



European Society for Blood and Marrow Transplantation

Autoimmune Diseases Working Party

Chair: Dominique Farge

Secretary: John Snowden

Study coordinator: Manuela Badoglio

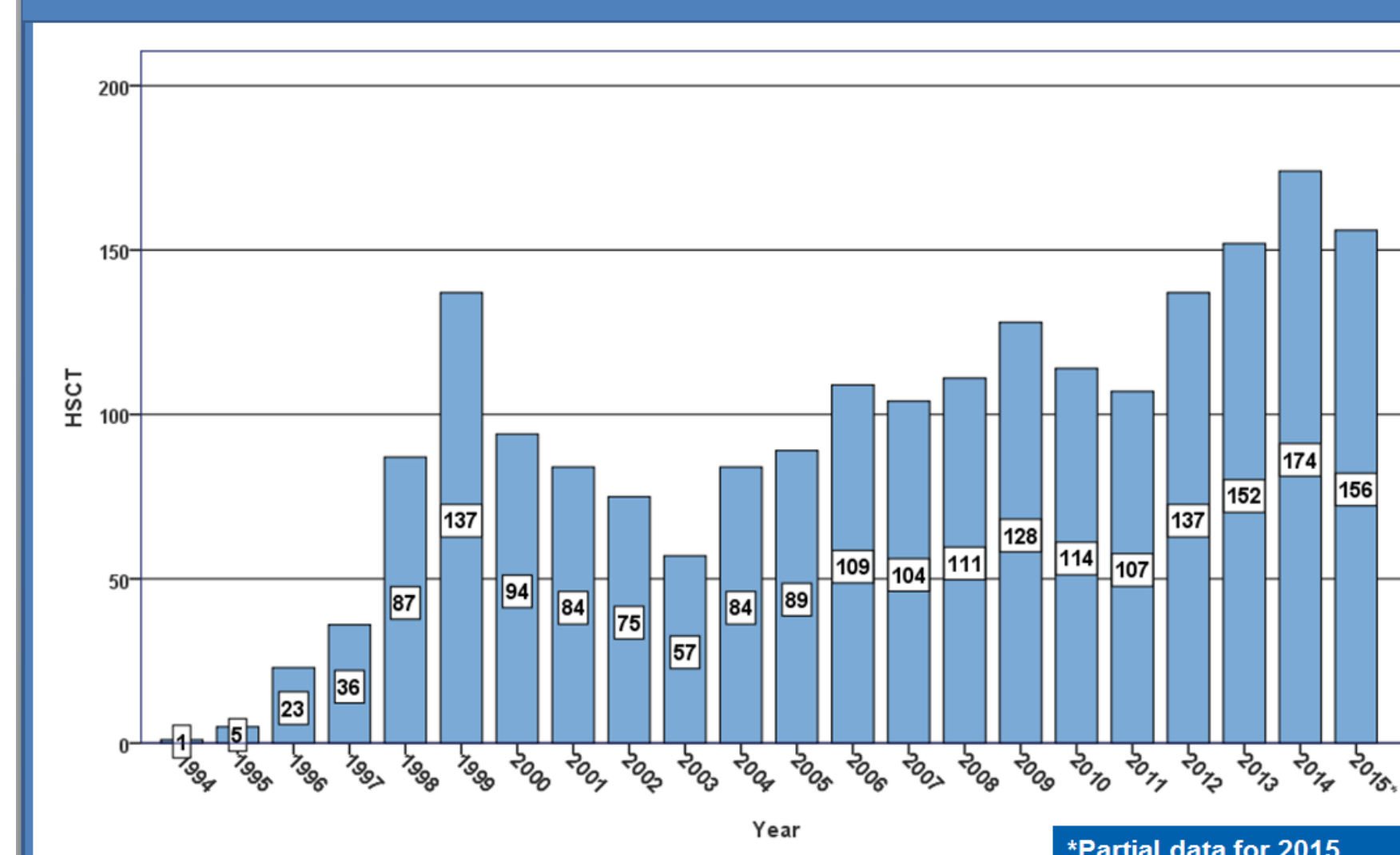
Number of HSCT for autoimmune diseases : 2069 EBMT Registry - February 2016

