



Autoimmune Diseases Working Party

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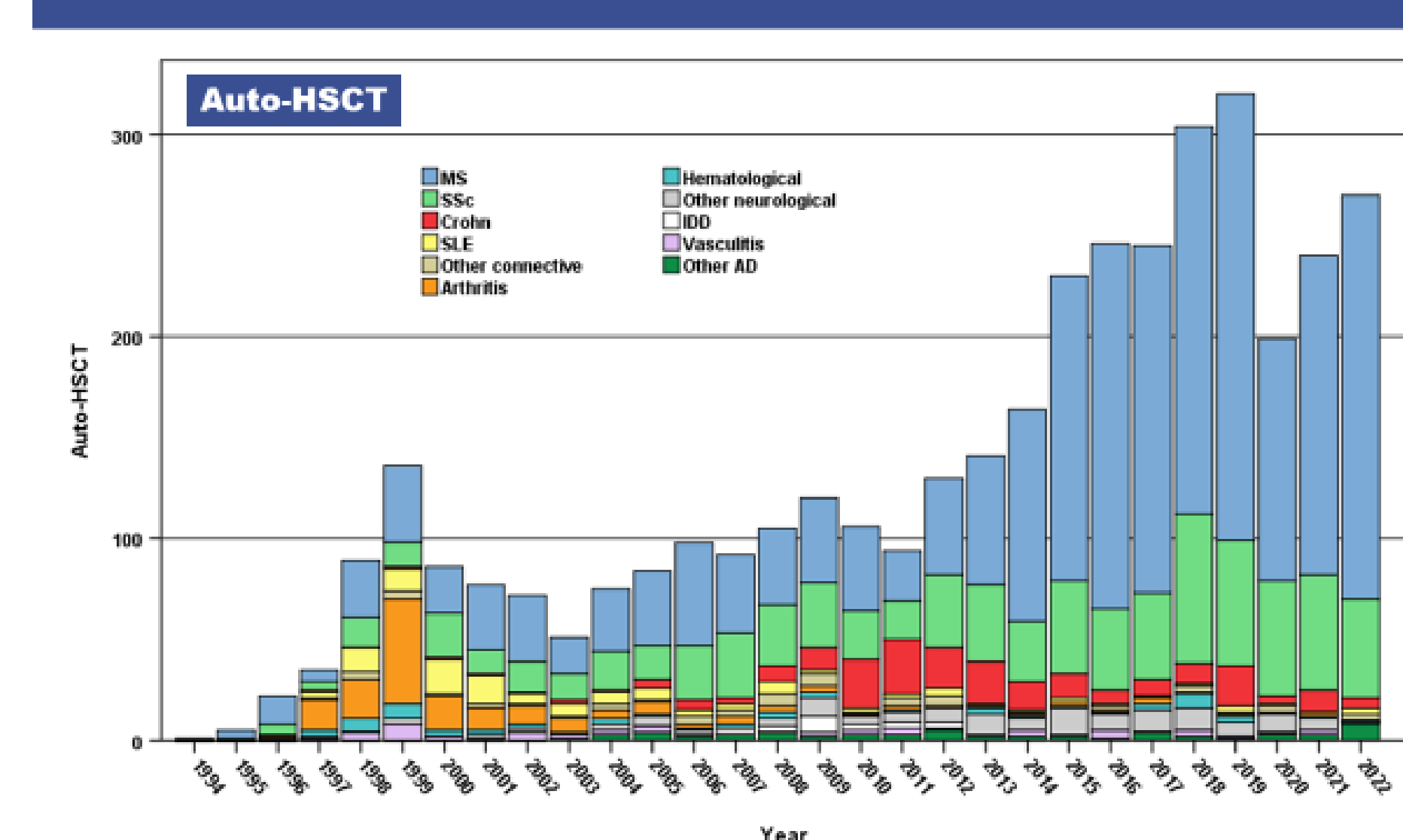
Number of HSCT for Autoimmune Diseases: 4140 EBMT Registry – March 2023

ADWP – Number of HSCT: 4140 EBMT Registry – March 2023	
Transplant procedures	4140
Patients	4055
Male/Female %	40/60
Paediatric/Adult %	9/91
Centres/Countries	323/44
Overall follow up (alive, median)	3y (<1-26)

