
EBMT
European Society
for Blood and Marrow Transplantation
Annual Report
/18

Registered address:
Rijnsburgerweg 10, 2333AA Leiden, The Netherlands
info@ebmt.org

For any enquiries or comments regarding this Annual Report
please contact our communication department at:
communications@ebmt.org

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Table of Contents

Message from the President

Nicolaus Kröger
EBMT President

In my first year as EBMT President, I am delighted and honoured to present the EBMT Activity Report summarising the remarkable achievements of our society in 2018.

On the following pages you can review the scientific and educational activities and productivity of our ten working parties within the last 12 months as evidenced by numerous publications in highly ranked peer-reviewed journals and by cutting-edge educational events. Taken together, these reflect the EBMT's vital role in advancing the field of stem cell transplantation and cellular therapies.

In 2018, the EBMT paved the way for the increasing need for up-to-date education for all involved healthcare providers starting with the launch of the online EBMT Nurses Textbook and the release of the new EBMT Handbook. The process for developing an e-learning platform has already started and will be available in 2019.

The requirements and challenges of the complex regulatory environment in our daily life have mandated continued progress in ensuring that the EBMT adheres to the highest professional and regulatory standards. By upgrading the safety and quality systems in the Clinical Trials Office in Leiden and by implementing a data protection and quality management coordinator in the Executive Office in Barcelona, the EBMT assures that personal data are protected properly in accordance with EU law and that clinical trials are performed according to high-level standards (i.e. Good clinical practice GCP).

Special thanks go to the numerous staff members and volunteers who were, throughout 2018, continuously involved in the new MACRO Registry Upgrade project. The new EBMT Registry, as the backbone of most of our scientific activities, will be launched in 2019. It will provide a robustly benchmarked system and support user-friendly documentation of transplant data and data from the new Cellular and Gene Therapy form.

In 2018, the EBMT decided to make patients voices more audible and founded a Patient, Family and Donor Committee to promote patient advocacy groups for stem cell transplantation and cellular therapies around the globe. Thanks to the very active and dedicated committee members, a new more appealing format of the Patient, Family and Donor Day will be presented at the EBMT's 45th Annual Meeting in Frankfurt, March 24–27 2019.

Probably the most important therapeutic advancement in the treatment of blood cancer in 2018 was the development of CAR-T cells and immune effector cells, which was confirmed by the EMA's approval for commercial CAR-T cell products, i.e. Kymriah and Yescarta, in August 2018. Because of our leading historical and pioneering role in the development of haematopoietic cell transplantation, the EBMT is pivotally positioned to play a leading role in the rapidly developing field of innovative cellular and gene therapies manufactured from haematopoietic tissues. The EBMT is, therefore, ideally placed to accelerate the translation of basic scientific advances into the clinic and integrate academic and commercial aspects into the development of somatic cell therapy and gene therapy products. EBMT staff and members were involved early by attending the EMA CAR-T cell therapy Registries Workshop and applied for a positive opinion for the EBMT Registry to collect CAR-T cell safety and outcome data (see page 44). In our CAR-T cell and immune effector cell strategy, a new Cellular and Gene Therapy form was implemented after harmonisation with CIBMTR; it includes the requirements of the EMA and the needs of the pharmaceutical industry for the upcoming post-authorisation safety studies (PASS). The EBMT has and will further provide educational tools and events focusing on specific issues related to the administration of CAR-T cells and other advanced therapy medicinal products, such as the 1st European CAR-T Cell Meeting in collaboration with the European Hematology Association (EHA), held in Paris in February 2019. Due to our experience with the JACIE accreditation process for transplant activities, which provides high quality in processing and applying cellular products as well as in patient care in haematopoietic stem cell transplantation, the EBMT will work on a specific set of standards that will cover the administration of immune effector cells, which would also be applicable outside the context of classical transplantation centres.

2018 was a year of remarkable progress on many fronts, and I would therefore like to express my sincere gratitude to all those who contributed to the fabulous activities of our society, including everyone who reported data and patient outcomes to the EBMT Registry.

Please continue your commitment to the EBMT in 2019 and enjoy reading the Activity Report for 2018.

About the European Society for Blood and Marrow Transplantation

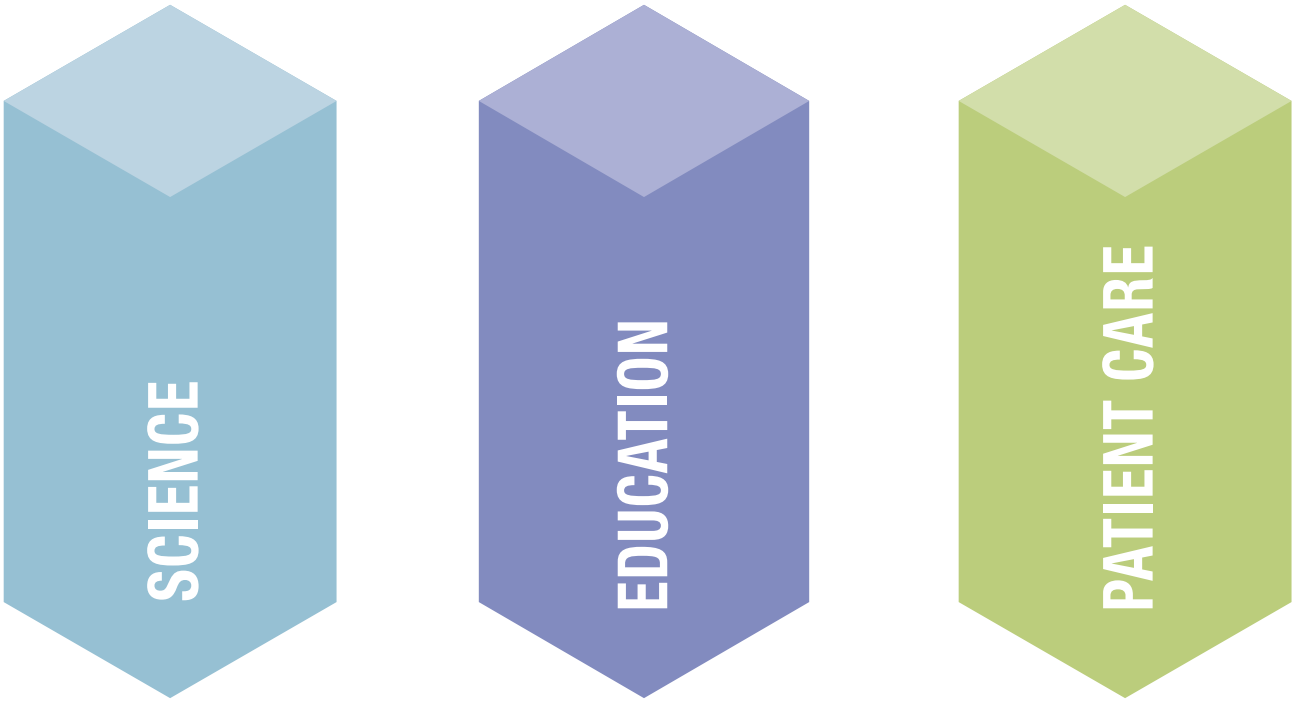
The EBMT is a not-for-profit medical and scientific organisation established in 1974.

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

EBMT members–more than 4,500 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 60 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**
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University Medical Centre
Utrecht, The Netherlands

4. **Scientific Council Chair with Research and Sciences Portofolio**
Mohamad Mohty
Hospital Saint Antoine
Paris, France

5. **Scientific Council Vice-Chair with Research and Sciences Portofolio**
Christian Chabannon
Institut Paoli Calmettes
Marseille, France

6. **Scientific Council Representative with the Registry Portofolio**
John Snowden
Sheffield Teaching Hospitals NHS Trust
Sheffield, UK

7. **Scientific Council Representative with the Education Portofolio**
Grzegorz W. Basak
Medical University of Warsaw
Warsaw, Poland

8. **Nurse Group President**
John Murray
Christie NHS Trust Hospital
Manchester, UK

9. **EBMT 2019 Annual Meeting Co-Presidents**
Peter Bader & Thomas Klingebiel
Klinikum der Johann-Wolfgang Goethe Universitaet
Frankfurt, Germany

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**
Joerg Halter
University Hospital of Basel
Basel, Switzerland

24. **Registry**
Per Ljungman
Karolinska University Hospital
Stockholm, Sweden

25. **Global**
Norbert-Claude Gorin
Hospital Saint Antoine
Paris, France

26. **Legal & Regulatory Affairs (LRAC)**
Christian Chabannon
Institut Paoli Calmettes
Marseille, France

27. **Pharmacist**
Tiene Bauters
Paediatric Clinic Prinses Elisabeth
Gent, Belgium

28. **Patient, Family and Donor**
Bregje Verhoeven
Hematon patient organisation
Utrecht, The Netherlands

Staff organisational chart

Organisational chart / Feb. 2019

Andreu Gusi
Executive Director

DATA OFFICE

Emmanuelle Polge
Data Office Operations Manager

- **Hervé Finel** — LWP Study Coord.
- **Manuela Badoglio** — ADWP Study Coord.
- **Arnaud Dalissier** — PDWP Study Coord.
- **Ivan de Marino** — TCWP Study Coord.
- **TBC** — ALWP & PDWP DM
- **Jessica Lemaitre** — VOD Registry Project Coord.
- **Mohamed Houhou** — ALWP Study Coord.
- **Audrey Mailhol** — ALWP Study Coord.
- **Irma Khvedelidze** — LWP & ALWP Study Coord.

Anja van Biezen
Business Developement & Operations Manager

- **Jennifer Hoek** — Senior DM
- **Cora Knol-Bout** — SAAWP Study Coord.
- **Paul Bosman** — Senior DM & Study Coord.
- **Jorinde Hoogenboom** — IEWP & CTIWP Junior Study Coord.
- **Anja Henseler** — CMWP & TCWP DM
- **Linda Koster** — CMWP Study Coord
- **Nina Knelange** — IDWP Study Coord
- **Anne Lippinkhof-Kozijn** — Junior Study Coord.
- **Sheree Hazelaar** — IEWP Study Coord.
- **Nienke Zinger** — CMWP Junior DM
- **Esther Hazelhoff** — CMWP & IDWP Junior DM
- **Tirlan Sirait** — CMWP Junior DM

STATISTICAL UNIT

- **Myriam Labopin** — Senior Statistician
- **Simona Iacobelli** — Senior Statistician
- **Ariane Boumendil** — Senior Statistician
- **Gloria Tridello** — Statistician
- **Junfeng Wang** — Statistician
- **Giulia Sbianchi** — Pharma Studies Statistician
- **Jacques-Emmanuel Galimard** — Statistician
- **Dirk-Jan Eikema** — Statistician
- **Christophe Peczynski** — Statistician

PROSPECTIVE CLINICAL STUDIES OFFICE

Marianne Mol
Clinical Studies Operations Manager

- **Alain Barrois** — Clinical Studies Coord.
- **Marleen van Os** — Clinical Studies Coord.
- **Annelies Kleijne** — DM Clinical Studies
- **Janette Symons** — Clinical Studies Coord.
- **Dyliana Georgieva** — Clinical Studies Coord.
- **Sofie Terwel** — Clinical Studies Coord.
- **Bianca Groenendijk** — Clinical Studies Coord.
- **Steffie van der Werf** — Clinical Studies Coord.
- **Merel Lammers-van Toorenburg** — Clinical Studies Coord.

REGISTRY OFFICE

Carmen Ruiz
Head of Registry

Debra Gordon
Registry Operational Manager

- **Richard Gumbs** — Information Analyst
- **Babatunde Oyenuga** — Information Analyst
- **Nigel Brook** — Project 2020 Manager
- **Shelley Hewerdine** — Data Management Coord.
- **Khrystyna Valkiv** — Data Quality Coord.
- **Lucas Stolarczyk** — Data Quality Coord.
- **Jude Aben** — Business Support Manager
- **Asterios Kasmiris** — Registry Helpdesk Coord.
- **Aikaterini Manolidou** — Registry Helpdesk Coord.
- **Helen Baldomero** — Transplant Activity Survey Coord.

JACIE

Eoin McGrath
JACIE Operations Manager

- **Raquel Espada** — Accreditation Coord.
- **Eva Controle** — Accreditation Coord.
- **Esther Zafra** — Accreditation Coord.
- **Aurea Villar** — HR Volunteers Coord.

EXECUTIVE OFFICE

Lissa Perteghella
Executive Assistant

- **TBC** — Education & Congress Manager
- **Alanna Mueller** — Scientific Events Coord.
- **Bora Çelikel** — Operations Coord.
- **Marta Herrero** — Membership Coord.
- **Irati Tomas** — Fundraising Coord. for educational events
- **Núria Planagumà** — Education & Events Coord.
- **Anna Bea** — Education & Events Coord.
- **Mélanie Chaboissier** — Communications Coord.
- **Lia Vazquez** — Marketing Coord.
- **Iris Bargalló** — Data Protection & Quality Management Coord.
- **Aurea Villar** — HR Coord.
- **Bas Natkiel** — Finance Controller
- **Liesbeth Hoekstra** — Accounting Coord.
- **Seppie Öztürk** — Finance assistant

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:

Physicians
2,898

Nurses
826

Data Managers
645

Laboratory Technicians
162

Quality Managers
270

Other
160

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

*FULL centres reporting**
518

INDIVIDUAL members
125

ASSOCIATE centres
53

*PROVISIONAL members (including centres and individuals)***
25

* Commit to submitting data on all patients treated in their centres
** New members which are pending approval at the General Assembly Meeting during the upcoming EBMT Annual Meeting.

Total Members:
4,961

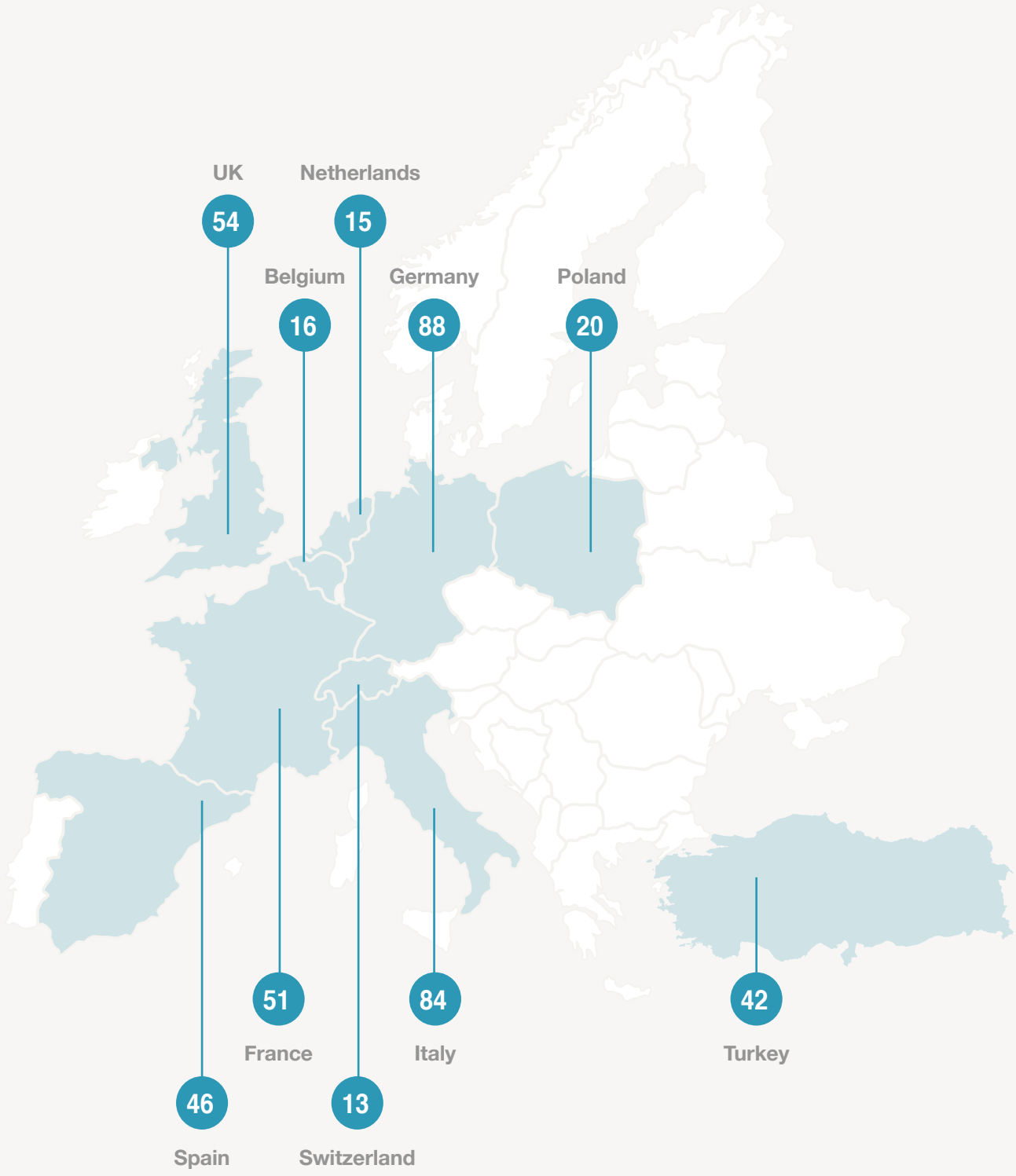
Total Centers:
583

18 centres and 13 individual members joined the EBMT in 2018

Source: ProMISe December 31, 2018

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

Our 583 centre members are located in 63 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

New and refreshed EBMT website

The new EBMT website went live on the 12th of March 2018 and was presented at the EBMT booth during the Annual Meeting in Lisbon.

We wanted to make our new website more user-centred to better serve our scientific community and welcome newcomers interested in the field of Bone Marrow Transplantation and Cellular Therapy. The new site enables users to learn more about the EBMT, share best practices and information, get involved in an active research project or submit a new study.

The responsive web design makes the new website look good on all devices (i.e. desktops, tablets and smartphones). Visitors to the website can see impactful changes such as a new modern design, improved navigation and search functions that significantly enhance their experience.

Also new is the full integration of the JACIE Accreditation site as a section within the main EBMT site. Of particular note is the updated section for patients and donors that contains useful information about the Registry, accredited centres, publications, research projects and patient associations that are relevant for this special target audience.

The EBMT Annual Meeting site was launched in September 2018 and is also now fully integrated within the main site **(https://www.ebmt.org/annual-meeting).**

The preparation of the new website required months of hard work from the many parties involved in the process. The EBMT site has a wealth of information and resources that are beneficial to data and quality managers, physicians, nurses, scientists and other healthcare professionals. This information is now well structured and organised, with consistent site navigation that directs the users, through smart topic filters, to the content that is most relevant to them.

The website is a work in progress. In 2019, there will be integration with the membership part of MACRO (the upcoming new Registry platform) with the objective to streamline the administrative functions and reduce most of the administrative work with regards to the management of memberships. It will also allow for personalisation of the site and the setting of access levels for sensitive information about studies and other internal Working Party topics. New features will be included such as the 'My EBMT' dashboard – where an EBMT member will be able to view the membership list, select his or her fields of interest, pay online, consult the status of payments and so on. So stay tuned!

www.ebmt.org

Legal and regulatory activities

The EBMT was very active in 2018 with regard to regulatory activities. The most significant efforts were dedicated to EBMT's request, presented in October 2017, to the **European Medicines Agency (EMA)** for qualification of the cellular therapy module of the EBMT Registry as suitable for performing pharmacoepidemiological studies for regulatory purposes, concerning CAR-T cell therapy for haematological malignancies. Major progress was made when EMA published their draft opinion in June 2018. The qualification opinion was released on February 28, 2019 (see page 44).

This achievement came on the back of months of engagement with EMA including two meetings in February when EBMT had a face-to-face meeting with the EMA in London followed two days later by the EMA CAR T-cell therapy registries workshop which included a much wider group of stakeholders.

In other areas, on 29-30 January, EBMT was represented at the **DG SANTÉ** (EU Commission, Public Health) meeting on data registries for Substances of Human Origin (SoHO) in Brussels presenting on the Registry and participating in wider discussions including sustainability, quality and data protection matters.

In March, EBMT was invited by the Council of Europe to a two-day event in Strasbourg under the title "Technical Meeting on National and EU-level tissue and cell activity data collection and reporting" under the auspices of the **Directorate for the Quality of Medicines & HealthCare (EDQM)**.

In July, EBMT attended the EU-organised meeting under the title "The way forward for **HTA (Health Technology Assessment) cooperation** – the views of stakeholders". In a time of breakthrough but expensive new therapies, health technology assessment requirements have to be taken into account in terms of getting access for patients to these treatments.

In November, EBMT responded to the public consultation by the EU Commission on their targeted stakeholder consultation on the draft Guidelines on **Good Clinical Practice for Advanced Therapy Medicinal Products**.

EBMT is also in the process of applying to EMA under their initiative for the involvement of healthcare professionals' organisations in the activities of the Agency. This process is expected to close in the first half of 2019.

EBMT is registered in the EU Transparency Registry under identification number 652992023103-09.

