]. Data-sharing initiatives are a logical way to address the scientific and economic challenges associated with clinical studies, providing a mechanism for increasing cost-effectiveness and efficiency through accelerated learning processes, whereby community members can learn from others’ successes and failures.

The Institute of Medicine (IOM) recently constituted the Committee on Strategies for Responsible Sharing of Clinical Trial Data. The committee is charged with developing principles and a framework for the sharing of clinical trial data. As a first step, the committee published a discussion framework document to elicit public comments.

PROSPECTIVE STEM CELL TRIALS REQUIRE SHARING OF QUALITY DATA

CIRM submitted comments to the IOM committee. These comments, summarized below, reflect our commitment to supporting and enabling high-quality stem cell discoveries to be brought to clinical testing. We view these comments as particularly pertinent to data sharing and information management needs arising in the context of...
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CIRM’s emphasis on clinical trial data sharing has been influenced by research performed during the lead-up to the release of the Alpha Stem Cell Clinics Request for Applications in 2013. CIRM engaged in discussions with individuals and organizations with expertise in clinical networks, patient advocates, and other interested parties, and a number of key messages emerged.

- **Data sharing will accelerate research:** The scientific and technical challenges involved in the development of cellular therapies can be best addressed through collaborative research efforts. There is a range of cross-cutting issues relating to cell product development, delivery systems, host immune response, and patient surveillance that require robust systems for data sharing.

- **Quality first:** Clinical studies generate large volumes of heterogeneous data ranging from discrete measurements to extensive annotations. These data will vary across various dimensions, including their precision, validity, reproducibility, and generalizability. Optimal approaches would involve aggregation of quality-controlled data that are accurate and reliable.

- **Focus on information management systems:** Scientists and clinicians increasingly require access to systems that enable the interrogation of “big data.” Management systems should not only enable data access but also support the development of tools and algorithms and allow for their reuse. The development and sharing of queries and algorithms may serve to accelerate discovery and advance safety and clinical efficacy across a range of health outcomes. Furthermore, depositing clinical data in appropriately administered information systems can effectively address regulatory concerns regarding privacy, confidentiality, and security.

- **De-risk data sharing:** Generators of clinical data will be less likely to participate if they perceive data sharing to be risky. An optimal data dissemination system would have governance procedures that de-risk participation for trial sponsors. De-risking requires an explicit acknowledgment that certain data may be made routinely available, some may be made available only to qualified investigators, and some may remain proprietary. Procedures should be set in place to develop appropriate protocols for data deposit and access.

- **Consider incentives for participation:** Consideration should be given to mechanisms for rewarding the deposit of data. For example, performance metrics for monetizing the deposit of high-quality data should be considered. Like an investigator’s publication record, such a system could be weighted in peer review and progress reporting. Monetization should also be evaluated from the perspective of system quality and sustainability. If quality data can serve to reduce sponsor costs, then reasonable fees or royalties may be appropriate. Contract research organizations have extensive experience and expertise in data generation, analysis, and management, but funding constraints may not allow them to participate in data sharing. Again, reimbursement mechanisms may bridge this gap.

**CONCLUSION**

The IOM Framework for Clinical Trial Data Sharing is an important policy discussion for CIRM. Organizationally, CIRM looks forward to applying lessons learned from its scientific programs to support evidence-based policy development. In the context of clinical trial data sharing, CIRM looks forward to applying lessons learned from clinical programs to support the creation of safe and effective therapies.

**AUTHOR CONTRIBUTIONS**

G.P.L., N.D.D., M.T.M., and E.G.F.: manuscript writing, final approval of the manuscript.

**DISCLOSURE OF POTENTIAL CONFLICTS OF INTEREST**

The authors indicate no potential conflicts of interest.

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