Stem Cells

What are stem cells?

Stem cells have the remarkable potential to develop into many different cell types in the body. Serving as a sort of repair system for the body, they can theoretically divide without limit to replenish other cells as long as the person or animal is still alive. When a stem cell divides, each new cell has the potential to either remain a stem cell or become another type of cell with a more specialized function, such as a muscle cell, a red blood cell, or a brain cell.

What classes of stem cells are there?

Common terms you may come across describing stem cells group them according to how many different types of cells they have the potential to produce. A fertilized egg is considered **totipotent**, meaning that its potential is total; it gives rise to all the different types of cells in the body. **Pluripotent** stem cells can give rise to any type of cell in the body except those needed to develop a fetus. Stem cells that can give rise to multiple different cell types are generally called **multipotent**.

Why are doctors and scientists so excited about stem cells?

Stem cells have potential in many different areas of health and medical research. To start with, studying stem cells will help us to understand how they transform into the dazzling array of specialized cells that make us what we are. Some of the most serious medical conditions, such as cancer and birth defects, are due to problems that occur somewhere in this process. A better understanding of normal cell development will allow us to understand and perhaps correct the errors that cause these medical conditions.

Another potential application of stem cells is making cells and tissues for medical therapies. Today, donated organs and tissues are often used to replace those that are diseased or destroyed. Unfortunately, the number of people suffering from these disorders far outstrips the number of organs available for transplantation. Stem cells offer the possibility of a renewable source of replacement cells and tissues to treat myriad diseases, conditions, and disabilities including Parkinson's and Alzheimer's diseases, spinal cord injury, stroke, burns, heart disease, diabetes, osteoarthritis and rheumatoid arthritis. There is almost no realm of medicine that might not be touched by this innovation.

Have stem cells been used successfully to treat any human diseases yet?

Blood-forming stem cells in bone marrow called hematopoietic stem cells (HSCs) are currently the only type of stem cell commonly used for therapy. Doctors have been transferring HSCs in bone marrow transplants for over 40 years. More advanced techniques of collecting, or "harvesting", HSCs are now used in order to treat leukemia, lymphoma and several inherited blood disorders.

The clinical potential of stem cells has also been demonstrated in the treatment of other human diseases that include diabetes and advanced kidney cancer. However, these newer applications have involved studies with a very limited number of patients, using stem cells that were harvested from people.

How do scientists get stem cells for medical and scientific use?

Pluripotent stem cells have been isolated from human embryos that are a few days old. Cells from these embryos can be used to create pluripotent stem cell "lines", cultures that can be grown indefinitely in the laboratory. Multipotent stem cell lines have also been developed from fetal tissue obtained from terminated pregnancies.

Stem cells can also be isolated from adult tissue. Thus far, these cells have been multipotent. Adult stem cells have not been found for all types of tissue, but discoveries in this area of research are increasing. For example, until recently it was thought that stem cells were not present in the adult nervous system, but in recent years such stem cells have been found in the brain.

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Why do scientists want to use stem cell lines?

Once a stem cell line is established from a cell in the body, it is essentially immortal, no matter how it was derived. That is, it does not have to be created again from the original embryo or adult. Once established, it can be grown in the laboratory indefinitely and widely distributed to other researchers.

In addition, before scientists can use any type of stem cell for transplantation, they must overcome attempts by a patient's immune system to reject the transplant. Human stem cell lines might in the future be modified with gene therapy or other techniques to overcome this immune rejection. Scientists might also be able to replace damaged genes or add new genes to stem cells in order to give them new characteristics that can ultimately help to treat diseases.

What will be the best type of stem cell to use for therapy?

Pluripotent stem cells, while having great therapeutic potential, face formidable technical challenges. First, scientists must learn how to control their development into all the different types of cells in the body. Second, the cells now available for research are likely to be rejected by a patient's immune system. Another serious consideration is that the idea of using stem cells from human embryos or human fetal tissue troubles many people on ethical grounds.

Until recently, there was little evidence that stem cells from adults could change course and provide the flexibility that researchers need in order to address all the medical diseases and disorders they would like to. New findings in animals, however, suggest that even after a stem cell has begun to specialize, it may be more flexible than previously thought.

There are currently several limitations to using adult stem cells. Although many different kinds of multipotent stem cells have been identified, the evidence that adult stem cells could give rise to all cell and tissue types is not yet conclusive. Adult stem cells are often present in only minute quantities and can therefore be difficult to isolate and purify. There is also evidence that they may not have the same capacity to multiply as embryonic stem cells do. Finally, adult stem cells may contain more DNA abnormalities—caused by sunlight, toxins and errors in making more DNA copies during the course of a lifetime. These potential weaknesses might limit the usefulness of adult stem cells.

Does NIH fund embryonic stem cell research?

Research on human embryonic stem cell lines may receive NIH funding if the cell line meets the following criteria: removal of cells from the embryo must have been initiated before August 9, 2001, when President Bush outlined this policy; and the embryo from which the stem cell line was derived must no longer have had the possibility of developing further as a human being. The embryo must have been created for reproductive purposes but no longer be needed for them. Informed consent must have been obtained from the parent(s) for the donation of the embryo, and no financial inducements for donation are allowed.

In order to ensure that federal funds are used to support only stem cell research that is scientifically sound, legal, and ethical, NIH examines stem cell lines and maintains a registry of those lines that satisfy the criteria at http://escr.nih.gov/.

Which research is best to pursue?

The development of stem cell lines that can produce many tissues of the human body is an important scientific breakthrough. This research has the potential to revolutionize the practice of medicine and improve the quality and length of life. Given the enormous promise of stem cells therapies for so many devastating diseases, NIH believes that it is important to simultaneously pursue all lines of research and search for the very best sources of these cells.

A more detailed primer on stem cells can be found at http://www.nih.gov/news/stemcell/primer.htm. For current, in-depth information on NIH and stem cell research, visit http://www.nih.gov/news/stemcell/index.htm.

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