February 2015 Newsletter

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Perspectives
By Paul Maris, MD

Every year in late September or early October, I am asked to provide performance reviews of employees who report to me. It’s not my favorite task, but it does provide an important opportunity for evaluation and reflection, and the process can lead to insights that enhance success. For the past two years, representatives of the CIBMTR Advisory Committee have conducted a formal annual review of each Working Committee. Three Working Committees received outstanding ratings in 2014: Immunobiology, Graft Sources and Manipulation, and Pediatric Cancer. All three of these Working Committees demonstrated high productivity through publications in high-impact journals. What factors contributed to the success of these committees?

The engagement and strong leadership of the respective Chairs and Scientific Directors of these Working Committees clearly contributed to the high level of performance in these committees. During the annual BMT Tandem Meetings, these Working Committees reviewed new proposals with robust discussion and careful consideration of a study’s potential impact. The Immunobiology Working Committee demonstrated outstanding performance in orchestrating a large and complex body of work with excellent results. Extensive updates were given for selected ongoing studies that required discussion. Critical consideration of
proposals by the Graft Sources and Manipulation Working Committee during the BMT Tandem Meetings led to important modifications in the plans for some studies. Performance of this committee has been strengthened by establishing strong working relationships with Eurocord and EBMT. Similarly critical discussions by the Pediatric Cancer Working Committee led to decisions not to approve two new proposals.

Comments from one committee noted that getting a proposed study accepted to move forward is increasingly competitive, which poses particular challenges for younger investigators. We encourage all Working Committees to include junior investigators in CIBMTR research studies with the mentorship of senior investigators. Ideally, the process of engaging younger investigators should begin when a new proposal is first formulated. The old adage, “See one, do one, teach one” in medical training is admittedly simplistic, but contains some truth, better stated as “See many, do many, teach many.”

Including junior investigators in research studies offers important opportunities for academic advancement and helps build the next generation that the CIBMTR will need to continue fulfilling its mission. The next generation of investigators will surely remember the relationships with their mentors as far more important than any individual publication. Likewise, when all is said and done, mentors are likely to derive more satisfaction from these relationships than from any list of publications.

Younger investigators can also learn new skills by paying close attention to presentations during the annual meeting and attempting to identify characteristics of proposals that generate interest, lively discussion, and strong support versus those that do not. Those lessons can then be taken to heart in developing a proposal that is more likely to be approved by the Working Committee.

On behalf of the Advisory Committee, I would like to congratulate all Working Committees for their excellent progress. I would especially like to thank Tom Shea, David Marks, Stela Davies, Michael Lill, and Joseph Pidala who worked with me as members of the Review Committee. We welcome any comments or suggestions that could be incorporated into the review process.

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### Plasma Cell Disorders and Adult Solid Tumors Working Committee

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<th>Yago Nieto, MD, PhD, Chair</th>
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<th>Jiaxing Huang, MS, MS Statistician</th>
<th>Parameswaran Hari, MD, MS, Scientific Director</th>
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Multiple myeloma is the most common indication for HCT in the US. The Plasma Cell Disorders and Adult Solid Tumors Working Committee strives to work with investigators from around the world to define the optimal utilization of transplant for not just multiple myeloma but other plasma cell disorders, such as light chain amyloidosis, Waldenstrom macroglobulinemia, plasma cell leukemia, etc. In addition, since 2014, adult solid tumors are also included within our focus.

Our committee includes Jingqing Huang, MS Statistician; Jennifer Le-Rademacher, PhD Statistician; Parameswaran Hari, MD, MS, Scientific Director; and Anita D’Souza, MD, Assistant Scientific Director. We currently have a great group of Chairs, including Amrita Krishnan, MD (City of Hope, CA); Yago Nieto, MD (MD Anderson Cancer Center, TX); Cristina Gasparetto, MD (Duke University, NC); and Tamer Mek, MD (Cornell University, NY). We also have 345 active members in our Working Committee. We have been fortunate to have commitment from outstanding previous Chairs, including David Vesole, MD, PhD (Hackensack University Medical Center, NJ); Donna Reece, MD (University of Toronto, Canada); Gustavo Milone, MD (Fundacion, Argentina); Angela Dispenzieri, MD (Mayo Clinic, MN); and Sagar Lonial, MD (Emory University, GA).

