





Annual Report /15

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OUR MISSION

The EBMT is a not-for-profit medical and scientific organisation established in 1974. It is dedicated to fighting life-threatening blood cancers and diseases and improving patients' lives.

EBMT members—more than 4,000 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 600 centres from over 60 countries, that perform or are involved in HSCT. The EBMT holds a central role in performing co-operative studies and disseminating state-of-the-art knowledge: the aim is to increase survival rates and enhance the quality of life of patients with life-threatening blood cancers and diseases.

OUR 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

Message from the President



“Our greatest challenge for the near future will be to refine the role of stem cell transplantation with or without novel agents, with the aim to cure diseases.”

One more year has passed and the new EBMT Annual Report is already here! When I look back, I reasonably consider that 2015 was a remarkable year for the EBMT. I am happy and thankful to all EBMT members, leaders, and staff for their strong support and commitment. Time runs very quickly. So, what have we done this year? Well, I could spend hours describing all of our activities, but I do not want this to become a very boring introduction.

The latest EBMT accomplishments are outlined in the following pages of this Annual Report and so I will not repeat them here. Nevertheless, I would like to stress that the retrospective database of the EBMT is an essential scientific backbone. A recent effort to establish a successor for ProMiSe has failed. Lessons have been learned! In addition novel challenges are arising such as developing a database for cellular therapies as well as linking clinical data to biobanking for defined subset of patients arising from defined EBMT activities. The Board decided to initiate a project tackling these novel technical and financial challenges called “Project 2020”. This project aims to implement a novel Registry system by 2020.

The EBMT continues to make strong and significant contributions to the fields of haematology and stem cell transplantation.

Over the past five years, haematology has been reinventing itself as a discipline at the forefront of medical innovation. As I am writing this, I am thinking about how dynamic our field is.

It is no secret that the haematology and transplant fields have changed drastically over the years. Many of the historical so-called “standard of care” approaches are being challenged and will be likely retiring in the near future! It is out with the old and in with the new!

More than four decades ago, EBMT pioneers revolutionised the medical field by introducing bone marrow transplantation into routine practice. Today, we are reinventing that milestone by pursuing the path of innovation for the benefit of our patients. The advent of newer transplant techniques and novel immunotherapy strategies are taking it one step further for those patients who are most in need. The unique position of stem cell transplanters provides a high-end opportunity for the development of such novel cellular therapies. Our greatest challenge for the near future will be to refine the role of stem cell transplantation with or without novel agents, with the aim to cure diseases. At the EBMT we are committed to designing effective and affordable treatment strategies for all patients who put their lives in our hands. The EBMT has a rich history of success, serving patients with yet incurable diseases, and we are all proud of being part of that legacy. It is a true pleasure and honour to engage the transplant community in the exploration of new frontiers and in advancing the boundaries of haematology.

Please enjoy reading this report, and do not forget to follow the EBMT news on Twitter @TheEBMT and @Mohty_EBMT.

Mohamad Mohty
EBMT President

EBMT structure

BOARD

Executive Committee

President, Secretary, Treasurer

Scientific Council Chair

Nicolaus Kröger

Scientific Council Co-Chair

Rafael Duarte

Scientific Council Education Representative

Dominique Farge Bancel

Scientific Council Registry Representative

Peter Dreger

Nurses Group President

Aleksandra Babic - Milan, Italy

Congress President

Miguel A. Sanz - Valencia, Spain

EXECUTIVE COMMITTEE

President

Mohamad Mohty - Paris, France

Secretary

Anna Sureda - Barcelona, Spain

Treasurer

Jürgen Kuball - Utrecht, The Netherlands

SCIENTIFIC COUNCIL - WORKING PARTIES

Severe Aplastic Anaemia

Carlo Dufour - Genoa, Italy

Autoimmune Diseases

Dominique Farge Bancel - Paris, France

Acute Leukaemia

Arnon Nagler - Tel Hashomer, Israel

Cellular Therapy and Immunobiology

Chiara Bonini - Milan, Italy

Infectious Diseases

Simone Cesaro - Verona, Italy

Inborn Errors

Andrew Gennery - Newcastle-Upon-Tyne, UK

Lymphoma

Peter Dreger - Heidelberg, Germany

Paediatric Diseases

Peter Bader - Frankfurt, Germany

Solid Tumours

Francesco Lanza - Cremona, Italy

Chronic Malignancies

Nicolaus Kröger - Hamburg, Germany

Complications and Quality of Life

Rafael Duarte - Madrid, Spain

COMMITTEES

Nuclear Accident

Ray Powles - London, UK

CT2

Charles Craddock - Birmingham, UK

Statistical

Myriam Labopin - Paris, France

JACIE

John Snowden - Sheffield, UK

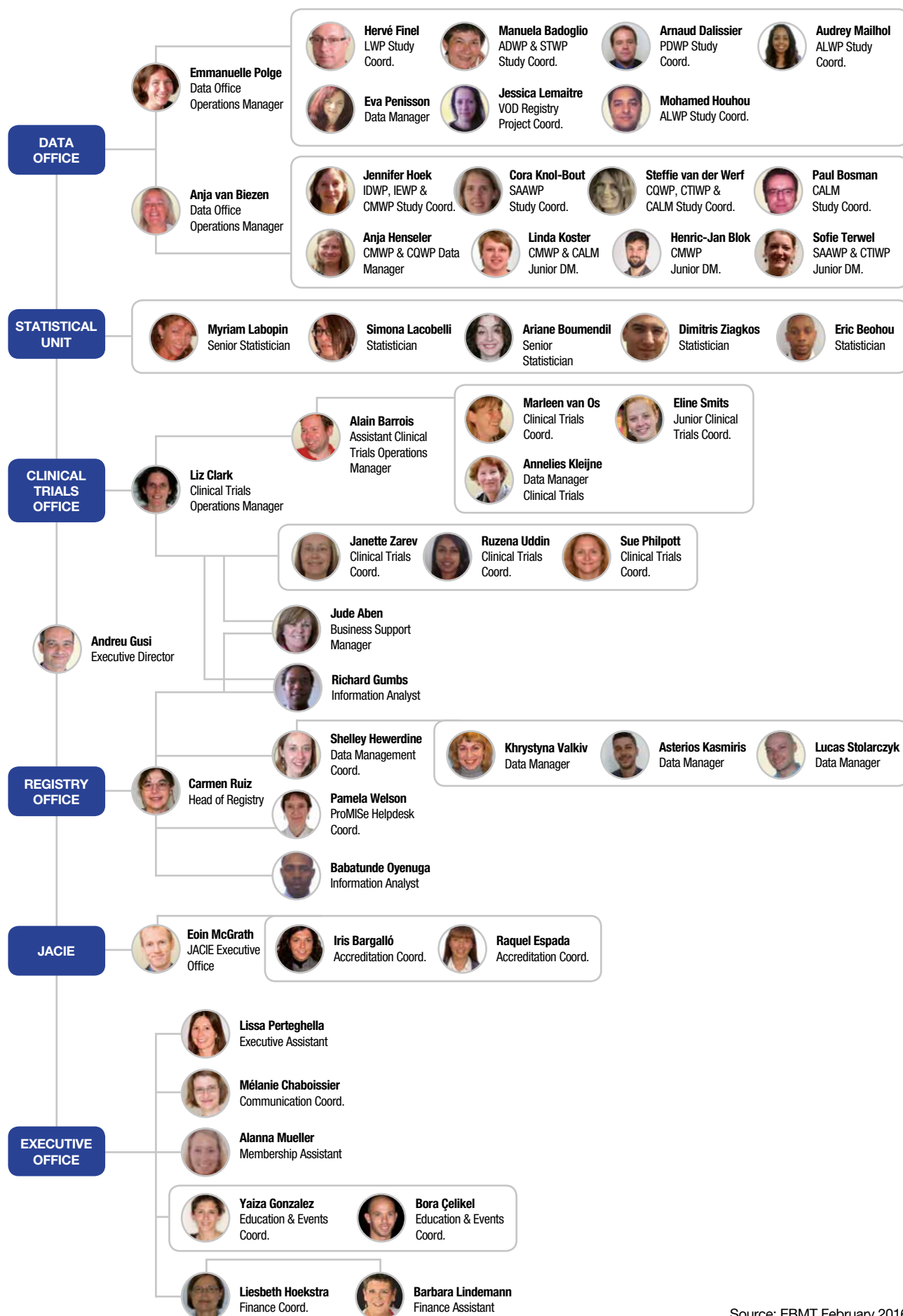
Donor Outcomes

Joerg Halter - Basel, Switzerland

Registry Committee

Per Ljungman - Stockholm, Sweden

Staff organisational chart



Source: EBMT February 2016

EBMT membership



The EBMT has a total of 4,740 members as of February 2016. Thirty-six new members joined the EBMT in 2015. The members belong to a unique collaborative network of peers involved in HSCT 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:



Total: 4,740

Source: ProMIs Feb. 2016

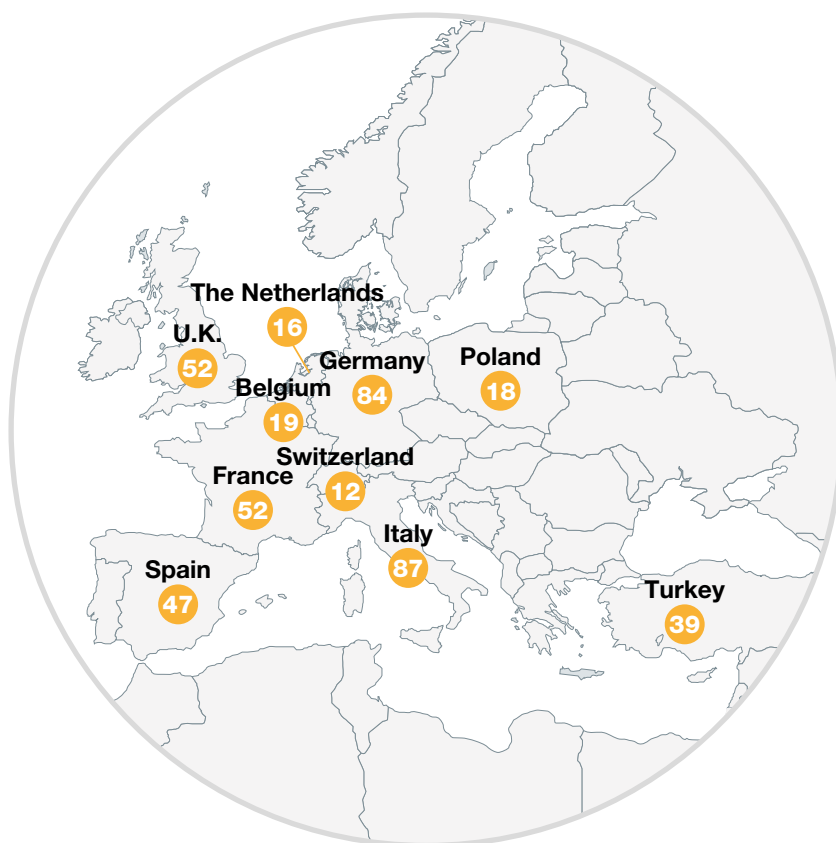
Our members can be classified as centre members or individual members.

Furthermore, our centre members can be divided into two categories: full members, or associate members. Our full members generously commit to submitting data on all patients treated in their centres on an annual basis, and enjoy the benefits of voting rights and eligibility for JACIE accreditation.

Provisional centres are new members which are pending approval at the General Assembly Meeting during the EBMT Annual Meeting.



Source: ProMIs Feb. 2016



Our 568 centre members are located in 55 different countries. Italy, Germany, UK, France, Spain, Turkey, Belgium, Poland, The Netherlands and Switzerland are the top 10 countries in terms of number of centres participating in the EBMT.

Membership benefits

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

- Eligibility to elect and stand as Board members
- Participation in EBMT studies
- Eligible for JACIE accreditation
- Access to the EBMT Registry
- Eligible for reduced fees to attend the EBMT Annual Meeting
- Discounts on subscriptions to the official journal of the EBMT, *Bone and Marrow Transplantation*.

Anna Sureda
EBMT Secretary

Joining the EBMT

Centres or independent persons that are active in the field of transplantation and 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. For any queries about EBMT membership, please contact: membership@ebmt.org or visit www.ebmt.org



Sciences



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The scientific activity reports



It is a great honour and pleasure to introduce the 2015 Scientific Reports of the EBMT, summarising the scientific activities and achievements of our Society over the last 12 months.

As you can see in the figures and in the detailed reports of the individual Working Parties (WPs) the activity and productivity of the EBMT are rapidly growing, reflecting the high enthusiasm and commitment of our members.

The EBMT aims to enable scientists and physicians involved in stem cell transplantation and cellular therapies to share their experience and develop cooperative studies. The EBMT coordinates its scientific activities through 11 WPs and the Nurses Group. The 11 WPs represent the Scientific Council. Some WPs are disease-related such as Acute Leukaemia, Chronic Malignancies, Autoimmune Diseases, Severe Aplastic Anaemia, Inborn Errors, Lymphomas, and Solid Tumours, while other WPs are more transversal-orientated such as Cellular Therapy and Immunobiology, Infectious Diseases, Complications and Quality of Life, and Paediatric Diseases. Through these WPs different types of studies are performed such as:

1. Registry studies derived from retrospective data collected from the EBMT registries
2. Non-interventional cohort studies
3. Interventional or prospective studies

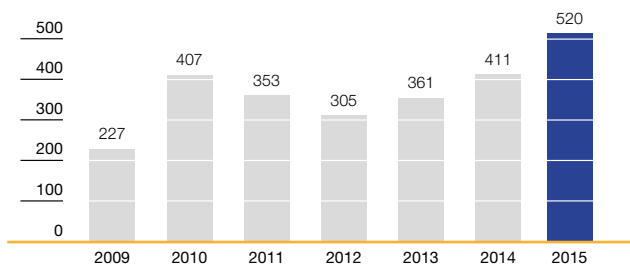
The studies conducted by the EBMT yield a significant amount of scientific knowledge, creating the basis for continual improvement of patient care. Beside these scientific activities, by performing studies there is increased activity in all WPs regarding educational events, which help to disseminate the newest developments and results throughout the scientific community. All these activities and achievements would not be possible without the support and commitment of all EBMT members and centres that timely report their data to the EBMT.

On behalf of the entire EBMT Scientific Council I would like to thank you all for the continuous support and encourage you and the members of your team to join one of the WPs, the regular meetings, and to actively participate in the scientific projects. I hope you will enjoy reading the 2015 scientific activity reports.

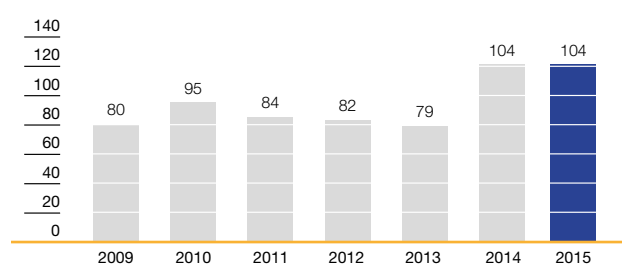
Best regards,

Nicolaus Kröger
Scientific Council Chair

Impact factor



Oral presentations



Severe Aplastic Anaemia Working Party (SAAWP)

Chair: **Carlo Dufour**



Major achievements

Starting the recruitment of two prospective randomised clinical trials:

1. The RACE study that compares standard IST (immunosuppressive treatment) (ATG+CsA) plus eltrombopag vs. standard IST alone. This EBMT study is financially supported by GSK, Pfizer and Alexion Pharmaceuticals. Thirty-seven EBMT centres will enroll patients. Five centres have already opened and 13 patients have been enrolled since recruitment began July 1st 2015.
2. The Moderate Aplastic Anaemia (MAA) study that compares Cyclosporin A (CsA) plus placebo vs. CsA plus eltrombopag in moderate aplastic anaemia. This study is 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. The first patients have already been recruited in the German centres.

Completing the first draft of the Bone Marrow Failure Textbook. 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.

