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By Coco Ballantyne on January 30, 2009

A new stem cell therapy improved the symptoms of early-stage multiple sclerosis (MS) in 80 percent of patients enrolled in a small clinical trial published today in *The Lancet Neurology*.

But lead author <u>Richard Burt</u>, chief of immunotherapy for autoimmune diseases at Northwestern University's Feinberg School of Medicine, cautions that the results have yet to be confirmed in large randomized clinical trials.

MS is a chronic debilitating neurological disorder that may cause symptoms including numbness of the arms and legs, and, in later stages of the disease paralysis and vision problems. Scientists believe that it's an autoimmune disorder in which the body's immune system – which ordinarily goes after invading germs—attacks healthy tissue. In the case of MS, the patient's immune system destroys protein sheaths that protect the nerve cells, or neurons, disrupting signals between the brain and the rest of the body. Using a method known as autologous non-myeloablative haemopoietic stem cell transplantation, Burt and his colleagues essentially eliminated misbehaving immune cells and replaced them with healthy ones (made from stem cells) in 21 patients (11 women and 10 men) with relapsingremitting MS, a common form of the disease in which symptoms come and go.

The way they did this: patients were given drugs that prompted their bone marrow to release immune stem cells (which have the ability to morph into any type of immune cell) into the blood; they then extracted the cells from the blood and gave patients drugs that wiped out their overactive immune systems. The researchers then injected the patients with their stem cells that had been removed earlier; the stem cells quickly divided, giving rise to a fresh batch of normal immune cells in the patients.

The idea behind the therapy, Burt says, is to "regenerate a new immune system" that recognizes healthy tissue and does not destroy the protein sheaths.

After an average follow up time 37 months, 17 of the patients (80 percent) scored better on a standard test used to gauge their vision, muscle strength, motor coordination, and other aspects of neurological function than they had before the trial. The other four patients did not improve, but they also didn't get any worse, Burt notes.

The next critical step is to figure out how the stem cell therapy stacks up against existing treatments for MS, such as tysabri and novantrone. These meds slow the disease by blocking or suppressing overactive immune systems, but they do not improve symptoms. Burt says he's currently conducting another clinical trial with 110 MS patients in which he is comparing the safety and effectiveness of the stem cell therapy and MS drugs.

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