EBMT Appual re

Annual report

<u>2024</u>



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Tobias Alexander ADWP Chair (ELECTED APRIL 2024, PREVIOUS CHAIR: Raffaella Greco) Germany

Major achievements

For autoimmune diseases (ADs) not sufficiently responding to state-of-the-art immunosuppressive or biologic therapies, haematopoietic stem cell transplantation (HSCT) has been successfully implemented as treatment option in carefully selected patients, with endorsement as standard indication for systemic sclerosis and multiple sclerosis from international societies, such as EULAR (Del Galdo et al., *Ann* *Rheum Dis.* 2024) and ECTRIMS (Muraro et al., *Nat Rev Neurol.* 2025). The EBMT Autoimmune Diseases Working Party (ADWP) has been central to development of these approaches. Currently, 4656 patients have been reported to the EBMT registry (Fig. 1).



Figure 1: Auto-HSCT cases reported to the EBMT Registry

Recent achievements from our working party include a position paper developed in collaboration the Patient Advocacy Committee (PAC) and Nurses Group of the EBMT on **patient related outcome parameters to be considered in HSCT recipients for ADs** (Alexander et al., *J Allergy Clin Immunol Glob*. 2024). Furthermore, we conducted a **survey on the centre policy on ATG usage** (Ismail et al., *Bone Marrow Transplant*. 2024) as well as **monitoring and managing viral reactivations post-transplantation for ADs** (Alexander et al., *Bone Marrow Transplant*. 2025). In cooperation with the EBMT Chronic Malignancies Working Party, we retrospectively analysed the **outcome of patients with VEXAS syndrome undergoing allogeneic HSCT** (Gurnari et al., *Blood Advances*. 2024).

Recently, innovative cellular therapies (i.e. CAR-T cells, mesenchymal cells, Tregsbased therapies) have been successfully adopted in ADs. To this end, we published an expert-based position statement and clinical practice recommendations from the EBMT Practice Harmonisation and Guidelines Committee on **innovative cellular therapies for ADs** (Greco et al, *EClinicalMedicine*. 2024) and provided an update

on the role of Chimeric Antigen Receptor (CAR) T-Cell Therapy in immunemediated neurological diseases (Brittain et al., Ann Neurol. 2024).

The ADWP is dedicated to promoting clinical activities, teaching and translational research on HCT together with innovative cellular therapies as AD treatment. We continued to expand the evidence-base and support best-practice with studies and guidelines, including significant collaborative outputs with the 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 (Fig 2.), and a harmonisation workshop on HSCT 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.



Joint Educational Meeting of the ADWP and CTIWP - Milan, Italy - 10-12 October 2024

Principal research studies

Comparison of Cyclophosphamide/ATG vs BEAM/ATG conditioning regimens in autologous HSCT for Multiple Sclerosis Study type Retrospective Studies Diseases Auto immune disorders Group Autoimmune Diseases Working Party (ADWP) Type of treatment Autologous Principal investigator Riccardo Saccardi Post AHSCT (Autologous Hematopoietic Stem Cell Transplantation) management for patients with systemic sclerosis: a prospective, non-interventional approach across Europe (NISSC-2) Study type Non-interventional Prospective Studies Diseases Group Autoimmune Diseases Working Party (ADWP) Type of treatment Autologous Principal investigator Pr Dominique Farge Observational Study in Multiple Sclerosis patients treated with autologous hematopoietic stem cell Transplantation (OMST) Study type Non-interventional Prospective Studies Diseases Auto immune disorders Group Autoimmune Diseases Working Party (ADWP) Type of treatment Autologous Principal investigator Riccardo Saccardi Incidence and outcome of CMV and EBV reactivations after autologous HSCT for autoimmune diseases: a retrospective survey from the Autoimmune Diseases Working Party (ADWP) of the European Society for Blood and Marrow Transplantation (EBMT) Study type **Retrospective Studies** Diseases Auto immune disorders Group Autoimmune Diseases Working Party (ADWP) Type of treatment

Principal investigator

Tobias Alexander CAR-T Cell Therapy in Autoimmune Diseases as Concomitant or Main Indication: a 2step Joint Study on behalf of EBMT ADWP and CTIWP Study type Closed Survey/Study Diseases Auto immune disorders Group Autoimmune Diseases Working Party (ADWP) Type of treatment CAR T Principal investigator Raffaella Greco

Key publications

2024 Innovative cellular therapies for autoimmune diseases: expert-based position statement and clinical practice recommendations from the EBMT practice harmonization and guidelines committee Group Autoimmune Diseases Working Party (ADWP) Practice Harmonisation and Guidelines Committee 1st listed author Raffaella Greco Journal EClinicalMedicine. 2024 Allogeneic hematopoietic cell transplantation for VEXAS syndrome: results of a multicenter study of the EBMT Group Autoimmune Diseases Working Party (ADWP) Chronic Malignancies Working Party (CMWP) 1st listed author Carmelo Gurnari Journal Blood Adv. 2024

The Role of Chimeric Antigen Receptor T-Cell Therapy in Immune-Mediated **Neurological Diseases** Group Autoimmune Diseases Working Party (ADWP) 1st listed author **Gavin Brittain** Journal Ann Neurol. 2024 ATG and other serotherapy in conditioning regimens for autologous HSCT in autoimmune diseases: a survey on behalf of the EBMT Autoimmune Diseases Working Party (ADWP) Group Autoimmune Diseases Working Party (ADWP) 1st listed author Azza Ismail Journal Bone Marrow Transplant. 2024 Patient-reported outcomes in HSCT for autoimmune diseases: Considerations on behalf of the EBMT ADWP, PAC, and Nurses Group Group Autoimmune Diseases Working Party (ADWP) Patient Advocacy Committee Nursing Research Committee **Nursing Scientific Committee** 1st listed author **Tobias Alexander** Journal J Allergy Clin Immunol Glob. See the full list of the ADWP 2024 publications



Indicators



Oral presentations	22	16	8
Poster presentations	1	4	1
International educational events	3	3	2

Major educational events



<u>Event</u>

50th Annual Meeting of the EBMT



Kingdom



9th International Transplant and Cellular Therapy Course



<u>Event</u>

Immunology and Cellular Therapies in Autoimmune Diseases

Oct 10, 2024 - Oct 12, 2024 / Milan, Italy Discover more

Webinars and e-courses

HSCT in Autoimmune Diseases E-Course Panel Discussion: Autologous HSCT in Multiple Sclerosis VISIT THE ADWP WEBPAGE