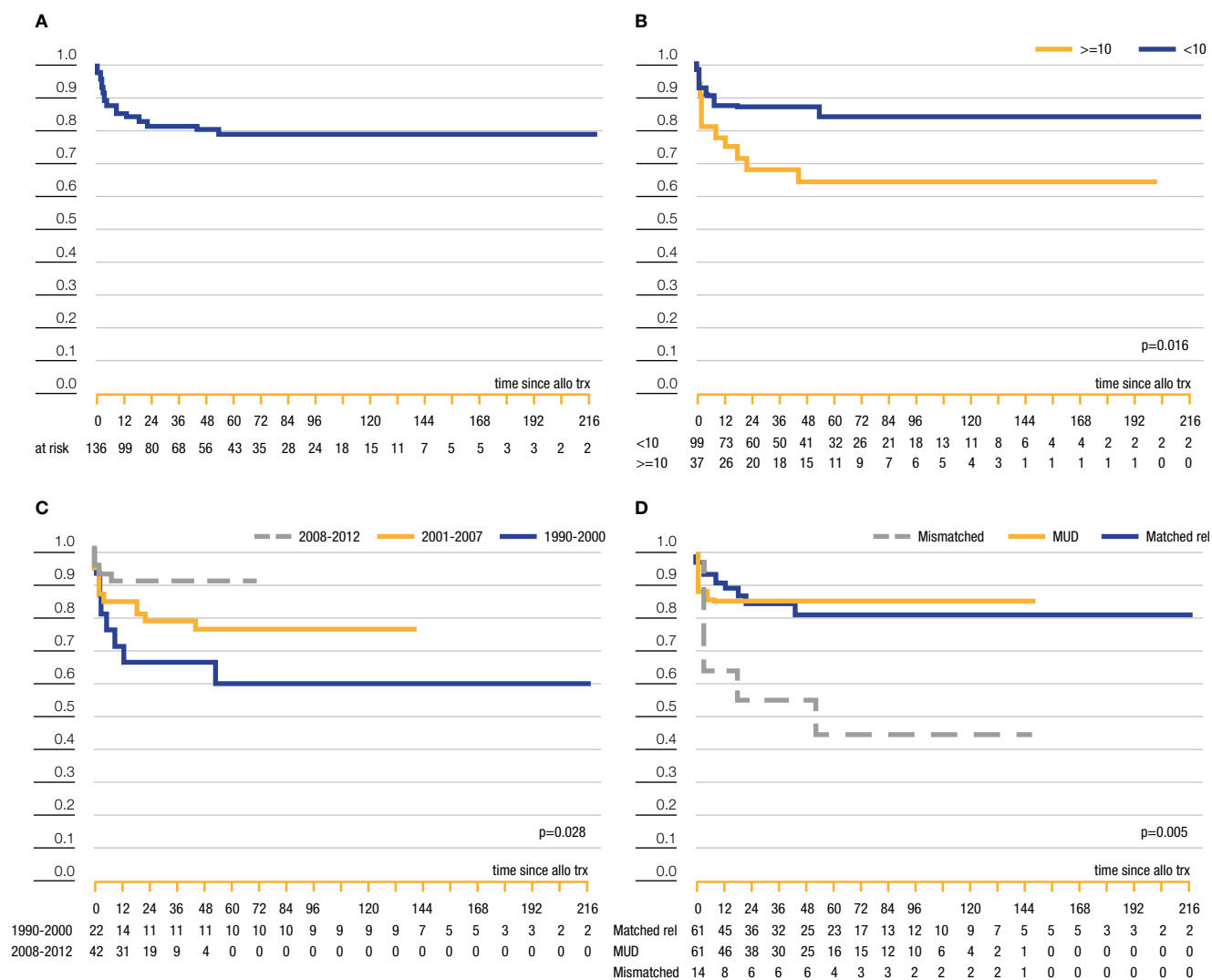
Principal research studies

1. Outcome of HSCT in 87 Dyskeratosis congenita patients. This is the largest cohort ever studied.
2. Campath vs. ATGAM in HSCT for aplastic anaemia
3. Outcome of HSCT in Fanconi Anaemia >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. Autoimmune phenomena post HSCT for aplastic anaemia in collaboration with the Autoimmune Diseases Working Party
5. HSCT in Pure Red Cell Aplasia (PRCA)

Key publications

1. Stem cell transplantation in severe congenital neutropenia: an analysis from the European Society for Blood and Marrow Transplantation. Fioredda F, *Blood* 2015 Oct 15
2. Current outcome of HLA identical sibling vs. unrelated donor transplants in severe aplastic anemia: an EBMT analysis. Bacigalupo A, *Haematologica* 2015 May
3. Similar outcome of upfront-unrelated and matched sibling stem cell transplantation in idiopathic paediatric aplastic anaemia. A study on behalf of the UK Paediatric BMT Working Party, Paediatric Diseases Working Party and Severe Aplastic Anaemia Working Party of EBMT. Dufour C, *Br J Haematol.* 2015 Nov
4. Outcome of aplastic anaemia in children. A study by the severe aplastic anaemia and paediatric disease working parties of the European group blood and bone marrow transplant. Dufour C, *Br J Haematol.* 2015 May
5. Second allogeneic stem cell transplant for aplastic anaemia: a retrospective study by the severe aplastic anaemia working party of the European society for blood and marrow transplantation. Cesaro S, *Br J Haematol.* 2015 Nov

Figure 1. Referring to publication n°1

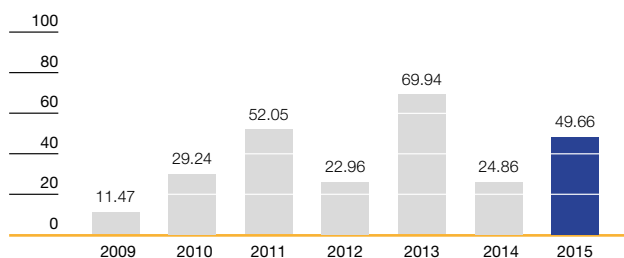


Overall Survival (OS) of Severe Congenital Neutropenia patients undergoing HSCT.

(A) OS. (B) OS by age. (C) OS by calendar period. (D) OS by donor type.

The curves in panels C and D are shown for the maximal follow-up and were not statistically analysed past the point of <5 patients. Mismatched includes both related and unrelated donors.

Impact factor (Aplastic Anaemia)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 6 | 9 | 10 |
| Poster Presentations | 8 | 1 | 5 |
| International Educational Events | 0 | 1 | 0 |

Autoimmune Diseases Working Party (ADWP)

Chair: **Dominique Farge-Bancel**



Major achievements

Over the last 20 years we have developed the largest database worldwide for autoimmune diseases with more than 2,000 cases reported. Since 2010, the yearly number of HSCT procedures registered has increased by 30%, reflecting a change in practice and the active role of all ADWP members.

In 2015, we completed the proof of efficacy for HSCT in multiple sclerosis and Crohn's disease with the publication of two major ADWP randomised trials (i.e. ASTIMS and ASTIC) in collaboration with other European Societies (i.e. ECTRIMS and ECCO), thus completing the previous achievement of the ASTIS trial in 2014 for systemic sclerosis. The efficacy of autologous HSCT is now established for AD.

The ADWP has continued to expand its activities using other types of immune-modulating cells (e.g. MSCs) from various sources of blood products (i.e. bone marrow, peripheral blood or cord blood, and placenta) either in the autologous or allogeneic settings. Refined analysis of immune reconstitution processes has confirmed the reset of autoimmunity after HSCT in AD.

Sustained positive clinical results and enhanced ADWP activity in otherwise refractory AD patients has attracted sustained and increased interest from patients, clinicians, and healthcare providers in the field.

It is now essential to adapt the ADWP clinical databases to biobanking performed in expert centres and to develop other types of cell therapy for various types of AD.

Finally, in 2015 the ADWP organised two educational and business meetings (see below).

Principal research studies

1. Autologous stem cell transplantation for progressive systemic sclerosis: a prospective non-interventional study across Europe (NIISC)
2. Does CD34+ selection change the outcome of HSCT in scleroderma patients?

3. Long-term outcomes after autologous haematopoietic stem cell transplantation for multiple sclerosis. A joint study from the Center for International Blood and Marrow Transplant Research (CIBMTR) and from the European Society for Blood and Marrow Transplantation (EBMT)
4. Autologous haematopoietic stem cell transplantation (aHSCT) for paediatric multiple sclerosis

Original Investigation

Autologous Hematopoietic Stem Cell Transplantation for Refractory Crohn Disease A Randomized Clinical Trial

Christopher J. Hawkey, FMedSci, Matthew Allez, PhD, Miranda M. Clark, BSc(Hons), Myriam Labopin, MD, James O. Lindsay, PhD, Elena Sturt, PhD, Gerhard Rogler, PhD, Montserrat Bovera, MD, Jack Satsangi, DPhil, Silvio Danese, PhD, Nigel Russell, MD, John Grisham, MD, Peter Johnson, MD, Jerome Larghero, MD, Catherine Thiebaut, PhD, Sandro Ardizzone, MD, Dean Dierckx, PhD, Adalberto Ibarici, MD, Timothy Littlewood, MD, Francesco Onida, MD, Urs Schanz, MD, Severine Vermeire, PhD, Jean-Frédéric Colombel, MD, Jean-Paul Jouxet, MD, Elizabeth Clark, MSc, Riccardo Saccardi, MD, Alan Tyndall, FRACP, Simon Travis, DPhil, Dominique Farge, PhD

IMPORTANCE Case reports and series suggest hematopoietic stem cell transplantation (HSCT) may benefit some patients with Crohn disease.

OBJECTIVE To evaluate the effect of autologous HSCT on refractory Crohn disease.

DESIGN, SETTING, AND PARTICIPANTS Parallel-group randomized clinical trial conducted in 11 European transplant units from July 2007 to September 2011, with follow-up through March 2013. Patients were aged 18 to 50 years with impaired quality of life from refractory Crohn disease not amenable to surgery despite treatment with 3 or more immunosuppressive or biologic agents and corticosteroids.

INTERVENTIONS All patients underwent stem cell mobilization before 1:1 randomization to immunoblation and HSCT (n = 23) or control treatment (HSCT deferred for 1 year [n = 22]). All were given standard Crohn disease treatment as needed.

MAIN RESULTS AND MEASURES Sustained disease remission at 1 year, a composite primary end point comprising clinical remission (Crohn Disease Activity Index [CDAI] <150 [range, 0-600]), no use of corticosteroids or immunosuppressive or biologic drugs for at least the last 3 months, and no endoscopic or radiological evidence of active (erosive) disease anywhere in the gastrointestinal (GI) tract. Secondary outcomes were individual components of the primary composite outcome and other measures of disease activity, laboratory results, quality of life and functional status, and GI tract imaging.

RESULTS Twenty-three patients underwent HSCT and 22 received standard treatment (controls). There were no statistically significant between-group differences in proportions of patients achieving sustained disease remission, CDAI less than 150 in the last 3 months, or freedom from active disease; there was a statistically significant difference among patients able to discontinue active treatment in the last 3 months. There were 76 serious adverse events in patients undergoing HSCT vs 38 in controls; 1 patient undergoing HSCT died.

| | No. (%) | HSCT | Control | Difference (95% CI), % | P Value |
|-----------------------------|-----------|----------|--------------------|------------------------|---------|
| Sustained disease remission | 2 (8.7) | 1 (4.3) | 1 (4.5) | 4.2 (-14.2 to 22.4) | .60 |
| Secondary outcomes | | | | | |
| No active treatment | 14 (60.9) | 5 (21.7) | 38.1 (9.3 to 59.3) | | .01 |
| CDAI <150 | 8 (34.8) | 7 (30.4) | 25.7 (1.1 to 47.1) | | .052 |
| Free of active disease | 8 (34.8) | 2 (8.7) | 25.7 (1.1 to 47.1) | | .054 |

CONCLUSIONS AND RELEVANCE Among adult patients with refractory Crohn disease not amenable to surgery who had impaired quality of life, HSCT, compared with conventional therapy, did not result in a statistically significant improvement in sustained disease remission at 1 year and was associated with significant toxicity. These findings do not support the widespread use of HSCT for patients with refractory Crohn disease.

TRIAL REGISTRATION clinicaltrials.gov Identifier: NCT00297193
JAMA. 2015;314(23):2534-2534. doi:10.1001/jama.2015.36700

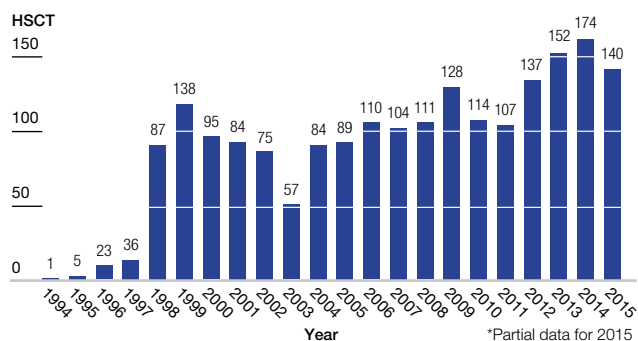
Supplemental content at jama.com

CME Quiz at jamanetwork.com and CME Questions page 2560

Author Affiliations: Author affiliations are listed at the end of this article.

Corresponding Author: Christopher J. Hawkey, FMedSci, Nottingham Digestive Diseases Centre, School of Clinical Sciences, Queen's Medical Centre, Nottingham NG7 2UH, United Kingdom (c.j.hawkey@nottingham.ac.uk).

HSCT for AD per year (1994-2015) (n=2052)



Key publications

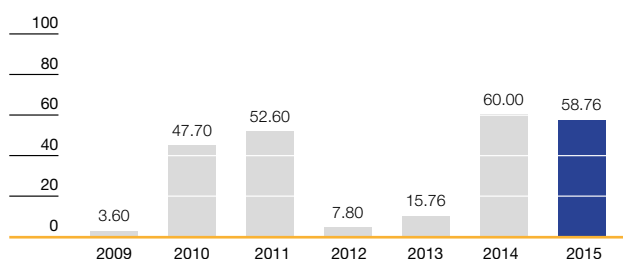
1. Autologous Hematopoietic Stem Cell Transplantation for Refractory Crohn Disease: A Randomized Clinical Trial. Hawkey CJ, *JAMA* 2015 Dec 15
2. Autologous hematopoietic stem cell transplantation in multiple sclerosis: a phase II trial. Mancardi GL, *Neurology* 2015 Mar 10
3. SCT for severe autoimmune diseases: consensus guidelines of the European Society for Blood and Marrow Transplantation for immune monitoring and biobanking. T Alexander, *Bone Marrow Transplant*. 2015 Feb
4. Autologous hematopoietic stem cell transplantation in neuromyelitis optica: a registry study of the EBMT Autoimmune Diseases Working Party. Greco R, *Mult Scler*. 2015 Feb

| | | | |
|-----------------------------|------------|---------------------------|-----------|
| MULTIPLE SCLEROSIS | 801 | HAEMATOLOGICAL | 97 |
| CONNECTIVE TISSUE | 595 | ITP | 31 |
| SSc | 433 | Evans' | 20 |
| SLE | 113 | AIHA | 25 |
| PM-DM | 18 | Other | 21 |
| Sjorgen | 3 | VASCULITIS | 49 |
| Antiphosph. Syndrome | 5 | Wegener's | 12 |
| Other/Unknown | 23 | Behcet's | 9 |
| ARTHRITIS | 184 | Takayasu | 2 |
| Rheumatoid arthritis | 85 | Microscopic poly. nodosa | 4 |
| Juvenile chronic arthritis: | | Classical poly. nodosa | 1 |
| Systemic JIA | 57 | Churg-Strauss | 2 |
| Other JIA | 18 | Other/Unknown | 20 |
| Polyarticular JIA | 14 | OTHER NEUROLOGICAL | 86 |
| Psoriatic arthritis | 4 | NMO | 22 |
| Other | 6 | CIDP | 31 |
| INFLAMMATORY BOWEL | 188 | Myasthenia gravis | 7 |
| Crohn's disease | 155 | Other/Unknown | 26 |
| Ulcerative colitis | 4 | INSULIN DEPENDENT | 20 |
| Other | 29 | DIABETES | |
| | | OTHER/Unknown | 32 |

Major educational courses

1. Workshop on Autoimmunity - March 23 2015 in Istanbul, Turkey
2. ADWP educational meeting on Stem Cell Therapy (HSCT & MSC) and Autoimmune Diseases - January 22-23 2016 in Berlin, Germany

Impact factor (Autoimmune Diseases)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 11 | 5 | 6 |
| Poster Presentations | 7 | 7 | 1 |
| International Educational Events | 1 | 1 | 1 |



Acute Leukaemia Working Party (ALWP)

Chair: **Arnon Nagler**



Major achievements

The ALWP had a very successful year in 2015. Acute leukaemia continues to be the number one indication for allogeneic stem cell transplantation (allo-SCT) in Europe. According to the recent report of the Transplant Activity Survey, out of 40,829 transplants performed annually in Europe, 33% (i.e. 11,853 transplants) are for leukaemias (96% allogeneic). Out of the total allogeneic transplantations, 36% are for acute myeloid leukaemia (AML) and 16% are for acute lymphoblastic leukaemia (ALL).

Numbers of T-cell repletion (T-replete) haploidentical allogeneic stem cell transplantations (Haplo-SCT) are increasing. Therefore, one of the ALWP main areas of interest in 2015 was comparing haplo-SCT for acute leukaemia to cord blood transplantation (CBT); assessing graft-versus-leukaemia (GVL) as well as the clinical relevance of human leukocyte antigen (HLA) (Lorentino F, oral session at the ASH meeting 2015). Other studies including optimal Graft versus Host Disease (GvHD) prophylaxis anti-thymocyte globulin (ATG) vs. post transplantation cyclophosphamide (PTCy); GvHD free relapse free survival (GRFS) post T-replete vs. T-deplete haplo-SCT and haplo-SCT for ALL are ongoing.

Other hot topics were:

- Allo-SCT in secondary leukaemias, comparing reduced intensity conditioning (RIC) to myeloablative conditioning (MAC) (Abstract in *Blood*, Savani B, Oral session at ASH 2015)
- Allo-SCT for refractory AML (*Lancet Hematology* 2015, Brissot et al., Oral session at ASH 2015)
- CBT RIC vs. MAC (Abstract in *Blood*, Baron F, Oral session at ASH 2015)
- Increased risk of GvHD with female cord blood unit (F CBU) to male host (Baron F, *J Hematol Oncol.* 2015)

We are also identifying overall survival (OS) predictors and related interactions in CBT using random survival forests in collaboration with Eurocord (Shouval R). 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. Jordie Esteve (Schmid C *Blood* 2015). As for ALL, with S. Giebel leading the ALL subcommittee, we have increased substantially our activities. We have been able to highlight that allo-SCT performed from unrelated donors in ALL have improved significantly over the last decade.

We also continue our educational activities and organised or contributed to some meetings focusing on relevant topics including haplo-SCT, relapse post allo-SCT and novel conditioning regimens.

Principal research studies

1. Myeloablative allogeneic stem cell transplantation for acute leukaemia. Outcomes after use of two standard ablative regimens in patients with refractory acute myeloid leukaemia: a retrospective, multicentre, registry analysis. Nagler A, *Lancet Haematol.* 2015
2. Identifying overall survival predictors and related interactions in umbilical cord blood transplantation using random survival forests: A Eurocord - Acute Leukaemia Working Party - Paediatric Diseases Working Party - EBMT Study. Shouval R, Ruggeri A, Gluckman E, Nagler A
3. Impact of conditioning regimen intensity on transplant outcome for secondary acute myeloid leukaemia. Savani B, Labopin M, Nagler A
4. Clinical relevance of HLA disparity on outcome after haploidentical haematopoietic stem cell transplantation in adults with de novo acute leukaemia. Lorentino F, Ciceri F, Mohty M, Nagler A

Key publications

1. Prediction of Allogeneic Hematopoietic Stem-Cell Transplantation Mortality 100 Days After Transplantation Using a Machine Learning Algorithm: A European Group for Blood and Marrow Transplantation Acute Leukemia Working Party Retrospective Data Mining Study. Shouval R, *J Clin Oncol.* 2015 Oct 1
2. Outcome of patients with distinct molecular genotypes and cytogenetically normal AML after allogeneic transplantation. Schmid C, *Blood* 2015 Oct 22
3. Comparison of outcomes after unrelated cord blood and unmanipulated haploidentical stem cell transplantation in adults with acute leukemia. Ruggeri A, *Leukemia* 2015 Sep
4. Is there a stronger graft-versus-leukemia effect using HLA-haploidentical donors compared with HLA-identical siblings? Ringdén O, *Leukemia* 2015 Aug 21

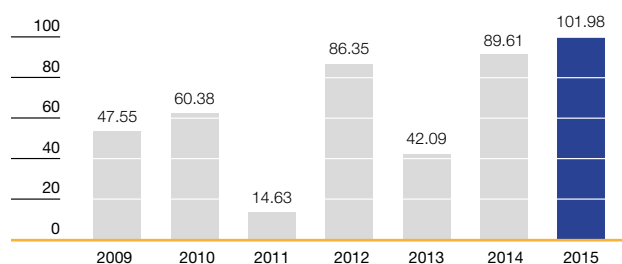


ALWP education course in Marseille

Major educational courses

1. Master Classes in Transplantation and Hematology (MATH)[®]: A Focus on Conditioning - January 30 2015 in Paris, France
2. Advances in Alternative Donor Stem Cell Transplantation: A Euro-Mediterranean Perspective - May 29–30 2015 in Marseille, France
3. 8th Educational Symposium of the Acute Leukaemia Working Party on “Reduction of leukemic relapse after ALLO-HSCT: How to move forward?” - November 27–28 2015 in Paris, France

Impact factor (Acute Leukaemia)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 9 | 18 | 22 |
| Poster Presentations | 13 | 13 | 15 |
| International Educational Events | 1 | 1 | 3 |

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 and to implement modern cellular therapies, based on cell and gene engineering approaches, to improve transplantation outcomes.

