





# Annual Report / 16

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#### **About us**

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

EBMT members—more than 4,000 physicians, nurses, scientists and other healthcare professionals—participate in a unique collaborative network of peers involved in haematopoietic stem cell transplantation (HSCT) and cellular therapy research. Membership encompasses more than 500 centres from over 50 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.



#### **MISSION**

To save the lives of patients with blood cancers and other life-threatening diseases by advancing the fields of blood and marrow transplantation and cell therapy worldwide through science, education and advocacy.

#### **VISION**



 Enhancing the scientific output of the organisation through strong support from the Working Parties to exploit the potential of the Registry, and continue generating high-quality retrospective and prospective data both in the autologous and allogeneic settings



Collaborating with the different disease-oriented cooperative groups



 Widening the scope of the Annual Meeting through the incorporation of high-level basic and translational research sessions



 Developing a broad annual educational events agenda in order to address more focused research and clinical topics



 Advocating for patients before the health authorities in order to maintain a high standard of care and high-quality research "The EBMT is committed to make strong and significant contributions to the fields of haematology, cellular therapy, immunotherapy and stem cell transplantation."



Mohamad Mohty EBMT President

## Message from the President

One more year has passed and the new EBMT Annual Report is already here! 2016 was a remarkable year for our society because we saw more milestones being reached in the life of EBMT, allowing us to look to the future with great enthusiasm. In the last few months, we introduced several novel features, applied new strategies throughout and strengthened our role as a stronger international medical and scientific society. All of this created exciting new times for our members. I can spend hours describing all of our activities, but I do not want it to become a very boring introduction, because what we achieved was beyond all expectations. I am incredibly proud to see how the EBMT board, scientific council, staff, individual members and centres, sponsors and other stakeholders across the organisation pulled together to grasp the opportunities to make a real and meaningful difference to our society.

As it is well highlighted in this report, all EBMT missions are now developing positively. We have enhanced our educational capacities with the introduction of the September International Transplant Course (ITC), and the different "Best of EBMT" events across the globe. Our annual event is expanding thanks to a new organisational and business model. As part of our mission, we have started working on a set of international recommendations for the clinical training of physicians to qualify them as being capable of performing stem cell transplantation procedures and taking care of such patients. The goal is to implement this global curriculum as a tool for the development of our field worldwide. In addition, different other educational tools will be designed for the benefit of our members and those involved in the field (e.g. smartphone apps, e-learning centre, nursing textbook, etc.) A new cellular therapy registry has been introduced and a modern registry replacing the current long-lived ProMISe registry is moving quickly. A new JACIE initiative is being progressively launched, because quality of patients' care is a fundamental issue. The EBMT is committed to make strong and significant contributions to the fields of haematology, cellular therapy, immunotherapy and stem cell transplantation.

I am very grateful to have been entrusted with the leadership of this wonderful organisation. Please enjoy reading this report, and do not forget to follow the EBMT news on Twitter @TheEBMT and @Mohty\_EBMT.

### EBMT structure

#### **Board**

#### Executive Committee, President

Mohamad Mohty Hospital Saint Antoine, Paris, France

#### 2. Executive Committee, Secretary

Rafael Duarte Hospital Universitario Puerta de Hierro, Madrid, Spain

#### 3. Executive Committee, Treasurer

Jürgen Kuball University Medical Centre, Utrecht, The Netherlands

#### 4. Scientific Council Chair

Nicolaus Kröger University Hospital Eppendorf, Hamburg, Germany

#### 5. Scientific Council Co-Chair

Arnon Nagler Chaim Sheba Medical Center, Tel-Hashomer, Israel

#### 6. Scientific Council Education Representative

Carlo Dufour
Institute G. Gaslini, Genova, Italy

#### 7. Scientific Council Registry Representative

Chiara Bonini€ Hospital San Raffaele, Milano, Italy

#### 8. Nurses Group President

Aleksandra Babic Istituto Europeo di Oncologia, Milano, Italy

#### 9. Congress President

Christian Chabannon Institut Paoli Calmettes, Marseille, Franco

#### Scientific council -Working parties

#### 10. Severe Aplastic Anaemia

Carlo Dufour Institute G. Gaslini, Genova, Italy

#### 11. Autoimmune Diseases

John Snowden Sheffield Teaching Hospitals NHS Trust, Sheffield, UK

#### 12. Acute Leukaemia

Arnon Nagler Chaim Sheba Medical Center, Tel-Hashomer, Israel

#### 13. Cellular Therapy & Immunobiology

Chiara Bonini Hospital San Raffaele, Milano, Italy

#### 14. Infectious Diseases

Jan Styczynski University Hospital, Collegium Medicum UMK, Bydgoszcz, Poland

#### 15. Inborn Errors

Andrew Gennery Great North Children's Hospital, Newcastle-Upon-Tyne, UK

#### 16. Lymphoma

Silvia Montoto St. Bartholomew's and The Royal London NHS Trust, London, UK

#### 17. Paediatric Diseases

Peter Bader Klinikum der Johann-Wolfgang Goethe Universitaet, Frankfurt, Germany

#### 18. Solid Tumour

Francesco Lanza Hospital Ravenna, Ravenna, Italy

#### 19. Chronic Malignancies

Nicolaus Kröger University Hospital Eppendorf, Hamburg, Germany

#### 20. Complications & Quality of Life

Rafael Duarte Hospital Universitario Puerta de Hierro, Madrid, Spain

#### **Committees**

#### 21. Nuclear Accident

Ray Powles
Cancer Center London, London,
UK

#### 22. CT2

Charles Craddock Queen Elizabeth Hospital, Birmingham, UK

#### 23. Statistical

Myriam Labopin Hospital Saint Antoine, Paris, France

#### 24. JACIE

John Snowden Sheffield Teaching Hospitals NHS Trust, Sheffield, UK

#### 25. Donor Outcomes

Joerg Halter University Hospital of Basel, Basel, Switzerland

#### 26. Registry

Per Ljungman Karolinska University Hospital, Stockholm, Sweden

#### 27. Global Committee

Norbert-Claude Gorin Hospital Saint Antoine, Paris, France

































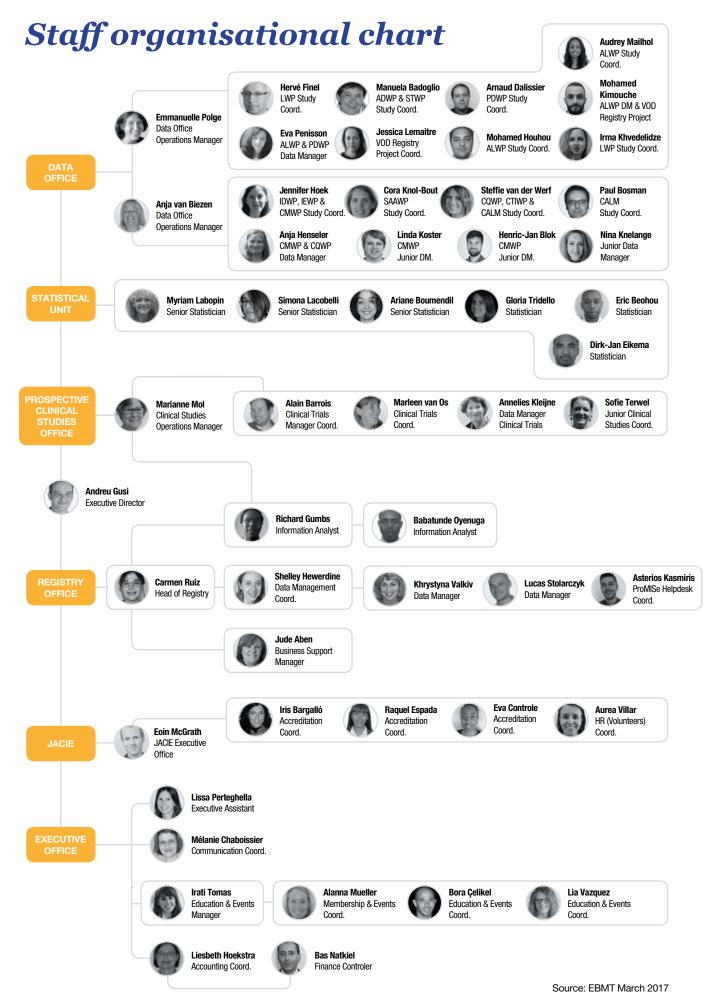












# EBMT membership

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

Our members are listed according to their role within their team. They are comprised of the following distribution of roles: Our members can be classified as centre members (full or associate); individual or provisional members.



Physician **2,697** 



Nurse 747



Data Manager 636



Laboratory Technician 124



Quality Manager **201** 



**Other 240** 





FULL centres reporting\*

**505** 

INDIVIDUAL members

**109** 

ASSOCIATE centres

39

PROVISIONAL members\*\*

**26** 

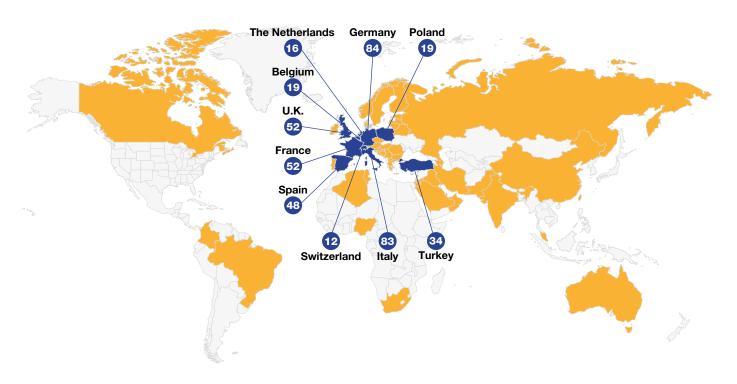
Source: ProMlse Jan. 2017

<sup>\*</sup> Commit to submitting data on all patients treated in their centre on an annual basis, and enjoy the benefits of voting rights and eligibility for JACIE accreditation.

<sup>\*\*</sup> New members (centres and individuals) which are pending approval at the General Assembly Meeting during the upcoming EBMT Annual Meeting.

Top ten countries in terms of number of centres participating in the EBMT

# Our 558 centre members are located in 57 different countries



#### **Membership benefits**

The benefits for those centres which are full EBMT members include the following:

- Eligibility to elect and stand as board members
- Participate in EBMT studies
- Be eligible for JACIE accreditation
- Have access to the EBMT Registry
- Be eligible for reduced fees to attend the EBMT Annual Meeting
- Receive discounts on subscriptions to the official journal of the EBMT, Bone and Marrow Transplantation.

\*There are special conditions for Chinese centre/individual member applicants:

- 50% discounted membership fees for the first year
- Sponsorship with two signatures on the application form is not mandatory, only the support of the EBMT Global Committee Representative (Norbert Claude Gorin)

#### **Joining the EBMT**

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.



# Sciences

The scientific activity reports	
Severe Aplastic Anaemia Working Party (SAAWP)	)
Autoimmune Diseases Working Party (ADWP)	
Acute Leukaemia Working Party (ALWP)	
Cellular Therapy & Immunobiology Working Party (CTIWP)	
Infectious Diseases Working Party (IDWP)	4
Inborn Errors Working Party (IEWP)	4
Lymphoma Working Party (LWP)	
Paediatric Diseases Working Party (PDWP)	
Solid Tumours Working Party (STWP)	1
Chronic Malignancies Working Party (CMWP)	
Complications & Quality of Life Working Party (CQLWP)	;
Publications 2016	;
EBMT Transplant Activity Survey 2015	;
The EBMT Registry	

# The scientific activity reports



This report is the result of the research carried out in 2016 by the 11 Working Parties of the EBMT, which represent the Scientific Council, and by the Nurses Group Research Committee.

The Working Parties exploit the potential of the patient registry – 39,000 transplants were registered during 2016 and a total of 589,444 transplants appeared as registered in the registry on December 31, 2016. The scientists use the data that are submitted continuously by more than 500 centres to carry three types of studies: retrospective data collection studies; non-interventional cohort studies and interventional studies.

As you can see from the graphs and from the Working Parties' activity reports on the following pages, 2016 was a very productive year resulting in a significant increase of impact factor manuscripts published in peer-reviewed journals such as *New England Journal of Medicine, Journal of Clinical Oncology and Blood.* 

Due to the strong commitment of the Working Party members, numerous abstracts were presented as oral communication or poster at several international meetings, not only at the EBMT 2016 Annual Meeting but also at ASH, EHA or BMT Tandem Meeting.

In addition, we would like to highlight the increase of educational activities. In 2016, the different Working Parties, Committees and Nurses Group performed 24 educational events with more than 1,300 attendees in total in addition to the Annual Meeting that welcomed 4,750 participants. Needless to say that the BMT community continues to see the EBMT as a major player in the scene of scientific productivity, education and dissemination.

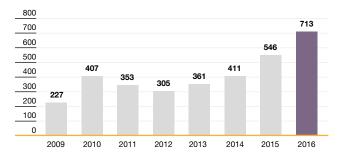
We are very pleased with the scientific outcomes which allow the EBMT to make solid advances in the field of haematopoietic stem cell transplantation and cellular therapy and thus increase survival rates and enhance the quality of life of patients with blood cancers and diseases.

On behalf of the EBMT Scientific Council, I would like to thank you all for your continuous support. I also take the opportunity to invite you and the members of your team to join the regular meetings of the Working Parties and to participate actively in our scientific projects. Together we will continue to make steady progress towards our mission.

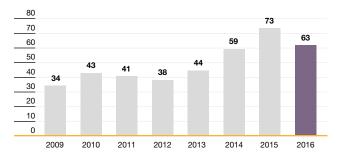
#### Nicolaus Kröger

Scientific Council Chair

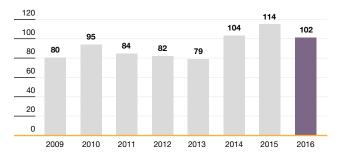
#### Impact factor



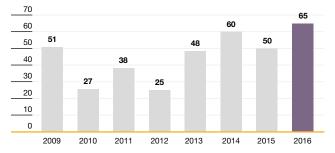
#### **Publications**



#### **Oral presentation**



#### Poster presentation



# Severe Aplastic Anaemia Working Party (SAAWP)



Chair: Carlo Dufour

#### **Major achievements**

## Progression of two prospective randomised clinical trials:

- 1. The RACE study that compares standard immunosuppressive treatment (IST) (ATG+CsA) plus Eltrombopag vs Standard IST alone. This EBMT study financially supported by GSK, Pfizer and Alexion Pharmaceuticals. Thirty-seven EBMT centres will enroll patients. Fifty-three patients have been enrolled since recruitment began on July 1st 2015.
- 2. The Moderate Aplastic Anaemia (MAA) study that compares Cyclosporin A (CsA) plus placebo vs. CsA plus eltrombopag in MAA This study in sponsored by the University of Ulm (Germany) and financially supported by GSK Germany. Sixteen EBMT centres from six countries (France, Germany, Italy, Switzerland, The Netherlands and the UK) will recruit patients. Nine patients have been recruited in German centres on December 2016.

