Autoimmune Diseases Working Party

Chair: John Snowden
Secretary: Tobias Alexander
Study coordinator: Manuela Badoglio

Number of HSCT for autoimmune diseases: 2268
EBMT Registry - February 2017

Transplant procedures 2208
Patients 2217
Male/Female % 36/64
Pediatric/adult % 11/89
Centres/Countries 354/40
Overall follow-up (months, t_HSCT = 2010) 2y (±1-20)

Autografts n=1223 Allografts n=142

- First 2104 112
- Second 20 24
- Third 1 6

Median age at 1st transplant: 37y (3-76), 12y (1-62)

The NISc study: ongoing
- Open, multi-centers, prospective non-interventional study
- Aiming to assess the effectiveness of Autologous HSCT for early severe or rapidly progressive Systemic Sclerosis (SSc)
- Recruitment is completed with 82 patients from 12 centres / 7 countries

Education
1. Updated recommendations for cardiopulmonary evaluation, screening, patient selection for stem cell therapy in poor prognosis systemic sclerosis.
2. ADWP/SAAWP joint Educational meeting, Paris, November 2016

New prospective non-interventional studies
1. NISMO: Autologous Hematopoietic Stem Cell Transplantation for Neuromyelitis Optica (NMO) and NMO spectrum disorders (R Grecco, Milan, Italy)
2. QMST: Multiple sclerosis patients treated with auto-HSCT (R Saccardi, Florence, Italy)
3. NISc II: Post transplant management of SSc patients treated with auto-HSCT (D Farge, Paris, France)

Key scientific advances
- Over 2000 HSCT in the largest data base worldwide
- In 2015, proof of efficacy for HSCT in multiple sclerosis and Crohn’s disease with the publication of two major ADWP randomised trials (i.e. ASTIMS and ASTIC) in collaboration with other European Societies (i.e. ECTRIMS and ECGO), thus completing the previous achievement of the ASTIS trial in 2014 for systemic sclerosis. The efficacy of autologous HSCT is now established for AD.
- The ADWP has continued to expand its activities using other types of immune-modulating cells (e.g. MSCs) from various sources of blood products (i.e. bone marrow, peripheral blood or cord blood, and placenta) either in the autologous or allogeneic settings. Refined analysis of immune reconstitution processes has confirmed the reset of autoimmunity after HSCT in AD.
- Sustained positive clinical results and enhanced ADWP activity in otherwise refractory AD patients has attracted an increased interest from patients, clinicians, and healthcare providers in the field.

ADWP - Recent publications
9. One year outcome of autologous stem cell transplantation in treatment refractory Crohn’s disease: identifying factors that predict benefit and harm. Lindsay JO et al. accepted Lancet Gastroenterology & Hepatology 2017

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