August 2015 Newsletter

Volume 21, Issue 3

Table of Contents:

Perspectives

By Paul Martin, MD

The American Society of Clinical Oncology (ASCO) recently published the initial work of its Value in Cancer Care Task Force (1). The Task Force presents a conceptual framework to assess the value of cancer care treatment options, as informed by randomized clinical trials comparing a new treatment against a current standard of care. This statement deserves attention from the HCT community.

The ASCO statement proposes one framework for potentially curative adjuvant or neoadjuvant therapy and another framework for non-curative treatment of advanced disease. Each framework represents an important step toward developing methods that can explain the value of cancer treatment to patients by estimating the net health benefit and the costs of treatment. Net health benefit is estimated as prolongation of survival weighed against the incidence and severity of high-grade acute toxicity, including treatment-related mortality. Due to lack of data, the current framework does not account for chronic low-grade toxicity. Costs

include drug-acquisition expenses and co-payments borne by patients. In the current framework, cost accounting does not include hospital use; emergency services; lost earnings; or expenses for travel, childcare, or caregiver support.

The work of the ASCO Task Force challenges us to consider how we might develop a similar approach to explain the value of HCT to patients. We would need at least two different frameworks, one for indolent diseases such as myelodysplastic syndromes and multiple myeloma that cannot be cured by conventional treatment and another for acute hematologic malignancies that can be cured by conventional treatment. Data directly comparing two different transplant methods might be available to address a few questions, but data comparing transplant and non-transplant alternatives have generally relied on donor versus no donor comparisons. Often, the most pertinent question is the optimal timing of transplantation during the disease course.

A transplant framework would have to account for chronic toxicity, including chronic GVHD and the effects of prolonged glucocorticoid treatment, impaired fertility, secondary cancers, and shortened life expectancy as well as the financial burdens associated with these complications. Patients typically become fully aware of these risks only when they move beyond the risks of reversible and sometimes irreversible regimen-related toxicity, infections, and acute GVHD during the first few months after transplantation.

Development of a transplant framework will require years of effort. Do we have any way to explain the value of transplantation in the meantime? As a qualitative approach, the Seattle group has assembled an annual anthology of comments from patients surviving for as long as 40 years after transplantation. These anthologies use the words of patients to characterize a wide spectrum of outcomes.

Patients rely on us for the specialized knowledge to evaluate their treatment options and to explain the efficacy, toxicity, effects on quality of life, and costs. In making patient-centered recommendations, we must also consider patient age and comorbidities as well as the values, goals, life circumstances, and religious or spiritual beliefs of each individual patient. Comments from survivors offer an emotionally engaging and eloquent additional approach that patients can use to understand the value of transplantation.

Publication Cited in this Article

1. Schnipper LE et al. **American Society of Clinical Oncology statement: A conceptual framework to assess the value of cancer treatment options.** Journal of Clinical Oncology. DOI:10.1200/JCO.2015.61.6706. Epub 2015 Jun 22.

Return to Top

Congratulations to 4 Working Committees

After the 2014-2015 review of the CIBMTR's Scientific Working Committees, four have been recognized as outstanding by the Advisory Committee:

- Chronic Leukemia
- Graft Sources and Manipulation
- Late Effects and Quality of Life
- Lymphoma

These committees were identified because of their strong promotion of studies that will have a notable impact on the field, willingness to forgo studies that will not have a significant impact on the field, and focus on the effective use of CIBMTR resources. In addition, these committees have cultivated external collaborations and are notably well-organized and highly productive.

Congratulations to the leadership of these committees:

- Edwin Alyea, MD – Chair, Chronic Leukemia
- Asad Bashey, MD, PhD – Chair, Graft Sources and Manipulation
- Minoo Battiwalla, MD, MS – Chair, Late Effects and Quality of Life
- Mary Eapen, MD, MS – Scientific Director, Graft Sources and Manipulation
- Timothy Fenske, MD, MS – Chair, Lymphoma
- Mary Flowers, MD – Chair, Late Effects and Quality of Life
- Mehdi Hamadani, MD – Scientific Director, Lymphoma
- Miguel-Angel Perales, MD – Chair, Graft Sources and Manipulation
- Uday Popat, MD – Chair, Chronic Leukemia
- Vanderson Rocha, MD, PhD – Chair, Graft Sources and Manipulation
- Wael Saber, MD, MS – Scientific Director, Chronic Leukemia
- Bipin Savani, MD – Chair, Late Effects and Quality of Life