EBMT is also a partner in the **Common representation of Substances of Human Origin's (SoHO) (CoRe SoHO)** along with the European Eye Bank Association, the European Blood Alliance and the European Association of Tissues Banks.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

SCIENCE

SCIENCE

The Scientific activity reports

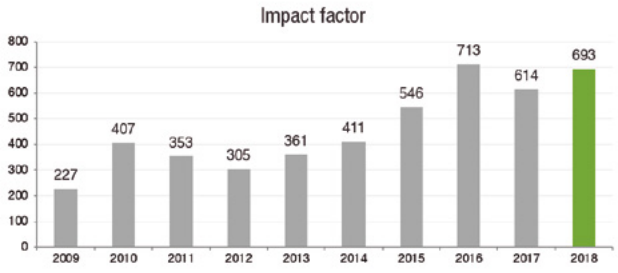
2018 was a most vibrant and exciting year, with many wonderful scientific achievements, and new opportunities. As the chairman of the scientific council of the society, it is a great honour for me to present the impressive scientific productivity of the EBMT, thanks to the contributions of all our members. Announcing and writing about these new studies is always a source of pride, mainly for two reasons. Firstly, because of the enormous satisfaction of offering the transplant community new evidence relevant to clinical practice, and secondly, because it is the most tangible evidence that the society is fulfilling its role and goals. I strongly believe that the positive energy and 'can-do' attitude of EBMT members have taken our society a long way.

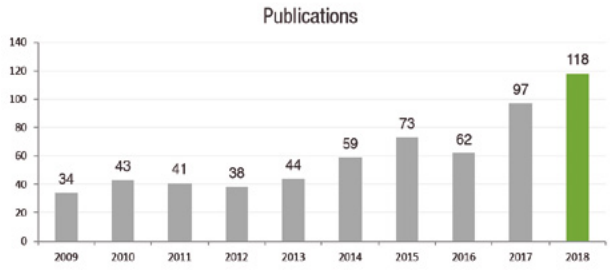
Over the last 12 months, the focus of the different working parties remained unwavering on delivering cutting-edge practical data and transplant guidelines, mainly based on the real-life experience gathered within the amazing EBMT Registry, as well as on the optimal management of transplant patients, especially with the advent of numerous targeted therapies which are being used before and after transplantation. The haplo-identical transplant approach continues to be an ever-evolving strategy that has been experiencing an impressive boom, particularly in acute leukaemia. It is already proving to be a valid transplant modality with outcomes being similar to those achieved with other stem cell sources and donors. At the same time, the EBMT Registry continues to be a timeless gateway to investigating the role of stem cell transplantation in rare disease entities or complications. Building on this background, the new Cellular Therapy module of the EBMT Registry became a reality in 2018 (see page 44). Such a new and modern module dedicated to all the novel innovative therapies represents a great success and a turning point for our strategy.

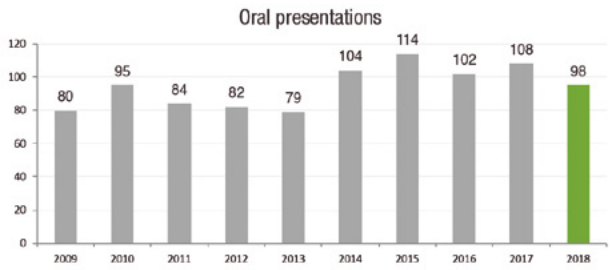
Besides the scientific publications, the EBMT has delivered in 2018 a record number of educational activities and events. The educational activities of the EBMT have been taken to new heights, in terms of both participation and scientific content. The EBMT's comprehensive educational portfolio covers the needs of both the youngest researchers and the most experienced ones and brings together top scientists from all over the globe to exchange ideas, allowing for more effective care and practice-changing discoveries. The launch of the new version of the EBMT Handbook can be considered as one of the highlights of 2018. This handbook is available for free download.

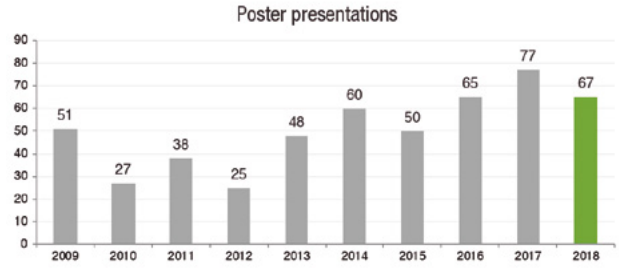
All the above achievements highlight the expanding global influence of the EBMT. The success of the EBMT is due to the effort and hard work of its members. The society would like to encourage newly emerging talent and support their ideas. We want to show our confidence in the upcoming generations and for them to take an active role in the transformations we are witnessing in the field. More than half of the researchers who took the lead in recent EBMT studies were less than 35 years old! Whether it is your first research project or your 50th, there is always something new to explore in the EBMT Registry. The EBMT working parties are the best umbrella to achieve a rewarding and useful clinical research experience in the constantly evolving field of stem cell transplantation and cellular therapy. Please join us and spread the word.

Mohamad Mohty
Chairman of the ALWP of the EBMT
Chairman of the scientific council of the EBMT









Severe Aplastic Anaemia Working Party (SAAWP)

Chair: Régis Peffaut de Latour
Elected in September 2018
(Previous Chair: Carlo Dufour)

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. 182 patients have been randomised so far out of 200 expected, in 28 EBMT sites open for recruitment. An anticipated last patient is expected in February/March 2019.

2. EMAA 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) have recruited patients. German centres have been opened and 31 patients have been enrolled up to early December.

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.

In October 2018, 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).

PRINCIPAL RESEARCH STUDIES

1. Transformed Fanconi Anaemia. PI: S. Giardino.

2. Haplo-identical HSCT with post-Cy in patients with aplastic anaemia. PI: P. Prata.

3. Long term follow-up of patient with SAA (update of the G-CSF trial). PI: A. Tichelli.

4. Use of Eltrombopag in Aplastic Anaemia. PI: M. Ecsedi.

5. Patient-reported symptom monitoring in aplastic anaemia and paroxysmal nocturnal hemoglobinuria. PI: B. Drexler.

KEY PUBLICATIONS

1. Transplant results in adults with Fanconi anaemia. Bierings M et al., *Br J Haematol*.

2. First line treatment of aplastic anaemia with thymoglobuline in Europe and Asia: Outcome of 955 patients treated 2001-2012. Bacigalupo A et al., *Am J Hematol*.

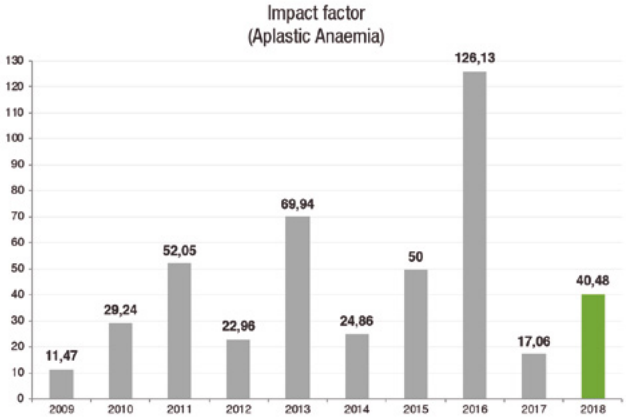
3. Transplant outcome for patients with acquired aplastic anaemia over the age of 40: has the outcome improved? Giammarco S et al., *Blood*.

4. Outcome of haematopoietic stem cell transplantation in dyskeratosis congenita. Fioredda F et al., *Br J Haematol*.

5. Allogeneic Hematopoietic Cell Transplantation in Patients Aged 50Years or Older with Severe Aplastic Anaemia. Rice C et al., *Biol Blood Marrow Transplant*.

MAJOR EDUCATIONAL COURSES

Joint Educational Meeting of the Autoimmune Diseases and Aplastic Anaemia Working Parties – 15-17 November 2018 in Florence, Italy.



	2014	2015	2016	2017	2018
Oral Presentations	9	23	20	13	17
Poster Presentations	1	5	1	9	3
International Educational Events	1	0	1	1	1

Autoimmune Diseases Working Party (ADWP)

Chair: John Snowden

MAJOR ACHIEVEMENTS

Autoimmune diseases (ADs) are now the fastest growing indication for HSCT, and the ADWP has central role in bringing together transplant and disease specialist communities. In 2018, the ADWP continued to expand the evidence-base and support best practice with registry-based studies and guidelines. The AD section of the EBMT Registry is now the largest database of its kind worldwide with almost 3,000 transplants and 2018 has been the most active year to date. Special consideration has been given to current EBMT Registry developments to accommodate the future needs of multispecialty interactions for comprehensive and sustainable long-term data collection in patients with ADs receiving HSCT and other cell therapies. The ADWP also remains actively involved with prospective clinical trials, securing funding (96000 euros) to support long-term data collection and analysis in ASTIClite, a UK NIHR randomised controlled clinical trial of autologous HSCT versus standard of care in Crohn's disease.

Education also continued to be central to global ADWP activities. In November, the ADWP educational meeting, organised jointly in Florence with the SAAWP, attracted the greatest number of delegates ever (over 170), reflecting growing interest in the field. Special workshop sessions in MS, systemic sclerosis and Crohn's disease evaluated the evidence-base, basic science and new clinical trial activity across Europe and beyond. The ADWP has been also engaged in the broader EBMT educational strategy, including the e-learning portal, the EBMT Curriculum and the EBMT Handbook.

'Implementation science' is now increasingly important to define how to best deliver HSCT in the context of biological and other modern therapies for ADs. The future depends on quality of outcomes and health economics. Along with disease specialist societies, the ADWP is working closely with JACIE, other EBMT Working Parties, the EBMT Nurses Group and Patient, Family & Donor Committee to assure best practice and clinical quality.

PRINCIPAL RESEARCH STUDIES

1. Allogeneic HSCT for Autoimmune Diseases (ADWP with PDWP/IEWP collaboration). Analysed and manuscript in preparation.

2. Autologous Stem Cell Transplantation for ANCA-positive vasculitis Analysed and manuscript in preparation. Abstract in oral session at EBMT 2017.

3. Autologous HSCT for progressive systemic sclerosis: a prospective non-interventional study across Europe (NISSC): Manuscript in preparation. Abstract in oral session at EBMT 2018, poster at ASH 2018.

4. NISSC-2: Post AHSCT management and mechanistic immunological reconstitution for patients with systemic sclerosis.

5. Comparison of Cyclo+ATG vs BEAM+ATG conditioning regimens in autologous HSCT for Multiple Sclerosis.

6. Late complications after autologous HSCT for autoimmune diseases: a retrospective survey from the ADWP and TCWP.

Figure 1: Attendees Joint Educational Meeting of the Autoimmune Diseases and Aplastic Anaemia Working Parties – 15-17 November 2018 in Florence, Italy

7. Retrospective studies of autologous HSCT for:

- **a.** Polymyositis-Dermatomyositis
- **b.** Behçet's Disease
- **c.** Rare neurological autoimmune diseases
- **d.** Immune cytopenias

8. Viral reactivations following HSCT for autoimmune disease: a retrospective EBMT survey.

9. Guidelines and recommendations for:

- **a.** Neurological diseases
- **b.** Paediatric autoimmune diseases (ADWP with IEWP/PWP collaboration)

10. 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.

KEY PUBLICATIONS

1. Hematopoietic stem cell therapy for autoimmune diseases - Clinical experience and mechanisms. Alexander T et al., *Journal of Autoimmunity*.

2. Autologous Haematopoietic Stem Cell Transplantation for Crohn's Disease: A Retrospective Survey of Long-term Outcomes from the European Society for Blood and Marrow Transplantation. Brierley CK et al., *J Crohns Colitis*.

3. Autologous Haematopoietic Stem Cell Transplantation (AHSCT) in Severe Crohn's Disease: A Review on Behalf of ECCO and EBMT. Snowden JA et al.; *J Crohns Colitis*.

4. Immune Reconstitution After Autologous Hematopoietic Stem Cell Transplantation in Crohn's Disease: Current Status and Future Directions. A Review on Behalf of the EBMT Autoimmune Diseases Working Party and the Autologous Stem Cell Transplantation In Refractory CD-Low Intensity Therapy Evaluation Study Investigators. Pockley AG et al., *Front Immunol*.

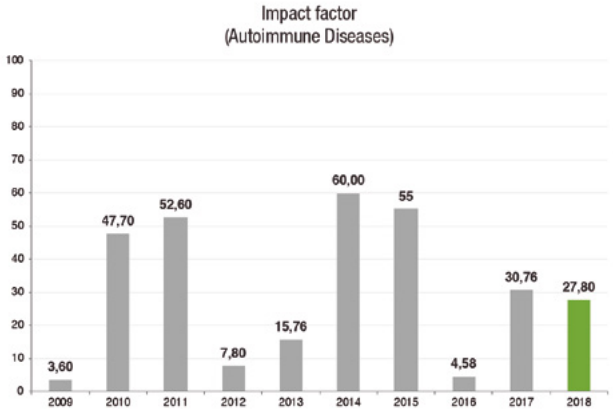
5. Autologous haematopoietic stem cell transplantation (aHSCT) for severe resistant autoimmune and inflammatory diseases - a guide for the generalist. Snowden JA et al., *Clinical Medicine*.

MAJOR EDUCATIONAL COURSES

1. Joint Educational Meeting of the Autoimmune Diseases and Aplastic Anaemia Working Parties – 15-17 November 2018 in Florence, Italy.

2. Educational and Working Party Sessions, EBMT Annual Meeting – March 2018 in Lisbon, Portugal.

3. Other international educational events: European Crohn´s and Colitis Organisation (ECCO) Congress; BSBMT Annual Scientific Meeting; EBMT Highlights (Saudi Arabia and India); EBMT International Transplant Course; Myelin Meeting.



	2014	2015	2016	2017	2018
Oral Presentations	5	6	11	10	11
Poster Presentations	7	1	0	3	5
International Educational Events	1	1	1	1	1

Acute Leukaemia Working Party (ALWP)

Chair: Mohamad Mohty
Elected in March 2018
(Previous Chair: Arnon Nagler)

MAJOR ACHIEVEMENTS

The number of HSCT procedures for patients with acute leukaemia is continuously growing as reflected by the growing content of our Registry. According to recently published studies by the ALWP, it is accompanied by improving results, due to both lower risk of relapse and decreased non-relapse mortality. This spectacular progress is possible thanks to many factors, including rapidly developing pharmacological therapies, which allow higher proportion of patients entering HSCT in high quality remission. Some drugs may be used for either prophylactic or pre-emptive treatment after HSCT, increasing the chance to maintain remission. On the other hand, almost all patients being in need of allogeneic HSCT (including those with refractory disease) may be offered such treatment thanks to the growing number of haplo-identical donors. Finally, the HSCT procedure has become safer due to the development of new reduced-toxicity conditioning regimens and post-transplant supportive care protocols.

In 2018, the ALWP performed several studies tackling some of these hot topics. The impact of ATG, a backbone for GVHD prophylaxis, has been investigated in different settings, with some positive results, of importance in daily practice. The use of bone marrow versus mobilised peripheral blood stem cells in haplo-identical transplants incorporating post-transplant cyclophosphamide remains a matter of debate. Post-transplant cyclophosphamide continues actually to generate a lot of enthusiasm, including in the non-haplo settings where it may have nullified the detrimental effect of HLA-mismatch. Refinement of the different conditioning regimens has been another focus of several studies, especially in the context of high risk and refractory disease. Furthermore, measurable residual disease is gaining a lot of interest as it can strongly predict outcome in different transplant approaches. Finally, post-remission strategies for the prevention of relapse following allogeneic transplantation are increasingly used with convincing efficacy.

The list of the ALWP achievements is huge, because this is a very exciting area of investigation, as reflected by the number of scientific publications and meeting abstracts. Also, the ALWP is very proud of the educational events organised annually in various European countries. Our activities are possible thanks to all EBMT members who report their data voluntarily.

PRINCIPAL RESEARCH STUDIES

1. Analysis of donor characteristics, timing, doses and schedule of post-transplant cyclophosphamide and immunosuppressive therapy post T-replete haploidentical transplantation.

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. Design of integrated pre-transplant scores for refining outcomes (e.g. novel conditioning score).

KEY PUBLICATIONS

1. Anti-thymocyte globulin improves survival free from relapse and graft-versus-host disease after allogeneic peripheral blood stem cell transplantation in patients with Philadelphia-negative acute lymphoblastic leukemia: An analysis by the Acute Leukemia Working Party of the EBMT. Czerw T et al., *Cancer*.

2. Cord blood transplantation is associated with good outcomes in secondary Acute Myeloid Leukemia in first remission. Baron F et al., *J Intern Med*.

3. Haploidentical versus unrelated allogeneic stem cell transplantation for relapsed/refractory acute myeloid leukemia: A report of 1578 patients from the Acute Leukemia Working Party of EBMT. Brissot et al., *Haematologica*.

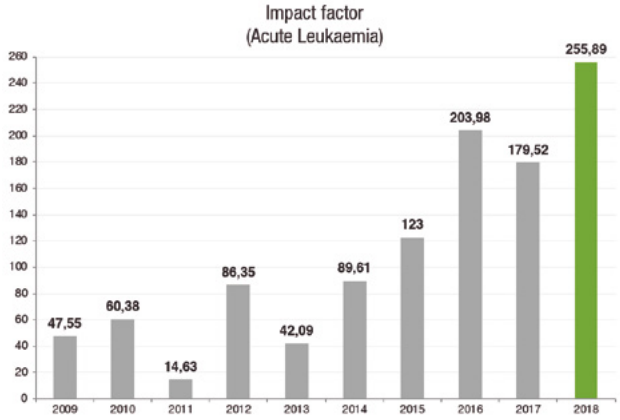
4. Association of Second Allogeneic Hematopoietic Cell Transplant vs Donor Lymphocyte Infusion With Overall Survival in Patients With Acute Myeloid Leukemia Relapse. Kharfan-Dabaja MA et al., *JAMA Oncol*.

5. Killer cell immunoglobulin-like receptor ligand mismatching and outcome after haploidentical transplantation with post-transplant cyclophosphamide. Shimoni A et al., *Leukemia*.

MAJOR EDUCATIONAL COURSES

1. Advances in Allogeneic Immunotherapy: where do we stand in 2018? – 20-22 April 2018 in Marseille, France.

2. Acute Leukaemia Working Party Scientific Meeting and Educational Symposium. "Advances in pre and post-transplant management of acute lymphoblastic leukaemia" – 16-17 November 2018 in Krakow, Poland.



	2014	2015	2016	2017	2018
Oral Presentations	18	22	30	39	28
Poster Presentations	13	15	19	23	18
International Educational Events	1	3	2	2	3

Figure 1: Lifetime Achievements Award in Acute Leukaemia 2018

Cellular Therapy and Immunobiology Working Party (CTIWP)

Chair: Christian Chabannon
Elected in March 2018
(Previous Chair: Chiara Bonini)

MAJOR ACHIEVEMENTS

The CTIWP develops activities focussed on clinical and biological monitoring of existing cellular therapies (mostly allogeneic hematopoietic cell transplantation), regardless of transplant indications. The CTIWP also explores ways to accelerate access to innovative somatic cell therapy medicinal products or gene therapies manufactured from hematopoietic cells: these include drug products engineered from hematopoietic stem cells, immune effector cells and mesenchymal stem cells, whether developed as investigational drugs or in the context of the hospital exemption by academic facilities, or developed and marketed by the industry.

2018 saw a burning actuality with the first approval of two autologous CAR-T Cells in Europe. CTIWP members invest a considerable amount of time to fully define conditions that are necessary to efficiently master interactions between hospitals and industry for the supply chain, to provide educational tools and organisational support to all categories of healthcare practitioners that are involved in delivery and administration of these innovative therapies, some of which produce impressive disease responses and clinical side-effects, and to organise long-term follow-up of treated patients as mandated by FDA and EMA. As part of these efforts, CTIWP members played an important role in designing an additional form that complements existing forms in the EBMT Registry: this Cellular and Gene Therapy form allows EBMT centers to register patients treated with all kind of advanced cell therapy medicinal products, whether adding, substituting or bridging to conventional cell transplant. In addition, CTIWP members work together to gain a comprehensive knowledge of ongoing activities in Europe in this field, and network complementary expertise and knowledge.

PRINCIPAL RESEARCH STUDIES

1. A survey of ongoing studies evaluating mesenchymal stem cells in Europe. Part I: manufacturing capacities. Part II: clinical studies (F Dazzi et al, Immune tolerance Subcommittee).

2. A survey of CAR-T cell activities in Europe (A Urbano-Ispizua et al, Immune Effector Cells Subcommittee).

3. A survey of immune monitoring practices in Europe (L Vago & C Bonini et al, Immune Monitoring Subcommittee).

4. Results of cord blood transplantations (A Ruggeri et al, Hematopoietic Stem Cells & Eurocord Subcommittee).

KEY PUBLICATIONS

1. Immune monitoring in allogeneic hematopoietic stem cell transplant recipients: a survey from the EBMT-CTIWP. Greco R et al., *Bone Marrow Transplant*.

2. Hematopoietic stem cell transplantation in its 60s: A platform for cellular therapies. Chabannon C et al., *Sci Transl Med*.

Figure 1: Jon Van Rood Award 2018

3. Manufacturing Mesenchymal Stromal Cells for the Treatment of Graft-versus-Host Disease: A Survey among Centers Affiliated with the European Society for Blood and Marrow Transplantation. Trento C et al., *Biol Blood Marrow Transplant*.

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*.

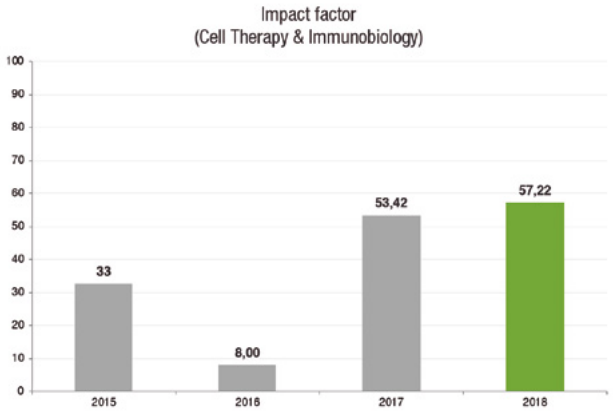
5. Handling, processing and disposal of stem cell products in Europe: A survey by the cellular therapy and immunobiology working party of the European Society for Blood and Marrow Transplantation. Holbro A et al., *Cytotherapy*.

