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Before the

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Concerning
STEM CELL RESEARCH

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Mr. Chairman and members of the Subcommittee: I am Lawrence Goldstein. I am here today as a representative of the American Society for Cell Biology. The Society represents 10,000 basic biomedical researchers throughout the United States and the world, most of whom work in our Nation's leading research universities and institutes. It is my pleasure to appear before you again and it is a particular honor to be here with Mr. Reeve who has been such an articulate and effective spokesperson on behalf of biomedical research.

I am a Professor in the Department of Cellular and Molecular Medicine at the University of California, San Diego School of Medicine. I am also an Investigator of the Howard Hughes Medical Institute. Before moving to San Diego I was a Professor in the Department of Cellular and Developmental Biology at Harvard University for 10 years. My research focuses on the molecular and genetic analysis of protein motors and their roles in neuronal function and neurodegenerative disease.

I want to thank you, Senator Specter and Senator Harkin, for ensuring through your leadership of this important subcommittee that the NIH is funded sufficiently to pursue the most promising basic and clinical research opportunities. One such opportunity is embryonic stem cell research. S.2015, "The Stem Cell Research Act Of 2000" which you have introduced, would allow federally-funded scientists to not only use, but also to derive embryonic stem cell lines for research purposes with the goal of developing new therapeutic strategies to treat devastating human disease. The American Society for Cell Biology stands firmly in support of this bill.

Just over a year ago, a milestone in biomedical research was achieved when human embryonic stem cell lines were obtained by growing cells from the inner cell mass of early stage human embryos. This discovery catalyzed a serious debate on Capitol Hill about whether federal funds should be used to support further research in this area. At issue is whether the merits of public funding and the dreadful burden of disease balance concerns about the origin of these special cells. Because of its great potential to treat disease and alleviate human suffering, the American Society for Cell Biology along with many other scientific organizations and societies have expressed strong support for Federal funding of this important research.
The American Society for Cell Biology supports The Stem Cell Research Act Of 2000 for four major reasons:

First, research work over the past 20 years using mouse embryonic stem cells has demonstrated the promise of embryonic stem, or ES, cells for basic research and potential disease therapy. These cells by themselves cannot form a mouse, but they can differentiate into any of the cell types that comprise a mouse. Mouse ES cells have been used to elucidate many important aspects of normal mouse embryology and development, but, most important, mouse ES cells are currently being used in a variety of "proof of therapeutic principle" experiments in several animal models of human disease. For example, these cells appear to be able to produce neural progenitors that can repair spinal cord damage and reconstitute brain cells that produce the chemicals that control cognition, motion and sensory perception. If reproducible with human ES cells we may be able to treat Parkinson's disease and Alzheimer's disease. We may be able to produce bone marrow cells to treat cancer and other hematopoietic diseases, and pancreatic cells to alleviate diabetes. In fact, we may be on the cusp of a new era of medicine, one in which cell therapy could restore normal function to a variety of affected tissues.

It is important to note that some have argued that so-called "adult stem cells", derived from adult tissues are of equivalent promise, less ethically compromised, and should therefore be pursued exclusively. But, notwithstanding important advances in the field of adult stem cell research, it is far too early to know if adult stem cells have the same potential as embryonic stem cells, whether they can be harvested in sufficient quantities to treat or cure disease, and whether they can grow indefinitely as can ES cells. For example, for juvenile diabetes, there is little reliable evidence at present that adult stem cells could be used for treatment, and thus embryonic stem cells are the best near-term candidates for therapy. Furthermore, embryonic, fetal and adult stem cells are very different from each other. It is not at all clear that they will prove to be interchangeable or equally receptive to manipulations that would make them useful for therapy. Thus, it is likely to take years to find out if adult stem cells will be useful for treating many diseases that may be treatable sooner with embryonic stem cells. For diseases that prove not to be treatable with adult stem cells, we risk unnecessary delays for patients who may die or endure needless suffering while the promise of adult stem cells is being tested. It is critical that we not prohibit or hinder research in any of these areas.

Second, there is great medical need and urgency for stem cell research. To understand the need for rapid research progress with human pluripotent stem cells, one need look no further than many common, and often fatal, diseases such as cancer, heart disease and kidney disease. These diseases are treatable in whole or in part by tissue or organ transplants, but there are persistent and deadly problems of rejection and a woefully inadequate supply of suitable donor organs and tissues. In addition, the grim arithmetic of most organ transplants requires those who are seriously ill to wait for the tragic accidental death of another person so that they may live. Worse, for juvenile diabetes and many other diseases, there is not even a suitable transplantation therapy or other cure. Unless we use federal funds for all aspects of human pluripotent stem cell research new
treatments for these conditions may be delayed by years, and many who might otherwise have been saved will surely die or endure needless suffering.

