CIBMTR®
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH
newsletter
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contents

PERSPECTIVES: PROCESS IMPROVEMENT AND THANK YOU

by Thomas Shea, MD

Chair, CIBMTR Advisory Committee; Professor, Department of Medicine - Division of Hematology and Oncology, & Director, Bone Marrow and Stem Cell Transplantation Programs, University of North Carolina

Over the past year, the Advisory Committee of the CIBMTR has created several task forces, one of which has developed a process for assessing the activity and effectiveness of the individual CIBMTR Working Committees. Metrics, such as the time from concept review to publication and the impact factor for published articles, were selected to provide the committees with goals and guidelines they could use to optimize the efficiency of their groups and enhance the quality of the research performed. The CIBMTR leadership is hopeful that these efforts will lead to even more effective use of the unique and invaluable resource that the registry provides.

Another effort to improve the overall productivity and quality of the CIBMTR process has been to assess the activity of each Working Committee and consolidate those in which there was overlap or merge those whose critical mass was small enough that sharing administrative and statistical resources with other committees would likely improve the process by helping achieve a critical mass of activity. To this end, several committees were merged with the hope that this consolidation would lead to a more efficient use of overall CIBMTR resources. As a result, the Advisory Committee voted to keep 11 committees as they've been and merge the remaining 8 committees into 4. The committees have been re-organized as follows:

- The **Immune Deficiencies and Inborn Errors of Metabolism Working Committee** will be combined with the **Non-Malignant Marrow Disorders Working Committee.** (A new name reflecting this expanded scope is yet to be determined.)
- The **Cellular Therapies Working Committee** will be combined with the **Autoimmune Diseases Working Committee.** (A new name is yet to be determined.)
- The **Health Policy and Quality of Life Working Committee** will be combined with the **International Studies Working Committee** to form the **Health Services and International Issues Working Committee.**
- The **Plasma Cell Disorders Working Committee** will be combined with the **Solid Tumors Working Committee** to form the **Plasma Cell Disorders and Adult Solid Tumors Working Committee.** (Pediatric solid tumors will be addressed in the Pediatric Cancer Working Committee.)

Finally, as I wind down my tenure as Chair of the CIBMTR Scientific Advisory Board and turn this role over to Paul Martin, I wanted to thank all of the great people in the CIBMTR and NMDP for their tireless and truly inspirational efforts to maintain the scientific value of these incredible

>> continued on page 2

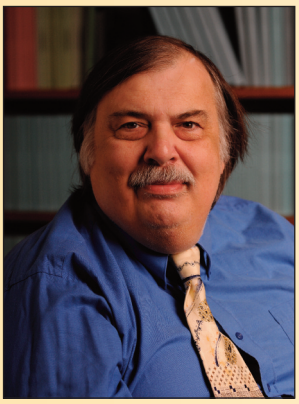
ABBREVIATIONS USED IN THIS NEWSLETTER:

AML	acute myeloid (myelogenous) leukemia
ASBMT	American Society for Blood and Marrow Transplantation
ASH	American Society of Hematology
BMT CTN	Blood and Marrow Transplant Clinical Trials Network
CIBMTR	Center for International Blood and Marrow Transplant Research
CLL	chronic lymphocytic leukemia
DHHS	Department of Health and Human Services
GVHD	graft-versus-host disease
HCT	hematopoietic (stem) cell transplant
HLA	human leukocyte antigen
HRSA	Health Resources and Services Administration
HSC	hematopoietic stem cell
MDS	myelodysplastic syndrome
NCI	National Cancer Institute
NHLBI	National Heart, Lung, and Blood Institute
NMDP	National Marrow Donor Program
NP/PA's	nurse practitioners and physician assistants
PBMTC	Pediatric Blood and Marrow Transplant Consortium
PCORI	Patient Centered Outcomes Research Institute
PI	principal investigator
QOL	quality of life
RCI BMT	Resources for Clinical Investigation in Blood and Marrow Transplantation
RIC	reduced-intensity conditioning
SCP	survivor care plan
SCTOD	Stem Cell Therapeutic Outcomes Database

