

Annual Report /13

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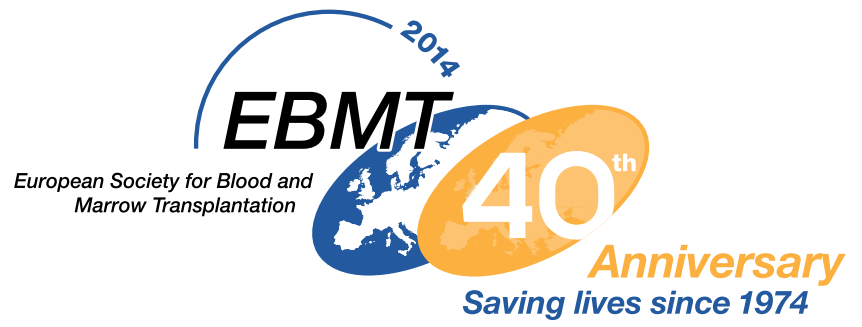
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The Mission and Vision

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

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 Burses 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

Enhancing the scientific output of the organisation through strong support from the working parties to exploit the potential of the registry, and continue generating high quality retrospective and prospective data both in the autologous and allogeneic settings;

Collaborating with the different disease-oriented cooperative groups;

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

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

Advocating for patients before the health authorities in order to maintain a high standard of care and high quality research.

2013

EBMT at a glance

4,323
members based in **563** transplant centres in **57** countries worldwide

4,242
participants from **60** countries attended EBMT 2013 Annual Meeting

44
publications in peer-reviewed journals

6,274
copies of the ESH-EBMT handbook distributed worldwide in 2013

13
educational events

7
Prospective Clinical Trials underway (in set-up, accruing patients or in follow-up) and **2** trials were closed to accrual in 2013

2
trials were closed to accrual in 2013

115
retrospective analyses underway and **18** non-interventional studies

478,586
transplants were registered in the EBMT Registry at the end of 2013

37,818
HSCTs submitted to the Activity Survey during 2013

Foreword by the EBMT President



Shortly after my election as President, I recall passing by the White House in Washington and being asked a very interesting question, *what would I like to leave as my legacy to the EBMT?* Some four years on, following the implementation of my Vision by the Board, I feel that this question has been answered by the very successful changes we that we have made together. Our prime mission has been to *increase the level of science across the organisation, advance clinical practice, improve the governance structure for effective and accountable implementation, and maximise resources to increase our activities.*

Looking back, I feel proud not only of our Society's achievements, but also proud of the passion, honesty and commitment of all the staff and members of EBMT involved in the process. During the last 4 years, the EBMT has; published more than 150 peer-reviewed manuscripts that give an accumulative impact factor of more than 1000, carried out more than 50 educational activities, presented more than 350 abstracts for oral presentations and posters, initiated 10 prospective clinical trials and reached a Registry record of 470,000.

The Clinical Trials Office has recently signed two new protocols (RACE and MCV), following 4 years without any new trials. The Society is now ready to develop and implement prospective clinical trials with scientific relevance, operational feasibility and financial sustainability.

The Board's far-reaching decision to update the Database (for the first time in 15 years) and set-up the Registry Upgrade Project, led to the Board successfully raising over 450,000€ from national registries, private foundations and pharmaceutical companies, to install state-of-the-art software that is designed to meet future data-collection and retrieval needs and facilitate studies. This will allow the EBMT to continue pursuing its 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.

The Board has reconfigured the organisational structure and the governance decision process of EBMT, leading to the creation of an Executive Director position. Both the Board and staff have gone through a major organisational change providing the scope to extend its professionalism as an organisation. It has devised a system of governance, whereby science, education and quality are well represented, resulting in the creation of two platforms; the Board of Association, which is 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 and committees have been reorganised and re-launched. In addition, the Society now has a solid, transparent and risk management control system in place, which allows it to take decisions considering present and future liabilities.

It has been a great personal sadness, not only for me but also for the wider EBMT community, to learn that two of our dear friends and colleagues, Prof. Alberto Graña and Prof. John Goldman passed away last year in October and December, respectively. The EBMT will honour them and remember their achievements during the Annual Meeting in Milan.

In its 40th Anniversary, I wish the Society and my successor, Prof Mohamed Mohty, every success, as he continues to drive our great organisation through future challenges with determination and passion.

Finally, I would like to thank all of the EBMT members and staff for placing their trust in me, for being such a pleasure to work with and for their generosity of spirit. I am delighted that the objectives I began with at the beginning of this journey have been achieved and memories of this time will remain with me.

My warmest regards to you all,

Alejandro Madrigal
EBMT President

Highlights of 2014 Strategic Plan by the EBMT Executive Director



In our process of establishing priorities for 2014 we are taking into account; objectives that are pending consolidation from the last Strategic Plan (2011-2013) lead by Alejandro Madrigal and the new EBMT Vision presented by Mohamad Mohty our president elect, in order to define the New Strategic Plan for 2014-2016.

2014 will be a year where the Registry, Data Offices and Statistical Unit will focus their efforts on;

- the implementation and data migration of the new Registry system, and in the training of EBMT Centres, with the support of National Registries and other Partners.
- supporting the Working Parties studies, with special emphasis on the CALM and VOD studies
- improving the data quality of the EBMT studies and data base.

After 4 years without a new Clinical Trial, EBMT has entered 2014 with 2 new clinical trials (Race and CMV Prophylaxis). There are also 5 trials in follow-up or final analysis phase (ASTIC, ASTIS, LYM-1, MMVAR, and RICMAC). Six trials are in the process of closure (RAGAA07, CLL, FLAGSHIP, MSC prophylaxis/treatment and ASTIMS) and we will also complete and implement the corrective and preventative actions (CAPA) proposed by the UK Medicines and Health Products Regulatory Agency (MHRA) report in preparation for the next inspection.

JACIE's main goals will be to; expand activities to regions beyond Western, Northern and Southern Europe, provide more direct support to building capacity among centres and improve the inspection process by training more inspectors and introducing quality managers as inspectors.

After the consolidation of our communication tools (website, newsletter and annual report), we now need to improve our social media presence. A Facebook; Twitter and LinkedIn accounts have been created with the objective to leverage the EBMT scientific output through our studies and publications.

In terms of marketing and fundraising we will work to; improve and develop the partnership between EBMT and Pharmaceutical companies, define the action plan to target companies, eg. Social Corporate Responsibility Budget (SCR) - a pilot run in France and improve our Marketing support to better present and sell educational and accreditation services.

As a result of the healthy experience of working in tandem with EBMT Working Parties and the Nurses Group since 2011, the Education and Events Unit aims to play an important part in the organisation of successful educational events during 2014 by ensuring that the EBMT's financial control mechanisms are employed in relation to such events. Annual Congress activities will focus on the smooth running of EBMT 2014 in Milan and preparations for EBMT 2015 in Istanbul. Following Milan 2014 a review of the organisational and logistical elements of the Congress will be conducted with MCI [EBMT's current Professional Congress Organiser (PCO)].

From the financial point of view, we will work to reduce the structural deficit at the end of the year with an action plan which should provide a balanced budget for 2015. Work on implementing a basic, robust and solid VAT Management system regarding all EBMT activities has already started with the aim to improve our internal control mechanisms and reduce legal, administrative and financial risks.

Andreu Gusi
EBMT Executive Director

EBMT 40th Anniversary: Progress goes faster every year



Similar to many prestigious scientific societies, EBMT originated from nothing: at most 5 to 10 people who were obsessed with their patients, their teams, and the dreams we all share to understand disease and, through the understanding of disease, to discover the deepest secrets of life. Our original targets, normal and leukemic stem cells, gene manipulation, cell expansion, cell therapy (at that time limited to bone marrow transplantation), and stem cell cryopreservation all masked an irrational and unformulated thought that cell therapy would somehow put us closer to immortality. When the original founders of EBMT first met in 1974 in the French ski resort of Courchevel, their minds were filled with the scientific highlights of that time, such as the discovery of almost intact frozen mammoths in Siberia, the first attempts at bone marrow transplantation with initial reports claiming an incredible 10% cure rate in end-stage full blown leukemic patients, the beginnings of HLA typing, and the dissection of immune cell populations. Over 40 years, EBMT has grown at an incredible rate: from the original 5 or so teams, it now includes more than 570 teams. While the first meetings were attended by fewer than 100 participants, the latest meetings in Florence, Goteborg, Paris, Geneva, London, and this year in Milan have attracted more than 4,500 participants. From the original few European countries, EBMT has now spread to cover the whole world including Asia, Australia and South America, and a few members from North America. EBMT, originally the "European group for bone marrow transplantation", has become the "European Society for Blood and Marrow Transplantation". For the last 30 years the EBMT annual meetings, which originally only involved physicians, have been coupled with the annual meeting of the nurses' group and, more recently, with groups involved with data management, quality management, cell therapy, and family and donor services. Over time, EBMT developed the largest worldwide registry on stem cell transplantation with records on more than 400,000 patients and more than 460,000 transplants from all over the world.

There are three major reasons for this success. The first two are closely linked to our decentralized structure, which relies on an incredibly dedicated network of volunteers. The third reason is the approach of "I bet on the improbable", which has been an unspoken but a very real tenet of EBMT.

The major scientific thread that EBMT members have always followed is the possibility to offer stem cell transplantation and/or another type of cell therapy to all patients in need. In the seventies, only 25% of patients below 35 years of age could be transplanted with an HLA identical sibling donor and 50% of these patients would experience acute graft versus host disease, and a further 50% of the survivors would suffer from chronic versus graft disease with a transplant-related mortality rate of approximately 30-50%. Cryopreservation of stem cells paved the way for autologous stem cell transplantation (ASCT), which was thought to offer an alternative to patients who could not be allografted. Nowadays, approximately 40,000 ASCTs are performed yearly worldwide, predominantly for lymphoid malignancies (lymphomas, myelomas, and autoimmune diseases). Since the first ASCT which was performed in Paris in 1977 until the year 2000, ASCT was also intensively tested in the consolidation therapy of patients with acute myelocytic leukemia in complete remission. In the early 90's, after the discovery and clinical introduction of cytokines such as GM-CSF and G-CSF, both in the autologous and allogeneic settings, peripheral blood stem cells (PBSC) progressively replaced marrow as a source of stem cells.

The discovery of the immune-mediated allogeneic graft versus leukemia/tumor effect was the leading incentive that pushed EBMT teams to find the means to offer this transplant modality to all patients in need. The first major shift was the establishment of unrelated donor registries, which now include more than 20 million potential donors worldwide. Unrelated cord blood banks expanded rapidly after the first cord blood transplant was performed in a child in Paris in the late 80's.

But at a time when all minds were looking at maximizing cytoreduction to increase tumor cell killing, the most improbable, courageous, yet successful approach was the introduction of the so-called reduced intensity conditioning (RIC) regimens, which has extended the age limit for an allogeneic transplant to elderly and unfit patients.



As we celebrate this 40th anniversary of EBMT, another improbable bet has been achieved through the advent of haplo mismatched transplants which allow finding a suitable donor for almost all patients in need.

In the 40 years of EBMT's existence, at least three Nobel prizes were awarded for research in stem cells and transplantation: in 1980 (Jean Dausset) for the HLA histocompatibility system discovery; in 1990 (Don Thomas) for allogeneic stem cell transplantation; and in 2012 (Shinya Yamanaka) for the discovery of induced pluripotent stem cells or iPS.

Since the beginnings of EBMT, there have been two unfulfilled prophecies: the first is the discovery of the causes of malignant diseases, and the second is the replacement of stem cell transplantation by targeted therapies. The latter has been already successfully achieved in chronic myeloid leukemia, and the development of other kinds of cell therapy such as tumor vaccination and the use of NK cells or engineered T cells makes it likely that EBMT will celebrate a radiant 50th anniversary in 2024. The 40th anniversary of EBMT is a tribute to all of the presidents, chairs, and secretaries of the working parties, and to our colleagues who have advanced the field with their passion-driven minds. Sadly, some of these have passed away; at this anniversary meeting we will unfortunately miss the former EBMT President John Goldman.

