EBMT

European Society for Blood and Marrow Transplantation

Annual Report /14

EBMT

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01

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Pierre Fabre Médicament

TERUMOBCT

Source: EBMT March 2015

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The Mission and Vision

The European Society for Blood and Marrow Transplantation (EBMT) is a non-profit organisation that was established in 1974 in order to allow scientists and physicians involved in clinical bone marrow transplantation to share their experience and develop cooperative studies. The EBMT is devoted to the promotion of all aspects associated with the transplantation of haematopoietic stem cells from all donor sources and donor types including basic and clinical research, education, standardisation, quality control, and accreditation for transplant procedures.

OUR MISSION

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

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.

2014 EBMT at a glance

3,896 members in 588 transplant centres in 61 countries

36,940 registered transplants in the EBMT registry

773 transplants using cord blood as source of stem cell, with 29 being done in the autologous setting

658 teams in 48 countries reporting to the 2013 activity survey performed a total of 39,209 transplants in 34,809 patients

223 research studies under way - 8 prospective clinical trials; 170 retrospective studies; 30 non-interventional studies

59 publications in peer-reviewed journals

13 educational events

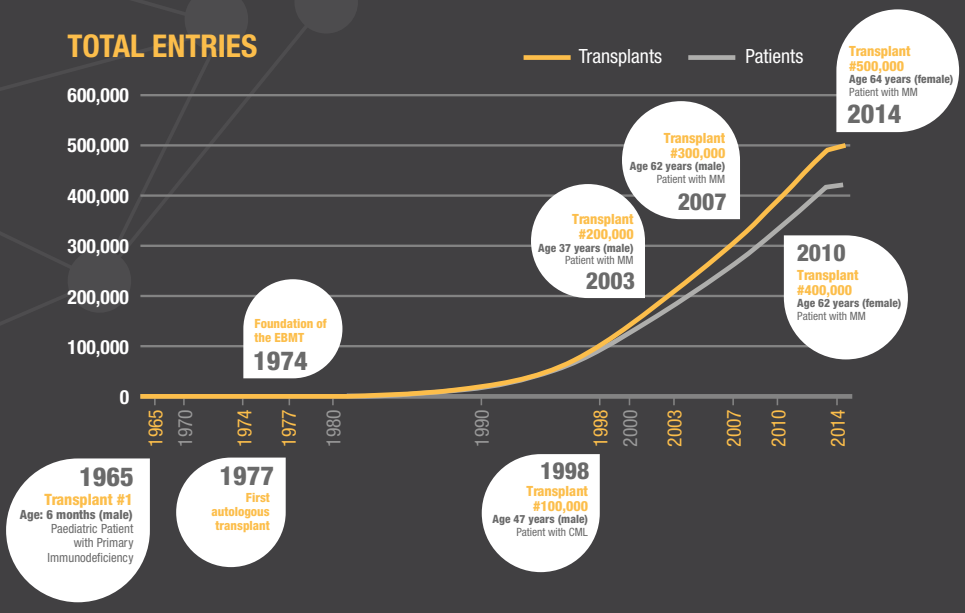
4,625 delegates from 83 countries at the EBMT 2014

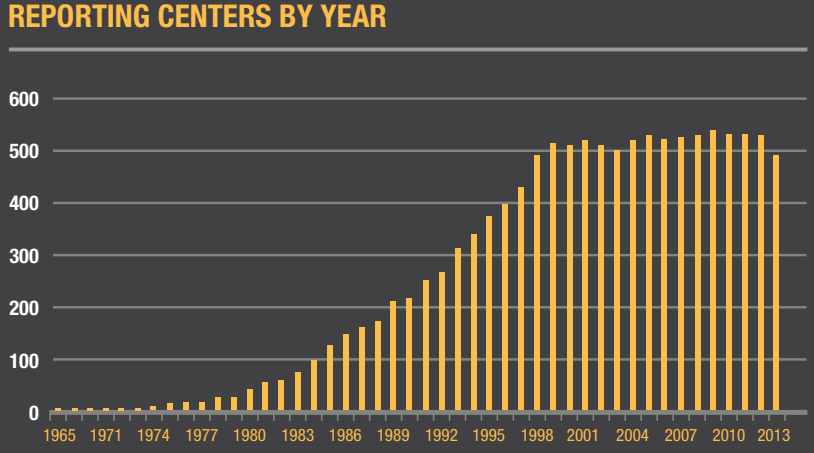
775 remaining copies of the ESH-EBMT handbook distributed at the major congresses and conferences

The EBMT registry has reached a total of

500,000 TRANSPLANTS

Here are some interesting facts about them

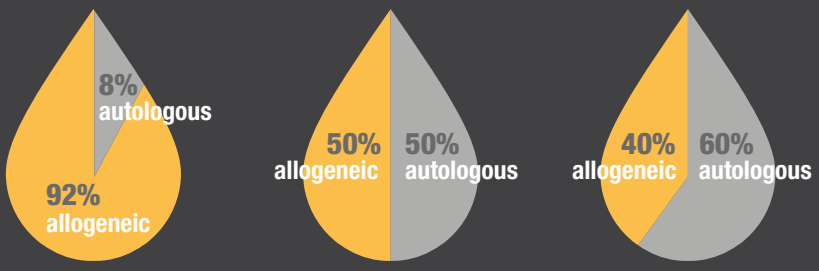




TYPES OF TRANSPLANT

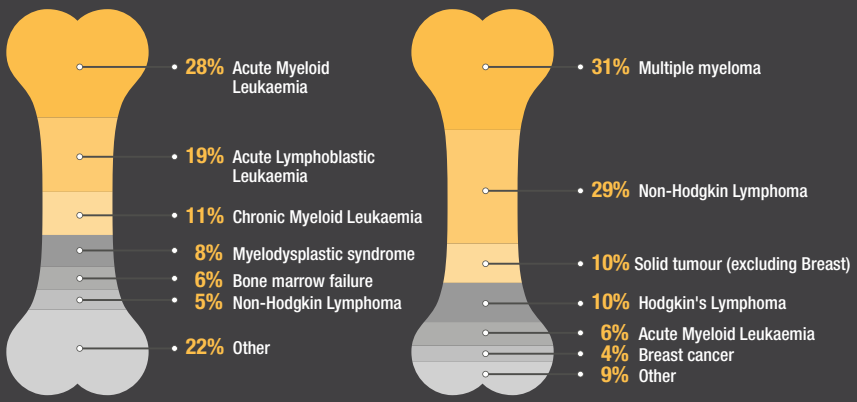
14% Paediatric

86% Adult

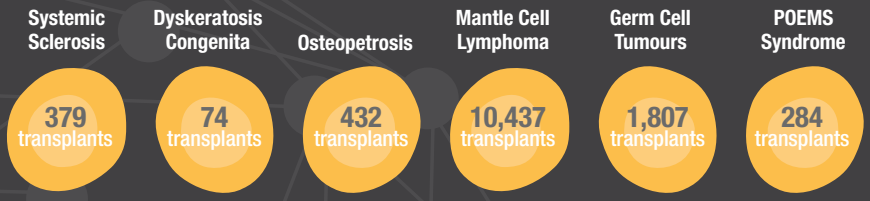


1979 **1993** **2014**

DIAGNOSED DISEASES

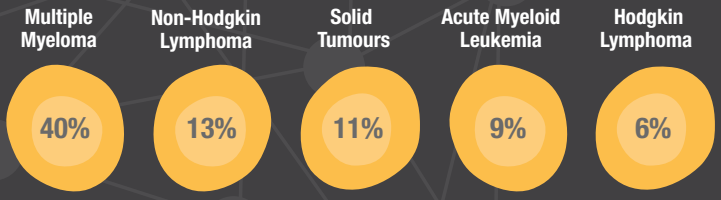


RARE DISEASES



MORE THAN ONE TRANSPLANT

Of those patients who receive more than one transplant, the main diagnosis is



Source: EBMT August 2014

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EBMT structure

BOARD

Executive Committee
President, President Elect, 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 Co-President and Scientific Chair
Taner Demirer & Muhit Özcan - Ankara, Turkey
Mutlu Arat - Istanbul, Turkey

EXECUTIVE COMMITTEE

President
Mohamad Mohty - Paris, France

Secretary
Anna Sureda - Barcelona, Spain

Treasurer
J.H. Frederik Falkenburg - Leiden, 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 & Immunobiology
Andrea Velardi - Perugia, Italy
Chiara Bonini€ Milan, Italy (elected in October 2014)

Infectious Diseases
Simone Cesaro - Verona, Italy

Inborn Errors
Andrew Gennery - Newscastle-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 - Barcelona, Spain

COMMITTEES

Education Committee
Enric Carreras - Barcelona, Spain

Nuclear Accident Committee
Ray Powles - London, UK

CT2-EBMT Committee
Hermann Einsele - Würzburg, Germany

Statistical Committee
Myriam Labopin - Paris, France

JACIE
Alessandro Rambaldi - Bergamo, Italy

Outreach Committee
Erzsebet Benedek - Targu-Mures, Romania

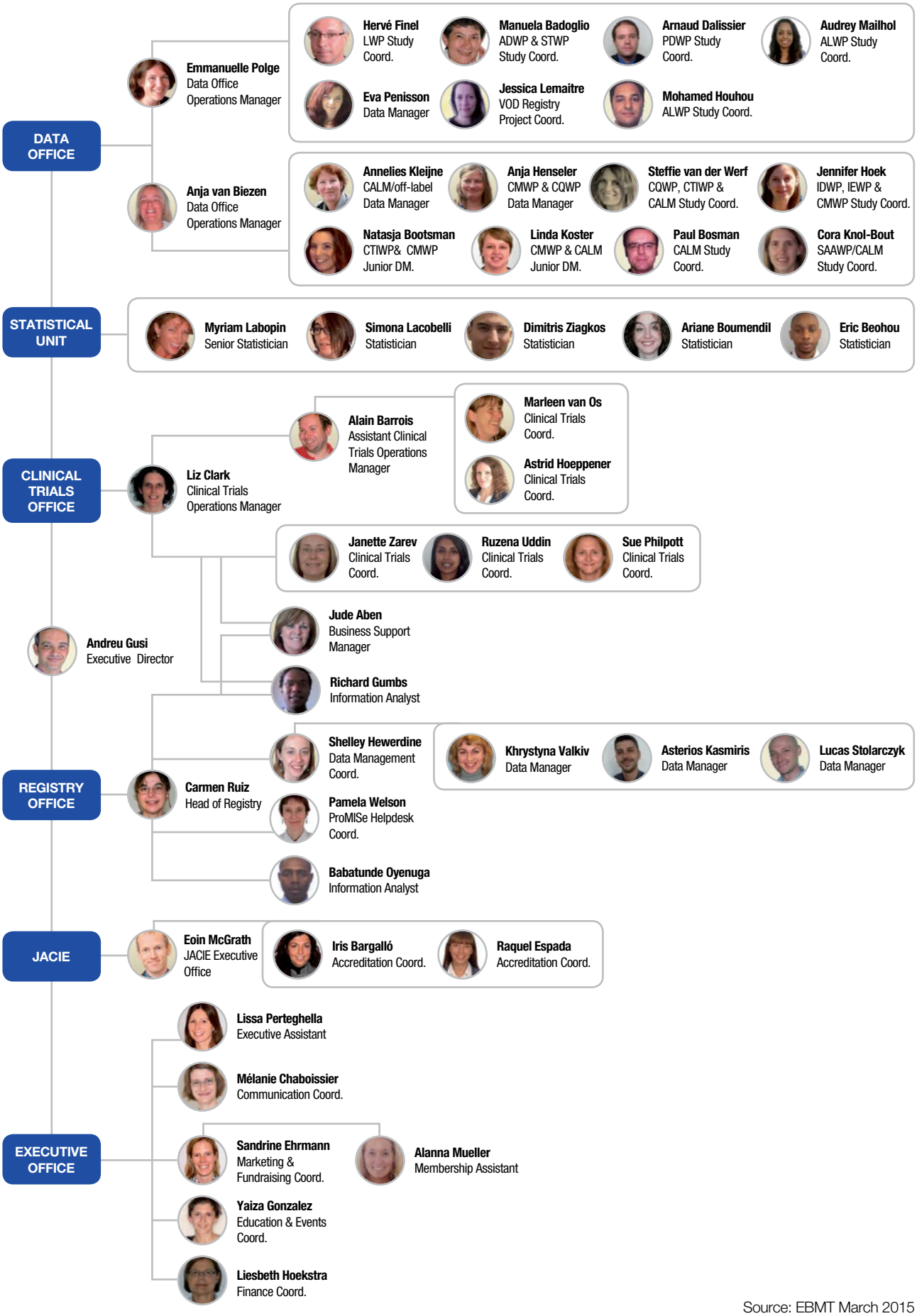
Donor Outcomes Committee
Joerg Halter - Basel, Switzerland

Cell Processing Committee
Christian Chabbanon - Marseille, France

Registry Committee
Per Ljungman - Stockholm, Sweden

Source: EBMT March 2015

Staff organisational chart



Source: EBMT March 2015

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Foreword by the EBMT President

This is the time of the year when we inevitably reflect on what has happened in the past 12 months, and what it means for the future.

The EBMT accomplishments are outlined in the following pages of this Annual Report, and I will not repeat them here. EBMT continues to make strong and significant contributions to the field of haematology and Stem Cell Transplantation. Today, the whole community worldwide acknowledges the solid and high reputation of EBMT's collaborative approach, which will soon culminate in the celebration of its 41st anniversary. Based on the EBMT's successful model, other professional societies are structuring themselves. However, we want EBMT to remain the premier society in the world for research and collaboration in Stem Cell Transplantation, cellular therapy and other related fields such as immunotherapy. Thanks to the dedication of all of you, members and staff, we have always managed to deliver state of the art science and research, focused on improving patient care and superb academic education.

The continuous success of EBMT should not overshadow the numerous challenges we will be facing in the near future. The treatment paradigm of several haematology diseases is radically shifting towards new concepts and approaches. The role of Stem Cell Transplantation (whether autologous or allogeneic) is questioned and needs to be refined. The advent of new drugs for several diseases should not be ignored. Our responsibility, as a leading international professional society, is to validate and propose effective and affordable treatment strategies which can be widely accessed.

As a matter of fact, change seems always frightening, rather than exciting. When I discuss with some colleagues, I am worried when I hear about fear, rather than optimism. Such scepticism reminds me of my school history classes, when we were told that the subjects of her Majesty Queen Victoria were quite suspicious of telephone and railways!

Some look with apprehension at the changes we have introduced into the structure of the annual meeting and of the registry, the use of social media, or the booming haplo transplant activity (among many examples). However, I feel deeply that these changes will enhance EBMT, and there should be no room for timidity in our approach to change. All of these changes are about opportunity, not threat. EBMT should feel more confident because the pace of change is accelerating. Our success in the next few years depends on recognising and adapting to the changing features of one's era. EBMT is committed to staying in the forefront of the game or ahead of it!

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

Take care.

Mohamad Mohty
EBMT President

05

The scientific activity reports

It is a great pleasure to introduce the 2014 scientific report of EBMT, summarising the scientific activities and achievements of our society for the last 12 months.

EBMT aims to allow scientists and physicians involved in Stem Cell Transplantation and cellular therapy to share their experience and develop co-operative studies. This happens mainly through its 11 working parties, which represent the Scientific Council, and different committees. These activities rely mainly on a large registry specifically devoted to the promotion and analyses of all aspects associated with transplantation of stem cells from all donor sources and donor types including prospective, retrospective and non-interventional studies. In the past and now studies conducted by EBMT yielded a significant amount of scientific knowledge creating the basis for continuously improving patient care. Furthermore, the increasing activity of educational events helped to disseminate the newest results into the scientific community.

All the activities and achievements were only possible thanks to the support and voluntary involvement of all EBMT members, and the centers who reported their data timely to EBMT.

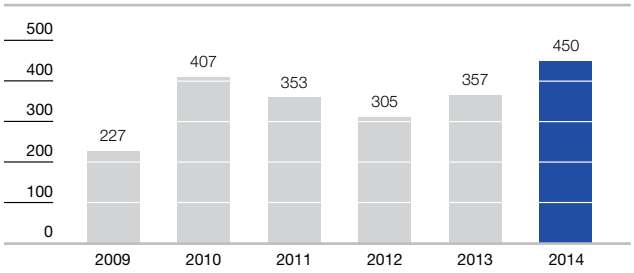
Despite the low resources and the strong worldwide scientific competition, our society was able to generate also in 2014 an amazing number of high impact factor manuscripts and communications at major meetings.

On behalf of the EBMT Scientific Council, I would like to thank you all for your continuous support. Furthermore we would like to invite you and the members of your team to join the regular meetings of the working parties and participate actively in our scientific projects.

