Autoimmune Diseases Working Party
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Number of HSCT for Autoimmune Diseases: 4140
EBMT Registry – 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 = 632) – March 2023

Principal research studies
1. MS Comparison of CYC-ATG vs. BEAM-ATG conditioning regimens in autologous HSCT for MS
2. NISSC P: 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 (OMIST).

Disease-specific retrospective studies of autologous HSCT
- Immune cytophenias
- 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 ReCONNECT).
- 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, CTINP and TCWP

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.

Key publications