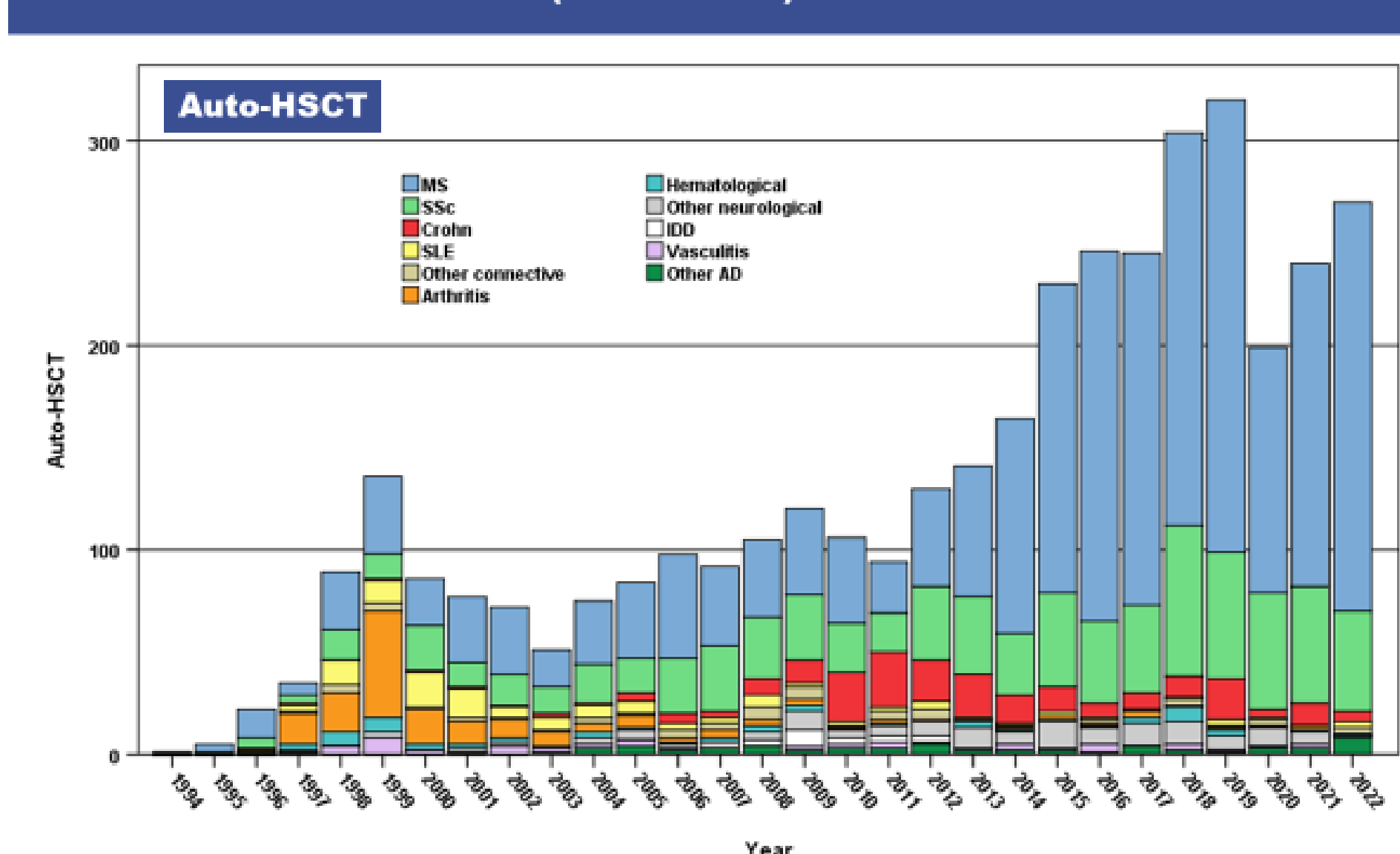
	Autografts n = 3854	Allografts n = 286
First	3818	237
Second	34	42
Third	2	6
Median age at 1st transplant	38y (3-76)	11y (<1-64)

