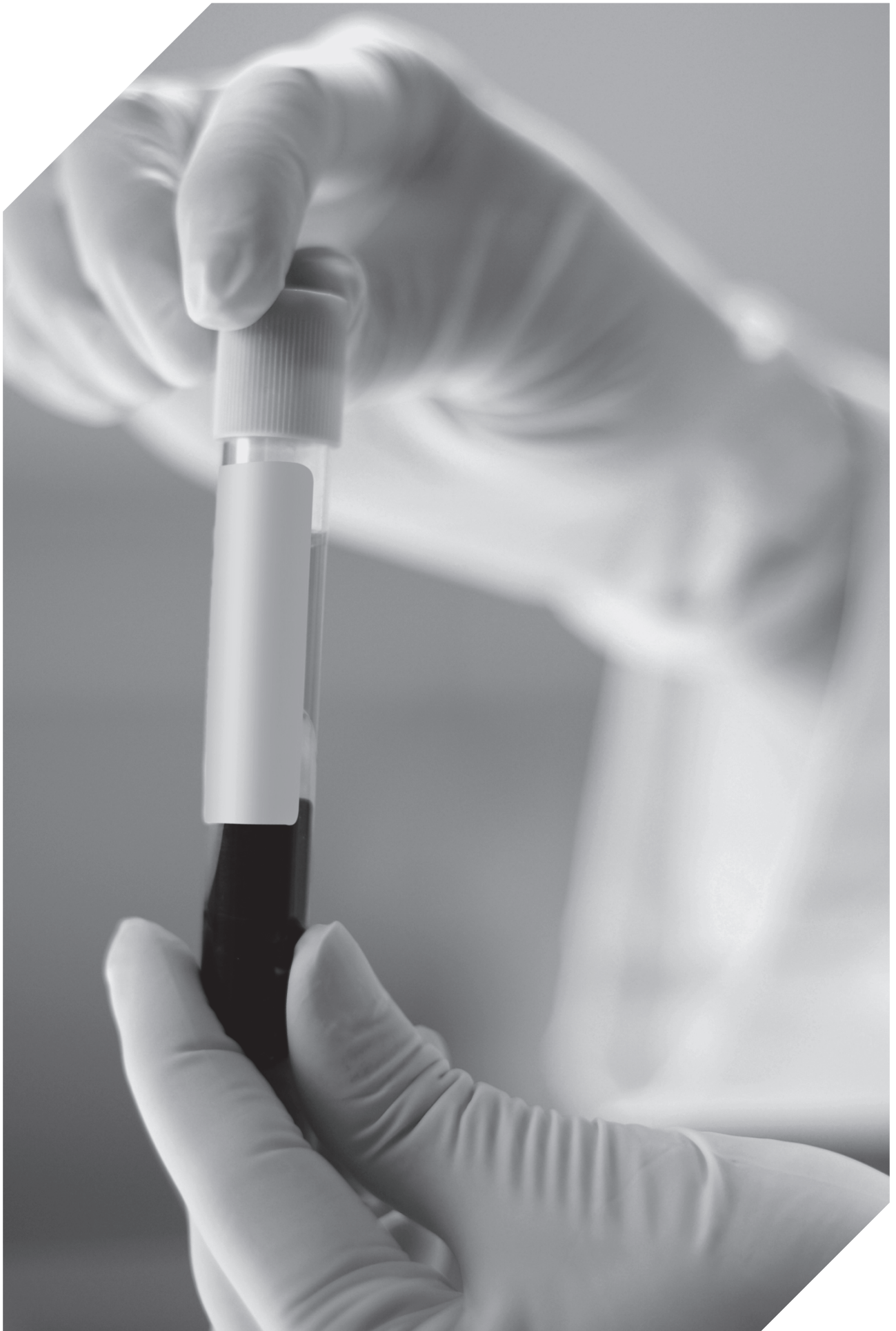


Annual Report

/12

**Annual Report**

**/12**





## Table of Contents

Introduction to the EBMT	<b>4</b>
2012 EBMT at a glance	<b>5</b>
Foreword by the EBMT President	<b>6</b>
Highlights of 2013 Strategic Plan by the EBMT Executive Director	<b>7</b>
Feature article: Global medicine Past, Present and Future Challenges	<b>8</b>
Hematopoietic Stem Cell Transplantation In Europe: Data and trends in 2011	<b>10</b>
The EBMT Registry Report	<b>12</b>
The scientific activity reports	<b>15</b>
Publications 2012 in peer-reviewed journals	<b>30</b>
Report of the clinical trials office	<b>33</b>
EBMT ED - coordinating educational activities	<b>34</b>
Standards and Accreditation - improving quality and safety in cellular therapy	<b>35</b>
Report of the statistical unit	<b>37</b>
The EBMT Nurses Group - improving patient care in haematology and HSCT	<b>38</b>
Information and communication	<b>40</b>
The Corporate Sponsors	<b>41</b>
EBMT financial highlights	<b>42</b>
Organigramme	<b>44</b>
The EBMT Board, Committee Chairs and Board of Counsellors in 2012	<b>46</b>



**The European Group 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.

The organisation is represented and led by the EBMT Board which consists of the following members: President, President-Elect, Secretary, Treasurer, Chairpersons of the Working Parties, the President of the EBMT Nurses Group and the President of the forthcoming annual EBMT meeting.

## 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**

**Increase the level of science across the organization with a view to advancing clinical practice;**

**Improve the governance structure for effective and accountable implementation of the Mission;**

**Maximize the resources to potentiate the activities of the Society.**

# 2012

## EBMT at a glance

**4,264**

members based in 570  
transplant centres in 57  
countries worldwide

**79**

retrospective  
analyses underway

**12**

educational  
events

**3,665**

participants from 77  
countries attended  
EBMT 2012

**450,110**

transplants were  
registered in the EBMT  
Registry at the end  
of 2012

**38**

publications in  
peer-reviewed  
journals

**22,225**

copies of the ESH-EBMT  
handbook distributed  
worldwide in 2012

**7**

Prospective Clinical  
Trials underway  
(in set-up, accruing  
patients or in  
follow-up) and 2 trials  
were closed to  
accrual in 2012

**35,478**

HSCTs submitted to  
the Activity Survey  
during 2012

## Foreword by the EBMT President



It is my pleasure to introduce this Annual Report and present our accomplishments for 2012.

I would like to congratulate all the EBMT Centers for the World's 1 millionth blood stem cell transplant, thanks to the collaborative work of medical scientists, physicians and nurses across the globe. This procedure has become a proven and essential therapy for many patients battling blood cancers like leukaemia and lymphoma, as well as other critical diseases.

It is also very important to thank all the EBMT Community for another successful year in terms of science. The EBMT extended 'CALM' – our largest non-interventional study – collecting data from 1,800 transplants from 53 centres by the end of 2012. Moreover, the results of the MMVAR and RATGAA07 Clinical Trials were published and following CT2 Committee selected two new Clinical Trials: EA-001 (TIGER) and Paediatric ALL to be set up.

One of the most important decisions taken by EBMT in the last 15 years was to upgrade our Registry Database. This was decided by a thorough, transparent and participative process involving centres, national registries, staff and board members, and resulted in the best technical solution being chosen out of 39 different providers. As President, I encourage our Partners to support this project not only financially, but also by making this new solution a continuation of Promise. I would like here to acknowledge the commitment of Ronald Brand and LUMC, who have been key in bringing EBMT to this exceptional situation.

The Board proposed a new governance structure for EBMT and this resulted in the creation of two platforms: the Board of Association - responsible for defining the strategic direction of the EBMT and running operations and the Scientific Council - responsible for defining the scientific and education policy. The structure of the EBMT working parties have been reorganised and all the EBMT Committees were revised and re-launched.

The Educational & Events Unit successfully managed the Geneva 2012 Annual Meeting and also supported several other important events, in addition to initiating and overseeing the PCO Tender process, the outcome being to work with MCI from Milan 2014.

The Fundraising & Communication Units have developed policies to improve their support of the EBMT Mission and Strategic Plan. Working groups have been created with EBMT staff and Board members to define objectives, activities and resources to improve communication and our capacity to generate funds. We are aiming to maximise our funding sources using different strategies, which involve working with our membership, corporate sponsorship, potentially the EU and foundations.

2012 has been a challenging and creative year for JACIE, which has automatised the inspector process for Accreditation by implementing new IT software and testing a new pilot project. This has substituted the old Sites Educational Meetings with a pre-preparation Centre visit and thus, JACIE has managed 35 inspections and currently holds 30 applications.

The EBMT greatly appreciates the support given by our Sponsors in order for us to reach our goals and help us maintain our activities, in particular the Registry Upgrade Project and the Educational Sessions.

To conclude, 2013 will see the beginning of our celebrations for the EBMT's 40th Anniversary. This is a huge milestone in our history and a unique opportunity for us to gain a greater visibility for the EBMT as a leading Society in HSCT science.

My warm regards to you all,

**Alejandro Madrigal**  
EBMT President

A handwritten signature in black ink, appearing to read 'Alejandro Madrigal', written in a cursive style.

# Highlights of 2013 Strategic Plan by the EBMT Executive Director



The last of our Strategic Plan (2011-2013) is upon us. 2012 has been a year in which we have finalised, consolidated and launched projects. There is still a lot to do but mainly 2013 will be the year to consolidate and close projects.

Science will continue to be the central axis for our efforts. We aim to:

---

1. Clarify the scientific vision (Scientific Policy)
2. Establish contacts with societies in order to involve these groups with EBMT projects (Political Mapping)
3. Set up 2 new EBMT co-sponsored funded CT and begin recruiting patients into those studies and launch new CT Application process – select new trials for 2013
4. Implement the Registry Upgrade solution and ensure the necessary funding (Migration and Training)
5. Implement a Quality System and Data to improve the quality of EBMT studies (standardization WP Study procedures and follow up)
6. Reinforce the EBMT Staff knowledge regarding diseases, the transplant procedures, including post transplant procedures like donor lymphocyte infusion and other secondary treatment
7. Launch the first EBMT-ASBMT Cell Therapy Training Course focusing on training young investigators in our respective societies to promote the translation of basic research from the laboratory to the clinic
8. Join project between EBMT and NHS

Following discussions regarding the new **Governance** framework, we aim to implement Board decision to:

---

9. Implement EBMT Governance framework (Board of the Association, Scientific Council, Committees, JACIE)
10. Implement a matrix organizational structure within our Data Offices (Coordination between Data Offices and Statistical Unit)
11. Improve transparency and accountability mechanisms - increasing control in order to decrease risk and improve efficiency - with the final aim of receiving an unqualified opinion from our auditors

In terms of **Maximizing Resources**, units will strive to achieve the following:

---

12. Balance the 2013 financial budget
13. Revision of the Membership fees Policy
14. Improve the funding of Data Office studies
15. Implement our Communication Policy to gain wider and stronger visibility and allow our stakeholders to better understand our mission, vision and strategy
16. Elaborate a Communication Plan for EBMT's 40th Anniversary (Milan in 2014)
17. Implement the Fundraising Policy in order to diversity sources, increase loyalty and increase funds
18. JACIE Office manage between 40 inspections per annum. In terms of applications, between 30-35 initial and reaccreditation requests are expected in 2013

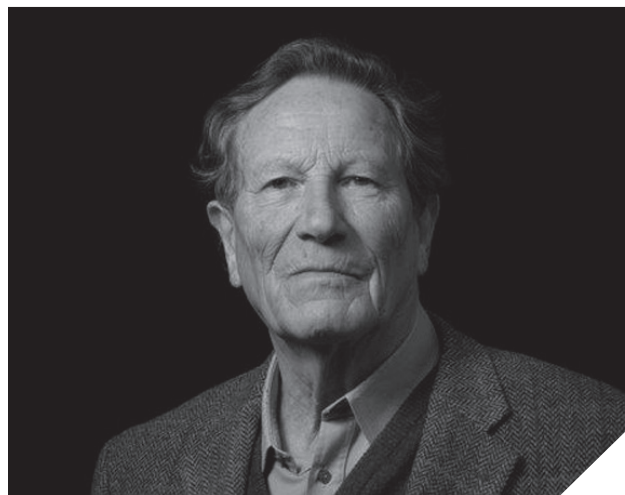
**Andreu Gusi**  
EBMT Executive Director

A stylized, handwritten signature of Andreu Gusi in black ink.



## Feature article:

# Global medicine Past, Present and Future Challenges



## Past

From the very beginning the ultimate goal of medicine has been to help all patients in need, with Hippocrates in our Western Society, as an inspiring example and every other culture having its own hero. The first major change from what was so far largely a one man's effort was the use of ether at the end of the nineteenth century, which introduced the second phase of medicine, i.e., teamwork, in this case, between anaesthetist and surgeon.

After that, modern medicine started opening the way to its third and ultimate phase: Global Medicine, in which all human beings are united in an effort to help those who are mortally ill, with blood transfusion as the most frequently used and best example. It made open-heart surgery and organ transplantation feasible.

When, at the end of the sixties, after decades of failure, three immune deficient patients were successfully transplanted with stem cells donated by HLA identical siblings, it was immediately clear that we needed to recruit large numbers of volunteers willing to donate stem cells to unrelated patients and that international cooperation was essential. Since then the disease free survival of patients with acute leukemia has substantially improved from a heart breaking 10% for patients in the early 70's to near 50% overall nowadays and for good risk cases even much better. However these early days should not be forgotten; patients suffered from the worst possible GVHD with 10 litres of diarrhoea, losing their skin, while the nurses and medical staff were faced with the hopeless task to make their suffering bearable. They all deserve our gratitude.

It took another twenty years before - on the initiative of John Goldman - informal talks were started, to come to an agreement under which conditions Hemopoietic Stem Cells (HSC) could be provided from one country to another. From that initiative originated the World Marrow Donor Association, which is now a truly global, highly active and interactive organisation, which from its beginning, kept track and reported on the number of HSC requests from unrelated stem cell donors and the number of transplants performed. A second important initiative was the start of regional organisations collecting data on patient outcome. From the mid-nineties Cord Blood HSC Transplantation (HSCT) became a reality and initiated Cord Blood banking. EBMT studies facilitated the rapid adoption of cord blood as an alternative to bone marrow or peripheral blood stem cells for transplantation.

## Present

It's important to highlight that the EBMT Registry with clinical data on more than the 30,000 transplants performed annually in Europe and neighbouring countries is a crucial tool for research in SCT. It has a direct impact on clinical practice, leading to improvement in patient outcomes.

Since 2006 a group of clinicians and other WMDA members persuaded the regional and global organisations to form together the Worldwide Network for Blood and Marrow Transplantation (WBMT) Group. They recently received the NGO status of WHO and sent out a communication proclaiming that the millionth stem cell transplant had been performed in December 2012. It is, without any doubt, a reason for a moment of reflection and respect for the patients who underwent this extreme therapy and the 500,000 donors, who made the allogeneic transplants possible. There is no doubt that in the good Hippocrates' tradition teaching by the 'experienced' has played an essential role in this improvement.

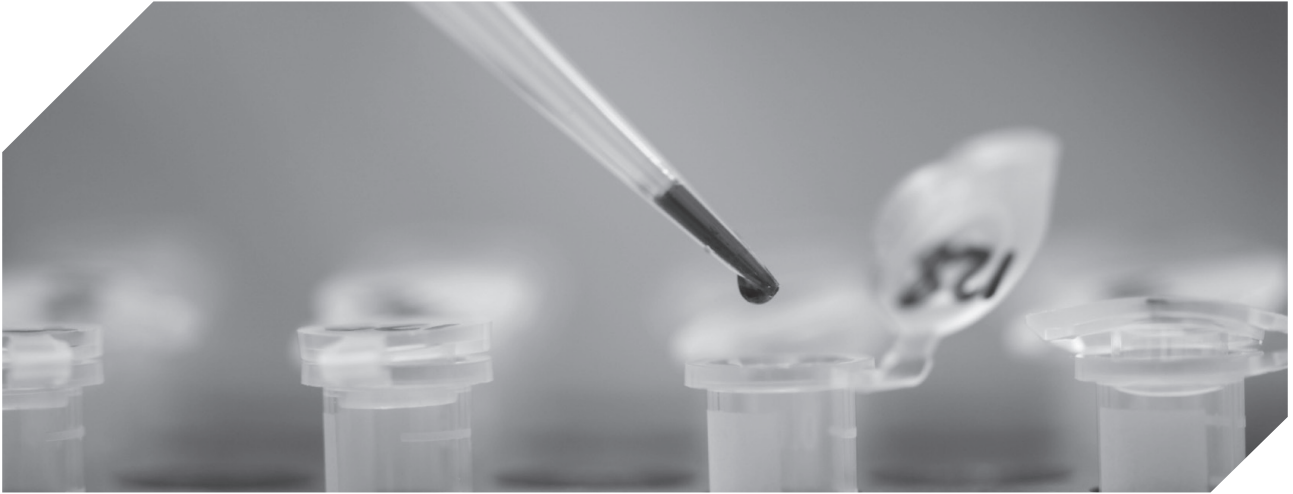
## Future Challenges

**Access to transplantation:** 50,000 patients were looking for an unrelated donor in 2011, but less than 20,000 received a transplant, while we agree that the present 20 million donors including the 500,000 Cord Blood units available can provide nearly all patients with a stem cell transplant. Our first challenge is to find out why they did not receive a transplant and identify preventable causes.

**Cost aspects:** These are not only a problem in emerging countries, but also in some of the richest. Experiments in "out-patient stem cell transplants centres" have shown that cost reduction is a feasible option.

**The multitude of different protocols:** Autologous, allogeneic identical, haploidentical, unrelated, cord blood transplants with or without a second donor. Hospitals in same language regions should form a network and refer patients to the hospital with the expertise needed.

**An adequate follow up of all transplanted patients:** with an overall survival of 50% with over 20% of the surviving patients suffering from chronic GVHD stem cell transplantation is still a developing therapy, which only is justified when the outcome is systematically analysed.



