February 2017 Newsletter

Volume 23, Issue 1

Table of Contents:
Perspectives
Autoimmune Diseases and Cellular Therapies Working Committee
Plasma Cell Disorders and Adult Solid Tumors Working Committee
CIBMTR Trivia
2017 BMT Tandem Meetings
2016 CIBMTR Annual Report
Team Spotlight: Survey Research Group
NCI / caDSR Collaboration
Stem Cell Therapeutic Outcomes Database
Second Annual CIBMTR Cellular Therapy Registry Forum
Blood and Marrow Transplant Clinical Trials Network
RCHT BMT: Mismatched Unrelated Donor Protocol
Health Services Research Program Updates
New Patient Summary of CIBMTR Research
CIBMTR on Facebook and Twitter
Our Supporters
Abbreviations

Perspectives
By Paul Martin, MD

After a three-year term, my service as Chair of the CIBMTR Advisory Committee will come to an end during the 2017 BMT Tandem Meetings. This will be my last Chair Perspective for the quarterly newsletter. I will not tweet #sad because I am not, and I will not deliver a farewell address because I will still be around for a while. However, this occasion offers an opportunity for reflection, both looking back and looking forward and from both a personal and an institutional perspective.

It has been said that nothing is more terrifying for a writer than a blank piece of paper, and I confess to having this feeling each time I begin writing the Chair Perspective for this newsletter. Looking back, I began with bathtubs and calculus problems. Along the way, I commented on the miseries and benefits of performance reviews, the pain of hearing “no”, picking winners (Working Committees and people), my second favorite three-word sentence,
celebration of anniversaries and our international scope, the yin and yang success of transplantation and burden of late effects, changing times and the advent of cellular therapy, and measuring value in cancer care.

In preparing to write this perspective, I was curious to see if I could find any formal statement describing the organizational values upheld by the CIBMTR. I found an eloquent mission statement that describes what we do, but the values were not explicitly named. In reflecting upon material online and my personal experience, it was easy to recognize seven key organizational values that shine through. These include scientific rigor, transparency, inclusiveness, respect for diversity, volunteer service, sharing, and collaboration. Perhaps you would name a few others, but in any case, these values are not merely aspirational words in a document. We, the people, live them at every level throughout the organization. They have served us well in the past, and they will continue to do so in the future.

I have many people to acknowledge and thank. Beyond the Advisory Committee Vice Chairs and Members at Large, the Working Committee Chairs, and the CIBMTR Scientific and Statistical Directors, several individuals deserve specific mention. Most of all I would like to thank Mary Horowitz for her outstanding leadership as Chief Scientific Director of the CIBMTR. Where would we be without Mary? Waleska Perez has led the way in the implementation of the process that we now use to evaluate the performance of Working Committees. In addition, I would like to thank Patty Steinert for outstanding administrative service and Doug Rizzo for his ability to manage the constantly evolving CIBMTR Research Database. Finally, I would like to thank the Project Officers who have supported the program for so many years of success and growth: Roy Wu (now retired), Bill Merritt, Nancy DiFronzo, Linda Griffith, Bob Hartzman, Shelly Grant, and Nawraz Shawir.

I am confident that we will have a smooth transition to new leadership with Rob Solfer as the incoming Chair. His year of service as Chair-Elect has given him good preparation for the role of Chair, and I look forward to the next two years of my service as Immediate-Past Chair as a way of enhancing continuity. We can now look forward to reading Chair Perspectives from Rob. He has a broad understanding of the continuing evolution in cellular therapy across the decades and a deep appreciation of the mission and values of the CIBMTR. He asks cogent questions; he is not afraid to say what he thinks, and best of all, he has a great sense of humor. Let’s welcome Rob!