The CTIWP aims to foster cellular therapy in Europe, through continuous exchanges between basic science findings, transplant immunobiology observations and implementation of cellular manufacturing approaches designed to answer unmet medical needs.

In 2015 the CTIWP launched and implemented:

1. Two retrospective and two multicentric prospective transplant immunobiology studies.
2. An effort to upgrade the EBMT registry forms and improve the EBMT ability to collect additional and high-quality clinical and biological information from patients undergoing standard HSCT alone or combined with innovative cellular therapy and cell-based gene therapy. This is a joint effort with the Solid Tumours, Autoimmune Diseases, and Inborn Errors WPs.
3. An upgrade of the EBMT Transplant Activity Survey to include cellular therapy and gene therapy.
4. A survey on currently used practices for minimally-manipulated cell products, with a view to harmonise recommendations.

Several studies that have been proposed by CTIWP subcommittee leaders are currently starting and include a survey of high-resolution typing in the EBMT database regarding the use of unrelated donors, and a survey on mesenchymal stem cell manufacture harmonisation.

One of the most important missions of the CTIWP is to disseminate and discuss scientific results. In 2015 we hosted:

1. The 4th Cell Therapy Day (during the 41st EBMT Annual Meeting in Istanbul) in collaboration with the local organising committee: the CTIWP Scientific Symposium provided attendees with practical, regulatory and scientific background information for the optimisation of cell transplantation and the development, evaluation and implementation of innovative cellular therapies.
2. The 1st EBMT Cellular Therapy and Immunobiology Working Party Scientific Symposium entitled: "From Transplantation to Gene Therapy: Cellular therapy in evolution". This first meeting was a great success; outstanding speakers delivered excellent presentations for a mixed audience allowing junior investigators to easily and freely interact with leaders in the field.

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 (inherited/non inherited paternal and maternal antigen IPA/NIMA vs. NIPA/IMA mismatching in graft versus host (GvH) vs. host versus graft (HvG) directions) on clinical outcomes in haplo-identical transplantation
3. Non-interventional prospective study on the role of donors vs. recipient NK cell allo-reactivity in haplo-identical SCT
4. Non-interventional prospective study on recipient pre-transplant thymic function as a biomarker of transplant outcome after allo-HSCT



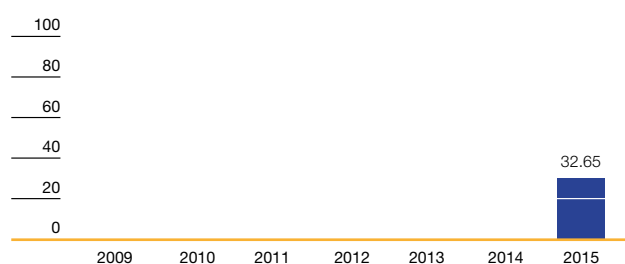
Key publications

1. Unrelated cord blood transplantation for adult patients with acute myeloid leukemia: higher incidence of acute graft-versus-host disease and lower survival in male patients transplanted with female unrelated cord blood-a report from Eurocord, the Acute Leukemia Working Party, and the Cord Blood Committee of the Cellular Therapy and Immunobiology Working Party of the European Group for Blood and Marrow Transplantation. Baron F, *J Hematol Oncol*. 2015 Oct 6
2. Unrelated cord blood transplantation for childhood acute myelogenous leukemia: The influence of cytogenetic risk group stratification. Michel G, *Leukemia* 2015 Sep 15
3. Outcomes of Cord Blood Transplantation Using Reduced-Intensity Conditioning for Chronic Lymphocytic Leukemia: A Study on Behalf of Eurocord and Cord Blood Committee of Cellular Therapy and Immunobiology Working Party, Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation, and the Société Française de Greffe de Moelle et Thérapie Cellulaire. Xavier E, *Biol Blood Marrow Transplant*. 2015 Aug
4. Comparison of outcomes after unrelated cord blood and unmanipulated haploidentical stem cell transplantation in adults with acute leukemia. Ruggeri A, *Leukemia* 2015 Sep

Major educational events

1. Activities during the 41st EBMT Annual Meeting, March 22–25 2015 in Istanbul, Turkey:
 - CTIWP Scientific Symposium - sponsored by Novartis
 - Jon J. Van Rood Award 2015 - sponsored by Neovii Pharmaceuticals
 - 4th Cell Therapy Day
2. The 1st CTIWP Scientific Symposium: “From Transplantation to Gene Therapy: Cellular therapy in Evolution” - November 11–13 2015 in Milan, Italy

Impact factor (Cell Therapy & Immunobiology)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 0 | 0 | 4 |
| Poster Presentations | 0 | 2 | 4 |
| International Educational Events | 1 | 1 | 1 |

Infectious Diseases Working Party (IDWP)

Chair: **Simone Cesaro**



Major achievements

In May 2015, the IDWP closed the prospective non-interventional study aimed at assessing the incidence of gram-negative bacteremia in the first six months after transplant, and among the isolated strains an evaluation of the rate of antibiotic resistant and multidrug resistant strains. This was a prospective, multinational study that will provide important epidemiological information on this important topic to the EBMT centres. The preliminary analysis on more than 485 episodes of gram-negative bacteremia collected from 444 patients coming from 58 centres and 28 different countries will be presented at the EBMT 2016 Annual Meeting. In these episodes, the 30 day mortality was 15% with a significant difference in deaths caused by resistant vs. sensitive to non-carbapenem beta-lactam antibiotics, 21% vs. 9%; caused by resistant vs. sensitive to carbapenem antibiotics, 40% vs. 10%; caused by resistant vs. sensitive to multi-drug-resistant (MDR) vs. non-MDR strains, 21% vs. 8%. Completion of the data analysis will allow the following to be defined: the main demographic, clinical and microbiological risk factors associated with gram-negative bacteremia and mortality in a HSCT setting.

Penack et al. published, on behalf of the IDWP, a large registry analysis evaluating the impact of a prior aspergillosis on long-term survival and on transplant-related complications in 1,150 patients with acute leukaemia who underwent allo-HSCT between 2005 and 2010. No significant impact of invasive aspergillosis (IA) on major transplant outcome variables was found. These data suggest that a history of IA should not generally be a contraindication to performing an allo-HSCT in patients with acute leukaemia. To confirm these original findings a prospective non-interventional study is in preparation.

In addition, the IDWP contributed to the organisation of the 6th edition of the European Conference on Infections in Leukaemia (ECIL) that was held in Sophia Antipolis (France) on 10th–12th September, 2015. The guidelines discussed in this meeting are of primary interest for the HSCT community because they deal with diagnosis, prophylaxis and treatment of haemorrhagic cystitis and polyomavirus BK (BKPyV) infection, the treatment of *Pneumocystis jirovecii* pneumonia, and an update of diagnosis, prophylaxis and treatment of Epstein-Barr virus related post-transplant lymphoproliferative disease (EBV-PTLD).

On 29th–30th October 2015, the IDWP organised the 18th training course that was held in Verona and attended by more than 60 young colleagues coming from 13 different European and non-European countries; 9 young colleagues were actively involved in the presentation of special cases.

Last but not least, in November 2015, the IDWP launched a new observational prospective study on hepatitis C infection (HCV) treatment approach in patients with HCV infection who underwent HSCT.

Principal research studies

1. A prospective study on the incidence of gram-negative bacteremia, risk factors and resistance to antibiotics
2. Risk factors and outcome of pneumocystis pneumonia (PcP) infection in HSCT
3. Treatment approach for patients with HCV infection and who underwent HSCT
4. Prospective study on the impact of prior aspergillosis on non-relapse mortality (NRM) after allo-HSCT in leukaemic patients
5. Retrospective study on human herpesvirus-6 (HHV6) encephalitis

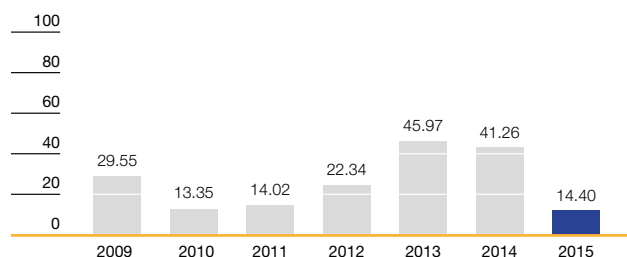


18th Training Course of the IDWP in Verona

Key publications

1. Influence of pre-existing invasive aspergillosis on allo-HSCT outcome: a retrospective EBMT analysis by the Infectious Diseases and Acute Leukemia Working Parties. Penack O, *Bone Marrow Transplant*. 2015 Oct 26
2. Current practices used for the prevention of central venous catheter-associated infection in hematopoietic stem cell transplantation recipients: a survey from the Infectious Diseases Working Party and Nurses' Group of EBMT. Snarski E, *Transpl Infect Dis*. 2015 Aug
3. Long-term persistence of the immune response to antipneumococcal vaccines after Allo-SCT: 10-year follow-up of the EBMT-IDWP01 trial. Gonnardier C, *Bone Marrow Transplant*. 2015 Jul
4. Discontinuation of empirical antibiotic therapy in neutropenic leukaemia patients with fever of unknown origin is ethical. Orasch C, *Clin Microbiol Infect*. 2015 Mar

Impact factor (Infectious Diseases)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 1 | 1 | 4 |
| Poster Presentations | 0 | 1 | 0 |
| International Educational Events | 1 | 1 | 1 |

Major educational courses

1. 6th European Conference on Infections in Leukaemia (ECIL) - September 10–12 2015 in Sophia Antipolis, France.
2. 18th Training Course of the Infectious Diseases Working Party - October 29–30 2015 in Verona, Italy.

Inborn Errors Working Party (IEWP)

Chair: **Andrew Gennery**



Major achievements

The IEWP has held three major meetings in addition to attending the EBMT 2015 Annual Meeting. Our first one day workshop – “How to transplant a patient with Primary Immunodeficiency” – was extremely successful. Following requests to repeat it, we departed from the norm and began our Annual Autumn Meeting with a similar workshop condensed into the preceding morning. Our Autumn Meeting had our highest number of attendees ever, including colleagues from North and South America, Australia and Malaysia. Notable highlights were the keynote lecture by Professor Notarangelo from Harvard University, “It is RAG* time: from repertoire abnormalities and anti-cytokine antibodies to NK** cells”, and in an IEWP first, Professor Hollander from Oxford and Basel Universities delivered his lecture for the Thymic Disorders session via Skype from Japan. Our small Newborn Screening for Severe Combined Immunodeficiency Working Group met in September 2015 and is producing European-wide guidelines for implementation of Newborn Screening for SCID, subsequent management guidelines and data collection. We have updated consensus guidelines for diagnosis, therapy and follow-up of osteopetrosis, available on the EBMT website https://www.ebmt.org/Contents/Research/TheWorkingParties/IEWP/Documents/00_OP_Guidelines_V3.pdf

A number of studies on outcomes of transplant in rare diseases are in progress, and we have published six major studies over the year. Increasingly we are collaborating with our sister organisation in North America (the Primary Immune Deficiency Treatment Consortium), giving greater power to our studies. The Gene Therapy and Metabolic Diseases subgroups continue to play an active role in the IEWP.

* recombination activating genes (RAG)

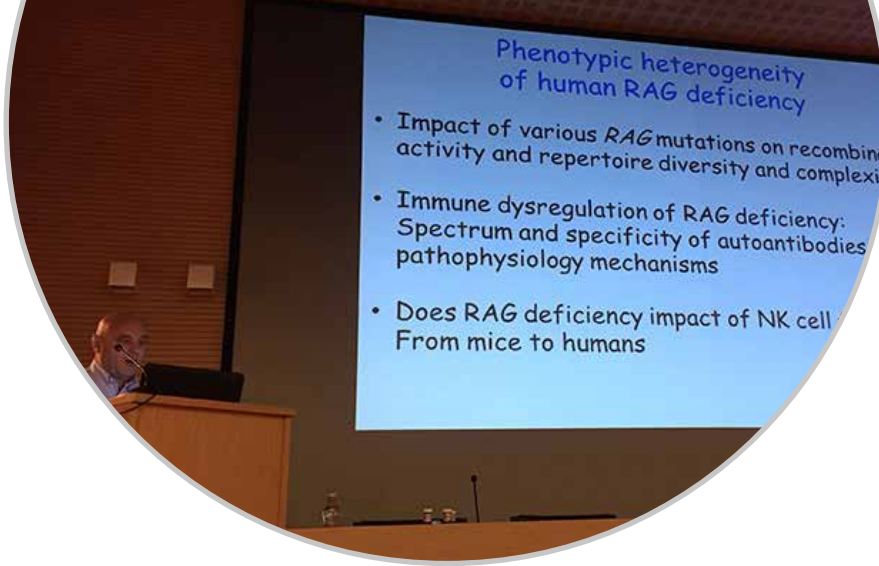
** natural killer (NK)

Principal research studies

1. Treosulfan-based conditioning for allogeneic haematopoietic stem cell transplantation in children with chronic granulomatous disease. Multicentre experience
2. Outcome of haematopoietic stem cell transplantation for DNA-Double strand breakage repair disorders: An European Society for Blood and Marrow Transplantation / European Society for Immunodeficiencies (EBMT/ESID) IEWP and Center for International Blood and Marrow Transplant Research / Primary Immune Deficiency Treatment Consortium (CIBMTR/PIDTC) survey
3. Outcome of haematopoietic stem cell transplantation for CD40 ligand deficiency: An EBMT/ESID IEWP and PIDTC survey
4. Reticular dysgenesis: International survey on clinical presentation, transplantation and outcome

Key publications

1. Nijmegen Breakage Syndrome: Clinical and Immunological Features, Long-Term Outcome and Treatment Options - a Retrospective Analysis. Wolska-Kuciel B, *J Clin Immunol.* 2015 Aug
2. The syndrome of hemophagocytic lymphohistiocytosis in primary immunodeficiencies: implications for differential diagnosis and pathogenesis. Bode SF, *Haematologica* 2015 Jul
3. DOCK8 deficiency: clinical and immunological phenotype and treatment options - a review of 136 patients. Aydin SE, *J Clin Immunol.* 2015 Feb
4. Multicenter experience in hematopoietic stem cell transplantation for serious complications of common variable immunodeficiency. Wehr C, *J Allergy Clin Immunol.* 2015 Apr

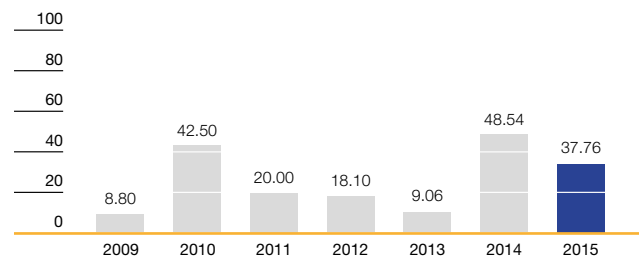


Luigi Notarangelo, speaker at the IEWP Autumn Meeting in Seville

Major educational courses

1. How to Transplant a Patient with Primary Immunodeficiency - April 24 2015 in Leuven, Belgium
2. Newborn Screening for SCID Working Group - September 30 2015 in London, UK
3. How to Transplant a Patient with Primary Immunodeficiency - October 16 2015 in Seville, Spain
4. IEWP Autumn Meeting - October 16–18 2015, Seville, Spain

Impact factor (Inborn Errors)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 3 | 2 | 5 |
| Poster Presentations | 0 | 2 | 2 |
| International Educational Events | 1 | 3 | 3 |



Lymphoma Working Party (LWP)

Chair: **Peter Dreger**



Major achievements

The LWP takes care of scientific and educational activities related to transplantations for lymphoma, which represents the largest single entity in the EBMT with over 120,000 registered transplantations to date. The LWP runs a Scientific Panel consisting of the LWP Chair (P Dreger, Heidelberg), the LWP Secretary (S Robinson, Bristol) and six additional members responsible for relevant subtopics, i.e. Hodgkin's lymphoma and educational affairs (A Sureda, Barcelona), Indolent lymphoma (S Montoto, London), T-cell lymphoma (N Schmitz, Hamburg), Aggressive B-cell lymphoma (H Schouten, Maastricht), Mantle cell lymphoma (O Hermine, Paris), and Outreach affairs (A Tanase, Bucharest). Key staff members in the Data Office in Paris are A Boumendil (Statistician) and H Finel (Study coordinator).

The most relevant scientific activities of the LWP in 2015 comprise conducting or completing 19 retrospective and 4 prospective non-interventional studies (involving 16 Principal Investigators (PIs) from 10 countries). See the section below for the most important ongoing studies. The LWP's study activities resulted in the publication of 10 scientific papers in 2015 with a cumulative Impact Factor of 68.926. See the section below for the most important publications.

Moreover, the 11th Annual LWP Educational Course held in Heidelberg, Germany, was a major success and continued the tradition of the LWP to be a prime supplier of state-of-the-art knowledge about lymphoma management and transplantation to young investigators and clinicians.

For the 4th time we were able to present the Jian-Jian Luan Award for Lymphoma Transplant research during the LWP session at the EBMT Annual Meeting in Istanbul. This prize is dedicated to LWP's former Study Coordinator Jian-Jian Luan, who had a fatal accident during an alpine hiking tour in December 2010. The award winner was Jorge Gayoso from Madrid, Spain, for a multicentre study on haplo-HCT in Hodgkin's lymphoma.

Finally, we managed to launch the new MED-A form that will largely improve data quality particularly for future lymphoma registry studies.

