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November 2016 Newsletter

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By Paul Martin, MD

Each year, CIBMTR leadership conducts a formal review of Working Committees (WC). Since 2014, we have given public commendations to committees with noteworthy performance. This year, the Advisory Committee recognized excellent performance by six WC: Acute Leukemia, Chronic Leukemia, Lymphoma, GVHD, Donor Health and Safety, and Infection and Immune Reconstitution. Why were these WC selected for this special recognition?

Criteria for the evaluation include the WC overview plan and portfolio activity profile, the impact and feasibility of proposals and studies, the conduct and effectiveness of the annual meeting based on surveys of attendees, and a variety of metrics to assess study progress and milestones. Milestones include protocol development, data file preparation, data analysis, manuscript preparation, manuscript submission, and publication. At least 80% of studies in the WC portfolio are expected to reach at least one milestone per year. The overall success rate from 2014 to 2016 is 69%. No more than 20% of the studies in a WC portfolio should be in progress for more than three years. The overall failure rate from 2014 to 2016 was 19%. At least 75% of WC study reports should be submitted as planned



in a given year. The overall WC success rate is 61%. Lastly, all manuscript writing should be completed within one year after data analysis is completed. From 2014 to 2016, 39 manuscripts took longer than a year to be completed.

The reviews and evaluations identified several characteristics of the WC that received commendations. All had a high level of WC member engagement and strong leadership, and all showed success in conducting studies likely to have high impact on clinical practice. The Acute Leukemia, Chronic Leukemia, and Donor Health and Safety WC were commended for transparency in explaining why certain proposals were not discussed during the annual meeting. The Acute Leukemia and Donor Health and Safety WC were commended for efforts to be inclusive in study participation by investigators. The GVHD WC was recognized for its success in working with investigators during the development of proposals before the BMT Tandem Meetings.

Between January 2015 and March 2016, the Lymphoma WC published reports in high-impact journals (Blood and the Journal of Clinical Oncology) while the Infection and Immune Reconstitution and Chronic Leukemia WC each had one publication in a high-impact journal (Blood and the Journal of Clinical Oncology, respectively). Additional WC with high-impact publications included Graft Sources and Manipulation (n = 3), Plasma Cell Disorders and Adult Solid Tumors (n = 2), Regimen-Related Toxicity (n = 1), and Primary Immune Deficiencies (n = 1). These reports were published in Leukemia (n = 2), Blood (n = 3), and the Journal of Clinical Oncology (n = 2). The Immunobiology Committee had a banner year for publications in high-impact journals (one each in the New England Journal of Medicine and JAMA).

From this year's review, CIBMTR leadership recognized the importance of publication in high-impact journals. The impact factor of a journal clearly differs from the impact of a study. The impact factor of a journal is influenced not only by the quality of the study but also by the breadth of the audience and the number of reports accepted for publication. The impact of a study relates to the conceptual advance or insight it provides for understanding disease pathophysiology or treatment. The CIBMTR WC are to be congratulated for conducting high-impact studies. With a more thoughtful approach, it should be possible to have a higher proportion of our studies published in high-impact journals so that we receive the recognition that our efforts deserve.

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[Graft Sources and Manipulation Working Committee](#)

Committee Leadership

Co-Chairs:

- [Sarah Nikiforow, MD, PhD](#), Dana Farber Cancer Institute – Pediatrics
- [Vanderson Rocha, MD, PhD](#), Churchill Hospital
- [Asad Bashey, MD, PhD](#), The Blood and Marrow Transplant Program at Northside Hospital
- [Miguel-Angel Perales, MD](#), Memorial Sloan Kettering Cancer Center – Adults

Scientific Director:

- [Mary Eapen, MD, MS](#)

Statistical Director:

- [Mei-Jie Zhang, PhD](#)

Statistician:

- [Andrew St. Martin, MS](#)

The Graft Sources and Manipulation Working Committee (GSWC) addresses scientific questions related to the comparative effectiveness of the three most commonly used graft types, quality, and manipulation. It is one of the most active and prolific committees of the CIBMTR. This committee has collaborated with other registries nationally and internationally, resulting in publications leading to practice changes with respect to graft choices when considering:

- HLA-matched sibling and unrelated donor transplantations for leukemia

- Use of T-cell depletion in reduced intensity transplants and pediatric cord blood transplants
- Selection of cord units
- Use of cord blood as a stem cell source for patients with hematological malignancies
- Choice of alternative graft source in patients with acute leukemia

Our primary collaborators are Eurocord and the Acute Leukemia Working Party of EBMT.

Committee membership is comprised of investigators of diverse backgrounds and experience in clinical transplantation and cell processing and manipulation, providing the opportunity for synergy in scientific interactions, stem cell technology development, and new ideas. The committee has a significant publication track record with 15 publications in the past 5 years, and several of the studies published in high impact journals were conducted jointly with Eurocord and EBMT. A full list of the GSWC's studies, including recent publications, is provided on the [GSWC Studies webpage](#).

The committee has several ongoing projects that address current issues related to donor and / or graft selection for allogeneic transplantation. In particular, several ongoing studies are looking at outcomes of T-cell replete haploidentical transplants with post-transplant cyclophosphamide, including identifying the best graft source and haploidentical donor as well as outcomes in older patients. The success of the committee is dependent on scientific interactions, new ideas, and active participation of junior and senior investigators. To learn more about our committee or to discuss ideas and new projects, contact one of the Committee Chairs or Scientific Director.

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[Primary Immune Deficiencies, Inborn Errors of Metabolism, and Other Non-Malignant Marrow Disorders Working Committee](#)

Committee Leadership

Co-Chairs:

- [Paolo Anderlini, MD](#), M.D. Anderson Cancer Center
- [Neena Kapoor, MD](#), Children's Hospital of Los Angeles
- [Vikram Mathews, MD](#), Christian Medical College Hospital
- [Jaap-Jan Boelens, MD, PhD](#), University Medical Center Utrecht, Pediatrics

Scientific Director:

- [Mary Eapen, MD, MS](#)

Statistical Director:

- [Soyoung Kim, PhD](#)

Statistician:

- [Kyle Hebert, MS](#)

The Primary Immune Deficiencies, Inborn Errors of Metabolism, and Other Non-Malignant Marrow Disorders Working Committee (PID-IEM-NMMD-WC) conducts clinical research on early and late outcomes following HCT. As many of the diseases are rare to ultra-rare, the patient population is small, so the best avenue to collect maximum information on BMT outcome for these diseases is to promote collaborative studies. PID-IEM-NMMD-WC collaborates with EBMT, Eurocord, rare disease registries, and individual highly specialized centers to report on transplant-related topics and further the field of knowledge in this area. The disorders covered by the committee may be broadly classified under the following categories: hemoglobinopathies, metabolic disorders, immune deficiency / dysregulation disorders, bone marrow failure syndromes, and hereditary disorders. The uniqueness of this WC is that we manage very different diseases. However, these diseases also have a lot in common; for example, patients are quite young, so the potential to affect their lives in a positive manner is magnified. Therefore we focus on areas quite valuable and not consistently achievable in other transplant settings, such as ways to preserve fertility and childbearing ability, so they can build a family of their own.

Committee membership draws investigators from diverse backgrounds, each with experience in transplantation for benign, often rare disorders. The uniquely different disorders and the common transplant goals provide a great opportunity

for synergy in scientific interactions and for members to bring forth new ideas. The committee has a good publication track record with 15 publications in the past 5 years, including 3 high-impact publications conducted jointly with Eurocord and EBMT. A full list of the PID-IEM-NMMD-WC studies, including recent publications, is provided on the [PID-IEM-NMMD-WC Studies webpage](#).

The PID-IEM-NMMD-WC has 11 ongoing studies. Each year, two to three new projects are selected during the BMT Tandem Meetings. The selection process includes input from the CIBMTR membership who score and prioritize studies based on interest and impact factors. As some of the disorders included within the purview of this committee face significant residual disease burden, even after a successful transplantation, studying late outcomes has special significance and is often the target of study proposals. We encourage active participation and new study proposals, discussion with the chairs as the success of the committee is highly dependent on this. In particular, we encourage the input of junior investigators. To learn more about our committee or to discuss ideas and new projects, contact one of the Committee Chairs or Scientific Director.

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[CIBMTR Trivia](#)

The CIBMTR fulfilled _____ requests for information and data this past fiscal year.

- A. 527
- B. 474
- C. 389
- D. 296

[Enter your answer online](#). If you answer correctly, you will be entered into a drawing to win a CIBMTR prize.

