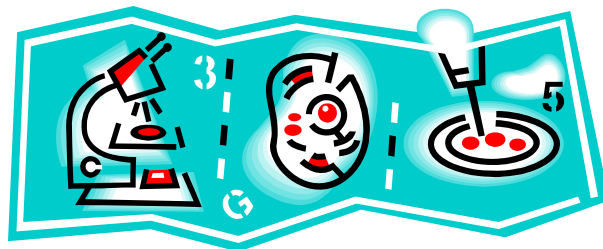


Studying both adult and embryonic stem cells helps scientists discover the processes by which cells develop and grow. Stem cells can also provide valuable models of disease, which helps researchers understand how illness and injury operate at the cellular level, which is often helpful for creating treatments. Because embryonic stem cells can transform into any type of cell, and adult stem cells have a limited capacity to form some types of cells, both may be useful therapeutically for diseases or conditions in which cells are missing or damaged.

RECENT STATEMENTS THAT NO ANIMAL HAS BEEN TREATED OR THAT NO PROGRESS HAS BEEN MADE USING HUMAN EMBRYONIC STEM CELLS ARE UNTRUE

While it is true that adult stem cells, which have been studied for more than 40 years, are farther along in the timeline of developing medical treatments, they do not exist for all kinds of tissue, are difficult to isolate, and are often not available in the quantities needed for treatment. Human embryonic stem cells, which were discovered less than ten years ago, are not bound by the limitations of adult stem cells.

Fortunately, the promising nature of embryonic stem cells, and their remarkable ability to become any other type of cell, have allowed rapid progress towards promising treatments in animal and laboratory experiments. Some examples are listed within.



2006

- **Spinal cord injury and Lou Gehrig's disease:** Scientists are using stem cells from a variety of sources to help animals with spinal cord injuries regain movement. Human embryonic and adult stem cells have been coaxed into becoming types of cells that repair damaged spinal cord insulation and replace damaged spinal cord nerve cells in rats. Scientists now report that they can use mouse embryonic stem cells to make functional motor neurons. Previously paralyzed rats treated with the motor neurons were able to move their legs again. This research gives scientists insight on how they might one day replace human motor cord neurons damaged by spinal cord injuries or diseases such as Lou Gehrig's Disease (ALS) and spinal muscular atrophies. ([Annals of Neurology 60\(1\) 32–44](#), laboratory of D. Kerr)
- **Liver disease:** Chronic liver diseases such as cirrhosis and hepatitis affect 25 million Americans. Although liver transplantation can help some of these individuals, there is an extreme shortage of transplantable organs. Scientists hope to overcome the organ shortage by using stem cells to replace lost liver function. A collaborative team of Japanese and NIH-funded scientists coaxed mouse embryonic stem cells into becoming liver-like cells. They used the new mouse liver-like cells in a bioartificial liver, an implanted device that uses liver cells to replace some liver function. Ninety percent of mice with liver failure that were implanted with the bioartificial liver survived, while mice with liver failure that did not receive the implant all died within two days. If scientists can repeat these results with human stem cells, the technique offers promise both to individuals born with liver problems and to those who develop liver disease later in life. ([Nature Biotechnology 24:1412–1419](#), laboratory of I. Fox)
- **Diabetes:** One hopeful prospect for treating diabetes is to replace the damaged or missing insulin-producing beta islet cells in the pancreas. Unfortunately, the supply of transplantable pancreatic islets is unable to meet the demand. Scientists have now succeeded in generating insulin-secreting cells from hESCs by allowing the cells to mimic pancreatic development. These hESC-derived precursor cells have almost as much insulin as adult beta cells. If scientists succeed at getting these cells to secrete enough insulin in response to blood sugar levels in human beings, they may one day be useful for treating diabetes. ([Nature Biotechnology advanced online publication](#), laboratory of Novocell Inc.)

- **Restoring vision loss:** Retinal pigment epithelium (RPE) cells within the eye play a vital role in the survival and maintenance of the rods and cones that detect light and color. Death of RPE cells may lead to age-related macular degeneration, a major cause of vision loss in persons aged 60 and older. RPE's have now been derived from human embryonic stem cells and may be used to restore vision in those suffering from macular degeneration. More recently, these same scientists report that they used these cells in rats with a genetic eye disease similar to age-related macular degeneration, a major cause of vision loss in persons aged 60 and older. After over a month of treatment with the RPE cells, visual acuity in the rats improved. ([Cloning and Stem Cells 6:217–245](#) and [Cloning and Stem Cells 8:189–199](#), laboratory of R. Lanza)