We have 10 ongoing projects that study outcomes of transplantation in germ cell tumors, light chain amyloidosis, Waldenstrom Macroglobulinemia, and multiple myeloma. Noteworthy accomplishments from this committee in the last 5 years include 17 peer-reviewed publications as well as 7 oral and 5 poster presentations at national and international conferences, including the American Society of Hematology, International Myeloma Workshop, and BMT Tandem Meetings. The Plasma Cell Disorders and Adult Solid Tumors Working Committee was recognized as an outstanding committee within the Clinical Outcomes Research Program of the CIBMTR in 2013.

This year we received 17 proposals, 7 of which will be presented at the BMT Tandem Meetings. We encourage all members of our Working Committee to actively participate in our committee, and we look forward to meeting everyone at the upcoming ASBMT / CIBMTR BMT Tandem Meetings in February.

Primary Immune Deficiencies, Inborn Errors of Metabolism and other Non-Malignant Disorders Working Committee

Shalini Sheng, MD, Chair
Harry Malech, MD, Chair
Jaap-Jan Boelens, MD, PhD, Chair
Paolo Anderlini, MD, Chair
Vikram Mathews, MD, Chair
Wensheng He, MS, MS Statistician

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. The committee takes advantage of the CIBMTR's large Research Database to study these outcomes. As many of the diseases are rare to ultra-rare, we promote collaborative studies with EBMT, Eurocord, rare disease registries, or 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 PID-EM-NMMD-WC is headed by six Chairs who are internationally recognized experts in the field. The current Chairs are Shalini Shenoy, MD (Washington University / St. Louis Children's Hospital, Missouri); Harry Malech, MD (NIAID-NIH); Jaap-Jan Boelens, MD, PhD (UMC Utrecht, Netherlands); Paolo Anderlini, MD (MD Anderson Cancer Center, Texas); Vikram Mathews, MD, MBBS (Christian Medical College Hospital, India); and Neena Kapoor, MD (Children's Hospital of Los Angeles, California). The Chairs are assisted by Statisticians Wensheng He, MS, Jennifer Le-Rademacher, PhD, and by Scientific Director, Mary Eappen, MS, MBBS.

The committee meets annually in person at the BMT Tandem Meetings, and several times during the year via teleconference calls. The mandate of the committee is to:

1. Ensure timely completion of projects
2. Assess priority areas and feasibility of proposed studies
3. Promote and develop the scientific agenda

Committee membership draws investigators from diverse backgrounds, each with experience in transplantation for benign and 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. Several of the high-impact publications were conducted jointly with Eurocord and EBMT.

The PID-EM-NMMD-WC has 12 ongoing projects. Two to three new projects are selected each year at the committee meeting held 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. The success of the committee is highly dependent on ongoing scientific interactions, new ideas, and active participation from both junior and senior investigators. Please contact one of the Chairs or the Scientific Director to learn more about the committee or to discuss ideas and new projects.

**2015 BMT Tandem Meetings on the Horizon**

*By D'Etta Waicolch, CMP*

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.

**Have you registered for the 2015 BMT Tandem Meetings in sunny San Diego yet?**

With February 11-15 quickly approaching, nearly 3,000 leading worldwide authorities will convene in San Diego to present the latest developments in blood and marrow transplantation during the BMT Tandem Meetings at the Manchester Grand Hyatt.

