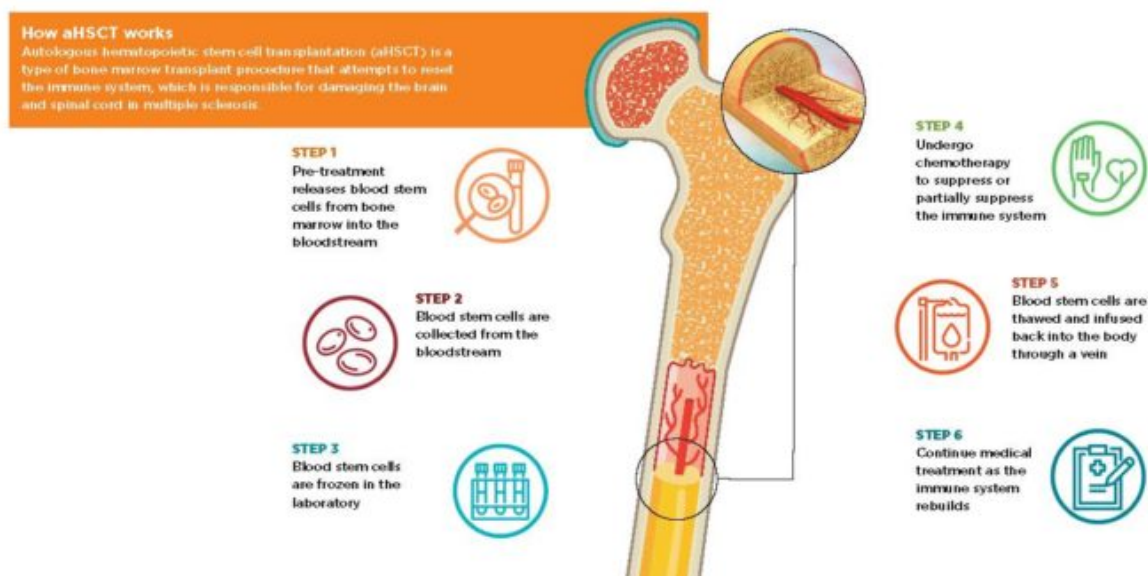


aHSCT: A promising treatment



Here's what you need to know about aHSCT and how to decide whether it's right for you.

by Vicky Uhland

For the last few years, discussions about stem cell replacement therapy have made their way into chat groups, social media postings and other conversations among people living with multiple sclerosis. When actress Selma Blair released a documentary in fall 2021 about her experiences with MS and her stem cell replacement procedure, those discussions entered the mainstream.

But despite this new scrutiny, questions still abound. For every report like Blair's showing how stem cell therapy stops disease progression, there are also reports of serious infections and other safety considerations.

To answer those questions, the National Multiple Sclerosis Society has made research into stem cell therapies a priority. Over the last 10 years, the Society has invested more than \$60 million into 68 stem cell studies.

These studies have produced a large enough body of evidence that the Society is now recommending a specific type of stem cell replacement therapy — autologous hematopoietic stem cell transplantation, or aHSCT — for certain people living with MS.

Here's what you need to know about aHSCT and whether it's right for you.

What is aHSCT?

aHSCT is a type of bone marrow transplant that's designed to reset the immune system and stop the inflammation that contributes to relapsing-remitting MS.

How does aHSCT work?

There's no universally agreed-upon aHSCT treatment protocol for MS. But in general, it's a one-time procedure that starts with treatment that stimulates the production of hematopoietic (blood-producing) stem cells in the bone marrow and promotes their release into the blood, plus chemotherapy that helps reduce the risk of relapses during this process. These stem cells are then harvested and frozen. This process can take five to 15 days.

After that, the patient is usually hospitalized and given another round of chemotherapy to kill their remaining immune cells. That's followed by the transplant of the previously harvested stem cells into the person's vein. Those cells migrate to the bone marrow, begin producing new white blood cells and, in essence, reboot the body's immune system.

"Instead of having an immune system that's programmed to attack the central nervous system, you've replaced it with naïve cells that aren't programmed to do that," says Aaron Miller, MD, medical director of the Corinne Goldsmith Dickinson Center for Multiple Sclerosis and a professor of neurology at the Icahn School of Medicine at Mount Sinai in New York.

The entire hospital stay can last about three weeks. The patient is usually given antibiotics during this time to combat infections.

Research shows the immune system gradually rebuilds itself within three to six months, but aHSCT follow-up appointments can last up to two years. These appointments include neurological and cognitive evaluations along with MRIs and blood tests to measure disease activity. MRIs are ongoing and don't stop after two years, however.

Who is a good candidate for aHSCT?

