



EBMT

European Society
for Blood and Marrow Transplantation

ANNUAL
REPORT
**20
19**

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Nicolaus Kröger
EBMT President

Foreword by the EBMT President

As president of our society I am very proud and honored to present the EBMT activity report summarising the amazing achievements of our society in 2019.

In our annual report you can follow the scientific and educational activities of our working parties and nurses' group as well as the different committees over the last twelve months, as evidenced by many achievements including an increasing number of publications in high rank peer reviewed journals and cutting-edge educational events. Together these activities reflect once again this year, EBMT's pioneering role in promoting and advancing the field of stem cell transplantation and cellular therapies.

By adhering to our EBMT educational strategy, in 2019 we increased our educational activities and prioritised our efforts and human resources into educational events.

In addition to the numerous educational events of the working parties in 2019, the newly established mid-size events such as the well-attended and successful EBMT GvHD summit in Warsaw and the international transplant course (ITC) in Barcelona reflect this strategy. In January we performed together with the European Hematology Association (EHA) an extremely successful and important 1st European CAR-T Cell Meeting in Paris, which with more than 600 attendees was "sold out" after only a few weeks after its announcement. The 2nd European CAR-T Cell Meeting in Sitges in early 2020, with more than 1,000 registrations, received similar attractiveness, demonstrating also our leading educational role in cell and gene therapy.

Thanks to the support of DKMS we could launch the e-learning platform and in 2020 several webinars and online learning modules will be released to improve knowledge of all health care providers working in the field of stem cell transplantation and cellular therapies. The new EBMT Handbook was launched in 2019 and has become a new standard in the field of stem cell transplantation where it can be used as an optimal preparation process in order to pass the first EBMT exam, which will be launched at EBMT's Annual Meeting in Madrid and will provide a personal qualification for physicians working in the field of stem cell transplantation and cellular therapy.

The 45th Annual Meeting of EBMT in Frankfurt was one of most successful and record-breaking EBMT congresses with more than 5,600 attendees from 91 countries and up-to-date and cutting-edge presentations about stem cell transplantation and cellular therapies. The inclusion of the

Patient, Family and Donor Day into the congress as well as the launch of the 1st Transplant Coordinator Day was very well received and was based on our strategy to provide patients with a voice in a patient-focused society as well as including all health care providers involved in stem cell transplantation and cellular therapies.

Early in 2019, the EBMT registry received a regulatory qualification from the European Medicine Agency (EMA) on the use of its patient registry to support novel CAR-T cell therapies which support our efforts to register all CAR-T cell and other immune effector cell treatments in our registry.

In order to collect all CAR-T cell procedures in Europe in our registry, we contacted national study groups, medical societies, health authorities and pharmaceutical industry to work on a governance structure, which will provide access to CAR-T cell results to all stakeholders.

In addition, in January 2020, EBMT announced a new collaboration with Novartis Pharmaceuticals to study the long-term outcomes of patients treated with CAR-T therapies.

Beside these achievements, we are still facing with the underestimated workload of data migration from ProMISe to the new registry system MACRO and for this reason we were not able to launch the new system in 2019. Nevertheless, I am very optimistic that we will solve this issue very soon.

As you can see and read on the following pages, 2019 has been a year of remarkable and amazing progress in many aspects and fields and I would like to express my deep gratitude to all who contribute to the fabulous activities of our society in 2019 including the centers of EBMT in Europe and around the world, who report patient outcome data to our registry.

Please enjoy reading the annual report and continue your commitment to EBMT in 2020. Please give us also any feedback and try to attend the different working party business meetings or educational events and contribute actively as part of our society and by moving the field forward in order to save more life of our patients.

Nicolaus Kröger

About the European Society for Blood and Marrow Transplantation

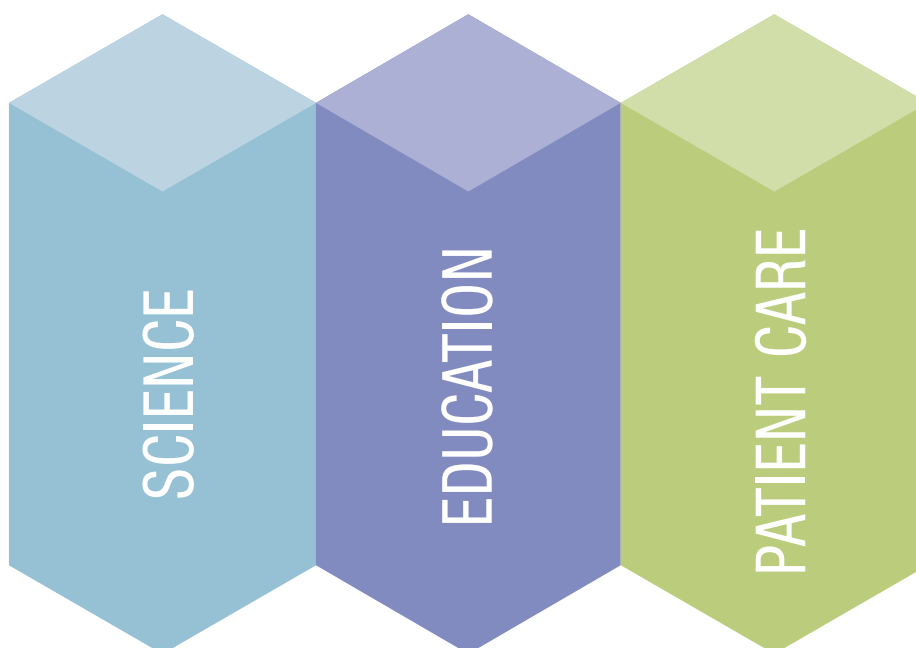
The EBMT is a not-for profit medical and scientific organisation established in 1974.

It is dedicated to fighting life-threatening blood cancers and diseases and improving patients' lives.

EBMT members—more than 5,000 physicians, nurses, scientists and other healthcare professionals—participate in a unique collaborative network of peers involved in haematopoietic stem cell transplantation (HSCT) and cellular therapy research. Membership encompasses more than 550 centres from over 70 countries, that perform or are involved in HSCT.

The EBMT holds a central role in performing co-operative studies and disseminating state-of-the-art knowledge: the aim is to increase survival rates and enhance the quality of life of patients with life-threatening blood cancers and diseases.

THE EBMT IS BUILT ON 3 PILLARS



EBMT STRUCTURE

BOARD

1. **Executive Committee, President**
Nicolaus Kröger
University Hospital Eppendorf
Hamburg, Germany
2. **Executive Committee, Secretary**
Rafael Duarte
Hospital Universitario Puerta de Hierro
Madrid, Spain
3. **Executive Committee, Treasurer**
Harry Dolstra
Radboud University Medical Centre
Nijmegen, Netherlands
4. **Scientific Council Chair with Research and Sciences Portfolio**
Mohamad Mohty
Hospital Saint Antoine
Paris, France
5. **Scientific Council Vice-Chair with Research and Sciences Portfolio**
Christian Chabannon
Institut Paoli Calmettes
Marseille, France
6. **Scientific Council Representative with the Registry Portfolio**
John Snowden
Sheffield Teaching Hospitals NHS Trust
Sheffield, UK
7. **Scientific Council Representative with the Education Portfolio**
Grzegorz W. Basak
Medical University of Warsaw
Warsaw, Poland
8. **Nurse Group President**
John Murray
Christie NHS Trust Hospital
Manchester, UK
9. **EBMT 2020 Annual Meeting President**
Rafael Duarte
Hospital Universitario Puerta de Hierro
Madrid, Spain

SCIENTIFIC COUNCIL -

WORKING PARTIES

10. **Severe Aplastic Anaemia**
Régis Peffault de Latour
Hospital Saint Louis
Paris, France
11. **Autoimmune Diseases**
John Snowden
Sheffield Teaching Hospitals NHS Trust
Sheffield, UK
12. **Acute Leukaemia**
Mohamad Mohty
Hospital Saint Antoine
Paris, France
13. **Cellular Therapy & Immunobiology**
Christian Chabannon
Institut Paoli Calmettes
Marseille, France

14. **Infectious Diseases**
Jan Styczynski
University Hospital, Collegium Medicum
UMK
Bydgoszcz, Poland
15. **Inborn Errors**
Arjan Lankester
Leiden University Hospital
Leiden, The Netherlands
16. **Lymphoma**
Silvia Montoto
St. Bartholomew's and The Royal London
NHS Trust
London, UK
17. **Paediatric Diseases**
Selim Corbacioglu
Children's Hospital Regensburg
Regensburg, Germany
18. **Chronic Malignancies**
Ibrahim Yakoub-Agha
Lille Hospital
Lille, France
19. **Transplant Complications**
Grzegorz W. Basak
Medical University of Warsaw
Warsaw, Poland

COMMITTEES

20. **Nuclear Accident**
Ray Powles
Cancer Center London
London, UK
21. **Statistical**
Simona Iacobelli
Hospital Saint Antoine
Paris, France
22. **JACIE**
John Snowden
Sheffield Teaching Hospitals NHS Trust
Sheffield, UK
23. **Donor Outcomes**
Nina Worel
Medical University of Vienna
Vienna, Austria
24. **Registry**
Per Ljungman
Karolinska University Hospital
Stockholm, Sweden
25. **Global**
Norbert-Claude Gorin
Hospital Saint Antoine
Paris, France
26. **Legal & Regulatory Affairs (LRAC)**
Jürgen Kuball
UMC Utrecht Utrecht,
Netherlands
27. **Pharmacist**
Tiene Bauters
Paediatric Clinic Prinses Elisabeth
Gent, Belgium
28. **Patient, Family and Donor**
Bregje Verhoeven
Hematopatient organisation
Utrecht, The Netherlands

1



2/9



3



4/12



5/13



6/11/22



7/19



8



10



14



15



16



17



18



20



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23



24



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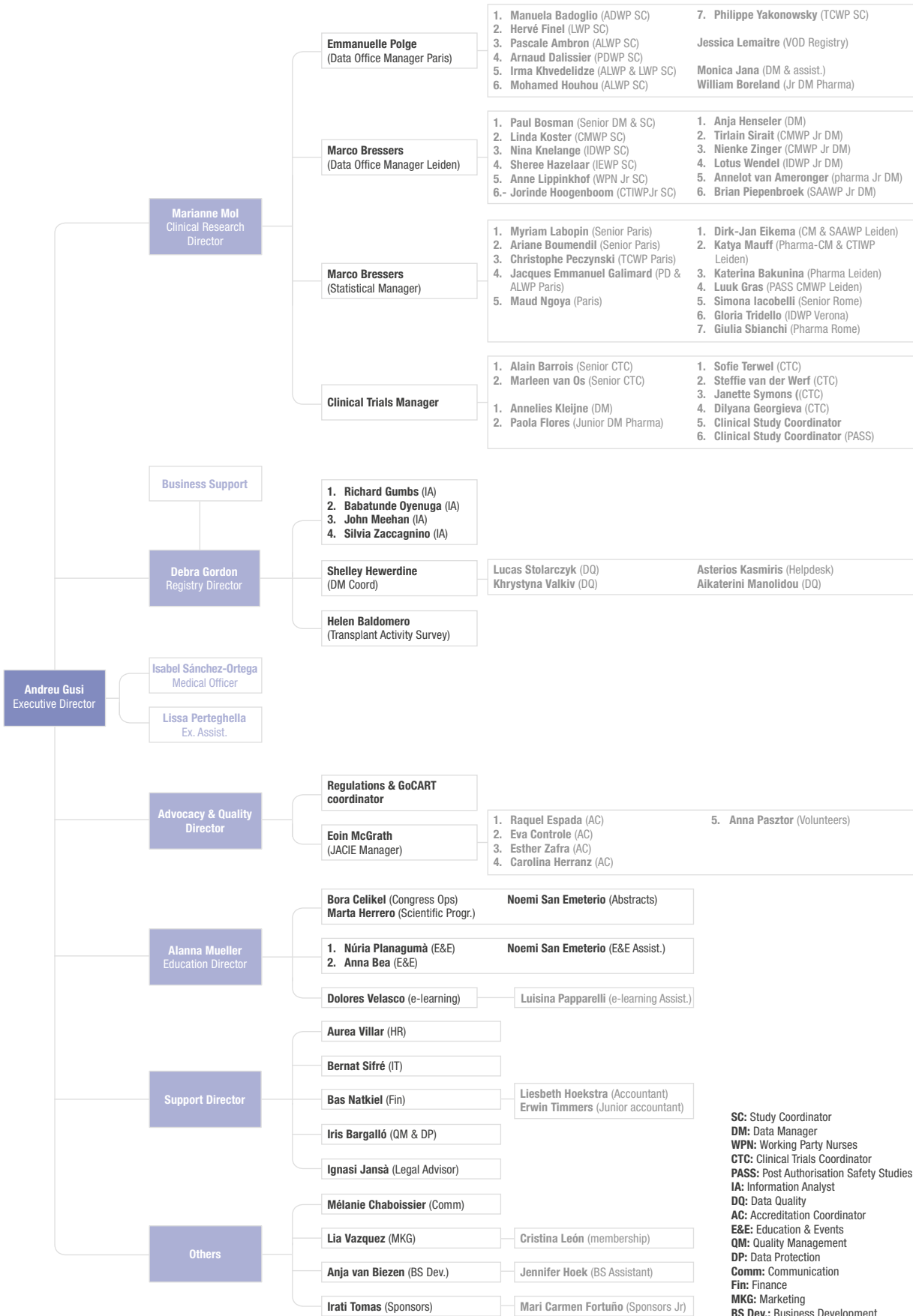
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28



STAFF ORGANISATIONAL CHART



EBMT MEMBERSHIP

The members belong to a unique collaborative network of peers involved in haematopoietic stem cell transplantation and cellular therapy research. They all have a common goal, and that is to enhance the quality of life of patients with life-threatening blood cancers and diseases.

Our members are listed according to their role within their team. They are comprised of the following distribution of roles:

Our members can be classified as centre members (full or associate); individual or provisional members.



Physicians
2988



Students
1



Nurses
905



Statisticians
4



Data Managers
924



Psychologists/Psychiatrists
3



Laboratory technicians
177



Scientists
11



Quality Managers
283



Patient Advocates
2



Pharmacists
20

Other
112



Transplant Coordinators
24

TOTAL MEMBERS:
5454



FULL centres reporting*
505

ASSOCIATE centres
54

INDIVIDUAL members
144

PROVISIONAL members
(including centres and individuals)**
18

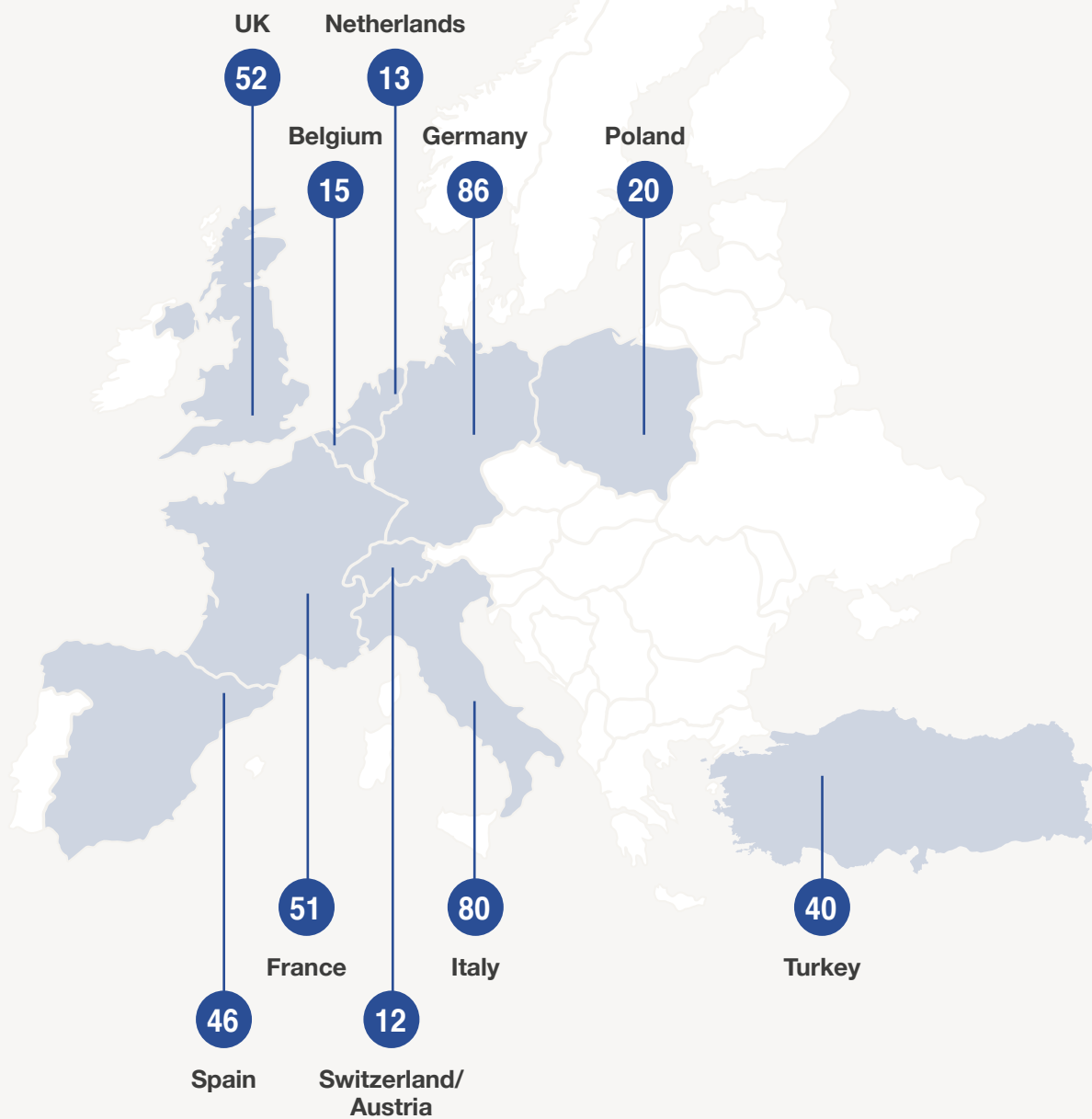
TOTAL CENTERS:
577

* Commit to submitting data on all patients treated in their centre

** New members which are pending approval at the General Assembly Meeting during the upcoming EBMT Annual Meeting.

18 centres and 38 individual members joined the EBMT in 2019

TOP 10 COUNTRIES IN TERMS OF NUMBER OF CENTRES PARTICIPATING IN THE EBMT



Our **577** centre members are located in **71** different countries

BECOME A MEMBER

Centres or independent persons that are active in the field of transplantation & cellular therapy related to any kind of haematopoietic stem cell, or any other organisation involved in the care of donors and recipients of HSCT, can become a member of the EBMT.

Members' rights, obligations, and fees are dependent upon the category of membership to which they pertain. Visit our website to get more details about each of the categories.

www.ebmt.org | membership@ebmt.org

LEGAL AND REGULATORY ACTIVITIES

EXPLORING THE EBMT REGISTRY AS A VALUABLE TOOL FOR HEALTH TECHNOLOGY ASSESSMENT (HTA) BODIES

EBMT has been recognized as one of the most significant registry holder in the EU as evidenced by being one of the first registries to successfully completing an EMA qualification process (see 2018 Annual Report). During the qualification process of the cellular therapy registry for CAR T therapies, observers from health technology assessment (HTA) bodies became aware of the EBMT. The European collaboration platform of HTAs from different countries (theEUnetHTA Joint Action) invited EBMT to qualify the cellular therapy registry for HTA purposes as a pilot test of their REQueST® Tool. The tool aims to support HTA organisations and other actors in guiding and evaluating registries for effective usage in HTA. The tool has been developed to be a comprehensive resource that covers all important aspects relating to the quality of registries. The standards set out in the tool are universal and essential elements of good practice and evidence quality that are, therefore, relevant for different types of registries.

INCREASING TRANSPARENCY AND BUILDING PARTNERSHIPS

EBMT has been registered in the EU Transparency Registry under identification number 652992023103-09. See <http://ec.europa.eu/transparencyregister/public/consultation/displaylobbyist.do?id=652992023103-09> for full details. This will allow for all external parties to get a closer insight into the organization. To increase the impact of health care professionals EBMT has partnered with the European Eye Bank Association, the European Blood Alliance and the European Association of Tissues Banks within the Common representation of Substances of Human Origin's (SoHO) (CoRe SoHO). Full details from the Transparency Register can be found at <http://ec.europa.eu/transparencyregister/public/consultation/displaylobbyist.do?id=501652723968-72>. Finally, EBMT is in the process of applying to EMA to participate in their framework for collaboration between EMA and academia. See more on this EMA initiative under <https://www.ema.europa.eu/en/partners-networks/academia>.

IMPACTING CHANGES IN THE REGULATORY ENVIRONMENT

Following several years of engaging with regulators and other stakeholders, EBMT is now recognised as the reference organisation for expert input on HSCT and cellular therapy at EU level. It is expected that 2020 will be an important year when the EU Tissues and Cells Directives are likely to be opened for review. On 20 February EBMT was invited to participate in the second DG SANTÉ (EU Commission, Public Health) meeting on data registries for Substances of Human Origin (SoHO) in Brussels presenting on our Registry and participating in wider discussions including sustainability, quality and data protection matters. Iris Bargalló, Debra Gordon and Eoin McGrath participated on behalf of EBMT.

On 1 April, EBMT met with Ms. Anne Bucher, Director of DG SANTÉ alongside the European Eye Bank Association, the European Blood Alliance and the European Association of Tissues Banks, associations that comprise the Common representation of Substances of Human Origin's (CoRe SoHO). The meeting was an opportunity to provide the then-newly appointed Director with the professionals' point of view on issues related to tissues and cells. Topics included donor/patient protection (monitor and prevent SARE, communicate risks, balance donor risk with patients benefits, use of SoHO as starting material for ATMP, etc); legislation: impact of EU regulation for SoHO sector; directives review and possible revision; SoHO

classification; current challenges; and impact for donation and clinical application; access to treatment and harmonization of practices.

In September, the Council of Europe's Directorate for the Quality of Medicines & HealthCare (EDQM) invited EBMT to participate in their new project, TO111 - *Harmonisation Activity Data Collection Exercises in the Field of Tissues and Cells in Europe*. This activity will contribute to improving the quality and accuracy of the data collected while avoiding duplication of efforts and decreasing the reporting duties of member States and tissue establishments. Furthermore, the results obtained may also be taken into account during potential updates of relevant EU Directives. Working groups are composed of experts from Health Authorities, professional societies and other relevant bodies. Prof. Per Ljungman will represent EBMT for this initiative.

