

## **Related Research: Medical Innovations at UofL and UK**

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Bob Evanosky said he and his wife, Sonya, were “handed three death sentences” in 2005, when all of their children were diagnosed with metachromatic leukodystrophy, a progressive and fatal genetic metabolic disorder that leads to muscle wasting, vision loss, paralysis and dementia.

But one of the Illinois couple’s sons – 8-year-old John has been offered hope through an experimental treatment pioneered by Dr. Suzanne Ildstad of the University of Louisville and Dr. Joanne Kurtzberg of Duke University. (His siblings including a twin brother, didn’t qualify for the clinical trail.)

Last year, John received a modified, safer version of a bone-marrow transplant similar to the one used to treat sickle cell disease. Bob Evanosky was the donor, and his marrow cells were sent to Louisville for processing before being given to John. Before the procedure, John was a quadriplegic on a ventilator who could not speak. The treatment is not expected to cure him, but his family and doctors hope his condition improves slightly and that he doesn’t reject the marrow.



Bob Evanosky said he is already noticing improvements and added that doctors plan to eventually wean John from anti-rejection drugs.

“We are starting to see subtle changes in John’s disposition,” said Evanosky, who lives outside Chicago. “There’s some improvement in his (breathing). There’s some movement in his body. He can move his arm and stretch. This probably is the most optimistic technology to come down the pike.”

### **Hand, Face and Similar Transplants:**

Today, a soldier needing a new hand to replace one lost in war would face a lifetime of anti-rejection medications. But research by Ildstad and her team could someday eliminate the need for such potentially dangerous drugs.

The research has been supported by the U.S. Department of Defense, which gave UofL’s Institute for Cellular Therapeutics a \$1.6 million grant last September.

Transplanting bone-marrow stem cells from the donor, along with a composite tissue transplant such as a hand transplant, is designed to create a “twin” immune system that helps the body recognize the transplanted tissue as part of itself. If it works, researchers said the potential for transplantation without anti-rejection drugs could revolutionize reconstructive surgery.

## **Multiple Sclerosis and Other Disorders**

UofL researchers believe safer bone-marrow transplants could also treat relapsing-remitting multiple sclerosis and a host of other autoimmune disorders.

In diseases such as MS, rheumatoid arthritis and lupus, the body's immune system mistakenly attacks its own organs, tissues and cells. Some diseases attack specific organs, while others attack several organs. Researchers believe bone-marrow transplants could halt the progression of such diseases.

Currently, however, bone-marrow transplants can be dangerous. A common and potentially fatal complication is graft-versus-host disease, in which cells in the donor marrow attack the recipient's body. Also, bone marrow or stem-cell transplants require donors and recipients to be a perfect genetic match.

But researchers are working on a less toxic "mini" bone-marrow transplant, which doesn't require a perfect match and may be performed on an outpatient basis. One clinical trial treats MS through a bone-marrow transplant with stem cells and "facilitating cells," which help the donor cells to "take."

## **Sickle Cell Research at UK**

Dr. Robert Means, an internal medicine professor at the University of Kentucky, also is studying sickle cell disease, focusing on a growth factor called P1GF, which regulates blood-vessel development. It is associated with inflammation, and is markedly increased in the bone marrow of sickle cell patients. Means, whose research so far is limited to laboratory cultures, said he wants to better understand the inflammation process and the role it may play in sickle cell disease.