

# California Institute for Regenerative Medicine

## 2015 SLAA REPORT

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December 31, 2015

Michael Cohen, Director  
California Department of Finance  
915 L Street  
Sacramento, CA 95814

Dear Mr. Cohen,

In accordance with the State Leadership Accountability Act (SLAA), the California Institute for Regenerative Medicine submits this report on the review of our systems of internal control and monitoring processes for the biennial period ended December 31, 2015.

Should you have any questions please contact C. Scott Tocher, Deputy General Counsel, at (510) 340-9159, [stocher@cirm.ca.gov](mailto:stocher@cirm.ca.gov).

### **BACKGROUND**

In 2004, California voters approved Proposition 71, the California Stem Cell Research and Cures Initiative. The statewide ballot measure authorized issuance of \$3 billion in general obligation bonds to finance funding for stem cell research and dedicated research facilities at California universities and research institutions. Proposition 71 created CIRM as a new state agency to administer the funding. CIRM is a small agency now staffed by approximately 50 FTE.

In authorizing these funds, Californians expected to speed the delivery of stem cell treatments and cures to patients with unmet medical needs, including a priority for funding pluripotent and progenitor cell research that was not receiving timely or sufficient federal funding. Additional potential benefits to Californians include propelling California into a leadership position in regenerative medicine, establishing California as the premier international location to advance stem cell medicine, stimulating the economy, reducing health care costs by replacing chronic treatments with cures, and ensuring that the State has the opportunity to benefit from the potential receipt of royalty payments arising from CIRM-funded treatments or technologies.

CIRM is governed by the Independent Citizen's Oversight Committee (ICOC), a 29-member board appointed by various state officials according to criteria specified in Proposition 71. ICOC members are public officials, appointed on the basis of their experience earned in California's leading public universities, non-profit academic and research institutions, patient advocacy groups, and the biotechnology industry. In addition to its fiduciary responsibility to the people of California, the Board is charged with: (1) adopting scientific, medical, ethical, and intellectual property policies; (2) making final funding decisions on grant and loan awards; and (3) providing oversight of CIRM. The mission of CIRM is to accelerate stem cell treatments to patients with unmet medical needs. CIRM does so pursuant to the highest ethical and medical standards, and seeks to discover and develop cures, therapies, diagnostics, and research technologies to relieve human suffering from chronic disease and injury. To date, CIRM has approved grants and loans totaling approximately \$2 billion. Of that amount, approximately \$1.57 billion has been disbursed to grantees as of the date of this report.

### **RISK ASSESSMENT PROCESS**

A. Proposition 71 requires an annual independent financial audit of CIRM. Health & Safety Code section 125290.30, subdivision (b), requires CIRM to commission an annual independent audit by a certified public accounting firm:

"The institute shall annually commission an independent financial audit of its activities from a certified public accounting firm, which shall be provided to the State Controller, who shall review the audit and

annually issue a public report of that review.”

Gilbert Associates, Inc. performed CIRM’s audit for the period from inception to June 30, 2005. Macias Gini & OConnell LLP (MGO) performed CIRM’s audit for each fiscal year ending June 30, from 2006-2015. All of the audit reports previously released may be found on the SCO website.

B. The annual independent financial audit is also reviewed separately by the State Controller.

As required by Section 125290.30(b), the annual independent financial audit of CIRM is provided to the State Controller, who then reviews the audit and issues a public report of that review. The SCO has reviewed and reported favorably on the independent financial audits of CIRM for every year through fiscal year through June 30, 2014.

C. CIRM’s financial practices are also reviewed each year by the Citizen’s Financial Accountability Oversight Committee.

In addition to the annual independent financial audit and the annual SCO review of that audit, Health & Safety Code section 125290.30(c) creates a Citizen’s Financial Accountability Oversight Committee (CFAOC):

"There shall be a CFAOC chaired by the State Controller. This committee shall review the annual financial audit, the State Controller's report and evaluation of that audit, and the financial practices of the institute."

The CFAOC is a six-member board chaired by the State Controller. The committee meets annually to review the financial practices and performance of CIRM. The SCO’s website has a page dedicated to the CFAOC’s proceedings. CFAOC meeting transcripts, annual reports, and other resources can be found there. (See <http://www.sco.ca.gov/eo/cfaoc/index.shtml>.)

D. CIRM is also subject to a triennial performance audit to ensure it is achieving economy, efficiency, and effectiveness in its use of resources.

In addition to the multiple levels of oversight described above, Health & Safety Code Section 125290.30(c) requires that CIRM commission a performance audit every 3 years beginning fy 2010–11. The performance audit, which is conducted in accordance with government auditing standards, examines the functions, operations, management systems, and policies and procedures of the institute to assess whether the institute is achieving economy, efficiency, and effectiveness in the employment of available resources. This includes a review of whether CIRM is complying with ICOC policies and procedures. The first performance audit was presented to the ICOC in May 2012 and included a review of, among other things: (1) CIRM’s policies and procedures for the issuance of contracts and grants and a review of a representative sample of contracts, grants, and loans executed by the institute; and (2) CIRM’s policies and procedures relating to the protection or treatment of IP rights associated with research funded or commissioned by CIRM.