EBMT is also a contributor to the EU [GAPP Joint Action](#) which will facilitate the development of a common and optimal approach to assessing and authorising preparation processes in blood and tissues establishments. The WP 6 2nd Expert Workshop took place in Paris on 20 May. WP6 will develop a Technical Annex offering overall guidance for regulators on authorisation of changes in donation, procurement and collection, processing, preservation, storage and distribution processes. The GAPP Interim Meeting meeting took place in Rome on 29-30 October with Dr. Harry Dolstra, EBMT Treasurer, participating as an expert in HSCT.

On 28 October at the [Conference on the Evaluation of the EU legislation on blood, tissues and cells](#), Prof. Jürgen Kuball, Chair of the EBMT Legal and Regulatory Affairs Committee, presented on behalf of EBMT. Eoin McGrath, EBMT employee, presented on behalf of CoRE SoHO (see above). The conference was attended by multiple stakeholders from the blood, tissues and cells sectors and the overall conclusion was that the Directives have served their purpose well since 2004 but are now in need of revision. Further announcements in this regard are expected in 2020.



On September 18-20, EBMT was represented at the [ICCBBA Global Forum on Medical Products of Human Origin \(MPHO\)](#) in Lisbon to celebrate 25 years of the ISBT128 coding system. This was an 'invitation only' international forum brought together delegates from more than 30 organizations and societies active in the entire range of MPHO, including representatives of health organizations, regulators, and professional societies. Discussed topics included common themes related to traceability, regulatory boundaries, ethics, biovigilance and healthcare technologies. The outcome of the Forum will be a report that will identify practical steps that can be taken towards harmonization and forwarding the guiding principles identified by the WHO.

Finally, EBMT proposed experts to contribute to drafting the next EDQM [Guide to the quality and safety of tissues and cells for human application](#), now in its 4th edition.

Jürgen Kuball, Chair EBMT Legal and Regulatory Affairs Committee
Eoin McGrath, JACIE Operations Manager

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THE SCIENTIFIC ACTIVITY REPORTS

2019 has passed and it proved to be a most vibrant and exciting year. In compiling the list of EBMT scientific achievements, I realise, once again, and am in total awe of how rich, varied, dynamic and rewarding our work has been in the past few years. Of course, there were also episodes of disappointment but, the successes far exceeded the tribulations and, for that, I marvel once more at how lucky we are to collaborate together in the fight against blood diseases, and in developing the field of hematopoietic cell transplantation and cellular therapy.

As the chairman of the scientific council of the society, it is always a great privilege for me to introduce the impressive scientific productivity of the EBMT. In the next few pages, you will see that over the last 12 months the different working parties have delivered some cutting-edge studies and guidelines, which are definitely impacting our practice. The amazing EBMT registry, including the newly established cellular therapy capturing process, continues to be the backbone of our activities and represents a unique flagship, making us proud. Despite the advent of many novel agents, the transplant approach continues to grow rapidly, particularly in acute leukaemia. The outcome of transplant patients is constantly improving, despite their increasingly older age, presence of comorbidities and many high-risk disease features. Interestingly, it is becoming clear in many diseases, that optimal outcomes are achieved when combining novel drugs, cellular therapies (including CAR-T cells) and hematopoietic cell transplantation.

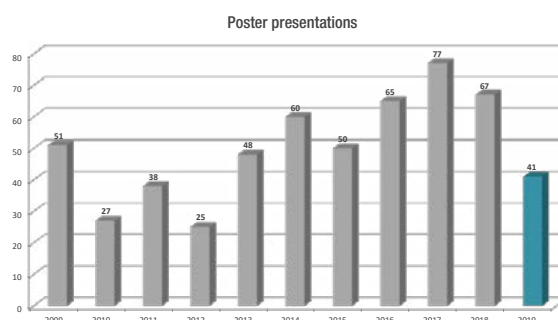
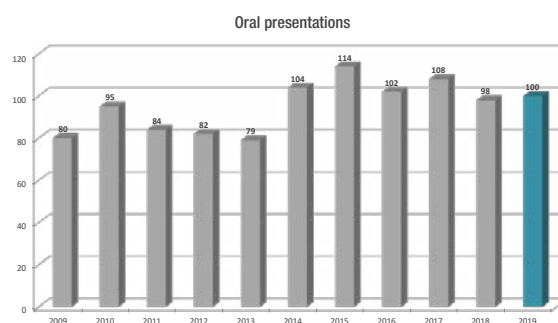
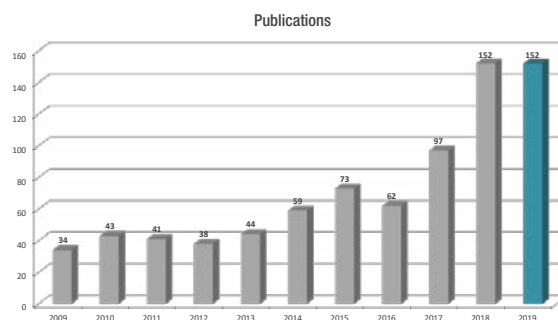
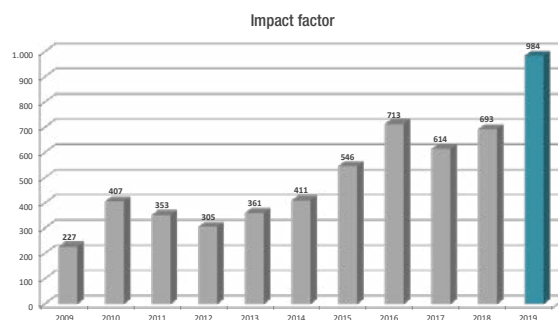
In 2019, beside the scientific publications, the EBMT has delivered an amazing number of educational activities and events. The new version of the EBMT Handbook has been a great success and has become extremely popular. The launch of the EBMT exam can be considered one of the highlights of 2019-2020.

The success of EBMT is also about the effort and hard work of its members. The EBMT is the ideal forum to develop the broader cellular therapy field and defend the interests of both our patients and members. The EBMT has a substantial advantage in sustaining its leadership role in what is a highly competitive environment. Please join us and spread the word.

Mohamad Mohty

Chairman of the ALWP of the EBMT

Chairman of the scientific council of the EBMT





Chair
Régis Peffaut de Latour

Severe Aplastic Anaemia Working Party (SAAWP)

MAJOR ACHIEVEMENTS

PROGRESSION OF TWO PROSPECTIVE RANDOMISED CLINICAL TRIALS:

1. RACE trial compares standard immunosuppressive therapy (ATG+CsA) plus Eltrombopag vs Standard IST alone in patients with severe aplastic anaemia. This is an EBMT study supported financially by Novartis, Pfizer and Alexion. 205 patients have been randomized. Accrual is thus finished since April 2019. The abstract entitled "Results of the EBMT SAAWP Phase III Prospective Randomized Multicenter RACE Study of Horse ATG and Ciclosporin With or Without Eltrombopag In Naïve SAA Patients" was granted the Van Bakkum Award and will be presented during the Presidential Symposium of the EBMT Annual Meeting 2020.
2. MAA study compares CsA plus Placebo vs CsA plus Eltrombopag in patients with moderate aplastic anaemia. This study is sponsored by the University of Ulm and supported financially by Novartis. 16 EBMT centres from 6 countries (Germany, Italy, France, The Netherlands, Switzerland and the UK) will recruit patients. Up to now, 41 patients have been enrolled and treated according to the study plan. The trial is still open for enrolment. There was no hint of an increased toxicity.

DATA QUALITY INITIATIVE

This initiative aims at improving the quality and follow-up data of patients receiving HSCT and the number of registration and the quality of data of those undergoing exclusive Immunosuppressive (IS) treatment. Until now, patients were selected between 2005 and 2014. The SAAWP had received the completed files from 69 centres for a total of 1,418 patients (1,173 with acquired bone marrow failure and 245 with constitutional disorders). This large dataset is now open for studies.

PRINCIPAL RESEARCH STUDIES

1. Haploidentical stem cell transplantation for congenital bone marrow failure: PI: Stefano Giardino
2. Stem Cell Transplantation for Diamond Blackfan Anaemia: PI: M. Miano
3. Upfront MUD transplantation in aplastic anemia: PI: A Petit
4. Renal failure in aplastic anemia: PI: B. Drexler
5. Comprehensive analysis of modified GRFS for severe aplastic anemia: PI: R Devillier

KEY PUBLICATIONS

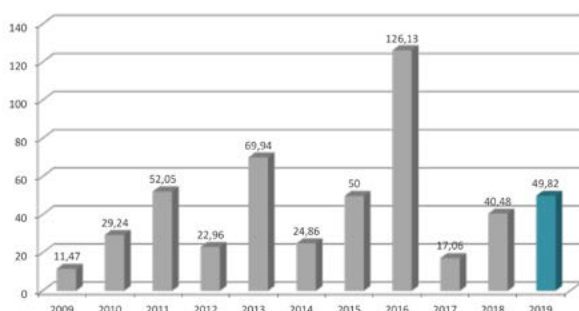
1. Long-term outcome of a randomized controlled study in patients with newly diagnosed severe aplastic anemia treated with antithymocyte globuline, cyclosporine, with or without G-CSF: a Severe Aplastic Anemia Working Party Trial from the European Group of Blood and Marrow Transplantation. Tichelli et al., *Haematologica*. DOI: 10.3324/haematol.2019.222562
2. Stem cell transplantation for congenital dyserythropoietic anemia: an analysis from the European Society for Blood and Marrow Transplantation. Miano M et al., *Haematologica*. DOI: 10.3324/haematol.2018.206623
3. Use of eltrombopag in aplastic anemia in Europe. Ecsedi M et al, *Ann Hematol*. DOI: 10.1007/s00277-019-03652-8
4. Allogeneic Hematopoietic Cell Transplantation in Patients Aged 50 Years or Older with Severe Aplastic Anemia. Rice C et al., *Biol Blood Marrow Transplant*. DOI: 10.1016/j.bbmt.2018.08.029
5. Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia: a joint study of the Autoimmune Diseases and Severe Aplastic Anaemia Working Parties (ADWP/SAAWP) of the European Society for Blood and Marrow Transplantation (EBMT). Miller PDE et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0680-4

6. Allogeneic stem cell transplantation for acquired pure red cell aplasia. Halkes C et al., *Am J Hematol*. DOI: 10.1002/ajh.25609
7. Impact of T-cell depletion strategies on outcomes following hematopoietic stem cell transplantation for idiopathic aplastic anemia: A study on behalf of the European blood and marrow transplant severe aplastic anemia working party. Samarasinghe S et al., *Am J Hematol*. DOI: 10.1002/ajh.25314
8. Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT. Risitano AM et al., *Front Immunol*. DOI: 10.3389/fimmu.2019.01157
9. Haploidentical transplantation and posttransplant cyclophosphamide for treating aplastic anemia patients: a report from the EBMT Severe Aplastic Anemia Working Party. Prata PH et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0773-0

MAJOR EDUCATIONAL COURSES

Joint EBMT-ESH Educational Meeting – Translational research conference on bone marrow failure – 13-15 November 2020 in Paris, France.

Impact factor
(Aplastic Anaemia)



	2015	2016	2017	2018	2019
Oral Presentations	23	20	13	17	8
Poster Presentations	5	1	9	3	1
International Educational Events	0	1	1	1	2



Chair
John Snowden

Autoimmune Diseases Working Party (ADWP)

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 (see below).

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.

PRINCIPAL RESEARCH STUDIES

1. **MS Comparison of CYC+ATG vs. BEAM+ATG conditioning regimens in autologous HSCT for Multiple Sclerosis.** ASH presentation 2019 (PI: Saccardi)
2. **Autologous HSCT for progressive systemic sclerosis: a prospective non-interventional study across Europe (NISSC):** Manuscript submitted. ACR presentation 2019 (PI: Farge)
3. **NISSC II - Post-AHSCT management and mechanistic immunological reconstitution for patients with systemic sclerosis** (PI: Farge)
4. **IRIS: Immune reset in SSc: prospective, multicentre study on immune reconstitution in SSc following auto-HSCT** (PI: Alexander)
5. **Late complications after autologous HSCT for AD: a retrospective survey from the ADWP and TCWP** (PI: Kirgizov)
6. **Viral reactivations: retrospective study on viral infections post auto-HSCT in Autoimmune Diseases** (PI: Alexander)
7. **Indications and outcomes of re-transplantation for AD** (Co-PIs Polushin/Kirgizov)
8. **Disease-specific retrospective studies of autologous HSCT in:**
 - a. Immune cytopenias (PI Cooper)
 - b. Rare neurological diseases (CIDP, Stiff Person Syndrome, myasthenia gravis, NMO and others, Co-PIs Sharrack/Brittain)
 - c. Behçet's Disease (PI Puyade)
 - d. Takayasu arteritis (PI Mekinian)
 - e. Juvenile and adult systemic arthritis/Still's disease (PI Alexander)
 - f. Polymyositis-Dermatomyositis (PI Alexander)
9. **Surveys**
 - a. HLH/MAS post-HSCT in adults (joint with TCWP, PI Sandler).

- b. National provision/reimbursement of HSCT and follow up in ADs (with EBMT NG and PAC, PI Jessop).

10. Guidelines and recommendations

- a. EBMT ADWP/Nurses Group guidelines for rehabilitation post HSCT for MS (Co-PI Jessop/Roberts)
b. HSCT in adult rheumatological autoimmune diseases (ADs): guidelines and recommendations from the EBMT Autoimmune Diseases Working Party (ADWP) +/- EULAR collaboration (PI Alexander)
c. Position paper with European Academy of Neurology EAN/ECTRIMS and EBMT (Co-PIs Muraro/Sharrack/Burman)

11. Prospective long-term follow of studies involving EBMT registry

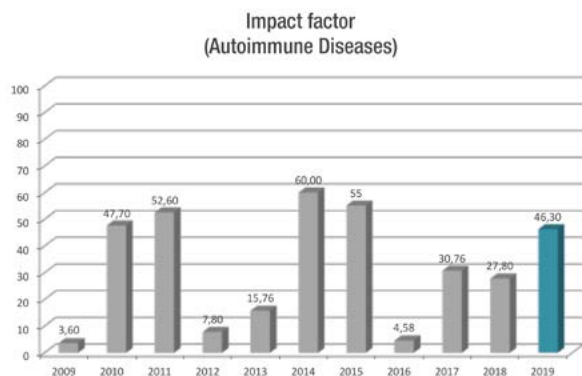
- a. ASTIClite: Via the ADWP, the EBMT secured funding (96000 euros) to support the data collection and analysis of the long-term follow-up study in this UK NIHR randomised controlled clinical trial of autologous HSCT versus standard of care in Crohn's disease (Co-PIs Lindsay/Snowden).
b. Harmonisation of long-term follow-up of clinical trials in MS (EBMT involvement TBC, Co-PIs Muraro/Sharrack).

KEY PUBLICATIONS

1. Autologous haematopoietic stem cell transplantation and other cellular therapy in multiple sclerosis and immune-mediated neurological diseases: updated guidelines and recommendations from the EBMT Autoimmune Diseases Working Party (ADWP) and the Joint Accreditation Committee of EBMT and ISCT (JACIE). Sharrack B et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0684-0
2. Allogeneic HSCT for Autoimmune Diseases: A Retrospective Study From the EBMT ADWP, IEWP, and PDWP Working Parties. Greco R et al., *Front Immunol*. DOI: 10.3389/fimmu.2019.01570
3. Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia: a joint study of the Autoimmune Diseases and Severe Aplastic Anaemia Working Parties (ADWP/SAAWP) of the European Society for Blood and Marrow Transplantation (EBMT). Miller PD et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0680-4
4. General information for patients and carers considering haematopoietic stem cell transplantation (HSCT) for severe autoimmune diseases (ADs): A position statement from the EBMT Autoimmune Diseases Working Party (ADWP), the EBMT Nurses Group, the EBMT Patient, Family and Donor Committee and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Jessop H et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0430-7
5. Evaluating the clinical effectiveness of autologous haematopoietic stem cell transplantation versus disease-modifying therapy in multiple sclerosis using a matching-adjusted indirect comparison: an exploratory study from the Autoimmune Diseases Working Party (ADWP) of the European Society of Bone and Marrow Transplantation (EBMT). Tappenden P et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0747-2

MAJOR EDUCATIONAL COURSES

1. Autumn Educational Meeting of the ADWP, 31 October-1 November 2019, Berlin, Germany.
2. ADWP Educational and Working Party Sessions, EBMT Annual Meeting, 24-27 March 2018, Frankfurt, Germany.
3. Invited lectures at other international educational events and courses.



	2015	2016	2017	2018	2019
Oral Presentations	6	11	10	11	16
Poster Presentations	1	0	3	5	2
International Educational Events	1	1	1	1	1



Professor John Snowden (ADWP Chair) at the opening session of the Autumn Educational Meeting of the Autoimmune Diseases Working Party, 31 October – 1 November 2019 in Berlin, Germany



Chair
Mohamad Mohty

Acute Leukaemia Working Party (ALWP)

MAJOR ACHIEVEMENTS

Acute leukaemia (both AML and ALL) remains the main indication for allogeneic HSCT. This activity is continuously growing despite the advent of many novel drugs, including CAR-T cells for young patients with relapsed ALL. The ALWP has always focused on exploiting the EBMT registry data in order to pave the way to further improving HSCT results, especially the risk of relapse and non-relapse mortality. Several ALWP studies performed over the last few months have aimed to understand the role of novel agents before ("bridge to HSCT") and after HSCT ("maintenance"). More and more, the success of the HSCT procedure is closely linked to interventions applied prior to HSCT, as well as after HSCT. In this respect, the role of measurable residual disease has gained a lot of interest and is proving to be a key determinant of the overall HSCT outcome. The optimization of the HSCT procedure itself is another field of interest to the ALWP. A significant number of studies have investigated the role of the so-called "post-Cy" GVHD prophylaxis regimen in different settings, beyond the classical haplo-identical donor setting.

In 2019, thanks to a terrific group of dynamic leaders and rising stars in the field of leukaemia, the ALWP has been extremely productive. The comparison between haploidentical donors and other donor types (e.g. HLA-matched sibling, HLA-matched unrelated, or cord blood cells) is increasingly showing the predominant role of haplo-identical donors as a major source of stem cell donation irrespective of disease status or conditioning regimen. This is why the use of post-transplant cyclophosphamide continues to generate a lot of enthusiasm, with some strong and consolidated results, including in the non-haplo setting where it may have nullified the detrimental effect of HLA-mismatch. The latter is of great interest, especially when considering advanced cases of AML or ALL, where it is probably not possible to wait long enough to find a fully matched donor. In this regard, the ALWP has proudly published the EBMT consensus recommendations for donor selection in haploidentical hematopoietic cell transplantation. This paper has rapidly become a worldwide reference for the community. Similarly, the ALWP developed and published a major project focused on re-defining and measuring transplant conditioning intensity in AML, because refinement of the different conditioning regimens (balance between anti-leukemia activity and toxicity) is of major concern, especially in the context of high risk and refractory disease. Finally, post-remission strategies for the prevention of relapse following allogeneic HSCT are increasingly used with convincing efficacy. The ALWP studies suggest that sorafenib (and probably other FLT3 inhibitors) can improve survival of FLT3-mutated AML after allogeneic HSCT. These are just a few examples of the key achievements of the ALWP, which has an amazing record of high impact publications. Our activities are possible thanks to all EBMT members who continue to report their data voluntarily.



"Advances in Allogeneic Immunotherapy" 15-17 November, 2019 in Marseille, France.

PRINCIPAL RESEARCH STUDIES

1. Analysis of donor characteristics in different disease and transplant settings
2. The role of measurable residual disease (MRD) in different transplant settings for acute leukaemia
3. Pharmacological agents to treat and prevent relapse post HSCT for acute leukaemia
4. Introduction of data-mining studies to predict transplant outcome, and design of strong consensus guidelines for the management of AML and ALL patients

3. Haploidentical vs. unrelated allogeneic stem cell transplantation for acute lymphoblastic leukemia in first complete remission: on behalf of the ALWP of the EBMT. Shem-Tov et al., *Leukemia*. DOI: 10.1038/s41375-019-0544-3
4. Outcomes of allogeneic haematopoietic stem cell transplantation from HLA-matched and alternative donors: a European Society for Blood and Marrow Transplantation registry retrospective analysis. Shouval et al., *Lancet Haematol*. DOI: 10.1016/S2352-3026(19)30158-9
5. Posttransplant cyclophosphamide vs antithymocyte globulin in HLA-mismatched unrelated donor transplantation. Battipaglia et al., *Blood*. DOI: 10.1182/blood.2019000487

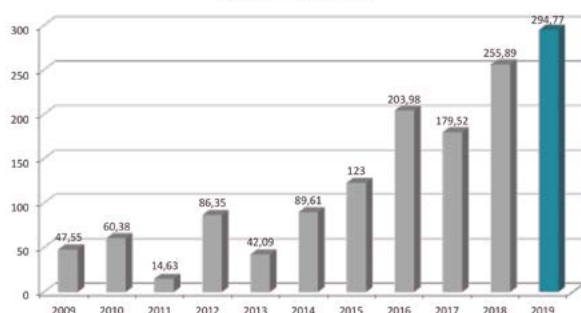
KEY PUBLICATIONS

1. Sorafenib improves survival of FLT3-mutated acute myeloid leukemia in relapse after allogeneic stem cell transplantation: a report of the EBMT acute leukemia Working Party. Bazarbachi et al., *Haematologica*. DOI: 10.3324/haematol.2018.211615
2. The European Society for Blood and Marrow Transplantation (EBMT) consensus recommendations for donor selection in haploidentical hematopoietic cell transplantation. Ciura et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0499-z

MAJOR EDUCATIONAL COURSES

“Advances in Allogeneic Immunotherapy” 15-17 November, 2019 in Marseille, France.

Impact factor
(Acute Leukaemia)



	2015	2016	2017	2018	2019
Oral Presentations	22	30	39	28	24
Poster Presentations	15	19	23	18	17
International Educational Events	3	2	2	3	1



Chair
Christian Chabannon

Cellular Therapy and Immunobiology Working Party (CTIWP)

MAJOR ACHIEVEMENTS

2019 has been an important year for the CTIWP. Registrations started for patients treated with CAR-T cells, using the newly created Cellular Therapy Form as accessible in ProMISe. While registering patients and entering follow-up is expected to be easier when the registry migrates to MACRO, transient dispositions already allow to do so, including the possibility to register patients whenever they become candidates to CAR-T cells treatment, and thus accumulate information that will be useful for Intent-to-treat (ITT) analyses. Starting in July 2019, the EBMT newsletter monthly published updated and cumulative figures showing rapid adoption of industry-manufactured CAR-T cells in most western European countries, as well as a robust clinical research activity that evaluates investigational CAR-T cells. This confirms the trend evidenced by the 2018 EBMT activity survey, and by the survey conducted under the leadership of Alvaro Urbano-Ispizua to identify active EU centers, and better understand the nature of ongoing activities (see below). Based on existing evidences, guidelines have been developed for practitioners and nurse, in collaboration with other EBMT Working Parties and with the EBMT Nurses Group. While some scientific projects are starting to emerge in the field of immune-effector cells, the EBMT community is working to define conditions in which to share and jointly exploit the full potential of collected data with all interested parties – including such professional associations as EHA and ESMO and disease-oriented cooperative groups - as part of a joint initiative.

