# Hematopoietic Stem Cell Transplantation: An Interview with Dr. Richard Burt

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**Could you please explain the stem-cell procedure that you're testing?**

We are testing a procedure called hematopoietic stem-cell transplantation (HSCT), but there are a lot of misnomers that can cause confusion for people who are not familiar with HSCT. First, hematopoietic stem cells are immune stem cells. So, perhaps a better term would be immune stem cell transplantation. Second, the word "transplant" can cause confusion. When people hear it they often think of a transplant for cancer and we do not use these cancer drugs; we don't use radiation or any cancer treatments. Nor does HSCT include transplantation of someone else's stem cells – we give the patient their own stem cells during the procedure.

We use standard immune suppressant drugs, commonly used to treat immune or autoimmune diseases, in a very short exposure and high dose to knock down the immune system, and then we give you back your own immune stem cells that we have collected before the procedure. Giving back your immune stem cells as a supportive blood product hastens recovery, but they're not necessary – you'd recover fine without them.

In MS, the immune inflammation that destroys myelin requires two signals to get started, one is exposure to myelin or something that looks like myelin and the second is a danger signal-something that tells the body there is a need for an immune attack. If either of these two signals are missing, you will not get the inflammation that causes MS. In HSCT, we are starting over-resetting the immune system. Think of it like a re-boot of your computer, we are wiping the hard

drive clean and starting over. The immune system has to learn all over again which things to fight (pathogens like bacteria and viruses) and which things to leave alone (myelin). We think what happens in HSCT is that the immune response is exposed to myelin in the absence of the danger signal and as a consequence is rendered harmless (tolerant), unable to do any more damage.

**Is there a type of MS that is most likely to benefit from HSCT?**

Yes, because what we're doing is stopping inflammation you need to treat when the disease in its inflammatory stage, which occurs in relapsing remitting MS. We don't think this treatment will be very helpful for people with progressive forms (primary or secondary) of MS. However, there are other forms of stem cell therapy, therapies designed to promote nervous system repair that hold great promise for treatment of all forms of disease, including progressive MS.

**Are you still recruiting people for your trial?**

Yes we are. The trial is still open and ongoing. You can learn more here.

**You mentioned you won't treat people who are in late secondary-progressive, are there certain types of people that are particularly appropriate or should consider the trial?**

The best way to answer that question is where does this approach lie in the treatment of MS? There are first-line therapies such as Avonex and Copaxone. If someone's MS is controlled well with first-line therapy, they should stick with their current therapy. But if they're not, and they're having frequent relapses, they'll go to second line therapy.

Our randomized trial is looking at people who have failed a first-line therapy and have opted for stem cell transplantation over a second-line treatment. Our goal of the trial is to establish in a randomized trial that this is the proper role for HSCT. Our early data suggests that HSCT does something that second-line therapy has never done: it reverses disability and significantly improves quality of life.

**Can you explain the terms myeloabalative and non-myeloablative?**

Our procedure is non-myeloablative. Non-myeloablative means that while we are removing much of the immune system with our drug regime, if we did not replace these cells with stem cells, the patient would be able to recover. Myeloablative is a procedure usually reserved for cancer therapy that completely eliminates the stem cell compartment. In this case, the patient would not be able to survive without a stem cell transplant. We feel that the myeloablative approach is too toxic for patients with MS and shouldn't be used in people with MS.

**There are different centers performing HSCT around the world, is the treatment standardized in any way?**

I hope that our trial will help standardize this approach when our clinical trial data is published,. We coordinate our trial at a few other centers around the world. The trial is also being run in

University of Sao Paulo, Brazil the University of Uppsala in Stockholm, Sweden. It's just been approved at the University of Sheffield in the UK, and I'm speaking in Sydney, Australia this month, because they want to open a center there as well. It is the same trial run in all locations.

**There are people who may not qualify for your trial or who are looking for other options. Is there any advice or caution you'd offer to people as they explore other options?**

We have developed certain criteria for accepting patients in our trial based on our experience and understanding how this therapy works. People do sometimes get upset when they don't qualify and sometimes they seek out other treatment centers.

Of course you can always find someone who doesn't have our level of experience understanding the limitations of the treatment and who may treat them, and of course that is your right to make your own treatment decisions, but you have to be cautious if somebody doesn't have limits or say 'this approach can't help this subset.' This is a very powerful technique and just like anything powerful it can be abused. If used the wrong way it can have adverse effects.

In the early development of something like this, experience of the care team is very important. You want to be sure to work with an experienced center that is dedicated to this form of transplantation. This is so promising and so exciting, but it is not without risks.

**Could you speak about the side effects and risks that people need to know about?**

Someone could die from our therapy, this is most likely to be the result of a runaway infection. We are very good at preventing that, we've had no deaths in trial participants with MS. In fact, of the 150 participants I will be reporting soon, we've only had one infection. Our procedure is not without risk, but you can minimize it if you follow proper procedures. That highest risk period lasts about eight days while they are recovering in the clinic.

Another risk to be aware of is the potential for infertility. It's age-dependent and many of our patients have gone on and gotten pregnant even in their 30's. We encourage people who are concerned about infertility to consider options such as sperm or egg storage prior to the procedure.

Most people get blood transfusions during this process. There is also a small risk of becoming infected with HIV or hepatitis. Again, the risk is very small. In my lifetime, I've probably transplanted about 1,500 patients and I've never had anyone get HIV or hepatitis from a blood transfusion, but you can never say for sure that there is no risk. Finally, late complications of this procedure unique to MS that may arise in a small subset are hypo or hyperthyroidism, or more rarely a drop in platelets that may require transient medical treatment to reverse.

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