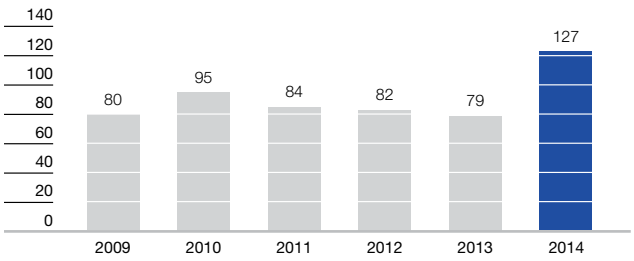
We hope you will enjoy reading our Scientific Activity Report 2014.

Nicolaus Kröger
Scientific Council Chair

Impact factor



Oral presentation



05

Severe Aplastic Anaemia Working Party

Chair: **Carlo Dufour**

Major achievements in 2014

Two important Prospective Randomized Clinical Trials:

1. RACE study that compares standard IST (ATG + CsA) plus Eltrombopag vs Standard IST alone. This EBMT study is financially supported by GSK and Pfizer. 31 EBMT Centres will enroll patients. The study is at the stage of contact with local Ethic Committees. Start will presumably be in 2015.

2. MAA study comparing CsA plus Placebo vs CsA plus Eltrombopag in Moderate AA. This study is sponsored by the University of Ulm (Germany) and financially supported by GSK Germany. 16 EBMT Centres will recruit patients. Start will hopefully be in 2015.

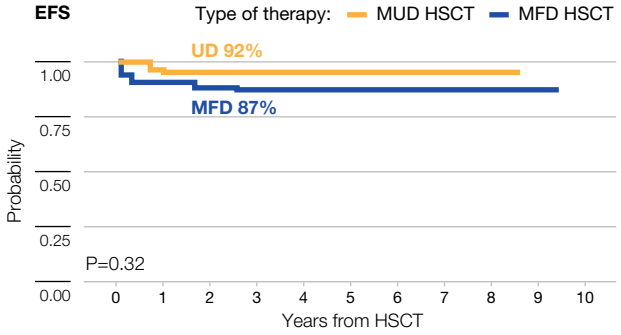
Two completions of two important studies both at the manuscript phase:

1. Similar outcome of upfront MUD and MSD HSCT in Idiopathic AA of Childhood and Adolescence. This comparative study was presented as an oral communication at the last ASH meeting in December 2013 in San Francisco and clearly shows in paediatric patients, that MUD HSCT performed as front line treatment fares the same as MSD HSD, far better than IST front line and better than rescue HSCT post IST failure. This finding is likely to change the treatment algorythm for idiopathic AA in children.

2. Outcome of HSCT in Severe Congenital Neutropenia. This is probably the largest study on transplant in this very rare disease. It shows that for patients requiring high dose of G-CSF or in transformation to MD/AML, HSCT, either from MS or MUD, is a satisfactory option with an OS rate of 82% and a TRM of 17%.

UD *vs* MFD HSCT

Similar EFS in UD and MFD HSCTs



Interval	Dx- neutrophil recovery
UD	MFD
0.39 yrs	0.31 yrs p= 0.93

Four major research studies

1. Outcome of aplastic anaemia in adolescence: a survey of the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation.

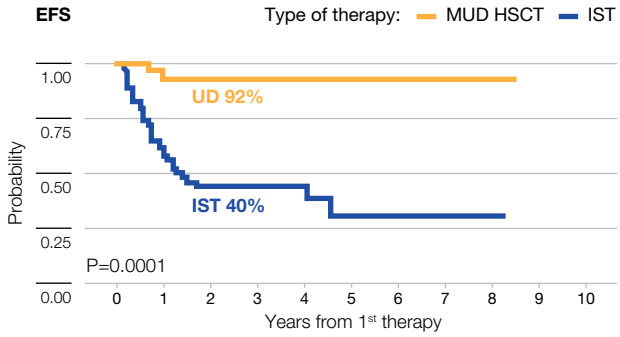
2. Similar Outcome of Upfront Unrelated and Matched Sibling Donor Haematopoietic Stem Cell Transplantation in Idiopathic Aplastic Anaemia of Childhood and Adolescence: A Cohort Controlled Study on Behalf of the UK Paediatric BMT WP, of the PD WP and of the SAA WP of the EBMT.

3. Outcome of aplastic anaemia in children: a study of the Severe Aplastic Anaemia and Paediatric Disease Working Parties of the EBMT.

4. Haematopoietic Stem Cell Transplantation in severe congenital neutropenia: A retrospective analysis from the EBMT.

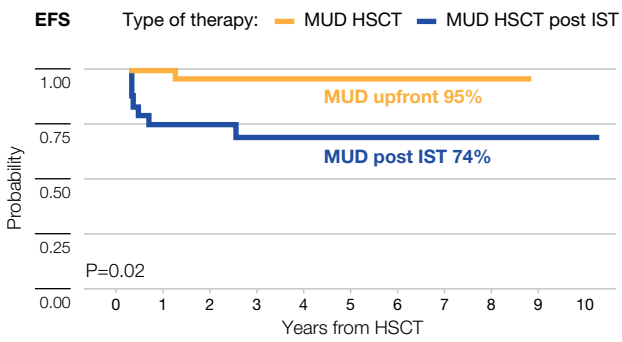
UD *vs* IST front-line

EFS of UD HSCT upfront is far better than IST front-line



MUD *vs* MUD post-failed IST

EFS of MUD HSCT upfront is better than MUD HSCT post failed IST



Best publications

1. Outcome of aplastic anaemia in adolescence: a survey of the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation. Dufour C et al., *Haematologica*.

2. Outcome of allogeneic Stem Cell Transplantation for patients transformed to myelodysplastic syndrome or Leukaemia from severe aplastic anaemia: a report from the MDS Subcommittee of the Chronic Malignancies Working Party and the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation. Hussein AA, et al., *Biol Blood Marrow Transplant.* 2014 Sep.

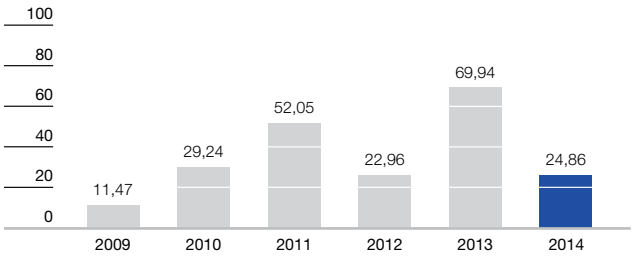
3. Immune suppression for childhood acquired aplastic anaemia and myelodysplastic syndrome: where next? Samarasinghe S et al., *Haematologica.* 2014 Apr.

4. Cyclophosphamide in severe aplastic anaemia? Peffault de Latour R., *Blood* 2014 Oct 30

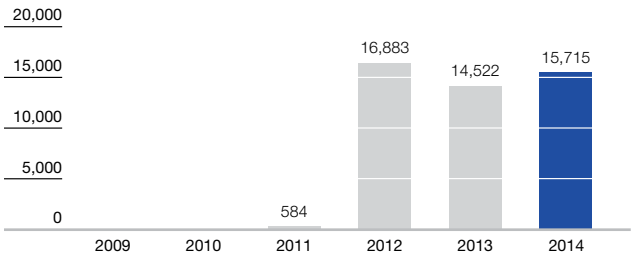
Major educational courses

1. EBMT Severe Aplastic Anaemia and Infectious Diseases Working Parties - Bruno Rotoli Memorial Joint Educational Course - September 29 – October 1 2014 in Naples, Italy

Impact factor (Aplastic Anaemia)



Overview of registered patient data (Aplastic Anaemia)



	2012	2013	**2014**
Oral Presentations	7	6	**9**
Poster Presentations	1	8	**1**
International Educational Events	1	0	**1**

05

Autoimmune Diseases Working Party

Chair: **Dominique Farge-Bancel**

Major achievements in 2014

Over the last 20 years (1994-2014), we have developed the largest database in the field with over 1,800 HSCT for Autoimmune Diseases and an ongoing increase in registrations, particularly in Multiple Sclerosis, Systemic Sclerosis and Crohn's disease in 2014.

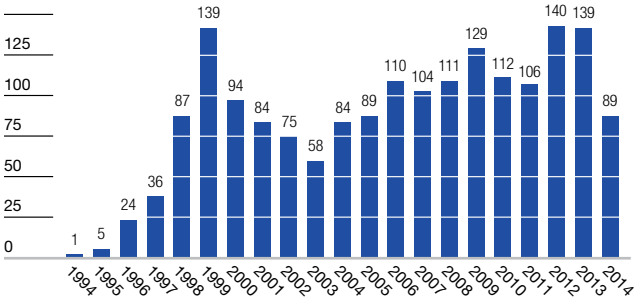
One major sponsored trial, ASTIS, was published in 2014 and another trial, ASTIC, was submitted for publication in November 2014. We also published guidelines for bio-banking and immune reconstitution, as well as several disease specific retrospective analyses. The proof of concept of the efficacy of autologous HSCT is now established for Scleroderma, Multiple Sclerosis, and Crohn's Disease.

We hosted two educational and business meetings, one in Milan in April and the other in Paris in November, to develop innovative activities based on the use of various types of immune-modulating cells (HSCs, MSCs or Tregs) and various sources of blood products (bone marrow, peripheral blood or cord blood and placenta) from either autologous or allogeneic donors, all aiming at resetting the autoimmune balance and inducing tolerance.

Overall 2014 has been a year of consolidating the field of HSCT in AD: the clinical results in otherwise refractory AD patients have raised interest from both patients and clinicians, as well as from healthcare providers.

It is now essential to capitalise on clinical data obtained with adapted clinical databases and bio-banking performed in expert centers to refine our understanding of the observed responses after HSCT for various types of AD in comparison with other approaches.

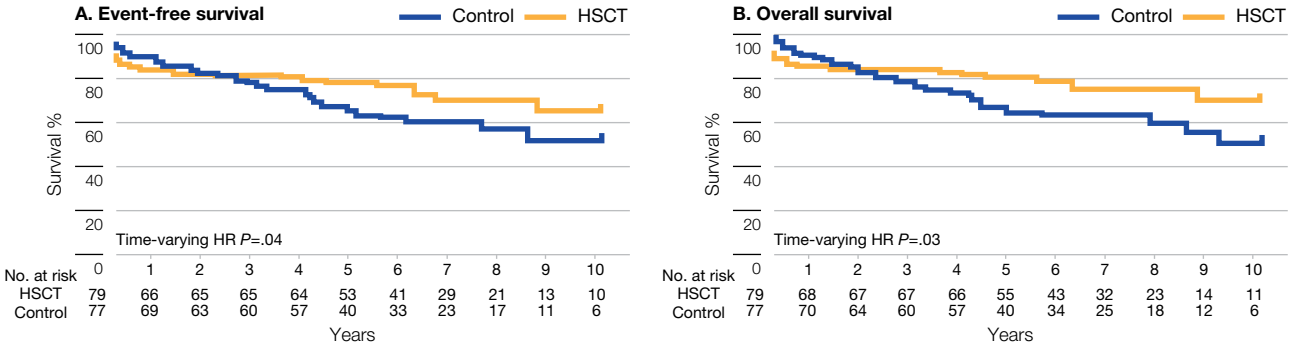
EBMT Registry - ADs
All HSCT per year (n=1817)
December 2014



MULTIPLE SCLEROSIS	**666**
CONNECTIVE TISSUE	**543**
SSc	384
SLE	109
PM-DM	20
Sjorgen	3
Antiphosph. Syndrome	5
Other/Unknown	22
ARTHRITIS	**176**
Rheumatoid arthritis	81
Juvenile chronic arthritis:	
Systemic JIA	55
Other JIA	18
Polyarticular JIA	14
Psoriatic arthritis	4
Other	5
INFLAMMATORY BOWEL	**169**
Crohn's disease	138
Ulcerative colitis	4
Other	22
HEAMATOLOGICAL	**93**
ITP	29
Evans'	19
AIHA	23
Other	22
VASCULITIS	**48**
Wegener's	12
Behcet's	9
Takayasu	2
Microscopic poly. nodosa	3
Classical poly. nodosa	1
Churg-Strauss	2
Other/Unknown	17
OTHER NEUROLOGICAL	**71**
Myasthenia gravis	7
Other/Unknown	66
INSULIN DEPENDENT DIABETES	**20**
OTHER	**29**

Autologous Haematopoietic Stem Cell Tranplantation vs Intravenous Pulse Cyclophosphamide in Diffuse Cutaneous Systemic Sclerosis. A Randomized Clinical Trial

Figure 2. Event-Free and Overall Survival During 10-Years Follow-up



Hazard ratios (HRs) and 95% CIs were calculated by Cox regression. Hazard ratios were time-varying. The hazard (slope of the survival curve) in the haematopoietic Stem Cell Transplantation (HSCT) group is initialy high because of treatment-related mortality but gradually improves. At 1-year follow-up, the HR alredy favors the HSCT group, which leads to the crossing of the survival curves at 2 years' follow-up. A. Three-month follow-up: HR, 2.01 (95% CI, 0.75-5.49); *P*=.17; 6-month follow-up: HR, 1.35 (95% CI, 0.62-2.96); *P*=.45 1 year follow-up: HR, 0.52 (95% CI, 0.28-0.96); *P*=.04; 2-year follow-up: HR, 0.35 (95% CI, 0.16-0.74); *P*=.006; 3-through 10 year follow-up: HR, 0.34 (95% CI, 0.16-0.74); *P*=.006. B, Three-month follow-up: HR, 2.40 (95% CI, 0.75-7.67); *P*=.14; 6-month follow-up: HR, 1.50 (95% CI, 0.61-3.68); *P*=.38; 1-year follow-up: HR, 0.48 (95% CI, 0.25-0.91; *P*=.02; 2-year follow-up: HR, 0.29 (95% CI, 0.13-0.65); *P*=.002; 3-through 10-years follow-up: HR, 0.29 (95% CI, 0.13-0.64); *P*=.002.

Van Laar, Jacob M., Dominique Farge, et al.. JAMA311 (24): 2490-98

Major 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 Research (CIBMTR) and from the European Society for Blood and Marrow Transplantation (EBMT)
4. Is CXCL4 a prognostic factor of response to HSCT in systemic sclerosis?

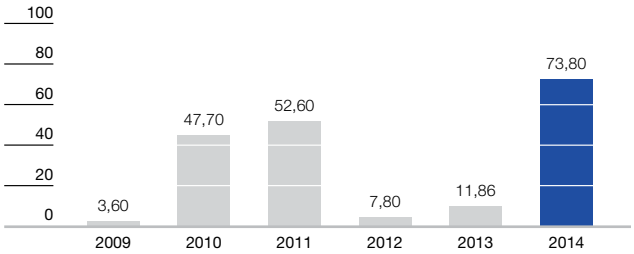
Best publications

1. Autologous Haematopoietic Stem Cell Transplantation vs Intravenous Pulse Cyclophosphamide in Diffuse Cutaneous Systemic Sclerosis: A Randomized Clinical Trial *JAMA 2014*
2. SCT for Severe Autoimmune Diseases: Consensus Guidelines of the European Society for Blood and Marrow Transplantation for Immune Monitoring and Biobanking. *Bone Marrow Transplantation* 2014
3. Autologous haematopoietic Stem Cell Transplantation in neuromyelitis optica: A registry study of the EBMT Autoimmune Diseases Working Party. *Mult Scler.* 2014
4. Onset and Outcome of Pregnancy after Autologous Haematopoietic SCT (AHSCT) for Autoimmune Diseases: A Retrospective Study of the EBMT Autoimmune Diseases Working Party (ADWP). *Bone Marrow Transplantation* 2014

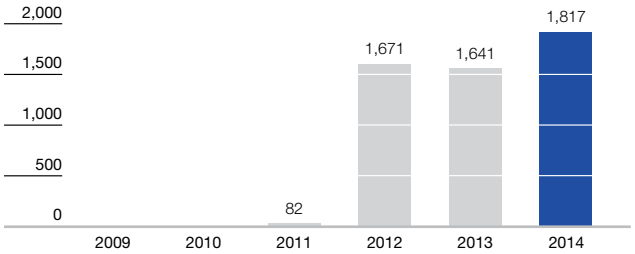
Major educational courses

1. Cellular therapies for severe autoimmune diseases – April 1 2014 in Milan, Italy
2. ADWP Educational meeting on Stem Cell Therapy (HSCT & MSC) and Autoimmune Diseases - November 28-29 2014 in Paris, France

Impact factor (Autoimmune Diseases)



Overview of registered patient data (Autoimmune Diseases)



	2012	2013	**2014**
Oral Presentations	7	11	**5**
Poster Presentations	1	7	**7**
International Educational Events	1	1	**1**

05

Acute Leukaemia Working Party

Chair: **Arnon Nagler**

Major achievements in 2014

2014 was a very productive and successful year for the ALWP. We tried and were able to continue the excellent work of the previous chairs of the ALWP and mainly Prof. Mohamad Mohty now the President of the Society.