Meanwhile, the CTIWP continues to explore the field of autologous and allogeneic hematopoietic cell transplantation, and several studies were pursued or initiated in 2019.

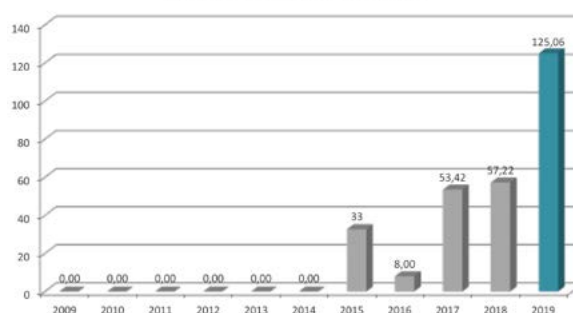
PRINCIPAL RESEARCH STUDIES

1. A survey of immune monitoring practices for viral infections post-HSCT in Europe (L Vago & R Greco et al, Immune Monitoring Subcommittee)
2. A study of the consequences of donor-recipient HLA matching on the outcome of allogeneic hematopoietic stem cell transplantation from unrelated donors (K Fleischhauer et al. Immunogenetics subcommittee, in collaboration with DKMZ)
3. Post-Transplant Cyclophosphamide (PTCy) prophylaxis after 9/10 or 10/10 HLA-matched unrelated donor transplantation for acute leukemia (F Lorentino, AL Ruggeri, collaboration with the EBMT Acute Leukemia Working Party, ALWP)
4. A survey of ongoing studies evaluating mesenchymal stem cells in Europe. Part I: manufacturing capacities.
5. A survey of CAR-T cell activities in Europe (A Urbano-Ispizua et al, Immune Effector Cells Subcommittee). Original data collected in Dec 2018, updated in May 2019
6. Development of guidelines for the safe administration of CAR-T Cells, intended for nurses. (R Ellard, liaison with the EBMT Nurses Group)
7. A survey of donor lymphocyte infusions following haplotype-mismatched hematopoietic cell transplantation (N Santoro, Immune Effector Cells subcommittee)
8. Results of cord blood transplantations (AL Ruggeri et al, Hematopoietic Stem Cells & Eurocord Subcommittee)
9. Retrospective study of soft tissue sarcomas undergoing autologous HSCT (Heilig CE, Pedrazzoli P et al, Solid Tumors Subcommittee)

KEY PUBLICATIONS

1. Management of adults and children undergoing CAR T-cell therapy: best practice recommendations of the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Yakoub-Agha I et al., *Haematologica*. DOI: 10.3324/haematol.2019.229781
2. Development of adaptive immune effector therapies in solid tumors. Comoli P et al., *Ann Oncol*. DOI: 10.1093/annonc/mdz285
3. Outcomes of allogeneic haematopoietic stem cell transplantation from HLA-matched and alternative donors: a European Society for Blood and Marrow Transplantation registry retrospective analysis. Shouval R et al, *Lancet Haematol*. DOI: 10.1016/S2352-3026(19)30158-9
4. Beneficial role of CD8+ T-cell reconstitution after HLA-haploidentical stem cell transplantation for high-risk acute leukaemias: results from a clinico-biological EBMT registry study mostly in the T-cell-depleted setting. Bondanza A et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-018-0351-x
5. Indications for haematopoietic stem cell transplantation for haematological diseases, solid tumours and immune disorders: current practice in Europe, 2019. Duarte RF et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0516-2

Impact factor
(Cell Therapy & Immunobiology)



	2015	2016	2017	2018	2019
Oral Presentations	4	1	7	3	16
Poster Presentations	4	8	3	2	2
International Educational Events	1	1	3	6	6

MAJOR EDUCATIONAL COURSES

1. 1st joint EBMT-EHA European CAR-T Cell Meeting - 14-16 February 2019 in Paris, France.

680 delegates
225 live stream registrations
85 submitted abstracts
78 speakers



2. The CTIWP Scientific Session at the 45th EBMT Annual Meeting including the delivery of the Jon van Rood Award to Itauá Leston Araujo – 26 March 2019 in Frankfurt, Germany.
3. The 8th edition of the Cell Therapy Day at the 45th EBMT Annual Meeting – 25 March 2019 in Frankfurt, Germany.



Chair
Jan Styczynski

Infectious Diseases Working Party (IDWP)

MAJOR ACHIEVEMENTS

The mission of IDWP is to share the experience and develop cooperative studies to increase education in the field of diagnosis, prophylaxis and treatment of infectious complications in HSCT patients.

IDWP Members continue their scientific and educational activity in the fields of: bacterial infections (D. Averbuch, M. Mikulska, C. Cordonnier, D. Engelhard, A. Verlinden, M. Yeshurun, E. Snarski), *viral infections* (P. Ljungman, K. Ward, R. de La Camara, M. Mikulska, K. Peruccio, O. Penack, J. Pinana, S. Cesaro, M. Schmidt-Hieber, L. Gil, H. Einsele, R. Martino, J. Styczynski), *fungal infections* (M. Mikulska, O. Penack, R. de La Camara, S. Cesaro, J. Styczynski), *parasitic infections* (L. Gil, R. Martino), *pneumocystis jiroveci infections* (C. Robin, C. Cordonnier), vaccinations (C. Cordonnier, M. Mikulska, P. Ljungman, D. Engelhard, S. Cesaro), *protective environment* (I. Agreiter, P. Donnelly, J. Styczynski, S. Cesaro) and *ECIL guidelines* (C. Cordonnier, M. Mikulska, P. Ljungman, D. Engelhard, S. Cesaro, J. Styczynski).

IDWP recent achievements were published in *Lancet Infectious Diseases*, *Haematologica*, *Journal of Antimicrobial Chemotherapy*, and *Bone Marrow Transplantation*.

PRINCIPAL RESEARCH STUDIES

Ongoing retrospective projects:

1. **Role of CMV, EBV, ADV, JCV, HHV6, HHV8 and HIV on outcomes of HCT**
2. **Infections with legionellosis, toxoplasmosis, tuberculosis, nocardiosis, JCV, HEV, ADV after HCT**
3. **Causes of deaths after HC.**

Ongoing prospective non-interventional projects:

4. **HHV6 infections after HCT**
5. **The incidence of gram-negative bacteremia, risk factors and resistance to antibiotics**
6. **Impact of pre-existing invasive aspergillosis on allo-HCT outcome**
7. **Risk factors and outcome of pneumocystis pneumonia (PcP) infection in HCT**
8. **Treatment approach for patients with HCV infection and who underwent HCT**
9. **Anti-infective prophylaxis and antibiotic use in patients undergoing HCT**
10. **Infections of CNS after HCT**



Figure 1. Participants of 22nd IDWP Educational Course in Krakow 17-19 October 2019.

KEY PUBLICATIONS

1. Vaccination of patients with haematological malignancies who did not have transplantations: guidelines from the 2017 European Conference on Infections in Leukaemia (ECIL 7). Mikulska M et al., *Lancet Infect Dis*. DOI: 10.1016/S1473-3099(18)30601-7
2. Vaccination of haemopoietic stem cell transplant recipients: guidelines of the 2017 European Conference on Infections in Leukaemia (ECIL 7). Cordonnier C et al., *Lancet Infect Dis*. DOI: 10.1016/S1473-3099(18)30600-5
3. Guidelines for the management of cytomegalovirus infection in patients with haematological malignancies and after stem cell transplantation from the 2017 European Conference on Infections in Leukaemia (ECIL 7). Ljungman P et al., *Lancet Infect Dis*. DOI: 10.1016/S1473-3099(19)30107-0

4. Guidelines from the 2017 European Conference on Infections in Leukaemia for management of HHV-6 infection in patients with hematologic malignancies and after hematopoietic stem cell transplantation. Ward KN et al., *Haematologica*. DOI: 10.3324/haematol.2019.223073
5. A survey on incidence and management of adenovirus infection after allogeneic HSCT. Cesaro S et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-018-0421-0

MAJOR EDUCATIONAL COURSES

1. 8th European Conference on Infections in Leukemia and Hematopoietic Cell Transplantation – 21-22 September 2019 in Sophie Antipolis, France.
2. 22nd Educational Course of the Infectious Diseases Working Party – 17-19 October 2019 in Krakow, Poland.

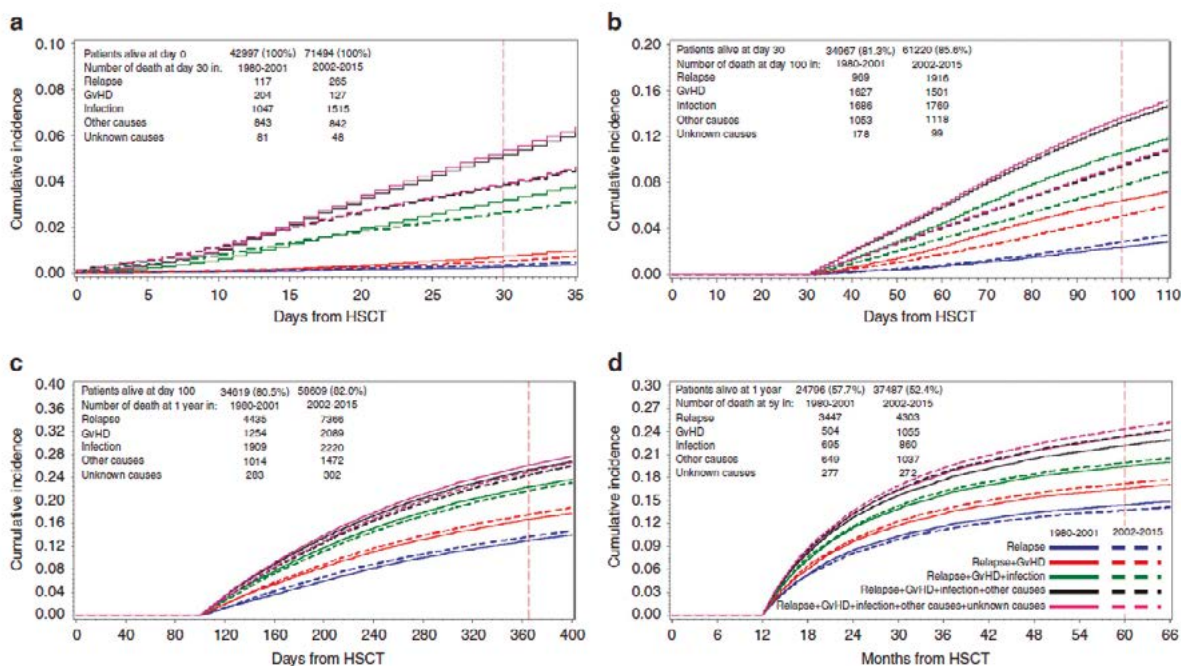
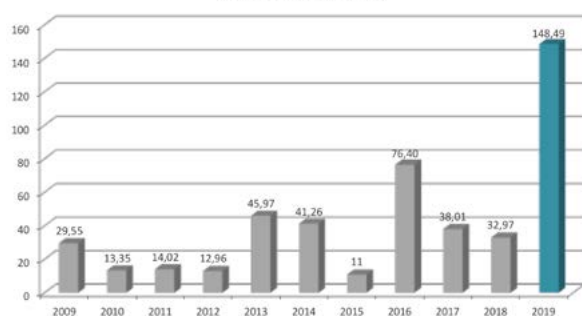


Figure 2. Decrease in mortality after HCT in EBMT centers between 2002-2015 in comparison to 1980-2001 (Styczynski et al, *Bone Marrow Transplant*, 2020 open access).

Impact factor
(Infectious Diseases)

	2015	2016	2017	2018	2019
Oral Presentations	3	6	1	3	1
Poster Presentations	0	0	4	3	2
International Educational Events	2	1	2	2	2



Chair
Arjan Lankester

Inborn Errors Working Party (IEWP)

MAJOR ACHIEVEMENTS

In 2019, the IEWP has continued and further expanded its activities in the field of stem cell therapies for inherited disorders of immunity, erythropoiesis and metabolism. As in previous years, IEWP actively participated in the EBMT International Transplant Course in Barcelona with presentations on HSCT in primary immune disorders (PID). In addition, presentations were given at the COSTEM meeting (HSCT in thalassemia) and the ADWP annual meeting (stem cell therapy in monogenic AID).

During this year's ESID focus meeting on Malignancy in PID, a very well attended IEWP session, was fully dedicated to optimizing the use of registries for PID with presentations on the value of clinical score in PID (M Seidel), registration of malignancies in PID (F Porta) and a registry for XIAP (S Burns).

This year, the Inborn Errors Working Party Annual Conference took place in London (host: Paul Veys), and special attention was given to the 40th anniversary of the stem cell therapy program in Great Ormond Street Children's Hospital. On the first educational day, a series of international experts presented excellent overviews on developments in the field of newborn screening for SCID, state-of-the-art in SCT and gene therapy in SCID and non-SCID PID, developments in gene editing, and novel treatment modalities in primary HLH. The second and third day included high-quality and interactive sessions on various aspects of stem cell therapies in inherited metabolic disorders and hemoglobinopathies, the evolving position of cellular therapy in treatment of immune regulatory disorders, AT and VEO-IBD, recent advances in the field of individualized conditioning and reports on ongoing IEWP studies. Cutting-edge presentations were given on thymic development (G Hollander) and the keynote lecture on RAG deficiency (L Notarangelo). The meeting was attended by many international colleagues reflecting both the excellent quality of the meeting as well as the strong international collaboration in the field of stem cell therapy for patients with these rare inherited disorders.

In 2019, fifteen excellent manuscripts were published on behalf of IEWP, including a series bundled as Golden

Research Topic in Frontiers in Pediatrics on HSCT for Primary Immunodeficiencies and Rare Metabolic Diseases. For 2020, IEWP has the aim to further extend its study activities, retrospective, prospective as well as biological, in collaboration with other WPs, ESID, SCETIDE, PIDTC and other international partners.

PRINCIPAL RESEARCH STUDIES

1. **Allogeneic HSCT in children and adults with chronic granulomatous disease**
2. **Outcome and immune reconstitution in SCID HSCT: IEWP-SCETIDE study**
3. **HSCT in Wiskott-Aldrich syndrome comparing Bu-Flu and Treo-Flu conditioning: IEWP-SCETIDE study**
4. **Long term outcome of HSCT for SCID: SCETIDE-IEWP-PIDTC study**
5. **Outcome of HSCT in Leukocyte Adhesion Deficiency 2008 – 2015: IEWP-PDWP study**
6. **HSCT for Erythropoietic Porphyria: EBMT-CIBMTR study**
7. **HSCT in Inherited Metabolic Diseases with focus on immune cytopenia: IEWP-Eurocord study**
8. **HSCT in LAL-Wolman's disease**
9. **HSCT in patients with IFN γ receptor deficiencies**
10. **CD27 and CD70 deficiency: clinical and immunologic features and HSCT outcomes**
11. **HSCT in IL7R deficient SCID: clinical and immunologic outcomes**
12. **HSCT in adolescent and adult patients with primary immunodeficiencies**
13. **Haploidentical SCT in PID comparing PT-Cy and TCR $\alpha\beta$ depletion approaches**



Inborn Errors Working Party Annual Conference – 12-13 October 2019 in London, UK

14. HSCT outcomes in hypomorphic RAG deficiency

15. Characteristics and HSCT outcome in Immunodeficiency, Centromere instability and Facial anomalies (ICF) syndrome

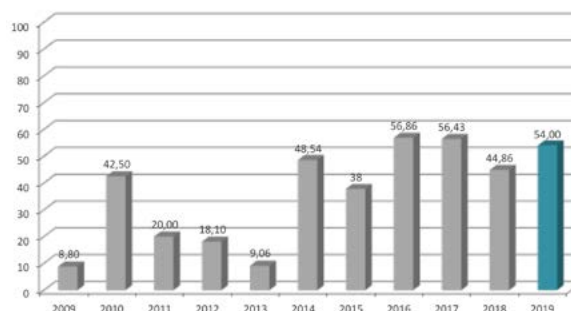
KEY PUBLICATIONS

1. Hematopoietic stem cell transplantation for CD40 ligand deficiency: Results from an EBMT/ESID-IEWP-SCETIDE-PIDTC study. Ferrua F et al., *J Allergy Clin Immunol*. DOI: 10.1016/j.jaci.2018.12.1010
2. Allogeneic HSCT for Autoimmune Diseases: A Retrospective Study From the EBMT ADWP, IEWP, and PDWP Working Parties. Greco R et al., *Front Immunol*. DOI: 10.3389/fimmu.2019.01570
3. Hematopoietic Stem Cell Transplantation as Treatment for Patients with DOCK8 Deficiency. Aydin S et al., *J Allergy Clin Immunol Pract*. DOI: 10.1016/j.jaip.2018.10.035
4. Hematopoietic Stem Cell Transplantation in Inborn Errors of Metabolism. Tan E et al., *Front. Pediatr*. DOI: 10.3389/fped.2019.00433
5. Allogeneic Hematopoietic Stem Cell Transplantation for Congenital Immune Dysregulatory Disorders. Bakhtiar et al., *Front Pediatr*. DOI: 10.3389/fped.2019.00461

MAJOR EDUCATIONAL COURSES

1. IEWP session on optimizing use of the registry at ESID Focus meeting PID and malignancy – 18-21 September 2019 in Brussel, Belgium.
2. Inborn Errors Working Party annual educational day '40 years of transplantation in Great Ormond Street' – 11 October 2019 in London, UK.
3. Inborn Errors Working Party Annual Conference – 12-13 October 2019 in London, UK.

Impact factor
(Inborn Errors)



	2015	2016	2017	2018	2019
Oral Presentations	5	2	8	8	9
Poster Presentations	2	3	2	1	0
International Educational Events	3	4	4	4	3



Chair
Silvia Montoto

Lymphoma Working Party (LWP)

MAJOR ACHIEVEMENTS

The LWP is an active working party composed of a core member panel of 10 physicians with an interest in lymphoma and cell therapies, 2 data-managers, 1 statistician, a nurse and a patients' representative. 2019 was a very successful year for the LWP with the completion and publication of many studies. Our portfolio and publication list highlights some of the core values of the LWP: collaboration and education. We have active and successfully collaborated with other EBMT working parties, as well as with external organisations such as CIBMTR. In addition to the research papers, the LWP has continued on the tradition of providing education and guidance through the publication of consensus guidelines and position papers on hot topics such as the role of haplo-transplants. Along the same lines, and given that lymphoma is currently one of the main indications for CAR-T cell therapy, we have been working with the CIBMTR on a position paper on the role of CAR-T cell therapy in patients with refractory lymphoma. We published 10 papers in 2019 and presented 11 studies at prestigious international scientific meetings such as at the International Conference in Malignant lymphoma (Lugano) and at the annual meeting of the European Hematology Association. In terms of education, the LWP run another successful educational course with the annual LWP educational course, which took place in Bristol, United Kingdom from 18th to 19th September 2019.

PRINCIPAL RESEARCH STUDIES

- 1. Allogeneic Transplantation in Double (Triple) - Hit Diffuse Large B-Cell Lymphoma: a retrospective study of the EBMT-Lymphoma Working Party.** N Schmitz
- 2. Post-transplant cyclophosphamide-Based Haploidentical Transplantation versus Matched Sibling or Well-matched Unrelated Donor Transplantation for peripheral T cell lymphoma: A CIBMTR Lymphoma Working Committee & EBMT Lymphoma Working Party Analysis.** P Dreger
- 3. Autologous Stem Cell Transplantation in Post-Transplantation Lymphoproliferative Diseaseorders. A retrospective analysis of the LWP of the EBMT.** T Eyre
- 4. Characteristics and outcomes of relapsed Hodgkin lymphoma after autologous stem cell transplant in the current era. A retrospective analysis of the LWP of the EBMT.** A Bazarbachi



15th Edition of the Lymphoma Working Party Educational Course - 19-20 September 2019 in Bristol, United Kingdom

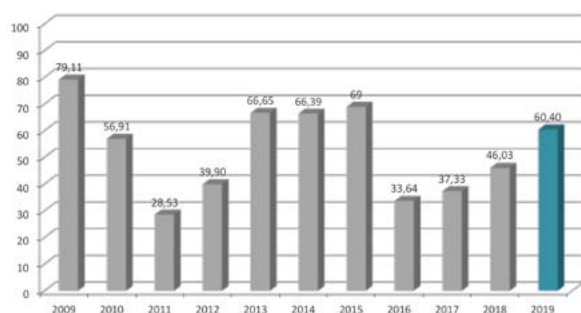
KEY PUBLICATIONS

1. Ibrutinib as a salvage therapy after allogeneic HCT for chronic lymphocytic leukemia. Michallet M et al; French Cooperative Group for CLL, SFGM-TC, and the EBMT Chronic Malignancy and Lymphoma Working Parties, *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0742-7
2. Influence of donor type, stem cell source and conditioning on outcomes after haploidentical transplant for lymphoma - a LWP-EBMT study. Bazarbachi A et al, *Br J Haematol*. DOI: 10.1111/bjh.16182
3. Haploidentical stem cell transplantation for patients with lymphoma: a position statement from the Lymphoma Working Party-European Society for Blood and Marrow Transplantation. Dietrich S et al, *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0583-4
4. Maintenance Therapies for Hodgkin and Non-Hodgkin Lymphomas After Autologous Transplantation: A Consensus Project of ASBMT, CIBMTR, and the Lymphoma Working Party of EBMT. Kanate AS et al, *JAMA Oncol*. DOI: 10.1001/jamaoncol.2018.6278
5. Autologous stem cell transplantation for HIV-associated lymphoma in the antiretroviral and rituximab era: a retrospective study by the EBMT Lymphoma Working Party. Hübel K et al, *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0480-x

MAJOR EDUCATIONAL COURSES

15th Edition of the Lymphoma Working Party Educational Course - 19-20 September 2019 in Bristol, United Kingdom.