Third, The Stem Cell Research Act of 2000 ensures that important avenues of medical research will not be restricted to the private sector. We agree with the National Bioethics Advisory Commission that, “relying on cell lines that might be derived exclusively by a subset of privately funded researchers who are interested in this area could severely limit scientific and clinical progress.” The effect of precluding federally-funded biomedical from such work will effectively bar the majority of the Nation's most prominent and most-qualified researchers from engaging in this critical research. Excluding these publicly funded investigators will close off scientific opportunities to those most qualified to make rapid and dramatic advances towards using stem cells for the treatment of disease.

We also know that a variety of poorly understood factors cause embryonic stem cells to lose their capacity to differentiate into all possible cell types. This loss of capacity may be caused by growth conditions, derivation conditions, or other variables of handling. Enabling individual publicly-funded investigators to derive cell lines using a variety of conditions in their own laboratories is the best route to finding out what conditions are critical to generate useful cells for therapeutic purposes. In the future, it is likely that cells prepared in one's own laboratory will have been derived, stored, and maintained in ways that maximize their potency for particular uses, whereas cells obtained from commercial sources are likely to be of unknown genetic background and history and therefore less useful for some important studies.

Federal funding is also the best way to guarantee that stem cell therapies are developed with the greatest consideration of the public good. Left to the private sector alone, stem-cell derived treatments may only be pursued for diseases that commercial companies project to yield the largest profit if treated. Thus, market forces could create a situation where deadly, but less widespread, diseases are ignored.

In fact, past experience has proven that allowing greater and more diverse access to breakthrough discoveries increases the likelihood of novel and innovative extensions. The Molecular Biology Revolution, which is responsible for enormous social, economic, and medical advances is the product of innovations and discoveries made by thousands of highly qualified and creative individual scientists who developed new techniques and applications. Our world would be very different today had this vital technology been restricted to just a few investigators.

Fourth and finally, the American Society for Cell Biology supports The Stem Cell Research Act of 2000 because it guarantees that this important biomedical research will proceed with the highest possible ethical standards, and with sensitivity to the public concern about the origins of these cells. The National Biomedical Ethics Advisory Commission held numerous hearings and debates on this issue and solicited input from the public, scientific experts, and religious leaders from different faiths. After careful deliberation, the National Biomedical Ethics Advisory Commission concluded that it
would be ethically permissible to prepare stem cell lines from embryos that had been obtained in the course of in vitro fertilization procedures, but were deemed by the donors and the physician to be in excess of the clinical need for the intended procedure: that is, those destined to be ethically and legally discarded.

The National Biomedical Ethics Advisory Commission also recommended specific regulatory and oversight procedures to ensure that the creation and use of human stem cells would meet the highest ethical standards. In fact, the draft guidelines developed by the NIH to govern use of embryonic stem cells specify similar criteria to those suggested by the National Biomedical Ethics Advisory Commission. Analogous procedures are wisely specified in the Stem Cell Act of 2000. These guidelines include complete separation between those who conduct research and those who donate stem cells, thus preventing any potential effort to develop embryos for the purpose of research. For federally funded research, this bill would also prohibit the use of embryos that are purchased or sold, and will continue to ensure that human embryos are not created for research purposes.

It is important to keep in mind that banning federal funding for human pluripotent stem cell research will not eliminate it. Such research will proceed in private industry and in other countries. This fact prompts serious concern that such work could be conducted in secret, without benefit of ethical regulation or public debate. Thus, prohibiting Federal funding will de facto prohibit public involvement and input into the future of this important field. Permitting peer-reviewed federal funds to be used for this research, combined with public oversight, is our best assurance that research will be of the highest quality and performed with proper ethical oversight and public input. Federal funding of this research will require the scientific community and the government to work together to establish an appropriate set of rules for this research. These rules will ensure the advancement of critical medical research and maintain respect for public sensibilities.

There are also serious ethical implications to not proceeding. Thus, while I am acutely and personally aware of the well meaning ethical concerns that have been expressed about the sacrifice of embryos to prepare stem cells, I am also cognizant that the embryos in question will be legally and ethically destroyed in any case. We must then ask: Is it ethical to literally throw away the opportunity to allow all people to benefit from the demise of these embryos? How can we justify not pursuing every reasonable means of finding cures for our friends, our parents, and our children, who will suffer and die if we do not find suitable therapies?

In thinking about the answers to these questions, I am reminded of the many parallels between our debate today about the potential use of human embryonic stem cells to treat disease and past debates about organ transplants and the proper treatment of donor families. The famous heart transplant surgeon Dr. Christian Barnard, stated it best in response to questions he received about the ethics of heart transplantation. He said: "Would it not be immoral to bury a heart when we have the ability to save a life?" We submit that the answer is the same for human embryonic stem cells. Ethics, scientific opportunity, and medical need can surely be balanced.
In closing, we have before us an unprecedented scientific opportunity to engage in a noble effort to develop new forms of medicine. That opportunity offers hope to the many millions of our citizens who rely on the shared stewardship of our scientists and our political leaders to enable science's achievements to relieve all people of the burden of serious disease.

Thank you for your courage in providing leadership on this most important — indeed, life-or-death — issue, and for inviting my testimony today.