MAJOR EDUCATIONAL COURSES

1. 2nd Cellular Therapy & Immunobiology Scientific Symposium – 18-20 January 2018 in Leiden, The Netherlands.

2. The CTIWP Scientific Session at the EBMT Annual Meeting including the delivery of the Jon van Rood Award to E Velardi and Dr A Galleu – 20 March 2018 in Lisbon, Portugal.

3. The 7th edition of the Cell Therapy Day at the EBMT Annual Meeting – 19 March 2018 in Lisbon, Portugal.



	2014	2015	2016	2017	2018
Oral Presentations	0	3	1	7	3
Poster Presentations	0	2	8	3	2
International Educational Events	1	1	1	3	6

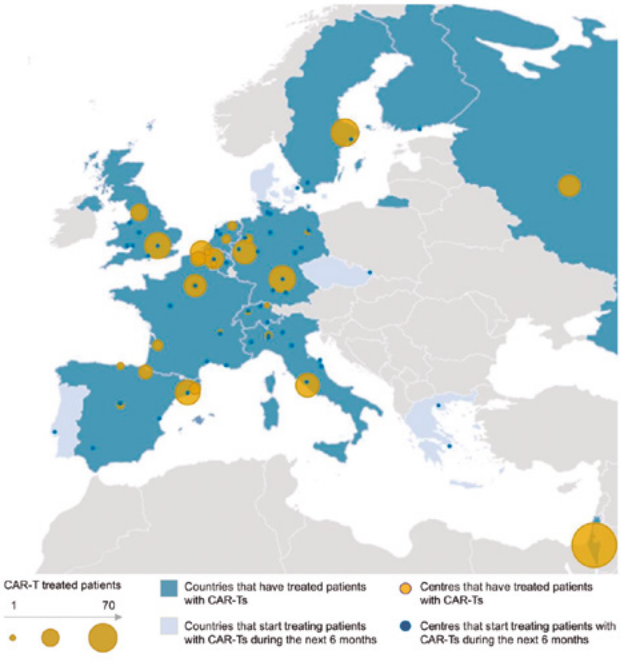


Figure 2: Map representing the CAR-T cell activitiy (December 2018). EBMT CTIWP.

Infectious Diseases Working Party (IDWP)

Chair: Jan Styczynski

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, O. Penack, S. Cesaro, M. Schmidt-Hieber, L. Gil, H. Einsele, R. Martino, J. Styczynski), *fungal infections* (M. Mikulska, O. Penack, S. Cesaro, J. Styczynski), *parasitic infections* (L. Gil, R. Martino), *pneumocystis jiroveci infections* (C. Robin, C. Cordonnier), *vaccinations* (C. Cordonnier, P. Ljungman, D. Engelhard, S. Cesaro), *protective environment* (I. Agreiter, P. Donnelly, J. Styczynski, S. Cesaro) and *ECIL guidelines* (chaired by C. Cordonnier).

IDWP recent achievements were published in *Clinical Infectious Diseases, Journal of Antimicrobial Chemotherapy, Bone Marrow Transplantation* and *Reviews in Medical Virology.*

PRINCIPAL RESEARCH STUDIES

ONGOING RETROSPECTIVE PROJECTS:

1. Causes of deaths after HSCT.

2. Infections with legionellosis, toxoplasmosis, tuberculosis, nocardiosis, JCV, HEV after HSCT.

3. Role of CMV, EBV, ADV, JCV, HHV6, HHV8 and HIV on outcomes of HSCT.

ONGOING PROSPECTIVE NON-INTERVENTIONAL PROJECTS:

4. HHV6 infections after HSCT.

5. The incidence of gram-negative bacteremia, risk factors and resistance to antibiotics.

6. Impact of pre-existing invasive aspergillosis on allo-HSCT outcome.

7. Risk factors and outcome of pneumocystis pneumonia (PcP) infection in HSCT.

8. Treatment approach for patients with HCV infection and who underwent HSCT.

9. Anti-infective prophylaxis and antibiotic use in patients undergoing HSCT.

10. Infections of CNS after HSCT.

11. Impact of candidemia on transplant outcome.

Figure 1. Participants of 21st IDWP Educational Course in Stockholm 25-27 October 2018.

KEY PUBLICATIONS

1. Incidence, Risk Factors, and Long-term Outcome of Acute Leukemia Patients With Early Candidemia After Allogeneic Stem Cell Transplantation: A Study by the Acute Leukemia and Infectious Diseases Working Parties of European Society for Blood and Marrow Transplantation. Cesaro S et al., *Clin Infect Dis*.

2. Protective environment for hematopoietic cell transplant (HSCT) recipients: The Infectious Diseases Working Party EBMT analysis of global recommendations on health-care facilities. Styczynski J et al., *Bone Marrow Transplant*.

3. Management of adenovirus infection in patients after haematopoietic stem cell transplantation: State-of-the-art and real-life current approach: A position statement on behalf of the Infectious Diseases Working Party of the European Society of Blood and Marrow Transplantation. Hiwarkar P et al., *Rev Med Virol*.

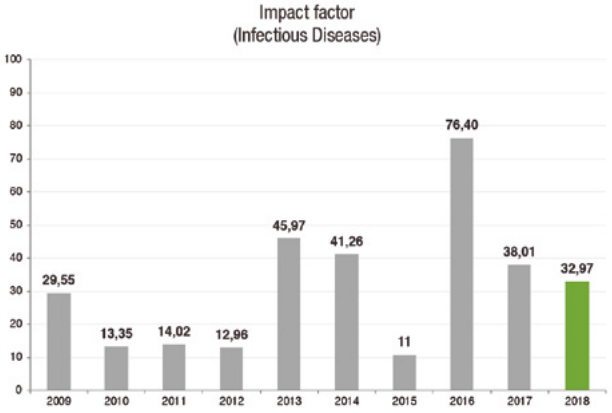
4. ECIL guidelines for the prevention, diagnosis and treatment of BK polyomavirus-associated haemorrhagic cystitis in haematopoietic stem cell transplant recipients. Cesaro S et al., *J Antimicrob Chemother*.

5. Fluoroquinolone prophylaxis in haematological cancer patients with neutropenia: ECIL critical appraisal of previous guidelines. Mikulska M et al., *J Infect*.

MAJOR EDUCATIONAL COURSES

1. 11th Paediatric Diseases, Infectious Diseases, Inborn Errors Working Parties and 6th Paediatric Nurses Group Meeting – 7-9 June 2018 in Verona, Italy.

2. 21st Educational Course of the Infectious Diseases Working Party – 25-27 October 2018 in Stockholm, Sweden.



	2014	2015	2016	2017	2018
Oral Presentations	1	3	6	1	3
Poster Presentations	1	0	0	4	3
International Educational Events	1	2	1	2	2

Inborn Errors Working Party (IEWP)

Chair: Arjan Lankester

MAJOR ACHIEVEMENTS

The IEWP can look back at a very interesting and productive year with many events in which IEWP has presented its activities. IEWP actively participated in the EBMT International Transplant Course in Barcelona with presentations on HSCT in primary immune disorders (PID) (A. Lankester) and hemoglobinopathies (J. de la Fuente). The 3rd thymus workshop (14 September in London) was organised in collaboration with the ESID clinical working party, and attended by a group of international experts in preclinical and clinical research.

The 4th Inborn Errors Working Party Annual Conference was organised in Leiden with special attention to the 50th anniversary of the first successful SCT in a SCID patient in Europe. On the first educational day, a series of international experts presented excellent overviews on developments in the field of SCT and gene therapy in SCID and non-SCID PID, SCID newborn screening, and long term outcome after SCT. The second and third day included sessions on various aspects of stem cell therapies and SCT indications in PID, hemoglobinopathies and inherited metabolic diseases, and reports on ongoing IEWP studies were presented.

During the ESID annual meeting, a very well attended IEWP session was fully dedicated to current management of chronic granulomatous disease patients including conventional treatment (S. Holland), SCT (T. Güngör) and gene therapy (C. Booth) followed by a panel discussion on CGD cases.

In 2018, six excellent manuscripts on IEWP studies were published. For 2019, IEWP has the ambition to further extend its study activities in collaboration with ESID, SCETIDE, PIDTC and other partners.

PRINCIPAL RESEARCH STUDIES

1. Allogeneic HSCT in children and adults with chronic granulomatous disease.

2. Outcome and immune reconstitution in SCID HSCT 2006-2015: 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 LAD HSCT 2008 – 2015: IEWP-PDWP study.

6. Domino HSCT for PID: IEWP-PIDTC study.

7. Cord Blood SCT for HLH (with Eurocord).

8. HSCT for Erythropoietic Porphyria: EBMT-CIBMTR study.

9. HSCT in Inherited Metabolic Diseases with focus on immune cytopenia: IEWP-Eurocord study.

10. HSCT in LAL-Wolman's disease.

11. HSCT in patients with IFNg receptor deficiencies.

KEY PUBLICATIONS

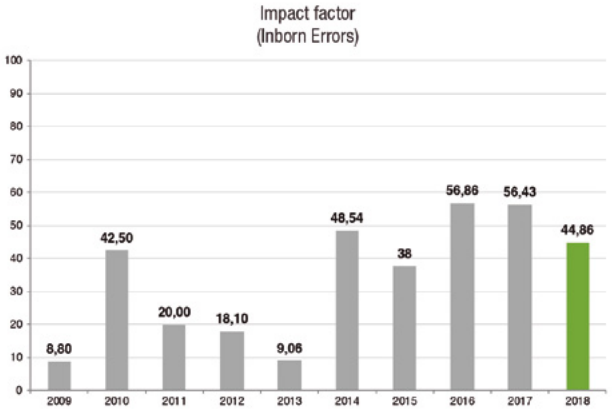
1. Current understanding and future research priorities in malignancy associated with inborn errors of immunity and DNA repair disorders: perspective of an interdisciplinary working group. Bomken S et al., *Frontiers in Immunology*.

2. Risk factors affecting outcome of unrelated cord blood transplantation for children with familial haemophagocytic lymphohistiocytosis. Furtado-Silva JM et al., *Br J Haematol*.

3. Outcome of domino hematopoietic stem cell transplantation in human subjects: An international case series. Belderbos ME et al., *J Allergy Clin Immunol*.

4. Early and late outcomes after cord blood transplantation for pediatric patients with inherited leukodystrophies. van den Broek BTA et al., *Blood Adv*.

5. Hematopoietic stem cell transplantation as treatment for patients with DOCK8 deficiency. Aydin SE et al., *J Allergy Clin Immunol Pract*.



	2014	2015	2016	2017	2018
Oral Presentations	2	5	2	8	8
Poster Presentations	2	2	3	2	1
International Educational Events	3	3	4	4	4

MAJOR EDUCATIONAL COURSES

1. 11th Paediatric Diseases, Infectious Diseases, Inborn Errors Working Parties and 6th Paediatric Nurses Group Meeting – 7-9 June 2018 in Verona, Italy.

2. Inborn Errors Working Party annual educational day '50 years of successful transplantation in PID' – 28 September 2018 in Leiden, The Netherlands.

3. 4th Inborn Errors Working Party Annual Conference – 28-30 September 2018 in Leiden, The Netherlands.

4. IEWP session "treatment of CGD in 2018 and beyond" during the annual meeting of the European Society for Immunodeficiencies (ESID) – 24-27 October 2018 in Lisbon, Portugal.

Lymphoma Working Party (LWP)

Chair: Silvia Montoto

MAJOR ACHIEVEMENTS

It is a pleasure to report that we have had another successful and very active year in the LWP. This group comprises physicians from across Europe along with a nursing and a newly incorporated patient representative. We have an active trials portfolio currently comprising 31 studies. The trial portfolio is managed through fortnightly teleconferences. Over the last year we have published 10 papers and presented 17 papers (8 oral presentations and 9 poster presentations) at international scientific meetings. Additionally we have conducted a number of successful collaborative projects with the CIBMTR Lymphoma Working Committee.

We are also actively involved in educational activities, the highlight of which is the annual LWP educational course this year hosted in Palma de Mallorca, Spain attracting over 90 participants. The 2019 course will be held in Bristol UK on the 18th-19th September 2019. We also contribute to the EBMT Annual Meeting educational program with lectures and debates and have additionally contributed several chapters to the new EBMT Handbook.

PRINCIPAL RESEARCH STUDIES

1. Durable benefit of rituximab maintenance post-autograft in patients with relapsed follicular lymphoma: 12-year follow-up of the EBMT Lymphoma Working Party Lym1 trial long term follow up of the EBMT LYM-1 trial. Pettengell, R.

2. Efficacy of donor lymphocyte infusions for the treatment of relapse post allogeneic transplant for lymphoid malignancies. Robinson S.

3. Influence of Donor Type, Stem Cell Source and Conditioning Regimen on Transplant Outcomes after Haploidentical Transplant with Post Transplant Cyclophosphamide for Lymphoma. Bazarbachi A.

4. Haploidentical stem cell transplantation for DLBCL. A joint study of the LWP EBMT and CIBMTR LWC. Dreger P and Hamadami M.

KEY PUBLICATIONS

1. Autologous stem cell transplantation for primary mediastinal B-cell lymphoma: long-term outcome and role of post-transplant radiotherapy. A report of the European Society for Blood and Marrow Transplantation. Avivi I et al., *Bone Marrow Transplant*.

2. Allogeneic Hematopoietic Stem Cell Transplantation for Relapsed Follicular Lymphoma. A Combined Analysis on Behalf of the Lymphoma Working Party of the EBMT and the Lymphoma Committee of the CIBMTR. Sureda A et al., *Cancer*.

3. Brentuximab vedotin for recurrent Hodgkin lymphoma after allogeneic hematopoietic stem cell transplantation: A report from the EBMT Lymphoma Working Party. Bazarbachi A et al., *Cancer*.

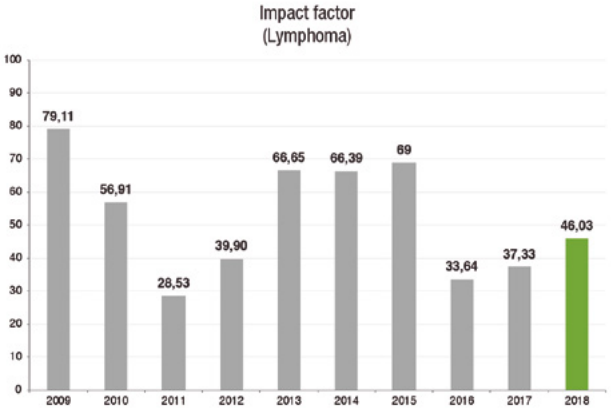
4. High-Dose Therapy and Autologous Stem Cell Transplantation in Marginal Zone

Lymphomas: A Retrospective Study by the EBMT Lymphoma Working Party and FIL-GITMO. Avivi I et al., *Br J Haematol*.

5. Brentuximab vedotin prior to allogeneic stem cell transplantation in Hodgkin lymphoma: a report from the EBMT Lymphoma Working Party. Bazarbachi A et al., *Br J Haematol*.

MAJOR EDUCATIONAL COURSES

1. 14th Edition of the Lymphoma Working Party Educational Course – 26-28 September 2018 in Palma de Mallorca, Spain.



	2014	2015	2016	2017	2018
Oral Presentations	8	6	3	8	8
Poster Presentations	11	4	5	8	9
International Educational Events	4	4	6	3	1

Figure 1. Jian-Jian Luan Award 2018

Paediatric Diseases Working Party (PDWP)

Chair: Selim Corbacioglu
Elected in September 2018
(Previous Chair: Peter Bader)

MAJOR ACHIEVEMENTS

In September 2018, after the extraordinary Chair elections, Selim Corbacioglu has been denominated as the new PDWP Chair, taking over from Peter Bader. Josu de la Fuente became Co-Chair and Katharina Kleinschmidt Secretary. The structure of the WP has been reorganised, with the introduction of specific subcommittees and the inauguration of the group of young investigators (WiPis).

The focus of the PDWP this year was on the educational level with the organisation of three educational courses/events:

1. The continuation of the "PDWP Supportive Care Workshop", initiated in 2017: Part 2 was held in January in Milano, Part 3 in Frankfurt in October. The objective was the elaboration of consensus position papers on supportive care measures in paediatric HSCT, like ward isolation and sterility policy, peri- and post-transplant food handling and vaccination.

2. In June 2018, the joint PDWP, IDWP, IEWP and Paediatric Nurses Group Meeting was held in Verona, Italy. All important and most discussed topics were addressed such as the different methods of T-cell depletion versus post-transplant cyclophosphamide in haploidentical transplantation as well as new approaches in CAR T-cell therapy in relapsed leukaemia. Much space was dedicated to case presentations by younger colleagues; a trend which will be intensified in the upcoming years: a dedicated subcommittee (WiPis) has been created to give maximum support to young investigators, for active participation in conferences and educational events.

One major mission of the PDWP is to continuously improve the quality of registry-based data. Currently, more then 20 PDWP studies are under evaluation or already ongoing (see below for most relevant studies).

Collaborative international prospective trials are a major focus of the WP and will be intensified in the upcoming years.

PRINCIPAL RESEARCH STUDIES

1. Haploidentical stem cell transplantation using PTCY for children with acute leukaemia.

2. Haematopoietic stem cell transplantation for sickle cell disease. An analysis on behalf of Eurocord, PDWP of EBMT, CIBMTR, USP and Ruby Hall Clinic.

3. Late effects after haematopoietic stem cell transplantation in patients with HLH.

4. The optimal alternative donor for ALL or AML: comparison between T-cell depleted haplo-HSCT and UCBT.

5. HSCT in children and adolescents with Non Hodgkin Lymphoma.

6. The use of TPO agonist post paediatric HSCT.

7. Pregnancy rates and pregnancy outcomes after HSCT in patients transplanted during childhood.

Figure 1. 11th Paediatric Diseases, Infectious Diseases, Inborn Errors Working Parties and 6th Paediatric Nurses Group Meeting

KEY PUBLICATIONS

1. Low Body Mass Index is Associated with Increased Risk of Acute GvHD after Umbilical Cord Blood Transplantation in Children and Young Adults with Acute Leukemia: a Study on Behalf of Eurocord and the EBMT Pediatric Disease Working Party. Paviglianiti A et al., *Biol Blood Marrow Transplant*.

2. Therapeutic Drug Monitoring of Busulfan for the Management of Pediatric Patients: Cross-Validation of Methods and Long-Term Performance. Choong E et al., *Ther Drug Monit*.

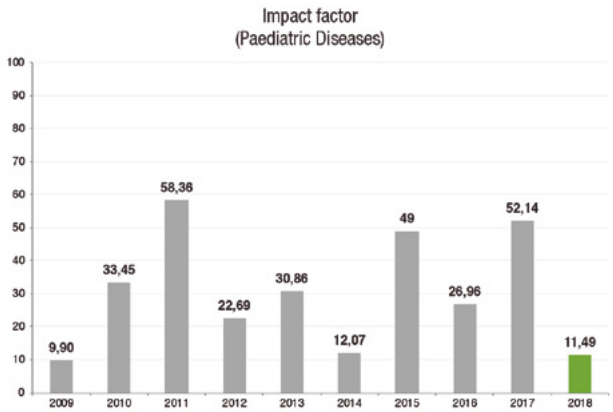
3. Second Hematopoietic Stem Cell Transplantation for Post-Transplantation Relapsed Acute Leukemia in Children: A Retrospective EBMT-PDWP Study. Yaniv I et al., *Biol Blood Marrow Transplant*.

MAJOR EDUCATIONAL COURSES

1. 11th Paediatric Diseases, Infectious Diseases, Inborn Errors Working Parties and 6th Paediatric Nurses Group Meeting – 7-9 June 2018 in Verona, Italy.

2. PDWP Supportive Care Workshop Part 2 in Milano, Italy.

3. PDWP Supportive Care Workshop Part 3 in Frankfurt, Germany.



	2014	2015	2016	2017	2018
Oral Presentations	10	6	8	6	5
Poster Presentations	7	3	7	1	7
International Educational Events	1	2	1	1	3

Chronic Malignancies Working Party (CMWP)

Chair: Ibrahim Yakoub-Agha
Elected in March 2018
(Previous Chair: Nicolaus Kröger)

MAJOR ACHIEVEMENTS

The CMWP had a strong year with publications covering all aspects of stem cell transplantation. In March 2018, I. Yakoub-Agha (Lille) was elected as CMWP Chair and P. Hayden (Dublin) became Secretary. They have continued to build on the achievements of the previous Chair, N. Kröger (Hamburg), the current EBMT President, who had completed a highly productive four-year term.

The CMWP is a unique Working Party in spanning several groups of diseases. Reflecting this range of activities, the internal organisation was rearranged resulting in six subcommittees: (1) Myelodysplasia including CMML (2) Plasma Cell Diseases (3) Ph. Neg Myeloproliferative Disorders (4) CML (5) CLL and (6) Practice Harmonisation and Guidelines. This last committee will focus on producing practical clinically relevant guidelines for haematologists in the field of HSCT.

CMML has been more clearly defined as a distinct entity in recent decades and the WP has been fortunate to have F. Onida (Milan) joined M. Robin (Paris) and C. Scheid (Cologne) as an MDS subcommittee vice-chair with expertise in this uncommon disorder. After a very successful term, L. Garderet handed over the chair of the Plasma Cell Disease subcommittee to S. Schönland (Heidelberg) who has been joined by M. Beksac (Ankara) as vice-chair. The new Ph. Neg MPN subcommittee is chaired by D. McLornan (London) and co-chaired by T. Czerw (Gliwice) and JC Hernandez Bolud (Valencia). Finally, we welcomed O. Tourniac (Clermont-Ferrand) as the new chair of the CLL subcommittee with M. van Gelder (Amsterdam) remaining as vice-chair. Y. Chalandon (Geneva) continues as chair of the CML subcommittee.