#### Bone Marrow Failure Textbook, First Edition

This book is an EBMT and ESH collaboration and it will be divided into two parts:

Part I: Acquired Aplastic Anaemia

Part II: Congenital Bone Marrow Failure Syndromes

There will be approximately 30 chapters comprising a total of 400 to 500 printed pages. The book will cover all aspects of bone marrow failure both in paediatrics and adults and also areas of supportive care; these are topics of great interest to physicians working with infectious diseases and also those involved in transfusion services.

Contributing authors will be world leading experts from the field of bone marrow failure, mostly from Western Europe, but also from North America and Asia.

The book will be of crucial importance for haematologists and physicians dealing with bone marrow transplantation in patients of all ages including paediatric, adult and geriatric.

#### Data quality Initiative

This initiative aims at improving the quality and follow up data of patients receiving Stem Cell Transplant and the number of registration and the quality of data from undergoing exclusive Immunosuppressive (IS) treatment.

The SAAWP data managers along with the Chair and the Secretary revised the data collection form (Med B) for transplanted patients and prepared a new one for those undergoing only IS. These forms were further revised by expert members of the SAAWP and afterwards a survey has been initiated among all EBMT centres to assess willingness to participate in either one or both initiatives.

#### Principal research studies

- 1. Outcome of HSCT in 87 Diskeratosis congenita patients. This is the largest cohort ever studied
- 2. Campath vs. ATGAM in HSCT for aplastic anaemia
- Outcome of HSCT in Fanconi Anemia >18 years old. A cohort of 180 patients collected also in the US (Cincinnati, NY) and in Brazilian centres. This is the largest cohort ever studied
- 4. Long-term side effects of G-SCF in AA
- 5. Use of Eltrombopag in AA

#### **Key publications**

1. Unrelated alternative donor transplantation for severe acquired aplastic anemia: a study from the French Society of Bone Marrow Transplantation and Cell Therapies and the Severe Aplastic Anemia Working Party of EBMT. Devillier R, *Haematologica* 2016 Jul.

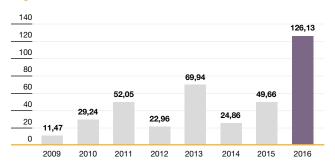
#### **Major educational courses**

SAAWP and ADWP Joint Meeting - November 9-11 2016 in Paris, France



SAAWP and ADWP Joint Meeting -November 2016 in Paris, France

#### Impact factor (Aplastic Aenemia)



	2014	2015	2016
Oral Presentations	9	10	20
Poster Presentations	1	5	1
International Educational Events	1	0	1

# Autoimmune Diseases Working Party (ADWP)



Chair: **John Snowden** 

#### **Major achievements**

Over the last 20 years we have developed the largest database worldwide for HSCT in autoimmune diseases with around 2,300 cases reported. Since 2013, the yearly number of HSCT procedures registered has increased by over 25%, and is now hitting 200 registrations annually, reflecting a significant change in transplant practice and the active role of many EBMT centres.

In 2016, Dominique Farge-Bancel completed her term as Chair and John Snowden took over having been Secretary. Dominique's substantial achievements included the publication of three major ADWP randomised trials (i.e. ASTIS, ASTIMS and ASTIC) in collaboration with other European Societies (i.e. EULAR, ECTRIMS and ECCO).

The efficacy of autologous HSCT is now established for AD and the future is about fine tuning to maximise safety and efficacy. Health economic considerations and implementation science are also essential to define how best to deliver HSCT in the context of biological and other modern therapies.

Laboratory studies continue to characterise the biological changes induced by HSCT - or "rebooting" - of dysfunctional immune systems resulting in clinical stabilisation and/or reversal of organ damage.

Sustained positive clinical results and enhanced ADWP activity in otherwise refractory AD patients continues to attract sustained and increased interest from patients, clinicians, and healthcare providers in the field. We continue to recommend that HSCT is only delivered by JACIE accredited centres while working closely with relevant disease specialists.

Finally, in 2016 the ADWP organised two educational and business meetings in Berlin and a joint meeting with the SAAWP in November (see below).

#### Principal research studies

- Autologous stem cell transplantation for progressive systemic sclerosis: a prospective noninterventional study across Europe (NIISC) – D. Farge
- Autologous haematopoietic stem cell transplantation for Crohn's disease: a retrospective survey of long-term outcomes - C Brierley & C Castilla Llorente
- Evaluating the cost-effectiveness of autologous haematopoietic stem cell transplantation (aHSCT) versus disease-modifying therapy in multiple sclerosis using a matching-adjusted indirect comparison (MAIC) - P Tappenden
- Retrospective study on Multiple Sclerosis paediatric patients treated with autologous HSCT
   J Burman & K Kirgizov
- 5. Allogeneic HSCT for autoimmune diseases R Greco



Members of the ADWP - April 2016 in Valencia

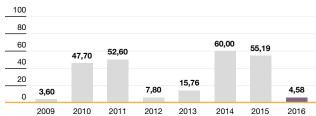
#### **Key publications**

- Does ex vivo CD34+ positive selection influence outcome after autologous hematopoietic stem cell transplantation in systemic sclerosis patients?. Oliveira MC, Bone Marrow Transplant. 2016 April
- Haematopoietic stem cell transplantation in autoimmune diseases: From basic science to clinical practice. Kelsey PJ, Curr Res Transl Med. 2016 Apr-Jun

#### **Major educational courses**

- ADWP Educational Course and Business Meeting January 22-23 2016 in Berlin, Germany
- Autologous transplantation in autoimmune diseases -Educational session during the EBMT Annual Meeting 2016 in Valencia, Spain
- Meet the expert session: Autologous transplant for autoimmune diseases during the EBMT Annual Meeting 2016 in Valencia, Spain
- SAAWP and ADWP Joint Meeting November 9-11 2016 in Paris, France

#### Impact factor (Autoimmune Diseases)



	2014	2015	2016
Oral Presentations	5	6	11
Poster Presentations	7	1	0
International Educational Events	1	1	1



# Acute Leukaemia Working Party (ALWP)



Chair: **Arnon Nagler** 

#### **Major achievements**

The ALWP had a very fruitful year in 2016. Acute leukemia continues to be the number one indication for allogeneic stem cell transplantation (allo-SCT) in Europe. According to the recent Transplant Activity Survey of the EBMT, out of 40,829 transplants performed annually in Europe, 33% (i.e. 11,853 transplants) are for acute leukemia (96% allogeneic). Out of the total allogeneic transplantations, 36% are for acute myeloid leukemia (AML) and 16% are for acute lymphoblastic leukemia (ALL).

Notably, there is a constant increase in numbers of transplants for acute leukemia with no saturation; in last year report the number of transplants for acute leukemia increased by 14% with 24% increase in Haploidentical transplants (Haplo-SCT). As for Haplo transplants the numbers of T cell repletion (T-replete) Haplo-SCT are constantly increasing. On the other hand the T cell deplete Haplo-SCT continue to be an attractive platform for cellular adoptive immunotherapy with manipulated and engineered T cells and T cell subsets (Mohty M. Oral ASH 2016). For this reason, one of the ALWP main areas of interest in 2016 continues to be Haplo-SCT. Toward this end, we compared the two leading strategies for graft versus host disease (GvHD) prophylaxis in Haplo-SCT post transplantation cyclophosphamide (PTCy) vs anti thymocyte globulin (ATG) with GvHD free relapse free survival (GRFS) being the primary end point Ruggeri A (Hematologica 2016). Similarly we compared myeloablative to reduced intensity conditioning for Haplos (Rubio MT, Savani B; J Hematol Oncol 2016) as well as BM vs PB grafts (Ruggeri A. Oral ASH 2016). We also made major efforts to assess the clinical relevance of mismatches of the human leukocyte antigen (HLA) on the unshared haplotype (Lorentino F). Few studies are performed assessing the novel end point GRFS: in post T-replete vs. T-deplete haplo-SCT; haplo-SCT for ALL; transplants from HLA matched siblings and unrelated donors and more.

Another very important topic for the ALWP during 2016 was assessing transplantation outcome by the machine learning approach and computer intelligence in large patient cohorts and in accordance to leukaemia risks. These two studies by Dr Shouval R were presented as orals at ASH 2016.

#### Other hot topics were:

- Allo-SCT for ALL: Results of allogeneic transplants from both siblings and moreover unrelated transplants have improved significantly over the last decade (Giebel S. Hematologica 2016) and in spite of the novel new therapies and monoclonal antibodies for ALL the number of transplants are not decreasing (Oral, EHA 2016). Two other studies assessing the role of ATG in ALL and comparing Allogeneic vs Autologous transplant in Ph+ ALL were presented as oral at ASH 2016 (Giebel S).
- CBT: Although it seems that numbers are decreasing the ALWP together with Eurocord performed some important studies focusing on CBT in 2016. Baron F assessed single vs double CBT (Oral EHA 2016) and revisited the topic of graft vs leukemia effect (GVL) in CBT. Finally and very importantly, we are for the first time identifying overall survival (OS) predictors and related interactions in CBT using random survival forests in collaboration with Eurocord (Shouval R,Oral at ASH 2016).
- Defining AML-related molecular markers and dissecting their influence on allo-SCT outcome continues to be one of ALWP's main efforts led by Dr. Jordi Esteve (Oral, Tandem 2016).





We also continued our educational activities and organised or contributed to some meetings focusing on relevant topics including haplo-SCT, allogeneic transplantation and immunotherapies in myeloid malignancies, relapse post allo-SCT and novel conditioning regimens.

#### **Principal research studies**

- 1. Allogeneic stem cell transplantation for refractory and secondary acute leukaemia Bipin S; Brisot E; Pavlu J.
- Identifying overall survival predictors in 1) allogeneic transplantation and 2) in umbilical cord blood transplantation using random survival forests in collaboration with Eurocord – Shouval R, Fien J, Labopin M, Ruggeri A, Baron F, Gluckman E.
- 3. Transplantation outcomes and trends in ALL focusing on conditioning, ATG, pre- and post- transplant TKIs, predicting cytogenetic markers and source of graft Giebel S; Czerw T; Esteve J; Schmid C.
- 4. Clinical outcome of haploidentical haematopoietic stem cell transplantation in adults with de novo acute leukaemia focusing on GVHD prophylaxis, conditioning, source of graft, HLA disparity assessing GRFS and comparing to other types of stem cell transplantations. Ciceri F, Ruggeri A, Piemontese S, Lorentino F, Mohty M.

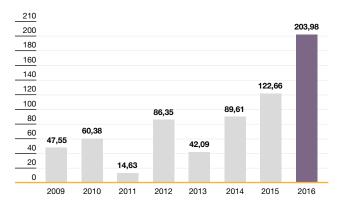
#### **Key publications**

- Post-transplant cyclophosphamide versus antithymocyteglobulin as graft versus host disease prophylaxis in aploidentical transplant. Ruggeri A, Haematologica 2016 Oct 6
- Prediction of Hematopoietic Stem Cell Transplantation Related Mortality- Lessons Learned from the In-Silico Approach: A European Society for Blood and Marrow Transplantation Acute Leukemia Working Party Data Mining Study. Shouval R, PLoS One 2016 Mar 4
- 3. Expanding transplant options to patients over 50 years-Improved outcome after reduced intensity conditioning mismatchedunrelated donor transplantation for patients with acute myeloid leukemia: A report from the Acute Leukemia Working Party of the EBMT. Savani BN, Haematologica 2016 June

#### **Major educational courses**

- Master Classes in Transplantation and Hematology (MATH)<sup>®</sup>: Exploring New Frontiers - January 28 2016 in Paris, France
- Educational Symposium of the Acute Leukaemia Working Party on "Allogeneic stem cell transplantation and immunotherapy in myeloid malignancies" - November 25–27 2016 in Marseille, France

#### Impact factor (Acute Leukaemia)



	2014	2015	2016
Oral Presentations	18	22	30
Poster Presentations	13	15	19
International Educational Events	1	3	2

# Cellular Therapy and Immunobiology Working Party (CTIWP)



# Chair: Chiara Bonini

#### **Major achievements**

The mission of the CTIWP is to understand and exploit the biological including immunological events occurring upon haematopoietic stem cell transplantation at large, and to implement modern cellular therapies based on cell and gene engineering approaches to improve transplantation outcomes.

In 2016 the CTIWP launched and implemented:

- A new Registry for Cellular Therapy, integrated within the EBMT Registry, designed to capture data on the most innovative cell and cell-based gene therapy approaches rapidly entering the clinical arena.
- Two retrospective and one multicentric prospective transplant immunobiology studies (one ready to be submitted for publication).
- 3. A survey on currently used practices for minimally-manipulated cell products, with a view to harmonised recommendations.
- 4. A survey on graft storage procedures in Europe (ready to be submitted for publication).
- 5. A survey on manufacturing of Mesenchimal Stem Cells in Europe.
- 6. A survey on policies of immunological monitoring of patients undergoing allogeneic HSCT.

One of the most important missions of the CTIWP is to disseminate and discuss scientific results. In 2016 the CTIWP hosted:

- The 5<sup>th</sup> Cell Therapy Day during the EBMT 2016 Annual Meeting, designed with the local organising committee, and the CTIWP Scientific Symposium that provided attendees with practical, regulatory and scientific background information for the optimisation of cell transplant and the development, evaluation and implementation of innovative cellular therapies.
- 2. A joint educational event of the CMWP and the CTIWP entitled "Come of Age... Immunotherapy in Multiple Myeloma". More than 120 physicians, scientists and health care providers attended this exciting cutting-edge meeting to discuss the most recent developments in the field.

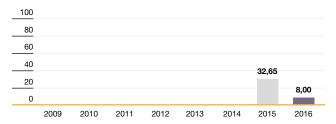


Jon J. van Rood Award 2016

#### **Principal research studies**

- 1. Identification of immunological biomarkers predictive of clinical outcome after haplo-identical SCT
- 2. The role of parent/child and haplo-identical siblings immune interactions (IPA/NIMA vs NIPA/IMA mismatching in GvH vs HvG directions) on clinical outcomes in haplo-identical transplantation
- Non-interventional prospective study on the role of donors vs recipient NK cell allo-reactivity in haplo-identical SCT
- A survey on manufacturing of Mesenchimal Stem Cells in Europe

#### Impact factor (Cell Therapy & Immunobiology)



	2014	2015	2016
Oral Presentations	0	4	1
Poster Presentations	0	4	8
International Educational Events	1	1	1

#### **Key publications**

- RIC versus MAC UCBT in adults with AML: A report from Eurocord, the ALWP and the CTIWP of the EBMT. Baron F, Oncotarget 2016 Jul 12
- Impact of cord blood banking technologies on clinical outcome: a Eurocord/Cord Blood Committee (CTIWP), European Society for Blood and Marrow Transplantation and NetCord retrospective analysis. Saccardi R, *Transfusion* 2016 Aug

#### **Major educational courses**

- 1. Activities during the EBMT 2016 Annual Meeting:
  - CTIWP Scientific Symposium Sponsored by Novartis -150 attendees
  - Jon J. van Rood Award 2016 Sponsored by Neovii
  - 5<sup>th</sup> Cell Therapy Day
- Joint educational event of the CMWP and the CTIWP: "Come of age... Immunotherapy in Multiple Myeloma" - September 30 - October 1 2016 in Berlin, Germany

# Infectious Diseases Working Party (IDWP)



Chair: **Jan Styczynski** 

#### **Major achievements**

*Mission:* share the experience and develop cooperative studies to increase education in the field of diagnosis, prophylaxis and treatment of infectious complications in HSCT patients.

