COVID-19 Updates
Quick Links
Patient Resources
Publication List
Newsletters
News Releases
Slides and Reports
Statistical Resources
CIBMTR 50th Anniversary
Get Involved

November 2015 Newsletter

Volume 21, Issue 4

Table of Contents:

Perspectives

By Paul Martin, MD

Bob Dylan's anthem The Times They Are A-Changin' heralded dramatic social and political changes in the early 1960's. In a more evolutionary way, the CIBMTR is at the doorstep of changing times by expanding its scope to include "cellular therapy," defined as the use of progenitor and stem cells for indications other than hematopoietic recovery, reversal of inherited disorders of metabolism, or treatment of primary immunodeficiency.

HCT is carried out with marrow, growth factor-mobilized blood cells, or cord blood cells from related or unrelated donors, with participation of transplant centers, the NMDP/Be The Match, and a network of cord blood banks. Cellular therapy will add considerable complexity by introducing innovative methods for genetic modification and cell expansion, with participation of transplant center investigators, industry sponsors, blood banks, and cell processing laboratories, all under close regulation by the FDA and other authorities.

The CIBMTR has given much thought to its vision and mission with respect to the advent of cellular therapy, based on previous experience in collecting data related to the use of mesenchymal stem cells for regenerative cell therapy of neurological diseases and poly-specific T cells for treatment of viral infections. Discussion of cellular therapy has been in progress for more than a year. Plans for the immediate future will focus on the use of cellular therapy for treatment of malignant diseases and viral infections since these indications are most closely aligned with the historical expertise of the CIBMTR.

The CIBMTR is now moving from its initial exploration of ideas into networking with potential partners and stakeholders, beginning with a meeting held on October 13, 2015, involving center representatives, biotech industry sponsors, the NCI, and the FDA. The next phase will involve modifying existing databases and building new databases in a forward-looking way to accommodate wide variability in cell and donor types, genetic modifications, methods of cell expansion, indications for treatment, cell doses, schedules and routes of administration, and concomitant treatment of patients.

The CIBMTR will face several new challenges in embracing a wider scope of cellular therapy research. For example, how do we collect information in sufficient detail to allow meaningful interpretation of results aggregated from multiple studies without overwhelming participating centers with an unduly high burden of work? To some extent, it should be possible to mitigate the burden by modifying selected data report forms that are already in use by the CIBMTR. How do we explain value added by the CIBMTR to investigators and sponsors who do not have strong ties to HCT? The answer here will likely involve marketing the expertise of the CIBMTR in relevant data collection and long-term follow-up to investigators and sponsors, since the FDA requires 15 years of follow-up for patients treated with genetically modified cells.

The 2004 agreement between the NMDP/Be The Match and the Medical College of Wisconsin that gave rise to the CIBMTR was accompanied by rebranding the original IBMTR. In extending the scope of the CIBMTR to include a broad definition of cellular therapy, it's not too soon to begin thinking about another change in our brand. Will the CIBMTR become the CICTR? Indeed, the times, they are a-changin'.

Return to Top

Chronic Leukemia Working Committee

Edwin Alyea,
MD,
Chair

Ronald Sobecks,
MD,
Chair

The focus of the Chronic Leukemia Working Committee is to define the optimal timing and improve the outcome of patients undergoing transplantation for MDS, CML, CLL, and myeloproliferative disorders. Over the last five years, the members of the committee published 11 manuscripts and presented several oral and poster presentations. Topics included comparing conditioning regimens and the intensity of the conditioning regimen in an attempt to improve outcomes and reduce regimen-related toxicity. One study used a large data set to validate and refine an index to predict a patient's risk of relapse after transplantation. Studies under

development focus on the optimal timing of transplantation in diseases where new non-transplant therapies have emerged. The committee is also responsible for reviewing and revising data collection forms, which will serve as the basis for future studies. Below we highlight a few published studies and proposals currently underway.