- Bronwen Shaw, MBChB, MRCP, PhD – Scientific Director, Late Effects and Quality of Life
- Sonali Smith, MD – Chair, Lymphoma
- Ronald Sobecks, MD – Chair, Chronic Leukemia
- Anna Sureda, MD – Chair, Lymphoma

Return to Top

Health Services and International Issues Working Committee

Yoshiko Atsuta,
MD, PhD,
Chair

Theresa Hahn,
PhD,
Chair

The Health Services and International Issues Working Committee (HSIS WC) has a complementary but distinct study portfolio from other disease or outcome based WCs of the CIBMTR. We focus on population-based studies, health services research, health care and resource utilization, disparities research, secondary data analysis, clinical practice variation (on a national and global scale), international studies, survey research, and more! We have experience merging CIBMTR data sets with those from the Japan Society for Hematopoietic Cell Transplantation, US CMS / Medicare, US Veteran's Administration Database, BMT CTN, Pediatric Health Information Service, and others. We are also familiar with the use of publicly available data such as SEER, US zip codes, US census, national GDP, etc. 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. Our WC gives the opportunity for international centers to ask research questions regarding their populations. If you want to ask and answer a really big question or have an idea for an innovative outcomes-related or international study, join us at the next BMT Tandem Meetings to see our committee in action.

Committee Leadership

Co-Chairs:

- Yoshiko Atsuta, MD, PhD, Nagoya University Graduate School of Medicine, Japan
- Carmem Bonfim, MD, PhD, Hospital de Clinicas – UFPR, Brazil
- Jignesh Dalal, MD, The Children's Mercy Hospitals and Clinics, Kansas City, MO
- Theresa Hahn, PhD, Roswell Park Cancer Institute, Buffalo, NY
- Nandita Khera, MD, Mayo Clinic Arizona and Phoenix Children's Hospital, Phoenix, AZ

Scientific Director:

- Wael Saber, MD, MS

Statisticians:

- Ruta Brazauskas, PhD
- Naya He, MPH

In the last five years, our combined WC has published 15 papers, including these examples:

- Knight et al. **Impact of socioeconomic status on gene expression in leukocytes of transplant recipients.** [Submitted]
- Khera, et al. **Comparison of characteristics and outcomes of patients enrolled vs not enrolled on the BMT CTN 0201 trial.** [Biology of Blood and Marrow Transplantation, In press]
- Ballen KK et al. **Hospital length of stay in the first 100 days after allogeneic hematopoietic cell transplantation for acute leukemia in remission: comparison among alternative graft sources.** Biology of Blood and Marrow Transplantation. 2014 Nov 1; 20(11):1819-1827. doi:10.1016/j.bbmt.2014.07.021. Epub 2014 Jul 23. PMC4194253.
- Kuwatsuka Y et al. **Graft-versus-host disease and survival after cord blood transplantation for acute leukemia: a comparison of Japanese versus White populations.** Biology of Blood and Marrow Transplantation. 2014 May 1; 20(5):662-667. doi:10.1016/j.bbmt.2014.01.020. Epub 2014 Feb 10. PMC4071962.
- Wood WA et al. **Survival improvements in adolescents and young adults after myeloablative allogeneic transplantation for acute lymphoblastic leukemia.** Biology of Blood and Marrow Transplantation. 2014 Jun 1; 20(6):829-836. doi:10.1016/j.bbmt.2014.02.021. Epub 2014 Mar 7. PMC4019683.
- Majhail NS et al. **Prevalence of hematopoietic cell transplant survivors in the United States.** Biology of Blood and Marrow Transplantation. 2013 Oct 1; 19(10):1498-1501. doi:10.1016/j.bbmt.2013.07.020. Epub 2013 Jul 29. PMC3779514.
- McCarthy PL Jr. et al. **Trends in use of and survival after autologous hematopoietic cell transplantation in North America, 1995-2005: significant improvement in survival for lymphoma and myeloma during a period of increasing recipient age.** Biology of Blood and Marrow Transplantation. 2013 Jul 1; 19(7):1116-1123. doi:10.1016/j.bbmt.2013.04.027. Epub 2013 May 6. PMC3694566.
- Eckrich M et al. **Hematopoietic cell transplantation in Latin America.** Hematology. 2012 Apr 1; 17(Suppl 1):S189-S191. doi:10.1179/102453312X13336169157059.

