PRESIDENT'S LETTER

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Few things are more fulfilling than setting goals and seeing them met—and to have a panel of outside reviewers agree that CIRM has been a real success. • In December 2006, our Governing Board adopted a strategic plan with very ambitious five-year and 10-year goals for the agency. These goals were set a year prior to my arrival at CIRM and to be frank, when I looked at the goals the first time I thought several of them might be a stretch in the given time frame, and one or two still may be difficult to achieve. But the progress in the past three years has certainly exceeded my initial expectations. • The 2006 plan called for a review of progress after three years, so during the second half of 2010, agency staff undertook a thorough self-assessment followed by an extensive review by an eight-person external advisory panel (EAP) made up of world leading stem cell researchers, science policy experts, and patient advocates and an ethicist (names of the panelists can be found here: http://www.cirm.ca.gov/Announcement_092810). Staff presented their self-assessment to the EAP in October and the panel presented its findings to our board in December. • We found that half the 10 five-year goals had already been met, including creating new methods of making stem cell lines. The remaining five-year goals are on a plausible track for completion in the next year or two, well within the five-year window. Also, CIRM grantees have published papers advancing progress toward nearly all 10 of the 10-year goals, including the ultimate goal of seeing embryonic stem cell–derived cells in clinical use. They have made sufficient progress to believe that these goals are achievable. This finding was hugely satisfying, as was the assessment of the EAP regarding the agency's early years: • "Progress during this first stage of CIRM's development has been remarkable; CIRM has built significant additional research capacity in the state, has attracted scores of talented young people to stem cell research, and has catalyzed large and important stem cell developments across the state. The EAP was most impressed with this rapid startup, the overall quality of the scientists and projects that have been funded, the development of major buildings and other facilities for stem cell research, the forging of a raft of important international partnerships and the innovative training programs that are in place."

However, the report doesn't leave time for CIRM to rest on these laurels. The panel provided a number of recommendations for CIRM to accelerate the field by making its funding more flexible, opportunistic and able to quickly respond to major discoveries, particularly those that are close to the clinic. The panel made some very thoughtful and helpful recommendations that will enable CIRM to deliver its mandate by becoming an even more effective organization.

The EAP report voiced confidence in CIRM's ability to be nimble and make the adjustments suggested. "The EAP feels confident that CIRM is poised to build on the success of the first stage to drive further growth toward its long-term mission of providing significant health and economic benefits to the people of California."

The panel grouped its findings into 10 key recommendations. Since December, CIRM's management team has been drafting a new operations plan that will spell out the tactics it proposes for carrying out those recommendations. We presented that plan to our board in March and here I would like to walk you through some of the

most important recommendations and our preliminary plans for carrying them out.

The first two recommendations simply encourage us to maintain the scientific excellence of what we fund and to continue to sustain fundamental discovery by supporting the most creative basic science. These simply require us to maintain the high standards and effective systems we have in place thanks to the highly dedicated members of our science office team.

In paving a path from fundamental to translational research the panel called for CIRM to develop a more aggressive, proactive approach to identify innovation across the whole therapeutic landscape. We believe we can accomplish this by more aggressively reviewing "hot areas" of breaking science and using the insights of an industry advisory group and of our collaborative funding partners around the globe. Once we find a hot spot of innovation we will need to move quickly to identify suitable California partners and arrange linkages no matter where the hot spot is.

The EAP said CIRM needs to create a process for prioritizing its portfolio, particularly its therapeutic candidates. We need a way to use expert advice to identify which programs deserve continued support and which do not. Our outside grant review panels' consideration of "relevance," along with the milestone/progress committee we are setting up, could go a long way toward setting priorities most likely to result in broadly adopted therapies. Relevance is a term used by industry to measure clinical impact on patients combined with a reasonable business/practice model for delivery.

Creating a "porous pipeline," the EAP said, would allow potential clinical projects to come from either inside or outside of CIRM's current funding, or even from outside of California. We believe we should be able to create more flexible funding processes that add a rolling funding cycle that could capture innovative projects at the time they are most ripe for support rather than only within set Request for Application schedules.

The panel asked that we not ignore social, ethical, regulatory and health care delivery issues, saying we should stimulate the research that is needed to move the field to everyday practice. To this end we are considering creating an advisory group to identify critical issues in these areas and hope to take a leadership role in developing standards for manufacturing and cell integrity for clinical use. We are also studying the possibility of establishing CIRM-sponsored clinical units for the delivery of cell therapies.

The EAP saw enabling more significant engagement with industry as critical to the next phase of CIRM. We were told we needed to be more accommodating to industry timelines and financial restraints. In response we will be looking for ways to streamline our grants award processes and developing mechanisms for industry to more quickly seek grant review. We will be increasing the number of industry reviewers on our grant review panels and we will be developing ways to encourage industry to use the patents and other intellectual property created by CIRM-funded projects.

The EAP also suggested we broaden our collaborative funding partnership program beyond the current nine countries and three U.S. states and foundations. We will be looking for ways to encourage multiple international and interstate partnerships on projects. At the same time we will continue looking for additional hubs of excellence around the world that can be added to the current network. We are also exploring options for partnering with the National Institutes of Health Clinical Center and its newly created National Center for Advancing Translational Sciences.

CIRM's efforts to increase its education and outreach activities with the public were strongly encouraged by the committee, which called for a significant increase in the breadth of this work. We intend to expand a program we began last year using a team of patient advocate outreach coordinators to work directly with the many disease-specific groups in the state. We also hope to enlist more of our grantee researchers in public education projects and expand our work with media outlets of all types.

The last recommendation from the EAP, to re-examine the roles of the Governing Board and CIRM managers, is an ongoing process. Our board is actively engaged in looking at the criteria its members believe are the top credentials for a new chair to succeed our visionary founding chair, Robert Klein.

As we enter CIRM's "phase II," looking for ways to implement these recommendations, it is clear to all of us at the agency that we are taking on these sometimes daunting tasks for one reason: to fulfill the mission of CIRM to accelerate the development of new therapies for patients. That is what we owe the voters of California. Thank you for your support.

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