The first Business and Educational Meeting took place in Dublin in January 2018. A productive Friday session was followed by an educational symposium on “Perspectives in the treatment of myeloma and other plasma cell disorders” on Saturday morning. We were fortunate to be joined by P. Hari of the CIBMTR and plans were laid for future collaborative projects. The rapid advances in the field of immunotherapy across a range of haematological malignancies were reviewed in Lille in September where an education session focussed on CAR T cell therapy in chronic haematological malignancies followed a well-attended business meeting.

PRINCIPAL RESEARCH STUDIES

1. Phase III: Vidaza vs allogeneic SCT in patients 55 to 69 years (N. Kröger, 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).

KEY PUBLICATIONS

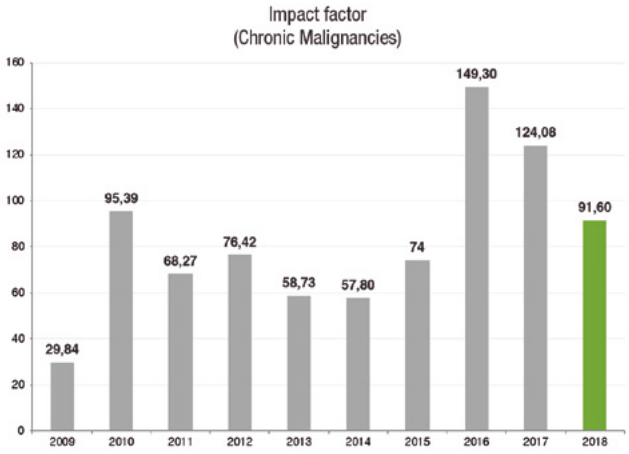
1. Outcome after relapse of myelodysplastic syndrome and secondary acute myeloid leukemia following allogeneic stem cell transplantation: a retrospective registry analysis on 698 patients by the Chronic Malignancies Working Party of the European Society of Blood and Marrow Transplantation. Schmid C et al., *Haematologica*.

2. HLA-Mismatched Donors in Patients with Myelodysplastic Syndrome: An EBMT Registry Analysis. Robin M et al., *Biol Blood Marrow Transplant*.

3. Melphalan 140 mg/m2 or 200 mg/m2 for autologous transplantation in myeloma: results from the Collaboration to Collect Autologous Transplant Outcomes in Lymphoma and Myeloma (CALM) study. A report by the EBMT Chronic Malignancies Working Party. Auner HW et al., *Haematologica*.

4. Outcome of patients with Myelofibrosis relapsing after allogeneic stem cell transplant: a retrospective study by the Chronic Malignancies Working Party of EBMT. McLornan DP et al., *Br J Haematol*.

5. High-risk chronic lymphocytic leukemia in the era of pathway inhibitors: integrating molecular and cellular therapies. Dreger P et al., *Blood*.



	2014	2015	2016	2017	2018
Oral Presentations	44	33	16	12	13
Poster Presentations	14	12	16	14	17
International Educational Events	4	2	4	2	2

MAJOR EDUCATIONAL COURSES

1. Chronic Malignancies Working Party Business & Educational Meeting – 26-27 January 2018 in Dublin, Ireland.

Educational symposium on “Perspectives in the treatment of myeloma & plasma cell disorders”.

2. Chronic Malignancies Working Party Business & Educational Meeting – 14-15 September 2018 in Lille, France.

Educational symposium on “CAR T Cell Therapy in Chronic Haematological Malignancies”.

Transplant Complications Working Party (TCWP)

Chair: Grzegorz Basak

MAJOR ACHIEVEMENTS

2018 has been a successful year for the TCWP in terms of both, education and research. The working party, chaired by G. Basak and supported by Z. Peric (Secretary), A. Harrington (Data Manager) and C. Peczynsky (Statistician), appointed new chairs of the three subcommittes: C. Koenecke for the Regimen-related toxicities and supportive care subcommittee, O. Penack for the Graft-versus-host disease subcommittee and H. Schoemans for the Late complications subcommittee as well as C. Eeltink for the Nurse Lead.

During this year, we have developed operating procedures for processing new studies and we continuously encourage EBMT members to join our working party with new proposals. We currently manage approximately 35 studies and we have successfully published 13 manuscripts this year. The TCWP has established strong links with late effects and quality of life subcommittee of the CIBMTR which resulted in very important consensus manuscripts on neurocognitive dysfunction, ocular graft-versus-host-disease and non-GVHD ocular complications. Moreover, together with the CIBMTR and National Institutes of Health, TCWP has recently published a position statement manuscript on the standardised GVHD assessment. The TCWP meets twice each year and conducts additional monthly teleconferences in order to follow the evolution of studies.

Finally, TCWP is strongly committed to educational activities. In November we organised a very dynamic educational course joined with the 4th Chronic GVHD symposium in Zagreb, Croatia. This meeting hosted a strong international faculty and gathered over 150 participants from 25 countries making it the most successful TCWP educational course so far.

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. Influence of inflammatory bowel disease on the outcome of allogeneic stem cell transplantation: A matched-pair analysis (Z. Peric).

4. Association of socioeconomic factors with survival of patients who experience severe classic acute graft versus host disease after allogeneic stem cell transplantation (A. Frankiewicz, S. Giebel).

5. Survival after acute graft-versus-host disease over time: big data analysis (H. Greinix, N. Kröger) - retrospective analysis of outcomes of >113,000 patients with aGvHD st II-IV transplanted in years 1990-2015.

Figure 1. Transplant Complications Working Party team

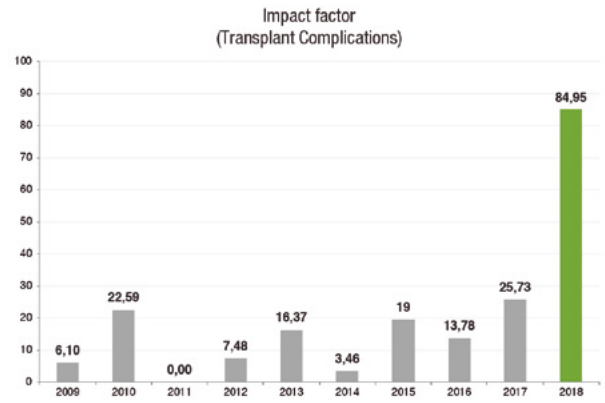
KEY PUBLICATIONS

1. Evaluation of Second Solid Cancers After Hematopoietic Stem Cell Transplantation in European Patients. Tichelli A et al., *JAMA Oncol*.

2. European experience and risk factor analysis of donor cell-derived leukaemias/MDS following haematopoietic cell transplantation. Engel N et al., *Leukemia*.

3. EBMT-NIH-CIBMTR Task Force position statement on standardized terminology & guidance for graft-versus-host disease assessment. Schoemans HM et al., *Bone Marrow Transplant*.

4. 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 et al., *Bone Marrow Transplant*.



	2014	2015	2016	2017	2018
Oral Presentations	1	2	2	4	2
Poster Presentations	2	1	3	10	2
International Educational Events	1	3	1	3	2

MAJOR EDUCATIONAL COURSES

1. 4th International cGvHD Symposium & Transplant Complications Working Party Educational Meeting – 8-10 November 2018 in Zagreb, Croatia.

Figure 2. 4th International cGvHD Symposium & Transplant Complications Working Party

Publications 2018

Title	First listed author	Journal	PMID
A survey on incidence and management of adenovirus infection after allogeneic HSCT.	Cesaro S	Bone Marrow Transplant.	30546071
Accuracy and usability of the eGVHD app in assessing the severity of graft-versus-host disease at the 2017 EBMT annual congress.	Schoemans HM	Bone Marrow Transplant.	29330389
Allogeneic Hematopoietic Cell Transplantation in Patients Aged 50Years or Older with Severe Aplastic Anemia.	Rice C	Biol Blood Marrow Transplant.	30194027
Allogeneic stem cell transplantation following relapse post autologous stem cell transplantation in adult patients with acute myeloid leukemia: A retrospective analysis of 537 patients from the Acute Leukemia Working Party of the EBMT.	Christopeit M	Am J Hematol.	30218444
Allogeneic Stem Cell Transplantation for Myelodysplastic Syndrome Patients with a 5q Deletion.	Garderet L	Biol Blood Marrow Transplant.	29196078
Allogeneic hematopoietic stem cell transplantation for relapsed follicularlymphoma: A combined analysis on behalf of the Lymphoma Working Party of the EBMT and the Lymphoma Committee of the CIBMTR.	Sureda A	Cancer.	29424927
AlloHSCT for inv(3)(q21;q26)/t(3;3)(q21;q26) AML: a report from the acute leukemia working party of the European society for blood and marrow transplantation.	Halaburda K	Bone Marrow Transplant.	29670208
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	Br J Haematol.	29468648
Anti-thymocyte globulin improves survival free from relapse and graft-versus-host disease after allogeneic peripheral blood stem cell transplantation in patients with Philadelphia-negative acute lymphoblastic leukemia: An analysis by the Acute Leukemia Working Party of the EBMT.	Czerw T	Cancer.	29603136
Association of Second Allogeneic Hematopoietic Cell Transplant vs Donor Lymphocyte Infusion With Overall Survival in Patients With Acute Myeloid Leukemia Relapse.	Kharfan-Dabaja MA	JAMA Oncol.	30003233
Autologous Haematopoietic Stem Cell Transplantation (AHSCT) in Severe Crohn's Disease: A Review on Behalf of ECCO and EBMT.	Snowden JA	J Crohns Colitis.	29325112
Autologous haematopoietic stem cell transplantation (aHSCT) for severe resistant autoimmune and inflammatory diseases - a guide for the generalist.	Snowden JA	Clin Med.	30072560
Autologous Haematopoietic Stem Cell Transplantation for Crohn's Disease: A Retrospective Survey of Long-term Outcomes from the European Society for Blood and Marrow Transplantation.	Brierley CK	J Crohns Colitis.	29788233
Autologous stem cell transplantation for primary mediastinal B-cell lymphoma: long-term outcome and role of post-transplant radiotherapy. A report of the European Society for Blood and Marrow Transplantation.	Avivi I	Bone Marrow Transplant.	29463854
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	Bone Marrow Transplant.	30531916
Bone marrow versus mobilized peripheral blood stem cells in haploidentical transplants using posttransplantation cyclophosphamide.	Ruggeri A	Cancer.	29360162
Brentuximab vedotin for recurrent Hodgkin lymphoma after allogeneic hematopoietic stem cell transplantation: A report from the EBMT Lymphoma Working Party.	Bazarbachi A	Cancer.	30351488
Brentuximab vedotin prior to allogeneic stem cell transplantation in Hodgkin lymphoma: a report from the EBMT Lymphoma Working Party.	Bazarbachi A	Br J Haematol.	29468647

Title	First listed author	Journal	PMID
Chronic graft-versus-host disease features in double unit cord blood transplantation according to National Institutes of Health 2005 cGVHD Consensus criteria.	Hayashi H	Bone Marrow Transplant.	29330402
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	Bone Marrow Transplant.	30361500
Clinical Utilization of Chimeric Antigen Receptor T Cells in B Cell Acute Lymphoblastic Leukemia: An Expert Opinion from the European Society for Blood and Marrow Transplantation and the American Society for Blood and Marrow Transplantation.	Kansagra AJ	Biol Blood Marrow Transplant.	30576834
Comparable outcomes of haploidentical, 10/10 and 9/10 unrelated donor transplantation in adverse karyotype AML in first complete remission.	Lorentino F	Am J Hematol.	30058714
Comparable results of autologous and allogeneic haematopoietic stem cell transplantation for adults with Philadelphia-positive acute lymphoblastic leukaemia in first complete molecular remission: An analysis by the Acute Leukemia Working Party of the EBMT.	Lorentino F	Am J Hematol.	30058714
Comparable survival using a CMV-matched or a mismatched donor for CMV+ patients undergoing T-replete haplo-HSCT with PT-Cy for acute leukemia: a study of behalf of the infectious diseases and acute leukemia working parties of the EBMT.	Cesaro S	Bone Marrow Transplant.	29330396
Comparative Outcomes of Myeloablative and Reduced-Intensity Conditioning Allogeneic Hematopoietic Cell Transplantation for Therapy-Related Acute Myeloid Leukemia with Prior Solid Tumor: a report from the ALWP of the EBMT.	Lee CJ	Am J Hematol.	30597620
Comparison of FLAMSA-based reduced intensity conditioning with treosulfan/fludarabine conditioning for patients with acute myeloid leukemia: an ALWP/EBMT analysis.	Sheth V	Bone Marrow Transplant.	30087463
Conditioning intensity in secondary AML with prior myelodysplastic syndrome/ myeloproliferative disorders: an EBMT ALWP study.	Sengsayadeth S	Blood Adv.	30143527
Cord blood transplantation is associated with good outcomes in secondary Acute Myeloid Leukaemia in first remission.	Baron F	J Intern Med.	30561052
Cord Blood Unit Dominance Analysis and Effect of the Winning Unit on Outcomes afterDouble-Unit Umbilical Cord Blood Transplantation in Adults with Acute Leukemia: A Retrospective Study on Behalf of Eurocord, the Cord Blood Committee of Cellular Therapy, Immunobiology Working Party, and the Acute Leukemia Working Party of the EuropeanGroup for Blood and Marrow Transplantation.	Tozatto-Maio K	Biol Blood Marrow Transplant.	29477777
Current Understanding and Future Research Priorities in Malignancy Associated With Inborn Errors of Immunity and DNA Repair Disorders: The Perspective of an Interdisciplinary Working Group.	Bomken S	Front Immunol.	30619276
Cyclophosphamide versus etoposide in combination with total body irradiation as conditioning regimen for adult patients with Ph-negative acute lymphoblastic leukemia undergoing allogeneic stem cell transplant: On behalf of the ALWP of the European Society for Blood and Marrow Transplantation.	Czyz A	Am J Hematol.	29574915
Distinct factors determine the kinetics of disease relapse in adults transplanted for acute myeloid leukaemia.	Craddock C	J Intern Med.	29214689
Early and late outcomes after cord blood transplantation for pediatric patients with inherited leukodystrophies.	van den Broek BTA	Blood Adv.	29344584
EBMT-NIH-CIBMTR Task Force position statement on standardized terminology & guidance for graft-versus-host disease assessment.	Schoemans HM	Bone Marrow Transplant.	29872128
ECIL guidelines for the prevention, diagnosis and treatment of BK polyomavirus-associated haemorrhagic cystitis in haematopoietic stem cell transplant recipients.	Cesaro S	J Antimicrob Chemother.	29190347
European experience and risk factor analysis of donor cell-derived leukaemias/MDS following haematopoietic cell transplantation.	Engel N	Leukemia.	30050122

Title	First listed author	Journal	PMID
European guidelines for primary antifungal prophylaxis in adult haematology patients: summary of the updated recommendations from the European Conference on Infections in Leukaemia.	Maertens JA	J Antimicrob Chemother.	30085172
Evaluation of Second Solid Cancers After Hematopoietic Stem Cell Transplantation in European Patients.	Tichelli A	JAMA Oncol.	30476975
Ex vivo and in vivo T cell-depleted allogeneic stem cell transplantation in patients with acute myeloid leukemia in first complete remission resulted in similar overall survival: on behalf of the ALWP of the EBMT and the MSKCC.	Malard F	J Hematol Oncol.	30342553
Family mismatched allogeneic stem cell transplantation for Myelofibrosis: Report from the Chronic Malignancies Working Party of EBMT.	Raj K	Biol Blood Marrow Transplant.	30408564
First line treatment of aplastic anemia with thymoglobuline in Europe and Asia: Outcome of 955 patients treated 2001-2012.	Bacigalupo A	Am J Hematol.	29498107
Fluoroquinolone prophylaxis in haematological cancer patients with neutropenia: ECIL critical appraisal of previous guidelines.	Mikulska M	J Infect.	29079323
Handling, processing and disposal of stem cell products in Europe: A survey by the cellular therapy and immunobiology working party of the European Society for Blood and Marrow Transplantation.	Holbro A	Cytotherapy.	29352666
Haploidentical transplantation is associated with better overall survival when compared to single cord blood transplantation: an EBMT-Eurocord study of acute leukemia patients conditioned with thiotepa, busulfan, and fludarabine.	Giannotti F	J Hematol Oncol.	30165887
Haploidentical transplantation outcomes for secondary acute myeloid leukemia: Acute Leukemia Working Party (ALWP) of the European Society for Blood and Marrow Transplantation (EBMT) study.	Li Z	Am J Hematol.	29536560
Haploidentical versus unrelated allogeneic stem cell transplantation for relapsed/ refractory acute myeloid leukemia: A report of 1578 patients from the Acute Leukemia Working Party of EBMT.	Brissot E	Haematologica.	30361416
Health-care professionals' perspective on discussing sexual issues in adult patients after haematopoietic cell transplantation.	Eeltink CM	Bone Marrow Transplant.	29247220
Hematopoietic stem cell therapy for autoimmune diseases - Clinical experience and mechanisms.	Alexander T	J Autoimmun.	29934135
Hematopoietic stem cell transplantation as treatment for patients with DOCK8 deficiency.	Aydin SE	J Allergy Clin Immunol Pract.	30391550
Hematopoietic stem cell transplantation for adult patients with isolated NPM1 mutated acute myeloid leukemia in first remission.	Poiré X	Am J Hematol.	30456896
Hematopoietic stem cell transplantation for adults with Philadelphia chromosome-negative acute lymphoblastic leukemia in first remission: a position statement of the European Working Group for Adult Acute Lymphoblastic Leukemia (EWALL) and the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation (EBMT).	Giebel S	Bone Marrow Transplant.	30385870
Hematopoietic stem cell transplantation in its 60s: A platform for cellular therapies.	Chabannon C	Sci Transl Med.	29643233
High-dose therapy and autologous stem cell transplantation in marginal zone lymphomas: a retrospective study by the EBMT Lymphoma Working Party and FIL-GITMO.	Avivi I	Br J Haematol.	29984825
High-dose therapy with BEAC conditioning compared to BEAM conditioning prior to autologous stem cell transplantation for non-Hodgkin lymphoma: no differences in toxicity or outcome. A matched-control study of the EBMT-Lymphoma Working Party	Robinson SP	Bone Marrow Transplant.	29884850

Title	First listed author	Journal	PMID
High-risk chronic lymphocytic leukemia in the era of pathway inhibitors: integrating molecular and cellular therapies.	Dreger P	Blood.	29997221
HLA-Mismatched Donors in Patients with Myelodysplastic Syndrome: An EBMT Registry Analysis.	Robin M	Biol Blood Marrow Transplant.	30172776
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	Bone Marrow Transplant.	29728701
Immune monitoring in allogeneic hematopoietic stem cell transplant recipients: a survey from the EBMT-CTIWP.	Greco R	Bone Marrow Transplant.	29666449
Immune Reconstitution After Autologous Hematopoietic Stem Cell Transplantation in Crohn's Disease: Current Status and Future Directions. A Review on Behalf of the EBMT Autoimmune Diseases Working Party and the Autologous Stem Cell Transplantation In Refractory CD-Low Intensity Therapy Evaluation Study Investigators.	Pockley AG	Front Immunol.	29670622
Impact of antithymocyte globulin doses in reduced intensity conditioning before allogeneic transplantation from matched sibling donor for patients with acute myeloid leukemia: a report from the acute leukemia working party of European group of Bone Marrow Transplantation.	Devillier R	Bone Marrow Transplant.	29330391
Impact of anti-thymocyte globulin on results of allogeneic peripheral blood stem cell transplantation for patients with Philadelphia-positive acute lymphoblastic leukaemia: An analysis by the Acute Leukemia Working Party of the EBMT.	Giebel S	Eur J Cancer.	30528805
Impact of Donor Type in Patients with AML Given Allogeneic Hematopoietic Cell Transplantation After Low-Dose TBI-Based Regimen.	Baron F	Clin Cancer Res.	29555662
Impact of extramedullary disease in patients with newly diagnosed multiple myeloma undergoing autologous stem cell transplantation: a study from the Chronic Malignancies Working Party of the EBMT.	Gagelmann N	Haematologica.	29419433
Incidence of Second Primary Malignancies after Autologous Transplantation for Multiple Myeloma in the Era of Novel Agents.	Sahebi F	Biol Blood Marrow Transplant.	29339268
Incidence, Risk Factors, and Long-term Outcome of Acute Leukemia Patients With EarlyCandidemia After Allogeneic Stem Cell Transplantation: A Study by the Acute Leukemia and Infectious Diseases Working Parties of European Society for Blood and Marrow Transplantation.	Cesaro S	Clin Infect Dis.	29481599
Is the use of unrelated donor transplantation leveling off in Europe? The 2016 European Society for Blood and Marrow Transplant activity survey report.	Passweg JR	Bone Marrow Transplant.	29540849
Killer cell immunoglobulin-like receptor ligand mismatching and outcome after haploidentical transplantation with post-transplant cyclophosphamide.	Shimoni A	Leukemia.	29907809
Late treatment-related mortality versus competing causes of death after allogeneic transplantation for myelodysplastic syndromes and secondary acute myeloid leukemia.	Schetelig J	Leukemia.	30573777
Long-term follow-up of IPEX syndrome patients after different therapeutic strategies: An international multicenter retrospective study.	Barzaghi F	J Allergy Clin Immunol.	29241729
Long-term outcome analysis of reduced-intensity allogeneic stem cell transplantation in patients with mantle cell lymphoma: a retrospective study from the EBMT Lymphoma Working Party.	Robinson SP	Bone Marrow Transplant.	29335632
Low Body Mass Index Is Associated with Increased Risk of Acute GVHD after UmbilicalCord Blood Transplantation in Children and Young Adults with Acute Leukemia: A Studyon Behalf of Eurocord and the EBMT Pediatric Disease Working Party.	Paviglianiti A	Biol Blood Marrow Transplant.	29288817