Transplant procedures	2069
Patients	2016
Male/Female %	39/61
Paediatric/ adult / %	11/89
Centres /Countries	249 / 40
Overall follow up (median)	2.7y (<1-19)

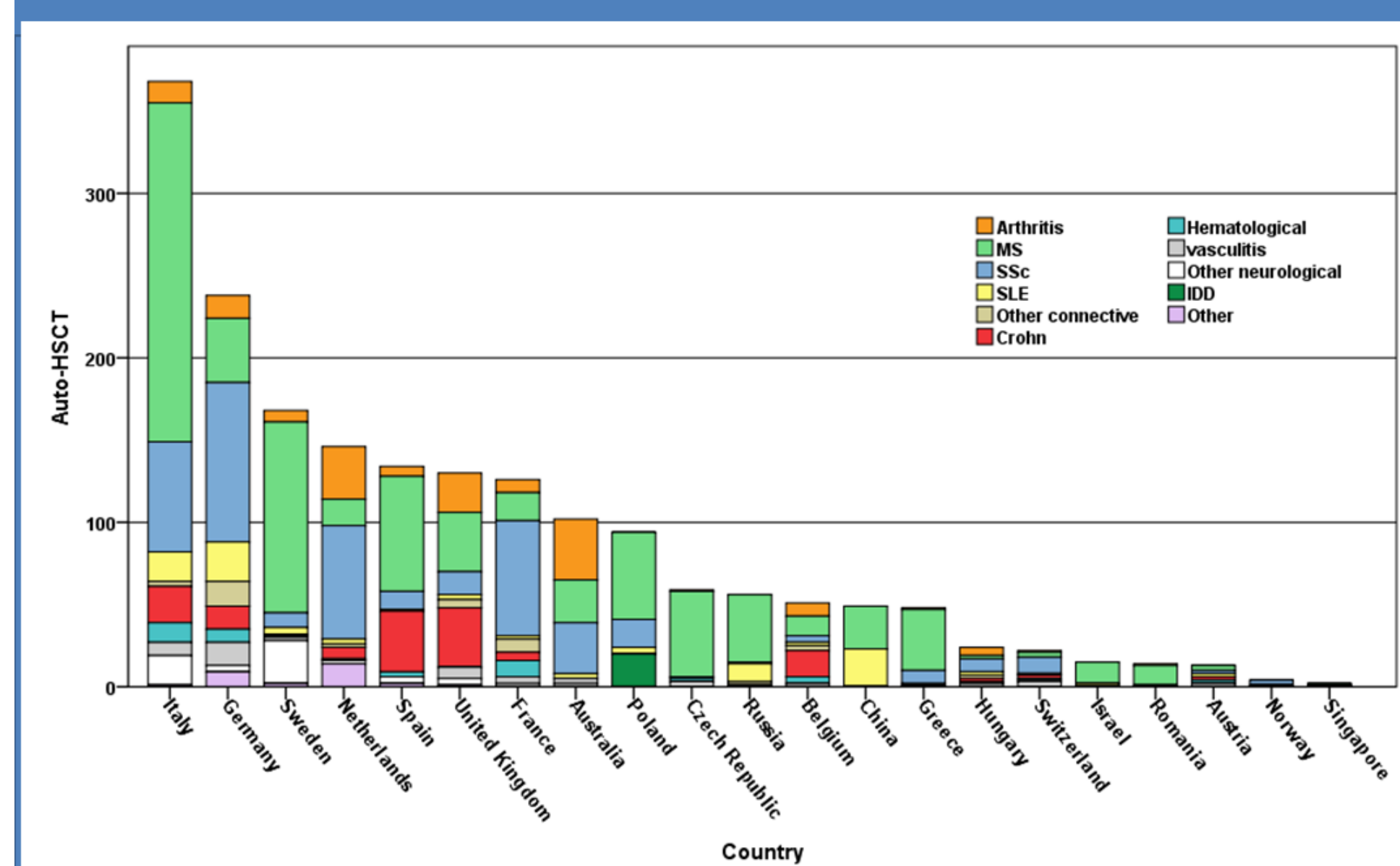
	Autografts n=1927	Allografts n=141
First	1905	111
Second	19	24
Third	1	6
Median age at 1st transplant	37y (3-76)	12y (<1-62)