In 2014 two new subcommittees were established within the ALWP: One for *Acute Lymphatic Leukaemia* (ALL) leaded by Dr Sebastian Giebel who is also the secretary of the ALWP and second one for *Cord Blood* (CB) *Transplantation* (in close collaboration with EuroCord) headed by Dr. Frederic Baron.

We felt that the progress and advances in the field of adult ALL which includes basing treatment decision on molecular minimal residual disease monitoring, the availability of TKIs pre and post transplants for Ph+ ALL as well as the emerging of novel monoclonal antibodies like blinatumomab, Bi-specific T-cell engagers (BiTEs) bispecific monoclonal antibodies and the Chimeric Antigen Receptor–Modified T Cells (CAR) based therapy justify the creation of an ALL subcommittee within the ALWP. Similar reasoning was the basis for establishing the CB subcommittee. We also nominated Dr. Bipin Savani as the head of the *reduced intensity* (RIC) subcommittee.

Alternative donor transplantation and novel compounds and formulations are among the most important developments in the field of allogeneic Stem Cell Transplantation for acute Leukaemia. We therefore devoted our education meetings to conditioning pre allogeneic transplantation, Haploidentical transplantation and recently the November 2014 education symposium was dedicated to unrelated donor transplantation for Acute Leukaemia. Discussed topics include donor availability; conditioning and indications for unrelated allogeneic Stem Cell Transplantation. These topics were extensively studied in 2014 (see below) and are the main topics for our ongoing studies.

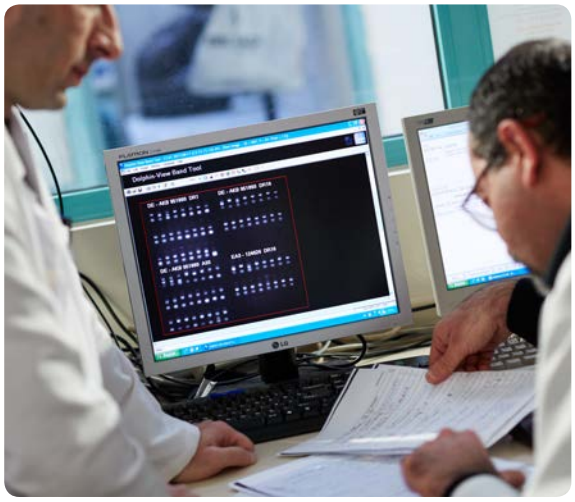
All of the above could not be achieved without the strong devotion, the hard and professional work and full commitment of the ALWP subcommittee leaders, Dr. Myriam Labopin and the ALWP office staff that deserve every possible credit.

Major research studies

1. Data mining study (R. Shouval) submitted to JCO (revise and respond to reviewers' comments) ASH 2014 POSTER
2. Comparative analyses of different post-remission strategies (alloHSCT vs. other) for patients with intermediate-risk AML and triple negative genotype: a CETLAM, AMLSG and EBMT joint study (R. Schlenk) ASH 2014 ORAL
3. Survey on unmanipulated graft haploidentical transplantation (S. Piemontese) accepted in *Leukaemia*
4. Impact of socio-economic factors on non-relapse mortality after alloHSCT for acute lymphoblastic Leukaemia (S. Giebel) Submitted to JCO (under reviewing)

Best publications

1. Prediction of non-relapse mortality in recipients of reduced intensity conditioning allogeneic Stem Cell Transplantation with AML in first complete remission. Versluis J et al. *Leukaemia* 2014 May 20
2. A survey on unmanipulated haploidentical haematopoietic Stem Cell Transplantation in adults with acute Leukaemia. Piemontese S et al. *Leukaemia* in press 2014
3. Tyrosine kinase inhibitors improve outcome of allogeneic haematopoietic Stem Cell Transplantation for adult patients with Philadelphia chromosome acute lymphoblastic Leukaemia in first complete remission: a study from the Acute Leukaemia Working Party of EBMT. E. Brissot et al. *Hematologica* in press 2014
4. Intravenous busulfan for autologous Stem Cell Transplantation in adult patients with acute myeloid Leukaemia: a survey of 952 patients on behalf of the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation. Nagler A et al. *Haematologica* 2014 Aug.

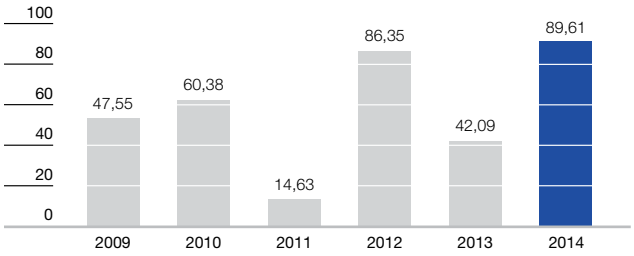


Major educational courses

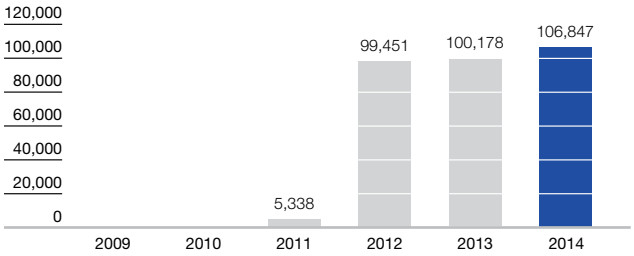
1. ALWP Meeting - March 30 2014 in Milan, Italy

2. ALWP Meeting and Educational Symposium - Unrelated Donor Haematopoietic Cell Transplantation for Acute Leukaemia - November 28-29 2014 in Paris, France

Impact factor (Acute Leukaemia)



Overview of registered patient data (Acute Leukaemia)



	2012	2013	**2014**
Oral Presentations	18	9	**18**
Poster Presentations	14	13	**13**
International Educational Events	1	1	**2**

05

Cellular Therapy & Immunobiology Working Party

Chair: **Andrea Velardi**

Chiara Bonini was elected Chair of the CTIWP in October 2014

Major achievements in 2014

In haploidentical transplantation for acute leukaemia, over 15 years' follow-up of transplants of T cell-depleted haematopoietic stem cells showed satisfactory outcomes in adults and children. Recent years have witnessed development of unmanipulated grafts combined with post-transplant cyclophosphamide, post-transplant rapamycin, or G-CSF-priming of donor bone marrow. All provide promising results. Current CTIWP studies mainly focus on exploiting natural and adaptive immunity to improve transplantation outcomes.

The CTIWP's major activities this year have been the performance, jointly with the PDWP (C. Peters) and the Alternative Donor Subcommittee of the ALWP (F. Ciceri), of two combined retrospective studies and one non-interventional prospective study on haploidentical Stem Cell Transplantation in adults and children with AML or ALL.

In 2014 we also launched a non-interventional prospective study on the clinical impact of pre-transplant thymic output.

As per previous years, the CTIWP session of the EBMT Annual Meeting in Milan featured the Jon van Rood Award Prize-giving Ceremony and the winner's presentation (best transplantation immunology paper published in the previous year).

In 2014 the CTIWP contributed to the scientific organisation of the 7th International Symposium on Haploidentical Stem Cell Transplantation. This now "classic", extremely well attended, important international educational event is a collaborative effort between the University of Perugia and the Weitzmann Institute. Its 7th edition was held at the Weitzmann Institute in Rehovot (Israel) in February 2014 and featured outstanding speakers from the US and Europe.

Four major research studies

1. **Retrospective study** (Principal Investigators: J. van Rood, A Velardi): designed to test whether mothers' immunity/tolerance towards paternal antigens in mother-to-child hapoidentical transplants are associated with better event-free survival under diverse protocols as was reported for T cell-depleted haploidentical transplants (Stern et al. *Blood* 2008). In addition, it will evaluate whether tolerance of non-inherited maternal antigens in sibling-to-sibling haploidentical transplants is associated with less GvHD/TRM as was reported in unrelated donor transplants (van Rood and coll. 2002).

2. **Retrospective study:** Attempts to identify immunological biomarkers that are predictive of clinical outcome after haploidentical Stem Cell Transplantation, under diverse protocols (Principal Investigators: A. Bondanza, C. Bonini, A. Toubert, A. Velardi). Several Centers are participating in these studies and the transplant cohort comprises over 400 patients.

3. **Non-interventional prospective study:** assesses the impact of donor vs recipient NK cell allo-reactivity in haploidentical haematopoietic transplantation under diverse (T cell depleted vs T cell replete) protocols (Principal Investigators: A. Velardi, L. Ruggeri). Several Centers from Europe and elsewhere are participating in this study. The study is enrolling patients for two years (September 2012-August 2014) and the estimated total number of transplants is over 200.

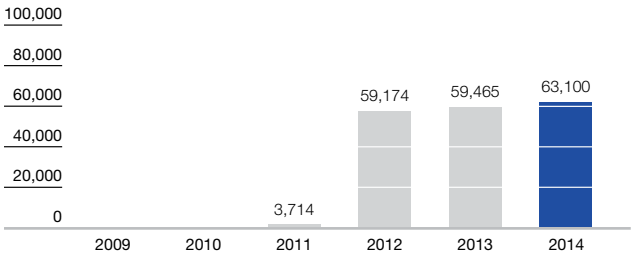
4. **Non-interventional prospective study:** Launched in 2014 jointly with the PDWP and the ALWP, this study assesses the impact of recipient pre-transplant thymic function (as evaluated by TRECs analysis) on outcomes after allogeneic Haematopoietic Stem Cell Transplantation (Principal Investigator: A. Toubert). The study is aiming at evaluating 800 patients with 2 year follow-up.

Major educational courses

1. Jon van Rood Award Prize-giving Ceremony and the winner's presentation - March 2014 in Milan, Italy

2. 7th International Symposium on Haploidentical Stem Cell Transplantation - February 2014 in Rehovot, Israel

Overview of registered patient data (Cell Therapy & Immunobiology)



The Cellular Therapy & Immunobiology, Infectious Diseases, and Complications & Quality of Life Working Parties are transversal Working Parties dealing with all patients regardless of diagnosis or age; the number shown for these Working Parties is the average of patients in the Registry for the other eight Working Parties.

	2012	2013	**2014**
Oral Presentations	0	0	**0**
Poster Presentations	0	0	**2**
International Educational Events	1	1	**1**

05

Infectious Diseases Working Party

Chair: **Simone Cesaro**

Major achievements in 2014

In 2014, IDWP gave a significant contribution in the most important areas of infectious complications after HSCT. Groll et al. published the first guidelines for the invasive fungal infections (IFI) in paediatric patients who undergo chemotherapy and/or HSCT. The working group who prepared the guidelines included experts from IDWP-EBMT, EORTC, ICHS, and ELN.

Ljugman et al. performed a large retrospective registry analysis on 49,542 HSCT patients recorded in ProMISe data base to assess the impact on the outcome of donor/recipient CMV serostatus. This study confirmed that there is a negative impact on overall survival if a CMV-seropositive unrelated donor is selected for a CMV-seronegative patient. Moreover, the study showed an advantage on OS if a CMV-seropositive patient who undergoes myeloablative conditioning regimen receives a CMV-seropositive donor.

Mikulska et al. published the results of a survey on bacteremia in haematological and HSCT patients. She found a reduction of the Gram-positive to Gram-negative infection ratio (55%:45% vs. 60%:40%), an increased rates of enterococci (8% vs. 5%) and Enterobacteriaceae (30% vs. 24%), a decreased rate of Pseudomonas aeruginosa (5% vs. 10%), and lower resistance rates for all bacteria. Nevertheless the median rates of ESBL-producers (15-24%), aminoglycoside-resistant Gram-negatives (5-14%) and carbapenem-resistant P. aeruginosa (5-14%) were substantial, and significantly higher in South-East vs. North-West Europe.

Bontant et al. assessed the current practice in the management of CMV infection in paediatric HSCT. They found that quantitative PCR was the most common monitoring tool and that all centers used pre-emptive strategy. In almost half of responding centre a prophylactic policy was also adopted mainly guided by the analysis of donor/receptor serologic status. Ganciclovir resulted the most common first-line agent for CMV disease. No uniform procedure was adopted for researching resistance strain, antiviral second-line therapy or cell therapy. They concluded that a better harmonisation of modality of treatment of CMV infection among paediatric centres would be needed.

Major research studies

1. NIS study on Gram-negative bacteremia episodes among EBMT Centres to assess incidence, type, and, most importantly, type and frequency of MDR together with outcome of HSCT. The participation to the study is depicted in the image below. The end of recruitment is expected for July-August 2015.
2. Previous proven/probable Aspergillosis and SCT outcome
3. Impact of EBV serostatus on outcome of allo-HSCT
4. Retrospective analysis of Candida spp infection in in HSCT performed over the last decade

Best publications

1. Fourth European Conference on Infections in Leukaemia (ECIL-4): guidelines for diagnosis, prevention, and treatment of invasive fungal diseases in paediatric patients with cancer or allogeneic haemopoietic Stem Cell Transplantation. Groll AH et al. *Lancet Oncol.* 2014
2. Donor CMV status influences the outcome of allogeneic Stem Cell Transplantation; a study by the European Group for Blood and Marrow Transplantation.Clinical Infectious Diseases. Ljungman P et al. *Clin Infect Dis* 2014
3. Aetiology and resistance in bacteraemias among adult and paediatric haematology and cancer patients. Mikulska M et al. *J Infect.* 2014
1. Survey of CMV management in paediatric allogeneic HSCT programs, on behalf of the Inborn Errors, Infectious Diseases and Paediatric Diseases Working Parties of EBMT. Bontant T et al. *Bone Marrow Transplant.* 2014

Major educational courses

1. EBMT Severe Aplastic Anaemia and Infectious Diseases Working Parties - Bruno Rotoli Memorial Joint Educational Course - September 29 – October 1 2014 in Naples, Italy

Castel dell'Ovo, Naples, Italy

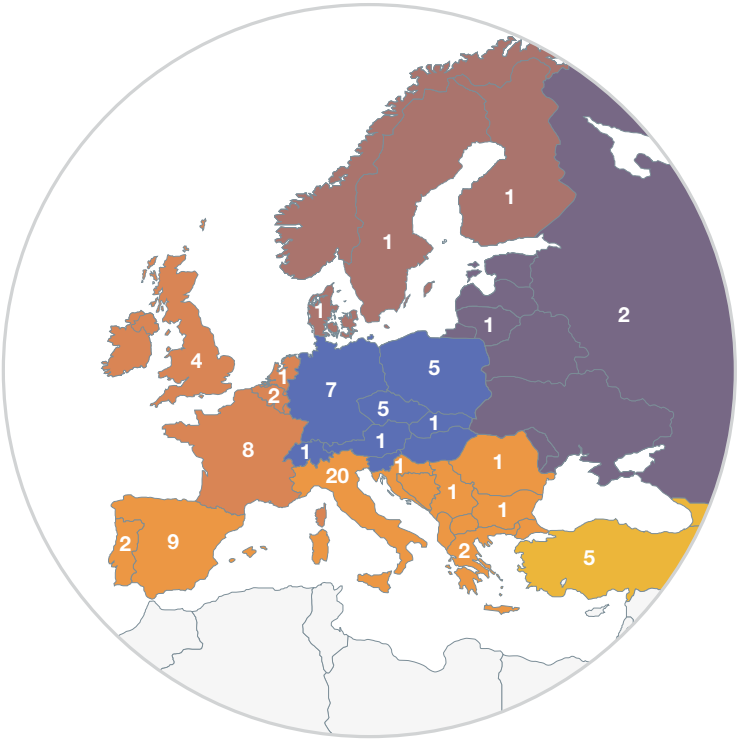
Speakers

NIS study on Gram-negative bacteremia

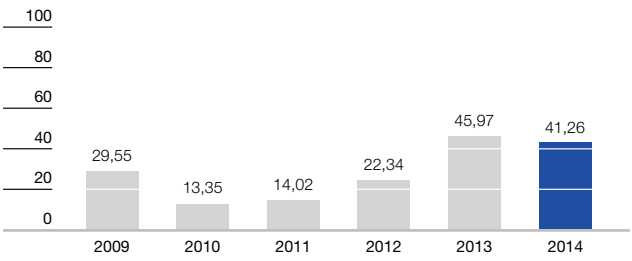
Nº of centres: 94
Nº of countries: 30

Nº of Countries by area

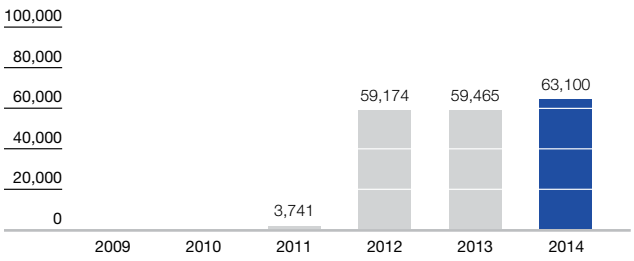
- 6 Central Europe
- 2 Eastern Europe
- 3 Northern Europe
- 4 Western Europe (partial view)
- 8 Southern Europe
- 1 Asian countries with territories in Europe
- 6 Outside Europe
  (Israeli: 4; China: 2; Cyprus: 1; Australia: 2; New Zeland: 1; Iran: 1)



Impact factor (Infectious Diseases)



Overview of registered patient data (Infectious Diseases)



The Cellular Therapy & Immunobiology, Infectious Diseases, and Complications & Quality of Life Working Parties are transversal Working Parties dealing with all patients regardless of diagnosis or age; the number shown for these Working Parties is the average of patients in the Registry for the other eight Working Parties.