Title	First listed author	Journal	PMID
Management of adenovirus infection in patients after haematopoietic stem cell transplantation: State-of-the-art and real-life current approach: A position statement on behalf of the Infectious Diseases Working Party of the European Society of Blood and Marrow Transplantation.	Hiwarkar P	Rev Med Virol.	29663594
Manufacturing Mesenchymal Stromal Cells for the Treatment of Graft-versus-Host Disease: A Survey among Centers Affiliated with the European Society for Blood and Marrow Transplantation.	Trento C	Biol Blood Marrow Transplant.	30031938
Measurable residual disease, conditioning regimen intensity, and age predict outcome of allogeneic hematopoietic cell transplantation for acute myeloid leukemia in first remission: A registry analysis of 2292 patients by the Acute Leukemia Working Party European Society of Blood and Marrow Transplantation.	Gilleece MH	Am J Hematol.	29981272
Melphalan 140 mg/m2 or 200 mg/m2 for autologous transplantation in myeloma: results from the Collaboration to Collect Autologous Transplant Outcomes in Lymphoma and Myeloma (CALM) study. A report by the EBMT Chronic Malignancies Working Party.	Auner HW	Haematologica.	29217776
Minimal residual disease status predicts outcome of acute myeloid leukaemia patients undergoing T-cell replete haploidentical transplantation. An analysis from the Acute Leukaemia Working Party (ALWP) of the European Society for Blood and Marrow Transplantation (EBMT).	Canaani J	Br J Haematol.	30117144
Neurocognitive dysfunction in hematopoietic cell transplant recipients: expert review from the late effects and Quality of Life Working Committee of the CIBMTR and complications and Quality of Life Working Party of the EBMT.	Buchbinder D	Bone Marrow Transplant.	29343837
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	Biol Blood Marrow Transplant.	30521975
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	Bone Marrow Transplant.	30531955
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	Biol Blood Marrow Transplant.	30481594
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	Bone Marrow Transplant.	30531954
Optimizing the pretransplant regimen for autologous stem cell transplantation in acute myelogenous leukemia: Better outcomes with busulfan and melphalan compared with busulfan and cyclophosphamide in high risk patients autografted in first complete remission: A study from the acute leukemia working party of the EBMT.	Gorin NC	Am J Hematol.	29644709
Outcome after relapse of myelodysplastic syndrome and secondary acute myeloid leukemia following allogeneic stem cell transplantation: a retrospective registry analysis on 698 patients by the Chronic Malignancies Working Party of the European Society of Blood and Marrow Transplantation.	Schmid C	Haematologica.	29101205
Outcome of a Salvage Third Autologous Stem Cell Transplantation in Multiple Myeloma.	Garderet L	Biol Blood Marrow Transplant.	29408334
Outcome of domino hematopoietic stem cell transplantation in human subjects: An international case series.	Belderbos ME	J Allergy Clin Immunol.	29981805
Outcome of haematopoietic stem cell transplantation in dyskeratosis congenita.	Fioredda F	Br J Haematol.	29984823
Outcome of patients with Myelofibrosis relapsing after allogeneic stem cell transplant: a retrospective study by the Chronic Malignancies Working Party of EBMT.	McLornan DP	Br J Haematol.	29808926

Title	First listed author	Journal	PMID
Outcomes of Advanced Hodgkin Lymphoma after Umbilical Cord Blood Transplantation: A Eurocord and EBMT Lymphoma and Cellular Therapy & Immunobiology Working Party Study.	Paviglianiti A	Biol Blood Marrow Transplant.	30031070
Outcomes of haploidentical stem cell transplantation for chronic lymphocytic leukemia: a retrospective study on behalf of the chronic malignancies working party of the EBMT.	van Gorkom G	Bone Marrow Transplant.	29255169
Outcomes of Haploidentical Transplantation in Patients with Relapsed Multiple Myeloma: An EBMT/CIBMTR Report.	Sahebi F	Biol Blood Marrow Transplant.	30243581
Outcomes of hematopoietic stem cell transplantation from unmanipulated haploidentical versus matched sibling donor in patients with acute myeloid leukemia in first complete remission with intermediate or high-risk cytogenetics: a study from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Salvatore D	Haematologica.	29748438
Post-remission strategies for the prevention of relapse following allogeneic hematopoietic cell transplantation for high-risk acute myeloid leukemia: expert review from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Lee CJ	Bone Marrow Transplant.	30104717
Post-transplant cyclophosphamide for graft-versus-host disease prophylaxis in HLA matched sibling or matched unrelated donor transplant for patients with acute leukemia, on behalf of ALWP-EBMT.	Ruggeri A	J Hematol Oncol.	29544522
Prevention and treatment of relapse after stem cell transplantation by cellular therapies.	Falkenburg F	Bone Marrow Transplant.	29795426
Prophylactic donor lymphocyte infusion after allogeneic stem cell transplantation in acute leukaemia - a matched pair analysis by the Acute Leukaemia Working Party of EBMT.	Schmid C	Br J Haematol.	30467839
Protective environment for hematopoietic cell transplant (HSCT) recipients: The Infectious Diseases Working Party EBMT analysis of global recommendations on health-care facilities.	Styczynski J	Bone Marrow Transplant.	29535381
Recommendations from the European Society for Blood and Marrow Transplantation (EBMT) for a curriculum in hematopoietic cell transplantation.	Mohty M	Bone Marrow Transplant.	29720705
Reduced Relapse Incidence with FLAMSA-RIC Compared with Busulfan/Fludarabine for Acute Myelogenous Leukemia Patients in First or Second Complete Remission: A Study from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Heinicke T	Biol Blood Marrow Transplant.	30009981
Refined graft-versus-host disease/relapse-free survival in transplant from HLA-identical related or unrelated donors in acute myeloid leukemia.	Battipaglia G	Bone Marrow Transplant.	29662244
Relapse and survival after transplantation for complex karyotype acute myeloid leukemia: A report from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation and the University of Texas MD Anderson Cancer Center.	Ciurea SO	Cancer.	29469961
Related donor transplants: has posttransplantation cyclophosphamide nullified the detrimental effect of HLA mismatch?	Robinson TM	Blood Adv.	29794073
Risk factors affecting outcome of unrelated cord blood transplantation for children with familial haemophagocytic lymphohistiocytosis.	Furtado-Silva JM	Br J Haematol.	30460979
Second Hematopoietic Stem Cell Transplantation for Post-TransplantationRelapsed Acute Leukemia in Children: A Retrospective EBMT-PDWP Study.	Yaniv I	Biol Blood Marrow Transplant.	29548831
Secondary malignancies after high-dose chemotherapy in germ cell tumor patients: a 34-year retrospective study of the European Society for Blood and Marrow Transplantation (EBMT).	Necchi A	Bone Marrow Transplant.	29367713

Title	First listed author	Journal	PMID
Single Dose Daily Fractionated Is Not Inferior To Twice A Day Fractionated Total Body Irradiation Prior To Allogeneic Stem Cell Transplantation For Acute Leukemia: A Useful Practice Simplification Resulting From The Sarasin Study.	Belkacemi Y	Int J Radiat Oncol Biol Phys.	29928948
Solid organ transplantation after haematopoietic stem cell transplantation in childhood: a multicentric retrospective survey.	Faraci M	Am J Transplant.	30586230
T-cell replete haploidentical stem cell transplantation attenuates the prognostic impact of FLT3-ITD in acute myeloid leukemia: A report from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Canaani J	Am J Hematol.	29498106
T-cell-depleted haploidentical stem cell transplantation results improve with time in adults with acute leukemia: A study from the Acute Leukemia Working Party of the European Society of Blood and Marrow Transplantation (EBMT).	Sestili S	Cancer.	29469924
The effect of NIMA matching in adult unrelated mismatched hematopoietic stem cell transplantation - a joint study of the Acute Leukemia Working Party of the EBMT and the CIBMTR.	Pingel J	Bone Marrow Transplant.	30279575
The eGVHD App has the potential to improve the accuracy of graft versus host disease assessment: a multicenter randomized controlled trial.	Schoemans HM	Haematologica.	29903762
The European Society for Blood and Marrow Transplantation (EBMT) Consensus Guidelines for the Detection and Treatment of Donor-specific Anti-HLA Antibodies (DSA) in Haploidentical Hematopoietic Cell Transplantation.	Ciurea SO	Bone Marrow Transplant.	29335625
The impact of advanced patient age on mortality after allogeneic hematopoietic Cell Transplantation for Non-Hodgkin's Lymphoma: A retrospective study by the EBMT Lymphoma Working Party.	Kyriakou C	Biol Blood Marrow Transplant.	30219698
Therapeutic Drug Monitoring of Busulfan for the Management of Pediatric Patients: Cross-Validation of Methods and Long-Term Performance.	Choong E	Ther Drug Monit.	29189665
Thiotepa, busulfan and fludarabine compared to busulfan and cyclophosphamide as conditioning regimen for allogeneic stem cell transplant from matched siblings and unrelated donors for acute myeloid leukemia.	Saraceni F	Am J Hematol.	30033639
Thiotepa-busulfan-fludarabine compared to busulfan-fludarabine for sibling and unrelated donor transplant in acute myeloid leukemia in first remission.	Saraceni F	Oncotarget.	29423053
Transplant outcome for patients with acquired aplastic anemia over the age of 40: has the outcome improved?	Giammarco S	Blood.	29549172
Transplant Outcomes for Secondary Acute Myeloid Leukemia: Acute Leukemia Working Party of the European Society for Blood and Bone Marrow Transplantation Study.	Sengsayadeth S	Biol Blood Marrow Transplant.	29678639
Transplant results in adults with Fanconi anaemia.	Bierings M	Br J Haematol.	29094350
Trends in patient outcome over the past two decades following allogeneic stem cell transplantation for acute myeloid leukemia. An ALWP/EBMT analysis.	Canaani J	J Intern Med.	30372796
Unmanipulated haploidentical in comparison with matched unrelated donor stem cell transplantation in patients 60 years and older with acute myeloid leukemia: a comparative study on behalf of the ALWP of the EBMT.	Santoro N	J Hematol Oncol.	29661208
Variability of nutritional practices in peritransplant period after allogeneic hematopoietic stem cell transplantation: a survey by the Complications and Quality of Life Working Party of the EBMT.	Peric Z	Bone Marrow Transplant.	29515252

EBMT Transplant Activity Survey 2017

In 2017, 683 teams reported 45,418 transplants in 41,100 patients (first transplant) to the annual activity survey. Of these, 18,281 HCT (40%) were allogeneic and 27,137 (60%) autologous (Table 1). When compared with 2016, the total number of transplants increased by 4.1% (3.6% allogeneic HCT and 4.4% autologous HCT). In addition, there were 4,318 second or subsequent transplants, 1,126 being allogeneic, mainly to treat relapse or graft failure and 3,192 autologous, the majority of which were part of multiple transplant procedures such as either tandem procedures, or as salvage autologous transplants for plasma cell disorders.

The total number of patients transplanted under the age of 18 in both dedicated and joint adult-paediatric units was 5,056 (3,725 allogeneic and 1,331 autologous). This is an increase of 7.8% (5.1% allogeneic, 16.2% autologous) when compared to 2016.

Main indications for HCT were myeloid malignancies (AML, CML, MDS or MD/MPN overlap and MPN): 10,147 (25% of total; 96% of which were allogeneic); lymphoid malignancies (ALL, CLL, HL, NHL and PCD): 26,488 (64%; 19% allogeneic); solid tumors: 1,607 (4%; 2% allogeneic); non-malignant disorders: 2,667 (7%; 81% allogeneic) and others: 191 (0.5%).

Figure 1 shows the distribution of disease indications for allogeneic (Figure 1a) and autologous (Figure 1b) HCT as a pie graph. In Figure 1a, AML is the most frequent indication (39%), of these 56% were for patients in CR1, 29% for patients with more advanced disease and 15% for patients with transformed AML, either therapy-related or from MDS/MPN. The number of patients has increased 3.6-fold over a twenty-year period from 4,751 in 1997 to 17,155 in 2017. During this time, the decline in HCT for CML from 25.3% to 1.9% can be clearly observed. Whereas AML, MDS and MPN have increased substantially during this period, the proportion of ALL transplants has decreased only slightly.

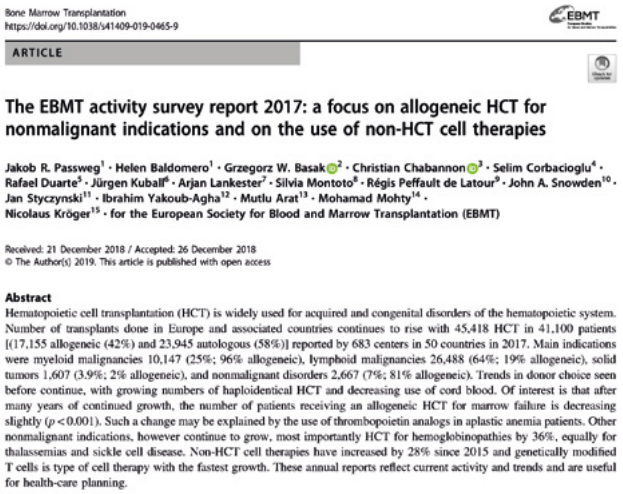
In Figure 1b, the most frequent indication is for plasma cell disorders (53%). The number of patients has increased 2-fold over a twenty-year period, from 12,199 in 1997 to 23,945 in 2017 with myeloma being the dominant indication; increasing from 18% to 53% during this time. Proportions of HCTs for Hodgkin and Non-Hodgkin lymphoma have remained stable. Autologous HCT for AML has decreased from 13% to 1.72%. Already well described is the change in autologous HCT for solid tumor where the decrease in use of this technology in breast cancer has decreased to 0.1%.

CELLULAR THERAPY USE

Table 2 shows cell therapies performed in EBMT centers in 2017. 2,825 patients received donor lymphocyte infusions. The majority of DLI's were given for relapse (1,284) and graft enhancement (661). Other reasons being for residual disease (422) and 'per protocol' (458). 1,202 patients received other forms of cell therapy, most commonly mesenchymal stromal cells (n=557; 91% allogeneic), mainly to treat graft versus host disease. Expanded / selected T lymphocytes (n=179; 100% allogeneic) and genetically modified T cells (n=151; 50% allogeneic) were the second most frequently reported cell therapy. The area with the highest growth is genetically modified T-cells. The main indications for cell therapies (excluding DLI) being GvHD or graft enhancement (n=688; 95% allogeneic), malignancy (n=253; 50% allogeneic), infections (n=124; 100% allogeneic), autoimmune disease (n=61; 43% allogeneic) and genetic disease (n=14; 7% allogeneic).

The annual activity survey of the EBMT reflects current activity and trends in the field of transplant technology. It is valuable for the dissemination of the most recent information on indications, donor and stem cell usage, which can ultimately be beneficial in health care planning.

Helen Baldomero, Jakob R Passweg
EBMT Activity Survey Office, Hematology, Department of Medicine, University Hospital, Basel, Switzerland



Bone Marrow Transplantation
https://doi.org/10.1038/s41409-019-0465-9

ARTICLE

The EBMT activity survey report 2017: a focus on allogeneic HCT for nonmalignant indications and on the use of non-HCT cell therapies

Jakob R. Passweg[1] · Helen Baldomero[1] · Grzegorz W. Basak[2] · Christian Chabannon[3] · Selim Corbacioglu[4] · Rafael Duarte[5] · Jürgen Kuball[6] · Arjan Lankester[7] · Silvia Montoto[8] · Régis Peffault de Latour[9] · John A. Snowden[10] · Jan Styczynski[11] · Ibrahim Yakoub-Agha[12] · Mutlu Arat[13] · Mohamad Mohty[14] · Nicolaus Kröger[15] · for the European Society for Blood and Marrow Transplantation (EBMT)

Received: 21 December 2018 / Accepted: 26 December 2018

Abstract
Hematopoietic cell transplantation (HCT) is widely used for acquired and congenital disorders of the hematopoietic system. Number of transplants done in Europe and associated countries continues to rise with 45,418 HCT in 41,100 patients [(17,155 allogeneic (42%) and 23,945 autologous (58%)] reported by 683 centers in 50 countries in 2017. Main indications were myeloid malignancies 10,147 (25%; 96% allogeneic), lymphoid malignancies 26,488 (64%; 19% allogeneic), solid tumors 1,607 (3.9%; 2% allogeneic), and nonmalignant disorders 2,667 (7%; 81% allogeneic). Trends in donor choice seen before continue, with growing numbers of haploidentical HCT and decreasing use of cord blood. Of interest is that after many years of continued growth, the number of patients receiving an allogeneic HCT for marrow failure is decreasing slightly ($p < 0.001$). Such a change may be explained by the use of thrombopoietin analogs in aplastic anemia patients. Other nonmalignant indications, however continue to grow, most importantly HCT for hemoglobinopathies by 36%, equally for thalassemias and sickle cell disease. Non-HCT cell therapies have increased by 28% since 2015 and genetically modified T cells is type of cell therapy with the fastest growth. These annual reports reflect current activity and trends and are useful for health-care planning.

Table 1:

NUMBERS OF HEMATOPOIETIC STEM CELL TRANSPLANTS IN EUROPE 2017 BY INDICATION, DONOR TYPE AND STEM CELL SOURCE.

	TRANSPLANT ACTIVITY 2017																	
	No. of patients																	
	Allogeneic												Autologous			Total		
	Family									Unrelated								
	HLA-id			Twin	Haplo >=2MM		Other family						BM	BM +		Allo	Auto	Total
	BM	PBPC	Cord	all	BM	PBSC	BM	PBPC	Cord	BM	PBPC	cord	only	PBPC	cord			
Myeloid malignancies	**380**	**2532**	**0**	**11**	**319**	**946**	**10**	**85**	**1**	**527**	**4804**	**157**	**7**	**368**	**0**	**9772**	**375**	**10147**
Acute myeloid leukemia	267	1802	0	7	235	716	7	70	1	350	3101	120	7	353	0	6676	360	7036
1st complete remission	195	1123		7	116	330	5	40		218	1655	64	4	289		3753	293	4046
not 1st complete remission	50	474			80	270	1	20	1	96	919	39	3	56		1950	59	2009
AML therapy related	8	53			14	46		3		15	130	8		2		277	2	279
AML from MDS/MPN	14	152			25	70	1	7		21	397	9		6		696	6	702
Chronic myeloid leukemia	35	84	0	0	6	22	0	2	0	25	161	0	0	0	0	335	0	335
chronic phase	22	35			2	6		2		14	55					136	0	136
not chronic phase	13	49			4	16				11	106					199	0	199
MDS or MD/MPN overlap	66	478		4	64	169	3	9		132	1097	34		5		2056	5	2061
MPN	12	168			14	39		4		20	445	3		10		705	10	715
Lymphoid malignancies	**297**	**1434**	**6**	**7**	**193**	**579**	**11**	**47**	**1**	**408**	**1963**	**69**	**29**	**21444**	**0**	**5015**	**21473**	**26488**
Acute lymphatic leukemia	245	731	6	3	89	285	9	38	1	340	876	58	5	85	0	2681	90	2771
1st complete remission	139	516	2	1	49	130	7	30		158	590	30	4	78		1652	82	1734
not 1st complete remission	106	215	4	2	40	155	2	8	1	182	286	28	1	7		1029	8	1037
Chronic lymphocytic leukemia	4	50			6	20		3		5	141	1		9		230	9	239
Plasma cell disorders - MM	5	122		1	12	17		2		8	192	1	4	12353		360	12357	12717
Plasma cell disorders - other		6				2				1	16			335		25	335	360
Hodgkin lymphoma	16	131	0	1	30	99		1		11	153	2	11	2141		444	2152	2596
Non Hodgkin lymphoma	27	394		2	56	156	2	3		43	585	7	9	6521		1275	6530	7805
Solid tumors	**3**	**1**	**0**	**0**	**10**	**20**	**0**	**0**	**0**	**0**	**2**	**0**	**48**	**1521**	**2**	**36**	**1571**	**1607**
Neuroblastoma	2				8	14					2		22	513	2	26	537	563
Soft tissue sarcoma/Ewing		1			1	6							4	225		8	229	237
Germinal tumors													2	370		0	372	372
Breast cancer														13		0	13	13
Other solid tumors	1				1								20	400		2	420	422
Non malignant disorders	**668**	**246**	**27**	**3**	**96**	**167**	**61**	**61**	**2**	**450**	**325**	**67**	**15**	**479**	**0**	**2173**	**494**	**2667**
Bone marrow failure - SAA	176	107		3	28	32	3	6		120	100	8		1		583	1	584
Bone marrow failure - other	54	23	3		8	14	7	10		62	35	3				219	0	219
Thalassemia	178	57	15		5	18	22	15	1	58	49	2	2	4		420	6	426
Sickle cell disease	110	41	6		11	14	6	2		19	5	1	1			215	1	216
Primary Immune deficiencies	125	10	3		33	81	15	19	1	144	102	21	6	7		554	13	567
Inh. disorders of Metabolism	23	3			8	6	7	7		44	29	32	5	5		159	10	169
Auto immune disease	2	5			3	2	1	2		3	5		1	462		23	463	486
Others	28	17			11	20	5	2		31	39	6		32		159	32	191
TOTAL PATIENTS	**1376**	**4230**	**33**	**21**	**629**	**1732**	**87**	**195**	**4**	**1416**	**7133**	**299**	**99**	**23844**	**2**	**17155**	**23945**	**41100**
TOTAL TRANSPLANTS	**1420**	**4411**	**33**	**21**	**708**	**1981**	**89**	**212**	**5**	**1492**	**7579**	**330**	**112**	**27022**	**3**	**18281**	**27137**	**45418**

Ref: Passweg et al. Bone Marrow Transplantation February 2019

Table 2:

NUMBERS OF CELL THERAPIES IN EUROPE 2017 BY INDICATION, DONOR TYPE AND CELL SOURCE.

