Center for American Progress

STEM CELL PRIMER

What are stem cells?

Stem cells are undifferentiated cells that have the capacity to self-renew, meaning they can replicate identical copies of themselves and become other cell types in the human body. Differentiation is the process by which these cells develop and mature into specialized cells, such as heart, pancreatic or nerve cells. Cells that cannot divide or differentiate are called somatic cells and make up most of the human body.

What types of stem cells are there?

Embryonic stem cells are derived from human embryos at the earliest stages of development. When a sperm fertilizes an oocyte, or egg cell, it eventually forms a ball of cells called a blastocyst. At this stage of development, about five to six days, the blastocyst contains an area called the "inner cell mass" from which embryonic stem cells can be derived. If these cells are extracted, they can be grown in cultures in the laboratory and will continue to replicate almost indefinitely. A human embryo at this stage of development is a mass of cells almost too small to be seen by the human eye and does not have any of the characteristics of human fetuses, such as developed limbs or a nervous system.

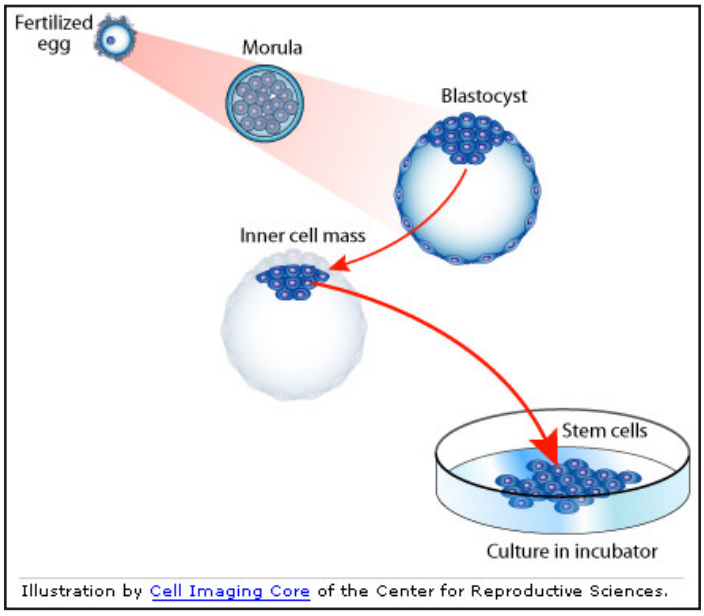

Figure 1. Source of Embryonic Stem Cells.
Courtesy of NICHD Grant for Specialized Cooperative Centers Program in Reproductive Research (SCCPRR). University of Kansas Medical Center, Center for Reproductive Sciences.
http://www.kumc.edu/stemcell/images.html

Adult stem cells can be found in various tissues throughout the human body including the brain, bone marrow, pancreas and liver. Stem cells from adult tissues can differentiate into a limited range of adult cells, usually only developing into the adult tissue in which they are found. For example, adult stem cells that are found in bone marrow are called hematopoietic stem cells and can differentiate into any of the types of somatic cells found in bone marrow or into additional hematopoietic stem cells.

What are the advantages of using embryonic stem cells rather than adult stem cells?

While some studies have shown that adult stem cells may have the ability to be coaxed into a wider variety of mature cells than initially believed, adult stem cells are still thought to be more limited than embryonic stem cells in their potential to differentiate into mature cells. Also, adult stem cells are rare within tissues in the human body and are difficult to isolate and grow in the laboratory. Scientists will have to work much harder to isolate, harvest and work with adult stem cells and may not be able to use them to treat certain diseases at all.

How are embryonic stem cells obtained?

Embryonic stem cells are derived from human embryos. There are approximately 400,000 excess embryos created for assisted reproductive treatments that remain frozen and unused in fertility clinics around the United States.[1] Researchers do not need nearly this many embryos to continue their research, so donated excess embryos would be sufficient to supply enough stem cells for research.

Embryonic stem cells can also be created in the laboratory using a method called somatic cell nuclear transfer, or SCNT (see Figure 2 below). In SCNT, the nucleus of an adult somatic cell, which houses the cell's chromosomes, is transferred into an enucleated oocyte (an egg cell without its own nucleus). Because somatic cells contain the full genetic complement of 46 chromosomes, following transplantation, the egg can be triggered to begin dividing as if a sperm and egg had fused. The embryonic stem cells that are derived from this egg are genetically identical to the adult somatic cell. It is hoped that embryonic stem cells created through SCNT could be used to create healthy cells for transplant into patients to cure their diseases. At present, however, this kind of treatment is not possible.

