Researchers use stem cells to treat children with life-threatening, blistering skin disease

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University of Minnesota Physician-researchers have demonstrated that a lethal skin disease can be successfully treated with stem cell therapy.

Medical School researchers John E. Wagner, M.D., and Jakub Tolar, M.D., Ph.D., in collaboration with researchers in Portland, Oregon, the United Kingdom, and Japan have for the first time used stem cells from bone marrow to repair the skin of patients with a fatal skin disease called recessive dystrophic epidermolysis bullosa, or RDEB. This is the first time researchers have shown that bone marrow stem cells can home to the skin and upper gastrointestinal tract and alter the natural course of the disease.

"Whether stem cells from marrow could repair tissues other than itself has been quite controversial," said Wagner, director of pediatric blood and marrow transplantation and clinical director of the Stem Cell Institute. "But in 2007 we found a rare subpopulation of marrow stem cells that could repair the skin in laboratory models. This astounding finding compelled us to test these stem cells in humans. This has never been done before."

"This discovery is more unique and more remarkable than it may first sound because until now, bone marrow has only been used to replace diseased or damaged marrow - which makes sense," said Tolar, associate professor of pediatric transplantation. "But what we have found is that stem cells contained in bone marrow can travel to sites of injured skin, leading to increased production of collagen which is deficient in patients

with RDEB."

Epidermolysis bullosa (EB), is a rare, genetic skin disease that causes skin to blister and scrape off with the slightest friction or trauma. It affects the skin and lining of the mouth and esophagus. Previously, there was no treatment and no chance for cure. In some countries, even euthanasia has been considered for newborns with the severest forms. If children with EB do not die of infection in their early life, many with the disease do not live beyond their 20s or 30s because they develop an aggressive form of skin cancer. While a few will live long term, the severest forms of EB are generally lethal.

"Bone marrow transplantation is one of the riskiest procedures in medicine, yet it is also one of the most successful," said Tolar. "Patients who otherwise would have died from their disease can often now be cured. It's a serious treatment for a serious disease."

Wagner and Tolar initiated the study in the fall of 2007. Since then, 10 children with the most aggressive forms of EB have been transplanted at the University of Minnesota Amplatz Children's Hospital. While all of the children have responded to the therapy, the magnitude of each response has varied.

"To understand this achievement, you have to understand how horrible this disease actually is," said Wagner. "From the moment of birth, these children develop blisters from the slightest trauma which eventually scar. They live lives of chronic pain, preventing any chance for a normal life. My hope is to do something that might change the natural history of this disease and enhance the quality of life of these kids."

Wagner and Tolar are measuring the progress each child makes after treatment in several ways. Clinically, the physicians monitor any improvements in health as well as in the strength of the recipient's skin

after transplant. They also use laboratory measures to determine how well the donor's cells are engrafting in - or becoming an integral part of - the skin, as well as measure the levels of collagen 7 - the protein missing in children with RDEB which is responsible for keeping layers of skin 'glued' to one another and to the body.

"What we now know is that after this treatment, healthy donor cells reside in the skin, collagen 7 consistently increases over time and the skin gradually becomes more resistant to blister formation." said Wagner. "This discovery expands the scope of marrow transplantation and serves as an example of the power of stem cells in the treatment of disease."

"While the treatment offers a chance for a better life, it comes with significant risk," said Tolar. "Two children have died from complications related to the treatment, so refinements are needed."

In fact, earlier this year Wagner and Tolar launched a new generation of the study by combining different stem cell populations. "We are fully conscious of what we have accomplished so far and the enormity of what else needs to be done," said Tolar. "But we have one goal—to take EB off the incurable list."

This breakthrough is another example of the University of Minnesota Medical School's continued excellence in regenerative medicine therapies. The world's first successful human bone marrow transplant was performed at the University in 1968, and since then, University of Minnesota Physicians have achieved many other world-firsts. Others include the first successful transplant to treat lymphoma (1975), the first use of umbilical cord blood to treat leukemia (1990), the first use of embryo selection to prevent a genetic disease and guarantee a human leukocyte antigen (HLA)-matched sibling ('savior sibling') and the first use of multi-unit umbilical cord blood transplantation to treat adults

(2000).

Provided by University of Minnesota

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