Norbert-Claude Gorin
EBMT honorary member

The scientific activity reports



Dear Friends and Colleagues,

It is a great pleasure to introduce the latest scientific report of EBMT, summarizing the scientific activities of our society for the last 12 months. As you know, the EBMT through its different working parties and committees aims to allow scientists and physicians involved in stem cell transplantation and cellular therapy to share their experience and develop co-operative studies. These activities rely mainly on a large registry specifically devoted to the promotion and analyses of all aspects associated with transplantation of stem cells from all donor sources and donor types including prospective and retrospective studies, and education in this field. Over the last decade, studies conducted by EBMT yielded a significant amount of scientific knowledge creating the basis for continuously improving patient care. In 2013, thanks to the support and voluntary involvement of all EBMT members, the society was able to generate an amazing number of high impact factor manuscripts and communications at major meetings, despite the context of low resources setting and strong worldwide scientific competition. I do thank you all for your continuous support, and you can count on the commitment of EBMT leadership to boost the scientific drive of our organisation.

Mohamad Mohty
Chairman, Scientific Council
President-Elect
Paris, France (CIC 775)

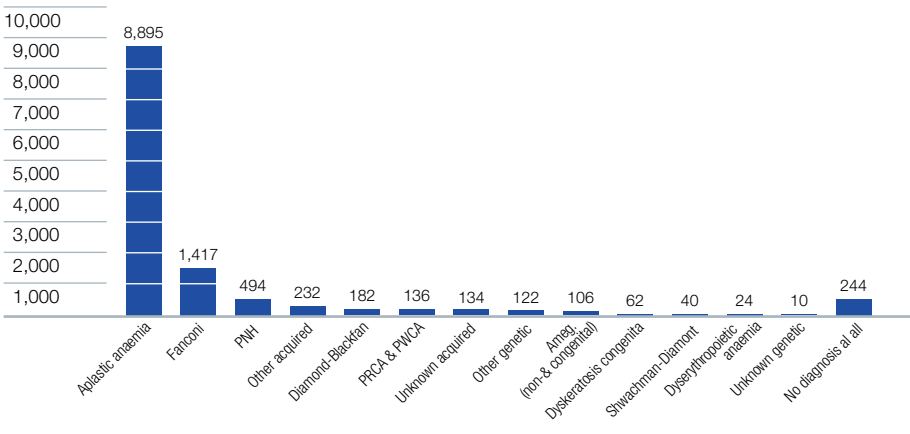


Severe Aplastic Anaemia Working Party
Chair: Judith Marsh

Introduction

The SAAWP focuses not only on aplastic anaemia (SAA), but other bone marrow failure (BMF) disorders such as PNH, and constitutional BMF syndromes including Fanconi anaemia and dyskeratosis congenita, as well as single lineage BMF disorders. The SAA database reports on a very large number of transplant as well as non-transplant treatment options, most frequently immunosuppressive therapy (IST) (see figure and table below. The table summarises the number of patients reported to the SAAWP of EBMT:

Major scientific successes and most relevant activities



Total patients	12,098
HSCT	9,658
Total HSCT	10,708
IST 1 st therapy	3,777
IST total therapies	4,927



Major scientific successes and most relevant activities

1. Publication of the complete algorithm for diagnosis and treatment of SAA, in a special issue of Bone Marrow Transplantation dedicated to SAAWP in 2013.
2. Excellent track record of prospective EBMT sponsored clinical trials published in high impact journals:
 - Prospective EBMT sponsored clinical trial comparing rabbit ATG with horse ATG, results published in Blood 2012.
 - EBMT prospective randomised clinical trial of horse ATG and ciclosporin with or without G-CSF, also published in Blood and awarded CME accreditation by Blood.
 - Approval in 2013 for a prospective randomised EBMT sponsored trial of horse ATG and ciclosporin with or without eltrombopag (see below).
3. Some key publications in 2013, for example:
 - Largest ever study published on Fanconi anaemia HSCT (Delatour et al, Blood) showing significant improvements using fludarabine based, non-irradiation conditioning regimens.
 - Largest study on syngeneic HSCT for SAA (Gerull et al, Haematologica).
4. Oral presentation at ASH 2013 (Bacigalupo et al): Current outcome of HLA identical sibling vs unrelated donor transplants in severe aplastic anaemia: An EBMT Analysis. This study has shown how much outcomes after unrelated donor HSCT for SAA have increased in recent years and now closely approaching outcomes after matched sibling donor HSCT.

Important objectives for 2014

1. Launch of a new prospective randomized clinical trial: A prospective Randomized multicenter study comparing horse Antithymocyte globulin (hATG) + Cyclosporine A (CsA) with or without Eltrombopag as front-line therapy for severe aplastic anemia patients (**RACE Study**). This study has been approved by EBMT Clinical Trials Unit and EBMT will be the sponsor. There will also be an important scientific component to this study, examining for clonal events with SNP-A karyotyping and somatic mutations using high throughput DNA sequencing.
2. Establishment of a PNH group within the SAAWP to lead on new proposals, such as standard conditioning protocols for AA/PNH HSCT, and the use of IST in PNH.
3. Comparative retrospective and observational studies of ATG versus alemtuzumab based conditioning for HSCT in SAA.
4. Plans for an EBMT/ESH book on Bone Marrow Failure disorders, both acquired and constitutional.
5. Combined Educational meeting of the SAA and Infectious Diseases Working Parties, with sessions on SAA, PNH and infectious disease issues relating to BMF, in late 2014.
6. Evaluation of alternative donor HSCT in patients failing IST and lacking a suitably matched BM donor, with proposals for a uniform EBMT protocol each for cord blood and haploidentical HSCT for SAA.

Judith Marsh
SAAWP Chair

Carlo Dufour
SAAWP Secretary



Autoimmune Diseases Working Party

Chair: Dominique Farge-Bancel

Introduction

During the year 2013, the EBMT ADWP members have continued to develop and enhance their ongoing clinical activities, educational meetings and research collaborative programs in the field of hematopoietic stem cell therapy (mostly autologous) and have enlarged their interest to stem cell therapy with the potential use of mesenchymal stem cells for Autoimmune Diseases (AD) at 3 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, including coordination of biobanking and immune reconstitution guidelines.
2. at the European level, by sustained interactions with respective AD scientific societies and research groups focused on scleroderma (SSc) (EULAR), lupus (Eurolupus), Inflammatory bowel disease (ECCO), and multiple sclerosis (ECTRIMS).
3. at a global level, by achieving common retrospective collaborative studies with the US (CIBMTR), Australia and Brazil and starting collaboration with leading Chinese groups (Nanjing and others) on stem cell transplantation on Multiple Sclerosis, Diabetes and Scleroderma.

Major scientific successes

1. **Confirmed results from the ASTIS trial** (Autologous Stem Cell Transplantation International Scleroderma), this unique and pivotal open label phase III trial, with 156 patients enrolled from 2001 to 2009 to compare autologous HSCT versus intravenous pulse cyclophosphamide. The ASTIS study (submitted to JAMA) shows that patients with poor prognosis early diffuse cutaneous SSc may benefit from early intensive immunosuppressive treatment.
2. **The early results from ASTIC trial** (Autologous Stem Cell Transplantation International Crohn's Disease), a randomised controlled trial co-sponsored by EBMT and ECCO, which compares early versus late immunoablation and HSCT in Crohn's disease (CD) over 1 year, completed recruitment of 46 patients (publication submitted to the Lancet).
3. **The final results from ASTIMS trial** (Autologous Stem Cell Transplantation International Multiple Sclerosis (MS) with 21 patients included and 4 years of follow up to compare the autologous HSCT in 9 patients versus 12 treated by Mitoxantrone have shown that autologous HSCT is significantly superior to Mitoxantrone in reducing MRI activity in severe MS. These results strongly support further phase III studies with primary clinical endpoints (submitted to *Neurology*).
4. The first **ADWP prospective non-interventional study in Systemic Sclerosis SSc (NISSc)** launched in 2013, aiming at enrolling 50 SSc patients in 3 years, has actively recruited.



The most relevant activities in 2013

1. With over 1700 HSCT for AD reported to the EBMT registry, the ADWP database is the largest worldwide. We have accomplished the first EBMT and CIBMTR retrospective collaborative study on MS, which was awarded the Van Bekkum award in London EBMT meeting 2013. We have initiated a similar study with Systemic Sclerosis in light of our good collaboration.
2. The educational meeting in Sheffield with representatives from the former Cell Therapy Committee enlarged our clinical and scientific activities. This best attended of all EBMT WP educational meetings this year - enabled over 100 participants to share knowledge and common interest on autoimmunity and stem cell transplantation and led to further development in the activities of the ADWP in cellular therapy.
3. The enlargement of our scientific interest to Mesenchymal Stem Cells and the biobanking project.

Important objectives for 2014

1. **Clinical activities:**
 - Development of the accreditation in the field of HSCT and cellular therapy in AD, after 15 years of experience in relation to quality of multidisciplinary care and patient outcomes for this unique field.
 - To launch new prospective clinical trials using combining HSCT and cellular therapy and the ADWP EBMT prospective non-interventional study on HSCT for Multiple Sclerosis.
 - To include new centers from Brazil, China and Australia.
 - To enrol 15 patients in the ADWP EBMT prospective NISSC for Systemic Sclerosis.
 - Update or development data collection forms for SSc, SLE, MS, Crohn's and Diabetes in association with the REMEDY registry update.
2. **Education:**
 - One educational meeting and two business meetings.
 - Elaboration of Good Clinical Practice Guidelines for biobanking.
 - Update of the 2005 Recommendations for heart and lung transplant evaluation in AD patients candidates for HSCT in common with worldwide experts.

Dominique Farge-Bancel
ADWP Chair



Acute Leukemia Working Party

Chair: Mohamad Mohty

Introduction

Transplant activity for acute leukemia continues to increase worldwide. The ALWP objectives are (i) to organize high level accredited educational activities pertinent to acute leukemia (latest symposium held in Marseille in Nov. 2013); (ii) to design and support prospective clinical trials in the field of acute leukemia across member centres, (iii) to generate high quality retrospective studies related to acute leukemia management and therapy; (iv) to increase within the EBMT registry the quality of data pertinent to SCT; and (v) to generate guidelines pertinent to the management of acute leukemia. Of note, during 2013, the ALWP hosted several post doctoral fellows from Austria, China, Croatia and Italy who actively contributed to the ongoing studies portfolio. Currently, the ALWP activities are organized within 6 subcommittees (SC) focused on specific fields of interest: autologous SCT SC (leader: NC Gorin), Immunotherapy SC (leader: C. Schmid), Alternative donors SC (leader: F. Ciceri), RIC SC (leader: A. Nagler), Molecular markers SC (leader: J. Esteve), and the Developing centers SC (leader: S. Giebel). In 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. These results will hopefully have a significant impact in the field.

Achievements and Objectives

In the autologous setting, the value of auto-SCT was investigated in different ALL and AML risk groups, but also in long term survivors after auto-SCT. Despite a low number of procedures performed in the registry, auto-SCT remains a valid option in specific subgroup of patients.

The RIC SC conducted several registry based surveys and studies evaluating different conditioning approaches in different age groups and populations, but also the role of high dose TBI in the era of novel so-called reduced-toxicity conditioning approaches. We also looked at the role of consolidation chemotherapy prior to allo-SCT in AML patients. Other important studies related to prediction of transplant-related mortality and toxicity, were also performed with some promising findings and the possibility to use such predictors in routine practice.

In addition to transplant techniques, we conducted several studies assessing the impact of molecular markers and novel targeted therapies on transplant outcome. The exact role of specific markers on transplant results is being refined, especially in AML patients with normal cytogenetics. In ALL, the use of TKI, either during induction phase or post-transplant, resulted in a better outcome in patients with Philadelphia-positive ALL. Other ongoing studies are focusing on the impact of hypomethylating agents as salvage therapy after relapse.

