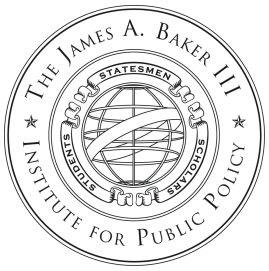

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Frequently Asked Questions: Stem Cells

By

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STEM CELL BIOLOGY

What are stem cells?

Stem cells are cells that have the ability to become many different types of cells in the body. Essential for normal development, stem cells enable the body's regenerative capacities so that tissues can undergo normal repair and replacement.

How do scientists currently obtain stem cells?

In humans, there are many sources of stem cells. Embryonic stem cells are typically isolated from a blastocyst, the scientific term for a fertilized egg around five to six days after fertilization. Cord blood stem cells are obtained from the umbilical cord and placenta. Adult stem cells can be found in several adult organs and tissues such as bone marrow, muscle, or brain.

What are some of the uses of stem cells?

Stem cells can be used to expand our knowledge about cell division, including the abnormal cell division associated with cancer. Stem cells also provide insight into how cells develop specialized functions. For example, an embryonic stem cell might generate a blood stem cell that, in turn, produces an immune cell in a process called "differentiation." Stem cells also help us understand early human development and can be used by researchers to determine toxicity of new drugs in advance of human testing. In addition, stem cells could potentially be used in regenerative medicine to replace injured or damaged organs and tissues.

What are the differences between adult and embryonic stem cells?

Embryonic stem cells are pluripotent, which means they can be induced to differentiate into any cell type. Most adult stem cells are multipotent, which means they can only differentiate into the types of cells found in their environment or in the particular tissue or organ where they reside.

Another key difference between embryonic and adult stem cells is the number of cells that can be isolated and grown *in vitro* (in the lab). Large numbers of embryonic stem cells can be grown *in vitro* from a single blastocyst. By contrast, adult stem cells are rare and methods of growing them still need to be perfected. Furthermore, due to their limited numbers, it is difficult to isolate a group of adult stem cells in pure form without having them contaminated with differentiated cells.

Why do stem cells have to come from an embryo? Why can't we use adult stem cells?

For some treatments and research, such as bone marrow transplants and heart disease, adult stem cells can be used in place of embryonic stem cells. But to study early development, only embryonic stem cells can be used because adult stem cells have progressed too far into a differentiated state. Furthermore, some tissues and organs do not have a sufficient population of adult stem cells, so the only way a cell-based treatment could be created would be using an embryonic stem cell. Finally, an ongoing challenge in any transplantation setting is the limitation of donors. Because embryonic stem cells can grow indefinitely before differentiating, they are best suited to fulfill the needs for cell and tissue transplantation in regenerative medicine.

Are there any alternatives to using embryonic stem cells?

Another option for creating embryonic-like stem cells without using human eggs is reprogramming normal cells to become pluripotent cells (known as induced pluripotent stem cells, or iPS cells). This innovative procedure has problematic aspects, though. To reprogram the cells, genes are introduced into the cells by way of a virus, which may cause adverse effects. Additionally, one of the genes necessary for the process contributes to unrestrained growth or cancer. However, research is currently being conducted to find alternative methods of reprogramming, such as using chemicals, instead of viruses, to "turn on" the genes. If scientists can resolves these issues, induced pluripotent stem cells would be a valuable source for cell therapies because donor stem cells could be reconfigured to be genetically identical to the recipient's cells, and the issue of immune rejection would be eliminated.

Why use embryonic stem cells if induced pluripotent cells are the same and do not require the destruction of an embryo?

Embryonic stem cells are the gold standard for stem cell research because of their ability to grow indefinitely before differentiating and potential to become any cell in the body. While induced pluripotent cells have great potential, their potential has not been thoroughly researched to determine how useful they will be therapeutically. In addition, induced pluripotent cells have been artificially manipulated, and it is uncertain how useful they are in illuminating basic scientific questions of early development and cell specialization.

Have stem cells cured any diseases yet?

There is a lot of research using both adult and embryonic stem cells for treatment of injuries and diseases. Adult stem cells from bone marrow have been used for years to treat cancers and problems that affect the

immune system. The use of stem cells in treating other diseases and conditions, such as heart and liver disease, is being investigated.

Embryonic stem cells are starting to be tested on humans to treat spinal cord injuries. In January 2009, the U.S. Food and Drug Administration (FDA) approved the first clinical trials using embryonic stems cells to treat acute spinal cord injuries.[1] Other potential therapies using embryonic stem cells include treatments for Parkinson's disease, juvenile diabetes, and retinal degeneration.

Induced pluripotent cells are new—the first human cells were created in 2007[2,3]—and they have yet to be utilized for therapeutic research. Before they are ready for clinical use, researchers need to find a better method for reprogramming the cells to be specialized.

Why have there been more therapies using adult stem cells than embryonic stem cells?

Research on adult stem cells dates back to the 1950s and has received federal funding for more than 50 years. Adult stem cells were first demonstrated in bone marrow in 1961,[4] with the first successful transplant in 1968.[5] By contrast, the first human embryonic stem cell was isolated in 1998.[6] Research on embryonic stem cells in the United States in the past decade has been severely limited by the restrictions on federal funding.

What are some of the ethical concerns associated with embryonic stem cell research?

This new area of research has great potential, but it is not without its controversies. Many ethical dilemmas surface because of the creation and destruction of human blastocysts, as well as the potential to clone an entire human being (reproductive cloning). Furthermore, since many embryos come from *in vitro* fertilization clinics, it is essential that clear guidelines be developed to ensure donations for research purposes include informed consent. Donors must be adequately advised on how the embryo will be used and what the donors' rights are.