An understanding of the factors, which make donation a safe and satisfying experience for all involved. Donation is generally safe however, little is known about the impact of donation on related donors.

Stem cell transplantation as a tool to induce tolerance for organ transplants.

These are big challenges – but no bigger than those faced by the patients we treat. The EBMT plays an essential role to improve the outcome of HSCT through 40 years of investigation towards patient care. Using our tradition of collaboration for both research and clinical care, there is no question that we can meet these challenges successfully.

**Jon van Rood**

Honorary Member of the EBMT

# Hematopoietic Stem Cell Transplantation In Europe: Data and trends in 2011

The transplant activity survey data office in Basel contacted 680 centres from 48 countries (39 European and 9 affiliated countries) for the 2011 annual survey; of which 649 teams from 46 countries (37 European, 9 affiliated countries) reported their numbers. This corresponds to a 95% return rate and includes 536 active EBMT member teams. 16 active teams failed to report in 2011 whilst 15 teams reported no activity due to transplant program development or closure.

The 2010 survey had for the first time reported more than 30'000 patients transplanted in a given year. This trend continues with an additional increase by 6.3% in 2011. This report is based on 35,478 transplants and 31,904 patients (13,401 allogeneic (42%), 18,503 autologous (58%)) in 2011. Main indications were leukaemias; 10,064 (32%; 94% allogeneic); lymphoid neoplasias; Non Hodgkin Lymphoma, Hodgkin Lymphoma, Plasma cell disorders; 18,318 (57%; 12% allogeneic); solid tumours; 1,517 (5%; 5% allogeneic); and non-malignant disorders; 1,825 (6%; 92% allogeneic). There were more unrelated donors than HLA identical sibling donors (54% versus 39%); proportion of peripheral blood as stem cell source was 99% for autologous and 73% for allogeneic HSCT. Cord blood was used in allogeneic transplants (6% of total).

Figures 1 and 2 show the distribution of diagnoses of patients reported in 2011 receiving autologous or allogeneic HSCT. Figure 3 shows the continued increase in unrelated donor transplantation, but also shows that the alternative family donor transplantation (haploidentical donors) continues to increase reaching 962 in 2011.

Data on the use of reduced intensity conditioning (RIC) for allogeneic HSCT was accessed. The numbers of RIC HSCT continued to increase from 1,436 in 2000 to 5,567 in 2011 (38%). We also observe an increasing use of donor lymphocyte infusions (DLI). In 1997, 305 patients were reported as having received DLI infusions after transplant; this has increased to 2,275 in 2011 and corresponds to 16% of patients with an allogeneic HSCT.

In spite of the economic crisis in Europe there does not appear to be a decrease in transplant activity since 2009 but rather a continued annual increase in the use of HSCT technology. In the last 10 years the overall number of transplants has increased by 53%. Allogeneic HSCT have doubled (7 272 to 14, 476) whilst, autologous have increased by 32% and continued to increase by about 1,100 HSCT per year since 2001.

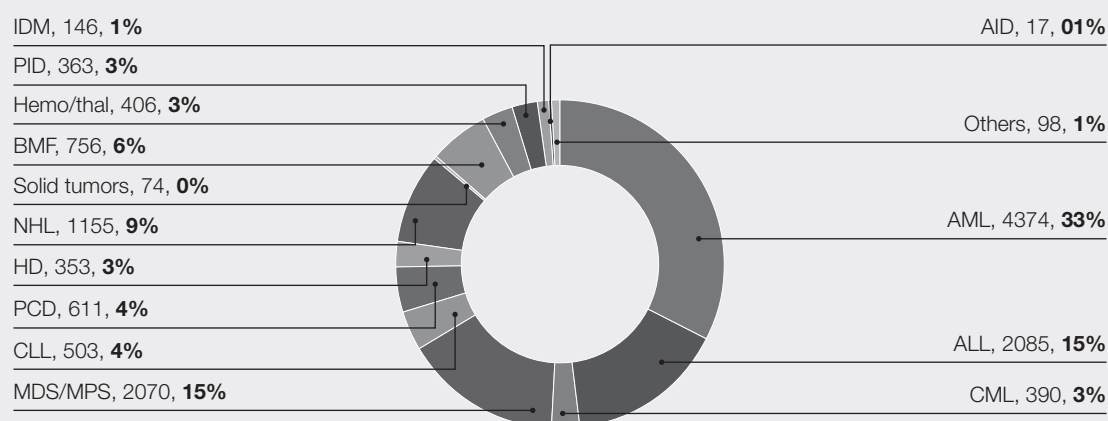
**Helen Baldomero**

EBMT Activity Survey Data Office, Basel, Switzerland

**Jakob Passweg**

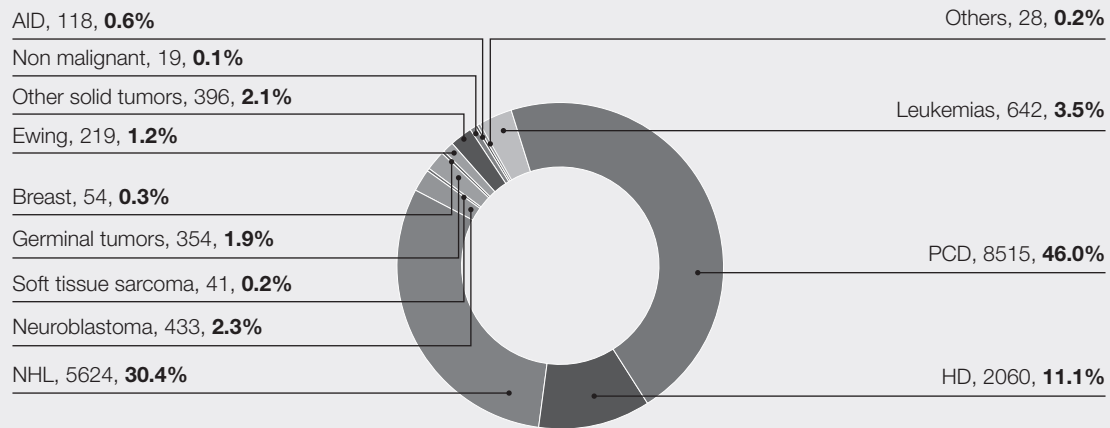
EBMT Activity Survey Data Office, Basel, Switzerland

**Figure 1:** main indications for allogeneic 1<sup>st</sup> transplants in 2011 (to be published in BMT 2013)

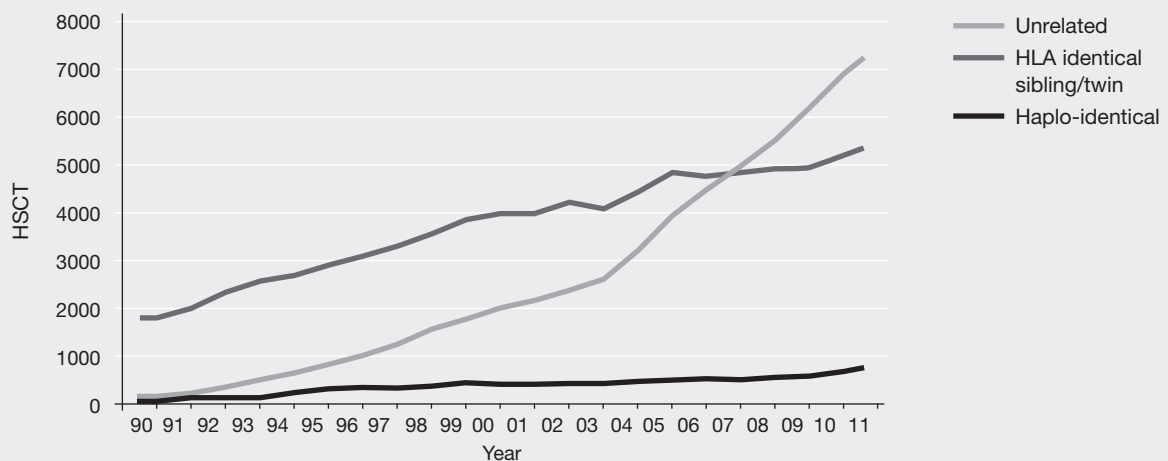




**Figure 2:** main indications for autologous 1<sup>st</sup> transplants in 2011 (to be published in BMT 2013)



**Figure 3:** shows the continued increase in the numbers of unrelated donor transplantation compared to HLA identical sibling donors and the gradual increase in alternative family donor transplantation (haploidentical donors)



# The EBMT Registry Report



## Data overview

A total of **376,796** patients and **442,841** transplants appear as registered in the EBMT Registry at the beginning of this year. Of these, over **30,000** transplants were registered during 2012, **78%** directly by the transplanting centres and the rest by a mixture of national registry and EBMT staff.

## Registry upgrade

During 2012 the Registry Upgrade project got into full swing with a series of events and tasks which culminated in the successful selection of a solution for the IT system to house the Registry in the future.

Oscar Alonso was hired to coordinate the project. He proceeded to contact providers asking for a demonstration of interest. Eleven proposals were received. These proposals were assessed by the Registry Upgrade (RU) Technical Committee, while the RU Scientific Committee assessed the financial proposals. Four providers were shortlisted and requested to do a presentation of their solution in front of an audience of EBMT and national societies staff and members. This took place at the end of August with three providers as one of the finalists withdrew at the last minute.

After months of work by representatives of all staff across the EBMT, the RU Scientific committee was able to propose a final solution which was accepted by the Board. In line with this Remedy Informatics has been asked to implement the new system for the Registry. Remedy Informatics already has presence in the BMT community and combines BMT content expertise with high-end technology. The Remedy Informatics' platform has been used to develop over 120 registries including BMT. It is intuitive and user friendly for both data entry and analysis and is footprint-free. The contract was finalised in December 2012. The delivery date is the 9th December, 2013.

## Data Sharing

### Belgian National Registry

In line with the Registry policy of facilitating data submission by transplant centres, the EBMT Registry is providing access to the Belgian National Registry to see data of all Belgian centres who have requested their data be seen by this organisation. This brings to 11 the number of national institutions seamlessly integrated with the EBMT and with which the EBMT shares the Registry system.

## AGNIS project

The objectives set for the AGNIS project regarding mapping and forwarding of selected forms were achieved. Documentation regarding business rules for the data transfer continues to be compiled.

The project underwent loss of funding with the consequent loss of 2 members of staff at Eurocord Studies. After this, Eurocord Registry proposed changes to the data flow which could not be accepted by the EBMT as it would entail reverse data flow through Eurocord Registry rather than directly from the CIBMTR, as proposed in the original project.

Due to this and because the implementation of the Registry upgraded system will modify considerably the existing work, reverse mapping was abandoned during 2012.

The focus for 2013 is to submit as much data as possible with the existing mapping while assessing the capabilities of the system selected for the Registry upgrade in the continuation of this project. Once the Registry upgrade has been finalised, the Registry will re-consider again the reverse mapping with the CIBMTR, and resume the dialogue with Eurocord Registry for future collaboration in electronic data exchange.

## New implementations

### Donor Outcome project

The Registry, in collaboration with the Donor Outcome Committee and the invaluable support of Swiss Transfusion SRC has successfully implemented the Donor outcome data collection forms. Donor registration and follow up is now open to all users. The focus of data collection is on serious adverse events during the donation procedure up to day 30 after the end of the procedure, and any adverse events during long term follow up. This is an important and integral part of allogeneic HSCT which will allow provision of data on short and long term donor safety and help to develop recommendations for donor eligibility, selection and outcome follow up based on donor health characteristics.

## Data quality

### Definitions group

The definitions group has continued its work to ensure clear and consistent definitions are available for the data managers from the centres regarding all data items present in the Med-AB forms. It has renewed its commitment to proactively





stimulate the international harmonisation of data items and is at present engaged in collaborating with the CIBMTR regarding changes to the Med-A/TED.

### **Data management**

Data management procedures that were initiated during 2010 and specifically aimed at increasing the data quality of the Registry were suspended during 2011 as the staggered replacement of four members of staff and their subsequent training made it impossible to find the time. Unfortunately, in face of financial difficulties the Registry continued to be unable to fully implement these procedures during 2012. However, the Registry did resume the very important task of approaching all centres and requesting they update the follow up of their patients. By the end of the year, a bit more than 10% of all centres approached have acknowledged or sent in the updates.

### **Registry Office move**

The Registry office was advised that due to re-organisation of the GSTT departments, they would need to move to a different location in the GSTT grounds. The move will take place in May 2013. During 2012, Registry staff have been involved in assessing the facilities on offer and liaising with GSTT planners to ensure the move is smooth and no absence of service is perceived by the EBMT members.

**Carmen Ruiz de Elvira**  
Head of Registry

**Anna Sureda**  
EBMT Secretary

# Support the EBMT Registry Upgrade Project

## The Registry today

- Holds over 400,000 transplant registrations since the early 70's
- More than 30,000 new registrations submitted in 2011
- More than 500 EBMT transplant centres in more than 50 countries

The European Group for Blood and Marrow Transplantation (EBMT)'s unique Registry is the biggest source of standardised medical data of patients receiving haematopoietic transplants in Europe available for medical research and has been a key asset in many of the medical advances in the field.

The current Registry system is reaching its limit, struggling to keep up with the improvement in technology seen during the past decade and the increased complexity of transplantation.

**Today, EBMT faces a crucial new challenge: to continue the Research in Stem Cell Transplantation, the Registry needs to be urgently upgraded.**

**If we don't upgrade the Registry, we will be unable to carry on with our mission of investigation, excellence in science and ultimately improving and saving patient's lives.**

*"Since 1974, the use of EBMT data and statistical resources have resulted in more than 560 publications in peer-reviewed scientific journals."*

*Alejandro Madrigal, EBMT President and Scientific Director of the Anthony Nolan Research Institute*

**The EBMT is a non-profit organisation  
Help us upgrade the Registry for the future of your patients  
<http://registryfunding.ebmt.org>**

**Make a  
donation**

## Your donation is absolutely key.

You can support this project either with an online donation using this secure online gateway <http://registryfunding.ebmt.org/> or by bank transfer. You can also contact the Registry upgrade team by email/phone

EBMT Executive Office - Calle Rossello 140 1º-1ª 08034 Barcelona (Spain) - Tel : (+34) 93 453 8570  
[registryupgrade@ebmt.org](mailto:registryupgrade@ebmt.org) - [www.ebmt.org](http://www.ebmt.org)

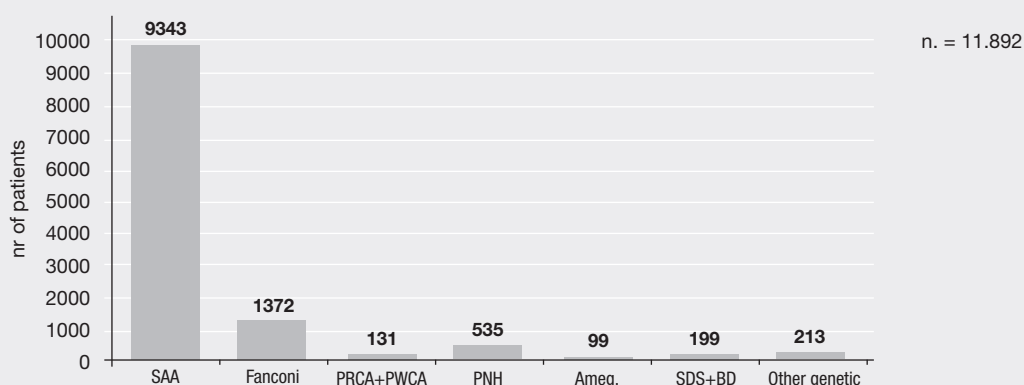
## The scientific activity reports



### Severe Aplastic Anaemia Working Party (SAAWP) – Chair: Judith Marsh

**Introduction:** The Severe Aplastic Anaemia Working Party (SAAWP) reports on AA and other rare acquired and inherited bone marrow failure (BMF) disorders (see figure 1). The AA database is the only “disease specific” database within the EBMT as data are not only collected on patients receiving transplant but also other forms of therapy, including immunosuppressive therapy (see table 1). Figure 2 shows the continued increase in BMT activity for SAA and other BMF disorders, most of which are for Fanconi anaemia. The change in practice to using BM in preference to PBSC as the stem cell source for SAA HSCT is shown in Figure 3, as we have shown better outcomes using BM. The SAAWP also actively promotes research in bone marrow failure disorders, in keeping with one of the key missions of the EBMT.

**Figure 1:** Activity as of February 2013



#### Major scientific advances in 2012:

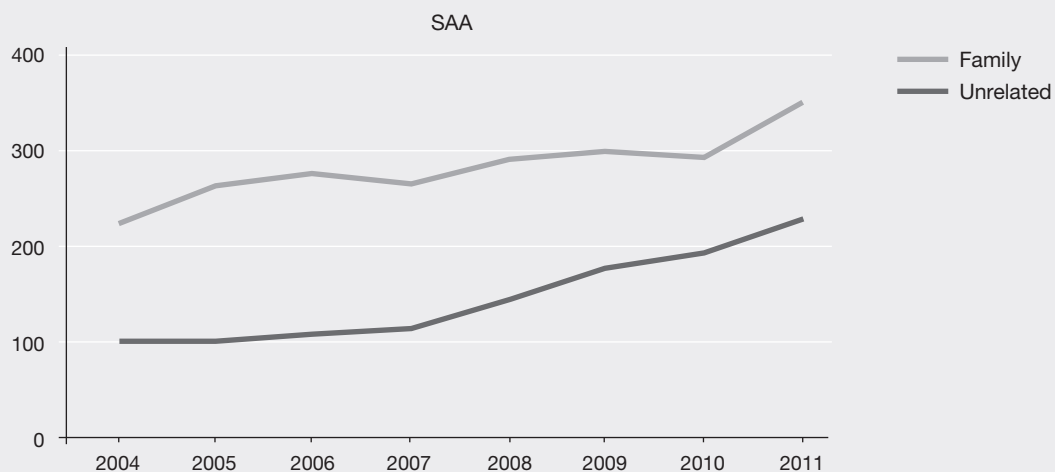
1. The complete treatment algorithm for SAA which has been a specific feature on the EBMT website, during 2012 has been summarised in the form of 12 manuscripts by the SAAWP members, and has been published accepted as a special issue of the journal Bone Marrow Transplantation in 2012 (see under Manuscripts). Thanks go to all the SAAWP contributors, but very special thanks to Mahmoud Aljurf who coordinated the BMT publication and Jakob Passweg who devised and established the complete treatment algorithm.
2. We held our second Joint Educational Meeting following on from the successful meeting of SAA, Late Effect and Autoimmune Diseases Working Parties held in November 2011 in Barcelona. In 2012, the Joint Educational Meeting was combined between Aplastic Anaemia and Complications and Quality of Life Working Parties in November 2012 in Budapest, Hungary.
3. The results of EBMT sponsored clinical trial of rabbit ATG (Thymoglobulin) in aplastic anaemia were published in Blood.

