



M E M O R A N D U M

TO: INDEPENDENT CITIZENS' OVERSIGHT
COMMITTEE (ICOC)

FROM: ELONA BAUM

SUBJECT: FINAL ADOPTION OF REGULATIONS FOR THE hPSC
BANKING INITIATIVE

DATE: MAY 23, 2013

I. Executive Summary

On December 8, 2011, CIRM's Governing Board ("ICOC") approved a concept plan for deriving and banking a comprehensive collection of disease specific human induced pluripotent stem cells (hiPSC). To implement this initiative, the Board also approved in 2012 interim intellectual property regulations to govern this unique program. Because interim regulations expire if not adopted permanently, staff initiated permanent adoption of the regulations to solicit public input. With the period for public comment now closed, the regulatory adoption process is nearly complete. The regulations (attached as Exhibit A), which are essentially unchanged from the interim regulations already approved by the Board, are now ready for final adoption at this meeting.

II. Background

Late in 2011 the ICOC approved a concept plan for deriving and banking a comprehensive collection of disease specific human induced pluripotent stem cells (hiPSC). This effort is funded through three separate Requests For Application (RFA) at a total cost of up to \$30M. These lines will serve as valuable tools in drug discovery and will be available to researchers worldwide. The Tissue Collection RFA No. 12-02 will fund clinicians and other scientists to identify, recruit and consent sufficient numbers of affected individuals within a disease population so as to effectively represent the disease's manifestations. Tissues will be collected and appropriate clinical, medical or diagnostic information, will be obtained to enable informed discovery of disease-related phenotypes and drug development activities using hiPSC-based models. These tissue samples will be provided (without charge) to the recipient of the CIRM hiPSC Derivation Award (RFA No. 12-03) for the production of the hiPSC lines. Once derived, characterized and released, the lines will be deposited in the CIRM hiPSC bank funded under RFA No. 12-04. The ICOC rendered awards under these initiatives in March of this year.

On May 8, 2012, the Intellectual Property and Industry Subcommittee ("IP Subcommittee") considered proposed interim regulations governing the hiPSC initiative. By a unanimous vote of the members present, the Subcommittee passed a motion recommending that the ICOC approve the proposed interim regulations. Since the IP Subcommittee meeting, the publication regulation was changed to include specific language

adapted to address the unique nature of this funding initiative rather than incorporating CIRM's IP regulations (defined below) in whole. In addition, the prohibition on exclusive licensing will be set forth in a Deposit Agreement between the hiPSC Repository and CIRM. The ICOC subsequently adopted the subcommittee's recommendation and adopted the proposed regulations on an interim basis and directed staff to begin the formal process for permanent adoption.

III. Need for a Different Regulatory Scheme

Under CIRM's current regulatory framework, awards are made in the form of a grant or a loan. If they are grants, CIRM's Intellectual Property and Revenue Sharing Regulations for For-Profit and Non-Profit Entities ("IP regulations") apply (as do other CIRM regulations such as the Grants Administration Policy). If they are in the form of a loan, then CIRM's Loan Administration Policy applies in addition to all provisions of the Intellectual Regulations, excluding Section 100608. The IP Regulations were drafted to address conventional drug discovery activities and did not contemplate creation of a comprehensive repository of cell lines intended for broad distribution. As a result, the IP Regulations contain a number of provisions which are either not applicable or worse could impede the success of the hiPSC bank. For instance, IP Regulations permit the exclusive licensing of CIRM funded inventions and technology. This would be counter-productive to the goals of the hiPSC repository which are predicated on wide spread access. Similarly the IP Regulations include provisions relating to revenue sharing. However, these regulations would have no practical effect for RFA Numbers 12-02 and 12-03 as the grantees under these awards are not entitled to charge a fee for the materials they transfer pursuant to the award. With respect to the repository, it is important to have flexibility to negotiate in a Deposit Agreement any terms for revenue sharing as the primary objective is to ensure that the cell lines are made available at low cost while at the same time ensuring the repository is self-sustaining. Provisions in the IP Regulations that warrant inclusion, such as the requirement relating to acknowledgment of CIRM in publications resulting from us of the cell lines, are maintained in the proposed regulations.

IV. Summary of Proposed Regulations

The proposed regulations provide, in pertinent part, the following:

- (i) Exempt from IP Regulations: Grantees will be exempt from the IP Regulations. These regulations are not consistent with the objectives of this initiative and if applied could actually be counter-productive.
- (ii) Ownership of the hiPSC Lines: Although the lines will be deposited in the repository, CIRM will be the actual owner of these cell lines. This permits CIRM to have complete control of this valuable resource and is consistent with the practice of NIH's Center for Regenerative Medicine, which is also creating a repository for iPSC lines and derived materials. (Note: under the existing IP Regulations the Grantee would own the lines.)
- (iii) Revenue Sharing and Pricing: As neither the awardee of the tissue generation RFA nor the awardee of the derivation RFA will be permitted to sell the materials they create with CIRM funding, there will be no revenue sharing regulations which apply to them. The repository will be permitted to charge a reasonable fee for the lines. However, to ensure the repository can become self-sustaining by the end of the project term, application of current revenue sharing provisions in the IP Regulations would likely be counter-productive. The Deposit Agreement between CIRM and the grantee of the repository RFA will set forth agreed to terms relating to revenue sharing and pricing.