Activity: IDWP is a transversal Working Party, with scientific interests involving various diseases. To continue the progress in the field of infectious diseases in transplant setting, we are focused both on prospective and retrospective studies. IDWP members continue their scientific and educational activities in the fields: bacterial infections (D. Averbuch, M. Mikulska, C. Cordonnier, D. Engelhard), viral infections (P. Ljungman, K. Ward, R. de la Camara, M. Mikulska, S. Cesaro, M. Schmidt-Hieber, H. Einsele, R. Martino, J. Styczynski), fungal infections (M. Mikulska, O. Penack, S. Cesaro), pneumocystis jiroveci infections (C. Robin, C. Cordonnier), vaccinations (C. Cordonnier, P. Ljungman, D. Engelhard, S. Cesaro) and ECIL guidelines (chaired by C.Cordonnier). IDWP recent achievements were published in Lancet Infectious Diseases, Journal of Clinical Oncology, Haematologica, Journal of Antimicrobial Chemotherapy, Bone Marrow Transplantation and Current Drug Targets.

**Science:** Currently ongoing prospective non-interventional projects include:

- 1. Treatment of HCV infection after HSCT.
- 2. Pneumocystis jirovecii Pneumonia after allo-HSCT.
- 3. Impact of Pre-existing invasive Aspergillosis on allo-HSCT.

Their results can significantly influence everyday transplant practice in the future. Therefore the IDWP would like to invite you to join our prospective studies. This progress will be possible only if many EBMT members continuously participate in such studies.

**Education:** The 19<sup>th</sup> Annual Educational Course of IDWP was organised together with CQLWP in Madrid and was a major success. The 20<sup>th</sup> IDWP Educational Course will be organised in Poznan (Poland) at the 25<sup>th</sup> Anniversary of the IDWP with special lectures given by all former IDWP Chairs.



Joint educational course of the CQWP and IDWP - October 2016 in Madrid, Spain

#### Principal research studies

- 1. Role of CMV, EBV and HHV6 on outcomes of HSCT.
- The incidence of gram-negative bacteremia, risk factors and resistance to antibiotics: a prospective study.
- Impact of pre-existing invasive aspergillosis on allo-HSCT outcome: prospective non-interventional study.
- Risk factors and outcome of pneumocystis pneumonia (PcP) infection in HSCT: prospective non-interventional study.
- Treatment approach for patients with HCV infection and who underwent HSCT: prospective non-interventional study.

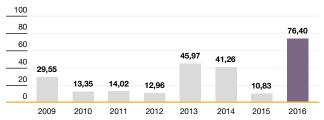
#### **Key publications**

- Impact of Donor Epstein-Barr Virus Serostatus on the Incidence of Graft-Versus-Host Disease in Patients With Acute Leukemia After Hematopoietic Stem-Cell Transplantation: A Study From the Acute Leukemia and Infectious Diseases Working Parties of the European Society for Blood and Marrow Transplantation. Styczynski J, J Clin Oncol. 2016 Jul 1
- Management of viral hepatitis in patients with haematological malignancy and in patients undergoing haemopoietic stem cell transplantation: recommendations of the 5th European Conference on Infections in Leukaemia (ECIL-5). Mallet V, Lancet Infect Dis. 2016 May
- Management of Epstein-Barr Virus infections and posttransplant lymphoproliferative disorders in patients after allogeneic hematopoietic stem cell transplantation: Sixth European Conference on Infections in Leukemia (ECIL-6) guidelines. Styczynski J, Haematologica 2016 Jul
- 4. Pneumocystis jirovecii pneumonia: still a concern in patients with haematological malignancies and stem cell transplant recipients. Cordonnier C, *J Antimicrob Chemother*. 2016 Sep

#### **Major educational courses**

 Joint Educational Course of the IDWP and the CQLWP: "Management of transplant complications – tricks of the trade" - October 27-29 2016 in Madrid, Spain

### Impact factor (Infectious Diseases)



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

# Inborn Errors Working Party (IEWP)



Chair: **Andrew Gennery** 

#### **Major achievements**

2016 has been a busy year for the IEWP. In terms of publications, six manuscripts were published or accepted for publication in high impact journals. A further two are in revision and several are in preparation. We had an oral presentation in the Presidential symposium at EBMT 2016 and a further oral presentation at the European Society of Immunodeficiencies 16<sup>th</sup> Biennial meeting, as well as several posters. Other than IEWP sessions at the EBMT Annual Meeting and European Society of Immunodeficiencies, IEWP took part in the 10<sup>th</sup> Scientific Meeting of the EBMT PDWP joint meeting with the IEWP and the 5<sup>th</sup> Meeting of the EBMT Paediatric Nurses in Rhodes. The IEWP Autumn meeting in Ghent was well attended with over 120 delegates. The meeting was preceded by an educational half day on 'How to Transplant Metabolic Diseases'. The meetings keynote lecture was Steve Holland from NIH, and there was a lively debate on the merits of the new TCD transplant methods over MMUD transplants for PID. The second IEWP Thymic workshop saw speakers from France, Italy, the Netherlands and UK joined by speakers from the USA and Israel for an extremely interactive and productive meeting. Despite this frenetic activity, there are many projects underway – so come and join the fun, and the science!

#### Principal research studies

- Treosulfan-based conditioning for allogeneic haematopoietic stem cell transplantation in children with chronic granulomatous disease. Multicentre experience
- 2. SCETIDE analysis of SCID HSCT outcome to 2013
- 3. IPEX syndrome Outcome with or without HSCT (joint with PIDTC)
- 4. Longterm outcome of HSCT for SCID (joint with PIDTC)
- 5. Outcome of LAD HSCT 2008 2015
- 6. Domino HSCT for PID (with PIDTC)
- Cord Blood SCT for HLH (with Eurocord)
- 8. HSCT for Porphyria (with CIBMTR)
- 9. HSCT for STAT3-GOF (with PIDTC)
- 10. HSCT for LRBA deficiency (with PIDTC)



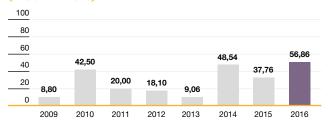
#### **Key publications**

- Outcomes after Unrelated Umbilical Cord Blood Transplantation for Children with Osteopetrosis. Chiesa R, Biol Blood Marrow Transplant. 2016 Nov
- A prospective study on the natural history of patients with profound combined immunodeficiency: An interim analysis. Speckmann C, J Allergy Clin Immunol. 2016 Sep 19
- Treosulfan-based conditioning for allogeneic HSCT in children with chronic granulomatous disease: a multicenter experience. Morillo-Gutierrez B, Blood. 2016 Jul 21

#### **Major educational courses**

- The 10<sup>th</sup> Scientific Meeting of the EBMT Paediatric Diseases Working Party conducted as joint meeting with the EBMT Inborn Errors Working Party and the 5th Meeting of the EBMT Paediatric Nurses - May 26-28 2016 in Island of Rhodes, Greece
- 2. IEWP Autumn Meeting 4-6 November 2016 in Ghent, Belgium
- 3. 2<sup>nd</sup> Inborn Errors Working Party Thymic Workshop 11 November 2016 in Leiden, The Netherlands

#### Impact factor (Inborn Errors)



	2014	2015	2016
Oral Presentations	2	5	2
Poster Presentations	2	2	3
International Educational Events	3	3	4



# Lymphoma Working Party (LWP)



Chair: Silvia Montoto

#### **Major achievements**

The LWP continues to be an active and dynamic research group producing numerous publications, scientific abstracts and educational events for the clinical community. In April 2016, Dr Silvia Montoto was appointed as the new chair of the LWP. She will took over from Prof Peter Dreger to whom we are very grateful for his hard work and productive tenure. Dr Montoto oversees the LWP which includes: A. Boumendil (statistician), H. Finel (data manager) and S. Robinson (scientific secretary), along with the following panel members: A. Sureda (Hodgkin lymphoma and Educational Affairs), P. Dreger (Indolent lymphomas), N. Schmitz (T cell lymphoma), H. Schouten (Aggressive lymphomas), S. Dietrich and O. Hermine (Mantle Cell lymphoma), C. Kyriakou (Lymphoplasmacytic lymphoma) and A. Tanase (Outreach Affairs). The LWP has also recently expanded its data management support with the appointment of a new part time position. In addition to the biannual Open Research Forum, fortnightly teleconferences are conducted to oversee the current research portfolio.

The success of the LWP is demonstrated by the number of publications, presentations at major international meetings (10 studies presented at EBMT, EHA and ASH) and educational events that have been completed this year. In addition to this, the LWP currently manages a portfolio of 29 on-going studies including prospective non-interventional studies. The LWP has successfully collaborated with the CMWP, the CIBMTR and with pharma on a number of projects.

Over the coming year the LWP will complete the data collection, analysis and manuscripts for several studies and are poised to launch three further studies.

The 13<sup>th</sup> Annual Educational Course will be held in Prague in the autumn of 2017. We will also look to further develop links with other transplant working groups and with pharma to help develop future research initiatives.

For more information, view the LWP webpage on www.ebmt.org and follow the LWP on Twitter: @LymphomaWP\_EBMT.

#### Principal research studies

- Efficacy of Brentuximab Vedotin before or after allogeneic transplant for classical Hodgkin lymphoma.
- 2. Checkpoint inhibition for treating Hodgkin and Non-Hodgkin lymphoma in the context of allogeneic stem cell transplantation
- Long-term follow-up of the EBMT-LWP Lym1 randomized study analysing the role of rituximab prior and post autologous stem cell transplantation (ASCT) in patients with relapsed follicular lymphoma (FL)



12th Educational Course of the LWP - September 2016 in Dublin, Ireland

#### **Key publications**

- Prospective noninterventional study on peripheral blood stem cell mobilization in patients with relapsed lymphomas. van Gorkom G, J Clin Apher. 2016 Sep 10
- Reduced intensity allogeneic stem cell transplantation for follicular lymphoma relapsing after an autologous transplant achieves durable long term disease control. An analysis from the Lymphoma Working Party of the EBMT. Robinson SP, Ann Oncol. 2016 Mar 8
- 3. Post-transplant cyclophosphamide-based haplo-identical transplantation as alternative to matched sibling or unrelated donor transplantation for non-Hodgkin lymphoma: a registry study by the European society for blood and marrow transplantation. Dietrich S, Leukemia 2016 Oct

### Impact factor (Lymphoma)



	2014	2015	2016
Oral Presentations	8	6	3
Poster Presentations	11	4	5
International Educational Events	4	4	6

#### **Major educational courses**

- 1. Activities during the EBMT 2016 Annual Meeting:
  - Open Research Forum (ORF)
  - Educational Session: "Transplant in the algorithm of lymphoid disorders"
  - Meet the expert: "Treatment of refractory Hodgkin disease"
- 2. LWP Panel meeting in Paris, France
- 3. ORF in Dublin, Ireland
- 12<sup>th</sup> Educational Course of the LWP: 'Treatment of malignant lymphoma: state-of –the-art and role of stem cell transplantation' - September 21-23 2016 in Dublin, Ireland



# Paediatric Diseases Working Party (PDWP)



Chair: **Peter Bader** 

#### **Major achievements**

The PDWP held its 10<sup>th</sup> Scientific Meeting of the EBMT Paediatric Diseases Working Party conducted as joint meeting with the EBMT Inborn Errors Working Party and the 5<sup>th</sup> Meeting of the EBMT Paediatric Nurses in May on the beautiful island of Rhodes. The local organiser S. Graphakos and P. Bader welcomed 120 participants from 24 countries and E. Trigoso saluted 37 nurses from 8 different countries. The wide-ranging program included up-to-date contributions on hemoglobinopathies, cellular therapy, GvHD, alternative transplantations, inborn errors, antiviral therapy, complications and late effects, like infertility, after SCT.

The latter topic had been extensively addressed in a PDWP driven "Expert Workshop on Fertility Preservation in the Context of HSCT" in Baden, Austria, in 2015. Results and recommendations for preservation of fertility are now summarised and published in two contributions in *Bone Marrow Transplantation*:

- Fertility preservation practices in paediatric and adolescent cancer patients undergoing HSCT in Europe: a population-based survey.
- State-of-the-Art Fertility Preservation in Children and Adolescents Undergoing Haematopoietic Stem Cell Transplantation: Report on the Expert Meeting of the PDWP of the EBMT in Baden, Austria, 29-30 September 2015.

A further publication in BMT indicating the "Incidence and severity of crucial late effects after allogeneic HSCT for malignancy under the age of three years: TBI is what really matters" completed the PDWP's significant contribution to the field of late effects after SCT in 2016.

#### Principal research studies

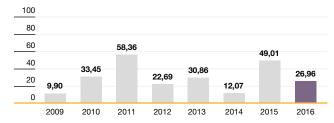
- 1. ALL SCTped 2012 FORUM ("For Omitting Radiation Under Majority Age")
- 2. Subsequent allogeneic SCT in paediatric patients: indications, procedures and outcome
- Haematopoietic stem cell transplantation for sickle cell disease: An analysis on behalf of Eurocord, PDWP of EBMT, CIBMTR, USP (Ribeirão Preto) and Ruby Hall Clinic
- 4. HSCT in children and adolescents with non-hodgkin lymphoma
- 5. Outcome of children developing grade III-IV acute graft-versus-host-disease after allogeneic haematopoietic stem cell transplantation



#### **Key publications**

- Incidence and severity of crucial late effects after allogeneic HSCT for malignancy under the age of 3 years: TBI is what really matters. Bresters D, Bone Marrow Transplant. 2016 Nov
- Association of CTH variant with sinusoidal obstruction syndrome in children receiving intravenous busulfan and cyclophosphamide before hematopoietic stem cell transplantation. Huezo-Diaz Curtis P, *Pharmacogenomics J*. 2016 Oct 25
- 3. Impact of Conditioning Regimen on Outcomes for Children with Acute Myeloid Leukemia Undergoing Transplantation in First Complete Remission. An Analysis on Behalf of the Pediatric Disease Working Party of the European Group for Blood and Marrow Transplantation. Lucchini G, Biol Blood Marrow Transplant. 2016 Dec 1

#### Impact factor (Paediatric Diseases)



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

#### **Major educational courses**

 The 10<sup>th</sup> Scientific Meeting of the EBMT Paediatric Diseases Working Party conducted as joint meeting with the EBMT Inborn Errors Working Party and the 5<sup>th</sup> Meeting of the EBMT Paediatric Nurses - May 26-28 2016 in Island of Rhodes, Greece



10th Scientific Meeting of the PDWP



Eugenia Trigoso, Chair of the Paediatric Nurses Committee



Stelios Graphakos, local organiser

# Solid Tumours Working Party (STWP)



# Chair: Francesco Lanza

#### **Major achievements**

#### Breast Cancer (BC)

In 2016, the STWP started a retrospective study with the main goal to assess long term efficacy and toxicity of autologous HSCT in metastatic BC. Autologous Haematopoietic Stem Cell Transplantation (AHSCT) has a low mortality rate and provides satisfactory long-term survival rates but needs to be further investigated in clinical trials. A Prospective clinical trial in triplenegative breast cancer is ongoing.