Committee Leadership

Co-Chairs:

- Edwin Alyea, MD, Dana Farber Cancer Institute, Boston, MA
- Uday Popat, MD, MD Anderson Cancer Center, Houston, TX
- Ronald Sobecks, MD, Cleveland Clinic, Cleveland, OH

Scientific Director:

- Wael Saber, MD, MS

Statisticians:

- Kwang Woo Ahn, PhD
- Zhen-Huan Hu, MPH

Immediate Past Chairs:

- Matt Kalaycio, MD, Cleveland Clinic, Cleveland, OH
- Richard Maiziarz, MD, Oregon Health Science Center

Dr. Edward Copelan has led a number of studies comparing ablative conditioning regimens and assessing outcomes and toxicity. In a recent study, the writing committee compared cyclophosphamide in combination with intravenous or oral busulfan and total body irradiation in patients with CML undergoing myeloablative transplantation. This study adds to a growing literature that busulfan-based conditioning regimens are more effective in disease control with an acceptable toxicity profile. A current study looks to define the appropriate timing of transplantation for patients with CML who have progressed on imatinib. The study requires comparing outcomes in a non-transplant patient database, which resides at an outside institution to the CIBMTR Research Database. This type of collaboration is strongly encouraged.

Dr. Philippe Armand led an important study refining the Disease Risk Index, which estimates a patient's risk of relapse after transplantation based on pre-transplant characteristics. This information is valuable to clinicians when discussing expectations about transplantation with patients and family. Equally important is the establishment of uniform criteria, which can be applied to patients in other clinical trials so that a more direct comparison of patient populations can be made and outcomes compared.

The committee published a number of studies focusing on myeloproliferative diseases. Myeloproliferative neoplasms are rare diseases, and registry studies have a unique role in reporting outcomes in this patient population after transplantation. One study compared the outcome of patients after ablative and non-ablative regimens. These data help guide clinicians in their choice of conditioning regimens for this difficult-to-treat group of diseases. A future study will compare transplantation with non-transplant strategies in order to define the role and timing of transplantation for these diseases.

The committee welcomes new participants as well as new proposals. We also encourage collaboration with other committees and the use of outside data sets, which can be used to better define the role and timing of transplantation as new non-transplant strategies emerge. The committee encourages individuals to help in revising and updating the data collection forms. With emergence of molecular data, database form revisions will be needed to strengthen the Research Database and provide a valuable resource for years to come. We encourage young investigators to participate in this effort as it is an excellent opportunity to become familiar with the current data and make use of this powerful resource. The next in-person meeting of the Chronic Leukemia Working Committee is at the CIBMTR/ASBMT BMT Tandem Meetings in Hawaii in February 2016. We look forward to seeing you there and welcoming new proposals. A link has been provided that outlines the work of this committee over the last several years and current proposals under development. We hope this information will stimulate your thoughts on new proposals.

Publications:

- CK00-02 - Ballen KK et al. **Outcome of transplantation for myelofibrosis.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2010 Mar 1; 16(3):358-367. doi:10.1016/j.bbmt.2009.10.025. Epub 2009 Oct 30. PMC2908949.