Our current portfolio contains 11 active studies:

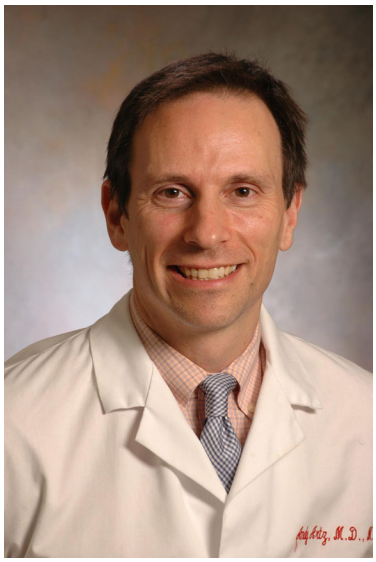
- IS09-02 - Outcomes comparison of the effect of HLA-matched sibling donor transplantation for severe aplastic anemia across different regions
- IS13-01 - Impact of ethnicity on GVHD rates after HLA-matched related bone marrow or PBSC transplantation for leukemia
- HS12-02 - Rates of transplantation in urban vs rural patients: Are rural patients less likely to receive an allogeneic transplant?
- HS13-01 - The effect of pre-transplant depression on outcomes of HCT for hematologic malignancies
- HS13-02 - Investigating inpatient health care utilization of matched sibling donor HCT for children with sickle cell disease
- HS14-01 - Investigating clinical outcomes and inpatient health care resource utilization of HCT for children with acute leukemia
- HS07-02b – Long-term financial impact of allogeneic HCT on patient and family
- IS10-02 - Total body irradiation prior to HCT: a pattern of care study
- IS10-01 - Outcomes of HCT for ALL: an international comparative analysis
- HS15-01 - Who is lost to follow-up in the CIBMTR registry?
- HS15-02 - Impact of socioeconomic status on pediatric HCT outcomes

The HSIS WC also collaborates 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 three studies, one of which is conducted through the WC:

- Patient and caregiver out-of-pocket costs after allogeneic transplant – this pilot study includes two phases. Phase 1 examined the feasibility of collecting out-of-pocket costs using a diary [citation below]. Phase 2 described the financial impact of transplant two years post-HCT [in manuscript preparation].
    - Majhail NS et al. **Pilot study of patient and caregiver out-of-pocket costs of allogeneic hematopoietic cell transplantation.** Bone Marrow Transplantation. 2013 Jun 1; 48(6):865-871. doi:10.1038/bmt.2012.248. Epub 2012 Dec 10. PMC3596484.

Return to Top

Regimen-Related Toxicity and Supportive Care Working Committee



Andrew Artz,
MD, MS,
Chair

The Regimen-Related Toxicities and Supportive Care Working Committee (RRTWC) was created to study factors associated with morbidity and mortality after HCT. RRTWC studies focus on patient- and transplant-related factors that may contribute to toxicity and mortality. The main goal of this committee is to advance approaches that make transplants safer.

Committee Leadership

Co-Chairs:

- Andrew Artz, MD, MS, University of Chicago Hospitals, Chicago, IL
- Alison Loren, MD, MS, University of Pennsylvania Medical Center, Philadelphia, PA
- Shin Mineishi, MD, University of Alabama at Birmingham, Birmingham, AL

Scientific Director:

- Marcelo C. Pasquini, MD, MS

Statisticians:

- Brent Logan, PhD
- Xiaochun Zhu, MS

Over the past year, the RRTWC has completed important studies focusing on conditioning regimens, early transplant complications, and comorbidities. Patient factors, unrelated to disease, are closely linked with outcomes. Age and performance score are frequently applied to assess eligibility and to estimate transplant or non-relapse related mortality. The development of the hematopoietic cell transplantation-comorbidity index (HCT-CI) by Sorror et al (Blood 2005) showed that a systemic evaluation of key comorbidities present at the time of transplant can assist in predicting post-transplant outcomes, such as non-relapse mortality and overall survival. The CIBMTR and the RRTWC collaborated with Sorror by incorporating the elements from HCT-CI in the pre-TED form with the intent of validating this tool (RT07-01 – Prospective validation of the impacts of the HCT–CI, alone and combined with aging on HCT outcomes for malignant diseases). The data was collected prospectively over two years, and the results were recently published (1). We validated and quantified the effect of higher comorbidity with adverse outcomes in allogeneic transplants in myeloablative and reduced intensity settings, as well as in autologous transplants. Interestingly, the study evaluated the degree of agreement between the HCT-CI coding from the TED form and a clinician at the same center. Among four centers, there was a significant variability in HCT-CI scores, which reinforces the need for education to standardize HCT-CI scores. Clearer guidance is now available, including an online calculator. An additional question currently being addressed is the ability of HCT-CI to predict early mortality after transplants for non-malignant diseases. The registry

now contains HCT-CI on all patients since 2007, which improves adjustment and comparisons for all CIBMTR studies.

