

# Stem Cells and the Future of Regenerative Medicine

## The Basics on Stem Cells

Stem cells are the primitive cells that give rise to the different tissue types in the body (for instance skin, muscle, and blood). Stem cells may be found in embryos during early stages of development, in fetal, umbilical cord, and placental tissue, and in some adult tissues. *Researchers hope they can use stem cells to generate specific tissues, such as nerve, heart, lung, or kidney tissue, which could then help repair damaged or diseased organs or provide alternatives for organ transplants.*

## The Promise of Stem Cells

Stem cell therapy could offer hope for millions who suffer from *debilitating diseases and conditions for which there are limited or no treatments*; cardiovascular disease, autoimmune diseases, diabetes, osteoporosis, cancer, and many more. These hopes are inspired by the historical success of using adult stem cells from bone marrow to successfully treat patients with leukemia and other cancers, inherited blood disorders, and diseases of the immune system.

However, stem cell research is still in its infancy, and there are substantial gaps in knowledge that pose obstacles to the realization of new medical therapies. Stem cell research using human embryonic stem cells, considered by many to be the most promising avenue of research, is controversial given the diverse views held in our society about the moral and legal status of the early embryo.

## The Debate

*When does an embryonic stem cell become a human being?* This is a fundamental question in the stem cell debate. *Few issues have provoked more debate than when life begins in this country and abroad.* Some religious traditions suggest that life begins several weeks or even months after conception. Others consider life to begin at conception. The acceptability of human ESCs for research is also influenced by the source of cells. Stem cell cultures derived from embryos produced in fertility clinics that are no longer needed for reproductive purposes are acceptable to some. Others accept the use of stem cells derived from embryos created specifically for research from eggs and sperm donated by volunteers who are unrelated to each other and have no reproductive intent.

## The Road to New Therapies: Multiple Avenues of Research

Most experts agree that medical therapies based on stem cells are still several years away. The National Academies' report *Stem Cells and the Future of Regenerative Medicine* recommends keeping as many avenues of research open as possible in order to promote rapid advances and efficient translation of outcomes to pave the way for therapeutic advances for millions of Americans. Therefore, research on both adult and embryonic stem cells is warranted.

Potential US Patient Populations for Stem Cell-Based Therapies.	
High incidence of the following conditions suggests that stem cell research could potentially help millions of Americans.	
Condition	Number of Patients
Cardiovascular disease	58 million
Cancers	8.2 million
Autoimmune diseases	30 million
Diabetes	16 million
Osteoporosis	10 million
Alzheimer's disease	5.5 million
Parkinson's disease	5.5 million
Burns (severe)	0.3 million
Spinal-cord injuries	0.25 million
Birth defects (per year)	0.15 million

## *Public vs. Private Funding*

In the view of the committee that authored the report, federal funding of basic research is essential to speed the progress of bridging laboratory research to the patient's bedside. *The National Institutes of Health (NIH) is the largest public sponsor of basic biomedical research in the United States*, with a budget of close to \$27 billion this year. Public sponsorship of such research ensures that many scientists can pursue a variety of research questions and that their results are made widely accessible to the public. Although private and nonprofit entities also support basic research, most private sector funding comes from companies with an interest in research that will yield commercial applications, such as new drugs, diagnostic tools, and medical devices. Additionally, results from privately funded research are often considered proprietary information and not openly exchanged or shared with the scientific community.

## *Need for Oversight*

Stem cell research should be conducted under established standards of open scientific exchange, peer review, and public oversight. *Oversight can ensure that the most efficient and responsible means necessary to achieve medical breakthroughs will be used and that the many ethical dilemmas and scientific uncertainties of which it raises will be addressed.* A national advisory board established by NIH could provide needed oversight for proposals for federal funding to work with embryonic stem cells to make sure they are justified on scientific grounds and that they meet current and future federally mandated ethical guidelines.

## **Perspectives on Public Policy**

In August 2001, President Bush articulated federal funding guidelines that permit research with cells from approximately 60 stem cell lines identified by the NIH. Currently, there is no federal law or policy prohibiting the private sector from creating stem cells for research. *The policies of most individual states currently permit private funding of the use of embryonic stem cells for research, although a few states have banned some forms of research with these cells.* California recently became the first state to authorize public state funding for research using embryonic stem cells. Its new law, effective Jan. 1, also requires doctors to tell individuals receiving infertility treatment about the possibility of donating excess embryos to research, and prohibits their sale.

### *Report Recommendations*

Human embryonic stem cells hold promise for treating some of the world's most debilitating diseases, but this research also raises many ethical and legal concerns. This research touches upon some of the most fundamental issues our society has grappled with over the centuries, including the definition of human life and the moral and legal status of the human embryo. *Stem Cells and the Future of Regenerative Medicine* makes several recommendations:

- Research with human stem cells should be conducted to make progress against human disease.
- Research on both embryonic and adult stem cells should be pursued.
- Current stem cell lines should be rigorously monitored for mutations, which may affect the results of future research.
- Human stem cell research should be publicly funded in order to ensure open scientific exchange, peer review, and public oversight.
- Strict federal guidelines should be established to ensure sound scientific grounds for proposed studies.
- A national advisory group composed of exceptional researchers, ethicists, and other stakeholders should be established at the National Institute of Health to oversee research on human embryonic stem cells.
- Research should be conducted with stem cells to investigate genetic manipulation techniques that will promote organ and tissue transplantation.

For More Information...

Copies of *Stem Cells and the Future of Regenerative Medicine* are available for sale from the National Academy Press; call (800) 624-6242 or (202) 334-3313, or visit the NAP homepage at [www.nap.edu](http://www.nap.edu).

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