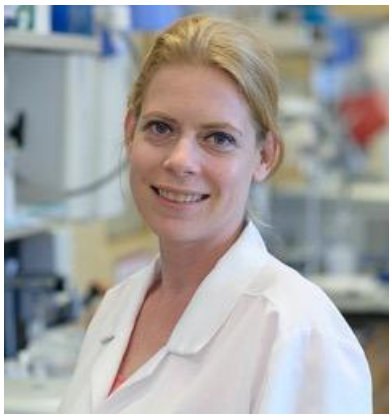
- CK03-02 - Goldman JM et al. **Relapse and late mortality in 5-year survivors of myeloablative allogeneic hematopoietic cell transplantation for chronic myeloid leukemia in first chronic phase.** Journal of Clinical Oncology. 2010 Apr 1; 28(11):1888-1895. doi:10.1200/JCO.2009.26.7757. Epub 2010 Mar 8. PMC2860369.
- CK07-02 - Kalaycio ME et al. **Allogeneic hematopoietic cell transplant for prolymphocytic leukemia.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2010 Apr 1; 16(4):543-547. doi:10.1016/j.bbmt.2009.11.021. Epub 2009 Dec 2. PMC2839005.
- CK03-01b - Khoury HJ et al. **Prognostic factors for outcomes in allogeneic transplantation for CML in the Imatinib era: a CIBMTR analysis.** Bone Marrow Transplantation. 2012 Jun 1; 47(6):810-816. doi:10.1038/bmt.2011.194. Epub 2011 Oct 10. PMC3896981.
- CK06-01 - Ballen KK et al. **Allogeneic hematopoietic cell transplantation for advanced polycythemia vera and essential thrombocythemia.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2012 Sep 1; 18(9):1446-1454. doi:10.1016/j.bbmt.2012.03.009. Epub 2012 Mar 24. PMC3499973.
- CK02-01a - Copelan EA et al. **Better leukemia-free and overall survival in AML in first remission following cyclophosphamide in combination with busulfan compared with TBI. Blood.** 2013 Dec 5; 122(24):3863-3870. doi:10.1182/blood-2013-07-514448. Epub 2013 Sep 24. PMC3854108.
- CK09-01 - Gupta V et al. **Reduced-intensity hematopoietic cell transplantation for patients with primary myelofibrosis: a cohort analysis from the Center for International Blood and Marrow Transplant Research.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2014 Jan 1; 20(1):89-97. doi:10.1016/j.bbmt.2013.10.018. Epub 2013 Oct 23. PMC3886623.
- CK12-04 - Saber W et al. **Impact of donor source on hematopoietic cell transplantation outcomes for patients with myelodysplastic syndromes (MDS).** Blood. 2013 Sep 12; 122(11):1974-1982. doi:10.1182/blood-2013-04-496778. Epub 2013 Jul 11. PMC3772501.
- CK06-03/CK07-01b - Sobecks RM et al. **Outcomes of human leukocyte antigen-matched sibling donor hematopoietic cell transplantation in chronic lymphocytic leukemia: myeloablative versus reduced-intensity conditioning regimens.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2014 Sep 1; 20(9):1390-1398. doi:10.1016/j.bbmt.2014.05.020. Epub 2014 May 28. PMC4174349.
- CK12-03 - Armand P et al. **Validation and refinement of the Disease Risk Index for allogeneic stem cell transplantation.** Blood. 2014 Jun 5; 123(23):3664-3671. doi:10.1182/blood-2014-01-552984. Epub 2014 Apr 17. PMC4047501.
- CK02-01b - Copelan EA et al. **Comparison of outcomes of allogeneic transplantation for chronic myeloid leukemia with cyclophosphamide in combination with intravenous busulfan, oral busulfan, or total body irradiation.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2015 Mar 1; 21(3):552-558. doi:10.1016/j.bbmt.2014.12.010. Epub 2014 Dec 17. PMC4329042.

Return to Top

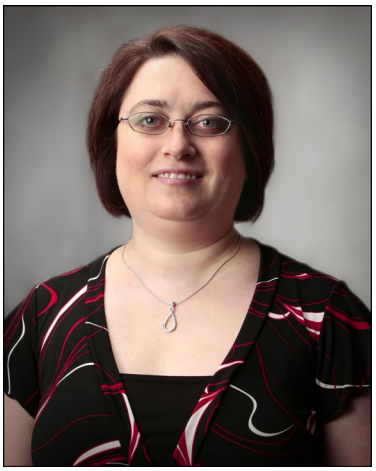
Infection and Immune Reconstitution Working Committee

Jeffery Auletta,
MD,
Chair

Krishna Komanduri,
MD,
Chair



Caroline Lindemans,
MD, PhD,
Chair



Marcie Riches,
MD, MS,
Chair

Based on the CIBMTR Summary Slides from 2014, infection is reported as the primary cause of death in up to 17% 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 the Infection and Immune Reconstitution Working Committee's efforts.