#### Germinal Cell Tumour (GCT)

In 2016, the main objective of the STWP research activity was to explore the role of AHSCT in rare clinical variants of GCT.

**Pure seminoma:** The optimal management of advanced seminoma that relapses after chemotherapy remains unknown. We retrospectively analysed outcomes with the use of HSCT. On multivariable Cox analysis, refractory disease was a significantly negative prognostic factor for both PFS and OS, while prior radiotherapy trended to significance for both. This retrospective analysis suggested that HDCT may represent a valuable therapeutic option after standard-dose chemotherapy failure.

**Mediastinal non-seminoma (MnS) GCT:** The role of HSCT in this disease category is under evaluation. The preliminary data of this retrospective analysis confirmed that the MnS was characterised by the poorest outcome with 5-year overall survival ranging from 40% to 45%. The use of AHSCT as both early intensification and at disease recurrence proved to be effective, given up-front, and may produce a 15%-20% absolute improvement in survival compared with standard dose CT.

**Refractory gestational trophoblastic neoplasia (GTN):** A few case reports reported of salvage AHSCT in patients with GTN. We conducted a retrospective analysis on 29 patients GTN treated with salvage HDC. Our study seems to support the notion that HDC based on carboplatin seems to be active in this heavily pretreated patient population with refractory GNT, and that AHSCT might represent a possible option for patients with refractory GNT.

The use of plerixafor for stem cell mobilization in solid tumors: The retrospective analysis performed in patients treated with HSCT showed that plerixafor achieved successful mobilization in > 80% of ST patients, independently of the disease category. A prospective study is planned in order to confirm the results obtained from this retrospective series.



#### **Principal research studies**

- Retrospective analysis of data on high-dose chemotherapy and autologous haemopoietic progenitor cell transplantation for metastatic breast cancer
- 2. Incidence and prevalence of therapy-related myeloid neoplasms and myelodysplastic/myeloproliferative diseases (t-MN) in breast carcinoma patients as a consequence of exposure to alkylating agents, topoisomerase II inhibitors and/or ionising radiations, including high-dose chemotherapy regimens followed by autologous stem cell transplantation
- Stem cell transplantation in soft tissue sarcoma a long-term follow-up: a retrospective study by the STWP on different histologic subtypes
- 4. Long-term results of salvage high-dose chemotherapy for germ cell tumours in a) female, b) adolescent, and c) patients aged over 40 yrs at time of HSCT, d) Incidence of secondary malignancies (SM) in patients with germ cell tumors (GCT) who received high-dose chemotherapy

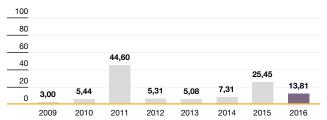
#### **Key publications**

- Salvage High-Dose Chemotherapy for Relapsed Pure Seminoma in the Last 10 Years: Results from the European Society for Blood and Marrow Transplantation Series 2002-2012. Necchi A, Clin Genitourin Cancer. 2016 Jun 27
- Is allogeneic transplant for solid tumors still alive? Bregni M, Bone Marrow Transplant. 2016 May
- High-Dose Chemotherapy and Autologous Hematopoietic Stem Cell Transplantation as Adjuvant Treatment in High-Risk Breast Cancer: Data from the European Group for Blood and Marrow Transplantation Registry. Martino M, Biol Blood Marrow Transplant. 2016 Mar

#### **Major educational courses**

- STWP Educational Meeting during the EBMT 2016 Annual Meeting
- STWP Educational Meeting July 27 2016 in Ravenna, Italy. 32 healthcare professionals attended the meeting. The session focused on the potential of the latest therapeutic advances for the treatment of breast cancer, including HSCT.

#### Impact factor (Solid Tumours)



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

# Chronic Malignancies Working Party (CMWP)



Chair: **Nicolaus Kröger** 

#### **Major achievements**

The CMWP is a disease-orientated WP covering diseases such as chronic myeloid and lymphocytic leukaemia, myelodysplastic syndromes and secondary leukaemia, myeloproliferative neoplasms, multiple myeloma, and other plasma cell disorders such as amyloidosis.

The mission of the CMWP is to contribute significantly to an improved outcome of stem cell transplantation in chronic haematological malignancies. In 2016 we continued our mission in organising high-quality educational events regarding the disease specific topics of our WP.

In January 2016 we hosted in Hamburg, a symposium on "Treatment option for relapse after autograft in Multiple".

In September 2016 we conducted an educational event in London on "Updates on MPN, MDS, Leukemia Stem Cells Biology and Haploidentical Stem cell Transplantation".

In September 2016 we performed together with the CTIWP a scientific symposium on "Come of age... Immunotherapy in Multiple Myeloma" and in November 2016 together with ASBMT in Hamburg the " $3^{\rm rd}$  International Workshop on Biology , Prevention and Treatment of Relapse after Stem Cell Transplantation".

A further major achievement in 2016 was the publication of the randomised EBMT labelled study "ATG to prevent chronic GvHD" in the *New England Journal of Medicine*.

Apart from this NEJM publication, the high activity and scientific productivity of the CMWP in 2016 is reflected by 17 manuscripts in peer-reviewed journals such as *Leukemia*, *Haematologica*, *British Journal of Haematology*, *Bone Marrow Transplantation and Biology of Blood and Marrow Transplantation*, resulting in a cumulative impact factor of 149 in 2016.

At international meetings such as ASH, EHA, and EBMT, active and highly motivated members of the CMWP presented more than 30 oral or poster presentations.

#### **Principal research studies**

- 5-Azacytidine vs Allogeneic Stem Cell Transplantation in elderly MDS patients (VidazaalloStudy). A prospective, randomised EBMT-labelled study
- 2. Reduced vs. standard conditioning in MDS/sAML (RICMAC study). A prospective randomized study of CMWP (Manuscript in 2017)
- 3. Effect of second generation TKI on outcome after allogeneic SCT for CML. A non-interventional study of the CMWP
- Role of Stem Cell Mobilization in Myeloma and Lymphoma. A prospective non-interventional study of CMWP and LWP (CALM Study)



3<sup>rd</sup> International Workshop on Biology, Prevention and Treatment of Relapse after Stem Cell Transplantation -November 2016 in Hamburg, Germany



"Come of age .. Immunotherapy for myeloma" Joint Educational Event of CMWP and CTIWP -September 2016, Berlin, Germany

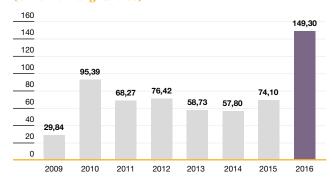
#### **Key publications**

- Antilymphocyte Globulin for Prevention of Chronic Graftversus-Host Disease. Kröger N, N Engl J Med. 2016 Jan 7
- Allogeneic hematopoietic cell transplantation for multiple myeloma in Europe: trends and outcomes over 25 years. A study by the EBMT Chronic Malignancies Working Party. Sobh M, Leukemia. 2016 Oct
- 3. Comparison of Intensive Chemotherapy and Hypomethylating Agents before Allogeneic Stem Cell Transplantation for Advanced Myelodysplastic Syndromes: A Study of the Myelodysplastic Syndrome Subcommittee of the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplant Research. Potter VT, Biol Blood Marrow Transplant. 2016 Sep

#### Major educational courses

- Treatment option for relapse after autograft in Multiple Myeloma - January 30 2016 in Hamburg, Germany
- Educational Event of the CMWP: "Updates on MPN, MDS, Leukemia Stem Cell Biology and Haploidentical transplantation" - September 24 2016 in London, UK
- Joint Educational Event of the CMWP and the CTIWP: "Come of age... Immunotherapy in Multiple Myeloma" - 30 September 30 - October 1 2016 in Berlin, Germany
- 3<sup>rd</sup> International Workshop on Biology, Preventation and Treatment of Relapse after Stem Cell Transplantation – November 4-5 2016 in Hamburg, Germany

#### Impact factor (Chronic Malignancies)



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



CMWP Business Meeting -September 2016 in London

# Complications & Quality of Life Working Party (CQLWP)



Chair: Rafael F. Duarte

#### **Major achievements**

The major goal of the CQLWP is to provide the highest quality of education and research in the broad field of noninfectious complications of hematopoietic cell transplantation. The CMWP organises these activities through a scientific panel which includes the WP Chair (R. Duarte) and Secretary (G. Basak), a Nurse Lead for transplant complications (D. Greenfield), and three subcommittees leaders focused on Regimen-related toxicity and supportive care (T. Ruutu), Graft-versus-host disease (H. Greinix) and Late complications (N. Salooja). Following R Duarte's election as EBMT Secretary during the EBMT 2016 Annual Meeting in Valencia, we are in the process to elect a new CQLWP Chair during the EBMT 2017 in Marseille.

In 2016, the CQLWP organised 2 business meetings, a Joint Educational Course with the Infectious Diseases WP on "Management of transplant complications – tricks of the trade", and participated in the organisation of the First International Transplant Course of the EBMT. Also, in 2016 we completed the development of the EBMT eGVHD App in collaboration with H. Schoemans and the University of Leuven. Initial results showing that this novel platform improves user satisfaction and diagnostic and scoring accuracy for chronic GvHD according to the 2014 NIH criteria were published in August. A final version for large-scale implementation has been developed, and we plan to launch it at the CQLWP Session in Marseille 2017. We have published four WP manuscripts and contributed to additional manuscripts from international collaborations such as the NIH Late Effects Initiative. At present, in addition to 10 additional manuscripts in preparation, we have 17 studies running and 7 new proposals. We hope that these studies, some of them summarised below, will be of interest to you. We would like to encourage you all to get involved in the work of the CQLWP, bringing your own ideas and proposals.

#### Principal research studies

- A prospective non-interventional study on the impact of uric acid levels on GvHD in recipients of allogeneic HCT (O. Penack)
- 2. Hematopoietic stem cell transplantation in patients aged ≥65 (G. Basak, I. Sanchez-Ortega)
- Prevalence of pregnancy and pregnancy outcome among survivors of stem cell transplantation (N Salooja)
- 4. Outcome of patients with secondary malignancies after HSCT (A. Tichelli)



Joint educational course of the CQWP and IDWP - October 2017 in Madrid, Spain

#### **Key publications**

- Development, preliminary usability and accuracy testing of the EBMT 'eGVHD App' to support GvHD assessment according to NIH criteria-a proof of concept. Schoemans H, Bone Marrow Transplant. 2016 Aug
- 2. The EBMT-ELN working group recommendations on the prophylaxis and treatment of GvHD: a change-control analysis. Ruutu T, *Bone Marrow Transplant*. 2016 Nov 28
- 3. Metabolic Syndrome and Cardiovascular Disease after Hematopoietic Cell Transplantation: Screening and Preventive Practice Recommendations from the CIBMTR and EBMT. DeFilipp Z, *Biol Blood Marrow Transplant*. 2016 Aug

#### **Major educational courses**

 Joint Educational Course of the CQWP and the IDWP: "Management of transplant complications – tricks of the trade" - October 27-29 2016 in Madrid, Spain

#### Impact factor (Complications & Quality of Life)

100								
80								
60								
40								
20		22,59			16,37		19,46	13,78
0	6,10			7,48		3,46		10,70
	2009	2010	2011	2012	2013	2014	2015	2016

	2014	2015	2016
Oral Presentations	1	2	2
Poster Presentations	2	1	3
International Educational Events	1	3	1



# **Publications 2016**



Title	First Listed Author	Journal	PMID
A prospective study on the natural history of patients with profound combined immunodeficiency: An interim analysis.	Speckmann C	J Allergy Clin Immunol.	27658761
Allogeneic haematopoietic stem cell transplant in patients with lower risk myelodysplastic syndrome: a retrospective analysis on behalf of the Chronic Malignancy Working Party of the EBMT.	Robin M	Bone Marrow Transplant.	27819688
Allogeneic hematopoietic cell transplantation for multiple myeloma in Europe: trends and outcomes over 25 years. A study by the EBMT Chronic Malignancies Working Party.	Sobh M	Leukemia	27118410
Allogeneic stem cell transplantation for patients with myelodysplastic syndrome 70 years of age or older: A retrospective study of the MDS subcommittee of the Chronic Malignancies Working Party (CMWP) of the EBMT.	Heidenreich S	Biol Blood Marrow Transplant.	27720995
Antilymphocyte Globulin for Prevention of Chronic Graft-versus-Host Disease.	Kröger N	N Engl J Med.	26735993
Association of CTH variant with sinusoidal obstruction syndrome in children receiving intravenous busulfan and cyclophosphamide before hematopoietic stem cell transplantation.	Huezo-Diaz P	Pharmacogeno- mics J.	27779248
Association of Macroeconomic Factors With Nonrelapse Mortality After Allogeneic Hematopoietic Cell Transplantation for Adults With Acute Lymphoblastic Leukemia: An Analysis From the Acute Leukemia Working Party of the EBMT.	Giebel S	Oncologist.	26869584
Autologous transplant remains the preferred therapy for relapsed APL in CR2.	Ganzel C	Bone Marrow Transplant.	27088379
Comparison of Intensive Chemotherapy and Hypomethylating Agents before Allogeneic Stem Cell Transplantation for Advanced Myelodysplastic Syndromes: A Study of the Myelodysplastic Syndrome Subcommittee of the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplant Research.	Potter VT	Biol Blood Marrow Transplant.	27264633
Development, preliminary usability and accuracy testing of the EBMT 'eGVHD App' to support GvHD assessment according to NIH criteria-a proof of concept.	Schoemans H	Transplant. Bone Marrow	27042834
Does ex vivo CD34+ positive selection influence outcome after autologous hematopoietic stem cell transplantation in systemic sclerosis patients?	Oliveira MC	Bone Marrow Transplant.	26642332
ECIL guidelines for preventing Pneumocystis jirovecii pneumonia in patients with haematological malignancies and stem cell transplant recipients.	Maertens J	J Antimicrob Chemother.	27550992
ECIL guidelines for the diagnosis of Pneumocystis jirovecii pneumonia in patients with haematological malignancies and stem cell transplant recipients.	Alanio A	J Antimicrob Chemother.	27550991
ECIL guidelines for treatment of Pneumocystis jirovecii pneumonia in non-HIV-infected haematology patients.	Maschmeyer G	J Antimicrob Chemother.	27550993
Economics and Outcome After Hematopoietic Stem Cell Transplantation: A Retrospective Cohort Study.	Gratwohl A	EBioMedicine.	26844291
Expanding transplant options to patients over 50 years. Improved outcome after reduced intensity conditioning mismatched-unrelated donor transplantation for patients with acute myeloid leukemia: a report from the Acute Leukemia Working Party of the EBMT.	Savani BN	Haematologica	26969081
Haematopoietic stem cell transplantation in autoimmune diseases: From basic science to clinical practice.	Kelsey PJ	Curr Res Transl Med.	27316390
Hematopoietic stem cell transplantation in Europe 2014: more than 40 000 transplants annually.	Passweg JR	Bone Marrow Transplant.	26901709
Hemopoietic stem cell transplantation in thalassemia: a report from the European Society for Blood and Bone Marrow Transplantation Hemoglobinopathy Registry, 2000-2010.	Baronciani D	Bone Marrow Transplant.	26752139
High CD3+ and CD34+ peripheral blood stem cell grafts content is associated with increased risk of graft-versus-host disease without beneficial effect on disease control after reduced-intensity conditioning allogeneic transplantation from matched unrelated donors for acute myeloid leukemia - an analysis from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Czerw T	Oncotarget.	27036034
High-dose chemotherapy and autologous hematopoietic stem cell transplantation as adjuvant treatment in high-risk breast cancer: data from the EBMT registry.	Martino M	Biol Blood Marrow Transplant.	26723932
Impact of conditioning intensity in T-replete haplo-identical stem cell transplantation for acute leukemia: a report from the acute leukemia working party of the EBMT.	Rubio MT	J Hematol Oncol.	26980295

