



Autoimmune Diseases Working Party

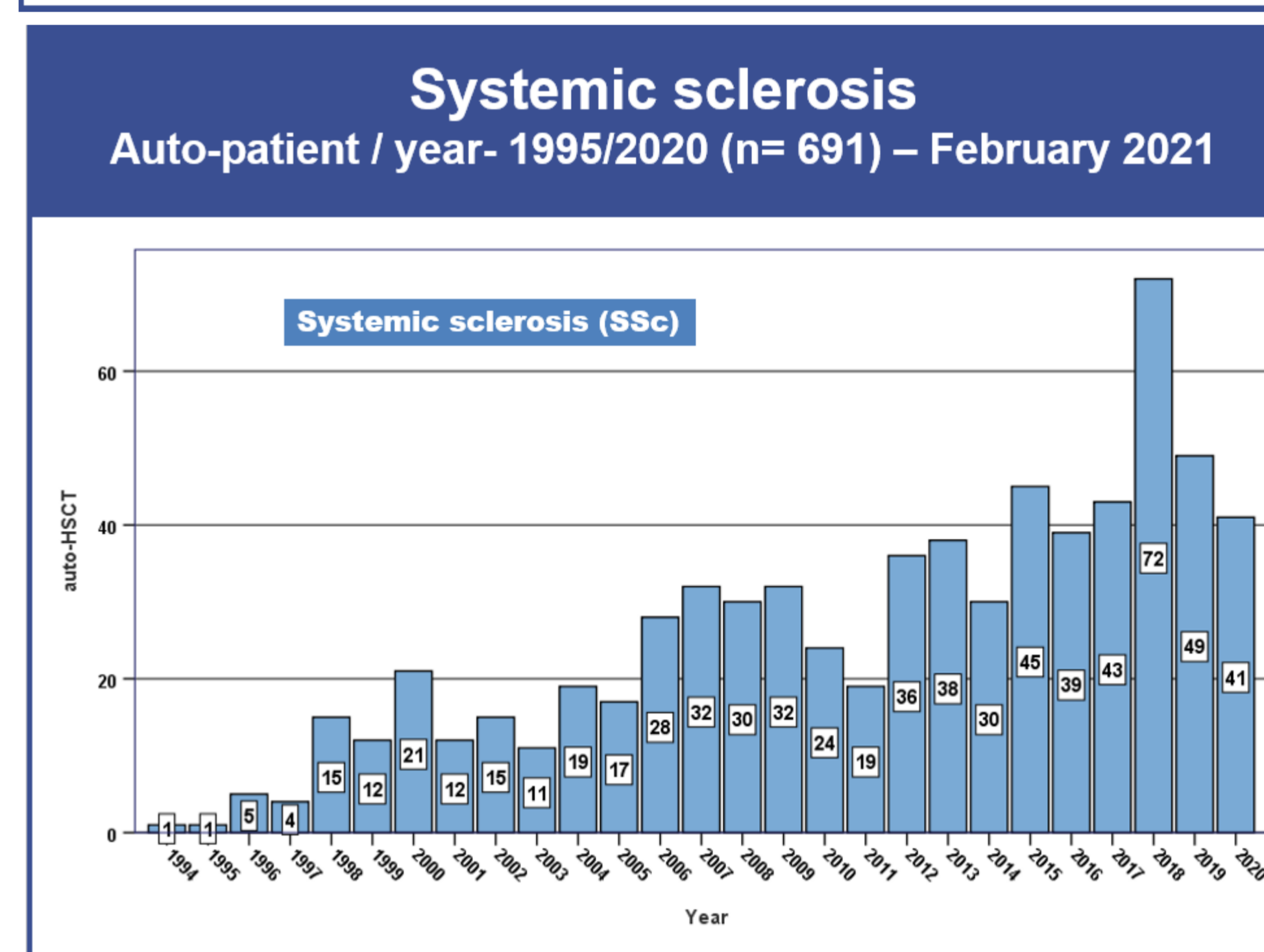
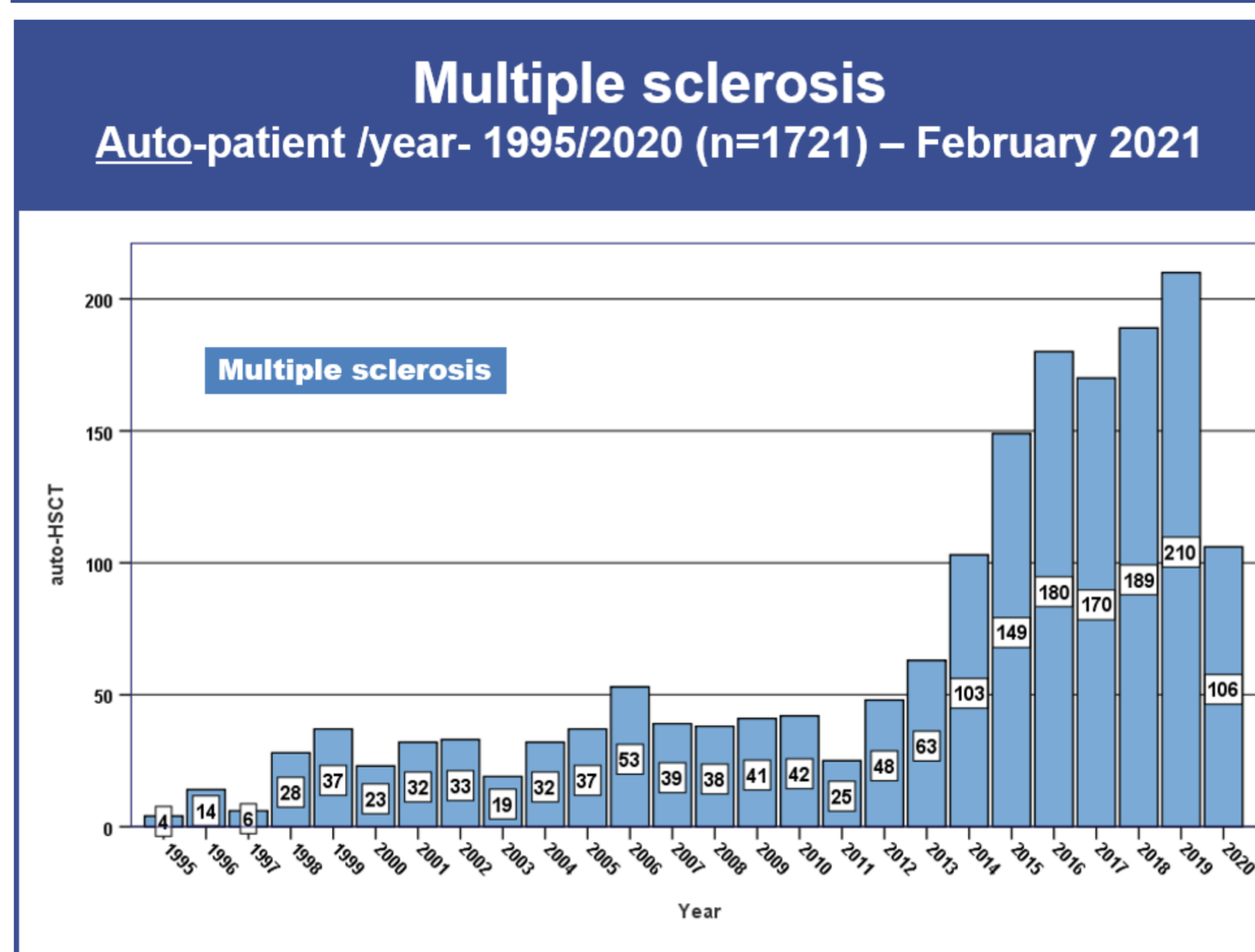
