Stem cell transplant reverses early-stage multiple sclerosis

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Researchers from Northwestern University's Feinberg School of Medicine appear to have reversed the neurological dysfunction of early-stage multiple sclerosis patients by transplanting their own immune stem cells into their bodies and thereby "resetting" their immune systems.

"This is the first time we have turned the tide on this disease," said principal investigator Richard Burt, M.D. chief of immunotherapy for autoimmune diseases at the Feinberg School. The clinical trial was performed at Northwestern Memorial Hospital where Burt holds the same title.

The patients in the small phase I/II trial continued to improve for up to 24 months after the transplantation procedure and then stabilized. They experienced improvements in areas in which they had been affected by multiple sclerosis including walking, ataxia, limb strength, vision and incontinence. The study will be published online January 30 and in the March issue of The *Lancet Neurology*.

Multiple sclerosis (MS) is an autoimmune disease in which the immune system attacks the central nervous system. In its early stages, the disease is characterized by intermittent neurological symptoms, called relapsing-remitting MS. During this time, the person will either fully or partially recover from the symptoms experienced during the attacks. Common symptoms are visual problems, fatigue, sensory changes, weakness or paralysis of limbs, tremors, lack of coordination, poor balance, bladder or bowel changes and psychological changes.

Within 10 to 15 years after onset of the disease, most patients with this relapsing-remitting MS progress to a later stage called secondary progressive multiple sclerosis. In this stage, they experience a steady worsening of irreversible neurological damage.

The 21 patients in the trial, ages 20 to 53, had relapsing-remitting multiple sclerosis that had not responded to at least six months of treatment with interferon beta. The patients had had MS for an average of five years. After an average follow-up of three years after transplantation, 17 patients (81 percent) improved by at least one point on a disability scale. The disease also stabilized in all patients.

In the procedure, Burt and colleagues treated patients with chemotherapy to destroy their immune system. They then injected the patients with their own immune stem cells, obtained from the patients' blood before the chemotherapy, to create a new immune system. The procedure is called autologous non-myeloablative haematopoietic stem-cell transplantion.

"We focus on destroying only the immune component of the bone marrow and then regenerate the immune component, which makes the procedure much safer and less toxic than traditional chemotherapy for cancer," Burt said. After the transplantation, the patient's new lymphocytes or immune cells are self-tolerant and do not attack the immune system.

"In MS the immune system is attacking your brain," Burt said. "After the procedure, it doesn't do that anymore."

In previous studies, Burt had transplanted immune stem cells into late-stage MS patients. "It didn't help in the late stages, but when we treat them in the early stage, they get better and continue to get better," he said.

Source: Northwestern University