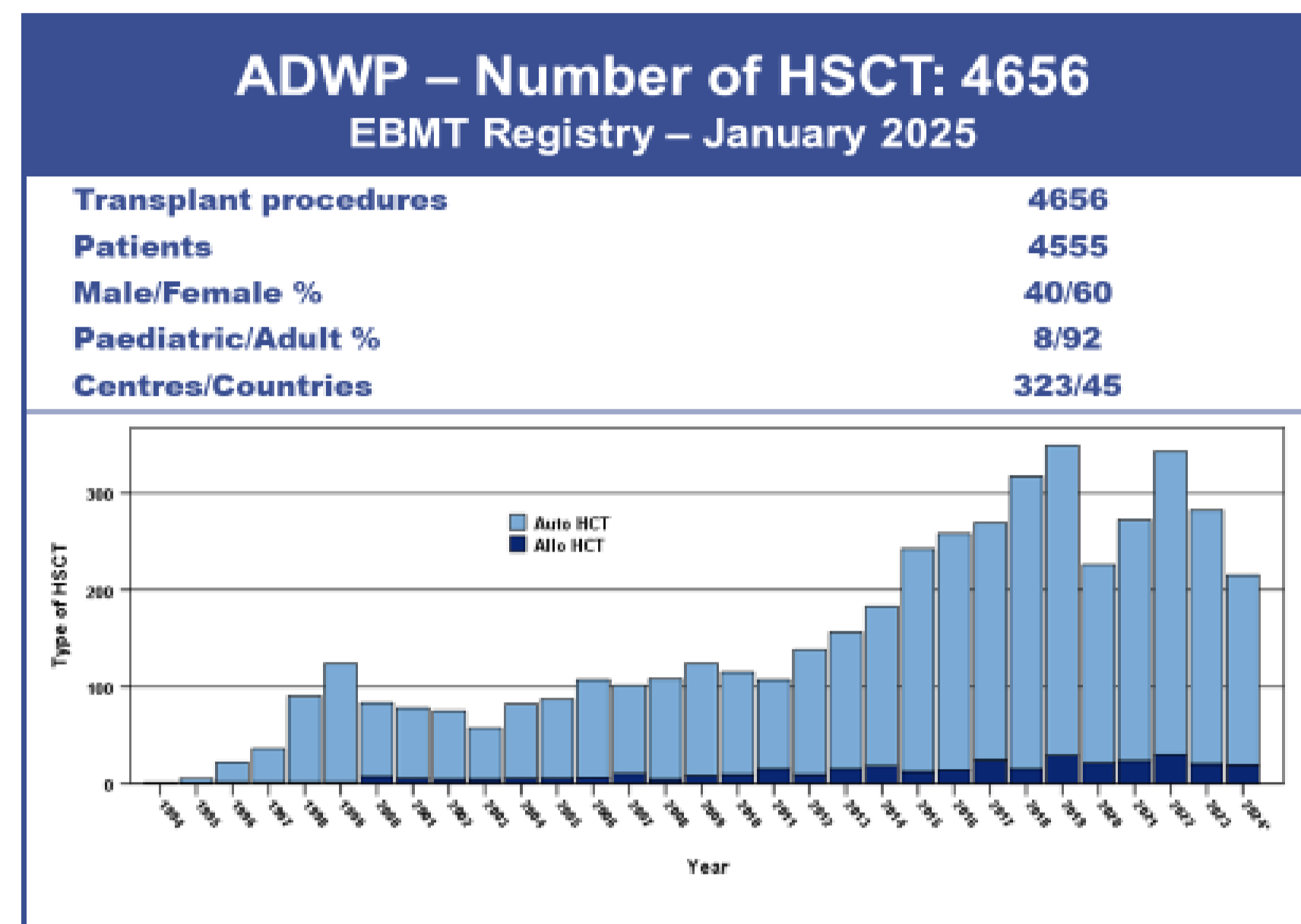


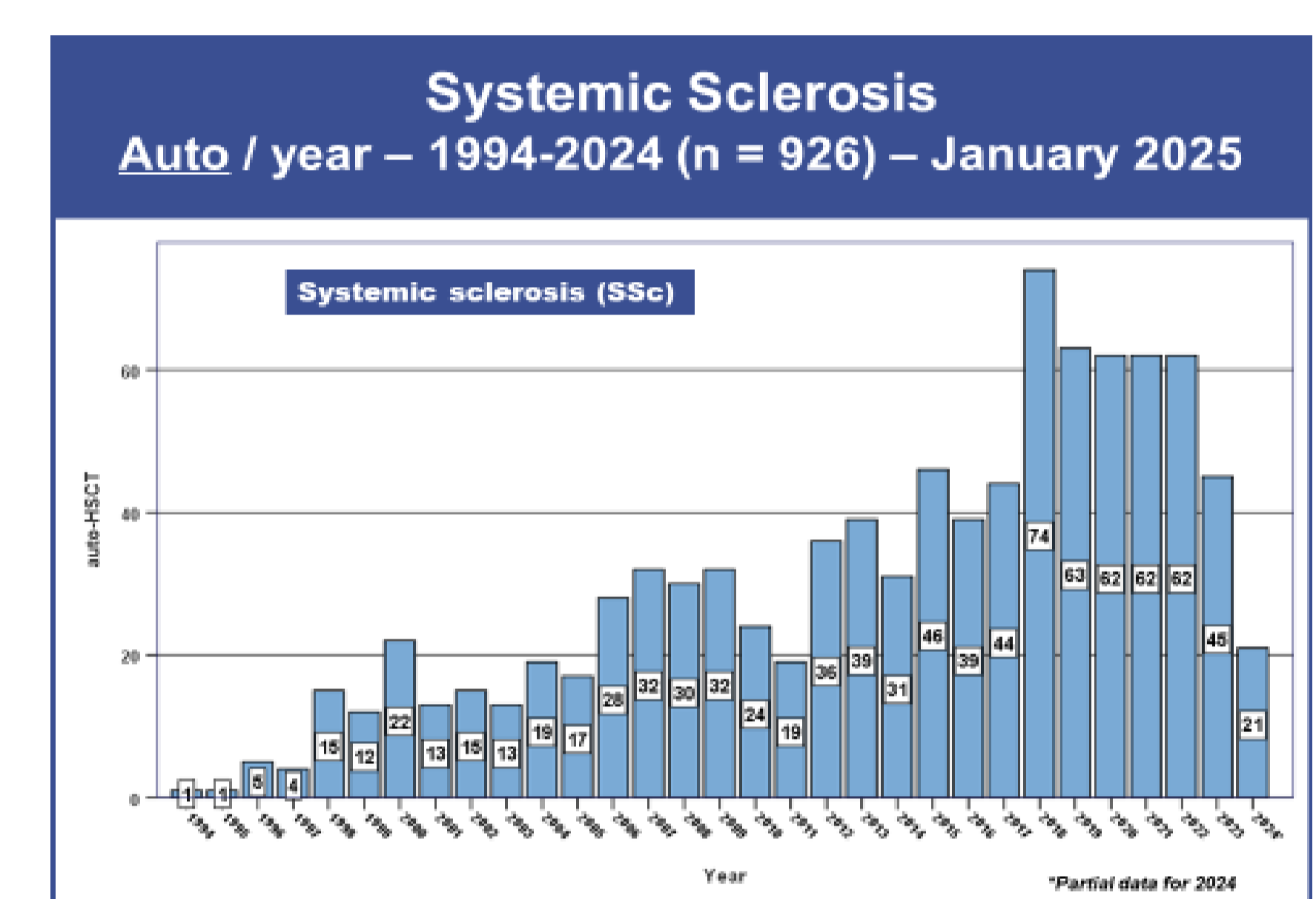