Title	First Listed Author	Journal	PMID
Impact of Conditioning Regimen on Outcomes for Children with Acute Myeloid Leukemia Undergoing Transplantation in First Complete Remission. An Analysis on Behalf of the Pediatric Disease Working Party of the European Group for Blood and Marrow Transplantation.	Lucchini G	Biol Blood Marrow Transplant.	27916512
Impact of cord blood banking technologies on clinical outcome: a Eurocord/Cord Blood Committee (CTIWP), European Society for Blood and Marrow Transplantation and NetCord retrospective analysis.	Saccardi R	Transfusion	27245270
Impact of Donor Epstein-Barr Virus Serostatus on the Incidence of Graft-Versus-Host Disease in Patients With Acute Leukemia After Hematopoietic Stem-Cell Transplantation: A Study From the Acute Leukemia and Infectious Diseases Working Parties of the European Society for Blood and Marrow Transplantation.	Styczynski J	J Clin Oncol.	27091716
Impact of drug development on the use of stem cell transplantation: a report by the European Society for Blood and Marrow Transplantation (EBMT).	Passweg JR	Bone Marrow Transplant.	27819687
Incidence and severity of crucial late effects after allogeneic HSCT for malignancy under the age of 3 years: TBI is what really matters.	Bresters D	Bone Marrow Transplant.	27348540
Investigating covariate-by-centre interaction in survival data.	Biard L	Stat Methods Med Res.	27166409
Is allogeneic transplant for solid tumors still alive?	Bregni M	Bone Marrow Transplant.	26808572
Long-term survival of patients with CLL after allogeneic transplantation: a report from the European Society for Blood and Marrow Transplantation.	van Gelder M	Bone Marrow Transplant.	27941763
Management of Epstein-Barr Virus infections and post-transplant lymphoproliferative disorders in patients after allogeneic hematopoietic stem cell transplantation: Sixth European Conference on Infections in Leukemia (ECIL-6) guidelines.	Styczynski J	Haematologica.	27365460
Management of viral hepatitis in patients with haematological malignancy and in patients undergoing haemopoietic stem cell transplantation: recommendations of the 5th European Conference on Infections in Leukaemia (ECIL-5).	Mallet V	Lancet Infect Dis.	27599653
Matched and mismatched unrelated donor compared to autologous stem cell transplantation for acute myeloid leukemia in first complete remission: a retrospective, propensity score-weighted analysis from the ALWP of the EBMT.	Saraceni F	J Hematol Oncol.	27589849
Metabolic Syndrome and Cardiovascular Disease after Hematopoietic Cell Transplantation: Screening and Preventive Practice Recommendations from the CIBMTR and EBMT.	DeFilipp Z	Biol Blood Marrow Transplant.	27184625
Metabolic syndrome and cardiovascular disease following hematopoietic cell transplantation: screening and preventive practice recommendations from CIBMTR and EBMT.	DeFilipp Z	Bone Marrow Transplant.	27548466
Outcome of conditioning intensity in acute myeloid leukemia with monosomal karyotype in patients over 45 year-old: A study from the acute leukemia working party (ALWP) of the European group of blood and marrow transplantation (EBMT).	[No authors listed]	Am J Hematol.	27093915
Outcome of patients with chronic myeloid leukemia and a low-risk score: allogeneic hematopoietic stem cell transplantation in the era of targeted therapy. A report from the EBMT Chronic Malignancies Working Party.	Koenecke C	Bone Marrow Transplant.	27111041
Outcomes after Unrelated Umbilical Cord Blood Transplantation for Children with Osteopetrosis.	Chiesa R	Biol Blood Marrow Transplant.	27470286
Outcomes of unrelated cord blood transplantation in patients with multiple myeloma: a survey on behalf of Eurocord, the Cord Blood Committee of Cellular Therapy and Immunobiology Working Party, and the Chronic Leukemia Working Party of the EBMT.	Paviglianiti A	Haematologica.	27229716
Pneumocystis jirovecii pneumonia: still a concern in patients with haematological malignancies and stem cell transplant recipients.	Cordonnier C	J Antimicrob Chemother.	27550990
Post-transplant cyclophosphamide versus antithymocyte-globulin as graft versus host disease prophylaxis in haploidentical transplant.	Ruggeri A	Haematologica	27758821
Post-transplant cyclophosphamide-based haplo-identical transplantation as alternative to matched sibling or unrelated donor transplantation for non-Hodgkin lymphoma: a registry study by the European society for blood and marrow transplantation.	Dietrich S	Leukemia	27146790



Title	First Listed Author	Journal	PMID		
Prediction of Hematopoietic Stem Cell Transplantation Related Mortality- Lessons Learned from the In-Silico Approach: A European Society for Blood and Marrow Fransplantation Acute Leukemia Working Party Data Mining Study.	Shouval R	PLoS One.	26942424		
Prognostic pre-transplant factors in myelodysplastic syndromes primarily treated by high dose allogeneic hematopoietic stem cell transplantation: a retrospective study of the MDS subcommittee of the CMWP of the EBMT.	Cremers EM	Ann Hematol.	27650829		
Prospective noninterventional study on peripheral blood stem cell mobilization in patients with relapsed lymphomas.	van Gorkom G	J Clin Apher.	27614935		
Reduced intensity allogeneic stem cell transplantation for follicular lymphoma relapsing after an autologous transplant achieves durable long term disease control.  An analysis from the Lymphoma Working Party Of the EBMT.	Robinson SP	oinson SP Ann Oncol.			
Relapse of AML after hematopoietic stem cell transplantation: methods of monitoring and preventive strategies. A review from the ALWP of the EBMT.	Tsirigotis P	Bone Marrow Transplant.	27295272		
RIC versus MAC UCBT in adults with AML: A report from Eurocord, the ALWP and he CTIWP of the EBMT.	Baron F	Oncotarget.	27250025		
Safety and efficacy of thiotepa-based conditioning for allogeneic transplantation in AML: a survey from the ALWP of the EBMT.	Eder S	Bone Marrow Transplant.	27643865		
Salvage High-Dose Chemotherapy for Relapsed Pure Seminoma in the Last 10 Years: Results From the European Society for Blood and Marrow Transplantation Series 2002-2012	Necchi A	Clin Genitourin Cancer.	27444987		
Sequential Intensified Conditioning Regimen Allogeneic Hematopoietic Stem Cell Fransplantation in Adult Patients with Intermediate or High-Risk Acute Myeloid Leukemia in Complete Remission: a Study From the ALWP of the EBMT.	Malard F	Biol Blood Marrow Transplant.	27816650		
Splenic irradiation before hematopoietic stem cell transplantation for chronic myeloid eukemia: long-term follow-up of a prospective randomized study.	Gratwohl A	Ann Hematol.	26994010		
Stem cell transplantation in multiple myeloma and other plasma cell disorders report from an EBMT preceptorship meeting).	Bruno B	Leuk Lymphoma.	26735310		
The EBMT-ELN working group recommendations on the prophylaxis and treatment of GvHD: a change-control analysis.	Ruutu T	Bone Marrow Transplant.	27892949		
The impact of HLA-matching on reduced intensity conditioning regimen unrelated donor allogeneic stem cell transplantation for acute myeloid leukemia in patients above 50 years-a report from the EBMT acute leukemia working party.	Rubio MT	J Hematol Oncol.	27488518		
Thiotepa-based conditioning for allogeneic stem cell transplantation in acute ymphoblastic leukemia - a survey from the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation.	Eder S	Am J Hematol.	27673280		
Freatment options for relapse after autograft in multiple myeloma - report from an EBMT educational meeting.	Garderet L	Leuk Lymphoma.	27650125		
reosulfan-based conditioning for allogeneic HSCT in children with chronic granulomatous disease: a multicenter experience.	Morillo-Gutierrez B	Blood	27216217		
Unmanipulated haploidentical versus matched unrelated donor allogeneic stem cell ransplantation in adult patients with acute myelogenous leukemia in first remission: A retrospective pair-matched comparative study of the Beijing approach with the EBMT database.	Sun Y	Haematologica	27081180		
Unrelated alternative donor transplantation for severe acquired aplastic anemia: a study from the French Society of Bone Marrow Transplantation and Cell Therapies and the Severe Aplastic Anemia Working Party of EBMT.	Devillier R	Haematologica	27056924		
Unrelated Cord Blood Transplantation for Acute Leukemia Diagnosed in the First Year of Life: Outcomes and Risk Factor Analysis.	Ruggeri A	Biol Blood Marrow Transplant.	27777140		
Unrelated donor versus matched sibling donor in adults with acute myeloid leukemia n first relapse: an ALWP-EBMT study.	Ruggeri A	J Hematol Oncol.	27639553		

# EBMT Transplant Activity Survey 2015



The activity survey reflects in a timely manner current trends in stem cell transplantation and is an essential tool for health care planning and health policy makers. It also reflects the culture of sharing information and data prevalent within the EBMT.

# Record number of 42,171 HSCT reported to the survey in 2015

687 centers from 48 countries (39 European and 9 affiliated countries) were invited to report their transplant activity for 2015. 655 teams reported and corresponds to a 95% return rate and includes 552 active EBMT member teams.

In 2015 a record number of 42,171 HSCT (17,302 HSCT (41%) allogeneic and 24,869 (59%) autologous) in 37,626 patients were reported to the survey. 4,545 of which were second or subsequent transplants (1,272 allogeneic and 3,273 autologous). The total number of patients transplanted under the age of 18 in both dedicated and joint adult-pediatric units was 4,490 (3,338 allogeneic and 1,152 autologous HSCT). Of these, 3,015 patients (67%, 2,570 allogeneic and 896 autologous) reporting a total of 3,466 transplants were performed in dedicated pediatric centers. When compared with 2014 the total number of transplants increased by 3.3% (2.1% allogeneic HSCT and 4.1% autologous HSCT) (12).

Indications for HSCT in 2015 are listed in detail in table 1. Main indications were **myeloid malignancies** (AML, CML, MDS/MPN and MPN): 9,413 (25% of total; 96% of which were allogeneic); **lymphoid malignancies** (ALL, CLL, HD, NHL and PCD): 24,340 (65%; 20% allogeneic); **solid tumors**: 1,516 (4%; 3% allogeneic); **non-malignant disorders**: 2,208 (6%; 90% allogeneic) and others: 149 (0.4%).

#### Abbreviations

AID: auto immune disease ALL: acute lymphoblastic leukemia AML: acute myeloid leukemia BMF: bone marrow failure CLL: chronic lymphocytic leukemia CML: chronic myeloid leukemia CR1: first complete remission HD: Hodgkin's disease IDM: inherited disease of metabolism MDS/MPN: myelodysplastic or myelodysplastic/myeloproliferative neoplasm MPN: myeloproliferative neoplasm NHL: Non-Hodgkin lymphoma PCD: plasma cell disorders PID: primary immune disease

Trends include continued growth in transplant activity during the period 2005 and 2015, with the highest percentage increase seen in middle income countries (allo 209%, auto 215%), and the lowest in very high income countries (allo 64%, auto 28%), for both allogeneic and autologous HSCT. In contrast the absolute growth is highest in the very high income countries (growth allo rates 114 transplants per 10x10<sup>6</sup> inhabitants, auto rates 85 for very high income countries; allo rates 35, auto rates 38 for middle income).

Use of haploidentical donors for allogeneic HSCT continues to increase 2,012 in 2015; a 291% increase since 2005. Growth is seen for all diseases with the highest growth seen in myeloid malignancies 1,008, with lymphoid malignancies 636, nonmalignant disorders 316 and 52 others (figure 1a). In AML, haploidentical HSCT increases similarly for patients with both advanced disease and those in CR1 (figure 1b). Both marrow and peripheral blood is used as stem cell source for haploidentical HSCT with higher numbers reported for the latter (figure 1c).

#### Additional cellular therapies

A total of 330 teams (in 35 countries) reported having performed **3,882 cellular therapies** in 2015. Of these, 2,940 patients received donor lymphocyte infusions. Indications were **graft enhancement:** 803 (27%); **residual disease:** 410 (14%); **relapsed disease:** 1,285 (44%) and **per protocol** 442 (15%).

Other cellular therapies were given either within the context of a HSCT or not. The majority were **MSCs** given for GvHD treatment (396) or for graft enhancement (45). 74 patients received MSCs for various other indications. The largest additional group of cellular therapies were selected or expanded T cells given to treat **infectious complications** (119 patients) or for **anti-malignant effects** (37). Other cellular therapies including **NK cells** (14), **TREGs** (35), **genetically modified T-cells** (14), dendritic cells (25) and expanded or genetically modified hematopoietic stem cells (36) were reported more rarely. 121 patients received cellular products for purposes of regenerative medicine.