	2012	2013	**2014**
Oral Presentations	1	1	**1**
Poster Presentations	0	0	**1**
International Educational Events	1	1	**1**

05

Inborn Errors Working Party

Chair: **Andrew Gennery**

Major achievements in 2014

2014 has been a busy year for the IEWP, in terms of structure, activity and output. Firstly, Fulvio Porta stepped down as secretary after 6 years. Arjan Lankester from Leiden has kindly stepped into the role. Two new sub-committees of IEWP have been formed, the *Gene Therapy sub-committee*, headed by Marina Cavazzana from Paris and Alessandro Auiti from Milan, and the *Metabolic Diseases sub-committee* headed by Rob Wynn from Manchester and Jaap jan Boelens from Utrecht. An *IEWP Studies committee* has also been formed to advise and guide members who wish to perform IEWP-related studies.

In addition to the IEWP joint sessions with PDWP at EBMT Spring meeting in Milan, earlier in the year, we also held an Educational day as part of the 9th PDWP meeting Jerusalem in May. A Newborn Screening workshop, led by Bobby Gaspar, met in Amsterdam in April, to consider formulating guidelines for diagnosing and transplanting newborn infants with Severe Combined Immunodeficiency, in anticipation of Newborn Screening for SCID being introduced in Europe over the next few years.

Our Autumn meeting was held in Munich in October, hosted by Michael Albert. In November our first Thymic workshop was held in London, led by Graham Davies and Georg Hollander, from which we are planning to establish guidelines for thymic transplantation.

We have initiated a successful collaboration between the EBMT and SCETIDE databases looking at the outcome of HSCT for CD40 ligand deficiency, and are involved with collaborative projects with the North American Primary Immune Deficiency Treatment Consortium.

Three important studies have been published on behalf of IEWP this year – more are in preparation and several have been presented through the year.

Major research studies

1. Haematopoietic Stem Cell Transplantation for CD40 ligand deficiency: results from an EBMT IEWP study
2. Multicenter experience in haematopoietic Stem Cell Transplantation for serious complications of common variable immunodeficiency
3. DOCK8 deficiency: clinical and immunological phenotype and treatment options
4. Outcomes Of Allogeneic Cord Blood Transplantation For Leukodystrophies; A Joint Study of Eurocord, Duke University Medical Center Cord blood committee of Cellular Therapy and Immunobiology and EBMT IEWP

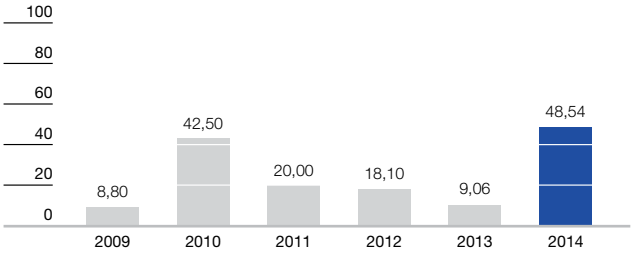
Best publications

1. Reduced-intensity conditioning and HLA-matched haemopoietic stem-cell transplantation in patients with chronic granulomatous disease: a prospective multicentre study. Lancet 2014
2. Survey of CMV management in paediatric allogeneic HSCT programs, on behalf of the inborn errors, infectious diseases and paediatric diseases working parties of EBMT. *Bone Marrow Transplant*
3. Haematopoietic Stem Cell Transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel. *Haematologica*
4. Patients with T /low NK IL-2 receptor chain deficiency have differentially-impaired cytokine signaling resulting in severe combined immunodeficiency. *Eur J Immunol*

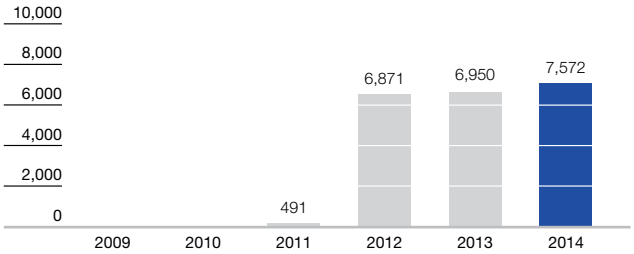
Major educational courses

1. NBS SCID Working Party - April 24 2014 in Amsterdam, Netherlands
2. Inborn Errors Working Party Educational Day - May 22 2014 in Jerusalem, Israel
3. IEWP Autumn Meeting - October 17-19, 2014 in Munich, Germany

Impact factor (Inborn Errors)



Overview of registered patient data (Inborn Errors)



	2012	2013	**2014**
Oral Presentations	0	3	**6**
Poster Presentations	0	0	**2**
International Educational Events	1	1	**3**

05

Lymphoma Working Party

Chair: **Peter Dreger**

Major achievements in 2014

The most relevant scientific activities in 2014 of the LWP comprise the conduction, completion, or preparation of 24 retrospective and 3 prospective non-interventional studies (involving 18 Principal Investigators from 10 countries). The LWP's study activities resulted in the publication of 12 scientific papers with a cumulative Impact Factor of 87.432 in 2014.

Moreover, the 10th Annual LWP Educational Course held in Nicosia, Cyprus, 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.

Finally, for the third time we were able to present the Jian-Jian Luan Award for Lymphoma Transplant research during the LWP session at the Milan Annual Meeting. 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. Award winner was Anne-Claire Mamez from Paris, France, for her research on DLI in lymphoma.

Major research studies

1. MALT lymphoma (together with FIL), PIs I Avivi (Tel Aviv) and A Conconi (Novara).
2. Zevalin-BEAM vs BEAM in alloHCT for follicular lymphoma, PI L Bento (Barcelona).
3. Waldenström's macroglobulinemia, PI C Kyriakou (London).
4. Haplotransplants in Hodgkin's and Non-Hodgkin's lymphoma, PIs A Sureda (Barcelona) and S Dietrich (Heidelberg).

Best publications

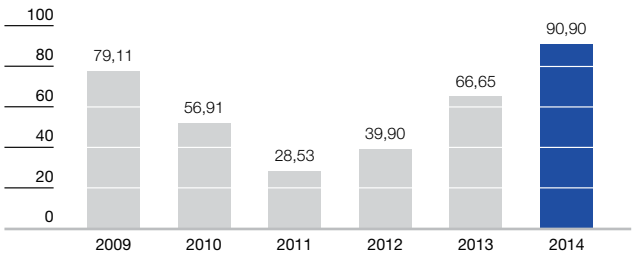
1. Allogeneic and Autologous Stem Cell Transplantation for hepatosplenic T cell lymphoma: a retrospective study of the EBMT Lymphoma Working Party. Tanase et al., *Leukaemia* 2014, Sep 19
2. Long-term outcome of allogeneic haematopoietic cell transplantation for patients with mycosis fungoides and sézary syndrome: a European Society for Blood and Marrow Transplantation Lymphoma Working Party extended analysis. Duarte et al., *J Clin Oncol* 2014
3. The first position paper on alloHCT in CLL in the ibrutinib era (together with the CMWP and the European research initiative on CLL (ERIC)). Dreger et al., *Blood* 2014
4. The EBMT/EMCL consensus project on the role of autologous and allogeneic Stem Cell Transplantation in mantle cell lymphoma. Robinson et al., *Leukaemia* 2014, Jul 18.

Major educational courses

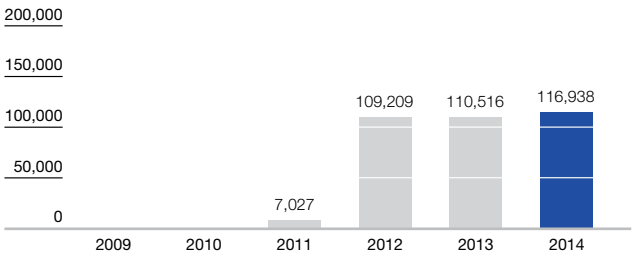
1. EBMT Lymphoma Working Party 10th educational course - Treatment of Malignant Lymphoma: State-of-the Art and the Role of Stem Cell Transplantation - October 16 – 17 2014 in Nicosia, Cyprus

10th educational course in the Castelliotissa Hall of the Lusignan palace in Nicosia, Cyprus

Impact factor (Lymphoma)



Overview of registered patient data (Lymphoma)



	2012	2013	**2014**
Oral Presentations	8	3	**8**
Poster Presentations	4	7	**11**
International Educational Events	1	1	**4**

05

Paediatric Diseases Working Party

Chair: **Peter Bader**

Major achievements in 2014

The PDWP held its 9th Bi-annual Meeting in Jerusalem in May 2014. We emphasized on cellular therapies, haploidentical SCT, positioning of intensive care treatment in the setting of SCT in childhood and adolescents and the improvement of supportive care in collaboration with our Nurses Group.

We published three major manuscripts:

1. A hallmark paper on HSCT in children with thalassemia major and sickle cell disease. Here we summarized the consensus from an expert meeting held in 2013, which outlined state of the art approaches with regard to indication and management of SCT in these patients.

2. A collaborative paper of IE, ID and PDWP outlined the management of CMV infections in paediatric SCT programs.

3. An important retrospective publication summarized the outcomes of treosulfan-based conditioning regimens for allogeneic SCT in children with acute lymphoblastic leukaemia.

Several manuscripts are currently under preparation to be submitted on behalf of the PDWP.

Finally, we started several retrospective studies within our WP and successfully initiated the prospective multinational randomized study “ALL SCTped 2012 FORUM” (Chair: C. Peters, Co-Chairs: P. Bader, F. Locatelli) aiming to demonstrate that a non-TBI conditioning (Flu/Thio/ivBu or Flu/Thio/Treo) results in a non-inferior survival as compared to conditioning with TBI/Etoposide in children after HSCT.

Major research studies

1. ALL SCTped 2012 FORUM (“For Omitting Radiation Under Majority Age”)

2. Haploidentical versus cord blood SCT in paediatric acute leukaemia

3. Outcome of children with chronic GvHD after allogeneic SCT

4. Optimal conditioning regime for paediatric AML: Comparison between Bu/Cy/(Mel) and Cy/TBI

Best publications

1. Treosulfan-based conditioning regimens for allogeneic HSCT in children with acute lymphoblastic leukaemia. *Ann Hematol.* 2014

2. Haematopoietic Stem Cell Transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel. *Haematologica*, 2014

3. Survey of CMV management in paediatric allogeneic HSCT programs, on behalf of the Inborn Errors, Infectious Diseases and Paediatric Diseases Working Parties of EBMT. *Bone Marrow Transplant,* 2014

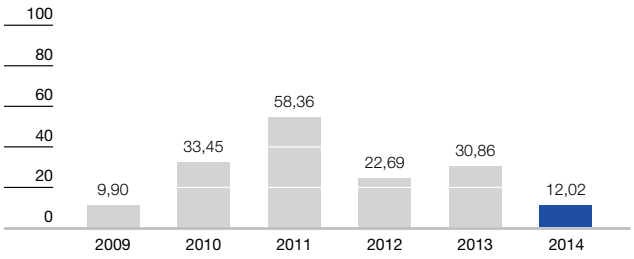
4. Combined cord blood and bone marrow transplantation from the same human leucocyte antigen-identical sibling donor for children with malignant and non-malignant diseases. *Br J Haematol.*, 2014

PDWP Board Meeting, September 11-12 2014 in Frankfurt am Main, Germany

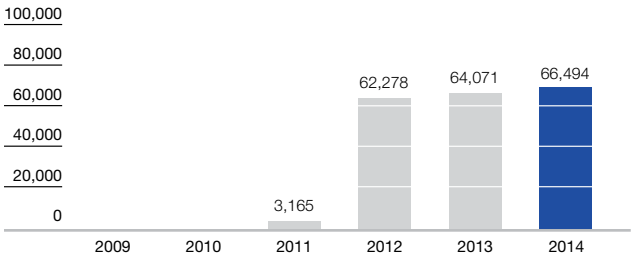
Major educational courses

1. 9th Meeting of the EBMT Paediatric Diseases Working Party - May 21-23 2014 in Jerusalem, Israel
2. Participation ESH training course on Haematopoietic Stem Cell Transplantation: Peter Bader, Christina Peters - May 8-10 2014 in Vienna, Austria
3. PDWP Expert Workshop on extra-corporal photopheresis (ECP) for the treatment of GvHD - January 17-18 2013 in Baden, Austria
4. PDWP Expert Workshop on Paediatric Intensive Care (PIC) in the context of SCT - November 6-7, 2013 in Vienna, Austria

Impact factor (Paediatric Diseases)



Overview of registered patient data (Paediatric Diseases)



	2012	2013	**2014**
Oral Presentations	13	3	**10**
Poster Presentations	1	5	**7**
International Educational Events	2	3	**1**

05

Solid Tumour Working Party

Chair: **Francesco Lanza**

Major achievements in 2014

The STWP assessed toxicity and efficacy of high-dose chemotherapy (HDC) and autologous hemopoietic progenitor cell transplantation (HPCT) in a large cohort of Breast Cancer (BC) patients. Based on the analysis of our large retrospective series, it can be stated that HDC with HSCT has low mortality rate and provides impressive long-term survival rates. Our results suggest that this treatment modality should be proposed in selected patient subgroups (triple negative and metastatic setting) and further investigated in prospective trials.

HPCT has a recognized indication in the salvage setting of advanced Germ Cell Tumor (GCT) and is steadily utilised worldwide. While the prognostic impact of response to prior lines of CT (i.e. definition of chemoresistance) is ascertained, that of response to induction/mobilization CT preceding single or multiple HPCT cycles is unknown. Our data showed that progression to induction CT prior to HPCT was independently and significantly associated with shorter PFS and OS, while response or progression to prior CT lines was not. This information could have important implications to refine patient eligibility to transplantation and enhance the prognostic risk grouping. Furthermore, the role played by paclitaxel-based regimens as a second or third-line salvage therapy for GCT was investigated. This might have an impact on the results with subsequent salvage HPCT in these patients. The EBMT-STWP sponsored a retrospective study on the outcomes of HDCT administered in the last 10 years. Hence, we aimed to study outcomes with HDCT after relapse to paclitaxel-CT to identify the level of chemoresistance in these patients. Interestingly, the administration of paclitaxel-based regimens before HDPT did not affect PFS/OS. Results were confirmed when excluding patients who were administered taxane-containing HDCT. Line of HDCT was not significantly prognostic too.

Moreover, long-term results of salvage high-dose chemotherapy for germ cell tumor in female patients as well as in paediatric/adolescent patients has been investigated, and results showed that these patients subgroups are characterised by different clinical characteristics and a peculiar response to treatment.

Major research studies

1. HSC collection and engraftment results in patients with germ cell tumors (GCT) who are candidates to myeloablative chemotherapy.
2. Long-term results of salvage high-dose chemotherapy for a) paediatric/adolescent and b) female- germ cell tumor patients.
3. Prospective studies of intensified chemotherapy with autologous Stem Cell Transplantation for triple-negative (neoadjuvant setting) and Her2 negative (metastatic setting) breast cancer.
4. Stem Cell Transplantation in Breast Cancer: a retrospective analysis.