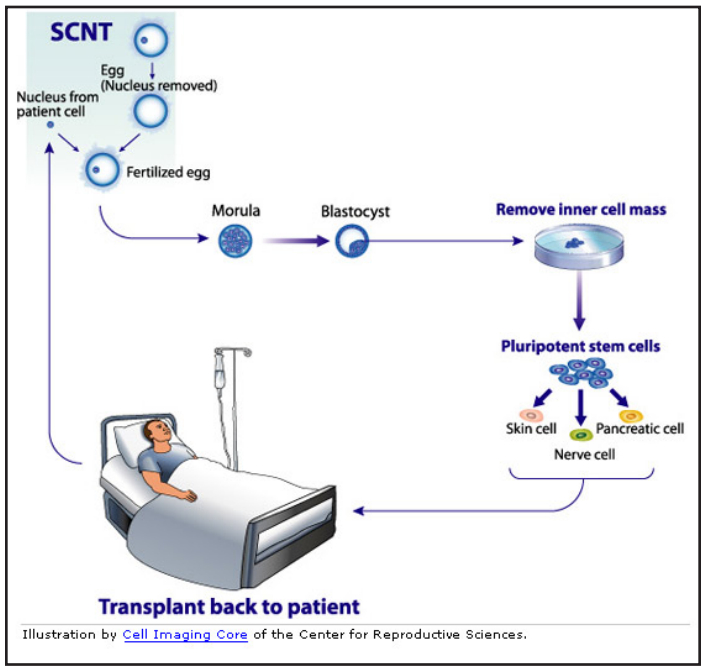

Figure 2. Somatic Cell Nuclear Transfer
Courtesy of NICHD Grant for Specialized Cooperative Centers Program in Reproductive Research (SCCPRR). University of Kansas Medical Center, Center for Reproductive Sciences. http://www.kumc.edu/stemcell/images.html

Alternative procedures have been developed to avoid deriving stem cells from embryos that could develop into human beings. Altered nuclear transfer, or ANT, involves altering the nucleus of the cell before it is transferred into a human egg. Altering the genetic material ensures that the resulting embryo would not be able to implant or normally develop in a woman's uterus.[2] Some argue that deriving stem cells from non-viable embryos reduces the ethical tension because they cannot develop into human beings.[3] Creating defective embryos, however, may only change, not eliminate, the ethical quandary if one considers the defective embryo to be human life.

While such alternative methods of nuclear transfer potentially could be developed, an effective, efficient and proven method of nuclear transfer to generate stem cells for research and treatment already exists. Millions of research dollars have been spent and would be spent pursuing these alternative research methods, when those funds could be used to advance stem cell research and the development of therapies using existing methods.

Why is stem cell research important?

Scientists believe that stem cells might be useful for a number of purposes (see Figure 3 on the following page):

1) **To create cells and tissue for transplants and therapies.** If stem cell research yields effective treatments, over 100 million Americans may benefit from stem cell therapies,[4] including

those with spinal cord injuries,[5] Parkinson's disease,[6] Lou Gehrig's disease (ALS),[7] and diabetes.[8] Stem cells, for example, have been injected into the brains of patients with Parkinson's disease to help repair their brain tissue. Damaged spinal cords may also be repaired using stem cells that can proliferate into new nerve cells. In the future, stem cells may be used to regenerate entire organs or even limbs, which, if created through SCNT, could be transplanted back into patients with no need for immunosuppressant drugs and reduced risk of transplant rejection.

2) **To improve our understanding of human development and the causes of birth defects, cancer and other degenerative diseases.** Stem cell research allows us to model diseases and problems in human development in the hopes of understanding and someday preventing a variety of health problems and conditions. For example, scientists in Australia have recently used embryonic stem cells to grow a human prostate in mice, allowing scientists to better study prostate cancer.[9]

3) **To teach us more about how drugs work in the human body and how to make them safer.** Rather than testing drugs in animals, which frequently react differently to drugs than humans, human stem cells might be used to test the safety of drugs. Scientists in Scotland and Italy have caused stem cells to become nerve cells, which can be used to test drugs for Parkinson's and Alzheimer's diseases.[10]

