NOTICE OF PROPOSED REGULATION AMENDMENTS

California Code of Regulations
Title 17. – Public Health
Division 4 - California Institute for Regenerative Medicine
Chapter 5, Section 100503

Date: August 31, 2018
Deadline for Submission of Written Comment: October 15, 2018 – 5:00 p.m.
Public Hearing Date: None Scheduled

Subject Matter of Proposed Amendments:
Grant Administration Policy for Clinical Stage Projects

Submittal of Comments:

Any interested party may present comments in writing about the proposed amendments to the agency contact person named in this notice. Written comments must be received no later than 5:00 p.m. on October 15, 2018. Comments regarding this proposed action may also be transmitted via e-mail to GAPComments@cirm.ca.gov or by facsimile transmission to (415) 396-9141.

Public Hearing:
At this time, no public hearing has been scheduled concerning the proposed regulations. If any interested person or the person's representative requests a public hearing, he or she must do so in writing no later than October 1, 2018.

Sections Affected: The proposed regulatory action amends Section 100503 to Chapter 5 of Title 17 of the California Code of Regulations, and the document incorporated by reference into section 100503.

Authority: Article XXXV of the California Constitution and Health and Safety Code Section 125290.40, subdivision (j).

Reference: Sections 125290.30, 125290.35, 125290.40, 125290.45, 125290.50, 125290.60, 125290.70, 125292.10, Health and Safety Code.

Informative Digest/Policy Statement Overview:
The California Institute for Regenerative Medicine ("Institute" or "CIRM") was established in 2005 after the passage in 2004 of Proposition 71 (the "Act"), the California Stem Cell Research and Cures Initiative. The statewide ballot measure established a new state agency to make grants and provide loans for stem cell research, research facilities and other vital research opportunities. The Independent Citizens' Oversight Committee ("ICOC") is the 29-member governing board for the Institute. The 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. The Act charges the ICOC with developing standards and criteria to make grant awards and to

develop standards and criteria for proper oversight of awards. (§ 125290.50.) To that end, CIRM adopted the CIRM Grants Administration Policy for Clinical Stage Projects ("GAP").

Existing section 100503 incorporates by reference the GAP and indicates that recipients of grants for clinical stage projects will be subject to this particular GAP. This section indicates that amendments to the policy will be applied to current active grants at the next budget period after the effective date of any amendments.

This grants administration policy incorporated by reference by section 100503 serves as the terms and conditions for Clinical Stage Projects funded by the California Institute for Regenerative Medicine (CIRM) pursuant to clinical stage funding opportunities. In addition, it provides guidance to applicants and Awardees regarding their responsibilities. Principal investigators, program directors, and organizational officials with grants management responsibilities are urged to read this document carefully and to refer to relevant sections for answers to questions that arise concerning the administration of CIRM awards. Applicants and Awardees may be required to document compliance with any and all provisions set forth in the policy.

In furtherance of CIRM's mission to accelerate the development of stem cell therapies to patients with unmet medical needs, CIRM seeks to continuously improve upon its policies and procedures to ensure a more streamlined, predictable process for awarding and administering grants. As part of that goal, we propose to initiate a new round of amendments to the Grants Administration Policy for Clinical Stage Projects (Clinical GAP).

The following changes are proposed. With the exception of these proposed amendments, the remainder of the Clinical GAP will remain in effect, except for minor technical/clarifying fixes.

A. Allowable and Unallowable Project Costs

These sections describe the types of direct project costs CIRM funding may and may not support. CIRM proposes generally to follow the NIH Grants Policy Statement on allowable costs and highlight areas of common interest to our Awardees as follows:

Allowable

- Insurance that is deemed necessary and specific to the project not otherwise covered by Facilities or Indirect costs, including clinical trial insurance and medical liability (malpractice) insurance when the project involves human subjects.

Unallowable

- Legal costs incurred in defending or prosecuting claims, whether equitable or monetary.
- Intellectual property costs including, but not limited to, invention, copyright, patent, licensing or royalty costs, filing fees, translation costs, examination fees, annuity costs and grant fees, and related attorney's fees.
- Routine, patient standard of care costs or any cost of care covered by a 3rd party provider.

B. Clinical Trial Registration Requirement

CIRM proposes to add a requirement for all CIRM-funded clinical trials to be registered and submit the results of the trial in accordance with FDAAA 801 requirements. These requirements include registering the trial no later than 21 days after the 1st patient is enrolled in the trial and publishing the results no later than 12 months after completion of the trial.