Principal research studies

1. Impact of B cell receptor inhibitors on lymphoma and CLL transplants (together with CMWP)
2. Brentuximab vedotin for bridging to transplant for anaplastic large cell lymphoma (ALCL) and Hodgkin lymphoma
3. Autologous HCT in HIV lymphoma in the era of combination antiretroviral therapy
4. Allogeneic transplants for NHL in the elderly

Key publications

1. Hematopoietic stem cell transplantation for T-cell large granular lymphocyte leukemia: a retrospective study of the European Society for Blood and Marrow Transplantation. Marchand T, *Leukemia* 2015 Sep 22
2. Second allo-SCT in patients with lymphoma relapse after a first allogeneic transplantation. A retrospective study of the EBMT Lymphoma Working Party. Horstmann K, *Bone Marrow Transplant*. 2015 Jun
3. Autologous stem cell transplantation for relapsed/refractory diffuse large B-cell lymphoma: efficacy in the rituximab era and comparison to first allogeneic transplants. A report from the EBMT Lymphoma Working Party. Robinson SP, *Bone Marrow Transplant*. 2015 Nov 30
4. Autologous hematopoietic stem cell transplantation for plasmablastic lymphoma: the European Society for Blood and Marrow Transplantation experience. Cattaneo C, *Biol Blood Marrow Transplant*. 2015 Jun

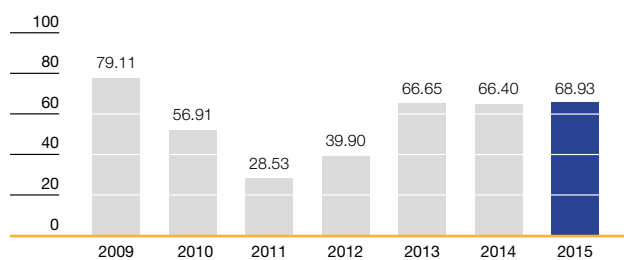


11th Educational course of the LWP in Heidelberg

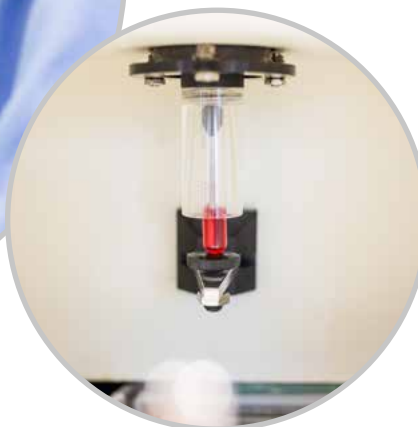
Major educational courses

1. 11th Educational Course of the LWP: "Treatment of Malignant Lymphoma: State-of-the-Art and Role of Stem Cell Transplantation" - September 24–26 2015 in Heidelberg, Germany

Impact factor (Lymphoma)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 3 | 8 | 8 |
| Poster Presentations | 7 | 11 | 4 |
| International Educational Events | 1 | 4 | 4 |



Paediatric Diseases Working Party (PDWP)

Chair: **Peter Bader**



Major achievements

The PDWP held its “5th EBMT Training Course for Paediatricians and Paediatric Nurses on HSCT in Children and Adolescents”, in Marrakech, Morocco, in order to promote this treatment modality in developing countries in Africa and the Middle East. More than 40 physicians from eight European, four Mediterranean and one Asian country formed an international audience and faculty. Participants had the opportunity to meet international experts in order to discuss all aspects of paediatric haematopoietic stem cell transplantation (HSCT): L Faulkner and N Novitzky gave insight in to their experiences of organising transplant units in developing countries (i.e. Pakistan, South Africa). P Bader promoted haplo-transplantation as a promising treatment option particularly in developing countries, where large donor registries will not be available in the near future.

Furthermore, the PDWP held an “Expert Workshop on Fertility Preservation in the Context of HSCT” in Baden, Austria. Thirty-three international experts in this field discussed strategies and methods in order to develop fertility preservation as a standard of care prior to paediatric HSCT.

C Peters published a key paper on behalf of the PDWP in the *Journal of Clinical Oncology*: in a prospective study within the acute lymphoblastic leukemia stem cell transplantation study (ALL-SCT-BFM 2003) it was proven that the outcomes among high-risk paediatric patients with ALL after HSCT were not affected by donor type [matched sibling donor (MSD) vs matched unrelated donor (MUD)]. This study provided the basis for the PDWP-driven prospective multinational randomised study “ALL SCTped 2012 FORUM” (Chair: C Peters, Co-Chairs: P Bader, F Locatelli) that aims to demonstrate that non- total body irradiation (TBI) conditioning results in non-inferior survival as compared to conditioning with TBI/etoposide in children after HSCT. Currently, recruitment is open in 78 centres in 17 countries and 179 patients have already been enrolled.

Principal research studies

1. Subsequent allogeneic SCT in paediatric patients: indications, procedures and outcome
2. ALL SCTped 2012 FORUM (“For Omitting Radiation Under Majority Age”)
3. The optimal alternative donor transplant for ALL or acute myelogenous leukemia AML: comparison between T-cell depleted haplo-HSCT and unrelated cord blood transplantation (UCBT)
4. Burden of late effects after HSCT for haematological malignancies in children transplanted before the age of three years

Key publications

1. Stem-cell transplantation in children with acute lymphoblastic leukemia: A prospective international multicenter trial comparing sibling donors with matched unrelated donors-The ALL-SCT-BFM-2003 trial. Peters C, *J Clin Oncol*. 2015 Apr 10
2. Combined cord blood and bone marrow transplantation from the same human leucocyte antigen-identical sibling donor for children with malignant and non-malignant diseases. Tucunduva L, *Br J Haematol*. 2015 Apr
3. Outcome of aplastic anaemia in children. A study by the severe aplastic anaemia and paediatric disease working parties of the European group blood and bone marrow transplant. Dufour C, *Br J Haematol*. 2015 May
4. Similar outcome of upfront-unrelated and matched sibling stem cell transplantation in idiopathic paediatric aplastic anaemia. A study on behalf of the UK Paediatric BMT Working Party, Paediatric Diseases Working Party and Severe Aplastic Anaemia Working Party of EBMT. Dufour C, *Br J Haematol*. 2015 Nov

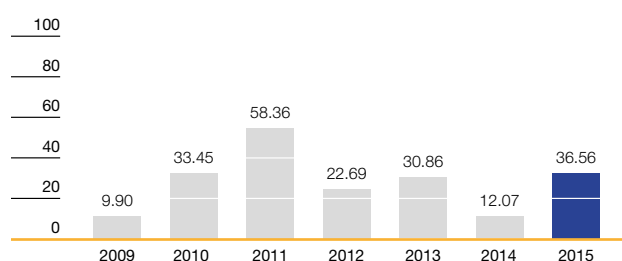


5th Training Course for Paediatricians and Paediatric Nurses on HSCT in Children and Adolescents in Marrakech

Major educational courses

1. 5th Training Course for Paediatricians and Paediatric Nurses on HSCT in Children and Adolescents - May 14–17 2015 in Marrakech, Morocco
2. PDWP Expert Workshop on Fertility Preservation in the context of HSCT - September 29–October 1 2015 in Baden, Austria
3. PDWP Scientific Symposium Honouring the Career of J. Cornish - September 30 2015 in Baden, Austria

Impact factor (Paediatric Diseases)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 3 | 10 | 6 |
| Poster Presentations | 5 | 7 | 3 |
| International Educational Events | 3 | 1 | 2 |



Solid Tumours Working Party (STWP)

Chair: **Francesco Lanza**



Major achievements

Germinal Cell Tumour (GCT)

The main aims of the STWP research activity in 2015 was to address the prognostic significance of response to induction chemotherapy (CT) preceding salvage high-dose CT (HDCT) courses, and prior taxane (TXL)-CT for advanced GCT, as this knowledge can inform trial design, stratification and eligibility criteria for HDCT.

Data were collected from 23 European centres and concerned treatment with salvage HDCT between 2002 and 2012. Both TXL used in prior CT lines of therapy and in induction-mobilisation regimens pre-HDCT were considered. While progressive disease (PD) to induction CT was independently prognostic for progression free survival (PFS) and overall survival (OS), TXL-regimens before HDCT did not affect the outcome. Stratification of trials for the latter factor did not appear to be required when accounting for the other clinical predictors. We demonstrated that the majority of patients with PD to induction CT usually progress after HDCT. In conclusion, in this population-based analysis we observed that results of HDCT as salvage therapy administered in the last 10 years were not influenced by the increasing use of TXL-containing salvage chemotherapy preceding HDCT. Moreover, while we have confirmed HDCT to be a substantially effective strategy irrespective of the number of prior regimens, an additional prognostic factor for clinical use was provided that was represented by the response to induction CT administered as part of the transplantation strategy.

Breast Cancer (BC)

In 2015, the STWP conducted a retrospective study with the main goal to assess toxicity and efficacy of adjuvant HDCT and autologous haematopoietic stem cell transplantation (AHSCT) in 583 high-risk breast cancer (BC) patients (>3 positive nodes) who were transplanted between 1995 and 2005 in Europe. Subgroup analysis demonstrated that OS was significantly better in patients with endocrine-responsive tumours, less than 10 positive lymph nodes and smaller tumour size. HER2 status did not affect survival probability. Adjuvant HDCT with AHSCT has a low mortality rate and provides impressive long-term survival rates in patients with high-risk BC. Our results suggest that this treatment modality should be considered in selected high-risk BC patients and further investigated in clinical trials. Along with some more recent phase III studies, retrospective analysis, and to some extent the results from meta-analysis, our results suggest a potential role for HDCT and autologous transplantation in high-risk BC.

The STWP educational course involved the participation of 7 speakers, 4 chairmen and 86 health-care professionals. The session focused on the potential of the latest therapeutic advances for the treatment of solid neoplasms, including cell therapies and gene therapies, and widely analysed the latest data on stem cell transplantation for ST. Moreover, the meeting discussed the latest biological improvements in the setting of the isolation of metastatic cells and nucleic acids, and critically evaluated the existing relationships between the field of clinical research and regulatory authorities.

Overall, the meeting was evaluated positively by the participants, which appreciated the objectivity of the discussions and the transparency of the presentations.

Principal research studies

1. High-dose chemotherapy and autologous haematopoietic stem cell transplantation as adjuvant treatment in high-risk breast cancer
2. Retrospective analysis of data on high-dose chemotherapy and autologous haematopoietic progenitor cell transplantation for metastatic breast cancer
3. 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
4. Long-term results of salvage high-dose chemotherapy for germ cell tumours in female and adolescent patients

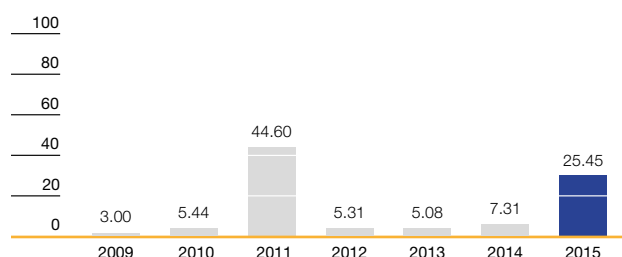


STWP educational course in Cremona

Key publications

1. High-Dose Chemotherapy With Autologous Hematopoietic Stem Cell Transplantation for High-Risk Primary Breast Cancer. Pedrazzoli P, *J Natl Cancer Inst Monogr*. 2015 May
2. Prognostic impact of progression to induction chemotherapy and prior paclitaxel therapy in patients with germ cell tumors receiving salvage high-dose chemotherapy in the last 10 years: a study of the European Society for Blood and Marrow Transplantation Solid Tumours Working Party. Necchi A, *Bone Marrow Transplant*. 2015 Dec 7
3. Breast cancer circulating biomarkers: advantages, drawbacks, and new insights. Ravelli A, *Tumour Biol*. 2015 Aug
4. Immune-related strategies driving immunotherapy in breast cancer treatment: a real clinical opportunity. Ravelli A, *Expert Rev Anticancer Ther*. 2015 Jun

Impact factor (Solid Tumours)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 0 | 6 | 4 |
| Poster Presentations | 0 | 2 | 3 |
| International Educational Events | 0 | 3 | 1 |

Major educational courses

1. EBMT Solid Tumours Working Party Educational Meeting March 2015 in Istanbul, Turkey
2. A Multispecialist Pathway Towards a Therapy and Health-Educational Model September 30–October 1 2015 in Cremona, Italy



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 2015 we continued our mission to organise high-quality educational events regarding the disease-specific topics of our WP. In January 2015 we hosted in Seville, a symposium on “The role of stem cell transplantation in multiple myeloma and related diseases in the era of novel drugs”. In September 2015 we conducted an educational event in Helsinki on “New developments in allo-geneic SCT for MDS and MPN”.

The EBMT-labelled randomised trial of the CMWP: “ATG to prevent chronic GvHD after HLA-identical sibling peripheral blood stem cell transplantation” received the prestigious Van Bekkum Award for the highest ranked abstract at the EBMT 2015 Annual Meeting in Istanbul. In addition, the first results of the randomised EBMT trial “Reduced vs. standard conditioning in MDS/sAML (RICMAC)” were presented in the Presidential Symposium of the EBMT 2015 Annual Meeting as one of the top five ranked abstracts.

In 2015, the CMWP published 13 manuscripts in peer-reviewed journals such as *Blood*, *Leukemia*, *Haematologica*, *British Journal of Haematology*, *Bone Marrow Transplantation and Biology of Blood and Marrow Transplantation*. At international meetings such as ASH, EHA, and EBMT, members of the CMWP presented more than 50 oral or poster presentations.

In 2015 we completed one consensus project in collaboration with the European Leukemia Net (ELN) on “Stem cell transplantation in myelofibrosis” (published in *Leukemia*) and we are in the final stages of another collaborative project with the ELN on “Stem cell transplantation in MDS”.

In addition, to harmonise response criteria for multiple myeloma, several teleconferences or face-to-face meetings were held together with the CIBMTR.

Overall, within our WP we have currently 11 active non-interventional studies, 3 prospective EBMT-sponsored or labelled studies ongoing and are working on more than 50 retrospective registry studies.

Principal research studies

1. ATG to prevent chronic GvHD after HLA-identical peripheral blood stem cell transplantation (ATGFamilyStudy). A prospective, randomised EBMT-labelled study
2. Reduced vs. standard conditioning in MDS/sAML (RICMAC study). A prospective randomised study of CMWP
3. The effect of 2nd generation TKI on the outcome after allogeneic SCT for Patients with CML: A non-interventional prospective study by the CMWP
4. Role of stem cell mobilisation and outcome in multiple myeloma and malignant lymphoma (CALM study). A non-interventional prospective study of CMWP and LWP

Key publications

1. Indication and management of allogeneic stem cell transplantation in primary myelofibrosis: a consensus process by an EBMT/ELN international working group. Kröger NM, *Leukemia* 2015 Nov
2. Impact of the revised International Prognostic Scoring System, cytogenetics and monosomal karyotype on outcome after allogeneic stem cell transplantation for myelodysplastic syndromes and secondary acute myeloid leukemia evolving from myelodysplastic syndromes: a retrospective multicenter study of the European Society of Blood and Marrow Transplantation. Koenecke C, *Haematologica* 2015 Mar
3. Impact of allogeneic stem cell transplantation on survival of patients less than 65 years of age with primary myelofibrosis. Kröger N, *Blood* 2015 May 21
4. Achievement of complete remission predicts outcome of allogeneic haematopoietic stem cell transplantation in patients with chronic myelomonocytic leukaemia. A study of the Chronic Malignancies Working Party of the European Group for Blood and Marrow Transplantation. Symeonidis A, *Br J Haematol.* 2015 Jul 26

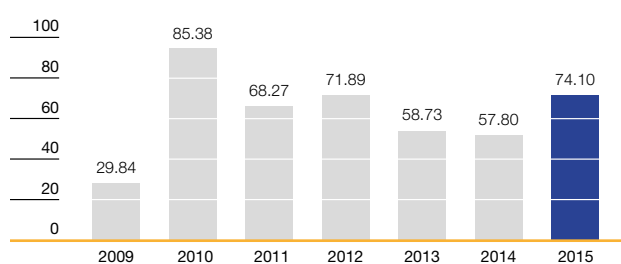


CMWP educational course in Helsinki

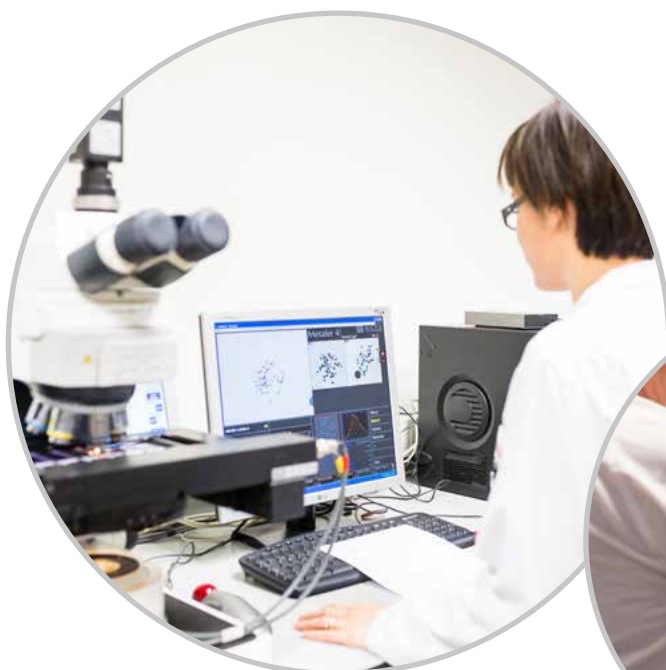
Major educational courses

1. "The Role of Stem Cell Transplantation in Multiple Myeloma and Related Diseases in the Era of Novel Drugs" - January 23–24 2015 in Seville, Spain
2. "New Developments in Allogeneic SCT for the Treatment of MDS and MPN" - September 11–12 2015 in Helsinki, Finland

Impact factor (Chronic Malignancies)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 41 | 44 | 33 |
| Poster Presentations | 9 | 14 | 12 |
| International Educational Events | 4 | 4 | 2 |



Complications and Quality of Life Working Party (CQLWP)

Chair: **Rafael F Duarte**



Major achievements

The CQLWP takes care of the scientific and educational activities of the EBMT in relation to transplant complications of a non-infectious nature. We organise these activities through a Scientific Panel that includes a WP Secretary (G Basak), a Nurse Lead for transplant complications (D Greenfield), three scientific subcommittees focused on GvHD (H Greinix), on conditioning-related complications and supportive care (T Ruutu) and on late complications (N Salooja), and a Working Party Chair (RF Duarte). Our main goal is to combine expertise to provide the EBMT with a strong WP focused on transversal research on transplant complications, in collaboration with other WPs and Committees within the EBMT, and external collaborations with international groups.

The educational highlight of 2015 was our WP course hosted by H. Schoemans on 29th–30th October in Leuven, Belgium (see below). The course was a big success, with more than 100 participants. It covered many topics across the scientific scope of our WP, with a particular focus on the management of under-recognised post transplantation complications including neurocognitive impairment after allogeneic transplantation, medication adherence, the caregiver's perspective on discussing sexuality after transplant, our recent guidelines on secondary cancer screening, iron overload before and after transplantation, conditioning dosing in obese patients and less common manifestations of graft-versus-host disease (GvHD) such as ocular, genital and neuro-GvHD. Notably, the course gave us the opportunity to organise in parallel a one-day symposium on the 28th October on transplant complications and management for nurses. A new educational course and business meeting will be held in October 2016 in Madrid, Spain.