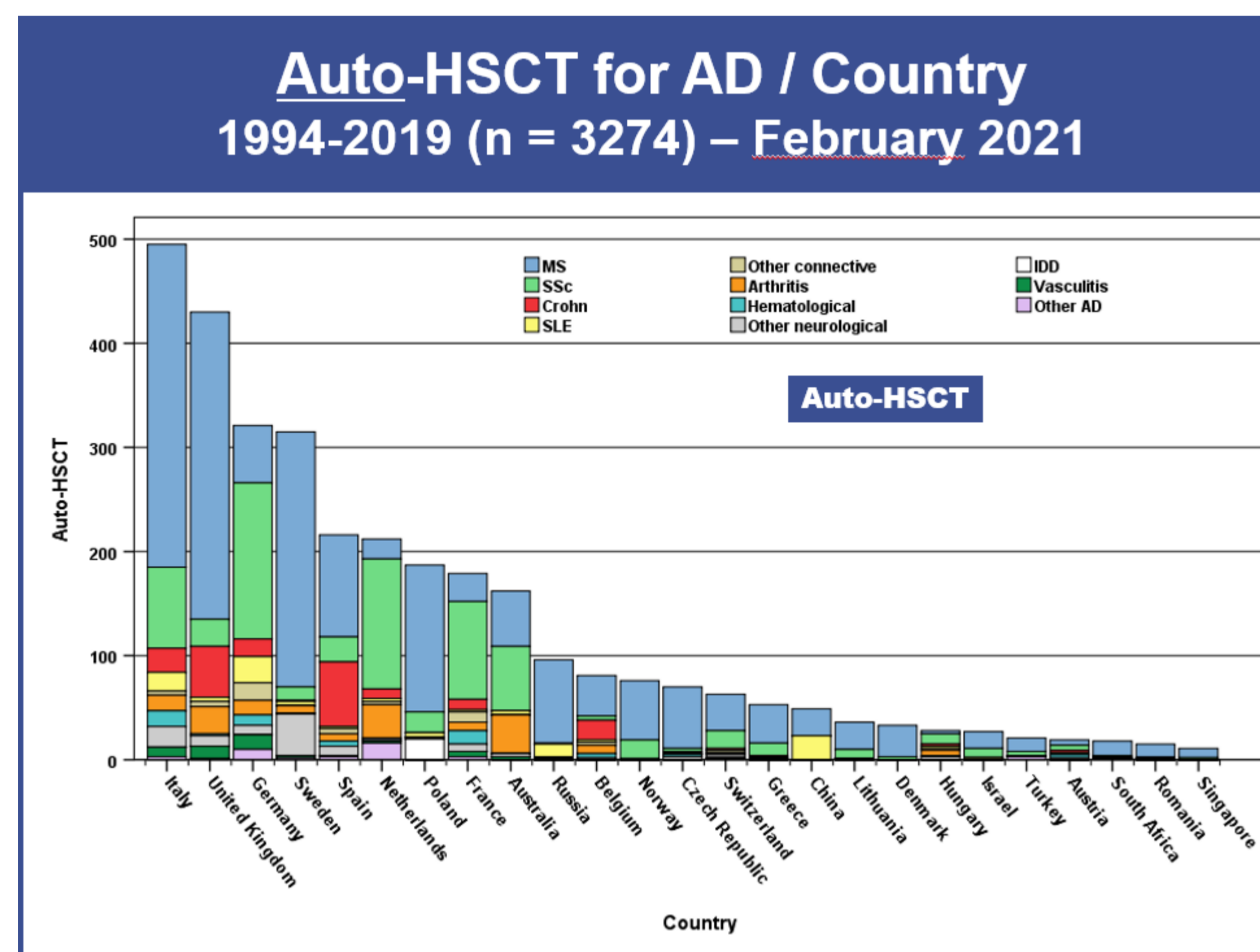
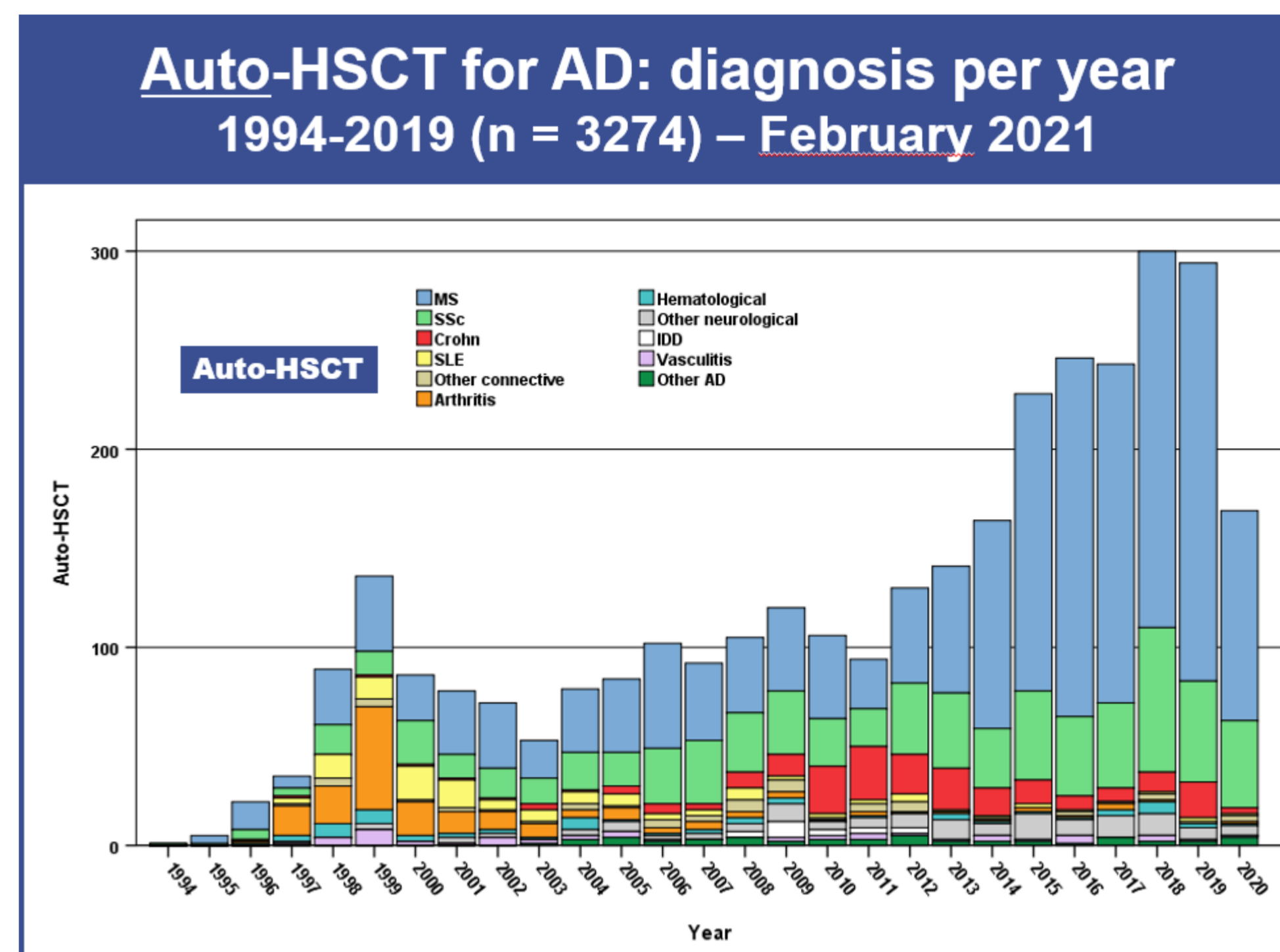
Raffaella Greco, ADWP Chair
Tobias Alexander, ADWP Secretary
Manuela Badoglio, ADWP Study Coordinator

