Comments to Proposed Changes to CIRM Regulation Entitled: Intellectual
Property Policy for Non–Profit Organizations, Issued Aug. 8, 2006

Dear Mr. Tocher:

California Healthcare Institute (CHI) welcomes this opportunity to comment on the California Institute for Regenerative Medicine’s (CIRM) proposed changes to regulations addressing Intellectual Property Policy for Non-Profit Organizations (IPPNPO). CHI represents the full biomedical sector of the California economy; our members include more than 250 of California’s leading life sciences companies, universities, and academic research institutions.

As the advocate for California’s statewide biomedical research and development community, CHI appreciates the time and effort the Independent Citizens’ Oversight Committee (ICOC) has directed towards the development of an intellectual property policy that conforms to the purpose and intent of Proposition 71, the California Stem Cell Research and Cures Act (Prop 71). We especially appreciate the consideration given to comments provided by stakeholders, such as CHI\(^1\), in the development of these revisions to the IPPNPO.

Still, CHI remains concerned that certain revised provisions of the IPPNPO addressing pricing, access requirements and revenue sharing may undermine the main goal of Prop 71: to advance stem cell research and science in order to produce breakthrough diagnostics, treatments and therapies.

Since commercialization is essential for the development and production of new medicines, CHI urges the ICOC to adopt IP policies that will promote and maximize licensing and technology transfer. In our view, certain remaining provisions present unnecessary, improper or overly burdensome requirements likely to undermine the commercial collaboration necessary for the development of new products.

Prop 71 has affirmed California as a global leader in stem cell research today, but it is likely that circumstances tomorrow may be very different. Other states are following California in funding and encouraging stem cell research. Recent votes in the Congress indicate growing momentum

\(^1\) Letter from D. Gollaher, President and CEO, CHI, to S. Tocher, CIRM, regarding comments to Proposed CIRM Regulation Entitled: Intellectual Property Policy for Non–Profit Organizations, June 15, 2006.
to ease current federal restrictions on embryonic stem cell research. And from Britain to Singapore, countries around the world are welcoming researchers and commercial collaborators. In light of this competitive environment, CHI suggests that some remaining provisions in the IPPNPO that create disincentives to technology transfer may make CIRM research funding less attractive and desirable than other funding sources, and may make California less attractive to investors, commercial partners and other collaborators. These provisions include:

- Requirements that permit exclusive licensing “only to persons that agree to have plans in place at the time of commercialization to provide access to resultant therapies and diagnostics for uninsured California patients” and only if “licensees will agree to provide to patients whose therapies and diagnostics will be purchased in California by public funds the therapies and diagnostics at a cost not to exceed the federal Medicaid price”;  
- Unlimited revenue-sharing provisions that, beyond a threshold amount, require grantees to pay 25% of net revenues received under a license agreement to the State of California for deposit into the State’s General Fund;  
- New broad and expansive language that seems to run counter to the decision to remove the Research Exemption language.

Finally, while we did not originally comment on IPPNPO provisions addressing biomedical materials, given concerns that the language, as written, may be inadvertently broad, we respectfully request that the ICOC specifically clarify and narrow the definition of “Biomedical Materials” to only those “Entities of biomedical relevance first produced as a consequence of CIRM-funded scientific research...” (Italicized for emphasis purposes only)

We address in detail our concerns regarding specific provisions below.

i. Title 17 of California Code of Regulations, Section 100306(d), as Revised

While appreciative of the improvement to access plan requirements incorporated into the revised IPPNPO, CHI remains nonetheless concerned, as detailed in our original comments, that the ICOC may be inappropriately using the IPPNPO to address health care access and pricing issues. While improving health care access and affordability are important goals, they were not the objective of Prop 71 and should not, therefore, be the subject of policies and regulations pertaining to Prop 71.

With regard to patient access plans in particular, California’s biotechnology industry has long shown a commitment to ensuring product access for patients in financial need. California companies regularly donate or provide reimbursement assistance for products to thousands of patients in the United State and worldwide every year. This is the case regardless of whether the

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2 Proposed Title 17 of California Code of Regulations, section 100306(d), as revised
3 Proposed Title 17 of California Code of Regulations, section 100308, as revised
4 Proposed Title 17 of California Code of Regulations, section 100306(a), as revised
5 Proposed Title 17 of California Code of Regulations, section 100304, as revised
6 Proposed Title 17 of California Code of Regulations, section 100301(d)
7 Letter from D. Gollaher, pp 4-5.
product was developed fully in-house or from a licensed technology. In the latter instance, this has also been the case without any mandate, requirement, or regulation tied to the funding source for that licensed technology, such as the NIH and relevant federal law such as the Bayh-Dole Act (P.L. 96-517, Amendments to the Patent and Trademark Act). CHI suggests that the IPPNPO provision permitting exclusive licensing “only to persons that agree to have plans in place at the time of commercialization to provide access to resultant therapies and diagnostics for uninsured California patients” is unnecessary on its face and should be removed. Maintaining our objection to this provision in general, we also urge the ICOC to revise any reference to access plans to patients in “financial need” as opposed to “uninsured” in order to more accurately connect with companies’ patient assistance plans.