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[1] Andrew Pollack, "F.D.A. Approves a Stem Cell Trial," *New York Times*, January 23, 2009, http://www.nytimes.com/2009/01/23/business/23stem.html.
[2] K. Takahashi et al., "Induction of Pluripotent Stem Cells from Adult Human Fibroblasts by Defined Factors," *Cell* 131, no. 5 (2007): 861–72, PMID 18035408.
[3] J. Yu et al., "Induced Pluripotent Stem Cell Lines Derived from Human Somatic Cells," *Science* 318, no. 5858 (2007): 1917–20, PMID 18029452.
[4] J.E. Till and E.A. McCulloch, "A direct measurement of the radiation sensitivity of normal mouse bone marrow cells," *Radiation Research* 14 (1961): 213–222, PMID 16722000.
[5] J.A. Hansen, "In Memoriam: Robert A. Good, M.D., Ph.D.," *J. Clin. Imm*. 23, no. 6 (2003): 539–40.
[6] J.A. Thomson et al., "Embryonic stem cell lines derived from human blastocysts," *Science* 282, no. 5391(1998): 1145–7, PMID 9804556.

POLICY

Was President George W. Bush really the first president to fund embryonic stem cell research?

Yes, President Bush did fund the first human embryonic stem cell grants. Human embryonic stem cells were isolated in late 1998. President Bill Clinton's administration discussed funding embryonic stem cell research, but did not fund any grants in the area.

What research did President Bush fund?

The Bush administration funded embryonic stem cell research using cells that were obtained before August 9, 2001. This consisted of 21 different cell lines, which were viable and available to U.S. researchers.

What research is President Barack Obama funding?

In March 2009, President Obama signed an executive order that removed the date restriction on embryonic stem cell lines. This allows research on lines created after 2001. The president asked the National Institutes of Health (NIH)—the agency responsible for funding the majority of U.S. biomedical research—to create guidelines for this new research. The guidelines, released in July 2009, allow for federal funding on embryonic stem cells that were obtained using private funding from leftover *in vitro* fertilized eggs with proper informed consent.[7] But, they do not allow funding for research using cells that were obtained from fertilized eggs created for research purposes or other methods, or cells obtained without proper documented informed consent from both the egg and sperm donor.

Can researchers use federal funds to destroy embryos and create embryonic stem cells?

No. Every year since 1996, U.S. Congress has passed an amendment on the funds for NIH that bans the destruction or harm of embryos for research. This amendment, called the Dickey-Wicker Amendment after the two representatives who wrote it, prohibits the use of federal funds to obtain embryonic stem cells, but does not apply to private funding.

How is the Obama policy different from the Bush policy?

The new guidelines allow the use of cell lines created after 2001, but only if they follow a rigorous informed consent process. Any lines created in the future must follow current guidelines and previously

[7] *National Institute of Health Guidelines for Human Stem Cell Research*, http://stemcells.nih.gov/policy/2009guidelines.htm.

existing lines must be reviewed and approved by an NIH panel of experts to determine if the informed consent process was upheld. This is more rigorous than the process for the approved Bush lines.

How does the Obama policy impact Texas?

With the expansion of cells eligible for federal funding, researchers across the country—including in Texas—can apply for money from the government to use embryonic stem cells that were created in private labs during the past 10 years. This would bring more federal dollars into the state, create more jobs, stimulate the Texas economy, allow researchers in Texas to participate in this revolutionary approach to health care, and free up foundation funding to be used in other areas of research.

How much stem cell research is going on in Texas?

The state of Texas receives approximately $1 billion from NIH for biomedical research each year. Of this, approximately $59 million was used for stem cell research in 2009 (including American Recovery and Reinvestment Act funds), with $6.5 million spent on human embryonic stem cell research (utilizing lines approved in the Bush administration and including Recovery Act funds).[8] As of May 2010, no human embryonic stem cell lines created in Texas were approved by NIH for federal funding, but two lines created at The University of Texas Health Science Center at Houston are pending approval.[9] The amount of private funding for human embryonic stem cell research within the state is unknown.

What would happen if Texas banned state funding for embryonic stem cell research?

The state of Texas does not currently fund embryonic stem cell research, but a ban could affect the use of state facilities. The ban would apply to any research currently underway, as well as federally funded biomedical research using cells approved during the Bush administration. It would also prevent researchers from applying for additional funds on newer lines.

How would a ban impact Texas' economy?

If a ban on embryonic stem cells were implemented in Texas, many researchers who are working in the field would likely leave the state to find new positions where there is more permissive regulation. In addition, a ban would create the perception that the state is anti-science and would hurt recruiting, especially of high-profile researchers. This could negatively impact the amount of federal funding brought into the state. Currently, Texas is fourth in the United States in receipt of federal research and

[8] National Institutes of Health, *Dollars Awarded by State*, http://report.nih.gov/award/trends/State_Congressional/StateOverview.cfm and *Estimates of Funding for Various Research, Conditions, and Disease Categories (RCDC)*, http://www.report.nih.gov/rcdc/categories/.
[9] National Institutes of Health, *NIH Human Embryonic Stem Cell Registry*. http://grants.nih.gov/stem_cells/registry/current.htm.

development expenditures.[10] As noted previously, Texas received more than $1 billion annually in federal funding from the NIH. This makes Texas fifth in the nation for biomedical research funding, but only sixth for stem cell and eighth for human embryonic stem cell research.[11]

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[10] National Science Board, *Science and Engineering Indicators 2010*; and National Science Foundation, appendix table 04-15 "R&D expenditures, by state,funding source, and performing sector: 2007," http://www.nsf.gov/statistics/seind10/append/c4/at04-15.pdf.
[11] National Institutes of Health, *Dollars Awarded by State*.