Impact factor
(Lymphoma)



	2015	2016	2017	2018	2019
Oral Presentations	6	3	8	8	7
Poster Presentations	4	5	8	9	4
International Educational Events	4	6	3	1	1



Chair
Selim Corbacioglu

Paediatric Diseases Working Party (PDWP)

MAJOR ACHIEVEMENTS

The scientific output in 2019 increased more than three times compared to 2018. In 2019, the PDWP introduced the position of a vice-chair and subcommittees (SC). These structural changes encourage autonomous expert groups to intensify their scientific activities. To that regard a common collaborative platform is also in discussion.

An international position paper, accepted for publication in *Lancet Haematology*, co-authored by PALISI and the EBMT PDWP, supports the modified EBMT criteria for children which will allow adaptation to the clinical circumstances in the US, potentially a milestone for unified pediatric criteria for the diagnosis of VOD/SOS.

Another early post-HSCT complication next to VOD/SOS, called transplant associated macrophage-activation syndrome (TAMAS), is not well defined and therefore underdiagnosed. It differs from macrophage-activation syndromes outside the transplantation setting. This is now increasingly recognized. The aim of a PDWP expert group, is to define diagnostic criteria for TAMAS and raise awareness.

A particular highlight of 2019 was the Educational Meeting in Regensburg, Germany with 123 attendees from 16 nations. A faculty of 20 experts presented a comprehensive state of the art overview of curative options for sickle cell disease (SCD). A

special issue of the *Journal Hematology/Oncology and Stem Cell Therapy* will present the reports open-access, allowing everybody access to the impressive medical advances in SCD. My gratitude extends to the editor-in-chief, Dr. Mahmoud Aljurf, for this outstanding opportunity.

Board meetings took place in Frankfurt, during the EBMT Annual Meeting and in October in Istanbul, at the 10th Eurasian Hematology Oncology Congress (EHOC 2019) and an ad hoc board meeting was held during the 61st ASH Annual Meeting in Orlando, resulting in constructive scientific projects for 2020.

For 2020, CAR-T therapy and gene editing will dominate the focus of attention, with the first patient with thalassemia ever being treated in a European country using the CRISPR/Cas technology. The prospective international trial using $\alpha\beta$ T-cell depleted haploidentical HSCT in SCD, an official EBMT trial, will start to recruit.

A Midterm Meeting, in Leiden, organized by the IEWP, the SAAWP and the PDWP, with focus on non-malignant diseases including SAA, hemoglobinopathies and immunodeficiencies and an educational/scientific meeting in October 2020 in London will focus on hot topics in the field.



5th International Sickle Cell Meeting SCD what are the curative options - 16 – 17 May 2019 in Regensburg, Germany.

PRINCIPAL RESEARCH STUDIES

1. **ALL-SCT Forum Trial (prospective) PI: Christina Peters**
2. **Second transplant in Sickle Cell Disease PI: Josu de la Fuente**
3. **Haploidentical stem cell transplantation using PTCY for children with acute leukemia PI: Annalisa Ruggeri**
4. **Haematopoietic stem cell transplantation for sickle cell disease. An analysis on behalf of Eurocord, PDWP of EBMT, CIBMTR, USP and Ruby Hall Clinic PIs: Fernanda Volt, Eliane Gluckman, Annalisa Ruggeri, Barbara Cappelli**
5. **Late effects after hematopoietic stem cell transplantation in patients with HLH. PI: Anna Carin Horne**
6. **The use of TPO agonist post pediatric HSCT PI: Asaf Yanir**
7. **Trends and outcomes of HSCT in Thalassemia inside the EBMT PI: Donatella Baronciani, Emanuele Angelucci**
8. **Pregnancy rates and pregnancy outcomes after HSCT in patients transplanted during childhood PI: Tamara Diesch**
9. **ABO Incompatibility in hemoglobinopathies PIs: Katharina Kleinschmidt, Selim Corbacioglu, Josu de la Fuente**
10. **A phase II stratified trial to assess haploidentical T-depleted stem cell transplantation in patients with sickle cell disease with no available sibling donor (prospective) PI: Selim Corbacioglu**

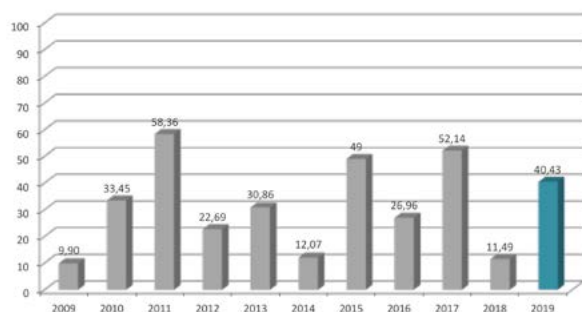
KEY PUBLICATIONS

1. Diagnosis, grading, and treatment recommendations for children, adolescents and young adult patients with sinusoidal obstructive syndrome: an international expert position statement. Mahadeo K et al., and the Pediatric Acute Lung Injury and Sepsis Investigators (PALISI) Network and the EBMT PDWP, *Lancet Haematol*. DOI: 10.1016/S2352-3026(19)30201-7
2. Allogeneic hematopoietic stem cell transplantation from unrelated donors is associated with higher infection rates in children with acute lymphoblastic leukemia-A prospective international multicenter trial on behalf of the BFM-SG and the EBMT-PDWP. Pichler H et al., *Am J Hematol*. DOI: 10.1002/ajh.25511
3. Allogeneic HSCT for Autoimmune Diseases: A Retrospective Study from the EBMT ADWP, IEWP, and PDWP Working Parties. Greco R et al., *Front Immunol*. DOI: 10.3389/fimmu.2019.01570
4. Transplant center practices for psychosocial assessment and management of pediatric hematopoietic stem cell donors. Wiener L et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0515-3
5. Gonadal Function after Busulfan Compared with Treosulfan in Children and Adolescents Undergoing Allogeneic Hematopoietic Stem Cell Transplant. Faraci M et al., *Biol Blood Marrow Transplant*. DOI: 10.1016/j.bbmt.2019.05.005

MAJOR EDUCATIONAL COURSES

5th International Sickle Cell Meeting “SCD: what are the curative options”? - 16 – 17 May 2019 in Regensburg, Germany.

Impact factor
(Paediatric Diseases)



	2015	2016	2017	2018	2019
Oral Presentations	6	8	6	5	8
Poster Presentations	3	7	1	7	1
International Educational Events	2	1	1	3	1



Chair
Ibrahim Yakoub-Agha

Chronic Malignancies Working Party (CMWP)

MAJOR ACHIEVEMENTS

The first CMWP Scientific Meeting of 2019 was held in the Novotel Paris Centre Tour Eiffel Hotel on the morning of 14th February just prior to the first joint EHA-EBMT European CAR T Cell Meeting. Reflecting the wide range of diseases covered by this WP, ongoing studies were based on analysis of EBMT registry data covering secondary myelodysplasia, chronic myelomonocytic leukaemia, myeloma, amyloidosis, myelofibrosis, chronic myeloid leukaemia and chronic lymphocytic leukaemia. The scientific meeting was well attended and provided an opportunity for WP members to liaise closely with the data managers based in Leiden and statisticians in planning projects for the year.

The 45th EBMT Annual Meeting took place in Frankfurt, Germany, from 24th to 27th March and the WP had oral presentations on the impact of stopping TKI after alloHCT in patients with chronic myeloid leukemia, high-risk cytogenetics in newly diagnosed myeloma, the role of renal impairment at diagnosis in myeloma and outcomes after second allo-HCT in myeloma as well as several posters.

In December 2018, the Practice Harmonisation and Guidelines subcommittee of the CMWP had proposed a joint working party project to draft EBMT-JACIE Best Practice Recommendations on the Management of Adults and Children undergoing CAR-T cell therapy. The proposal was approved by the EBMT board on January 12th, 2019. An online survey was sent to fifty physicians in active CAR-T centres to solicit feedback on current approaches to the topics covered in the proposed guidelines. Their responses and the current medical literature in the field were discussed over three teleconferences in February and March and a two-day CAR-T workshop which took place in Lille in April. The guidelines were submitted to *Haematologica* in June, revised and accepted in November. This collective EBMT endeavour will hopefully provide guidance to physicians and nurses in best practice in this fast-moving field.

The second scientific and educational meeting of 2019 took place in Istanbul on 18th & 19th October and was organised by our colleague and local host, Meral Beksac from Ankara.

The large number of attendees spent a very enjoyable two days in the Vilayetler Evi hotel on the Bosphorus. In addition to a productive review of ongoing studies, the symposium on Saturday had informative sessions on Myelofibrosis (N. Kroeger), CLL (O. Tournilhac, O. Arslan), and Myelodysplastic Syndrome (Z. Gulbaş), Myeloma (L. Rosiñol, M. Beksac, F. Gay), Cellular therapies (S. Manier, I. Yakoub-Agha, P. Hayden) and GVHD (S. Civriz). An important advance at this meeting was the parallel nursing educational session organised by the CMWP Nursing Lead, Maaïke de Ruijter.



2nd Chronic Malignancies Working Party Business & Educational Meeting – 18-19 October 2019 in Istanbul, Turkey.

PRINCIPAL RESEARCH STUDIES

1. **Phase III: Vidaza vs allogeneic SCT in patients 55 to 69 years (N Kroger, U Platzbecker)**
2. **EMN RIC Allo Trial**
3. **Retrospective comparison between RIC and MAC for MF**
4. **Risk factors and outcomes after unrelated cord blood transplantation (UCBT) for patients with Chronic Myeloid Leukemia (CML): a collaborative study from the CMWP and Eurocord (H de Lavallade)**
5. **Retrospective study on dynamic IPSS in allo-HCT for MDS**
6. **Retrospective study on impact of cytogenetic in allo-HCT for complex karyotype MDS**
7. **Retrospective studies on allo-HCT for therapy-related MDS**

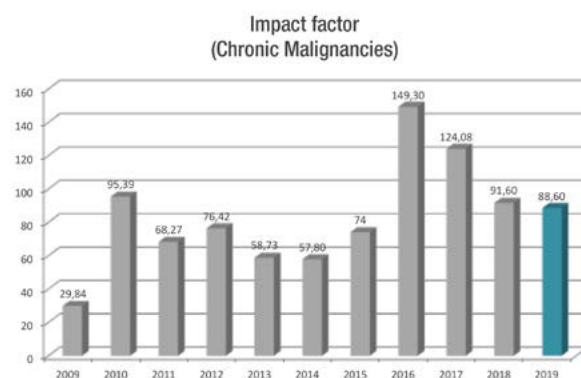
KEY PUBLICATIONS

1. Late Treatment-Related Mortality versus Competing Causes of Death after Allogeneic Transplantation for Myelodysplastic Syndromes and Secondary Acute Myeloid Leukemia. Schetelig J et al., *Leukemia*. DOI: 10.1038/s41375-018-0302-y
2. Tandem Autologous Stem Cell Transplantation Improves Outcomes in Newly Diagnosed Multiple Myeloma with Extramedullary Disease and High-Risk Cytogenetics: A Study from the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation. Gagelmann N et al., *Biol Blood Marrow Transplant*. DOI: 10.1016/j.bbmt.2019.07.004
3. Myeloablative and Reduced-Intensity Conditioned Allogeneic Hematopoietic Stem Cell Transplantation in Myelofibrosis: A Retrospective Study by the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation. McLornan D et al., *Biol Blood Marrow Transplant*. DOI: 10.1016/j.bbmt.2019.06.034
4. Allogeneic Stem Cell Transplantation for Blast Crisis Chronic Myeloid Leukemia in the Era of Tyrosine Kinase Inhibitors: A Retrospective Study by the EBMT Chronic Malignancies Working Party. Radujkovic A et al., *Biol Blood Marrow Transplant*. DOI: 10.1016/j.bbmt.2019.06.028
5. Management of Adults and Children Undergoing CAR T-Cell Therapy: Best Practice Recommendations of the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Yakoub-Agha I et al., *Haematologica*. DOI: 10.3324/haematol.2019.229781

MAJOR EDUCATIONAL COURSES

1. Chronic Malignancies Working Party Business & Educational Meeting – 18-19 October 2019 in Istanbul, Turkey.

Educational symposium on Chronic Malignancies and Cellular Therapies



	2015	2016	2017	2018	2019
Oral Presentations	33	16	12	13	9
Poster Presentations	12	16	14	17	8
International Educational Events	2	4	2	2	1



Chair
Grzegorz W. Basak

Transplant Complications Working Party (TCWP)

MAJOR ACHIEVEMENTS

2019 was a very successful year for the TCWP, both in terms of education and research. The working party is chaired by Grzegorz Basak, supported by Zinaida Peric (Secretary) and divided into the three subcommittees: Regimen-related toxicities and supportive care subcommittee chaired by Christian Koenecke, Graft-versus-host disease subcommittee chaired by Olaf Penack and Survivorship subcommittee chaired by Helene Schoemans. We currently manage approximately 40 studies and we have successfully published 12 manuscripts this year. Our work has been presented as oral presentations during the most important annual congresses - EBMT and ASH. Moreover, one of our studies (on allogeneic transplantations in patients with IBD) has been selected as a highlight of the EBMT.

The TCWP meets twice each year and conducts additional monthly teleconferences in order to follow the evolution of studies. We continued to keep strong links with Late effects and quality of life subcommittee of the CIBMTR which have resulted in very important collaboration and consensus manuscripts on transplant complications.

Finally, TCWP is strongly committed to educational activities and we continuously encourage EBMT members to join our working party with new proposals. Last May we organized a very dynamic first EBMT GVHD Summit. This meeting hosted a strong international faculty and gathered over 200 participants receiving a very positive feedback from the transplant community.

PRINCIPAL RESEARCH STUDIES

1. **EASIX score to predict outcomes of allo-HSCT (O. Penack, T. Luft) – validation and further investigation of new and easy score based on LDH, platelets and creatinine**
2. **Complications of CTX-based stem cell transplantation (G. Basak) non-invasive prospective study**
3. **Fecal transplant for GVHD (J. Bilinski)**
4. **Steroid-refractory GvHD (Z. Peric)**
5. **Risk Factors and Frequency of Lethal Central Nervous System Toxicity in Allogeneic Stem Cell Transplantation (C. Schultze-Florey)**



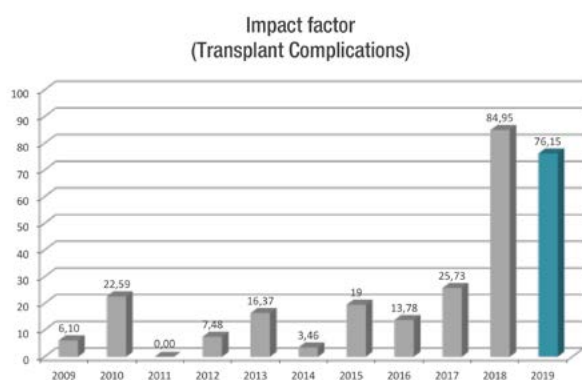
1st EBMT GVHD summit - 16-18 May 2019 in Warsaw, Poland.

KEY PUBLICATIONS

1. Association of uric acid levels before start of conditioning with mortality after alloSCT: a prospective, non-interventional study of the EBMT Transplant Complication Working Party. Penack O et al., *Hematologica*. DOI: 10.3324/haematol.2019.228668
2. European experience and risk factor analysis of donor cell-derived leukaemias/MDS following haematopoietic cell transplantation. Engel N et al., *Leukemia*. DOI: 10.1038/s41375-018-0218-6
3. Frequency of lethal central nervous system neurotoxicity in patients undergoing allogeneic stem cell transplantation: a retrospective registry analysis. Schultze-Florey CR et al., *Bone Marrow Transplant*. DOI: 10.1038/s41409-019-0738-3

MAJOR EDUCATIONAL COURSES

1st EBMT GVHD summit - 16-18 May 2019 in Warsaw, Poland.



	2015	2016	2017	2018	2019
Oral Presentations	2	2	4	2	2
Poster Presentations	1	3	10	2	4
International Educational Events	3	1	3	2	2

EBMT PUBLICATIONS 2019

TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
2017 ECIL 7 vaccine guidelines.	Cordonnier C	<i>Lancet Infect Dis.</i>	31250818
A prospective non-interventional study on the impact of transfusion burden and related iron toxicity on outcome in myelodysplastic syndromes undergoing allogeneic hematopoietic cell transplantation.	Cremers EMP	<i>Leuk Lymphoma.</i>	30997844
A survey on incidence and management of adenovirus infection after allogeneic HSCT.	Cesaro S	<i>Bone Marrow Transplant.</i>	30546071
A Toll-like receptor 2 genetic variant modulates occurrence of bacterial infections in patients with sickle cell disease.	Tozatto-Maio K	<i>Br J Haematol.</i>	30908604
Allogeneic haemopoietic transplantation for acute myeloid leukaemia in second complete remission: a registry report by the Acute Leukaemia Working Party of the EBMT.	Gilleece MH	<i>Leukemia.</i>	31363160
Allogeneic Hematopoietic Cell Transplantation in Patients Aged 50Years or Older with Severe Aplastic Anemia.	Rice C	<i>Biol Blood Marrow Transplant.</i>	30194027
Allogeneic Hematopoietic Stem Cell Transplantation for Congenital Immune Dysregulatory Disorders.	Bakhtiar S	<i>Front Pediatr.</i>	31799221
Allogeneic hematopoietic stem cell transplantation for patients with relapsed/refractory systemic anaplastic large cell lymphoma. A retrospective analysis of the Lymphoma Working Party of the European Society for Blood and Marrow Transplantation.	Domingo-Domènech E	<i>Bone Marrow Transplant.</i>	31695173
Allogeneic hematopoietic stem cell transplantation from unrelated donors is associated with higher infection rates in children with acute lymphoblastic leukemia-A prospective international multicenter trial on behalf of the BFM-SG and the EBMT-PDWP.	Pichler H	<i>Am J Hematol.</i>	31095771
Allogeneic HSCT for Autoimmune Diseases: A Retrospective Study From the EBMT ADWP, IEWP, and PDWP Working Parties.	Greco R	<i>Front Immunol.</i>	31333680
Allogeneic peripheral blood stem cell transplantation with anti-thymocyte globulin versus allogeneic bone marrow transplantation without anti-thymocyte globulin.	Baron F	<i>Haematologica.</i>	31413093
Allogeneic stem cell transplantation for acquired Pure Red Cell Aplasia.	Halkes C	<i>Am J Hematol.</i>	31396977
Allogeneic Stem Cell Transplantation for Blast Crisis Chronic Myeloid Leukemia in the Era of Tyrosine Kinase Inhibitors: A Retrospective Study by the EBMT Chronic Malignancies Working Party.	Radujkovic A	<i>Biol Blood Marrow Transplant.</i>	31271884
Allogeneic stem cell transplantation in second complete remission for core binding factor acute myeloid leukemia: a study from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Halaburda K	<i>Haematologica.</i>	31439677
Allogeneic stem cell transplantation using HLA-matched donors for acute myeloid leukemia with deletion 5q or monosomy 5: a study from the Acute Leukemia Working Party of the EBMT.	Poiré X	<i>Haematologica.</i>	31048355
Allogeneic stem-cell transplantation with sequential conditioning in adult patients with refractory or relapsed acute lymphoblastic leukemia: a report from the EBMT Acute Leukemia Working Party.	Bazarbachi AH	<i>Bone Marrow Transplant.</i>	31562398
An international survey on the management of patients receiving CAR T-cell therapy for haematological malignancies on behalf of the Chronic Malignancies Working Party of EBMT.	Hayden PJ	<i>Curr Res Transl Med.</i>	31182380
Analysis of data collected in the European Society for Blood and Marrow Transplantation (EBMT) Registry on a cohort of lymphoma patients receiving plerixafor.	Sureda A	<i>Bone Marrow Transplant.</i>	31570781
Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT.	Risitano AM	<i>Front Immunol.</i>	31258525
Antilymphocyte globulin for matched sibling donor transplantation in patients with myelofibrosis.	Robin M	<i>Haematologica.</i>	30655365
Anti-thymocyte globulin for graft-versus-host disease prophylaxis in patients with intermediate- or high-risk acute myeloid leukaemia undergoing reduced-intensity conditioning allogeneic stem cell transplantation in first complete remission - a survey on behalf of the Acute Leukaemia Working Party of the European Society for Blood and Marrow Transplantation.	Ofran Y	<i>Br J Haematol.</i>	29468648
Association of aplastic anaemia and lymphoma: a report from the severe aplastic anaemia working party of the European Society of Blood and Bone Marrow Transplantation.	Rovó A	<i>Br J Haematol.</i>	29265360
Association of uric acid levels before start of conditioning with mortality after alloSCT: a prospective, non-interventional study of the EBMT Transplant Complication Working Party.	Penack O	<i>Haematologica.</i>	31601686

TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia: a joint study of the Autoimmune Diseases and Severe Aplastic Anaemia Working Parties (ADWP/SAAWP) of the European Society for Blood and Marrow Transplantation (EBMT).	Miller PDE	<i>Bone Marrow Transplant.</i>	31554929
Autologous haematopoietic stem cell therapy for multiple sclerosis: a review for supportive care clinicians on behalf of the Autoimmune Diseases Working Party of the European Society for Blood and Marrow Transplantation.	Ismail A	<i>Curr Opin Support Palliat Care.</i>	31599815
Autologous haematopoietic stem cell transplantation (HSCT) for anti-neutrophil cytoplasmic antibody (ANCA)-associated vasculitis: a retrospective survey of patients reported to European Society for Blood and Marrow Transplantation (EBMT) registry.	Alexander T	<i>Bone Marrow Transplant.</i>	31848450
Autologous haematopoietic stem cell transplantation and other cellular therapy in multiple sclerosis and immune-mediated neurological diseases: updated guidelines and recommendations from the EBMT Autoimmune Diseases Working Party (ADWP) and the Joint Accreditation Committee of EBMT and ISCT (JACIE).	Sharrack B	<i>Bone Marrow Transplant.</i>	31558790
Autologous hematopoietic stem cell transplantation for relapsed/refractory systemic anaplastic large cell lymphoma. A retrospective analysis of the lymphoma working party (LWP) of the EBMT.	Domingo-Domènech E	<i>Bone Marrow Transplant.</i>	31695174
Autologous stem cell transplantation for HIV-associated lymphoma in the antiretroviral and rituximab era: a retrospective study by the EBMT Lymphoma Working Party.	Hübel K	<i>Bone Marrow Transplant.</i>	30804486
Autologous stem cell transplantation in refractory Crohn's disease - low intensity therapy evaluation (ASTIClite): study protocols for a multicentre, randomised controlled trial and observational follow up study.	Snowden JA	<i>BMC Gastroenterol.</i>	31151436
Benchmarking of survival outcomes following haematopoietic stem cell transplantation: A review of existing processes and the introduction of an international system from the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE).	Snowden JA	<i>Bone Marrow Transplant.</i>	31636397
Beneficial role of CD8+ T-cell reconstitution after HLA-haploidentical stem cell transplantation for high-risk acute leukaemias: results from a clinico-biological EBMT registry study mostly in the T-cell-depleted setting.	Bondanza A	<i>Bone Marrow Transplant.</i>	30531916
CD19 chimeric antigen receptor-T cells in B-cell leukemia and lymphoma: current status and perspectives.	Mohty M	<i>Leukemia.</i>	31690821
Central nervous system disorders after hematopoietic stem cell transplantation: a prospective study of the Infectious Diseases Working Party of EBMT.	Schmidt-Hieber M	<i>J Neurol.</i>	31664549
Clinical and morphological practices in the diagnosis of transplant-associated microangiopathy: a study on behalf of Transplant Complications Working Party of the EBMT.	Moiseev IS	<i>Bone Marrow Transplant.</i>	30361500
Clinical applications of donor lymphocyte infusion from an HLA-haploidentical donor: consensus recommendations from the acute leukemia working party of the EBMT.	Dholaria B	<i>Haematologica.</i>	31537691
Clinical utilization of Chimeric Antigen Receptor T-cells (CAR-T) in B-cell acute lymphoblastic leukemia (ALL)—an expert opinion from the European Society for Blood and Marrow Transplantation (EBMT) and the American Society for Blood and Marrow Transplantation (ASBMT)	Kansagra AJ	<i>Bone Marrow Transplant.</i>	31092900
Comparable Long-Term Outcome after Allogeneic StemShimoni A-Cell Transplantation from Sibling and Matched Unrelated Donors in AML Patients Older than 50 years. A Report on Behalf of the ALWP of EBMT.	Shimoni A	<i>Biol Blood Marrow Transplant.</i>	31271887
Conditioning Perspectives for Primary Immunodeficiency Stem Cell Transplants.	Shaw P	<i>Front Pediatr.</i>	31781522
Conditioning-based outcomes after allogeneic transplantation for myeloma following a prior autologous transplant (1991-2012) on behalf of EBMT CMWP.	Hayden PJ	<i>Eur J Haematol.</i>	31737951
Consensus recommendations for the role and competencies of the EBMT clinical pharmacist and clinical pharmacologist involved in hematopoietic stem cell transplantation.	Langebrake C	<i>Bone Marrow Transplant.</i>	31101890
Current practice in vitamin D management in Allogeneic Haematopoietic Stem Cell Transplantation: a survey by the Transplant Complications Working Party of the EBMT.	Ros-Soto J	<i>Biol Blood Marrow Transplant.</i>	31229642
Death after hematopoietic stem cell transplantation: changes over calendar year time, infections and associated factors.	Styczyński J	<i>Bone Marrow Transplant.</i>	31455899
Development of adaptive immune effector therapies in solid tumors.	Comoli P	<i>Ann Oncol.</i>	31435646
Diagnosis, grading, and treatment recommendations for children, adolescents, and young adults with sinusoidal obstructive syndrome: an international expert position statement.	Mahadeo KM	<i>Lancet Haematol.</i>	31818728
Donor selection for a second allogeneic stem cell transplantation in AML patients relapsing after a first transplant: a study of the Acute Leukemia Working Party of EBMT.	Shimoni A	<i>Blood Cancer J.</i>	31740656

TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
European experience and risk factor analysis of donor cell-derived leukaemias/MDS following haematopoietic cell transplantation.	Engel N	<i>Leukemia.</i>	30050122
Evaluating the clinical effectiveness of autologous haematopoietic stem cell transplantation versus disease-modifying therapy in multiple sclerosis using a matching-adjusted indirect comparison: an exploratory study from the Autoimmune Diseases Working Party (ADWP) of the European Society of Bone and Marrow Transplantation (EBMT).	Tappenden P	<i>Bone Marrow Transplant.</i>	31745252
Evaluation of Second Solid Cancers After Hematopoietic Stem Cell Transplantation in European Patients.	Tichelli A	<i>JAMA Oncol.</i>	30476975
Family Mismatched Allogeneic Stem Cell Transplantation for Myelofibrosis: Report from the Chronic Malignancies Working Party of European Society for Blood and Marrow Transplantation.	Raj K	<i>Biol Blood Marrow Transplant.</i>	30408564
Fludarabine/busulfan versus fludarabine/total-body-irradiation (2 Gy) as conditioning prior to allogeneic stem cell transplantation in patients (≥ 60 years) with acute myelogenous leukemia: a study of the acute leukemia working party of the EBMT.	Heinicke T	<i>Bone Marrow Transplant.</i>	31645668
Fludarabine-treosulfan compared to thiotepa-busulfan-fludarabine or FLAMSA as conditioning regimen for patients with primary refractory or relapsed acute myeloid leukemia: a study from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation (EBMT).	Saraceni F	<i>J Hematol Oncol.</i>	31023346
Frequency of lethal central nervous system neurotoxicity in patients undergoing allogeneic stem cell transplantation: a retrospective registry analysis.	Schultze-Florey CR	<i>Bone Marrow Transplant.</i>	31695171
General information for patients and carers considering haematopoietic stem cell transplantation (HSCT) for severe autoimmune diseases (ADs): A position statement from the EBMT Autoimmune Diseases Working Party (ADWP), the EBMT Nurses Group, the EBMT Patient, Family and Donor Committee and the Joint Accreditation Committee of ISCT and EBMT (JACIE).	Jessop H	<i>Bone Marrow Transplant.</i>	30705338
Gonadal Function after Busulfan Compared with Treosulfan in Children and Adolescents Undergoing Allogeneic Hematopoietic Stem Cell Transplant.	Faraci M	<i>Biol Blood Marrow Transplant.</i>	31082473
Graft-versus-host disease and graft-versus-leukaemia effects in secondary acute myeloid leukaemia: a retrospective, multicentre registry analysis from the Acute Leukaemia Working Party of the EBMT.	Baron F	<i>Br J Haematol.</i>	31612473
Guidelines for Secondary Solid Cancers Among HSCT Recipients-In Reply.	Tichelli A	<i>JAMA Oncol.</i>	31070680
Guidelines for the management of cytomegalovirus infection in patients with haematological malignancies and after stem cell transplantation from the 2017 European Conference on Infections in Leukaemia (ECIL 7).	Ljungman P	<i>Lancet Infect Dis.</i>	31153807
Guidelines from the 2017 European Conference on Infections in Leukaemia for management of HHV-6 infection in patients with hematologic malignancies and after hematopoietic stem cell transplantation.	Ward KN	<i>Haematologica.</i>	31467131
Haploidentical stem cell transplantation for patients with lymphoma: a position statement from the Lymphoma Working Party-European Society for Blood and Marrow Transplantation	Dietrich S	<i>Bone Marrow Transplant.</i>	31150019
Haploidentical transplantation and posttransplant cyclophosphamide for treating aplastic anemia patients: a report from the EBMT Severe Aplastic Anemia Working Party.	Prata PH	<i>Bone Marrow Transplant.</i>	31844137
Haploidentical vs. unrelated allogeneic stem cell transplantation for acute lymphoblastic leukemia in first complete remission: on behalf of the ALWP of the EBMT.	Shem-Tov N	<i>Leukemia.</i>	31427719
Hematopoietic cell transplant nurse coordinators' perceptions of related donor care: a European survey from the EBMT Nurses Group.	Polomeni A	<i>Bone Marrow Transplant.</i>	31578465
Hematopoietic Cell Transplantation for MHC Class II Deficiency.	Lum SH	<i>Front Pediatr.</i>	31921728
Hematopoietic Stem Cell Transplantation as Treatment for Patients with DOCK8 Deficiency.	Aydin SE	<i>J Allergy Clin Immunol Pract.</i>	30391550
Hematopoietic stem cell transplantation for CD40 ligand deficiency: Results from an EBMT/ESID-IEWP-SCETIDE-PIDTC study.	Ferrua F	<i>J Allergy Clin Immunol.</i>	30660643
Hematopoietic Stem Cell Transplantation for Primary Immunodeficiencies.	Gennery AR	<i>Front Pediatr.</i>	31737589
Hematopoietic Stem Cell Transplantation in Inborn Errors of Metabolism.	Tan EY	<i>Front Pediatr.</i>	31709204
Hematopoietic stem cell transplantation with unrelated cord blood or haploidentical donor grafts in adult patients with secondary acute myeloid leukemia, a comparative study from Eurocord and the ALWP EBMT	Ruggeri A	<i>Bone Marrow Transplant.</i>	31150016
High dose chemotherapy and autologous hematopoietic cell transplantation for Wilms tumor: a study of the European Society for Blood and Marrow Transplantation.	Spreafico F	<i>Bone Marrow Transplant.</i>	31534191
Ibrutinib as a salvage therapy after allogeneic HCT for chronic lymphocytic leukemia.	Michallet M	<i>Bone Marrow Transplant.</i>	31700137

TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
Ibrutinib for bridging to allogeneic hematopoietic cell transplantation in patients with chronic lymphocytic leukemia or mantle cell lymphoma: a study by the EBMT Chronic Malignancies and Lymphoma Working Parties.	Dreger P	<i>Bone Marrow Transplant.</i>	29728701
Impact of antithymocyte globulin on outcomes of allogeneic hematopoietic cell transplantation with TBI.	Nagler A	<i>Blood Adv.</i>	31262738
Impact of conditioning intensity on outcomes of haploidentical stem cell transplantation for patients with acute myeloid leukemia over 45 years of age.	Santoro N	<i>Cancer.</i>	30620383
Impact of primary disease on outcome after allogeneic stem cell transplantation for transformed secondary acute leukaemia.	Kröger N	<i>Br J Haematol.</i>	30820933
Impact of T-cell depletion strategies on outcomes following hematopoietic stem cell transplantation for idiopathic aplastic anemia: A study on behalf of the European blood and marrow transplant severe aplastic anemia working party.	Samarasinghe S	<i>Am J Hematol.</i>	30328134
Incidence and outcome of Kaposi sarcoma after hematopoietic stem cell transplantation: a retrospective analysis and a review of the literature, on behalf of infectious diseases working party of EBMT.	Cesaro S	<i>Bone Marrow Transplant.</i>	31435035
Indications for haematopoietic stem cell transplantation for haematological diseases, solid tumours and immune disorders: current practice in Europe, 2019.	Duarte RF	<i>Bone Marrow Transplant.</i>	30953028
Individualized prediction of leukemia-free survival after autologous stem cell transplantation in acute myeloid leukemia.	Shouval R	<i>Cancer.</i>	31225904
Influence of donor type, stem cell source and conditioning on outcomes after haploidentical transplant for lymphoma - a LWP-EBMT study.	Bazarbachi A	<i>Br J Haematol.</i>	31498883
Late treatment-related mortality versus competing causes of death after allogeneic transplantation for myelodysplastic syndromes and secondary acute myeloid leukemia.	Schetelig J	<i>Leukemia.</i>	30573777
Leukemia relapse following unmanipulated haploidentical transplantation: a risk factor analysis on behalf of the ALWP of the EBMT.	Piemontese S	<i>J Hematol Oncol.</i>	31272508
Long Term Outcome and Immune Function After Hematopoietic Stem Cell Transplantation for Primary Immunodeficiency.	Gennery AR	<i>Front Pediatr.</i>	31616648
Long-term outcome after allogeneic hematopoietic cell transplantation for myelofibrosis.	Robin M	<i>Haematologica.</i>	30733269
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Long-term outcome of LRBA deficiency in 76 patients after various treatment modalities as evaluated by the immune deficiency and dysregulation activity (IDDA) score.	Tesch VK	<i>J Allergy Clin Immunol.</i>	31887391
Maintenance Therapies for Hodgkin and Non-Hodgkin Lymphomas After Autologous Transplantation: A Consensus Project of ASBMT, CIBMTR, and the Lymphoma Working Party of EBMT.	Kanate AS	<i>JAMA Oncol.</i>	30816957
Management of adults and children undergoing CAR t-cell therapy: best practice recommendations of the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE).	Yakoub-Agha I	<i>Haematologica.</i>	31753925
Management of growth failure and growth hormone deficiency after pediatric allogeneic HSCT: Endocrinologists are of importance for further guidelines and studies.	Lawitschka A	<i>Pediatr Hematol Oncol.</i>	31633441
Measurable residual disease at myeloablative allogeneic transplantation in adults with acute lymphoblastic leukemia: a retrospective registry study on 2780 patients from the acute leukemia working party of the EBMT.	Pavlů J	<i>J Hematol Oncol.</i>	31647022
Myeloablative and Reduced-Intensity Conditioned Allogeneic Hematopoietic Stem Cell Transplantation in Myelofibrosis: A Retrospective Study by the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation.	McLornan D	<i>Biol Blood Marrow Transplant.</i>	31284069
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Non-GVHD ocular complications after hematopoietic cell transplantation: expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.	Inamoto Y	<i>Bone Marrow Transplant.</i>	30809032
Ocular graft-versus-host disease after hematopoietic cell transplantation: Expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.	Inamoto Y	<i>Bone Marrow Transplant.</i>	30531954
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TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
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Outcome of allogeneic hematopoietic stem cell transplantation in patients over 69 years of age with acute myeloid leukemia: on behalf of the acute leukemia working party of the EBMT.	Ringdén O	<i>Biol Blood Marrow Transplant.</i>	31181255
Outcomes of allogeneic haematopoietic stem cell transplantation from HLA-matched and alternative donors: a European Society for Blood and Marrow Transplantation registry retrospective analysis.	Shouval R	<i>Lancet Haematol.</i>	31477550
Post remission consolidation by autologous HCT for AML in CR1, negative implications for subsequent allogeneic HCT in CR2. A Study by the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation (EBMT).	Passweg JR	<i>Biol Blood Marrow Transplant.</i>	31759159
Posttransplant cyclophosphamide vs antithymocyte globulin in HLA-mismatched unrelated donor transplantation.	Battipaglia G	<i>Blood.</i>	31270102
Pre-transplantation Risks and Transplant-Techniques in Haematopoietic Stem Cell Transplantation for Acute Leukaemia.	Gratwohl A	<i>EClinicalMedicine.</i>	31709412
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Prognostic factors for adult single cord blood transplantation among European and Japanese populations: the Eurocord/ALWP-EBMT and JSHCT/JDCHCT collaborative study.	Kanda J	<i>Leukemia.</i>	31409921
Prognostic impact of EBV serostatus in patients with lymphomas or chronic malignancies undergoing allogeneic HCT	Styczynski J	<i>Bone Marrow Transplant.</i>	31363166
Prognostic significance of recurring chromosomal abnormalities in transplanted patients with acute myeloid leukemia.	Canaani J	<i>Leukemia.</i>	30846862
PTCy-based haploidentical vs matched related or unrelated donor reduced-intensity conditioning transplant for DLBCL.	Dreger P	<i>Blood Adv.</i>	30723110
Reduced intensity conditioning regimens including alkylating chemotherapy do not alter survival outcomes after allogeneic hematopoietic cell transplantation in chronic lymphocytic leukemia compared to low-intensity non-myeloablative conditioning.	Andersen NS	<i>J Cancer Res Clin Oncol.</i>	31468122
Relapse of Aplastic Anemia with Majority Donor Chimerism (Donor-Type Aplasia) Occurring Late after Bone Marrow Transplantation.	Shaw A	<i>Biol Blood Marrow Transplant.</i>	31733299
Reporting data of patients receiving CAR T cell therapy into the EBMT registry: Guidelines of the Francophone Society of Bone Marrow Transplantation and Cellular Therapy (SFGM-TC).	Vasseur A	<i>Bull Cancer.</i>	31831153
Results from a multicenter, noninterventional registry study for multiple myeloma patients who received stem cell mobilization regimens with and without plerixafor.	Morris C	<i>Bone Marrow Transplant.</i>	31534192
Risk factors affecting outcome of unrelated cord blood transplantation for children with familial haemophagocytic lymphohistiocytosis.	Furtado-Silva JM	<i>Br J Haematol.</i>	30460979
Risk factors and outcomes according to age at transplantation with an HLA-identical sibling for sickle cell disease.	Cappelli B	<i>Haematologica.</i>	31018975
Role of Age and Hematopoietic Cell Transplantation-Specific Comorbidity Index in Myelodysplastic Patients Undergoing an Allograft: A Retrospective Study from the Chronic Malignancies Working Party of the European Group for Blood and Marrow Transplantation.	Carré M	<i>Biol Blood Marrow Transplant.</i>	31647984
Role of physical therapy pre and post hematopoietic stem cell transplantation - White paper report.	Mohammed J	<i>Biol Blood Marrow Transplant.</i>	30658224
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Single-Dose Daily Fractionation Is Not Inferior to Twice-a-Day Fractionated Total-Body Irradiation Before Allogeneic Stem Cell Transplantation for Acute Leukemia: A Useful Practice Simplification Resulting From the SARASIN Study.	Belkacemi Y	<i>Int J Radiat Oncol Biol Phys.</i>	29928948
Sorafenib improves survival of FLT3-mutated acute myeloid leukemia in relapse after allogeneic stem cell transplantation: a report of EBMT acute leukemia Working Party.	Bazarbachi A	<i>Haematologica.</i>	30792203
State-of-the-art review: allogeneic stem cell transplantation for myelofibrosis in 2019.	McLornan DP	<i>Haematologica.</i>	30872371
Stem cell transplantation for congenital dyserythropoietic anemia: an analysis from the European Society for Blood and Marrow Transplantation.	Miano M	<i>Haematologica.</i>	30679331

TITLE	1 ST LISTED AUTHOR	JOURNAL	PMID
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Tandem Autologous Stem Cell Transplantation Improves Outcomes in Newly Diagnosed Multiple Myeloma with Extramedullary Disease and High-Risk Cytogenetics: A Study from the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation.	Gagelmann N	<i>Biol Blood Marrow Transplant.</i>	31288095
The Disease-Risk Stratification Scheme (DRSS), a Contemporary Risk-Stratification System for Allogeneic Stem Cell Transplantation.	Shouval R	<i>Blood.</i>	31724016
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The impact of anti-thymocyte globulin on the outcomes of Patients with AML with or without measurable residual disease at the time of allogeneic hematopoietic cell transplantation.	Nagler A	<i>Leukemia.</i>	31728052
The prognostic impact of the cytomegalovirus serostatus in patients with chronic hematological malignancies after allogeneic hematopoietic stem cell transplantation: a report from the Infectious Diseases Working Party of EBMT.	Schmidt-Hieber M	<i>Ann Hematol.</i>	30993417
The rise of autologous HCT for autoimmune diseases: what's behind it and what does it mean for the future of treatment? A update on behalf of the EBMT Autoimmune Diseases Working Party.	Snowden JA	<i>Expert Rev Clin Immunol.</i>	31414932
Transplant center practices for psychosocial assessment and management of pediatric hematopoietic stem cell donors.	Wiener L	<i>Bone Marrow Transplant.</i>	30971776
Transplant outcomes for patients with therapy-related acute myeloid leukemia with prior lymphoid malignancy: an ALWP of EBMT study.	Gatwood KS	<i>Bone Marrow Transplant.</i>	31527819
Trends in the use of hematopoietic stem cell transplantation for adults with acute lymphoblastic leukemia in Europe: a report from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation (EBMT).	Giebel S	<i>Ann Hematol.</i>	31392462
Umbilical cord blood versus unrelated donor transplantation in adults with primary refractory or relapsed acute myeloid leukemia: a report from Eurocord, the Acute Leukemia Working Party and the Cord Blood Committee of the Cellular Therapy and Immunobiology Working Party of the EBMT.	Baron F	<i>Blood Cancer J.</i>	30979868
Unmanipulated haploidentical versus HLA-matched sibling allogeneic hematopoietic stem cell transplantation in relapsed/refractory acute myeloid leukemia: a retrospective study on behalf of the ALWP of the EBMT.	Battipaglia G	<i>Bone Marrow Transplant.</i>	30718798
Use of busulfan in conditioning for allogeneic hematopoietic stem cell transplantation in adults: a survey by the Transplant Complications Working Party of the EBMT	Ruutu T	<i>Bone Marrow Transplant.</i>	31160806
Use of eltrombopag in aplastic anemia in Europe.	Ecsedi M	<i>Ann Hematol.</i>	30915499
Vaccination of haemopoietic stem cell transplant recipients: guidelines of the 2017 European Conference on Infections in Leukaemia (ECIL 7).	Cordonnier C	<i>Lancet Infect Dis.</i>	30744963
Vaccination of patients with haematological malignancies who did not have transplantations: guidelines from the 2017 European Conference on Infections in Leukaemia (ECIL 7).	Mikulska M	<i>Lancet Infect Dis.</i>	30744964

EBMT TRANSPLANT ACTIVITY SURVEY

2018 ACTIVITY SURVEY ON HEMATOPOIETIC CELL TRANSPLANTATION AND HEMATOPOIETIC CELLULAR THERAPIES

HEMATOPOIETIC CELL TRANSPLANTATION

In 2018, 701 centers reported 47,468 transplants in 42,901 patients (first transplant) from 50 countries to the annual activity survey. Of these, 19,630 HCT (41%) were allogeneic and 27,838 (59%) autologous (Table 1a). In addition, there were 4,567 second or subsequent transplants. When compared with 2017, the total number of transplants increased by 4.5% (7.4% allogeneic HCT and 2.6% autologous HCT). The number of pediatric patients (<18 at HCT) transplanted in both dedicated pediatric and joint adult-pediatric units was 5,368 (4,075 allogeneic and 1,293 autologous). This is an increase of 9.4% allogeneic HCT and a decrease of 3% autologous HCT compared to 2017.

Main indications for HCT were myeloid malignancies (AML, CML, MDS or MD/MPN overlap and MPN): 10,679 (97% allogeneic HCT); lymphoid malignancies (ALL, CLL, HL, NHL and PCD): 27,318 (64%; 19% allogeneic); solid tumors: 1,625 (3.8%; 3% allogeneic); non-malignant disorders: 3,063 (7%; 81% allogeneic) and others: 216 (0.5%).

Important trends in allogeneic HCT include a 49% increase for chronic phase CML (although transplant numbers remain low) and a 24% increase in aplastic anemia. Growth is observed generally in well-established indications such as AML in CR1 and ALL, more so in advanced disease than with patients in CR1. In autologous HCT, there is an ongoing increase in autoimmune diseases (by 19%), predominantly due to activity in multiple sclerosis.

The numbers of HCT using bone marrow or peripheral blood stem cells from haploidentical and unrelated donors appear to increase simultaneously; 16.8% increase for haploidentical donors, 6.6% for unrelated donors. Cord blood use continues to decline; by 15% since 2017.