In the field of alternative donors, the ALWP launched major studies evaluating important questions in this area, such as comparison of outcome of haplo versus other donor sources, the optimal conditioning for haplo, etc. Indeed, the year 2013 saw a widespread diffusion of haploidentical transplantation, mainly related to the use of high-dose post-transplantation cyclophosphamide, allowing for the infusion of non-T cells depleted haploidentical grafts, with an acceptable incidence of GvHD as compared to standard unmanipulated transplants from HLA-matched related and unrelated donors. The ALWP focused on refining the registry to include detailed information of unmanipulated T-cell repleted haploidentical transplantation for acute leukemia, with the aim to generate rapid and reliable data in this field.

Finally, we pursued the work on the impact of socio-economic, geopolitical and center-related factors potentially influencing results of allogeneic SCT. Interestingly, it appears that center experience is the most important factor predicting results of RIC-alloSCT for patients with acute leukemia, while in patients with ALL in CR1, non-relapse mortality after myeloablative allo-SCT was strongly related to Health Care Expenditure and the Human Development Index.

Mohamad Mohty
ALWP chair



Immunobiology Working Party

Chair: Andrea Velardi

Introduction

Haploidentical transplantation: over 15 years' follow-up of transplants of T cell-depleted hematopoietic stem cells showed satisfactory outcomes in adults and children and revealed that donor-versus-recipient NK cell alloreactions efficiently control leukemia relapse. Recent years have witnessed development of unmanipulated grafts combined with post-transplant cyclophosphamide, post-transplant rapamycin, or G-CSF-priming of donor bone marrow. All provide promising results (reviewed by A Velardi, Blood 2013;121:719-20.). Current IWP studies mainly focus on exploiting immunology to improve haploidentical transplantation outcomes.

Major scientific successes

The IWP's major scientific successes this year have been the 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 2013 we also started planning a non-interventional perspective study on the clinical impact of pre-transplant thymic output.

The first retrospective study (Principal Investigators: J. van Rood, A Velardi) 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, it will evaluate whether tolerance of non-inherited maternal antigens in sibling-to-sibling haploidentical transplants is associated with less GvHD/TRM as was reported in unrelated donor transplants (van Rood and coll. 2002).

The second retrospective study will attempt to identify immunological biomarkers that are predictive of clinical outcome after haploidentical stem cell transplantation, under diverse protocols (Principal Investigators: A. Bondanza, C. Bonini, A Toubert, A Velardi).

Fourteen Centers from Europe and elsewhere have agreed to participate in these studies and the transplant cohort comprises over 600 patients.

The non-interventional prospective study will assess the impact of donor vs recipient NK cell allo-reactivity in haploidentical hematopoietic transplantation under diverse (T cell depleted vs T cell replete) protocols (Principal Investigators: A Velardi, L Ruggeri).

Eleven 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 200.

Jointly with the PDWP and the ALWP, in 2013 the IWP designed another non-interventional perspective study to assess the impact of recipient pre-transplant thymic function (as evaluated by TRECs analysis) on outcomes after allogeneic Hematopoietic Stem Cell Transplantation (Principal Investigator: A Toubert). A feasibility survey has been sent out to European Centres in December 2013. The study is aiming at evaluating 800 patients with 2 year follow-up.

Relevant activities in 2013.

The IWP organized two Educational Events in 2013. The first was the IWP Session of the EBMT Annual Meeting (London). 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 third "Perugia classic" IWP Educational Meeting on 28-29 September 2013 that featured extremely well attended (100 participants) lectures on haploidentical transplantation and immune tolerance given by outstanding European experts.



Objectives for 2014

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, data analyses is currently under way.

With regards to the non-interventional perspective study on NK cell alloreactivity in haploidentical transplantation, in 2014 we will continue enrolling patients and start performing interim analyses.

In 2014 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 the IWP session of the 2013 Annual EBMT meeting (Milan) and the 4th "Perugia classic" educational event in Autumn 2014.

Andrea Velardi

IWP Chair

Infectious Diseases Working Party

Chair: Simone Cesaro

Introduction

The increasing availability over last three decades of new and more potent antibacterial, antiviral, and antifungal drugs, combined with the improvement of immunosuppressive strategies and the use of adoptive immunotherapy has allowed to improve the results of prophylaxis and treatment of infections after HSCT. Nevertheless, the infections remain the major causes of morbidity and mortality both in the early and in the late post-transplant period and their occurrence is favored. Since its foundation, the Infectious Disease Working Party performed epidemiological, retrospective, and prospective studies that gave important contributions to the knowledge of the more frequent infectious complications such as bacteremia, CMV infection, EBV infection, aspergillosis, viral respiratory infection as well as how to deal with rarer infections (pneumocystosis, toxoplasmosis, zygomycosis, HHV-6, BK virus infection). Moreover, the increasing number of transplant centers requires IDWP 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

IDWP is currently organized to conduct clinical research allocating part of its resources to a dedicated Data Manager and Statistician time at EBMT Office in Leiden. This change in its structure has permitted to finalize and publish several important studies in the last 2 years such as: the relationship between BK and virological response to cidofovir in patients with hemorrhagic cystitis, the response to rituximab-based therapy and risk factor analysis in post-transplant lymphoproliferative disease after hematopoietic stem cell transplant, a survey among EBMT centers on the environmental policy to prevent infections, the etiology and resistance pattern of bacteremias among adult and pediatric patients with cancer. IDWP is an active and important partner of ECIL (European Conference on Infection in Leukemia) since its foundation. In 2013, the guidelines regarding the topic discussed at ECIL 3 and ECIL 4 meetings have been published: European guidelines for prevention and management of influenza in HSCT and leukemia patients, European guidelines for diagnosis and treatment of human respiratory syncytial virus, parainfluenza, metapneumovirus, rhinovirus, and coronavirus, European guidelines for empirical antibacterial therapy for febrile neutropenic patients in the era of growing resistance, European guidelines for diagnosis and treatment of mucormycosis in patients with hematological malignancy, European guidelines on targeted therapy against multi-resistant bacteria in leukemic and hematopoietic stem cell transplant recipients. Noteworthy, the guidelines on prevention, diagnosis and therapy of invasive fungal infection prepared by the pediatric group of ECIL 4 has been accepted in the prestigious journal Lancet Oncology. Moreover, as part of ECIL 5 conference, held in Sophia Antipolis (Nice, France), on 20-21 September 2013, IDWP was involved in the organization of scientific program. Fifty-seven experts from 21 European countries met to discuss and prepare guideline on other important topics such as the prevention of Pneumocystis jirovecii infection, the management of viral hepatitis in hematology patients, and to update the antifungal prophylaxis and therapy guidelines. All contents of the ECIL 5th Conference will be available as slide format at EBMT website.



The most relevant activities in 2013

In 2013, IDWP completed the analysis regarding 2 important retrospective studies: the impact of CMV donor/recipient serostatus in the outcome of HSCT and the response to rituximab-based therapy for PTLT. Moreover, a survey on the adherence to international guidelines for prevention of infection caused by central venous catheter among EBMT centers was launched and the analysis of the data are ongoing. A project covering an important topic of the recent years is the incidence and mortality among EBMT centres by Gram negative bacteremia is due to start in February 2014. Educational initiatives have been traditionally a key activity of IDWP through its annual 2-day training course on infectious disease topics in SCT. In 2013, we changed the format and decided to perform a joint course with Transplant Complications and Quality of Life WP. The Joint Course was held in Barcelona, on 31 October-1 November and the program was focused on infection and non-infectious complications in patients with GVHD. In order to maintain the infectious topics more integrated with other initiatives of EBMT, this year the training course will be organized with the Severe Aplastic Anemia WP in Naples.

Important objectives for 2013

We are collaborating with EBMT Board to support a large, prospective, randomized, study, sponsored by Merck company, regarding the use of a new antiviral, letermovir, as prophylaxis of CMV infection in adult HSCT patient. This study is of paramount importance because could contribute to change the attitude of transplant centers to use pre-emptive versus prophylaxis treatment for CMV infection by drugs, orally available, such as letermovir or CMX001. 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.

Simone Cesaro
IDWP Chair

Inborn Errors Working Party

Chair: Andy Gennery

Introduction

The IEWP is dedicated to the improvement in outcomes of transplantation and gene therapy for inborn errors of metabolism including primary immunodeficiencies and metabolic diseases. We do this through exchange of ideas, transplant and gene therapy data, international collaborative retrospective studies, development of transplant guidelines and education and training of transplant physicians around the world. The IEWP has had a 2013 budget of €1,000 - 16,000 from the EBMT and €5,000 from CELL-PID. We have been sponsored by Medac, Therakos, Gentium, Eurocept International, Sanquin and Baxter for this year's IEWP autumn meeting, held in Leiden.

Major Scientific successes

Many IEWP publications over the last 25 years have defined the indication for transplant in inborn errors and led the way in which transplants for these conditions are undertaken. The activities, meetings and publications of IEWP are essential sources of information for transplant physicians dealing with these rare and difficult diseases. A network of contacts from the group responds to urgent clinical questions from transplant physicians around the world. The IEWP has participated in 8 retrospective studies during 2013, and representatives from the group have spoken at numerous international meetings.

Most relevant activities

Retrospective data on outcomes of transplantation using various HSCT cell sources for children with Hurlers syndrome were published by the group in 2013. The IEWP is PI on a major EU FP7 grant 'CELL-PID' and is promoting educational and training opportunities of the network. The IEWP 'Guidelines' document for transplantation of severe immunodeficiencies, updated in 2012 is freely available on the EBMT website (<http://www.ebmt.org/guidelines2012>), and remains a key reference source for those in the field.

The IEWP has organised 2 educational activities in 2013 and plans 4 more for 2014. The group is developing links with sister consortia, particularly the North American Primary Immune Deficiency Treatment Consortium (PIDTC).

Important objectives for 2014

We will publish further high impact retrospective studies (2-3 are already in the process of being published, and 1 is in press). We are strengthening collaborative working with other consortia dedicated to improve transplant outcome for inborn errors, including the PIDTC. To that end we are establishing a collaborative project between PIDTC and IEWP, and at least one joint retrospective study is being prepared for publication. Further IEWP-initiated prospective studies are being explored. We will be meeting to discuss the Europe-wide implementation of Newborn Screening for Severe Combined Immunodeficiency, to develop common approaches, data collection and treatment strategies.

Andrew Gennery
IEWP Chair





Lymphoma Working Party

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 120,000 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

During recent years consist in several retrospective registry studies which had significant scientific impact in the field of lymphoma treatment. From 2010 to 2013, the LWP has published 19 studies with a mean Impact Factor of >10.

Most relevant scientific activities in 2013

The most relevant scientific activities of the LWP comprise the conduction, completion, or preparation of 24 retrospective and 3 prospective non-interventional studies (involving 18 Principal Investigators from 10 countries). The LWP is no longer aiming at running formal clinical trials. The LWP's study activities resulted in the publication of 9 scientific papers with a cumulative Impact Factor of 66.653 in 2013. The most important papers reported (1) the first prospective study on rituximab maintenance after autoHCT in follicular lymphoma, Pettengel et al, JCO 2013;31:1624-30; (2) the first study on autologous HCT in enteropathy-associated T cell lymphoma, Jantunen et al, Blood 2013;121:2529-32; and (3) the first comprehensive on the outcome of patients with Hodgkin's lymphoma relapsing after autologous HCT, Martinez et al, Ann Oncol 2013; 24:2430-34. Another major achievement was the EBMT follicular lymphoma transplant consensus, a guideline project helping to define the indications for HCT in follicular lymphoma in the 2010s. This paper was coordinated by Silvia Montoto and published in Haematologica 2013;98:1014-1021.

Moreover, the 9th Annual LWP Educational Course held in Barcelona, Spain, 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.

For the second time we were able to present the Jian-Jian Luan Award for Lymphoma Transplant research during the LWP session at the London 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 Luca Castagna from Milan, Italy, for his research on haplotransplants in lymphoma.

Objectives for 2014

In Jian-Jian's spirit, however, we hope that we can achieve most of the planned important objectives for 2014, i.e. continue the numerous studies mentioned, attract and launch important new ones, publish the 10 scientific study currently in manuscript phase, and perform the 10th LWP Educational Course (October 16-17, Cyprus, Local Organizer Chara Kyriakou) even more successfully than before.

Peter Dreger

LWP Chair



Paediatric Diseases Working Party

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.
- Raise rare paediatric issues in Expert Workshops, view the available literature, grade the evidence, come to a consensus on the best practise treatment, to publish and to develop further cooperation and projects.

Major Scientific successes:

- Initiation of the international ALL SCTped 2012 FORUM trial. Study start in Austria on April 13th 2013. 20 countries will follow.



Main activities in 2013:

- The 4th training course for paediatricians and paediatric nurses on HSCT in children and adolescents was an interactive educational EBMT course[®], 23rd to 25th May 2013. The course explored the major issues on the impact of hematopoietic stem cell transplantation in children and adolescents. Many European leading paediatric haematologists/oncologists were included among the speakers; the course was addressed specifically to postgraduate/resident level young physicians and paediatric nurses. 100 physicians and 50 nurses participated. Participants presenting selected abstracts were awarded with grants covering registration and housing. Due to the decision “going to Eastern Europe” we could integrate many people from Romania in this meeting and initiate several cooperations and friendships.
- PDWP organised an Expert Workshop on ECP treatment in Vienna from January 17th to 18th in Vienna. The group explored the major issues on GVHD staging, indications and different techniques of ECP treatment after haemopoietic stem cell transplantation. A prospective study on the evaluation of ECP is in discussion, but the issue shows difficult to realize.
- From November 6th to 7th 2013 the EBMT PDWP PICU Expert Workshop took place in Vienna exploring the necessary links between the HSCT and the ICU ward. An abstract out of this consensus meeting was forwarded to the EBMT congress in Milan, further cooperation and projects will follow.
- The collaboration with the European Medicines Evaluation Agency (EMA) and the implementation of a network for paediatric research at EMA (EnprEMA) was continued. PDWP became 1st category member of this network.
- Two business meetings were held in 2013 to discuss projects, strategies and work plans for the next working period. The most important project developments were: the selection of the two candidates for the next PDWP chair election in 2014, the preparational works for the educational meeting in Bukarest and the PDWP meeting 2014 in Jerusalem, the finalization of several publications.

Objectives for 2014

- Continue and support the conduct of the ALL SCTped 2012 study (prospective, randomized, open multicenter multinational study for allogeneic HSCT in children and adolescents with ALL) in several countries.
- PDWP Meeting in Jerusalem (May 21st to 23rd): Scientific course for physicians and nurses in cooperation with the IE WP.
- Expert Workshop on Survivorship after HSCT in childhood or adolescence and Survivor Pass Tool.

Christina Peters
PDWP Chair



Solid Tumours Working Party

Chair: Francesco Lanza

Introduction

The STWP is dedicated to pre-clinical, translational and clinical studies of cell therapy for solid tumours, including autologous (auto) and allogeneic (allo) stem cell transplant (SCT), active and adoptive immunotherapy, lymphoablative therapy with expanded T cells. The existence of a dose-response effect in epithelial tumours has been explored in a number of studies; however, the role played by auto- and allo-SCT is still a matter of investigation. The benefit of high-dose chemotherapy (HDCT) in selected subgroups of patients has become clearer in germ cell tumor (GCT), some subgroups of breast cancer, renal cell carcinoma, soft tissue sarcoma, Ewing's sarcoma, and medulloblastoma.

The story of stem cell transplantation in solid tumors demonstrates the importance of adopting an internationally co-ordinated approach to the investigation of this treatment modality. There needs to be an increased emphasis on prospective trials that are statistically robust and have well defined criteria for patient selection. Only these will be able to demonstrate whether SCT, alone or incorporated into programs with novel therapeutic modalities, is worthwhile in patients for whom conventional treatments have often limited impact on survival.

Major Scientific Successes

Despite the great potential, cell therapy programs for cancer control still has a marginal role in the management of patients with solid tumors, although its use in the setting of melanoma and other malignancies seems ready for development as a routine therapy. This is due to limitations inherent to the technologies and products employed, and to the financial and structural burden that are associated with cell therapy. The STWP is performing a number of studies aimed at better investigating these hot issues. As far as GCT is concerned, recent retrospective data suggest that HDCT may be superior to conventional chemotherapy. To test this hypothesis, an international phase III randomised trial is planned. This study is widely known as the TIGER study and is a global investigator led collaboration

Main activities

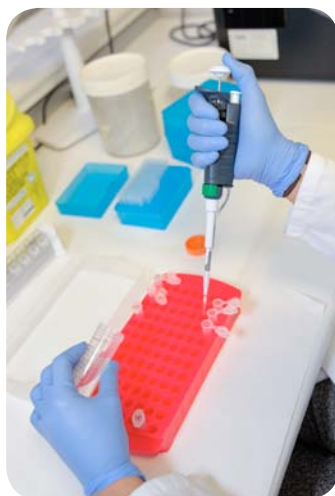
In 2013, STWP promoted the scientific activity of this Committee, prioritise new prospective and retrospective clinical trials in various solid tumours. Such trials provided a unique opportunity to understand, through mechanistic investigations, the role of cancer cells in the development and progression of some tumours, and improved the design of future trials. An increased awareness of ethical and biological issues regarding cancer stem cells was also achieved by STWP activity.

Objectives for 2014

Objectives for 2014: a phase II randomized, open-label neo-adjuvant study of standard chemotherapy regimen compared to high dose chemotherapy regimen with autologous stem cell transplantation in patients with triple negative breast cancer (BC) is planned in 2014. In BC, an international survey evaluating toxicity and efficacy of high-dose chemotherapy (HDC) and autologous SCT in a large cohort of BC will be released. Furthermore, a phase II randomized, open-label study of standard chemotherapy regimen compared to high dose chemotherapy regimen with autologous stem cell transplantation in patients with Metastatic BC will start in 2014.

Finally, HDCT plus autologous SCT using the TI-CE regimen (paclitaxel & ifosfamide followed by high-dose carboplatin and etoposide) will be compared to standard chemotherapy with Paclitaxel, Ifosfamide, and Cisplatin in the context of the “Tiger” study. Overall survival will be the primary endpoint of this study.

Francesco Lanza
STWP Chair



Chronic Malignancies Working Party

Chair: Nicolaus Kröger

Introduction

The CMWP is a disease-orientated working party covering diseases such as chronic myeloid and lymphocytic leukemia, myelodysplastic syndromes, myeloproliferative neoplasms, multiple myeloma, and amyloidosis.

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.

The CMWP is divided in 4 subcommittees:

1. Myelodysplastic syndromes.
2. Plasma cell disorders.
3. Myeloproliferative neoplasms.
4. Chronic lymphocytic leukemia.

Major scientific success

In 2013 the CMWP published 11 manuscripts in peer-reviewed journals such as Blood, Leukemia, PLOS one, BMT, BBMT, and American Journal of Hematology.

At international meetings members of CMWP presented results as oral presentation at 5 different events. 10 oral presentation were done at the EBMT Annual Meeting 2013, 4 during the ASH Annual Meeting, 2 at the International MDS Meeting and one at both EHA and International Myeloma Meeting.

This year ended with 2 ongoing prospective studies: RICMAC (recruitment closed) and Vidaza Allo Study (ongoing in Germany). In addition, 5 observational studies (NIS) are ongoing for 2014:

- Allogeneic SCT in amyloidosis
- CALM-study
- Iron overload in MDS
- Allogeneic SCT in CLL with del 17p
- 2. Generation TKI before allogeneic SCT in CML

More than 30 retrospective studies for MDS, plasma-cell disorders, CML, MPN, and CLL are also in ongoing status for 2014.

Most relevant activities

During this last year, 3 business meetings were held in Cologne, London and Lyon, as well as 2 sub-chair meetings (Leiden).

As for educational events, the CMWP has organised 3 events each one covering different topics. These were: "Role of molecular genetics and cytogenetics in MDS" in Cologne with 2 speakers, a two-day symposium on "Managing and integrating allogeneic stem cell transplantation in the management of myelofibrosis" in Hamburg with a faculty of 16 speakers, and last but not least "From bone marrow transplantation to composite tissue allograft", an educational meeting held in Lyon with 1 speaker.



Two consensus projects are still ongoing:

- Allogeneic stem cell transplantation for MDS (in collaboration with ELN).
- Allogeneic stem cell transplantation for myelofibrosis (in collaboration with ELN).

Important objectives for 2014

The CMWP is planning to perform several educational events in 2014 with the aim to cover interesting topics such as:

- New drugs and transplantation in CLL (01.02.2014 Leiden/NL).
- Role of transplantation in MDS (13.09.2014 Lille/France).
- Workshop on transplantation in multiple myeloma (to be announced).

Apart from that, we will also run the following projects:

- Start of collaborative initiative with CIBMTR (in MM and MDS).
- Launch new NIS: Ruxolitinib prior allogeneic SCT in MPN.
- Launch new (EBMT-labelled) prospective studies:
 - 5-Aza plus lenalidomide plus DLI for relapse in MDS after allogeneic SCT (phase-II).
 - Ruxo vs. allo SCT in myelofibrosis (phase-III).
- Start of consensus regarding autologous SCT in older patients with multiple myeloma (in collaboration with CIBMTR).

Nicolaus Kröger

CMWP Chair



Complications and Quality of Life Working Party

Chair: Rafael F. Duarte

Introduction

The CQLWP takes care of the scientific and educational activities of the EBMT in relation to transplant complications of a non-infectious nature. Our main goal is to combine expertise to provide the Society with a strong WP focused on transversal research on transplant complications, including early and long-term transplant-related complications, as well as GVHD, in collaboration with other WPs and Committees within the Society, and external collaborations with international groups.

The CQLWP organizes its activities through a scientific panel, including the WP Chairman, Rafael Duarte, the Secretary, Grzegorz Basak, and three additional leaders for our scientific subcommittees (SC), Hildegard Greinix, in the GVHD SC, Tapani Ruutu, in the early complications SC, and Nina Salooja as new leader of our long-term complications SC, all working in close collaboration with the team of the EBMT Leyden office. In addition, in 2013 the structure of the WP has been further strengthened with the appointment of Diana Greenfield as a Nurse Lead for transplant complications. Diana is a consultant nurse in survivorship and late effects, and a very active member of our WP. This new role of a nurse lead in a WP has been developed in collaboration with Elisabeth Wallhult, President of the Nurses Group, with whom we share the excitement for this post to become a pioneering model for the collaboration between the NG and other EBMT WPs.

The most relevant activities in 2013 and objectives for 2014

We are very happy about the big success of our first training workshop on assessment of chronic GVHD according to the NIH criteria, held at the EBMT 2013 congress in London. This quick response indicates a high level of interest and need on this topic, which will remain a priority in 2014, with the preparation of the second edition of these criteria, in which several members of our WP are participating as overseas experts. The educational highlight of 2013 was our course on Clinical Management of Infective and Non-Infective Complications of GVHD, held in Barcelona on October 31st – November 1st, a joint initiative of the CQLWP and the IDWP. This was the first EBMT wide course on GVHD in many years, and was not only a big success from an educational perspective with more than 70 participants, but also financially, as it provided over 15,000 Euros net benefit for the CQL and ID WP. In 2014, we have organized a new educational course and business meeting, across the scientific scope of our WP, which will be hosted by Grzegorz Basak in Warsaw, Poland, October 23rd – 25th.

