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Perspectives

By Robert Soiffer, MD

When explaining the risks of alloHCT for malignant disease to patients, providers typically emphasize potentially life-threatening complications, such as GVHD, opportunistic infection, organ damage, hemorrhage, and graft rejection. After learning of the potential morbidity and mortality associated with alloHCT in such consent sessions, transplant candidates are sometimes surprised to also face the risk of disease recurrence despite transplantation. Indeed, over the past 30 years, funding for clinical- and lab-based transplant research has focused far more on prevention and treatment of GVHD, rather than on addressing the issue of relapse. This may be understandable given the still somewhat mysterious nature of GVHD and its devastating consequences, yet disease recurrence remains the single leading cause for failure after alloHCT. Indeed, a review of investigational studies listed on ClinicalTrials.gov reveals a relative paucity of interventional trials devoted specifically to the prevention and/or treatment of disease relapse after alloHCT.

A practical barrier to developing innovative trials is the lack of access to investigational agents to test in the post alloHCT setting. Early phase studies involving new drugs for relapse often exclude patients who have undergone transplant given the potential for complications associated with HCT that may be

completely independent of a new experimental product. Most commonly, trials using novel agents are only conducted when such agents have been approved for other indications. This often leads to off-protocol use of these agents, compromising enrollment on prospective trials. A hopeful exception to this phenomenon will be the large prospective BMT CTN led international Phase III placebo controlled randomized trial to determine the benefit of administering a FLT3 TKI (gilteritinib) as maintenance after alloHCT for patients with FLT3-ITD AML (NCT02997202).

The CIBMTR and its numerous Scientific Working Committees skillfully address many issues critical to the success of transplantation, including donor source, GVHD prophylaxis, GVHD treatment, late complications, and regimen related toxicities. These topics are very specifically related to the process of transplantation and appropriately engage the transplant scientific community. The disease specific committees (acute leukemia, chronic leukemia, lymphoma, plasma cell disorders, etc.) examine transplant outcomes by disease sub-type, focusing largely on the tools within the transplanter's armamentarium. However, for us to truly make significant progress in curing the malignancies for which HCT is performed in the first place, we need to work more closely with our colleagues addressing basic disease biology and novel drug development. We need to find a way to convince pharmaceutical and biotech companies to partner with us to explore synergies between new agents and graft-versus-tumor responses in prospective clinical trials. We also need to further explore avenues to seamlessly link the remarkable registry capacity of the CIBMTR with cooperative group and single institution databases so that we can understand where and when HCT should be applied.

Hopefully, the next five years will see us bring genomics, immunobiology, small molecule inhibitors, antibodies, and engineered cell therapies together so that we can figure out what it is we have set out to do – to cure all our patients with cancer.

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Randy Mills, PhD, Joined Be The Match as CEO

Be The Match Board of Directors selected C. Randal (Randy) Mills, PhD , to join Be The Match as CEO.

Randy started as CEO on June 30. Dr. Chell is now CEO Emeritus and will spend his time until November supporting Randy's onboarding and transition.

Randy is a mission-driven leader with more than 20 years of experience leading companies focused on adult stem cell therapies and regenerative medicine. He spent the last three years as CEO of the California Institute for Regenerative Medicine (CIRM) where he transformed the organization into one internationally recognized for its speed, innovation, and productivity. Until recently, Randy also served as Chairman of KeraLink (formally TBI), an international non-profit organization focused on reversing corneal blindness through ocular transplantation.

During his time as CEO of Orisis Therapeutics, Inc., Randy won approval for the first stem cell drug to treat pediatric GVHD and created an international expanded access program for a cell therapy drug that treats transplant patients suffering from refractory GVHD. He also made tissue transplantation safer when he invented BioCleanse®, the first sterilization system for human tissue transplantation that was accepted by the FDA, while at Regeneration Technologies, Inc.