An unprecedented 613 abstracts were submitted to this year’s meeting. In addition to an outstanding scientific program, the 2015 meetings offer peripheral sessions for BMT pharmacists, BMT center administrators, coordinators, investigators, medical directors, clinical research professionals / data managers, transplant nurses, and advanced practitioners. Along with state-of-the-art educational offerings, industry-supported satellite sessions and product theaters will broaden the spectrum of presentations.
Scientific Program Chairs for the 2015 meetings, Paul Veys, MD, for the CIBMTR and Krishna Komanduri, MD, for ASBMT, will preside over the Best Oral Abstract Session on Friday, February 13. Later that afternoon, a tribute to Nancy King McLain, 52-year transplant survivor, will be held just prior to the Mortimer M. Botwin and E. Donnell Thomas lectures.

Keep an eye on www.cibmtr.org or www.asbmt.org to create your own personal meeting agenda. After registering, take advantage of special conference guest room rates at the Manchester Grand Hyatt and neighboring hotels. Remember to reserve your ticket to the Saturday evening Tandem Reception aboard the USS Midway to end a memorable week with colleagues and good friends.

Questions regarding support opportunities at the 2015 BMT Tandem Meetings may be directed to Sherry Fisher at sfisher@mcw.edu.

For general information, please email the conference office at bmttandem@cs.com.

We look forward to seeing you in San Diego in a few short weeks!

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Clinical Research Professionals / Data Managers Conference
By Lori Colt, BA

Planning for the upcoming Clinical Research Professionals / Data Managers Conference began before the 2014 BMT Tandem Meetings wrapped up its final sessions in Grapevine, Texas. The next destination for learning and discovery, to be held in San Diego, on February 10 and 11, 2015, will include selected topics such as infection, cytogenetics, center specific / center volume reporting, and the ATG protocol. Together, with guidance from senior leadership, coordinators, and volunteer data managers, the chosen presenters will provide training and educational opportunities to data management staff who are responsible for completing and submitting recipient data to the CIBMTR.

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Center-Specific Outcomes and Registry Models
By Carol Daleysh, BS, CPA

The SCTOD is part of the HRSA-funded C.W. Bill Young Cell Transplantation Program that collects data on all allogeneic transplants performed in the US and on transplants performed elsewhere using cellular products that originated in the US. Recent activity within the SCTOD has focused on the Center-Specific Survival Report and Center Outcomes Forum.

Center-Specific Survival Analysis

HCT outcomes reports for US HCT centers are needed to provide information requested by patients, insurers, and government agencies and to comply with current laws. The SCTOD contract requires that the CIBMTR conduct an analysis of one-year survival rates at each transplant center in the US and make these data available to the public. An un-blinded version of the 2014 Center-Specific Survival Report, which includes first allogeneic HCTs performed between 2010 and 2012, was recently distributed to transplant center medical directors and US payers. This is consistent with the goal of the CIBMTR to increase transparency of the Center-Specific Survival Report. The report is meant to be useful as a quality improvement tool for transplant centers. The data are also made available to the public on the Be The Match website.

Center-Specific Outcomes Analysis Forum

In order to fairly address the complex issues and maintain a transparent scientific approach to center outcomes reporting, a fourth Center-Specific Outcomes Analysis Forum was held in June 2014 in collaboration with the NMDP’s Defining Quality and Value in Stem Cell Transplant meeting. Participants included representatives of the HCT community, including transplant physicians and center directors, the ASBMT Committee on Quality Outcomes, governmental funding agencies, patients, private payers, and statisticians. A summary of the meeting was distributed to US Medical Directors and posted on the CIBMTR website.

Registry Models Publication in the New England Journal of Medicine

Program. This accomplishment highlights the value of the Program to the medical community.

Blood and Marrow Transplant Clinical Trials Network

By Amy Feley, MA

The BMT CTN, with its 20 core and approximately 100 affiliate centers, has enrolled over 7,400 patients since 2003. The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with NMDP 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 Fred Appelbaum, MD (Fred Hutchinson Cancer Research Center), Steve Devine, MD (Ohio State University Medical University) continues to serve as Vice-Chair, and Gina Laport, MD (Stanford University) is serving as Immediate Past-Chair.

See you at the 2015 BMT Tandem Meetings!

