

Frequently Asked Questions

1. Why do researchers need embryonic stem cells now that they can reprogram adult skin cells to create induced pluripotent stem cells, or iPS cells, which mimic embryonic stem cells?

Opponents of embryonic stem cell research tout iPS, but researchers still need access to embryonic stem cells because the study of iPS cells is in its infancy, and the reprogramming approach may have serious limitations. The standard technique to create iPS cells uses viruses to transfer the reprogramming genes into the skin cells.

Researchers need to determine whether the reprogrammed cells really can give rise to specialized cells that are indistinguishable from the specialized cells formed by embryonic stem cells.

Research on human embryonic stem cells, somatic cell nuclear transfer and 'adult' or tissue-specific stem cells needs to continue in parallel. All are part of a research effort that seeks to expand our knowledge of how cells function, what fails in the disease process, and how the first stages of human development occur. It is this combined knowledge that will ultimately generate safe and effective therapies.

2. Why do UNMC scientists need to use embryonic stem cell lines created after 2001? Aren't the lines funded under President George W. Bush sufficient?

Hundreds of stem cell lines have been created since 2001, and they are much more flexible and easier to work with than the lines formed almost a decade ago. Because of the new lines' potential, it's doubtful that projects that are restricted to the old lines will be funded by the National Institutes of Health (NIH) or other competitive funding agencies.

3. What are the potential uses of human stem cells?

Stem cell research contributes to a fundamental understanding of how organisms develop and grow, and how tissues are maintained throughout adult life. This is knowledge that is required to work out what goes wrong during disease and injury and ultimately how these conditions might be treated. The development of a range of human tissue-specific and embryonic stem cell lines will provide researchers with the tools to model disease, test drugs and develop increasingly effective therapies.

Replacing diseased cells with healthy cells, a process called cell therapy, is a promising use of stem cells in the treatment of disease; this is similar to organ transplantation only the treatment consists of transplanting cells instead of organs. Currently, researchers are investigating the use of adult, fetal and embryonic stem cells as a resource for various, specialized cell types, such as nerve cells, muscle cells, blood cells and skin cells that can be used to treat various diseases.

In theory, any condition in which there is tissue degeneration can be a potential candidate for stem cell therapies, including Parkinson's disease, spinal cord injury, stroke, burns, heart disease, Type 1 diabetes, osteoarthritis, rheumatoid arthritis, muscular dystrophies and liver diseases.

In addition, retinal regeneration with stem cells isolated from the eyes can lead to a possible cure for damaged or diseased eyes and may one day help reverse blindness. Bone marrow transplantation (transfers blood stem cells) is a well-established treatment for blood cancers and other blood disorders.

4. What are the obstacles that must be overcome before the potential uses of stem cells in cell therapy will be realized?

Here are just a few of the challenges that lie ahead. Firstly, a source of stem cells must be found. The process of identifying, isolating

and growing the right kind of stem cell, for example a rare cell in the adult tissue, is painstaking. In general, embryonic and fetal stem cells are believed to be more versatile than tissue-specific stem cells. Secondly, once stem cells are identified and isolated, the right conditions must be developed so that the cells differentiate into the specialized cells required for a particular therapy. This too will require a great deal of experimentation. Thirdly, a system that delivers the cells to the right part of the body must be developed and the cells once there must be encouraged to integrate and function in concert with the body's natural cells. Furthermore, just as in organ transplants, the body's immune system may be suppressed to minimize the immune reaction set off by the transplanted cells.

While results from animal models are promising, the research on stem cells and their applications to treat various human diseases is still at a preliminary stage. As with any medical treatment, a rigorous research and testing process must be followed to ensure long-term efficacy and safety.

5. Are stem cells currently used in therapies today?

Hematopoietic stem cells (HSCs) or blood stem cells, present in the bone marrow are the precursors to all blood cells. Blood stem cells are currently the only type of stem cells commonly used for therapy. Doctors have been transferring blood stem cells by bone marrow transplant for more than 40 years. Advanced techniques for collecting or "harvesting" HSCs are now used to treat leukemia, lymphoma and several inherited blood disorders. Cord blood, like bone marrow, is stored as a source of HSCs and is being used experimentally as an alternative to bone marrow in transplantation.

New clinical applications for stem cells are currently being tested therapeutically for the treatment of musculoskeletal abnormalities, cardiac disease, liver disease, autoimmune and metabolic disorders (amyloidosis), chronic inflammatory diseases (lupus) and other advanced cancers. However, these new therapies have been offered only to a very limited number of patients and are experimental in nature rather than standard practice.

6. Why are researchers interested in developing disease-specific or patient-specific pluripotent stem cells?

The development of patient-specific or disease-specific pluripotent stem cells has great therapeutic promise for three reasons. Firstly, these cells could provide a powerful new tool for studying the basis of human disease and for discovering new drugs. Secondly, the resulting embryonic stem cells could be developed into a needed cell type, and if transplanted into the original donor, would be recognized as 'self', thereby avoiding the problems of rejection and immunosuppression that occur with transplants from unrelated donors.

7. Why derive embryonic stem cell lines following somatic cell nuclear transfer (SCNT)?

The derivation of patient-specific human embryonic stem cell lines using this technique (see 'What is somatic cell nuclear transfer?') Firstly, these cells could provide a powerful new tool for studying the basis of human disease and for discovering new drugs. Secondly, the resulting embryonic stem cells could be developed into a needed cell type, and if transplanted into the original donor, would be recognized as 'self', thereby avoiding the problems of rejection and immunosuppression that occur with transplants from unrelated donors.

8. What is regenerative medicine?

The goal of regenerative medicine is to repair organs or tissues that are damaged by disease, aging or trauma, such that function is restored, or at least improved.

The term regenerative medicine is often used nowadays to describe medical treatments and research that use stem cells (either adult or embryonic) to restore the function of organs or tissues. This can be achieved in different ways; first, by administering stem cells, or specific cells that are derived from stem cells in the laboratory; or second, by administering drugs that coax stem cells that are

already present in tissues to more efficiently repair the involved tissue.

Source: International Society for Stem Cell Research