Our committee helps investigators identify additional patient characteristics associated with outcomes. Artz conducted a study [RT10-01 – C-reactive protein (CRP), albumin and ferritin to predict non-relapse mortality after allogeneic HCT] to validate the impact of ferritin, albumin, and CRP on overall survival and transplant-related mortality. Assessment of ferritin and CRP was done by ELISA at the University of Chicago while albumin levels were reported by centers. The results confirmed that these three biomarkers were associated with mortality post-transplant, independent of comorbidity and other factors, and they could be added to a pre-transplant assessment to better refine predictions of morbidity and mortality.

The RRTWC conducted several studies to assess the impact of obesity on transplant outcomes. The most recent study on obesity by Aplenc and Bunin et al (RT 0902 - Effect of body mass in children with hematologic malignancies undergoing allogeneic bone marrow transplantation) focused on children with malignant diseases. The results demonstrated no significant impact of obesity, measured by age-adjusted body mass index, on transplant outcomes (2).

Conditioning regimen strongly influences transplant toxicity and, as a modifiable factor, is particularly relevant. The RRTWC assisted in the creation of the currently used working definition of conditioning regimen intensity for CIBMTR analyses (3).

During the past year, the RRTWC conducted several studies addressing different aspects of conditioning regimens. Hong et al (RT12-04 - Comparison of non-myeloablative conditioning regimens for lymphoproliferative disorders) compared non-myeloablative regimens used for transplants in lymphoma based on the use of total body irradiation (TBI) (4). The study demonstrated that TBI-based non-myeloablative conditioning regimens for lymphoproliferative diseases are currently used less frequently than chemotherapy-based regimens. The latter are similar to regimens commonly used in the non-transplant setting. The outcomes after transplant between regimens with or without TBI were similar. Chen and Lane et al (RT11-01 Impact of conditioning regimen on outcomes for patients with lymphoma undergoing high-dose therapy with autologous HCT) analyzed different conditioning regimens for autologous transplant in lymphoma (5). The study compared commonly used conditioning regimens and demonstrated disparate outcomes according to the regimen and the type of lymphoma histology. The results suggest a more tailored approach in selecting a high dose regimen for lymphoma. For example, the use of busulfan-based regimens in Hodgkin disease was associated with worse outcomes than a BEAM (carmustine, etoposide, cytarabine, melphalan) regimen, and CBV (cyclophosphamide, carmustine and etoposide) with standard carmustine dose (300mg/m^2) in follicular lymphoma was associated with better outcomes than other regimens.

Another conditioning question related to the sequence of agents was answered by the publication by Holter-Chakrabarty et al (RT 1201 - The sequence of cyclophosphamide and myeloablative TBI in HCT for patients with acute leukemia) where the sequence of TBI and cyclophosphamide (Cy) for acute leukemia was explored (6). The findings demonstrated that CyTBI and TBICy were equally common and not defined by center. Outcomes were similar between the two different sequences, arguing against previous concerns with altered TBI sequence and worse outcomes. The RRTWC also developed a prospective cohort study sponsored by Otsuka Pharmaceutical Development and Commercialization to compare intravenous busulfan (BU) based myeloablative conditioning regimen to TBI based conditioning. The results of this large cohort study demonstrated that the outcomes with Bu were superior to TBI for myeloid malignancies undergoing myeloablative regimens (7).

Primary graft failure was also addressed by the RRTWC. The first study by Schriber et al reported on dismal outcomes for second allogeneic transplants for patients who experienced primary graft failure (8). In a follow-up study by Olsson et al (RT 0901 Primary graft failure following allogeneic HCT for the treatment of hematological malignancies – Leukemia 2015), factors associated with the development of graft failure were developed and a score was proposed to identify patients with high risk of graft failure (9).

The RRTWC collaborates with Hahn and Sucheston-Campbell on a large genome-wide association study to identify genetic determinants for early mortality after unrelated donor HCT [RT09-04/IB09-06a - Adjudication of cause-specific mortality after unrelated donor allogeneic HCT for acute leukemia and myelodysplastic syndrome: The primary endpoint of a future genome-wide association study (GWAS)]. The adjudication of causes of death reported to the CIBMTR and a proposal for ranking and categorization for this and other studies was recently published (10).

Moving forward, the RRTWC has a comprehensive portfolio of new studies. The major themes of approved studies include exploring toxicity in pediatric transplants, assessing determinants of critical care in transplant patients, reporting on incidence and prognostic factors for endothelial injury syndromes, and updating conditioning regimen intensity definitions.

Publications Cited in this Article

1. Sorror ML et al. **Prospective validation of the predictive power of the HCT-CI: A CIBMTR study.** Biology of Blood and Marrow Transplantation. Epub 2015 Apr 7.
2. Aplenc R et al. **Effect of body mass in children with hematologic malignancies undergoing allogeneic bone marrow transplantation.** Blood. 2014 May 29; 123(22):3504-3511.
3. Bacigalupo A et al. **Defining the intensity of conditioning regimens: working definitions.** Biology of Blood and Marrow Transplantation. 2009 Dec 1; 15(12):1628-33.
4. Hong S et al. **Comparison of non-myeloablative conditioning regimens for lymphoproliferative disorders.** Bone Marrow Transplant. 2014 Mar 1; 50(3):367-374.
5. Chen YB et al. **Impact of conditioning regimen on outcomes for patients with lymphoma undergoing high-dose therapy with autologous HCT.** Biology of Blood and Marrow Transplantation. 2015 Jun;21(6):1046-53.
6. Holter-Chakrabarty JL et al. **The sequence of cyclophosphamide and myeloablative TBI in HCT for patients with acute leukemia.** Biology of Blood and Marrow Transplantation. 2015 Jul;21(7):1251-7.
7. Bredeson C et al. **Prospective cohort study comparing intravenous busulfan to TBI in HCT.** Blood. 2013 Dec 5;122(24):3871-8.
8. Schriber J et al. **Second unrelated donor HCT for primary graft failure.** Biology of Blood and Marrow Transplantation. 2010 Feb 18.
9. Olsson RF et al. **Primary graft failure after myeloablative allogeneic HCT for hematologic malignancies.** Leukemia. 2015 Mar 16.
10. Hahn T et al. **Establishment of definitions and review process for consistent adjudication of cause-specific mortality after allogeneic unrelated-donor HCT.** Biology of Blood and Marrow Transplantation. 2015 May 29.

Return to Top

How You Can Help HCT Research Remain an NHLBI Priority

Participate in the NHLBI Strategic Visioning Process

This past Spring, Gary Gibbons, MD, Director of the NHLBI, invited the NHLBI community "to help identify the most compelling questions and critical challenges that we must tackle in the coming years if we are to take full advantage of emerging scientific opportunities and bold new approaches for actively promoting human health, as well as significantly reducing and preventing disease." More than 1,200 ideas were submitted by the deadline of May 15, including a number related to HCT research. These ideas aligned with the NHLBI's four strategic goals:

- Promote human health
- Reduce human disease
- Advance translational research
- Develop workforce and resources

Individuals were encouraged to vote either for or against ideas. CIBMTR investigators submitted many questions and challenges, including these 7, which were in the top 40 most popular ideas:

- Allogeneic transplantation as a safe and universally available therapeutic strategy for treating non-malignant blood diseases (Richard Jones, MD)
- Can we leverage existing registries to perform prospective trials and reduce the cost of doing research? (Sergio Giralt, MD)
- The importance of the microbiome in recovery after hematopoietic stem cell transplantation (Mary Horowitz, MD, MS)
- What is the place of curative therapies in the management of sickle cell disease? (Lakshmanan Krishnamurti, MD)
- Transplantation across HLA barriers in aplastic anemia (Joseph Antin, MD)
- How can we develop more selective immunosuppression for allogeneic hematopoietic cell transplantation? (Krishna Komanduri, MD)
- Cellular therapy of blood diseases (Mary Horowitz, MD, MS)

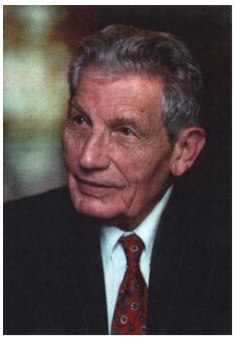
The NHLBI and advisory groups reviewed and synthesized the ideas and created the Draft Strategic Research Priorities, on which the NHLBI is now seeking public input. Share your comments to emphasize the importance of HCT research. In particular, take note of these priorities related to HCT and the work of the CIBMTR:

- 2.A.3: What are the mechanisms for the late development of complications or new clinical programs after HCT? How can these consequences be deterred early and prevented to reduce the high rates of mortality following HCT?

- 2.C.1: How can improved methods for HCT or gene therapeutic approaches be used to cure sickle cell disease?
- 3.D.4: How can one leverage existing registries to perform prospective trials at reduced cost?
- 3.G.1: How can we optimize HCT safety and make the procedure a more universally effective treatment for non-malignant blood and immune disorders?
- 3.G.2: How can we use novel agents to provide more selective immunosuppression for patients who receive allogeneic HCT? Such therapies would target donor immune responses that are related to graft-versus-host disease while preserving pathogen-specific host immunity so that HCT can be applied to a broader range of non-malignant diseases and to recipients who lack well-matched donors.

Return to Top

In Remembrance of Dr. Dick W van Bekkum



Dick van Bekkum, one of the pioneers of BMT, passed away on July 17 after a short illness. He would have turned 90 on July 30. Professor van Bekkum dedicated his life to science and medicine, contributing to the HCT field in numerous ways, including seminal contributions to the IBMTR. Read the obituary written by Bob Lowenberg to remember Dick van Bekkum's life and many achievements.

Return to Top

We Want Your Feedback to Improve the Newsletter

Our goal is to make the CIBMTR newsletter as engaging and useful to our audience as possible. Do you have any recommendations for improvement? For instance, you might suggest additional topics you would like us to include, different formatting techniques, or alternate distribution tactics. Please fill out this short survey to share your ideas: http://www.surveygizmo.com/s3/2209717/2015-CIBMTR-Newsletter-Survey.
We appreciate your feedback!

Return to Top

2016 BMT Tandem Meetings

By Tia Houseman and D'Etta Waldoch Snyder, CMP

The BMT Tandem Meetings - the combined annual meetings of the CIBMTR and ASBMT - are North America's largest international gathering of HCT 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 HCT during the BMT Tandem Meetings at the Hawaii Convention Center in Honolulu, Hawaii, February 18-22, 2016. 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 2016 meetings offer peripheral sessions for BMT Pharmacists, BMT Center Administrators, Coordinators, Investigators, Medical Directors, Clinical Research Professionals /

Meeting Topics and Special Sessions

- CAR-T Cells
- Modifying Genome
- ICU in HCT
- Precision Medicine
- Novel Immunotherapeutic Strategies
- Choosing a Donor in 2016
- Acute GVHD Biology
- Psychological Factors
- Haplo: Asia vs Europe vs USA
- Autologous BMT / Novel Strategies
- Mucosal Immunology and Transplant
- Stats Session
- Iron
- Chronic GVHD beyond B-Cells
- BM Failure Syndrome
- Donor Economics

Plus:

- Mortimer M. Bortin Lecture
- E. Donnell Thomas Lecture
- Oral Abstracts and Poster Sessions
- CIBMTR Working Committee Meetings
- Meet-the-Professor Luncheon Sessions

Data Managers, Transplant Nurses, and Advanced Practitioners.

The online registration, abstract, and housing site will open in a couple of days, and agendas will firm up over the next few months. The early registration and abstract deadline is October 1. After registering, take advantage of special conference guest room rates offered at several hotels near the Hawaii Convention Center. Don't forget to reserve your ticket to the Sunday evening Tandem Reception to end a memorable week with colleagues and old friends!

Questions regarding support opportunities at the 2016 BMT Tandem Meetings may be directed to Sherry Fisher at slfisher@mcw.edu. For general information, please email the conference office at bmttandem@mcw.edu.

We look forward to seeing you in Honolulu!

Return to Top

Patient Resources Available on the HRSA Blood Cell Transplant Website

By Carol Doleysh

The SCTOD is part of the HRSA-funded C. W. Bill Young Cell Transplantation Program (Program), which collects data on all allogeneic transplants performed in the US and on transplants performed elsewhere using cellular products that originated in the US.

Managed by the Program, the HRSA Blood Cell Transplant website features basic transplant, cord blood, and donor information; a description of the Program and its contractors; and a search feature for patient survival and center volumes data. An increasing amount of information useful to patients is being made available on this website, including the Transplant Activity Report for HCTs performed from 2008 to 2012 and center volumes data for HCTs performed from 2009 to 2013.

The Transplant Activity Report was posted for the first time in 2015 and provides static reports of the number of transplants performed at US transplant centers. These data include all types of transplants, including autologous, related allogeneic, and unrelated allogeneic as reported by transplant centers. The tables provide annual transplant numbers by age of patient; cell source (bone marrow, peripheral blood stem cells, and umbilical cord blood); disease; donor type (autologous, unrelated allogeneic, and related allogeneic); gender; race; state of transplant center; and year.