ADWP – Number of HSCT: 4140 EBMT Registry – March 2023	
▶ MULTIPLE SCLEROSIS	2132
▶ CONNECTIVE TISSUE	1028
SSc	835
SLE	126
PM-DM	18
Sjogren	6
Antiphosph. Syndrome	6
Other/Unknown	37
▶ ARTHRITIS	209
Rheumatoid arthritis	82
Juvenile chronic arthritis :	
*Systemic JIA	74
*Articular JIA	22
*Other JIA	19
Psoriatic arthritis	3
Other	9
▶ INFLAMMATORY BOWEL	283
Crohn's disease	233
Coeliac disease	18
Other	32
▶ HAEMATOLOGICAL	161
ITP	42
AIHA	36
Evans'	24
Other	60
▶ VASCULITIS	67
Granulomatosis with Polyangiitis (GPA)	12
Behcet's	18
Eosinophilic GPA	2
Polyarteritis	4
Takayasu	3
Other/Unknown	28
▶ OTHER NEUROLOGICAL	146
NMO	29
CIDP	65
Myasthenia gravis	11
Other/Unknown	41
▶ INSULIN DEPENDENT DIABETES	20
▶ OTHER	94

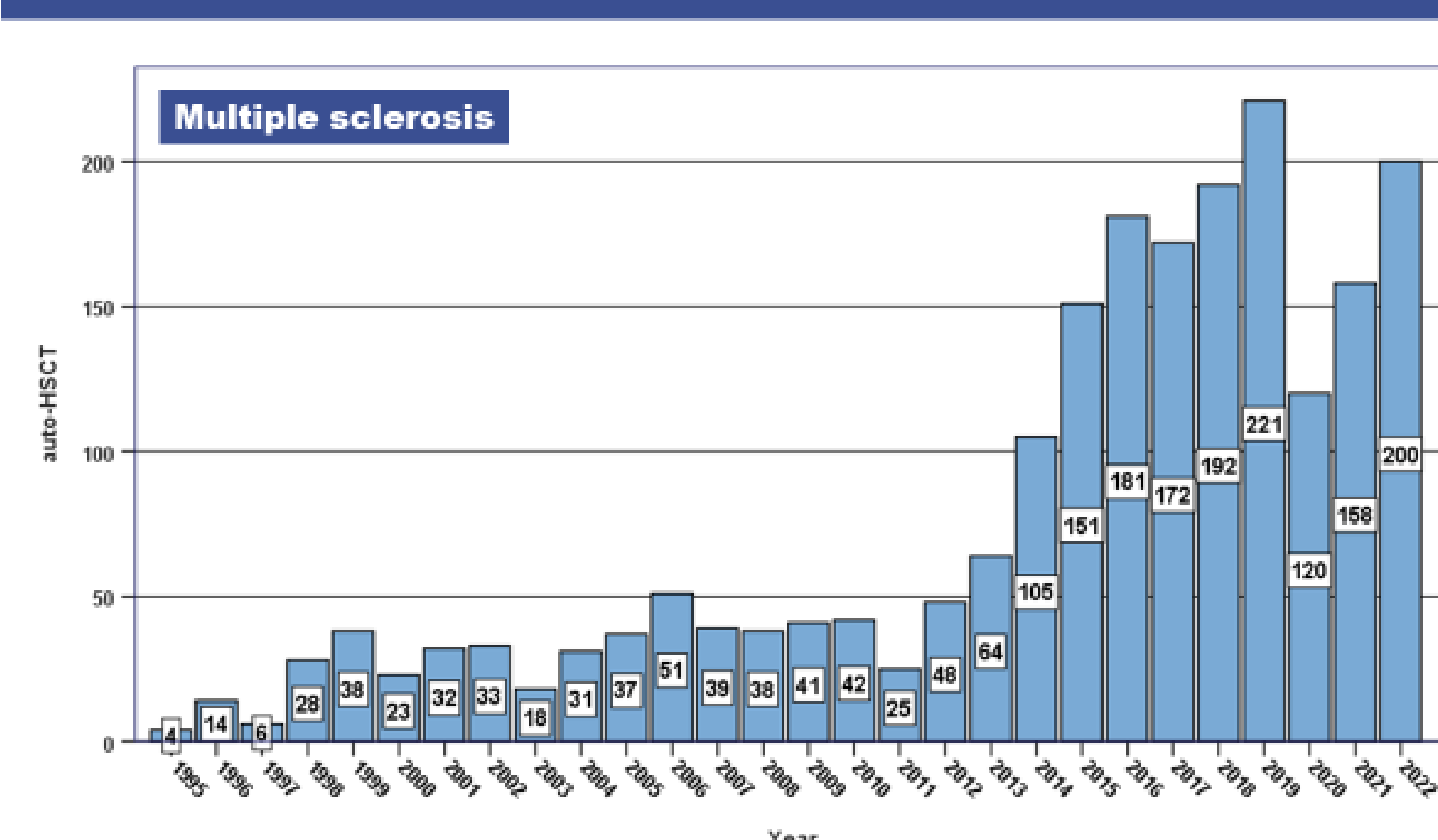
Auto-HSCT for AD: diagnosis per year 1994-2022 (n = 3837) – March 2023