In 2015, we also continued the international collaborative work started in 2013 through the EBMT-National Institutes of Health (NIH) Task Force on chronic GvHD and survivorship issues after transplant. We have created an electronic application, the EBMT GvHD App, which improves not only the accuracy to diagnose and score chronic GvHD according to the updated 2014 NIH criteria, but also increases user satisfaction on a platform compatible with computers and mobile devices. Large-scale implementation of the EBMT GvHD App will get underway in 2016. We have also continued our line of collaborations on transplant guidelines with our Center for International Blood and Marrow Transplant Research (CIBMTR) colleagues, with joint guidelines on secondary solid cancer screening as well as on metabolic syndrome and cardiovascular diseases after transplant.

Over 20 studies including retrospective registry studies, practice surveys and prospective studies are currently underway, many coming to the end of their cycle. 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. We look forward to meeting you at our business meeting and WP session at the EBMT 2016 Annual Meeting.





CQLWP educational course in Leuven

Principal research studies

1. A prospective non interventional study on the impact of uric acid levels on GvHD in recipients of allogeneic HCT (O Penack)
2. Incidence and outcome of pregnancy following HCT (N Salooja)
3. Survey of nutritional practices in HCT (G Basak)
4. Metabolic syndrome in HCT recipients (D Greenfield, J Snowden)

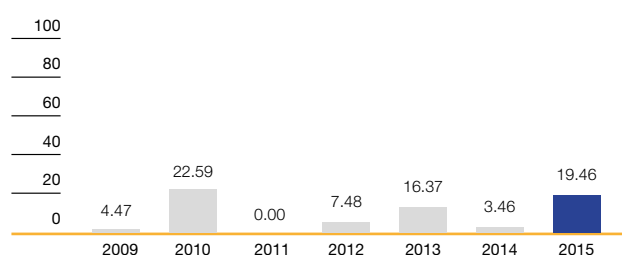
Key publications

1. Allogeneic hematopoietic stem cell transplantation in solid organ transplant recipients: a retrospective, multicenter study of the EBMT. Basak GW, *Am J Transplant*. 2015 Mar
2. Paediatric reduced intensity conditioning: analysis of centre strategies on regimens and definitions by the EBMT Paediatric Diseases and Complications and Quality of Life WP. Lawitschka A, *Bone Marrow Transplant*. 2015 Apr
3. Second allogeneic transplantation for relapse of malignant disease: retrospective analysis of outcome and predictive factors by the EBMT. Ruutu T, *Bone Marrow Transplant*. 2015 Dec
4. Secondary solid cancer screening following hematopoietic cell transplantation. Inamoto Y, *Bone Marrow Transplant*. 2015 Aug

Major educational courses

1. "Thinking Outside the Box: Going beyond 'Survival after Stem Cell Transplant'" - October 29–30 2015 in Leuven, Belgium

Impact factor (Complications and Quality of Life)



| | 2013 | 2014 | 2015 |
|----------------------------------|------|------|------|
| Oral Presentations | 2 | 1 | 2 |
| Poster Presentations | 2 | 2 | 1 |
| International Educational Events | 2 | 1 | 3 |

Publications 2015



| Title | First Listed Author | Journal | PMID |
|---|---------------------|--------------------------------------|----------|
| Allogeneic Hematopoietic Stem Cell Transplantation in Solid Organ Transplant Recipients: A Retrospective, Multicenter Study of the EBMT. | Basak GW | <i>Am J Transplant.</i> | 25648262 |
| Aplastic Anemia: Alternative Immunosuppressive Treatments and Eltrombopag. A report from the 2014 EBMT Educational Meeting from the Severe Aplastic Anaemia and Infectious Diseases Working Parties. | Risitano AM | <i>Curr Drug Targets.</i> | 25619749 |
| Autologous Hematopoietic Stem Cell Transplantation for Refractory Crohn Disease: A Randomized Clinical Trial. | Hawkey CJ | <i>JAMA</i> | 26670970 |
| Autologous hematopoietic stem cell transplantation for plasmablastic lymphoma: the European Society for Blood and Marrow Transplantation experience. | Cattaneo C | <i>Biol Blood Marrow Transplant.</i> | 25783635 |
| Autologous hematopoietic stem cell transplantation in multiple sclerosis: A phase II trial. | Mancardi GL | <i>Neurology</i> | 25672923 |
| Autologous hematopoietic stem cell transplantation in neuromyelitis optica: A registry study of the EBMT Autoimmune Diseases Working Party. | Greco R | <i>Mult Scler.</i> | 25078274 |
| Autologous stem cell transplantation for adult acute leukemia in 2015: time to rethink? Present status and future prospects. | Gorin NC | <i>Bone Marrow Transplant.</i> | 26281031 |
| Autologous stem cell transplantation for relapsed/refractory diffuse large B-cell lymphoma: efficacy in the rituximab era and comparison to first allogeneic transplants. A report from the EBMT Lymphoma Working Party. | Robinson SP | <i>Bone Marrow Transplant.</i> | 26618550 |
| Breast cancer circulating biomarkers: advantages, drawbacks, and new insights. | Ravelli A | <i>Tumour Biol.</i> | 26307395 |
| Characteristics and outcomes of aplastic anemia in HIV patients: a brief report from the severe aplastic anemia working party of the European Society of Blood and Bone Marrow Transplantation. | Pagliuca S | <i>Bone Marrow Transplant.</i> | 26479981 |
| Chemotherapy Dose Adjustment for Obese Patients Undergoing Hematopoietic Stem Cell Transplantation: A Survey on Behalf of the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation. | Shem-Tov N | <i>Oncologist</i> | 25480827 |
| Comparison of outcomes after unrelated cord blood and unmanipulated haploidentical stem cell transplantation in adults with acute leukemia. | Ruggeri A | <i>Leukemia</i> | 25882700 |
| Comparison of upfront tandem autologous-allogeneic transplantation versus reduced intensity allogeneic transplantation for multiple myeloma. | Sahebi F | <i>Bone Marrow Transplant.</i> | 25798673 |
| Conditioning intensity in middle-aged patients with AML in first CR: no advantage for myeloablative regimens irrespective of the risk group-an observational analysis by the Acute Leukemia Working Party of the EBMT. | Passweg JR | <i>Bone Marrow Transplant.</i> | 26030052 |
| Current outcome of HLA identical sibling vs. unrelated donor transplants in severe aplastic anemia: an EBMT analysis. | Bacigalupo A | <i>Haematologica</i> | 25616576 |
| Current practices used for the prevention of central venous catheter-associated infection in hematopoietic stem cell transplantation recipients: a survey from the Infectious Diseases Working Party and Nurses' Group of EBMT. | Snarski E | <i>Transpl Infect Dis.</i> | 25953418 |
| Definition of GvHD-free, relapse-free survival for registry-based studies: an ALWP-EBMT analysis on patients with AML in remission. | Ruggeri A | <i>Bone Marrow Transplant.</i> | 26657834 |
| Discontinuation of empirical antibiotic therapy in neutropenic leukaemia patients with fever of unknown origin is ethical. | Orasch C | <i>Clin Microbiol Infect.</i> | 25658572 |
| DOCK8 Deficiency: Clinical and Immunological Phenotype and Treatment Options - a Review of 136 Patients. | Aydin SE | <i>J Clin Immunol.</i> | 25627830 |
| Does ex vivo CD34+ positive selection influence outcome after autologous hematopoietic stem cell transplantation in systemic sclerosis patients? | Oliveira MC | <i>Bone Marrow Transplant.</i> | 26642332 |
| Efficacy and outcome of allogeneic transplantation in IgD and non secretory myeloma. A report on behalf of the myeloma subcommittee of the Chronic Malignancies Working Party of the EBMT. | Morris C | <i>Biol Blood Marrow Transplant.</i> | 25708221 |
| European Group for Blood and Marrow Transplantation Centers with FACT-JACIE Accreditation Have Significantly Better Compliance with Related Donor Care Standards. | Anthias C | <i>Biol Blood Marrow Transplant.</i> | 26597079 |
| European Society for Blood and Marrow Transplantation Analysis of Treosulfan Conditioning Before Hematopoietic Stem Cell Transplantation in Children and Adolescents With Hematological Malignancies. | Boztug H | <i>Pediatr Blood Cancer.</i> | 26398915 |

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| Hematopoietic SCT in Europe 2013: recent trends in the use of alternative donors showing more haploidentical donors but fewer cord blood transplants. | Passweg JR | <i>Bone Marrow Transplant.</i> | 25642761 |
| Hematopoietic stem cell transplantation for T-cell large granular lymphocyte leukemia: a retrospective study of the European Society for Blood and Marrow Transplantation. | Marchand T | <i>Leukemia</i> | 26460210 |
| High-dose chemotherapy and autologous hematopoietic stem cell transplantation as adjuvant treatment in high-risk breast cancer: data from the EBMT registry. | Martino M | <i>Biol Blood Marrow Transplant.</i> | 26723932 |
| High-Dose Chemotherapy With Autologous Hematopoietic Stem Cell Transplantation for High-Risk Primary Breast Cancer. | Pedrazzoli P | <i>J Natl Cancer Inst Monogr.</i> | 26063892 |
| High-dose therapy and autologous stem cell transplantation for extra-nodal NK/T lymphoma in patients from the Western hemisphere: a study from the European Society for Blood and Marrow Transplantation. | Fox CP | <i>Leuk Lymphoma.</i> | 25899403 |
| Higher busulfan dose intensity appears to improve leukemia-free and overall survival in AML allografted in CR2: An analysis from the Acute Leukemia Working Party of the European Group for Blood and Marrow Transplantation. | Kharfan-Dabaja MA | <i>Leuk Res.</i> | 26003666 |
| Immune-related strategies driving immunotherapy in breast cancer treatment: a real clinical opportunity. | Ravelli A | <i>Expert Rev Anticancer Ther.</i> | 25927868 |
| Impact of allogeneic stem cell transplantation on survival of patients less than 65 years of age with primary myelofibrosis. | Kröger N | <i>Blood</i> | 25784679 |
| Impact of conditioning with TBI in adult patients with T-cell ALL who receive a myeloablative allogeneic stem cell transplantation: a report from the acute leukemia working party of EBMT. | Cahu X | <i>Bone Marrow Transplant.</i> | 26618548 |
| Impact of CR before and after allogeneic and autologous transplantation in multiple myeloma: results from the EBMT NMAM2000 prospective trial. | Iacobelli S | <i>Bone Marrow Transplant.</i> | 25621805 |
| Impact of the revised International Prognostic Scoring System, cytogenetics and monosomal karyotype on outcome after allogeneic stem cell transplantation for myelodysplastic syndromes and secondary acute myeloid leukemia evolving from myelodysplastic synd. | Koenecke C | <i>Haematologica</i> | 25552702 |
| In patients older than 55 years with AML in first CR, should we search for a matched unrelated donor when an old sibling donor is available? | Peffault de Latour R | <i>Bone Marrow Transplant.</i> | 26367237 |
| Indication and management of allogeneic stem cell transplantation in primary myelofibrosis: a consensus process by an EBMT/ELN international working group. | Kröger NM | <i>Leukemia</i> | 26293647 |
| Influence of pre-existing invasive aspergillosis on allo-HSCT outcome: a retrospective EBMT analysis by the Infectious Diseases and Acute Leukemia Working Parties. | Penack O | <i>Bone Marrow Transplant.</i> | 26501769 |
| Is there a stronger graft-versus-leukemia effect using HLA-haploidentical donors than with HLA-identical siblings? | Ringdén O | <i>Leukemia</i> | 26293645 |
| Long-term outcome and prognostic factors of second allogeneic hematopoietic stem cell transplant for acute leukemia in patients with a median follow-up of 40 years. | Andreola G | <i>Bone Marrow Transplant.</i> | 26389832 |
| Long-term persistence of the immune response to antipneumococcal vaccines after Allo-SCT: 10-year follow-up of the EBMT-IDWP01 trial. | Cordonnier C | <i>Bone Marrow Transplant.</i> | 25867652 |
| Monosomal karyotype as an adverse prognostic factor in patients with acute myeloid leukemia treated with allogeneic hematopoietic stem-cell transplantation in first complete remission. A retrospective survey on behalf of the ALWP of EBMT. | Brands-Nijenhuis AV | <i>Haematologica</i> | 26589909 |
| Multicenter experience in hematopoietic stem cell transplantation for serious complications of common variable immunodeficiency. | Wehr C | <i>J Allergy Clin Immunol.</i> | 25595268 |
| Nijmegen Breakage Syndrome: Clinical and Immunological Features, Long-Term Outcome and Treatment Options - a Retrospective Analysis. | Wolska-Ku€nierz B | <i>J Clin Immunol.</i> | 26271390 |
| Outcome of allogeneic hematopoietic stem-cell transplantation for adult patients with AML and 11q23/MLL rearrangement (MLL-r AML). | Pigneux A | <i>Leukemia</i> | 26082270 |
| Outcome of aplastic anaemia in children. A study by the severe aplastic anaemia and paediatric disease working parties of the European group blood and bone marrow transplant. | Dufour C | <i>Br J Haematol.</i> | 25683884 |
| 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). | Poiré X | <i>Am J Hematol.</i> | 26010466 |
| Outcome of patients with distinct molecular genotypes and cytogenetically normal AML after allogeneic transplantation. | Schmid C | <i>Blood</i> | 26351297 |
| Outcomes after use of two standard ablative regimens in patients with refractory acute myeloid leukaemia: a retrospective, multicentre, registry analysis. | Nagler A | <i>Lancet Haematol.</i> | 26685771 |
| Paediatric reduced intensity conditioning: analysis of centre strategies on regimens and definitions by the EBMT Paediatric Diseases and Complications and Quality of Life WP. | Lawitschka A | <i>Bone Marrow Transplant.</i> | 25621804 |
| Peripheral blood stem cell graft compared to bone marrow after reduced intensity conditioning regimens for acute leukemia - A report from the ALWP of the EBMT. | Savani BN | <i>Haematologica</i> | 26565001 |

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| Prediction of Allogeneic Hematopoietic Stem-Cell Transplantation Mortality 100 Days After Transplantation Using a Machine Learning Algorithm: A European Group for Blood and Marrow Transplantation Acute Leukemia Working Party Retrospective Data Mining Stud | Shouval R | <i>J Clin Oncol.</i> | 26240227 |
| Prognostic impact of progression to induction chemotherapy and prior paclitaxel therapy in patients with germ cell tumors receiving salvage high-dose chemotherapy in the last 10 years: a study of the European Society for Blood and Marrow Transplantation S. | Necchi A | <i>Bone Marrow Transplant.</i> | 26642334 |
| Reduced intensity conditioning allogeneic hematopoietic cell transplantation for adult acute myeloid leukemia in complete remission - a review from the Acute Leukemia Working Party of the EBMT. | Sengsayadeth S | <i>Haematologica</i> | 26130513 |
| Indications for allo- and auto-SCT for haematological diseases, solid tumours and immune disorders: current practice in Europe, 2015 | Sureda A | <i>Bone Marrow Transplant.</i> | 25798672 |
| SCT for severe autoimmune diseases: consensus guidelines of the European Society for Blood and Marrow Transplantation for immune monitoring and biobanking. | Alexander T | <i>Bone Marrow Transplant.</i> | 25387090 |
| Second allogeneic stem cell transplant for aplastic anaemia: a retrospective study by the severe aplastic anaemia working party of the European society for blood and marrow transplantation. | Cesaro S | <i>Br J Haematol.</i> | 26304743 |
| Second allogeneic transplantation for relapse of malignant disease: retrospective analysis of outcome and predictive factors by the EBMT. | Ruutu T | <i>Bone Marrow Transplant.</i> | 26367221 |
| Second allo-SCT in patients with lymphoma relapse after a first allogeneic transplantation. A retrospective study of the EBMT Lymphoma Working Party. | Horstmann K | <i>Bone Marrow Transplant.</i> | 25751644 |
| Second reduced intensity conditioning allogeneic transplant as a rescue strategy for acute leukaemia patients who relapse after an initial RIC allogeneic transplantation: analysis of risk factors and treatment outcomes. | Vrhovac R | <i>Bone Marrow Transplant.</i> | 26437057 |
| Similar outcome of upfront-unrelated and matched sibling stem cell transplantation in idiopathic paediatric aplastic anaemia. A study on behalf of the UK Paediatric BMT Working Party, Paediatric Diseases Working Party and Severe Aplastic Anaemia Working P. | Dufour C | <i>Br J Haematol.</i> | 26223288 |
| Sinusoidal obstruction syndrome/veno-occlusive disease: current situation and perspectives-a position statement from the European Society for Blood and Marrow Transplantation (EBMT). | Mohty M | <i>Bone Marrow Transplant.</i> | 25798682 |
| Stem cell transplantation in severe congenital neutropenia: an analysis from the European Society for Blood and Marrow Transplantation. | Fioredda F | <i>Blood</i> | 26185129 |
| Stem-cell transplantation in children with acute lymphoblastic leukemia: A prospective international multicenter trial comparing sibling donors with matched unrelated donors-The ALL-SCT-BFM-2003 trial. | Peters C | <i>J Clin Oncol.</i> | 25753432 |
| T replete haploidentical versus autologous stem cell transplantation in adult acute leukemia: a matched pair analysis. | Gorin NC | <i>Haematologica</i> | 25637051 |
| The impact of graft-versus-host disease prophylaxis in reduced-intensity conditioning allogeneic stem cell transplant in acute myeloid leukemia: a study from the Acute Leukemia Working Party of the European Group for Blood and Marrow Transplantation. | Rubio MT | <i>Haematologica</i> | 25769546 |
| The syndrome of hemophagocytic lymphohistiocytosis in primary immunodeficiencies: implications for differential diagnosis and pathogenesis. | Bode SF | <i>Haematologica</i> | 26022711 |
| Thiotepa-based high-dose therapy for autologous stem cell transplantation in lymphoma: a retrospective study from the EBMT. | Sellner L | <i>Bone Marrow Transplant.</i> | 26569093 |
| Thiotepa-based versus total body irradiation-based myeloablative conditioning prior to allogeneic stem cell transplantation for acute myeloid leukemia in first complete remission: a retrospective analysis from the Acute Leukemia Working Party of the Europ. | Eder S. | <i>Eur J Haematol.</i> | 25807864 |
| Treosulfan-based conditioning regimens for allogeneic haematopoietic stem cell transplantation in children with non-malignant diseases. | Slatter MA | <i>Bone Marrow Transplant.</i> | 26259076 |
| Tyrosine kinase inhibitors improve long-term outcome of allogeneic hematopoietic stem cell transplantation for adult patients with Philadelphia chromosome positive acute lymphoblastic leukemia. | Brissot E | <i>Haematologica</i> | 25527562 |
| Unrelated cord blood transplantation for adult patients with acute myeloid leukemia: higher incidence of acute graft-versus-host disease and lower survival in male patients transplanted with female unrelated cord blood-a report from Eurocord, the Acute Le | Baron F | <i>J Hematol Oncol.</i> | 26445106 |
| Unrelated cord blood transplantation for childhood acute myelogenous leukemia: The influence of cytogenetic risk group stratification. | Michel G | <i>Leukemia</i> | 26369981 |
| Where does allogeneic stem cell transplantation fit in the treatment of chronic lymphocytic leukemia? | Dreger P | <i>Curr Hematol Malign Rep.</i> | 25651976 |
| Is allogeneic transplant for solid tumors still alive? | Bregni M | <i>Bone Marrow Transplant.</i> | 26808572 |
| Secondary solid cancer screening following hematopoietic cell transplantation. | Inamoto Y | <i>Bone Marrow Transplant.</i> | 25822223 |

6th Edition of the EBMT Indications Manuscript

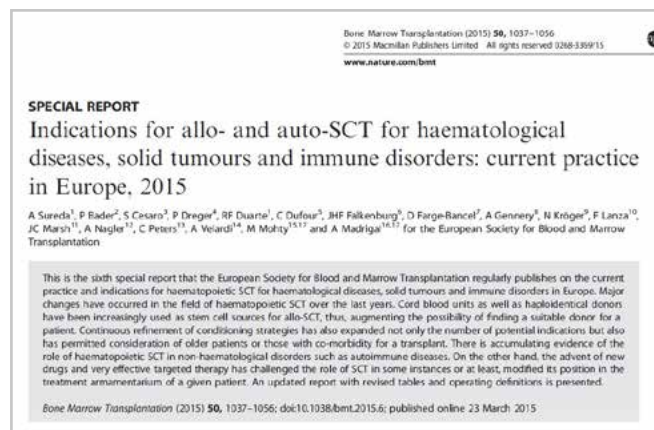
The 6th Edition of the EBMT Indications Manuscript was published in *Bone and Marrow Transplantation* in 2015. It is the result of a common effort from every single member of the EBMT Board representing each Working Party as well as the Executive Committee.

