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A Life Sciences Crucible

Stem Cell Research and Innovation Done Responsibly and Ethically

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Introduction and Summary

A Call to Innovation

It is time for the United States to stake its claim as the world leader in regenerative medicine, which promises to become a vital component of the cutting edge of life sciences research and innovation in the 21st century. To ensure research in this newly emerging field of life sciences is conducted responsibly and ethically, the federal government must reform its stem cell research policy in order to fund embryonic stem cell research that is robust and comprehensive as well as cautious and principled.

Regenerative medicine is a new therapeutic approach that works by cultivating a small sample of a patient's own cells, reprogramming them, and using them to heal the patient without the risk of rejection or severe side effects that usually result from introducing foreign therapeutic materials. The potential therapies range from transforming the pancreatic cells of diabetics so they can produce insulin to reconnecting the nerves in severed spinal cords. Indeed, there have already been some modest clinical applications where heart muscles and cartilage have been repaired with stem cells derived from bone marrow.

But that is just the tip of the iceberg. The greatest potential for regenerative medicine lies in scientists' ability to tap into the process of cell differentiation and development. This can only be achieved by tracing the development of human cells from the very beginning. To do so, scientists need to conduct research on embryonic stem cells so that they can discover how these all-purpose cells can change into any one of the more than 200 different cell types in the human body.

Moreover, by studying the development of embryonic stem cells scientists will be able to discover how the human genome goes about manifesting itself and creating unique individual persons. These efforts will provide us with unprecedented insights into human development, how it can go wrong, and how it can be fixed.

Opponents of embryonic stem cell research argue that there have been many scientific advances made using stem cells that do not come from embryos, such as bone marrow-derived stem cells, which are a type of adult stem cell. Opponents also point to so-called induced pluripotent stem cells, which are created when adult cells—say, skin cells—are reprogrammed to become all-purpose "pluripotent" cells. These arguments are valid, but

only up to a point. The reason: embryonic stem cells are both the original "master cells" capable of turning into any cell in the body as well as the "gold standard" against which all other stem cells must be compared

Scientists determine whether other types of stem cells hold the promise of delivering the kinds of regenerative medicine envisioned by life scientists by analyzing the surfaces of these alternative cells to see whether they have the same proteins and therefore the same capabilities as embryonic stem cells. Evidence suggests that these stem cell-specific proteins activate certain chemical pathways in the stem cells, which in turn allow them to maintain their pluripotency. Regardless of what type of stem cells prove to be the most useful, this process of embryonic stem cell comparison must be carried out for each therapeutic application, whether for Alzheimer's disease, Parkinson's disease, spinal cord injuries, or any of the other myriad conditions for which stem cell therapy might be possible.

Just as important: embryonic stem cells must be studied so that scientists can learn more about developmental biology. It is a longstanding research paradigm to study failures of development by determining when, where, and how genes malfunction. The ultimate goal is to develop a guidebook that will tell us exactly how each gene or combination of genes contributes to the development of a unique individual. This will greatly enhance our understanding of basic genetics and could allow scientists to develop drugs that can prevent the diseases from developing in the first place.

Additionally, embryonic stem cells can aid in the refinement of these new drugs since the cells can be differentiated into specific cell types upon which scientists can quickly test whether a drug has a desired effect. This will make the drug development process and then the clinical trial process much safer and more efficient.

The bottom line is that embryonic stem cell research is good science. It is necessary science, and it needs to be part of America's federally funded biomedical research enterprise if America is to retain its status as a global scientific leader. That's why embryonic stem cell research must be conducted responsibly and ethically, and why the incoming Obama administration must outline new federal research and funding oversight guidelines for embryonic stem cell research that are cautious and principled.

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A New Federal Embryonic Stem Cell Research Agenda

The first step toward renewing U.S. life sciences leadership must be taken by the executive branch. President Barack Obama has the option of either issuing an executive order or issuing a presidential memorandum to govern stem cell research. Either way, the primary objective of the executive document must be to lift the existing temporal restriction on the federal funding of embryonic stem cell research.

JEFF MILLER/UNIVERSITY OF WISCONSIN-MADISON

Developmental biologist James Thomson works at a microscope in his research lab at the University of Wisconsin-Madison.

Currently, federal funding is only available for research on the 21 lines of embryonic stem cells that were derived before August 9, 2001. Once this arbitrary limit is lifted, the National Institutes of Health will be able to issue grants to scientists who wish to research embryonic stem cells in accordance with guidelines for ethically derived cells, including:

- The stem cells must come from embryos that were originally created at *in vitro* fertilization clinics for the purpose of fertility treatment but are now stored at these IVF clinics because more were created than required to fulfill the patient's clinical need
- Proper written informed consent is obtained from the donors
- As part of the informed consent process, the embryo donors along with the physician determine that the embryos will never be implanted in a womb and would otherwise be destroyed
- There are no financial inducements and the donors understand the purpose of the research is not to eventually confer therapeutic benefits upon the donors

Embryonic stem cell research requirements along these lines should also be codified in legislation by the incoming 111th Congress and become law so that future presidents cannot obstruct this research.

In addition, it is current policy that no embryonic stem cells will be derived using federal funds. Federal funds will only pay for research on stem cells that have already been derived with private funds.

To enforce these ethical guidelines and to ensure that all stem cell research (embryonic or otherwise) is conducted cautiously and responsibly so as not to threaten the safety or autonomy of research subjects or the donors of research materials, the following administrative oversight requirements should be included either in the president's document or in legislation that should be passed in the first session of the 111th Congress:

- The National Institutes of Health should require that all research be conducted under the review of a stem cell research oversight committee that adheres to the standards put forth in the regulations issued by NIH and HHS as informed by the National Academies or the International Society for Stem Cell Research guidelines. Any embryonic stem cells that are not in compliance with these rules, or are derived from embryos that are not in compliance with these rules, will not be eligible for federal funding.

- The one caveat to this requirement is that the 21 cell lines that were approved by the Bush administration should be grandfathered into the new policy because federal funding has already been provided for research that is now well underway.

- The NIH or the Department of Health and Human Services should adopt these rules no more than 90 days after the executive order lifts the existing restriction.

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If these requirements are articulated in a presidential document, then the 111th Congress should also codify them in legislation. The legislation should provide broad, principled ethical standards so that the science itself can evolve in the direction that experimentation and serendipity takes it—alongside easily adapted regulations governing the research based on the broad ethical standards approved by Congress. Specifically, the legislation should charge the Department of Health and Human Services with the duty to update at regular intervals its regulations for embryonic stem cell research in light of new science.

These policy guidelines will ensure that human embryonic stem cell research is carried out with the highest ethical standards. It will also ensure that U.S. public and private biomedical research laboratories live up to the highest scientific standards. In the pages that follow, we will examine in detail why these new guidelines are necessary and proper given the history of stem cell research during the Bush administration and the advances that have been made in the science since James Thomson of the University of Wisconsin at Madison first created this new life sciences arena 10 years ago. In short, this paper will demonstrate that our policy recommendations are based on good science and sound ethical principles.

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