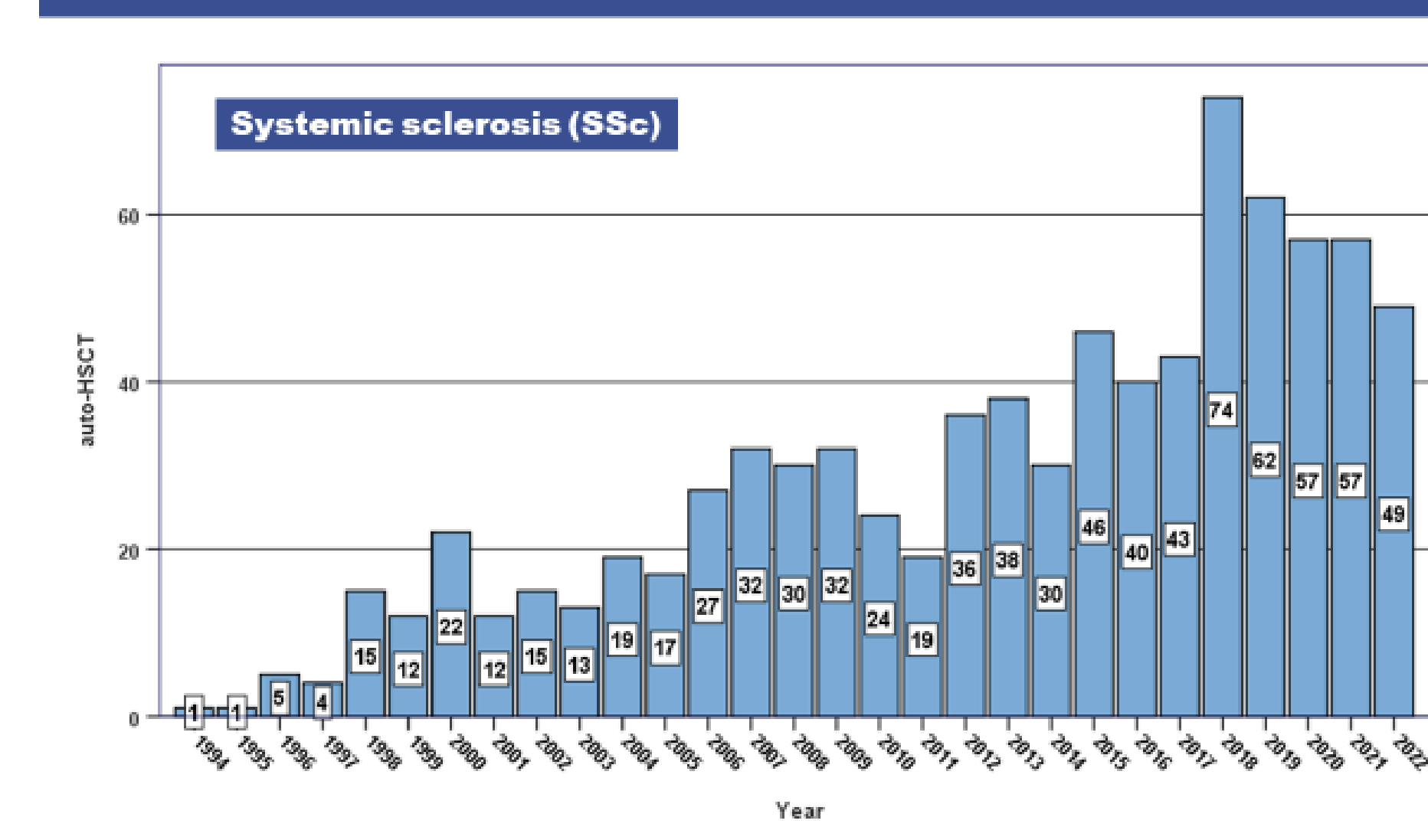
Auto-HSCT for AD: diagnosis per year 1994-2022 (n = 3837) – March 2023