#### Helen Baldomero Jakob R Passweg

EBMT Activity Survey Office, Hematology, Department of Medicine, University Hospital, Basel, Switzerland



Figure 1 Trend in haplo-identical HSCT in Europe 1990-2015

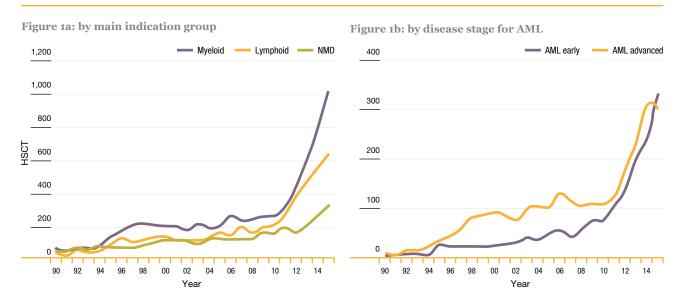
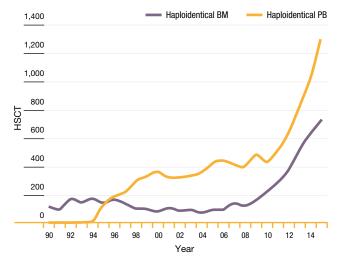


Figure 1c: by stem cell source



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Table 1.

Table 1.  Transplant activity 2015	TRANSPLANT ACTIVITY 2015 No. of patients  Allogeneic Autologous Total																	
activity 2015										A	utologou	IS	Total					
		Family				Unrelated												
		HLA-id		Twin	Haplo :	>=2MM	01	ther fam	ily				BM	BM +		Allo	Auto	
	BM	PBPC	Cord	all	BM	PBSC	BM	PBPC	Cord	BM	PBPC	cord	only	PBPC	cord			
Myeloid malignancies	412	2439	5	9	310	603	28	68	0	525	4425	170	9	410	0	8994	419	9413
Acute myeloid leukemia	290	1750	5	6	208	460	15	52	0	330	2940	133	9	404	0	6189	413	6602
1 <sup>st</sup> complete remission	201	1077	5	3	100	196	10	29	0	203	1537	79	6	349	0	3440	355	3795
not 1 <sup>st</sup> complete remission	64	485	0	3	81	199	4	21	0	90	864	37	3	50	0	1848	53	1901
AML therapy related	11	57	0	0	4	18	0	1	0	9	115	6	0	2	0	221	2	223
AML from MDS/MPN	14	131	0	0	23	47	1	1	0	28	424	11	0	3	0	680	3	683
Chronic myeloid leukemia	26	115	0	0	14	12	0	2	0	27	196	6	0	3	0	398	3	401
chronic phase	14	48	0	0	2	5	0	1	0	14	78	1	0	1	0	163	1	164
not 1st chronic phase	12	67	0	0	12	7	0	1	0	13	118	5	0	2	0	235	2	237
MDS or MD/MPN MPN	87 9	442 132	0	3 0	68 20	108 23	12	9 5	0	146 22	971 318	28 3	0	3 0	0	1874 533	3	1877 533
	-	-	-	-	-	-		-	-			-	-		-		-	
Lymphoid malignancies	338	1410	5	11	222	356	16	41	1	392	1984	114	26	19424	0	4890	19450	24340
Acute lymphatic leukemia	264	641	5	2	101	185	15	27	1	309	851	93	3	81	0	2494	84	2578
1 <sup>st</sup> complete remission	164	457	0	2	49	92	9	12	1	146	556	43	1	65	0	1531	66	1597
not 1st complete remission	100	184 83	5 0	0	52 7	93	0	15 3	0	163 7	295	50 2	2	16 36	0	963 255	18 36	981 291
Chronic lymphocytic leukemia Plasma cell disorders - MM	9	194	0	2	13	13 14	0	ა 1	0	20	131 285	3	5	10856	0	538	10861	11399
Plasma cell disorders - other	0	15	0	2	0	1	0	0	0	20	9	0	0	326	0	29	326	355
Hodgkin's lymphoma	12	130	0	2	55	61	1	2	0	9	166	4	8	2062	0	442	2070	2512
Non Hodgkin lymphoma	47	347	0	3	46	82	0	8	0	45	542	12	10	6063	0	1132	6073	7205
Solid tumors	5	2	1	1	2	19	0	1	0	2	4	1	47	1430	1	38	1478	1516
Neuroblastoma	3	1	1	0	2	12	0	0	0	0	1	1	27	459	1	21	487	508
Soft tissue sarcoma/Ewing	0	Ö	0	0	0	5	0	1	0	0	2	0	10	205	0	8	215	223
Germinal tumors	0	0	0	0	0	0	0	0	0	0	0	0	1	350	0	0	351	351
Breast cancer	0	0	0	0	0	0	0	0	0	0	0	0	0	29	0	0	29	29
Other solid tumors	2	1	0	1	0	2	0	0	0	2	1	0	9	387	0	9	396	405
Non malignant disorders	648	241	28	4	64	131	72	49	4	419	227	98	9	214	0	1985	223	2208
Bone marrow failure - SAA	185	105	5	3	16	22	11	3	1	124	79	12	0	0	0	566	0	566
Bone marrow failure - other	68	24	4	0	8	20	9	12	1	73	31	11	0	0	0	261	0	261
Thalassemia	148	66	9	1	5	12	17	7	1	55	19	1	1	3	0	341	4	345
Sickle cell disease	92	11	6	0	10	4	3	1	0	14	5	0	0	0	0	146	0	146
Primary Immune deficiencies	130	26	3	0	19	65	27	22	1	120	77	37	2	6	0	527	8	535
Inh. disorders of Metabolism	23	5	1	0	6	7	5	3	0	30	14	35	4	0	0	129	4	133
Auto immune disease	2	4	0	0	0	1	0	1	0	3	2	2	2	205	0	15	207	222
Others	31	10	1	0	8	16	5	1	0	17	27	7	0	26	0	123	26	149
TOTAL PATIENTS	1434	4102	40	25	606	1125	121	160	5	1355	6667	390	91	21504	1	16030	21596	37626
Re/additional transplants	55	257	0	2	96	257	4	12	0	71	497	21	7	3266	0	1272	3273	4545
TOTAL TRANSPLANTS	1489	4359	40	27	702	1382	125	172	5	1426	7164	411	98	24770	1	17302	24869	42171

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# The EBMT Registry

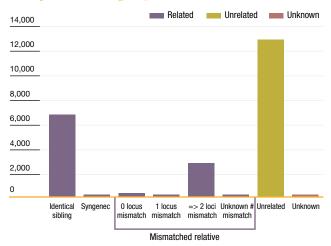


#### 39,000 transplants reported to the Registry in 2016

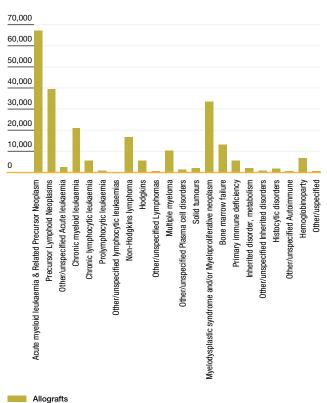
The Registry had a total of 499,118 patients and 589,444 transplants by the December 31, 2016. Of these, approximately 39,000 transplants were registered during 2016, which sees an increase in registrations over previous years. This does not seem to reflect an increase in the number of registered transplants by year of transplant, but seems to be due to some centres catching up with incomplete reporting in the past.

The percentage of transplants directly entered into the Registry by data managers from the transplanting centres remains at 76% of the transplants - the same as during 2015. The rest of the registered transplants were entered by a mixture of National Registry and EBMT Staff, including the upload of data from some paediatric German centres that do not submit data directly to the EBMT.

#### **Donor type** HSCT performed during 2015-2016







#### 130,000 120,000 100,000 80,000 60,000 40,000 20,000 Other/unspecified Acute leukaemia Chronic lymphocytic leukaemia Prolymphocytic leukaemia Other/unspecified lymphocytic leukaemias Non-Hodgkins lymphoma Hodgkins Multiple myeloma Other/unspecified Autoimmune Acute myeloid leukaemia & Related Precursor Neoplasm Precursor Lymphoid Neoplasms Hemoglobinoparty Other/uspecified nherited disorder, metabolism



Autografts

Autografts





#### Project 2020: Registry upgrade

During 2016, the process of evaluating the companies interested in providing the infrastructure for the Registry upgrade continued. By the middle of the year, three of the companies were selected for visiting. The evaluation team was overseen by the Head of the Registry and made up of representatives from various groups: study coordinators, data managers, statisticians and information analysts. The team had face-to-face meetings with representatives of the companies, engaged in detailed discussions on the technical requirements and provided and participated in demonstrations before evaluating the final proposals. The results were submitted to the Steering committee of Project 2020.

Elsevier was appointed on February 1st 2017 to work on the new registry system. The development will be based on MACRO, a powerful, flexible and user friendly Electronic Data Capture system that can be customised further to meet EBMT Registry requirements.

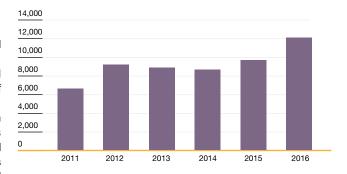
#### **Data sharing**

# A Growable Network Information System (AGNIS) project

The new EBMT Med-A, implemented during 2015, has been fully mapped to the new TED (CIBMTR form). Data of approximately 20,000 registrations from 46 centres has been forwarded by EBMT to the CIBMTR during 2016. Four TED related data collection forms have been automated. A new form to help with the automation of data submission status is being implemented. Testing continues and more centres are expected to be brought into the regular data flow during 2017.

#### Human Leukocyte Antigen (HLA)

During 2016, HLA data were reported for more than 12,000 transplants. Part of the increase was due to the upload of HLA data coming from the Anthony Nolan donor registry for those patients who had transplants from their donors. However, even without this, the number of HLA registered has increased.



#### **Data collection and quality**

#### Med-A

Harmonisation of the MED-B forms with the new Med-A that was implemented in 2015 has been finalised and the manuals have been updated. A MED-A Merge module is used to generate case report forms and Excel downloads for data quality checks. Work to bring this module in line with the new Med-A has proceeded but it remains unfinished.

#### Data management training

The EBMT Board decided that 2016 would be the last year the Registry office would enter data for centres, thus launching a "paperless" registry. Centres for which the Registry office was doing data entry were informed and offered an extra training day to take place in Paris in January. As most of the centres sending paper forms to the Registry office were outside Europe, many of these centres were unable to take advantage of this offer. Training went ahead with those centres that could attend, and courses on data entry for Med-A and for HLA were provided on the 16th January 2017.

#### Cell therapy

New forms for the collection of cell therapies other than transplantation were developed during 2015. They have been implemented in the Registry database and a manual is currently being written. A small number of selected centres submitted completed forms to the Registry so that testing could go ahead with real data. The Data collection forms are ready for data entry and centre training will take place during the Annual meeting 2017.

#### Carmen Ruiz de Elvira

Head of the EBMT Registry

# Education





49



Infographic EBMT 42<sup>nd</sup> Annual Meeting

Awards 47

Educational events 2016

# **EBMT 2016** 42ND ANNUAL MEETING OF THE EUROPEAN **SOCIETY FOR BLOOD AND MARROW TRANSPLATION**



3 - 6 April 2016 Valencia, Spain

The EBMT Annual Meeting is the Society's flagship event. It brings together scientists, physicians, nurses, data managers, patients, quality managers, statisticians, pharmacists, biologists and technicians from Europe and all over the world. The exciting scientific programme, inclusive lectures, "Meet the expert", "How do I ...?" sessions, lunch controversies, and so on, are all designed to cover the key issues relating to HSCT and cellular therapy research.

















#### **Attendance**

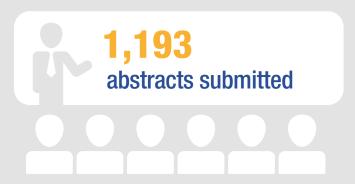


participants from



countries

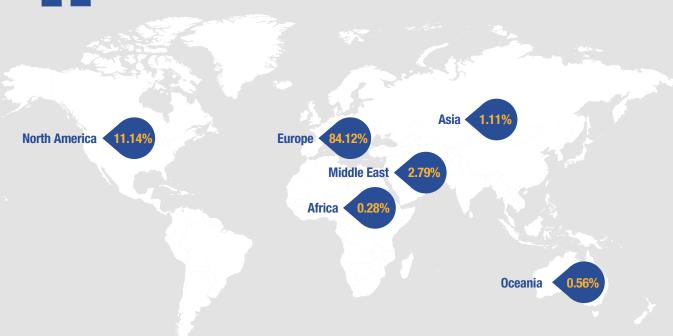
### **Programme**



#### **S**essions



180 sessions presented by 359 speakers







63%
of the delegates
rated the educational
sessions as "very good"
to "excellent1



**73%** of the delegates rated the scientific content as "very good" to "excellent"<sup>2</sup>

#### **New features**



1st pharmacist day with518 attendees



## Top five participants' areas of interest<sup>3</sup>



## **Sponsoring and Exhibition**

**71** 

companies, associations or groups supported the 42<sup>nd</sup> Annual Meeting

**45** 

exhibiting organisations in **916m**<sup>2</sup> exhibition area

28

sponsored sessions

#### **Digital activities**



**608** people sent **4,601** tweets to the Twitter wall generating **9,536,530** potential impressions



2,819 page views of the EBMT-TV online



1,028 downloads of EBMT 2016 App



1,793 delegates registered and are using the E-materials portals



6,191 visitors generating 17,317 views of the online Abstract Book

#### **Networking activities**



2,500 delegates attended the opening session and welcome reception



**200** delegates participated in the EBMT 2016 Walk for Life



92% of the delegates said they liked the Walk for life



450 delegates attended the flamenco concert and dinner



**81%** of the delegates would recommend the EBMT Annual Meeting to a friend or colleague<sup>7</sup>

<sup>&</sup>lt;sup>1-2</sup> EBMT 2016 Delegates survey (547 espondents)

<sup>&</sup>lt;sup>3</sup> EBMT 2016 Delegates survey (616 espondents)

<sup>&</sup>lt;sup>4</sup> Statistics from 1 April to 6 May 2016

<sup>&</sup>lt;sup>5</sup> Statistics from 1 April to 8 May 2016

<sup>&</sup>lt;sup>6</sup> Nature Publishing Group statistics from 19 April to 6 May 2016

<sup>&</sup>lt;sup>7</sup> EBMT 2016 Delegates survey (536 espondents)

### **Awards**





**The Van Bekkum Award** for the best abstract submitted to the physician's programme, sponsored by the EBMT, was presented to Maddalena Noviello for the abstract entitled: *Multiple inhibitory receptors are expressed on central memory and memory stem T cells infiltrating the bone marrow of AML patients relapsing after allo-HSCT.* 



**The Basic Science Award**, sponsored by Clinigen Group Ltd, was presented to P. Santos E Sousa for the abstract entitled: *Tissue instruction dictates spatial diversity of effector T cell programs in graft-versus-host disease.* 



**Honorary Membership** awarded to Tapani Ruutu (Finland) and Christian Gisselbrecht (France).



**The Jian-Jian Luan Award** for Lymphoma Transplant, sponsored by the EBMT, was presented to Alessandro Rambaldi for the abstract entitled: *Randomized Trial comparing R-CHOP-14* versus High Dose Sequential chemotherapy in High Risk Patients with Diffuse Large B-Cell Lymphomas. Final results from the GITIL-RHDS0305 study.