Autoimmune Diseases and Cellular Therapies Working Committee

Committee Leadership

Co-Chairs:
- Ian D. Lewis, MD, PhD, Royal Adelaide Hospital
- David H. McKenna, MD, University of Minnesota
- Stefanie Sarantopoulos, MD, PhD, Duke University Medical Center

Scientific Director:
- Marcelo Paquini, MD, MS

Statistical Director:
- Ruta Brazauskas, PhD

Statisticians:
- Kyle Bo-Subait, MPH

The Autoimmune Diseases and Cellular Therapy Working Committee (ACWC) was formed in 2014 via the merging of two existing committees, the Autoimmune Diseases WC and the Cellular Therapy WC. This created a unique committee with a broad scope and diverse applications. The unifying aspect of these two areas is the requirement of outreach to other specialties for collaboration in projects. There is also overlap with the use of cellular therapy for treatment of autoimmune diseases.
The ACWC's mission is to promote studies of HCT and other cellular therapies for autoimmune disease and to investigate novel uses of cellular therapies for both malignant and non-malignant indications. Committee leadership is comprised of individuals with expertise in transplantation, cellular therapy, cell processing, and autoimmune diseases.

### Autoimmune Disease

Autoimmune disease remains an uncommon transplant indication. To date, physicians have primarily transplanted patients with multiple sclerosis (MS) and scleroderma but also patients with systemic lupus erythematosus, inflammatory bowel diseases, autoimmune cytopenias, diabetes, and other diseases. Most transplants are autologous, which rely on immune-ablative regimens to control autoimmunity. Applications of allogeneic transplant remain mostly investigational.

From 2007 to 2016, 398 autologous and 67 allogeneic HCTs were reported to the CIBMTR by 80 centers.

The main challenge for this committee remains the availability of disease specific data for more comprehensive studies. Disease assessment in autoimmune diseases is often complex and requires organ specific complexity assessments, and input from disease specialties is ideal. Data collection for these diseases is different from other more common hematological or malignant disease transplant indications, in which disease specific information is readily available to data managers. The CIBMTR has undertaken several efforts to improve data collection, including simplifying report forms. For example, the committee collaborated with the EBMT Autoimmune Working Party to harmonize the MS forms. Similar efforts for scleroderma forms are ongoing. Still, studies in this committee require additional active data collection to supplement information about disease status at transplant, disease response, and progression events after transplant.

One recently completed study, AI 0901 (PIs: Paolo Muraro, MD, and Ricardo Saccardi, MD), was made possible by the collaboration of the EBMT Autoimmune Working Party and centers from Brazil with high activity in transplants for autoimmune disease. In this study, 283 autologous transplant recipients with MS were analyzed, making this the largest study with the longest follow-up to date. The analysis demonstrated that responses to transplant are long lasting, and patients who receive transplants while in earlier and more inflammatory forms of the disease (relapsing remitting) have better disease control. This study emphasized the importance of transplant as a therapeutic option for MS patients and will lead to further studies, which are anticipated to result in a change in practice. Given the success of the MS study, the committee is focusing on similar collaborations to describe long-term outcomes of patients who receive transplants for scleroderma (AC 14-01 – PI: Dominique Farge, MD). This study is led by the EBMT group, and the CIBMTR is assisting in reaching out to US centers to collect scleroderma specific data and long-term follow-up.

### Cellular Therapy

Cellular therapy application is a rapidly emerging area with a very large scope. The CIBMTR initiated efforts to study the use of cellular therapy in 2008 through the SCTOD, which assessed alternate uses of hematopoietic cells in regenerative medicine. At that time, applications in cardiology were rapidly expanding the use of autologous stem cells with the intent to restore or heal cardiomyocytes. The CIBMTR then established the Cellular Therapy Working Committee to oversee the initial development of a database specific for cellular therapy with pertinent data elements to this new field. This required a major modification in the structure of data collection that, until then, used transplant as the main milestone, with all subsequent outcomes being referenced to that event. The approach was to developed a decisional node during the registration process at the level of setting up the CIBMTR Identification (CRID) to define whether the procedure being performed was a hematopoietic cell transplant or a cellular therapy. In order to specifically define a transplant, the data manager needed to address if the therapy intent included hematopoietic recovery / replacement or not. Patients assigned to the cellular therapy track receive a single form called Form 4000 or CTRM (Cellular Therapy for Regenerative Medicine Form). The initial intent was to capture activity, and it incorporates limited outcome data related to infusion adverse events but had no follow-up. The CIBMTR reached out to the cardiology community for collaboration at that time; however, the use of cellular therapy was mainly investigational and implementation of a database was felt to be premature.