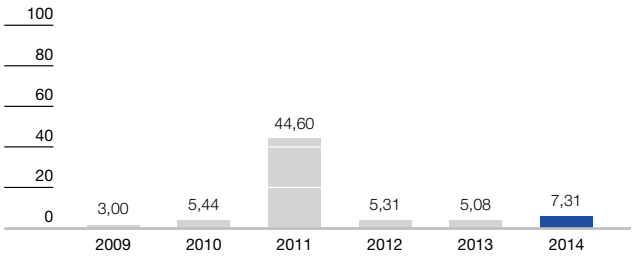
Four best publications

1. Erythropoiesis-stimulating agents in allogeneic and autologous haematopoietic Stem Cell Transplantation. Martino M et al. *Expert Opinion in Biological Therapy. 2014*
2. High-dose chemotherapy for germ cell tumors: do we have a model? An expert opinion on behalf of the European Society for Blood and Marrow Transplantation, Solid Tumors Working Party (EBMT-STWP). Necchi A et al. *Expert Opinion in Biological Therapy* 2014 Sep 22

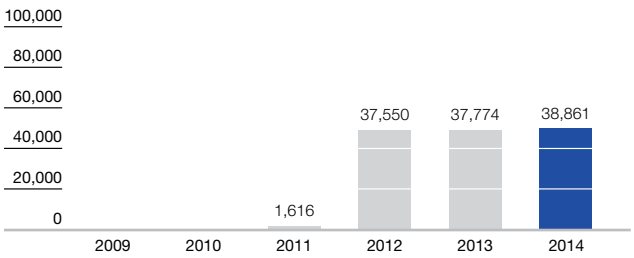
Major educational courses

1. EBMT Solid Tumors Working Party Educational Meeting - October 31 2014 in Florence, Italy

Impact factor (Solid Tumors)



Overview of registered patient data (Solid Tumors)



	2012	2013	**2014**
Oral Presentations	0	0	**6**
Poster Presentations	0	0	**2**
International Educational Events	0	0	**3**

05

Chronic Malignancies Working Party

Chair: **Nicolaus Kröger**

Major achievements in 2014

In 2014, the CMWP intensified its mission to perform high quality educational event regarding disease specific topics of our Working Party. In February 2014 we performed in Leiden an exciting symposium on "The Role of Allogeneic Stem Cell Transplantation in Chronic Lymphocytic Leukaemia". In September 2014, together with the French SFGM-TC, we performed in Lille an educational event on "Stem Cell transplantation in MDS" and we started a 2-day EBMT Preceptorship meeting on "Stem Cell Transplantation in Multiple myeloma and other Plasmacell-Disorders" in Torino.

In 2014 the CMWP published 13 manuscripts in peer-reviewed journals such as *Leukaemia, Haematologica*, and *American Journal of Haematology.*

At international meetings such as ASH, EHA, EBMT or BMT Tandem meeting, members of CMWP presented more than 20 oral or poster presentations.

We started 2 consensus projects about Stem Cell Transplantation in MDS as well as in myelofibrosis both in collaboration with ELN which will be finalised and published in 2015.

Two brainstorm meetings with CIBMTR for common projects in MDS/MPN and Myeloma were performed.

Overall, in the CMWP we have currently 10 non-interventional studies, 3 prospective EBMT sponsored or labelled studies ongoing and are working on more than 35 retrospective registry studies.

Major research studies

1. Prospective analysis of prognostic pre-transplant factors in myelodysplastic syndromes primarily treated by allogeneic haematopoietic Stem Cell Transplantation: a study on behalf of the MDS subcommittee of the Chronic Malignancies Working Party of the EBMT

2. Reduced versus 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. Allogeneic Stem Cell Transplantation for CLL with del17p. A non-interventional study of CMWP

Four best publications

1. Early administration of donor lymphocyte infusions upon molecular relapse after allogeneic haematopoietic Stem Cell Transplantation for chronic myeloid Leukaemia: a study by the Chronic Malignancies Working Party of the EBMT. Chalandon Y et al., *Haematologica* 2014 Sep

2. Impact of the International Prognostic Scoring System cytogenetic risk groups on the outcome of patients with primary myelodysplastic syndromes undergoing allogeneic Stem Cell Transplantation from human leukocyte antigen-identical siblings: a retrospective analysis of the European Society for Blood and Marrow Transplantation-Chronic Malignancies Working Party. Onida F et al., *Haematologica* 2014 Oct

3. Trends in autologous haematopoietic cell transplantation for multiple myeloma in Europe: increased use and improved outcomes in elderly patients in recent years. Auner HW et al., *Bone Marrow Transplant.* 2014 Nov 10

4. Outcome of allogeneic Stem Cell Transplantation for patients transformed to myelodysplastic syndrome or Leukaemia from severe aplastic anaemia: a report from the MDS Subcommittee of the Chronic Malignancies Working Party and the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation. Hussein AA et al., *Biol Blood Marrow Transplant.* 2014 Sep

Business meeting and educational event "Stem Cell Transplantation in MDS, September 2014 in Lille, France

Four major educational courses

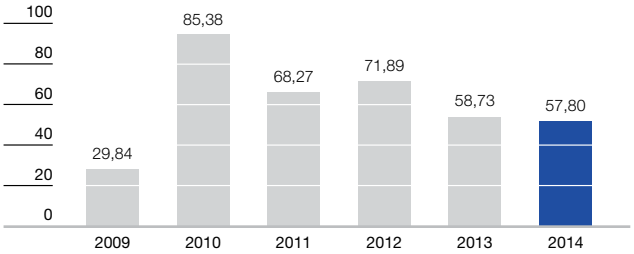
1. Educational event: "The Role of Allogeneic Stem Cell Transplantation in Chronic Lymphocytic Leukaemia in the era of novel drugs" - February 1 2014 in Leiden, The Netherlands.

2. Educational event: "Stem Cell Transplantation in MDS" - September 12 2014 in Lille, France.

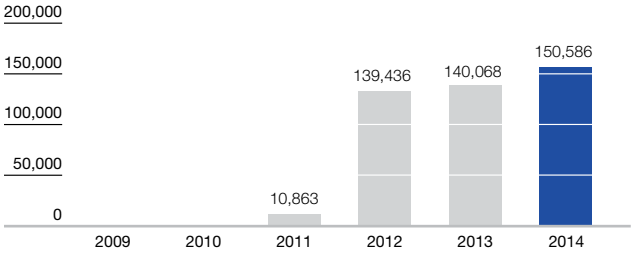
3. Preceptorship meeting: "Stem Cell Transplantation in Multiple Myeloma" - September 25-26 2014 in Torino, Italy.

4. Scientific meeting: "Stem Cell Transplantation in Multiple myeloma in EBMT and CIBMTR" - April 2014 (during EBMT Annual meeting) in Milano, Italy.

Impact factor (Chronic Malignancies)



Overview of registered patient data (Chronic Malignancies)



	2012	2013	**2014**
Oral Presentations	28	41	**63**
Poster Presentations	4	9	**14**
International Educational Events	1	4	**4**

05

Complications & Quality of Life Working Party

Chair: **Rafael Duarte**

Major achievements in 2014

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 including Grzegorz Basak (WP Secretary), Diana Greenfield (Nurse Lead for transplant complications), Hildegard Greinix (GVHD subcommittee), Nina Salooja (Late Complications subcommittee), Tapani Ruutu (Conditioning-related complications and supportive care subcommittee) and Rafael Duarte (WP Chair). Our main goal is to combine expertise to provide the Society with a strong WP focused on transversal research on transplant complications, in collaboration with other WPs and Committees within the Society, and external collaborations with international groups.

The educational highlight of 2014 was our WP Course, hosted by Grzegorz Basak in Warsaw, Poland, on October 23rd – 24th. The course was a big success, with more than 70 participants, and covered many topics across the scientific scope of our WP, including organisational aspects of long-term transplant care and services, the perspective on the management of organ-specific complications from expert support specialists, a particular focus on the impact of conditioning-regimen intensity on transplant complications, and the first international educational session on the revised NIH recommendations of chronic GVHD 2014, in which several members of our WP have participated as overseas experts in the various working committees. Of note, the course gave us an opportunity to enjoy a keynote lecture by Professor Wiktor-Jedrzejczak and celebrate the 30th anniversary of haematopoietic transplantation in Poland. In 2015, a new educational course and business meeting will be hosted on October 29th and 30th in Leuven, Belgium, by Hélène Schoemans.

In 2014, we have also continued the international collaborative work started in 2013 through the EBMT-NCI Task Force on chronic GVHD and survivorship issues after transplant, with a first publication on an international analysis of clinical practice on chronic GVHD and NIH-criteria, and we also started a new line of collaborations on postransplant guidelines with our colleagues at the CIBMTR, with a first manuscript on secondary solid cancer screening following Haematopoietic Cell Transplantation. A good number of studies are coming to the end of their cycle, and many new proposals have been recently launched in 2014. We hope that many of these studies, some of them summarized below, will be of your interest. We would encourage you all to attend our business meeting and WP Session in Istanbul 2015 to learn more about these and other important studies.

Please, do feel invited to bring in your own proposals and ideas, and to get involved in the work of the CQLWP.

Major research studies

1. Prospective observational registry to collect safety and outcome data in patients with severe hepatic VOD treated with Defitelio® or supportive care (PI: M Mohty, CIC-775)
2. Cross-sectional non-interventional study on metabolic syndrome after HCT (PIs: D Greenfield & J Snowden, CIC-778)
3. Retrospective analysis of the prevalence and outcome of pregnancy following HCT (PI: N Salooja, CIC-205)
4. Prospective non-interventional study of the association between uric acid levels and danger signals and graft-versus-host disease (PI: O Penack, CIC-807)

Best publications

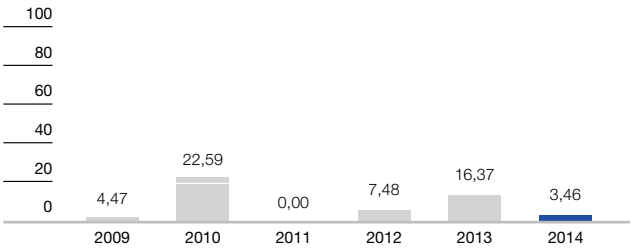
1. Uptake and use of recommendations for the diagnosis, severity scoring and management of chronic GVHD: an international survey of the EBMT-NCI Chronic GVHD Task Force. RF Duarte et al. *Bone Marrow Transplant*.

2. Allogeneic haematopoietic Stem Cell Transplantation in solid organ transplant recipients: a retrospective, multicenter study of the EBMT. G Basak et al. *Am J Transplant*.

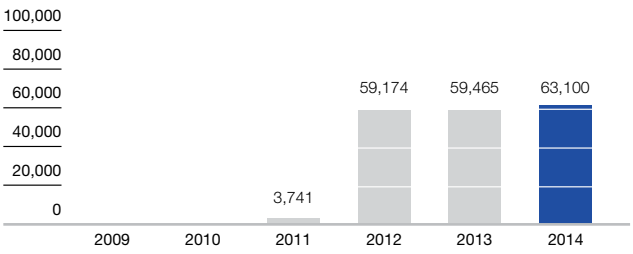
Major educational courses

1. EBMT Complications and Quality of Life Working Party educational course - October 23-24 2014 in Warsaw, Poland

Impact factor (Complications and Quality of Life)



Overview of registered patient data (Complications and Quality of Life)



The Cellular Therapy & Immunobiology, Infectious Diseases, and Complications & Quality of Life Working Parties are transversal Working Parties dealing with all patients regardless of diagnosis or age; the number shown for these Working Parties is the average of patients in the Registry for the other eight Working Parties.

	2012	2013	**2014**
Oral Presentations	0	0	**1**
Poster Presentations	0	2	**2**
International Educational Events	1	2	**1**

06

Educational events 2014

- **CMWP:** Chronic Malignancies Working Party
- **PDWP:** Paediatric Diseases Working Party
- **SAAWP:** Severe Aplastic Anaemia Working Party
- **IDWP:** Infectious Diseases Working Party
- **LWP:** Lymphoma Working Party
- **IEWP:** Inborn Errors Working Party
- **CQLWP:** Complications and Quality of Life Working Party
- **STWP:** Solid Tumours Working Party
- **ADWP:** Autoimmune Diseases Working Party
- **ALWP:** Acute Leukaemia Working Party

Leiden, The Netherlands
1 Feb 2014
65 attendees
CMWP Educational Event: The role of Stem Cell Transplantation in Chronic Lymphocytic Leukaemia in the era of novel drugs

Vienna, Austria
8 - 10 May 2014
200 attendees
18th ESH-EBMT Training Course on HSCT

Jerusalem, Israel
21 - 23 May 2014
100 attendees
9th Meeting of the PDWP, 4th Meeting of the Paediatric Nurses including IEWP Educational Day

Lille, France
13 Sept 2014
30 attendees
CMWP in conjunction with the SFGM-TC - Critical Issues in Allogeneic Stem Cell Transplantation for MDS

Torino, Italy
25 - 26 Sept 2014
60 attendees
CMWP Meeting - Stem Cell Transplantation in Multiple Myeloma

Naples, Italy
29 Sept-1 Oct 2014
100 attendees
SAA and ID WPs: Bruno Rotoli Memorial Joint Educational Course

Nicosia, Cyprus
16 -17 Oct 2014
60 attendees
LWP 10th Educational Course - Treatment of Malignant Lymphoma: State-of-the Art and the Role of Stem Cell Transplantation

Munich, Germany
17 - 19 Oct 2014
90 attendees
IEWP Conference

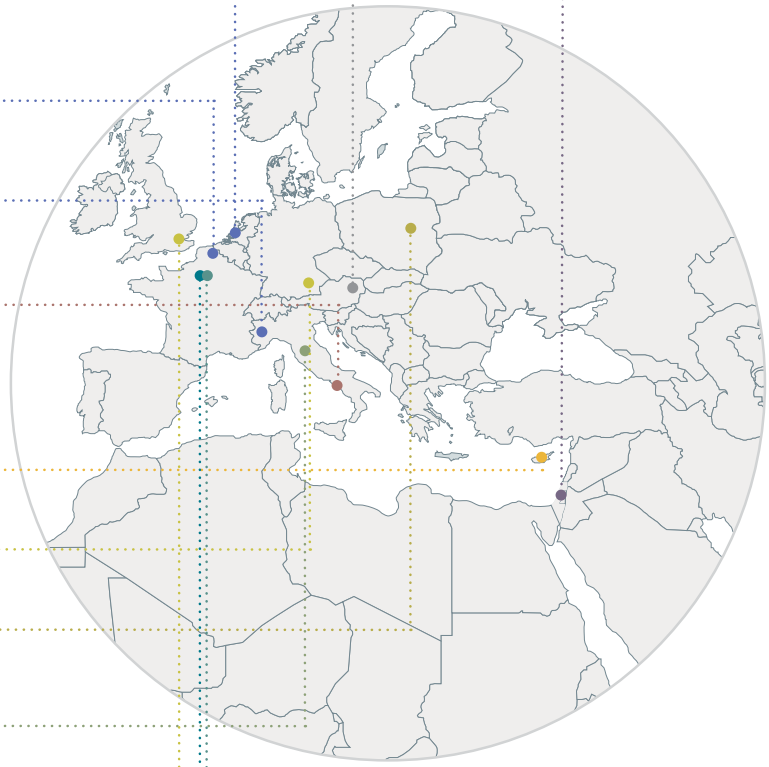
Warsaw, Poland
23 - 24 Oct 2014
70 attendees
CQLWP Educational Course

Florence, Italy
31 Oct 2014
60 attendees
STWP and Nurses Group Educational Meeting

London, UK
10 Nov 2014
40 attendees
IEWP - Thymic Disorders Workshop

Paris, France
28 - 29 Nov 2014
20 attendees
ADWP Educational Meeting on Stem Cell Therapy

Paris, France
28 - 29 Nov 2014
80 attendees
ALWP Meeting and Educational Symposium - Unrelated Donor Haematopoietic Cell Transplantation for Acute Leukaemia



Publications 2014

Title	First Listed Author	*Journal*	PMID
Outcome of aplastic anaemia in adolescence: a survey of the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation.	Dufour C	*Haematologica*	25085353
Outcome of allogeneic Stem Cell Transplantation for patients transformed to myelodysplastic syndrome or Leukaemia from severe aplastic anaemia: a report from the MDS Subcommittee of the Chronic Malignancies Working Party and the Severe Aplastic Anaemia Working Party of the European Group for Blood and Marrow Transplantation.	Hussein AA	*Biol Blood Marrow Transplant.*	24910382
Autologous haematopoietic Stem Cell Transplantation in neuromyelitis optica: A registry study of the EBMT Autoimmune Diseases Working Party.	Greco R	*Mult Scler.*	25078274
Onset and outcome of pregnancy after autologous haematopoietic SCT (AHSCT) for autoimmune diseases: a retrospective study of the EBMT autoimmune diseases working party (ADWP).	Snarski E	*Bone Marrow Transplant.*	25387098
Risk factors for outcomes after unrelated cord blood transplantation for adults with acute lymphoblastic Leukaemia: a report on behalf of Eurocord and the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Tucunduva L	*Bone Marrow Transplant.*	24986801
Comparing i.v. BU dose intensity between two regimens (FB2 vs FB4) for allogeneic HCT for AML in CR1: a report from the Acute Leukaemia Working Party of EBMT.	Kharfan-Dabaja MA	*Bone Marrow Transplant.*	24978140
Prediction of non-relapse mortality in recipients of reduced intensity conditioning allogeneic Stem Cell Transplantation with AML in first complete remission.	Versluis J	*Leukaemia*	24913728
Intravenous busulfan for autologous Stem Cell Transplantation in adult patients with acute myeloid Leukaemia: a survey of 952 patients on behalf of the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Nagler A	*Haematologica*	24816236
Extreme heterogeneity of myeloablative total body irradiation techniques in clinical practice: a survey of the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Giebel S	*Cancer.*	24804873
Who is the best haematopoietic stem-cell donor for a male patient with acute Leukaemia?	Ringdén O	*Transplantation.*	24798307
Impact of in vivo T-cell depletion on outcome of AML patients in first CR given peripheral blood stem cells and reduced-intensity conditioning allo-SCT from a HLA-identical sibling donor: a report from the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Baron F	*Bone Marrow Transplant.*	24419525
Impact of postremission consolidation chemotherapy on outcome after reduced-intensity conditioning allogeneic Stem Cell Transplantation for patients with acute myeloid Leukaemia in first complete remission: a report from the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Yeshurun M	*Cancer*	24338939
A prospective registration study to determine feasibility of haematopoietic SCT in adults with acute Leukaemia: planning, expectations and reality.	Labopin M	*Bone Marrow Transplant.*	24241579
Improving results of autologous Stem Cell Transplantation for Philadelphia-positive acute lymphoblastic leukaemia in the era of tyrosine kinase inhibitors: a report from the Acute Leukaemia Working Party of the European Group for Blood and Marrow Transplantation.	Giebel S	*Eur J Cancer.*	24210524
Tyrosine kinase inhibitors improve long-term outcome of allogeneic haematopoietic Stem Cell Transplantation for adult patients with Philadelphia chromosome positive acute lymphoblastic Leukaemia.	Brissot E	*Haematologica*	25527562
Chemotherapy Dose Adjustment for Obese Patients Undergoing Haematopoietic Stem Cell Transplantation: A Survey on Behalf of the Acute Leukaemia Working Party of the European Society for Blood and Marrow Transplantation.	Shem-Tov N	*Oncologist*	25480827
A survey on unmanipulated haploidentical haematopoietic stem cell transplantation in adults with acute Leukaemia.	Piemontese S	*Leukaemia*	25434302

