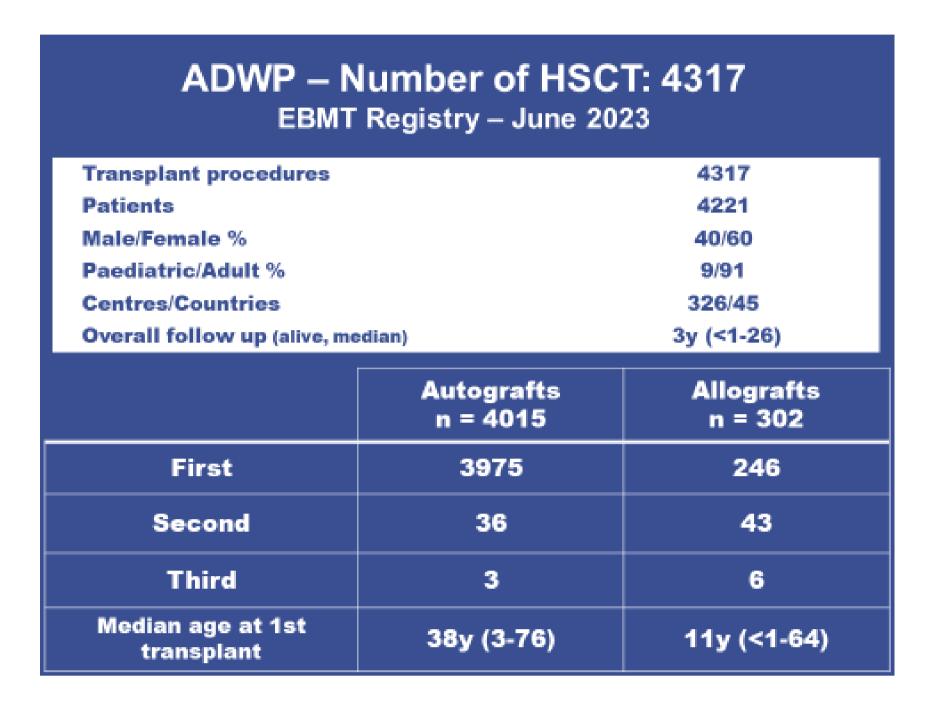


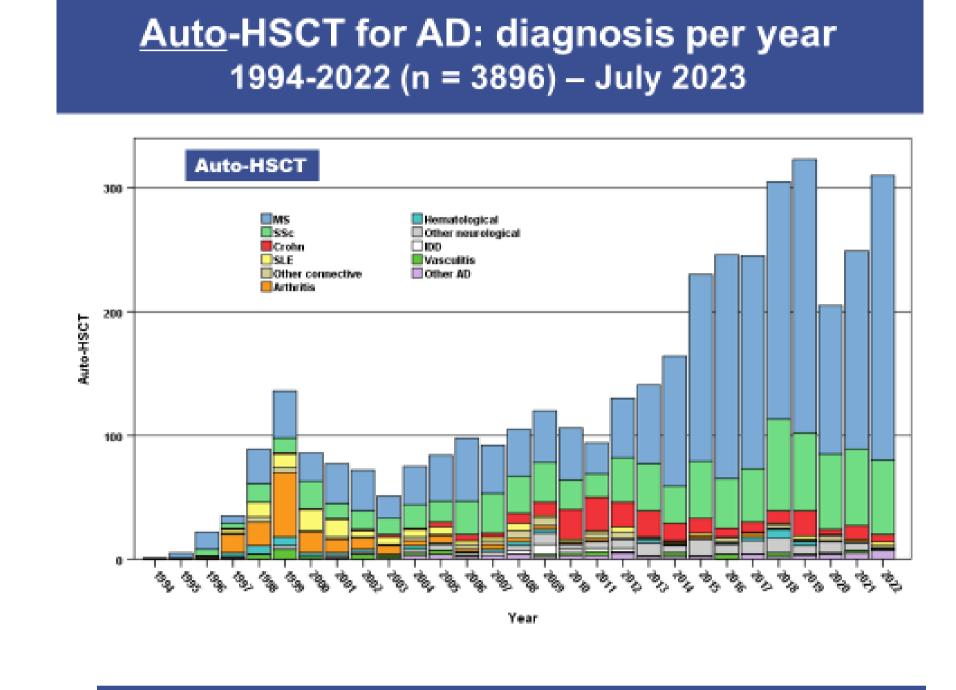
# Autoimmune Diseases Working Party

Raffaella Greco, ADWP Chair Tobias Alexander, ADWP Secretary Manuela Badoglio, ADWP Study Coordinator Ariadna Domènech, Nurse representative

