Autoimmune Diseases Working Party

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Number of HSCT for Autoimmune Diseases: 3372
EBMT Registry – August 2020

ADWP - Number of HSCT: 3372
EBMT Registry – August 2020

Transplant procedures	3372
Patients	3306
Male/Female %	40/60
Paediatric/Adult %	9/91
Centres/Countries	305/44
Overall follow up (alive, median, Yr HSCT < 2017)	4y (<1-24)

	Autografts n=3151	Allografts n=221
First	3122	184
Second	27	31
Third	2	6
Median age at 1st transplant	38y (3-76)	11y (<1-64)

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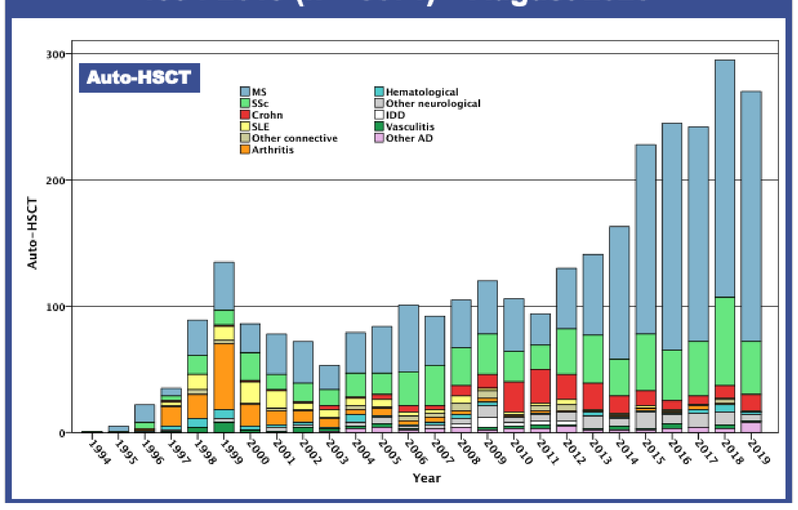
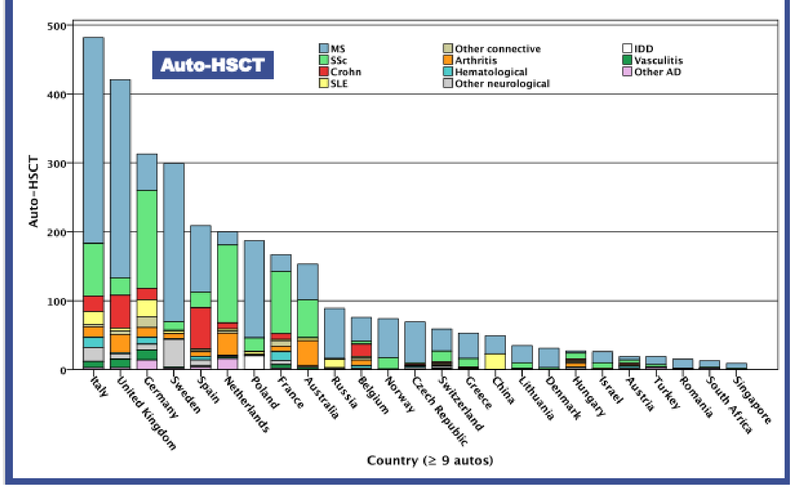
▶ MULTIPLE SCLEROSIS	1671	▶ HAEMATOLOGICAL	131
▶ CONNECTIVE TISSUE	834	ITP	37
SSc	658	AIHA	30
SLE	118	Evans'	26
PM-DM	18	*Other*	38
Sjogren	4	▶ VASCULITIS	63
Antiphosph. Syndrome	6	Wegener's	14
Other/Unknown	30	Behcet's	13
▶ ARTHRITIS	193	Takayasu	3
Rheumatoid arthritis	82	Polyarteritis	4
Juvenile chronic arthritis :		Churg-Strauss	2
**Systemic JIA*	64	*Other/Unknown*	27
**Other JIA*	19	▶ OTHER NEUROLOGICAL	128
**Polyarticular JIA*	17	NMO	26
Psoriatic arthritis	3	CIDP	60
Other	8	Myasthenia gravis	10
▶ INFLAMMATORY BOWEL	252	*Other/Unknown*	32
Crohn's disease	206	▶ INSULIN DEPENDENT DIABETES	20
Ulcerative colitis	4	▶ OTHER	80
Other	42		