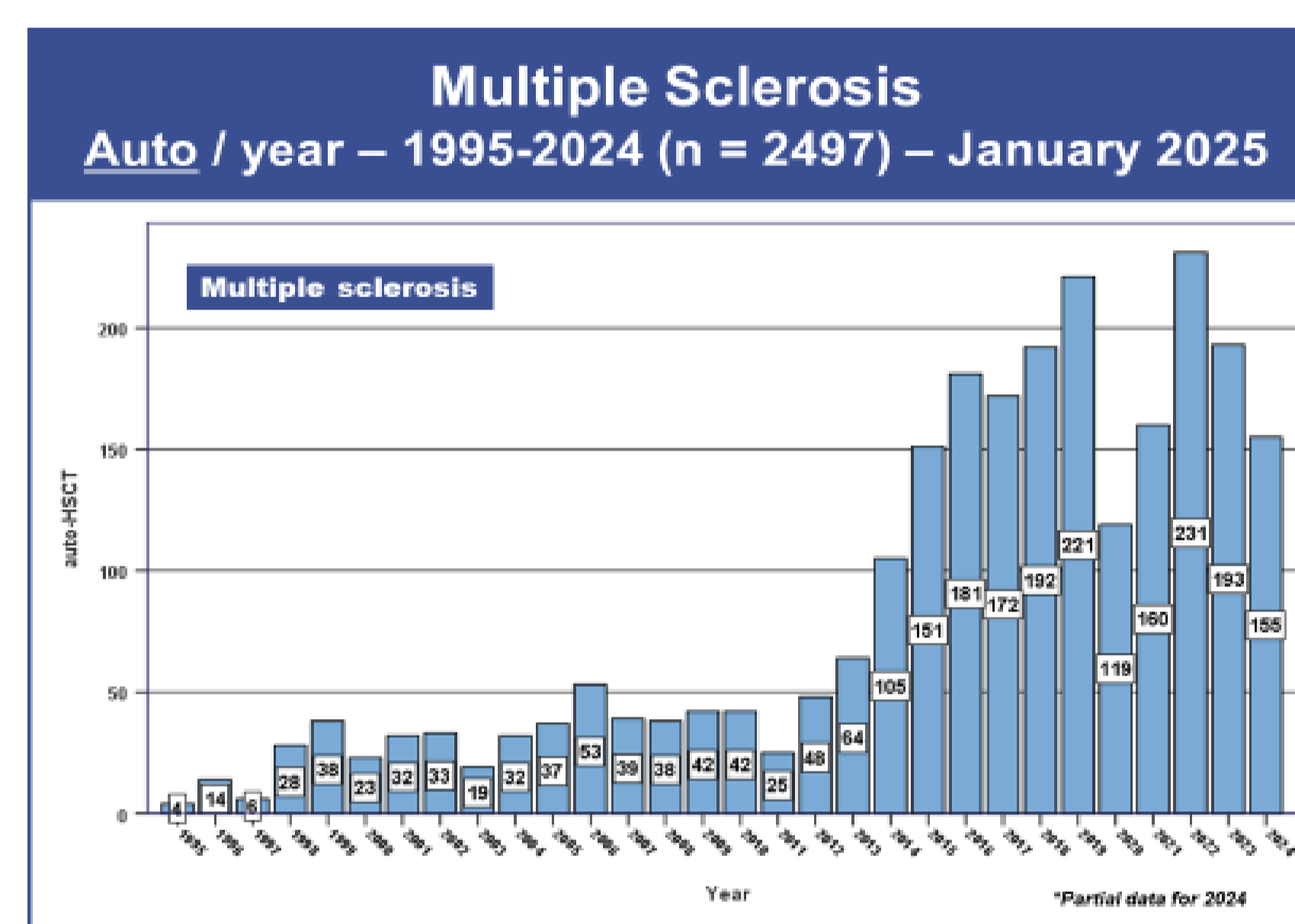
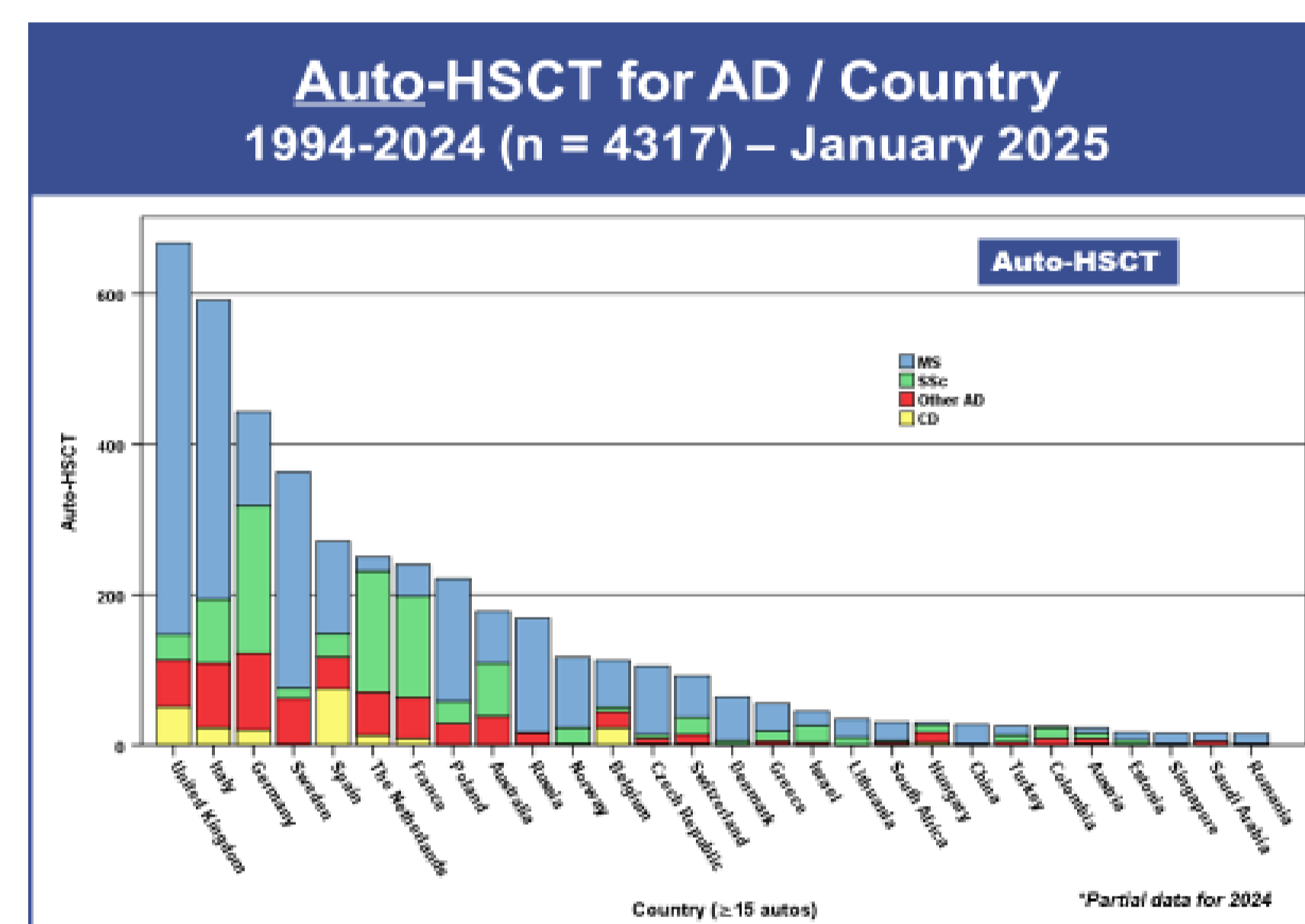