► MULTIPLE SCLEROSIS	811	► HAEMATOLOGICAL	97
► CONNECTIVE TISSUE	599	ITP	31
SSc	438	Evans'	20
SLE	113	AIHA	25
PM-DM	17	Other	22
Sjogren	3	► VASCULITIS	50
Antiphosph. Syndrome	5	Wegener's	13
Other/Unknown	22	Behcet's	9
► ARTHRITIS	182	Takayasu	2
Rheumatoid arthritis	85	Polyarteritis	4
Juvenile chronic arthritis :		Churg-Strauss	2
*Systemic JIA	57	Other/Unknown	20
*Other JIA	18	► OTHER NEUROLOGICAL	85
*Polyarticular JIA	15	NMO	22
Psoriatic arthritis	4	CIDP	31
Other	6	Myasthenia gravis	7
► INFLAMMATORY BOWEL	190	Other/Unknown	26
Crohn's disease	157	► INSULIN DEPENDENT DIABETES	20
Ulcerative colitis	4	► OTHER/ Unknown	35
Other	24		

HSCT for AD per year

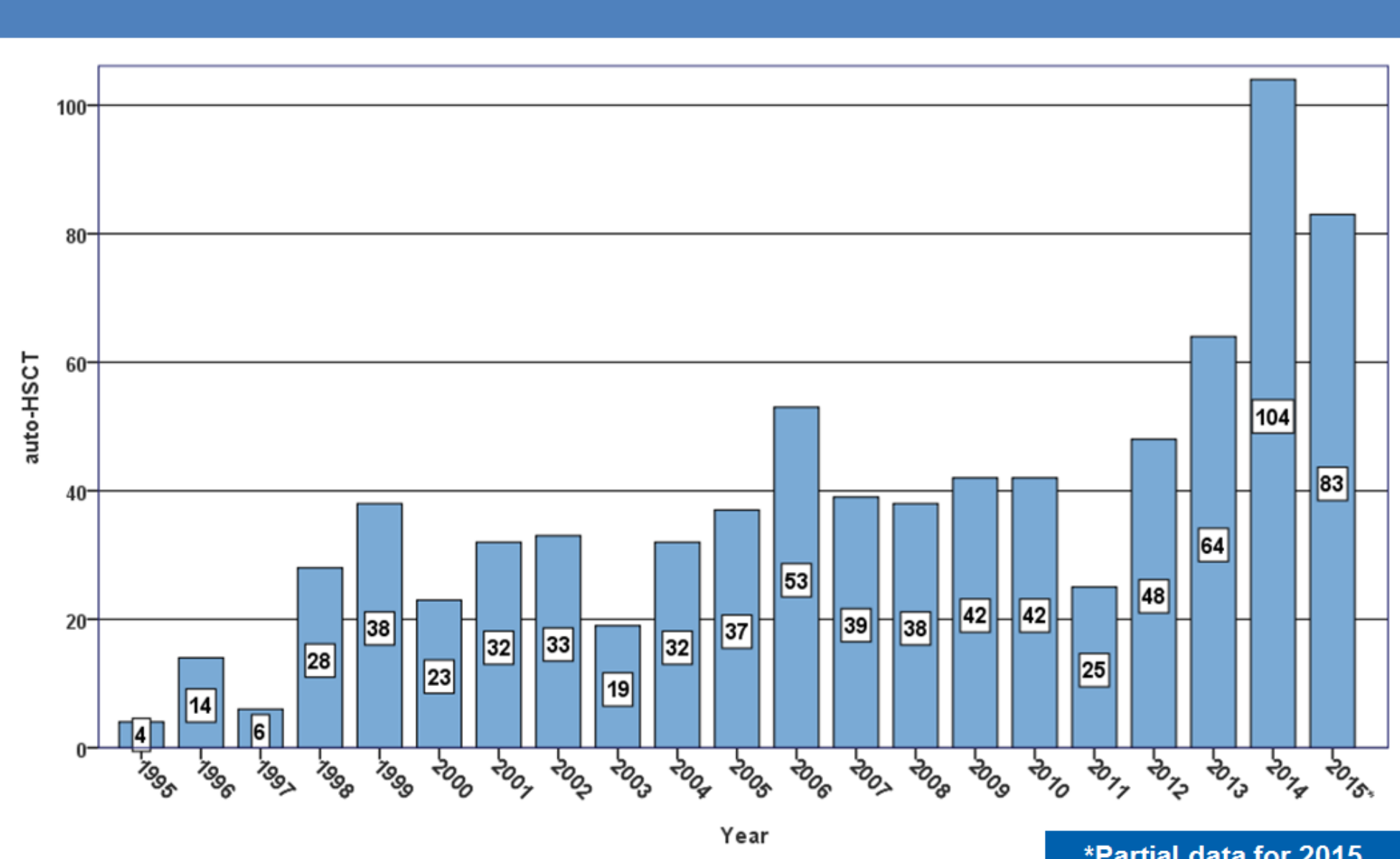


Auto-HSCT /diagnosis per country (n= 1927)



