aHSCT: Who Might Benefit?



Autologous hematopoietic stem cell transplantation (aHSCT), an emerging treatment for some forms of MS, is getting a lot of attention lately. Based on a thorough review of research evidence and clinical experience, the National MS
Society has endorsed aHSCT as an additional treatment approach for people with relapsing-remitting MS. This means, in our view, it should no longer be considered experimental for some people, and we want health care providers, health insurers, and people living with MS and their families to know about it.

I don't recall another time when the Society has endorsed any particular MS therapy – but this is important because unlike medications, this actual procedure in MS does not really fall under the jurisdiction of the FDA. The progress we've made in refining treatment protocols and results from clinical studies have revealed more about how this treatment works and who is most likely to benefit. It is my pleasure to provide a brief update on this promising treatment for MS.

Who Can Benefit from aHSCT?

Finding ways to stop MS means finding the right treatment approach for each person as quickly as possible. Despite having many treatment options for people living with **relapsing-remitting MS**, finding the right one for any one person sometimes involves time-consuming trial and error. For some with very active relapsing-remitting MS, none of the disease-modifying therapies work very well.

According to research, aHSCT is a potential treatment option for people who:

- have relapsing-remitting MS
- are less than 50 years of age
- have had MS for less than 10 years
- have "breakthrough" disease activity (new active MRI brain lesions and/or relapses) despite treatment with one of the highly effective, second-generation therapies, **or** those who are unable to take one.

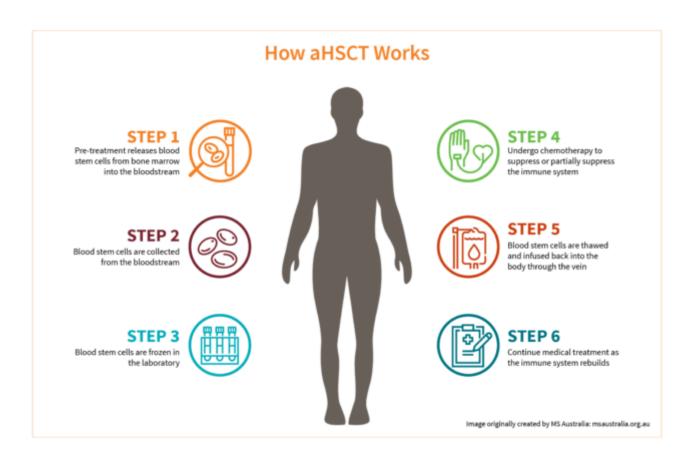
Even for people who fit this profile, aHSCT may not be the right treatment. For example, being older, having greater disability, and having certain health conditions (like heart or lung disease) can increase the risk of life-threatening side effects following aHSCT. And unfortunately, for those living with progressive forms of MS, aHSCT has generally not been found to be beneficial.

How aHSCT Works

aHSCT is not a single treatment (like a pill or shot), but is a series of medications and procedures that take place as an in-patient at a hospital. The goal of the treatment is to reset, or "reboot," the immune system so that it will stop attacking the nervous system.

- 1. This rebooting occurs after a person's immune system cells are nearly erased with strong drugs.
- 2. Then their own (autologous) bone marrow stem cells are reinfused into the vein.
- 3. These stem cells gradually create a new immune system that for reasons we still don't fully understand-often reduce or sometimes even eliminate the immune cells responsible for disease attacks

During the weeks and months that it takes for the immune system to repopulate, the individual is vulnerable to all kinds of infections. But in the right medical hands and in people with the specific profile, this treatment can be a lifesaver. Another exciting thing about aHSCT is that it is a one-time procedure.



Finding an aHSCT Clinic

A word of caution – there are <u>clinics</u> in the U.S. and abroad that may not have experience in aHSCT and MS. Some overstate their experience online with testimonials to attract new patients. It's crucial that aHSCT is performed at a treatment location that is accredited by the <u>Foundation for Accreditation of Cellular Therapy (FACT)</u>. An MS specialist can assist with selecting a treatment center for care during and after aHSCT.

Since MS is different for each person, individuals should discuss treatment options with their MS specialist or physician to determine what is best for their MS.

Evolving Research

There are still some questions about who would benefit most from aHSCT and how this procedure stacks up against more powerful disease-modifying therapies that are now available. To answer these questions, the **BEAT-MS clinical trial** is recruiting eligible participants who are randomly assigned to undergo aHSCT or receive certain disease modifying treatments to compare the costs, safety and effectiveness of the two treatments. For details and to locate the site nearest you, visit the **study website**.

Editor's Note: **Read more** about aHSCT as a treatment for certain people with relapsing-remitting MS, or **watch our Ask an MS Expert** episode on aHSCT: