May 2017 Newsletter

Volume 23, Issue 2

Table of Contents:

Perspectives

By Robert Soiffer, MD

Ask five BMT experts, “What is the best approach to GVHD prophylaxis?” and you will undoubtedly get at least five different answers. Not only will the approaches vary, but the responses will likely be delivered with unshakable self-confidence. Unfortunately, “expert” opinion often falls short. They are, after all, only opinions. Usually prospective randomized trials represent the gold standard in determining optimal clinical strategies. Sometimes those randomized trials may be underway, but definitive answers are years off in the future. Sometimes randomized trials are just not feasible. Yet, doctors are still faced with the dilemma of rendering clinical decisions and devising treatment plans for patients who must receive immediate intervention. For decades, the CIBMTR has stepped into the void and shed light onto a host of controversies critical to the field. It has conducted multiple studies that have directly altered prevailing opinion on the superiority of one approach over another, influencing everyday clinical practice and impacting the lives of our patients. This is why I am so honored to have the opportunity to serve as the incoming Chair of the CIBMTR Advisory Committee.

The CIBMTR relies on the voluntary submission of de-identified patient, disease, and treatment variables from a vast array of transplant centers in the nation and around the world. Participating institutions are willing to devote their time and

effort because they know, as an organization, the CIBMTR will impartially and exhaustively analyze data to arrive at conclusions that will help their patients today and in the future. What is remarkable is that the CIBMTR engages investigators from transplant centers, both big and small, to promote inclusion and widespread participation in these analyses. Given the often fiercely held opinions among transplanters, the spirit of collaboration fostered by the organization is most remarkable.

Of course, every team needs leadership. As Scientific Director, Dr. Mary Horowitz has done more than any single individual to improve transplant outcomes by careful evaluation of real-life data. Now, Mary would likely not agree with the statement above because of her humility. She would state, correctly so in part, that she merely represents the dedicated team at the CIBMTR and BMT CTN and the multitude of cooperating institutions that provide data and samples. However, there is no doubt in my mind that the success of the CIBMTR and BMT CTN would not have been possible without her wisdom, vision, hard work, and leadership. No one represents “Team Science” more ably than her.

Now with Dr. Horowitz and her colleagues ably at the helm, I have asked myself exactly what should I be doing as Chair of the Advisory Committee, especially since they seem to have it under control. I was fortunate enough during this past year to observe Dr. Paul Martin in his role as Chair. I first met Paul while attending the ASH Annual Meeting as a fellow in Jerry Ritz’s lab. I distinctly remember him stopping by my poster and talking with me for ten minutes about my presentation. I was struck by his quiet even handed advice, praising the merits of my work, gently pointing out some shortcomings, and suggesting some future directions. Ever since then, I have marveled at Paul’s ability to quickly size up a clinical or scientific issue, separating the wheat from the chaff and pinpointing exactly what needs to get done. There are no shortcuts with Paul, and he always sets high standards. It is because of these attributes that he has helped to make CIBMTR even more successful than it has been in the past. So, I guess as new challenges arise in my new role in the coming years, I will have to ask myself, “What would Paul Martin do?” and I know that we will be on the right path.

Return to Top

Donor Health and Safety Working Committee

Committee Leadership

Co-Chairs*:

- Galen Switzer, PhD, University of Pittsburgh Medical Center
- Michael Pulsipher, MD, Children's Hospital of Los Angeles
- Nirali Shah, MD, MHSc, National Cancer Institute

Scientific Director:

- Bronwen Shaw, MBChB, MRCP, PhD

Ex Officio Senior Advisor:

- Dennis Confer, MD

Statistical Director:

- Brent Logan, PhD

Statistician:

- Deidre Kiefer, MPH
- Pintip Chitphakdithai, PhD

* The committee would also like to acknowledge the important contributions to the research focus and operations of the committee by Paul O’Donnell, MD, who ended his tenure on the committee in 2016.

The research priority of the Donor Health and Safety Working Committee (DHSWC), now in its twelfth year, is to understand the impact of donation on both related and unrelated hematopoietic stem cell donors. Several important retrospective and prospective studies over the past five years are increasing our understanding of the medical and psychosocial risks involved in marrow or PBSC donation and have changed, or are likely to change, clinical practice in the management of adult and pediatric donors.

