



A Catalyst for Cures: Embryonic Stem Cell Research

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Executive Summary

Researchers who study human embryonic stem (hES) cells are working to devise cures for some of our most intractable diseases. On this 10th anniversary of the discovery that hES cells could be grown in culture, top U.S. stem cell scientists reflected on the promises and challenges of research with these extraordinary cells. These conversations reveal progress in areas unanticipated a decade ago, and validate the researchers' dreams that patients are likely to experience real benefit. They also lead to the unmistakable conclusion that in order to harvest the full rewards of the scientific successes thus far, the Federal government must resume its traditional role of full partner by lifting the restrictions imposed on Federal funding.

The phase 1 goals of hES cell research have been achieved. hES cells have become an important vehicle for learning about tissue development and discovering the genes involved in self-renewal. And researchers have learned how to coax hES cells to form many kinds of cells in the human body, including heart cells, motor neurons, and dopamine-producing brain cells.

With the knowledge gained in the past decade, stem cell research is more promising than ever. Researchers at U.S. universities, medical centers, and in industry are moving toward safer and speedier drug development and devising hES cell-based treatments. These efforts may move the study of disease from people to Petri dishes. They are growing the cell types that are damaged or die in various forms of disease, such as Lou Gehrig's disease (amyotrophic lateral sclerosis) and using them for drug discovery. They are growing human heart cells and liver cells and testing whether new drugs are safe against these sensitive tissues—before the drugs ever enter testing in patients. Improvements in predicting failures before patient testing could save \$100 million in development costs per drug, according to a report by the U.S. Food and Drug Administration.

Access to hES cells is crucial to continued progress. These pluripotent cells that can self-renew are an unmatched research tool for understanding the body and what goes wrong in disease. Researchers refer to them as “the gold standard” because these are the cells with the greatest potential for making any cell type in the body. Study of these cells has led to the development of other potential sources of self-renewing cells, such as the reprogramming of adult skin cells to make induced pluripotent stem (iPS) cells. The ability to make iPS cells demonstrates the power of hES cell research to transform science and create new medical opportunities. Researchers will continue to test iPS cells against hES cells to determine their potential and limitations. Continued research on hES cells may reveal other ways to accomplish regenerative medicine, but the paradigm-shifting discoveries come when scientists have access to all avenues of exploration.

Federal restrictions are hindering collaborations and slowing research. Biotech firms that are eager to share cell lines with researchers to speed discoveries, are restricted from doing so if those researcher are receiving Federal funding for that research. Seldom does the Federal government decline to fully support research that has such potential to solve major public health problems. Stem cell research is one of the most exciting fields of study for young researchers, yet many are hesitant to enter a field with an uncertain future and funding restrictions. In addition, the restrictions fly in the face of the diversity requirements established by the Federal government for clinical research. The federally approved lines do not represent the diversity in our society, which is a critical part of ensuring that new medicines work for everyone.

Biotech firms are betting on the future of stem cell research and pharmaceutical companies have begun to invest as well. They see an electrifying future in using hES cells as tools for testing and developing new drugs. Some firms are moving swiftly toward the clinic to test cell therapies in humans. For example, Geron Corporation and scientists from University of California, Irvine, are exploring the challenges of implanting hESC-derived glial cells in patients to repair acute spinal cord injuries. They are close to clinical testing. Researchers need the time and support to address safety concerns for using hESC-derived therapies in humans. To explore what happens to cells once they are transplanted, determine how the body reacts, reveal any health risks, and see how the therapy interacts with important medications.

Based on hES cell research, scientists see great promise in efforts to improve therapies for diabetes, Parkinson's disease, macular degeneration, cancer, spinal cord injuries, and heart disease. The time for removal of restrictions, expanded support, and implementation of relevant oversight guidelines is now.

Experts interviewed for this paper:

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Main Body

Human embryonic stem cell (hESC) research has reached a 10-year milestone. In 1998, James Thomson and John Gearhart separately announced they had successfully grown the first human pluripotent stem cell lines in culture, cells that can self-renew and give rise to various cell types in the body. Despite limited funding, scientists have made great strides in using these primary cells to understand what goes wrong in disease and begin to devise promising new therapies for devastating conditions, such as heart disease, spinal cord injury, and diabetes. Conversations with some of the nation's top stem cell researchers—in academia and industry—make clear that, with removal of limits on Federal funding, hESC research will fulfill its promise in broader ways than originally anticipated.