CELLULAR THERAPIES

In the 2018, 1,325 patients in 28 countries received hematopoietic cellular therapies (excluding donor lymphocyte infusions). These immune effector therapies qualify as medicinal products rather than cell transplants and are defined as an infusion of cells, undergoing substantial manipulation after collection, either selection and/or expansion, or genetic modification (Table 1b). The most widely used cellular therapy ahead of CAR-T cells in 2018 are mesenchymal stromal cells (n=460; 87% allogeneic), their use being mainly to treat graft versus host disease and expanded/selected T lymphocytes (n=122; 98% allogeneic). A notable increase seen was in gene modified T-cells, notably CAR-T cells from 151 to 301 (100% increase) in patients treated in 2018 (figure 1a, 1b). Since only two approved products received a centralized marketing approval from EMA in August 2018, it is likely that the reported activity for 2018 partially reflects clinical studies, either industry-sponsored or academia-sponsored. Whilst most other cellular therapies appear to be decreasing slightly, dendritic cells, which have increased from 44 in 2017 to 77 (75%) in 2018. In addition, 3,096 un-manipulated donor lymphocyte infusions (DLI) were reported, an increase of 9.6% since 2017. The majority of DLI's were given for relapse (1,345) and graft enhancement (738).

The well-established practice of transparently sharing data on activity of advanced therapy medicinal products manufactured from hematopoietic cells used by the annual survey continues to reflect current activity and trends in the field of transplant technology. It is valuable for the dissemination of the most recent information on HCT and cellular therapies, and is used for benchmarking of data completeness, which can ultimately be beneficial in health care planning.

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ARTICLE

The EBMT activity survey on hematopoietic-cell transplantation and cellular therapy 2018: CAR-T's come into focus

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Abstract

Hematopoietic-cell transplantation (HCT) is widely used for acquired and congenital disorders of the hematopoietic system. Number of transplants performed in Europe and associated countries continues to rise with 47,468 HCT in 42,901 patients [19,630 allogeneic (41%) and 27,838 autologous (59%)] reported by 701 centers in 50 countries in 2018. Main indications were myeloid malignancies 10,679 (25%; 97% allogeneic), lymphoid malignancies 27,318 (64%; 19% allogeneic), solid tumors 1,625 (4%; 3% allogeneic), and nonmalignant disorders 3,063 (7%; 81% allogeneic). This year's analysis focuses on cellular therapies with the marked growth in CAR T-cell therapies from 151 in 2017 to 301 patients reported in 2018. Other cellular therapy numbers show less significant changes. Important trends in HCT include a 49% increase in allogeneic HCT for chronic phase CML (although transplant numbers remain low) and a 24% increase in aplastic anemia. In autologous HCT, there is an ongoing increase in autoimmune diseases (by 19%), predominantly due to activity in multiple sclerosis. This annual report reflects current activity and highlights important trends, useful for health care planning.

Table 1:

NUMBERS OF HEMATOPOIETIC STEM CELL TRANSPLANTS IN EUROPE 2018 BY INDICATION, DONOR TYPE AND STEM CELL SOURCE.

	TRANSPLANT ACTIVITY 2018																	
	No. of patients																	
	Allogeneic												Autologous			Total		
	Family									Unrelated								
	HLA-id			Twin	Haplo >=2MM		Other family						BM	BM +		Allo	Auto	
	BM	PBPC	Cord	all	BM	PBSC	BM	PBPC	Cord	BM	PBPC	cord	only	PBPC	cord			
Myeloid malignancies	312	2745	1	11	401	1102	6	79	1	461	5147	119	3	291	0	10385	294	10679
Acute myeloid leukemia	220	1959	1	7	267	801	5	54	0	263	3279	87	3	290	0	6943	293	7236
1st complete remission	146	1280		5	135	399	5	34		175	1790	39	3	238		4008	241	4249
not 1st complete remission	57	442	1	2	93	267		14		56	942	36		43		1910	43	1953
AML therapy related	7	68			8	32				7	162	2		1		286	1	287
AML from MDS/MPN	10	169			31	103		6		25	385	10		8		739	8	747
Chronic myeloid leukemia	12	107	0	0	13	34	0	1	0	21	176	8	0	0	0	372	0	372
chronic phase	8	60			6	15		1		17	90	5				202	0	202
not chronic phase	4	47			7	19				4	86	3				170	0	170
MDS or MD/MPN overlap	68	493		2	86	205	1	16		154	1275	22		1		2322	1	2323
MPN	12	186		2	35	62		8	1	23	417	2				748	0	748
Lymphoid malignancies	350	1395	3	9	239	722	11	37	1	392	2135	75	37	21912	0	5369	21949	27318
Acute lymphatic leukemia	288	727	3	2	112	362	8	19	1	331	1046	66	4	70	0	2965	74	3039
1st complete remission	167	519	2	1	39	169	4	14		174	660	31	3	61		1780	64	1844
not 1st complete remission	121	208	1	1	73	193	4	5	1	157	386	35	1	9		1185	10	1195
Chronic lymphocytic leukemia	6	39		1	1	20		1		7	116	1		10		192	10	202
Plasma cell disorders - MM	8	113		2	15	38		2		6	199	1	1	12758		384	12759	13143
Plasma cell disorders - other		12			1	3					15	1		387		32	387	419
Hodgkin lymphoma	14	105		1	50	96	2	1		5	154	2	14	2107		430	2121	2551
Non Hodgkin lymphoma	34	399		3	60	203	1	14		43	605	4	18	6580		1366	6598	7964
Solid tumors	5	3	0	0	4	25	0	0	0	3	7	0	32	1545	1	47	1578	1625
Neuroblastoma	4	1			4	21					1		23	495		31	518	549
Soft tissue sarcoma/Ewing	1					2					1		4	241		4	245	249
Germinal tumors		2				1							1	380		3	381	384
Breast cancer										2				23		2	23	25
Other solid tumors						1				1	5		4	406	1	7	411	418
Non malignant disorders	736	351	29	7	117	174	69	56	1	510	388	49	7	568	1	2487	576	3063
Bone marrow failure - SAA	214	147	1	4	31	39	6	6		144	124	6		3	1	722	4	726
Bone marrow failure - other	68	21	3	1	15	13	4	10		64	31	2		1		232	1	233
Thalassemia	169	74	14	1	3	15	15	14		66	43			8		414	8	422
Sickle cell disease	110	46	8		12	13	9	4		17	6					225	0	225
Primary Immune deficiencies	136	43	1	1	52	85	29	15	1	149	150	14	3	4		676	7	683
Inh. disorders of Metabolism	37	13	2		3	8	5	6		64	33	27	3	3		198	6	204
Auto immune disease	2	7			1	1	1	1		6	1		1	549		20	550	570
Others	38	29			12	16	4	14	1	27	45	9		21		195	21	216
TOTAL PATIENTS	1441	4523	33	27	773	2039	90	186	4	1393	7722	252	79	24337	2	18483	24418	42901
Re/additional transplants	29	177	1	2	75	296	10	10		51	474	22	7	3413		1147	3420	4567
TOTAL TRANSPLANTS	1470	4700	34	29	848	2335	100	196	4	1444	8196	274	86	27750	2	19630	27838	47468

Table 2:

NUMBERS OF CELLULAR THERAPIES IN EUROPE 2018 BY INDICATION, DONOR TYPE AND CELL SOURCE.

Number of patients	DLI	CAR T		selected/expanded T cells or CIK		Regulatory T cells (TREGS)		Genetically modified T cells		Natural Killer cells		Dendritic cells		Mesenchymal stem cells		Genetically modified CD34+ cells		Other cell therapies		Total excluding DLI	Total
2018		Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	allo	auto
GvHD				2		38						22		318		4		19		403	0
Graft enhancement	738			6						1		1		39		6		106	41	159	41
Autoimmune dis.												4		7	16					7	20
Genetic disease														2		9	6			11	6
Infection				97		1		5						10			1	4	2	117	3
Malignancy		19	282	15	2					12		21	29		10	1		24	2	92	325
DLI for residual disease	433																			0	0
DLI for relapse	1345																			0	0
DLI per protocol	580																			0	0
Regenerative medicine									1	1				25	33			10	71	36	105
Total	3096	19	282	120	2	39	0	5	1	14	0	44	33	401	59	20	7	163	116	825	500

Figure 1b:

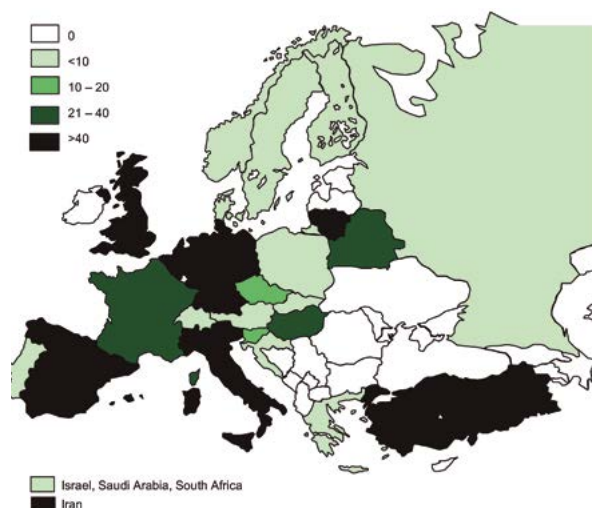
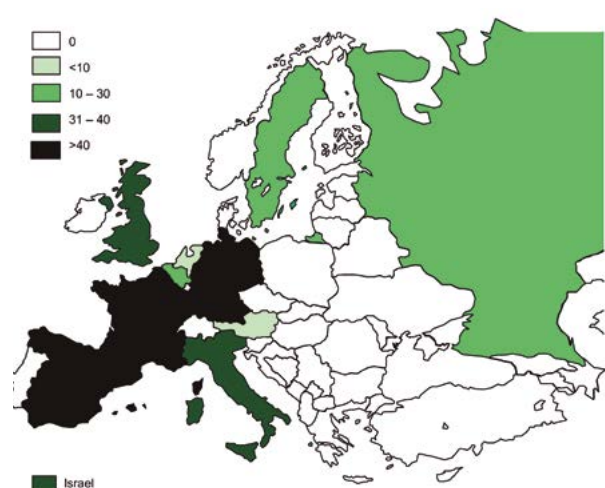
ABSOLUTE NUMBERS OF PATIENTS RECEIVING OTHER NON CAR-T CELLULAR THERAPIES

Figure 1a:

ABSOLUTE NUMBERS OF PATIENTS RECEIVING CAR-T CELLULAR THERAPIES**Abbreviations:**

AML; acute myeloid leukemia, ALL; acute lymphoblastic leukemia, CML; chronic myeloid leukemia, MDS or MD/MPN overlap; myelodysplastic or myelodysplastic/ myeloproliferative neoplasm, MPN; myeloproliferative neoplasm, CLL; chronic lymphocytic leukemia, PCD; plasma cell disorders, MM; multiple myeloma, HL; Hodgkin lymphoma, NHL; Non-Hodgkin lymphoma; BMF; bone marrow failure, SAA; severe aplastic anemia. CR1; first complete remission.

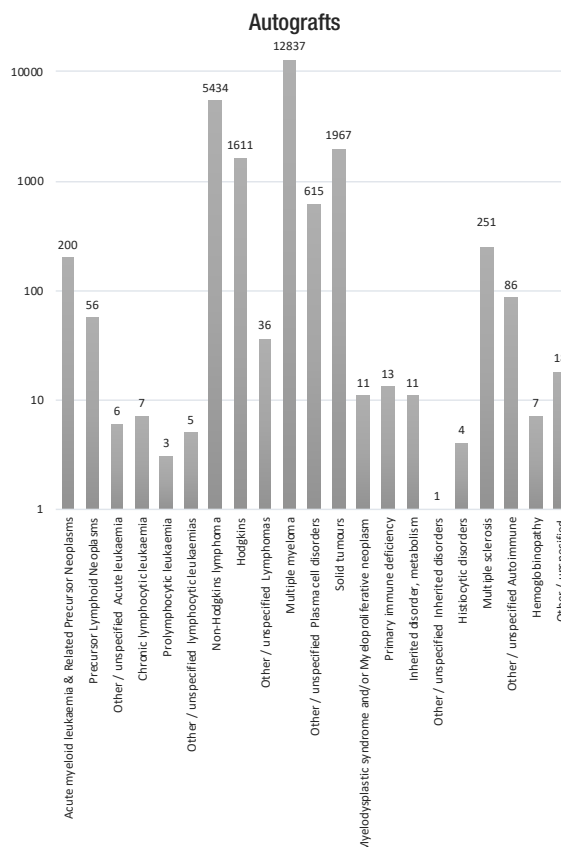
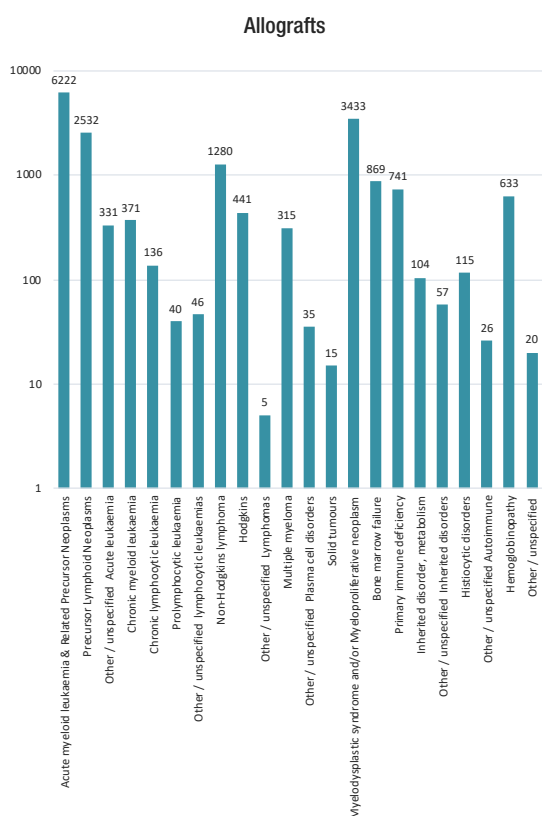
DLI; donor lymphocyte infusions, CAR T; chimeric Antigen receptor T-Cells, in vitro selected and or expanded T-Cells or cytokine activated, such as virus specific T-cells; Cytokine induced Killer Cells (CIK); Regulatory T-Cells (TREGS); Genetically modified T-Cells other than CAR-T; Natural Killer Cells (NK); Dendritic Cells; Mesenchymal Stromal Cells; in vitro expanded CD34+ Cells; and genetically modified CD34+ Cells.

THE EBMT REGISTRY

NEW REGISTRATIONS FOR HSCT IN 2019

By the end of 2019, the entire Registry included at least 709,000 transplant registrations in total. New registrations during 2019 remained at a very similar level to the previous year. We include the charts below.

17767 allografts and 23179 autografts were registered during 2019. Note that these numbers also include some transplants performed during earlier years.



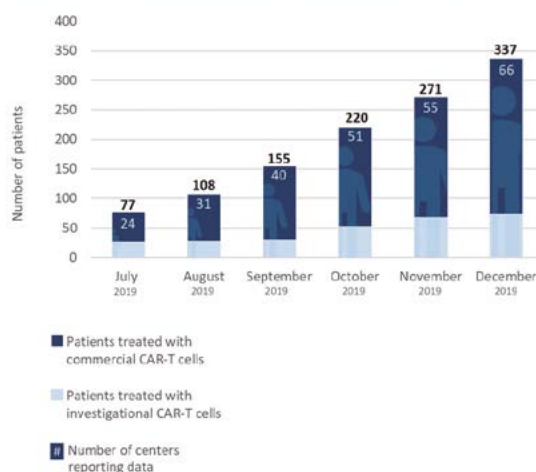
Graphs: Logarithmic scale.

NEW REGISTRATIONS FOR CELL THERAPY IN 2019

Through the network of our EBMT newsletter the Registry has actively encouraged centres to report on all cases involved in the treatment of patients using a CAR-T product whether commercial or investigational. The 1st graph below illustrates the steady growth of new reported cases into the EBMT Registry during 2019 and the reflected number of centres.

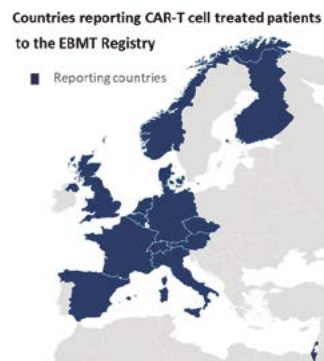
All centres should continue to complete the current Cellular Therapy Form outlined in ProMiSe on a timely basis to create an accurate patient representation.

Number of CAR-T cell treated patients registered in the EBMT Registry



Source: EBMT Registry, December 2019

The 2nd graph represents a geographical approach in blue of all our reporting countries who are continually processing data into the EBMT Registry. We look forward to welcoming more countries in 2020 as the number of CAR-T products on the market steadily increase.



Source: EBMT Registry, December 2019

REGISTRY UPGRADE

Substantial work has continued on this project throughout 2019 where the production environment was made available allowing the data transfer from ProMiSe to MACRO to begin.

Due to ongoing technical challenges that have hampered our progress, the validation process will continue during 2020.

The Registry Upgrade still remains the highest priority within the EBMT and is being closely monitored by our internal stakeholders.

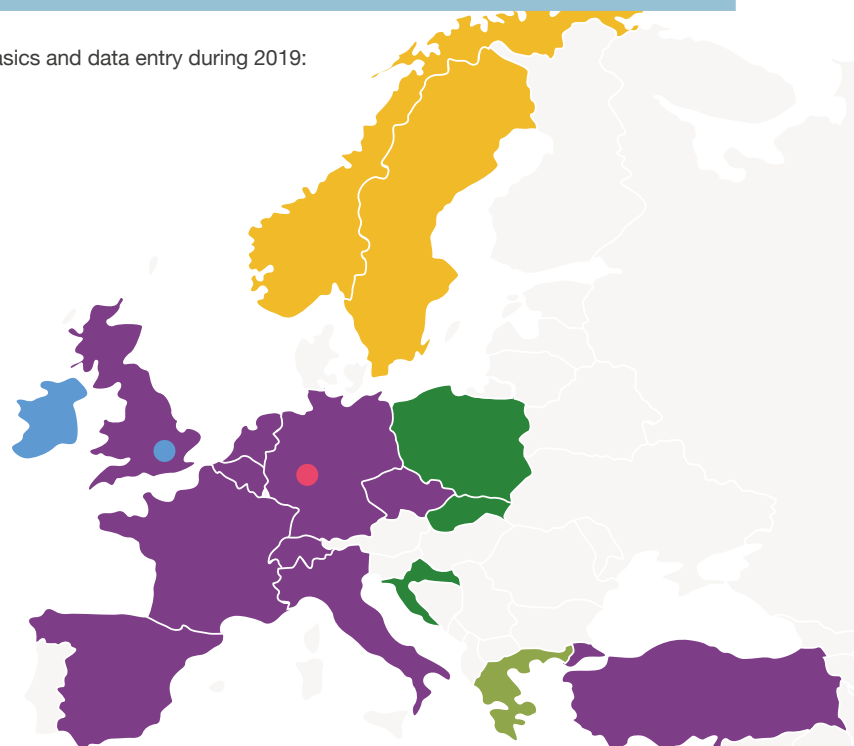
As background to our project, last February the Registry rolled out a data entry training program to EBMT staff and data managers in the National Registries and Centres.

Almost all affiliated national societies have been providing training to centres in their region and we are very grateful for their collaboration. A summary is provided below.

MACRO TRAINING ROLLOUT

Around 600 users have been trained in MACRO basics and data entry during 2019:

- **EBMT registry:**
London, UK
- **EBMT Annual Meeting:**
Frankfurt, Germany
- **National society:**
Turkey
Spain
Belgium
UK
France
Czech Republic
Germany
Italy
Switzerland
The Netherlands
- **Medical University of Warsaw:**
Warsaw, Poland
- **Evangelismos Hospital:**
Athens, Greece
- **Karolinska Institute:**
Stockholm, Sweden



MACRO training is ongoing and will continue until go-live and beyond, in particular online training, face to face sessions at the EBMT Annual Meetings and via the national societies. Data retrieval training is in development.

CELLULAR THERAPY MED-A FORM COMPLETION

The EBMT is in the process of finalising the Cellular Therapy Med-A Form which plans to be a robust stand-alone document reflecting on current standard of treatment across our Hospital community.

This important data collection form (DCF) is to be the basis of all PASS CAR-T Studies and will eventually be implemented into our new MACRO Electronic Data Capture system replacing ProMISe.

Any patient data that has been registered using the current Cellular Therapy Med-A Form in ProMISe will be migrated into MACRO during our ongoing data migration process which is currently taking place at the London Registry Office as of Q4 2019.

As competent authorities require a 15-year follow-up of recipients the staff at all our centres including data managers and clinical departments will be instrumental in collating all the necessary patient data both at the time of treatment and subsequent follow-up visits.

REGISTRY RESOURCE

In 2019 the Registry has transitioned from the previous Head Carmen Ruiz de Elvira to Debra Gordon who joined EBMT Registry in November 2018. EBMT would like to thank Carmen for her exceptional contribution, dedication and commitment that she has shown to the organisation over the past 20 years and wishes her well in her future endeavours.

EBMT welcomes Debra as Head of Registry.

The Registry has also proceeded to hire a new data manager experienced in MACRO alongside the expansion of our IT department which now has greater technical expertise much needed for the challenges of MACRO and delivery of our upcoming Registry IT Projects.

REGISTRY DOCUMENTATION

AND COMMERCIAL AUDIT

The Registry successfully prepared and participated in a commercial audit in Q4 2019.

A variety of SOPs were written including Training, DCF Creation and Documentation Management.

TIMELINES

In review of upcoming project milestones it is planned that MACRO will be partially operational in 2020. Looking forward to collaborating with you in the coming year.