SCTOD Highlights

New or updated data has recently been made available on these websites:

Transplant Activity Report – summary tables about HCTs performed from 2008 to 2012

Center Volumes Data – data has been updated so queries can be run by center or disease on HCTs performed from 2009 to 2013

Return to Top

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 8,000 patients since 2003. The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with NMDP/Be The Match and The Emmes Corporation. Together, these three organizations support all BMT CTN activities.

The BMT CTN Steering Committee is currently under the leadership of Chair Fred Appelbaum, MD (Fred Hutchinson Cancer Research Center), Chair-Elect Steve Devine, MD (Ohio State University Medical University), and Vice-Chair Rick Jones (Johns Hopkins University).

Clinical Trials: Open Enrollment

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

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

- BMT CTN 07LT - Continued, long-term follow-up and **lenalidomide maintenance therapy** for patients who have enrolled on BMT CTN 0702
- 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 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 1301 PROGRESS II - Randomized, Phase III trial of **calcineurin inhibitor-free interventions for prevention of graft-versus-host disease**
- BMT CTN 1302 - Phase II double-blind placebo controlled trial of **maintenance ixazomib after allogeneic HCT for high risk multiple myeloma**
- BMT CTN 1304 / DFCI 10-106 - Phase III study comparing conventional dose treatment using a combination of lenalidomide, bortezomib, and dexamethasone (RVD) to high-dose treatment with peripheral stem cell transplant in the **initial management of myeloma** in patients up to 65 years (Note: this study is managed by Dana Farber Cancer Institute, but BMT CTN has endorsed the study and is providing accrual credit to BMT CTN centers)
- BMT CTN 1505 RECRUIT - Randomized intervention trial to increase **minority patient recruitment**

Recent Presentations

BMT CTN Investigators presented the following abstracts at the APOS / IPOS World Congress on Psycho-Oncology in July:

- Syrjala et al. Cancer and treatment distress measurement over time in a multicenter cohort of hematopoietic cell transplantation (HCT) recipients (BMT CTN 0902)
- Knight et al. Pre-transplant health-related quality of life factors as predictors of outcomes following hematopoietic cell transplantation (BMT CTN 0902)

BMT CTN Publications

There are 51 BMT CTN published articles, including 14 primary analyses. The following manuscript was recently published.

- Khera N et al. **Comparison of characteristics and outcomes of trial participants and nonparticipants: example of BMT CTN 0201 trial.** Biology of Blood and Marrow Transplantation. Epub 2015 Jun 11.

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

- Like us on Facebook: www.facebook.com/bmtctn
- Follow us on Twitter: @BMTCTN

Return to Top

RCI BMT Accomplishments

By Rebecca Drexler

Over the past few months, the RCI BMT opened new studies, closed current studies to accrual, prepared study data, implemented a new Survey Research Group system, and made a final decision on a new electronic data capture and study management system.

Three of our trials recently closed to accrual when they met their protocol accrual goals. RCI BMT also opened three new trials recently of which all three have had their first accruals. We are actively collecting data on 14 different studies and projects managed by RCI BMT staff. Staff members are working with the statistical and protocol team members to prepare data sets on four studies for either upcoming abstracts or manuscripts.

The Survey Research Group call tracking enhancement project went live on March 23. This new call tracking system increases the stability and efficiency of study, time point, subject, and contact attempt management and processes. It allows the

Survey Research Group to view and manage all activities for multiple studies in one place and automates several workflow, analysis, and study management activities that were previously very manual, difficult, and inconsistent across studies.
One of the most valuable features of the system is it auto-assigns contact attempt tasks to staff members daily. When a new subject is added to the system, the first contact attempts for each current and future time point are automatically created. As staff members contact a subject within their time point window, they can select when the next contact attempt should be by day and timeframe (morning, afternoon, evening, or anytime) as well as the task type (call, email, letter, etc).
Another valuable feature of this new system is the reporting functionality. With data about all studies, subjects, and time points in one place, staff members can create and save complex reports. These reports can incorporate multiple data points, and staff members can easily refresh the reports with the latest data or review any specified period of time. Currently staff members are using the system for four studies, and the plan is to add all future studies the Survey Research Group supports.
Final approval was made to proceed with Medidata Solutions, Inc. RAVE® and CTMS™ for the RCI BMT clinical trial data collection and trial management system. We are excited to begin implementing this interconnected system, which will allow RCI BMT to effectively and efficiently provide support for a wide array of clinical trials and research studies.