**Table 1:** Bone Marrow Failure Treatments (as of March 2012)

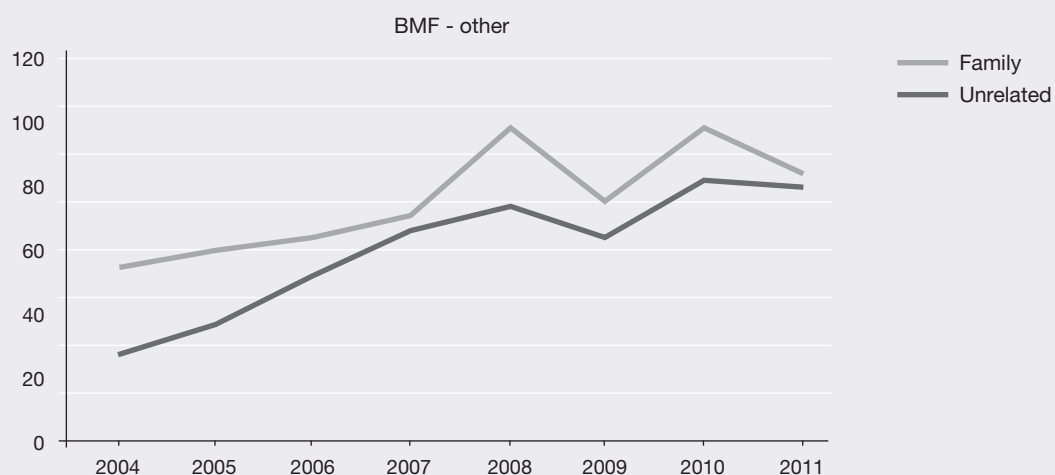
<b>Total patients</b>	<b>11,279</b>
HSCT pts	<b>8,866</b>
Total HSCT	<b>9,839</b>
IS first therapy pts	<b>3,690</b>
IS treatments	<b>4,796</b>

IS, immunosuppressive therapy, mostly ATG based treatment; total HSCT includes patients who had more than one HSCT.

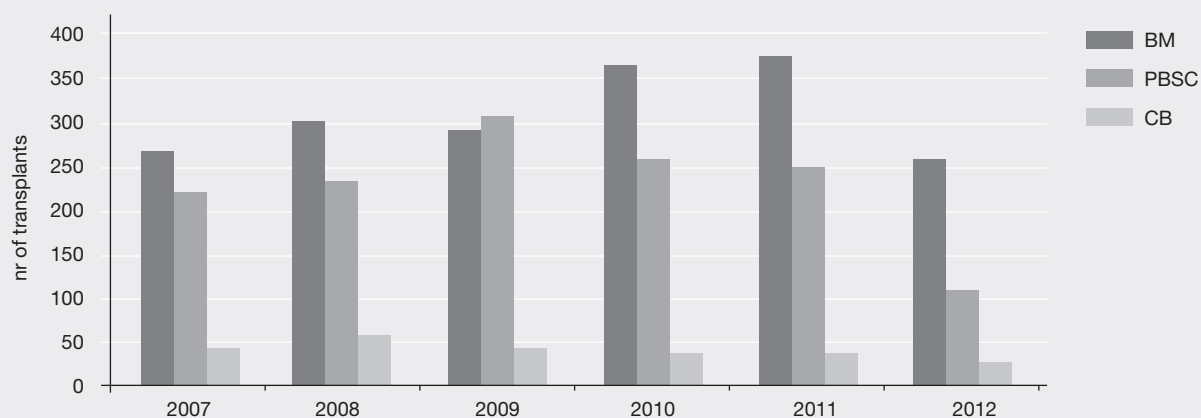
**Figure 2a:** Data from the Activity Survey of the European Group of Blood and Marrow Transplantation. Frequencies of allogeneic Transplants for severe aplastic anemia (SAA)



**Figure 2b:** Data from the Activity Survey of the European Group of Blood and Marrow Transplantation. Frequencies of allogeneic Transplants for other bone marrow failure disorders (mostly Fanconi anaemia)



**Figure 3:** Stem cell source 2006-2012





### The most relevant activities in 2012:

#### EBMT oral presentations:

1. Marsh J. Chairman's report. **Bone Marrow Transplant**. 2012; S32; 160a.
2. Dufour C et al. Outcome of aplastic anaemia in children. A survey on behalf of the SAA and PDWP of the EBMT. **Bone Marrow Transplant**. 2012; S33; 161a.
3. Stanghellini MT et al. A survey of fully haploidentical stem cell transplantation in patients with severe aplastic anaemia: a study by the Severe Aplastic Anaemia Working Party (SAAWP) and Paediatric Diseases Working Party (PDWP) of the European Blood and Marrow transplantation Group (EBMT). **Bone Marrow Transplant** 2012; S34: 163a.
4. Peffault de Latour R et al. Characteristics and outcome of severe aplastic anaemia in HIV positive patients. **Bone Marrow Transplant** 2012; S34: 164a.
5. Cugno C et al. Second haematopoietic stem cell transplantation from unrelated donors in severe aplastic anaemia. A retrospective analysis from the Aplastic Anaemia Working Party (SAA-WP). **Bone Marrow Transplant**. 2012; S215: 662a.

#### EBMT poster presentations:

1. Maury S et al. Unrelated stem cell transplantation for acquired aplastic anaemia: a donor versus no-donor analysis. **Bone Marrow Transplant** 2012; S218: 671a.

#### ASH 2012 oral presentations:

1. Carlo Dufour, Marta Pillon, Rosi Oneto, Genny Franceschetto, Andrea P Bacigalupo, Jakob R. Passweg, Gerard Socie, Antonio M. Risitano, Andre Tichelli, Regis Peffault de Latour, Alicia Rovó, Hubert Schrezenmeier, Christina Peters, Mahmoud Aljurf, and Judith C Marsh Outcome of Aplastic Anemia in Children. A Survey On Behalf of the SAA and PDWP of the EBMT Blood (ASH Annual Meeting Abstracts), Nov 2012; 120: 643.

#### Educational and Working Party Meetings:

1. Joint Education Meeting of the EBMT Aplastic Anaemia and Complications and Quality of Life Working Parties, Budapest, Hungary. 1st-3rd November 2012.  
Overall cost: EU 40,381.29; income EU 55,074.00; EBMT contribution: EU17,000.00.  
Website link: <http://www.ebmt.org/3WPs2012>
2. SAAWP Business meeting, Budapest, 2nd November 2012.
3. SAAWP Business meeting, Geneva 2nd April 2012.

**Table 2:** Number of patients registered

	Number of patients
<b>2010</b>	705
<b>2011</b>	592
<b>2012</b>	447
<b>Total</b>	<b>1744</b>



## Autoimmune Disease Working Party (ADWP) – Chair: Dominique Farge-Bancel

**Introduction:** During the year 2012, the EBMT ADWP members have further developed and enhanced clinical activities, educational meetings and research collaborative programs in the field of stem cell therapy for Autoimmune Diseases (AD) at 3 complementary levels:

1. at each country level, by identifying dedicated centers where both Hematologists and Autoimmune Disease Specialists work in tandem following EBMT standards and ADWP guidelines,
2. at the European level, by fostering interactions with the respective AD scientific societies and research groups focused on scleroderma (EULAR), lupus (Eurolupus), Inflammatory Bowel's diseases (ECCO), Multiple Sclerosis (ECTRIMS) and organizing joint educational or research sessions on cell therapy in AD during their annual meetings with presentation of the EBMT activity, guidelines and ongoing protocols.
3. at the international level, by initiating collaborative studies with the US (CIBMTR and Chicago Northwestern), Brasil and China on stem cell transplantation on Multiple Sclerosis, Diabetes and Scleroderma.

### Major scientific success(es):

1. **First results from the ASTIS** (Autologous Stem Cell Transplantation International Scleroderma) trial were successively released and presented on behalf of the EBMT/EULAR Scleroderma Study Group at the Scleroderma International Congress (February 2012, Madrid), at EULAR in (June 2012, Berlin) and ASH (December 2012, Atlanta). This pivotal open label phase III trial has enrolled 156 patients from 2001 to 2009 and randomised patients to the autologous HSCT arm or to intravenous pulse cyclophosphamide treatment. "Data indicate that haematopoietic stem cell transplantation (HSCT) results in better long term survival than conventional treatment for patients with poor prognosis early diffuse cutaneous systemic sclerosis. The ASTIS trial was a unique collaborative project of 27 multidisciplinary teams from 10 countries conducted under the auspices of two leading organizations in the respective fields, the European Group for Blood and Marrow Transplantation (EBMT; [www.ebmt.org](http://www.ebmt.org)) and the European League Against Rheumatism (EULAR; [www.eular.org](http://www.eular.org)). The primary endpoint of the trial was event-free survival, defined as survival until death or development of major organ failure. The ASTIS study shows that patients with poor prognosis early diffuse cutaneous systemic sclerosis may benefit from early intensive immunosuppressive treatment".
2. **The ASTIC trial** (Autologous Stem Cell Transplantation International Crohn's Disease Trial), a randomised controlled trial co-sponsored by EBMT and ECCO and funded by the Broad Foundation, which compares early versus late immunoablation and hemopoietic stem cell transplantation (HSCT) in Crohn's disease (CD) over 1 year, has completed recruitment of 46 patients. Patients with impaired quality of life due to active CD, despite >3 immunosuppressive agents were included and all underwent mobilisation before randomisation to immediate (1 month) or delayed (13 months) HSCT. Clinical (CDAI), endoscopic (SES-CD) quality of life and safety data are compared 1 year after mobilisation alone or after mobilisation and HSCT. Early safety results were presented at EBMT (April 2012, Genova) in 2012. The final results of the trial will allow a rational evaluation of the effectiveness and safety of HSCT to be discussed.
3. **First ADWP prospective non interventional study in Scleroderma** aiming at enrolling 50 SSc patients in 3 years approved and launched.

### The most relevant activities in 2012:

1. With over 1500 HSCT for AD reported to the EBMT registry, the ADWP data base is the largest worldwide for stem cell transplantation.
2. Joint educational meeting with the Immunobiology Working Party members, which enabled 80 participants to share knowledge and common interest on autoimmunity and stem cell transplantation and led to further development in ADWP specific biobanking project.
3. 3 major publications from ADWP retrospective studies, focused on specific autoimmune diseases subjects, illustrating our broaden research activities.

### Important objectives for 2013:

1. **Clinical activities:**
  - Development of the ADWP EBMT prospective non interventional study on HSCT for Multiple Sclerosis;
  - Enrolment of 15 SSc patients in the ADWP EBMT prospective non interventional study on HSCT for Systemic Sclerosis;
  - Update or development of Data Collection forms for SSc, SLE, MS, Crohn's and Diabetes in link with the registry update.
2. **Education:**
  - 1 combined educational meeting with the IWP and 2 business meetings
  - Update of the 2005 Recommendations for heart and lung transplant evaluation in AD patients candidates for HSCT
  - Elaboration of Good Clinical Practice Guidelines for biobanking
3. **Clinical and translational research:**
  - In agreement with statistical prerequisite and AD experts advice, achieving consensus for endpoints definition for each type of Autoimmune Diseases
  - Publication of ASTIS, ASTIC and ASTIMS first manuscripts
  - Completing 4 of the ongoing retrospective studies



## Acute Leukaemia Working Party (ALWP) – Chair: Mohamad Mohty

**Introduction:** Transplant activity for acute leukaemia continues to increase worldwide. In the ALWP registry more than 85000 transplant procedures for AML and ALL (auto and allo-HSCT) were registered thus far. The ALWP objectives are:

1. Organize high level accredited educational activities pertinent to acute leukaemia (latest symposiums: Nantes in 2008, Barcelona in 2009, Milan in 2010, Warsaw in 2011, and Milan in 2012);
2. Design and support prospective clinical trials in the field of acute leukaemia across member centres (the pan-European elderly AML randomized trial is currently recruiting patients: ClinicalTrials.gov Identifier: NCT00766779);
3. Generate high quality retrospective studies addressing different issues related to acute leukaemia management and therapy;
4. Increase within the EBMT registry the quality of data pertinent to HSCT for acute leukaemia; and
5. Generate guidelines pertinent to the management of acute leukaemia.

Over the last 12 months, the studies portfolio of the ALWP has grown quickly, and generated an amazing number of published manuscripts and communications at major meetings such as the EBMT and ASH meetings.

**Achievements and Objectives:** During the year 2012, the ALWP activities were organized and structured within the 6 sub-committees (SC) focused on specific fields of interest: autologous stem cell transplantation SC, immunotherapy SC, alternative donors SC, reduced-intensity conditioning (RIC) SC, Molecular markers SC, and the Developing centres SC.

We were able to demonstrate that Health Care Expenditure strongly influences leukaemia-free survival (LFS) in European countries. An analysis comparing results of the European and Eastern-Mediterranean countries (EMBMT) did not reveal significant differences, however, the populations and transplant procedures differed markedly. Also, we demonstrated that centre experience is the strongest predictor of outcome in the setting of RIC. The latter results might be useful for planning stem cell transplant strategies at national and regional level. The major scientific task for 2013 will be to evaluate whether the introduction of JACIE accreditation may overcome differences in outcome related to socioeconomic factors and centre experience.

The year 2012 saw also a widespread diffusion of haploidentical transplantation, mainly related to the use of high-dose post-transplantation cyclophosphamide, allowing for the infusion of T cells harvested from the bone marrow of haploidentical donors with an acceptable incidence of GvHD as compared to standard unmanipulated transplants from HLA-matched related and unrelated donors. The work of the Alternative Donors SC of the ALWP focused on refining the registry to include detailed information of unmanipulated T-cell repleted haploidentical transplantation for acute leukaemias, with aim for 2013 to perform comparative studies with transplantation from other donor sources including autologous stem cells.

In the field of immunotherapy, main achievements comprised a large study on acute lymphocytic leukaemia (ALL) relapse after allogeneic stem cell transplantation (allo-SCT), and an analysis on AML relapsing after RIC allo-SCT. Finally, a comprehensive analysis of long-term outcome (>10 years of follow-up) after second transplants for relapsed AML is being prepared for publication. Future major activities will include a comprehensive analysis of the role of 5-Azacytidine for the treatment of post transplant relapse, but also the investigation of the use of prophylactic DLI for prevention of relapse after allo-SCT, and the efficacy of sequential therapy for refractory AML.

In the field of molecular markers of AML, one of the main achievements of the ALWP was the publication of a study that analyzed the influence of FLT3-ITD mutation in patients undergoing allo-SCT for normal karyotype AML. Moreover, a study on the outcome of allo-SCT for patients with T-ALL, identifying a beneficial effect of radiotherapy, was presented as an oral communication during the ASH 2012 meeting. Another study focusing on the use of TKIs in patients undergoing allo-SCT for Ph-positive ALL since 2003 has gathered the experience of 473 Ph+ALL patients (accepted for presentation at the Plenary Session of the next EBMT meeting). The main objectives for 2013 will be the completion of the major ongoing studies: effect of TKIs on the outcome of allo-SCT for Ph-positive ALL, the role of allo-SCT in T-ALL, the influence on the combined assessment of FLT3-ITD and NPM1 mutational status on allo-SCT for normal karyotype AML, as well as another study on the role of autologous and allo-SCT for patients with acute promyelocytic leukaemia in second complete response.



The RIC subcommittee performed several studies during 2012 to dissect the role of IV Busulfan for allo-SCT in AML both in CR1 and in resistant disease. The IV Busulfan based RIC transplants were compared to the historical myeloablative TBI-based conditioning. Transplantation outcomes were similar using IV Busulfan conditioning in comparison to TBI-based transplants. GVHD incidence was higher with TBI vs. IV Busulfan while relapse rate was higher with IV Busulfan. In AML patients in relapse transplanted from unrelated donors, we observed better survival using IV Busulfan-based regimens in comparison to patients receiving TBI. In the field of GVHD, a large series showed that grade I acute and limited chronic GVHD (but not other GVHD grades) were associated with lower relapse risk after allo-SCT. An important objective for the upcoming months would be the impact of the number of consolidation courses prior to RIC allo-SCT in AML patients.

Finally, during the year 2012, around 600 autologous stem cell transplantation (ASCT) procedures (mainly for AML) were reported to the ALWP registry. The ASCT SC is currently focusing on studies aiming to determine the exact role of ASCT in the therapeutic strategy for AML and ALL, including results of long term follow-up studies. Of note, the role of post transplant maintenance is proposed for investigation because this is of great importance to decrease the incidence of relapse after ASCT (e.g. introduction of pre- and post-transplant TKIs in ASCT for ALL, and 5-Azacytidine in the setting of AML).