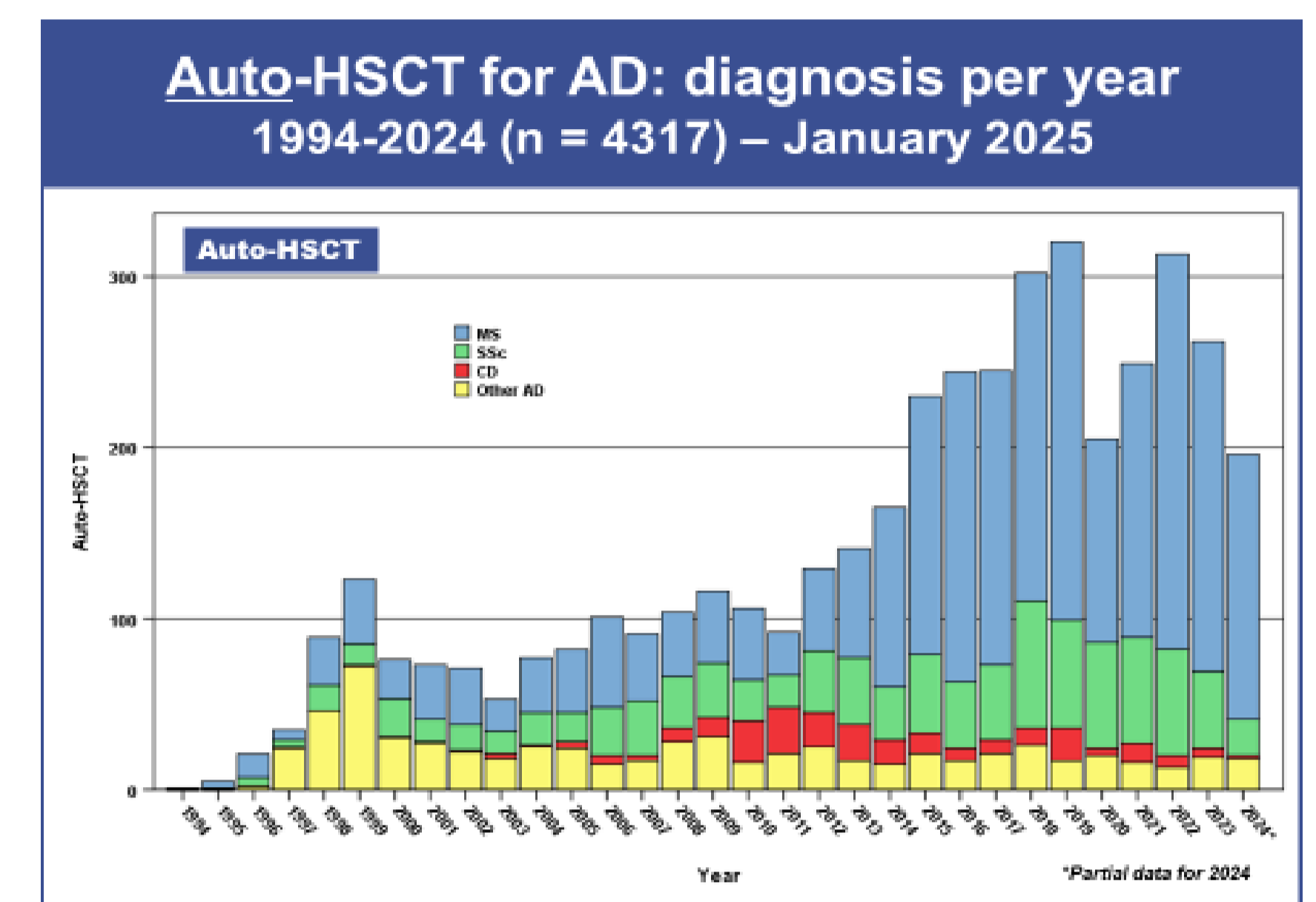
Tobias Alexander, ADWP Chair  
 Elisa Roldan, ADWP Secretary  
 Manuela Badoglio, ADWP Study Coordinator  
 Claudia Boglione, Nurse representative