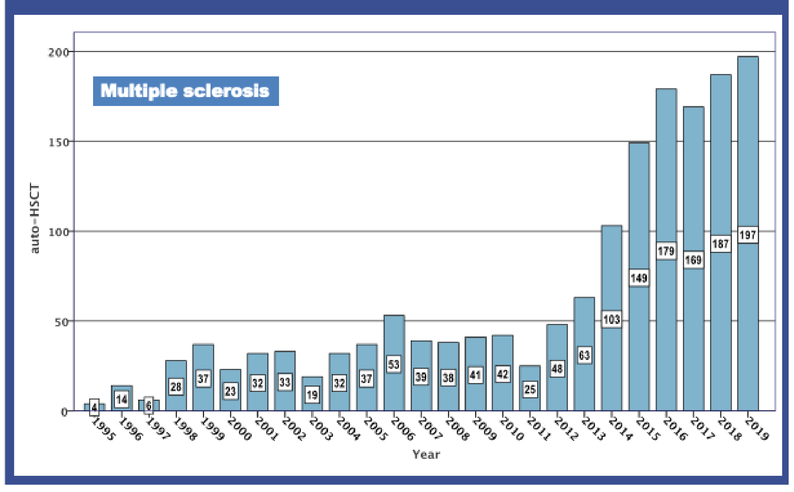
Auto-HSCT for AD: diagnosis per year
1994-2019 (n = 3071) – August 2020



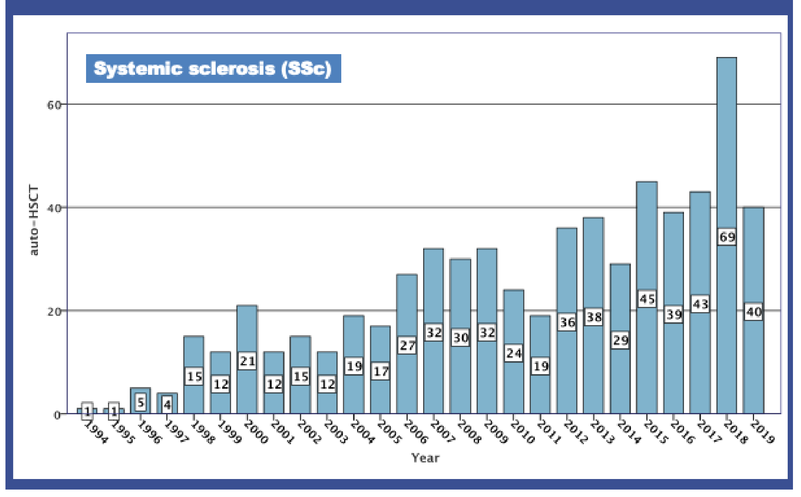
Auto-HSCT for AD / Country
1994-2019 (n = 3071) – August 2020



Multiple sclerosis
Auto-patient /year- 1995/2019 (n=1598) – August 2020



Systemic sclerosis
Auto-patient / year- 1995/2019 (n= 637) – August 2020



Principal Research Studies

1. **NISSC-2: Post AHSCT management and mechanistic immunological reconstitution for patients with systemic sclerosis.** *Ongoing*
2. **Comparison of Cyclo+ATG vs. BEAM+ATG conditioning regimens in autologous HSCT for multiple sclerosis.** *Data collection*
3. **Late complications after autologous HSCT for ADs.** *Ongoing*
4. **Incidence and outcome of CMV and EBV reactivations after autologous HSCT for autoimmune diseases.** *Preparation*
5. **Prospective non-interventional on patients with multiple sclerosis (OMST).** *On hold*
6. **Autologous SCT for polymyositis-dermatomyositis.** *Preparation*
7. **Autologous HSCT for Behcet disease.** *Ongoing*
8. **HSCT for Takayasu diseases.** *Manuscript.*
9. **sHLH EMBT Survey.** *Closed*
10. **Outcomes of HSCT for autoimmune cytopenias.** *Preparation*
11. **Autologous SCT for CIDP, SPS, NMO MG and other immune mediated neurological diseases.** *Preparation*
12. **Autologous SCT for myositis**. *Preparation*