One of the main developments that became a reality for the CQLWP in 2013 was the establishment of a joint EBMT-NCI Task Force on chronic GVHD and survivorship issues after transplant, in collaboration 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. Our formal first



research collaboration started in 2013, with an initial study carried out in parallel in all EBMT allogeneic transplant centers, as well as in America through the CIBMTR, which was presented at the CQLWP Session at the EBMT 2013 in London, and has also been recently published in Bone Marrow Transplantation. From this initial study, a prospective collaboration has been organized by the Task Force, and a NIH R21 research grant has been submitted in 2013 to continue this research collaboration both in US and EBMT centers. Special gratitude goes 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.

We have also seen major contributions from the WP this year in the fields of early and late complications after transplant. A number of important studies have been published in 2013, showing us data on the increase of suicide and accidental deaths in transplant recipients, paternity wishes in long-term survivors and the impact of ongoing GVHD in fertility and azoospermia. Also, additional studies have been completed and presented in abstract format, such as our EBMT experience on the outcome of hematopoietic cell transplantation in recipients of solid organ transplants, or the occurrence of donor type secondary leukemia after allogeneic transplantation. A particularly important event in early transplant complications in 2013 was the approval and marketing authorization in October in Europe of Defibrotide for the treatment of severe hepatic veno-occlusive disease. On request of the EMA, and in collaboration between the EBMT and Gentium, the CQLWP will launch in 2014 an exciting prospective observational registry to collect safety and outcome data in patients diagnosed with severe VOD treated with Defitelio®.

A good number of studies are coming to the end of their cycle, and many new proposals on transplant-related complications have been recently launched or will be launch this year in 2014: an EBMT assessment of the metabolic syndrome, a study on long-term sexual function, a new proposal to investigate the prevalence and outcome of pregnancy among transplant survivors, a study on current practice in growth hormone treatment in transplanted children and adolescents, a survey on current practice of extracorporeal photopheresis in the treatment of GVHD, a prospective analysis of the association between uric acid and acute GVHD, or a prospective non-interventional study on second-line treatment of corticosteroid-refractory acute GVHD. I would encourage you all to attend our business meeting and WP Session in Milano 2014 to learn more about some of these important studies. Please, do feel invited to bring in your own proposals and ideas, and to get involved in the work of the CQLWP.

Rafael Daurte

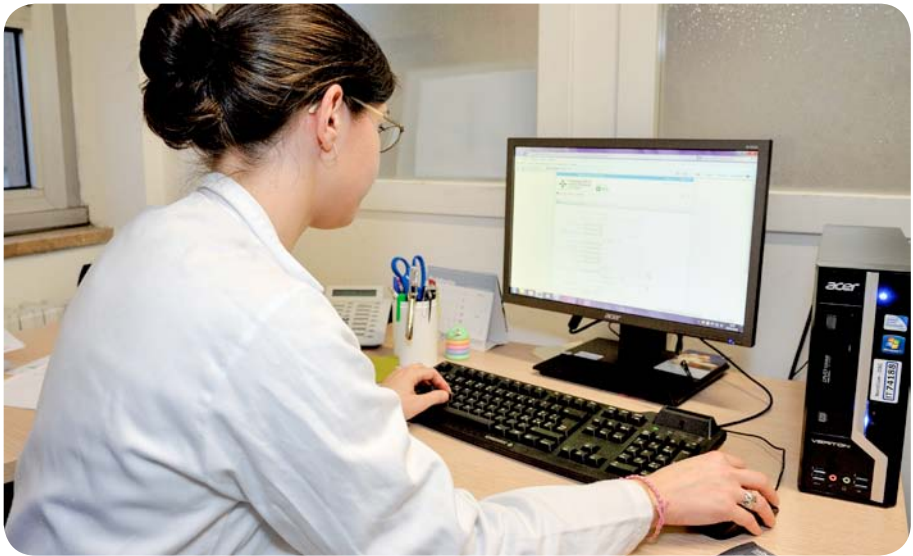
CQLWP Chair

Publications 2013 in peer-reviewed journals

WP	Title	First Listed Author	Journal	PMID
EBMT Activity Survey Office	Hematopoietic SCT in Europe: data and trends in 2011.	Passweg, JR, et al	Bone Marrow Transplant	23584439
Eurocord	Analysis of risk factors influencing outcomes after cord blood transplantation in children with juvenile myelomonocytic leukemia: a EUROCORD, EBMT, EWOG-MDS, CIBMTR study.	Locatelli F, et al	Blood	23926304
Eurocord	Outcome of patients with hemoglobinopathies given either cord blood or bone marrow transplantation from an HLA-identical sibling.	Locatelli F, et al	Blood	23692854
Eurocord & ADWP	New autoimmune diseases after cord blood transplantation: a retrospective study of EUROCORD and the Autoimmune Disease Working Party of the European Group for Blood and Marrow Transplantation.	Daikeler T, et al.	Blood	23247725
Eurocord, SFHI & SFGM-TC	Impact of donor-specific anti-HLA antibodies on graft failure and survival after reduced intensity conditioning-unrelated cord blood transplantation: a Eurocord, Société Francophone d'Histocompatibilité et d'Immunogénétique (SFHI) and Société Française de Greffe de Moelle et de Thérapie Cellulaire (SFGM-TC) analysis.	Ruggeri A, et al	Haematologica	23242594
Eurocord & SFGM-TC	Incidence and risk factors of EBV reactivation after unrelated cord blood transplantation: a Eurocord and Société Française de Greffe de Moelle-Thérapie Cellulaire collaborative study.	Dumas PY, et al	Bone Marrow Transplant.	22773124
Eurocord	Outcomes of transplantation using various hematopoietic cell sources in children with Hurler syndrome after myeloablative conditioning.	Boelens JJ, et al	Blood.	23493783
AAWP	Outcome of patients activating an unrelated donor search for severe acquired aplastic anemia.	Maury S, et al	Am J Hematol	23804195
AAWP	Allogeneic hematopoietic stem cell transplantation in Fanconi anemia: the European Group for Blood and Marrow Transplantation experience.	Peffault de Latour R, et al	Blood	24144640
AAWP	Syngeneic transplantation in aplastic anemia: pre-transplant conditioning and peripheral blood are associated with improved engraftment: an observational study on behalf of the Severe Aplastic Anemia and Pediatric Diseases Working Parties of the European Group for Blood and Marrow Transplantation.	Gerull S, et al	Haematologica	23894010
AAWP	Diagnosis of acquired aplastic anemia.	Rovó A, et al	Bone Marrow Transplant	23165495
ADWP	Long-term outcomes of hematopoietic stem cell transplantation for severe treatment-resistant autoimmune cytopenia in children.	Rabusin M, et al	Bone Marrow Transplant	23253561
ADWP	Autologous haematopoietic stem cell transplantation for systemic lupus erythematosus: data from the European Group for Blood and Marrow Transplantation registry.	Alchi B, et al	Lupus	23257404
ALWP	CMV serostatus still has an important prognostic impact in de novo acute leukemia patients after allogeneic stem cell transplantation: a report from the Acute Leukemia Working Party of EBMT.	Schmidt-Hieber M, et al	Blood	24037724

WP	Title	First Listed Author	Journal	PMID
ALWP & Eurocord	Comparison of outcomes after single or double cord blood transplantation in adults with acute leukemia using different types of myeloablative conditioning regimen, a retrospective study on behalf of Eurocord and the Acute Leukemia Working Party of EBMT.	Ruggeri A, et al	Leukemia	24005245
ALWP	Allogeneic hematopoietic stem-cell transplantation for acute myeloid leukemia in remission: comparison of intravenous busulfan plus cyclophosphamide (Cy) versus total-body irradiation plus Cy as conditioning regimen--a report from the acute leukemia working party of the European group for blood and marrow transplantation.	Nagler A, et al	J Clin Oncol	23980086
ALWP & CMWP	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	The impact of center experience on results of reduced intensity: allogeneic hematopoietic SCT for AML. An analysis from the Acute Leukemia Working Party of the EBMT.	Giebel S, et al	Bone Marrow Transplant	22773125
CMWP	Allogeneic Stem Cell Transplantation for Myelofibrosis with Leukemic Transformation: A Study from the Myeloproliferative Neoplasm Subcommittee of the CMWP of the European Group for Blood and Marrow Transplantation.	Alchalby H, et al	Biol Blood Marrow Transplant	24201159
CMWP	Improved PFS after autologous stem cell transplantation does not translate into better Quality of Life in CLL: lessons from the randomized EBMT-Intergroup study.	de Wreede LC, et al	Am J Hematol	24123244
CMWP	Comparison of allogeneic stem cell transplantation and non-transplant approaches in elderly patients with advanced myelodysplastic syndrome: optimal statistical approaches and a critical appraisal of clinical results using non-randomized data.	Brand R, et al	PLos One	0074368
CMWP	From nuclear to a global family: more donors for MDS.	Kröger N	Blood	
CMWP	Reduced intensity-conditioned allogeneic stem cell transplantation for multiple myeloma relapsing or progressing after autologous transplantation: a study by the European Group for Blood and Marrow Transplantation.	Auner HW, et al	Bone Marrow Transplant	23708704
CMWP	Autologous/reduced-intensity allogeneic stem cell transplantation vs autologous transplantation in multiple myeloma: long-term results of the EBMT-NMAM2000 study.	Gahrton G, et al	Blood	23482933
CMWP	Allogeneic stem cell transplantation for older advanced MDS patients: improved survival with young unrelated donor in comparison with HLA-identical siblings.	Kröger N, et al	Leukemia	22821073
CMWP	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	23164802
CMWP	Prophylaxis and treatment of GVHD: EBMT-ELN working group recommendations for a standardized practice.	Ruutu T, et al	Bone Marrow Transplant	23892326
IDWP	Response to rituximab-based therapy and risk factor analysis in epstein barr virus-related lymphoproliferative disorder after hematopoietic stem cell transplant in children and adults: a study from the Infectious Diseases Working Party of the European Group for Blood and Marrow Transplantation.	Styczynski J, et al	Clin Infect Dis	23771985

Status of clinical trials and completion of GCP inspection: a report of 2013 from the Clinical Trials Office



Prospective clinical trials continue to be important to the EBMT, which is fully committed to performing high quality academic prospective clinical trials, in order to improve patient care.

In 2013 the Clinical Trials Office (CTO) completed its first routine GCP inspection by the MHRA (UK Regulatory Authority) resulting in numerous findings that require implementing to further improve quality management of prospective clinical trials. The inspection and the subsequent aftermath took a very considerable effort and working hours from all staff.

At the end of 2013, there was 1 prospective clinical trial recruiting patients, 1 trial in the early set-up stages (RACE) and 1 pharmaceutical sponsored trial (CMV Prophylaxis) in the advance stage for which the CTO will provide recruitment and retention service. The two new trials will be open for recruitment in the summer of 2014. Two new trials which were supposed to start under EBMT (co)sponsorship are not going forward anymore.

There are 4 trials under analysis which should be published in 2014 (ASTIC, ASTIS, MMVAR and RICMAC,). The CTO made 2 publications in 2013 reporting the LYM1 trial (Pettengell, R. et.al. J Clin Oncol. 2013 May 1;31(13): 1624-30) and the CLL QoL (de Wreede, L et.al. Am J Hematol. 2013 Oct 9. doi: [Epub ahead of print]). Several oral and poster presentations were made at EBMT and ASH.

Trials in set-up stage:

- RACE (A prospective Randomized multicenter study comparing horse Antithymocyte globuline (hATG) + Cyclosporine A (CsA) with or without Eltrombopag as front-line therapy for severe aplastic anemia patients).
- CMV Prophylaxis (A Phase III Randomized, Placebo-controlled Clinical Trial to Evaluate the Safety and Efficacy of MK-8228 (Letermovir) for the Prevention of Clinically Significant Human Cytomegalovirus (CMV) Infection in Adult, CMV-Seropositive Allogeneic Hematopoietic Stem Cell Transplant Recipients).