CHI also remains concerned with the IPPNPO provision permitting exclusive licensing only to those licensees that “agree to provide to patients whose therapies and diagnostics will be purchased in California by public funds the therapies and diagnostics at a cost not to exceed the federal Medicaid price.”

The application of “federal Medicaid price” to non-Medicaid purchasers for drug and biologic therapies could have the unintended consequence of increasing, rather than decreasing, prices for therapies resulting from CIRM-funded research. Such products would not be exempt from Medicaid Best Price\(^8\) calculations, which could result in higher Medicaid rebate liability for manufacturers of these products. Manufacturers would be unable to sustain such sweeping discounts, resulting in higher pricing or fewer discounts for Medicaid (and other) purchasers, and by extension, those purchasing with “public funds” under this proposed regulation.

Aside from the policy issue described above, the term “federal Medicaid price” is not defined in federal statutory language, and therefore it is not clear how this regulation would be implemented. Basing CIRM drug reimbursement on the state Medi-Cal program’s cost net of federally-mandated Medicaid rebates is impractical. Medicaid rebates are confidential and calculated by the federal government. The state would have to attempt to replicate this calculation. However, rebates are calculated quarterly based on two complex factors that could not be accurately determined by the state. The first is detailed utilization data submitted by the state to each drug manufacturer. This figure is often disputed, with disputes resolved through a Centers for Medicaid and Medicare Services (CMS)-governed dispute resolution process. A similar process would be required for “resultant therapies and diagnostics” under this regulation. The second factor is a complex calculation of rebate per drug unit based on the Average Manufacturers Price (AMP), the Best Price (BP), and a statutory rebate formula. The AMP and BP are confidential figures submitted by the manufacturer to CMS, not the State.\(^9\) Further, federal law and regulations permit AMPs to be restated within a three-year period and, accordingly, rebates paid are adjusted during this time. Thus, aside from the confidentiality associated with the Medicaid rebates, there is not a readily available “reimbursement” amount or Medicaid price that could be applied to CIRM drugs and biologics.

\(^8\) Best price is the lowest price paid to a manufacturer for a brand name drug, taking into account rebates, chargebacks, discounts or other pricing adjustments, excluding nominal prices. Prices charged to certain governmental purchasers are statutorily excluded from best price including prices charged to the VA, DoD, Indian tribes, FSS, State pharmacy assistance programs, Medicaid, and 340B covered entities.

\(^9\) At present AMPs are confidential, but the Deficit Reduction Act of 2005 will require that AMPs be made public in the future.
The term “public funds” is also not defined and is, therefore, ambiguous. In this construction, public funds could refer to products purchased through state and federal funds, including those purchased by the Veterans Administration, state employee health plans, state hospitals, state university employee health plans, local government employee health plans, et al. Given the many state residents covered by plans that involve “public funds,” this provision would, in practice, impose price controls on these products.

CHI suggests that this one-sentence provision fails to capture the complexities of the issue it intends to address. It would prove exceedingly difficult to implement, manage, and coordinate. Therefore we urge its deletion.

Finally, recognizing that it takes 10 to 15 years and more than $800 million to develop a potential new medicine from a basic research discovery to a product approved by the FDA, it is more than likely that any “resultant product or therapy” developed with CIRM funding will have received funding from other sources, such as the NIH, as well. For inventions funded with both state and federal money, therefore, the IPPNPO threatens to create an additional layer of complexity and uncertainty – a substantial disincentive -- for potential licensees, investors, and other stakeholders. The result would place grantees receiving CIRM funding at a competitive disadvantage with others facing simpler, less complex, and less risky technology transfer processes. Therefore, CHI suggests that these IPPNPO provisions be limited to those resultant products for which CIRM funding was the only public source of funding and that, in particular, where federal sources of funding were involved, explicitly state that federal policy prevail.

ii. Title 17 of California Code of Regulations, Section 100308, as Revised

CHI again suggests, as detailed in our original IPPNPO comments, that revenue sharing payments as required in the IPPNPO are likely to pale in comparison to the financial benefits to the state from CIRM-funded research and subsequent technology transfer and product commercialization resulting from job creation, exports, increased income taxes, payroll taxes, capital gains taxes, corporate income taxes – in short from a broad range of economic factors.\(^\text{10}\) We also call attention to the fact that revenues to grantees derived by licensing agreements will support continued research and education efforts, as is required by the IPPNPO.\(^\text{11}\) Therefore, in order to obtain the maximum potential reinvestment into stem cell research and the development of new medicines and therapies – activities at the core of Prop 71 – we urge the ICOC to limit revenue sharing on CIRM-funded, licensed inventions up to the total dollar amount of CIRM-provided research dollars. CHI strongly believes this to be in accordance and consistent with Prop 71’s purpose and intent.\(^\text{12}\)

\(^{10}\) Letter from D. Gollaher, pp 6-7.