The second performance audit conducted by Moss Adams LLP commenced in late 2014 and covered CIRM’s operations in the fiscal year (July 1, 2013 – June 30, 2014). The FY 2013-2014 Performance Audit included, but was not limited to, a review of all of the following:

Policies and procedures for the issuance of contracts and a review of a representative sample of contracts:

Policies and procedures for the issuance of grants and loans and a review of a representative sample of grants and loans; and

Policies and procedures relating to the protection or treatment of IP rights associated with research funded or commissioned by CIRM.

Finally, CIRM’s President, General Counsel, Deputy General Counsel, and Dir. of Finance met together

and with the Board and staff to review strategic goals and risks, including the issues identified by previous audits, to compile the items described herein.

Identified Risks are prioritized using a risk impact/probability analysis, accounting for the prioritization indicated. Risks with a higher quantitative impact and probability are prioritized higher than risks with lower such characteristics.

## **EVALUATION OF RISKS AND CONTROLS**

### **Operations- External- Partner Agencies/Grantees—Conflicting Objectives, Program Coordination**

Though CIRM intends to greatly increase the number of clinical trials and treatments in the Institute's portfolio, CIRM also intends to support only projects of the highest quality. The bar must not be lowered in order to achieve the strategic plan goals. In fact, as has been the practice under the CIRM 2.0 Clinical Program, CIRM intends to raise the bar for all CIRM programs in order to ensure that we fund only projects that have exceptional merit. Although CIRM will make every effort to identify and recruit promising, high caliber projects to California for partnership with the Institute, achieving the major goals in this area depends on the existence of such projects. It is possible that stem cell research is simply not advanced enough to support achievement of CIRM's target goals for the number of clinical trials and therapeutics candidates or that those projects are outside of California and the investigators are not interested in bringing their projects to California.

CIRM plans to mitigate this risk by aggressively recruiting high quality projects and designing CIRM programs that are so compelling to drug developers that they are willing to relocate all or part of their projects to California.

### **Operations- External- Staff—Recruitment, Retention, Staffing Levels**

Although Proposition 71 did not provide a sunset date for CIRM, the Institute's life is limited by two related factors. First, Proposition 71 authorized CIRM to spend a total of \$3 billion in bond proceeds. Second, of that \$3 billion, CIRM may spend no more than six percent, or \$180 million, plus donated funds and interest earned on its funds, for administrative purposes. In the absence of the authorization of additional funds or some alternative source of revenue, therefore, CIRM cannot continue to exist once its current funding expires. The uncertainty relating to CIRM's longevity creates a challenge for CIRM in recruiting and retaining talented team members. This challenge is compounded by the fact that the State's contribution towards an employee's retirement benefits vests after five years of service.

Fortunately, CIRM has long benefited from the fact that its team members are drawn to the Institute because of its mission, not based on financial rewards. Nonetheless, CIRM is evaluating its policies to enhance its ability to attract and retain the top talent that the Institute has enjoyed since its inception.

### **Compliance- External- Complexity or Dynamic Nature of Laws or Regulations**

Respondents to CIRM's strategic plan survey overwhelmingly identified the regulatory environment for stem cell treatments as the biggest roadblock to the field. The uncertainty of the regulatory pathway for stem cell treatments results in project delays and increased costs, and it dissuades investment in the field by venture capitalists, pharmaceutical companies, and the biotech sector. CIRM has established a working relationship with the FDA to address these concerns, but if the FDA is unwilling to take steps to improve the regulatory environment, it could remain a substantial obstacle to accomplishing CIRM's mission.

To address this risk, CIRM is engaging in a dialogue with the FDA and other stakeholders about the opportunities for reform, including consideration of the Japanese model, or alternatively,

other pathways such as the development of California-specific standards for the approval of stem cell treatments for use by patients in the State of California.

### **Operations- External- Technology—Data Security**

CIRM stores data and files that are both critical to the ongoing function of CIRM and confidential to our applicants and grantees. CIRM is taking various steps to better protect and more efficiently manage this information by, among other things, moving files and data to cloud-based storage and retrieval systems. In addition, CIRM relocated its primary offices from San Francisco to Oakland in 2015. Since the relocation CIRM has not conducted a security audit to determine that the new infrastructure and policies are sufficiently protecting and efficiently managing critical CIRM files and data.

(Description of the control; how the control helps to mitigate the risk; if the control is new or existing)

Equipment –Verify that all data center equipment is working properly and effectively. Equipment utilization reports, equipment inspection for damage and functionality, system downtime records and equipment performance measurements all help the auditor determine the state of data center equipment. Additionally, ensure preventative maintenance policies are in place and performed.