For any comments, suggestions or questions regarding the EBMT Registry please email registryhelpdesk@ebmt.org

Debra Gordon
Head of the Registry



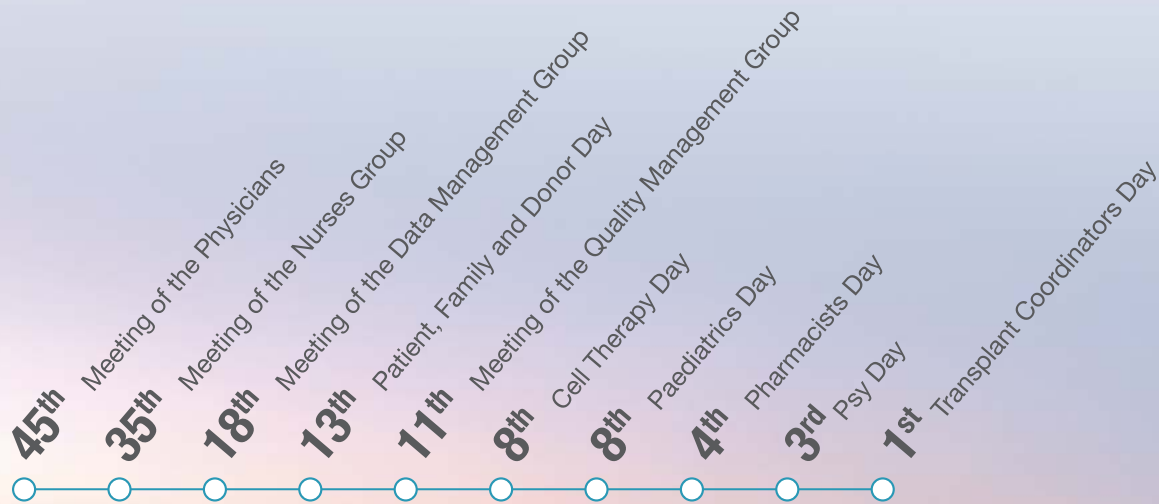
EDUCATION

EBMT 45 th Annual Meeting	45
Awards	48
Educational events 2019	50
New EBMT e-learning platform	51



45th Annual Meeting
of the European Society for
Blood and Marrow Transplantation

24th–27th March 2019 · Frankfurt, Germany



Attendance:

More than
5,640
participants



From
94
countries

Top ten countries:

- Germany
- United Kingdom
- Italy
- France
- Spain
- United States
- Switzerland
- Netherlands
- Belgium
- Russia





150 attendees to
the Patient, Family and Donor Day



159 people watched
the live-streamed sessions

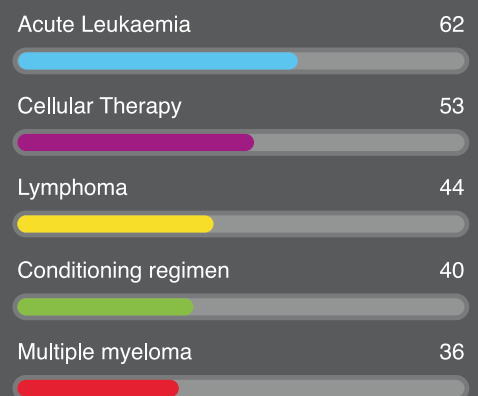
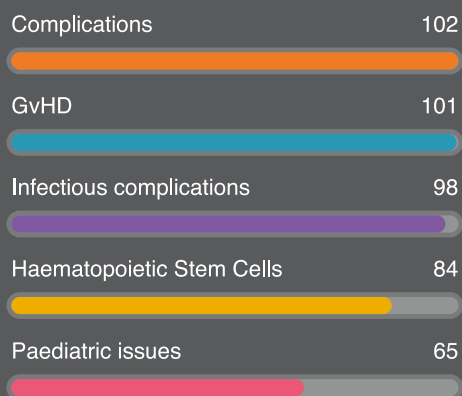
Programme:



Number of submitted abstracts

1,068

Top 10 categories of submitted abstracts



Invited Faculty

456



Sessions

183



Presentations

495



Oral abstract presentations

207



Poster abstract presentations

829



Awards granted

26



New at EBMT 2019:

Guided poster tours, Late-breaking abstracts and the EBMT Hub in the Exhibition Area.

Sponsoring and exhibition:

73

companies, associations
or groups supported
EBMT 2019

37

sponsored sessions
(industry symposium,
theatre, How do I...?)

63

exhibiting organisations in
over 1.500 m² exhibition
area

Digital activities:



4,552 tweets

from 870 participants, generating 9,806,000 impressions



23,848 page views

of the EBMT TV online



3,657 mobile app

downloads

EDUCATION

Networking activities:



750

delegates attended
the opening session
and welcome reception



570

delegates attended
the networking event
at the Gesellschaftshaus
Palmengarten

Delegates gave the Annual Meeting a **rating of 8,7 out of 10**

EBMT 2019 delegates' survey **2,635 respondents**

AWARDS

OPENING SESSION



VAN BEKKUM AWARD to German MDS Study Group and Cooperative German Transplant Study Group for their abstract entitled *AZACYTIDINE (5-AZA) INDUCTION FOLLOWED BY ALLOGENEIC STEM CELL TRANSPLANTATION VERSUS CONTINUOUS 5-AZA IN ELDERLY MDS PATIENTS (55-70 YEARS). A PROSPECTIVE RANDOMIZED STUDY (VIDAZAALLO STUDY)*



BASIC SCIENCE AWARD to Melody Smith (US) for her abstract entitled *INTESTINAL MICROBIOME ANALYSES IDENTIFY BIOMARKERS FOR PATIENT RESPONSE TO CAR T CELL THERAPY*

LYMPHOMA WORKING PARTY SESSION



JIAN-JIAN LUAN AWARD to Chiara De Philippis (Italy) for her abstract entitled: *CHECKPOINT INHIBITOR TREATMENT BEFORE HAPLOIDENTICAL TRANSPLANTATION IN RELAPSED OR REFRACTORY HODGKIN LYMPHOMA (HL) PATIENTS IS ASSOCIATED WITH HIGHER PFS WITHOUT INCREASED TOXICITIES*

PRESIDENTIAL SYMPOSIUM



HONORARY MEMBERS

Per Ljungman (Sweden) and Mauricette Michallet (France)



CLINICAL ACHIEVEMENT AWARD

Wiesław Jędrzejczak (Poland) and James Biggs (Australia)

CELLULAR THERAPY & IMMUNOBIOLOGY

WORKING PARTY SESSION



JON J. VAN ROOD AWARD to Itauá Leston Araujo (Brazil) for his abstract entitled: *HUMAN THYMOPOIESIS IS INFLUENCED BY A COMMON GENETIC VARIANT WITHIN THE TCRA-TCRD LOCUS*

2ND POSTER SESSION - 10 BEST YOUNG ABSTRACT AWARDS

1. **MARIA SPERANZA MASSEI** (Italy)
2. **FRANCESCA LORENTINO** (Italy)
3. **SERENA ALBANESE** (Italy)
4. **VALENTÍN ORTIZ-MALDONADO** (Spain)
5. **NICO GAGELMANN** (Germany)

6. **MARTHE C.J ROEX** (The Netherlands)
7. **SARA PICCINELLI** (Italy)
8. **MENG LV** (China)
9. **WESLEY HUISMAN** (The Netherlands)
10. **SERGEI BLAGOV** (Russian Federation)

5 BEST YOUNG POSTER AWARDS

1. **SAMAR ELBAHY** (UK)
2. **MARIA LAMMOGLIA COBO** (Germany)
3. **NICOLAAS G. VAN DER MAAS** (The Netherlands)
4. **KATRINE KIELSEN** (Denmark)
5. **MANON DELAFOY** (France)



CLOSING CEREMONY



Francesco Manfredi on behalf of Eliana Ruggiero

THE BEST CLINICAL POSTER AWARD, sponsored by Springer, was presented to Eliana Ruggiero (Italy) for her poster entitled: *LONGITUDINAL TRACKING OF WT1-SPECIFIC T CELLS ALLOWS TO GENERATE A LIBRARY OF WT1-SPECIFIC T CELL RECEPTORS (TCR), FOR TCR GENE EDITING OF ACUTE LEUKEMIA*



THE BEST SCIENCE POSTER AWARD, sponsored by Springer, was presented to Johannes Schetelig (Germany) for his poster entitled: *DOES DONOR KIR-GENOTYPE IMPACT OUTCOME AFTER UNRELATED HEMATOPOIETIC STELL CELL TRANSPLANTATION FOR MYELODYSPLASTIC SYNDROMES OR SECONDARY ACUTE MYELOID LEUKEMIA?*



EBMT RESEARCH FELLOWSHIP GRANT in the field of "haematopoeitic stem cell transplantation in acute leukaemia" was presented to Mirjam Belderbos (The Netherlands) for her research on tracing the clonal dynamics of single HSCs in human hematopoietic stem cell recipients

EBMT NURSES GROUP AWARDS



DISTINGUISHED MERIT AWARD to Aleksandra Babic (Italy) for her services to the EBMT Nurses Group.



BEST RESEARCH ABSTRACT ORAL PRESENTATION to Cristina Canaleta Ros (Spain) for her abstract entitled *PERCEPTION OF PATIENTS ABOUT THE INSERTION OF CENTRAL VENOUS CATHETER: PREPARATION, FUNCTIONALITY AND BODY IMAGE - STUDY PRELIMINARY RESULTS*



BEST ORAL PRESENTATION to Sabine Valenta (Switzerland) for her presentation entitled *RE-ENGINEERING FOLLOW-UP CARE AFTER ALLOGENEIC STEM CELL TRANSPLANTATION: PATIENTS' AND CLINICIANS' PERSPECTIVES OF EHEALTH ENHANCED SUPPORT CARE - THE SMILE STUDY*



BEST POSTER to Stéphanie Schmitt (France) for her poster entitled *"GREFFÉ & GOURMET": CREATION OF A RECIPE BOOK FOR TRANSPLANT PATIENTS*

BEST PAEDIATRICS ORAL PRESENTATION to James Evans (UK) for his presentation entitled *GASTROSTOMY FEEDING REDUCES THE NEED FOR PARENTERAL NUTRITION IN CHILDREN UNDERGOING ALLOGENIC BONE MARROW TRANSPLANTATION*



BEST PAEDIATRICS POSTER PRESENTATION to Julie Guest (UK) for her poster presentation entitled *INCREASING THE IMPACT OF MEANINGFUL PATIENT RESEARCH - BUILDING A DEEPER UNDERSTANDING OF THE LIFE WORLD AND BURDEN OF ILLNESS IN A CHILD AND ADOLESCENT CLINICAL APHERESIS COMMUNITY*

EDUCATIONAL EVENTS 2019

EBMT-EHA 1st European CAR T Cell Meeting

14-16 February 2019
Paris, France
680 attendees

EBMT 45th Annual Meeting

Frankfurt, Germany
24-27 March 2019
5,640 attendees

PDWP Meeting: Sickle Cell Disease, what are the curative options?

16-17 May 2019
Regensburg, Germany
174 attendees

1st EBMT GVHD Summit

16-18 May 2019
Warsaw, Poland
234 attendees

JACIE Inspector Training Course

13-14 June 2019
Barcelona, Spain
31 attendees

EBMT 4th International Transplant Course

6-8 September 2019
Barcelona, Spain
293 attendees

LWP 15th Educational Course

19-20 September 2019
Bristol, UK
73 attendees

NG 11th EBMT Nurses International Study Day & 3rd Nurses Research Study Day

3-4 October 2019
Amsterdam, The Netherlands
108 / 82 attendees

IEWP Annual Conference

11-13 October 2019
London, UK
176 attendees

IDWP 22nd Educational Course

17-19 October 2019
Krakow, Poland
121 attendees

CMWP 2nd Scientific & Educational Meeting

18-19 October 2019
Istanbul, Turkey
115 attendees

ADWP Educational Meeting

31 October - 1 November 2019
Berlin, Germany
119 attendees

JACIE Inspector Training Course

14-15 November 2019
Barcelona, Spain
30 attendees

ALWP Scientific Meeting

15 November 2019
Marseille, France

- ALWP: Acute Leukaemia Working Party
- IEWP: Inborn Errors Working Party
- LWP: Lymphoma Working Party
- IDWP: Infectious Diseases Working Party
- CMWP: Chronic Malignancies Working Party
- PDWP: Paediatric Diseases Working Party
- ADWP: Autoimmune Diseases Working Party
- NG: Nurses Group
- JACIE

NEW EBMT E-LEARNING PLATFORM

EBMT is committed to the lifelong education of healthcare professionals in the field of HSCT and Cellular Therapy. Nowadays, online learning is key to the development of knowledge and skills and, in the professional world, learning must be continuously ongoing in order to achieve excellence. For this reason, the e-learning initiative is a critical component of EBMT's strategy, and the Society will officially launch its platform at the EBMT Annual Meeting in Madrid, 30 August - 2 September 2020.

With e-learning, space and time barriers disappear. e-learning programmes offer a flexible learning environment where learners can set their own pace, become responsible for their learning process, and be more autonomous. In online teaching settings, the roles of both teachers and learners change in a unique and beneficial way.

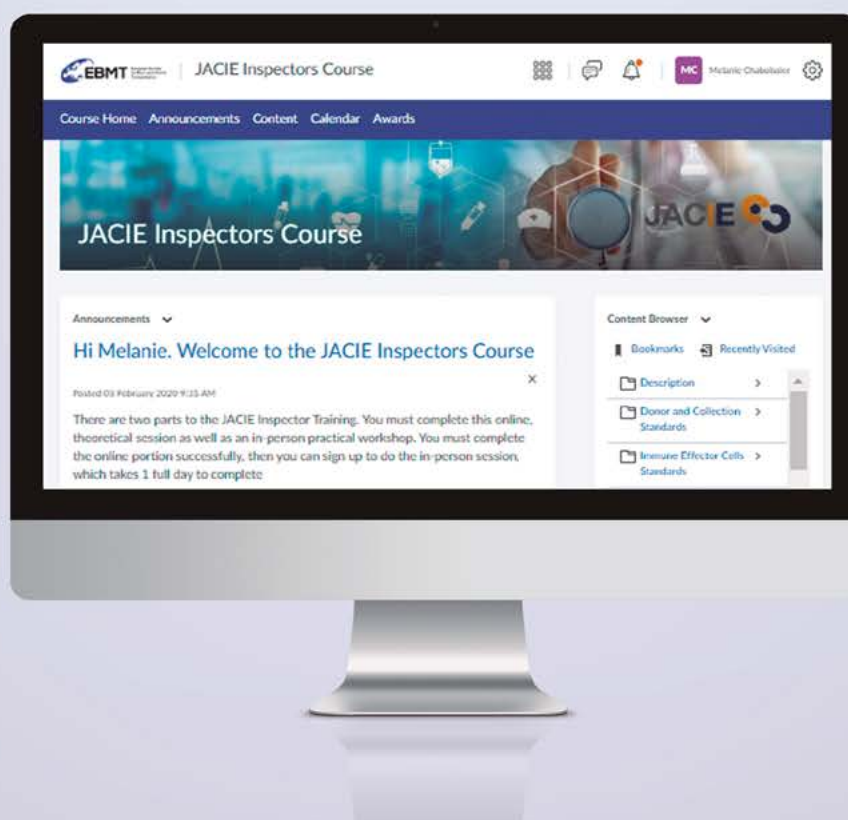
Via the EBMT e-learning platform, users will be able to access e-courses and resources related to EBMT initiatives, and have access to the newest educational material designed by experts in the field of HSCT and Cellular Therapy together with pedagogical and online learning experts, with content that is constantly kept up to date. In addition, online forums available throughout select courses allows users to connect and collaborate with each other.

With the launch of the e-learning initiative at EBMT 2020 in Madrid, EBMT Members can test the platform, as well as access the first e-learning materials:

- Nurses e-course (based on The European Blood and Marrow Transplantation Textbook for Nurses)
- Educational webinars
- CAR-T cell e-course
- JACIE Inspector course

If you are interested in viewing the e-learning platform and registering for any of the above programs, or future programs, please visit **ebmt.org**

For any queries please contact: **elearning@ebmt.org**



PATIENT CARE

EBMT Nurses Group Activity Report 53

Highlights of the JACIE's Activities 55

EBMT NURSES GROUP

ACTIVITY REPORT

THE EBMT NURSES GROUP (NG) IS COMMITTED TO PATIENT CARE THROUGH EDUCATION, RESEARCH AND INTERNATIONAL NETWORK COLLABORATION.

The EBMT NG is one of the leading groups in the field of haematology and HSCT nursing. It is dedicated to improving the care of patients receiving HSCT and promoting excellence in and through evidence-based practice. The NG's mission is to enhance and value the nursing role all over the world, supporting and sharing knowledge through communication, advocacy, research, training and education.

ANNUAL MEETING

The nurses and allied health care professions programme at EBMT 2019 in Frankfurt was a great success from the first session on Sunday 24th through to Wednesday 27th March and was our 35th Nurses Meeting. Over the 4 days of congress there were many stimulating and inspiring sessions, covering the many complications our patient's face, from diagnosis through a variety of treatments and the transplant process and the multitude of effects this procedure leads to.

The Nurses Group was pleased to be able to host two satellite symposiums throughout the conference, supported by Jazz Pharmaceuticals and Mallinckrodt that were extremely well attended and received excellent feedback.

Grants for 2019 were given to five nurses, these grants enable nurses to attend our Annual Meeting, travel to other EBMT centres and share excellent practice and generate ideas and improve working practices. The winners for 2019 have all supplied fantastic feedback either within the EBMT Newsletter or by an oral presentation.

PAEDIATRIC TRAINING DAYS

The Paediatricians and Paediatric Nurses held a training course in Regensburg on sickle cell disease on 16th and 17th May and this was a great success with nurses across Europe attending. Details are within the PDWP's report update.

NURSES INTERNATIONAL STUDY DAY AND

NURSES RESEARCH STUDY

Annually EBMT NG provides an autumn educational meeting. 2019 saw the 11th Nurses International Study Day and the 3rd Nurses Research Study. This year we were hosted in Amsterdam and had a fantastic reception with a great turn out of local nurses. As usual we offer a wide spread of topics, with talks on chronic GvHD, endocrine dysfunction and care of the young adult, a cornucopia on offer that was a stimulating and thought-provoking programme.

The second day saw the now permanent research day in the calendar. The presentations are all aimed specifically at nurses. The research abstract and general abstract finalists talked through their winning pieces of work, giving hints and tips for future writers. A workshop in the afternoon made this a more practical day. Helping nurses turn an idea into a reality by developing their question using research methodology. This led to feedback of >90% satisfaction of a good meeting and people happy to attend again next year, a success.



11th Nurses International Study Day and the 3rd Nurses Research Study

MEDICAL WORKING PARTY NURSES

This role is now firmly established as a valuable resource in each of the medical working parties. The goal to develop collaborative nursing/medical projects for patient benefit continues to develop at pace. The roles have begun to adapt to the needs of the WP with many nurses taking more responsibility for education.

Most notably this year is publications from Helen Jessop (Autoimmune Disease) and Rose Ellard (Cellular therapy & Immunobiology). There are also papers in press from the paediatric committee.

As a consequence of collaboration, the Nurses Group had a session at the GvHD Summit meeting in Warsaw. This saw nurse speakers from across Europe give hints and tips on how to care for patients suffering with the effects of GvHD. We were also lucky enough to have a patient, Chris Lewis come along and tell us of his journey, a truly humbling experience.

JOINT EBMT EHA COLLABORATION

The joint EBMT EHA CAR-T Cell Meeting in Paris in January was a success and also had a nurse's program which was well received and evaluated. The collaboration between the EBMT Nurses Group and nurse representatives from EHA was repeated in Sitges January 2020 at this sold out event.

INTERNATIONAL TRAINING DAY

This is the first time that the Nurses Group has been invited along to the EBMT International Transplant Course. A well-attended meeting held in Barcelona each October. The NG had a whole day and used it wisely to give presentations based on the JACIE competencies. The meeting was very well received and we will be back next year, please sign up early to avoid disappointment.

GLOBAL EDUCATIONAL COMMITTEE

The Global Educational Committee (GEC) in collaboration with the EBMT NG aims to coordinate and organise outreach meetings in cooperation with other non-profit associations with the same mission. Together they coordinate and provide a range of educational activities for nurses and allied health professionals within the field of Haematology and HSCT.

On the 1st and 2nd August the GEC visited Brasilia attending the LABMT annual meeting and giving 2 days of presentations. The GEC also visited Paraguay 2-3rd September and Argentina 30th October to 1st November and participated in the programs of education. Information about all of these can be seen on the GEC posters in Madrid 2020.



23rd Congress of the Brazilian Society of BMT (SBTMO) and 3rd Meeting of the Latin American Blood and Marrow Transplantation Society (LABMT)

NURSING RESEARCH COMMITTEE

We have had one publication this year in *Bone Marrow Transplantation* Polemeni et al Haematopoietic cell transplant nurse coordinators' perceptions of related donor care: a European survey from the EBMT Nurses Group.

There are several projects currently recruiting or about to be launched by the EBMT nurses research group.

THE TEXTBOOK FOR NURSES

The EBMT Nurses Group launched this ground breaking book in March 2018, the first of its kind. There have been approx. 290,000 downloads in the 18 months since its launch. This unique, comprehensive publication informs and guides readers through the myriad of difficulties associated with HSCT. In line with the EBMT strategic plan the text book is now being developed into an e-learning document. At the end of each chapter there will be 10 questions. Once passed a certificate will be produced that can become part of the individuals portfolio of evidence of training and will be an integral part of the JACIE process.

THE EBMT NURSES GROUP ON TWITTER

Follow us now for instant updates on what's going on in the world of nursing: **@TheEBMT_Nurses**

The EBMT NG has a wealth of nurses dedicated to improving knowledge and sharing this with its members. It can be seen from the variety of activities generated in 2019 that this vibrant team is moving the nursing agenda forward and improving patient care.

John Murray

President EBMT Nurses Group



HIGHLIGHTS OF THE JACIE'S ACTIVITIES



The Joint Accreditation Committee ISCT-Europe & EBMT (JACIE) was established in 1998. It promotes high-quality patient care and laboratory performance in the collection, processing and administration of cellular therapy through a profession-led, voluntary accreditation scheme.

JACIE works continuously with international partner organisations to develop and maintain standards for the provision of quality medical and laboratory practice in HSCT, performs on-site inspections, and accredits those programmes that demonstrate compliance with these standards. JACIE also provides training for inspectors and centres on the accreditation process.

Since 2000, **473 transplant programmes and facilities in 34 countries** in Europe and beyond have applied to JACIE and **737 inspections** (first-time and reaccreditation) have been performed. **345** applicants have achieved accreditation at least once with practically all centres repeating the process after completing the first accreditation cycle. There are over **300** registered inspectors, all volunteers drawn from the HSCT and cellular therapy field.

Following the first two joint JACIE-FACT inspections performed in Latin America in 2018 and 2019, a further 8 centres from the region have presented themselves to be inspected during 2020-2021. This 'step-wise' format is being piloted in collaboration with the Latin American Group for Bone Marrow Transplantation (LABMT).

In terms of activity, 2019 was very busy with 101 applications received, by far the highest number ever received. With 71 inspections, 2019 was JACIE's second-busiest year while the 78 awarded accreditations established a new record.

43 applications received in 2019 included Immune Effector Cells in the scope of their accreditation processes.

