



Industry Engagement and Commercialization Plan

December 2012

Introduction and Executive Summary

Proposition 71, established the California Institute for Regenerative Medicine (CIRM), and provides \$3 billion for stem cell research. Its purpose is to accelerate stem cell research and ultimately the development of transformative new therapies. Additionally, Proposition 71 seeks to: “benefit the California economy by creating projects, jobs, and therapies that will generate millions of dollars in new tax revenues;” and “advance the biotech industry in California to world leadership, as an economic engine for California’s future.”

CIRM has already succeeded in making significant contributions towards the economic development goals of Proposition 71. Thus far, CIRM funding projected to be disbursed through 2014 has created 38,000 job years or 4,222 jobs/year over 9 years and it has generated tax revenues of \$286M. Going forward, this Industry Engagement and Commercialization Plan (“Business Plan”) serves as CIRM’s blueprint for continuing to support Proposition 71’s mission by engaging industry and supporting commercialization of CIRM’s funded research. It sets forth the following four strategic objectives:

- Attract Follow-On Financing and Co-funding of CIRM Funded Research
- Support of Company Creation/Growth/Relocation
- Early Engagement of Top Tier Biopharmaceutical Companies in Order to Access Critical Expertise
- Assume a Leadership Role in Business Related Areas the are Critical for Supporting the Field

In addition, this Business Plan sets forth key initiatives that will support these objectives. While CIRM expects that its strategic objectives will remain generally unchanged year to year, CIRM fully expects its initiatives in support of these priorities will change periodically.

Underlying Considerations and Rationale

In part because of the enormous expense of drug development, attracting and funding the highest caliber of stem cell science will not be enough to ensure delivery of break-through therapies to patients. Rather, to enhance the likelihood of success, CIRM must seek to engage industry in a number of contexts. Without the support of industry, there is significant risk that CIRM will have seeded the field and built a platform for continued research, but translation to FDA approved therapies will be significantly impeded, young California regenerative medicine companies could close and an opportunity to develop California into a global leader in regenerative medicine will be lost. By focusing on the strategic objectives identified within this Business Plan and through execution of the initiatives described below, CIRM will best position itself to advance the development of ground-breaking therapies, improving the lives of patients while at the same time supporting a robust regenerative medicine industry within California.

The importance of engaging industry was recognized by the panel of global experts that reviewed CIRM in 2010. In their report, (http://www.cirm.ca.gov/files/PDFs/Administrative/CIRM-EAP_Report.pdf, Report of the External Advisory Panel), they noted that “by engaging industry in a proactive manner, CIRM can further execute on its mission and provide the framework for continued growth beyond Proposition 71.” The advisory panel recommended that CIRM “expand engagement with the healthcare industry and explore innovative partnerships that will catalyze the movement of research from the laboratory to the clinic.” The Report did not identify the scope, nature or context of the recommended industry engagement. Rather it stated, “the precise role of industry needs to be explored and defined. It is not likely that one size fits all.”

Strategic Objectives and Supporting Initiatives

Strategic Objectives:

CIRM has identified the following four strategic objectives that will guide its commercialization and industry engagement activities:

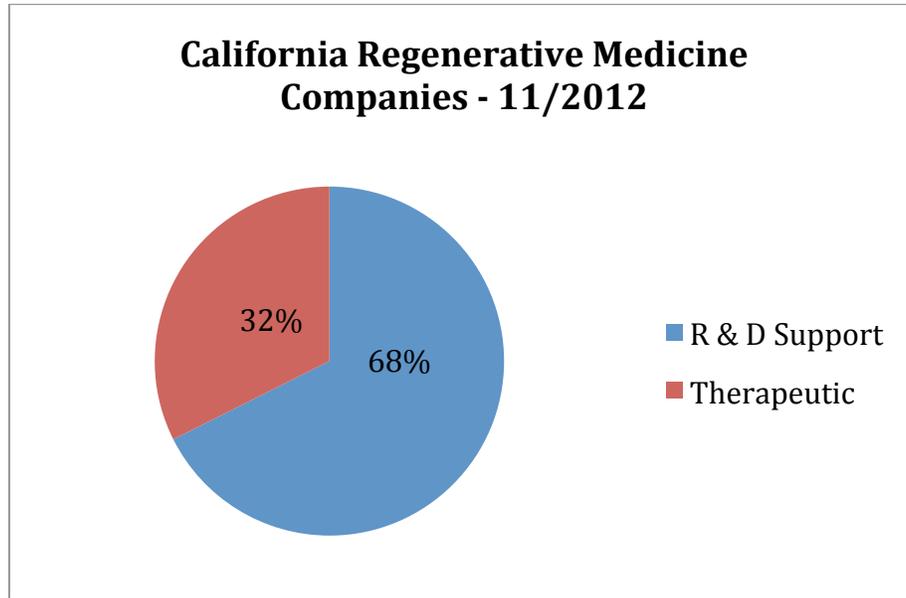
Strategic Objectives			
Attracting Follow On Financing and Co- Funding	Early Access to BioPharma’s Expertise	Company Creation, Growth, & Relocation	Leadership & Industry Support