Number of HSCT for Autoimmune Diseases: 3514 EBMT Registry – February 2021

ADWP - Number of HSCT: 3514 EBMT Registry – January 2021	
Transplant procedures	3514
Patients	3442
Male/Female %	40/60
Paediatric/Adult %	9/91
Centres/Countries	310/44
Overall follow up (alive, median)	3y (<1-24)

	Autografts n=3277	Allografts n=237
First	3245	197
Second	29	34
Third	2	6
Median age at 1st transplant	38y (3-76)	11y (<1-65)

ADWP - Number of HSCT: 3448 EBMT Registry – November 2020	
▶ MULTIPLE SCLEROSIS	1738
▶ CONNECTIVE TISSUE	886
▶ ARTHRITIS	196
▶ INFLAMMATORY BOWEL	258
▶ HAEMATOLOGICAL	139
▶ VASCULITIS	64
▶ OTHER NEUROLOGICAL	134
▶ INSULIN DEPENDENT DIABETES	20
▶ OTHER	79



Principal research studies

- 1- MS Comparison of CYC+ATG vs. BEAM+ATG conditioning regimens in autologous HSCT for MS
- 2- NISSC II: Post-AHSC management and mechanistic immunological reconstitution for patients with systemic sclerosis
- 3- Late complications after autologous HSCT for Autoimmune Diseases
- 4- Viral reactivations: retrospective study on viral infections post auto-HSCT in Autoimmune Diseases
- 5- Indications and outcomes of re-transplantation for Autoimmune Diseases
- 6- Prospective non-interventional on patients with multiple sclerosis (OMST).