C. Delete Description of Scoring

CIRM proposes to remove the paragraph in Section II.E. Application Review that describes clinical application scoring. The current statement is inconsistent with the GWG bylaws, which modified the definition of a Tier 3 score. Given the existing conflict and the potential for future inconsistencies if the Board approves future changes to the GWG bylaws regarding scoring, CIRM suggests removing this language from the Clinical GAP.

D. Prior Approval Request – Change in California Organization Eligibility

CIRM proposes to add a requirement for a CIRM-funded Awardee to immediately report whether their organization's status as a California-based or Non-California-based organization has changed in light of the fact that such status will determine the scope of allowable project costs.

If a California-based Organization becomes a Non-California-based organization, we will propose to reduce the remainder of the award effective the date the organization's status changed to only cover the allowable project costs available to Non-California-based organizations. If a Non-California-based organization becomes a California-based organization, we propose to maintain the existing award amount as approved by the ICOC but allow the Awardee to reallocate remaining funds to also cover any additional allowable project costs available to California-based organizations.

E. Award Conversion

Under this section, recipients of CIRM's Clinical Stage Program awards have the option to treat their awards as loans within the earlier of the submission of an application for marketing approval by the Food and Drug Administration or seven years from the effective date of the award. Unless the parties agreed to different terms, the awardee would be required to repay the loan balance within ten days of making the loan election at a rate that would escalate based on the date of repayment. The loan election would become final only after the awardee has satisfied the terms of the election. If an awardee does not make this election, its award would be treated as a grant. The proposed amendments make nonsubstantive amendments to delete reference to non-existent funding opportunities and propose optional language regarding payback terms.

F. Award Termination

This section describes the circumstances under which failure of compliance may lead to CIRM action with regard to the award, including termination of the Award. CIRM proposes to broaden this provision to address the circumstance where and Awardee or the Award become ineligible based on the criteria for that program.

Anticipated Benefits of the Proposed Regulation:
To the extent the regulation facilitates use of the funds and encourages development of intellectual property and return to the state as required by law, and to the extent California institutions apply for and receive research funds, such requirements are indirectly attributable to increased economic activity spurred by the investment research funds in the state and resultant positive business and employment development. Also, to the extent the regulation makes it possible for the expenditure of research funds in the state, and to the extent that research results in medical treatments and cures for chronic disease and injury, the regulation indirectly benefits the health and welfare of California residents who will benefit from such treatments and cures.

Consistency and Compatibility with Existing State Regulations:
CIRM has conducted an evaluation for any other regulations on this area and has concluded that this is the only regulation concerning administration of CIRM-funded awards for late-stage research projects. Therefore, the proposed amendments are neither inconsistent nor incompatible with any other existing state regulations.

Incorporated by Reference Documents: California Institute for Regenerative Medicine Grants Administration Policy (GAP) for Clinical Stage Projects, Sections "II" through and including "VI" in their entirety; As to Section "I", only part "I.B.", " (Abbreviations), "I.C.," ("Defined Terms"), "I.D" ("Types of Support") and "I.E." ("Roles and Responsibilities"), Rev. 06/17.

DISCLOSURES REGARDING THE PROPOSED AMENDMENTS:
CIRM has made the following initial determinations:

Mandate on local agencies and school districts: None.

Effect on Small Business:
CIRM has determined that the proposed amendments will have no impact on small businesses. The regulation implements conditions on awarding and administering grants for stem cell research. This research is conducted almost exclusively by large public and private nonprofit institutions. As such, the amendments to the regulation are not expected to adversely impact small business as defined in Government Code Section 11342.610.

Impact on Local Agencies or School Districts:
CIRM has determined that the proposed amendments do not impose a mandate on local agencies or school districts, nor do they require reimbursement by the state pursuant to Part 7 (commencing with Section 17500) of Division 4 of the Government Code because the amendments do not constitute a "new program or higher level of service of an existing program" within the meaning of Section 6 of Article XIII of the California Constitution. CIRM has also

determined that no nondiscretionary costs or savings to local agencies or school districts will result from the proposed amendments.

Costs or Savings to State Agencies:
CIRM has determined that no savings or increased costs to any agency will result from the proposed amendments.

Effect on Federal Funding to the State:
CIRM has determined that no costs or savings in federal funding to the state will result from the proposed amendments.