Committee Leadership

Co-Chairs:

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

Scientific Director:

- Marcie Riches, MD, MS

Statisticians:

- Kwang Woo Ahn, PhD
- Soyoung Kim, PhD
- Min Chen, MS

Our 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 factors

such as graft type, donor-recipient mismatch, recipient age, and GVHD incidence. Furthermore, infections prior to the transplant also impact transplant outcomes. Given the complexity of these factors, our analyses rely heavily on novel statistical techniques provided by our excellent MS Statistician, Min Chen, and our PhD Statistician, Kwang Woo Ahn. The committee welcomes Soyoung Kim, a new PhD Statistician who joined our group in September 2015. We look forward to her ideas and contributions.

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 dedicated 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 has formed a task force with the recently formed ASBMT Infectious Disease Special Interest Group to redesign data collection mechanisms. This will include improved information collection regarding antimicrobial prophylaxis and greater detail regarding certain infectious complications. Dr. Riches is currently collaborating with a group of investigators to pilot these proposed collection forms in an oligo-center prebiotic trial that is currently in development.

Several manuscripts from our committee are currently in press, under review, or ready for submission. These include:

- IN05-02: Risk Factors and impact of non-Aspergillus mold infections (NAMI) following allogeneic HCT (in press, Bone Marrow Transplantation)
- IN06-01: The incidence, mortality, and timing of Pneumocystis jiroveci pneumonia after HCT (minor revisions requested)
- IN09-01: Outcomes of allogeneic HCT for patients with hematologic malignancies (AML, ALL, MDS, CML) with and without fungal infections — pre-existing fungal infection is not a contraindication for subsequent HCT (under review)
- IN10-01: Comparison of infection rates among acute leukemia patients receiving alternative donor HCT (draft manuscript circulated)
- IN12-01: Impact of early cytomegalovirus reactivation in cord blood stem cell recipients in the current era (draft manuscript circulated)
- IN12-01: Early cytomegalovirus reactivation increases transplant-related mortality without attenuating hematologic disease relapse risk following allogeneic HCT (draft manuscript circulated)

While all studies to date have focused on infections, the committee would like to expand 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.

Return to Top

CIBMTR Trivia

The CIBMTR's Research Database has information on approximately _____ patients.

1. 290,000
2. 315,000
3. 390,000
4. 415,000

Click here to enter your answer. If you answer correctly, you will be entered into a drawing to win a CIBMTR prize!

Return to Top

CIBMTR Abstracts at ASH

CIBMTR investigators will present the following 25 abstracts at ASH:

Title	Type	Session/Location
Saturday, December 5, 2015		
RT09-04/IB09-06: Combined donor and recipient non-HLA genotypes show evidence of genome- wide association with transplant related mortality (TRM) after HLA-matched unrelated donor blood and marrow transplantation (URD-BMT) (DISCOVeRY- BMT study)	Oral	9:30-11:00 am, presentation 9:30 am, W304

CK11-02: A prognostic system predictive of outcomes in persons undergoing allogeneic hematopoietic cell transplantation for myelodysplastic syndrome	Oral	9:30- 11:00 am, presentation 10:15 am, W304
MM13-02: Autologous hematopoietic cell transplantation in patients with high risk multiple myeloma: post-transplant responses do not translate to longer survival	Poster	5:30-7:30 pm, Hall A
LK13-04: Comparison of post allogeneic hematopoietic cell transplantation (HCT) outcomes after matched related donor versus matched unrelated donor HCT in adults with acute lymphoblastic leukemia	Poster	5:30-7:30 pm, Hall A
LK14-02: Outcomes of allogeneic transplantation in patients aged ≥60 years with acute myeloid leukemia in second complete remission: a CIBMTR cohort analysis	Poster	5:30-7:30 pm, Hall A
CK14-01: Outcomes after umbilical cord blood transplantation for myelodysplastic syndromes: a Center for International Blood and Marrow Transplant Registry (CIBMTR®) study	Poster	5:30-7:30 pm, Hall A

