



Coalition for the Advancement of Medical Research

Dedicated to Advancing Stem Cell Research

STEM CELL RESEARCH DEVELOPMENTS 2008

Overview

Exciting stem cell breakthroughs capture the public's attention and receive considerable news coverage. CAMR applauds these advances and continues to follow the lead of the science community which remains committed to the promise of human embryonic stem cell research. Therefore, CAMR will continue to work to enact the Stem Cell Research Enhancement Act.

CAMR's ultimate mission is to help end the suffering of the 100 million Americans with diseases and conditions that may someday be treated or even cured through progress in the field of human embryonic stem cell research. CAMR supports all ethical research that unlocks the secrets of pluripotent cells -- cells that can develop into any cell type. The world's leading scientists assert that embryonic stem cell research remains the most promising key to demystifying these cells. The recent breakthroughs, while laudable, by no means obviate the need for further embryonic stem cell research. Embryonic stem cell research is more important now than ever.

Recent Developments

This fact sheet contains summary descriptions of recent stem cell-related developments that have been in the news. The first, induced pluripotent stem cells (iPS), garnered the most attention as it reprogrammed adult cells to a pluripotent state. The second breakthrough, conducted by a company called Advanced Cell Technology, uses a controversial technique to remove cells from an early-stage embryo. Theoretically, this process does not harm the embryo but further research is needed to understand and effectively replicate this process. Finally, an announcement from the company Stemgen has refocused attention on somatic cell nuclear transfer (SCNT), a technique supported by CAMR. The announcement of any type of breakthrough is the beginning of a long process. Any development will require years of validation before it can reach the stage of progress we now have with human embryonic stem cell research. Scientists and other experts are available for briefings or meetings to provide further details about any of this research.

Induced Pluripotent Stem Cells (iPS) by Drs. Thomson and Yamanaka

Dr. James Thomson of the University of Wisconsin and Dr. Shinya Yamanaka of Kyoto University published studies in 2007 that offer a new approach for developing what appear to be pluripotent cells. Similar studies have since been published by researchers at Harvard University. In all of the studies, genes were delivered via a virus agent to an adult cell. The genes reprogram the cell and "turn back the clock" -- reverting the cell to a pluripotent state that can be used to generate stem cell lines. Because iPS uses adult cells (one study used discarded tissue from newborn circumcisions) and does not require a human egg or embryo, many assert that this research ends the need for embryonic stem cell research. However, there are several safety concerns about this technique such as the high frequency of tumor development and the hazards associated with using a virus to deliver the genes. Because of this, Thompson, Yamanaka and other scientists continue to see the need for embryonic stem cell research.

What Leading Scientists are Saying about iPS

Certain, important medical questions can only be examined through embryonic stem cell research. As Dr. George Daley, a member of the Harvard Stem Cell Institute's Executive Committee and President of the International Society for Stem Cell Research has noted, "despite success in generating iPS cells, we are not abandoning our efforts to derive new human stem cell lines by nuclear transfer. We are not yet certain which type of cell will prove most useful for medical applications. Besides, nuclear transfer is an experimental method that asks very important questions that will never be answered by reprogramming skin cells with defined genes."

The iPS discoveries were derivatives of embryonic stem cell research. It is only because embryonic stem cell research was conducted first, that we have iPS now. Dr. Yamanaka has written, "...the recent advancements in iPS cell research would not be possible if it were not for the many years of dedicated hES cell research [human embryonic stem cell research] that preceded them. We cannot support the notion that iPS cell research can advance without hES [human embryonic stem] cell research." (Cell Stem Cell, October 2007, coauthored article with Drs. Insoo Hyun, Konrad Hochedlinger, and Rudolf Jaenisch)

There are safety concerns associated with the iPS model. As Dr. Story Landis, chair of the NIH stem cell task force, asserted in public comments at the Parkinson's Action Network Forum (February 2008) one of the "major potential hopes for stem cells is that scientists could use pluripotent stem cells to create tissue replacement therapies for diseases." However, Landis said scientists working on iPS successfully reprogrammed these cells by using viruses to introduce the reprogramming genes, one of which is known to cause cancer, thereby making this method in its current form "absolutely" unsuitable for any kind of transplant. "So while this is a huge scientific step forward, there are many unanswered questions," she said.

We do not know enough about pluripotent cells to know if the iPS versions are identical and it is unlikely that they will behave like exact copies. Dr. Landis stated at the American Health Lawyers Association's (AHLA) annual conference (January 2008), "The game isn't over because we don't actually know that these cells [iPS] are identical to human embryonic stem cells. There are in fact differences," she said. "The likelihood and it is my personal belief, that you end up with something identical to that pristine human embryonic stem cell is about zero. We don't know. It's a very interesting question, and scientists are certainly looking at that." Continued embryonic stem cell research is required to answer those questions. Rather than obviating the need for embryonic stem cell research, iPS provides another testament to the importance of such research.