It is time for the government to become a full scientific partner in supporting the broad range of stem cell research so that the greatest public benefit can be achieved on the shoulders of the last 10 years' accomplishments.

Human embryonic stem cell research has achieved its phase one goals.

In 1998, researchers imagined that hES cells could be made into any kind of cell in the body. Ten years ago, this was a hope. Today it is fact. Researchers have shown that stem cells from embryos have the ability to become many of the roughly 210 cell types in the human body. They have coaxed hES cells to form heart cells, dopamine-producing brain cells, motor neurons, bladder tissue, kidney tissue, and others.

Two out of three major, early goals for hES cells have been met:

1. hES cells would be a vehicle for learning about tissue development and about the relationship of tissues and genes. They would lead to the discovery of the genes involved in self-renewal. Those promises have been realized.
2. hES cells would offer a path to new treatments. Now that scientists know how to make heart muscle and dopamine-producing cells, for example, the goals of cell therapies have moved from the theoretical to the concrete. hESC-derived cells are beginning to be used for early toxicity screening and new drug discovery, as well.
3. There would be widespread use and testing of these cells. Restrictive Federal policies severely diminished that expectation.

“Everything we expected hES cells to do, they are doing,” says James Thomson, University of Wisconsin. “They’ve proven themselves.”

**“10 years ago, human embryonic stem cells offered hope.
Today they offer solutions.”**

— Hans Keirstead, University of California, Irvine

Stem cell research is more promising today than it was 10 years ago.

“In the next decade, most advances will come from drugs that affect progression of disease. And we’ll get there by using hES cells as test beds for new therapeutics,” says Doug Melton, Harvard Stem Cell Institute.

After 10 years of experience with hES cells, scientists know what needs to be done to make ES-based cells useful for patients—and the opportunities go far beyond cell transplantation.

The scientific community is asking questions it would not have asked if it didn't have access to hES cells. And hES cell studies led to the unexpected development of induced pluripotent stem (iPS) cells, adult cells that are reprogrammed to an embryonic stem cell-like state by being forced to express factors important for maintaining pluripotency. Now we even have proof that you can take a fully mature cell and put genes into it and drive it in a different direction. Pancreatic exocrine cells, for example, can be transformed to pancreatic beta cells, the cells that are destroyed in type 1 diabetes. All of these advances are a result of hES cell research.

The big revolution in the next 10 years will focus on the ability to make the cell types that get sick and use them for drug discovery, removing the study of disease from people to a Petri dish. This has never been possible before—and it's one of the main reasons the drug pipeline has not been flowing with new, effective drugs.

The rise of serious heart risks from drugs that treat chronic conditions has become a top concern at the U.S. Food and Drug Administration (FDA). A potential solution comes from companies making panels of hES cells—cardiac, liver, kidney—to test new drugs for toxicities before they ever enter testing in patients. Only the most effective and safest candidates would move on to animal studies and later patient testing. Imagine if the makers of Vioxx had been able to test this drug on heart cells in a Petri dish and learn about its toxicities before it could cause those estimated 28,000 heart attacks and sudden cardiac deaths in humans.

Today, a new medicinal compound entering Phase 1 testing, usually after a decade or more of preclinical screening and evaluation, has about an 8 percent chance of reaching the market. Unpleasant surprises during human studies are the biggest contributor to the cost of bringing a new drug to market. Improving predictive toxicology testing before new drugs are tested in people would have major consequences: "...a 10-percent improvement in predicting failures before clinical trials could save \$100 million in development costs per drug."

Source: *FDA Report: Innovation and Stagnation — Challenge and Opportunity on the Critical Path to New Medical Products*, March 2004 (<http://www.fda.gov/oc/initiatives/criticalpath/whitepaper.html>).