Sunday, December 6, 2015

SC11-06: Outcome of patients 65 years and older with myelodysplastic syndrome (MDS) receiving allogeneic hematopoietic stem cell transplantation compared to patients 55-64 years of age	Oral	7:30-9:00 am, presentation 7:30 am, W304
LY15-01: Survival after T-cell replete haploidentical related donor transplant using post-transplant cyclophosphamide compared with matched unrelated donor (MUD) transplant for lymphoid malignancies	Oral	7:30-9:00 am, presentation 7:45 am, W304
LY14-02: Reduced-intensity allogenic hematopoietic cell transplantation (allo-HCT) provides durable progression-free survival (PFS) in a subset of diffuse large B-cell lymphoma (DLBCL) patients relapsing after autologous (auto-) HCT	Oral	7:30-9:00 am, presentation 8:30 am, W304
LY06-03: Allogeneic stem cell transplantation for relapsed / refractory (R/R) follicular lymphoma (FL). A joint study between the European Society for Blood and Marrow Transplantation (EBMT) and the Center for International Blood and Marrow Transplant Research (CIBMTR)	Oral	7:30-9:00 am, presentation 8:45 am, W304
HS13-01: The impact of pre-transplant depression on outcomes of allogeneic and autologous hematopoietic stem cell transplantation	Oral	12:00-1:30 pm, presentation 12:00 pm, W340
BMT CTN 0201: 5 year results of BMT CTN 0201: unrelated donor bone marrow Is associated with better psychological well-being and less burdensome chronic GVHD symptoms than peripheral blood	Oral	12:00-1:30 pm, presentation 1:15 pm, W340
RT09-04/IB09-06: Genome-wide association study of overall and progression-free survival after HLA-matched unrelated donor blood and marrow transplantation (DISCOVeRY-BMT study)	Oral	4:30-6:00 pm, presentation 4:30 pm, Tangerine 3 (WF3-4)
RCI BMT 09 PLEX: A phase II study evaluating the safety and efficacy of subcutaneous plerixafor for the mobilization and transplantation of HLA-matched sibling donor hematopoietic stem cells in recipients with hematological malignancies	Oral	4:30-6:00 pm, presentation 5:30 pm, W230
MM15-01: Post-transplant therapy is more important than induction regimen choice in autologous hematopoietic cell transplantation (AHCT) recipients for multiple myeloma (MM)	Oral	4:30-6:00 pm, presentation 5:45 pm, W224CDGH
IB12-03: Investigating effect of genetic ddmixture and donor/recipient genetic disparity on transplant outcomes	Poster	6:00-8:00 pm, Hall A
IB11-01: Evaluation of the impact of non-inherited maternal antigens on the outcome of HLA mismatched unrelated donor hematopoietic stem cell transplantation for hematological malignancies on behalf of the ALWP of the EBMT and the CIBMTR	Poster	6:00-8:00 pm, Hall A