Most notable in the past two years is the Related Donor Safety (RDSafe) study, supported by the DHSWC and an NIH R01 grant held by committee Chairs, Drs. Pulsipher and Switzer. This study examined many aspects of the donation process on donor outcomes, including severity of adverse events and donor quality of life. It has already led to a number of publications, and more data is forthcoming. Prior to the RDSafe study, no prospective study of a large cohort of related donors had been performed. A few examples of future analyses include a comparison of toxicities experienced by related versus unrelated donors as well as between pediatric related and adult related donors.

A follow-up CIBMTR survey of donor practice patterns has also been completed, leading to changes in international guidelines regarding the work-up and management of adult donors. Based on the important results from this survey, a parallel survey of donor practice patterns in pediatrics is now developed and will be circulated later this year. Other ongoing studies focus on the impact of second donations on donors, the quality of bone marrow harvest on transplant outcomes, and the utility of autologous blood donation in donors.

View planned, in-progress, and completed studies and publications on the Donor Health and Safety Working Committee webpage. Based on the 2015-2016 final review of the Advisory Committee, the DHSWC was given an "outstanding" productivity review.

The DHSWC is pleased to see a steady increase in attendance at the committee meetings over the past three BMT Tandem Meetings. We encourage participation from the transplant community, especially new members, in ongoing studies or through the submission of new proposals.

Return to Top

CIBMTR Trivia

How many people attended our 2017 BMT Tandem Meetings in Orlando, FL?

A. 3,491
B. 3,522
C. 3,649
D. 3,784

Enter your answer online. If you answer correctly, you will be entered into a drawing to win a CIBMTR prize.

Return to Top

Team Spotlight: Audit and Monitoring Team

The CIBMTR Audit and Monitoring Team, led by Senior Manager Deb Christianson, is comprised of ten staff members located in Minneapolis.

The audit team performs data audits at all participating transplant centers as part of the CIBMTR's overall data quality assurance program. While onsite, auditors compare data in source documents maintained at the transplant center with data contained in the CIBMTR Research Database. Audits are conducted at regular intervals to ensure the accuracy of data submitted to the CIBMTR Research Database. Each transplant center is audited once in a four-year cycle. In fiscal year 2017, the audit team will travel to 63 total centers, including 13 international centers. The overall goal of ongoing audits is to ensure the quality and accuracy of the database by identifying systemic and non-systemic errors in reporting, requesting corrective action for errors identified during the audit, and providing training to transplant center data management staff.

In addition to performing data audits at transplant centers, the audit team is involved in the forms revision process, works closely with CIBMTR Information Technology to continue improving FormsNet capabilities for the audit program, and is responsible for providing training resources to data management staff, including developing eLearning modules and managing the CIBMTR Forms Instruction Manual. A recent significant accomplishment was publishing new manuals to correspond with several new forms released in January 2017: the Post-TED (Form 2450), Post-HCT Follow-up (Form 2100), and CML disease inserts (Form 2012 and 2112).

The monitoring team currently monitors six clinical trials. The team works closely with the RCI BMT team, participating in the set-up of new clinical trials, developing the monitoring plan, and completing the monitoring for the trials. In addition to RCI BMT trials, the monitoring team monitors the BMT CTN 1202 trial and a sponsor-

initiated trial through a Seattle group. A significant monitoring accomplishment in 2016 was closing out the monitoring of two clinical trials: 09-Plex and 11-TREO.

Back row left to right:
Justin Peterson, Deb Christianson, Edna Eich, and Ashley Birch

Front row left to right:
Nalini Singh, Mandi Proue, Ariana Hendrickson, Kelli Basa, and Will Affield

Not pictured:
Kelly Newport and Lauren Wendland

Return to Top

2017 BMT Tandem Meetings

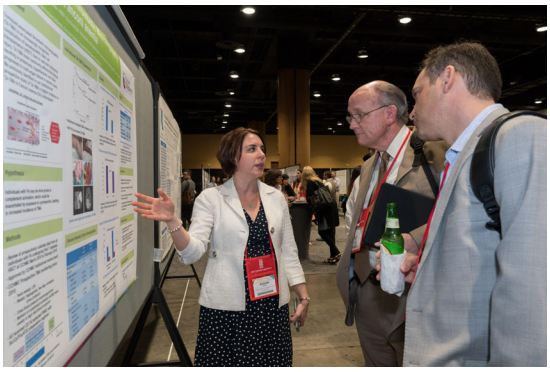
By Tia Houseman

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

View conference photos.