**The Jon van Rood Award** for the best paper in the immunobiology of allogeneic hematopoietic transplantation, sponsored by Neovii Pharmaceuticals, was presented to Yael Zlotnikov-Klionsky for her paper entitled: *Perforin-Positive Dendritic Cells Exhibit an Immuno-regulatory Role in Metabolic Syndrome and Autoimmunity.* 



The Best Young Abstract Awards for the best abstracts submitted for Oral and Poster presentations, sponsored by the EBMT, were presented to 11 young investigators: Nicole Santoro (Italy) O003; Alexandra Laberko (Russian Federation) O004; Daniela Weber (Germany) O006; Pietro Merli (Italy) O007; Justine Decroocq (France) O016; Sara Tognarelli (Germany) O024; Johan Törlén (Sweden) O032; Matthias Felber (Switzerland) O074; Reona Sakemura (Japan) O166; Rebecca Schultze-Florey (Germany) P543; Mehtap Sirin (Germany) P645

**The Best Clinical Poster Award,** sponsored by Nature Publishing Group, was presented to David Miklos for his poster P124 entitled: *Multicenter Open-Label Phase 1b/2 Study of Ibrutinib in Steroid-Dependent/Refractory Chronic Graft Versus Host Disease (cGVHD).* 

The Best Science Poster Award, sponsored by Nature Publishing Group, was presented to Dimin Nie for his poster P097 entitled: Endothelial microparticles carrying hedgehog-interacting protein aggravate endothelial damage in the development of acute graft-versus-host disease after allogeneic hematopoietic stem cell transplantation.



#### **Nurses Group Awards**

**The 8th Distinguished Merit Award** was presented to Corien Eeltink (Amsterdam, The Netherlands).



**The Best Oral Presentation Award** was presented to Sarah Ware (London, UK) for her presentation N0005 entitled: *Holistic assessment of patients' concerns prior to autologous High Dose Melphalan (HDM) Haematopoietic Stem Cell Transplantation (HSCT) for Plasma Cell Disorders (PCD).* 



**The Best Poster Award** was presented to Iris Agreiter for her poster NP036 entitled: Help, Mum or Dad has cancer: *literature* search on how nurses can support children and parents.

# **Educational** events 2016

Berlin, Germany 22-23 Jan. 2016	ADWP ADWP Educational Course and Business Meeting
Paris, France 28 Jan. 2016	ALWP "Master Classes in Transplantation and Hematology (MATH)®: Exploring new frontiers"
Hamburg, Germany 30 Jan. 2016 70 attendees	<b>CMWP</b> Treatment option for relapse after autograft in Multiple Myeloma
Budapest, Hungary 11-14 May 2016	<b>ESH-EBMT</b> 20th Annual ESH-EBMT Training Course
Leiden, Netherlands 24-27 May 2016 26 attendees	<b>EBMT</b> 4 <sup>th</sup> EBMT Statistical Course
Island of Rhodes, Greece 26-28 May 2016 120 attendees	PDWP-IEWP-NG The 10th Scientific Meeting of the EBMT Paediatric Diseases Working Party conducted as joint meetin with the EBMT Inborn Errors Working Party and the 5th Meeting of the EBMT Paediatric Nurses
Southampton, United Kingdom 2 - 3 June 2016 21 attendees	JACIE JACIE Inspector Training Course
Ravenna, Italy 27 July 2016 32 attendees	STWP STWP Educational Meeting
Barcelona, Spain 9-11 Sept. 2016 250 attendees	<b>EBMT</b> 1st EBMT International Transplant Course
Bellinzona, Switzerland 16 Sept. 2016	JACIE-NG Transplant Procedures Update for Physicians and Nurses Operating in JACIE Accredited Transplant Centers Focus on Competency Maintenance
Dublin, Ireland 21-23 Sept. 2016 80 attendees	<b>LWP</b> 12 <sup>th</sup> Educational Course of the LWP: 'Treatment or malignant lymphoma: state-of –the-art and role or stem cell transplantation'
London, UK 24 Sept. 2016 50 attendees	CMWP Educational Event of the CMWP: "Updates on MPN, MDS, Leukemia Stem Cell Biology and
oo attoriaddd	Haploidentical transplantation"
Berlin, Germany 30 Sept1 Oct. 2016 100 attendees	

- ALWP: Acute Leukaemia Working Party
- IEWP: Inborn Errors Working Party
- STWP: Solid Tumours Working Party
- CQLWP: Complications and Quality of Life 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



Barcelona, Spain 6-7 Oct. 2016 20 attendees	JACIE JACIE Inspector Training Course
Gothenborg, Sweden 14 Oct. 2016 65 attendees	NG Nurses Group International Study Day
Nijmegen, Netherlands 27-28 Oct. 2016 25 attendees	JACIE JACIE Inspector Training Course
Madrid, Spain 27-29 Oct. 2016 85 attendees	CQWP-IDWP Joint Educational Course of the CQWP and the IDWP: "Management of transplant complications – tricks of the trade"
Hamburg, Germany 4-5 Nov. 2016 200 attendees	<b>CMWP</b> 3rd International Workshop on Biology, Preventation and Treatment of Relapse after Stem Cell Transplantation
Ghent, Belgium 4-6 Nov. 2016 120 attendees	IEWP IEWP Annual Conference
Paris, France 9-11 Nov. 2016 110 attendees	<b>SAAWP-ADWP</b> SAAWP and the ADWP Joint Meeting
Leiden, The Netherlands 11 Nov. 2016 45 attendees	IEWP 2 <sup>nd</sup> Inborn Errors Working Party Thymic Workshop
Vigo, Spain 11 Nov. 2016	<b>NG</b> Spanish Nurses Group: 10th Day for Nurses in HSCT
Stockholm, Sweden 24 - 25 Nov. 2016 25 attendees	JACIE Inspector Training Course
Marseille, France 25-27 Nov. 2016	ALWP Educational Symposium of the ALWP on Allogeneic Stem Cell Transplantation and Immunotherapy in Myeloid Malignancies



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# Haematology and HSCT Nursing



The EBMT Nurses Group: committed to patient care through education, research and international network collaboration.

The EBMT Nurses Group (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 care through evidence-based practice.

**MISSION:** 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 Healthcare Professionals (AHPs) programme of the Annual Meeting 2016 in Valencia, as well as the Education Day, were organised by the Scientific Committee. Over 500 nurses attended the Annual meeting, with over a hundred abstracts submitted from 60 countries worldwide. The latest evidence in nursing research was presented by noteworthy speakers contributing to a dynamic meeting.

The presentations of the Education Day and International Study Day are available on the Nursing section of www.ebmt.org.

The Educational Meeting on "Transplant procedures update for physicians and nurses operating in JACIE accredited transplant centers with focus on competency maintenance" took place in Bellinzona, Switzerland on September 16, 2016. A new online platform, certified by EBMT and JACIE, was launched including e-sessions and lectures that were recorded during this Educational

Meeting. All these materials are at your disposal free of charge. Watch all the videos and complete the related questionnaire to obtain a Certificate of Competence validated by EBMT and JACIE.



#### **Materials**

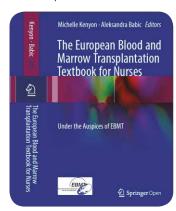
The Veno-Occlusive Disease (VOD) Learning Programme is available on the EBMT website in eight languages and has been updated.

Practical guides on **Paroxysmal Nocturnal Haemaglobinuria: 'Understanding PNH'**, were launched in 2015 and include a brochure and diary for patients, and a practical guide for nurses and AHPs.

All these materials have been translated in five languages - English, German, Italian, Spanish and French and are available on the Nursing section of ebmt.org.

The EBMT NG has worked on a textbook for nurses that will be released by the end of 2017. This textbook provides nurses with a

comprehensive and informative guide covering all aspects of transplant nursing, from basic principles to advanced concepts. It takes the reader on a journey through the history of transplant nursing, presenting concepts to help nurses improve the patient experience, as well as research and auditing methods.



#### Research

The Research Committee is currently collaborating on three studies with the Complications and Quality of Life Working Party (CQWP).

- A survey exploring the barriers and facilitators to AHPs discussing sexual concerns with people following HSCT, the results of this study are about to be submitted for publication.
- 2. A study looking into practices around nutritional care; The data of this study is currently being analysed.
- 3. A study of sexual functioning in adults three and fifteen years post allogeneic SCT (S-FAST). This study is currently open to recruitment and any centre interested in participating can contact the Leiden study office for information (50 euros are being offered for each patient entered once forms have been completed): <a href="mailto:CQWPebmt@lumc.nl">CQWPebmt@lumc.nl</a>



Training Course for HSCT nurses - February 26-27 2016 in Guangzhou, China

Other ongoing projects include several collaborative research projects where the Research Committee is supporting and facilitating NG members' initiatives. A survey of the perceptions of HSCT coordinators on related donor (RD) care for example, was completed in 2016 with the support of the Paris Study office. The survey suggests that RD care is not standardised and that many aspects can be improved, notably ethical aspects.

The rolling programme of research co-ordinated by the Research Committee also has studies in the planning stage, such as a collaborative study to look at the impact of nursing standards of care and skill-mix of staff in JACIE centres, focusing on patient safety, which will have the potential to validate JACIE standards from a nursing perspective.

2017 will see the commencement of an exciting new development within the Research Committee, which will expand capacity for the NG to develop collaborative working with all the Working Parties (WP). This is to be achieved by each WP having a nurse member who will work with the support of the Research Committee to facilitate collaborative, nurse-led research relevant to each WP.

#### **Collaboration**

We had successful National Groups & Forums Chairs Meetings 2016, following the International Study Day at the Gothic Tower in Gothenburg, Sweden on October 14 2016. The meeting was organised together with the Sahlgrenska University Hospital and the theme was based on patient participation.

The next, 9<sup>th</sup> International Study Day, following by the first International Research Nurse Day and National Groups & Forums meeting, will take place in Manchester, UK on October 5-7 2017.

The EBMT NG Global Educational Committee, together with the association Nurses No Frontiers arranged another outreach project aiming to promote HSCT nursing education and practice in low and middle income countries. The Training course for HSCT nurses was organised at the Tata Memorial Centre in Parel, Mumbai in India on December 9-10 2016. This event was attended by 203 participants from 12 Indian states and 39 hospitals. This also highlights the importance of having similar programs to update the nurses' knowledge and skills and to standardise various nursing protocols.

The Paediatric Committee and Paediatric Diseases Working Party (PDWP) had the 5<sup>th</sup> EBMT training course for paediatricians and paediatric nurses on HSCT in children and adolescents in Rhodes, Greece on May 26-28, 2016. This gave nurses from different hospitals from Europe and Greece the possibility to network and share experiences.

The next 6<sup>th</sup> Meeting of the EBMT Paediatric Nurses will take place in Copenhagen, Denmark on June 8-10 2017 themed 'Developing Aspects and Future Directions with Emphasis on Immune Therapy and Toxicity'.

Our achievements during 2016 have set the scene for further development and growth in terms of research, education and international collaboration. We look forward to being able to extend this in 2017, continuing our commitment to promoting excellence in patient care.

#### Aleksandra Babic

EBMT Nurses Group President



Training Course for HSCT nurses - December 9 2016 in Mumbai, India

# Standards and Accreditation - JACIE



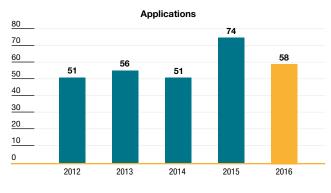
2016 was very successful year for JACIE with the joint-highest number of inspections ever and a new record of accreditation awards. 2016 had the second highest number of applications in the past 5 years and the  $4^{\text{th}}$  highest of all time. These numbers all point to the extraordinary success and impact of JACIE in our field but also to the historical and continuous effort that goes into this initiative.

#### **Applications**

**58 applications** (28 first-time and 30 reaccreditation) received (see figure 1).

Figure 1.

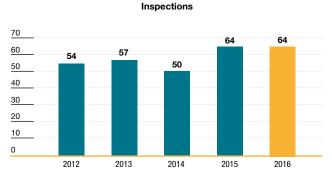
Number of applications received for the past five years



#### **Inspections**

**64 inspections** (21 first-time and 43 reaccreditation) performed equalling the 2015 record (see figure 2). The key factors in being able to complete this high number of inspections is the invaluable commitment of the inspectors and the excellent support provided by the two Accreditation Coordinators, Iris Bargalló and Raquel Espada.

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

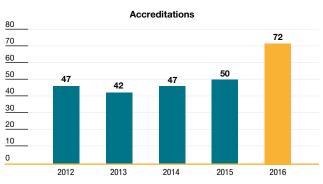


#### **Accreditations**

**72 accreditations** (33 first-time and 39 reaccreditation) awarded (See figure 3). Not surprisingly, 2016 has set a new record with the increase due to the high number of centres inspected in 2015 (64) many of whom went on to finalise their corrections and achieve accreditation in 2016.

Figure 3.

Number of accreditations awarded for the past five years



#### **7<sup>th</sup> Edition of FACT-JACIE Standards**

Work on preparing the 7<sup>th</sup> edition of the FACT-JACIE Standards started in June 2016 with a meeting of the FACT and JACIE teams in Barcelona. Throughout out the second half of 2016, the standards sub-committees worked on their respective sections of the Standards. One significant development for this edition is that a dedicated Quality Management sub-committee has been incorporated into the process which will bring to bear the particular experience and knowledge of quality managers.

#### **Educational events**

Four well-attended JACIE training courses were held in 2106: Southampton (UK), Barcelona, (Spain), Nijmegen (The Netherlands) and Arlanda (Sweden) with 91 participants in total. In addition, participants from South America joined the Spanishlanguage webinars in October and November run in conjunction with FACT. A 'refresher' webinar on the accreditation process was run for German inspectors in November and JACIE was also represented in a number of other events and projects throughout the year (See figure 4).

#### Other

The JACIE website recorded 18,841 unique visitors, a 10% rise over 2015. All EBMT Newsletters now incorporate a JACIE section. The JACIE Twitter account @JACIE\_EBMT grew to 415 followers.