In addition to the above achievements, 2012 saw the birth of an important partnership between the ALWP and the team of haematology of Suzhou (University of Sochoow, China). The major objective of this partnership is the comparison of transplant outcome in China versus EBMT centres. For this purpose, 2 Chinese colleagues are already hosted in the ALWP office in Paris. They are currently working on importing and adapting the Chinese database to the EBMT database.

---

## Immunobiology Working Party (IWP) - Chair: Andrea Velardi

**Introduction:** Haploidentical transplantation: over 15 years' follow-up of protocols using high-intensity conditioning regimens and transplantation of a mega-dose of extensively T cell-depleted peripheral blood hematopoietic progenitor cells showed satisfactory outcomes in adults and children. Major issues are delayed immune reconstitution especially in adults (whose thymic output is limited) and potential lack of (T cell-mediated) GVL effect. The absence of post-transplant pharmacological immune suppression revealed donor versus recipient NK cell alloreactions which eradicated acute myeloid leukemia, favored engraftment, protected from GVHD and improved survival (Ruggeri et al., Science 2002). Furthermore, infusion of donor regulatory T cells efficiently protected against otherwise lethal doses of conventional T cells given to improve immune reconstitution (Di Ianni et al., Blood 2010). Recent years have witnessed development of diverse approaches to haploidentical transplantation: non-T cell-depleted grafts combined with new strategies to attenuate/modulate donor T cell alloreactivity and help prevent GVHD, i.e., post-transplant high-dose cyclophosphamide, post-transplant rapamycin, G-CSF-priming of donor bone marrow and intensified post-transplant immune suppression. All provide promising results that need, however, to be confirmed in longer-term follow-ups (reviewed by A Velardi in Blood 2013, "Inside Blood". Current IWP studies focus on exploiting immunology to improve haploidentical transplantation outcomes.

**Major scientific success(es):** The IWP's major scientific successes this year have been the design, approval and launch, 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 perspective study on haploidentical stem cell transplantation in adults and children with AML or ALL. In 2012 we also started planning a non-interventional perspective study on the clinical impact of pre-transplant thymic output.

The first retrospective study is investigating the role of parent/child and sibling/sibling immune interactions (IPA/NIMA vs NIPA/IMA in GvH vs HvG directions) in clinical outcomes after haploidentical transplantation (Principal Investigators: J. van Rood, A Velardi). It is designed to test whether mothers' immunity/tolerance towards paternal antigens in mother-to-child haploidentical 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, this study 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).



The second retrospective study will attempt to identify immunological biomarkers (such as T cell subset counts and pathogen-specific immune responses) that are predictive of clinical outcome after haploidentical stem cell transplantation, under diverse protocols (Principal Investigators: A. Bondanza, C. Bonini, A. Toubert, A. Velardi).

These combined studies will evaluate transplants that were performed before June 2012. Data analysis will be started after June 2013 to ensure a one-year minimum follow-up. So far, 14 Centers from Europe and elsewhere have agreed to participate in these studies and the transplant cohort comprises over 700 patients. The data analysis is expected to be completed by the end of 2013.

The non-interventional prospective study will assess the impact of donor vs recipient NK cell allo-reactivity in haploidentical hematopoietic transplantation under diverse protocols (Principal Investigators: A. Velardi, L. Ruggeri). The working hypothesis is that NK cell alloreactivity exerts GvL effects under diverse protocols (i.e., T replete grafts) as originally demonstrated in T cell-depleted haploidentical transplants.

At present 16 Centers from Europe and elsewhere have agreed to participate. The study is enrolling patients for two years (September 2012-August 2014) and the estimated total number of transplants is over 180. Data analysis will be performed at one and two years follow up.

Jointly with the PDWP and the ALWP, in 2012 the IWP designed another non-interventional perspective study to assess the impact of recipient pre-transplant thymic function on outcomes after allogeneic Hematopoietic Stem Cell Transplantation (Principal Investigator: A. Toubert). This study will test the hypothesis that pre-transplant T cell Receptor Excision Circles (TRECS) are a useful prognostic biomarker of thymic function and transplant outcome. The protocol has been drawn up and reviewed. It will soon be circulated to the EBMT Board Scientific Council and then Centers will be invited to participate.

**Most relevant activities in 2012:** The IWP organized two Educational Events in 2012.

The first was the IWP Session of the EBMT Annual Meeting (Paris). As per previous years, it featured the Jon van Rood Award Prize-giving Ceremony and the winner's presentation (best transplantation immunology paper published in the previous year).

We also held the first ever educational meeting that was jointly organized with the ADWP (Chair: Dominique Farge) in Paris. See Programme below.

**Important objectives for 2013:** As the one-year follow-up of the two combined retrospective studies (NIMA/IPA effects and immune biomarkers in haploidentical transplantation) ends in June 2013, we will perform data analyses after this date and start writing up abstracts for international meetings and papers for publication.

With regards to the non-interventional perspective study on NK cell alloreactivity in haploidentical transplantation, in 2013 we will continue enrolling patients and start performing interim analyses.

In 2013 we will launch the non-interventional perspective study on the role of pre-transplant thymic function in allogeneic hematopoietic transplantation and start enrolling patients.

We are organizing two educational events. One is the IWP session of the 2013 Annual EBMT meeting (London). In addition to outstanding invited speakers, it features two ex aequo Jon van Rood Award Winners out of 19 excellent submissions. Final programme is as follows.

Introduction of the Jon van Rood Awards. H.-J. Kolb

Jon van Rood Award: IL-7 and IL-15 instruct the generation of human memory stem T cells from naive precursors. N. Cieri

Jon van Rood Award: Endogenous HLA class II epitopes that are immunogenic in vivo show distinct behavior toward HLA-DM and its natural inhibitor HLA-DO. A.N. Kremer



Novel perforin positive regulatory DCs in immune tolerance. Y. Reisner Allogenicity & immunogenicity of regenerative stem cell therapy. D. Charron GvHD in unusual places: thymic and brain GvHD. M. van den Brink Polymorphism of NK-cell receptors that recognize HLA-C. P. Parham.

The IWP is also organizing its own two-day educational event in Perugia, in Spring or Autumn 2013, that will feature European experts and will cover topics such as, Tolerance induction, Immunopathogenesis of GvHD, Invasive fungal infections in the immune compromised host, Thymocyte development, Role of thymic function in transplantation outcomes, Immune regulatory functions of mesenchymal stem cells, Prevention and treatment of GvHD with mesenchymal stem cells, Strategies for immunotherapeutic interventions against pathogens and leukemia, Immunotherapy of leukemia with TCR transduced T cells, Innovative haploidentical transplantation protocols, such as negative depletion of CD3+ and TcR alpha/beta+ T cells, regulatory T cell-based haploidentical transplants, G-CSF primed bone marrow transplantation, bone marrow transplantation with cyclophosphamide or rapamycin post-transplant.

---

## Infectious Diseases Working Party (IDWP) – Chair: Simone Cesaro

**Introduction:** Despite the recent availability of new and more potent antiviral and antifungal drugs, the improvement of immunosuppressive strategies and the use of adoptive immunotherapy have improved the results of prophylaxis and treatment of infections after HSCT, these remain one of the major causes of morbidity and mortality both in the early and in the late post-transplant period. Therefore, there is a need for epidemiological, retrospective, and prospective studies to improve the treatment of the more frequent infections (febrile bacteremia, CMV infection, EBV infection, aspergillosis, viral respiratory infection) but also to increase the knowledge of how to deal with rarer infections (typhilitis, pneumocystosis, toxoplasmosis, zygomycosis, HHV-6, BK virus infection). Moreover, the increasing number of transplant centers requires EBMT continues to develop educational initiatives for younger colleagues or for centers that are going to start new ways of transplant (allogeneic, haplo, RIC).

**Major scientific successes:** One of the main achievement was the decision to promote the clinical research by providing IDWP with a dedicated Data Manager and Statistician time at EBMT Office in Leiden. In 2012, several studies, both retrospective and prospective, were finalized and/or published such as the response to cidofovir treatment for BK virus-associated hemorrhagic cystitis, the long-term outcome of transplanted patients with chronic HCV infection, the results of standard isolation procedure to prevent infection across EBMT centers, and the impact of donor/recipient CMV serostatus on survival after myeloablative and reduced intensity conditioning regimen. IDWP is a scientific partner of ECIL initiative (European Conference on Infection in Leukemia) from its beginning and this collaboration continued at the forth ECIL Conference, held in September 2011, that had as topics the treatment of infections caused by resistant germs, the diagnosis and treatment of respiratory infections by influenza or other viruses, the diagnosis and treatment of adenovirus infections, the diagnosis, the prophylaxis, and treatment of fungal infection in pediatric patients. All these topics were discussed among major European experts and guidelines for diagnosis, prophylaxis, and treatment have been published or are in the submission process. Moreover, all contents of the ECIL 4th Conference are available freely in slide format at EBMT website.

**The most relevant activities in 2012:** Several important studies have been carried out in 2012 and some have been closed whereas others are still ongoing, such as the impact of rituximab treatment on survival of patient with PTLD, the surveys on the filtered air rooms and management of CVC in the EBMT centers, and the impact of chromosomally integrated human herpesvirus-6 (CI HHV-6) in patients after allogeneic SCT. IDWP continues to be an official partner of ECIL initiative for the definition and publication of guidelines in different infectious disease topics and is involved in the scientific board of ECIL-5 that is planned for September 2013. Educational initiatives have been traditionally a key activity of IDWP through its annual 2-day training course on infectious disease topics in SCT. The 15<sup>th</sup> Annual Training Course of IDWP on Diagnosis and Treatment of Infectious Diseases was held in Würzburg (Germany), on 27-29 September 2012. In order to make this event more integrated with other initiatives of EBMT, this year the training course will be organized with Transplant-Related Complication and QoL WP and it will focus on GVHD and its infectious and non infectious implications.

**Important objectives for 2013:** Considering the general financial crisis over main part of Europe and the strict regulation for clinical studies that requires more resources for projection, data managing, and administrative needs, one of the main challenge for IDWP is to maintain, and possibly to increase, the scientific initiatives by launching new studies, and, at the same time, finding new financial resources to support them. We are working in this new direction for a project on bacterial infection in transplanted patients searching also the partnership with other scientific associations. There are ongoing or in preparation for 2013 2 retrospective studies and 2 non interventional studies. The intention of IDWP is to continue to promote the clinical research into several aspects of infections in HSCT patients, to stimulate the participation of younger colleagues, supporting them in their study proposals, and to increase collaboration with other working parties in order to pursue projects of common interest.





## Inborn Errors Working Party (IEWP) – Chair: Bobby Gaspar

**Introduction:** The IEWP is dedicated to the improvement of outcome of transplantation for inborn errors of metabolism including severe congenital immunodeficiencies and metabolic diseases. We aim to do this through exchange of ideas and transplant data, international collaborative retrospective studies, development of transplant guidelines and education of transplant physicians worldwide.

The IEWP has had a 2012 budget of 28,000€ - 21,000€ from the EBMT and 5,000€ from CELL-PID. We have been sponsored by Medacs, Pierre-Fabre, Orphan Europe, Therakos, bluebird Bio, and Baxter for the IEWP autumn meeting.

**Major scientific successes:** Numerous IEWP publications over the last 20 years have been essential for defining the indication for transplant in inborn errors and shaping the way transplants for these conditions are undertaken. The activities and publications of IEWP have become an essential source of information for transplant physicians dealing with these rare and difficult diseases.

The IEWP has participated in 7 retrospective studies during 2012.

**The most relevant activities in 2012:** Retrospective data on a number of conditions were published including outcome of ADA-SCID and a collaborative retrospective study with the EUCORD on the outcome of transplantation for SCID using either cord blood or haplo identical transplants. The IEWP is also PI on a major EU FP7 grant 'CELL-PID' and will help promote the educational and training opportunities of the network. The IEWP also completed and made available its "Guidelines" document for transplantation of severe immunodeficiencies and this was updated for 2012. This is now freely available on the EBMT website (<http://www.ebmt.org/guidelines2012>).

The IEWP has organised 2 educational activities and plans 2 more for 2013. Other relevant activities range from educational sessions, to abstract presentations in important international meetings such as ASH, ASCO, etc.

**Important objectives for 2013:** We will aim to produce further high impact retrospective studies (2-3 are already in the process of being published). We will also increase collaborative working with other consortia dedicated to improve transplant outcome for inborn errors, including the North American Primary Immunodeficiency Consortium.

---

## Lymphoma Working Party (LWP) – Chair: Peter Dreger

**Introduction:** The EBMT Lymphoma Working Party (LWP) takes care of scientific and educational activities related to transplantations for lymphoma, which represents the largest single entity in the EBMT with over 110,000 registered transplantations to date. The LWP runs a scientific panel consisting of the LWP chairperson (P Dreger), the LWP secretary (S Robinson) and 6 additional members being responsible for relevant subtopics, i.e. Hodgkin's lymphoma and educational affairs (A Sureda), Indolent lymphoma (S Montoto, chairperson of the indolent lymphoma subcommittee), T cell lymphoma (N Schmitz), Aggressive B cell lymphoma (H Schouten), Mantle cell lymphoma (Olivier Hermine), and Outreach affairs (Alina Tanase).

**Major scientific successes:** The major scientific successes of the LWP during recent years consist in several retrospective registry studies which had significant scientific impact in the field of lymphoma treatment. From 2009 to 2012, the LWP has published 18 studies with a mean Impact Factor of >10.

**Scientific Activities in 2012:** The most relevant scientific activities in 2012 of the LWP comprise the conduction, completion, or preparation of 30 retrospective and 3 prospective non-interventional studies (involving 20 Principal Investigators from 12 countries). The LWP is no longer aiming at running formal clinical trials. The LWP's study activities resulted in the publication of 7 scientific papers with a cumulative Impact Factor of 49.799 in 2012. The most important papers reported (1) the first prospective study on alloHCT for Hodgkin's lymphoma *Haematologica* 2012;97:310-317; (2) the to date largest study on allogeneic and

autologous HCT in Richter's transformation (together with the CMWP; JCO 2012;30:2211-2217); and (3) the to date largest study on allogeneic HCT in blastic plasmacytoid dendritic cell neoplasia (together with the ALWP; Blood epub 30.11.2012). Another major achievement was the finding that in the rituximab era TBI does appear to be with an increased NRM compared to BEAM provides better disease control when used as high-doses regimen prior to autologous HCT for follicular lymphoma. This study was presented during the Plenary session of the Annual EBMT meeting in Geneva by Inas El-Najjar from London, UK.

Moreover, the **8<sup>th</sup> Annual LWP Educational Course** held in Bucharest, Romania, was a major success and continued the tradition of the LWP to be a prime supplier of state-of-the-art knowledge about lymphoma transplantation to young investigators and clinicians.

Finally, thanks to the initiative and support by Professor Norbert-Claude Gorin we were happy to move to perfectly renovated new facilities for the EBMT office in Paris in June 2012.

For the first time we were able to present the the **Jian-Jian Luan Award** for Lymphoma Transplant research during the LWP session at the Geneva 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 A Elmaagacli from Essen, Germany, for his research on the impact of CMV exposition on disease control after alloHCT for lymphoma.

**Important objectives for 2013:** In Jian-Jian's spirit, however, we hope that we can achieve most of the planned **important objectives for 2013**, i.e. continue the numerous studies mentioned, attract and launch important new ones, publish most of the 10 scientific manuscripts planned for release in 2013, and perform the 9th LWP Educational Course (October 8-9, Barcelona, Local Organizer A Sureda) even more successfully than before.

---

## Paediatric Diseases Working Party (PDWP) – Chair: Christina Peters

**Introduction:** The PDWP is dedicated to:

- Support research and education to improve the availability, safety, and efficacy of hematopoietic stem cell transplantation and other cellular therapeutics for children and adolescents.
- Engage and promote active co-operation with all EBMT working parties treating children and adolescents, particularly with the Inborn Errors WP, Aplastic Anaemia, Infectious Diseases, Late Effects and Immunobiology, in an effort to meet the total needs of the full spectrum of paediatric HSCT patients.
- Initiate and perform prospective, collaborative, GCP-conform studies for malignant and non malignant paediatric diseases. All clinical trials adhere to the ethical considerations for clinical trials on medicinal products conducted with the paediatric population website and is a prerequisite for any clinical trial under the umbrella of EBMT.
- Develop further the close collaboration with experts from the Leukaemia/Chemotherapy national frontline studies in order to define the optimal timing of HSCT and incorporate transplant recommendations into the disease specific protocols.
- Implement the new regulations on paediatric medicines from the European Medicines Agency (EMA), which aim to ensure that drugs used to treat children are properly tested and the availability is expanded.
- Offer physicians and nursing staff from small or new centres practical training and fellowships in experienced transplantation units through a European Collaborative Paediatric HSCT network.
- Further develop established Paediatric Standards within the Accreditation Process through JACIE to guarantee and maintain a high quality of patient care and experience.

**Scientific Successes:** On request of the European Medicines Agency (EMA) a meta-analysis on the use of treosulfan in children was performed. Patients below 18 years with malignant or non malignant diseases who underwent HSCT between January 2005 and July 2010 registered in the EBMT database were eligible. To investigate a potential non-linear association between dose and outcome, fractional polynomials were used. 843 patients met the inclusion criteria and 75% could be included into the analysis. The whole data collection, follow up, evaluation and the statistical report was finished and was presented during the Annual EBMT meeting in Geneva 2012 and at the IBMTR Tandem meeting in February 2013. Two manuscripts are in preparation.

