COMMENTS OF THE BIOTECHNOLOGY INDUSTRY ORGANIZATION ON CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE'S NOTICE OF PROPOSED REGULATION ADOPTION CALIFORNIAN CODE OF REGULATIONS TITLE 17.—PUBLIC HEALTH PROPOSED REGULATIONS: INTELLECTUAL PROPERTY POLICY FOR NON-PROFIT ORGANIZATIONS

JUNE 19, 2006

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BY ELECTRONIC MAIL TO NONPROFITIPREGS@CIRM.CA.GOV

Mr. C. Scott Tocher
Interim Counsel
California Institute for Regenerative Medicine
250 King Street
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Comments on Proposed Regulation: Adoption of Californian Code of Regulations, Title 17.—Public Health, Proposed Regulations: Intellectual Property Policy for Non-Profit Organizations

Dear Mr. Tocher:

The biotechnology industry is one of the most research and development intensive and capital-focused industries in the world. It is the bedrock for the entire world of biomedical research. In 2003, the U.S. biotech industry spent $17.9 billion on research and development and put into the hands of the public more than 300 biotech drug products and vaccines and hundreds of medical diagnostic tests. This, despite the decades-long development time and the complex regulatory process the industry must face before bringing its products to market. The U.S. system of commercializing scientific discoveries—the funding of basic research, transferring technology from the academic sector, taking advantage of the availability of risk capital, and leveraging the fact that investors are willing to take risks—has paid off. The United States currently leads the world in the area of biotechnology because strong patent laws and flexible technology transfer systems have provided favorable incentives to mitigate the high risks.

At the federal level, rapid commercialization of scientific discovery did not fully come about until the enactment of the Bayh-Dole Act in 1980. Prior to enactment of this legislation, publicly funded research was owned by the government and offered for licensing on a non-exclusive basis or simply

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dedicated to the public. There was little incentive for businesses to undertake the financial risk to develop a product. The result was that only 5% of publicly funded discoveries were ever developed into new or improved products.[i] The Bayh-Dole Act allowed universities and research institutions to patent and retain title to their inventions. Moreover, the Act allowed for flexibility in licensing of publicly funded inventions. The motivation to license the technology in expectation of royalty payments was created. This provided a necessary impetus for the transfer of publicly-sponsored research to the private sector, thereby dramatically stimulating the commercialization of federal government-supported research. The result among other things is the existence of innovative new therapeutics, diagnostics and tools, industrial processes and agricultural products for the benefit of society.

The California Institute for Regenerative Medicine (CIRM), in its stewardship of the public funds dedicated to stem cell research, has proposed policies that are antithetical to the federally tested system of technology transfer created through the Bayh-Dole Act. Although the CIRM draft IP policies are often described as going "beyond" Bayh-Dole these policies, if implemented, would actually take California backward to the pre-Bayh-Dole era when few promising publicly funded technologies ever reached the hands of the public.

BIO AND THE BIOTECHNOLOGY INDUSTRY

The Biotechnology Industry Organization (BIO) is a trade association of more than 1,100 companies, universities, research institutions, and affiliated organizations worldwide. BIO members are engaged in biotechnology research on medicines, diagnostics, agriculture, and environmental and industrial applications. BIO represents an industry that has already provided more than 300 million people with benefits from more than 250 commercially approved drugs, biologics and vaccines. [ii] Over 20 percent of BIO's membership resides in the state of California—the birthplace of biotechnology. BIO's members, the majority of whom are involved in the development of healthcare related products and services, have a long history of focusing their efforts on intractable diseases including various cancers, AIDS, Alzheimer's disease, heart disease, diabetes, multiple sclerosis and arthritis. In addition, biotechnology companies and researchers are responsible for the development of hundreds of medical diagnostic tests, many biotechnology-derived foods, environmental products and other industrial products. [iii]

In California over 1200 "spin-off" companies have been established over the years through the effects of Bayh-Dole from Stanford, and this is just one example. Successful "spin-off" ventures help bring valuable products to market, and also develop the vibrant Silicon Valley which leads in high tech, biotech, and medical device industries. This thriving business ecosystem, in turn enables further R&D initiatives and two-way technology flow between academia and industry.

CIRM'S IP POLICIES

BIO supports the goals of the California Stem Cell Research and Cures Act (Prop 71) which is designed to fund stem cell research as well as other opportunities for the development of regenerative medical diagnostics, treatments and therapies. The potential for the development of innovative products and technologies in California as a result of its stem cell initiative is extraordinary. The existence of the infrastructure necessary to realize the promise of stem cell research in California – in the form of unsurpassed research institutions and intellectual capital as well as a robust venture capital and commercial development community – further enhances the chances that successful cures, therapies, diagnostics, and tools will result from the CIRM's efforts. But as we have learned too well in the federal setting prior to the enactment of the Bayh-Dole Act, an infrastructure without an environment and framework conducive to commercialization will only generate promising research that will languish on the shelves of California's pre-eminent research institutions for years to come. Further, without financial incentives, industry is unlikely to license technologies developed by Universities with CIRM funds, which will result in the loss of additional funding for further research and development in this critical area.

There are two major components of the proposed intellectual property policies for non-profit organizations (IPPNPO) which reduce the likelihood that CIRM-funded discoveries will reach patients and other end users. These concerns are also salient to intellectual property policies in general.