WP	Title	First Listed Author	Journal	PMID
IDWP	Environmental prevention of infection in stem cell transplant recipients: a survey of the Infectious Diseases Working Party of the European Group for Blood and Marrow Transplantation.	Hicheri Y, et al	Transpl Infect Dis	23465046
IDWP, EORTC, ICHS & ELN	European guidelines for prevention and management of influenza in hematopoietic stem cell transplantation and leukemia patients: summary of ECIL-4 (2011), on behalf of ECIL, a joint venture of EBMT, EORTC, ICHS, and ELN.	Engelhard D, et al	Transpl Infect Dis	23363310
IEWP	Outcomes of transplantation using various hematopoietic cell sources in children with Hurler syndrome after myeloablative conditioning.	Boelens JJ, et al	Blood	23493783
LWP	Stem cell transplantation can provide durable disease control in blastic plasmacytoid dendritic cell neoplasm: a retrospective study from the European Group for Blood and Marrow Transplantation.	Roos-Weil D, et al	Blood	23203822
LWP	Hematopoietic stem cell transplantation in patients with lymphomatoid granulomatosis: a European group for blood and marrow transplantation report.	Siegloch K, et al	Biol Blood Marrow Transplant	23948061
LWP	Indications for hematopoietic stem cell transplantation in patients with follicular lymphoma: a consensus project of the EBMT-Lymphoma Working Party.	Montoto S, et al	Haematologica	23813647
LWP	Identification of prognostic factors predicting outcome in Hodgkin's lymphoma patients relapsing after autologous stem cell transplantation.	Martínez C, et al	Ann Oncol	23712545
LWP	Rituximab purging and/or maintenance in patients undergoing autologous transplantation for relapsed follicular lymphoma: a prospective randomized trial from the lymphoma working party of the European group for blood and marrow transplantation.	Pettengell R, et al	J Clin Oncol	23547078
LWP	Autologous stem cell transplantation for enteropathy-associated T-cell lymphoma: a retrospective study by the EBMT.	Jantunen E, et al	Blood	23361910
LWP	The outcome of reduced intensity allogeneic stem cell transplantation and autologous stem cell transplantation when performed as a first transplant strategy in relapsed follicular lymphoma: an analysis from the Lymphoma Working Party of the EBMT.	Robinson SP, et al	Bone Marrow Transplant	23771004
PDWP	Challenges and opportunities for international cooperative studies in pediatric hematopoeitic cell transplantation: priorities of the Westhafen Intercontinental Group.	Schultz RK, et al	Biol Blood Marrow Transplant	23883618
PDWP	Glutathione S-transferase gene variations influence BU pharmacokinetics and outcome of hematopoietic SCT in pediatric patients.	Ansari M, et al	Bone Marrow Transplant	23292236
STWP	Allogeneic stem cell transplantation for patients with advanced rhabdomyosarcoma: a retrospective assessment.	Thiel U, et al	Br J Cancer	24149176
CQWP	Increase of suicide and accidental death after hematopoietic stem cell transplantation: a cohort study on behalf of the Late Effects Working Party of the European Group for Blood and Marrow Transplantation (EBMT).	Tichelli A, et al	Cancer	23512286
CQWP	Paternity wishes in long-term survivors after allogeneic hematopoietic SCT. A study of the late effects working party of the EBMT.	Rovó A, et al	Bone Marrow Transplant	23292242
Graft Quality Committee	Individual quality assessment of autografting by probability estimation for clinical endpoints: a prospective validation study from the European group for blood and marrow transplantation.	Lanza F	Biol Blood Marrow Transplant	23988403



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).
- 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).
- 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)].
- RICMAC (Dose-reduced Versus Standard Conditioning Followed by Allogeneic Stem Cell Transplantation in Patients with MDS (Myelodysplastic syndromes) or secondary AML).

In summary it is hoped that the CTO will be successful in implementing the new prospective clinical trial that will be initiated in 2014 using the new processes that have been developed during 2010-2013. The second objective of the CTO is to complete and implement the Corrective Actions and Preventative Actions proposed to the MHRA for the new trial, bearing in mind that MHRA will re-inspect us within 6 months of enrolling a patient in the RACE trial. This will improve the level of quality inherent in the trials and also the efficiency of the operational processes.

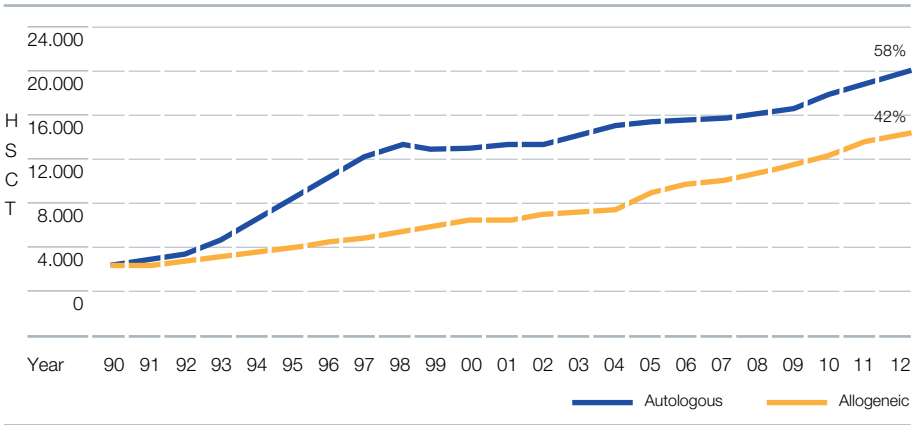
Alain Barrois
Leiden Clinical Trials Office Manager

Data and trends in haematopoietic stem cell transplantation in 2012



The EBMT activity survey has been conducted annually since 1990. 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% in 2012 when compared with 2011 suggesting that HSCT remains an increasingly important treatment modality in the era of targeted antibody and molecular therapy. In the 2012 survey, 661 from 680 centers in 48 countries reported 37'818 HSCT in 33'678 patients [14'165 allogeneic (42%), 19,513 autologous (58%)] to the 2012 survey. Use of allogeneic HSCT increases more than of autologous HSCT, e.g. in 2002 33% of the transplants were allogeneic and 67% autologous (figure 1). Main indications in 2012 were leukemias; 10,641 (32% of total; 95% of which were allogeneic); lymphoid neoplasias including Non Hodgkin lymphoma, Hodgkin lymphoma, and plasma cell disorders; 19,336 (57%; 11% allogeneic); solid tumors; 1,630 (5%; 3% allogeneic); and non-malignant disorders; 1,953 (6%; 90% allogeneic). The number of unrelated donor transplants increased by 5.4% from 7,799 to 8,224. HSCT for some indications continues to increase but not for others. The most significant increases in allogeneic HSCT were for AML in CR1 (12%) and for MPN (15%). For autologous HSCT there was a decrease in activity for acute leukemia (8% for AML and 22% for ALL) but an increase for plasma cell disorders by 7% and Non Hodgkin lymphoma by 4%. Autologous HSCT for autoimmune disease has increased by 50%.

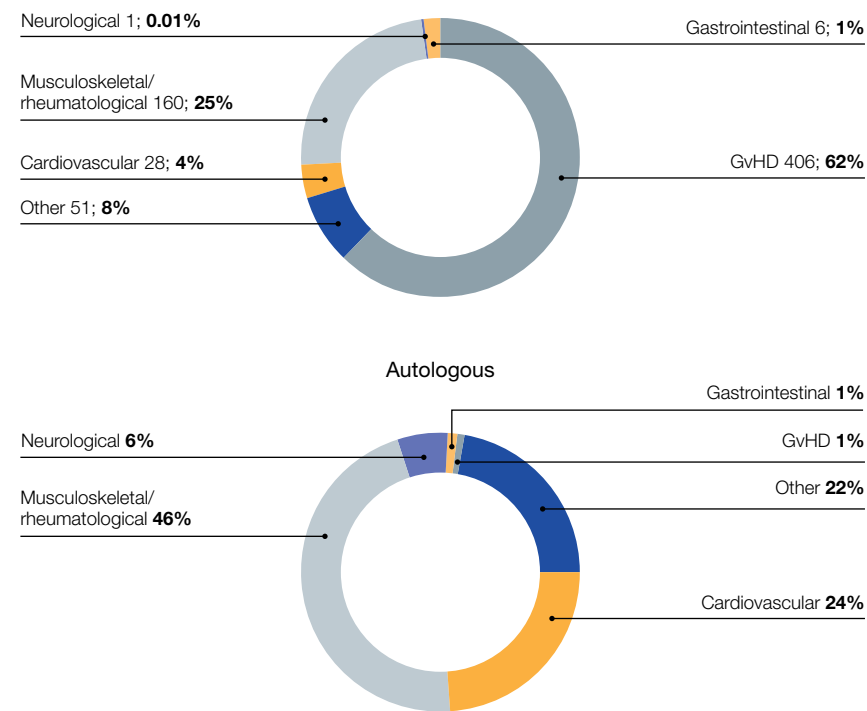
Figure 1. Increase in the numbers of allogeneic and autologous HSCT since 1990



Pediatric HSCT

For the first time in the 2012 survey data on the number of pediatric transplants were collected performed in either dedicated pediatric transplant centers or those centers performing transplants in both adults (>18 years of age at transplant) and pediatrics. Approximately twice as many pediatric patients were transplanted in dedicated pediatric centers as opposed to combined pediatric adult centers. It varies considerably by country but does not appear to be related to the size of the country but maybe to the policies within the country. 4,041 transplants, 2,877 (71%) allogeneic and 1,164 (29%) autologous, were reported in patients under the age of 18. Main indications for pediatric allogeneic HSCT is acute lymphoblastic leukemia (520; 26%), and primary immune deficiencies (315; 16%). For autologous HSCT it is solid tumors (504; 66% including 267 (53%) neuroblastomas) and lymphomas (111; 15%).

Figure 2: Main indications for cellular therapies reported to the dedicated survey in 2012, not including traditional hematopoietic stem cell transplants reported to the activity survey (preliminary data).



Novel cellular therapies

Since 2008 the activity survey office together with Ivan Martin, department of Biomedicine, University Hospital Basel, under the auspices of TERMIS EU, ICRS-EU and ISCT-EU, have been collecting data on novel cellular and engineered tissue therapies in Europe. In 2012, 135 teams from 27 countries reported 1,758 patients to the dedicated survey. The main indications were cardiovascular: 294 (266 autologous); musculoskeletal/rheumatological: 672 (512 autologous); neurological: 65 (64 autologous); gastrointestinal: 21 (15 autologous) and other: 296 (245 autologous). 410 patients received mesenchymal stromal cells for prevention/treatment of GvHD (377) or prevention/treatment of graft failure (33) (figure 2). The more detailed and completed survey on cellular therapies will be published later in the year.

The annual survey continues to provide data on use of hematopoietic stem cell transplantation throughout Europe and reflects to the community our current activity.

Helen Baldomero
EBMT Activity Survey Data Offices

Jakob Passweg



The Registry Upgrade and AGNIS projects: an overview of the main activities of the Registry Office

Data overview

A total of 406,099 patients and 478,594 transplants appear as registered in the EBMT Registry at the beginning of this year. Of these, over 34,000 transplants were registered during 2013, 78% directly by the transplanting centres and the rest by a mixture of national registry and EBMT staff.

Registry upgrade

The main activity for Registry staff during 2013 has been the Registry Upgrade project. The first months saw the development of detailed documentation pertaining to the enhancements necessary to serve the EBMT requirements which culminated in a ~700 page document. Remedy, given the zero footprint requirements, opted to build the Registry in a different platform than the one demonstrated to EBMT during 2012 and 2013 at the Annual Conference. Remedy has struggled to keep deadlines set for 2013 and early 2014, which have been postponed.

