



## The Facts: Embryonic Stem Cell Research

### **The Problem**

The most significant problem facing embryonic stem cell research is the arbitrary restriction of research on embryonic stem cell lines created before August 9, 2001. This cutoff date has significantly impeded the science's progress. It is time to expand the federal policy so that this groundbreaking research can move forward. The current policy has tied the hands of researchers:

- Of the 78 stem cell lines promised by President Bush, only 22 are available to researchers
- These 22 lines are aged, contaminated with mouse cells and were developed with outdated techniques
- There are at least 125 new stem cell lines that federally funded researchers cannot use

### **Why do we need to research embryonic stem cells?**

Embryonic stem cells possess a unique ability to develop into any type of cell as they mature and develop. This ability means embryonic stem cells offer a potentially unlimited source of cells for organ transplantation, drug discovery, and the study of human development. Early research suggests they are capable of developing into various specific types of cells, making them more useful. Unfortunately, adult stem cell lines have not displayed this ability to differentiate into specific types of cells.

### **Where Do Embryonic Stem Cells Come From?**

Most embryonic stem cells are derived from embryos that were fertilized in an in vitro fertilization clinic and then donated for research purposes with informed consent of the donors. These embryos would have otherwise been discarded or destroyed. It is estimated that 400,000 surplus embryos are currently in frozen storage at fertilization clinics in the U.S.

### **The Castle-DeGette Bill**

The Castle DeGette Stem Cell bill (H.R. 810) expands the federal policy and implements stricter ethical guidelines for this research. Under this legislation, stem cell lines will be eligible for federal research only if they meet the following requirements:

- The embryos used to derive the stem cells were originally created for fertility treatment purposes and are in excess of clinical need;
- The individuals seeking fertility treatments have decided to discard these excess embryos
- The individuals for whom the embryos were created have provided written informed consent for the donation.

If the President's restrictive policy continues, this research will move ahead without the federal ethical controls, support and oversight. The Castle-DeGette bill requires NIH to determine the ethical guidelines and monitor the research

## **What are stem cells?**

Stem cells are the foundation cells for every organ, tissue and cell in the body. They are like a blank microchip that can ultimately be programmed to perform any number of specialized tasks. Stem cells are undifferentiated, or "blank" cells that do not yet have a specific function. Under proper conditions, stem cells begin to develop into specialized tissues and organs. Stem cells offer the potential for supplying cells and tissues, which can be used to treat various diseases.

## **Where do stem cells come from?**

All human beings start their lives from a single cell, called the zygote, which is formed after fertilization. The zygote divides and forms two cells; each of those cells divides again, and so on. Pretty soon, about five days after conception, there is hollow ball of about 150 cells called the blastocyst. The blastocyst is smaller than a grain of sand and contains two types of cells, one which forms embryonic stem cells.

Adult stem cells can also be found in very small numbers in various tissues in the adult body. For example, bone marrow stem cells are found in the marrow of the bone and they give rise to all specialized blood cell types. Unlike embryonic stem cells, adult stem cells are typically programmed to form different cell types of their own tissue. Adult stem cells have not yet been identified in all vital organs. In some tissues like the brain, although stem cells exist, they are not very active, and thus do not readily respond to cell injury or damage.

## **What are the potential uses of human stem cells?**

Most of the body's specialized cells cannot be regenerated if they are seriously damaged or diseased. Stem cells can be used to generate healthy and functioning specialized cells, which can then replace diseased or dysfunctional cells.

Replacing diseased cells with healthy cells, called cell therapy, is similar to the process of organ transplantation except cells are transplanted instead of organs. Some conditions or injuries can be treated through transplantation of entire healthy organs, but in the United States there is a shortage of available organs. Stem cells can serve as an alternate and renewable source.

Currently, researchers are investigating the use of adult 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. For example, in Parkinson's disease, embryonic stem cells may be used to form a special kind of nerve cell, a kind that secretes dopamine. These nerve cells can theoretically be transplanted into a patient where they will re-wire the brain and restore function, thus treating the patient.



# Stem Cells and Diseases

## The Promise of Stem Cells

Studying stem cells will help us 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 needing a transplant far exceeds the number of organs available for transplantation. Pluripotent stem cells offer the possibility of a renewable source of replacement cells and tissues to treat a myriad of diseases, conditions, and disabilities including Parkinson's and Alzheimer's diseases, spinal cord injury, stroke, burns, heart disease, diabetes, osteoarthritis and rheumatoid arthritis.

## Have human embryonic stem cells successfully treated any human diseases?

Scientists have been able to do experiments with human embryonic stem cells (hESC) only since 1998, when a group led by Dr. James Thomson at the University of Wisconsin developed a technique to isolate and grow the cells. Moreover, Federal funds to support hESC research have been available since only August 9, 2001, when President Bush announced his decision on Federal funding for hESC research. Because many academic researchers rely on Federal funds to support their laboratories, they are just beginning to learn how to grow and use the cells. Thus, although hESC are thought to offer potential cures and therapies for many devastating diseases, research using them is still in its early stages.

Adult stem cells, such as blood-forming stem cells in bone marrow (called hematopoietic stem cells, or HSCs), are currently the only type of stem cell commonly used to treat human diseases. 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 adult stem cells has also been demonstrated in the treatment of other human diseases that include diabetes and advanced kidney cancer. However, these newer uses have involved studies with a very limited number of patients.