- (iv) Publication: Researchers using lines from the CIRM funded repository would be required to acknowledge CIRM's funding.

V. Recommendation

Staff recommends the ICOC adopt the proposed regulations attached hereto as Exhibit A.

1 Adopt 17 Cal. Code of Regs. Chapter 9, section 100900 to read:

2 **Chapter 9. Repository for Induced Pluripotent Stem Cells**

3 **and Embryonic Stem Cells**

4 **§ 100900. Definitions.**

5 (a) hPSC Repository. The repository established pursuant to CIRM Request for
6 Applications 12-04.

7 (b) hPSC Bank RFAs. The Request for Applications, numbers 12-02, 12-03 and 12-04,
8 that provide funding for the creation of disease specific human induced pluripotent stem cells
9 and a repository and distribution center for storing cell lines.

10 (c) Miscellaneous. All other capitalized and undefined terms shall have the meanings as
11 set forth in CIRM's Intellectual Property and Revenue Sharing for Non-Profit and For-Profit
12 Grantee regulations (17 Cal. Code of Regs. sections 100600 et seq.) and CIRM's Grants
13 Administration Policy for Academic and Non-Profit Institutions (17 Cal. Code of Regs. Section
14 100500).

15 Note: Authority cited: Article XXXV, California Constitution; Section 125290.40(j), Health and
16 Safety Code. Reference: Section 125290.30, Health and Safety Code.

1 Adopt 17 Cal. Code of Regs. Chapter 9, section 100901 to read:

2 **§ 100901. Application of Specific CIRM Policies.**

3 (a) Intellectual Property. Awards made pursuant to the hPSC Bank RFAs are exempt
4 from CIRM's Intellectual Property and Revenue Sharing for Non-Profit and For-Profit Grantees
5 regulations (17 Cal. Code of Regs. sections 100600 et seq.).

6 (b) Grant Administration. CIRM's Grants Administration Policy for Academic and Non-
7 Profit Institutions (17 Cal. Code of Regs. section 100500, as amended) shall apply to awards
8 made pursuant to the hPSC Bank RFAs (12-02, 12-03, 12-04).

9 (c) Scientific and Medical Accountability Standards. CIRM's Scientific and Medical
10 Accountability Standards (17 Cal. Code of Regs. sections 100010 et seq., as amended) shall
11 apply to awards made pursuant to the hPSC Bank RFAs (12-02, 12-03, 12-04).

12 Note: Authority cited: Article XXXV, California Constitution; Section 125290.40(j), Health and
13 Safety Code. Reference: Section 125290.30, Health and Safety Code.

1 Adopt 17 Cal. Code of Regs. Chapter 9, section 100902 to read:

2 **§ 100902. Ownership of Intellectual Property and Materials.**

3 (a) All intellectual property of Grantees, Collaborators, and their subcontractors owned
4 prior to engaging in the activities set forth in the hPSC Bank RFAs (12-02, 12-03, 12-04) shall
5 remain the property of the Grantees, Collaborators, and their subcontractors. CIRM shall have
6 no rights to pre-existing intellectual property.

7 (b) CIRM shall own all hiPSC lines generated pursuant to the hPSC Bank RFAs (12-02,
8 12-03, 12-04) and shall retain all rights of such ownership including but not limited to rights to
9 license or otherwise transfer.

10 Note: Authority cited: Article XXXV, California Constitution; Section 125290.40(j), Health and
11 Safety Code. Reference: Section 125290.30, Health and Safety Code.

1 Adopt 17 Cal. Code of Regs. Chapter 9, section 100903 to read:

2 **§ 100903. Revenue Sharing.**

3 CIRM may negotiate revenue sharing provisions with the hPSC Repository, taking into
4 account the interest in ensuring that the hPSC Repository becomes self-sustaining.

5 Note: Authority cited: Article XXXV, California Constitution; Section 125290.40(j), Health and
6 Safety Code. Reference: Section 125290.30, Health and Safety Code.

1 Adopt 17 Cal. Code of Regs. Chapter 9, section 100904 to read:

2 **§ 100904. Publication.**

3 A researcher using a cell line obtained from the hPSC Repository that was derived in
4 whole or in part using CIRM funding shall, in any written publication and oral presentation,
5 acknowledge CIRM's funding and identify by name the hPSC Repository.

6 Note: Authority cited: Article XXXV, California Constitution; Section 125290.40(j), Health and
7 Safety Code. Reference: Section 125290.30, Health and Safety Code.