### Main Activities in 2012:

- In June 2012 the PD WP course was held in Prague. International speakers reported on the most important topics on hematopoietic stem cell transplantation in children and adolescents. Furthermore, selected abstracts from physicians and nurses highlighted the developments in this field. The course was addressed to physicians and paediatric nurses. Participants presenting selected abstracts were awarded with grants covering registration and housing.
- To update and harmonize treatment standards for children and adolescents with congenital bone marrow failures, the PD WP organised an expert workshop in Vienna in October 2012. A consensus manuscript on indication, timing and measures for stem cell transplantation was elaborated and is prepared for publication.
- The collaboration with the European Medicines Agency (EMA) and the implementation of a network for paediatric research at EMA (EnprEMA) was continued. PDWP is 1st category member of this network.
- Three business meetings were held in 2012 to discuss projects, strategies and work plans for the next working period. The most important project developments were: the reorganisation of the PDWP study process, the full integration of the new data manager and the preparational works the PDWP Educational Course 2013 in Bucharest and for the new prospective EBMT study on HSCT in paediatric and adolescent ALL.
- Strategy meeting of the International SCT consortium (BBMT, COG, EBMT, IBMTR and others): In September 2012 the first get together was held in Frankfurt. An expert group of leading physicians from the United States and Europe elaborated possible collaborations on emerging paediatric topics (e.g. minimal residual disease definition; definition of outcome measures, new approaches for non malignant diseases)



### Important objectives for 2013:

- Initiate the ALL SCTped study: prospective, randomized, open multicenter multinational study for allogeneic HSCT in children and adolescents with ALL.
- PDWP Educational Course in Bukarest in cooperation with the Paediatric Nurses Group.
- Expert Workshop for Extracorporeal Phototherapy in children and adolescents: January 2013, Vienna
- Strategy meeting of the International SCT consortium (BBMT, COG, EBMT, IBMTR and others): February 2013, Salt Lake City

---

## Solid Tumours Working Party (STWP) – Chair: Marco Bregni

**Introduction:** The science and the practice of oncology have undergone profound changes in the last few years. The understanding of the molecular mechanisms of cancer has produced overwhelming numbers of new drugs that interfere with major pathways of cancer pathogenesis. The introduction of mechanism-based targeted therapies to treat human cancer is one of the most successful and fruitful endeavours in clinical oncology. Hanahan and Weinberg have identified ten major hallmarks of cancer (1), involved in the pathogenesis of some, or perhaps all cancers, that can be targeted by specific drugs: many of these drugs are still investigational, some others are in clinical trials, many are already approved for therapeutic use.

The improved knowledge of the biology of cancer not only has introduced new drugs in the therapeutic armamentarium, but also has better defined drug sensitivity of tumors: e.g., HER2-positive breast cancer is not considered susceptible anymore to alkylating agents that are used in high-dose chemotherapy. As a consequence, the indications for stem cell transplantation, either autologous or allogeneic, have been drastically reduced, and limited to few solid tumors.

**Major scientific success(es):** In 2012 the STWP scientific activity has focused on retrospective studies and on organizing the TIGER prospective study (see below). This year, new and exciting proposals have been discussed and approved, mainly in the area of germ cell tumors (GCT). The numbers of autologous transplants for GCT in the Registry are more than 400 per year, with an increasing trend. Germ cell tumors (GCT) represent the most common malignancy affecting adolescent and young adult men in both Europe and the United States. Early stage disease, which affects the majority of GCT patients, is nearly universally curable with local or short-course systemic therapy, such that current efforts are focused on defining the least toxic means to achieve cure rather than improving efficacy. In contrast, up to 30 percent of patients with advanced GCT will not be cured with initial chemotherapy with or without surgery and therefore require salvage treatment. Furthermore, up to 20 percent of advanced GCT patients will ultimately die from disease progression. At present, the two major salvage approaches are HDCT with autologous stem cell transplant (ASCT) or conventional-dose chemotherapy (CDCT) incorporating cisplatin, ifosfamide, and either vinblastine (Velp) or paclitaxel (TIP). Due to a lack of conclusive randomized trials, it remains unclear whether sequential HDCT or CDCT represents the optimal initial salvage approach. Defining standards and optimizing outcomes of salvage treatment thus represents one of the most pressing issues in GCT treatment at present. In addition to the prospective study, in several retrospective studies the STWP will analyze: a. the effect of autografting in rare forms of GCT (e.g., adolescents, females); b. the optimal timing for mobilization of peripheral blood stem cells in GCT patients; c. the effects of allografting in refractory GCT. The activity of the Transplant Centers in 2012 has focused, even if at a lesser extent, on other tumors such as Ewing sarcoma and breast cancer. Ewing sarcoma family tumors (EFT) are rare small round cell tumors arising either in bone or soft tissues. Usually, they occur in children, adolescents and young adults, and in these age groups, they are the second most common bone cancer. Chemotherapy treatment of patients with EFT has been based for decades on a four-drug combination of vincristine, doxorubicin, cyclophosphamide and actinomycin D (VACAc regimen), however relapse occurs in >30% of patients. In slow-responder patients, intensification with high-dose chemotherapy has reported increased event-free survival compared to historical series (2).



High-dose chemotherapy (HDCT) in high-risk primary breast cancer has been analyzed in a joint MD Anderson/EBMT meta-analysis published in 2011: results show that disease-free survival, but not overall survival, is increased by HDCT compared to conventional-dose chemotherapy. Now these results have been confirmed by a new metaanalysis (3). Patients with triple-negative tumors appear to benefit most from HDCT, but confirmation would need a prospective randomized trial.

Other retrospective study proposals in cooperation with ASBMT (Transplant trends in solid tumors in US and Europe in the last 20 years) and with MD Anderson Cancer Center on rare forms of breast cancer (i.e., inflammatory carcinoma) are underway.

**The most relevant activities in 2012:** In 2012 the STWP has focused on the set up and organization of an international, randomized, prospective Phase III trial of high-dose chemotherapy and autologous stem cell transplant vs conventional dose chemotherapy in the salvage treatment of relapsed/resistant germ cell tumors (TIGER trial, or EA-001 trial). This project, that involves the Alliance (formerly CALGB) in US and different scientific organizations as the sponsors in Europe, will have EBMT as scientific coordination. The study aims to definitively settle the question on whether autologous transplant is the best therapy for relapsed GCT, by comparing 2-yr OS after conventional-dose chemotherapy (TIP) or with high-dose chemotherapy and autologous transplant according to T1-CE protocol. Overall survival will be the primary endpoint. Approximately 400 patients will be required. Superiority for HDCT is the primary endpoint. Stratifications factors include the The Lorch Score (IGCCCGr), Randomisation will occur in both the Alliance and EBMT. The data from the 2 Sponsors will be combined on a 6 monthly basis.

This will be the only prospective study of STWP. In this study, EBMT Clinical Trial Office will provide data management according to GCP practice. Already 4 countries (UK, Italy, Germany and France) have joined the study, and others (Ireland, Denmark, Sweden) are willing to join. The study will start in the second half of 2013 in Europe.

**Important objectives for 2013:** The most important objective for 2013 for the STWP will be to start the TIGER prospective study. In the medium run, the STWP interests will have to include also immunotherapy and adoptive cell therapy studies, that are of increasing importance in solid tumors.

---

## Chronic Malignancies Working Party (CMWP) – Chair: Nicolaus Kröger

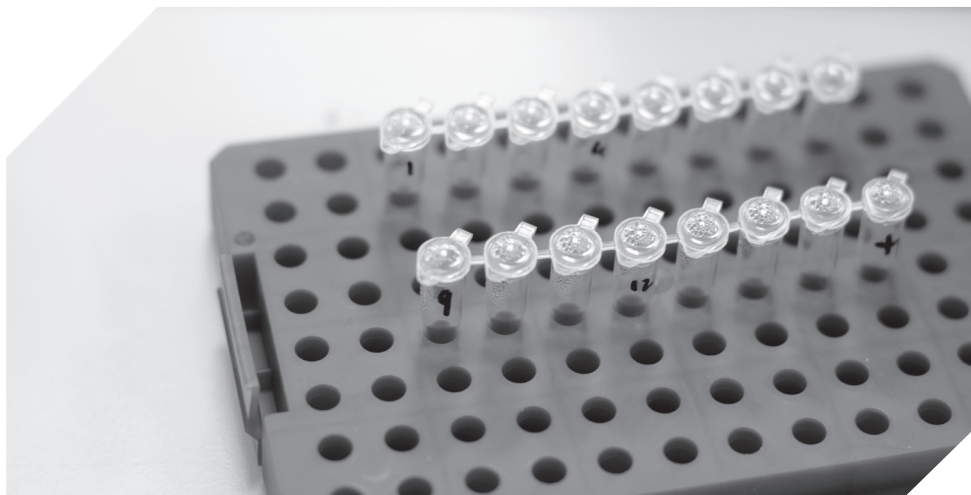
**Introduction:** In April 2012 a new chair for the renamed Chronic Malignancies Working Party (former: Chronic Leukemia Working Party) was elected (Nicolaus Kröger Hamburg).

The CMWP promotes and conduct educational activities and clinical investigations on hematopoietic stem cell transplantation in chronic haematological malignancies.

The mission of the CMWP is to contribute significantly to an improved outcome of stem cell transplantation in chronic hematological malignancies by

1. performing high quality retrospective registry studies
2. performing prospective clinical trials and non-interventional studies (NIS)
3. improving quality of data in collaboration with other WP and the registry committee
4. promoting advanced training and scientific interaction by performing "Educational Courses" and "Scientific Meetings"
5. collaboration with national and international Transplant – and Non-transplant (disease-specific) Groups
6. disseminating knowledge by up-to date information to the transplant and non-transplant scientific community, patient organization and lay public





**Activities, Achievements and Objectives:** During 2012 two prospective clinical trials, 4 non-interventional observational studies and 31 retrospective registry studies were active. Due to limitation of personal resources within the CMWP we improved in 2012 the process from designing to publishing studies by developing and implementing a working flow (SOP) for prospective and retrospective studies as well as for non-interventional trials. The Chronic Malignancies Working Party started an initiative to improve data quality in collaboration with the statistical unit of the EBMT.

In 2012 the CMWP published 15 manuscripts in peer-reviewed journals such as Journal of Clinical Oncology, Blood, Leukemia, British Journal of Hematology and Bone Marrow Transplantation.

Numerous abstracts from CMWP members were accepted as oral or poster presentation at EBMT annual meeting in Geneva as well as at ASH in Atlanta, including invitations to "Meet the Professor" Session.

In 2012, three business meetings were held in Hamburg (January); in Geneva (April at EBMT) and in Paris (September). At the meetings in Hamburg and Paris scientific educational activities were included focussing 2012 on lectures given by leading experts about biological and treatment aspects in multiple myeloma. The CMWP has been restructured into 4 subcommittees: 1. MDS, 2. Plasmacell Disorders, 3. Chronic Lymphocytic Leukemia and 4. Myeloproliferative Disorders

**MDS:** The numbers of allogeneic stem cell transplantation for Myelodysplastic Syndromes are steadily increasing and reached now more than 1.500/anno. The important prospective randomized trial comparing dose-reduced vs. standard myeloablative conditioning (NCT 00682396) has been closed in December 2012 and preliminary results will be presented at the annual EBMT Meeting in London. The non-interventional observation study about impact of iron overload prior to stem cell transplantation in MDS has nearly reached the planned accrual of 200 patients.

Major efforts have been made to analyse the impact of cytogenetic on outcome after transplantation and in 2013 the impact of IPSS-revised, the impact of monosomal karyotype as well as the new cytogenetic risk score will be analysed. More focus will be given in 2013 to molecular abnormalities detected by next generation sequencing. Analysis using sophisticated methods such as multi-state models have been done in 2012 to compare non-transplant with transplant approach in MDS and in 2012 a new prospective EBMT-labeled study has been started to compare in elderly MDS patients 5-Azacytidine and allogeneic stem cell transplantation.

**Plasmacell-Disorders:** The plasmacell disorder subcommittee finalized a retrospective study on autologous stem cell transplantation for POEMS syndrome and the effect on early RIC allogeneic transplantation for multiple myeloma. The prospective study for relapsed patients (NMVAR) was published in JCO and further subanalyses are planned for 2013. A long-term follow-up of the prospective auto-allo study (NMAM 2000) confirmed the benefit of auto-allo vs. auto SCT and the manuscript is submitted for publication. An observational allogeneic study for high risk young myeloma patients is planned for 2013 as well as an EBMT consensus on autologous stem cell transplantation for multiple myeloma. A new prospective study for autologous SCT with Bendamustine/Melphalan combination is planned for 2013.

**Chronic Lymphocytic Leukemia:** In the subcommittee CLL a data quality initiative was started and received a positive feedback from the centers. Now more detailed analyses on allogeneic SCT in CLL can be performed. The non-interventional allo SCT study for patients with del17 has included the planned 50 patients in 2012 and an prolongation up to 100 patients is planned for 2013. Important data on quality of life from the prospective autologous transplantation study in CLL have been generated and submitted for publication.

**Myeloproliferative Disorders:** The non-interventional observation study on second generation TKI before allogeneic SCT in CML has recruited more than 270 patients by the end of 2012 and the target of 390 should be reached in 2013. Retrospective analysis for CML with good EBMT risk score have shown an excellent survival of about 90% after allogeneic stem cell transplantation. Myelofibrosis has become an increasing transplant indication and more than 400 patients have received in 2011 an allogeneic stem cell transplantation. The only prospective trial in allogeneic SCT so far for myelofibrosis from the CMWP has shown excellent long-term results with good quality of life. New observational as well as prospective trial including JAK inhibitors one of the major objectives for MPN in 2013. Furthermore, in collaboration with European Leukemia Net (ELN) a consensus conference





and publication for allogeneic stem cell transplantation in PMF will be performed in 2013. Finally, a 1,5 day educational event will take place on 25<sup>th</sup> and 26<sup>th</sup> of May 2013 in Hamburg titled: *"How to manage and integrate allogeneic stem cell transplantation in the treatment of myeloproliferative neoplasms"* and participation from EBMT centers is highly anticipated.

**Complications:** The former subcommittee "complications" has moved into the "Transplant-related Complications and Quality of Life after SCT" Working Party. However, important studies on DSMO toxicity, stem cell transplantation in organ transplant recipients, secondary allogeneic transplantations and mega-file study on GvHD and transplant outcome have been finalized in 2012 and will be submitted in 2013.

Finally, in an ELN/EBMT collaborative effort a consensus recommendation for GvHD prophylaxis and treatment has been achieved and the manuscript is submitted for publication.

---

## Complications and Quality of life Working Party (CQWP) – Chair: Rafael F. Duarte

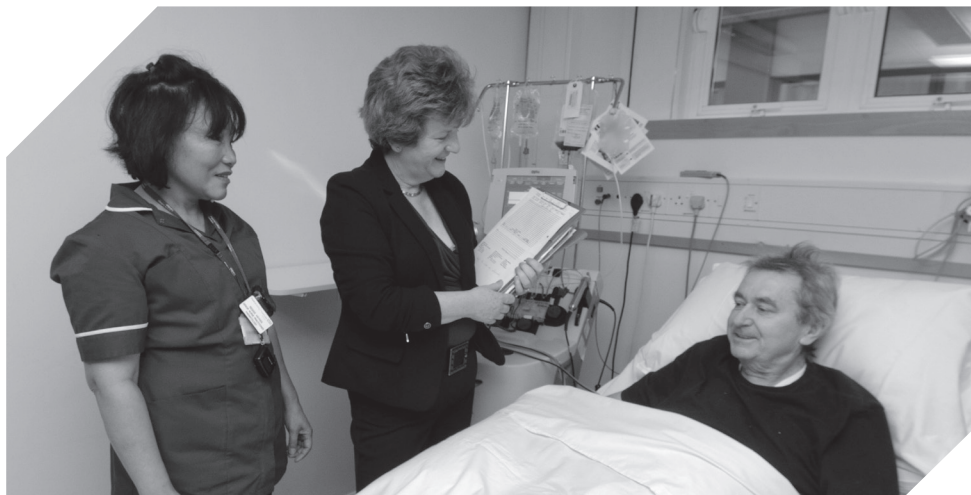
**Introduction:** In this year since Geneva 2012, the former Late Effects WP became a new Complications and Quality of Life WP (CQWP). This new CQWP not only ensures continuity of the WP's strong record in long-term complications, but it also extends its scope to incorporate early non-infective complications, and for the first time in EBMT, we centralize in one WP the work of the Society on GVHD, the commonest and most important complication of allogeneic transplantation. Our main goal at the CQWP is to combine expertise across transplant complications and to provide us all with a strong WP focused on transversal research in this field in collaboration with other WPs within EBMT, and external collaborations with international groups.

As I briefly summarize in this report, in less than a year, the new CQWP has made successful developments to making significant progress in all these areas. This has only been made possible thanks to the commitment and collaboration of our WP Secretary and Subcommittee Leaders, Grzegorz Basak, Hildegard Greinix, Alicia Rovó and Tapani Ruutu, and the support and vision from the EBMT ExCom and my fellow WP-Chairpersons.

**The most relevant activities of the CQWP in 2012:** I would like to start with one of the highlights of 2012, the publication of the recommendations for screening and preventive practices in long-term survivors after HCT, which are the result of very broad collaboration of EBMT with several other international HCT societies, and have a critical relevance for clinical practice of the transplant community worldwide.

From the perspective of education and training, I would like to mention the success of our joint educational meeting of the Severe Aplastic Anaemia WP and CQWP, held in Budapest in November. The course was financially self-sufficient, and already included specific topics on early complications and GVHD in keeping with the new scope of our WP. In addition to the EBMT ED unit and my co-chairs Judith Marsh and André Tichelli, I would like to stress the important contribution of Tamás Masszi, EBMT Education Committee Chair and Local Organizer, whose enthusiastic hard work was key to the success of the course.