GV12-01: Outcomes of grades II-IV acute graft-versus-host disease post-allogeneic hematopoietic stem cell transplantation: how much progress was achieved?	Poster	6:00-8:00 pm, Hall A
RT09-04c: Evidence for heterogeneous genetic associations with acute lymphoblastic leukemia (ALL) by cytogenetics and sex in high risk patients treated with matched unrelated donor allogenic blood or marrow transplant (URD-BMT)	Poster	6:00-8:00 pm, Hall A
Monday, December 7, 2015		
HS13-02: A study of predictors of clinical outcomes and healthcare utilization in children with sickle cell disease undergoing allogeneic hematopoietic cell transplantation	Oral	7:00-8:30 am, presentation 8:15 am, Tangerine 1 (WF1)
BMT CTN 0601: A multicenter phase II trial of unrelated donor reduced intensity bone marrow transplantation for children with severe sickle cell disease (SCURT): results of the Blood and Marrow Transplant Clinical Trials Network (BMT CTN 0601) study	Oral	10:30-12:00 pm, presentation 10:30 am, W230
BMT CTN 0805: Multicenter US intergroup study of intensive chemotherapy plus dasatinib follwed by allogeneic stem cell transplant in patients with philadelphia chromosome positive acute lymphoblastic leukemia younger than 60	Oral	Presentation 5:15 pm, W224CDGH
GV12-02: Upper gastrointestinal acute graft-versus-host disease adds minimal prognostic value when present in isolation or in addition to grade I or other grade II-defining GVHD manifestations	Oral	4:30-6:00 pm, presentation 5:30 pm, W230
LK14-03: Autologous transplant, and not ATO alone, remains the preferred therapy for relapsed APL: a report from the CIBMTR, EBMT, and two specialized centers	Oral	6:15-7:45 pm, presentation 7:00 pm, W314
Tuesday, December 8, 2015		
BMT CTN 0901: Results of a phase III randomized, multi-center study of allogeneic stem cell transplantation after high vs reduced intensity conditioning in patients with myelodysplastic syndrome (MDS) or acute myeloid leukemia (AML)	Oral	7:30-9:30 am, presentation 9:15 am, Hall D

Return to Top

2016 BMT Tandem Meetings Return to Honolulu, Hawaii

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 blood and marrow transplant Clinicians and Investigators, Laboratory Technicians, Advanced Practice Professionals, Transplant Nurses, Pharmacists, Administrators, and Clinical Research Associates since 1999.

Leading experts will convene at the Honolulu Convention Center in Honolulu, Hawaii, February 18-22 (note: Thursday-Monday) to present the latest developments in blood and marrow transplantation during the BMT Tandem Meetings. Scientific Program Chairs for the 2016 meetings are Corey Cutler, MD, for the CIBMTR and Pavan Reddy, MD, for ASBMT.
Visit the 2016 BMT Tandem Meetings homepage to register and view additional details. At the early registration deadline, there were more than 1,300 participants registered. The last day for general registration rates is January 16, 2016, and the last day to book your hotel is January 20, 2016. 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 Tandem Reception on Sunday evening at the Royal Hawaiian Hotel on the Ocean Lawn and in the Monarch Room.

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

BMT CTN Spotlight on Sickle Cell Disease

By Amy Foley, MA

The BMT CTN's mission is to conduct large, multi-institutional clinical trials to improve the outcomes of HCT for patients, including those with rare and non-malignant disorders such as sickle cell disease (SCD).

Patients with severe SCD have progressive perfusion-related vascular complications like stroke, acute chest syndrome, and vaso-occlusion-related pain. Both disease and therapy can cause morbidity and early mortality, especially when complications persist or progress despite supportive care measures.

The BMT CTN has completed its first SCD trial, BMT CTN 0601. This study, using unrelated donors for transplantation of children with SCD, was undertaken because of the limitations of identifying suitably matched siblings as donors. The trial employed a reduced intensity-conditioning regimen and bone marrow grafts from volunteer donors or banked umbilical cord blood. The trial's primary objective was event-free survival at 1-year. (The events are death or graft failure.) The primary results will be presented at the ASH annual meeting in December. (See details in ASH Presentations section below). The cord blood arm of the trial was closed earlier for excess graft failure (Kamani N et al. Biology of Blood and Marrow Transplantation, 2012).

