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On behalf of the
Biotechnology Industry Organization (BIO)

Special Committee on Aging
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Regarding Stem Cell Research

Good afternoon. Mr. Chairman and members of the Committee, my name is Doug Doerfler. I am President and CEO of MaxCyte, a biotechnology company based in Gaithersburg, Maryland. I am here today representing the Biotechnology Industry Organization (BIO).

BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers and related organizations in all 50 U.S. states and 33 other nations. BIO members are involved in the research and development of health care, agriculture, industrial and environmental biotechnology products.

Thank you for the opportunity to present testimony today on the promise of embryonic stem cell research and in support of S. 471, the Stem Cell Research Enhancement Act of 2005.

My company uses patented technology to develop cell-based therapies. Because many diseases and disabilities are caused by cellular malfunction, cell-based therapies have the potential to provide medical breakthroughs in

the treatment of patients suffering from a variety of diseases. At MaxCyte, we are working to develop therapeutic products – including stem cell products – that will have uses in oncology, pulmonary, infectious and autoimmune disease and other illnesses that affect millions of people. Our company will help turn research discoveries into therapies for patients.

I want to make two points at the outset of my testimony. First, my company does not perform embryonic stem cell research. And second, BIO supports all types of stem cell research – including research using cord blood and adult stem cells.

But Mr. Chairman, I am here today to say that to help speed the development of all types of cell-based therapies, we need expanded federal support of embryonic stem cell research.

That's why BIO supports S. 471. Research with existing cell lines has demonstrated the enormous potential of this work. However, scientists have found that these lines are not genetically diverse, may be difficult to grow, and may be contaminated by animal proteins.

That's where your legislation makes its mark. It appropriately expands support for this research by making more cell lines eligible for federal funding while creating a framework to ensure that this research is performed ethically.

The legislation will allow federal funding of research using embryonic stem cells provided the cells were obtained from *in vitro* fertilization procedures that are either in excess or otherwise do not qualify for transplantation. The bill also requires the informed consent of the donors and prohibits any financial inducement to donate. Moreover, the bill calls on the National Institutes of Health (NIH) to develop appropriate guidelines to govern this process. We believe this is an important step and is similar to the way the Asilomar Conference helped ease public anxiety and spur the development of recombinant DNA technology during the 1970s.

This Committee has heard – and will continue to hear – about the potential benefits of embryonic stem cell research regarding cures and treatments for diseases and disabilities. Embryonic stem cells have the capacity to be turned into any of the body's cell types, meaning they could possibly be

developed into replacement cells and tissue for patients whose own cells are malfunctioning. This has not yet been shown to be true for adult stem cells.

It is that potential that has thus far generated the most enthusiasm within the scientific community. According the NIH and the National Academies of Science, human embryonic stem cells have shown incredible promise toward developing breakthrough treatments for a variety of intractable diseases including various cancers, kidney disease, diabetes, multiple sclerosis, Parkinson's Disease and Alzheimer's Disease.

However, I would like to discuss other reasons to support this research.

The majority of companies currently involved in stem cell research use non-controversial sources of cells such as umbilical cord and bone marrow.

It's important to emphasize that embryonic stem cell research will have a positive impact on these areas of research and will further the development of cell-based therapies.

For example, embryonic stem cell research will lead to greater scientific understanding of cell differentiation – the process by which our cells become specialized to perform certain functions – and proliferation – the process where cells expand, or multiply for controlled use as a potential therapeutic.

In addition, if this bill is enacted, more genetically diverse cell lines will be available for funding. Scientists will then be able to learn more about how and when genetic anomalies cause cells to malfunction. This could help researchers understand the root causes of many diseases and therefore lead to the development of truly breakthrough therapies.

Expanded support of embryonic stem cell research could also go a long way toward reducing the time and expense needed for drug discovery. Using embryonic stem cells in drug testing could be significant because of the cells' ability to turn into all types of human cells. New chemical or biological compounds meant to treat diseases could be tested in specific human cells prior to their use in live human beings.

In addition to time saved, this process could reduce adverse events from drug candidates because tests on human cells might reveal harmful side effects before the drug is given to patients.

Finally, Mr. Chairman, I've heard opponents of your bill say that it is not necessary to expand federal support for stem cell research because many states are moving forward with their own programs.

Nothing could be farther from the truth. Put simply, while state support is enormously helpful, there is no substitute for an increased commitment from the NIH. First of all, the NIH can jump start an area of research with an influx of funds.

But just as importantly, the NIH provides the infrastructure and a uniform set of rules for the scientific community – especially as basic research is turned into therapies. The biotech industry has worked effectively for many years with the NIH and academia to develop products for patients. Forcing companies to deal with a patchwork of state regulations and requirements will create inefficiencies and confusion that could inhibit critical collaborations and slow development of treatments for patients.

In conclusion, embryonic stem cell research holds the promise to dramatically improve our nation's ability to develop cures and treatments for disease. Whether these therapies are the direct result of this research or come about due to the advances in scientific knowledge that will come from this work, it seems clear that our nation should increase its commitment.

BIO supports your legislation, Mr. Chairman, because it will expand federal support for this important research.

Thank you for the opportunity to testify today.