More work is needed to validate the iPS results. Dr. Kevin Eggan, a leading researcher at Harvard University stated in a Science article (February 2008) that "to validate iPS cells, scientists must make huge [numbers]...from many different people and compare them in a battery of tests with embryonic stem cells." Validating the results requires moving forward with embryonic stem cell research.

Federal limitations should be overturned and removed. After his iPS announcement, Dr. Thomson asserted in an editorial co-authored by Dr. Alan Leshner (chief executive of the American Association for the Advancement of Science and executive publisher of the journal *Science*) in *The Washington Post* (December 3, 2007) that, “We hope Congress will override the president's veto of the Stem Cell Research Enhancement Act. Further delays in pursuing the clearly viable option of embryonic stem cells will result in an irretrievable loss of time, especially if the new approach fails to prove itself.”

All avenues of research should be pursued. Writing in the journal *Cell Stem Cell*, Drs. Yamanaka, Konrad Hochedlinger (Harvard Stem Cell Institute’s Principal Investigator) and Rudolf Jaenisch (MIT’s Whitehead Institute) assert that “We hold that research into all avenues of human stem cell research must proceed together. Society deserves to have the full commitment of scientific inquiry at its service. And science is a practice that works best when it is approached with an open and creative mind. Research into one approach can inspire new ideas in unpredictable and exciting ways.”

Dr. Landis’ comments at the AHLA conference further bolster this position: "We simply don't know where the advances are going to come from for any particular disease, and as an institute director, we're responsible for 600 diseases, common diseases, rare diseases, and to say that all the answers to neurogenerative diseases are going to come from adult stem cells or reprogrammed stem cells, I think that's just unreasonable."

The iPS discoveries were possible only because embryonic stem cell research was conducted first. Dr. Yamanaka has written, “...the recent advancements in iPS cell research would not be possible if it were not for the many years of dedicated hES cell research [embryonic stem cell research] that preceded them. We cannot support the notion that iPS cell research can advance without hES [embryonic stem] cell research.” (*Cell Stem Cell*, October 2007, coauthored article with Drs. Insoo Hyun, Konrad Hochedlinger, and Rudolf Jaenisch)

Advanced Cell Technology

In January 2008, a published study in the journal *Cell Stem Cell* announced that Advanced Cell Technology (ACT), a private company, created a pluripotent stem cell line from a single cell removed from an early stage embryo without causing harm to the embryo. This process of inducing a single cell to replicate is sometimes called “parthenogenesis.” The technique of removing an embryonic cell from a blastocyst prior to implantation is often used in fertility clinics for pre-implantation genetic testing and is controversial. While now growing in use and acceptance, the long-term effects of removing the single cell from an embryo prior to implantation are not known nor fully understood. Further research is needed to answer safety questions regarding this procedure.

Concerns about the ACT Study

- The results from the ACT study have not been replicated elsewhere. ACT is a privately held company and aspects of its work are not publicly available for scrutiny.
- The “Dickey-Wicker” amendment that has been included in the Labor-Health and Human Services-Education Appropriations bill for the past several years, severely limits research and experimentation on human embryos. This has prevented federally funded scientists from doing the long-term studies needed to confirm the safety of the technique.

Therapeutic Cloning (SCNT) by Stemagen

In January 2008, private company Stemagen announced it had successfully performed a technique called somatic cell nuclear transfer (SCNT), otherwise known as therapeutic cloning, on human cells. Stemagen is the first company to announce that it successfully performed SCNT on human cells. In this process an unfertilized human egg cell (oocyte) is used to develop pluripotent stem cell lines. The genetic material is removed from the egg, and genetic material from a donor is placed in the egg. Scientists are able to induce cellular division and create pluripotent stem cells. These stem cells are a perfect match to the genetic donor and have the potential to be used for a variety of treatments. CAMR fully supports SCNT and joins with the entire scientific and ethics communities in opposing human cloning, also called reproductive cloning.

- Therapeutic cloning (SCNT) continues to be a valid and important avenue to pursue in the field of stem cell research and must remain a legal option for scientists.
- Human reproductive cloning is not supported by any ethical group or scientist. CAMR strongly supports efforts to ban human reproductive cloning by banning implantation of blastocysts created by SCNT. CAMR does not support overly broad bans that would also prohibit SCNT research.

Conclusion: CAMR Supports All Ethical Research

- CAMR supports all ethical research that will help end the suffering of the more than 100 million Americans with diseases and conditions that may someday be treated or even cured through progress in the field of embryonic stem cell research.
- Embryonic stem cell research remains the most promising avenue of research to cure diseases and end suffering.
- Other avenues of study, including iPS, are very new and will require years of validation before they can reach the stage of progress we now have with embryonic stem cell research.
- Too many patients and their families are suffering and we must not abandon the important work done to date with embryonic stem cell lines. While CAMR supports all ethical research, it is imperative that the Stem Cell Research Enhancement Act (S.5), or similar legislation, be enacted, and the federal barriers to full funding for embryonic stem cell research be removed.

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