

Stem cells: What they are and what they do



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Stem cells and derived products offer great promise for new medical treatments. Learn about stem cell types, current and possible uses, ethical issues, and the state of research and practice.

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You've heard about stem cells in the news, and perhaps you've wondered if they might help you or a loved one with a serious disease. You may wonder what stem cells are, how they're being used to treat disease and injury, and why they're the subject of such vigorous debate.

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Here are some answers to frequently asked questions about stem cells.

What are stem cells?

Stem cells are the body's raw materials – cells from which all other cells with specialized functions are generated. Under the right conditions in the body or a laboratory, stem cells divide to form more cells called daughter cells.

These daughter cells either become new stem cells (self-renewal) or become specialized cells (differentiation) with a more specific function, such as blood cells, brain cells, heart muscle cells or bone cells. No other cell in the body has the natural ability to generate new cell types.

Why is there such an interest in stem cells?

Researchers and doctors hope stem cell studies can help to:

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- **Increase understanding of how diseases occur.** By watching stem cells mature into cells in bones, heart muscle, nerves, and other organs and tissue, researchers and doctors may better understand how diseases and conditions develop.
- **Generate healthy cells to replace diseased cells (regenerative medicine).** Stem cells can be guided into becoming specific cells that can be used to regenerate and repair diseased or damaged tissues in people.

People who might benefit from stem cell therapies include those with spinal cord injuries, type 1 diabetes, Parkinson's disease, amyotrophic lateral sclerosis, Alzheimer's disease, heart disease, stroke, burns, cancer and osteoarthritis.

Stem cells may have the potential to be grown to become new tissue for use in transplant and regenerative medicine. Researchers continue to advance the knowledge on stem cells and their applications in transplant and regenerative medicine.

- **Test new drugs for safety and effectiveness.** Before using investigational drugs in people, researchers can use some types of stem cells to test the drugs for safety and quality. This type of testing will most likely first have a direct impact on drug development first for cardiac toxicity testing.

New areas of study include the effectiveness of using human stem cells that have been programmed into tissue-specific cells to test new drugs. For the testing of new drugs to be accurate, the cells must be programmed to acquire properties of the type of cells targeted by the drug. Techniques to program cells into specific cells continue to be studied.

For instance, nerve cells could be generated to test a new drug for a nerve disease. Tests could show whether the new drug had any effect on the cells and whether the cells were harmed.

Where do stem cells come from?

- **Embryonic stem cells.** These stem cells come from embryos that are three to five days old. At this stage, an embryo is called a blastocyst and has about 150 cells.

These are pluripotent (ploo-RIP-uh-tunt) stem cells, meaning they can divide into more stem cells or can become any type of cell in the body. This versatility allows embryonic stem cells to be used to regenerate or repair diseased tissue and organs.

- **Adult stem cells.** These stem cells are found in small numbers in most adult tissues, such as bone marrow or fat. Compared with embryonic stem cells, adult stem cells have a more limited ability to give rise to various cells of the body.

Until recently, researchers thought adult stem cells could create only similar types of cells. For instance, researchers thought that stem cells residing in the bone marrow could give rise only to blood cells.

However, emerging evidence suggests that adult stem cells may be able to create various types of cells. For instance, bone marrow stem cells may be able to create bone or heart muscle cells.

This research has led to early-stage clinical trials to test usefulness and safety in people. For example, adult stem cells are currently being tested in people with neurological or heart disease.

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- **Adult cells altered to have properties of embryonic stem cells (induced pluripotent stem cells).** Scientists have successfully transformed regular adult cells into stem cells using genetic reprogramming. By altering the genes in the adult cells, researchers can reprogram the cells to act similarly to embryonic stem cells.

This new technique may allow researchers to use reprogrammed cells instead of embryonic stem cells and prevent immune system rejection of the new stem cells. However, scientists don't yet know whether using altered adult cells will cause adverse effects in humans.

Researchers have been able to take regular connective tissue cells and reprogram them to become functional heart cells. In studies, animals with heart failure that were injected with new heart cells experienced improved heart function and survival time.

- **Perinatal stem cells.** Researchers have discovered stem cells in amniotic fluid as well as umbilical cord blood. These stem cells also have the ability to change into specialized cells.

Amniotic fluid fills the sac that surrounds and protects a developing fetus in the uterus. Researchers have identified stem cells in samples of amniotic fluid drawn from pregnant women to test for abnormalities – a procedure called amniocentesis.

More study of amniotic fluid stem cells is needed to understand their potential.

Why is there a controversy about using embryonic stem cells?

Embryonic stem cells are obtained from early-stage embryos – a group of cells that forms when a woman's egg is fertilized with a man's sperm in an in vitro fertilization clinic. Because human embryonic stem cells are extracted from human embryos, several questions and issues have been raised about the ethics of embryonic stem cell research.

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The National Institutes of Health created guidelines for human stem cell research in 2009. The guidelines define embryonic stem cells and how they may be used in research, and include recommendations for the donation of embryonic stem cells. Also, the guidelines state embryonic stem cells from embryos created by in vitro fertilization can be used only when the embryo is no longer needed.

Where do these embryos come from?

The embryos being used in embryonic stem cell research come from eggs that were fertilized at in vitro fertilization clinics but never implanted in a woman's uterus. The stem cells are donated with informed consent from donors. The stem cells can live and grow in special solutions in test tubes or petri dishes in laboratories.

Why can't researchers use adult stem cells instead?