Currently, predictive toxicology is limited to studies in rodents that are inbred. They don't come in the diversity that humans do. Even among humans, different populations metabolize drugs differently. A series of cells from different hES cell lines or iPS cell lines from humans of different genetic backgrounds would be extremely powerful. In addition, a drug may appear effective in mice, but not work in humans. And, there are drugs that failed in mice, but were effective in humans. Imagine moving the whole pharmaceutical industry to doing all drug screening on human cells, and not having to put a patient at risk

The most ambitious proposal is commonly referred to as cellular replacement therapy. In just 10 years, scientists have been able to go from a primary discovery—finding they can grow hES cells in a dish—to being ready to test a therapy that involves transplanting cells derived from hES cells into patients. The FDA is preparing for hES cells' entering clinical studies. Its Cellular, Tissue and Gene Therapies Advisory Committee met in April 2008 to determine how to measure the safety of hES cell therapies. In Spring 2008, the biotech company Geron submitted the first IND (investigational new drug) application to the FDA to use hES-derived cells in human therapy, for the repair of acute spinal cord injury.

"We will use ES cells in the clinic," says Melissa Carpenter, a consultant and former researcher at Geron and Novocell. "When Geron gets its clinical trial approved by the FDA, others will come along."

"I'd be very surprised if, during the course of my scientific career, the next 20 years, we don't have much better therapies for Parkinson's disease, based on the fact that we have these hESC-derived tissues in culture," says James Thomson. After gaining a much better understanding of the mechanisms of the disease using nerve cells derived from hES cells and iPS cells, scientists will devise treatments to stop progression in patients with early disease.

**"Human embryonic stem cells are still incredibly valuable.
We set them aside at our own peril."**

—George Daley, Harvard Medical School

Leading scientists are adamant that hES cells still have a crucial role.

"At this time it is still undetermined which stem cell types will prove the most useful for regenerative medicine, as most likely each will have some utility. Therefore, the need for research with human embryonic stem cells still exists despite the availability of new cell sources." From NAS report, *2008 Amendments to the National Academies' Guidelines for Human Embryonic Stem Cell Research*.

hES cells are an unbeatable research tool to understand the body and what goes wrong in disease. Just like telescopes opened new vistas to distant galaxies, hES cells offer unprecedented access to the human body. Scientists are using hES cells to grow limitless quantities of various tissues, such as heart muscle cells. It will be a vast improvement over today's studies of the physiology of the human heart, which rely on limited biopsy samples from sick hearts.

Some opposed to hESC research have argued that we don't need hES cells anymore, now that iPS cells have been developed. But if we have learned anything in the history of stem cell research, it is that we have not been very good at predicting which cells are most useful for which applications. To devise new therapies, research must continue with all types of stem cells. If we allow research on hES cells to wither, who knows how many other breakthroughs, like adult cell reprogramming, will go undiscovered. Although iPS cells show great promise, preliminary studies indicate they are not identical to hES cells and may not be as useful for some applications. And there appears to be significant variation between iPS cell lines, probably more so than between human ES cell lines. Further studies are necessary. In terms of safety, iPS cells are much further from the clinic than are ES cells. At present, they are made with genes and viruses that can cause cancer.

"We are doing very careful comparisons of how well iPS cells and hES cells make motor neurons, and how functional those cells truly are," says Kevin Eggan, Harvard University. "NIH should be funding both activities."

Every study of iPS cells requires hES cells for controls and comparisons. These comparisons are crucial for moving the iPS field forward. Researchers must test the safety and efficacy of iPS cells against hES cells. Continued research on hES cells and others may reveal other ways to accomplish regenerative medicine. But we are not there yet.

Finally, and importantly, Federal restrictions on hES cell lines are a social justice issue. The Federal lines do not represent the diversity in our society. If hES cells have the potential to change the future of medicine, our Federal government has imposed restrictions that might lead to minorities being left out of that future. The same Federal government that insists on enrolling diverse patients in any clinical trial to ensure that new medicines work in everyone, insists that researchers do all work on hES cells that are from a small number of sources.

What about adult stem cells?

Many scientists have been studying adult stem cells and learning more about their utility and their limitations. So far, adult stem cells have only successfully been used in a very narrow area: blood system reconstitution, including bone marrow transplant, umbilical cord transplant, and peripheral blood transplant.

“The argument that there are 60 to 70 diseases that can be cured with adult stem cells was never credible.”

— Sean Morrison, University of Michigan

With access to all avenues of exploration, scientists make unexpected, paradigm-changing discoveries.

