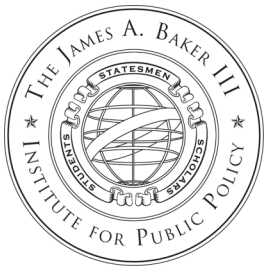

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Frequently Asked Questions: Stem Cells

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Kirstin Matthews is a fellow in science and technology policy at the Baker Institute. Matthews' research focuses on the intersection between traditional biomedical research and public policy. Her current projects include the Civic Scientist Lecture Series and Outreach Program; policy studies in research and development funding, global health and climate change; and the Baker Institute International Stem Cell Policy Program. The mission of the program is to bring together scientists, ethicists, policymakers, media experts, and community and business leaders to find new ways to engage the general public in a dialogue on these vital issues. The program includes a U.S. conference series titled "Stem Cells: Saving Lives or Crossing Lines," a series of international conferences, major public policy research and publications, workshops to bring together scholars and scientists from the international community, and conference reports on the workshops. Below, she addresses some frequently asked questions about stem cells.

BIOLOGY

What are stem cells?

Stem cells are cells that have the ability to replicate themselves for indefinite periods and to divide, producing one copy of themselves and one cell of a different type.

How do scientists currently obtain stem cells?

In humans, there are many sources of stem cells. Embryonic stem cells are 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 or "differentiate." For example, an embryonic stem cell might produce a

blood stem cell, which in turn produces an immune cell, in a process called differentiation. They also help us understand early human development. Stem cells 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 can be induced to differentiate into any cell type, but adult stem cells cannot. Most adult cells 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 volume 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 stems. 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 tissue and organs do not have a population of adult stem cells, so the only way a cell-based treatment could be created would be using an embryonic stem cell.

Are there any alternatives to using embryonic stem cells?

Another option for creating embryonic-like stem cells without using human eggs is deprogramming normal cells to become pluripotent cells (known as induced pluripotent stem cells, or iPS cells). This innovative procedure has problematic aspects, though. To deprogram 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 cancer. However, research is currently being conducted to find alternative methods of "turning on" the genes, such as using chemicals instead of viruses. If scientists can resolves these issues, 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. While induced pluripotent cells have great potential, that potential can only be analyzed when compared to embryonic stem cells to determine if the cells are functioning correctly. 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.

[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.

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 the clinic, researchers need to find a better method for deprogramming the cells as well as reprogramming the cells to be specialized again.

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 been supported 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] But research on embryonic stem cells in the United States over the past decade was severely limited by lack of federal funding

What are some of the ethical concerns to be aware of when it comes to 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). The donation of embryos for research purposes requires informed consent that adequately advises donors on how the embryo will be used and their rights after the embryo is given.

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[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.

[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. The Clinton 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 Aug. 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 will allow 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 draft guidelines (released in April 2009 for public review)[7] allow for federal funding on embryonic stem cells that were obtained (using private funding) from leftover *in vitro* fertilized eggs with proper informed consent. The guidelines do not allow funding for research using cells that were obtained from fertilized eggs created for research purposes or cells without documented informed consent from both the egg and sperm donor.

[7] Draft National Institute of Health Guidelines for Human Stem Cell Research, http://www.nih.gov/ and http://edocket.access.gpo.gov/2009/E9-9313.htm.

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

Not currently. Every year since 1996, U.S. Congress has passed an amendment on the funds to the NIH that bans the destruction or harm of embryos for research. This amendment prohibits the use of federal funds to obtain embryonic stem cells, but does not apply to private funding.

How does the executive order 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 and free up foundation funding to be used in other areas of research.

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 currently underway and federally funded biomedical research using cells approved during the Bush administration. It would also prevent researchers' ability to apply for additional funds on newer lines.

How would a ban impact Texas' economy?

If a ban on embryonic stem cells were implemented in Texas, researchers who are working in the field would likely leave 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 into Texas. This could negatively impact the amount of federal funding brought into the state. Currently Texas is third in the nation in receipt of federal research and development expenditures.[8] Last year, Texas received more than $1

[8] From Senator K.B. Hutchinson's Web site, http://hutchison.senate.gov/issue_science.html.

billion in federal funding from the National Institutes of Health alone, making it fifth in the nation for NIH funding.[9]

[9] National Institutes of Health, "Dollars Awarded by State for 2008," http://report.nih.gov/award/trends/State_Congressional/StateOverview.cfm.