The CIBMTR started to collect information on cellular therapy in 2011. These data were mainly contributed by transplant centers also involved in cellular therapy protocols who were interested in sharing data on indications, type of cells utilized, and number of patients / infusions performed. Since its implementation, data for more than 600 patients who received cellular therapy for several indications were reported by 17 centers. The most common indication is the use of autologous cord
blood unit for treatment of congenital neurologic disorders, mainly cerebral palsy and congenital hydrocephalus. However, this did not represent all activity, as only a small number of centers reported this data to the CIBMTR.

As the cellular therapy field has evolved over the past five years so have the types of indications, and a number of cellular therapies for treatment of hematologic malignancies using genetic modified cells started to be reported to the CIBMTR. The committee recognized a need to adapt to the evolving nature and practices on cellular therapy and developed a task force to revise the data collection practices and expand the focus of cellular therapy to include the use of treatment of malignancies. To better capture new data available on cellular therapies, and in conjunction with the Form Revision process, the CIBMTR expanded its cellular therapy data collection forms. Over the past year, a team of experts worked to develop new cellular therapy forms. These forms include:

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<thead>
<tr>
<th>Cellular Therapy Forms</th>
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<tr>
<td>Pre-Cellular Therapy Essential Data (Pre-CTED)</td>
<td>4000</td>
<td>Pre-infusion form, required once per course of cellular therapy</td>
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<tr>
<td>Cellular Therapy Infusion</td>
<td>4006</td>
<td>Required for each infusion</td>
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<tr>
<td>Post-Cellular Therapy Essential Data (Post-CTED)</td>
<td>4100</td>
<td>Non-Genetically Modified: 100 days, 6 months, 1-6 years, and then every other year. Genetically Modified*: Required at 100 days, 6 months, 1-6 years, and then every other year.</td>
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*Genetically modified products reported to the CIBMTR will have a modified forms due schedule. Per FDA requirements, follow-up on genetically modified products is required for 15 years.

The new pre-CTED functions like the pre-TED in collecting pre-infusion data for cellular therapies. The Cellular Therapy Infusion Form (F4006) was adapted from the transplant infusion form (F2006), which was expanded to accommodate information related to the product manufacturing and characterization as well as details of the infusion. The post-CTED form (F4100) collects post-infusion data, similar to the F2100. A unique component of the post-CTED is the inclusion of cellular therapy-specific outcomes, such as cytokine release syndrome, neurotoxicity, and persistence of the cellular product. There are different reporting schedules for infusions with non-genetically modified products versus those with genetically modified products, which comply with regulatory requirements.

The new CTED series of forms was released July 2016 for a pilot period of one year in order to beta test the system and the forms. This pilot project is being funded by the NCI and includes eight centers. Input from these initial users will be used to revise the forms and optimize the system. In January 2016, with the release of the post-TED form, all the donor cellular infusions that were collected in transplant forms will be directed through the cellular therapy track to better understand the indications and cellular therapy used and to capture cellular therapy-specific outcomes. As part of the development of the new cellular therapy registry, the CIBMTR reached out to the EBMT and Japan to initiate the harmonization of cellular therapy data elements. All cellular therapy forms have been reviewed with these international partners, and overlapping fields have been harmonized. The next version of the CTED series of forms will include updates from both the pilot study and from the harmonization with international partners.

Research activities in cellular therapy have mainly focused on having a robust database to the development of studies. There are a few retrospective studies focused mainly on uses of donor cellular infusions after transplant:

- **CT10-01** led by Noelle Frey, MD, and David Porter, MD, analyzed donor cellular infusion and second transplants for treatment of disease relapse. By applying a statistical model that started at the time of disease relapse using any treatment as a time-dependent covariate, the investigators were able to analyze both the activity and impact of these therapies on survival. The data revealed that the overall impact of these DCI and second transplants on post-transplant survival is small, highlighting the need for development of novel approaches for treatment of disease relapse. This study is currently in the manuscript development phase.
- **CT13-01** led by Gorgun Akpek, MD, will look at the use of donor cellular infusion (DCI) for treatment of post-transplant infections. This DCI indication
utilized non-manipulated lymphocytes, third party cytotoxic T lymphocytes, or genetic modified cells. The study will collect infection related outcomes on recipients of these cellular infusions.