Randy is a biotech pioneer, innovator, and entrepreneur who has advanced each company he led and made significant contributions to science. He also serves on several boards and has been recognized for his work by organizations such as Ernst & Young, Deloitte, and the World Regenerative Medicine Congress.

Randy's experience and understanding of the importance of our research will make him a great partner to the CIBTMR. Please join us in welcoming him.

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Infection and Immune Reconstitution Working Committee

Committee Leadership

Co-Chairs:

- Jeffery Auletta, MD, Nationwide Children's Hospital, Columbus, OH
- Caroline Lindemans, MD, PhD, University Medical Center Utrecht
- Krishna Komanduri, MD, University of Miami

Scientific Director:

- Marcie Riches, MD, MS, University of North Carolina Hospitals

Statistical Director:

- Soyoung Kim, PhD, CIBMTR

Statistician:

- Min Chen, MS, CIBMTR

Based on the 2016 CIBMTR Summary Slides, infection is reported as the primary cause of death in up to 18% of patients receiving autologous and allogeneic transplantation. However, transplant clinicians recognize that infection accounts for significantly greater morbidity in our patients. This burden of infections and its correlation with post-transplant immune reconstitution is the focus of our committee's efforts.

The Infection and Immune Reconstitution Committee faces unique challenges due to the complex interactions of multiple time-dependent co-variates as well as the complexities associated with reporting multiple (and often recurrent) infections caused by a diverse range of pathogens. The rates of post-transplant infections are also associated with a variety of factors, including graft type, donor-recipient mismatch, recipient age, and GVHD incidence. Furthermore, infections prior to the transplant impact transplant outcomes. Given the complexity of these factors, our analyses rely heavily on novel statistical techniques provided by our excellent Statistician, Min Chen, MS, and our Statistical Director, Soyoung Kim, PhD, with the assistance of Kwang Woo Ahn, PhD.

The committee recognizes that data collection for infections and immune reconstitution is complex and particularly demanding. In part, the significant effort required to document infection endpoints has contributed to under-reporting biases evident in prior analyses. Given this complexity, we greatly appreciate the time involved by all data managers to provide us with the high-quality data needed to understand and improve infection-related transplant outcomes. To streamline and improve the quality of infection-related data, the committee leadership formed a task force with the ASBMT Infectious Disease Special Interest Group to redesign data collection mechanisms.

In January 2017, the CIBMTR released revisions to Form 2100 for infection prophylaxis, revised organisms of interest for capture, and capture at limited sites of interest. Revisions to the Data Manager Manual to facilitate collection accompanied the release. Furthermore, revisions to the fungal forms (2046 / 2146) and new forms to obtain detail on CMV / Adenovirus / EBV / HHV-6 (2150), and upper respiratory viruses (2149) are anticipated for release later this year. These revisions, developed in collaboration with data managers, capture limited information about diagnosis, treatment, and response to therapy. These forms are triggered forms based upon the organism reported and accounting for the site of infection. While limited in scope, the data will provide investigators a more global picture of infection complications following transplant. Dr. Riches presented these forms at the Data Managers Meeting and to the committee at the 2017 BMT Tandem Meetings in Orlando.

The past academic year has been quite productive. In addition to the activities of forms revision, investigators published four manuscripts of study results. Four additional studies currently have five manuscripts in preparation of final results. While all studies to date have focused on infections, the committee desires to expand our efforts to study correlates of immune recovery, including lymphocyte subsets and immunoglobulin levels, especially as the quantity and quality of these data improve over time. The committee welcomes proposals to further investigate these issues.

View planned, in-progress and completed studies and publications on the Infection and Immune Reconstitution Working Committee webpage.