The Indications Manuscript has significantly increased in length, complexity and number of references over time with the objective to give a comprehensive overview to the reader of the indications of HSCT in different diseases and different population of patients.

It is one of the mostly read and cited manuscripts in *Bone and Marrow Transplantation* and it is extensively used not only for scientific and educational purposes but also for regulatory purposes. In this sense, the elaboration of the Indications Manuscript represents a major task to be undertaken by the EBMT Board in order to accomplish all the prior described objectives.

The treatment of haematological malignancies has significantly changed in the last few years and the position of HSCT in the therapeutic armamentarium of a given patient has been modified by the introduction of targeted therapies in the market. In addition to that, new sources of hematopoietic stem cells, e.g. haploidentical stem cells, are being increasingly used in the allogeneic setting and have increased the possibility to find a donor for a patient to virtually 100%. Therefore, taking into consideration that this scientific field is moving so quickly, the Indications Manuscript will be updated every two years rather than every four years. Updated versions will focus on significant changes happening in the specific indications for stem cell transplantation in the various malignant and non-malignant diseases. We hope that all these changes to be done in future editions will make the Indications Manuscript an even more attractive and useful tool for the transplantation community.

Anna Sureda
EBMT Secretary



EBMT Transplant Activity Survey 2014



Haematopoietic Stem Cell Transplantation (HSCT) in Europe 2014: more than 40,000 reported transplants.

A record number of 40,829 HSCTs (16,946 (42%) allogeneic and 23,883 (58%) autologous) in 36,469 patients were reported by 656 centres in 47 countries for the 2014 survey. There were 410 teams (62%) performing both allogeneic and autologous transplants; 227 teams (35%) restricted their activity to autologous HSCTs, 13 teams (2%) restricted their activity to allogeneic transplants only and six teams (1%) reported having performed no transplants in 2014. Of the 656 active centres, 118 (18%) centres performed transplants on both adult and paediatric patients, 431 (66%) centres performed transplants on adults only and an additional 107 (16%) centres were dedicated paediatric transplant centres. Compared with the 2013 survey, the total number of transplants increased by 4.1% (4.5% allogeneic HSCTs and 3.8% autologous HSCTs).

Recent trends include continued increase in the use of haplo-identical family donors (by 25%), slower growth for unrelated donor HSCTs and a continued decrease in cord blood use.

When compared to Western European countries, a much higher growth rate in actual transplant activity is observed in Eastern European countries despite lower transplant rates in general. For autologous HSCT we see for the first time since 1990 that over 50% of patients had plasma cell disorders.

Compared to 2013, there were increases in allogeneic HSCT for acute myeloid leukaemia (AML) in first complete remission (CR1) by 13%, myeloproliferative neoplasms (MPN) by 14% and severe aplastic anaemia (SAA) by 12%. A decrease was seen in chronic lymphocytic leukaemia (CLL), by 21%. For autologous HSCT there were increases in the following: in myeloma by 5%, amyloidosis by 44%, Hodgkin's lymphoma by 8% and autoimmune disease by 40%.

Main indications in 2014 were leukaemias, 11,853 (33% of total transplants; 96% of which were allogeneic); lymphoid neoplasias including Non-Hodgkin's lymphoma, Hodgkin's lymphoma, and plasma cell disorders, 20,802 (57%; 11% allogeneic); solid tumours, 1,458 (4%; 3% allogeneic); and non-malignant disorders, 2,203 (6%; 88% allogeneic) (figures 1a, 1b). Autologous HSCT for non-malignant disorders predominantly included patients with autoimmune disorders.

Figure 1a: Main indication for transplant: allogeneic HSCT

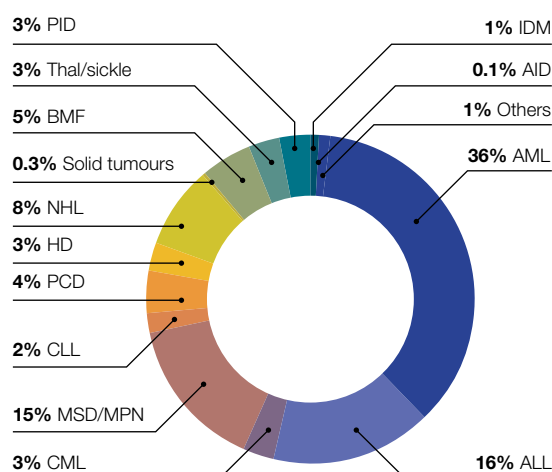
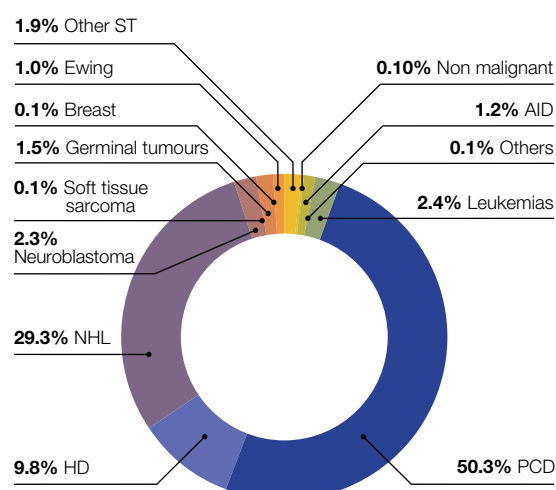


Figure 1b: Main indication for transplant: autologous HSCT



Abbreviations:

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

EBMT Survey on Cellular and Engineered Tissue Therapies

The other main survey performed by the Activity Survey Data Office describes the activity in Europe for the year 2013 in the area of *cellular and engineered tissue therapies*, excluding haematopoietic stem cell treatments for the reconstitution of haematopoiesis. A total of 318 teams from 31 countries responded to the Survey; 145 teams from 25 countries reported treating 2,187 patients, while a further 173 teams reported no activity. Indications were musculoskeletal/rheumatological disorders (45%; 89% autologous), cardiovascular disorders (20%; 99% autologous), haematology/oncology, predominantly prevention or treatment of GvHD and haematopoietic stem cell (HSC) graft enhancement (19%; <1% autologous), neurological disorders (3%; 100% autologous), gastrointestinal disorders (2%; 32% autologous) and other indications (11%; 67% autologous). The majority of autologous cells (88%) were used to treat musculoskeletal/rheumatological (57%) and cardiovascular (27%) disorders, whereas allogeneic cells were used mainly for haematology/oncology (64%). The reported allogeneic and autologous cell types for both allogeneic and autologous therapies were mesenchymal stem/stromal cells (MSCs) (49%), HSCs (28%), chondrocytes (11%), dendritic cells (2%), keratinocytes (1%) and others (9%). In 46% of the grafts, cells were delivered following *ex vivo* expansion, sorted in 17% of the reported cases and transduced in only 3%. Of treatments administered, 33% were delivered intravenously or intra-arterially, and of the remaining 67%, 37% used a membrane/scaffold, 28% a suspension and 2% a gel. The data are compared to those previously collected to identify trends in a still unpredictably evolving field.

Helen Baldomero **Jakob Passweg**
EBMT Activity Survey Data Office, Basel, Switzerland



The EBMT Registry

Data overview

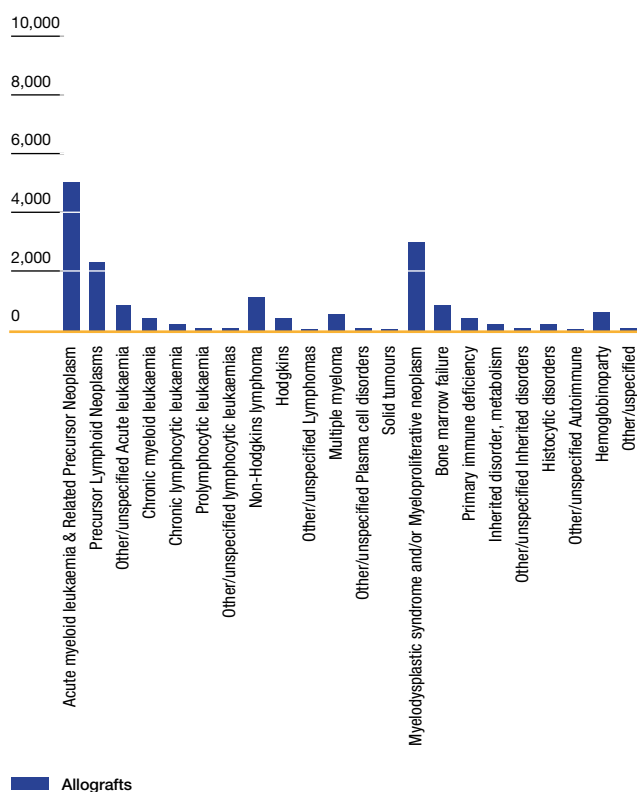
A total of 467,805 patients and 552,344 transplants appear as registered in the EBMT Registry at the beginning of 2016. Of these, 35,316 transplants were registered during 2015. The total number of registrations is slightly lower than last year, which may indicate that some centres are behind with their data reporting.

Data managers from the transplanting centres directly entered 78% of the transplants (a 4% increase on last year), with the rest being entered by a mixture of National Registry and EBMT staff.

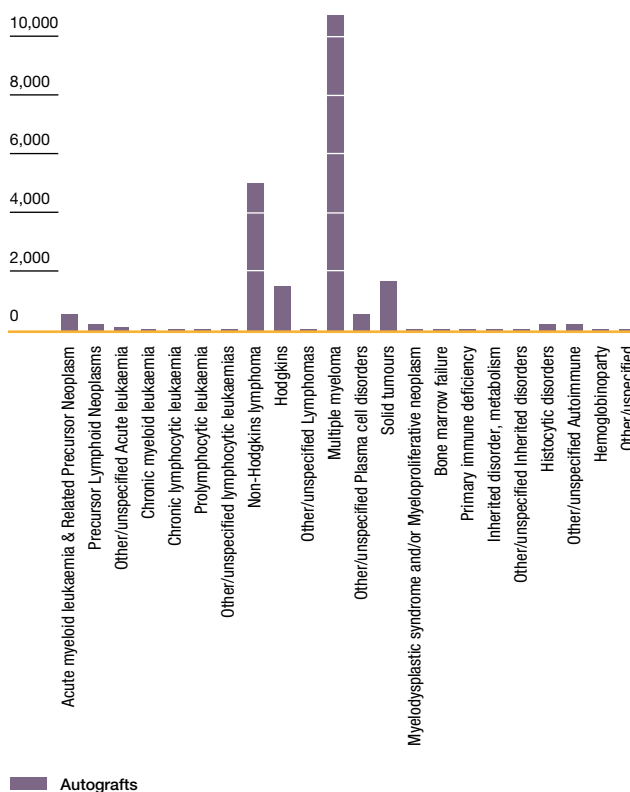
“Project 2020”

The EBMT is focusing on achieving a new Registry system to improve the level of science and facilitate cooperative studies and to continue serving HSCT community and improve patient care. With this in mind, the EBMT Board has launched a new project called “project 2020” with the objective to implement a novel Registry system by 2020. Please read the “Message from the President” for additional information about this project.

Allografts



Autografts





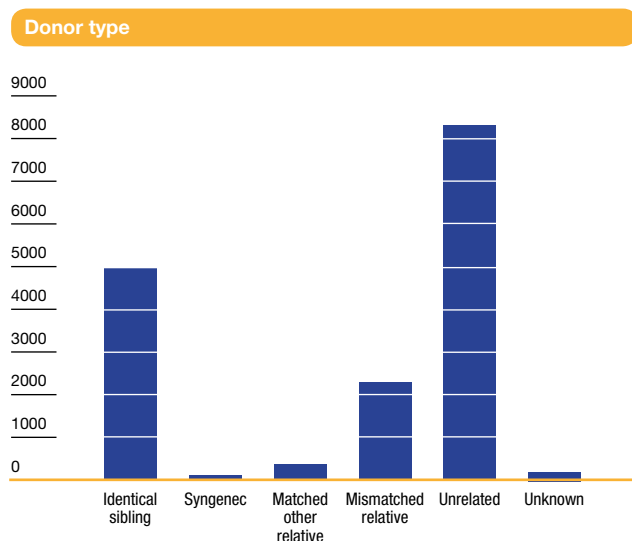
Data sharing

A Growable Network Information System (AGNIS) project

During 2015, data were transferred to the Center for International Blood & Marrow Transplantation (CIBMTR) for selected member centres, both for first registrations and for follow-ups. The forwarding of data is still not regular as the CIBMTR imposes some restrictions on which types of transplants are being accepted which will be raised as the project proceeds.

Human Leukocyte Antigen (HLA) data

The Registry Office was expecting to upload HLA data from selected donor registries to the system but it had to be postponed due to formatting problems with the incoming files and the lack of time to re-identify the patient/donor pairs. The Registry Office and a certain number of centres and National Registries are continuing to enter HLA typing reports directly in the Registry Database. During 2015, HLA data were reported for more than 7,000 patients (about 50% of the number of allogeneic transplants reported during the year).



Data collection and quality

Med-A

The new extended MED-A form was approved by the Board and after thorough testing at the Registry Office we were able to implement it in the Registry as far as it was possible with the current system. EBMT data are now collected on day 0 as well as day 100, in addition to the follow-ups. By submitting data as close as possible to the transplant, we expect to increase the accuracy of the data and facilitate the work of the data managers. The expanded dataset also allows researchers access to more disease-specific data. However, these changes do entail an increased workload for the data managers. The implementation was finalised in December 2015 and it is now possible to enter data. However, harmonisation of the MED-B form and approval of the changes needed for the manuals are still pending. Work is also being done on updating the QueriesP2 module for data merging.

Data management training

During 2015, the Registry Office launched new training videos for entering and retrieving EBMT data using the current system ProMISe. The online videos cover data entry (beginners and advanced), HLA entry and basic data retrieval. They provide remote training for new users and also act as useful refreshers for those who have already attended training sessions.

Cell therapy

Data collection forms have been drafted to enhance data collection for cell therapies not aimed at haematopoietic transplants. These forms are being discussed within the Cell Therapy Committee of the CTIWP.

Carmen Ruiz de Elvira

Head of the EBMT Registry

Education

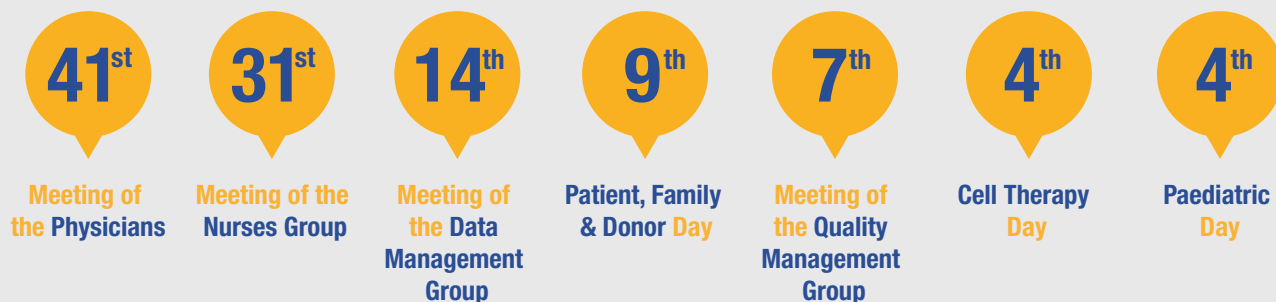
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| Infographic EBMT 41st Annual Meeting | 43 |
| Awards | 47 |
| Educational events 2015 | 49 |

EBMT 2015 41ST ANNUAL MEETING

OF THE EUROPEAN SOCIETY FOR BLOOD AND MARROW TRANSPLANTATION

22nd - 25th March 2015
Istanbul, Turkey

The EBMT Annual Meeting is the Society's flagship event. It brings together scientists, physicians, nurses, patients, statisticians, quality managers, data managers, 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



¹ EBMT 2015 Delegates survey (562 respondents)

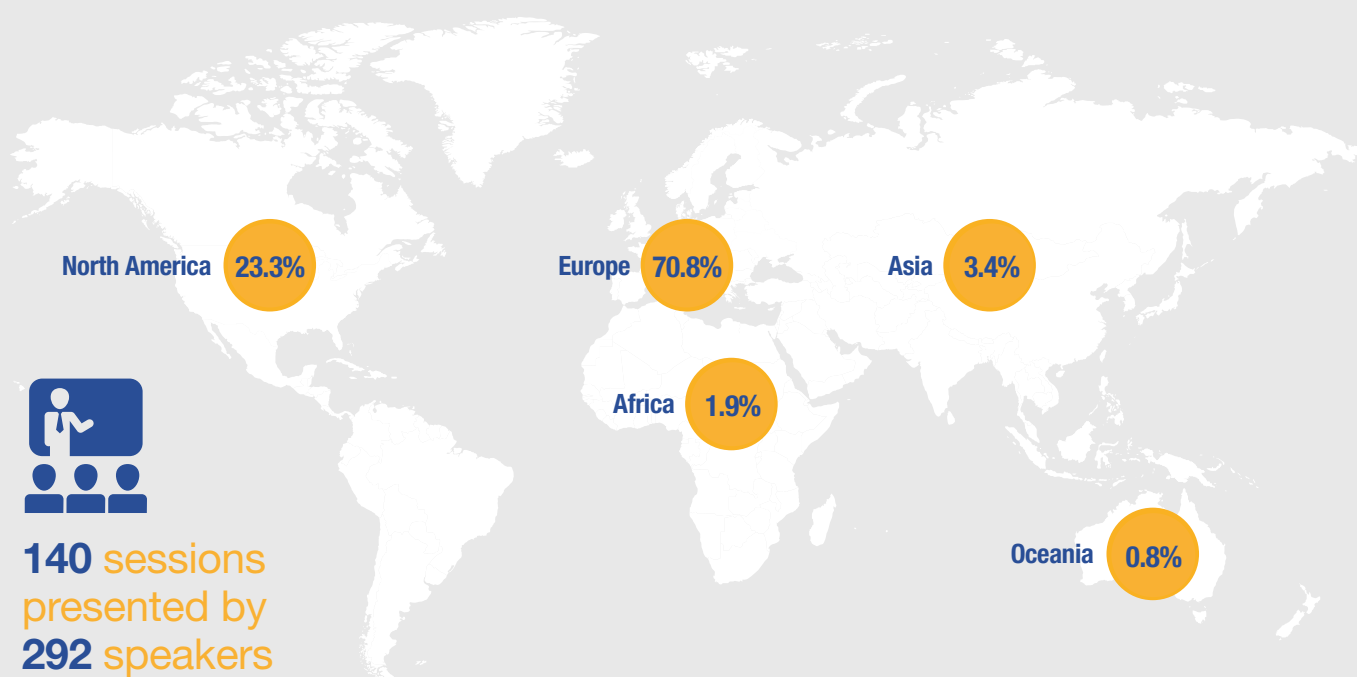


¹ **87%** of delegates rated Istanbul as a "very good" to "excellent" host city

Programme



Regional spread of speakers



New features to the Annual Meeting Programme



Meet the expert



How do I... ?