“If you’re a botanist, you don’t study one type of tree to learn everything about trees,” says Ole Isacson, Harvard Medical School.

In 2006, Yamanaka first reported he had turned mouse adult skin cells into stem cells. Then late in 2007, Yamanaka, Thomson, and Daley each reported that they had successfully turned human adult skin cells into stem cells. The development of these iPS cells was unexpected. The prevailing wisdom was that, once a cell had become differentiated or specialized, it could not be turned back by any method other than nuclear transfer. But that’s just what iPS does. iPS cells are a victory for ES cells. They demonstrate the power of stem cell science to lead to unexpected and field-changing breakthroughs.

Recall the recombinant DNA debates of the mid 1970s. Some thought recombinant DNA technology was so dangerous it should be banned outright. But reason prevailed, and science went forward, and recombinant DNA has dramatically changed human medicine. For example, insulin was cloned using recombinant DNA technology. If decision-makers had sided with fear, and stopped this research, millions of people who are thriving on recombinant insulin would have died.

Somatic cell nuclear transfer (SCNT) is another example of a technology with promise that has faced unexpected challenges. Oocyte availability, for example, has been problematic. Yet the challenges are worth overcoming.

“SCNT is the only known procedure for completely and normally reprogramming a cell,” says John Gearhart, University of Pennsylvania. Because SCNT is more efficient than iPS cell technology for reprogramming cells, and can be done without inserting new genes, continued studies of SCNT could help scientists find the linchpin to make reprogramming factors more efficient and effective. SCNT will also provide fundamental insights into how an egg reprograms that will teach a great deal about basic biology.

The bottom line is, all areas of stem cell research should remain open. “It’s not our job to guess right now what we will need in the future and how,” says Sean Morrison, University of Michigan. “This is a war against disease and it needs to be fought with all weapons.”

Federal restrictions are hindering collaborations and slowing research, and they are discouraging young, fresh minds from being able to safely enter an extremely exciting field.

“When I was at Novocell, we developed an hES cell line under clinical manufacturing conditions, as close to GMP (good manufacturing practice) conditions as possible,” says Melissa Carpenter. “The idea was to take this cell line and use it in the clinic. We would have also liked to share this cell line more broadly with other researchers, but it is administratively complex to do so since even though non-approved cell lines can be used by NIH-funded researchers, the researcher can’t use NIH money for the research using the lines. Without those restrictions, we’d learn so much more and so much faster.” U.S. companies are going to foreign researchers for collaborations. In addition, researchers setting up their first lab are less likely to go into a politically controversial field where NIH funding is questionable, when they are under pressure to get grants and achieve tenure.

“Young researchers face a double whammy: flat NIH funding on top of 20 years of tepid support from the Federal government, and even opposition to a lot of the research,” says George Daley.

An ethical framework exists.

“Embryonic research is not the unregulated ‘wild west’ of science,” says Sean Morrison.

Guidelines for the ethical oversight and ongoing monitoring of stem cell research have been developed by the National Academies and the International Society for Stem Cell Research. Many institutions have already adopted these guidelines, which require each institution conducting stem cell research to have a protocol-by-protocol ethical review of the work by a committee of ethicists, scientists, legal experts, and community members. These so-called ESCRO (Embryonic Stem Cell Research Oversight) committees are focused specifically on the ethical implications of using human embryonic stem cell lines in scientific research. Additional supervision is afforded by other pre-existing federally mandated oversight committees, such as institutional review boards in the case of human subjects research, animal care committees, and committees on biological safety. If Federal policies change, there is already a framework in place to devise Federal guidelines on the use of these material.

Biotech firms are revving up, focused on toxicity screening and drug development. A few are aggressively pursuing hES cell-based therapies. Big Pharma is also beginning to invest in stem cells.

“Embryonic stem cells are a source of cells for predictive toxicology and drug discovery,” says Melissa Carpenter.

“Industry is embracing this approach,” says Doug Melton. “They tell us this is the best idea for solving their pipeline problems.”

See Appendix for a list of industry examples.

The political climate is changing.

