

## **The Role of the University in Promoting Human Embryonic Stem Cell Research and Stem Cell Therapies**

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When asked about what the University should do to support or promote stem cell research, my view as a physician-scientist is that the University should allow and promote innovation. Specifically, innovation in a direction that will help us learn new knowledge about diseases and possible treatments to develop novel therapies that will push the envelope and help better treat and potentially cure those conditions that are currently incurable. As a faculty member in the Stem Cell Institute and the Department of Medicine at the University of Minnesota, my area of research interest is to use stem cells to understand blood cell development. There are many different views on the question of how we push this field of stem cell research in general, and nuclear transfer in particular, toward novel clinical therapies. In my academic life, I routinely go back and forth between the clinic and the lab. This allows clear understanding of the needs and challenges in the areas of basic and clinical research, and how to potentially bring these areas together.

As an example of the potential for successful stem cell therapies, it is informative to recall the scientific and technological innovation that developed in the field of hematology and bone marrow transplant (“BMT”). The first successful BMT anywhere in the world was performed at the University of Minnesota in 1968.<sup>1</sup> This was not the first BMT attempted, but was the first that succeeded. The patient was an

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1. Richard A. Gatti et al., *Immunological Reconstitution of Sex-Linked Lymphopenic Immunological Deficiency*, 292 LANCET 1366, 1366–69 (1968).

infant with an immunodeficiency that would typically be lethal because his body could not effectively fight common infections. He received bone marrow cells from his sister, and those cells engrafted his bone marrow and made all the blood cells he needs for an entire lifetime, including lymphocytes needed to fight infections. It is important to recognize that bone marrow transplantation is stem cell therapy. We can take bone marrow that contains blood stem cells from one person, typically a brother or sister, and transplant those to a patient with life threatening diseases, typically blood cell cancers or immunodeficiencies.<sup>2</sup> Under the proper conditions those blood stem cells will live in the body of the recipient for the rest of his/her life.

Thirty-eight years have passed since that first successful BMT, making that the longest period of time that we know a stem cell therapy will work. However, even despite considerable progress over four decades, these transplants still have a high rate of problems that cause severe, potentially lethal side effects in the recipient.<sup>3</sup> It still seems that the successful cases occur when the stars are aligned properly: you need the right donor and a recipient who is suffering from a life-threatening disease but not so sick that the treatment being done to save a life would unfortunately kill a patient instead. We have learned about the types of cancers where stem cell therapy (in the form of BMT) is effective. However, 20% or more of recipients of allogeneic transplants (from one person to another) will still die in the first year from the treatment alone.<sup>4</sup> Therefore, because of the dangers involved in the procedures, we are still not going to do an allogeneic transplant in many cases. Despite thirty-eight years of research on this, this is how far we have advanced. It is great for the patients when these transplants work—we can actually cure those diseases that would be otherwise be incurable. However, with the continued high rate of morbidity and mortality we are only going to do it on the most severe cases. For less severe diseases (*i.e.*, autoimmune diseases), the benefit

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2. Edward A. Copelan, *Hematopoietic Stem-Cell Transplantation*, 54 NEW ENG. J. MED. 1813, 1813 (2006).

3. *Id.*; see also E. Donnall Thomas & Karl G. Blume, *Historical Markers in the Development of Allogeneic Hematopoietic Cell Transplantation*, 5 BIOLOGY BLOOD & MARROW TRANSPLANTATION 341 (1999).

4. Copelan, *supra* note 2, at 1822.

may not be worth the risk.

In my perspective, we now have an established experience and body of knowledge regarding stem cell-based treatments in the form of BMT. We have room to make more real paradigm-shifting treatment changes in the field of cancer therapy. Currently, if you have diseases such as lung cancer or multiple myeloma or kidney cancer, it is great for drug companies and those performing the clinical trials if the pharmaceutical companies develop a new drug that improves your life span by six months. From my vantage point, however, this incremental advance is less than optimal. We want treatments that will not just treat but cure the underlying diseases. That is exactly what stem cell therapies have to offer. There is considerable interest at the University of Minnesota to facilitate stem cell research using everything at our disposal to push this field forward in the most innovative and most productive way possible. These directions include adult stem cells, embryonic stem cells, and potentially nuclear transfer. This is an important and rapidly developing area. The Stem Cell Institute at the University of Minnesota (and at other institutions) helps promote advances to this field, but clearly we have a long way to go before we can successfully cure a wide range of devastating diseases. The next ten years will be really exciting. New treatments and cures will come from these endeavors and support of stem cell research. However, it is also essential to communicate that many of these new treatments may not be directly cell-based therapies. Instead, these treatments will come about from these new cell-based assays and tools developed from the basic research. In my view this is really where things are, and hopefully where the field is going.

While I do not have formal training in bioethics, I certainly have a first hand perspective of the issues that are considered by patients, physicians, and researchers and have considered ethical and public policy issues related to human embryonic stem cell and nuclear transfer research. Clearly there are differences of opinion regarding ethical perspectives on this type of research. However, my view as a physician who cares for patients who die of cancer becomes quite clear, and I think typical for many working closely in this field, especially with patients who may one day benefit from advances in stem cell research. I feel that it is actually morally imperative that stem cell research, that can and will impact so many diseases, be moved forward in as expeditious a manner as possible. The list

of diseases that are often touted in terms of potential stem cell-based therapies include spinal cord injury, diabetes, Parkinson's disease, cancer, and others. All of these diseases often have no good treatments and cause significant suffering and deaths of millions of patients every year. Therefore, I think reinforcing what the role of the University in this process would be to facilitate working with policy makers, as well as have appropriate oversight by ethical and clinical trial review committees, so that these basic research studies and eventual clinical trials can be done in as open and productive a manner as possible. This is an exciting area of research that implicates science, ethics, and public policy in a field that truly represents twenty-first century medicine.