Given the successful accrual of the 0601 study, the BMT CTN has been recognized as a network that effectively brings together hematologists and transplant physicians to treat SCD patients on transplant clinical trials. This collaboration has resulted in the development of two subsequent SCD protocols:

- BMT CTN 1503 HCT for young adults with SCD: This trial will compare adolescents and young adults with severe SCD receiving bone marrow transplantation from a matched sibling or unrelated adult donor to those receiving standard of care. Although young adults with severe SCD have a lower life expectancy than the general population, it remains to be seen whether the mortality rate from SCD exceeds the mortality associated with transplantation.
- BMT CTN 1507 Reduced intensity conditioning before HLA-haploidentical bone marrow transplantation in patients with symptomatic SCD: This Phase II trial will test whether transplantation of bone marrow grafts from haplo-identical relatives in children, adolescents, and young adults will result in sustained donor engraftment and disease cure.

Both trials are slated to be released to centers and posted on the BMT CTN website by early 2016.

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 12 trials open to accrual and 9 in development. The following BMT CTN trials were recently opened for accrual:

- 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

ASH Presentations

BMT CTN Investigators will present these abstracts at the ASH annual meeting in December:

- BMT CTN 0902: Lee et al. **Unrelated donor bone marrow is associated with better psychological well-being and less burdensome chronic GVHD symptoms**; #270, 12/6/2015 1:15 pm ET, W340
- Primary Results! SWOG #S0805 / BMT CTN 0805: Ravandi et al. **Multi-Center US Intergroup study of intensive chemotherapy plus dasatinib followed by allogeneic stem cell transplant in patients with Philadelphia chromosome positive acute lymphoblastic leukemia younger than 60**; 12/7/15 5:15 pm ET, W224
- Primary Results! BMT CTN 0601: Shenoy et al. **A multicenter Phase II trial of unrelated donor reduced intensity bone marrow transplantation for children with severe sickle cell disease (SCURT): Results of the Blood and Marrow Transplant Clinical Trials Network (BMT CTN 0601) study**; #619, 12/7/2015 10:30 am ET, W230

BMT CTN Publications

There are 57 BMT CTN published articles, including 16 primary analyses. The following manuscripts were recently published.

- Primary Results! Anderlini P et al. **Cyclophosphamide conditioning in patients with severe aplastic anaemia given unrelated marrow transplantation: a phase 1-2 dose de-escalation study.** Lancet Haematology. 2015 Sep 1. 2(9):367-375. Epub 8 Sep 15.
- Young et al. **Infections following transplantation of bone marrow or peripheral-blood stem cells from unrelated donors.** Biology of Blood and Marrow Transplantation. Epub 2015 Sep 23.
- Giralt S et al. American Society of Blood and Marrow Transplant, European Society of Blood and Marrow Transplantation, Blood and Marrow Transplant Clinical Trials Network and International Myeloma Working Group **Consensus Conference on Salvage Hematopoietic Cell Transplantation in Patients with Relapsed Multiple Myeloma.** Biology of Blood and Marrow Transplantation. Epub 2015 Sep 28.
- Primary Results! Devine SM et al. **A Phase II study of allogeneic transplantation for older patients with AML in first complete remission using a reduced intensity conditioning regimen: Results from CALGB 100103 (Alliance)/BMT CTN 0502.** Journal of Clinical Oncology. [In press].
- Wood WA et al. **Patient-reported physical functioning predicts the success of hematopoietic cell transplantation (BMT CTN 0902).** Cancer. [In press].

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

Patient-Centered Outcomes Research (PCOR): Let's Get Going!

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

Patients are at the center of all our efforts within the transplant community. However, sometimes we lose our focus and forget to ask patients how they're doing and how they think we're doing! Therefore, for the first time this year, four transplant recipients shared their personal experiences and perspectives on HCT care delivery at the third annual Aligning Quality and Value in HCT meeting sponsored by NMDP/Be The Match Payer Policy on July 15-16 in Minneapolis, MN. More than 100 researchers, administrators, payers, and representatives of professional organizations attended.
The patient-based session provided novel insights into the HCT care delivery process and clearly drove discussions throughout the meeting. At the meeting's conclusion, the most highly audience-rated areas of interest to tackle in the next one to two years were measures of quality of life and care coordination. Within the broad area of quality of life, key topics lending themselves to measuring outcomes included a return to "normal," financial health, psychosocial well-being, and long-term follow-up care issues. Within the area of care coordination, voiced interests were timely HLA typing, referral for transplant consultation, a multidisciplinary team approach to post-transplant complications including GVHD management, and communication at times of care transitions. Furthermore, the primary theme that emerged from the meeting was: "We need to create and retain a patient-centered focus."