I. Attracting Follow-On Financing and Co-Funding: This strategic objective seeks to expand the reach of CIRM’s funding and to increase the likelihood of commercialization of CIRM programs by linking them to future sources of capital. Linking CIRM’s development pipeline with potential future (if not present) funding sources is of critical importance. CIRM is not intending to fund Phase 3 clinical trials and will not have the funding available to support even earlier stage clinical trials for all of the projects it is currently funding. By linking its development projects to global biopharmaceutical companies and/or venture capital CIRM enhances the likelihood of commercialization of many of its funded projects. In addition, by obtaining co-funding for drug discovery projects, CIRM will be able to leverage its funding and thereby support a greater number of programs. Co-funding can also serve as a proxy for projects that are more likely to attract future investment.

II. Early Engagement with Major BioPharma to Access Critical Expertise: Engaging biopharmaceutical companies early in translational research will benefit CIRM funded programs by providing access to expertise in areas which include regulatory, manufacturing and clinical trial design. Many academics are new to drug development and lack in-depth knowledge of the regulatory requirements and technical aspects of translating research discoveries into drugs. By engaging biopharma early, academic programs will benefit from the expertise they will be able to access and as a result, these programs should become more competitive in terms of investment opportunities.

III. Support Company Creation/Growth/Relocation: This economic development objective is multi-faceted: growing California regenerative medicine companies, in particular those that are within CIRM’s funding mandate, attracting high quality, high impact companies to California (e.g. research support companies as well as therapeutic companies) and supporting companies that are “spun-out” of academia based in whole or part from CIRM funded research. (See Appendix A for companies that have received CIRM funding and Appendix B for a list of companies arising from CIRM research to date). Proposition 71’s funding mandate is “funding pluripotent stem cell and progenitor cell research that cannot, or is unlikely to, receive timely or sufficient federal funding, unencumbered by limitations that would impede the research...[and other] vital research opportunit[ies].” Regardless of whether a company has received CIRM funding, a number of programs and initiatives that CIRM pursues will support the industry as a whole. (See Strategic Objective IV below).

With respect to supporting California's regenerative medicine industry it bears noting that as of November, 2012, data compiled by UniverCell Market¹ showed a total of 58 California companies designated as regenerative medicine companies developing therapeutics and 121 California companies were identified as tool and regenerative medicine research support companies. Some of the therapeutic companies listed by UniverCell Market appeared to be young start-ups. In addition, a number of the companies were focused in areas that are outside of CIRM's funding mandate.



¹ Information downloaded on November 15, 2012 from website at <https://www.univercellmarket.com/@offers/Regional+Profiles/North+America+++California/>

In addition to supporting existing California regenerative medicine companies, attracting high impact companies to California will also aid Proposition 71's goal of building an "economic engine" for California. The prospect of funding has attracted some interest thus far. Through CIRM's Strategic Partnership Funding Program, CIRM was able to attract a noteworthy and respected young company to establish a small office in California and importantly to hire a local manufacturer and conduct its planned clinical trial(s) within California. CIRM has also been in discussions with a number of other companies interested in establishing operations in California in order to access California's talent pool, which CIRM has helped develop, and possibly CIRM funding as well. Nonetheless, attracting significant company investment into California will be challenging as CIRM does not have the ability to offer significant incentives that economic development agencies commonly have.

Lastly, CIRM will seek to support company start-ups that emerge from research supported by CIRM. In this context it is recognized that not all research programs should be transformed into companies. However, already CIRM has been contacted by CIRM grantees seeking CIRM's assistance and CIRM will endeavor to adopt initiatives that can provide some support to these young companies.

IV. Assume a Leadership Role in Business Related Areas the are Critical for Supporting the Field: The field of regenerative medicine can benefit as a whole from efforts aimed at addressing reimbursement issues, increasing access to capital and facilitating a business model for autologous

drugs in particular. As such CIRM will take a leadership role, working closely with industry organizations and companies, to support policies and forums which address these issues and other business related needs of critical importance to the industry

Initiatives Supporting CIRM' Strategic Objectives:

There are a number of initiatives, small and large that will support the four strategic objectives. Many of the initiatives discussed below are in the very early formation stage. Some have already commenced. Initiatives may change, some new initiatives will emerge and some will be discontinued. In short, we consider this Business Plan to be a living document.