PERSPECTIVES: PROCESS IMPROVEMENT AND THANK YOU CON'T

>> continued from page 1

organizations. This culture of excellence extends from the leadership provided by Mary Horowitz and Jeff Chell through the protocol, statistical, and IT staff to the investigators and site specific data managers that make up the backbone of our effort. I think it is fair to say that the CIBMTR and NMDP have formed a collaboration that is the envy of every organization in the world that tries to establish a data registry using the lessons of the past to make the future better. On a lighter note, I will miss Paula Watry's ability to keep me on task as well as D'Etta Waldoch's uncanny ability to keep golfers happy in Salt Lake City and skiers content in Tampa. Our patients thank all of you for the quality and quantity of discoveries that you've achieved, and I thank you for letting me be a part of it over the last four years.



In Remembrance of *Dr. John Klein*

1950 - 2013

John Klein, PhD, was Director of the Division of Biostatistics at the Medical College of Wisconsin and Statistical Director of the CIBMTR. He received his BA and MS degrees from the University of Wisconsin - Milwaukee, and his PhD in Statistics from the University of Missouri at Columbia in 1980. Dr. Klein joined the Medical College of Wisconsin and CIBMTR in 1993, coming from the Ohio State University where he was Professor of Statistics and Preventive Medicine and Statistical Director of The Ohio State University Comprehensive Cancer Center.

Under his leadership, the CIBMTR advanced the statistical science of clinical outcomes research in blood and marrow transplantation. His expertise and insistence on statistical rigor in all studies were key to establishing the CIBMTR's reputation for high quality clinical research.

Dr. Klein's more than 180 publications include not only clinical outcomes studies but numerous papers describing his own, National Institutes of Health-funded, methodologic research addressing issues in survival analysis, competing risks, and multistate modeling.

Dr. Klein was an elected member of the International Statistical Institute and a Fellow of the American Statistical Association, two of the highest honors accorded to those in the statistical fields. His book, *Survival Analysis: Techniques for Censored and Truncated Data,* 2nd edition, with Melvin Moeschberger, is a standard biostatistics textbook. He was Associate Editor of *Lifetime Data Analysis and of Biometrics.*

In addition to his work with the CIBMTR, Dr. Klein collaborated with investigators in the Medical College of Wisconsin's Departments of Emergency Medicine and Orthopedics and with investigators at Marquette University's School of Engineering. Dr. Klein was a dedicated and respected graduate and medical school educator and mentor. He advised more than 30 graduate students and in 2005 was named Outstanding Graduate School Mentor by the Medical College of Wisconsin.

Those of us who worked with John respected him for his expertise and for the high standards to which he held us. We were also grateful for his unselfish willingness to work one-on-one with whomever needed his help, from medical student to new investigator to tenured faculty. He will be deeply missed but his impact on us and our work will live on.

LAY SUMMARIES OF CIBMTR RESEARCH

by Jessica Gillis-Smith, MPH

The CIBMTR 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. One of the main initiatives of this committee over the past year was to develop and implement a sustained process of translating published research articles into lay summaries.

Committee members determine which research articles are most applicable to patients and family members, and the lay summary is created collaboratively with the CIBMTR's Medical Writer, the first author of the research article, Consumer Advocacy Committee members, and members of the NMDP Patient Services Writing Team. Through the new process, 7 summaries have been added to the CIBMTR Patient Resources webpage at www.cibmtr.org, bringing the total number of available summaries to 22.

In Remembrance of

Congressman C.W. Bill Young

1930-2013

Congressman C.W. Bill Young served over 50 years in public office, including 22 terms in Congress as a representative of the Thirteenth Congressional District of Florida. At various times during his tenure, Congressman Young served as the Chairman of the House Appropriations Subcommittee on Defense, and from 1999-2005, he served as Chairman of the House Appropriations Committee, overseeing the entire federal discretionary budget.

Throughout his service on the Appropriations Committee, Congressman Young was a leading advocate for increased biomedical research. He led the fight for federal funding for a variety of medical issues, including an increased immunization rate for preschoolers, improved public health programs nationwide, and cures for Parkinson's and Alzheimer's Diseases.

In 1986, Congressman Young was instrumental in creating a national registry for bone marrow donors, now known as the "C.W. Bill Young Marrow Donor Recruitment and Research Program" in his honor. The registry lists more than nine million volunteer donors and has served as a model for similar programs through the world. The Program has made more than 55,000 transplants possible, and it facilitates an average of 15 blood and marrow transplants every day of the year.