Although research into adult stem cells is promising, adult stem cells may not be as versatile and durable as are embryonic stem cells. Adult stem cells may not be able to be manipulated to produce all cell types, which limits how adult stem cells can be used to treat diseases.

Adult stem cells also are more likely to contain abnormalities due to environmental hazards, such as toxins, or from errors acquired by the cells during replication. However, researchers have found that adult stem cells are more adaptable than was first thought.

What are stem cell lines and why do researchers want to use them?

A stem cell line is a group of cells that all descend from a single original stem cell and are grown in a lab. Cells in a stem cell line keep growing but don't differentiate into specialized cells. Ideally, they remain free of genetic defects and continue to create more stem cells. Clusters of cells can be taken from a stem cell line and frozen for storage or shared with other researchers.

What is stem cell therapy (regenerative medicine) and how does it work?

Stem cell therapy, also known as regenerative medicine, promotes the repair response of diseased, dysfunctional or injured tissue using stem cells or their derivatives. It is the next chapter in organ transplantation and uses cells instead of donor organs, which are limited in supply.

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Researchers grow stem cells in a lab. These stem cells are manipulated to specialize into specific types of cells, such as heart muscle cells, blood cells or nerve cells.

The specialized cells can then be implanted into a person. For example, if the person has heart disease, the cells could be injected into the heart muscle. The healthy transplanted heart muscle cells could then contribute to repairing defective heart muscle.

Researchers have already shown that adult bone marrow cells guided to become heart-like cells can repair heart tissue in people, and more research is ongoing.

Have stem cells already been used to treat diseases?

Yes. Doctors have performed stem cell transplants, also known as bone marrow transplants. In stem cell transplants, stem cells replace cells damaged by chemotherapy or disease or serve as a way for the donor's immune system to fight some types of cancer and blood-related diseases, such as leukemia, lymphoma, neuroblastoma and multiple myeloma. These transplants use adult stem cells or umbilical cord blood.

Researchers are testing adult stem cells to treat other conditions, including a number of degenerative diseases such as heart failure.

What are the potential problems with using embryonic stem cells in humans?

For embryonic stem cells to be useful in people, researchers must be certain that the stem cells will differentiate into the specific cell types desired.

Researchers have discovered ways to direct stem cells to become specific types of cells, such as directing embryonic stem cells to become heart cells. Research is ongoing in this area.

Embryonic stem cells can also grow irregularly or specialize in different cell types spontaneously. Researchers are studying how to control the growth and differentiation of embryonic stem cells.

Embryonic stem cells might also trigger an immune response in which the recipient's body attacks the stem cells as foreign invaders, or the stem cells might simply fail to function normally, with unknown consequences. Researchers continue to study how to avoid these possible complications.

What is therapeutic cloning, and what benefits might it offer?

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Therapeutic cloning, also called somatic cell nuclear transfer, is a technique to create versatile stem cells independent of fertilized eggs. In this technique, the nucleus, which contains the genetic material, is removed from an unfertilized egg. The nucleus is also removed from the cell of a donor.

This donor nucleus is then injected into the egg, replacing the nucleus that was removed, in a process called nuclear transfer. The egg is allowed to divide and soon forms a blastocyst. This process creates a line of stem cells that is genetically identical to the donor's cells – in essence, a clone.

Some researchers believe that stem cells derived from therapeutic cloning may offer benefits over those from fertilized eggs because cloned cells are less likely to be rejected once transplanted back into the donor and may allow researchers to see exactly how a disease develops.

Has therapeutic cloning in people been successful?

No. Researchers haven't been able to successfully perform therapeutic cloning with humans despite success in a number of other species.

However, in recent studies, researchers have created human pluripotent stem cells by modifying the therapeutic cloning process. Researchers continue to study the potential of therapeutic cloning in people.

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References

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1. Stem cell information – Frequently asked questions (FAQs). National Institutes of Health. <https://stemcells.nih.gov/info/faqs.htm>. Accessed July 23, 2018.
2. Stem cell basics. National Institutes of Health. <https://stemcells.nih.gov/info/basics.htm>. Accessed July 23, 2018.
3. Nelson TJ, et al. Stem cell therapy and congenital heart disease. *Journal of Cardiovascular Development and Disease*. 2016;3:1.
4. Terashvili M, et al. Stem cell therapies in cardiovascular disease. *Journal of Cardiothoracic and Vascular Anesthesia*. In press. Accessed July 23, 2018.
5. Samsonraj RM, et al. Concise review: Multifaceted characterization of human mesenchymal stem cells for use in regenerative medicine. *Stem Cells Translational Medicine*. 2017;6:2173.
6. Blood-forming stem cell transplants. National Cancer Institute. <https://www.cancer.gov/about-cancer/treatment/types/stem-cell-transplant/stem-cell-fact-sheet>. Accessed July 23, 2018.
7. Abbaspanah B, et al. Advances in perinatal stem cells research: A precious cell source for clinical applications. 2018;13:595.
8. Routine tests during pregnancy. The American College of Obstetricians and Gynecologists. <https://www.acog.org/Patients/FAQs/Routine-Tests-During-Pregnancy>. Accessed July 23, 2018.
9. Stem cell facts. International Society for Stem Cell Research. <http://www.closerlookatstemcells.org/>. Accessed July 23, 2018.
10. Matoba S, et al. Somatic cell nuclear transfer reprogramming: Mechanisms and applications. *Cell Stem Cell* 2018;23:1.
11. National Institutes of Health guidelines for human stem cell research. National Institutes of Health. <https://stemcells.nih.gov/policy/2009-guidelines.htm>. Accessed July 23, 2018.