1. Strategic Partnership Funding Program – The Strategic Partnership Funding Program has already been approved by the Governing Board of CIRM and it serves as a cornerstone of CIRM's efforts to engage industry and satisfy the Follow-On Funding and Co-Funding Strategic Objective.
2. Appointment of a CIRM Business Development Officer - CIRM has appointed a Vice President of Business Development and has recently hired a Business Development Officer whose responsibilities will include enhancing industry engagement in CIRM initiatives, facilitating linkages of CIRM funded programs to potential funding sources, and mentoring academic start-up companies.
3. Designation of Industry Collaborators - CIRM will foster relationships with biopharmaceutical companies having an expressed interest in funding and/or supporting through in-kind services regenerative medicine research ("CIRM Industry Collaborators") and it will facilitate linkages between its Industry Collaborators and California researchers. As appropriate, Industry Collaborators will be identified on CIRM's website and within RFAs as possible co-funding and/or co-development partners. Industry Collaborators will also be included in CIRM workshops and other programs of interest.
4. Relocation Support - Encouragement of companies engaged in critical research support activities (e.g. process development/manufacturing, pre-clinical animal models) to establish operations in California. CIRM will explore various mechanisms to create sufficient incentives for high impact companies to locate in California. Possible mechanisms include relocation enhancements to grant awards, the pre-purchasing of a certain level of services, and perhaps as part of CIRM's Alpha Clinic program.

5. Leadership & Industry Support – May include:

- a. Stem Cell Meeting on the Mesa partnering conference – continued support of the annual scientific conference and partnering forum of this conference
- b. Summit/ Workshop to identify areas of interest for co-funding pre-competitive technologies and for identifying bottlenecks in research and development
- c. Company Creation Workshop – to assist principle investigators in identifying the process of and tools needed to establish companies Areas could include reimbursement, manufacturing capacity/infrastructure, and technology transfer issues.
- d. Participation in efforts by industry organizations to pursue reimbursement pathways that will support the regenerative medicine industry

The following table summarizes some of the key initiatives discussed above and which of the strategic objective(s) they serve.

	Follow On Funding/Co-Funding	Company Creation/Growth/ Relocation	Early Engagement of Biopharma	Leadership & Industry Support
Strategic Partnership Funding Program*	XX	XX	XX	XX
CIRM Industry Collaborators	XX		XX	
Relocation Support for High Impact Companies		XX		
Pre-Competitive Consortia/ Tool and Tech. Workshop	XX		XX	XX
Est. of a CIRM BD function including creation of a supporting database, marketing materials, and continuous outreach activities *	XX	XX	XX	XX

* - Indicates that initiative has been implemented

Measures of Success

In order to evaluate the successful implementation of this Business Plan, CIRM will track its performance in the following key areas over the next five years:

- Increased industry funding through programs such as the Strategic Partnership Fund
 - Recruitment of new stem cell operations to California
 - Creation of new companies based on CIRM funded programs
 - Formation of industry relationships between CIRM, CIRM grantees and biopharmaceutical companies
 - Formation of new alliances beyond already existing relationships.
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Appendix A – Companies Receiving CIRM Funding

CIRM has made 23 awards to 14 For-Profit Entities for a total of \$166 Million

Name of For-Profit Grantee	Total Amount of Award	Total Number of Awards
BioTime, Inc.	\$ 4,721,706	1 – Early Translational I award - \$4,721,706
BlueBird Bio	\$9,363,335	1 Strategic Partnership award – \$9,363,335
Capricor	\$19,782,136	1 Disease Team II- \$19,782,136
Escape Therapeutics, Inc	\$1,453,040	1 Transplantation Immunology - \$1,453,040
Fluidigm Corporation	\$2,693,424	2 Tools & Technology I - \$749,520 & Tools & Technology II - \$1,943,904
Gamma Medica-Ideas, Inc.	\$2,478,347	2 Tools & Technology I - \$949,748 & Tools & Technology II - \$1,528,599
GMR Epigenetics	\$1,452,693	1 Tools & Technologies II - \$1,452,693
iPierian, Inc.	\$ 7,123,887	2 Early Translational II - \$5,665,887 & Basic Biology II - \$1,458,000
OncoMed Pharmaceuticals Inc	\$65,120	1 Disease Team II Planning - \$65,120
Stem Cells, Inc.	\$40,098,050	3 Disease Team II Planning - \$98,050 & two Disease Team Awards each for \$20,000,000
Vala Sciences, Inc.	\$906,629	1 Tools & Technology I - \$906,629
Viacyte (formerly Novocell)	\$36,356,426	5 Early Translational I - \$5,405,397 & Tools & Technology I - \$827,072 & Disease Team Planning - \$48,950 & Disease Team I - \$19,999,937 Strategic Partnership Award - \$10,075,070
VistaGen Therapeutics, Inc.	\$971,558	1 Tools & Technology I - \$971,558
Wintherix, LLC	\$99,110	1 Disease Team II Planning - \$99,110
Total	\$115,719,868.00	23

EXHIBIT B – COMPANIES ARISING IN WHOLE OR IN PART FROM CIRM FUNDING

1. Regenerative Patch Technologies (RPT, Inc.)
 2. Oceanside Biotechnology LLC
 3. Didmi, Inc.
 4. Telerogen, Inc.
 5. Neurona, Inc.
 6. CytoRay, Inc.
 7. ChemRegen
 8. jCyte, Inc.
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