The 2017 BMT Tandem Meetings celebrated another record-breaking year with nearly 3,500 registered from more than 40 countries! This year the meetings were held February 22-26, at the Gaylord Palms Convention Center in Orlando, Florida. Countries with the highest representation, aside from the United States, were Canada, Brazil, Colombia, and Germany. Forty-eight percent of attendees were first time attendees. Many of the first-time attendees were abstract presenters and live stream attendees, with the rest falling under various categories such as exhibitors, general attendees, and invited faculty.

Program Co-Chairs Marcel van den Brink, MD, PhD, for ASBMT and David Marks, MBBS, PhD, for CIBMTR, along with the Scientific Organizing Committee, put together an excellent program for this year's meeting. New this year, we closed the BMT Tandem Meetings with three late breaking abstracts. Also included were 5 breakfast symposia and 4 lunch symposia, 5 plenary sessions, 9 concurrent sessions, 96 oral abstracts, 7 product theaters, 6 best abstract awards, 2 poster sessions, 15 Working Committee meetings, and 8 Meet-the-Professor sessions. In addition to an outstanding scientific program, parallel sessions were held for Clinical Research Professionals/Data Managers, BMT CTN Coordinators, BMT CTN Investigators, Pediatricians, Administrative Directors, Transplant Nurses, Pharmacists, and Nurse Practitioners with the addition of a new session this year entitled "Building a PCOR Collaborative Community".



The CIBMTR Distinguished Service Award was presented jointly to David Gómez-Almaguer, MD, and Guillermo J. Ruiz-Argüelles, MD. They have focused their efforts on making changes to BMT procedures to render them affordable for persons living in developing countries; employing these changes, they have grafted more than 1,000 persons with both malignant and non-malignant diseases. The two have worked together in two different institutions in Mexico. Their partnership has done more to advance the field of stem cell transplanttation in Mexico and Latin America than any other investigators in the region. The purpose of the Distinguished Service Award is to recognize individuals who have made outstanding contributions by promoting transplantation research and care in developing countries, by advancing the field despite unique challenges, by expanding the availability of transplantation, by disseminating research results as a way of improving outcomes and quality of life, or by collaborating with organizations to increase data exchange and research collaborations world-wide. *Revista de Hematologia* published an article about the award presentation titled *Presentation of the 2017 Distinguished Service Awards by the CIBMTR, February 24, 2017 (Hematologia Revista Mexicana 2017 January,18(1):33-35.)*

Richard E. Champlin, MD, of the University of Texas MD Anderson Cancer Center in Houston, TX, presented the Mortimer M. Bortin Lecture, speaking on *Use and Role of Allogeneic Hematopoietic Stem Cell Transplantation in the Era of Targeted Therapies*. The Mortimer M. Bortin Lecture commemorates the Founding Scientific Director of the International Bone Marrow Transplant Registry, now the CIBMTR. Dr. Bortin's foresight and dedication were critical to the development of the CIBMTR as a global resource for HCT research.

Jerome Ritz, MD, presented the E. Donnall Thomas Lecture "Modulating Immune Reconstitution after Stem Cell Transplantation". In honor of Dr. Thomas, the E. Donnall Thomas Lecture recognizes an eminent physician or scientist, either a clinician or investigator, who has contributed meritoriously to the advancement of BMT knowledge.

ASBMT presented the Lifetime Achievement Award to Hans Messner, MD, PhD, Professor of Medicine at the University of Toronto and Princess Margaret Cancer

Center. They also presented three Biology of Blood and Marrow Transplantation Editorial Awards, and seven ASBMT New Investigator Awards.

A new meet and greet opportunity was offered this year with a Networking Reception in the exhibit hall Thursday evening. Attendees enjoyed beverages and visited many of the 74 booths set up in the exhibit hall. The BMT Tandem Reception was held on Saturday evening on the Coquina Lawn and in Wreckers Night Club at the Gaylord Palms where attendees connected with colleagues and danced to the music of the band Blonde Ambition.

Save the date and watch for details on the 2018 BMT Tandem Meetings in Salt Lake City, Utah, in the coming months. Contact Sherry Fisher at slfisher@mcw.edu for information regarding support opportunities for next year's meeting.

On behalf of the 2018 BMT Tandem Meetings team as well as Scientific Organizing Chairs, Fred Appelbaum, MD, for the CIBMTR and Jerome Ritz, MD, for ASBMT, we look forward to seeing you at the Salt Palace Convention Center in Salt Lake City, Utah, February 21-25, 2018.

