

Testimony to House Committee on Energy and Commerce, Subcommittee on Health
“Stem cell science: the foundation for future cures” May 8, 2008
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Thank you for the invitation to speak today on the subject of stem cell science. My name is George Daley and I am an Associate Professor of Biological Chemistry, Medicine, and Pediatrics at Children’s Hospital Boston and Harvard Medical School, a core faculty member of the Harvard Stem Cell Institute, an investigator of the Howard Hughes Medical Institute, and the current President of the International Society for Stem Cell Research (ISSCR), the major professional organization of stem cell scientists worldwide. My laboratory studies blood development, blood cancer, and experimental transplant therapies for diseases like sickle cell anemia, immune deficiency and leukemia. In my clinical duties at Children’s Hospital, I care for patients with these devastating blood diseases, and see first hand the need for better treatments. Stem cell research offers hope.

Let me recount the stories of two patients I cared for recently at Children’s Hospital that illustrate the shortcomings of current therapies. One was a young African-American boy with sickle cell anemia, suddenly struck down by what we call a pain crisis. When I saw him in the emergency room, he was writhing on the gurney, and whimpering in pain. Despite powerful, high doses of intravenous morphine, I was unable to give that child adequate relief from his pain and suffering for several days. A second case was an infant who suffered repeated infections and had spent half his young life in the hospital hooked up to intravenous antibiotics. His disease was immune-deficiency, and unfortunately he had no sibling donors for a potentially curative adult stem cell transplant. Stem cell research is laying the foundation for improved treatments for these kids, and countless other children and adults with debilitating, life-threatening diseases.

All stem cells—whether from embryonic, fetal, neonatal, or adult sources—hold great promise. The crowning scientific achievement of the twentieth century was the sequencing of the human genome, and the dominant mission of twenty-first century science is to discover how that blueprint drives the formation of tissues and organs, and how tissues are sustained, repaired, and rejuvenated over time. Stem cell research goes to the core of human biology and medicine.

Much excitement in stem cell research has focused on a property of embryonic cells called pluripotency—the capacity to generate all of the tissues in an organism. Recently, several laboratories, including my own, reported that a small set of genes linked to pluripotency in embryonic stem (ES) cells can be inserted into human skin cells to induce pluripotency—to endow skin cells with this same remarkable capacity to become a seed for all tissues in the body. By using gene-based reprogramming to make these so-called induced pluripotent stem cells (called “iPS cells”), scientists can now produce customized, patient-specific stem cells in the Petri dish. In a matter of weeks, we can take cells from a patient’s forearm and transform them into pluripotent stem cells that we believe closely approximate embryonic stem cells. This is a major breakthrough in medical research, empowering scientists to create cellular models of human disease. It may also mean that one day we will treat patients with rejuvenated and repaired versions of their own tissues.

Realizing this promise will take time. A key concern is that the viruses used to carry the reprogramming genes into human skin cells can cause cancer. Moreover, the genes and pathways the viruses stimulate are themselves associated with cancer, raising the concern that even if viruses can be eliminated from the process, the reprogrammed cells might remain predisposed to cancer. For these reasons, iPS cells may never be suitable for use in patients. I sincerely hope that iPS cells are the long-sought-after customized patient-specific stem cell, but much more research must be done.

Even with iPS cells in hand, my laboratory will continue to study embryonic stem cells. First, we need to directly compare the capacity of these two types of stem cells to generate specific tissues. Some very preliminary data has suggested that iPS cells may be less potent than embryonic stem cells in making blood, while others are noting a deficiency in making heart muscle cells. It will take years for scientists to understand the similarities and differences between these two valuable classes of pluripotent stem cells. Even with iPS cells in hand, my laboratory will continue to investigate somatic cell nuclear transfer as a means of generating pluripotent stem cells. Reprogramming by nuclear transfer is faster and may entail very different mechanisms than gene-based reprogramming. Learning why may lead to better methods for making iPS cells.

The iPS breakthrough is being heralded by opponents of embryonic stem cell research as a solution to the long-smoldering debate over the necessity of embryonic stem cell research. We have heard the arguments for many years, first made when multi-potential adult progenitor cells (MAPCs) were reported in 2002, and later when stem cells were isolated from Fat and Amniotic fluid: we are told that alternatives are available that preclude the need for embryonic stem cell research. Congress has been wise to not yield to such arguments. Indeed, it was embryonic stem cell research that led directly to the breakthrough in iPS cells, and my own laboratory was poised to generate iPS cells in large part because of our experience and expertise in deriving and culturing human embryonic stem cells. Today, it would again be a mistake to place limits on the tools available to biomedical scientists to pursue the next medical breakthroughs. The right course for biomedical science and ultimately the right decision for patients and our health care system, is to expand the scope of federal funding for all forms of stem cell research, including the many lines of embryonic stem cells created after the President's artificial deadline of August 9th, 2001.

Yesterday, in my address to the Congressional Biomedical Research Caucus, I was asked the question: "Do we still need research on embryonic stem cells?" to which I replied a resounding "Yes." Embryonic stem cells remain the gold standard today and will remain so for the foreseeable future. If we are to maximize the pace of scientific discovery and accelerate development of new treatments for disease, we must continue to vigorously pursue all forms of stem cell research, using ES cells derived from embryos, pluripotent stem cells generated by nuclear transfer and gene-based reprogramming, and adult stem cells. Passage of the bill HR-810 originally proposed by members Castle and Degette remains a worthy goal.