Title	First Listed Author	*Journal*	PMID
Comparison of outcomes after single or double cord blood transplantation in adults with acute Leukaemia using different types of myeloablative conditioning regimen, a retrospective study on behalf of Eurocord and the ALWP of the EBMT.	Ruggeri A	*Leukaemia*	24005245
Impact of minimal residual disease on outcomes after umbilical cord blood transplantation for adults with Philadelphia-positive acute lymphoblastic leukaemia: an analysis on behalf of Eurocord, Cord Blood Committee and the Acute Leukaemia working party of the EBMT.	Tucunduva L	*Br J Haematol.*	24961645
Fourth European Conference on Infections in Leukaemia (ECIL-4): guidelines for diagnosis, prevention, and treatment of invasive fungal diseases in paediatric patients with cancer or allogeneic haemopoietic stem-cell transplantation.	Groll AH	*Lancet Oncol.*	24988936
Aetiology and resistance in bacteraemias among adult and paediatric haematology and cancer patients.	Mikulska M	*J Infect.*	24370562
Donor cytomegalovirus status influences the outcome of allogeneic stem cell transplant: a study by the European group for blood and marrow transplantation.	Ljungman P	*Clin Infect Dis.*	24850801
Reduced-intensity conditioning and HLA-matched haemopoietic stem-cell transplantation in patients with chronic granulomatous disease: a prospective multicentre study.	Güngör T	*Lancet*	24161820
Survey of CMV management in paediatric allogeneic HSCT programs, on behalf of the inborn errors, infectious diseases and paediatric diseases working parties of EBMT.	Bontant T	*Bone Marrow Transplant.*	24162611
Haematopoietic Stem Cell Transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel.	Angelucci E	*Haematologica*	24790059
The impact of total body irradiation on the outcome of patients with follicular lymphoma treated with autologous stem-cell transplantation in the modern era: a retrospective study of the EBMT Lymphoma Working Party.	El-Najjar I	*Ann Oncol.*	25193988
Outcome of patients with HTLV-1-associated adult T-cell Leukaemia/lymphoma after SCT: a retrospective study by the EBMT LWP.	Bazarbachi A	*Bone Marrow Transplant.*	25029232
Allogeneic and Autologous Stem Cell Transplantation for hepatosplenic T cell lymphoma: A retrospective study of the EBMT Lymphoma Working party.	Tanase A	*Leukaemia*	25234166
Long-term outcome of allogeneic haematopoietic cell transplantation for patients with mycosis fungoides and sézary syndrome: a European society for blood and marrow transplantation lymphoma working party extended analysis.	Duarte RF	*J Clin Oncol.*	25154828
The role of allogeneic Stem Cell Transplantation in Hodgkin's lymphoma.	Sureda A	*Curr Treat Options Oncol.*	24752768
Matched unrelated donor allogeneic transplantation provides comparable long-term outcome to HLA-identical sibling transplantation in relapsed diffuse large B-cell lymphoma.	Avivi I	*Bone Marrow Transplant.*	24510071
Alternative donor haematopoietic Stem Cell Transplantation for mature lymphoid malignancies after reduced-intensity conditioning regimen: similar outcomes with umbilical cord blood and unrelated donor peripheral blood.	Rodrigues CA	*Haematologica*	23935024
Treosulfan-based conditioning regimens for allogeneic HSCT in children with acute lymphoblastic leukaemia.	Boztug H	*Ann Hematol.*	25231927
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.*	25521756
High-dose chemotherapy for germ cell tumors: do we have a model?	Necchi A	*Expert Opin Biol Ther.*	25243977
Erythropoiesis-stimulating agents in allogeneic and autologous haematopoietic Stem Cell Transplantation	Martino M	*Expert Opin Biol Ther.*	25315815
Allogeneic Stem Cell Transplanatation for Myelofibrosis with Leukemic Transformation: A Study from the Myeloproliferative Neoplasm Subcommittee of the CMWP of the European Group for Blood and Marrow Transplantation	Alchalby H	*Biol Blood Marrow Transplant.*	24201159
Imporved PFS after autologous Stem Cell Transplantation does not translate into better Quality of Life in CLL: lessons from the randomized EBMT-Intergroup study	de Wreede LC	*Am J Hematol.*	24123244
Prophylaxis and treatment of GVHD: EBMT-ELN working group recommendations for a standardized practice	Ruutu T	*Bone Marrow Transplant.*	23892326
Allogeneic haematopoietic Stem Cell Transplantation in patients with polycythemia vera or essential thrombocythemia transformed to myelofibrosis or acute myeloid Leukaemia: a report from the MPN Subcommittee of the Chronic Malignancies Working Party	Lussana F	*Haematologica*	24389309

Title	First Listed Author	*Journal*	PMID
Impact of the International Prognostic Scoring System cytogenetic risk groups on the outcome of patients with primary myelodysplastic syndromes undergoing allogeneic Stem Cell Transplantation from human leukocyte antigen-identical siblings: a retrospective analysis of the European Society for Blood and Marrow Transplantation-Chronic Malignancies Working Party.	Onida F	*Haematologica*	25085359
Early administration of donor lymphocyte infusions upon molecular relapse after allogeneic haematopoietic Stem Cell Transplantation for chronic myeloid Leukaemia: a study by the Chronic Malignancies Working Party of the EBMT.	Chalandon Y	*Haematologica*	24997146
Trends in autologous haematopoietic cell transplantation for multiple myeloma in Europe: increased use and improved outcomes in elderly patients in recent years.	Auner HW	*Bone Marrow Transplant.*	25387088
Should the standard dimethyl sulfoxide concentration be reduced? Results of a European Group for Blood and Marrow Transplantation prospective noninterventional study on usage and side effects of dimethyl sulfoxide.	Morris C	*Transfusion*	24964911
Unrelated cord blood transplantation for patients with primary or secondary myelofibrosis.	Robin M	*Biol Blood Marrow Transplant.*	24946719
Comparison of Unrelated Cord Blood and Peripheral Blood Stem Cell Transplantation in Adults with Myelodysplastic Syndrome after Reduced-Intensity Conditioning Regimen: A Collaborative Study from Eurocord and Chronic Malignancies Working Party.	Robin M	*Biol Blood Marrow Transplant.*	25529382
Uptake and use of recommendations for the diagnosis, severity scoring and management of chronic GVHD: an international survey of the EBMT-NCI Chronic GVHD Task Force.	Duarte RF	*Bone Marrow Transplant.*	23955633
Nurses' practice patterns in relation to adherence-enhancing interventions in stem cell transplant care: a survey from the Nurses Group of the European Group for Blood and Marrow Transplantation.	Kirsch M	*Eur J Cancer Care (Engl)*	24393127
Basic oral care for haematology-oncology patients and haematopoietic stem cell transplantation recipients: a position paper from the joint task force of the Multinational Association of Supportive Care in Cancer/International Society of Oral Oncology (MASCC/ISOO) and the European Society for Blood and Marrow Transplantation (EBMT).	Elad S	*Support Care Cancer.*	25189149
Haematopoietic SCT in Europe: data and trends in 2012 with special consideration of paediatric transplantation.	Passweg JR	*Bone Marrow Transplant.*	24637898
[Recommended screening and preventive practices for long-term survivors after haematopoietic cell transplantation].	Majhail NS	*Rinsho Ketsueki.*	24975331
Sensitivity of hematological malignancies to graft-versus-host effects: an EBMT megafile analysis.	Stern M	*Leukaemia*	24781016
Autologous haematopoietic stem cell mobilisation in multiple myeloma and lymphoma patients: a position statement from the European Group for Blood and Marrow Transplantation.	Mohty M	*Bone Marrow Transplant.*	24686988
Allogeneic haematopoietic SCT for adults AML using i.v. BU in the conditioning regimen: outcomes and risk factors for the occurrence of hepatic sinusoidal obstructive syndrome.	Nagler A	*Bone Marrow Transplant.*	24535127
Managing high-risk chronic lymphocytic Leukaemia during transition to a new treatment era: Stem Cell Transplantation or novel agents?	Dreger P	*Blood*	25301705
The EBMT/EMCL consensus project on the role of autologous and allogeneic Stem Cell Transplantation in mantle cell lymphoma.	Robinson S	*Leukaemia*	25034148
Outcome and prognostic factors in patients with mantle-cell lymphoma relapsing after autologous stem-cell transplantation: a retrospective study of the European Group for Blood and Marrow Transplantation (EBMT).	Dietrich S	*Ann Oncol.*	24585719
Autologous haematopoietic Stem Cell Transplantation vs intravenous pulse cyclophosphamide in diffuse cutaneous systemic sclerosis: a randomized clinical trial.	van Laar JM	*JAMA*	25058083
Regulation of advanced therapy medicinal products will affect the practice of haematopoietic SCT in the near future: a perspective from the EBMT cell-processing committee.	Chabbanon C	*Bone Marrow Transplant.*	25501349

08

EBMT Transplant Activity Survey 2013

Recent trends in the use of alternative donors showing more haplo-identical donor but fewer cord blood transplants.

This year's annual report based on the 2013 survey provides us with the most recent data on activity, transplant rates and indications and focuses on the use of donors other than HLA identical siblings and matched unrelated donors for allogeneic HSCT.

658 teams in 48 countries reporting to the 2013 survey performed a total of 39,209 transplants in 34,809 patients. Of these, 16,211 (41%) were allogeneic; 22,998 (59%) autologous. This is an increase of 3.7% (5.6% allogeneic HSCT and 2.4% autologous HSCT) when compared to 2012 and 26% when compared to five years previously indicating that HSCT continues to be an increasingly important treatment modality in the era of targeted antibody and molecular therapy.

Main indications (table 1) were Leukaemias; 11,190 (32% of total; 96% of which were allogeneic); lymphoid neoplasias including Non Hodgkin lymphoma, Hodgkin lymphoma, and plasma cell disorders; 19,958 (57%; 11% allogeneic); solid tumors; 1,543 (4%; 4% allogeneic); and non-malignant disorders; 1,975 (6%; 91% allogeneic). As seen in previous years, the majority of HSCT for lymphoid malignancies were autologous while most transplants for Leukaemia were performed using stem cells from allogeneic donors. Autologous HSCT for non-malignant disorders predominantly include patients with autoimmune disorders. When compared to 2012 there were increases in allogeneic HSCT for AML in CR1 (10.7%), MPN (11.1%) and NHL (12.5%). For autologous HSCT there was an increase for plasma cell disorders by 6.1% and a decrease in activity for AML (18%) and HD (10%) which may be related to the availability of monoclonal antibodies.

Noteworthy in this years' survey is the increase in the use of allogeneic HSCT more than autologous HSCT thus narrowing the gap in numbers of autologous and allogeneic HSCT and in allogeneic HSCT the increasing use of alternative donor transplants, where an impressive trend for more haplo-identical HSCT has been observed (figure 1a). The increase in haplo-identical HSCT coincides with the publications of the post-transplant cyclophosphamide GvHD prophylaxis in haplo-identical HSCT. This is accompanied by a slight decrease in HSCT using cord blood pointing to the fact that mismatched unrelated cord blood and haplo-identical donors are in competition for patients who do not have a sibling or matched unrelated donor (figure 1a).

Figure 1b shows the use of sibling and unrelated donor HSCT both continuing to increase, please pay attention to the fact that the scale in figure 1a is 5x the scale in figure 1b. When comparing the use of donors for allogeneic HSCT in countries with high transplant rates it is obvious that there are important differences. Some may be explained by availability of sibling donors as there are differences in family size across Europe. There are, however, 3 fold differences in transplant rates for sibling and unrelated donor HSCT among countries and even larger differences in the use of unrelated cord blood and haplo-identical donors probably reflecting availability, financial issues as well as differences in the interpretation of results of recent studies and local experience.

The annual survey continues to provide valuable and up-to-date information on use of haematopoietic Stem Cell Transplantation throughout Europe. It not only reflects current practice but also provides essential material for health care planning and health policy makers.

Helen Baldomero **Jakob Passweg**
EBMT Activity Survey Data Offices

Table 1. Numbers of haematopoietic Stem Cell Transplants in Europe 2013 by indication, donor type and stem cell source

	Family Donor								Unrelated Donor			Total Allogenic HSCT	Autologous			Total Allogenic HSCT	Total HSCT
	HLA-identical			other relative, MM, haplo-id.			twin						BM	BM+			
	BM	PBPC	Cord	BM	PBPC	Cord	BM	PBPC	BM	PBPC	cord		only	PBPC	cord		
Leukaemias	**725**	**3140**	**15**	**332**	**557**	**2**	**5**	**24**	**795**	**4675**	**420**	**10690**	**10**	**489**	**1**	**500**	**11190**
Acute myeloid leukaemia	309	1599	5	169	271	1	2	9	315	2337	211	5228	7	373	0	380	5608
1st complete remission	232	1123	2	88	118	1	1	9	209	1340	114	3237	6	313	0	319	3556
not 1st complete remission	77	476	3	81	153	0	1	0	106	997	97	1991	1	60	0	61	2052
Acute lymphatic leukaemia	276	688	9	86	185	1	2	8	271	767	112	2405	3	72	0	75	2480
1st complete remission	148	486	3	41	77	1	1	6	141	477	60	1441	0	57	0	57	1498
not 1st complete remission	128	202	6	45	108	0	1	2	130	290	52	964	3	15	0	18	982
Chronic myeloid leukaemia	31	107	0	18	20	0	0	0	36	173	13	398	0	3	0	3	401
chronic phase	20	51	0	6	6	0	0	0	21	65	5	174	0	0	0	0	174
not 1st chronic phase	11	56	0	12	14	0	0	0	15	108	8	224	0	3	0	3	227
MDS, MDS/MPN overlap	93	476	1	42	69	0	1	6	134	882	58	1762	0	11	1	12	1774
MPN	11	129	0	9	8	0	0	0	22	257	16	452	0	5	0	5	457
Chronic lymphocytic leukaemia	5	141	0	8	4	0	0	1	17	259	10	445	0	25	0	25	470
Lymphoproliferative disorders	**94**	**721**	**0**	**80**	**111**	**0**	**1**	**17**	**108**	**1097**	**55**	**2284**	**51**	**17623**	**0**	**17674**	**19958**
Plasma cell disorders	19	226	0	6	16	0	1	10	29	295	4	606	6	9788	0	9794	10400
Hodgkin's lymphoma	14	102	0	34	55	0	0	1	20	161	23	410	21	1859	0	1880	2290
Non Hodgkin lymphoma	61	393	0	40	40	0	0	6	59	641	28	1268	24	5976	0	6000	7268
Solid tumors	**2**	**5**	**0**	**4**	**34**	**0**	**0**	**0**	**7**	**8**	**0**	**60**	**54**	**1428**	**1**	**1483**	**1543**
Neuroblastoma	0	2	0	2	26	0	0	0	2	5	0	37	22	464	1	487	524
Soft tissue sarcoma	0	0	0	0	3	0	0	0	1	0	0	4	5	19	0	24	28
Germinal tumors	0	0	0	0	0	0	0	0	0	0	0	0	2	353	0	355	355
Breast cancer	0	1	0	0	0	0	0	0	0	0	0	1	0	42	0	42	43
Ewing	1	0	0	1	2	0	0	0	1	1	0	6	15	200	0	215	221
Other solid tumors	1	2	0	1	3	0	0	0	3	2	0	12	10	350	0	360	372
Non malignant disorders	**601**	**227**	**47**	**86**	**111**	**1**	**1**	**3**	**355**	**245**	**119**	**1796**	**4**	**175**	**0**	**179**	**1975**
Bone marrow failure	237	141	12	30	29	1	1	3	168	121	29	772	0	1	0	1	773
Hemoglobinopathies	216	62	22	24	11	0	0	0	34	14	1	384	0	1	0	1	385
Primary Immune deficiencies	120	18	10	28	56	0	0	0	118	89	57	496	2	2	0	4	500
Inh. disorders of Metabolism	25	6	1	3	11	0	0	0	28	16	31	121	1	1	0	2	123
Auto immune disease	3	0	2	1	4	0	0	0	7	5	1	23	1	170	0	171	194
Others	23	20	0	5	7	0	0	0	27	26	12	120	0	23	0	23	143
TOTAL PATIENTS	**1445**	**4113**	**62**	**507**	**820**	**3**	**7**	**44**	**1292**	**6051**	**606**	**14950**	**119**	**19738**	**2**	**19859**	**34809**
TOTAL TRANSPLANTS	**1529**	**4403**	**64**	**574**	**992**	**5**	**8**	**49**	**1361**	**6560**	**666**	**16211**	**129**	**22867**	**2**	**22998**	**39209**