- **AC16-01**, led by Vivek Roy, MD, will look at patterns of use and outcomes of donor lymphocyte infusion (DLI) after HLA-haploidentical allogeneic HCT. As the number of haploidentical donor transplants are on the rise, there is an interest in describing the activity of using DLI after these transplants.

In summary, the ACWC has a number of exciting projects and a large focus on building the infrastructure for future studies. For both areas of focus, there is increasing activity. The committee invites young and experienced investigators to continue to participate and submit their research ideas.

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

**Committee Leadership**

**Co-Chairs**

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<th>Co-Chair</th>
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<tr>
<td>Amita Y. Krishnan, MD</td>
<td>City of Hope National Medical Center</td>
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<tr>
<td>Cristina Gasparetto, MD</td>
<td>Duke University Medical Center</td>
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<tr>
<td>Yeigo L. Nieto, MD, PhD</td>
<td>Anderson Cancer Center</td>
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<td>Tomer M. Mark, MD, MD</td>
<td>University of Colorado Hospital</td>
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**Scientific Director**

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<td>Parameswaran Hari, MD, MS</td>
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<td>Anita D’Souza, MD</td>
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<td>Raphael Fraser, PhD</td>
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<td>Omar Dávila-Avello, MPH, MS</td>
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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 only multiple myeloma but also other plasma cell disorders, such as light chain amyloidosis, Waldenstrom macroglobulinemia, and plasma cell leukemia. In addition, since 2014, adult solid tumors are also included within our focus.

We have six ongoing projects, including validation of R-ISS staging in multiple myeloma, germ cell tumors, third transplant in myeloma, various graft sources for performing allogeneic transplants in myeloma, and others. Noteworthy accomplishments from this committee in the last year include six peer-reviewed publications in 2016, four oral presentations at the American Society of Hematology, and one oral presentation at the BMT Tandem Meetings later this month.
This year we received 14 proposals, 8 of which will be presented at the BMT Tandem Meetings. The Plasma Cell Disorders and Adult Solid Tumors Working Committee is always seeking interesting and novel ideas for study as well as encouraging the involvement of junior investigators and those wanting to break into the field of blood and marrow transplantation and outcomes research. We believe early involvement will encourage long-term participation of young investigators in committee activities in the future.

The success of the Committee depends on new ideas and testable hypotheses as well as participation by individuals with different perspectives and scientific backgrounds. The Plasma Cell Disorders and Adult Solid Tumors Working Committee encourages all investigators with an interest in our disease focus to propose a study. Information on how to propose a study can be found on the CIBMTR How to Propose a Study webpage.

To learn more or to discuss your research ideas and proposals, contact one of the members of the Working Committee Leadership

This year, we bid farewell to Amrita, our outgoing Chair at the end of February. We welcome our new Chair, Shaji Kumar, MD, Mayo Clinic.

We encourage our 345 active working committee members to actively participate. We look forward to seeing everyone at the upcoming BMT Tandem Meetings in Orlando, FL.

CIBMTR Trivia
The CIBMTR provides detailed information and training to help centers submit high quality data in an efficient manner. We offer ___ online courses for centers to view.

A. 10
B. 15
C. 20
D. 25

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

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.

Have you registered for the 2017 BMT Tandem Meetings, taking place February 22-26 in Orlando, Florida?

More than 3,000 leading worldwide authorities will convene in Orlando to present the latest developments in BMT at the Gaylord Palms Convention Center.

More than 550 abstracts from more than 25 countries were submitted to this year’s meeting. We invite you to join us for the Best Oral Abstract Session on Friday, February 24. New this year, Scientific Program Chairs for the 2017 BMT Tandem Meetings, David Marks, MBBS, PhD, for the CIBMTR and Marcel van den Brink, MD, PhD, for the ASBMT will moderate a Late Breaking Abstracts session during which three late breaking abstracts will be presented as the final session of the meeting on Sunday.

In addition to an outstanding scientific program, the 2017 meetings offer peripheral sessions for BMT pharmacists, center administrators, coordinators, investigators, medical directors, clinical research professionals / data managers, transplant
nurses, and advanced practice professionals. Along with state-of-the-art educational offerings, industry supported satellite sessions and product theaters will broaden the spectrum of presentations.