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Health Services and International Studies Working Committee

Committee Leadership

Co-Chairs:

- Jignesh Dalal, MD, Seidman Cancer Center-University Hospitals Cleveland Medical Center
- Theresa Hahn, PhD, Roswell Park Cancer Institute
- Nandita Khera, MD, Mayo Clinic Arizona and Phoenix Children's Hospital
- William Wood, MD, MPH, University of North Carolina Hospitals

Scientific Director:

- Wael Saber, MD, MS, CIBMTR

Statistical Director:

- Ruta Brazauskas, PhD, CIBMTR

Statistician:

- Naya He, MPH, CIBMTR

Consumer Advocacy Committee Representative

- Jack Aiello

The main goal of Health Services and International Studies Working Committee (HSIS WC) is to help improve the practice of HCT through health services research at a global level. The HSIS WC is the result of the 2013 merger of the Health Policy / Psychosocial Issues WC and the International Studies WC. We bring together an enthusiastic and diverse group of BMT investigators worldwide, representing varied clinical and research backgrounds. Studies in this WC include population-based studies to understand better questions around disparities in access and outcomes of HCT, practice patterns in HCT, and impact of HCT related variables on outcomes other than survival, such as costs and health care utilization. To complete these studies, we not only query the CIBMTR Research Database, but we also link it to other large databases both nationally and internationally to get a true population-based perspective. In addition, our WC strives to ensure good quality registry data by understanding gaps in follow-up, lead efforts in the area of improving international data collection, and help improve outcomes reporting by the CIBMTR by studying the impact of social determinants on outcomes. Our WC gives international centers the opportunity to ask research questions regarding their populations.

In the last five years, our combined WC published 19 papers, and our current portfolio contains 10 active studies. The HSIS WC works closely with the CIBMTR Health Services Research Program operated by NMDP/Be The Match's Patient and Health Professional Services department. The Health Services Research Program typically conducts investigator-initiated studies that require expertise and resources beyond those usually needed for CIBMTR studies. Currently, the HSIS WC and Health Services Research Program are partnering on a study, Safety and Cost-Effectiveness of Outpatient Autologous Stem Cell Transplantation: A Multicenter Retrospective Case-Control Study Using Propensity Score Matching.

If you have an idea for an innovative outcomes-related or international study, let us know. View planned, in-progress and completed studies and publications on the Health Services and International Studies Working Committee webpage.

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CIBMTR Trivia

How many lay summaries of CIBMTR research, written specifically for patients and their loved ones, are posted on the CIBMTR website?

A. 15
B. 30
C. 60
D. 120

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

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2018 BMT Tandem Meetings

By Tia Houseman

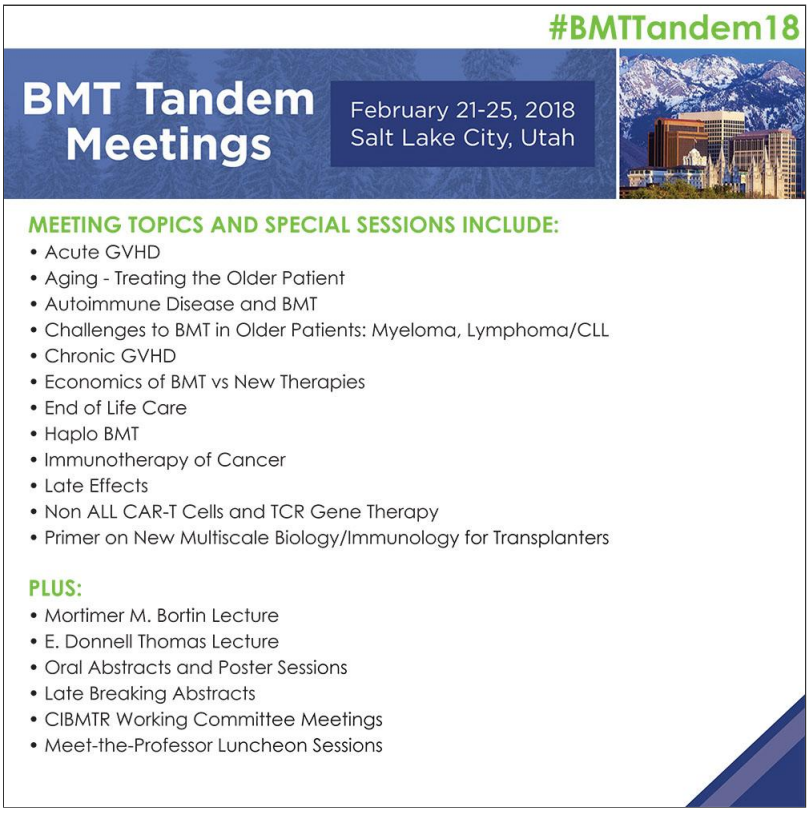
The BMT Tandem Meetings - the combined annual meetings of the CIBMTR and ASBMT - are North America's largest international gathering of BMT clinicians and investigators, laboratory technicians, advanced practice professionals, transplant nurses, pharmacists, administrators, and clinical research associates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

