

NEW RESULTS SHOW THAT AUTOLOGOUS HAEMATOPOIETIC STEM CELL TRANSPLANTATION INCREASES SURVIVAL RATE IN PATIENTS WITH SIFFUSE CUTANEOUS SYSTEMIC SCLEROSIS

Embargo date: Tuesday, June 24th 2014

Barcelona, 24th of June 2014. Final results from the Autologous Stem Cell Transplantation International Scleroderma (ASTIS) trial have been published in the Journal of the American Medical Association (JAMA). This data carries more comprehensive evidence that autologous haematopoietic stem cell transplantation (HSCT) confers better long term survival than intravenous pulse cyclophosphamide treatment for diffuse cutaneous sclerosis.

From March 2001 to October 2009, 156 patients from 10 countries with early diffuse cutaneous systemic sclerosis were recruited and followed up until the end of October 2013. After registration, patients were randomly assigned to receive HSCT or 12 monthly intravenous pulses of cyclophosphamide with the aim to compare survival rates of both treatments. The primary end point of ASTIS was event-free survival, defined as time from randomization until the occurrence of death or persistent major organ failure.

ASTIS trial is an international, multi-centre, investigator-based, open label, phase III trial that involved 29 centers. It is a unique groundbreaking collaborative project coordinated by two leading entities in the fields of the study, the European Society for Blood and Marrow Transplantation (EBMT) and the European League Against Rheumatism (EULAR).

Even though HSCT therapy shows more treatment-related mortality in early stages of the follow up, long term event-free and overall survival rates are higher in the HSCT treatment group than in the control group. Furthermore, HSCT treatment is proven to be far more effective for non-smoking patients rather than for smoking patients. "These results are very encouraging and shed light on new research on the type of patient that will benefit from immunosuppressive therapy" said Professor Jaap van Laar (Newcastle University), Professor Dominique Farge Bancel (APHP) and Alan Tyndall (Basel University).

Systemic sclerosis is a rare heterogeneous autoimmune connective tissue disease characterized by vasculopathy, autoantibody formation, low-grade inflammation, and fibrosis in skin and internal organs. It affects to 2-22 people per one million every year and the diffuse cutaneous form of the condition – which is a more severe form of the disease - make up to 30% of the total cases. This severe autoimmune disease can lead to premature death by lung fibrosis and pulmonary hypertension with no curative treatment yet discovered.

About the EBMT:

The EBMT is a non-profit organisation established in 1974 to allow scientists and physicians involved in clinical bone marrow transplantation to share their experience and develop cooperative studies. The EBMT aims 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.

For further information please visit www.ebmt.org

About EULAR:

The EULAR is the organisation which represents the patient, health professional and scientific societies of rheumatology of all the European nations. EULAR aims to promote, stimulate and support the research, prevention treatment and rehabilitation of rheumatic diseases.

To find more information go to www.eular.org