Social media



9 Awards

given to researchers for their outstanding contributions to science and to the EBMT



²**83%**

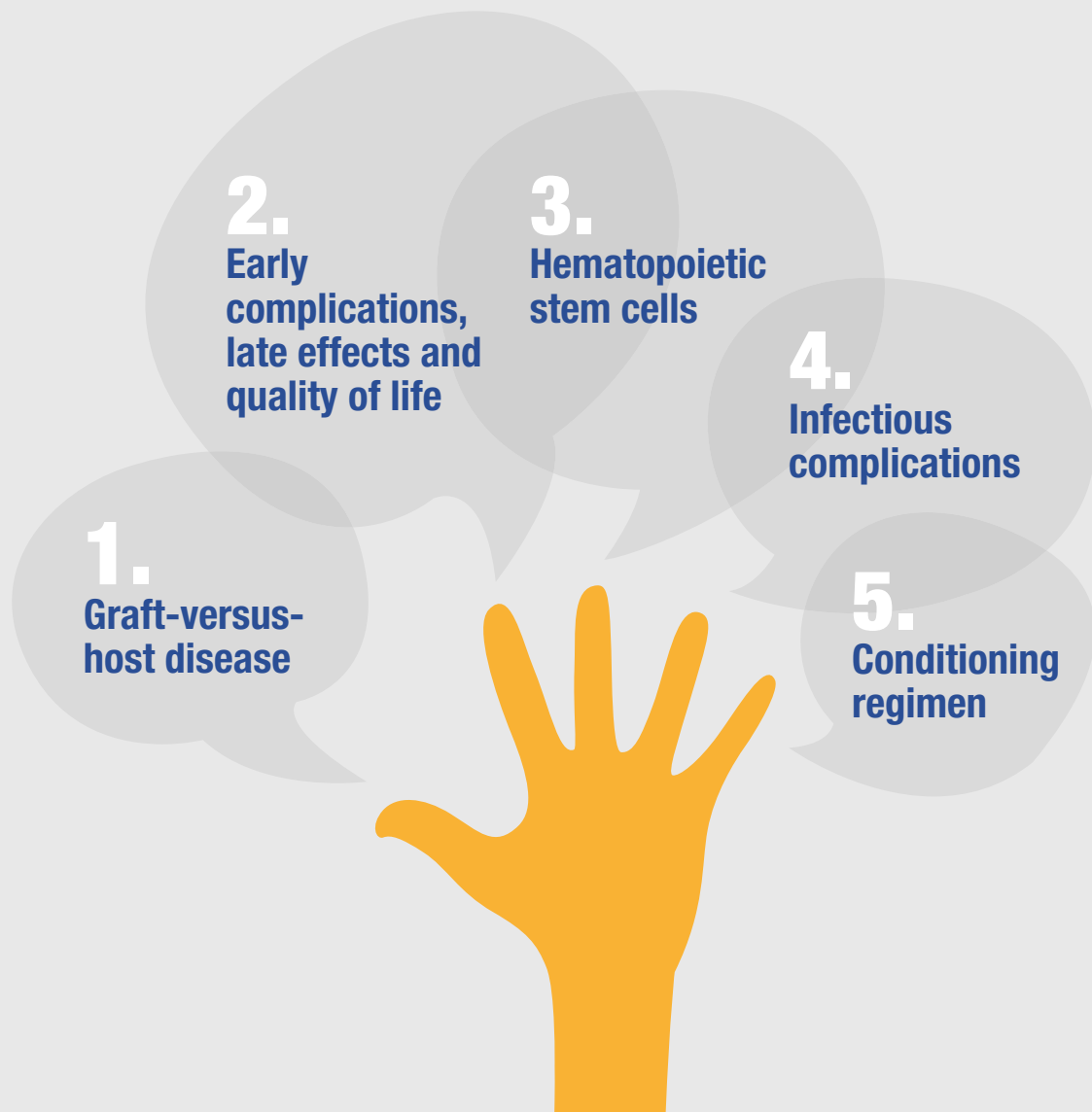
of the delegates rated the scientific programme as "very good" to "excellent"



³**90%**

of participants rated the Patient, Family and Donor Day as "very good" to "excellent"

⁴ Top five participants' areas of interest



² EBMT 2015 Delegates survey (514 respondents)

³ EBMT 2015 Delegates survey (503 respondents)

⁴ EBMT 2015 Delegates survey (563 respondents)

Sponsoring and Exhibition

53

companies, associations or groups supported the 41st Annual Meeting

41

exhibiting organisations in **780m²** exhibition area

24

sponsored sessions

Networking events



2,499

delegates attended the opening session and welcome reception



817

delegates attended the networking evening

Online tools



315

people sent **1,690** tweets using #EBMT15



Reaching **937,795** people



953

downloads of EBMT 2015 App



2,957

subscriptions to the online learning portal



5,870

visitors generating **17,030** views of the online Abstract Book⁵

⁵ Nature Publishing Group statistics from 20th March to 23rd of June

Awards



From left to right: T. Demirer, F. Bonifazi, M. Mothy, N. Kröger, C. Solano

Van Bekkum Award presented to three investigators of the ATGfamily study, Nicolas Kröger (principal investigator and lead author), Francesca Bonifazi (national study coordinator for Italy) and Carlos Solano (national coordinator for Spain and presenting author) for the abstract entitled: *Improved GvHD/Relapse-free survival after HLA-identical sibling PBSC transplantation with anti-lymphocyte globulin. A prospective, multicenter, multi-national randomized phase III trial (ATGfamily study).*



From left to right: M. Mothy, M. Özcan, T. Demirer, R. Shouval, A. Babic, A. Nagler, J. Gissing

Basic Science Award, sponsored by Clinigen Group plc, presented to Roni Shouval, lead author of the abstract entitled: *Exploring Limiting Factors in the Prediction of Allogeneic HSCT Related Mortality: An In-Silico Machine Learning Analysis of the Acute Leukemia Working Party (ALWP) Registry of the EBMT.*



From left to right: T. Demirer, M. Mothy, T. de Witte, M. Özcan, M. Arat

Honorary Membership awarded to Theo de Witte.



From left to right: P. Dreger, J. Gayoso

Jian-Jian Luan Award for Lymphoma Transplant presented to Jorge Gayoso, lead author of the abstract entitled: *Haploidentical transplantation (HAPLO-HSCT) with busulfan based Reduced Intensity Conditioning (RIC) regimens and Post-Transplant Cyclophosphamide (PTCy) as GvHD prophylaxis in patients with relapsed/refractory Hodgkin Lymphoma (HL): Spanish Experience.*



From left to right: J. Petan, L. Martin Schwab, R. Zeiser, M. Hauri-Hohl, H.J Kolb, C. Bonini, C. Chabannon

Jon J. van Rood Award for the Best Paper in the Immunology of Allogeneic Haematopoietic Transplantation, sponsored by Neovii Pharmaceuticals, presented to Robert Zeiser and Lukas Martin Schwab for: *Neutrophil granulocytes recruited upon translocation of intestinal bacteria enhance graft-versus-host disease via tissue damage*. And to Mathias M. Hauri-Hohl for his paper entitled: *A regulatory role for TGF- β signaling in the establishment and function of the thymic medulla*.

The Best Clinical Poster Award, sponsored by Nature Publishing Group, presented to Miguel Angel Diaz for his poster entitled: *Outcome and risk factors for pediatric patients receiving an haploidentical transplantation using CD3/CD19 depleted grafts*.

The Best Science Poster Award, sponsored by Nature Publishing Group, presented to Julia Marie Ritter for her poster entitled: *T-cell receptor deep sequencing analysis of EBV specific T cells before and after adoptive transfer in a patient after allogeneic stem cell transplantation*.



From left to right: E. Trigo, A. Babic, H. Rip-Mekelenkamp

Nurses Group – Best Oral Presentation Award presented to Hilda Rip-Mekelenkamp and her colleagues from The Netherlands for: *Parental experiences and perspectives of end-of-life decision-making in allogeneic paediatric stem cell transplant*.

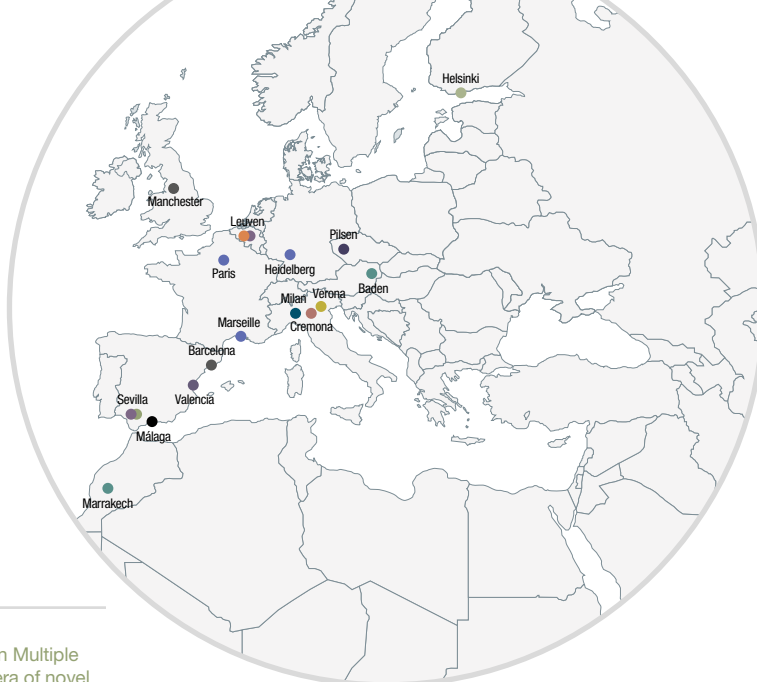


From left to right: A. Babic, C. Bompont, S. Erdal

Nurses Group – the 7th Distinguished Merit Award was presented to Caroline Bompont.

Nurses Group – Best Poster Award presented to Fameeda Palk for: *Safe discharge of a non-English speaking family from the international paediatric HSCT unit at Great Ormond Street Hospital*.

Educational events 2015



| | |
|--|--|
| Seville, Spain 23 - 24 Jan. 2015 55 attendees | CMWP "The role of stem cell transplantation in Multiple Myeloma and related diseases in the era of novel drugs" |
| Paris, France 30 Jan. 2015 97 attendees | ALWP "Master Classes in Transplantation and Hematology (MATH) [®] : A Focus on Conditioning" |
| Leuven, Belgium 24 Apr. 2015 40 attendees | IEWP IEWP Workshop: How to transplant a patient with primary immunodeficiency |
| Marrakech, Morocco 14 - 17 May 2015 40 physicians 25 nurses attendees | PDWP The 5 th EBMT Training Course for Paediatricians and Paediatric nurses on HSCT in children and adolescents Developing hematopoietic stem cell transplantation in Africa |
| Málaga, Spain 21 - 23 May 2015 205 attendees | ESH-EBMT 19 th Annual ESH-EBMT Training Course |
| Marseille, France 29 - 30 May 2015 158 attendees | ALWP ALWP Workshop: Advances in alternative donor stem cell transplantation: A Euro-Mediterranean perspective |
| Manchester, UK 2 - 3 July 2015 27 attendees | JACIE JACIE 28 th Inspector Training Course |
| Helsinki, Finland 11 - 12 Sept. 2015 65 attendees | CMWP CMWP Business Meeting and educational course: "New developments in allogeneic SCT for the treatment of MDS and MPN" |
| Heidelberg, Germany 24 - 26 Sept. 2015 93 attendees | LWP 11 th Educational Course of the LWP: "Treatment of Malignant Lymphoma: State-of-the-Art and Role of Stem Cell Transplantation" |
| Baden, Austria 29 Sept. - 1 Oct. 2015 33 attendees | PDWP PDWP Expert Workshop on Fertility Preservation in the context of HSCT |

| | |
|---|--|
| Cremona, Italy 30 Sept. - 1 Oct., 2015 86 attendees | STWP STWP Educational Course: A multispecialist pathway towards a therapy and health-educational model |
| Barcelona, Spain 1 - 2 Oct. 2015 26 attendees | JACIE 29 th JACIE Training Course |
| Pilsen, Czech Republic 9 Oct. 2015 80 attendees | NG Nurses Group International Study Day |
| Seville, Spain 16 Oct. 2015 80 attendees | IEWP IEWP Educational Meeting 'How to transplant a patient with Primary Immunodeficiency' |
| Seville, Spain 16 - 18 Oct. 2015 130 attendees | IEWP IEWP Autumn Meeting |
| Leuven, Belgium 29 - 30 Oct. 2015 100 attendees | CQLWP CQLWP Educational Course: Thinking Outside the Box: Going beyond 'Survival after Stem Cell Transplant' |
| Verona, Italy 29 - 30 Oct. 2015 63 attendees | IDWP 18 th Training Course of the IDWP |
| Valencia, Spain 6 Nov. 2015 145 attendees | NG Spanish Nurses Group: 9 th Day for Nurses in HSCT |
| Milan, Italy 11 - 13 Nov. 2015 250 attendees | CTIWP The 1 st CTIWP Scientific Symposium: "From Transplantation to Gene Therapy: Cellular therapy in Evolution" |
| Paris, France 27 - 28 Nov. 2015 83 attendees | ALWP Winter Meeting & 8 th Educational Symposium of the ALWP: "Reduction of leukemic relapse after ALLO-HSCT: How to move forward?" |

- **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**

Patient Care



Haematology and HSCT Nursing

51

Standards and Accreditation

53

Haematology and HSCT Nursing



The EBMT Nurses Group (NG): committed to high-quality patient care through **education**, **research** and international **collaboration**.

The EBMT NG, representing more than 800 nurses and allied health professionals, 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. The EBMT NG's Mission is to enhance and value the nursing role all over the world, supporting and sharing knowledge through communication, advocacy, research, training and education.

Education

Once again the Scientific Committee organised the Nurses Programme of the EBMT 2015 Annual Meeting in Istanbul and the Education Day, both of which had increased attendance. Over 500 nurses attended the EBMT 2015 Annual Meeting, 126 abstracts were submitted, and noteworthy speakers contributed to a dynamic meeting. The presentations of the Education Day and of the International Study Day are available on the Nursing section of the EBMT website.

Materials

Patient safety and how we as nurses can contribute to reducing risk and hopefully avoiding errors, which can occur during the numerous complex activities that we undertake in our daily practice, was evidenced by launching the first EBMT NG Documentary entitled: "Do we all do it the same way?".



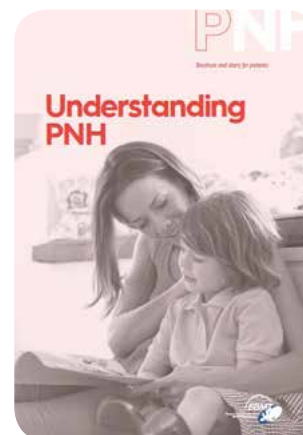
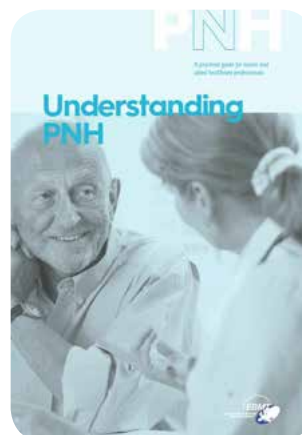
The Veno-Occlusive Disease (VOD) Learning Programme, that was already available on the EBMT website has now been translated in eight languages.

Active management of severe veno-occlusive disease



Veno-occlusive disease (VOD) Learning Programme

Practical guides on Paroxysmal Nocturnal Haemoglobinuria: "Understanding PNH", have been launched and include a brochure and diary for patients, and a practical guide for nurses and allied healthcare professionals. All materials are available on the EBMT website.



Research

The Research Committee has performed research looking at the barriers and facilitators in discussing sexual concerns with people following HSCT, and together with the Complications and Quality of Life Working Party (CQWP), is leading on a study (S-FAST) of sexual functioning in adults post allogeneic Stem Cell Transplantation.

Other ongoing research projects include several collaborative projects where the Research Committee is supporting and facilitating NG members' initiatives.



Collaborations

We had a successful National Groups & Forums Chairs Meetings 2015, following the International Study Day at the Pilsner Urquell Brewery Conference & Meeting Centre in Pilsen, in the Czech Republic on the 8th and 9th of October 2015. The meeting was organised together with the Haemato-Oncology Department of the University Hospital in Pilsen.

