



PRESS RELEASE

Blood Stem Cell Transplantation is more effective than existing drugs in suppressing severe Multiple Sclerosis

Barcelona, February 25, 2015 – High dose chemotherapy and transplantation of patients’ own blood stem cells may be more effective than standard treatment with the approved drug Mitoxantrone in severe affected patients with Multiple Sclerosis (MS), according to a new study published in the February 11, 2015, online issue of Neurology®, the medical journal of the American Academy of Neurology.

The study was supported by the European Society for Blood and Marrow Transplantation (EBMT) and all the members of the Autoimmune Diseases Working Party (ADWP) as well as the EBMT Clinical Trials Officers. The ASTIMS study involved 21 MS patients treated in 7 European Centres; 12 were randomized to receive Mitoxantrone and 9 to transplantation. The average age of patients was 36 years, and all had advanced disability due to MS. The study primarily focused on MS activity monitored by Magnetic Resonance Imaging (MRI) of the brain and spinal cord. Patients were followed for 4 years after the treatment.

Stem Cells were collected from the patients’ blood and frozen in liquid nitrogen. High dose chemotherapy was then administered with the aim of destroying the abnormal immune system. The patients’ blood stem cells were then thawed and infused intravenously, like an ordinary blood transfusion, leading to the generation of a newly ‘re-set’ immune system. This procedure, usually referred to as autologous transplantation, is commonly used for the treatment of many haematological diseases, such as lymphomas and leukaemias. In the last 15 years, however, it has also been increasingly employed in the treatment of severe autoimmune diseases, such as MS. This is the first randomized trial to be completed in MS.

Prof. Mancardi from the University of Genova (Italy) coordinated the neurological part of the study: “This process appears to reset the immune system,” said Dr. Giovanni Mancardi. “With these results, we can speculate that stem cell treatment may profoundly affect the course of the disease.” In the 4 years of follow-up, transplantation reduced of 79% the number of new brain lesions detected by MRI as compared to patients treated with Mitoxantrone; also inflammatory or ‘enhancing’ lesions were totally absent in transplanted patients while 56 percent of patients treated with Mitoxantrone showed such lesions.

“Most of the side effects reported in transplanted patients were expected and resolved with time” said Dr. Riccardo Saccardi, from the Careggi University Hospital in Florence, Italy, who coordinated the haematological aspects. “We used a transplant technology at intermediate intensity, which is the most used in Europe for these patients. Appropriate selection of patients with autoimmune diseases who are candidate for stem cell transplantation - is crucial. MS patients in the early phase of the disease, showing clinical and MRI signs of flare despite the administration of a standard treatment show the best outcome”. Recently the EBMT Clinical Practices Guidelines for Stem Cell Transplantation on Autoimmune Diseases have been updated by all the ADWP members under the coordination of Prof Dominique.Farge Bancel from Saint Louis Hospital in Paris and EBMT ADWP Chair and Pr John Snowden from Sheffield University and EBMT ADWP Secretary. These guidelines are available on the EBMT website www.ebmt.org.

Over a thousand MS patients have now undergone autologous transplantation worldwide, most of them reported to the EBMT Registry. Published data of other preliminary studies and retrospective analyses have also been promising, but this is the first study in which transplant is directly compared with an approved treatment in severe, treatment resistant MS.

“More research is needed with larger numbers of patients who are randomized to receive either the stem cell transplant or an approved therapy, but it’s very exciting to see that this treatment may be so superior to a current treatment for people with severe MS that is not responding well to standard treatments,” Pr Mancardi said.



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About the European society for Blood and Marrow Transplantation (EBMT) and the Autoimmune Diseases Working Party (ADWP)

The EBMT is a non-profit organisation established in 1974 to allow scientists and physicians to promote transplantation of haematopoietic stem cells and cell therapy including basic and clinical research, education, standardisation, quality control, and accreditation. The EBMT develops cooperative studies to save the lives of patients with blood cancers and other life-threatening diseases by advancing the fields of blood and marrow transplantation and cell therapy worldwide through science, education and advocacy.

Since 1997, the EBMT Autoimmune Disease Working Party (ADWP) is dedicated to foster awareness and clinical collaboration on Stem Cell Transplantation for Autoimmune Diseases. The EBMT ADWP database is the largest collaborative platform of the field and it has actively contributed to the completion of clinical and research studies for more comprehensive understanding of best available current therapeutic strategies in Autoimmune Diseases in rapidly progressive or severe Systemic Sclerosis, Multiple Sclerosis, Lupus, Rheumatoid Arthritis and Juvenile Arthritis, Immune Cytopenia, Inflammatory Bowel diseases and other ADs, and more recently in Early Acute Insulin Dependent Diabetes.

For further information about the EBMT, please visit the website: www.ebmt.org and follow us on Twitter: [@TheEBMT](https://twitter.com/TheEBMT)

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