Major Achievements

- Autoimmune diseases (ADs) continue to be the fastest growing indication for autologous HSCT across EBMT, with the ADWP central to bringing together HCT and disease specialist communities. The Autoimmune Diseases section of the EBMT Registry is the largest database of its kind worldwide and this year exceeded the landmark of 3,000 transplants, with registered activity being the highest ever. In 2019, the ADWP continued to expand the evidence-base and support best practice with registry-based studies and guidelines, including significant collaborative outputs with other EBMT Working Parties, JACIE, the EBMT Nurses Group and Patient Advocacy Committee.
- Education continued to be central to global ADWP activities in 2019. In November, the ADWP educational meeting in Berlin attracted the greatest number of delegates ever for a single ADWP meeting, reflecting evolving interest in the field across all disciplines, with a repeat already planned for September 2020 in London. In addition, we continued to build closer links with partners outside EBMT, particularly in the Americas and Russia.
- 'Implementation science' remains central to delivery of HCT for AD within our health services. The future depends on quality of outcomes and health economics versus non-transplant biological treatments, and ADWP activity has also focussed on these aspects. Updated EBMT recommendations for HSCT and cell therapy in neurological diseases provided a major impetus and resource for clinicians and health service providers. Strategic priorities for the ADWP include ongoing work with disease specialist societies (such as the European Academy of Neurology/ECTRIMS, EULAR and ECCO), whilst working closely with others within EBMT and JACIE to assure best practice, clinical quality and patient-centred care in HCT for ADs.

Key Publications

- **Diagnosis and Management of Secondary HLH/MAS Following HSCT and CAR-T Cell Therapy in Adults; A Review of the Literature and a Survey of Practice Within EBMT Centres on Behalf of the ADWP and TCWP.** Sandler. R et al. (2020). *Front Immunol. Mar 31;11:524*
- **Evaluating the clinical effectiveness of autologous HSCT versus disease-modifying therapy in multiple sclerosis using a matching-adjusted indirect comparison: an exploratory study from the ADWP EBMT .** Tappenden P et al. (2020). *Bone Marrow Transplant. Jul;55(7***):1473-1475**
- **Autologous HSCT with reduced-intensity conditioning regimens in refractory Takayasu arteritis: a retrospective multicenter case-series from the ADWP of the EBMT.** Laurent C et al. (2020). *Bone Marrow Transplant. Apr 22. [Epub].*
- **Autologous HSCT for autoimmune diseases : Current indications and mode of action, a review on behalf of the EBMT ADWP.** Alexander T et al. (2020). *Rheumatol. Jun;79(5):419-428.*
- **Autologous HSCT and other cellular therapy in multiple sclerosis and immune-mediated neurological diseases: updated guidelines and recommendations from the EBMT ADWP and JACIE and ISCT.** Sharrack, B et al. (2020). *Bone Marrow Transplant. 55, 283-306.*
- **Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia: a joint study of the ADWP/SAAWP of the EBMT.** Miller, P.D.E et al. (2020). *Bone Marrow Transplant. 55, 441-451.*
- **Autologous HSCT for progressive systemic sclerosis: a prospective non-interventional study across Europe (NISSC).** Henes, J et al. (2020). *Haematologica Jan 16;haematol.2019.230128.*
- **Autologous HSCT for ANCA-associated vasculitis: a retrospective survey of patients reported to EBMT registry.** Alexander, T et al. (2020). *Bone Marrow Transplant Jul;55(7):1512-1515.*