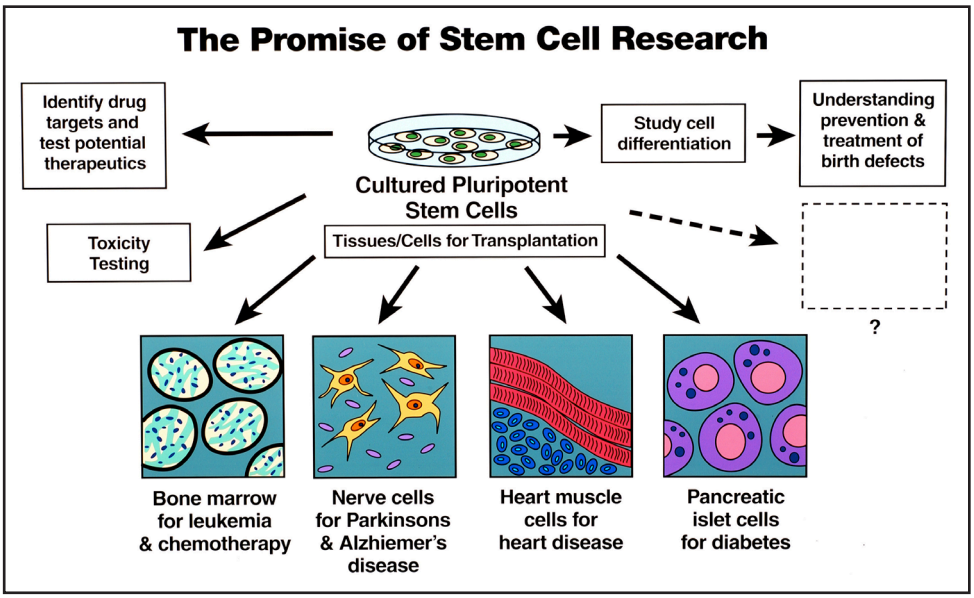

Figure 3. Potential Uses of Stem Cells.
Courtesy of National Institutes of Health. Stem Cell Information. http://stemcells.nih.gov/staticresources/info/media/DSC_1185.jpg

Why do we need additional lines of embryonic stem cells that are eligible for federal funding?

There are too few useful stem cell lines eligible for federal funding in the United States. President Bush claimed in 2001 that there were at least 60 stem cell lines eligible for funding, but in reality there are just 22 stem cell lines available.[11] Many of these stem cell lines were created using mouse cells to grow them, making them unsuitable for use in humans. Also, even if existing lines self-replicate forever, they will eventually develop mutations. We need to have federal funding for new lines with the necessary properties to be used to find cures for human diseases.

What are chimeras and how do they relate to the debate over stem cells?

A chimera is an organism containing distinct populations of cells from two different sources arising from different fertilization events. For example, chimeras are created when tissues from a human are transplanted into a laboratory animal or when a human being receives a heart transplant from another person.

Chimeras are necessary for a wide variety of medical research using animal models. They allow scientists to study the effect of human diseases and medicines on human tissue — which is far more effective than using animal models — without putting actual people in danger.

Where can I learn more about stem cell research?

NIH Stem Cells Basics

National Academy of Sciences Report on Stem Cell Research

University of Kansas Medical Center's Stem Cell Research 101

1. Hoffman, D.I., et al. Cryopreserved embryos in the United States and their availability for research. *Fertility and Sterility. 2003.* 79: 1063-1069.
2. A. Meissner and R. Jaenisch. Generation of nuclear transfer-derived pluripotent ES cells from cloned Cdx2-deficient blastocysts. *Nature.* 2006. Jan 12;439(7073):212-5.
3. A. Meissner and R. Jaenisch. Politically correct human embryonic stem cells? *New England Journal of Medicine.* 2006 Mar 16;354(11):1208-9
4. CAMR Fact Sheet. "Don't Deny Hope."
5. Christopher Reeve Foundation. Action Center on Stem Cells/SCNT.
6. The Michael J. Fox Foundation for Parkinson's Research. What's the status of research into a cure?
7. ALS Association. "A Primer on Stem Cells."
8. Juvenile Diabetes Research Foundation. "Advocacy: Stem Cells."
9. Taylor RA; Cowin PA; Cuhna GR; Pera M; Trounson AO; Pederson J; Risbridger GP. "Formation of human prostate tissue from embryonic stem cells." *Nature Methods*. 2006. 3: 179-181.
10. BBC News. "Scientists Make Nerve Stem Cells." August 16, 2005.
11. National Institutes of Health. Information on Eligibility Criteria for Federal Funding of Research on Human Embryonic Stem Cells.