Figure 1a. Absolute numbers of haplo-identical and cord blood HSCT in Europe 1998-2013

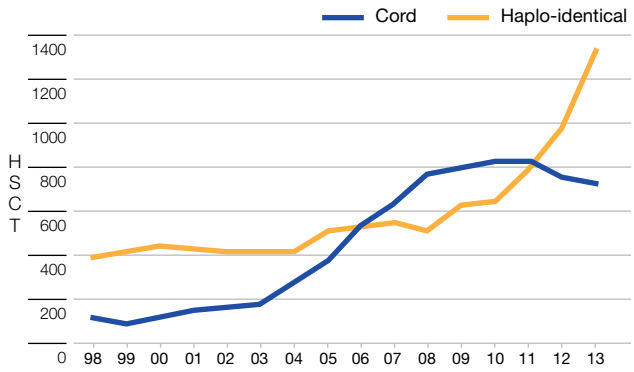
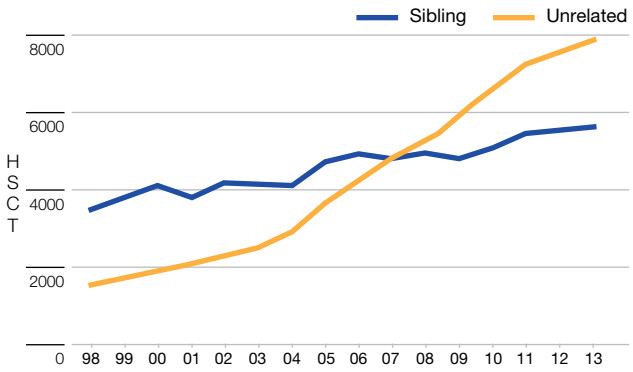


Figure 1b. Absolute numbers of sibling donor and unrelated donor HSCT in Europe 1998-2013



Bone Marrow Transplantation (2015), 1–7 Epub

09

The Registry Office

Data overview

A total of **435,219** patients and **515,355** transplants appear as registered in the EBMT Registry at the beginning of 2015. Of these, **36,940** transplants were registered during 2014. The number of transplants registered during 2014 that used cord blood as source of stem cells was **773**, with **29** being done in the autologous setting.

Data managers from the transplanting centres directly entered **74%** (4% drop on last year) of the transplants with the rest being entered by a mixture of national registry and EBMT staff.

Registry upgrade

This project has suffered an important setback during 2014. As we reported in 2013, the provider had been unable to keep to the deadlines and it was clear half way through the year that they were in financial difficulties. In October, they ceased business and their assets were eventually auctioned and bought by another company.

The EBMT is in negotiations with the new company to see whether they can continue with the project, but this is not guaranteed and the company is still assessing the percentage of the project that can be considered to have been done and the cost of continuing with it. In the meantime, the project is on hold. The EBMT is maintaining communication with other clients that were left in the same situation and is open to approaches from other possible providers.

ProMISe version 3

Consequent to the delay of the Registry upgrade project, the Registry needs to move from ProMISe version 2 to version 3, as the providers of ProMISe are unwilling to maintain version 2 any longer. ProMISe version 3 has enhanced security features and some extra functionality. The underlying structure and database are the same as in the previous version.

Data Sharing

AGNIS project

During 2014 we have seen improvements done to the underlying code developed in the EBMT office which have sped up the data submission. Under the existing testing conditions, over 8,000 forms (Med-A, subdivided in two parts to make allowances for CIBMTR TED structure) achieved the status of submitted and accepted. Work was also done on reconciliation of patient and donor records. Documentation regarding business rules for the data transfer continues to be compiled.

Data quality

The Registry office continued a sustained effort to fill in essential missing information and fix problems which would have rendered data migration to the new system difficult. A series of Data Quality queries were set in ProMISe, specifically to address these issues. A session to demonstrate them took place during the annual meeting, and communication with the centres regarding them has continued throughout the year. The 5-year follow up request project has been relaunched, with responses already being received.

Data collection

Med-A

The Registry Committee in collaboration with the Definitions group, has successfully come up with a new extended Med-A. The new Med-A was meant to be implemented in the new system. Given the circumstances, the Registry is reviewing its content to assess how much of it can be implemented in our current system.

Donor follow up

Changes to the navigation necessary to allow the registration of donor follow up after DLI or other cell therapy treatments have been implemented in the Registry.

Carmen Ruiz de Elvira
Head of the EBMT Registry

The EBMT Nurses Group

The EBMT Nurses Group (NG) plays an essential role in Haematology and Haematological Stem Cell Transplantation (HSCT) nursing. We continue to build on high levels of nursing representation in EBMT clinical centres, with nurses and allied health professional contacts in over 50 countries worldwide. The group is dedicated to improving the care of patients receiving SCT and works towards promoting excellence in care through recognising and building upon evidence based practice. The EBMT NG mission is to enhance and value the nurses' role all over the world, supporting and sharing knowledge through communication, advocacy, research, training and education.

The NG Sub Committees have continued their efforts, making 2014 a productive and rich year.

Education

The ***Scientific Committee*** organised the Nurses Programme at the Annual Meeting in Milan as well as the Education Day, which continues to increase in the number of attendees. Both were a real success with 526 nurses attending the meeting, over 130 abstracts submitted, and excellent speakers contributing to an exciting meeting. The Distinguished Merit Award was awarded to Monica Fliedner, for her dedication and strategic work emphasizing research, education and collaboration as fundamentals contributing to the successful development of the Nurses Group.

The *VOD project* was launched as a demonstration of our commitment to educational initiatives.

The *International Study Day* was held in Florence in collaboration with the National Italian Transplant (GITMO).

During 2014, the Swiss EBMT NG launched the *Lymphoma Learning Programme*, designed to support nurses and other health care professionals in delivering optimal care to lymphoma patients.

All these materials are available for download from the EBMT website.

The ***Paediatric Committee*** and Paediatric Diseases Working Party (PDWP) had the 4th Nurses Group meeting in Jerusalem, Israel, with over 65 nurses attending from 17 countries.

A new educational project for nurses and patients relating to Paroxysmal Nocturnal Haemoglobinuria began this year and will be launched during 2015.

Research

In 2014 the ***Research Committee*** published work relating to adherence:

Nurses' practice patterns in relation to adherence-enhancing interventions in Stem Cell Transplant care: a survey from the Nurses Group of the European Group for Blood and Marrow Transplantation. Kirsch M et al., *Eur J Cancer Care*.

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

Other ongoing research projects include the evaluation of quality of life during SC mobilisation and apheresis, and several collaborative research projects where the Research Committee is supporting and facilitating NG member initiatives.

Communication

The ***Communication and Networking Committee*** is responsible for delivering nursing news to the EBMT Newsletters and has updated and maintained the nursing section of the website. The challenge for 2015 will be to explore social media communication as an innovative way to communicate with the nursing community.

Collaboration

We had successful National Groups & Forums Chairs Meetings in Frankfurt and in Florence this year. As for 2015, the International Study Day will be in Pilsen, Czech Republic.

Our collaboration continues with the major oncology and nursing societies, where we presented the EBMT NG at the EONS 9th meeting in 2014.

Further links have been forged with Saudi Society for BMT, where EBMT NG representatives were invited speakers at the 4th Annual Meeting of SSBMT / EBMT in Riyadh, Saudi Arabia.

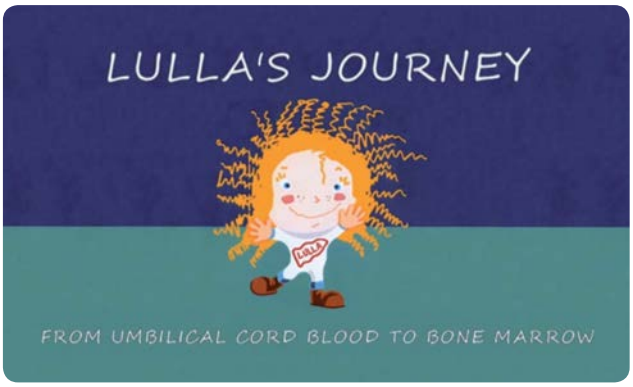
Together with MASCC and ISOO, a position paper about Basic Oral Care has been developed: Basic oral care for haematology-oncology patients and haematopoietic Stem Cell Transplantation recipients: a position paper from the joint task force of the Multinational Association of Supportive Care in Cancer/International Society of Oral Oncology (MASCC/ISOO) and the European Society for Blood and Marrow Transplantation (EBMT). Elad S et al., *Support Care Cancer*.

Collaboration with patient associations and foundations has been initiated, and this year a short film for children, shown at the opening of the Annual Meeting was an excellent beginning. The movie "Lulla's Journey" aims to explain the transplant process to children using children as actors in the story of a stem cell pathway from cord bank through bone marrow to hospital discharge. This movie is also available on the website.

Another collaboration is between EBMT NG & charitable foundations for an outreach project aiming to promote HSCT nursing education and practice in low and middle income countries.

Our achievements during 2014 have been higher than our expectations in terms of research, education and collaboration. In 2015, we look forward to continuing our accomplishments and transferring this into high quality care for our patients.

Aleksandra Babic
EBMT Nurses Group President



40th Anniversary edition of the EBMT Annual Meeting

The 40th anniversary edition of the EBMT Annual Meeting saw 4,622 participants gather in Milan for the 5 day event. The Scientific Programme, designed by the local Organising Committee under the leadership of the Congress President Marco Bregni, covered a wide range of topics in varying session formats.

The 2014 edition of the EBMT Abstract Book included a record total of 1,153 abstracts and was visited more than 1500 times on its new online platform.

40th Anniversary celebrations

2014 was marked by the EBMT 40th anniversary. One of the key successes of this special milestone was the creation of the EBMT timeline 1974 – 2014. The 20-meter long timeline was displayed in the exhibition area of MiCo Congress center in Milan. Moreover, a printed leaflet was distributed in the delegates' bags and an online version was posted on the EBMT website. You can view this timeline on www.ebmt.org.

The Opening Session ended in a cheerful note on the occasion of the 40th Anniversary. President Alejandro Madrigal presented a big birthday cake that was served at the welcome reception.

EBMT Stand

The exhibition area welcomed 60 exhibitors. Along with its main stand, the EBMT had a dedicated space for the 23 posters on clinical trials, studies and registry and the latest transplant activity survey.

Huge birthday cake to celebrate the 40th Anniversary

Awards and Prices

In the 40th EBMT Annual Meeting, 12 different awards have been given to remarkable researchers for their outstanding contributions to science and to the EBMT.

Van Bekkum Award to Stephan Grupp, lead author of the abstract entitled: *T CELLS ENGINEERED WITH A CHIMERIC ANTIGEN RECEPTOR (CAR) TARGETING CD19 (CTL019 CELLS) PRODUCE SIGNIFICANT IN VIVO PROLIFERATION, COMPLETE RESPONSES AND LONG-TERM PERSISTENCE WITHOUT GVHD IN CHILDREN AND ADULTS WITH RELAPSED, REFRACTORY ALL*

Basic Science Award supported by Clinigen to Nicoletta Cieri, lead author of the abstract entitled: *TRACKING T CELL DYNAMICS IN THE FIRST MONTH AFTER ALLOGENEIC HSCT OFFERS A UNIQUE OPPORTUNITY TO UNVEIL THE MECHANISM OF MEMORY STEM T CELL FORMATION IN HUMANS*

Oustanding Contribution to the EBMT to Ronald Brand, for his continuous and dedicated work in the development of ProMISe EBMT Registry.

Honorary Membership to Shaun McCann and Massimo Martelli.

Clinical Achievement Award to Rose Hamladji for her lifelong achievements in the field of Bone and Marrow Transplantation. Professor Hamladji was a pioneer in Haematology in Algeria.

Jian-Jian Luan Award for Lymphoma Transplant Research to Anne-Claire Mamez, lead author of the abstract entitled: *EFFECT OF IMMUNOMODULATION FOR PERIPHERAL T-CELL LYMPHOMA IN RELAPSE AFTER ALLOGENEIC HAEMATOPOIETIC STEM CELL TRANSPLANT. A SFGM-TC STUDY ON 64 PATIENTS*

EBMT
European Society for Blood and Marrow Transplantation
40th Anniversary
Saving lives since 1974

Jon van Rood Award sponsored by Neovii Biotech to Sanja Stevanovic, lead author of the abstract entitled: *HLA CLASS II UPREGULATION DURING VIRAL INFECTION LEADS TO HLA-DP-DIRECTED GRAFT-VERSUS-HOST DISEASES AFTER CD4+ DONOR LYMPHOCYTE INFUSION*

The Best Clinical Poster Award sponsored by Nature Publishing Group to Hyeoung-Joon Kim, for his poster entitled: *CLINICAL IMPLICATION OF TET2 MUTATION WITH NORMAL KARYOTYPE ACUTE MYELOID LEUKAEMIA IN YOUNGER PATIENTS*

The Best Science Poster Award sponsored by Nature Publishing Group to Sarah Oelsner, lead author of the poster entitled: *GENETICALLY MODIFIED CYTOKINE-INDUCED KILLER (CIK) CELLS FOR TARGETED CANCER THERAPY*

Nurses Group - Distinguished Merit Award to Monica Fliedner for her long-standing dedication and strategic work emphasizing research, education and collaboration as fundamentals contributing to the successful development of the Nurses Group.

Nurses Group - Best Oral Presentation Award to Barbara Gresch for her team's presentation: *MEDICATION NON-ADHERENCE TO TAKING IMMUNOSUPPRESSANTS AFTER ALLOGENEIC HAEMATOPOIETIC STEM CELL TRANSPLANTATION IS ASSOCIATED WITH cGvHD: PROVIVIOMED - A MULTICENTER CROSS-SECTONAL STUDY*

Nurses Group - Best Poster Award to Laure Tardieu for her team's poster: *EVALUATION OF A DISCHARGE INSTRUCTION BOOKLET FOR ALLOGRAFT PATIENT*

12

JACIE - Standards and Accreditation

Since 2000, 354 transplant programmes and facilities in Europe and beyond have applied to the Joint Accreditation Committee-ISCT & EBMT (JACIE). 406 inspections (first-time and reaccreditation) have been performed. Over 245 have achieved accreditation at least once with practically all centres repeating the experience after their initial accreditation. There are over 300 registered inspectors, all volunteers drawn from the cellular therapy field.

In 2014, **52** applications (25 first-time and 27 reaccreditation) were received and **50** inspections (24 first-time and 26 reaccreditation) were performed. **46** accreditations (26 first-time and 20 reaccreditation) were awarded.

JACIE is a regulatory requirement in 6 countries – Belgium, Croatia, France, Italy, Switzerland and The Netherlands and is cited in various national and international guidelines. More information is available at www.jacie.org/about/national-regulations.

The review process to prepare the **6th edition of the Standards** continued throughout 2014. The public consultation on the first draft generated a record number of submissions with over 1,563 comments from 180 participants from 15 countries. The new edition will be released at the end of February 2015.

Seven education events were run either on the initiative of national societies and groups with JACIE support or directly by JACIE: Brussels (February); Antalya (March); Malaga (March); Paris (April); Zagreb (May); Portugal (June); Barcelona (October).

