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Committee of Health, Education Labor, and Pensions

My name is Dr. George Q. Daley and I'd like to begin by thanking the members of the Committee for inviting me here today. I believe passionately in the scientific value of stem cell research, and I am eager to present my views to the Committee.

I am an Associate Professor at the Harvard Medical School based at the Boston Children's Hospital. I am Associate Director of the Children's Hospital Stem Cell Program and a founding member of the Harvard Stem Cell Institute. I serve on the Public Policy committee of the American Society for Cell Biology, which represents over 10,000 scientists, and I am President-Elect of the International Society for Stem Cell Research, the world's leading organization of stem cell scientists, which has grown to over 2500 members in just over 4 years.

As a practicing physician-scientist, I run a busy research laboratory at the Children's, where we study adult stem cells of the blood—both their normal regulation and their pathology, as in leukemia—and we study the formation of blood during embryonic development. For this, we use embryonic stem cells. I also care for adults and kids with malignant and genetic bone marrow conditions—diseases like leukemia and lymphoma, immune deficiency, and sickle cell anemia. Many of these diseases can be cured by bone marrow transplantation—a form of stem cell therapy that harnesses the power of adult blood stem cells, or as you will hear (or have heard) from Dr. Wagner, from stem

cells in Umbilical Cord Blood. While transplants are effective for some, the reality is that marrow replacement represents a heroic attempt at a life saving therapy for fatal diseases. The transplantation regimen itself is highly toxic. I would not wish this therapy on anyone who was not otherwise facing a potentially terminal illness. As a direct response to these shortcomings of adult stem cell therapies, my lab investigates the formation of blood stem cells from embryonic stem cells, and is pursuing strategies for making rejection proof, autologous tissues for transplantation. Our current treatments for many blood diseases are stone-age, and only through research can we hope to make progress. I believe that embryonic stem cell research holds the key to treating many blood diseases.

Stem cells come in many varieties. Even the term "stem cell" is a very general term. It defines a generic category of cells that has many members with different properties. It's about as specific as the category "seed". Seeds of all types share many properties, but an apple seed makes apple trees and an orange seed makes oranges. When we compare apples and oranges no one confuses the two. To a biologist, the distinctions between seeds are crucial, as are the distinctions between different types of stem cells. No credible biologist would argue that one type of seed can teach you all you need to know about all seeds and all fruit. Yet somehow, when we speak about stem cells in the current debate, people tend not to appreciate the differences, and consider them all interchangeable.

The media has covered a long list of "breakthroughs" that purportedly represent new

sources of stem cells that substitute for embryonic stem cells. Initially, it was the Multipotential Adult Progenitor Cell from Catherine Verfaillie's lab in Minnesota, later it was the fat stem cell, then umbilical cord blood stem cells, and stem cells from testes. Just last week we heard reports about stem cells from amniotic fluid. All of these new types of stem cells are important tools for research and may even one day yield new therapies. However, none of them is the equivalent of embryonic stem cells. Perhaps they can do some of the things that embryonic cells can do, but they cannot do all of them. The differences between these other stem cells and embryonic stem cells are very, very important.

We have also heard that there are alternative means of generating embryonic stem cells without sacrificing embryos. There have been exciting recent developments that claim "reprogramming" of adult cells back to their primitive embryonic state, either by cell fusion with existing embryonic stem cells, or by introducing a small number of genes. Again, these achievements are noteworthy and fascinating, but they have not yet produced cells that faithfully mimic or replace the functions of true ES cells.

After many years of competing claims, ES cells remain the most versatile of all stem cells. ES cells are the gold standard for the biological concept of pluripotency, and it has been known from over 20 years of research in the mouse that ES cells can make all the cells of the body. ES cells have unique properties and they fulfill a unique purpose in biological research. Human ES cells are irreplaceable tools for understanding the earliest stages of human development. They are unique precisely because they come

from the earliest human embryos--before implantation into the womb, before even the most rudimentary human form has begun to take shape. Understanding how these primitive cells orchestrate the process of human development represents one of the greatest goals of modern biology. Figuring out how amniotic stem cells work or fat stem cells work will not teach us about the earliest days of human development. Many different types of stem cells—adult and embryonic--may prove useful for therapies. But embryonic stem cells are the only stem cells that have been proven to form all cells in the body, and this feature alone makes them worthy of study.

With regards to medicine, it is sometimes said by opponents of ES cell research that ES cells have never cured anyone. This is a patently unfair assertion because human ES cells have only been around for only 9 years, and even now cannot be considered routinely available to scientists in the United States. However, the detractors of ES cells are naïve in trivializing the contributions that ES cells have made to biomedical research. Mouse ES cells have been used extensively to model human disease and to study how gene variations influence cancer, heart disease, neurodegeneration, metabolic disease, and many, many others. Indeed, a paper published in 2003 reported that gene knock-out strains of mice, which derive from ES cells, provided key target validation for the effects of the 100 best-selling drugs (Zambrowicz and Sands, Nature Reviews, 2003). It is therefore fair to say that ES cells have already saved lives--not directly through cell replacement therapies--but indirectly through key insights into human disease and the development of new drugs.

In closing, I want to stress that there is no credible scientific argument that would justify studying only adult stem cells to the exclusion of embryonic stem cells. Medical science does not advance fastest by cutting off fruitful avenues of research that the overwhelming majority of scientists and leading scientific societies like the ASCB and the ISSCR believe are vital. We must promote embryonic and adult stem cell research with equal vigor. We need a more conducive Federal policy for human embryonic stem cell studies, and Senate passage of Stem Cell Bill would be a healthy start. This vital research should not be left up to the States to fund. We need to stop making pseudo-scientific arguments against embryonic stem cell research, and get on with the scientific challenges ahead.

Thank you.