Return to Top

Health Services Research Program

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

The Health Services Research Program is committed to a science-based approach that values innovation, as well as collaborative and strategic partnership with external organizations and professionals. Our overarching goal is to discover answers to improve health and expand understanding of the conduct, organization, outcomes, and effectiveness of HCT.

Program Leadership

Medical Director:

- Linda Burns, MD

Leadership:

- Elizabeth Murphy, EdD, RN
- Ellen Denzen, MS

Statisticians:

- Lih-Wen Mau, PhD, MPH
- Christa Meyer, MS
- Jaime Preussler, MS

The Health Services Research Program partners with the Health Services and International Issues Working Committee (HSIS WC) and the NMDP/Be The Match Payer Policy Department to maximize clinical, research, and policy expertise in the following focus areas:

- Economic aspects of HCT (e.g., costs and cost-effectiveness)
- Healthcare disparities in access to and outcomes of HCT (e.g., health status and quality of life)
- Survivorship care and healthcare utilization
- Practice patterns and impact on HCT outcomes
- Program evaluation

We recently initiated a number of exciting research and patient-centered projects:

- Reimbursement analysis of HCT in older patients (using linked CMS claims and CIBMTR clinical data)
- HCT multidisciplinary care teams: Burnout, moral distress, and career satisfaction
- Education needs assessment with adults who received HCT at age 65 or older
- Transplant center financial barriers to HCT: health insurance coverage and reimbursement

Our research portfolio also includes on-going research (select studies):

- Costs and utilization of allogeneic HCT compared to chemotherapy alone as initial therapy for older patients with AML (in partnership with HSIS WC)
- Individualized care plans for HCT survivors (PCORI funded; partnership with RCI BMT)
- Payer partnered approach to community-based referral for HCT (NCCN/Pfizer grant)
- Easy-to-read informed consent forms for HCT multicenter trials (NHLBI ancillary grant to BMT CTN; BMT CTN 1205)

Recent Health Services Research Program peer-review publications:

- Besse KL, et al. **Estimating demand and unmet need for allogeneic HCT in the US using geographic information systems.** Journal of Oncology Practice. 11(2):e120-e130. doi:10.1200/JOP.2014.000794. Epub 2015 Mar 1. PMC4371120.
- Clauser SB, et al. **Patient centeredness and engagement in quality-of-care oncology research.** Journal of Oncology Practice. 2015 May 1; 11(3):176-179. doi:10.1200/JOP.2015.003749. Epub 2015 Apr 7.
- Majhail NS, et al. **National survey of HCT center personnel, infrastructure and models of care delivery.** Biology of Blood and Marrow Transplantation. 2015 Jul 1; 21(7):1308-1314. doi:10.1016/j.bbmt.2015.03.020. Epub 2015 Mar 31. PMC4466059.
- Preussler JM, et al. **Variation in Medicaid coverage for HCT.** Journal of Oncology Practice. 2014 Jul 1; 10(4):e196-e200. doi:10.1200/JOP.2013.001155. Epub 2014 Apr 8. PMC4135085.

Finally, we are planning next steps for the Health Economics Interest Group, which held its first meeting at the BMT Tandem Meetings in February. Interest Group members will receive an update in August.
For any questions about the Health Services Research Program or the Health Economics Interest Group, please contact Ellen Denzen at edenzen@nmdp.org.

Return to Top

New Format for Patient Summaries of CIBMTR Research

By Jessica Gillis-Smith, MPH

The 2015 lay summaries of CIBMTR publications have a new format, promoting ease of reading and engagement with patients and their loved ones. Seven summaries have recently been posted on the CIBMTR Patient Resources webpage:

- Allogeneic transplant may be a good treatment option for patients 40 years or older with non-Hodgkin lymphoma
- Guidelines on cancer screening for transplant recipients
- Guidelines for transplant and multiple myeloma
- Fertility preservation options before transplant
- Patients with lymphoma have similar survival after transplant whether the unrelated donor is matched, mismatched, or cord blood
- More patients who have a transplant from an unrelated donor survive than in the past
- More teens and young adults with ALL survive after blood or marrow transplant than in the past

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

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

Return to Top

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- Follow us on Twitter: @CIBMTR

Return to Top

CIBMTR Advisory Committee

The Advisory Committee, made up of members from across the globe, maintains careful oversight of the CIBMTR research agenda. The 2015 committee members are listed on the CIBMTR website, and we sincerely thank each one for their time and efforts.

Return to Top

Our Supporters

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Return to Top

Abbreviations

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

Return to Top

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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