The Registry is currently waiting to assess a set of deliverables (forms, platform configuration, reports and dashboards). Depending on the completeness of these deliverables, the Registry will organise train-the-trainers courses. The intention is for a selected number of EBMT and national registry staff to learn and then go out to train the centres. A first attempt at doing this last December had to be aborted as the configuration of the system was insufficient for this purpose.

Within the context of the implementation of the new system, the EBMT Board has reviewed the rules concerning access to the Registry. Without jeopardising the work that national registries may need to do for Health Authorities, the EBMT will tighten the rules for providing free licenses, in favour of paying members or entities that provide obvious benefits to the EBMT.

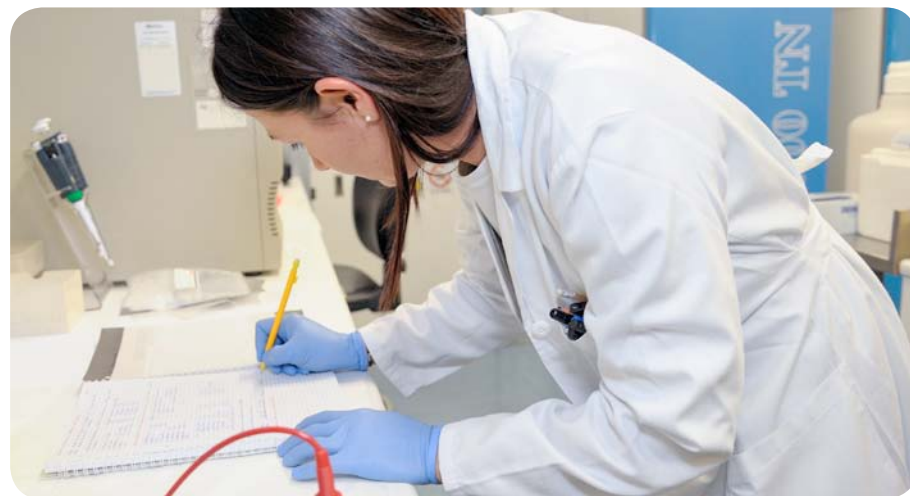
Data Sharing

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 focus during 2013 was to submit as much data as possible with the existing mapping. Difficulties were found – some due to changes to the AGNIS side. Despite this, data transfer is now possible, albeit still not with full capability.

Once the Registry upgrade has been finalised, the Registry will re-consider the reverse mapping with the CIBMTR, and resume the dialogue with Eurocord Registry for future collaboration in electronic data exchange. In the meantime, the Registry has suggested that Eurocord Registry write a project proposal to submit to the EBMT.



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. Regarding the collaboration with the CIBMTR assessing proposed changes to the Med-A/TED, the Definitions group submitted a document to the Scientific Council recommending some changes and rejecting others. This document was passed to the Registry Working Group for further processing (see below).

Registry Working Group

The Registry Committee, under the leadership of Per Ljungman, was asked by the Scientific Council to work on a review of the EBMT's data collection strategy in view of the upcoming changes to the Registry system. Face to face meetings and teleconferences have been taking place during 2013 and the results have been discussed in the Scientific Council. These discussions have resulted in a request to the Working Parties to submit suggested changes to the data collection forms by end of February 2014. As mentioned above, the Med-A changes recommended by the Definitions group within the CIBMTR collaboration, will be included here.

Registry Office move

The Registry office moved to new premises during the middle of 2013, with the usual –and not so usual (flooding)– associated problems. It is our pleasure to say that all problems have now been solved.

Carmen Ruiz
Head of Registry



The EBMT Nurses Group: towards an excellent patient care through international collaboration, education and science

As a division and established part of the main organisation, The EBMT Nurses Group (NG) plays an important role in the field of Haematology and Haematological Stem Cell Transplantation (HSCT) nursing. We have now nursing representation in all EBMT clinical centres, and we reach nurses and allied health professionals in over 50 countries worldwide.

The group is dedicated to improving the care of patients receiving HSCT by supporting and educating nurses and allied health care professionals. We recognize the need to strengthen the base for our practice through scientific evidence and promote nursing and collaborative research.

We have our own committees and through their hard and dedicated work, we made the following achievements in 2013:

Education

The Master Class initiative in Russia from 2012 continued and expanded to both paediatric and adult settings in Moscow and St. Petersburg. It was well received and the involved centres have been able to use the Master Classes as a starting point for their work towards new routines and improved patient care.

The Education Day prior to the Annual Meeting, organized by the **Scientific Committee**, has grown increasingly popular. During the last congress which was held in London, we had the highest number of participants ever and had to limit the number to 350 due to space restrictions.

A learning programme about Immune thrombocytopenia (ITP) was presented and is now available in our website.

The work with an educational tool for Venous Occlusive Disease (VOD) commenced and the result will be launched in Milan 2014.

A project called "Lulla's Journey" started and will be completed for presentation in Milan. It is a DVD for children, about a Stem Cell (Lulla) and it is way from cord bank through bone marrow to hospital discharge.

In May the **Paediatric Committee** and the Paediatric Diseases Working Party (PDWP) arranged their bi-annual course in Bucharest, Romania.

The Annual International Study Day was successfully repeated, this time in Barcelona in collaboration with the Spanish Nurses National EBMT Group.

Nurse members of the EBMT participated and presented in the Eastern Mediterranean Blood and Marrow Transplantation (EMBMT) meeting in Oman which was a great opportunity to network and plan for future collaboration with colleagues from the Middle Eastern countries.

EBMT NG was also involved in the 1st National Congress of GIIMA in Italy.

We continued our representation in the Skeletal Care Academy, an initiative regarding cancer-related bone disease.



Science

The Annual meeting continues to be our most important arena for presenting scientific results and 2013 was no exception with a large number of posters and oral sessions.

In London, the 5th Distinguished Merit Award was bestowed to Mairead NiChonghaile (Ireland). She received the award for her energy, passion, extensive knowledge and holistic approach serving the EBMT in its mission to improve care for patients and donors.

Research regarding Infection control is ongoing in collaboration with the Infectious Diseases Working Party (IDWP), and in this area, we can easily envision many aspects for future projects together.

The **Research Committee** is also involved in several studies about late effects together with the Complications and Quality of Life Working Party (CQWP). During 2013, the NG attained special recognition by formal appointment of a NG representative in the CQWP; something which we hope will serve as a model for other Working Parties in the coming year.

Communication

The **Communication and Networking Committee** has been responsible for delivering nursing news to the Newsletters. Together with the EBMT Communication Coordinator, they started late in 2013 to adapt the website to better suit the needs of nurses and the changes will be introduced early in 2014.

Collaboration

Apart from the collaborations mentioned above, the EBMT NG is represented in the Educational Committee and the revision of the JACIE standards.

Our collaboration with the National Groups/Forums has continued, and for 2014, we plan to further strengthen the ties to and include new groups and countries in our organisation and network.

With our colleagues in the European Oncology Nurses Society (EONS), we have started a joint educational project. We are also invited to present at the EONS9 meeting in 2014.

Together with MASCC, we have worked on a position paper about Basic Oral Care that will be submitted for publication in the beginning of 2014.

The characteristics of 2013 have been in line with our goals which were Science, Education and Collaboration. For 2014, we will set forth to establish a structure for our activities that will ameliorate our primary objective of improving patient care.

Elisabeth Wallhult
EBMT Nurses Group President

The Statistical Unit in 2013: Improving methodology in HSCT research

Studies

The main contribution of the EBMT Statistical Unit is in performing high quality prospective and retrospective studies in collaboration with 11 EBMT Working Parties. The EBMT statisticians are responsible of the adherence of the study to the methodological requisites of good scientific research projects. As such, statisticians are involved in planning, analysis, interpretation of results, and scientific communication. In 2013 they contributed to a total of about 118 studies performed by the Leiden and Paris offices and to several EBMT clinical trials:

- 93 retrospective registry-based studies
- 15 observational prospective studies or surveys
- 10 interventional prospective trials

The EBMT statisticians authored 27 manuscripts published in 2013 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 2013 were:

- The first edition of the EBMT Statistics Course (Leiden, 22-24 May 2013).
- The publication of the updated EBMT Statistical Guidelines in Bone Marrow Transplantation (2013 Mar;48 Suppl 1:S1-37).
- The statistical events at the EBMT annual meeting in London:
 - Statistical Symposium.
 - Round Table.
 - Course in basic statistics.
 - Consultancy for participants.
- One educational session for data managers during the EBMT annual meeting.
- The statistical course at the ESH-EBMT Training Course on Blood and Marrow Transplantation.
- One oral presentation at the annual meeting of the International Society of Clinical Biostatistics, based on EBMT studies.
- Two statistical workshops 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 2013 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

EBMT ED, consolidating collaborations to deliver high-quality education

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 2013 the educational events listed in the table below were staged by the EBMT, its Working Parties, Nurses Group, and long-term collaborators.

Dates	Event	Location
1-2 February 2013	EBMT Chronic Malignancies Working Party Business Meeting	Cologne, Germany
25-28 April 2013	ESH-EBMT Training Course	Sicily, Italy
22-24 May 2013	EBMT Statistics Course	Leiden, The Netherlands
23-26 May 2013	4 th EBMT Training Course for Paediatricians and Paediatric Nurses on HSCT in Children and Adolescents: Interactive Educational EBMT PDs Course	Bucharest, Romania
25-26 May 2013	EBMT Chronic Malignancies Working Party Educational Course	Hamburg, Germany
13-15 September 2013	EBMT Inborn Errors Working Party Meeting	Leiden, The Netherlands
28-29 September 2013	EBMT Immunobiology Working Party Educational Course	Perugia, Italy
3-4 October 2013	JACIE Inspector Training Course	Barcelona, Spain
4 October 2013	EBMT Nurses Group / Spanish Nurses Working Group International Study Day	Barcelona, Spain
9-11 October 2013	EBMT Lymphoma Working Party 9 th Edition of the Educational Course on Stem Cell Transplantation in Lymphomas	Barcelona, Spain
31 October-1 November 2013	EBMT Complications and Quality of Life and Infectious Diseases Working Parties Joint Educational Course	Barcelona, Spain
22-23 November 2013	EBMT Acute Leukemia Working Party Meeting and Symposium	Marseille, France
29-30 November 2013	EBMT Autoimmune Diseases Working Party Educational Meeting - Including a joint symposium with the UK Scleroderma Group	Sheffield, UK

The third year of the 2011-2013 EBMT Strategic Plan saw the EBMT Education and Events (E&E) Unit further consolidate its role and increase working relationships with relevant actors within EBMT and beyond to deliver high-quality educational products to the EBMT membership. In addition to assisting with other events, the E&E Unit played an important role in the organisation of four Working Party and Nurses Group Educational Meetings, three of which took place in Barcelona, Spain, and one in Sheffield, UK, during the autumn period.

A further edition of the flagship Joint Training Course on Blood and Marrow Transplantation also took place in Syracuse, Italy from 25 - 28 April. The collaboration with ESH around this course and the EBMT Handbook on Haematopoietic Stem Cell Transplantation continues to bear fruit, with 6274 copies of the latest edition distributed during 2013. A Turkish-language translation of the Handbook was also finalised and the possibility of translation into further languages is a subject that will be studied further in the near future.

EBMT Annual Meeting - London 2013

The 39th EBMT Annual Meeting was held in the ExCel London, UK, from 7 – 10 April 2013. The congress attracted over 4,200 active participants from 80 countries. The scientific and educational quality of the meeting was extremely high, thanks to both a meticulously scheduled programme by Congress President, Jane Apperley, and her Local Organising Committee and a record 1,244 abstract submissions.

In addition to the scientific programme, participants also enjoyed a spectacular gala dinner at the London Guildhall – a fitting end to a very successful congress. EBMT is also very grateful to its Annual Meeting symposia organisers, sponsors and exhibitors, who participated in many different ways to the success of the event.