Among a number of new developments in the structure and functioning of the CQWP, I would like to highlight the establishment of a new collaboration with the NIH – National Cancer Institute (NCI). This joint EBMT-NCI Task Force was one of the projects included in my candidate manifesto last year, has a primary focus on GVHD and survivorship issues after transplant, and brings together the EBMT President, Secretary and several key members of the CQWP and the GVHD subcommittee with a broad and trans-disciplinary group of U.S. colleagues from the NCI transplant program and chronic GVHD study group, the U.S. chronic GVHD consortium, and the NCI Division of Cancer Control and Population Sciences. After several months of productive discussions and planning, we held a first business meeting of the task force in Bethesda (MD) after ASH in December, and since then, a formal first research collaboration is underway, starting with a survey on clinical practice on chronic GVHD and NIH-criteria to be carried out in parallel in March 2013 in all EBMT allogeneic transplant centers, as well as in America through the CIBMTR.



The results of the survey will be presented at the CQWP Session at the EBMT 2013 annual meeting in London. The survey will provide an important snapshot of current practice in chronic GVHD worldwide, and will help to develop research programs for dissemination and implementation of chronic GVHD management criteria into clinical practice. Such dissemination and implementation strategies and research are particularly important from the perspective of the CQWP and a Society such as EBMT, with fellow members from multiple countries and socio-economic backgrounds. As a Society, we must not only excel in science, as per our EBMT Mission, we must also make sure that such excellence does not stay on the bookshelves, that it fully transfers and gets implemented into clinical benefit for patients. Among so many colleagues that are making this important international collaboration come true in such a short time-frame, I would like to express particular gratitude to Alejandro Madrigal and Steve Pavletic, with whom this potential collaboration was discussed from the start, to Dr. Ted Trimble, for embracing this idea and providing scientific and logistic support from the NCI – Center for Global Health that he directs, and to Hildegard Greinix and Stephanie Lee, for generously putting their top class expertise to the common service of this collaboration.

Finally, a good number of studies have come to the end of their cycle and will be published during this year. This is also a requirement for the CQWP to optimize an efficient use of our data management and statistical resources for the increasing number of new studies in the extended scope of the WP. I would encourage you all to attend the WP Session and learn more about some of these important studies, such as Tapani Ruutu's study on the EBMT – ELN recommendations for a standardized practice on prophylaxis and treatment of GVHD, Grzegorz Basak's retrospective study on haematopoietic stem cell transplantation in recipients of solid-organ transplants, Nicole Engel's study on donor type secondary leukaemia, or Phillip Schwarze's study on current practice in growth hormone treatment in children and adolescents after HCT.

**Important objectives for 2013:** There are many exciting challenges ahead for 2013. From an educational perspective, we are very happy to see that our first training workshop on assessment of chronic GVHD according to the NIH criteria in daily practice, planned by Hildegard Greinix as leader of the GVHD Subcommittee, and to be held at the EBMT 2013 congress in London, has been fully booked to the maximum of 150 participants in just a few days from the launch in the congress website. This quick response indicates a high level of interest and need on this topic, and is encouraging for the organization of future courses. In particular, we are looking forward to our upcoming educational course on the clinical management of infective and non-infective complications of GVHD, which will be held in Barcelona in October 2013 as a joint initiative of the CQWP and the Infectious Diseases WP. Furthermore, 2013 will be an exciting year in terms of development of research collaborations with other WPs, and internationally through the EBMT-NCI Task Force following from the results of our joint survey.

The CQWP is a fairly new WP, and I would like to end this brief activity report from our first inviting you all to consider joining us, to be proactive and bring in your own proposals and ideas, and to get involved in the work of our WP.

## Publications 2012 in peer-reviewed journals



WP	Title	First Listed Author	Journal	PMID
EBMT Activity Survey Office	The EBMT activity survey: 1990-2010.	Passweg JR, et al	Bone Marrow Transplant.	22543746
ALWP	Comparison of conditioning regimens of various intensities for allogeneic hematopoietic SCT using HLA-identical sibling donors in AML and MDS with <10% BM blasts: a report from EBMT.	Martino R, et al	Bone Marrow Transplant.	23208314
ALWP	Clofarabine-containing conditioning regimen for allo-SCT in AML/ALL patients: a survey from the Acute Leukemia Working Party of EBMT.	Chevallier P	Eur J Haematol.	22702414
ALWP	Impact of graft-versus-host disease after reduced-intensity conditioning allogeneic stem cell transplantation for acute myeloid leukemia: a report from the Acute Leukemia Working Party of the European group for blood and marrow transplantation.	Baron F, et al	Leukemia. 2012	22699419
ALWP	Mobilized peripheral blood stem cells compared with bone marrow from HLA-identical siblings for reduced-intensity conditioning transplantation in acute myeloid leukemia in complete remission: a retrospective analysis from the Acute Leukemia Working Party of EBMT.	Nagler A, et al	Eur J Haematol.	22650267
ALWP	Bone marrow or peripheral blood stem cell transplantation from unrelated donors in adult patients with acute myeloid leukaemia, an Acute Leukaemia Working Party analysis in 2262 patients.	Ringdén O, et al	J Intern Med. 2012	22519980
ALWP	Impact of cytogenetics risk on outcome after reduced intensity conditioning allo-SCT from an HLA-identical sibling for patients with AML in first CR: a report from the acute leukemia working party of EBMT.	Chevallier P, et al	Bone Marrow Transplant. 2012	22504932
ALWP	Mobilized peripheral blood stem cells compared with bone marrow as the stem cell source for unrelated donor allogeneic transplantation with reduced-intensity conditioning in patients with acute myeloid leukemia in complete remission: an analysis from the Acute Leukemia Working Party of the European Group for Blood and Marrow Transplantation.	Nagler A, et al.	Biol Blood Marrow Transplant. 2012	22446014
ALWP	Impact of FLT3 internal tandem duplication on the outcome of related and unrelated hematopoietic transplantation for adult acute myeloid leukemia in first remission: a retrospective analysis.	Brunet S, et al	J Clin Oncol. 2012	22291086

WP	Title	First Listed Author	Journal	PMID
ALWP	Outcomes and prognostic factors of adults with acute lymphoblastic leukemia who relapse after allogeneic hematopoietic cell transplantation. An analysis on behalf of the Acute Leukemia Working Party of EBMT.	Spyridonidis A, et al	Leukemia. 2012	22290066
ALWP	Treatment, risk factors, and outcome of adults with relapsed AML after reduced intensity conditioning for allogeneic stem cell transplantation.	Schmid C, et al	Blood. 2012	22167752
SAA	Allogeneic stem cell transplantation in paroxysmal nocturnal hemoglobinuria.	Peiffault de Latour R, et al	Haematologica. 2012	22689687
SAA	Prospective study of rabbit antithymocyte globulin and cyclosporine for aplastic anemia from the EBMT Severe Aplastic Anaemia Working Party.	Bacigalupo A, et al	Haematologica. 2012	22315497
SAA	Bone marrow versus peripheral blood as the stem cell source for sibling transplants in acquired aplastic anemia: survival advantage for bone marrow in all age groups.	Bacigalupo A, et al	Haematologica. 2012	22315497
ADWP	Long-Term Outcomes of Hematopoietic Stem Cell Transplantation for Severe Treatment-Resistant Autoimmune Cytopenia in Children.	Rabusin M, et al	Biol Blood Marrow Transplant. 2012	23253561
ADWP	A prospective, randomized, controlled trial of autologous haematopoietic stem cell transplantation for aggressive multiple sclerosis: a position paper.	Saccardi R, et al	Mult Scler. 2012	22383228
ADWP	Haematopoietic SCT in severe autoimmune diseases: updated guidelines of the European Group for Blood and Marrow Transplantation.	Snowden JA, et al	Bone Marrow Transplant. 2012	22002489
CLWP	Monosomal karyotype predicts poor survival after allogeneic stem cell transplantation in chromosome 7 abnormal myelodysplastic syndrome and secondary acute myeloid leukemia.	van Gelder M, et al	Leukemia. 2012	23164802
CLWP	Donor lymphocyte infusions for the treatment of chronic myeloid leukemia relapse following peripheral blood or bone marrow stem cell transplantation.	Basak GW, et al	Bone Marrow Transplant. 2012	23178548
CLWP	Superiority of the triple combination of bortezomib-thalidomide-dexamethasone over the dual combination of thalidomide-dexamethasone in patients with multiple myeloma progressing or relapsing after autologous transplantation: the MMVAR/IFM 2005-04 Randomized Phase III Trial from the Chronic Leukemia Working Party of the European Group for Blood and Marrow Transplantation.	Garderet L, et al	J Clin Oncol. 2012	22585692
CLWP	Prophylaxis and treatment of GVHD after allogeneic haematopoietic SCT: a survey of centre strategies by the European Group for Blood and Marrow Transplantation.	Ruutu T, et al	Bone Marrow Transplant. 2012	22410750
CLWP	Hematopoietic stem cell transplantation in T-prolymphocytic leukemia: a retrospective study from the European Group for Blood and Marrow Transplantation and the Royal Marsden Consortium.	Wiktor-Jedrzejczak W, et al	Leukemia. 2012	22116553
IEWP	Transplantation in patients with SCID: mismatched related stem cells or unrelated cord blood?	Fernandes JF, et al	Blood. 2012	22308292
IEWP	Outcome of hematopoietic stem cell transplantation for adenosine deaminase-deficient severe combined immunodeficiency.	Hassan A, et al	Blood. 2012	22791287
IDWP	Relationship between clinical and BK virological response in patients with late hemorrhagic cystitis treated with cidofovir: a retrospective study of European Group for Blood and Marrow Transplantation.	Cesaro S, et al	Bone Marrow Transplant. 2012	23222380

<b>WP</b>	<b>Title</b>	<b>First Listed Author</b>	<b>Journal</b>	<b>PMID</b>
IDWP	Long-term follow-up of HCV-infected hematopoietic SCT patients and effects of antiviral therapy.	Ljungman P, et al	Bone Marrow Transplant. 2012	22158388
LWP	High-dose therapy and autologous stem cell transplantation in first relapse for diffuse large B cell lymphoma in the rituximab era: an analysis based on data from the European Blood and Marrow Transplantation Registry.	Mounier N, et al	Curr Opin Oncol. 2012	23079783
LWP	The role of hematopoietic stem cell transplantation in the treatment of relapsed/refractory Hodgkin's lymphoma.	Sureda A, et al 2012	Curr Opin Oncol. 2012	23079783
LWP	Autologous and allogeneic stem-cell transplantation for transformed chronic lymphocytic leukemia (Richter's syndrome): A retrospective analysis from the chronic lymphocytic leukemia subcommittee of the chronic leukemia working party and lymphoma working party of the European Group for Blood and Marrow Transplantation.	Cwynarski K, et al	J Clin Oncol. 2012	22547610
LWP	Autologous stem-cell transplantation in patients with mantle cell lymphoma beyond 65 years of age: a study from the European Group for Blood and Marrow Transplantation (EBMT).	Jantunen E, et al	Ann Oncol. 2012	21467125
LWP	Allogeneic stem cell transplantation after reduced intensity conditioning in patients with relapsed or refractory Hodgkin's lymphoma. Results of the HDR-ALLO study - a prospective clinical trial by the Grupo Español de Linfomas/Trasplante de Médula Osea (GEL/TAMO) and the Lymphoma Working Party of the European Group for Blood and Marrow Transplantation.	Sureda A, et al	Haematologica. 2012	21993674
PDWP	Risk of complications during hematopoietic stem cell collection in pediatric sibling donors: a prospective European Group for Blood and Marrow Transplantation Pediatric Diseases Working Party study.	Styczynski J, et al	Blood. 2012	22160619
PDWP	Defibrotide for prophylaxis of hepatic veno-occlusive disease in paediatric haemopoietic stem-cell transplantation: an open-label, phase 3, randomised controlled trial.	Corbacioglu S, et al	Lancet. 2012	22364685
PDWP	Impact of pretransplant minimal residual disease after cord blood transplantation for childhood acute lymphoblastic leukemia in remission: an Eurocord, PDWP-EBMT analysis.	Ruggeri A, et al	Leukemia. 2012	22555150
CQWP	Recommended screening and preventive practices for long-term survivors after hematopoietic cell transplantation.	Majhail NS, et al	Hematol Oncol Stem Cell Ther. 2012	22446607
CQWP	Recommended screening and preventive practices for long-term survivors after hematopoietic cell transplantation.	Majhail NS, et al	Bone Marrow Transplant. 2012	22395764
CQWP	Cardiovascular complications in long-term survivors after allogeneic hematopoietic stem cell transplantation.	Rovó A, et al	Semin Hematol. 2012	22221782
CQWP	Recommended screening and preventive practices for long-term survivors after hematopoietic cell transplantation.	Majhail NS, et al	Biol Blood Marrow Transplant. 2012	22178693



## Report of the clinical trials office



The EBMT maintains its commitment to perform high quality academic prospective clinical trials, in order to improve patient care. The CTO has now completed the reorganisation and process improvement exercise, which was started in 2010. At the end of 2012, there was 1 prospective clinical trial recruiting patients, 2 trials in the advanced set-up stages (EA-001 and ALL SCTped Forum 2012), which will open to recruitment by the summer 2013, and 2 trials were closed to accrual in 2012 (RICMAC at 129/160 patients and ASTIC at 45/48 patients). The Plerixafor trial has been put on hold owing to restructuring at Sanofi Genzyme.

There are 4 trials under analysis which should be published in 2013 (ASTIC, RICMAC, MMVAR and ASTIS). The CTO made 2 publications in 2012 reporting the MMVAR and RATGAA07 trials (Garderet L et al, JCO 2012; 30: 2475-82 and Marsh J et al, Blood 2012; 119: 5391-6) and there were several oral and poster presentations at EBMT and ASH.

Of the 6 new clinical trials submitted to the CT2-EBMT committee in 2011, one will be jointly sponsored with the Alliance in the US (EA-001) and one will be sponsored by St Anna Kinderkrebsforschung in Vienna in collaboration with the EBMT (ALL SCTped Forum 2012). There is one proposal currently being reviewed by the CT2 committee, for sponsorship in 2013.

### Trials in the advanced set-up stage

- **EA-001** (TIGER: A Randomized Phase III Trial comparing Conventional-Dose Chemotherapy using Paclitaxel, Ifosfamide, and Cisplatin (TIP) with High-Dose Chemotherapy using mobilizing Paclitaxel plus Ifosfamide followed by High-Dose Carboplatin and Etoposide (TI-CE) as First Salvage Treatment in Relapsed or Refractory Germ Cell Tumors)
- **ALL SCTped Forum 2012:** (Allogeneic Stem Cell Transplantation in Children and Adolescents with Acute Lymphoblastic Leukaemia)

### Trials open to recruitment

- **HCT vs. CT elderly AML** (A Randomized Phase III study comparing conventional chemotherapy to low dose total body irradiation-based conditioning and hematopoietic cell transplantation from related and unrelated donors as consolidation therapy for older Patients with AML in first Complete Remission.)

### Trials under analysis

- **ASTIC** (Autologous Stem Cell Transplantation for Crohn's Syndrome)
- **RICMAC** (Dose-reduced Versus Standard Conditioning Followed by Allogeneic Stem Cell Transplantation in Patients with MDS (Myelodysplastic syndromes) or secondary AML)
- **MMVAR** (A Randomized controlled study of Velcade (Bortezomib) plus Thalidomide plus Dexamethasone compared to Thalidomide plus Dexamethasone for the treatment of myeloma patients progressing or relapsing after autologous transplantation (MMVAR))
- **ASTIS** (High dose immunoablation and autologous haematopoietic stem cell transplantation versus monthly intravenous pulse therapy cyclophosphamide for the treatment of patients with severe systemic sclerosis.)

In summary, prospective clinical trials continue to be important to the EBMT. However, they also continue to be highly challenging owing to the regulatory environment and current economic constraints. It is hoped that the CTO will be successful with the new prospective clinical trials that will be initiated in 2013 and that there will be improvements in and harmonisation of the requirements for academic clinical trials. In the meantime, the CTO will continue to implement efficiencies and to increase investment in our clinical trials. This should ensure our financial sustainability. 2013 is a very important year for the EBMT Clinical Trials Offices.

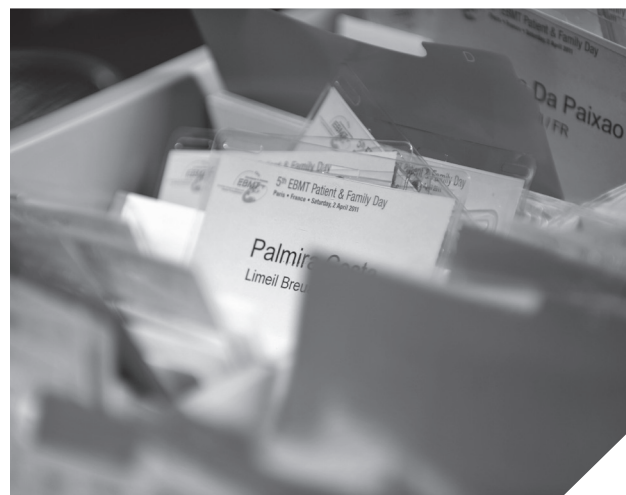
**Liz Clark**

Clinical Trials Operations Manager

**Hermann Einsele**

CT2 Committee Chair

## EBMT ED - coordinating educational activities



EBMT ED is the educational arm of the EBMT. It is the umbrella under which a range of educational opportunities are offered to the EBMT members. During 2012 the educational events listed in the table below were staged by the EBMT, its Working Parties, Nurses Group, and long-term collaborators.

The second year of the 2011-2013 EBMT Strategic Plan included the creation of an EBMT Education and Events (E&E) Unit. During 2012 the unit sought to establish itself, document its development, and continue to collaborate with traditional partners and reinforce and build on the working relationships established in 2011. In addition to assisting with other events, the E&E Unit played an important role in the organisation of the Joint WP Educational Meetings in Budapest and Paris in November 2012.

A further edition of the flagship Joint Training Course on Blood and Marrow Transplantation also took place in Sofia, Bulgaria from 22 – 25 April. The collaboration with ESH around this course and the EBMT Handbook on Haematopoietic Stem Cell Transplantation continues to bear fruit with future editions of both ventures already in the pipeline. Negotiations to publish a Turkish-language translation of the Handbook were also commenced in 2012.

