

Report of the SAB

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President

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Agenda Item #6

Presentation re SAB/Management

Recommendations - 1

- The SAB advises CIRM to identify, through a prioritization process, the top 6 to 8 projects, with clear relevance to the remit of CIRM's stem cell mission, *and to set aside the funding to ensure the projects can proceed to phase 1 and 2a clinical trials as rapidly as possible, without financial impediments.*
- Agreement will cement CIRM reputation, achieve primary strategic goal and provide the necessary verification of Prop 71. Also recommended independently by President's Business Advisory Group.
- SP 5 yr Goal VIII: CIRM will have funded 10 therapies in phase I or II clinical trials, in at least 5 different therapeutic areas, based on stem cell research.

Presentation re SAB/Management Recommendations - 2

- The SAB recommends continued funding of training programs at all levels to develop a work force of trained individuals, which will be valuable as cell therapies burgeon
- Continue to enable human resources to expand in California with likely new industries/ companies, manufacturing and clinical trials, reagents for the field, equipment, spin-offs and expanding research strengths

Presentation re SAB/Management Recommendations - 3

- the SAB does not recommend continued funding of the 17 shared labs; these should operate on a revenue neutral basis; although essential as a safe haven during NIH funding ban, *the importance of these resources to CIRM's mission and achieving sustainability of earlier investments is not as compelling.*
- While recognizing the shared labs have helped to train and equip new scientists in general, stem cell derivation handling and differentiation procedures – institutions should continue them as revenue neutral or fold them into the institute/center general infrastructure for general training activities.

Presentation re SAB/Management

Recommendations - 4

- recommends continued support for basic research, but *felt restriction of CIRM funding in some RFAs to projects using only human cells was too prescriptive*, and doesn't take into account the benefits that model organism research can offer.
- Human cells rather than priority from the beginning, as these are systems more closely mimic the human condition, by their nature are more complex to develop and traditionally have not been well supported by NIH. Innovative ideas that could be demonstrated with research on model systems have been included in Basic Science RFAs in recent years. Management believes we should continue to emphasize study of human cell systems, but potentially transformative studies using other organisms also will continue to be eligible for support.

Presentation re SAB/Management Recommendations - 5

- The SAB had a very positive view of interactions between CIRM and the commercial sector.
- They noted an advantage of leveraged funding from the commercial sector of externally validating the quality of science and the likelihood of success.
- *They also recommended that for the prioritized set of projects, that it is important to ensure they can be funded without requiring matched leverage funding until after proof-of-concept (phase 2a) when successful programs should readily obtain external support.*

Presentation re SAB/Management Recommendations - 6

- how would you best make the case that CIRM was a great innovation in public funding of cutting edge science and whether it has delivered, and could continue to deliver in the future, value to the citizens of California and to the field of regenerative medicine?
- advancing a project to *successful achievement of clinical proof of concept will be important to making this case to the public*. Careful selection of these projects, and effective support, will be key to showing that CIRM is delivering on its promise.