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[2017 BMT Tandem Meetings](#)

By Tia Houseman



The BMT Tandem Meetings - the combined annual meetings of the CIBMTR and ASBMT - are North America's largest international gathering of blood and marrow transplant clinicians and investigators, laboratory technicians, advanced practice professionals, transplant nurses, pharmacists, administrators, and clinical research associates since 1999.

Leading experts will convene at the Gaylord Palms Convention Center in Orlando, Florida, February 22-26, 2017, to present the latest developments in blood and marrow transplantation during the BMT Tandem Meetings. Scientific Program Chairs for the 2017 meetings are David Marks, MD (CIBMTR), and Marcel van den Brink, MD, PhD (ASBMT).

Visit the [2017 BMT Tandem Meetings website](#) to register and view additional conference details. The last day for general registration rates is January 25, 2017. After registering, take advantage of special conference guest room rates at a wide variety of hotels within the BMT Tandem Meetings room block. The last day to book your hotel is February 14, 2017. Remember to reserve your ticket to the Reception beginning on the Gaylord Palms Coquina Lawn then moving to Wreckers Night Club, just off the lawn, for dancing and a fun night to remember.

Questions regarding support opportunities at the 2017 BMT Tandem Meetings may be directed to [Sherry Fisher](#), Director of Advancement for the CIBMTR. For general information, please email bmttandem@mcw.edu.

We look forward to seeing you in Orlando!



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[MS Biostatisticians Contribute to the CIBMTR's Purpose](#)

A key component of the CIBMTR research activities is the support of the Master's-level (MS) Biostatisticians. MS Biostatisticians manage and coordinate the activities of the CIBMTR Scientific Working Committees. They also direct the progress of research studies. The MS Biostatistician assigned to a WC is responsible for the following:

1. Serves as primary statistician for the WC
2. Manages and coordinates the activities of the WC
3. Identifies potential eligible patients in a study
4. Describes patient population and outcomes
5. Performs statistical analyses
6. Assists in the preparation of scientific reports and manuscripts
7. Assists in the preparation of materials for presentation at national and international meetings

In addition to WC research studies, MS Biostatisticians are involved in the following areas:

1. Information requests: Respond to physician requests for information pertinent to patient care and requests from patients, media, and corporate partners for information about transplant activity captured by the CIBMTR registry.
2. Clinical trial planning support: Check feasibility, prepare datasets, describe patient population, determine outcomes, and perform statistical analyses.
3. Government reporting: Prepare data and analyses to support government deliverables under the SCTOD contract and annual reports for FDA Investigational New Drug protocols.
4. Collaborate on the development of program materials, including educational materials, training manuals, marketing materials, websites, and reports.
5. Collaborate with other CIBMTR functional areas and NMDP Operational departments to meet their research data and analysis needs.

The CIBMTR Statistical Center is comprised of 18 dedicated MS Biostatisticians (14 located in Milwaukee and 4 in Minneapolis) that work under the dual leadership of Waleska S. Pérez and Stephen Spellman, providing critical support to the CIBMTR's mission by successfully completing hundreds of observational and prospective clinical research studies and handling the Information Resource Program.

Milwaukee



Left to right, starting at top: Omar Dàvila, Khalid Bo-Subait, Kenny Hu, Andrew St. Martin, Michael Hemmer, Xiaochun Zhu, Kyle Hebert, Hai-Lin Wang, Jeanette Carreras, Naya He, Mingwei Fei, Min Chen, Heather Millard, Waleska Perez, and Alyssa DiGilio

Minneapolis



Left to right: Steve Spellman, Deidre Kiefer, Mike Haagenson, and Pintip Chitphakdithai

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FACT / CIBMTR Data Audit Collaboration

As of January 1, 2017, the Foundation for the Accreditation of Cellular Therapy (FACT) and CIBMTR will implement a collaborative program of data auditing designed to reduce duplicative efforts, enhance quality improvement efforts, and provide support to accredited programs. With this collaborative program:

- FACT clinical inspectors will no longer perform a data audit during the on-site FACT inspection. This will eliminate the need for data sheets to be prepared only for FACT inspectors and allow the clinical inspector to focus on adequacy of corrective actions and quality improvement.
- All verification of the accuracy of data against source data will be handled by the CIBMTR audit teams on-site according to their current practices and schedules. The current CIBMTR process will not change.
- The FACT / CIBMTR Data Audit Committee will review CIBMTR audit reports and corrective action plans to assess compliance with standards, implementation of effective corrective action, and improvements.
- Timeliness and completeness of data submission will also be assessed by the Committee using CPI reports from the CIBMTR indicating “in good standing”.