**Dan Wilde**  
EBMT Education and  
Events coordinator

**Tamàs Maszi**  
Chair of the Educational  
Committee

Date	Event	Venue
22-25 Apr 2012	16 <sup>th</sup> Edition of the ESH-EBMT Training Course on Blood and Marrow Transplantation	Sofia, Bulgaria
7-9 Jun 2012	8 <sup>th</sup> Meeting of the EBMT Paediatric Diseases Working Party, 3 <sup>rd</sup> Meeting of the EBMT Paediatric Nurses including an Inborn Errors Working Party Educational Day	Prague, Czech Republic
21-22 Sep 2012	EBMT Chronic Malignancies Working Party Meeting, including educational lectures	Paris, France
27-29 Sep 2012	EBMT Infectious Diseases Working Party Training Course	Würzburg, Germany
4-5 Oct 2012	JACIE Inspector Training Course	Barcelona, Spain
5 Oct 2012	EBMT Nurses Group / UK (NAP) International Study Day	London, United Kingdom
18-19 Oct 2012	8 <sup>th</sup> Edition of the EBMT Lymphoma Working Party Educational Course on Stem Cell Transplantation in Lymphomas	Bucharest, Romania
1-3 Nov 2012	EBMT Severe Aplastic Anaemia and Late Effects Working Parties Joint Educational Meeting	Budapest, Hungary
2-4 Nov 2012	EBMT Inborn Errors Working Party Workshop and Educational Meeting	Barcelona, Spain
9-10 Nov 2012	EBMT Acute Leukemia Working Party Business Meeting & Symposium "Novel Agents in Stem Cell Transplantation"	Milan, Italy
15-17 Nov 2012	GITMO Allogeneic Workshop and 1st National JACIE Inspector Course in Italy	Turin, Italy
16-17 Nov 2012	EBMT Autoimmune Diseases and Immunobiology Working Parties Joint Educational Meeting	Paris, France

## Standards and Accreditation - improving quality and safety in cellular therapy



Since 2000, the Joint Accreditation Committee-ISCT & EBMT has received almost 300 applications from centres in Europe and beyond and over 300 inspections have been performed. Over 196 have achieved accreditation at least once with practically all centres repeating the experience after their initial accreditation. This achievement is all the more remarkable for being based on a voluntary programme delivered in the context of diverse regulations, languages and resources.

In 2012, **49 applications** (36 first-time and 13 reaccreditation) were received and **53 inspections** (31 first-time and 22 reaccreditation) were performed. **48 accreditations** (31 first-time and 17 reaccreditation) were awarded.

JACIE is now a regulatory requirement 5 countries – Belgium, 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](http://www.jacie.org/about/national-regulations).

The 5<sup>th</sup> edition of the **Standards** was released on 1 March 2012 following 18 months of tremendous effort by the members of the Standards sub-committees.

Three **Inspector Training courses** with a total of **82** participants were run either on the initiative of national societies groups or individuals with JACIE support (Malaga, March 2012 and Turin, November 2012) or directly by JACIE (Barcelona, October 2012).

One **Centre Preparation Course** with 27 participants was run as part of the Turkish Hematology Society annual meeting in Antalya in March.

In 2012, JACIE rolled out a new training format by offering **Orientation Visits** to applicant centres on-site by experienced JACIE staff. Two pilot visits were completed in May and one full visit was completed shortly afterwards.

An online version of the Standards exam for inspectors was launched to coincide with the new edition of the Standards providing automatic scoring and indicating the correct answers thus supporting inspectors' continuous training.

A travel agency service and standardized *per diem* payment were introduced for inspectors to avail of.

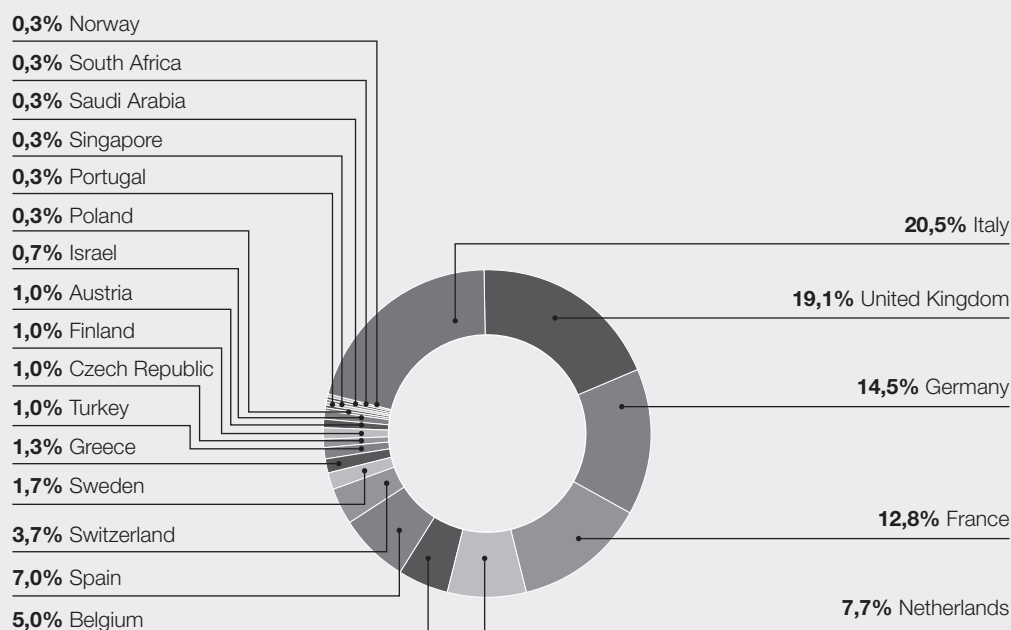
An online collaboration tool, Teambox, was introduced in September to support the organization of inspections by centralizing all communications and documentation in a single point of contact.

In late 2012, the EBMT JACIE representatives Alessandro Rambaldi (Bergamo, IT), Christian Chabannon (Marseille, FR) and Nina Worel (Vienna, AT) were joined by Massimo Dominici (Modena, IT), ISCT President-elect and Stephan Mielke (Wurzburg, DE) Europe, Regional Treasurer (Europe).

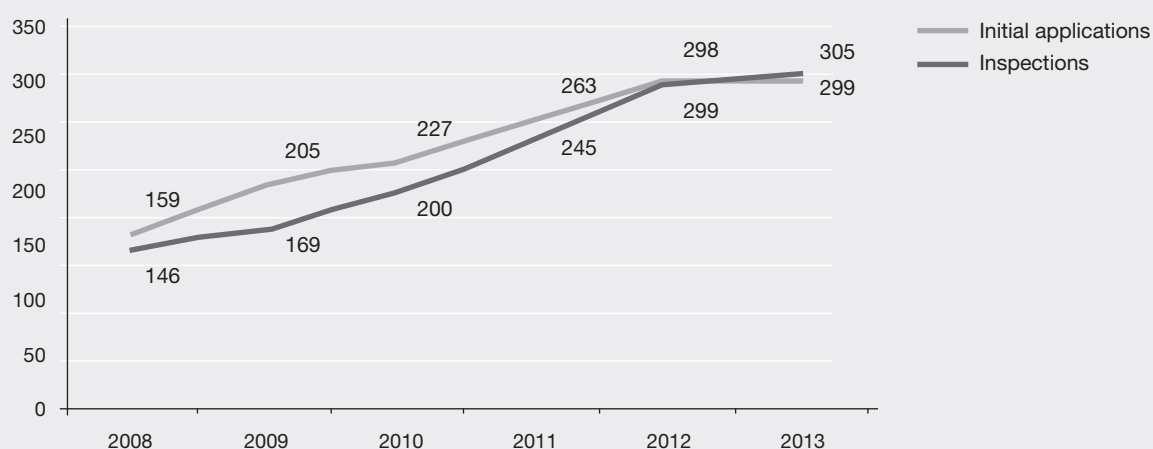
As ever, I would like to express my appreciation and admiration for the inspectors, Board members and other volunteers for their amazing hard work, commitment and dedication.

**Eoin McGrath**  
JACIE Executive Officer

## % distribution of total initial applications by country



## Cumulative initial applications & all inspections (initial & reaccreditation)



## 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 through 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 HSC transplantation. 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.

# Report of the statistical unit



## Studies

The main contribution of the EBMT Statistical Unit is in performing high quality prospective and retrospective studies. The EBMT statisticians support all EBMT Working Parties providing consultancy or full support, from planning to analysis to publication. In 2012 they contributed to a total of about 100 studies performed by the Leiden and Paris offices and to several EBMT clinical trials:

- 79 retrospective registry-based studies
- 22 observational prospective studies or surveys
- 7 interventional prospective trials

The EBMT statisticians authored 25 manuscripts published in 2012 in high-rank journals, and several oral and poster presentations were given at international congresses.

## Education

The EBMT statisticians are involved in several educational projects aimed at the diffusion of good methodology and appropriate statistical methods in stem cell transplant research; the educational activities are performed in cooperation with the EBMT Statistical Committee. The main achievements in 2012 were:

- The finalization of the updated EBMT statistical guidelines (publication expected in March 2013)
- The statistical symposium and the round table during the EBMT annual meeting
- One educational session during the EBMT annual meeting
- Two oral presentations at the annual meeting of the International Society of Clinical Biostatistics, based on EBMT studies
- The courses in basic statistics and consultancy for participants to the EBMT annual meeting
- The statistical course at the ESH-EBMT Training Course on Blood and Marrow Transplantation
- The statistical educational meeting for exchanges among scientists co-operating with EBMT

## Support to the EBMT Clinical Trial Office, Registry and Data Offices

As a further contribution to the activities of EBMT, the Statistical Unit works in cooperation with the other units, to guarantee adequate methodological support. In 2012 in particular the EBMT statisticians contributed to:

- The selection of prospective clinical trials proposals for the CT2 Committee
- The process of registry upgrade
- The initiatives for improvement of data quality

**Simona Iacobelli**  
Statistical Unit Coordinator

**Myriam Labopin**  
Statistical Committee Chair



# The EBMT Nurses Group - improving patient care in haematology and HSCT

During 2012 the Nurses Group (EBMT NG) became a more integrated part of the main organisation. Accordingly there should now be a Principal Nurse at every centre, an important change which reflects and confirms the role of nurses and allied health professionals as significant contributors in the haematological and stem cell transplant setting as well as within the EBMT organisation. We still act independently on nursing issues while authority and strength has been added by sharing by-laws and constitution.

## Scientific Committee

The Scientific Committee (SC) plans and coordinates the Nurses programme of the annual meeting. The 28th Meeting of the Nurses Group was well organised with submitted abstracts and presentations of high scientific level.

The 8<sup>th</sup> Pre-Meeting Education Day attracting 243 participants is one of the most important annual educational initiatives by the SC. During the meeting the fourth Distinguished Merit Award was awarded to Barry Quinn (UK) who since 1986 through his commitment to EBMT has managed to raise the profile of nursing.

## Research Committee

In 2012 the activities of the Research Committee have been focused on several studies including late effects together with the Complications and Quality of Life Committee, infection control in collaboration with the Infectious Diseases Working Party (IDWP) and adherence, of which the latter was submitted for publication in December.

## Communication and Networking Committee (CNC)

The CNC is working on the continuous production and edition of the nursing content in the Newsletter maintaining contact and communication both with National Groups/Forums and individual members.

They are also an important link between the NG and the EBMT Communication Coordinator.

## Paediatric Committee

The Paediatric Committee has an established collaboration with the Paediatric Diseases Working Party (PDWP) and takes an active part in shared activities such as the meeting of the EBMT Paediatric Nurses, the EBMT PDWP and Inborn Errors WP in Prague, Czech Republic in June 2012, as well as the planning of the bi-annual course to be held in Bucharest, Romania in May 2013.

The Paediatric Committee developed and performed a Master Class in principals of patient care in a Scientific Center of Paediatric Hematology, Oncology and Immunology in Moscow in October 2012. This enterprise was a great achievement which will be followed by similar classes in both paediatric and adult settings in St Petersburg and Moscow during 2013.

## National Groups and Regional Forums

One way of collaborating with colleagues is through contact with the National Groups and Regional Forums.

The Belgian group joined in early 2012 and became the 12th member of the 9 National Groups and 3 Regional Forums. There is constant interest in the EBMT in countries where no National Group has been formed yet, and we expect this network to continue to grow.

Following the success of the first International Study Day arranged by the EBMT Swiss Nurses Group and the EBMT-NG in 2011, the National Group collaboration continued with the 2<sup>nd</sup> Study Day in London, UK in October 2012.

This was hosted by the EBMT UK Nurses and Allied Health Professionals (NAP) Group. It was well attended with international representation both of attendees and speakers and offered a very interesting program of high quality.

## Educational Initiatives

In order to reach the goal of the EBMT-NG to expand and optimise existing educational projects we have continued to invest in education during 2012.



Activities such as the Pre-Meeting Education Day, contribution to a course in Turkey completing and presenting the MDS slide deck and ITP project, the International Study Day, Paediatric Meeting and Paediatric Master Class are examples of what have been achieved in the past year. Late in 2012 we started a new project for education in VOD which we anticipate to complete during 2013.

In order to assure a high scientific level and a structured approach for all EBMT-NG events the Scientific Committee has been given the main responsibility for educational issues.

Our primary objectives of improving care for the patients remains for 2013 and is why education will be continue to be in focus. We aim to identify not only educational needs but also areas of expertise among our members. To facilitate this efficient communication with members, both through the Principal Nurses and individually, will be an important task.

**Elisabeth Wallhult**  
EBMT Nurses Group President

## About the EBMT Nurses Group

The EBMT Nurses Group (NG) is one of the leading groups in the field of Haematology and Haematological Stem Cell Transplantation (HSCT) nursing. It represents nurses and allied health professionals from over 500 transplant centres from over 50 countries worldwide.

The group is dedicated to improving the care of patients receiving HSCT and works towards promoting excellence in the provision of blood and marrow transplant and haematological care by supporting nurses and health care professionals in the provision of evidence based practice. By recognising and building upon good practice, the group provides information and forums to support and share knowledge in research, education and training and clinical practice.

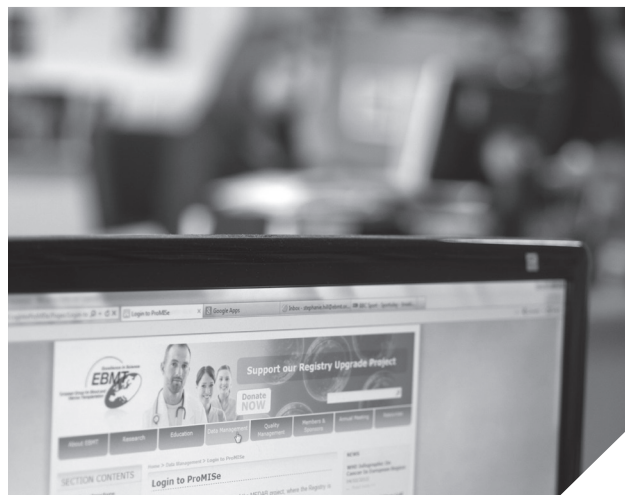
## Information and communication

In June 2012, the Communications Policy was presented to the Board during the Board Meeting in Amsterdam. This document defines our communication objectives to:

1. Enhance communication towards EBMT Members
2. Strengthen communications and public relations programmes as a future asset for fundraising
3. Develop greater visibility for the EBMT and position the Society as the primary source for credible scientific information about SCT, cellular therapy and gene therapy.

With this in mind, EBMT Members, Sponsors, and EU/National Authorities have been identified as main target audiences. However, it is important for the EBMT to consider other audiences such as Board Members, Working Parties, EBMT Staff, Patients, Donors and the Media.

Concerning the upcoming 40th Anniversary, a PR campaign has been defined with the help of the Anniversary Working Group, which is made by the Communications Working Group and members of EBMT Staff that have volunteered to participate in the project. This PR Campaign includes the publication of Anniversary Papers at the BMT Journal and the celebration during EBMT 2014 among others.



In June 2012, a survey was sent to all recipients to assess the existing Newsletter. The outcome will help us upgrade the newsletter, which will include new features to facilitate navigation within the Newsletter and a new layout and content distribution.

As for the Registry Upgrade Project, the Communications Coordinator and the Marketing and Fundraising Coordinators have been closely working together with the Registry Office to develop an effective communications strategy towards Members. The aims of that communications strategy are to extensively communicate each milestone of the project as well as to promote the crowd funding platform hosted in the EBMT Website.

**Mélanie Chaboissier**

Communications Coordinator

## The Corporate Sponsors



18 Corporate Sponsors supported the day-to-day work of the society in 2012.

With their generous support, EBMT Corporate Sponsors join the mutual efforts of EBMT Members to promote all aspects of blood and marrow transplantation. The EBMT aims for long-term relations with its Corporate Sponsors in order to ensure a safe development of its activities, thanks to their first of commitment of three years.

In 2012, GENTIUM upgraded its support to the EBMT and became our Platinum Sponsor.

The Industry Meeting, a one-hour meeting between the Board and our Corporate sponsors was organised during the Annual Meeting in Geneva, with a specific focus on the EBMT Strategic Plan (outcomes for 2011 and highlights for 2012), EBMT major projects to develop in partnership with the Industry (The Registry Upgrade, The development of Clinical Trials, The Educational Events), The Activity Survey (key figures) and the presentation of the EBMT 2013 in London.

Additionally, two new Corporate Sponsors joined the EBMT: Clinigen and Sandoz Pharmaceuticals, and we are extremely grateful for their support.