Please join us after the Best Abstracts Session on Friday afternoon as the CIBMTR Distinguished Service Award is presented to David Gómez-Almaguer, MD, and Guillermo J. Ruiz-Arguelles, MD. This award is presented to both doctors for their service and commitment to the CIBMTR and its missions. 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. We invite you to attend the Mortimer M. Bortin Lecture, presented by Richard E. Champlin, MD, and E. Donnall Thomas Lecture, presented by Jerome Ritz, MD.

Visit the 2017 BMT Tandem Meetings home site to create your own personal agenda, register, and view additional details. As of late-January, more than 2,700 attendees were registered. On-site registration rates went into effect on January 25. After registering, take advantage of special conference guest room rates at a wide variety of hotels within the BMT Tandem Meetings room block.

Remember to reserve your ticket to the BMT Tandem Meetings Reception on Saturday, February 25, at the Gaylord Palms starting on the Coquina Lawn and moving just off the lawn for dancing inside of Wreckers.

**Tandem Goes Mobile!**

You may now download the official BMT Tandem Meetings app for quick and easy access to the most current version of schedules, attendee list, venue information, and more! Search for BMT Tandem Meetings in your app store. Enter bmt2017 for access verification and after downloading the app, enter the same email address used to register for the meeting for additional features. The app is free for all attendees. With Wi-Fi available throughout all BMT Tandem Meetings rooms, users can:

- View and search the meeting program schedule
- Vote / participate in interactive sessions
- Complete session surveys
- Search for speakers
- Check out who is exhibiting and find their booth on a map
- Create a personal schedule
- Message other attendees
- Access other meeting information

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!

![Image of the Gaylord Palms Hotel](image)

Return to Top

**2016 CIBMTR Annual Report**

We recently published the CIBMTR 2016 Annual Report. The format of the report this year focuses on information most important to transplant center personnel and other partners. We explain who we are, what we do, how we share knowledge, how we collect and manage data, and what we will do next. Review the electronic version to access links directly, or pick up a hard copy at the CIBMTR booth at the BMT Tandem Meetings.
Team Spotlight: Survey Research Group

The CIBMTR Survey Research Group (SRG) is part of the Prospective Research team and supports research studies that require direct contact with participants, primarily by phone. Participants include related and unrelated HCT donors, HCT transplant survivors, patients awaiting transplant or an HCT donor, and patient caregivers. Providing support for studies for RCI BMT, Health Services Research, BMT CTN, Bioinformatics, and National Marrow Donor Program, SRG staff members contact participants to complete telephone assessments or to follow-up on study materials that have been sent out.

SRG is comprised of three Survey Research Associates and a Supervisor who are supported by the Prospective Research Clinical Trials Assistant. On any given day, the SRG makes 150-200 contact attempts to donors or patients and completes 25-40 assessments or study calls. In fiscal year 2016, the group made and received 32,513 phone calls and sent 2,286 emails, 296 texts, and 1,345 letters or other mailed materials leading to the completion of 7,339 assessments or other study activities.

The SRG works on five to eight different research studies at any given time. Some of SRGs current studies include:

- **Long-Term Donor Follow-Up** – SRG conducts brief, biennial health assessments of unrelated donors. Some contracted donor centers conduct their own assessments, but SRG is responsible for approximately 14,500 of the about 21,500 enrolled donors in this study.

- **Millennial Member Project** – This study, directed by the University of Pittsburgh, aims to develop strategies to improve availability of donors aged 18-30. SRG contact registry members who are:
  - Continuing with donation or opting out after being reached for clinical trial.
  - New registry members, to obtain permission for the University of Pittsburgh to send them a link to an online survey.

- **BMT CTN 1102** – This trial compares overall survival and quality of life of patients with myelodysplastic syndrome who are able to find a suitable donor for HCT with those who are not able to do so within 90 days of enrollment. SRG conducts 20-minute quality of life surveys at six time points over a three-year period.

