



European Society for Blood and  
Marrow Transplantation

# Autoimmune Diseases Working Party

Chair: John Snowden

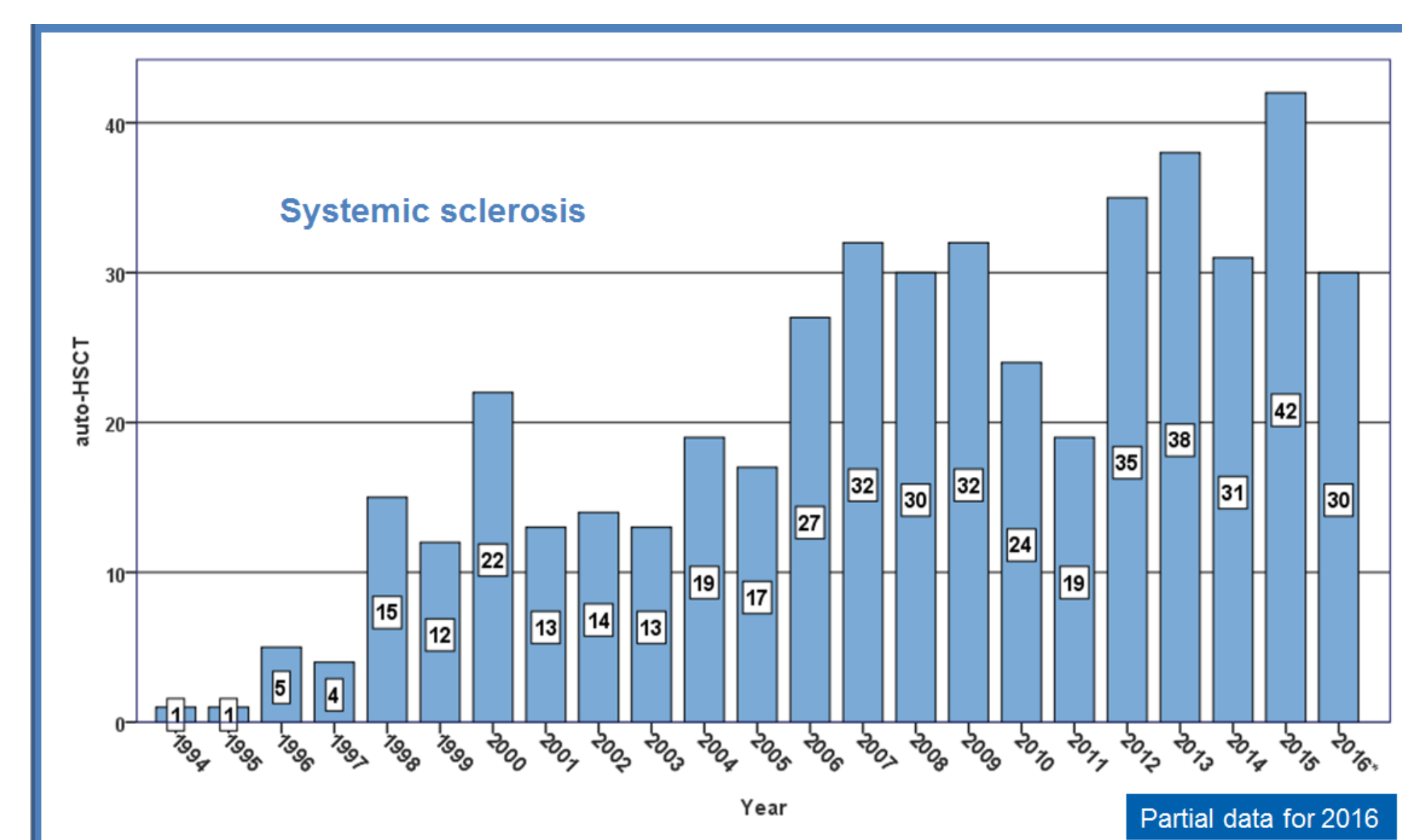
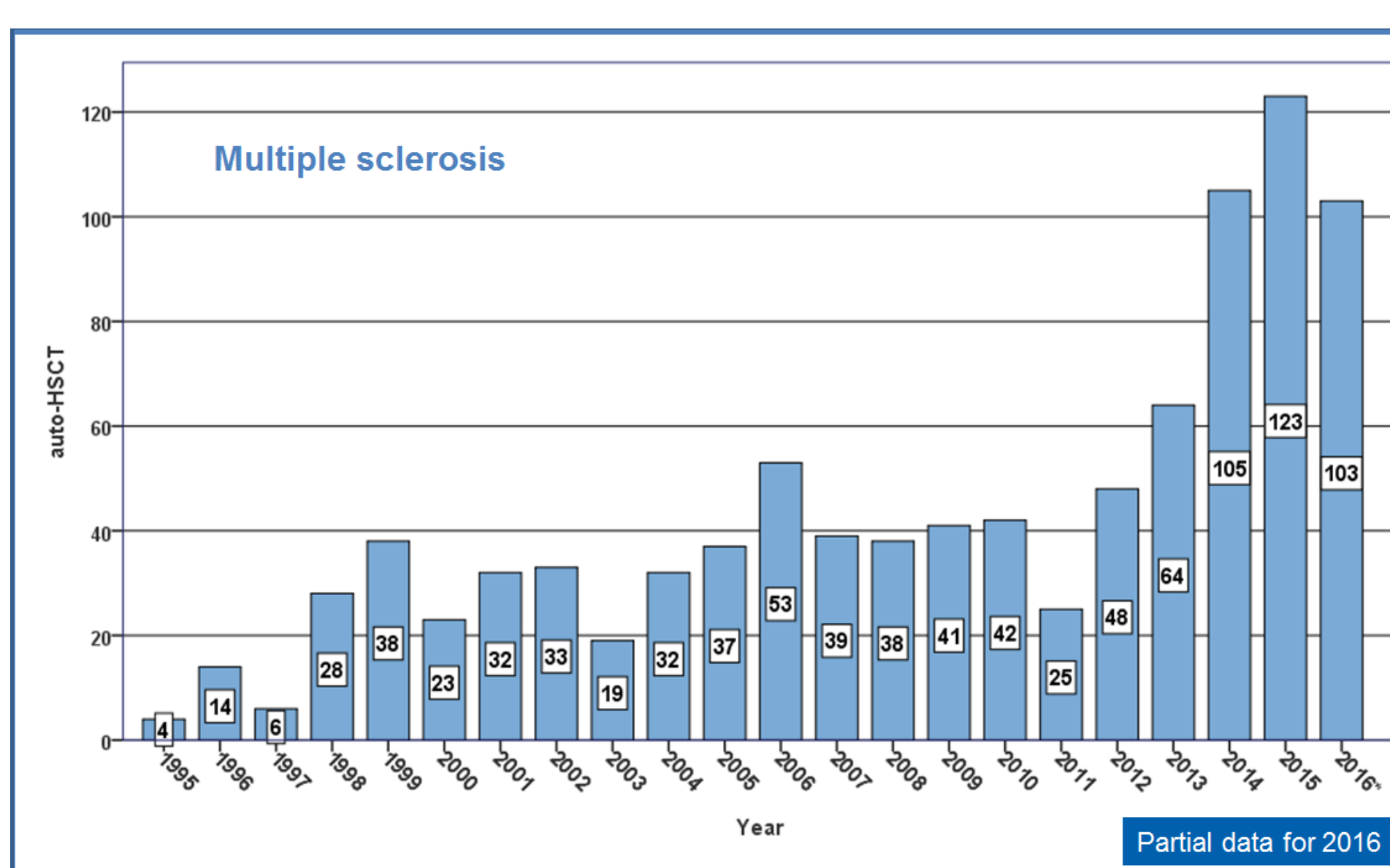
