BIOTECHNOLOGY INDUSTRY ORGANIZATION
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May 26, 2009

NIH Stem Cell Guidelines
MSC 7997
9000 Rockville Pike
Bethesda, MD 20892-7997

Re: Draft National Institutes of Health Guidelines for Human Stem Cell Research (FR Doc. E9-9313)

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the National Institutes of Health (NIH) for the opportunity to submit comments on the draft guidelines for human stem cell research, published in the Federal Register on April 23, 2009.

BIO represents more than 1,200 biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental biotechnology products, thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment.

Our comments follow.

1. **BIO supports federal funding for human embryonic stem cell research.** BIO is pleased that the draft guideline provides a strong endorsement for human embryonic stem cell research. This research has consistently demonstrated its potential to lead to cures and treatments for diseases and disabilities such as diabetes, Parkinson's Disease, ALS, and spinal cord injury. That's why BIO has supported numerous legislative and administrative actions over the past decade that were designed to support this research and facilitate the commercialization of its results into treatments for patients.

Although BIO member companies are typically not NIH grantees, it is critically important that the NIH expand its funding of human embryonic stem cell research to advance scientific knowledge. Biotechnology companies – and the FDA – often rely on published academic studies (often done with NIH grants) to provide basic information about relevant science. Companies also develop, manufacture and commercialize discoveries that originate from academic labs into treatments for patients.

In addition, by removing the previous limits on funding, Executive Order 13505 and these guidelines send the signal to the private markets that this field will be advancing without restriction, making it easier for companies to receive the private financial support necessary to commercialize the research.

2. **BIO supports the provision in the draft guidelines that distinguishes embryonic stem cells from embryos.** Some research opponents have argued that federal law prohibits expanded human embryonic stem cell research funding. The draft guidelines make clear in Section I that while NIH is prohibited from funding research using embryos, human embryonic stem cells are not embryos. This is scientifically accurate since although embryonic stem cells can be turned into virtually any cell in the body, they cannot become a human being. Therefore expanded funding of research using these cells is lawful.

3. **BIO supports the overall approach of the draft guidelines.** The draft guidelines make clear that researchers using human embryonic stem cells can receive federal funding regardless of when the cells were derived. In addition, the draft specifies that the cells must be derived under an ethical framework. BIO supports the requirement that researchers meet high ethical standards to give confidence to patients and embryo donors that the research will be done in accordance with the highest standards.

BIO supports the specific requirements in the draft guideline that require the cells to have been derived from leftover embryos from *in vitro* fertilization (IVF) clinics that are in excess of clinical need and were going to be discarded, that the donors voluntarily consented to provide embryos for research, and that there was no financial inducement to donate. These are similar to the funding rules promulgated by the Bush Administration and found in the Stem Cell Research Enhancement Act, legislation that was passed by Congress several times.

4. **BIO is concerned that some existing cell lines may not be eligible for funding under the terms of the draft guidelines or will be perceived as not ethically derived.** Scientists have derived hundreds of embryonic stem cell lines over the past decade. Some of these lines were eligible for funding under previous NIH rules. These cells have demonstrated the potential of this research and have been used in thousands of experiments. They also have been the source of numerous important breakthroughs. In addition, some of these cells are currently being used in the first clinical trial using human embryonic stem cells.

Unfortunately, our reading of the draft guideline would seem to disqualify some of these cell lines from research funding. Specifically, some of the requirements listed in Section II B, while appropriate at the present time, may not have been consistently followed in every IVF clinic over

the past decade. They were not required by the Bush Administration or the NIH. Thus, it is unclear if some or many embryonic stem cell lines currently in use meet these requirements.

If existing cell lines are not eligible for federal funding, research will slow as scientists derive and distribute newly eligible lines, and experiments with existing lines will have to be adjusted. In addition, scientists will be forced to segregate their lab space and equipment between that which is federally approved and that which isn't. Thus, universities will be forced to continue the practice of creating duplicate laboratory space for embryonic stem cell researchers, driving up costs and creating inefficiencies.

In addition, prohibiting funding for research using existing lines would hamper the continuity of research with a particular cell line and the ability to compare results obtained with current cell lines and new ones. It also hurts companies that distribute these lines and have built enhanced (engineered) cell lines to accelerate scientific research using these parental lines.

Moreover, it could call into question the validity of the cells being used in the ongoing clinical trial. NIH should affirmatively state that the lines currently being used in the ongoing clinical trial were ethically derived. It would be a disservice to patients for the trial to be delayed or halted because federal officials have retrospectively decided that the process by which the embryonic stem cells were obtained is ethically tainted.

In addition, BIO urges NIH to develop a process whereby existing cell lines can still be eligible for funding. This process should guarantee that the embryos were donated under strict ethical rules using standards that were accepted at the time of the derivation. For example, the final guideline could require documentation that the standards developed in Bush Administration and in the Stem Cell Research Enhancement Act (S. 5) were followed. That is, the embryos were from IVF clinics, they were in excess of clinical need and were to be discarded, they were originally created for reproduction purposes, and the donor provided written informed consent and was not financially induced to donate. Alternatively, the final guidelines could require that when an IRB reviews a research proposal, it should determine that ethical standards were met.

5. **BIO urges the NIH to re-visit these guidelines as technology advances.** The draft guidelines take the unusual position of stating affirmatively what the NIH will not fund. Since they specify in detail the process by which eligible cell lines will be derived, it is unnecessary to spell out what research is not eligible for funding.

In addition, BIO opposes the prohibition on funding for research using embryonic stem cells from somatic cell nuclear transfer (SCNT) and parthenogenesis.

Parthenogenesis has yielded several pluripotent human stem cell lines that have therapeutic potential for immune matching and are currently being used in privately-funded U.S. laboratories and internationally as unique research tools to study immune reaction and cell differentiation. While SCNT and other techniques have not yet successfully yielded embryonic stem cells, NIH should not permanently close the door on new technologies that have merit. For example, many scientists believe these techniques can be useful for identifying genetic causes of disease.

Thus, BIO urges the NIH to remove the language specifically prohibiting funding for SCNT and parthenogenesis. Further, the agency should specify in the final guidelines that it will periodically review and update its guidelines to account for scientific and technological advances.

Thank you for the opportunity to comment.

Respectfully submitted,

/s/

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