Left to right: Sarah Lease, Cynthia Kunakom, Christine Jacob, Deborah Mattila and Hilary Bauer

NCI / caDSR Collaboration

One barrier to collaborative research is the different ways in which organizations define, collect, and store data. Such inconsistencies make it difficult to share, aggregate, and analyze data. Ultimately, this hinders the research needed to fulfill the CIBMTR mission to advance HCT and cellular therapy worldwide to increase survival and enrich quality of life for patients. Data standards help overcome this barrier by establishing a common language for sharing and analyzing data. The cancer Data Standards Registry and Repository (caDSR) is a publicly accessible data dictionary maintained by the National Cancer Institute (NCI). Approximately 40 organizations develop, maintain, and share the more than 55,000 common data elements contained in the caDSR.
For nearly ten years, the CIBMTR has contributed to the development of data standards within the caDSR. What began as a small project to support the electronic transmission of data has become the foundation upon which recipient data is collected. To date, common data elements have been created for more than 19,200 FormsNet database fields. This represents those database fields associated with 99% of recipient forms submitted via FormsNet. These common data elements are defined by a hard-working team of Metadata Analysts from both the Milwaukee and Minneapolis campuses of the CIBMTR: Angela Kummerow, Michele Nych, Sandy Sorensen, and Wendy Zhang.

The CIBMTR has also become a recognized leader within the caDSR community. The team members are frequently asked to participate in data quality, harmonization, and other projects. The caDSR is also working on several key initiatives including a redesign of their database and supporting applications. As part of these key initiatives, the CIBMTR and the caDSR will engage in a collaboration over the next year. During this time, Robinet Renner, Manager of the Metadata Analysis Team, will dedicate a portion of her time toward the caDSR initiatives. She will assist with the design of the new caDSR applications, lead data quality projects, and oversee the work of the Core NCI Metadata Analysis Team. This presents the CIBMTR with an opportunity to deepen our partnership with the caDSR, reaffirm our commitment to standardized data, and to influence the future design of the caDSR. It is also a testament to the CIBMTR’s reputation for creating high-quality common data elements.

Stem Cell Therapeutic Outcomes Database
By Carol Doleysh

The SCTOD is part of the US Health Resources and Services Administration-funded C.W. Bill Young Cell Transplantation Program that collects data on all allogeneic HCTs performed in the US and on transplants performed using cellular products that originated in the US.

Center Outcomes
Outcomes reporting in allogeneic HCT is necessary to provide information requested by patients, insurers, and government agencies and to comply with current laws. The SCTOD contract requires the CIBMTR to conduct an analysis of one-year survival rates at each transplant center in the US annually. The report generated by the CIBMTR 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.

The 2016 Center-Specific Outcomes Report, which includes first allogeneic HCT performed between 2012 and 2014 in the US, was distributed in December to Center Directors and Payors. The data was also updated on the Be the Match website. Additional tools accessible to transplant centers for quality improvement work include Center Performance Analytics and the Survival Calculator. Access to these is through the secure CIBMTR Portal. For additional information, contact cibmtr-portalhelp@mcw.edu.

In order to be included in the analysis, transplant centers were required to have at least one year of follow-up on more than 90% of related and unrelated HCT recipients. A description of the methodology used in generating this report is available on the US Transplant and Survival Statistics on Related Sites webpage.

Center Outcomes Forum
In order to fairly address the complex issues and maintain a transparent scientific approach to center outcomes reporting, a Fifth Center Outcomes Forum was held in October 2016 in collaboration with the NMDP’s Implementing Quality and Value in HCT meeting. Participants included representatives of the HCT community, including transplant physicians and center directors, the ASBMT, governmental funding agencies, patients, private payors, and statisticians. A summary of the meeting will be distributed to US Medical Directors and posted on the Center-Specific Outcomes Analysis webpage. The major topics addressed at the 2016 forum included:

- Measuring quality of life in transplant recipients
- Quality improvement activities of transplant centers
- Handling unintended consequences of public outcomes reporting
- Possible future outcomes metrics for public reporting
- Addressing cost of care and "value"

Return to Top
Second Annual CIBMTR Cellular Therapy Registry Forum  
By Tiffany Hunt, MS, CCRP, and Emilie Love, CSPO

The SCTOD in October marked the second annual CIBMTR Cellular Therapy Registry Forum. This two-day event, held in Minneapolis, hosted nationwide attendees from the cellular therapy community, industry, and regulatory groups. Day one included an overview of the cellular therapy world and where it is headed. Presenters described their practices at multiple centers, shared updates from industry such as Kite Pharma and Atara Biotherapeutics, and explained the work the FDA and FACT are doing around cellular therapies. Day two focused on the CIBMTR’s cellular therapy data collection initiative. Data managers from several centers reviewed the cellular therapy data collection forms in detail, answered questions, and gathered suggestions for the first form revisions. This successful meeting marked another step-forward for the CIBMTR Cellular Therapy Registry.