**Sandrine Ehrmann**  
Marketing and Fundraising Coordinator

### Platinum - 100.000 €



### Gold - 50.000 €



### Silver - 20.000 €



### Bronze - 10.000 €



# EBMT

## financial highlights

In 2012, EBMT has faced challenging financial situations with strength and all EBMT Units have faced adjustments in order to strengthen the current and future financial stability of the organisation during this difficult economic European crisis. Expenses and income growth were accommodated to a similar limit.

More transparency; control and accountability are still on process to be improved, and EBMT decided to start to work with a new international audit firm (Ernst & Young) with the objective in the mid term to have an unqualified opinion.

With the objective to reduce financial risk and gain financial stability and assure that the money is expended according our Mission, EBMT has developed its strategy for diversification, retention of sources and assure that the 80% of our expenses are Mission expenses.

In that direction, EBMT works to assure its non-earmarked income (Membership, Sponsoring, Annual Meeting ) in order to cover structural cost (Registry and Management) and launch non-commercial academic retrospective studies and educational activities through our Working Parties network.

The earmarked income comes from pharma grants allocated for specific studies and educational activities, for our Clinical Trial Office and also Working Parties network. Among the different studies CALM Project (Genzyme) has been the largest grant. The costs of JACIE are covered by the contributions from the centers that are accredited.

In 2012 EBMT has assured that 81% of its Budget was dedicated to its Mission (Studies, Registry, Accreditation and Education), the 19% has been dedicated to Management (Board and Executive Office expenses).

EBMT will end the year with a total loss of - 118K€ coming due to decrease in its capacity to raise funds for Clinical Trials. EBMT is closing the year with a total expenses of 3.350K€, representing +3% deviation from its initial Expenses Budget. From the Income side, EBMT is closing the year with a total income of 3.232K€, representing +1% deviation from initial expectations.

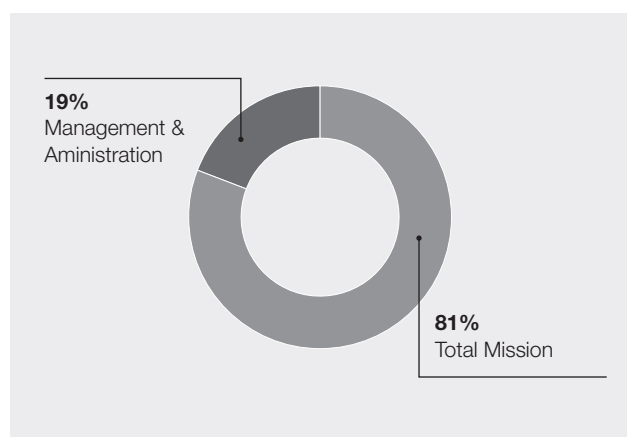
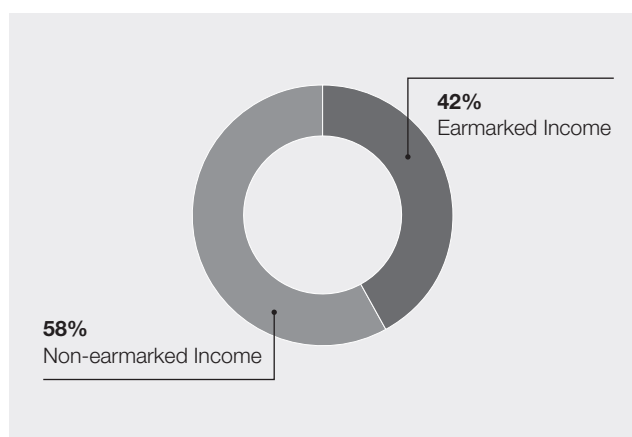
**Frederik Falkenburg**  
EBMT Treasurer

SOURCE of INCOME 2012	Budget	Final Income	
in 1,000€		€	%
Membership	490	408	13
Sponsoring	473	430	13
Annual Meeting	925	923	29
Others	90	109	3
<b>Non-earmarked Income</b>	<b>1.978</b>	<b>1.870</b>	<b>58</b>
Studies & CT & Education	846	1.039	32
Accreditation (JACIE)	333	314	10
Other Grants	50	10	0
<b>Earmarked Income</b>	<b>1.230</b>	<b>1.363</b>	<b>42</b>
<b>TOTAL Income</b>	<b>3.207</b>	<b>3.232</b>	<b>100</b>

HOW EBMT SPEND THE MONEY 2012	Budget	Final Expenses	
in 1,000€		€	%
Retrospectives Studies	881	1.064	32
Prospective Studies	558	566	17
Educational Activities	218	188	6
EBMT Registry	508	486	15
Accreditation Process (JACIE)	297	274	8
Nurses Activities	55	85	3
Committees Activities	17	3	0
Registry Upgrade	75	54	2
<b>Total Mission</b>	<b>2.609</b>	<b>2.721</b>	<b>81</b>
<b>Management &amp; Administration</b>	<b>640</b>	<b>629</b>	<b>19</b>
<b>TOTAL Cost</b>	<b>3.249</b>	<b>3.350</b>	<b>100</b>

NET RESULT 2012			
<b>TOTAL Income</b>	<b>3.207</b>	<b>3.232</b>	
<b>TOTAL Cost</b>	<b>3.249</b>	<b>3.350</b>	
<b>TOTAL Surplus/Deficit</b>	<b>-42</b>	<b>-118</b>	



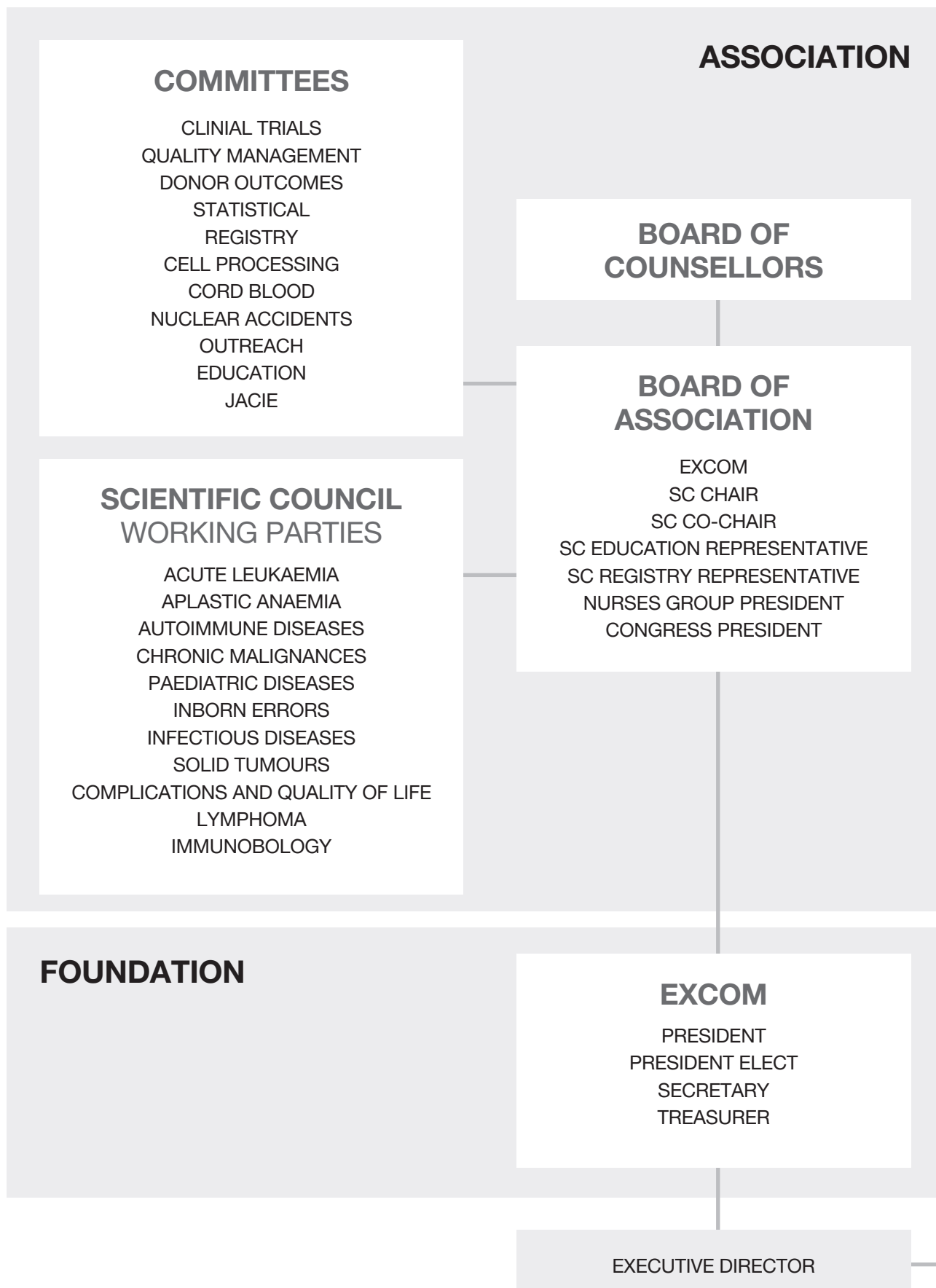


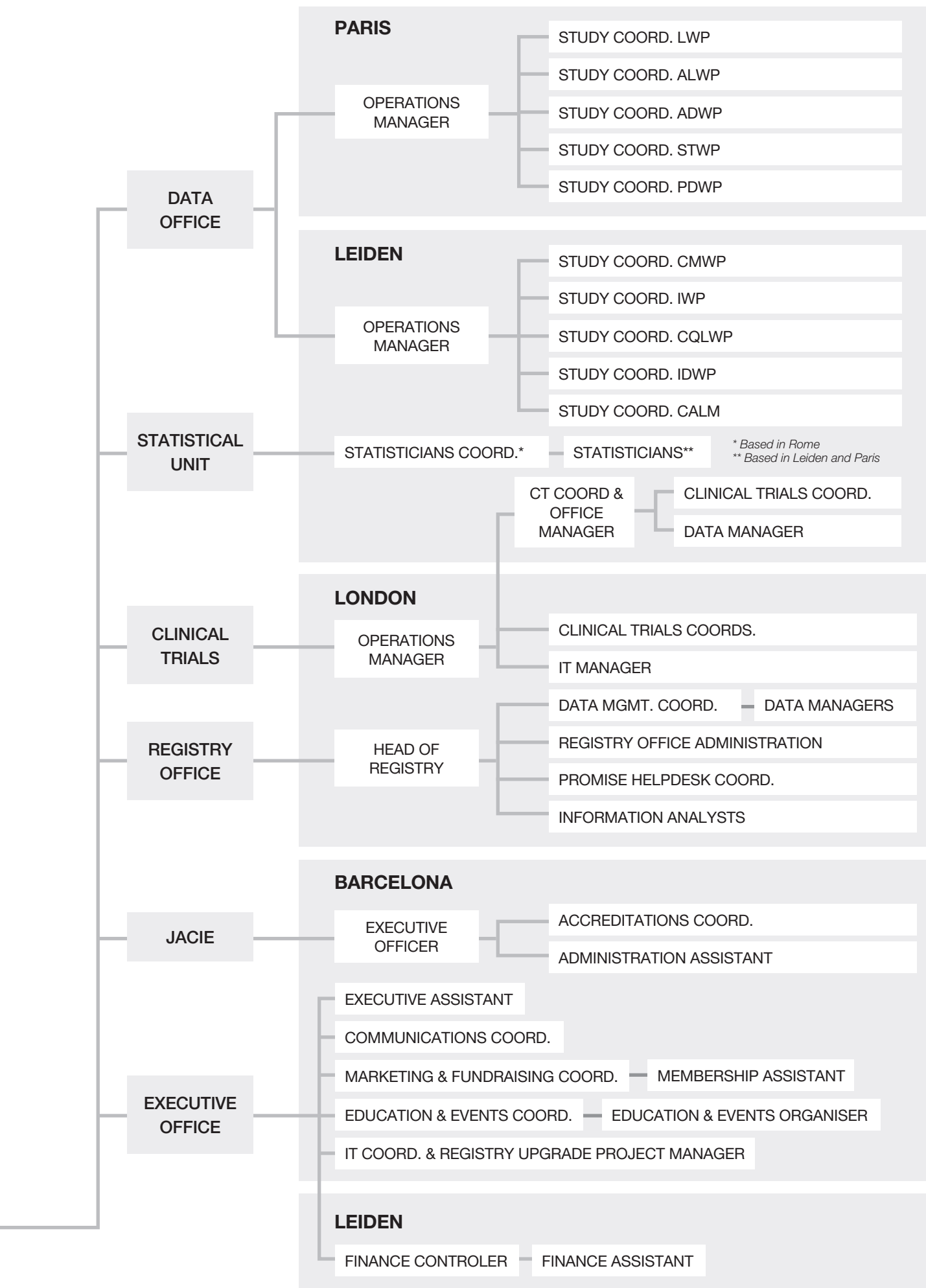
BALANCE 2012		
(financial situation at the of the year)		
in 1,000	€	%
Cash & equivalents	4.108	79
Other current assets	1.082	21
<b>Total Net Assets</b>	<b>5.190</b>	<b>100</b>
Earmarked funds	350	7
Non-earmarked funds	2.293	44
Other Reserves	135	3
<b>Total Reserves</b>	<b>2.778</b>	<b>54</b>
Current Liabilities	2.412	46
<b>Total Liabilities and Reserves</b>	<b>5.190</b>	<b>100</b>

EBMT Staff 2012		
(at the end of the year)	FTEs	%
Study Coordinator and Statisticians for Retrospectives Studies	14	37
Study Coordinator and Statisticians Prospective Studies	7,39	20
Educational Activities	1,50	4
Registry	7,80	21
Accreditation Process (JACIE)	2,00	5
Communication Coordinator	1,00	3
Fundraising and Membership Staff	1,30	3
Management Staff	2,67	7
<b>Total Staff</b>	<b>38</b>	<b>100</b>

# Organigramme





# The EBMT Board, Committee Chairs and Board of Counsellors in 2012

## Executive Committee

### President

Alejandro Madrigal  
London, UK

### Secretary

Anna Sureda  
Cambridge, UK

### Treasurer

J.H. Frederik Falkenburg  
Leiden, The Netherlands

## EBMT Working Parties Chairs, Nurses' Group President and Congress President

### Aplastic Anaemia

Judith Marsh  
London, UK

### Autoimmune Diseases

Dominique Farge Bancel  
Paris, France

### Immunobiology

Andrea Velardi  
Perugia, Italy

### Infectious Diseases

Simone Cesaro  
Verona, Italy

### Lymphoma

Peter Dreger  
Heidelberg, Germany

### Solid Tumours

Marco Bregni  
Milan, Italy

### Acute Leukaemia

Mohamad Mohty  
Nantes, France

### Chronic Malignancies

Nicolaus Kröger  
Hamburg, Germany

### Inborn Errors

Bobby Gaspar  
London, UK

### Transplant-related Complications and Quality of Life after SCT

Rafael Duarte  
Barcelona, Spain

### Paediatric Diseases

Christina Peters  
Vienna, Austria

### Nurses Group President

Elisabeth Wallhult  
Göteborg, Sweden

### Congress President 2013

Jane Apperley  
London, UK

## EBMT Committee Chairs

### Education Committee

Tamás Masszi - Budapest, Hungary

### Nuclear Accident Committee

Ray Powles - London, UK

### CT2-EBMT Committee

Hermann Einsele - Würzburg, Germany

### Statistical Committee

Myriam Labopin - Paris, France

### Quality Management Committee

Pierre Donot - Lyon, France

### Outreach Committee

Elisabeth Benedek

### Donor Outcomes Committee

Joerg Halter

### Cell Processing

Christian Chabannon

### Registry Committee

Per Ljungman

### Cord Blood Committee

Vanderson Rocha

## Board of Counsellors

John Goldman - London, UK  
Andrea Bacigalupo - Genova, Italy  
Gösta Gahrton - Stockholm, Sweden

Bob Löwenberg - Rotterdam, The Netherlands  
Mary Horowitz - Wisconsin, USA





Thank you to Jane Apperley and her team from the Imperial College,  
Department of Haematology, Hammersmith Hospital, London.

#### **Barcelona**

EBMT Executive Office  
JACIE Accreditation Office

C/ Rosselló 140, 1<sup>o</sup>-1<sup>a</sup>  
08036 Barcelona  
Spain

EBMT Executive Office  
Tel: +34 93 453 8570  
Fax: +34 93 451 9583

[info@ebmt.org](mailto:info@ebmt.org)

JACIE Accreditation Office  
Tel: +34 93 453 8711  
Fax: +34 93 451 9583  
[jacie@ebmt.org](mailto:jacie@ebmt.org)

[www.ebmt.org](http://www.ebmt.org)

#### **London**

EBMT Central Registry Office  
EBMT Clinical Trials Office

12<sup>th</sup> Floor, Tower Wing  
Guy's Hospital  
Great Maze Pond  
SE1 9RT London  
United Kingdom

EBMT Central Registry Office  
Tel: +44 207 188 8408  
Fax: +44 207 188 8411  
[registryhelpdesk@ebmt.org](mailto:registryhelpdesk@ebmt.org)

EBMT Clinical Trials Office  
Tel: +44 207 188 8402  
Fax: +44 207 188 8406

#### **Leiden**

EBMT Data Office  
EBMT Clinical Trials Office

Rijnsburgerweg 10  
2333 AA Leiden  
The Netherlands

EBMT Study Office  
Tel: +31 (0)71 526 4746  
Fax: +49 71 149 008 723  
[EBMTDOLeiden@lumc.nl](mailto:EBMTDOLeiden@lumc.nl)

EBMT Clinical Trials Office  
Tel: +31 (0)71 526 5005

#### **Paris**

EBMT Data Office

Hôpital Saint Antoine  
184, rue du Faubourg Saint Antoine  
75571 Paris Cedex 12  
France

Tel: +33 1 71 97 04 85  
Fax: +33 1 71 97 04 88