Stem cell therapy for relapsing MS proves effective and safe, study finds

POSTED 7:01 AM, JANUARY 16, 2019, BY CNN WIRE

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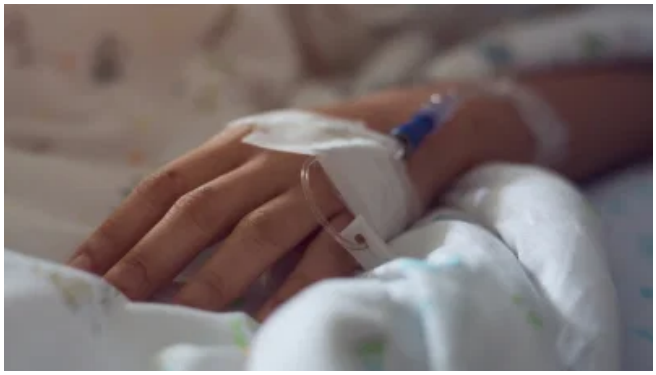

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A stem cell transplant using a lower-dose regimen of chemotherapy plus immune system suppressors is more effective at preventing disease progression compared to currently used disease-modifying therapies, according to the new study published Tuesday in the journal JAMA. (The stem cell treatment is known as "autologous hematopoietic stem cell transplantation" or HSCT.)

"I never use the word 'cure' — never," said Dr. Richard K. Burt, lead author of the study and chief of Immunotherapy and Autoimmune Diseases at Northwestern University Feinberg School of Medicine in Chicago. However, only a minority of patients receiving HSCT relapse by the five year mark, he said. "The vast majority don't."

How does this experimental stem cell therapy work?

HSCT essentially reboots the immune system.

“Out with the old, in with the new” is the goal, said Burt, adding that it is a “one-time treatment. You’re done, you’re off drugs.” First a patient’s blood stem cells are collected and then the patient is treated with chemotherapy drugs. Then, blood stem cells are returned to the patient to jump start the development of a new immune system.

In contrast, disease-modifying therapies work differently. These are a dozen or more drugs designed to be chronic, continuous treatment that targets and modulates the immune system.

The new comparison study took place at medical centers in the US, UK, Sweden and Brazil, where 110 patients with relapsing-remitting MS participated in the randomized clinical trial, a gold standard medical test. Patients received either the HSCT protocol or a different class of disease-modifying therapy than they’d previously used.

HSCT proved to be the more effective treatment: Of 55 patients receiving HSCT, only three patients showed disease progression at one year, the study showed. Yet, 34 of 55 patients in the disease-modifying therapy group showed disease progression at one year. Disease progression was measured using the Expanded Disability Status Scale, a method for monitoring changes in symptoms over time.

Among the HSCT group, the proportion of patients with disease progression was (roughly) 2% up to two years, 5% at three years, and 10% at 4 and 5 years. Meanwhile, the proportion of patients with no evidence of disease — defined as no progression, no relapses, and no new or enlarging lesions on MRI scans — was (nearly) 98% at one year, 93% at two years, 90% at three years, and 78% at four and five years.

By contrast, almost one quarter of the patients in the disease-modifying therapy group showed disease progression at one year, more than half at two years, and just under three-quarters at five years. And the proportion of patients with no evidence of disease was (about) 40% at six months, 21% at one year and 3% by years four and five.

What are the side effects of HSCT?

Side effects of HSCT can include infertility, said Burt, who noted that women can choose to preserve their eggs before treatment. And some patients developed autoimmune thyroid disease, a treatable condition. He noted that the disease-modifying treatments also have side effects.

Bruce Bebo, executive vice president of research at the National MS Society, said the study is “too small to really be definitive, but it does add to a growing amount of evidence that suggests this approach has benefits.”

The disease-modifying treatments tested in the study did not include two of the most recent and most effective drugs. “It is a gap in our knowledge,” said Bebo, who was not involved in the research. “We didn’t have [them] when the study started.”

In past studies of HSCT, patients with progressive MS did not respond to the therapy, noted Bebo. About 85% of patients are diagnosed with relapsing-remitting multiple sclerosis, where attacks of symptoms — such as dizziness, pain, and blurred vision — are followed by periods of remission. Yet, a "substantial portion" of relapsing MS patients evolve to develop progressive MS, where symptoms no longer wax and wane and instead worsen over time, explained Bebo.

"In the early days, HSCT was also riskier than it is now. Protocols have been fine-tuned since the early days," said Bebo, who said some past patients died. Today, the risk of mortality is "really, really low," said Bebo, who added that the chemotherapy drugs used by Burt "have been used to treat blood cancers for many many years."

Ultimately, HSCT could be a beneficial treatment for some patients with MS, said Bebo: "Probably people with an aggressive form of the disease who are not responding to other types of therapy."

Bebo advises patients to "be really cautious about a facility advertising stem cell treatment for MS." Other experimental stem cell approaches are "less mature" and less studied, he said, and they may endanger a patient's health.

Burt said, "This should be done in a major university medical center." Bebo added that "you have to make sure it's being done by a center with a great deal of experience. You really have to do your homework and find a place that has a published track record."

Going forward, Burt will fine tune HSCT to make it safer.

"The hope is to change the natural history of this disease," said Burt. "This data suggests we're doing it."

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