Blood and Marrow Transplant Clinical Trials Network  
By Amy Foley, MA

The BMT CTN, with its 20 core and approximately 100 affiliate centers, has enrolled more than 9,300 patients since 2003. The CIBMTR shares administration of BMT CTN Data and Coordinating Center with NMDP and The Emes 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 (Baylor College of Medicine), started her term as Vice-Chair on January 1.

See you at the 2017 BMT Tandem Meetings!

Are you an investigator wanting to get involved? The **CIBMTR and BMT CTN Orientation session** will be held on Thursday, February 23, 7:00 – 8:15 am EST in Naples 1-3. Presenters Drs. Marcelo Pasquini and Bronwen Shaw will provide specific ways to participate in both programs.

All meeting attendees are welcome to join us at the **BMT CTN Investigators Meeting** showcasing upcoming and accruing studies. This year’s agenda includes results from the 0702 STaMINA myeloma study and 0201 PBSC vs marrow 5-year follow-up as well as overviews of the upcoming 1506 AML Maintenance Therapy trial and risk stratification trials in acute GVHD. The meeting will be held Wednesday, February 22, 7:00 – 8:15 am EST in Sun B.

The **BMT CTN Coordinators Meeting** will be held on Wednesday, February 22, 8:30 am-4:30 pm EST in Sun A. The meeting will cover BMT CTN processes and study overviews and, as always, will feature presentations from several BMT CTN Investigators. We hope to see all of the BMT CTN study coordinators there! Mark your conference calendars to attend the BMT CTN abstracts oral presentations:

- **Shenan Holtan, MD**
  - **Follistatin and Endoglin:** Potential biomarkers of endothelial damage and non-relapse mortality after myeloablative allogeneic hematopoietic cell transplantation in Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0402 [Session J – Saturday, February 25, 6:30 – 6:45 pm EST]

- **Nandita Khera, MD, MPH**
  - **Understanding physicians’ perspectives about translating research into clinical practice:** Example of Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0201 results [Session G - Friday, February 24, 11:45 am – 12:00 pm EST]

- **Joseph Pidala, MD, PhD**
  - **Multi-state modeling identifies determinants of successful immune suppression discontinuation:** Secondary analysis of BMT CTN 0201 and 0402 trials [Session K – Saturday, February 25, 6:15 – 6:30 p.m. EST]

Do not forget to 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.
To get up-to-date information about BMT CTN studies, meetings, and news:

Facebook.com/bmtctn
Twitter.com/bmtctn (@BMTCTN)

Return to Top

**RCI BMT: Mismatched Unrelated Donor Protocol**

In support of the Be The Match objective to determine the value of allogeneic unrelated HCT as an effective therapy, the CIBMTR developed the 15-MMUD study, a multi-center, Phase II trial of HLA-mismatched unrelated donor bone marrow transplantation with post-transplantation cyclophosphamide for patients with hematologic malignancies. The study will evaluate outcomes in patients who do not have an HLA-identical sibling or 8/8 match and undergo bone marrow transplant using a mismatched unrelated donor (MMUD).

The goal of this study is to demonstrate survival, engraftment, and GVHD rates similar to those observed after haploidentical (related) donor bone marrow transplant. Establishing similar rates would optimize and expedite future donor selection with the added ability to select a MMUD for patients without a matched donor. Increasing the donor pool for each patient would improve the likelihood of finding a donor available to donate in a more timely manner. Expanded MMUD options would also benefit patients from ethnic minorities who are more likely to find a mismatched than a matched unrelated donor on international donor registries. With more than 20 million unrelated donors currently listed on the international donor registries, multiple mismatched donors are expected to be available for each patient.

Development for the 15-MMUD study began in October 2015. The protocol received approval from both the CIBMTR / RCI BMT Data Safety Monitoring Board and the NMDP Institutional Review Board within seven months of development initiation, and the first study site was opened to enrollment in August 2016 (within 10 months of development initiation). Eighty recipients will be enrolled during a two-year accrual period, with 40 subjects receiving select full-intensity conditioning regimens and 40 subjects receiving a select reduced-intensity conditioning regimen. Ten transplant centers will conduct the study, with eight sites currently open to accrual. Our first study subject was enrolled four months after the first site activation.

