



Canadian Bone Marrow Transplantation Trial: Quick Facts

Updated: March 2017

What was the study?

- In 2000, the Multiple Sclerosis Society of Canada and Multiple Sclerosis Scientific Research Foundation (MSSRF) funded a multi-centre clinical trial, entitled 'Targeting Multiple Sclerosis as an Autoimmune Disease with Intensive Immunoablative Therapy and Immunological Reconstitution'. The trial received a total of \$6,475,344 in funding.
- The aim of the trial was to determine whether immunoablation and autologous hematopoietic stem cell transplantation (IAHSCT) in people with MS can stop the disease.
- The study, now referred to as the **Canadian Bone Marrow Transplantation (BMT) trial**, was led by Drs. Mark Freedman and Harry Atkins from the Ottawa Hospital Research Institute.
- Recruitment for the trial began in 2000, and the first transplant was completed in 2001. A total of 24 participants underwent IAHSCT, which involved receiving a potent chemotherapy regimen to remove the immune system, followed by transplantation with stem cells. The purpose of the procedure was to remove the abnormal immune system and replace it with a healthy new one.
- Due to the serious side effects associated with chemotherapy, only those individuals who had aggressive, highly inflammatory relapsing-remitting MS (RRMS) were considered and accepted to the trial.
- The stem cells used for the treatment were autologous, meaning they were collected from the bone marrow of the participants themselves. This eliminated the risk of rejection as the cells are from the individual and not from a donor.
- Participants were monitored over a period of up to 13 years post-treatment. Researchers assessed levels of disability, number and size of inflammatory brain lesions, changes in brain volume over time, and measures of social wellbeing.
- [Additional experiments](#) were conducted to further understand the participants' responses to the treatment. These included comprehensive clinical, MRI and immunological studies.
- Results of the Canadian BMT Trial were published on June 9, 2016 in [The Lancet](#).

What did they find?

- Published results from the BMT trial demonstrate that IAHSCT is highly effective in stopping new inflammatory disease. Specifically, no new relapses or brain lesions were observed in 23 participants during the entire follow-up period.
- Researchers also found that 70% of participants showed no evidence of disability progression over the long-term, 40% experienced unexpected recovery in various functions like strength and improved movement coordination, and some participants experienced marked improvements in several measures of social wellbeing.
- One participant died of complications from liver failure approximately 2 months after undergoing the IAHSCT procedure.

What does it mean?

- Publication of the clinical trial results in a peer-reviewed scientific journal indicates that the data has been reviewed thoroughly by experts, and allows medical professionals and key decision makers to evaluate the treatment's efficacy, safety, and eligibility for Canadians living with MS.
- While the results are highly encouraging, IAHSCT remains to be a complex procedure that involves intense, potentially toxic chemotherapy. Thus the treatment is warranted for those who have aggressive, highly inflammatory relapsing-remitting MS (RRMS) who have not responded to disease-modifying therapies.
- It is unclear whether IAHSCT can benefit those who have experienced a high level of MS-related disability over a prolonged period of time and/or exhibit low levels of inflammation in the brain and spinal cord.
- [Other IAHSCT trials](#) have been completed or are currently taking place around the world, which include different methods, eligibility criteria, and chemotherapy cocktails. Together this body of research, in addition to the Canadian BMT trial, greatly enhances the MS community's understanding of the full risks and benefits associated with IAHSCT as a treatment for MS, and paves the way for ongoing research on treatments involving stem cells for all forms of MS. The BMT Trial, in particular, offers a unique perspective on the sustained effect of the treatment over the long term.
- Currently, The Ottawa Hospital is accepting referrals for eligible patients for IAHSCT. Speak to your neurologist if you are exploring this treatment option. Questions related to the procedure, payment/coverage details, eligibility, monitoring, etc. are to be directed to The Ottawa Hospital. More information can be found on their [website](#).
- With funding from the MSSRF, Dr. Mark Freedman recently initiated a [clinical trial involving mesenchymal stem cells](#) with co-lead investigator Dr. James Marriott from the University of Manitoba.

Supporting information

- [MS Update: Landmark MS Scientific Research Foundation funded study demonstrates that “rebooting” the immune system stops disease activity and leads to long-lasting recovery in people with aggressive multiple sclerosis](#)
- [MS Update: Multiple Sclerosis Scientific Research Foundation funded study advances understanding of risky but effective stem cell treatment](#)
- [Press release](#)