Number of patients	DLI	MSC		NK cells		selected/ expanded T cells or CIK		Regulatory T cells (TREGS)		Genetically modified T cells		Dendritic cells		Expanded CD34+ cells		Genetically modified CD34+ cells		Other	
		Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto	Allo	Auto
GvHD		413	5			8		36										13	
Graft enhancement	661	55	18	1		14		5		57			4	6		1		44	8
Autoimmune dis.		6	14	19										1					21
Genetic disease		1															13		
Infection		3				113												8	
Malignancy				6		42		24	8	16	78	5	35	2				32	5
DLI for residual disease	422																		
DLI for relapse	1284																		
DLI per protocol	458																		
Regenerative medicine		31	11	1		2								2				4	11
Total	**2825**	**509**	**48**	**27**	**0**	**179**	**0**	**65**	**8**	**73**	**78**	**5**	**39**	**11**	**0**	**1**	**13**	**101**	**45**

Figure 1:

RELATIVE PROPORTION OF DISEASE INDICATIONS FOR HCT IN EUROPE 2017.

Fig 1a: *RELATIVE PROPORTION OF ALLOGENEIC HCT*

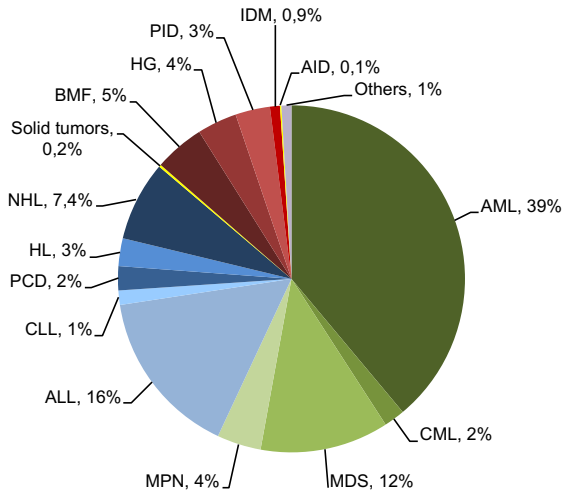
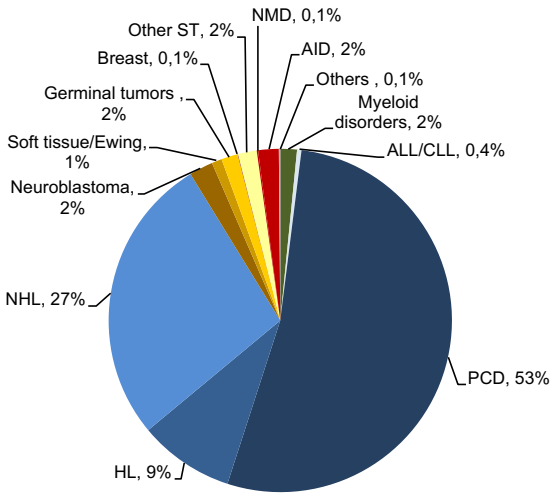


Fig 1b: *RELATIVE PROPORTION OF AUTOLOGOUS HCT*



Abbreviations:

HCT; hematopoietic stem cell transplant, 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, SAA; severe aplastic anemia. CR1; 1st. complete remission, DLI; donor lymphocyte infusion, MSC; mesenchymal stromal cells.

The EBMT Registry

During 2018 the EBMT Registry received a draft positive qualification opinion from the European Medicines Agency (EMA) regarding its Cellular Therapy module. The qualification opinion was released on February 28, 2019 (see page 44). In addition, the data collection form (MED-A) for Cellular Therapy has undergone further review during the year. More detail is included below.

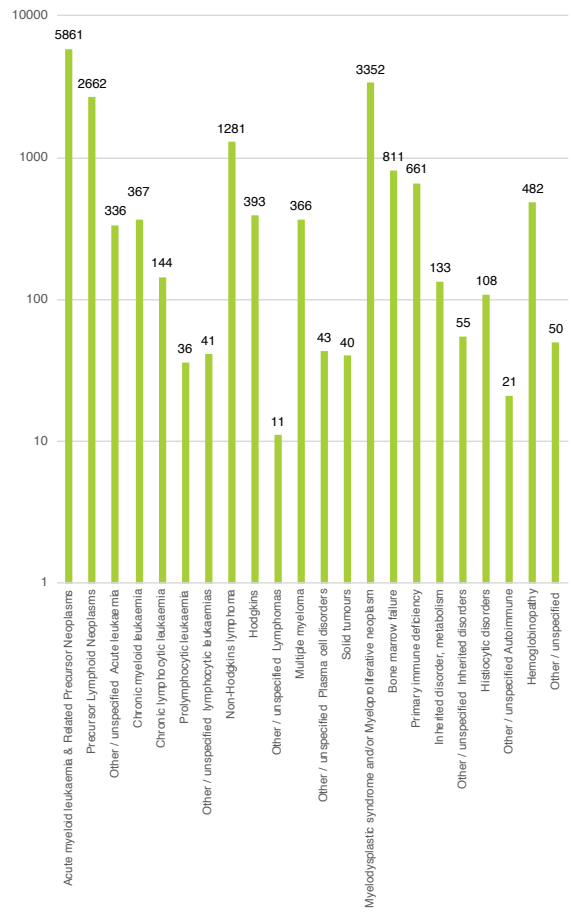
At the Registry we are continuing to work with Elsevier, our chosen supplier for the New Registry System. The solution named MACRO is due to replace ProMISe and go live in the first half of 2019. Please see below for more information on the progress and plan.

By the end of 2018, the entire Registry included at least 666,000 transplant registrations in total. New registrations (allografts) entered during 2018 increased by approximately 10% on the previous year. We include the charts below.

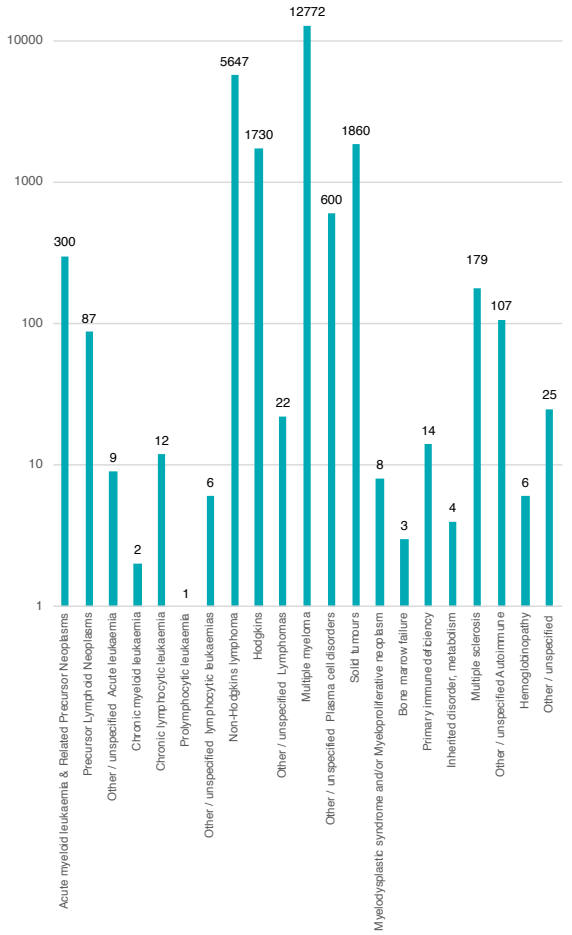
NEW REGISTRATIONS

17,256 allografts and 23,397 autografts were registered during 2018. This includes mainly recent transplants, but also some transplants performed during earlier years.

Allografts



Autografts



Graphs: Logarithmic scale.

Figure 1. Data Manager Training in Lisbon

POSITIVE QUALIFICATION OPINION - EMA

In the summer of 2018, EMA issued the first two marketing authorisations for CAR T-cells medicines in the European Union. Among the conditions imposed by EMA is the utilisation of a patient registry as part of the risk management measures for these two new medicines in order to monitor long-term safety and efficacy.

The EBMT had already formally engaged with the European Medicines Agency (EMA) regarding the EBMT Registry and this led to the publication by EMA in July 2018 of their draft positive qualification opinion on the cellular therapy module of the EBMT Registry for public consultation. The qualification opinion was released on February 28, 2019 (see page 44). The publication of the draft EMA opinion has driven a sharp increase in interest among pharmaceutical companies in collaborating with EBMT to collect long-term safety data for Chimeric Antigen Receptor (CAR) T-cell therapy and Immune Effector Cells.

EBMT will effectively be a key collaborator in rolling out these novel therapies by supporting post-authorisation follow-up and supplying data to facilitate risk-benefit evaluations.

CELL THERAPY

During the last year, the EBMT has further worked on the Cell Therapy Med-A, resulting in the creation of a new Cellular and Gene Therapy Form. This will incorporate the remaining "must have" items required by EMA.

The new form is being implemented in MACRO, the upcoming new registry system that will go live in 2019. The data that have been registered using the current Cell Therapy Med-A form in ProMISe will be migrated to MACRO during the normal data migration process, and the transferred registrations can then be completed using the new Cellular and Gene Therapy Form.

Competent authorities will require manufacturers to perform long-term (15 years) follow-up of recipients which implies collecting significant amounts of clinical data. With this requirement in mind, the EBMT has been working with commercial CAR-T manufacturers to review and validate the Cell Therapy form. This form is expected to be fully implemented shortly. CAR T teams and clinical departments will be key to collecting the data on their patients, both at the time of treatment and subsequent follow-up.

PROJECT 2020: REGISTRY UPGRADE

As announced in the last report, Elsevier was appointed in February 2017 to work on the new registry system (MACRO). During 2018, comprehensive testing of Data Entry continued, alongside the initial testing of the new Data Retrieval function, following further customised development for EBMT. This led to some delays in the project timeline that had been planned.

In addition to EBMT staff, the pool of beta testers was increased and a number of National Registry staff and Centre data managers volunteered their time and gave important feedback. We are very grateful for their support.

END USER ENGAGEMENT

During 2018, presentations took place at the BSBMT Data Manager Training Days (UK) and at the EBMT Annual Meeting in Lisbon. During the EBMT congress we also provided taster sessions using MACRO in the computer room.

TRAINING

A Train the Trainer session took place in February 2019 in London, primarily aimed at National Registry staff who will be providing training to their centres, with support from the EBMT Registry. Group training sessions are already planned in Turkey, Spain and at the EBMT Annual Meeting in March 2019. Further regional sessions are being planned by other National Registries, and others will be planned for those centres outside of National Registries. Suggestions for host venues will be very welcome

TIMELINE

We aim to go live as soon as possible after the centre training has started taking place, after the EBMT Annual Meeting in March. We will keep members informed of the next steps. We look forward to working together on the new Registry in 2019.

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

Carmen Ruiz de Elvira
Head of the EBMT Registry

EMA's qualification opinion on Cellular therapy module of the EBMT Registry

ON FEBRUARY 28, 2019, THE EBMT RECEIVED A REGULATORY QUALIFICATION FROM THE EUROPEAN MEDICINE AGENCY (EMA) ON THE USE OF ITS PATIENT REGISTRY TO SUPPORT NOVEL CAR T-CELL THERAPIES.

The EBMT Registry has been qualified by EMA as a suitable platform for the collection of data for post-authorisation safety studies. The registry is now considered suitable to perform pharmacoepidemiological studies for regulatory purposes, concerning Chimeric Antigen Receptor (CAR) T-cell therapy used in the treatment of haematological malignancies.

CAR T-cell immunotherapies are advanced therapy medicinal products (ATMPs) manufactured from human primary living cells procured by hospitals or blood banks. While clinical results of CART-cell products so far have been impressive, these highly personalised medicines can also have substantial adverse effects leading to severe complications in patients including death. Furthermore, some ATMPs may be considered as a once-in-a-lifetime treatment and long-term follow-up is needed to demonstrate the sustainability of efficacy. This is where registries such as EBMT become essential actors with a proven long-term follow-up of large cohorts of patients undergoing complex therapeutic procedures such as haematopoietic cell transplantation and immune effector cells.

The EBMT developed a specific Cellular and Gene Therapy Form to standardise registration of patients treated with cellular therapies such as CAR T-cells in Europe. Furthermore EBMT contributed to the EMA-facilitated consensus with regulators and industry on establishing the minimal dataset needed for long-term surveillance of patients receiving CAR T-cells.

The qualification opinion came as a result of extensive interaction with EMA starting in late 2016 when the EBMT first responded to the **EMA's Patient Registry Initiative** and subsequently in the formal qualification opinion request process.

The EMA recognises the value of its registry and this recognition will lead to improved communication among the various stakeholders, including registry owners, regulators and marketing authorisation holders, giving confidence to users on the data collected and ultimately bring safe and effective therapies to the patients.

The EBMT acknowledges the important contributions of EBMT volunteers to this achievement: Chiara Bonini, Christian Chabannon, Jürgen Kuball, with the support of EBMT staff including Eoin McGrath, Carmen Ruiz de Elvira and Anja van Biezen.

The *full qualification opinion report* is available on **www.ebmt.org** and **www.ema.europa.eu**

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EDUCATION

EDUCATION

EBMT 2018
44TH ANNUAL MEETING
OF THE EUROPEAN SOCIETY FOR BLOOD AND MARROW TRANSPLATION

EBMT
European Society
for Blood and Marrow Transplantation

18 - 21 March 2018
Lisbon, Portugal

- **44th** Meeting of the Physicians
- **34th** Meeting of the Nurses Group
- **17th** Meeting of the Data Management Group
- **12th** Patient, Family & Donor Day
- **10th** Meeting of the Quality Management Group
- **7th** Cell Therapy Day
- **7th** Paediatric Day
- **3rd** Pharmacist Day
- **2nd** Psy Day

Attendance

5,261 delegates from **87** countries

Programme

1,213
abstracts submitted

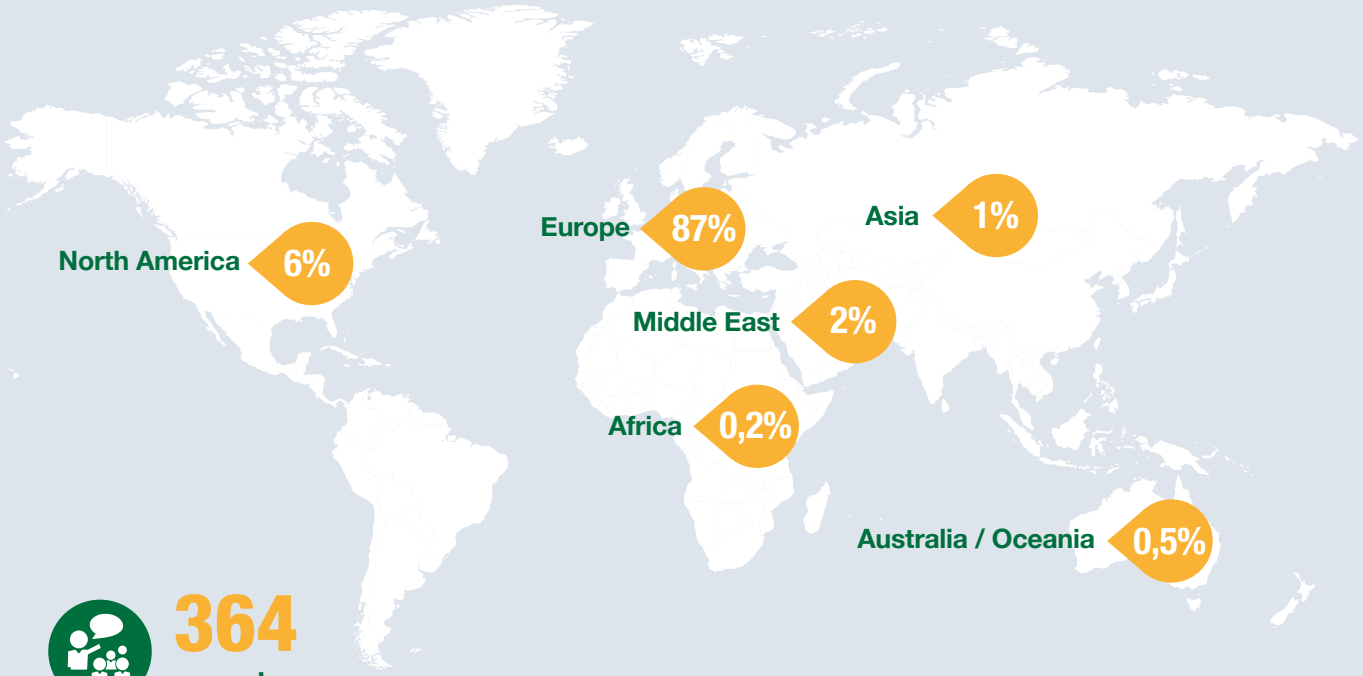
192
oral presentations

971
posters presented

171
sessions

15
awards

Continent of origin of the speakers:



364
speakers

Sponsoring and Exhibition

24
companies, associations or groups supported EBMT 2018

33
sponsored sessions (industry symposium, industrial theatre, How Do I...?)

51
exhibiting organisations in over **1,170** m2 exhibition area

Digital Activities

#EBMT18

945 people sent **5,312** tweets to the Twitter wall generating **20,411,265** impressions and reaching **13,495,218** people

7,191 page views of the EBMT TV online (WebsEdge and YouTube)

3,627 mobile app downloads

Networking Activities

850
delegates attended the opening session and welcome reception

600
delegates attended the networking event in the Patio da Gale

Delegates gave the Annual Meeting a rating of **8** out of **10**
(EBMT 18 delegates' survey - 1,856 respondents)

Awards

VAN BEKKUM AWARD

The Van Bekkum Award for the best abstract submitted to the physician's programme, sponsored by EBMT, was presented to Richard K. Burt (United States) for the abstract entitled: ***NON-MYELOABLATIVE HAEMATOPOIETIC STEM CELL TRANSPLANTATION VERSUS CONTINUED DISEASE MODIFYING THERAPIES (DMT) IN PATIENTS WITH HIGHLY ACTIVE RELAPSING REMITTING MULTIPLE SCLEROSIS (RRMS)***

BASIC SCIENCE AWARD

The Basic Science Award, sponsored by Chimeric Antigen Receptors for Advanced Therapies (CARAT), was presented to Fermín Sánchez-Guijo (Spain) for the abstract entitled: ***THE INCORPORATION OF EXTRACELLULAR VESICLES FROM MESENCHYMAL STROMAL CELLS INTO HEMATOPOETIC PROGENITORS INCREASE THEIR CLONOGENIC CAPACITY AND THEIR ENGRAFTMENT ABILITY***

HONORARY MEMBERSHIP

Honorary Membership awarded to Enric Carreras (Spain), Dietrich Niethammer (Germany) (represented by Peter Bader) and Ray Powles (United Kingdom)

CLINICAL ACHIEVEMENTS

The Clinical Achievements Award awarded to Boris Afanasiev (Russian Federation)

JIAN-JIAN LUAN AWARD

The Jian-Jian Luan Award for Lymphoma Transplant Research, sponsored by EBMT, was presented to Anne-Claire Mamez (Switzerland) for the abstract entitled: ***ALLOGENEIC STEM CELL TRANSPLANTATION FOR PERIPHERAL T-CELL LYMPHOMAS: A STUDY OF 284 PATIENTS FROM THE SOCIETE FRANCOPHONE DE GREFFE DE MOELLE ET DE THERAPIE CELLULAIRE***

JON VAN ROOD AWARD

The Jon van Rood Award for the best paper in the immunobiology of allogeneic hematopoietic transplantation, sponsored by EBMT, was presented to Antonio Galleu (United Kingdom) for the paper entitled: ***APOPTOSIS IN MESENCHYMAL STROMAL CELLS INDUCES IN VIVO RECIPIENT-MEDIATED IMMUNOMODULATION*** and to Enrico Velardi (United States) for the paper entitled: ***PRODUCTION OF BMP4 BY ENDOTHELIAL CELLS IS CRUCIAL FOR ENDOGENOUS THYMIC REGENERATION***

LIFETIME ACHIEVEMENT AWARD IN ACUTE LEUKAEMIA

The EBMT Lifetime Achievement Award in Acute Leukaemia awarded to Eliane Gluckman

BEST YOUNG ABSTRACTS

The Best Young Abstract Awards for the best abstracts submitted for Oral and Poster presentations, sponsored by the EBMT, were presented to ten young investigators:

1. *Mattia Algeri (Italy)*
2. *Jon Badiola Gonzalez (Spain)*
3. *Nico Gagelmann (Germany)*
4. *Federica Galaverna (Italy)*
5. *Mojibade Hassan (United States)*
6. *Hardikkumar Jetani (Germany)*
7. *Nayoun Kim (Korea, Republic of)*
8. *Marthe C.J. Roex (The Netherlands)*
9. *Serena Scala (Italy)*
10. *Anke Verlinden (Belgium)*