Enric Carreras
Chair of the Educational Committee

Standards and Accreditation - making advances in the international BMT community

Since 2000, the Joint Accreditation Committee-ISCT & EBMT (JACIE) has received 311 applications from centres in Europe and beyond and 360 inspections (first-time and reaccreditation) have been performed. Over 220 have achieved accreditation at least once with practically all centres repeating the experience after their initial accreditation.

In 2013, **57 applications** (26 first-time and 31 reaccreditation) were received and **57 inspections** (33 first-time and 24 reaccreditation) were performed. **43 accreditations** (27 first-time and 16 reaccreditation) were awarded.

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

The review process to prepare the 6th edition of the **Standards** commenced in June 2013 and was followed by 4 months of work by the members of the FACT-JACIE Standards sub-committees via regular teleconferences. The 6th edition of the Standards is expected to be published in late 2014.

Three **Inspector Training courses** with a total of 51 participants were run either on the initiative of national societies groups with JACIE support (Paris, January 2013 and Israel, October 2013) or directly by JACIE (Barcelona, October 2013).

In 2013, JACIE performed **2 Orientation Visits** to applicant centres on-site by JACIE staff.

In 2013, the JACIE web site recorded 11,209 unique visitors – 19% more than 2012. 11 newsletters were mailed during 2013 with an average open rate of 25%. In addition 12 other communications were issued concerning surveys or specific issues e.g. inspectors expenses announcements.

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

Eoin McGrath
JACIE Executive Officer

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

Figure 1. Number of initial applications per country



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.

Information and Communication



In 2013, our main focus was the launch of the new e-Newsletter based on a Content Management System (CMS) solution offering new design, layout and functionalities. With this new CMS, the e-Newsletter receives an increasing number of unique visitors, which reached 450 on our last issue of 2013. The January e-Newsletter has been read by 540 unique visitors.

The EBMT external website has remained the main gateway for the different target audiences interested in the scientific outputs, educational events, membership, etc. It has to be underlined that the presentation of the EBMT publications, studies and clinical trials on the Research section of the website has been enhanced enabling visitors to use filters to clarify their search. Besides, a “Nursing” section has been created to gather all the information the BMT nurses might need including materials (practical guides, learning programmes, videos, slide bank); educational events and scholarship; membership, etc.

The website statistics show that in 2013 there were more than 74,000 individual visitors (16% increase), who visited the website almost 148,000 times and viewed on average 3 pages per visit.

In addition, the EBMT launched its presence on the social media and created facebook and twitter accounts. In 2014, the Society's objective will be to attract more followers by posting more information and articles.

Moreover, in 2013, we started to work on the preparation of the 40th Anniversary of the EBMT. Most of the celebrations will take place during the 2014 Annual Meeting in Milan focusing on the scientific achievements in the field of HSCT these last 40 years and the people behind these achievements. The EBMT Communications Unit wants to acknowledge the work of Professor Norbert-Claude Gorin, Eliane Gluckman and Carole Charley and other EBMT members that voluntarily committed to the project providing very useful support and information.

EBMT participated in key conferences such as ASH and for the first time, APBMT. It was an outstanding opportunity to showcase the EBMT's activity to transplant physicians from the Asia-Pacific countries.

And of course, the EBMT continued with recurrent publications such as the e-Newsletter, Annual Report and regular communications about its activities.

Mélanie Chaboissier
Communications Coordinator

The Corporate Sponsors

Platinum - 100.000€



Gold - 50.000€



Silver - 20.000€



Bronze - 10.000€€



18 Corporate Sponsors supported the day-to-day work of the society in 2013

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.

Gentium supports the EBMT with Platinum Sponsorship since 2012. In 2013, GILEAD joined back the EBMT and became our Gold Sponsor.

The EBMT launched three major projects in 2013 in partnership with the Industry:

1. The Registry Upgrade project.
2. The development of Studies.
3. The development of Educational Events.

Industry Meeting in EBMT 2013

The Industry Meeting, a one-hour meeting between the EBMT and our Corporate Sponsors was organised during the Annual Meeting in London, with a specific focus on four key subjects:

- The EBMT Strategic Plan: outcomes for 2012 and highlights for 2013.
- The EBMT Activity Survey: key figures for 2011 with 649 Centres participating.

The updates on EBMT major projects presented last year and developed in partnership with the Industry, among which the presentation of an example of a EBMT Study run in collaboration with the Industry (MEDAC) presented by Prof. Christina Peters, Paediatric WP Chair.

Focus on the Registry Upgrade Project

The Board of the EBMT acknowledged that the EBMT Registry represents a key tool for the scientific research and regulatory activities of the Society and its partners. Together with a consortium of partners including national outcome and donor registries, the Board agreed in 2012 **to update the Registry System with state-of-the-art software to enable EBMT and its partners to meet future data collection and retrieval needs.**

This project has been made possible thanks to the support of National Registries, Association, Hospitals and institutions and many sponsors such as DKMS with a key financial support. We also would like to thank Miltenyi Biotec for its financial support to the implementation of the New Registry. Its grant will be dedicated to the training sessions to Data Managers and National Registries to make the New Registry a real success in 2014.

Sandrine Ehrmann
Marketing & Fundraising Coordinator



EBMT financial highlights

In 2013, 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 have been reduced around 10% in relation of 2012.

Although EBMT is not required by law to have a third party audit the financial statements, EBMT has started to introduce mechanisms of control, accountability and transparency in order in the future to be fully audited. For that reason, EBMT has prepared in cooperation with the auditors Ernst & Young an internal control framework. This internal control framework is almost fully completed and implemented in the organization but still some work should be done in 2014 with the objective to further 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.

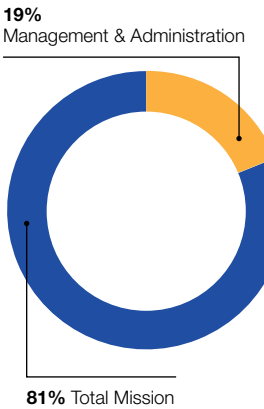
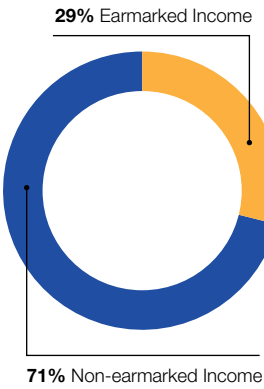
In that direction, EBMT works to assure its non-earmarked income (Membership, Sponsoring, Annual Meeting) in order to cover structural cost (Registry and Management) and launch non-commercial academic retrospective studies and educational activities through our Working Parties network. The earmarked 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 2013 EBMT has maintained 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 - 166K€ due to decreased funds for Studies, membership outstanding payment fees and JACIE change of accounting principles. EBMT is closing the year with a total expenses of 3.070K€ and a total Income of 2.904K€.

The Registry Upgrade project has received very generous donations from our partners (480K€) which will allow EBMT to cover part of the Registry Upgrade investment project. The complexity and delay of the project has caused an increase of internal and external resource of EBMT Staff allocated to the project. EBMT is obliged to continue trying to raise the additional 171K€ necessary for the Registry Upgrade project in order not to further reduce the EBMT reserves which should be kept at the present level to maintain a healthy financial situation.

Fred Falkenburg
EBMT Treasurer



SOURCE of INCOME	2012		2013	
	in 1,000€	Final Income	Final Income	
Membership	408	13%	565	19%
Sponsoring	430	13%	470	16%
Annual Meeting	923	29%	983	34%
Others	109	3%	58	2%
Non-earmarked Income	1.870	58%	2.076	71%
Studies & CT & Education	1.039	32%	371	13%
Accreditation (JACIE)	314	10%	165	6%
Other Grants	10	0%	293	10%
Earmarked Income	1.363	42%	830	29%
TOTAL Income	3.232	100%	2.906	100%

HOW EBMT SPEND THE MONEY	2012		2013	
	in 1,000€	Final Expenses	Final Expenses	
Retrospectives Studies	1.064	32%	771	25%
Prospective Studies	566	17%	383	12%
Educational Activities	188	6%	236	8%
EBMT Registry	486	15%	497	16%
Accreditation Process (JACIE)	274	8%	301	10%
Nurses Activities	85	3%	51	2%
Committees Activities	3	0%	10	0%
Registry Upgrade	54	2%	251	8%
Total Mission	2.721	81%	2.500	81%
Management & Administration	629	19%	570	19%
TOTAL Cost	3.350	100%	3.070	100%

Net Result	2012		2013	
	Final Expenses		Final Expenses	
TOTAL Income	3.232		2.906	
TOTAL Cost	3.350		3.070	
TOTAL Surplus/Deficit	-118		-164	

BALANCE 2013 (financial situation at the of the year)	2012		2013	
	in 1,000-€€	in€ %	in€	%
Cash & equivalents	4.108	79%	2.263	50%
Other current assets	1.082	21%	2.275	50%
Total Net Assets	5.190	100%	4.538	100%
Earmarked funds	350	7%	287	6%
Non-earmarked funds	2.293	44%	2.247	50%
Other Reserves	135	3%	79	2%
Total Reserves	2.778	54%	2.613	58%
Current Liabilities	2.412	46%	1.925	42%
Total Liabilities and Reserves	5.190	100%	4.538	100%

EBMT Staff (at the end of the year)	2012		2013	
	FTEs	%	FTEs	%
Study Coordinator and Statisticians for Retrospectives Studies	14	37%	15	41%
Study Coordinator and Statisticians Prospective Studies	7,39	20%	6,14	16%
Educational Activities	1,50	4%	1,20	3%
Registry	7,80	21%	8,15	22%
Accreditation Process (JACIE)	2,00	5%	1,80	5%
Communication Coordinator	1,00	3%	0,85	2%
Fundraising and Membership Staff	1,30	3%	1,30	3%
Management Staff	2,67	7%	2,65	7%
Total Staff	37,67		37,37	

The EBMT Board, Committee Chairs and Board of Counsellors in 2013

Executive Committee

President Alejandro Madrigal - London, UK	President Elect Mohamad Mohty - Paris, France	Secretary Anna Sureda - Barcelona, Spain	Treasurer J.H. Frederik Falkenburg - Leiden, The Netherlands
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EBMT Working Parties Chairs, Nurses' Group President and Congress President

Aplastic Anaemia Judith Marsh - London, UK	Acute Leukaemia Mohamad Mohty - Paris, France
Autoimmune Diseases Dominique Farge Bancel - Paris, France	Chronic Malignancies Nicolaus Kröger - Hamburg, Germany
Immunobiology Andrea Velardi - Perugia, Italy	Inborn Errors Andrew Gennery - Newcastle-Upon-Tyne, UK
Infectious Diseases Simone Cesaro - Verona, Italy	Transplant-related Complications and Quality of Life after SCT Rafael Duarte - Barcelona, Spain
Lymphoma Peter Dreger - Heidelberg, Germany	Paediatric Diseases Christina Peters - Vienna, Austria
Solid Tumours Francesco Lanza - Cremona, Italy	Nurses Group President Elisabeth Wallhult - Göteborg, Sweden
	Congress President 2014 Marco Bregni - Milan, Italy

EBMT Committee Chairs

Education Committee Enric Carreras - Barcelona, Spain	Nominations Committee Anna Sureda - Barcelona, Spain
Nuclear Accident Committee Ray Powles - London, UK	Outreach Committee Elisabeth Benedek
CT2-EBMT Committee Hermann Einsele - Würzburg, Germany	Donor Outcomes Committee Joerg Halter - Basel, Switzerland
Statistical Committee Myriam Labopin - Paris, France	Cell Processing Committee Christian Chabbanon - Marseille, France
JACIE Alessandro Rambaldi - Bergamo, Italy	Registry Committee Per Ljungman - Stockholm, Sweden
	Cord Blood Committee Vanderson Rocha - Sao Paulo, Brazil

Board of Counsellors

Andrea Bacigalupo - Genova, Italy	Bob Löwenberg - Rotterdam, The Netherlands
Gösta Gahrton - Stockholm, Sweden	Mary Horowitz - Wisconsin, USA



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