# Study Group Working Notes #2: Banking CIRM Funded Cell Lines

Study Group: Banking

Reference: Section 100008

Background: The Standards Working Group and the public have identified the need for cell-lines, cell products, and other materials derived using CIRM funding to be made available. Public comment have centered on concerns that materials derived with CIRM funds be widely available and not patented for exclusive use or profit-making. In response to this need, the Standards Working Group agreed that CIRM materials should be in public domain and included the following language in section 100009 of the Interim Regulations:

*Cell lines derived or modified in any way with CIRM-funds are required to be shared through a well recognized stem cell bank that will make the lines widely available to investigators.*

Subsequently, CIRM has received comment that there are technical challenges related to the centralized banking of cell lines. One concern is that the maintenance of cell lines is challenging. Experience suggests that quality tends to be highest when cells are maintained in the laboratories where they were derived. In time, the art of maintaining cells lines may evolve to the point where central banks can achieve an equivalent standard of quality.

Further the SWG felt it was not appropriate to include all the specific banking requirements in the existing NA Guidelines, so the following language was included in the Interim Regulations.

*Institutions engaged in CIRM-Funded hES derivation or research shall be encouraged at present and possibly mandated in the future to create or participate in central repositories for hES cell lines, including through partnerships or augmentation of existing quality research cell lines repositories, and shall adhere to high ethical, legal, and scientific standards consistent with Section 100009(a) and Section 100007.*

Options: The Standards Working Group and participants in the public sessions are looking for assurances that CIRM-funded research will result in high quality materials being available in the public domain to support additional research and ultimately effective therapies. A critical question at this juncture is **how can this goal be realized most effectively**?

Two questions have emerged in relation to this goal (1) is a central bank the most effective means of ensuring high quality materials are available and (2) what specific steps should be taken to promote sharing?

Question 1: There is anecdotal evidence to suggest that a central bank may not be the most effective mechanism in the near term for storing cell lines. Should the SWG consider focusing on the goal of sharing cell lines without mandating the exact mechanism for sharing?

Question 2: Currently, the interim guidelines *encourage but do not require* a variety of specific steps be taken to support material documentation, exchange and quality control – see section 100009. The draft Recommended Regulations do not contain this language. Many of the specific steps described in this section may be included as part of the cooperative agreement between CIRM and its grantee institution(s) rather than detailed in the CIRM guidelines. Also issues of who has access to the materials, and what the intellectual property arrangements will be.

Why are these details better suited for the cooperative agreement?

- Many of the specific steps identified in 100009 are more accurately described as best practices for material and data management. Such practices generally change over time requiring flexibility incompatible with the rulemaking process required to revise regulations. Therefore, more flexible cooperative agreements should be the vehicle for detailing CIRM's expectations for banking and distribution.

- The power of the purse is what gives CIRM enforcement ability. The CIRM Science Office will be providing direct oversight of grantee agreements. Therefore, the Science Office, through its agreements with funded institutions, is best positioned to enforce these agreements.

Should the draft recommended regulations continue to omit the detailed language?

Recommendation and/or Proposed Language:

Question 1:

Require sharing which would not preclude a cell bank, but also do not prescribe a bank.

*Cell lines derived or modified in any way with CIRM-funds are required to ~~be shared through a well recognized stem cell bank that will make the lines~~ widely available to [California?] investigators.*

Language could be included to ensure sharing protocols are *satisfactory* to CIRM. However, the details could be covered in the CIRM Grants Policy.

Question 2: The Standards Working Group should consider regulatory language that makes their intent explicit, but defers any specific requirements related to material documentation, exchange and quality control to the CIRM. CIRM would be in a position through its grants program to establish requirements related to material documentation, exchange and quality control as such language becomes available. The following amendments to the Interim Regulations should be considered.

*CIRM-funded research involving the derivation of cell-lines, cell-derived materials or related products should be shared ~~through a CIRM-approved stem cell bank~~*

*~~intended~~ to make the cells and materials widely available to investigators. The CIRM-funded institutions shall adhere to CIRM requirements for ~~participation in central repositories~~*

The additional question remains, to what extent can an open-source bank address concerns about patenting and intellectual property. For example, does the requirement that materials be deposited in the bank, alleviate public concerns that materials may fall under exclusive use or be unaffordable to the public?