Public opinion strongly favors embryonic stem cell research. Nearly three-quarters (73%) of Americans believe that President-elect Obama should keep his pledge to lift existing Federal restrictions on embryonic stem cell research, according to a national poll conducted for CAMR in January 2009 by Opinion Research Corporation. November’s U.S. election also showed the public’s support. In Michigan, voters decided to let scientists derive new human embryonic stem cell lines from embryos donated by couples getting fertility treatments. Michigan had one of the country’s most restrictive laws on embryonic stem cell research and the ballot proposal to loosen restrictions succeeded despite an extremely well-funded opposition campaign. A Colorado measure that would define a fertilized egg as a human being was also defeated.

We've only just begun.

“It’s like discovering nuclear power...and now we have to figure out how to use it,” says Ole Isacson. His group discovered they could make dopamine-producing cells from hES cells in 2002. But it took much more work to learn how to modify the factors that control the process, and how to do it consistently and with high efficiency. Research takes time. A few examples from history:

- The poliovirus was first isolated in 1909. It took 45 years to get to the Salk vaccine.
- HIV was first isolated in 1983. Bringing all the power of modern virology—and billions of dollars to bear—hundreds of scientists are still working on combating this virus.
- The first attempt at bone marrow transplantation between an unrelated donor and recipient was in 1955 by E. Donnall Thomas—after many years of research. All six patients died. He went back to the lab to figure out why. The first successful transplant of these adult stem cells occurred in 1969—14 years later. It took years more of clinical testing to get it right, and bone marrow transplantation only became a common procedure in the 1980s. If opponents, after 10 years of study of these adult stem cells, had said ‘there’ve been no cures, let’s stop’, then adult cell transplantation would not exist and countless lives would not have been saved.

Scientists need the time and support to overcome safety concerns for using ES cells in patients. There won’t be products for patients unless scientists can devise ways to eliminate risk. Parkinson’s researcher Ole Isacson is sorting stem-cell derived neural cells to remove those that have tumor-cause potential. This isn’t headline-grabbing, but it’s critical science. Researchers need to explore what happens to cells once they are transplanted, determine the immune system’s reaction to the cells, and see if there are cancer risks. Are the cells compatible with any drugs the patient might be taking for their disease or other conditions? Can the cells tolerate those drugs?

Progress is Tangible on Several Disease Fronts.

Cancer chemotherapy: Researchers are studying ES cells, close cousins to cancer cells, to learn how cancer cells replicate. ES cells may be used as a drug target for cancer cells. They may also be used to personalize cancer therapies. Before a cancer patient takes a chemotherapy regimen that is extremely toxic, doctors could take a skin cell from the patient, and through the iPS process, create liver cells and heart cells. The chemotherapy could be tested first on those patient’s “own” cells. A new drug regimen would only be given to the patient after it is clear that the drugs will kill the cancer, not the patient.

Diabetes: “Everything we’ve learned says we will get there. If there are seven steps to turn an ES cell into a pancreatic beta cell, we’ve solved two of the steps,” says Doug Melton. “We will get there, but it may be a year or a decade.” Geron and Novocell are developing cell therapy. Geron has shown it can put hES cell-derived therapy in mice and improve function and extend life.

Spinal cord injury: Spinal cord cell types were the first high purity, commercially scalable cells derived from hES cells. Hans Keirstead’s group at UC Irvine has been working with Geron to address the four major challenges in treatment development: manufacturing the product so it is suitable for human use, studies to show the treatment works in animals, and is safe in animals, and development of a clinical plan to apply the treatment to humans. Geron has submitted an IND to the FDA to test its product to activate glial cell repair within 7 days of spinal cord injury.

Parkinson’s disease: Several groups have made dopamine-producing nerve cells from hES cells and iPS cells. They have shown they work in animal models. Scientists are also using the nerve cells to study the mechanisms of the disease in hopes of devising treatments that will stop its progression.

Age-related macular degeneration: hES cells converted into the special cells that line the base of the retina have rescued vision in rats with a form of this blinding disease.

Amyotrophic lateral sclerosis (ALS) or Lou Gehrig's disease: Ordinary skin cells taken from patients with ALS were transformed into iPS cells and then motor neurons, the cells that waste away and die in ALS. Now the researchers can make unlimited supplies of these cells to uncover the mechanisms behind this disease and screen for drugs that can prolong life.