We are delighted to announce the Memorandum of Understanding that has been signed between the EBMT and the Haematology Society of Australia and New Zealand (HSANZ) Nurses Group. The HSANZ will attend the EBMT 2016 Annual Meeting in Valencia and present their group and recent activities.

We are strengthening collaborations with other healthcare professional communities that share common interests, such as the Multinational Association of Supportive Care in Cancer (MASCC)/ International Society of Oral Oncology (ISOO) regarding basic oral assessment; a position paper about basic oral care has been prepared.

Another collaboration is between the EBMT NG and another nonprofit association, Nurses No Frontiers, for an outreach project aiming to promote HSCT nursing education and practice in low- and middle-income countries. The Training Course for HSCT Nurses – China in Guangzhou Hospital has been organised and will take place in February 2016.

The NG Paediatric Committee and Paediatric Diseases Working Party (PDWP) held the 5th EBMT Training Course for Paediatricians and Paediatric nurses on HSCT in children and adolescents entitled “Developing hematopoietic stem cell transplantation in Africa” on the 14th–17th May 2015 in Marrakesh, Morocco. This gave nurses from different cities and hospitals in Morocco the possibility to network and share experiences with European nurses.

Our work during 2015 has set the scene for further development and growth in terms of research, education and collaboration. We look forward to being able to extend this in 2016, continuing our commitment to promoting excellence in patient care.

Aleksandra Babic
EBMT Nurses Group President

5th Training Course for Paediatricians and Paediatric Nurses on HSCT in Children and Adolescents in Marrakech



Standards and Accreditation

Improving safety and quality in transplant and cellular therapy

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.

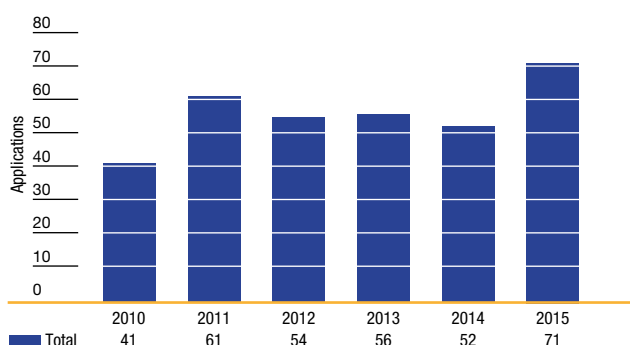
JACIE works continuously with international partner organisations to develop and maintain standards for the provision of quality medical and laboratory practice in HSCT, performs on-site inspections, and accredits those programmes that demonstrate compliance with these standards. JACIE also provides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

Since 2000, 360 transplant programmes and facilities in 25 countries in Europe and beyond have applied to JACIE and 473 inspections (first-time and reaccreditation) have been performed. Over 270 applicants have achieved accreditation at least once with practically all centres repeating the process after completing the initial accreditation cycle. There are over 300 registered inspectors, all volunteers drawn from the cellular therapy field.

Applications

In 2015, 71 applications (26 first-time and 45 reaccreditation) were received. The deadline in February 2015 to apply under the 5th edition of the Foundation for the Accreditation of Cellular Therapy (FACT)-JACIE Standards led to a high number of applications in the early part of the year but this momentum continued throughout the rest of the year, making 2015 our best year to date for applications (See figure 1).

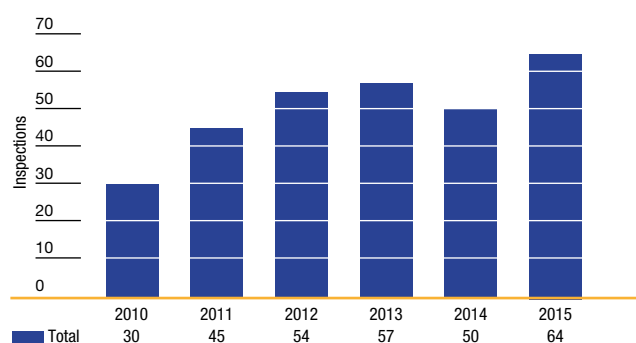
Figure 1. Number of applications received for the past six years



Inspections

In 2015, 64 inspections (29 first-time and 35 reaccreditation) were performed. Again, 2015 was a record year, surpassing the previous high of 57 inspections carried out in 2013. A key factor in being able to schedule a high number of inspections was the expansion of the JACIE Office team to two Accreditation Coordinators. However, it bears repeating that it is the incredible commitment of the inspectors that makes all of this possible (See figure 2).

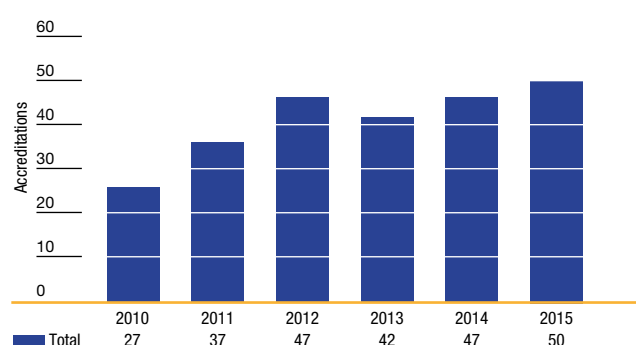
Figure 2. Number of inspections performed for the past six years



Accreditations

In 2015, 50 accreditations (25 first-time and 25 reaccreditation) were awarded. Not surprisingly, this also established a new record (See figure 3).

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



6th Edition of FACT-JACIE Standards

The 6th edition of the FACT-JACIE Standards was released in March 2015. This edition introduced new elements including benchmarking of outcome, specific pharmacist and physician-in-training standards, minimum continuous education requirements, use of accredited chimerism testing techniques, ambulatory care and late effects.

Educational events

Two successful JACIE training courses were held: one in Manchester, UK and one in Barcelona, Spain. Fifty-three participants attended in total. In addition, 24 participants from South America joined the Spanish-language webinar in May. JACIE was also represented in a number of other events and projects (See figure 4).



Figure 4. Training, Events, Representation & Participation

Ankara, Turkey
January
EU-TR Project

Riyadh, S. Arabia
9 May
SSBMT Workshop

Split, Croatia
3-4 October
EATB Congress

Tehran, Iran
16-17 January
Iranian Society of HSCT

Cairo, Egypt
6 June
EHOG Annual Meeting

Dublin, Ireland
6 November
Information Day

Brussels, Belgium
6 March
BHS National Meeting

Manchester, UK
2-3 July
JACIE Training

Paris, France
11 December
ARTHIQS Joint Action

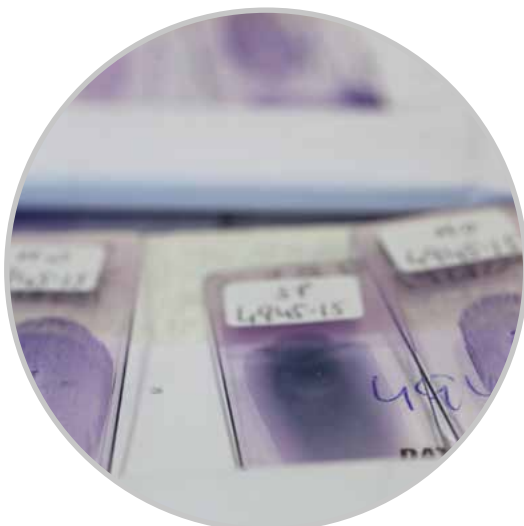
Malaga, Spain
7 March
GETH Meeting

Rome, Italy
23 September
ARTHIQS Joint Action

Online Webinar
(in Spanish)
20 May
III LABMT-JACIE

Warsaw, Poland
24 April
Praktyka
Hematologiczna

Barcelona, Spain
1-2 October
Inspector Course





Participants at the JACIE Training Course, Manchester, UK

Other

Prof. John Snowden was appointed Chair of the JACIE Committee by the EBMT Board in March 2015, succeeding Alessandro Rambaldi. The Medical Director's position was filled at the end of 2015 by the appointment of Dr. Riccardo Saccardi.

The JACIE website recorded 17,055 unique visitors, a 32% rise over 2014. Four newsletters were published and since late 2015, JACIE News is now fully incorporated into the EBMT Newsletter. The JACIE Twitter account grew to 280 followers.

JACIE, represented by Eoin McGrath, was invited to form part of the External Assessment Board of the Assisted Reproductive Technologies & Haematopoietic stem cells Improvements for Quality & Safety throughout Europe (ARTHIQS) Joint Action of the EU (www.arthiqs.eu). JACIE also contributed to the EU-Turkey Technical Assistance Project on Alignment in Human Tissues and Cells (tinyurl.com/zjvu487). JACIE experts were invited to contribute to 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).

The JACIE Office team expanded to three with the incorporation of Raquel Espada as the second Accreditations Coordinator alongside Iris Bargalló. Raquel became a permanent member of the team after originally joining in 2015 to cover Iris' maternity leave.

As ever, I would like to express my appreciation and admiration for the Inspectors, Committee Members and other volunteers for their tremendous hard work, commitment and dedication.

Eoin McGrath

JACIE Executive Officer

Institutions awarded accreditation in 2015

AZ Sint-Jan Brugge-Oostende AV (Bruges, Belgium); Institut Jules Bordet (JJB); Hôpital Universitaire des Enfants Reine Fabiola (HUDERF); Hospital Saint-Pierre (Brussels, Belgium); Hôpital de Jolimont (Haine-Saint-Paul, Belgium); UZ Leuven (Leuven, Belgium); Hôpital Robert Debré (Paris, France); Hôpital Saint Louis, Assistance Publique – Hôpitaux de Paris AP-HP (Paris, France); Hôpital Saint-Antoine – Assistance Publique – Hôpitaux de Paris (Paris, France); CHU Poitiers (Poitiers, France); Universitätsklinikum Knappschafts Krankenhaus Bochum GmbH (Bochum, Germany); Universitätsklinikum Greifswald (Greifswald, Germany); Universitätsmedizin der Johannes Gutenberg Universität Mainz (Mainz, Germany); University Medical Center of Johannes Gutenberg University (Mainz, Germany); University Hospital Giessen and Marburg (Marburg, Germany); University of Münster (Münster, Germany); Medizinische Klinik 5, Klinikum Nürnberg Nord (Nürnberg, Germany); Univ. Hospital Regensburg, HSCT (Regensburg, Germany); University of Debrecen Clinical Center. (Debrecen, Hungary); Hadassah University Medical Center (Jerusalem, Israel); Ospedale C. e G. Mazzoni (Ascoli Piceno, Italy); Azienda Ospedaliero Universitaria Consorziale Policlinico di Bari (Bari, Italy); Azienda Ospedaliera Universitaria di Bologna, Policlinico Sant'Orsola-Malpighi (Bologna, Italy); Centro Unico di Trapianto PP.OO. "R.Binaghi" e "Microciternico" (Cagliari, Italy); Fondazione IRCCS Istituto Nazionale dei Tumori (Milan, Italy); Istituto Europeo di Oncologia (Milan, Italy); Azienda Ospedaliero Universitaria di Modena Policlinico (Modena, Italy); Azienda USL Di Piacenza (Piacenza, Italy); Azienda Ospedaliero-Universitaria Pisana (A.O.U.P.) (Pisa, Italy); Arcispedale Santa Maria Nuova IRCCS Reggio Emilia (Reggio Emilia, Italy); AO S. Camilo Forlanini (Rome, Italy); Istituti Clinico Humanitas (Rozzano, Italy); Azienda Ospedaliera "Card. Panico" (Tricase, Italy); Azienda Ospedaliera Ospedale di Busto Arsizio (Varese, Italy); Academic Medical Center (Amsterdam, Netherlands); Leids Universitair Medisch Centrum (LUMC) (Leiden, Netherlands); Leids Universitair Medisch Centrum (LUMC) (Leiden, Netherlands); St. Antonius Ziekenhuis (Nieuwegein, Netherlands); Isala Klinieken (Zwolle, Netherlands); Banc de Sang i Teixits (Barcelona, Spain); Hospital de la Santa Creu i Sant Pau (Barcelona, Spain); Sahlgrenska University Hospital (Göteborg, Sweden); Universitätsspital Basel (Basel, Switzerland); Ospedale regionale di Bellinzona e Valli (Bellinzona, Switzerland); Kantonsspital St.Gallen (St. Gallen, Switzerland); Bristol Royal Hospital for Children & Bristol Haematology/Oncology Centre (Bristol, United Kingdom); Filton Blood Centre (Bristol, United Kingdom); Blood and Bone Marrow Transplant Centre (Leeds, United Kingdom); Royal Liverpool University Hospital (Liverpool, United Kingdom); University College Hospital (London, United Kingdom); Newcastle Upon Tyne Hospitals NHS Foundation Trust (Newcastle upon Tyne, United Kingdom); Sheffield Children's NHS Foundation Trust (Sheffield, United Kingdom)

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

Financial highlights



The EBMT has got for the first time in its history an “unqualified opinion”, we can be proud! The EBMT gained financial stability and provided assurances that the money is spent and allocated according to our Mission. Today, the EBMT has mechanisms of control in place, that demonstrate clear lines of accountability through transparency. These mechanisms have been controlled by external auditors from Ernest & Young and we are glad to announce that during the audit in 2015 the EBMT received an “unqualified opinion” based on the progress of the organisation during the last three years in financial, governance and structural changes.

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 second 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
- better funding for the Clinical Trials and WP Studies
- and last but not least a better capacity of the organisation to be in full control and follow up on its activities.

Spending our money on our missions

In 2015 the EBMT maintains that 82% of its expenses has been dedicated and allocated to its Mission (Studies, Registry, Accreditation and Education) and the remaining 18% allocated to Management (Board and Executive Office expenses). The EBMT continues to develop and build on its strategy for diversification and retention of resources. Continuing in this direction, the EBMT also works hard to assure its ‘non-earmarked’ income (Membership, Sponsoring, Annual Meeting) covers the structural cost of the Society (Registry and Management) and investment in non-commercial academic retrospective and educational studies/activities through our Working Parties (WPs). Our ‘earmarked’ income comes from Pharma grants, which are allocated to specific educational studies/activities for our Clinical Trials Office and WPs.

Tax control framework

Since 2014 the EBMT is acting on the flow chart of the tax control framework and will further implement this framework into the financial policy of the EBMT in 2016 in order to allow a thorough discussion with all local tax authorities.

Investing in “structural innovation”

Innovation of the EBMT depends on the creativity of all members, WPs as well as optimal tools to employ our ideas. Therefore our Society needs to invest in supporting these ideas with optimal structures and tools. After reaching full financial control, the Board decided in early 2016 to use current benefits of our Society from 2015 for structural innovation. In order to secure this ongoing process for the next years the Board also agreed to develop a fixed budget for the future in order to guarantee continuous structural innovation within our Society. The first project that will benefit from this changing financial policy is the “Project 2020”. Please read the “Message from the President” for additional information about this project.

Financial conclusion

The EBMT has achieved full control of all financial aspects, which allows embarking on long-term obligations. The EBMT is also acting on the tax control framework since two years and will implement this framework further into the financial policy of the EBMT in 2016. With this full financial control the Board agreed to preserve 300K€ from 2015 and 300K€ for each upcoming year for structural innovation. The fixed budget for structural innovation will within the next years mainly be used to secure the “Project 2020”. Even with adding a budget of 300K€ for structural innovation for 2015 and earmarked reserves for 223K€, the EBMT will be closing the year 2015 with a total expenses of 3,112K€ and a total income of 3,775K€, thus with a total positive balance of 140K€. The net profit of 140K€ will be returned to the reserves in order to further mitigate financial risks of a continuous “structural innovation” such as “project 2020” and in turn will strengthen the financial and overall position of the EBMT.

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

Jürgen Kuball
EBMT Treasurer

SOURCE OF INCOME

| | 2014 | | 2015 | |
|-----------------------------|--------------|-------------|--------------|-------------|
| | in K€ | % | in K€ | % |
| Membership | 556 | 16% | 614 | 16% |
| Sponsoring | 490 | 14% | 580 | 15% |
| Annual Meeting | 1,220 | 35% | 1,198 | 32% |
| Others | 120 | 3% | 51 | 1% |
| Non-earmarked Income | 2,386 | 69% | 2,443 | 65% |
| Studies & CT & Education | 646 | 19% | 775 | 21% |
| Accreditation (JACIE) | 292 | 8% | 397 | 11% |
| Other Grants | 153 | 4% | 160 | 4% |
| Earmarked Income | 1,092 | 31% | 1,332 | 35% |
| TOTAL Income | 3,447 | 100% | 3,775 | 100% |

HOW EBMT SPENDS THE MONEY

| | 2014 | | 2015 | |
|--|--------------|-------------|--------------|-------------|
| | in K€ | % | in K€ | % |
| Retrospectives Studies | 911 | 28% | 1,012 | 33% |
| Prospective Studies | 367 | 11% | 452 | 15% |
| Educational Activities | 149 | 5% | 95 | 3% |
| EBMT Registry | 506 | 15% | 546 | 18% |
| Accreditation Process (JACIE) | 363 | 11% | 356 | 11% |
| Nurses Activities | 65 | 2% | 103 | 3% |
| Committees Activities | 10 | 0% | 4 | 0% |
| Registry Upgrade/Provision | 386 | 12% | 0 | 0% |
| Total Mission | 2,758 | 84% | 2,567 | 82% |
| Management & Administration | 525 | 16% | 545 | 18% |
| TOTAL Cost | 3,282 | 100% | 3,112 | 100% |

Net Result

| | 2014 | 2015 |
|---------------------------------------|--------------|--------------|
| | in K€ | in K€ |
| TOTAL Income | 3,477 | 3,775 |
| TOTAL Cost | 3,282 | 3,112 |
| Structural Innovation Reserves | | 300 |
| Other Earmarked | 54 | 223 |
| TOTAL Net Result | 141 | 140 |

BALANCE

(financial situation at the 31 December 2015)

| | 2014 | | 2015 | |
|---------------------------------------|--------------|-------------|--------------|-------------|
| € | in K€ | % | in K€ | % |
| Cash & equivalents | 3,073 | 50% | 4,739 | 66% |
| Other current assets | 3,046 | 50% | 2,431 | 34% |
| Total Net Assets | 6,119 | 100% | 7,170 | 100% |
| Earmarked funds | 462 | 8% | 985 | 14% |
| Non-earmarked funds | 2,267 | 37% | 2,400 | 33% |
| Provision | 417 | 7% | 374 | 5% |
| Total Reserves | 3,146 | 51% | 3,759 | 52% |
| Current Liabilities | 2,973 | 49% | 3,411 | 48% |
| Total Liabilities and Reserves | 6,119 | 100% | 7,170 | 100% |



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