\(^{11}\) Proposed Title 17 of California Code of Regulations, section 100308(d)

\(^{12}\) In particular, provision for “an opportunity for the state to benefit from royalties, patents, and licensing fees that result from the research.” Proposition 71, Sec. 3, Purpose and Intent
iii. Title 17 of California Code of Regulations, Section 100306(a), as Revised

CHI gratefully acknowledges the ICOC’s attention and consideration of concerns, as addressed in our original comments, regarding the original IPPNPO research use exemption provision, which resulted in the recommendation to delete that section. If, in the future, circumstances and evidence indicate that research is being undermined or constrained by IP protections, we would fully support reconsideration of this issue.

Nonetheless, we are concerned with the addition of new language providing that “grantee organizations retain the right to practice the use of CIRM-funded patented inventions for any non-profit purpose, including sponsored research and collaborations.” Given the recommendation of the ICOC IP Task Force as supported at the August 2, 2006 ICOC meeting, the addition of this language was not only unexpected, but, as written, is vague, overbroad and ambiguous. As such, it creates most of the same obstacles to private investment in CIRM-funded research-related IP as the original RUE provision, and thus runs counter to CIRM goals.

CHI requests the removal of this new sentence as unnecessary and counterproductive. As noted at the July 14th meeting of the IP Task Force, researchers’ access to IP is not currently a problem. In addition, the IPPNPO provides sufficient assurances that an access problem will not develop in the case of CIRM-funded IP. Provisions in the IPPNPO already require licensees to bring IP to “practical application” – including wide availability to the public (not just researchers) on reasonable terms. Moreover, the IPPNPO provides for enforcement mechanisms, such as march-in and termination of licenses, in the event a CIRM funded invention is not made widely available.

If language must be retained, we suggest that the ICOC adopt narrower and more concise language to minimize unintended consequences of the new language:

“In licensing CIRM-funded patented inventions, Grantee Organizations may retain the right to practice the use of CIRM-funded patented inventions and to allow other non-profit Grantee Organizations to practice the invention for their non-profit educational and basic research purposes.”

Conclusion

CHI appreciates this opportunity to comment on the revised CIRM Intellectual Property Policy for Non-Profit Organizations. We believe that a strong IPPNPO will advance CIRM-funded stem cell research and, ultimately, treatments for millions here in California and worldwide. This, in turn, will improve California’s health care system, benefit the California economy, and

[14] Title 17 of California Code of Regulations, section 100307, as originally proposed
[15] Title 17 of California Code of Regulations, section 100306(a), as revised
[16] Title 17 of California Code of Regulations, section 100306(c) and 100306(f)
[17] Title 17 of California Code of Regulations, section 100310
[18] Title 17 of California Code of Regulations, section 100306(f)
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further stimulate the state’s biotechnology industry. We hope that the ICOC will give careful consideration to our comments and incorporate them into the final IPPNPO.

In summary, to promote technology transfer and commercial collaboration on CIRM-funded inventions and to limit barriers to stakeholder participation in research, licensing, and commercialization, CHI suggests that the ICOC:

- Remove or specifically narrow provisions in Section 100306(d) pertaining to patient access plans.
- Remove provisions in Section 100306(d) that reference Medicaid pricing.
- Limit pricing and access requirement provisions in Section 100306(d) to those resultant products for which CIRM funding was the only public source of funding and that, in particular, where federal sources of funding were involved, affirm that federal policy prevails.
- Refine that revenue sharing requirements in Section 100308 be limited up to the total dollar amount of CIRM-provided research dollars.
- Remove, clarify, or narrow new “retain the right to practice” language added to Section 100306(a).
- Clarify and narrow the definition of “Biomedical Materials” in Section 100301(d), as referenced in the revised Section 100304, to only those “Entities of biomedical relevance first produced as a consequence of CIRM-funded scientific research…”

We look forward to continuing to work with the ICOC as it finalizes this policy, and we would be happy to further discuss these comments in additional detail.

Thank you for your attention to this important matter.

Sincerely,

David L. Gollaher, Ph.D.  
President and CEO  
CHI – California Healthcare Institute