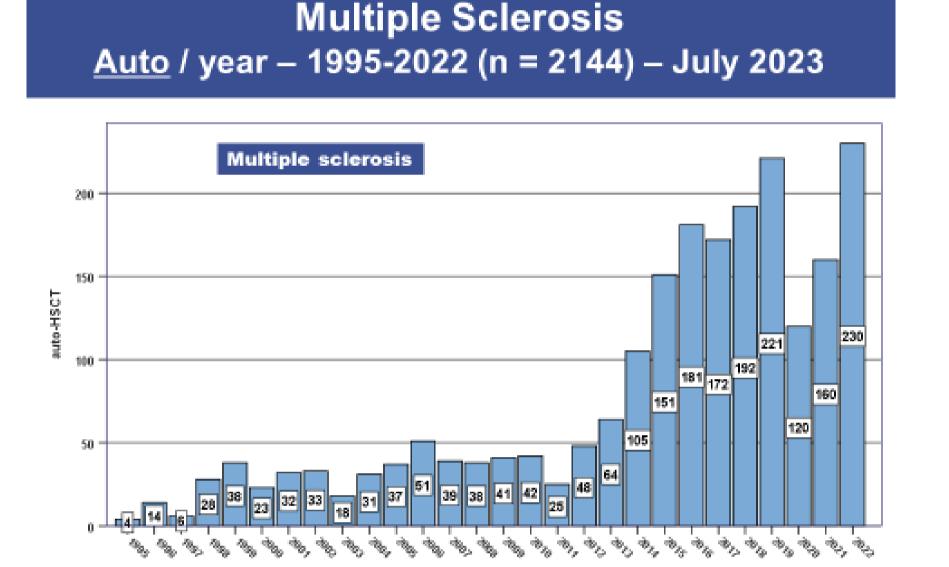
# Number of HSCT for Autoimmune Diseases: 4317 EBMT Registry

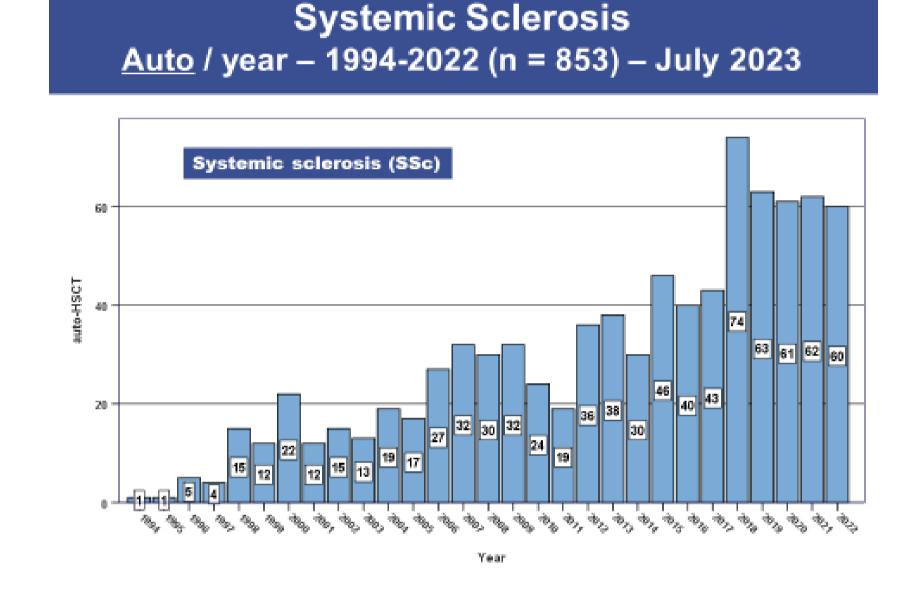


### ADWP – Number of HSCT: 4317 EBMT Registry - July 2023 2240 ► HAEMATOLOGICAL ► MULTIPLE SCLEROSIS 166 **▶** CONNECTIVE TISSUE PM-DM 6 ► VASCULITIS 67 Sjogren Granulomatosis with Polyangitis (GPA) 12 Antiphosph. Syndrome Other/Unknown ARTHRITIS 212 Eosinophilic GPA Polyarteritis 4 6 1 Rheumatoid arthritis Juvenile chronic arthritis: Takayasu 76 Other \*Systemic JIA 153 \*Articular JIA 22 ► OTHER NEUROLOGICAL NMO 30 \*Other JIA CIDP Psoriatic arthritis Myasthenia gravis ▶ INFLAMMATORY Other BOWEL Crohn's disease 236 ► INSULIN DEPENDENT DIABETES 20 18 ► OTHER 104 Coeliac disease 32



# Auto-HSCT for AD / Country 1994-2023 (n = 4015) – July 2023





### Principal research studies

- MS 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
- Late complications after autologous HSCT for Autoimmune Diseases
- Viral reactivations: retrospective study on viral infections post auto-HSCT in Autoimmune Diseases
- Prospective non-interventional on patients with multiple sclerosis (OMST).

## Retrospective studies on autologous HSCT

- Immune cytopenias
- Rare neurological diseases (CIDP, Stiff Person Syndrome, myasthenia gravis, NMO and others),
- Adult onset Still's disease (AOSD)
- Polymyositis-Dermatomyositis
- Indications and outcomes of re-transplantation for autoimmune diseases

### Surveys

- Survey of current practice: ATG and other serotherapies in conditioning regimens for autologous HSCT in autoimmune
- Definition of relapse in SSc after HSCT (DELPHI panel)

### 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

### 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.

## **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 (HCT) 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 HCT 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 HCT 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 a successful joint Midterm Educational Meeting on New indications in Immune Dysregulatory, Autoinflammatory and Autoimmune Diseases, and Harmonization Workshop on innovative cellular therapies for ADs. Moreover, a section specifically designed for ADs was developed within the EBMT e-learning platform with contributions from key experts in the field.

### **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. Outcome of SARS-CoV2 infection in hematopoietic stem cell transplant recipients for autoimmune diseases. Greco R, et al; on behalf of the EBMT COVID19 Task Force, ADWP and IDWP. J Autoimmun. 2023 Apr; 136:103024. doi:10.1016/j.jaut.2023.103024.
- 3. Innovative cellular therapies for autoimmune diseases: expert-based position statement and clinical practice recommendations from the EBMT practice harmonization and guidelines committee. Greco R et al. eclinical Medicine Journal, 2024.
- 4. 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.
- 5. 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.
- 6. 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