- Policies and Procedures – Determine all data center policies and procedures are documented and located at CIRM. Important documented procedures include: data center personnel job responsibilities, back up policies, security policies, employee termination policies, system operating procedures and an overview of operating systems.
- Physical security / environmental controls – assess the security of the CIRM's data center. These include: Air conditioning units, raised floors, humidifiers and uninterruptible power supply.
- Backup procedures –Verify that the client has backup procedures in place in the case of system failure.

### **Operations- Internal- FI\$Cal Conversion**

Because of challenges in implementing the the State's new FI\$Cal system, the utility of the program has not yet been optimized, which presents challenges to CIRM staff in utilizing the system. Due to various factors, DGS/CFS is currently not utilizing FI\$Cal to process CIRM payments. Payments are submitted to the State Controller's Office via paper claims, and then uploaded to FI\$Cal, resulting in duplication of efforts and inefficiencies. CIRM is not able to obtain timely or accurate financial data. Additionally, CIRM has experienced operational inefficiency as a result of system issues, such as conversion, preparation of invoices for processing in both systems and training, for example.

To address this risk, CIRM is working with DGS/CFS, FI\$Cal, and SCO to address the issues that are requiring manual claim schedules and creating FI\$Cal system backlogs. Additionally, CIRM is closely managing its internal financial systems to ensure they are current and accurate.

### **Operations- Internal- Oversight, Monitoring, Internal Control Systems**

The agency's legal team's mission is to support execution of CIRM programs by establishing effective policies, ensuring the fair and efficient review of applications, and applying our rules uniformly. To do so the agency must establish clear and effective policies for the submission and review of applications and for the administration and oversight of awards and seize every opportunity to make established policies more effective and easier to understand. In some cases, however, the policies and procedures employed by CIRM have not been evaluated and updated since their adoption to determine their effectiveness and opportunities for improvement. Accordingly, the agency may be operating less efficiently and/or with less compliance with internal policies and goals.

CIRM 2.0 Core: In addition to the overhaul of research and development activities, CIRM's

general operating activities (accounting, legal, HR, etc.) are being updated and refined to reflect current best practices necessary to accomplish CIRM's mission. This process, termed "2.0 Core" will ensure that the Institute operates in a manner that is both efficient and responsive.

### **Operations- External- Business Interruption, Safety Concerns**

In 1999, a patient who was participating in a gene therapy trial at the University of Pennsylvania died during the trial, leading to a substantial set-back for the field of gene therapy. Fortunately, to date, stem cell clinical trials have not encountered similar challenges, but a generalized concern about putting cells into the human body could deter patients from participating in stem cell clinical trials.

CIRM plans to address this risk by ensuring that the trials it funds are conducted with appropriate consent and under the highest standards, and if a trial encounters safety concerns, CIRM will take prompt action to address those concerns, including terminating the award if warranted. CIRM also intends to engage in education efforts to ensure that patients have complete information about potential clinical trials.

### **ONGOING MONITORING**

Through our ongoing monitoring processes, the California Institute for Regenerative Medicine reviews, evaluates, and improves our systems of internal controls and monitoring processes. The California Institute for Regenerative Medicine is in the process of formalizing and documenting our ongoing monitoring and as such, we have determined we partially comply with California Government Code sections 13400-13407.

### **Roles and Responsibilities**

As the head of California Institute for Regenerative Medicine, C. Randal Mills, President and CEO, is responsible for the overall establishment and maintenance of the internal control system. We have identified Chila Silva-Martin, Director of Finance, James Harrison, General Counsel, as our designated agency monitor(s).

### **Frequency of Monitoring Activities**

CIRM holds weekly senior management meetings. Meeting topics include discussion of current and potential challenges to achievement of strategic goals, as well as updates on control activities and an assessment of the success of mitigation activities. The senior management meetings consist of individual unit chiefs charged with implementation of the controls, each of which oversees implementation of various components of the controls and evaluates the control's performance.

### **Reporting and Documenting Monitoring Activities**

Because of the relatively small size of the agency (50 employees), senior management is in daily close contact and communication with all units within the organization and receive real-time feedback on success or challenges of monitoring practices conducted and the overall monitoring performance within each team. CIRM encourages all members of the CIRM team to communicate to the rest of the team if they identify an opportunity that should be considered to better assist CIRM with fulfilling its mission and goals. Through its almost monthly governing board meetings, the CIRM executive team updates periodically the governing board on the performance of individual units in the overall achievement of the strategic goals and efforts to mitigate challenges identified in this report.

### **Procedure for Addressing Identified Internal Control Deficiencies**

Designated monitors meet with the CIRM senior executive team on a weekly basis where progress on control performance can be shared and options for improvement discussed, designed and implemented.

## **CONCLUSION**

The California Institute for Regenerative Medicine strives to reduce the risks inherent in our work through ongoing monitoring. The California Institute for Regenerative Medicine accepts the responsibility to continuously improve by addressing newly recognized risks and revising risk mitigation strategies. I certify our systems of internal control and monitoring processes are adequate to identify and address material inadequacies or material weaknesses facing the organization.

C. Randal Mills, President and CEO

cc: Department of Finance  
Legislature  
State Auditor  
State Library  
State Controller  
Secretary of Government Operations