Leading authorities from around the world will present the latest developments in blood and marrow transplantation February 21-25, 2018, during the BMT Tandem Meetings at the Salt Palace Convention Center in Salt Lake City, Utah. Along with state-of-the-art educational offerings, industry-supported satellite sessions and product theaters will broaden the spectrum of presentations. In addition to an outstanding scientific program, the 2018 meetings offer peripheral sessions for BMT pharmacists, center administrators, coordinators, investigators, medical directors, clinical research professionals / data managers, transplant nurses, and advanced practitioners.

The online registration, abstract, and housing site opens mid-August. The early registration and abstract deadline is October 3. After registering, take advantage of special conference guest room rates offered at several hotels in the BMT Tandem Meetings housing block near the Salt Palace Convention Center. Don't forget to reserve your ticket to the Saturday evening Tandem Reception to end a memorable week with colleagues and friends!

Questions regarding support opportunities at the 2018 BMT Tandem Meetings may be directed to Sherry Fisher, Director of Advancement for the CIBMTR. For general information, email bmttandem@mcw.edu.

We look forward to seeing you in Salt Lake City!



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Team Spotlight: Health Services Research Program

The CIBMTR Health Services Research Program includes Lih-Wen Mau, PhD Investigator / Supervisor; Tatenda Mupfudze, PhD-level Health Services Research Analyst; Christa Meyer and Jaime Preussler, MS-trained Analysts; and Ellen Denzen, MS, Senior Manager. Oversight is provided by Linda Burns, MD, Vice President and Senior Scientific Director, and Elizabeth Murphy, Vice President, Patient and Health Professional Services and Education and Training. The Program is located in Minneapolis at the National Marrow Donor Program/Be The Match Coordinating Center.

The Program's vision is to conduct research that contributes knowledge to the transplant field and informs policy, clinical practice, and survivorship issues, while

fostering a culture of learning, integrity, and excellence. The overarching goals are to identify and address barriers to access to HCT, improve practice of HCT, and demonstrate the value of cellular therapies (compared to other therapies) and survivorship care through research and dissemination of findings.
Data sources are varied and include public and commercial administrative claims linked to the CIBMTR Research Database clinical outcomes, prospective assessments, survey research, and qualitative interviews. The Health Services Research Program currently has 11 studies in progress and has published 18 papers since its creation in 2006. Select studies include:

- **Health Economics Research Portfolio**
  - **Reimbursement Analysis of HCT in Older Patients with AML (HSR 15-01):** Aims to describe trends in HCT-related reimbursement within the Medicare patient population using linked CMS administrative claims data and CIBMTR clinical data.
  - **Cost-Effectiveness Analysis of Allogeneic HCT versus Chemotherapy Alone in Patients with AML (HSR 16-05):** Aims to describe health care costs, utilization, and cost-effectiveness for patients treated with either chemotherapy alone or allogeneic HCT for AML using Optum Clinformatics and National Death Index data.
- **Survivorship and Quality of Life Portfolio**
  - **Developing a Patient-Centered HCT Outcomes Research Agenda** (funded by PCORI Eugene Washington Engagement Award): Engages patients, caregivers, and other key stakeholders in developing a patient-centered HCT outcomes research agenda; prioritized recommendations can inform the development of comparative effectiveness research protocols as well as recommend focus areas to public and private funders.
  - **Two webinars are planned** for this fall to disseminate the prioritized research questions as they were presented at the 2017 BMT Tandem Meetings in February.
- **Treatment Decision-Making Support portfolio**
  - **Impact of BMT CTN 0201 on Clinical Practice (HSR 16-01):** Aims to determine impact of BMT CTN 0201 (PBSC vs BM) on clinical practice and to understand how research findings are translated into practice among HCT physicians and transplant centers.
  - **AML Matters program:** In partnership with the ASH, the Oncology Nursing Society, and The France Foundation, the program aims to identify knowledge gaps and practice trends among multidisciplinary health professionals and deliver innovative education interventions to support treatment decision-making for patients with AML. The AML Matters Summit was held at the NMDP/Be The Match headquarters in Minneapolis on July 28.

The Health Services Research Program collaborates closely with internal and external partners by contributing research design and methodology expertise, grant proposal analytic support, administrative support and data collection services. Examples of partners include the BMT CTN, RCI BMT, Survey Research Group and NMDP/Be The Match Payer Policy, independent investigators, ASBMT, ASH, and Oncology Nursing Society. Funding for studies is provided in part by PCORI, the National Comprehensive Cancer Network / Pfizer, NIH, HRSA, and others.

Back row left to right:
Jaime Preussler, Christa Meyer, Ellen Denzen, Beth Murphy, Linda Burns

Front row left to right:
Tatenda Mupfudze, Robynn Erdman, Lih-Wen Mau

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Data Collection

By Tiffany Hunt, MS, CCRP, and Emilie Love, CSPO

Over the past year, teams of experts worked to revise and develop CIBMTR data collection forms was to capture the newest and most relevant data. As a result, 10 revised forms were released in July:

Form Name	Form Number
Pre-Cellular Therapy Essential Data (Pre-CTED)	4000
Cellular Therapy Infusion	4006
Post-Cellular Therapy Essential Data (Post-CTED)	4100
AML Disease Inserts	2010 / 2110
ALL Disease Inserts	2011 / 2111
Pre-TED Disease Classification (AML and ALL sections only)	2402
Fungal Infection Forms	2046 / 2146

Release Highlights

The Disease Classification Form 2402 (formerly Pre-TED Disease Classification) and the ALL Disease Inserts are now used to capture data for cellular therapies. This change was made to ensure all necessary disease specific data are captured, reduce duplication across forms, and provide consistency in data reporting.

For more details on form changes and the form revision process, please contact Emilie Love or Tiffany Hunt .

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Blood and Marrow Transplant Clinical Trials Network Update

By Amy Foley, MA

The BMT CTN has enrolled more than 9,500 patients. The Network was established in 2001 and is 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 new 7-year cycle started July 1 and ushered in 20 Core Centers, 9 of which are consortia of two or more centers. Congratulations to the continuing and new Core and Consortium Centers!

Clinical Trials: Open Enrollment

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

There are 12 trials open to accrual, 2 released to centers, and 2 in development. The following BMT CTN protocols were recently released to centers:

- BMT CTN 1506: A Multi-center, Randomized, Double-blind, Placebo-controlled Phase III Trial of the FLT3 Inhibitor Gilteritinib Administered as Maintenance Therapy Following Allogeneic Transplant for Patients with FLT3/ITD AML.
- BMT CTN 1507: Reduced Intensity Conditioning for Haploidentical Bone Marrow Transplantation in Patients with Symptomatic Sickle Cell Disease.

BMT CTN Publications

There are 76 BMT CTN published articles, including 21 primary analyses. The following secondary analysis manuscripts were recently published, representing the tenth and fourth 0302 and 0402 publications, respectively!