BEST CHINESE ORAL & POSTER ABSTRACTS

The Best Chinese oral abstract Award was presented to Honghu Li (China) for the abstract entitled: *PRIMITIVE NEUTROPHILS DERIVED IL6 POSITIVELY REGULATES EMBRYONIC HEMATOPOIETIC STEM CELL EMERGENCE*

The Best Chinese poster abstract Award was presented to Zhao Wu (China) for the abstract entitled: *POTENT ANTI-LEUKEMIA ACTIVITIES OF HUMANIZED CHIMERIC ANTIGEN RECEPTOR MODIFIED T(CAR-T) CELL THERAPY IN CHINESE PATIENTS WITH RELAPSED/ REFRACTORY ACUTE LYMPHOBLAST LEUKEMIA*

BEST LATIN AMERICAN ABSTRACTS

The Best Latin American Abstract Award was presented to Carmem Bonfim (Brazil) for the abstract entitled: *EXCELLENT OUTCOME FOR 91 FANCONI ANEMIA PATIENTS UNDERGOING MATCHED RELATED TRANSPLANTS USING CYCLOPHOSPHAMIDE 60MG/KG IN CURITIBA, BRAZIL* and to Monica Magdalena Rivera Franco (Mexico) for the abstract entitled: *DONOR AND RECIPIENT HYPERCHOLESTEROLEMIA BEFORE ALLOGENEIC STEM CELL TRANSPLANTATION ASSOCIATES WITH INCREASED INCIDENCE OF ACUTE GRAFT-VERSUS-HOST DISEASE*

NATURE PUBLISHING POSTER AWARDS

The Best Clinical Poster Award, sponsored by Nature Publishing Group, was presented to Britta Eiz-Vesper (Germany) for her poster #A129 entitled: *VIRUS-SPECIFIC T CELLS FROM STEM CELL, FAMILY AND THIRD PARTY T CELL DONORS: PATIENT MONITORING, DONOR SELECTION AND GMP-COMPLIANT MANUFACTURING*

The Best Science Poster Award, sponsored by Nature Publishing Group, was presented to Lars Wallstabe (Germany) for his poster #A094 entitled: *CAR-T CELLS TARGETING αVβ3 INTEGRIN CONFER COMPLETE REMISSION OF EPITHELIAL CANCERS IN PRE-CLINICAL MODELS IN VIVO*

NURSES GROUP AWARDS

Distinguished Merit Award to Jacqui Stringer (United Kingdom)

Best research abstract oral presentation to Jantina Kortleve-Kadijk (The Netherlands) for the abstract entitled: *A CRITICAL APPRAISED TOPIC (CAT): WHAT IS THE NEED FOR HYDRATION AFTER AUTOLOGOUS STEM CELL TRANSPLANTATION?*

Best oral presentation to Joanne Ellis (United Kingdom) for the abstract *ANALYSIS OF PARENT, STAFF AND WARD EXPERIENCE FOLLOWING THE IMPLEMENTATION OF A ROBUST, VALIDATED TEACHING TOOL FOR PARENTS OF CHILDREN WHO REQUIRE (HOME) PARENTERAL NUTRITION*

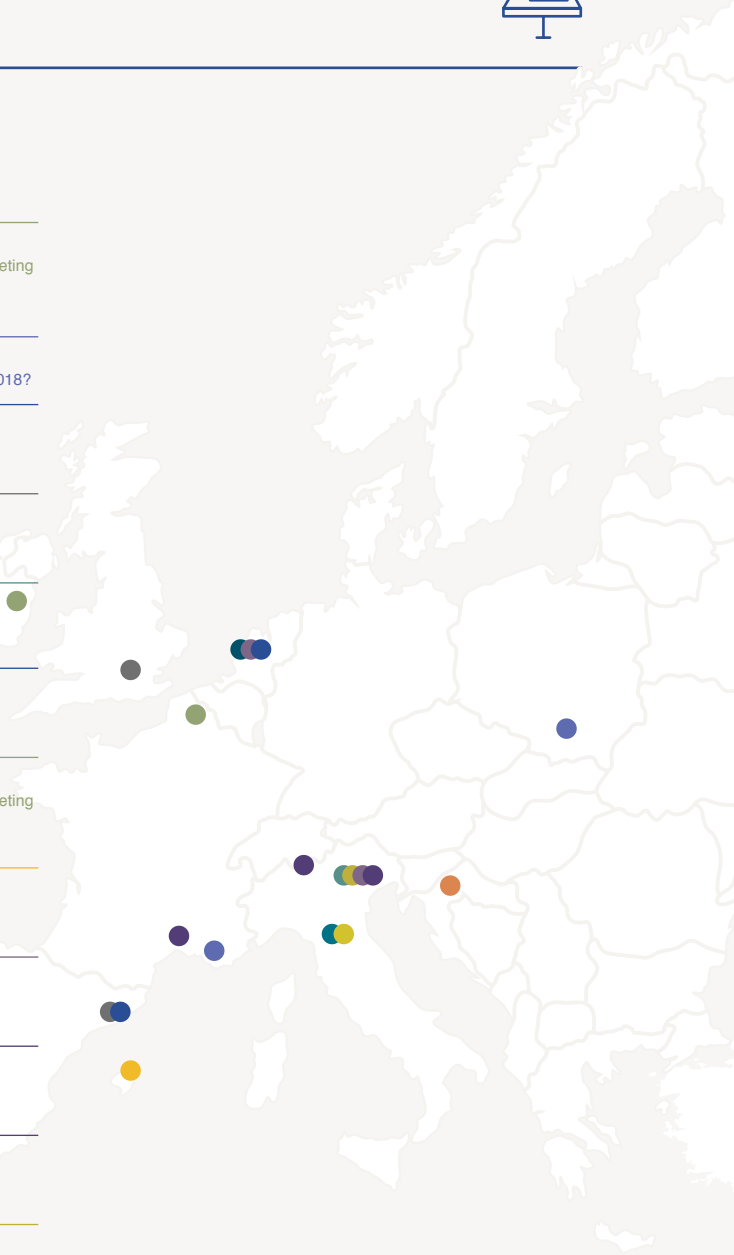
Best paediatric oral presentation to Shohei Nakajima (Japan) for the abstract entitled: *PREDICTORS OF PARENTAL PSYCHOLOGICAL DISTRESS DURING THE ACUTE PHASE OF PEDIATRIC HEMATOPOIETIC STEM CELL TRANSPLANTATION IN JAPAN: A MULTICENTER PROSPECTIVE LONGITUDINAL STUDY*

Best poster to Teresa Solano (Spain) for the poster entitled: *IMPLEMENTATION OF LATE-EFFECTS FOLLOW-UP NURSE CONSULTATION FOR ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION RECIPIENTS: HIGH INCIDENCE OF NUTRITIONAL, PSYCHOLOGICAL AND SEXUAL DYSFUNCTIONS*

Best paediatric poster to Victoria Cassels (United Kingdom) for the poster entitled: *STANDARDISING DISCHARGE INFORMATION FOR NURSES WORKING ON A PAEDIATRIC STEM CELL TRANSPLANT WARD*

Educational events 2018

Leiden, The Netherlands 18-20 January 2018 150 attendees	**CTIWP** 2nd Cellular Therapy & Immunobiology Scientific Symposium
Dublin, Ireland 26-27 January 2018	**CMWP** Chronic Malignancies Working Party Business & Educational Meeting Educational symposium on "Perspectives in the treatment of myeloma & plasma cell disorders"
Marseille, France 20-22 April 2018	**ALWP** Advances in Allogeneic Immunotherapy: where do we stand in 2018?
Leiden, The Netherlands 22-25 May 2018 21 attendees	**Statistical** 6th Edition EBMT Statistics Course
Barcelona, Spain 24-25 May 2018 29 attendees	**JACIE** Inspector Training Course
Verona, Italy 7-9 June 2018 178 attendees	**PDWP / IDWP / IEWP / NG** 11th Paediatric Diseases, Infectious Diseases, Inborn Errors Parties and 6th Paediatric Nurses Group Meeting
Barcelona, Spain 7-9 September 2018 240 attendees	**EBMT** 3rd EBMT International Transplant Course
Lille, France 14-15 September 2018	**CMWP** Chronic Malignancies Working Party Business & Educational Meeting Educational symposium on "CAR T Cell Therapy in Chronic Haematological Malignancies"
Palma de Mallorca, Spain 26-28 September 2018 92 attendees	**LWP** 14th Edition of the Lymphoma Working Party Educational Course
Leiden, The Netherlands 28-30 September 2018 158 attendees	**IEWP** 4th Inborn Errors Working Party Annual Conference
Montpellier, France 4-5 October 2018 71 attendees	**NG** 10th EBMT Nurses International Study Day 2nd Nurses Research Study Day
London, UK 12 October 2018 Therapy	**NG** Emerging Therapies - Let's focus on CAR T Cell
Stockholm, Sweden 25-27 October 2018 50 attendees	**IDWP** 21st Educational Course of the Infectious Diseases Working Party
London, UK 8-9 November 2018 27 attendees	**JACIE** Inspector Training Course
Zagreb, Croatia 8-10 November 2018 195 attendees	**TCWP** 4th International cGvHD Symposium & Transplant Complications Working Party Educational Meeting
Bellinzona, Italy 15 November 2018	**NG** Swiss Nurses Working Group Study Day
Florence, Italy 15-17 November 2018 151 attendees	**ADWP / SAAWP** Joint Educational Meeting of the Autoimmune Diseases and Aplastic Anaemia Working Parties
Krakow, Poland 16-17 November 2018 95 attendees	**ALWP** Acute Leukaemia Working Party Scientific Meeting and Educational Symposium. "Advances in pre and post-transplant management of acute lymphoblastic leukaemia"
Barcelona, Spain 22-23 November 28 attendees	**JACIE** Inspector Training Course
Mumbai, India December 2018	**NG** 2nd training course for HSCT Nurses



- **ALWP:** Acute Leukemia Working Party
- **IEWP:** Inborn Errors Working Party
- **TCWP:** Transplant Complications Working Party
- **LWP:** Lymphoma Working Party
- **SAAWP:** Severe Aplastic Anaemia Working Party
- **IDWP:** Infectious Diseases Working Party
- **CMWP:** Chronic Malignancies Working Party
- **PDWP:** Paediatric Diseases Working Party
- **ADWP:** Autoimmune Diseases Working Party
- **CTIWP:** Cellular Therapy and Immunobiology Working Party
- **NG:** Nurses Group
- **JACIE**

The new EBMT Handbook

The EBMT Handbook Hematopoietic Stem Cell Transplantation and Cellular Therapies was released in a new format in January 2019. The project started in 2017 and was coordinated by Enric Carreras, one of the editors together with Carlo Dufour, Mohamad Mohty and Nicolaus Kröger.

This new formatted EBMT Handbook which follows the long tradition of the ESH-EBMT Handbook, addresses the latest developments and innovations in stem cell transplantation and cellular therapy. In 93 chapters written by 175 leading experts in the field, the book covers all types of stem cell and bone marrow transplantation, including haplo-identical stem cell and cord blood transplantation, but also indications for transplantation, the management of early and late complications as well as the new and rapidly evolving field of cellular therapies. Other important issues such as quality management and JACIE accreditation, stem cell collection, conditioning, donor selection, HLA typing, graft manipulation, ethical issues, psychological support, and quality of life are also properly addressed.

This book provides an unparalleled description of current practices to enhance readers' knowledge and practice skills.

The book is downloadable in PDF and all participants of the EBMT 45th Annual Meeting will be able to get a free-hard copy.

The open-access and print edition (Springer Open) have been possible thanks to the generous unrestricted educational grant from the Fondation José Carreras pour la lutte contre la leucémie, Genève.

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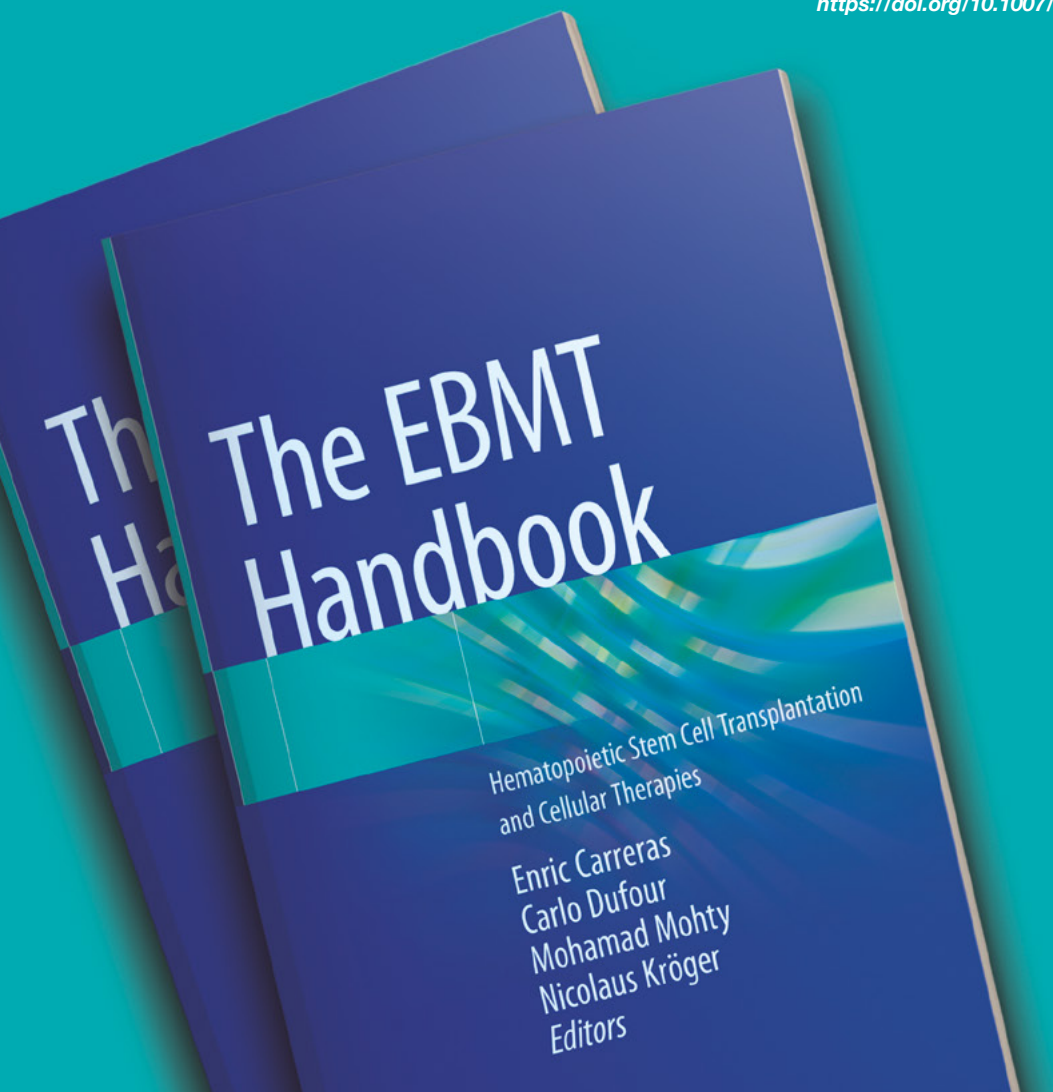


PATIENT CARE

EBMT Nurses Group

Haematology and HSCT Nursing

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.

EDUCATION

The nurses and allied health care professions programme at EBMT 2018 in Lisbon was a great success from the first session on Sunday 18th through to Wednesday 21st March and was our 34th nurses meeting. Over the 4 days of congress there were many stimulating and inspiring sessions, covering the many complications our patients face from diagnosis to their initial treatment and through and beyond the transplant process.

This year we had 2 satellite symposiums throughout the conference, supported by Jazz Pharmaceuticals and Mallinckrodt that were extremely well attended and gave good feedback.

Grants for 2018 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 2018 were, M. Jafr, who received the educational grant, C. Lim, travel grant recipient, O. Niculita, the grant to attend the annual paediatric training day, C. Locca, the exchange grant winner and M. Achrekar was our outreach nurse from Mumbai India who attended the Annual Meeting. All have supplied fantastic feedback either within the EBMT NG newsletter or by oral presentation.

PAEDIATRIC TRAINING DAYS

The 6th Paediatricians & Paediatric Nurses training course on HSCT in Children and Adolescents was held from 7th to 9th June in Verona, Italy, and was a great success with nurses across Europe attending. Details are within the PDWP update.

INTERNATIONAL NURSES TRAINING DAY AND SECOND RESEARCH NURSES DAY

Annually EBMT NG provides an autumn education meeting. 2018 saw the 10th training day and the 2nd research day. This year we were hosted in Montpellier and had a fantastic reception with a great turn out of local nurses. The 10th training day saw 71 nurses from across Europe join together to learn. As usual we offer a wide spread of topics, with talks on MDS, DLI, GvHD and care of the young adult, a cornucopia on offer that was a stimulating and thought provoking programme.

The second day saw the return of the research day, a new idea for 2017 that we repeated this year and has now become a permanent day in the calendar. There were 42 attendees with presentations 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.

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. We have 2 current vacancies. The nurses and their respective working parties are:

1. Infectious diseases WP - **Iris Agreiter** (Italy)
2. Lymphoma WP - **Erik Aerts** (Switzerland)
3. Acute leukaemia WP - **Elisabeth Wallhult** (Sweden)
4. Paediatric diseases WP - **Eugenia Trigoso Arjona** (Spain)
5. Severe Aplastic Anaemia WP – vacancy advertised
6. Autoimmune Diseases WP - **Helen Jessop** (UK)
7. Transplant Complications WP - **Corien Eeltink** (Netherlands)
8. Cellular therapy & Immunobiology WP – **Rose Ellard** (UK)
9. Chronic malignancy WP - vacancy advertised
10. Inborn errors WP – **Hilda Mekelenkamp** (Netherlands)

GLOBAL EDUCATIONAL COMMITTEE

The Global Educational Committee 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 14th and 15th December 2018 the GEC visited the ACTREC hospital in Mumbai, India to give a series of presentations focussed on JACIE Nursing specific standards. Approx. 220 nurses attended this educational program. Feedback was once again excellent and will be published in 2019.

Figure 1. 2nd training course for HSCT Nurses, India

NURSING RESEARCH COMMITTEE

We have had two publications this year in *Bone Marrow Transplantation* and there are several projects currently recruiting or about to be launched by the EBMT nurses research group.

S-FAST Collaboration with TCWP – Ethical approval has been given for several European countries and data collection continues.

Nutritional Surveys Collaboration with TCWP – Manuscript is currently being prepared and is due for submission spring 2019.

Survey on protective environment in HSCT settings collaboration with IDWP –Questionnaire designed due for release spring 2019.

CVC study on preferred insertion sites collaboration with IDWP – Study open and enrolling.

ATG survey – Looking at centre specific practices when treating patients with ATG, survey open and recruiting.

Infection Control questionnaire for LMIC; EBMT /WHO collaboration – Data collected and analysed, manuscript being written. An abstract has been written and accepted for Frankfurt annual conference.

Cutaneous cGvHD QoL questionnaire – Assess current position with regard to care of patient's skin that has chronic GvHD and the impact of cutaneous cGvHD on morbidity / QoL in adult and paediatric population, study in development.

NG Paediatric committee Collaboration with PDWP – Safe Transfer from SCT unit to ICU: your experience, data has been collected and this is now closed and will be presented in Frankfurt with the PDWP.

Related donor care manuscript has been written and is currently under review.

Specialized Paediatric Palliative Care Services in European Paediatric Hematopoietic Stem Cell Transplant Centres, data has been collected and will be presented in Frankfurt.

Nurse competencies and patient outcomes in India Global Education Committee project – Data collected and currently being analysed.

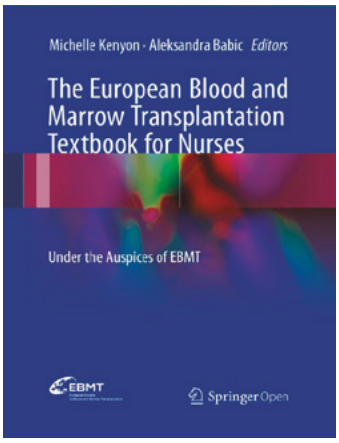
PUBLICATIONS

1. Health-care professionals' perspective on discussing sexual issues in adult patients after haematopoietic cell transplantation. Eeltink CM, *Bone Marrow Transplant*. 2018 Mar.

2. Variability of nutritional practices in peritransplant period after allogeneic hematopoietic stem cell transplantation: a survey by the Complications and Quality of Life Working Party of the EBMT. Peric Z, *Bone Marrow Transplant*. 2018 Aug.

THE TEXTBOOK FOR NURSES

RELEASED: MARCH 2018



The EBMT nurses Group launched this ground breaking book in March 2018, the first of its kind. There have been approx. 150,000 downloads. This unique, comprehensive publication informs and guides readers through the myriad of difficulties associated with HSCT. This textbook, for adult and paediatric nurses paves a journey through the history of transplant nursing, including essential and progressive elements to help nurses improve their knowledge and benefit the patient experience, as well as a comprehensive introduction to research and auditing methods. A must have for all levels of HSCT nurse and other professionals specialising or wishing to specialise in this area.

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 2018 that this vibrant team is moving the nursing agenda forward and improving patient care.

John Murray
President EBMT Nurses Group

Standards and Accreditation - JACIE

JACIE
Joint Accreditation Committee
ISCT | EBMT

The Joint Accreditation Committee ISCT & 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, **434 transplant programmes and facilities in 33 countries** in Europe and beyond have applied to JACIE and **665 inspections** (first-time and reaccreditation) have been performed. **320 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 cellular therapy field.