APPLICATIONS

101 APPLICATIONS RECEIVED

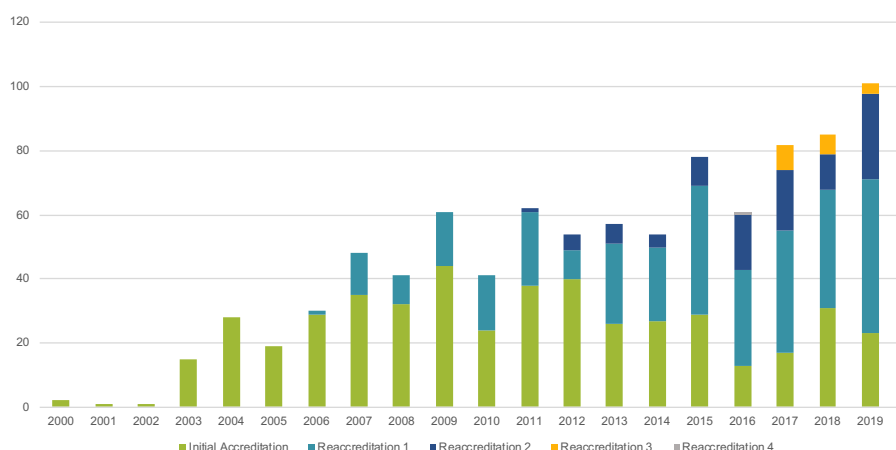


Figure 1. Number of applications received per year

INSPECTIONS

71 INSPECTIONS PERFORMED

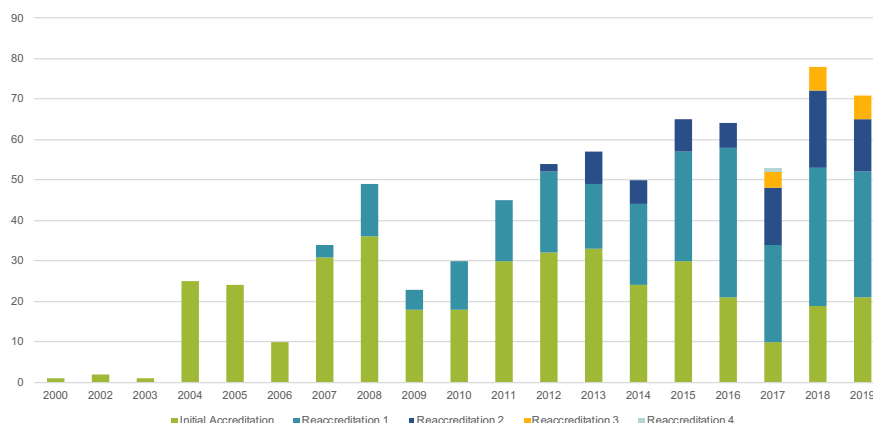


Figure 2. Number of inspections performed per year.

ACCREDITATIONS

78 ACCREDITATION AWARDS

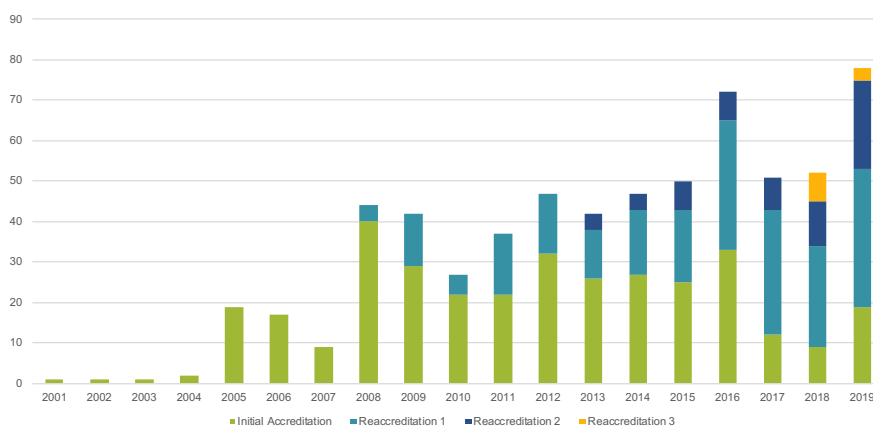


Figure 3. Number of accreditations awarded per year.

8TH EDITION OF FACT-JACIE STANDARDS

The process to prepare the 8th edition of the FACT-JACIE Standards kicked-off in mid-2019. The sub-committees commenced their review during the last months of 2019 and will complete their work in early 2020. The first draft will be opened for public consultation in April 2020 during 3 months.

JACIE COMMITTEE

Chair: John Snowden, EBMT (UK)
 Rafael Duarte, EBMT (ES)
 Nina Worel, EBMT (AT)
 Riccardo Saccardi, EBMT (IT)
 Fermín Sanchez-Guijo, ISCT (ES)
 Franco Bambi, ISCT (IT)

EDUCATIONAL EVENTS

Two JACIE training courses were held during 2019, both in Barcelona, Spain with **38** participants in total from **17** countries while **6** other meetings and events were dedicated to or included accreditation. Furthermore, in September, a joint JACIE-FACT workshop was held at the WBMT/WHO meeting in Asunción, Paraguay.



Figure 4. JACIE training course



Figure 5. FACT-JACIE workshop, Asunción, Paraguay



Figure 6. JACIE training courses

PUBLICATIONS

On behalf of or including direct JACIE participation

Saccardi R, McGrath E, Snowden AJ. JACIE Accreditation of HSCT Programs. The EBMT handbook. 2019:35-40. ISBN 978-3-030-02278-5

Benchmarking of survival outcomes following haematopoietic stem cell transplantation: A review of existing processes and the introduction of an international system from the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Snowden JA et al., *Bone Marrow Transplant*.

Management of adults and children undergoing CAR t-cell therapy: best practice recommendations of the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Yakoub-Agha I et al., *Haematologica*.

OTHER PUBLICATIONS

Indications for haematopoietic stem cell transplantation for haematological diseases, solid tumours and immune disorders: current practice in Europe, 2019. Duarte RF et al., *Bone Marrow Transplant*.

Special report: Summary of the first meeting of African Blood and Marrow Transplantation (AfBMT) group, Casablanca, Morocco, April 19-21, 2018 held under the auspices of the Worldwide Network for Blood and Marrow Transplantation (WBMT).

An international survey on the management of patients receiving CAR T-cell therapy for haematological malignancies on behalf of the Chronic Malignancies Working Party of EBMT. Hayden PJ et al., *Curr Res Transl Med*.

General information for patients and carers considering haematopoietic stem cell transplantation (HSCT) for severe autoimmune diseases (ADs): A position statement from the EBMT Autoimmune Diseases Working Party (ADWP), the EBMT Nurses Group, the EBMT Patient, Family and Donor Committee and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Jessop H et al., *Bone Marrow Transplant*.

Consensus recommendations for the role and competencies of the EBMT clinical pharmacist and clinical pharmacologist involved in hematopoietic stem cell transplantation. Langebrake C et al., *Bone Marrow Transplant*.

Providing both autologous and allogeneic hematopoietic stem cell transplants (HSCT) may have a stronger impact on the outcome of autologous HSCT in adult patients than activity levels or implementation of JACIE at Belgian transplant centres. Poirel HA et al., *Bone Marrow Transplant*.

Microbiological evaluation of environmental cleanliness in haematopoietic cell transplant patient rooms: implementing JACIE standards. Zeneli A et al., *J Hosp Infect*.

Pre-transplantation Risks and Transplant-Techniques in Haematopoietic Stem Cell Transplantation for Acute Leukaemia. Gratwohl A et al., *EClinicalMedicine*.

Worldwide Network for Blood and Marrow Transplantation (WBMT) recommendations for establishing a hematopoietic cell transplantation program (Part I): Minimum requirements and beyond. Pasquini MC et al., *Hematol Oncol Stem Cell Ther*.

For 2020, a group of experts has commenced the drafting of an EBMT Quality Management Guide for HSCT with publication expected in early 2021.

TWITTER

The JACIE Twitter account @JACIE_EBMT has grown to 1310 followers.

We would like to express our continued appreciation and admiration for the Inspectors, JACIE Committee Members, Accreditation, Standards and Quality Manager Committee members, other volunteers and the JACIE Office team for all their tremendous hard work, commitment and dedication.

John Snowden

Chair, JACIE Committee

Eoin McGrath

JACIE Operations Manager

Institutions awarded accreditation in 2019

AZ Sint-Jan Brugge-Ostende AV, Brugge, Belgium; Hôpital Erasme, Brussels, Belgium; Centres Hospitaliers de Jolimont, Haine Saint Paul, Belgium; UZ Leuven, Leuven, Belgium; ULICE (Centre de Thérapie Cellulaire – Université de Liège), Liège, Belgium; Institute of Haematology and Blood Transfusion, Prague, Czech Republic; University Hospital Motol, Prague, Czech Republic; Helsinki University Central Hospital, Helsinki, Finland; CHU Grenoble, Grenoble, France; CHRU de LILLE & Etablissement Français du Sang (EFS) – Nord de France, Lille, France; Institut Paoli-Calmettes, Marseille, France; CHU de Nantes, Nantes, France; Hôpital Necker Enfants-Malades, Paris, France; Centre Henri Becquerel, Rouen, France; Institut de Cancérologie Lucien Neuwirth, Saint-Priest, France; Cellex Collection Center, Cologne, Germany; Universitätsklinikum Köln (AöR), Cologne, Germany; Universitätsklinikum Essen, Essen, Germany; University of Duisburg-Essen, Essen, Germany; Hannover Medical School, Hannover, Germany; Institute of Transfusion Medicine, Kiel, Germany; University Hospital Schleswig-Holstein, Campus Kiel, Kiel, Germany; University Hospital Giessen and Marburg, Campus Marburg, Marburg, Germany; Klinikum Oldenburg AOR, Oldenburg, Germany; DRK-Blutspendedienst West gGmbH, Zentralbereich Stammzelle (ZBST), Ratingen, Germany; Universitätsklinikum Regensburg, Regensburg, Germany; Aghia Sophia Children's Hospital, Athens, Greece; G. Papanicolaou Hospital, Thessaloniki, Greece; Blood Bank Landspítali University Hospital, Reykjavik, Iceland; Children's Health Ireland at Crumlin, Dublin, Ireland; Hadassah University Hospital, Jerusalem, Israel; Hadassah University Medical Center, Jerusalem, Israel; U.O.C. Ematologia, Ospedale C.e G. Mazzoni, Ascoli Piceno, Ascoli Piceno, Italy; Oncoematologia Pediatrica – Ospedale dei Bambini – Centro Trapianti Midollo Osseo Monica e Luca Folonari, Spedali Civili di Brescia, Brescia, Italy; Ospedale Santa Maria Goretti, Latina, Italy; Ospedale San Raffaele S.r.l., Milan, Italy; Azienda Ospedaliera Universitaria Maggiore della Carità, Novara, Italy; AOR VILLA SOFIA CERVELLO, Palermo, Italy; Ospedale Civile di Pescara, Pescara, Italy; Centro Trapianti Midollo Osseo "Alberto Neri", Reggio Calabria, Italy; Arcispedale Santa Maria Nuova IRCCS Reggio Emilia, Reggio Emilia, Italy; Azienda Ospedaliera S. Giovanni Addolorata, Rome, Italy; Fondazione Policlinico Universitario A. Gemelli - Università Cattolica S. Cuore Roma, Rome, Italy; OSPEDALE PEDIATRICO BAMBINO GESÙ, Rome, Italy; AOU Città della Salute e della Scienza di Torino, Turin, Italy; AOU Città della Salute e della Scienza di Torino, Ospedale Infantile Regina Margherita, Turin, Italy; VU Medical Centre, Amsterdam, Netherlands; University Medical Centre Groningen, Groningen, Netherlands; Leiden University Medical Centre, Leiden, Netherlands; Leiden University Medical Centre, Leiden, Netherlands; St Antonius Ziekenhuis, Nieuwegein, Netherlands; Haga Hospital, The Hague, Netherlands; King Faisal Specialist Hospital & Research Centre, Riyadh, Saudi Arabia; Blood Services Group, Health Sciences Authority, Singapore, Singapore; Netcare Pretoria East Hospital, Pretoria, South Africa; Hospital Universitari Vall d'Hebron, Barcelona, Spain; Hospital Infantil Universitario Niño Jesús, Madrid, Spain; Hospital Universitario 12 de Octubre, Madrid, Spain; Hospital Marques de Valdecilla & Banco de Sangre y Tejidos de Cantabria, Santander, Spain; Klinik Hirslanden, Zürich, Switzerland; Klinik Hirslanden Zürich, Zürich, Switzerland; University Children's Hospital Zürich, Zürich, Switzerland; Erciyes University Medical Faculty, Kayseri, Turkey; NHS Blood and Transplant, Birmingham, United Kingdom; University Hospitals Birmingham NHS Foundation Trust, Birmingham, United Kingdom; Addenbrookes Hospital, Cambridge, United Kingdom; NHS Greater Glasgow & Clyde, Glasgow, United Kingdom; Scottish National Blood Transfusion Service, Glasgow, United Kingdom; Alder Hey NHS Foundation Trust, Liverpool, United Kingdom; St Mary's Hospital, London, United Kingdom; The Royal Free London NHS Foundation Trust, London, United Kingdom; The Royal Marsden NHS Foundation Trust, London, United Kingdom; Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle upon Tyne, United Kingdom; Sheffield Children's NHS Foundation Trust, Sheffield, United Kingdom; Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield, United Kingdom; University Hospital of North Midlands, Stoke-on-Trent, United Kingdom; South Warwickshire NHS Foundation Trust, Warwick, United Kingdom;

Full list available at www.ebmt.org/jacie-accredited-centres



Harry Dolstra
Treasurer

Financial Report and Highlights 2019

EBMT has closed 2019 with a positive financial result and continues to invest in its strategic goals in line with its mission. Furthermore, EBMT Finance has obtained an “unqualified opinion” from an independent auditor’s judgement bureau, demonstrating that EBMT is maintaining its high standard of modern management, sustaining its financial stability and provides assurance that money is being spent and allocated to our mission.

During my first year as EBMT Treasurer, the EBMT Board continued to implement the financial model, which allowed the creation of a continuous reserve of income for scientific and educational projects. This permitted further increase in staff positions as well as the strategic plan to modernize and improve the EBMT organizational structure (Figure 1).

SPENDING OUR FINANCIAL RESOURCES ON OUR MISSIONS

Our increasing annual income has been used to further work on our mission and improve the organizational structure of EBMT in order to strengthen our growing organization for achieving our mission goals. The budget needed for management (i.e. board and executive office expenses) decreased to 8,8% which is resulting in allocating 91,2% to our scientific studies, Registry, accreditation and education including the Annual Meeting, e-learning and educational events (Figure 2 Financial highlights 2019). Therefore, EBMT remains a very efficient organization when assessed by international rankings (A/A+ in line with CharityWatch).

IMPLEMENTING THE WELL-BALANCED STRATEGIC

FINANCIAL ROADMAP

In 2019, the main challenge was to implement and optimize EBMT’s organization through a well-balanced financial roadmap for the future in order to be able to cover all financial challenges in line with our ambitions but also further strengthen a strong backbone. The EBMT Board decided to partially release allocated reserves in 2020 (1,067 k€) to support our roadmap and EBMT organization for the next few years which covers the following strategic topics:

1. IMPLEMENTING MACRO REGISTRY

In 2019 we continued to work on the migration of the Registry into MACRO. This project will be finalized in 2020 and has the highest financial priority. Reserves have been earmarked to cover all expected costs. In addition, reserves are being used and held for any unexpected expenses.

2. RESTRUCTURING THE ORGANIGRAM

In 2019 we restructured the managerial infrastructure of the EBMT and organized it in six departments, which are the Science, Registry, Educational, JACIE, Support and Business departments. Each department is led by a department head that together with the Executive Director form the EBMT management team. Furthermore, we recruited a Medical Officer to support all medical aspects within the organization, a HR Coordinator and IT Coordinator to better support our organization and mission. Further implementation of the restructured organigram will continue in 2020 to strengthen the efficiency and mission goals of the EBMT.

3. SETTING UP SUCCESSFUL BUSINESS UNIT

In 2019 we continued with setting up the business unit in order to increase and streamline the process of setting up retrospective and prospective studies in collaboration with pharma, institutions and non-profit organizations. The funded studies are a growing segment of the activities of EBMT, and my compliments go to the business, operational and finance teams in the Leiden office to streamline the contracting of the scientific studies.

4. STARTING THE CART PASS STUDIES

In receiving the qualification opinion from EMA on the cellular therapy module of the EBMT Registry, the Board decided to strengthen manpower to support this initiative, which will collect post-market evidence for safety and efficacy of different commercial CART products. The first contract with Novartis has been signed and more companies are likely to follow. The finance involved in these contracts allow more manpower to execute these PASS studies and increase the Registry data and quality. In addition, this registry tool will also be helpful for all researchers who are executing clinical studies with immune effector cells in order to gather more comprehensive data sets.

5. LAUNCHING THE “BENCHMARKING” PROJECT

In order to finance the “benchmarking project”, which will allow centres to know their own clinical transplantation outcomes compared to the rest of Europe (Snowden et al. BMT 2019), we have increased the JACIE fees. Furthermore, for the first time we have an internal Medical Officer within the EBMT organization to support benchmarking reporting. In 2020 we will give back valuable information to each reporting centre about the quality of their daily clinical care.

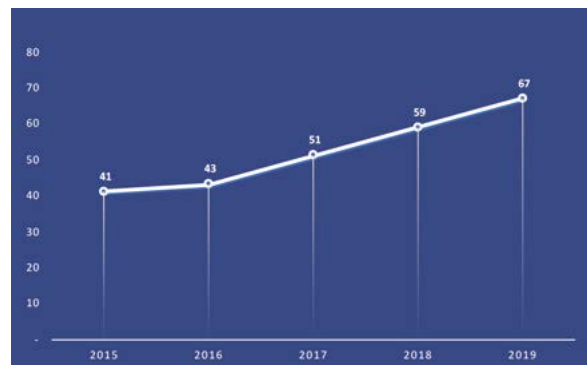
6. CONSOLIDATING THE EVENTS AND EDUCATIONAL UNIT

The in-housing process of the Annual Meeting and educational events by the Barcelona office continues to be a huge success in order to increase the EBMT's positioning and visibility and to improve financial control.

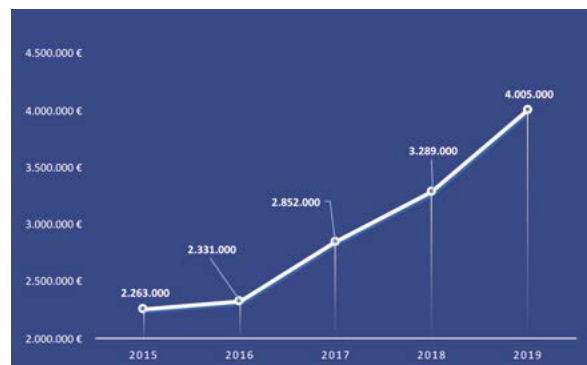
7. IMPLEMENTING E-LEARNING TOOLS

We further invested in developing a sophisticated e-learning platform, which will allow the community to further educate themselves by online training.

(A) NUMBER OF FTE EMPLOYEES



(B) TOTAL SALARY COSTS



(C) GROSS INCOME

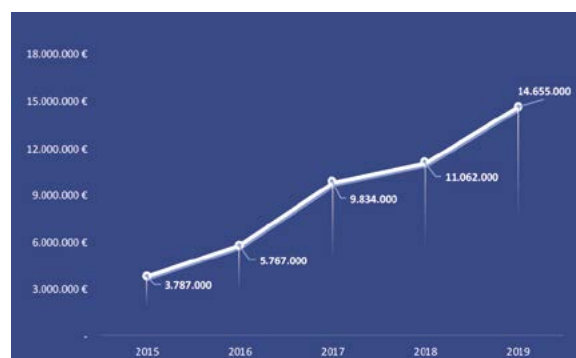


Figure 1. Growth in: (A) FTE Employees (B) Salary Costs (C) Gross Income

FINANCIAL CONCLUSION

The continued positive financial development of the organization has allowed EBMT to further build on its strategic goals and improve its structure. Current highlights include in-housing educational event organization, e-learning, registry upgrade, cellular therapy registry, benchmarking system and CART PASS studies. Despite all these substantial strategic financial investments in 2019 for staff and IT, EBMT will be closing the year 2019 with a positive total result of 4,158 k€ (expenses of 10,497 k€ and a total income of 14,655 k€). A total of 500 k€ will be earmarked in line with the Board's decisions for structural innovation (300 k€) and registry implementation (200 k€). For other running projects 197 k€ will be earmarked as well. The residual budget of 3,461 k€ will be returned to our reserves and used to further secure our key staff positions and main strategic projects in case of any unforeseen serious adverse financial event. Additionally, 1,067 k€ will be used in 2020 to strategically invest in the goals mentioned above.

It has been my pleasure to serve EBMT during my first year as Treasurer. I would like to express my appreciation for the finance team in the Leiden office, Bas Natkiel, Liesbeth Hoekstra and Seppie Öztürk for their hard work and dedication.

Harry Dolstra
EBMT Treasurer

SOURCE OF INCOME

*EBMT Members donations***€591,000**

4,0%

*Scientific Sponsors donations***€703,000**

4,8%

*Annual Meeting***€7,749,000**

52,9%

*Results from investments***€116,000**

0,8%

*Grants for Studies, Clinical Trials
& Education***€4,629,000**

31,6%

*Accreditation (JACIE)***€867,000**

5,9%

*Total Income:***€14,655,000**

100%

DESTINATION OF RESOURCES

*Scientific Studies***€2,375,000**

22,7%

*EBMT Registry***€863,000**

8,2%

*Annual Meeting***€4,020,000**

38,3%

*Educational Activities***€1,672,000**

15,9%

*Standards & Accreditation
(JACIE)***€643,000**

6,1%

*Management and administration***€924,000**

8,8%

*Total Expenditure***€10,497,000**

100%

FINANCIAL OUTCOME

<i>Total Income:</i>	€14,655,000	100%
<i>Total Costs:</i>	€10,497,000	71,6%
<i>Gross Result:</i>	€4,158,000	28,4%
<i>Earmarked:</i>	€697,000	4,8%
<i>Net Result:</i>	€3,461,000	23,6%

Figure 2. Financial highlights 2019

At the time of the report release date (May 5, 2020), the audit process is pending completion.

EBMT'S PARTNERS

The EBMT would like to thank the 27 partners for their generous support in 2019.

DISTINGUISHED PARTNERS



LONG-TERM PARTNERS



PARTNERS OF EXCELLENCE



Partners from 01-01-2019 until 31-12-2019

Thank you to Professor Rafael Duarte and his team from the Hospital Universitario Puerta De Hierro in Madrid, Spain, for providing the necessary permission and organising the photoshoot.



Rafael Duarte



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