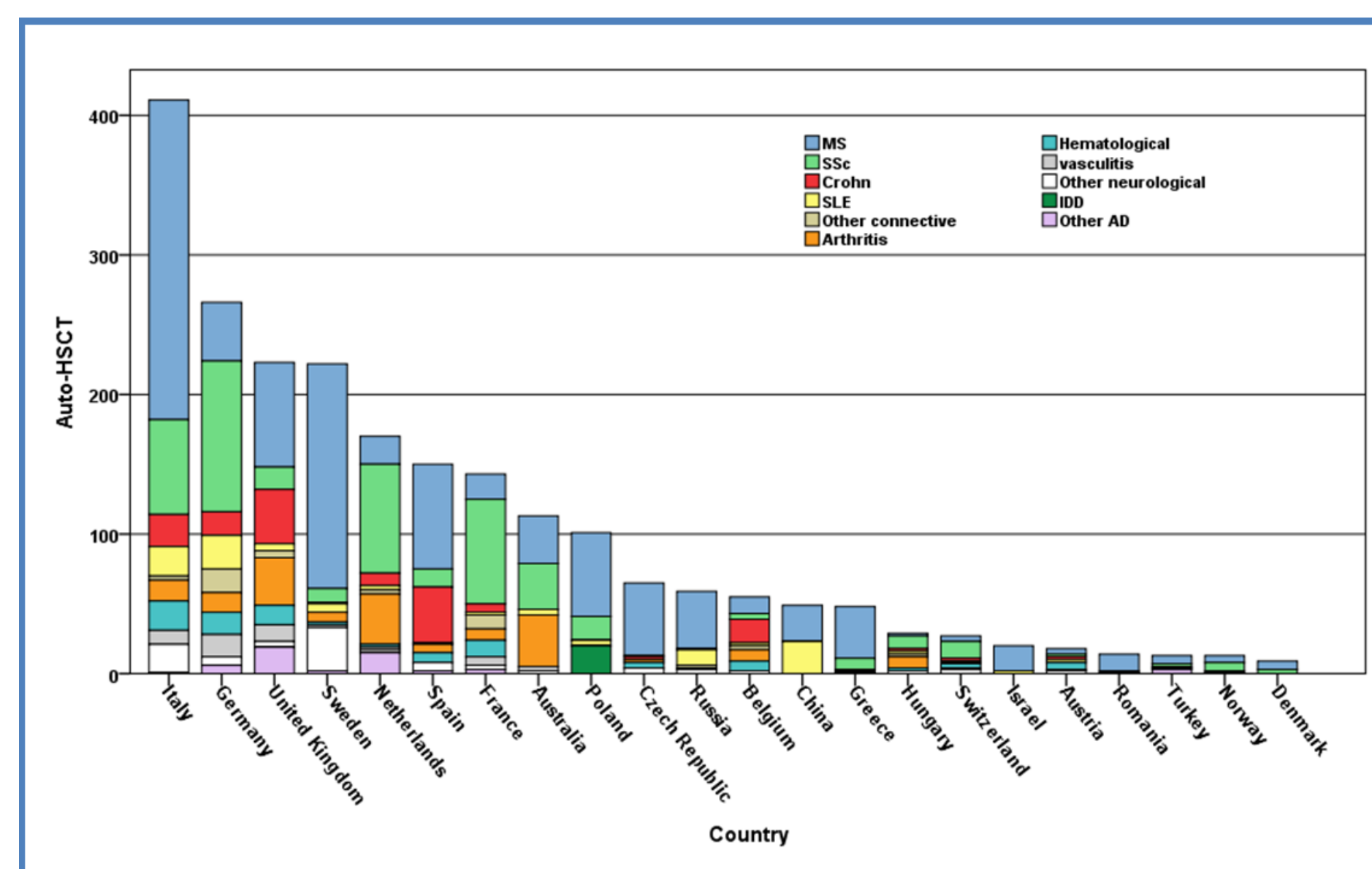
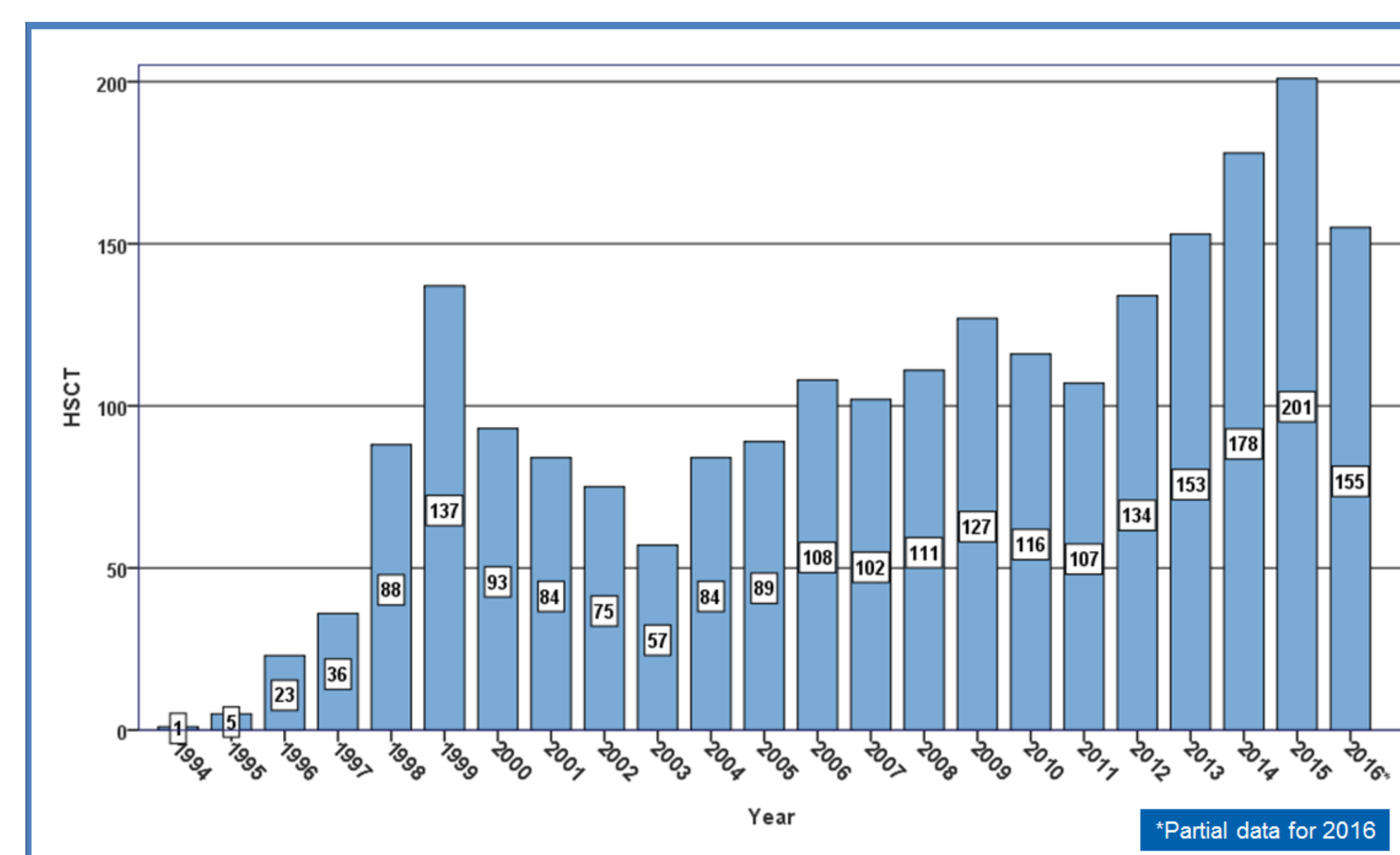
Secretary: Tobias Alexander