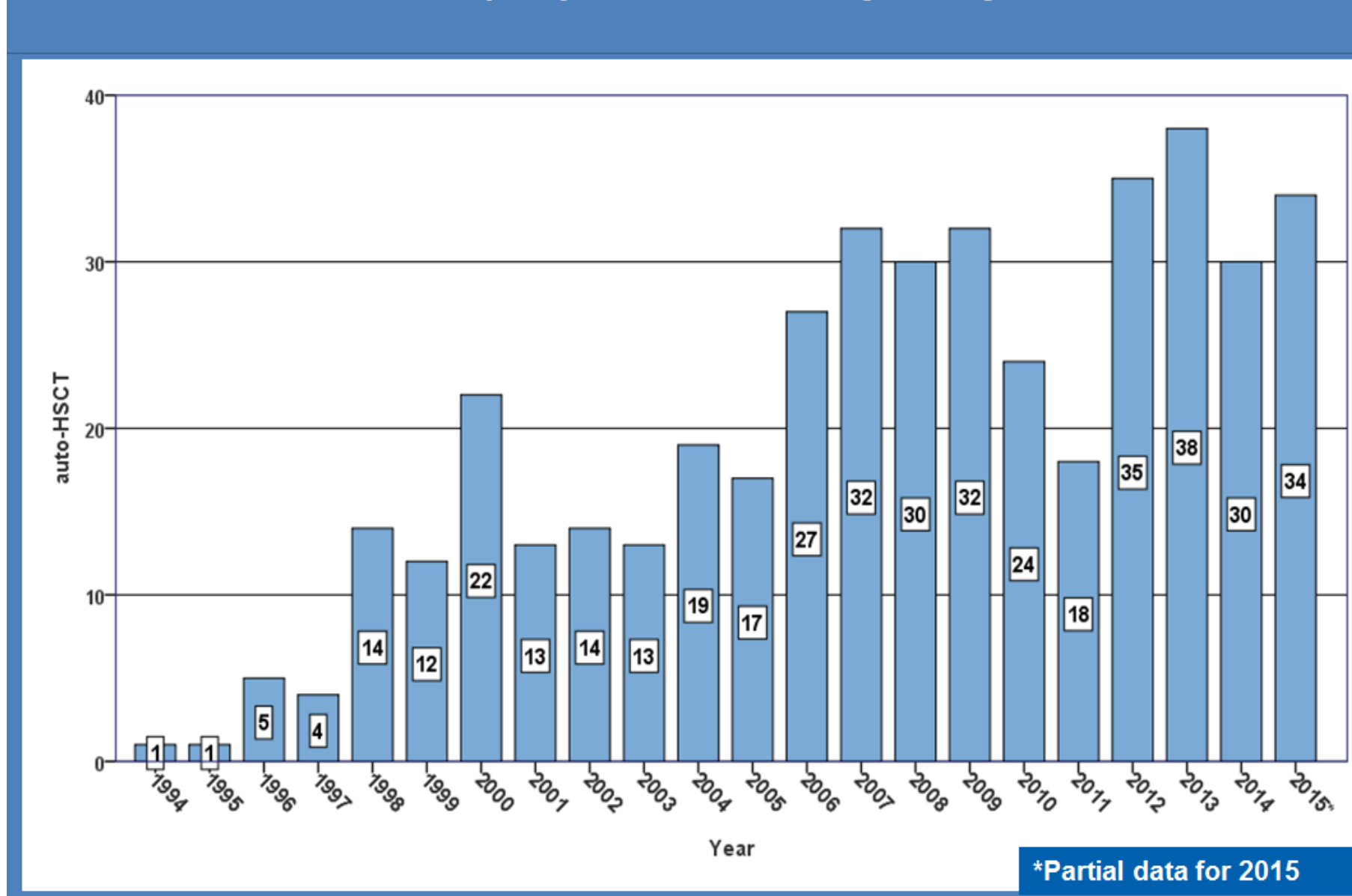
Multiple sclerosis

Auto per year- 1995/2015* (n=806)



Systemic sclerosis

Auto per year-1994/2015 (n=438)



The NISSc 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

Major educational courses

- 1- Workshop on Autoimmunity - March 23, 2015 in Istanbul, Turkey
- 2- ADWP educational meeting on Stem Cell Therapy (HSCT & MSC) and Autoimmune Diseases - January 22-23 2016 in Berlin, Germany

New prospectives non-interventional studies

- 1- NISMO: Autologous Hematopoietic Stem Cell Transplantation for Neuromyelitis Optica (NMO) and NMO spectrum disorders (R Grecco, Milan, Italy)
- 2- OMST: Multiple sclerosis patients treated with auto-HSCT (R Saccardi, Florence, Italy)
- 3- NISSC 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 ECCO), 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 – 2015 publications