All attendees are welcome to join us at the BMT CTN Investigators Meeting showcasing upcoming studies in development and primary results from recently-completed analyses. The meeting will be held Wednesday, February 11, 2:45 – 4:30 pm PT, in the Harbor Ballroom ABC.

Reminder to study coordinators: registration is required for the BMT CTN Coordinators Meeting held on Tuesday and Wednesday February 10-11 in the Harbor Ballroom DEF. The meeting will cover BMT CTN processes and study overviews and, as always, will feature presentations from several BMT CTN Investigators. Hope to see you there!

Come support your colleagues as they present their BMT CTN ancillary study abstracts on Thursday, February 12, 4:45 – 6:45 pm PT:

- Shaman Holtan, MD
  - Prognostic Impact of Follistatin in Acute Graft-Versus-Host Disease: Results from BMT CTN 0302 and 0802 [Session E – GVH / GVL: Seaport Ballroom DE]
- Jo-Anne Young, MD
  - More Infections with Transplantation of Bone Marrow, Versus Peripheral-Blood Stem Cells, from Unrelated Donors [Session F – Allo Transplants: Harbor Ballroom ABC]

Stop by the BMT CTN booth – BMT CTN Investigators and Data and Coordinating Center staff will be there during breaks to answer your questions and provide ideas for how your center can get involved.

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 at http://www.bmtctn.net.

There are eight trials open, one released to sites, and seven in development. The following BMT CTN trials are open or will soon be opened for enrollment:

- BMT CTN 0903 - Phase II study for allogeneic transplantation for hematologic malignancy in HIV + patients
- BMT CTN 1101 - Phase III study comparing HLA-haploidentical related donor or bone marrow vs. double umbilical cord blood (haplo vs. double cord) with RIC for patients with hematologic malignancy
- BMT CTN 1102 - Biologic assignment trial comparing RIC HCT to hypomethylating therapy or best supportive care in patients aged 50-75 with intermediate-2 and high risk myelodysplastic syndrome
- BMT CTN 1202 - Prospective cohort of biologic samples for the evaluation of biomarkers predicting risk of complications and mortality following allogeneic HCT
- BMT CTN 1203 PROGRESS I - Randomized Phase II study of novel approaches for GVHD prophylaxis compared to CIBMTR controls
- BMT CTN 1204 - RIC for children and adults with hemophagocytic syndromes or selected primary immune deficiencies
- BMT CTN 1205 - Easy-to-read Informed consent for HCT clinical trials
BMT CTN Publications

There are 48 BMT CTN published articles, including 14 primary analyses. The following manuscripts were recently accepted / published. (** Manuscripts reporting primary results.)


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

- Like us on Facebook: [www.facebook.com/bmtctn](http://www.facebook.com/bmtctn)
- Follow us on Twitter: @BMTCTN

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- Follow us on Twitter: @CIBMTR

CIBMTR Advisory Committee

The Advisory Committee, made up of members from across the globe, maintains careful oversight of the CIBMTR research agenda. The committee members are listed on the CIBMTR website, and we sincerely thank all of our committee members for their time and efforts, particularly the following individuals who will complete their service in February:

- Jan Cornelissen - Member at Large (Non-North America)
- Mitchell Horowitz - Member at Large (North America)
- Michael Lili - Member at Large (North America)
- David Marks - Vice-Chair (Europe)
- Philip McCarthy - Member at Large (North America)
- Alok Srivasta - Vice-Chair (Asia / Africa / Australia)
- Jeffrey Szer - Member at Large (Non-North America)
- André Tichelli - Member at Large (Non-North America)

Our Supporters

The CIBMTR is supported by Public Health Service Grant / Cooperative Agreement 5U24CA076510 from the NCI, NHLBI, and NIAID; a Grant / Cooperative Agreement 5U10HL069294 from NHLBI and NCI; a contract HHSH250201200016C with 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, which are
listed on the CIBMTR website.

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

Last Updated: 4/1/2015 3:39 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

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