Heart disease: Scientists have made cardiac cells from hES cells that beat in a dish. Geron has a very large program in this area. It will likely be their second clinical application. They've seen functional improvements in animal models. A group in Wisconsin is building toxicology screens using cardiomyocytes, since heart toxicity is the most common drug toxicity today, along with liver toxicity. This group is testing drugs that have already failed clinical testing on cardiomyocytes from hESCs as early predictors of drug failure. They're not putting cells into patients, but it will have a huge impact on patients.

Appendix:

Biotech and pharmaceutical companies are investing in embryonic stem cell research.

Cellartis, in Sweden, signed an agreement with **Pfizer** to develop an hES cell platform for human toxicity screening in vitro, using liver cells and heart cells from hES cells.

Cellular Dynamics International (CDI), in Madison, Wisconsin, uses iPS cells and hES cells to produce large quantities of fully functional cardiomyocytes and other cell types for human in vitro drug safety screening. Differentiation protocols originally developed with human ES cells are now being used with human iPS cells to dramatically extend the genetic diversity of cells examined in drug screens. CDI is collaborating with Roche to test compounds for cardiotoxicity and build predictive models using iPS cell derived cardiomyocytes.

Geron Corporation in Menlo Park, California, filed an IND to begin clinical testing of its hES cell-derived oligodendroglial progenitor cells as a therapeutic for acute spinal cord injury. The FDA put the application on hold in May 2008. More recently, Geron published data with researchers from University of Alberta, showing the successful engraftment of hESC-derived pancreatic islet-like clusters (ILCs) in diabetic mice (*Cell Proliferation*, published online October 19, 2008). After transplantation, the ILCs continued to express important pancreatic islet proteins, responded to high levels of glucose in the blood, and extended the survival of recipient animals. The company is also testing hESC-derived heart cells, hESC-derived cartilage progenitor cells for joint repair, hESC-derived hepatocytes for treatment of liver failure, and hESC-derived osteogenic progenitors for bone regeneration.

Novocell in Southern California is expected to be first at bringing an hESC-based diabetes treatment to patient studies.

Stem Cells Inc., in Palo Alto, California, reported October 30 that its proprietary purified human neural stem cells, when transplanted into rats, can protect the retina from progressive degeneration, which leads to loss of vision in diseases such as age-related macular degeneration and retinitis pigmentosa. This study was conducted by researchers at Oregon Health & Science University.

VistaGen, in South San Francisco, California, working with scientists in Toronto and at New York's Mount Sinai School of Medicine, identified key biochemical pathways that enabled them to produce large numbers of functional, highly pure, cardiomyocytes (heart cells) from hES cells (*Nature Biotechnology*, published online Sep 28, 2008). The aim is to use these cells for improved drug safety screening and development, to reduce clinical development failures and lower drug development costs. Earlier in the year (*Nature*, April 23, 2008), the company reported it had encouraged ES cells to grow into three different types of cells important to cardiovascular development (cardiomyocytes, endothelial cells, and vascular smooth muscle cells).

AstraZeneca, GlaxoSmithKline, and Roche—joined with five agencies of the UK government to launch **Stem Cells for Safer Medicines (SCASM)**, a novel public-private consortium. Structured as an independent company, its mission is to use hES cells to catch drug-safety problems in petri dishes, not in patients. It's also hoped that the technology developed will eventually replace some of the millions of animals currently sacrificed in preclinical testing.

GlaxoSmithKline Plc committed in June 2008 to give the Harvard Stem Cell Institute at least \$25 million over five years to explore using stem cells to fight cancer, diabetes, and obesity along with nerve, heart, and musculoskeletal diseases.

Pfizer is putting together a large group of pharma companies in Cambridge, MA, and in Cambridge, U.K. that are actively engaged in cell replacement therapy. The Massachusetts group will focus on heart disease and diabetes. The UK shop is ophthalmology and central nervous system. The first uses will be early-stage safety testing. But eventually, work could shift to creating new tissues to be used to treat disease—new heart cells, for example, could repair damaged tissue. Pfizer is also funding EyeCyte, a startup in San Diego that's working on a treatment for retina damage with stem cells identified in bone and blood-marrow that could repair damaged blood vessels in the eye.

“Pfizer has put its flag in the ground that there is a future in regenerative medicine,” Corey Goodman, Pfizer's President of Biotherapeutics and Bioinnovation Center, told *Forbes*.