2005

- **Parkinson's and Lou Gehrig's disease:** Human embryonic stem cells (hESCs) injected into the brains of embryonic mice can take cues from the mouse brain environment and differentiated into nerve cells and supporting cells typically found in the brain. Mice with functioning human nerve cells and supporting cells provide a valuable model system for learning how the human brain develops and for testing drugs to treat human nervous system diseases such as Lou Gehrig's and Parkinson's disease. ([Proceedings of the National Academy of Sciences of the USA 102\(51\):18644–8](#), laboratory of R. Gage)
- **Spinal cord injury and paralysis:** Transplanted stem cells have the potential to help repair or replace damaged nerve cells in individuals who have suffered a spinal cord injury. NIH-supported scientists injected human neural stem cells into the damaged spinal cords of rats. Four weeks after the injections, rats treated with human cells regained use of their back paws and were able to coordinate steps between their front and back paws. Although more tests must be done before this kind of stem cell therapy is ready for use in humans, these studies provide hope that human stem cells may one day restore mobility to individuals with spinal cord injuries. ([Proceedings of the National Academy of Sciences of the USA 102:14069–14074](#), laboratory of A.J. Anderson)
- **Hemophilia:** Hemophilia is a rare inherited disorder in which the blood does not clot normally. Individuals with hemophilia can be treated with infusions of blood clotting factors, but these only help for a short time. NIH-supported scientists used stem cells to cure mice

suffering from a disorder similar to human Hemophilia B. If these results can be repeated in human beings, doctors may one day be able to use human embryonic stem cells (hESCs) to restore blood clotting abilities to individuals with hemophilia. ([Proceedings of the National Academy of Sciences of the USA 102:2958–2963](#), laboratories of O. Smithies and J. Frelinger)

- **Multiple sclerosis:** Multiple sclerosis and some other nervous system disorders are caused by damage of a protective coating around nerve cells called the myelin sheath, which is formed when cells known as oligodendrocytes wrap themselves around the axon of the nerve. Scientists have formed oligodendrocytes from human embryonic stem cells and used them to restore the myelin sheath in mice. If this work can be repeated in humans, it may enable scientists to help individuals with nervous system disorders recover some of their mobility and sensations. ([Glia 49:385–396](#), laboratory of H.S. Keirstead)
- **Lou Gehrig's Disease and spinal cord injury:** Individuals who suffer spinal cord or motor neuron diseases such as Lou Gehrig's disease (amyotrophic lateral sclerosis or ALS), currently have no treatment option available to reverse their condition. NIH-funded scientists have directed human embryonic stem cells into cells that express markers and transmit nerve impulses in a manner similar to motor neurons. If they are able to function in human beings after transplantation, these cells may also serve as a renewable source of replacement motor neurons to treat spinal cord injury and motor neuron diseases. ([Nature Biotechnology 23:215–221](#), laboratory of S-C. Zhang)

2004

- **Heart Disease:** Mice with severe heart defects appeared to return to normal heart function following injections of embryonic stem cells. ([Science 306:247–252](#), laboratory of R. Benezra) In addition, heart muscle cells derived from human embryonic stem cells were also able to restore heart rhythm in 11 out of 13 pigs whose biological pacemaker had been damaged. If this work can be repeated in human beings, scientists may be able to use these cells to replace human heart pacemakers rather than the current implanted electronic devices. ([Nat Biotech 22:1282–1289](#), laboratory of L. Gepstein)

- **Parkinson's Disease:** In 2002, scientists reported that they had successfully derived dopaminergic neurons from mouse embryonic stem cells. (*Nature* 418:50–56, 2002, laboratory of R. McKay) When grafted into rat models of Parkinson's disease, the cells were able to improve motor function. More recent work has produced human dopamine producing cells from human embryonic stem cells, which may be used to treat humans with Parkinson's disease. (*PNAS* 101:12543–8, laboratory of L. Studer)

2003

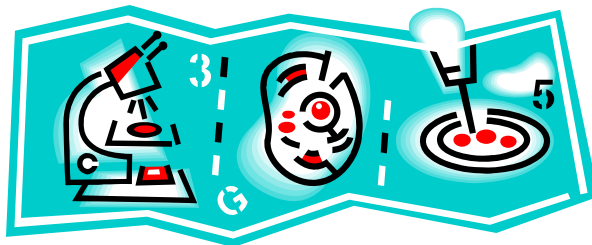
- **Infertility treatment:** Scientists report production of functional sperm in mice from human embryonic stem cells, which may be used to treat men who are suffering from sterility or infertility. (*Nature* 427:148–154, laboratory of G.Q. Daley) In addition, oocytes (eggs) have been generated from embryonic stem cells. This has important implications for: creation of new hESC lines, generation of tissue for transplantation, generation of human oocytes, and infertility treatment. (*Science* 300:1251–1256, 2003, laboratory of H.R. Schöler)
- **Lou Gehrig's Disease and Spinal Cord Injury:** Using pluripotent cells derived from human embryonic germ cells, scientists have been able to partially restore paralyzed rats' ability to move. The rats serve as an animal model of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease. This work provides hope that scientists may one day be able to use embryonic stem cells to restore movement to patients suffering from Lou Gehrig's disease. (*J Neurosci* 23:5131–5140, 2003, laboratories of J.D. Gearhart and J.D. Rothstein)

2002

- **Diabetes:** scientists at Stanford University recently reported that they could use mouse embryonic stem cells to "cure" a mouse model of diabetes. Their results suggest that embryonic stem cells could serve as a source of insulin-producing replacement tissue and provide hope that this technique, adapted to human embryonic stem cells, may lead to a cure for human diabetes patients. (*PNAS USA* 99:16105–16110, 2002, laboratory of S.K. Kim)

Information source: <http://stemcells.nih.gov>

BREAKTHROUGHS USING EMBRYONIC STEM CELLS



Compiled by Carrie D. Wolinetz, Ph.D.

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