Studies show that aHSCT can be safe and effective in people who:

- **Have relapsing-remitting MS.** Research shows that aHSCT isn't as effective for progressive MS because it can't regrow damaged nerves or repair damaged myelin.
- **Are younger than age 50.** Miller says older people can have weaker immune systems and comorbidities (like heart disease or other age-related diseases) that may make them more susceptible to complications from aHSCT.
- **Were diagnosed with MS less than 10 years ago.** People who have had the disease longer may have more disability, which can make the aHSCT procedure more dangerous, Miller says.
- **Are unable to take a high-efficacy medication (such as Tysabri, Lemtrada, Ocrevus, Rituxan or Kesimpta),** or are still developing new lesions or having relapses despite taking these medications. Some MS specialists also consider Mavenclad to be part of this group.

Jeffrey Cohen, MD, professor at the Cleveland Clinic Lerner College of Medicine and director of experimental therapeutics at the Mellen Center for MS Treatment and Research, says the Cleveland Clinic gets a “tremendous number of calls” about aHSCT.

“But unfortunately, many of the people who approach us are not good candidates,” he says. “People are not so clear on who is likely to benefit, or they tend to underestimate how risky the procedure is.”

How effective is aHSCT?

Like most things regarding MS, there’s no definitive answer. Some people who have had aHSCT have gone into long-term remission, and some have even seen their symptoms reversed because the nervous system has been able to repair itself. Others have had MS lesions and relapses return after a period of time.

Over the last three years, the Cleveland Clinic has performed about eight aHSCT procedures, and Cohen says he’s “very impressed” with the results.

“It takes about three to four months to fully recover from the transplant, but even within that time frame, everyone we’ve treated started to feel better pretty quickly,” he says. “They’ve been able to walk better and have less fatigue and brain fog.”

What are the dangers of aHSCT?

The main risk comes from the chemotherapy regimen. Depleting the immune system makes people vulnerable to infections that can be life-threatening.

Cohen says there’s debate among practitioners and researchers about how intense aHSCT-related chemotherapy should be.

“More potent regimens have more health risks, but the benefit appears to be more durable,” he says. “Low-intensity chemotherapy is better tolerated, but there’s some concern that the benefits of the transplant wear off sooner in some people.”

Chemotherapy can also cause infertility in women, Miller says, so it’s a good idea to harvest your eggs if you want to become pregnant in the future.

How much does aHSCT cost, and does insurance cover it?

Prices vary based on the individual patient and their treatment plan, but in general, the total cost for aHSCT — including the care needed before and after the procedure — is around \$150,000, says Sean Grande, the National MS Society’s vice president of healthcare access. Grande adds that the \$150,000 is for people who are uninsured or whose insurance doesn’t cover the procedure, so it might not indicate what a person may actually pay.

Some, but not all, private health insurance plans cover aHSCT for people with MS — but only if specific criteria are met, Grande says. And that’s where it gets dicey. Insurance providers set their own coverage criteria and approval processes based on clinical guidelines

determined from an evidence perspective. They'll also consider the patient's medical history and the exact nature of the stem cell procedure.

Medicare doesn't currently cover aHSCT for people with MS, Grande says. On the other hand, under the Medicaid program, individual states have broad discretion in determining whether they cover aHSCT for people with MS. Your state's Medicaid agency can answer questions about benefits and coverage.

No matter your source of insurance coverage, getting approved for aHSCT is complicated. It can help to work with an MS specialist and an accredited aHSCT treatment center to navigate the process.

If your insurer denies coverage, don't despair. Public and private insurers have been reviewing their coverage guidelines and eligibility criteria for aHSCT as more scientific evidence emerges, says Hope Nearhood, the National MS Society's director of MS information and resources. Appealing their decision could still be successful — especially if you work with an MS specialist or accredited treatment center to demonstrate that aHSCT is clinically beneficial in people with MS.

Where can I get aHSCT?

The Society recommends that people only undergo aHSCT at centers accredited by the Foundation for the Accreditation of Cellular Therapy (FACT), which has a stringent set of quality and safety standards.

The Society also recommends that aHSCT treatment center transplant teams include neurologists who are MS experts, along with hematologist-oncologists who are experienced in performing aHSCT on people with MS.

Because of treatment costs and insurance woes, some people may be tempted to go outside the U.S. for an aHSCT procedure. But many international stem cell transplant clinics don't have the same sanitation and clinical quality standards as FACT-accredited centers.

"Some clinics abroad or even in the U.S. have websites that look like advertisements for stem-cell transplants — including cosmetic procedures — and can't support their claims with peer-reviewed research," Nearhood says. "That's a red flag that they can have a lack of regulatory oversight."

Vicky Uhland is a writer and editor in Lafayette, Colorado.

Learn more about [aHSCT and find a treatment center](#).

Read about one woman's experience with aHSCT.