First, the proposed policy mandates that grantee organizations exclusively license CIRM-funded patented inventions only to organizations that plan to provide access of resultant products to uninsured California patients. The policy further mandates that the licensees agree to provide any resulting therapies that will be purchased by California public funds at a cost not to exceed the federal Medicaid price. These proposed licensing restrictions restrain free markets by imposing a de facto price control over the resulting

product. In its proposal, CIRM recognizes that economic incentives will be necessary to enable commercial development. [iv/] However, the requirement to only grant exclusive licenses to organizations with plans to provide resultant products at Medicaid prices in effect reduces incentives for companies to commercialize such products.

Drug development in the biopharmaceutical industry is a very high risk endeavor. A study has shown that pharmaceutical companies spend an average of $800 million and 10-14 years to develop a single pharmaceutical product[v]. Companies will only invest in such high risk endeavors if there is a potential to recoup the development cost. At the same time, the vast majority of BIO's members are start-ups that that currently have no products on the market. Rather they rely on their patent assets to generate R&D financing from the private sector. For example, in 2002, only approximately 1.6 percent of the industry's R&D funding originated from government sources; the remaining 98.4% came from private sector financing. In many instances these patent assets are licensed far upstream in the development time-line, making it difficult for either a company or its investors to know what pricing and access regimes will be viable. Without flexibility in product pricing and structure of license arrangements, the ability to secure private funding to support the development of innovative stem cell products would be severely hindered.

A second area of concern in the IPPNPO is the research use exemption provision. This provision requires that CIRM funded inventions be provided to all "California research institutions" "for research purposes" "at no cost".

Research tools are core enabling technologies for future stem cell research. Currently, many companies license research tool IP from universities, enhance it, and commercialize the resulting products. Most of these products are then made widely available to the research community through websites, catalogues, in house supply centers, and the like. The benefit to the research community is enhanced biological understanding and faster, simpler, and more repeatable tools and techniques of experimentation. In research tools, as in therapeutics, the opportunity to patent and to commercialize a product is a powerful incentive for innovation.

The IPPNPO provision interrupts this well functioning system by requiring that all CIRM funded IP be made available to all California researchers at no cost. This requirement greatly reduces the incentive for a company to commercialize a CIRM funded research tool, since the California research

market must be served at no cost. As a result, promising CIRM funded research tools are likely to remain on the shelf, undeveloped, as was so often the case in the pre Bayh Dole era at the NIH.

These provisions substantially reduce or eliminate the incentives to commercialize patented stem cell-related technologies and products even in spite of the generous funding provided by Proposition 71. The opportunity for firms to take the inventions supported by Prop 71, and further invest their own money and efforts into creating innovative tools, technologies and products for chronic diseases will be negated by strict obligations and inflexible licensing schemes proposed by the CIRM.

CONCLUSION

BIO urges CIRM to carefully reconsider these issues in the formation of any policies. Failure to do so would likely have significant undesirable consequences on CIRM's ability to achieve its goals. For example, during the 1990's two issues similar to those presented by CIRM's proposed policies arose out of public policy initiatives. Concerns that health care reform proposals from the early 1990's could lead to price controls led to perturbations in the market for biotechnology investment. The impact potential price controls on the biotechnology industry was immediate and powerful. The capital markets crashed and investment nearly dried up.

A similar result occurred in 1999 when President Clinton and Prime Minister Blair were cited in the press as supporting the notion that certain classes of patented genetic information should be freely available to all at the time the human genome was "unraveled." Despite a clear correction by the President the next day, it took six months for the biotechnology capital markets to recover.

In both cases, a threat to free-market protection of intellectual property drove investors away from biotechnology and research. The Bayh-Dole Act was designed to facilitate the transfer of publicly funded research to the private sector for further development and commercialization. The careful balance set forth in the act has been hugely successful. The impact of the Act is evident today in the over 1400 biotechnology companies in the United States and hundreds of biotechnology products in the marketplace.

We have learned from history that attempts to control prices or restrict flexibility in licensing are likely to disincentivize biotechnology companies

from undertaking the huge risks to bring innovative products and services to all Americans.

BIO urges CIRM to reconsider its proposed IP policy and look to the Bayh-Dole Act as an overwhelmingly successful example of a framework that has achieved goals similar to that of CIRMs'.

Sincerely,

James C. Greenwood
President & CEO
Biotechnology Industry Organization

[i] Association for University Technology Managers, Annual Report, 2003

[ii/] Facts about the biotechnology industry are derived from data compiled by (BIO) *at* <www.bio.org/er/statistics.asp>.

[iii/] Kenneth I. Shine, President, Institute of Medicine, "Welcome," *in* National Research Council, *Serving Science and Society in the New Millennium*. Washington, D.C.: Nat'l Academy Press, 1998. (proclaiming that, whereas "the 20th century will be known as the century of physics and astronomy ... [b]ut the 21st century will be the century of the life sciences in all their ramifications.").

[iv/] Section 100306, lines 9-15 of CIRM Proposal.

[v] http://csdd.tufts.edu/NewsEvents/RecentNews.asp?newsid=29, May 13, 2003, Press Release.