Multiple Sclerosis Auto / year – 1995-2022 (n = 2112) – March 2023



Systemic Sclerosis Auto / year – 1994-2022 (n = 832) – March 2023



Principal research studies

- 1- MS Comparison of CYC+ATG vs. BEAM+ATG conditioning regimens in autologous HSCT for MS
- 2- NISSC II: Post-AHSCT 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
- 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),
- Juvenile and adult systemic arthritis/Still's disease
- Polymyositis-Dermatomyositis

Surveys

- Survey of national provision/reimbursement of HSCT and follow up in Autoimmune Diseases
- Survey of current practice: ATG and other serotherapies in conditioning regimens for autologous HSCT in autoimmune diseases

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/recommendations on behalf of ECTRIMS and EBMT/ADWP

Prospective long-term follow of studies involving EBMT registry

- GoCART project; Autoimmune and autoinflammatory complications after CAR-T cell therapy - a joint study on behalf of the EBMT - ADWP, CTIWP and TCWP

Key publications

1. Autoimmune manifestations in VEXAS: Opportunities for integration and pitfalls to interpretation. Bruno A et al. *J Allergy Clin Immunol.* 2023 Mar 20;S0091-6749(23)00231-2. doi: 10.1016/j.jaci.2023.02.017. Online ahead of print. PMID: 36948992 Review.
2. New insights in systemic lupus erythematosus: From regulatory T cells to CAR-T-cell strategies. Doglio M et al. *J Allergy Clin Immunol.* 2022 Dec;150(6):1289-1301. doi: 10.1016/j.jaci.2022.08.003.
3. Hematopoietic stem cell transplantation and cellular therapies for autoimmune diseases: overview and future considerations from the Autoimmune Diseases Working Party (ADWP) of the European Society for Blood and Marrow Transplantation (EBMT). Alexander T, Greco R. *Bone Marrow Transplant.* 2022 Jul;57(7):1055-1062. doi: 10.1038/s41409-022-01702-w. PMID: 35578014.
4. Immune Reconstitution Following Autologous Hematopoietic Stem Cell Transplantation for Multiple Sclerosis: A Review on Behalf of the EBMT Autoimmune Diseases Working Party. Cencioni MT et al. *Front Immunol.* 2022 Feb 1;12:813957. doi: 10.3389/fimmu.2021.813957. PMID: 35178046
5. Haematopoietic stem cell transplantation for severe autoimmune diseases in children: A review of current literature, registry activity and future directions on behalf of the autoimmune diseases and paediatric diseases working parties of the European Society for Blood and Marrow Transplantation. Achini-Gutzwiller FR et al. *Br J Haematol.* 2022;198:24-45. <https://doi.org/10.1111/bjh.18176>
6. Textbook 'Hematopoietic Stem Cell Transplantation and Cellular Therapies for Autoimmune Diseases' (edited by Burt R, Farge D, Ruiz MA, Saccardi R, Snowden JA): <https://www.routledge.com/Hematopoietic-Stem-Cell-Transplantation-and-Cellular-Therapies-for-Autoimmune/Burt-Farge-Ruiz-Saccardi-Snowden/p/book/9781138558557>

Major achievements

Over the last 25 years, hematopoietic stem cell transplantation (HSCT) has been increasingly used to treat patients affected by severe and refractory autoimmune diseases (ADs). HSCT for ADs is recently facing a unique developmental phase across EBMT centres. Autologous HSCT has become an integral and standard-of-care part of treatment algorithms in multiple sclerosis (MS) and systemic sclerosis (SSc). Recently, novel cellular therapies (i.e. CAR-T cells, mesenchymal cells) have been successfully adopted in ADs.

The EBMT Autoimmune Diseases Working Party (ADWP) has been central to development of these approaches (Fig 1). The ADWP is dedicated to promoting clinical activities, teaching and translational research on autologous/allogeneic HSCT together with novel approaches of cellular therapy as specific treatment of severe ADs, in a specific challenge 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 4,100 HSCT registrations for ADs (Dec 2022, Fig 2).

A multidisciplinary approach is key in this field, and 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, Trainee-Committee and the Nurses-Group.

Education is central in ADWP activities, including a successful ADWP Educational Meeting in London, together with a workshop on autologous HSCT in MS jointly done with ECTRIMS. Moreover, a section specifically designed for ADs was developed within the EBMT e-learning platform, including a recent webinar on 'autologous HSCT in MS' with key experts in the field.