Return to Top

BMT Documentation Tools available in Epic EMR

By J.D. Rizzo, MD and Vincent Ho, MD

New standardized BMT documentation tools are now available in the Epic Foundation System for BMT programs who are using Epic as their electronic medical record (EMR) system. Over the last three years, a collaborative group including an Epic development team and physicians, data professionals, and IT staff members from nearly 20 transplant centers have created worksheets and tools for BMT programs, resulting in standardized flowsheets for the longitudinal scoring of acute and chronic GVHD and a revised BMT SmartForm available in the Epic Foundation System. These standardized tools are based on published criteria, such as the 2014 NIH Consensus Criteria for Chronic GVHD, and coordinated with CIBMTR reporting standards where relevant. Key functionality of these tools include automation for calculating the overall acute GVHD grade and global chronic GVHD severity scores based on the organ stages entered as well as easy migration of data from these tools into progress notes via the use of SmartPhrases.

Most of the data fields in the forms have been associated with standardized Common Data Elements (CDEs) previously defined by the CIBMTR in the NCI Cancer Data Standards Repository (caDSR) to ensure future interoperable data exchange. The CDEs are embedded in the acute GVHD flowsheet and BMT SmartForm and will be incorporated into the chronic GVHD flowsheet in the next few months.

If you are an employee of an Epic customer, you can register for an Epic UserWeb account and view this post containing more information about the updates. Please share this information with your clinical and EMR development staff.

The EMR User Group will continue work with the Epic development team to develop standardized content for integration in the Epic EMR. Our group recognizes that other large EMR systems exist, and our decision to collaborate with Epic was based on the large numbers of BMT centers already using this system, together with the shared enthusiasm from the Epic team. As such, we will strive to maintain transparency in the data elements the group has developed. While the CIBMTR will not share proprietary content specific to a given EMR, the data elements agreed upon for inclusion in these documentation tools can be made available as an Excel table upon request to contactus@cibmtr.org.

Return to Top

Clinical Research Professionals' Data Management Conference

By Sharon Meiers and Kay Gardner

The 2017 Clinical Research Professionals' Data Management Conference was held during the BMT Tandem Meetings in Orlando, Florida, and 225 individuals attended this three-day training and educational opportunity. Highlights from this year's conference included educational presentations on cytogenetics, cellular therapies, and chronic GVHD. Based on feedback from previous conference participants, the third day of in-depth training was added for the first time this year. Topics included Multiple Myeloma and Forms Journey. In 2018, we will again include a third day with in-depth training, and we hope to increase our attendance. To access presentations and related materials from this year's conference, visit the 2017 Clinical Research Professionals / Data Management Conference webpage.

Save the date for our 2018 conference, February 20-22 at the Salt Lake Palace in Salt Lake City, Utah.

Return to Top

BMT CTN Transition Period

By Amy Foley, MA

The BMT CTN, with its 20 Core and approximately 100 Affiliate centers, has enrolled more than 9,300 patients. The Network was established in 2001 and is at the end of its third grant cycle funded by the NHLBI and NCI. Last year the NHLBI shared welcome news that the BMT CTN grant would be renewed for a fourth grant cycle. This seven-year cycle starts July 1 and will include 18 Core Centers, several of which are anticipated to still be consortia comprised of two or more centers.

Clinical Trials: Open Enrollment

The BMT CTN encourages widespread transplant community participation in clinical trials. If your center is interested in participating, please visit the BMT CTN website.

There are 11 trials open to accrual, 1 released to centers. and 4 in development. The following BMT CTN trial was recently released to centers:

- BMT CTN 1502: Optimizing Cord Blood and Haploidentical Aplastic Anemia Transplantation (CHAMP)

BMT CTN Publications

There are 73 BMT CTN published articles, including 21 primary analyses. The following primary results manuscripts were recently published.

- BMT CTN 0601: Shenoy S et al. A trial of unrelated donor marrow transplantation for children with severe sickle cell disease. Blood. 2016 Nov 24; 128(21): 2561-2567, Epub 2016 Sept 13. PMC5123194. PubMed Link
- SWOG S0805 / BMT CTN 0805: Ravandi F et al. US intergroup study of chemotherapy plus dasatinib and allogeneic stem cell transplant in

Philadelphia chromosome positive ALL. Blood Advances. 2016 Dec 27; 1(3): 250-259. Journal Link
- BMT CTN 0901: Scott BL et al. Myeloablative vs. reduced intensity hematopoietic cell transplantation for acute myeloid leukemia and myelodysplastic syndrome. Journal of Clinical Oncology. 2017 Apr 1; 35(11): 1154-1161. Journal Link

About the BMT CTN

The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with NMDP/Be The Match® and The Emmes Corporation®. Together, these three organizations support all BMT CTN activities. The BMT CTN Steering Committee is currently under the leadership of Chair Steve Devine, MD, Ohio State University. Rick Jones, MD, Johns Hopkins, is Chair-Elect, and Helen Heslop, MD, Baylor College of Medicine, is Vice-Chair.