Programs submitting an Annual Report or Renewal Application to FACT after January 1, 2017, will find new questions related to this program on those reports. Programs that have already submitted renewal applications will notice there is no change in the upcoming inspection. Between now and January, there may be some overlap in processes.

CIBMTR audits will remain every four years as scheduled (unless you request and pay for an interim special audit). Your center will respond to these audits to the principal auditor as usual and according to the time-frames defined by the CIBMTR.

FACT will receive information from your center annually and manage the processes on an on-going basis, depending on the needs of the program. FACT on-site inspections will continue to occur every three years.

On-site, clinical FACT inspectors will have access to CIBMTR data audit information and reports. They will review documentation of internal data audits and implementation of corrective action plans. Ultimately, successful FACT accreditation will depend on satisfactory audit results.

If your center struggled with CIBMTR data audits in the past, you won't immediately lose your FACT accreditation. Initially, centers will be given a grace period to show improvement in critical field and random error rates. During this grace period, centers will be expected to learn from prior difficult audits, design appropriate investigations, implement effective corrective actions, and follow up to ensure the improvements are sustained. This new process is designed to help your center identify the issues that may be barriers to improvement and develop strategies to be successful.

For more information about the FACT / CIBMTR data audit collaboration, contact [Heather Conway, CQA \(ASQ\)](#), FACT Quality Manager.

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[Health Services Research Program Updates](#)

By Linda Burns, MD; Ellen Denzen, MS; Beth Murphy, EdD, RN; and Stephanie Farnia, MPH

Register for Patient-Centered Outcomes Research (PCOR) Webinar and Symposia

Our Patient-Centered Outcomes Research Institute (PCORI) supported project, Engaging Patients in Developing a Patient-Centered HCT Research Agenda, has several exciting upcoming events planned. Don't miss out – register today!

Upcoming Webinar:

- **Wednesday, November 16:** *Patient Reported Outcomes Research in Hematopoietic Cell Transplantation* will highlight the research of experts in the field: Heather Jim, PhD (H. Lee Moffitt Cancer Center and Research Institute); Bill Wood, MD, MPH (University of North Carolina Hospitals); and Bronwen Shaw, MD, PhD (Medical College of Wisconsin, CIBMTR – Milwaukee). [Register](#).

Upcoming Symposia:

Join us for the Second PCOR symposium on December 2, 2016, in San Diego, California! Following the First Symposium at the 2016 BMT Tandem Meetings, six Working Groups were established, focusing on patient-centered topics: Patient, Caregiver and Family Education and Support; Physical Health and Fatigue; Emotional, Cognitive and Social Health; Sexual Health and Relationships; Models of Care Delivery / Survivorship and Late Effects; and Financial Burden.

Tasked with identifying patient-centered questions that lend themselves to comparative effectiveness clinical studies, Working Groups will present the results of their deliberations for feedback at this symposium. A panel of patients and caregivers will also discuss their engagement in the Working Groups.

Title: *Prioritizing a PCOR HCT Research Agenda*

Date / time: Friday, December 2, from 5 to 10 pm (dinner served at 5 pm)

Place: Legends Room, Hard Rock Hotel, San Diego (across from convention center)
[Register](#)

Save the Date: The third (and final) PCOR symposium will be held during the 2017 BMT Tandem Meetings on Saturday, February 25, 2017, from 12:15 to 4:45 pm EST. Plan to attend to provide input into the proposed research agenda for patient-reported outcomes in HCT.

Palliative Care: An Unmet Need for Patients

We need your input! The delivery of effective palliative care is an unmet need for patients with hematologic diseases and those undergoing transplant. The Health Services Research Program, in collaboration with the ASBMT Palliative Care Task Force under the leadership of Dr. Effie Petersdorf, is conducting a survey of physicians regarding perceptions and availability / utilization of palliative care options for their patients to inform future work. Be sure to watch your email inbox in the coming month for the survey. Your input is invaluable in this critical area.

Health Care Costs and Utilization

Administrative claims databases provide information on costs and utilization, but

without outcomes data, any interpretation of the value of transplant and / or non-transplant therapy is limited. We have recently completed merging a Medicare dataset from the Centers for Medicare and Medicaid (CMS) with CIBMTR outcomes data to create a unique dataset for exploring the value of therapy for older patients with AML and MDS. Additional projects include utilizing Optum data linked to the National Death Index to explore costs, utilization, and outcomes for patients with a variety of diseases for which transplant is indicated.