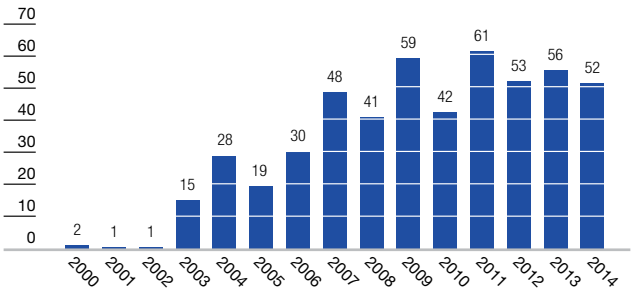
In addition, two webinars in Spanish (June and November) were organised with the LABMT (Latin American Group for Blood and Marrow Transplantation) to introduce JACIE and the Standards to transplant professionals in South America.

In 2014, the JACIE web site recorded 12,898 unique visitors. 6 newsletters were mailed during 2014 with an average open rate of 22%. In addition 16 other communications were issued concerning surveys or specific issues (e.g. inspectors expenses announcements). The JACIE Twitter account has 130 followers.

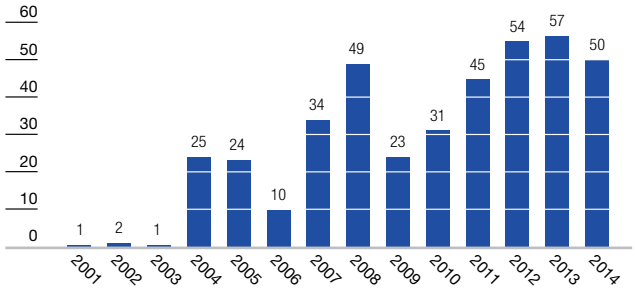
As ever, I would like to express my appreciation and admiration for the Inspectors, Committee members and other volunteers for their tremendous hard work, commitment and dedication.

Eoin McGrath
JACIE Executive Officer

Applications



Inspections



Accreditations

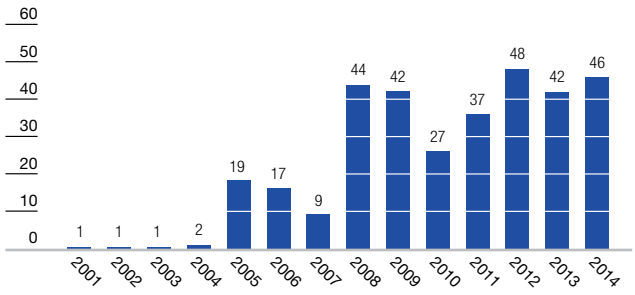
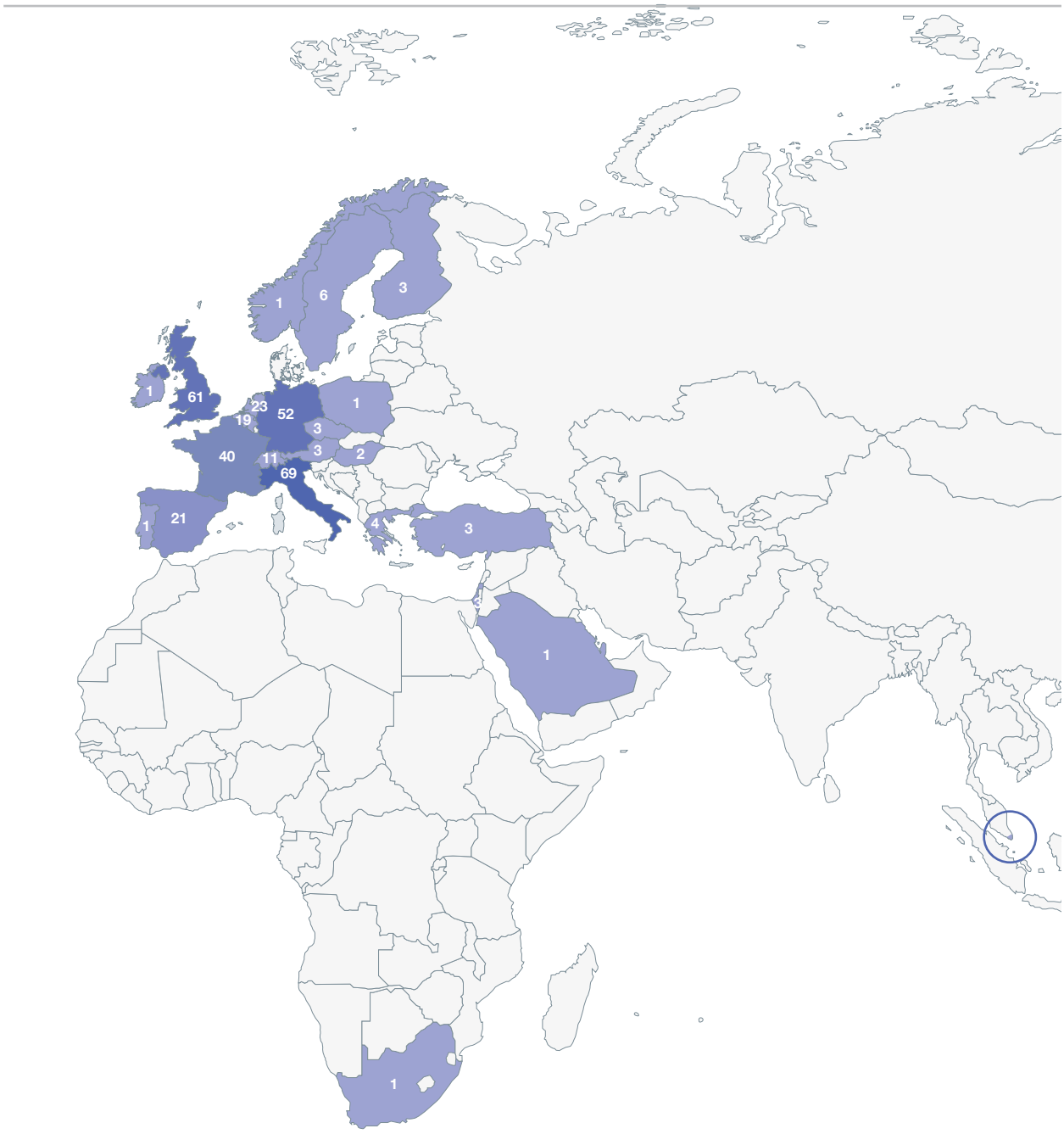
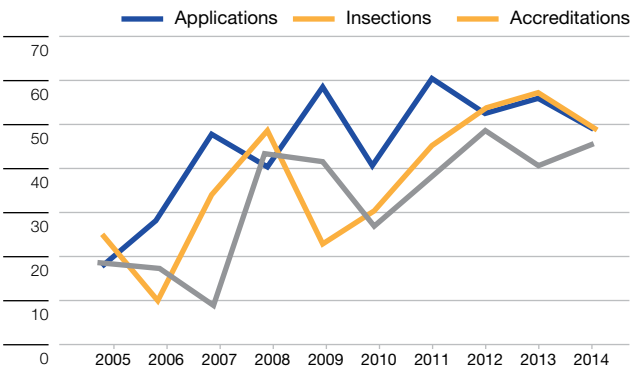


Figure 1. Number of initial applications per country since 2000



Activity



About JACIE

The Joint Accreditation Committee-ISCT (Europe) & EBMT was established in 1998 with the primary aim of promoting high quality patient care and laboratory performance in the collection, processing and administration of cellular therapy though voluntary accreditation based on standards developed by professionals working in the field. Accreditation is awarded following successful completion of a rigorous process including on-site inspection. JACIE in collaboration with the US-based Foundation for the Accreditation of Cellular Therapy (FACT) develops standards for the provision of quality medical and laboratory practice in HSCT. Accreditation in general is increasingly being used by regulators and other organisations as an independent, impartial, and transparent means of assessing the competence of healthcare providers and this also holds true for JACIE with regulators in a number of European countries including JACIE among the requirements for transplant programmes.

13

EBMT financial highlights

EBMT has improved its financial situation after 2 years of adjustments. This improvement is due to better results on the income from the Annual Meeting, better funding for the Clinical Trials and Working Party Studies and last but not least a better capacity of the organisation to control and follow up on its activities. After 3 years of governance and structural changes, we have succeeded in reducing the financial risks. We have gained financial stability and provided assurances that the money is spent and allocated according to our Mission. Today, EBMT has mechanisms of control in place that demonstrate clear lines of accountability through transparency, which has resulted in an official audit of the financial statements by third party auditors during 2014.

Regarding EBMT, the structure of decision making now in place prevents conflicts of interest and provides a clear segregation of responsibilities. However, the organisation still needs time to further consolidate the Scientific Council and the Board of Association roles.

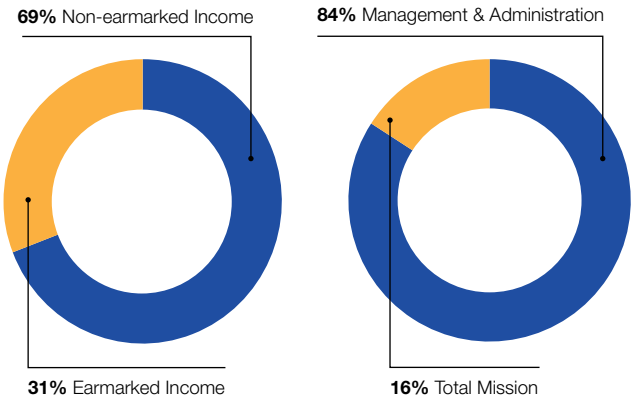
In 2014 EBMT maintains that 82% of its expenses has been dedicated and allocated to its Mission (Studies, Registry, Accreditation and Education) and the remaining 18% allocated to Management (Board and Executive Office expenses).

EBMT continues to develop and build on its strategy for diversification and retention of resources. Continuing in this direction, 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. Our 'earmarked' income comes from Pharma grants, which are allocated to specific educational studies/activities for our Clinical Trials Office and Working Parties.

EBMT will end the year with a total surplus of 141€ thanks to the success of the 2014 Annual Meeting in Milan along with an increase in study grants. EBMT will be closing the year with an expenses total of 3,282K€ and a total income of 3,477K€. In the past and for several years, EBMT was obliged to use part of the 'reserves' to balance the budget. The 2014 surplus will be returned to the reserves and in turn will now strengthen the financial position of EBMT. This financial situation therefore, allows an increase in personnel to be allocated to scientific studies, which will be overseen by the Working Parties in 2015.

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

Fred Falkenburg
EBMT Treasurer



SOURCE OF INCOME

	2013		2014	
€	in K€	%	in K€	%
Membership	565	19%	556	16%
Sponsoring	470	16%	490	14%
Annual Meeting	983	34%	1,220	35%
Others	58	2%	120	3%
Non-earmarked Income	**2,078**	**71%**	**2,386**	**69%**
Studies & CT & Education	370	13%	646	19%
Accreditation (JACIE)	165	6%	292	8%
Other Grants	293	10%	153	4%
Earmarked Income	**828**	**29%**	**1,092**	**31%**
TOTAL Income	**2,906**	**100%**	**3,477**	**100%**

HOW EBMT SPENDS THE MONEY

	2013		2014	
	in K€	%	in K€	%
Retrospectives Studies	771	25%	911	28%
Prospective Studies	383	12%	367	11%
Educational Activities	176	6%	149	5%
EBMT Registry	497	16%	506	15%
Accreditation Process (JACIE)	301	10%	363	11%
Nurses Activities	51	2%	65	2%
Committees Activities	10	0%	10	0%
Provision Reg Upgr & Personnel			365	11%
Registry Upgrade	251	8%	21	1%
Total Mission	**2,440**	**79%**	**2,758**	**84%**
Management & Administration	**630**	**21%**	**525**	**16%**
TOTAL Cost	**3,070**	**100%**	**3,282**	**100%**

Net Result

	2013	2014
	in K€	in K€
TOTAL Income	**2,906**	**3,477**
TOTAL Cost	**3,070**	**3,282**
Earmarked		**54**
TOTAL Surplus/Deficit	**-164**	**141**

BALANCE

(financial situation at the of the year)

	2013		2014	
€	in K€	%	in K€	%
Cash & equivalents	2,263	47%	3,073	50%
Other current assets	2,572	53%	3,046	50%
Total Net Assets	**4.835**	**100%**	**6,119**	**100%**
Earmarked funds	408	8%	462	8%
Non-earmarked funds	2,126	44%	2,267	37%
Provision	79	2%	417	72%
Total Reserves	**2,613**	**54%**	**3,146**	**51%**
Current Liabilities	2,222	46%	2,973	49%
Total Liabilities and Reserves	**4,835**	**100%**	**6,119**	**100%**

EBMT Staff

(at the end of the year)

	2013		2014	
	FTEs	%	FTEs	%
Study Coordinator and Statisticians for Retrospectives Studies	15,28	41%	16,10	41%
Study Coordinator and Statisticians Prospective Studies	6,14	16%	6,92	18%
Educational Activities	1,20	3%	1,20	3%
Registry	8,15	22%	7,80	20%
Accreditation Process (JACIE)	1,80	5%	2,45	6%
Communication Coordinator	0,85	2%	0,80	2%
Fundraising and Membership Staff	1,30	3%	1,30	3%
Management Staff	2,65	7%	2,90	7%
Total Staff	**37,67**		**39,47**	

Thanks to Taner Demirer, Muhit Özcan, Mutlu Arat and the team of the Ankara University Faculty of Medicine for organising the photoshoot.

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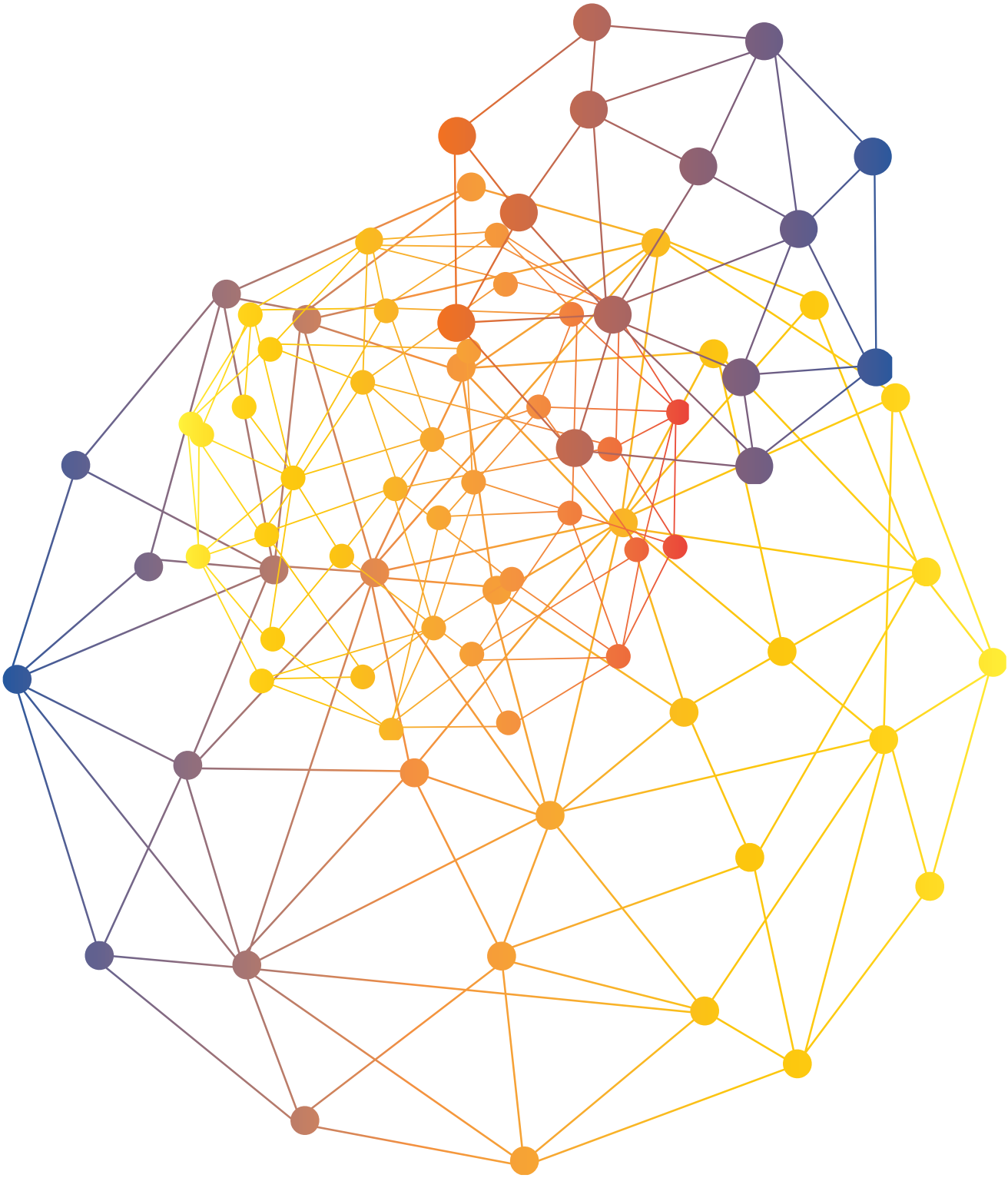

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