## Number of HSCT for Autoimmune Diseases: 4656 EBMT Registry



**ADWP – Number of HSCT: 4649**  
 EBMT Registry – January 2025

▶ MULTIPLE SCLEROSIS	2506	▶ HAEMATOLOGICAL	190
▶ CONNECTIVE TISSUE	1101	ITP	43
SSC	927	AIHA	38
SLE	108	ALS (primary)	30
PM-DM	19	Evans'	26
Sjogren	6	Other	53
Antiphosph. Syndrome	7	▶ VASCULITIS	72
Other/Unknown	34	Granulomatosis with Polyangiitis (GPA)	12
▶ ARTHRITIS	207	Behcet's	19
Rheumatoid arthritis	75	Eosinophilic GPA	2
Juvenile chronic arthritis:		Polyarteritis	4
*Systemic JIA	79	Takayasu	3
*Articular JIA	22	Other	32
*Other JIA	19	▶ OTHER NEUROLOGICAL	150
Psoriatic arthritis	4	NMO	31
Other	8	CIDP	69
▶ INFLAMMATORY BOWEL	286	Myasthenia gravis	11
Crohn's disease	236	Other	39
Coeliac disease	18	▶ INSULIN DEPENDENT DIABETES	20
Other	32	▶ OTHER	125



### Principal research studies

- Prospective non-interventional study in patients with multiple sclerosis (OMST)
- Comparison of CYC/ATG vs. BEAM/ATG conditioning regimens in autologous HSCT for MS
- NISSC II: Post-AHSCT management and mechanistic immunological reconstitution for patients with systemic sclerosis
- NISSC I: Long Term Extension study on long-term outcomes of auto-HSCT for systemic sclerosis
- Viral reactivations: retrospective study on viral infections post auto-HSCT in autoimmune diseases