To get up-to-date information about BMT CTN studies, meetings, and news:

facebook.com/bmtctn

twitter.com/bmtctn (@BMTCTN)

Return to Top

Health Services Research Program: Health Care Costs / Utilization and PCOR

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

Health Care Costs and Utilization

Interested in working with health claims databases to measure costs and resource utilization in HCT? We are! Six criteria for using these datasets for cost analyses were recently reviewed by the Health Services Research Program in collaboration with the NMDP/Be The Match Payer Policy department (Preussler JM et al, Biol Blood Marrow Transplant 2016:1736-1746). We applied these criteria in an assessment of health care costs and utilization for privately insured patients in the US aged 50-64 years with acute myeloid leukemia who were treated either with chemotherapy alone or allogeneic HCT (Preussler JM et al. Biol Blood Marrow Transplant 2017 March 2; Epub). Adjusted mean one-year costs were $280,788 for chemotherapy and $544,178 for allogeneic HCT. Patients receiving chemotherapy alone had a mean of 4 hospitalizations, 52.9 inpatient days, and 52.4 outpatient visits in the year following AML diagnosis; patients receiving allogeneic HCT had 5 hospitalizations, 92.5 inpatient days, and 74.5 outpatient visits.

Administrative claims data 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 linked Centers for Medicare and Medicaid (CMS) Medicare data with CIBMTR outcomes data to create a unique dataset for exploring the value of therapy for older patients with a variety of diseases treated with HCT (Meyer C et al. Biol Blood Marrow Transplant; 2017, abstract 476). Our initial analysis of acute myeloid leukemia and myelodysplastic syndrome is in progress.

If you are interested in collaborating with the Health Sevices Research Program, view the Health Services Research webpage for further information.

Patient-Centered Outcomes Research (PCOR)

Our PCORI-supported project, Engaging Patients in Developing a Patient-Centered HCT Research Agenda, held its third and final symposium at the 2017 BMT Tandem Meetings. Six Working Groups outlined their final recommendations on HCT research questions that matter most to patients. Unique to this symposium, Working Group co-chairs, patients, and caregivers delivered joint presentations, providing unique and complementary perspectives. Research and funding opportunities within PCORI, BMT CTN, and CIBMTR were also presented, followed by a discussion of next steps in building an international collaborative PCOR community in transplantation. Stay tuned as we disseminate results later this year via webinars and publications.

New enduring activity: If you missed last Fall's webinar on PCOR in HCT that featured the research programs of Dr. Heather Jim, Dr. Bill Wood, and the patient-reported outcomes pilot project within CIBMTR as reported by Dr. Bronwen Shaw, you can view it on the Be The Match Clinical Education webpage. Viewing this online webinar, you are eligible to earn one CME hour for physicians, allied health professionals, and nurses.

Contact Ellen Denzen, MS, Senior Manager of Health Services Research with questions.

Return to Top

A New Patient Summary of CIBMTR Research

By Jessica Gillis-Smith, MPH

One new patient summary of CIBMTR publication has been posted on the CIBMTR Patient Resources webpage.

- Blood or marrow transplant can work well to treat B-cell acute lymphoblastic leukemia for older patients
  - Reduced-intensity BMT can work well for older patients with B-cell ALL.
  - For patients who had BMT when the disease was in 1st complete remission, almost half (45%) were alive 3 years later.

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.

Return to Top

CIBMTR on Facebook and Twitter

Like us on Facebook and follow us on Twitter to stay up-to-date with important news and events. We promote our publications, share important content from other organizations, and advertise our key meetings and events. Join us today!

facebook.com/theCIBMTR

twitter.com/CIBMTR (@CIBMTR)

Return to Top

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 HHSH250201200016C 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.

Return to Top

Abbreviations

Need an acronym defined? Review our list of common abbreviations.

Return to Top

Last Updated: 5/1/2017 3:03 PM

CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program®/Be The Match® and the Medical College of Wisconsin