Disease-specific retrospective studies of autologous HSCT

- Immune cytopenias
- Rare neurological diseases (CIDP, Stiff Person Syndrome, myasthenia gravis, NMO and others, Behçet's Disease)
- Juvenile and adult systemic arthritis/Still's disease
- Polymyositis-Dermatomyositis

Surveys

- Survey of national provision/reimbursement of HSCT and follow up in Autoimmune Diseases

Guidelines and recommendations

- JACIE-ADWP COVID 19 Guidelines. Hematopoietic stem cell transplantation for autoimmune diseases in the time of COVID-19: EBMT guidelines and recommendations
- HSCT in adult rheumatological autoimmune diseases : guidelines and recommendations from the EBMT ADWP in collaboration with European Reference Network (ERN) for rare and complex connective tissue diseases (ERN ReCONNET)
- Position paper with European Academy of Neurology EAN/ECTRIMS and EBMT

Prospective long-term follow of studies involving EBMT registry

- a- Harmonisation of long-term follow-up of clinical trials in MS (EBMT involvement TBC)

Major achievements

Autoimmune diseases (ADs) are a major cause of morbidity. HSCT has evolved for >20 years as a specific treatment of patients with severe ADs, through eradication of the pathologic, immunologic memory and profound immune 'resetting'.

HSCT for ADs is today facing a unique developmental phase across EBMT, with the ADWP central to bringing together HSCT and disease specialist communities. The ADs section of the EBMT Registry is the largest database of its kind worldwide, reporting more than 3,300 transplants. The ADWP is continuing to expand the evidence-base and support best practice with studies and guidelines, including significant collaborative outputs with other EBMT Working Parties, JACIE, the EBMT Nurses Group and Patient Advocacy Committee.

Education continued to be central to global ADWP activities, despite the global COVID-19 pandemic and its extensive worldwide impact in 2020. The ADWP educational meeting was a unique event, being the first virtual and the most successful meeting of the working party, with 230 attendees from 36 countries, attracting hematologists, ADs experts and nurses. In addition, we continued to build closer links with partners outside EBMT, particularly in the Americas and Russia.

'Implementation science' remains central to delivery of HSCT for AD within our health services. The future depends on quality of outcomes and health economics versus non-transplant biological treatments. Strategic priorities for the ADWP include ongoing work with international disease-specialist societies, whilst working closely with others within EBMT and JACIE to assure best practice, clinical quality and patient-centred care in HSCT for ADs.

Key publications

- Hematopoietic Stem Cell Transplantation for Autoimmune Disease. Alexander T, et al. *Annu Rev Med.* 2020 Oct 26. *Annu Rev Med.* 2021 Jan 27;72:215-228.
- Rehabilitation before and after Autologous HSCT for patients with Multiple Sclerosis : Consensus guidelines and recommendations for best clinical practice on behalf of the ADWP, Nurses Group and Patient Advocacy Committee of the EBMT. Roberts F et al, *Frontiers in Neurology*, 2020, 11 December 2020
- Diagnosis and Management of Secondary HLH/MAS Following HSCT and CAR-T Cell Therapy in Adults; A Review of the Literature and a Survey of Practice Within EBMT Centres on Behalf of the ADWP and TCWP. Sandler RD, et al. *Front Immunol.* 2020 Mar 31;11:524
- Autologous HSCT and other cellular therapy in multiple sclerosis and immune-mediated neurological diseases: updated guidelines and recommendations from the EBMT ADWP and JACIE. Sharrack B, et al. *Bone Marrow Transplant.* 2020 Feb;55(2):283-306.
- Evaluating the clinical effectiveness of autologous HSCT versus disease-modifying therapy in multiple sclerosis using a matching-adjusted indirect comparison: an exploratory study from the ADWP EBMT. Tappenden P et al. (2020). *Bone Marrow Transplant.* Jul;55(7):1473-1475.
- Autologous HSCT with reduced-intensity conditioning regimens in refractory Takayasu arteritis: a retrospective multicenter case-series from the ADWP of the EBMT. Laurent C et al. (2020). *Bone Marrow Transplant.* Apr 22.
- Autoimmune cytopenias (AIC) following allogeneic HSCT for acquired aplastic anaemia: a joint study of the ADWP/SAAWP of the EBMT. Miller, P.D.E et al. (2020). *Bone Marrow Transplant.* 55, 441-451.
- Autologous HSCT for progressive systemic sclerosis: a prospective non-interventional study across Europe (NISSC). Henes, J et al. (2020). *Haematologica* Jan 16
- Autologous HSCT for ANCA-associated vasculitis: a retrospective survey of patients reported to EBMT registry. Alexander, T et al. (2020). *Bone Marrow Transplant.* Jul;55(7):1512-1515.
- Autologous HSCT for autoimmune diseases: Current indications and mode of action, a review on behalf of the EBMT ADWP. Alexander T et al. (2020). *Rheumatol.* Jun;79(5):419-428.