Study data will be captured using the Medidata Rave web-based electronic data capture platform. After development of study case report forms (CRF) was complete, CRF design and testing in Rave were completed within 90 days. By utilizing Rave, study sites are able to complete study CRFs and report critical study data points in a timely manner for this important study.

As a result of cross-organizational collaborative teamwork, the 15-MMUD study was able to achieve aggressive study development milestones. The dedication and commitment from groups at the CIBMTR and Be The Match as well as transplant center protocol team members contributed to successful study activation less than one year after project initiation.

Return to Top

**Health Services Research Program Updates**

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

**Patient-Centered Outcomes Research (PCOR) Update and Final Symposium**

We’re making notable progress in our Patient-Centered Outcomes Research Institute (PCORI) supported project, Engaging patients in developing a patient-centered HCT research agenda. We held two webinars in October and November that each had more than 100 attendees. The first, entitled Transplant outcomes that matter most to patients, highlighted a patient / caregiver and social worker panel that provided unique perspectives on the transplant journey. The second webinar, Patient reported outcomes research in hematopoietic cell transplantation, highlighted the research of experts in the field, including that of Heather Jim, PhD; Bill Wood, MD, MPH; and Bronwen Shaw, MD, PhD. This webinar will be turned into an enduring program on the NMDP/Be The Match website with the offer of CME.

We held our second symposium, Prioritizing a PCOR HCT Research Agenda, in December. There was lively discussion and attendee input as the six Working
Groups reported on their progress to date in identifying research questions that matter most to patients. A patient/caregiver panel provided their perspectives on working with researchers, a highpoint of the program.

Save the Date: PCOR Symposium: Building a PCOR Collaborative Community
The final PCOR Symposium, Building a PCOR Collaborative Community, will be held during the BMT Tandem Meetings on Saturday, February 25, 2017, 12:15 – 4:45 pm EST. We need your input on the proposed research agenda for patient-reported outcomes in HCT.

The six Working Groups, focused on the patient-centered topics listed below, will provide their final recommendations regarding research questions.

- 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
- Financial Burden

Unique to this symposium, we asked Working Group Co-Chairs to prepare joint presentations with patients/caregivers. Following the Working Groups’ reports, research and funding opportunities within PCORI, BMT CTN, and CIBMTR will be presented. We will discuss next steps in building a collaborative PCOR community in transplantation. CME will be provided.

While this is an open meeting, we request that you pre-register for planning purposes:
Palliative Care, an Unmet Need for Patients: January Survey
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 Drs. Effie Petersdorf and Thomas Leblanc, 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 in January for this survey. Your input is invaluable in this critical area. While this survey is for physicians, upcoming surveys will obtain input from other providers and patients.

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. The Health Services Research Program recently completed merging a dataset from the Centers for Medicare and Medicaid (CMS) with CIBMTR outcomes data to create a unique data set for exploring the value of therapy for older patients with acute myeloid leukemia and myelodysplastic syndromes. This work will be presented at the BMT Tandem Meetings. 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
The HCT Value and Health Economics Special Interest Group (SIG), in collaboration with the Administrator’s SIG, will hold a combined session at the BMT Tandem Meetings on Friday, February 24, 3:00 – 6:00 pm EST. The agenda incorporates outstanding speakers on a variety of topics addressing the numerous issues in quality and value we grapple with in HCT. Dr. David Dickens will speak on payer advocacy; Dr. Vinay Prasad will provide his assessment of how drug pricing influences patient care, and Dr. Nathan Dowden will give a view from the payer and biotechnology industry perspective on reimbursement and drug pricing. We will include international topics as well, such as exporting transplantation across national borders and medical tourism. Mark your calendars and join us for an interactive and timely discussion. CME will be provided.
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.

- Some children who donate cells to a brother or a sister have a lower quality of life and need extra support
• About 20% of donors (1 in 5) at each time point had very poor QOL.
• Parents tended to overestimate their child’s QOL.
• Younger children were more likely to have a lower QOL.

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

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