2018 stands out for 2 significant events. The first joint JACIE-FACT inspection in Latin America was performed in November in Córdona, Argentina. A further 11 centres from the region have presented themselves to be accredited during 2019-2020. This is the first region in which the StepWise approach to accreditation is being tested. Secondly, several centres in England achieved accreditation for immune effector cell administration in conjunction with NHS England as part of their CAR-T cell therapy roll-out strategy. Both of these special initiatives impacted on the JACIE's applications, inspections and accreditations activities in 2018.

APPLICATIONS 2018

75 APPLICATIONS

(30 first-time and 45 reaccreditation) received

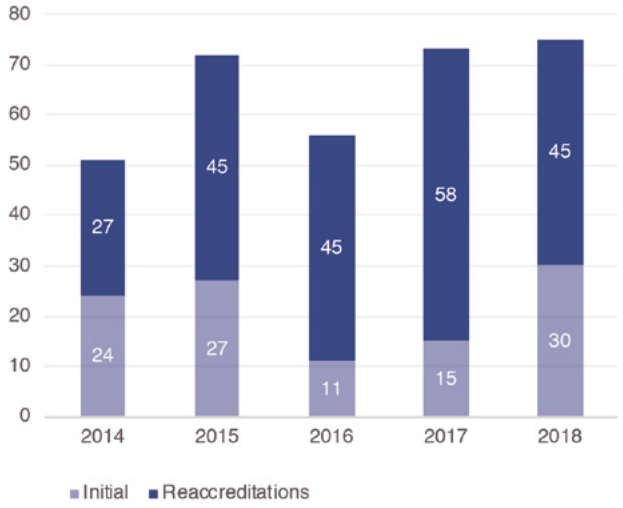


Figure 1. Number of applications received for the past five years

INSPECTIONS 2018

78 INSPECTIONS

(19 first-time and 59 reaccreditation)

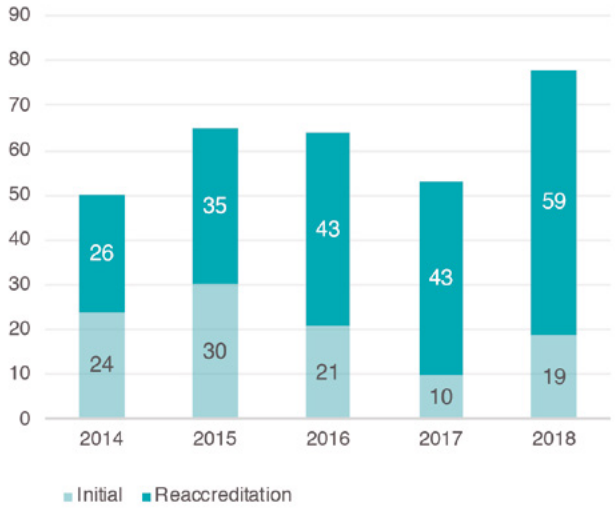


Figure 2. Number of inspections performed for the past five years

ACCREDITATIONS 2018

51 ACCREDITATIONS

(9 first-time and 42 reaccreditation) awarded

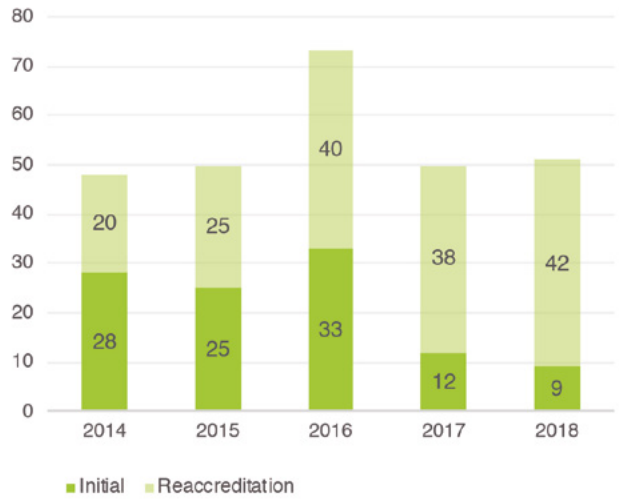


Figure 3. Number of accreditations awarded for the past five years

EDUCATIONAL EVENTS

Three JACIE training courses were held during 2018 - two in Barcelona, (Spain) and another in London (United Kingdom) with 60 participants in total. Furthermore, a joint JACIE-FACT workshop was held at the AfBMT congress in Casablanca, Morocco. Finally, JACIE was represented at the International Congress on Stem Cell and Regenerative Medicine in Tehran, Iran.

7TH EDITION OF FACT-JACIE STANDARDS

The 7th edition of the FACT-JACIE Standards was published on 1 March 2018. This edition featured Immune Effector Cells, first developed in edition 6.1. These particular standards have sparked a lot of interest given the recent upsurge in CAR-T cellular therapy activity.

IMMUNE EFFECTOR CELLS (IEC)

In early 2018, the National Health Service of England incorporated JACIE accreditation for IEC administration into its criteria for designation of centres to administer CAR-T cell therapy. JACIE performed focussed inspections of 8 centres using a bespoke checklist for IECs between September and October with all of those centres achieving accreditation. Sincere thanks to all of the inspectors for their invaluable contribution to the success of this project.

Figure 1. Participants at the JACIE Inspector Training Course in London, UK.

JACIE EVENTS 2018

Figure 2. JACIE Information Day in Antalya, Turkey

OTHER

The JACIE website has now been fully incorporated into the new EBMT website. The JACIE Twitter account @JACIE_EBMT has grown to 921 followers.

We would like to express our 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 2018

Medical University of Vienna, Vienna, Austria; Cliniques Universitaires St Luc, Brussels, Belgium; Jessa Ziekenhuis, campus Virga Jesse, Hasselt, Belgium; AZ Turnnhout vzw, Campus Sint-Elisabeth, Turnnhout, Belgium; University Hospital Centre Zagreb, Zagreb, Croatia; Centre Hospitalier Lyon Sud (CHLS), Lyon, France; Institut d'Hématologie et d'Oncologie Pédiatrique, IHOPe - Lyon, Lyon, France; Hôpitaux Universitaires Pitié Salpêtrière - Charles - Foix, Paris, France; Institut Gustave Roussy, Villejuif, France; Institut Gustave Roussy, Villejuif, France; Evangelisches Krankenhaus Essen-Werden, Essen, Germany; St Franziskus Hospital, Medizinische Klinik I, Flensburg, Germany; Univ. Hospital Regensburg, HSCT, Regensburg, Germany; University Medical Center Würzburg, Würzburg, Germany; University Medical Center Würzburg, Würzburg, Germany; MEDYAG Kft., Debrecen, Hungary; Azienda Ospedaliera Brotzu, Cagliari, Italy; Aorn Santobono Pausilipon, Napoli, Italy; Sanquin Research, Amsterdam, Netherlands; The Netherlands Cancer Institute-Antoni van Leeuwenhoek Hospital (NKI-AVL) Antoni van Leeuwenhoek Hospital, Amsterdam, Netherlands; Maastricht University Medical Center, Maastricht, Netherlands; University Medical Center Utrecht, Utrecht, Netherlands; University Medical Center Utrecht, Utrecht, Netherlands; Hospital Duran i Reynals, Institut Català d´Oncologia, Barcelona, Spain; Hospital Universitari Vall d´Hebron, Barcelona, Spain; Hospital Universitario Morales Meseguer, Murcia, Spain; Skane University Hospital, Lund, Sweden; University Hospital Berne Paediatric Hematology / Oncology, Berne, Switzerland; Hôpitaux Universitaires de Genève, Geneva, Switzerland; Birmingham Women's and Children's NHS Foundation Trust, Birmingham, United Kingdom; Queen Elizabeth Hospital, Birmingham, United Kingdom; Blackpool Victoria Hospital, Blackpool, United Kingdom; Bristol Royal Hospital for Children, Bristol, United Kingdom; Bristol Royal Hospital for Children, Bristol, United Kingdom; Royal Devon and Exeter NHS Foundation Trust, Exeter, United Kingdom; Hull and East Yorkshire Hospitals NHS Trust, Hull, United Kingdom; Great Ormond Street Hospital, London, United Kingdom; Great Ormond Street Hospital, London, United Kingdom; Imperial College Healthcare NHS Trust, London, United Kingdom; King's College Hospital NHS Foundation Trust, London, United Kingdom; London Bridge Hospital, London, United Kingdom; University College London Hospitals NHS Foundation Trust, London, United Kingdom; Manchester University Hospitals NHS Foundation Trust, Manchester, United Kingdom; Manchester University Hospitals NHS Foundation Trust, Manchester, United Kingdom; The Christie NHS Foundation Trust, The Christie Clinic and The Christie NHS Foundation Trust, Manchester, United Kingdom; Norfolk and Norwich University Hospital NHS Foundation Trust, Norwich, United Kingdom; Nottingham University Hospitals NHS Trust, Nottingham, United Kingdom; Nottingham University Hospitals NHS Trust, Nottingham, United Kingdom; Poole Hospital NHS Foundation Trust, Poole, United Kingdom; NHS Blood and Transplant, Southampton, United Kingdom; The Wessex Blood and Marrow Transplant Program, Southampton, United Kingdom;

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

Financial Report and Highlighs 2018

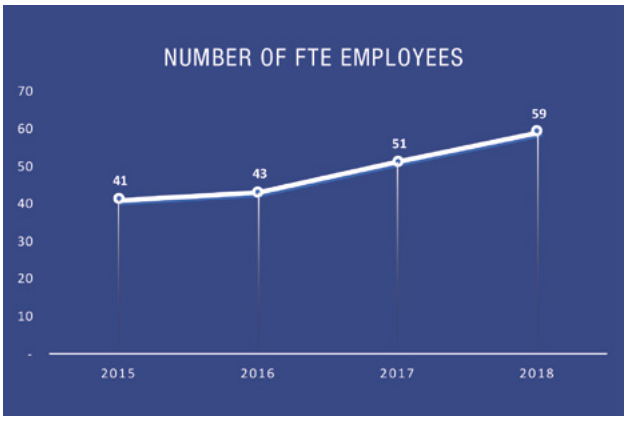
Jürgen Kuball
EBMT Treasurer

For the 4th consecutive time, EBMT has obtained an "unqualified opinion", this demonstrates that EBMT is maintaining its high standard of modern management, improving its financial stability and provides assurance that money is being spent and allocated according to our mission.

EBMT HAS CLOSED FOR A 5TH CONSECUTIVE YEAR WITH A POSITIVE RESULT AND CONTINUES TO INVEST IN ITS STRATEGIC GOALS IN LINE WITH ITS MISSION.

During the last four years as EBMT Treasurer, the EBMT Board substantially changed the financial model, which allowed the creation of a continuous reserve of income for strategic projects. This permitted a one-third increase in staff positions but also augmented financial obligations (Figure 1). Therefore, at the very same time we increased our reserves from 2,267 k€ in 2014 to 5,260 k€ in 2018 to cover salary costs in case of any unexpected event.

(A)



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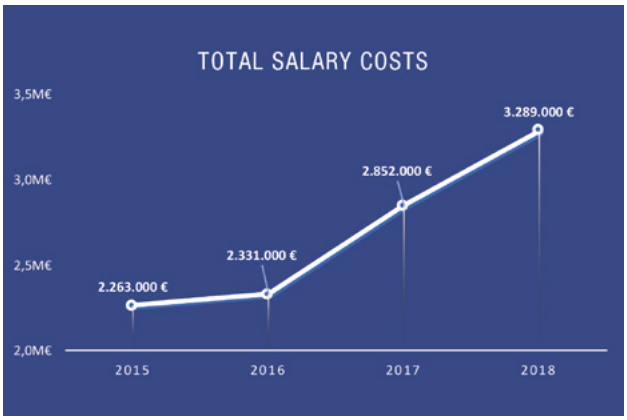

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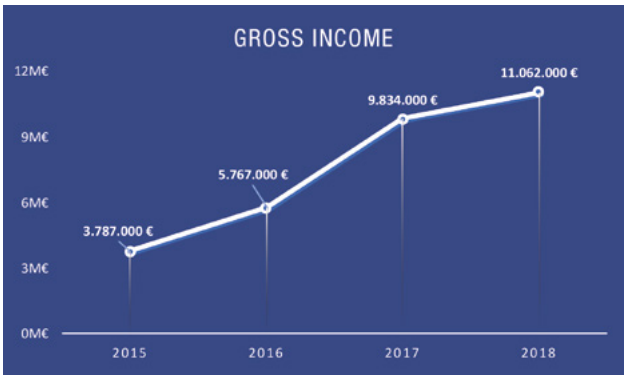

Figure 1. Growth in (A) FTE Employees (B) Salary costs (C) Income

SPENDING OUR FINANCIAL RESOURCES ON OUR MISSIONS

Our increasing annual income has been mainly used to further work on our mission as well as to improve the executive structure of EBMT in order to keep pace with the growing organisation. The budget needed for management (i.e. board and executive office expenses), therefore, slightly increased from 8% to 10%, still resulting in an allocation of 90% to our main missions (i.e. studies, Registry, accreditation and education, including the Annual Meeting) (Figure 2 Financial highlights 2018). Thereby, EBMT remains a very efficient organisation when assessed by international rankings (A/A+ in line with CharityWatch).

DEVELOPING A WELL-BALANCED STRATEGIC FINANCIAL ROADMAP FOR THE FUTURE

In 2018, the main challenge was to develop a well-balanced financial roadmap for the next four years in order to be able to cover all financial challenges in line with our ambitions but also to secure a strong backbone. To cover future financial challenges, we earmarked 300 k€ per year for the last few years. The EBMT Board decided to partially release such allocated reserves in 2019 (1,153 k€) to support our roadmap for the next few years which covers the following strategic topics:

1. IMPLEMENTING THE NEW EBMT REGISTRY (MACRO)

This project will be closed in 2019 and has the highest financial priority. Reserves have been earmarked to cover all expected costs. Also, additional reserves are being held for any unexpected expenses.

2. INTRODUCING THE "BENCHMARKING" CONCEPT

We have increased the JACIE fees to cover the "benchmarking project", which will allow centres to know how their own clinical transplantation outcomes compare to the rest of Europe. For the very first time, this will give back the most valuable information to each reporting centre: How is the quality of our daily clinical care and can we learn from over-performers to improve clinical outcomes of all centres?

3. JACIE AND LOW TO MIDDLE-INCOME COUNTRIES

The Board decided to further reach out to countries outside Europe to implement JACIE as a quality system for transplantation centres. We will, therefore, financially support this initiative.

4. IMPLEMENTING THE CELLULAR THERAPY PROJECT AND PASS STUDIES

In receiving the qualification opinion from EMA on the cellular therapy module of the EBMT Registry in February 2019 (see page 44), the board decided to strengthen manpower further to support this initiative, which will collect post-market evidence for safety and efficacy of different commercial CAR T products. In addition, this registry tool will also be helpful for all researchers who are executing clinical studies with CAR T and other advanced therapy medicinal products (ATMPs) in order to gather more comprehensive data sets on efficacy and safety in this very exciting field.

5. INVESTING IN IT AND HUMAN RESOURCES TO ALLOW PROPER MANAGEMENT OF THE ORGANISATION

With a growing organisation, we need to increase the managerial infrastructure and modernise software tools in order to further strengthen the efficiency of the EBMT.

The Board decided to recruit a medical officer who will be dedicated to supporting all medical aspects within the organisation; this has been mainly handled by volunteers until now.

6. CONSOLIDATING THE EVENTS AND EDUCATIONAL UNIT

The in-housing process of the Annual Meeting has been a huge success in order to increase the EBMT's positioning and visibility and to improve financial control as requested by auditors. We will further invest in this important aspect of our mission, and my compliments go to the Barcelona office.

7. IMPLEMENTING E-LEARNING TOOLS

We plan to invest in an e-learning platform, which will allow the community to further educate themselves. The recently published EBMT Handbook 2019 is an excellent example of the EBMT educational activities.

FINANCIAL CONCLUSION

The very positive financial development of the organisation during recent years has allowed EBMT to further build on its strategic goals and consolidate its structure. Current highlights include the website renewal, registry upgrade, cellular therapy registry, and the development of an EBMT-based benchmarking system which has been initiated in 2019 and will be further developed in the next few years. Despite all these substantial strategic key financial investments in 2018 for IT and staff, EBMT will be closing the year 2018 with a positive total result of 2,106 k€ (expenses of 8,956 k€ and a total income of 11,062 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 582 k€ will be earmarked as well. The residual budget of 1,024 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, 1,153 k€ will be used in 2019 to strategically invest in the goals mentioned above.

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

Jürgen Kuball
EBMT Treasurer

SOURCE OF INCOME

EBMT Members donations
€626,000
5,7%

Scientific Sponsors donations
€282,000
2,5%

Annual Meeting
€6,551,000
59,2%

Results from investments
-€44,000
-0,4%

Grants for Studies, Clinical Trials & Education
€2,922,000
26,4%

Accreditation (JACIE)
€725,000
6,6%

Total Income:
€11,062,000
100%

DESTINATION OF RESOURCES

Scientific Studies
€2,135,000
23,8%

EBMT Registry
€829,000
9,3%

Annual Meeting
€3,233,000
36,1%

Educational Activities
€1,209,000
13,5%

Standards & Accreditation (JACIE)
€694,000
7,7%

Management and administration
€856,000
9,6%

Total Expenditure
€8,956,000
100%

FINANCIAL OUTCOME

Total Income:	€11,062,000	100%
Total Costs:	€8,956,000	81%
Gross Result:	€2,106,000	19%
Earmarked:	€1,082,000	10%
Net Result:	€1,024,000	9%

Figure 2. Financial highlights 2018

At the time of the report was printed (March 6, 2019), the audit process had not been completed yet.

The Corporate Sponsors

The EBMT would like to thank the 22 corporate sponsors for their generous support in 2018.

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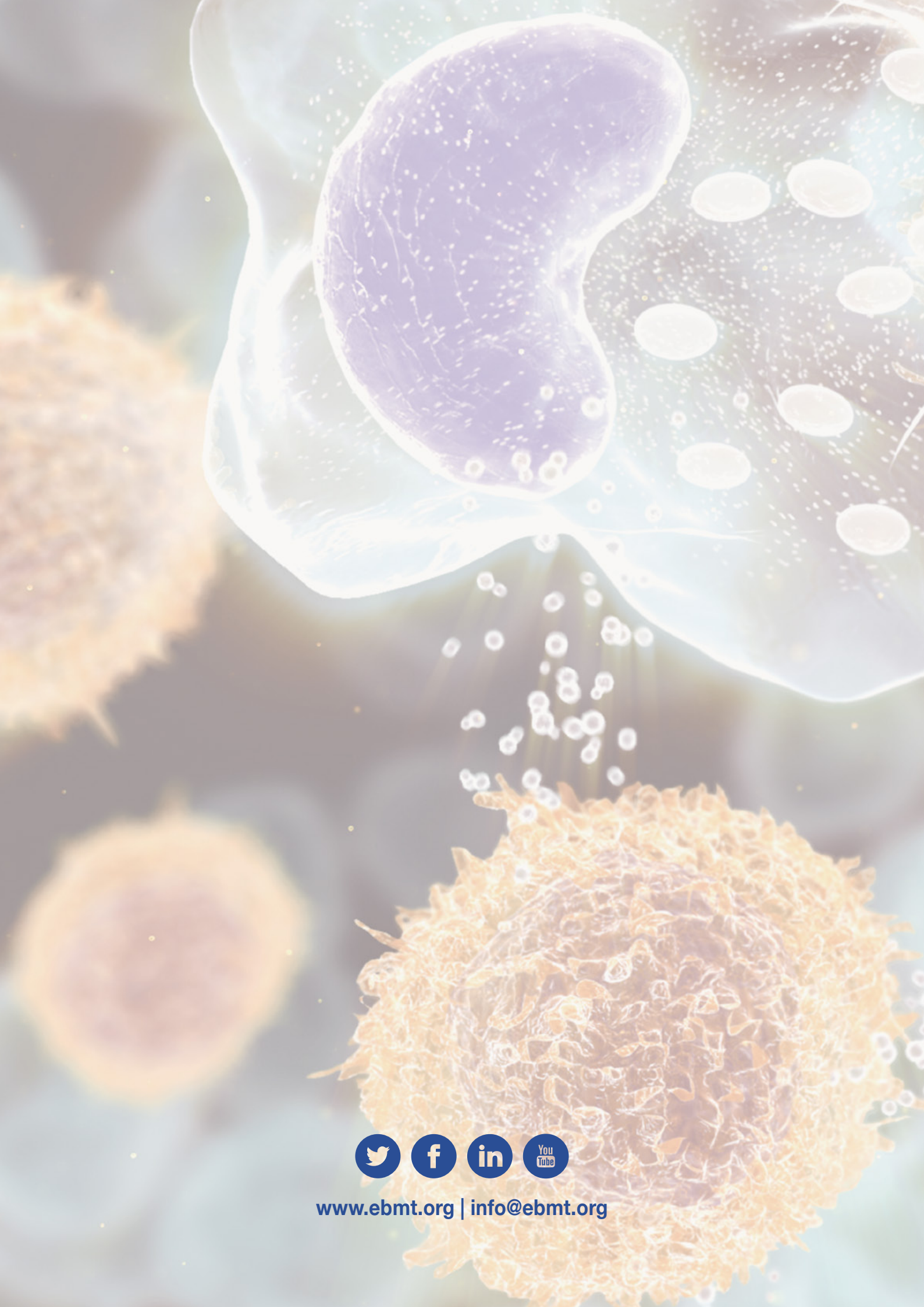
for providing the necessary permission and organising the photoshoot.



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