EBMT through JACIE, represented by Eoin McGrath, was invited to form part of the External Assessment Board of the Vigilance and Inspection for the Safety of Transfusion, Assisted Reproduction and Transplantation Joint Action (www.vistart-ja.eu) of the EU. Eoin also participates in the same capacity for the ARTHIQS Joint Action (www.arthiqs.eu). EBMT experts continued to work on the new guide to the Quality and safety of tissues and cells for human application published by the European Directorate for the Quality of Medicines and Healthcare (EDQM) which is expected to be released in early 2017.

The JACIE Office team expanded to four with the incorporation of Áurea Villar in a new position of HR (Volunteers) Coordinator. Áurea is responsible for the recruitment and retention strategy for JACIE Inspectors.

As ever, we would like to express our appreciation and admiration for the Inspectors, fellow 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

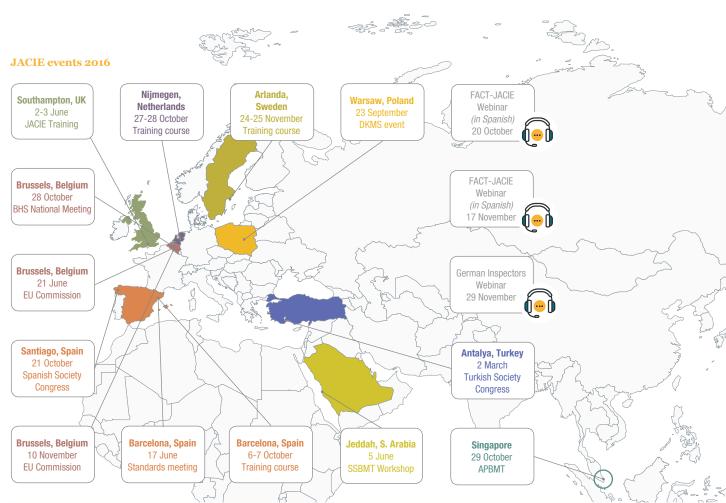


Figure 5.
Participants at the JACIE Training Course, Nijmegen, The Netherlands



#### **About JACIE**

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

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

Since 2000, 399 transplant programmes and facilities in 26 countries in Europe and beyond have applied to JACIE and 544 inspections (first-time and reaccreditation) have been performed. Over 300 applicants have achieved accreditation at least once with practically all centres repeating the process after completing the first accreditation cycle. There are over 260 registered inspectors, all volunteers drawn from the cellular therapy field.

#### Institutions awarded accreditation in 2016

Ziekenhuis Netwerk Antwerpen (Antwerp, Belgium); UZ Brussels (Brussels, Belgium): Grand Hôpital de Charlerroi (Charleroi, Belgium): CHU UCL Namur (Yvoir, Belgium); Turku University Central Hospital (Turku, Finland); CHU Amiens (Amiens, France); CHRU Besançon & EFS Bourgogne Franche-Comté (Besançon, France); CHU Brest & Etablissement Français du Sang (Brest, France); CHU Estaing of Clermont Ferrand (Clermont Ferrand, France); CHU Limoges & EFS - Aquitaine Limousin (Limoges, France); Hospital TENON - Assistance Publique-Hopitaux de Paris (Paris, France); Insitute for Transfusion Medicine Dessau (Dessau-Roßlau, Germany); Center for Pediatrics and Adolescent Medicine, Medical Center University of Freiburg (Freiburg, Germany); Universitätsklinikum Freiburg (Freiburg, Germany); Bayerische Stammzellbank gGmbH (Gauting, Germany); Einrichtung für Transfusionsmedizin am Universitätsklinikum Halle (Halle, Germany); Universitätsklinikum Halle (Halle, Germany); Universitätsklinikum Hamburg-Eppendorf (Hamburg, Germany); Staedtisches Klinikum Karlsruhe (Karlsruhe, Germany); Universitätsklinikum Magdeburg A. ö. R (Magdeburg, Germany); Klinik für Kinder- und Jugendmedizin -Pädiatrische Hämatologie und Onkologie-Universitätsklinikum Münster (Münster, Germany); DRK-Blutspendedienst NSTOB Institut Bremen-Oldenburg (Oldenburg, Germany); Azienda Ospedaliera Papa Giovanni XXIII (Bergamo, Italy); Azienda Sanitaria dell'Alto Adige (Bolzano, Italy); Azienda Spedali Civili Brescia (Brescia, Italy); Ospedale Civile di Civitanova Marche Area Vasta 3 Marche (Civitanova Marche (Macerata), Italy); Azienda Ospedaliero Universitaria di Ferrara (Ferrara, Italy); Dipartamento Oncologico III La Maddalena (Palermo, Italy); Fondazione IRCCS Policlinico San Matteo (Pavia, Italy); Fondazione IRCCS Policlinico San Matteo (Pavia, Italy); Fondazione IRCCS Policlinico San Matteo (Pavia, Italy); Azienda Ospedaliera di Perugia (Perugia, Italy); Azienda Ospedaliera Regionale San Carlo (Potenza, Italy); Ospedale Pediatrico Bambino Gesù (Rome, Italy); Policlinico Umberto I (Rome, Italy); Policlinico Umberto I (Rome, Italy); Presidio Ospedaliero "S.G Moscati" (Taranto, Italy); Ospedale San Bortolo - Vicenza (Vicenza, Italy); American University of Beirut Medical Center (Beirut, Lebanon): Medisch Spectrum Twente (Enschede, Netherlands); Radboud University Nijmegen Medical Centre (Nijmegen, Netherlands); Erasmus MC (Rotterdam, Netherlands); Princess Máxima Center (Utrecht, Netherlands); King Faisal Specialist Hospital & Research Centre (Riyadh, Saudi Arabia); Singapore General Hospital (Singapore, Singapore); Institut Català d'Oncologia - Badalona (Badalona, Spain); Hospital Universitario de Gran Canaria Dr. Negrín (Gran Canaria, Spain); Hospital General Universitario Gregorio Marañon (Madrid, Spain); HOSPITAL UNIVERSITARIO DE LA PRINCESA (Madrid, Spain); Hospital Universitario Fundación Jiménez Diaz IDC Salud (Madrid, Spain); Hospital Universitario Ramon v Caial (Madrid, Spain): Hospital Universitario Virgen Del Rocío (Sevilla, Spain); Hospital Clinico Universitario de Valencia (Valencia, Spain); Drottning Silvias Barn och Ungdomssjukhus (Göteborg, Sweden); Karolinska University Hospital, Huddinge (Stockholm, Sweden); Akademiska Sjukhuset (Uppsala, Sweden); Luzerner Kantonsspital (Luzern, Switzerland); Universitätsspital Zürich (Zürich, Switzerland); Baskent University Adana Hospital, Teaching and Medical Research Center (Adana, Turkey); Gloucestershire Hospitals NHS Foundation Trust (Cheltenham, United Kingdom); Dudley Group of Hospitals NHS Trust (Dudley, United Kingdom); Western General Hospital & Scottish National Blood Transfusion Service (SNBTS) (Edinburgh, United Kingdom); Barts Health NHS Trust (London, United Kingdom); St. George's University Hospitals NHS Foundation Trust (London, United Kingdom); Central Manchester University Hospitals NHS Foundation Trust (Manchester, United Kingdom); Royal Manchester Children's Hospital (Manchester, United Kingdom); The Christie NHS Foundation Trust (Manchester, United Kingdom); John Radcliffe Children's Hospital (Oxford, United Kingdom); NHS Blood and Transplant (Oxford, United Kingdom); Oxford University Hospitals NHS Trust / Buckinghamshire Healthcare NHS Trust (Oxford, United Kingdom); Biovault Technical Ltd (Plymouth, United Kingdom); The Great Western Hospital NHS FT (Swindon, United Kingdom);

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

# **JACIE Inspectors Quotes**



#### **Richard Willfred Olaussen**

Consultant, Section for Cell Therapy, Department of Oncology at Oslo University Hospital, Norway

As a new JACIE inspector in 2016, I have had the delightful privilege of taking part in two inspections as both a trainee and inspector. After many years' experience in quality management inspections of blood banks, JACIE represented something substantially new in formal quality assessment. The JACIE quality management system incorporates and integrates GMP production-standards with well-established clinical practice in a non-intrusive, self-explanatory and acceptable way, which all professionals would recognize and consent to. The system extends modern manufacturing principles of cellular products into the clinical realm in a supportive, inspiring and trustworthy way that provide thorough confidence in the entire process involved in cell therapy management. All aspects of the system are carefully considered and continuously updated based on experiences from practitioners and regulatory bodies. The follow-up and friendly support from JACIE at all stages is indeed helpful. Thus I'm very proud to be part of such an organization.

#### **Hellmut Ottinger**

Clinical Coordinator of the KMT Klinik of Essen University Hospital, Germany

Beside my clinical engagement I hold a double commitment: Since almost 10 years I am the JACIE Representative for Germany and – beside Prof. Martin Bornhäuser from Dresden – one of the most active Module-B (Clinical) JACIE inspectors in my country (4 inspections per year as Team Leader in 2015 and 2016). In Germany JACIE accreditation for transplant centers is not requested by law and not affiliated with financial reimbursement. Nevertheless, for me, there is no doubt that the JACIE Quality Management system is the best being available for Germany. Thus, for me it is a continuous challenge to convince reluctant program directors of German HCT units to join JACIE, and I am always happy if I win. Being active as a JACIE Inspector results in considerable work load in my rare free time, but it is worthwhile. No one more than I has had the opportunity to look into "the kitchen" of so many German transplant units, and my conclusions were always positive: No rivalry between German HCT units makes sense. We are all facing the same problems. Every JACIE Inspection Exit Meeting should and could define the needs of the inspected unit for further development, e.g. the need of a full time employed QM designee. In dealings with a local administration, external inspectors may be more mighty with their recommendations than local program directors. A positive inspection by a JACIE inspector from a different unit may be the base for cooperation of the inspected center in a multicenter clinical study in the field of HCT.

#### **Eva Alonso**

Hospital Germans Trias i Pujol (HGTiP) Badalona, Barcelona, Spain

When the Inspection day arrived, I was totally calm having completed all of my preparations. The only thing left was to observe how the expert in my speciality was performing the inspection in order to learn as much as possible from him and from the centre. The atmosphere during the inspection both among the inspectors during the different phases of the inspection and with our colleagues at the hospital was very positive. This helped me to feel really comfortable and perform my tasks properly.

I am grateful to the JACIE office team for their support at all times, to the inspector who I accompanied for his empathy, humility and kindness, and to all the inspectors for those 'coffee-break moments' where we discussed critical points and had some enriching discussions. Finally I am thankful to the transplant team for their cordiality and kindness that really facilitated the execution of the inspection - I felt at home from the very first moment!



# Financial highlights



**Jürgen Kuball** EBMT Treasurer

The EBMT is maintaining its high standard of modern management and has, for the second time, obtained an "unqualified opinion" (see below). We can be proud! This demonstrates that the EBMT is improving its financial stability and provides assurance that money is spent and allocated according to our mission.

What is an "unqualified opinion"? It is an independent auditor's judgment that a company's financial records and statements are fairly and appropriately presented, and in accordance with Generally Accepted Accounting Principles (GAAP).

## EBMT has closed for a third consecutive year with a positive result.

This improvement of our financial outcome in the last years is due to:

- better results on the income from the Annual Meeting
- a better capacity of the organisation to be in full control and follow up on its activities.

# **Spending our money** on our mission

In 2016, 82% of EBMT's expenses have been dedicated and allocated to its mission (studies, registry, accreditation and education) and the remaining 18% allocated to management (board and executive office expenses).

# **Professionalising** educational events

As part of our mission to professionalise all our educational events, the EBMT Board has taken the strategic decision to start a process to fully in-house all education events including the Annual Meeting management until 2020. As a first step, all accounting and financial flows have been centralised within the EBMT and plans are underway to further invest in a team to support all educational events.

# **Investing in "structural innovation"**

Over the last few years and in 2016, the board has earmarked more than 300k€ Euros per year for structural innovation. As a consequence of this strict financial policy, we have achieved earlier than anticipated a major financial milestone, which has allowed us to commit to and start on the urgently needed new registry. Consequently our budget for "structural innovation" will from now on not only be dedicated to the registry but also open for other innovative tools, which need a startup funding such as new educational tools.

#### Stay connected with EBMT

Part of our positive outcome will be used to upgrade our appearance in the World Wide Web during 2017. This new web appearance will also allow EBMT to integrate future educational tools.

#### **Further professionalising JACIE**

JACIE is an ongoing success story of EBMT. In order to keep up with our success and the increasing numbers of accreditations, as well as all new developments in quality management, we plan to strengthen our JACIE team in Barcelona as well as improve support to our valuable inspectors. In addition, EBMT will embark on supporting a project to better allow benchmarking for centers. However, this further professionalisation has meant an increase in accreditation fees, which has been approved by the Board of Association and will be implemented in 2017.

#### Tax control framework

Since 2014, EBMT is improving the tax management and will further implement a new tax framework into the financial policy of the EBMT in 2017 with our partner E&Y in order to allow a thorough discussion with all local tax authorities.

#### **Financial conclusion**

In 2016, EBMT has successfully achieved full control of all financial aspects, which will pave the way forward in 2017 as EBMT embarks on long-term obligations such as the new registry, new webpage and JACIE. With this full control of all financial aspects, the Board has agreed to invest 1,200 k€ in the new registry, with the project starting in 2017. EBMT will be closing the year 2016 with a total expenses of of 2,907 k€ and a total income of 4,654 k€, which means a total positive balance of 1,747 k€. The net profit of 1,747 k€ will be returned to the reserves in order to consolidate further the financial reserves of the organisation as well as serve the mission of the EBMT. This includes mitigating financial risks during the development of the new registry, investing in future "structural innovations", and professionalising educational tools.

For more information regarding the Audit report please visit www.ebmt.org

# Source of income during 2016

# Destination of resources during 2016



**€635,000** 14%

**EBMT Members donations** 



**€650,000** 14%

Scientific Sponsors donations



**€1,947,000** 42%

Annual Meeting financial result



**€960,000** 20%

Grants for Studies, Clinical Trials & Education



**€462,000** 10%

Accreditation (JACIE)



**€1,377,000** 47%

Scientific Studies



**€303,000** 1

**EBMT** Registry



**€317,000** 11%

**Educational Activities** 



**€391,000** 14%

Standards & Accreditation (JACIE)



**€2,388,000** 82%

**EBMT Total Mission Cost** 



**€519,000** 18%

Management & Administration



**€4,654,000** 100%

**Total Income** 



**€2,907,000** 100%

**Total Expenses** 



Christian Chabannon

Gérard Michel

Thank you to the following people for providing the necessary permission and organising the photoshoot:

- Professors Christian Chabannon, Didier Blaise and team members at Institut Paoli-Calmettes, comprehensive cancer center.
- Professor Gérard Michel, Ms Marie Angéli and team members at La Timone University Hospital (AP-HM), all in Marseille, France.