Study coordinator: Manuela Badoglio

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

Transplant procedures	2268	
Patients	2217	
Male/Female %	39/61	
Paediatric/ adult / %	11/89	
Centres /Countries	254 / 40	
Overall follow up (median, Yr HSCT < 2016)	3y (<1-20)	
	Autografts n=2125	Allografts n=142
First	2104	112
Second	20	24
Third	1	6
Median age at 1st transplant	37y (3-76)	12y (<1-62)

► MULTIPLE SCLEROSIS	955	► HAEMATOLOGICAL	101
► CONNECTIVE TISSUE	643	ITP	31
SSc	477	AIHA	25
SLE	115	Evans'	21
PM-DM	18	Other	24
Sjogren	3	► VASCULITIS	54
Antiphosph. Syndrome	5	Wegener's	14
Other/Unknown	25	Behcet's	10
► ARTHRITIS	183	Takayasu	2
Rheumatoid arthritis	81	Polyarteritis	4
Juvenile chronic arthritis :		Churg-Strauss	2
*Systemic JIA	58	Other/Unknown	22
*Other JIA	18	► OTHER NEUROLOGICAL	93
*Polyarticular JIA	16	NMO	24
Psoriatic arthritis	3	CIDP	37
Other	7	Myasthenia gravis	7
► INFLAMMATORY BOWEL	199	Other/Unknown	25
Crohn's disease	166	► INSULIN DEPENDENT DIABETES	20
Ulcerative colitis	4	► OTHER	20
Other	29		



### 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

### 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 perspectives 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)

### ADWP – Recent publications

1. **Onset and outcome of pregnancy after autologous haematopoietic SCT (AHSCT) for autoimmune diseases.** Snarski E, et al.. *BMT*. 2015 Feb;50(2):216–20.
2. **Autologous hematopoietic stem cell transplantation in neuromyelitis optica: a registry study.** Greco R, et al. *Mult Scler*. 2015 Feb;21(2):189–97.
3. **Does ex vivo CD34+ positive selection influence outcome after autologous hematopoietic stem cell transplantation in systemic sclerosis patients?** Oliveira MC, et al.. *BMT* 2015 Dec 7;
4. **Autologous Hematopoietic Stem Cell Transplantation for Refractory Crohn Disease: A Randomized Clinical Trial.** Hawkey CJ, et al. *JAMA*. 2015 Dec 15;314(23):2524–34.
5. **Autologous hematopoietic stem cell transplantation in multiple sclerosis: a phase II trial.** Mancardi GL, et al. *Neurology*. 2015 Mar 10;84(10):981–8.
6. **SCT for severe autoimmune diseases: consensus guidelines of the European Society for Blood and Marrow Transplantation for immune monitoring and biobanking.** Alexander T, et al. *BMT* 2015 Feb;50(2):173–80.
7. **Hematopoietic SCT in Europe 2013: recent trends in the use of alternative donors showing more haploidentical donors but fewer cord blood transplants.** Passweg JR, et al. *BMT*. 2015 Apr;50(4):476–82.
8. **Indications for allo- and auto-SCT for haematological diseases, solid tumours and immune disorders: current practice in Europe, 2015.** Sureda A, et al.. *BMT*. 2015 Aug;50(8):1037–56.
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

### 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.