

Medical Update Memo

February 9, 2009

Researchers Report Good Results from Small Study of Stem Cell Therapy to “Reboot” Immune System in MS

SUMMARY

Dr. Richard Burt and colleagues at Northwestern University report on the safety and benefits from an early phase study of experimental transplantation of individuals’ own blood stem cells to turn off the immune attacks in MS. Unlike some of the previous studies of this procedure, this one involved 21 people with relapsing-remitting disease, rather than people with later stage, progressive disease. (The study results were reported early online on January 30, 2009 in *Lancet Neurology*.)

Details

In this uncontrolled trial, they found that the procedure was relatively safe, and after an average of 37 months, none had progressed and a significant portion experienced a reversal of at least 1 point on their disability (EDSS) scores, and 76% remained free from relapses. As pointed out by the investigators, it will take larger-scale, controlled trials to determine whether this expensive, potentially risky procedure is superior to other approved treatment options. Controlled trials of this procedure are now recruiting participants.

Background : Autologous hematopoietic stem cell transplantation is similar to bone marrow transplantation to treat cancer, except that it uses the patients’ own blood or bone marrow stem cells. In treating MS, the procedure generally involves removing, from the blood stream, a person’s cells that are capable of regenerating into new immune cells. These “stem cells” are stored, and the rest of his or her immune cells are destroyed by various chemotherapy or other regimens. Then the stored stem cells are reintroduced by injection. Eventually they grow and repopulate the body with immune cells. The hope is that the new immune cells will no longer attack myelin or other brain tissue, so that the person has perhaps a completely new immune system.

This attempt to “reboot” the immune system has been studied in over 300 individuals with MS around the world with varying degrees of success. Earlier studies focused on people with secondary-progressive MS, and in these patients this procedure was not found to reverse disability. In addition, earlier attempts with this therapy had relatively high fatality rates because of the methods used to kill the immune cells (called “conditioning”) before

transplantation. Investigators have been experimenting with different conditioning regimens to make the procedure safer.

This Study: This study by Northwestern University researchers used a new, low intensity conditioning regimen and involved people with relapsing-remitting MS who had signs of very active immune attacks who had not been helped by standard therapies such as interferon beta. The average age of participants was 33 years and the median duration of their disease was five years, with moderate disability scores ranging from 2.0 to 5.5 points. After transplantation, many of the patients required blood or platelet transfusions due to low platelet counts. They were released from the hospital after an average of 11 days.

Results: Depending on the conditioning regimen used, the investigators found that the procedure was relatively safe. After an average of 37 months, none had progressed and a significant portion experienced a reversal of at least one point on their disability (EDSS) scores. Sixteen (76%) did not experience clinical relapses after transplantation, while five did, occurring six to 16 months after their transplantations. Those five were treated with immune-suppressing drugs and are considered by the investigators to be in remission.

Comments: Research on the safety and effectiveness of hemopoietic stem cell transplantation is ongoing several centers in the United States, Canada and abroad to determine whether it can halt progression or bring on remission of disease. As these experienced investigators point out, "Whether the data from this trial are superior to those achieved with continuation of standard therapies for relapsing-remitting MS can be assessed only in a randomized trial." It is also not yet clear how much potential benefit may have been due to the transplanted stem cells and how much may have been due to the medications used in the conditioning regimen, as those medications have themselves been preliminarily reported in other studies to be of therapeutic benefit in MS.

This procedure is strictly investigational, and it carries the risk of death because the body is nearly helpless against infection during the several weeks it takes for the immune system to be restored. There is no proof yet that it can cure a person with MS.

With information from the National MS Society (USA)

National Research and Programs

Offert en français.

Disclaimer

The Multiple Sclerosis Society of Canada is an independent, voluntary health agency and does not approve, endorse or recommend any specific product or therapy, but provides information to assist individuals in making their own decisions.