- 0302: Arora M et al. **Pharmacogenetics of steroid responsive acute graft-versus-host disease.** Clinical Transplantation. 2017 May 1;31(5). Epub 2017 Apr 4. PMC5413396. https://www.ncbi.nlm.nih.gov/pubmed/28266732
- 0402: Holtan SG et al. **Low EGF after myeloablative allotransplantation: Association with severe acute GVHD in BMT CTN 0402.** Bone Marrow Transplantation. 2017 Jun 5. [Epub ahead of print] https://www.ncbi.nlm.nih.gov/pubmed/28581470

About the BMT CTN

The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with National Marrow Donor Program/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)

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

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

Survivorship

The Health Services Research Program is excited to announce a new research collaboration to integrate health informatics in a scalable stepped care self-management program for HCT survivors after. Co-principal investigators of the five-year NIH R01 funded project are Karen Syrjala, PhD, and K. Scott Baker, MD, MS, from the Fred Hutchinson Cancer Research Center and Navneet Majhail, MD, MS, from the Cleveland Clinic. The Health Services Research Program will lend its qualitative research expertise to the development, conduct, and interpretation of patient focus groups as well statistical support for the grant.

Analysis is underway for the HSR-13 Survivorship Care Plan prospective, randomized study that compared individualized care plans for transplant survivors (based on CIBMTR data) with institutional standard care. The project is funded by PCORI and led by Co-PIs Navneet Majhail, MD, MS, from the Cleveland Clinic; Scott Baker, MD, MS, from the Fred Hutchinson Cancer Center; and Beth Murphy, EdD, RN, of the Health Services Research Program. Purushottam Laud, PhD, of the Medical College of Wisconsin and Jamie Preussler, MS, from the Health Services Research Program are conducting the analyses. We look forward to submitting our results for presentation at the ASH Annual Meeting.

Both projects involve numerous contributions by members of the RCI BMT staff led by Roberta King.

Patient-Centered Outcomes Research webinars planned for September and October

Another PCORI-supported project, Engaging Patients in Developing a Patient-Centered HCT Research Agenda, held its final symposium at the 2017 BMT Tandem Meetings. Six Working Groups outlined their final recommendations on HCT research questions that matter most to patients. Working Group Co-Chairs, patients, and caregivers delivered joint presentations that provided unique and complementary perspectives. Webinars are planned on Tuesday, September 26, 1:00 – 2:00 pm CT and Wednesday, October 18, 2:00 – 3:00 pm CT to review the top research priorities for each of the Working Groups. Make plans to tune in as we move forward to establish the research agenda and build a collaborative research community that is patient-centered.

Register today:

- Tuesday , Sept. 26 | Setting a Patient-Centered Outcomes Research Agenda in HCT: Priorities in Sexual, Physical, and Emotional Health
- Wednesday , Oct. 18 | Setting a Patient-Centered Outcomes Research Agenda in HCT: Priorities in Education, Care Delivery, and Financial Burden

For any questions about the HSR Program, contact Ellen Denzen, MS, Senior Manager, Health Services Research or visit the Health Services Research webpage.

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Four New Patient Summaries of CIBMTR Research

By Jessica Gillis-Smith, MPH

Four new patient summaries of CIBMTR publication were recently posted on the CIBMTR Patient Resources webpage.

- Cord blood transplant helps some people with myelodysplastic syndromes
  - If someone with MDS does not have a matched family member or unrelated donor, a cord blood transplant might help.
- After donating blood-forming cells, older people report similar quality of life as younger people
  - Siblings 60 and older can donate blood-forming cells.
  - Older donors have similar quality of life after donation as younger donors.
- Less chronic GVHD after cord blood transplant for acute leukemia
  - Patients had less chronic GVHD after cord blood transplants.
  - Patients who received ATG had less acute GVHD.
- How to make transplant work best for myelodysplastic syndromes
  - There are many things to think about before using transplant to treat MDS. Doctors and patients should think about the timing, the donor, the preparative regimen, and the cells.

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 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.

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Abbreviations

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

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Last Updated: 10/30/2017 2:02 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