

What to know about autologous hematopoietic stem cell transplantation



by Vicky Uhland

For the last few years, stem cell replacement therapy has received a lot of buzz in the multiple sclerosis community. But for every report of how the procedure has stopped someone's disease progression, there have been concerns and questions about serious infections and other safety considerations.

To answer these questions, the National Multiple Sclerosis Society has made research into stem cell therapies a priority. Over the last 10 years, the Society has supported 68 stem cell studies, and its current multi-year investment into stem cell research totals more than \$60 million.

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.

"So 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 for 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 (Tysabari, Lemtrada, Ocrevus, Rituxan, or Kesimpta) or are still developing new lesions or having relapses despite taking these medications. Some MS specialists consider Mavenclad in this group.

How effective is aHSCT?

Like most things regarding MS, there's no definitive answer. Effectiveness varies from person to person. 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.

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. 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 Society's vice president of healthcare access. Grande adds that the \$150,000 is for those who are uninsured or when insurance does not cover the procedure and does not indicate what a person might 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 take into account the patient's medical history and the exact nature of the stem cell procedure.

Medicare does not currently cover aHSCT for people with MS, Grande says. But because of the structure of the Medicaid program, individual states have broad discretion in determining whether it covers 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 going to take a lot of paperwork. Grande recommends working with your MS specialist and an accredited aHSCT treatment center to navigate the process.

If your plan 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 Society's director of MS information and resources and advocacy and healthcare access. Appealing their decision could still be successful — especially if you work with your MS specialist or accredited treatment center to demonstrate that aHSCT is clinically beneficial in MS.

Where can I get aHSCT?

The National MS Society recommends that people only undergo aHSCT at centers accredited by the [Foundation for Accreditation of Cellular Therapy](#), FACT center, 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." It is always important to talk to your MS specialist for help with choosing a reputable clinic.

How can I find out more about aHSCT?

You can contact an MS Navigator at 800-344-4867 or ContactUsNMSS@nmss.org for general information about aHSCT, help locating a treatment center and questions about health insurance coverage.

Participate in an aHSCT clinical trial

There is currently one clinical trial on aHSCT and MS in the United States, called BEAT-MS. Researchers are recruiting 156 people ages 18 to 55 who have highly active, treatment-resistant, relapsing MS.

Trial participants will be randomly assigned to either undergo aHSCT or receive certain disease-modifying therapies. The objective is to compare the costs, safety and effectiveness of the two treatments. For details and locations of the participating treatment centers, visit [the study website](#).

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