Save the date: The HCT Value and Health Economics Special Interest Group (SIG), in collaboration with the Administrator's SIG, will be hold a combined session at the BMT Tandem Meetings on Friday, February 24, 2017, with an agenda that will appeal to everyone interested in addressing the numerous issues in quality and value that we're grappling with in HCT. Stay tuned for more details.

AML Webinar

An AML educational webinar to support optimal timing of referral for HCT consultation and address survey-identified knowledge gaps is scheduled in November. It will be case-based with plenty of time for audience questions. Please join us, and advertise the webinars to your trainees, faculty, and referring physicians and clinics. CME will be offered at no cost.

- **Thursday, November 17: *Therapeutic Options for Older Patients***
Moderator: Fred Appelbaum, MD
Speakers: James Foran, MD, and Laura Michaelis, MD
[Register](#)

Contact [Ellen Denzen, MS](#), Senior Manager of Health Services Research with questions.

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[Seven New Patient Summaries of CIBMTR Research](#)

By Jessica Gillis-Smith, MPH

Seven patient summaries of CIBMTR publications were posted on the [CIBMTR Patient Resources webpage](#) this year:

- [Transplant from half-matched donors may be as good as matched donors for people with lymphoma](#)
 - People were just as likely to be alive 3 years after transplant whether they had a matched sibling transplant or a haploidentical transplant.
 - Fewer people got GVHD after a haploidentical transplant.
- [More older people with non-Hodgkin lymphoma get transplant](#)
 - Transplant is a treatment option for healthy older people with NHL
- [Transplant may be better when it uses bone marrow compared to peripheral blood stem cells from unrelated donors](#)
 - At 5 years after transplant from an unrelated donor, people who got bone marrow had better quality of life than people who got PBSC.
People who got bone marrow:
 - Were more likely to be back at work.
 - Said they felt better emotionally.
 - Had fewer symptoms of chronic GVHD.
- [Transplant often affects family finances](#)
 - At 2 years after transplant:
 - Most patients and caregivers thought transplant affected their family finances a medium or large amount.
 - Half of patients who worked before transplant were back to work.
- [Younger adults with high risk multiple myeloma may benefit from allo transplant](#)
 - Allo transplant may help people with high risk myeloma or myeloma that comes back early after an auto transplant live longer without the disease.
 - Younger people and people with no myeloma signs or symptoms before allo transplant lived longer without the disease.
- [Comparing 2 types of blood and marrow transplant \(BMT\): Double cord blood and haplo-cord](#)
 - For both types of transplant, about half of patients were alive 1 year after transplant.
 - Patients who had a haplo-cord transplant had less GVHD, and the disease came back less often.
- [Transplant outcomes for children and young adults with chronic myeloid leukemia \(CML\)](#)
 - Transplant outcomes are similar in children and young adults with CML.

- Transplant works better with bone marrow from sibling donors.
- Taking TKIs before transplant doesn't affect how well transplant works.

Summaries are created through a collaborative process involving CIBMTR Consumer Advocacy Committee members; CIBMTR and NMDP/Be The Match Medical Writers, Communications Specialists, and Patient Education Specialists; and CIBMTR Scientific Directors. Developing these summaries is one of the main initiatives of the Consumer Advocacy Committee.

The [Consumer Advocacy Committee](#) was created in 2005 as a subcommittee of the Advisory Committee to communicate CIBMTR research results and data to the non-medical community and to provide patient and donor perspectives during the development of the CIBMTR research agenda. Many members have personal experience as a donor, recipient, or family member.

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[Our Supporters](#)

The CIBMTR is supported by Public Health Service Grant/Cooperative Agreement 5U24-CA076518 from the National Cancer Institute (NCI), the National Heart, Lung and Blood Institute (NHLBI) and the National Institute of Allergy and Infectious Diseases (NIAID); a Grant/Cooperative Agreement 5U10HL069294 from NHLBI and NCI; a contract HSSH250201200016C with Health Resources and Services Administration (HRSA/DHHS); two Grants N00014-13-1-0039 and N00014-14-1-0028 from the Office of Naval Research; and grants from our [corporate and private contributors](#).

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[Abbreviations](#)

Need an acronym defined? Review our [list of common abbreviations](#).

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Last Updated: 11/1/2016 9:22 AM

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