- Onset and Outcome of Pregnancy after Autologous Haematopoietic SCT (AHSCT) for Autoimmune Diseases: A Retrospective Study of the EBMT Autoimmune Diseases Working Party (ADWP) – Snarski E et al. *Bone Marrow Transplant.* 2015 Feb;50(2):216-20
- Autologous Hematopoietic Stem Cell Transplantation in Neuromyelitis Optica: A Registry Study of the EBMT Autoimmune Diseases Working Party- Greco, Raffaella et al. *Mult Scler.* 2015 Feb;21(2):189–97
- SCT for Severe Autoimmune Diseases: Consensus Guidelines of the European Society for Blood and Marrow Transplantation for Immune Monitoring and Biobanking - Alexander, Tobias et al. *Bone Marrow Transplant.* 2015 Feb;50(2):173–80.
- Autologous Haematopoietic Stem Cell Transplantation in Multiple Sclerosis: a phase II trial-ASTIMS - Mancardi, GL et al. *Neurology.* 2015 Mar 10;84(10):981–8.
- Does Ex Vivo CD34+ Positive Selection Influence Outcome after Autologous Hematopoietic Stem Cell Transplantation in Systemic Sclerosis Patients? Oliveira Maria Carolina et al. *Bone Marrow Transplantation* 2015, Dec 7. doi:10.1038/bmt.2015.299