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Stem Cell Transplants Offer First-Ever MS Treatment That Reverses Disability

Written by Jeri Burtchell on January 22, 2015

Only 2 years after getting a stem cell transplant, half of volunteers showed improvement in their disability scores — a first for any MS therapy.

Dr. Richard K. Burt performed the first hematopoietic stem cell transplant (HSCT) for a multiple sclerosis (MS) patient in the United States at Chicago's Northwestern Memorial Hospital. Now Burt, Chief of the Division of Medicine-Immunotherapy and Autoimmune Diseases at [Northwestern](#) University's Feinberg School of Medicine, is making headlines again.

Burt and his colleagues published the results of their newest HSCT study earlier this week in the [Journal of the American Medical Association](#). Their results show that HSCT could be the first MS therapy to reverse disability. Though the study group was small, the results have experts hopeful.

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For this trial, 151 patients underwent a stem cell transplant. First, their immune systems were tamped down using low-dose chemotherapy. Then, doctors used HSCT therapy, involving an infusion of the patients' own stem cells, previously harvested from their blood, to reboot their immune systems. After a short stay in the hospital, the volunteers went about their normal lives, needing no "maintenance" drugs.

Over the next several years, the volunteers were periodically given a series of tests to measure their disability. One test, known as the Expanded Disability Status Scale, or EDSS, measures cognition, coordination, and walking, among other things. Participants underwent MRI scans and completed questionnaires to measure their overall quality of life.

The researchers found that at two years post-transplant half of the patients showed a marked improvement in disability. Of the patients who were followed for four years, more than 80 percent remained relapse-free.

Since 1993, the FDA has approved [12 disease-modifying therapies \(DMTs\)](#) to treat relapsing-remitting MS (RRMS). All are designed to suppress the immune system to one degree or another. These drugs cost about \$5,000 per month and they must be taken indefinitely, since relapses will occur if the drugs are stopped. While patients now have many options to stave off disease progression, no DMT has been proven to reverse disability.

HSCT costs about \$125,000 per patient. "Although we haven't done a cost analysis, given how expensive Tysabri is, and Fingolimod, [since HSCT is a one-time treatment] it should start paying for itself around 18 months," Burt told Healthline.

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Who Should Have a Stem Cell Transplant?

“The caveat,” Burt conceded, “is this is not effective in progressive MS.” He pointed out the tendency among neurologists to try one DMT after another until the patient is out of options before offering HSCT. “But by then [the patient has] entered secondary progressive and most likely nothing will help.”

“If you’re doing well on first-line therapies, interferons or Copaxone, good, that’s where you should stay,” Burt added. “But if you’re having frequent relapses, two or more a year despite those therapies ... I think that’s the group that, rather than going to Tysabri or Fingolimod, should be given this therapy because it’s so much more beneficial. Plus, if you wait until you’ve had all those other [DMTs] then you increase the risk of this treatment.”

Even after stopping the drug, patients who have taken natalizumab (Tysabri) continue to have an increased risk of primary multifocal leukoencephalopathy (PML) for many months. If they were to undergo HSCT during that time, the risk for this rare but serious brain infection carries over and would make the procedure more dangerous.

In their study, Burt pointed out, “we had no opportunistic infections, no PML, nothing, but my worry is, if you’ve had many years of prior treatment with Tysabri, and you’re [positive for John Cunningham virus], then you could get PML and people think it’s our transplant but it’s really all that prior Tysabri.”

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Getting Her Life Back

One of Burt's trial patients, Roxane Beygi, spoke on a panel at the Vatican Adult Stem Cell Conference in 2013. In a [video](#) of the event, she describes her life before the study.

Despite being on a DMT prior to the study, Beygi was relapsing regularly and could barely walk. She had trouble writing, brushing her teeth, and even performing simple tasks like drinking from a glass.

“Since I had my transplant, my life changed completely,” said Beygi, speaking more than two years after treatment, “[Before the transplant] I had major fatigue where I couldn't even get out of bed. ... Now I get up at 6 ... and a lot of the time I'm studying and exercising until like 1 a.m.”

Beygi ended her presentation by thanking Dr. Burt for giving her life back. She called him her “hero.”

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Although HSCT is currently only available in clinical trials and for “compassionate use” in certain cases, Burt is hopeful that more studies will lead the FDA to approve stem cell transplantation for MS.

In fact, his team is currently conducting a larger study comparing HSCT to FDA-approved DMTs at three centers worldwide. The trial is currently enrolling, and interested patients can learn more at http://www.stemcell-immunotherapy.com/research_clinical.html.

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