Effect on Housing Costs:
CIRM has determined that the proposed amendments will have no effect on housing costs.

Significant Statewide Adverse Economic Impact Directly Affecting Businesses:
CIRM has made an initial determination that the proposed amendments will not have a significant statewide adverse economic impact directly affecting businesses, including the ability of California Businesses to compete with businesses in other states.

Cost Impacts on Representative Private Persons or Businesses:
CIRM has made an initial determination that the adoption of these amendments will not have a significant cost impact on representative private persons or businesses. CIRM is not aware of any cost impacts that a representative private person or business would necessarily incur in reasonable compliance with the proposed amendments.

Results of Economic Impact Analysis:
The above analysis is based on that fact that the proposed amendments do not impose new requirements on existing business operations or functions of other agencies or individuals but implement standards for seeking and using state grant funds for scientific research. In most cases, such grants include funds to cover overhead and other indirect costs of the research, including most compliance activities. CIRM has made an initial determination that it is unlikely the proposed amendments will impact the creation or elimination of jobs, the creation of new businesses or the elimination of existing businesses, or the expansion of businesses currently doing business within the State of California, nor directly impact the health and welfare of California residents, worker safety, and the state's environment. However, applicants and awardees of CIRM funds for clinical stage projects would have a clear understanding of their responsibilities in accepting and using state funds for stem cell research, which ultimately benefit the citizenry of California. In addition, To the extent the regulation facilitates use of the funds and encourages invention and return to the state as required by law, and to the extent California institutions apply for and receive research funds, such requirements are indirectly attributable to increased economic activity spurred by the investment research funds in the state and resultant positive business and employment development. Also, to the extent the regulation makes it possible for the expenditure of research funds in the state, and to the extent that research results in medical treatments and cures for chronic disease and injury, the regulation indirectly benefits the health and welfare of California residents who will benefit from such treatments and cures.

Consideration of Alternatives:
In accordance with Government Code Section 11346.5, subdivision (a)(13), CIRM must determine that no reasonable alternative it considered, or that has otherwise been identified and brought to its attention, would be more effective in carrying out the purpose for which the action is proposed or would be as effective and less burdensome to affected private persons or would be more cost-effective to affected private persons and equally effective in implementing the statutory policy or other provision of the law than the proposal described in this Notice. CIRM invites interested persons to present statements or arguments with respect to alternatives to the proposed amendments at the scheduled hearing or during the written comment period.

Availability of Statement of Reasons and Text of Proposed Regulations:
CIRM has prepared an Initial Statement of Reasons, and has available the express terms of the proposed amendments, all of the information upon which the amendments are based, and a rulemaking file. A copy of the Initial Statement of Reasons and the proposed text of the regulation may be obtained from the agency contact person named in this notice. The information upon which CIRM relied in preparing this proposal and the rulemaking file are available for review at the address specified below.

Availability of Changed or Modified Text:
After holding the hearing and considering all timely and relevant comments, CIRM may adopt the proposed amendments substantially as described in this notice. If CIRM makes modifications that are sufficiently related to the originally proposed text of the amendments, it will make the modified text (with the changes clearly indicated) available to the pubic for at least 15 days before it adopts the regulations as amended. Requests for the modified text should be addressed to the agency contact person named in this notice. CIRM will accept written comments on any changes for 15 days after the modified text is made available.

Agency Contact:
Written comments about the proposed regulatory action; requests for a copy of the Initial Statements of Reasons, the proposed text of the amendments; and inquiries regarding the rulemaking file may be directed to:

Scott Tocher
Deputy General Counsel
California Institute for Regenerative Medicine
1999 Harrison Street, #1650
Oakland, CA 94612
(415) 740-8735

Questions on the substance of the proposed regulatory action may be directed to:

Gabe Thompson
Director of Portfolio Operations and Performance
1999 Harrison Street, Suite 1650 | Oakland, CA 94612-3515
Phone/Fax: 510-340-9166

The Notice of Proposed Regulatory Amendment, the Initial Statement of Reasons and any attachments, and the proposed text of the amendments and existing regulation are also available on CIRM's website, www.cirm.ca.gov.

Availability of Final Statement of Reasons:
Following its preparation, a copy of the Final Statement of Reasons mandated by Government Code Section 11346.9, subdivision (a), may be obtained from the contact person named above.

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