#### Retrospective studies on autologous HSCT

- Secondary Autoimmune and inflammatory Diseases after allo-HCT
- Rare neurological diseases (CIDP, Stiff Person Syndrome, myasthenia gravis, NMO and others),
- Fertility and pregnancy outcomes after auto-HSCT for AD.
- Indications and outcomes of re-transplantation for autoimmune diseases

#### Surveys

- Survey of current practice: CAR-T Cell Therapy in Autoimmune Diseases

#### Guidelines and recommendations

- HSCT Autologous haematopoietic stem cell transplantation for rheumatic diseases: best practice recommendations from the EBMT Practice Harmonization and Guidelines committee
- Position paper/recommendations on behalf of ECTRIMS and EBMT/ADWP for the indication and management of MS patients undergoing auto-HSCT

#### Studies involving EBMT registry, GoCART & CART Task Force

- GoCART project; Autoimmune and autoinflammatory complications, HLH/MAS included, of CAR-T cell therapy - a joint study on behalf of the EBMT - ADWP, CTIWP and TCWP
- CAR-T Cell Therapy in Autoimmune Diseases as Concomitant or Main Indication: a 2-step Joint Study on behalf of EBMT ADWP and CTIWP

### Key publications

1. Innovative cellular therapies for autoimmune diseases: expert-based position statement and clinical practice recommendations from the EBMT practice harmonization and guidelines committee. Greco R, Alexander T, Del Papa N, et al. *EClinicalMedicine*. 2024 Feb 10;69:102476. doi: 10.1016/j.eclinm.2024.102476.
2. Allogeneic hematopoietic cell transplantation for VEXAS syndrome: results of a multicenter study of the EBMT. Gurnari C, Koster L, Baaij L et al. *Blood Adv*. 2024 Mar 26;8(6):1444-1448. doi: 10.1182/bloodadvances.2023012478.
3. The Role of Chimeric Antigen Receptor T-Cell Therapy in Immune-Mediated Neurological Diseases. Brittain G, Roldan E, Alexander T et al. *Ann Neurol*. 2024 Sep;96(3):441-452. doi: 10.1002/ana.27029. Epub 2024 Jul 17. PMID: 39015040
4. Autologous haematopoietic stem cell transplantation for treatment of multiple sclerosis and neuromyelitis optica spectrum disorder - recommendations from ECTRIMS and the EBMT. Muraro PA, Mariottini A, Greco R, et al. *Nat Rev Neurol* 2025 Jan 15. doi: 10.1038/s41582-024-01050-x.
5. Patient-reported outcomes in HSCT for autoimmune diseases: Considerations on behalf of the EBMT ADWP, PAC, and Nurses Group. Alexander T, Tassy N, Domenech A et al., *J Allergy Clin Immunol. Clin Immunol Glob*. 2024 May 21;3(3):100283. doi: 10.1016/j.jacig.2024.100283.

### Major achievements

Autoimmune diseases (ADs) are a heterogeneous group of diseases, characterized by loss of immune tolerance, high chronicity, with substantial morbidity and mortality. Over the last 3 decades, hematopoietic stem cell transplantation (HSCT) has been increasingly used to treat patients affected by severe and refractory ADs, and is recently facing a unique developmental phase across EBMT centres. Autologous HSCT has become a standard-of-care part of treatment algorithms in multiple sclerosis and systemic sclerosis. Recently, innovative cellular therapies (i.e. CAR-T cells, mesenchymal cells, Tregs-based therapies) have been successfully adopted in ADs.

The EBMT Autoimmune Diseases Working Party (ADWP) has been central to development of these approaches. The ADWP is dedicated to promoting clinical activities, teaching and translational research on HSCT together with innovative cellular therapies as AD treatment, to re-induce self-tolerance by resetting the immune system. The ADs section of the EBMT Registry is the largest database of its kind worldwide, with over 4,300 HCT registrations.

A multidisciplinary approach is key in this field, and ADWP is continuing to expand the evidence-base and support best-practice with studies and guidelines, including significant collaborative outputs with EBMT Working-Parties, JACIE, Trainee-Committee, PAC and Nurses-Group.

Education is central in ADWP activities, including an educational Meeting on "Immunology and Cellular Therapies in Autoimmune Diseases" held in Milano, and a harmonization workshop on HCT in rheumatic diseases that took place in Lille 2024. Moreover, a section specifically designed for ADs was developed within the EBMT e-learning platform with contributions from key experts in the field.