This patient-based session clearly demonstrated how impactful patient engagement can be and the urgent need to include patient-centered outcomes research (PCOR) within the Health Services Research Program's portfolio. PCOR addresses the questions and concerns most relevant to patients, with other key

stakeholders including caregivers, healthcare providers, researchers, payers, and policy makers. Involvement by patients and other non-researchers guides research prioritization and innovations in care delivery and impacts healthcare policy. Importantly, PCOR helps patients and their care providers make informed healthcare decisions and, ultimately, improves healthcare delivery.

For more than a decade, Dr. Douglas Rizzo has led CIBMTR efforts to collect patient-reported outcomes (PRO). A quality of life study was performed in the late 1990's, and PRO have been collected in a substantial number of BMT CTN studies. Led by Drs. Bronwen Shaw and Douglas Rizzo, the CIBMTR is currently completing feasibility analyses of collecting PRO directly from patients after enrollment through the transplant center.

The CIBMTR and Health Services Research Program will lead efforts in PCOR and measure development within the HCT community. Stay tuned for more details in the coming months about how we plan to get going and how you can be involved. In the meantime, contact Ellen Denzen, Senior Manager of the Health Services Research Program, at edenze@nmdp.org with any questions or suggestions.

Return to Top

Five New Patient Summaries of CIBMTR Research

By Jessica Gillis-Smith, MPH

Five patient summaries of CIBMTR publications have recently been posted on the CIBMTR Patient Resources webpage:

- Bone marrow donors have more side effects at hospitals that do fewer bone marrow collections
  - Bone marrow donors had more side effects at hospitals that did fewer bone marrow collections.
- Transplant can help some children with hypodiploid acute lymphoblastic leukemia
  - Children with hypodiploid ALL did worse when they had a transplant later (in their 2nd complete remission).
  - Children with 43 or fewer chromosomes in their diseased cells had a higher risk of the leukemia coming back and dying after transplant compared to children with 44 or 45 chromosomes.
  - While more children may live longer because doctors can find better matched donors, disease factors still affect transplant outcomes.
- A new way to measure acute graft-versus-host disease can help doctors predict transplant outcomes
  - The Refined Acute GVHD Risk Score is a way for doctors to predict the likelihood that:
    - A patient will be alive six months after starting treatment for acute GVHD.
    - Acute GVHD will get better with steroids.
- New blood test can predict the severity of acute graft-versus-host disease
  - Biomarkers can predict whether acute GVHD will be mild or severe and whether it is likely to respond to treatment.
- Patients with myeloma or lymphoma can have excellent outcomes after an outpatient autologous transplant
  - Outcomes (results) were similar in the inpatient and outpatient transplant groups.

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

Join the CIBMTR on Facebook and Twitter

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

- Like us on Facebook: www.facebook.com/theCIBMTR

- 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 all of our committee members for their time and efforts.

Return to Top

Our Supporters

The CIBMTR is supported by Public Health Service Grant/Cooperative Agreement 5U24-CA076518 from the National Cancer Institute (NCI), the National Heart, Lung and Blood Institute (NHLBI) and the National Institute of Allergy and Infectious Diseases (NIAID); a Grant/Cooperative Agreement 5U10HL069294 from NHLBI and NCI; a contract HHSH250201200016C with Health Resources and Services Administration (HRSA/DHHS); two Grants N00014-13-1-0039 and N00014-14-1-0028 from the Office of Naval Research; and grants from our corporate and private contributors, which are listed on the CIBMTR website.

Return to Top

Abbreviations

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

Return to Top

Last Updated: 4/18/2016 5:11 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