Bill often remarked that his proudest achievement was establishing the national registry for blood and marrow donors. We in the blood and marrow transplant field cannot thank him enough for his dedication and service in this endeavor and so many others.

FORMSNET UPGRADES AND DATA OPERATIONS SITE VISITS

by Marie Matlack and Janet Brunner, PA-C

FormsNet Upgrades

Last December marked the completion of the recipient module, the first phase of a multi-year project to upgrade FormsNet, the CIBMTR's electronic data capture system now known as FormsNet[SM]3. The next module to be upgraded in FormsNet3 will be the Donor module, which collects data on NMDP unrelated donors and their donation process. This module has just completed development, and quality assurance testing is underway. Currently, this module is due to be released in FormsNet3 in February 2014. Clinical Trials will be the last module to be upgraded.

The first project for the FormsNet3 recipient module was implemented on October 29, 2013, with the release of 26 revised recipient forms. The forms were reviewed and updated by a team that included physicians, scientific directors, clinical research coordinators, network data managers, statisticians, and metadata and information technology staff. The revised forms incorporate changing treatment practices, and they utilize the enhanced features of FormsNet3. For example, for recipients participating in a BMT CTN clinical trial, the revised Pre-TED (Form 2400) has a drop down box listing all the current BMT CTN studies, allowing a specific study to be selected.

The 26 revised forms consisted of the following:

TED/CRF Forms

- 2804 - CIBMTR Recipient ID Assignment
- 2400 - Pre-TED
- 2000 - Recipient Baseline Data
- 2004 - Infectious Disease Markers
- 2005 - Confirmation of HLA Typing
- 2006 - HCT Infusion

Disease-Specific Forms

- 2010/2110 - Acute Myelogenous Leukemia
- 2011/2111 - Acute Lymphoblastic Leukemia
- 2014/2114 - Myelodysplasia / Myeloproliferative Neoplasms
- 2015/2115 - Juvenile Myelomonocytic Leukemia
- 2016/2116 - Plasma Cell Disorders (forms 2016/2116 PCD and 2017/2117 AMY have been combined)
- 2018/2118 - Hodgkin and Non-Hodgkin Lymphoma
- 2019/2119 - Waldenstrom's Macroglobulinemia
- 2034/2134 - X-Linked Lymphoproliferative Syndrome
- 2039/2139 - Hemophagocytic Lymphohistiocytosis
- 2056/2156 - Pigmentary Dilution Disorders (new forms)

Updated instruction manuals are now available on www.cibmtr.org for all the revised forms except Pigmentary Dilution Disorders.

Network Center Site Visits

In April 2013, the CIBMTR began a new initiative to ensure stakeholder engagement and satisfaction with the services we provide. The Site Team visited 20 centers

>> continued on page 4

FORMSNET UPGRADES AND DATA OPERATIONS SITE VISITS

>> continued from page 3

this spring and summer. The objective was to focus on understanding the user perspective of FormsNet3 and AGNIS. Center participation helped determine the scope of the Summer Performance Release, improve the blank form format on the CIBMTR, and drive a new perspective of customer satisfaction.

Below are the immediate results that were delivered:

1. An enhancement release for FormsNet3 took place in late August 2013, resulting in improved performance across the application and enhanced printing functions.

2. The 15 most commonly used blank recipient forms are reformatted and on our website. While the results differ from the archived "pretty PDFs", the blank forms more closely resemble the format in FormsNet3. We are hopeful that this new format will fit better in the work flows described during the site visits. As of the Forms Revision release in October, the remaining forms are available. Other notable improvements:

- The number of pages was reduced by approximately 50% and included enhanced formatting (e.g., Form 2400 decreased from 62 pages to 25 pages).

- "Else go to" instructions were removed.

- Vertical lists of question options were changed to horizontal in the paper forms.

3. As a part of testing for the August release, three transplant centers participated in user acceptance testing to ensure that the performance release met user needs. They discovered conditions unique to the user environment that were able to be resolved prior to release.

Many thanks go out to our business partners at Stanford, Vanderbilt, and City of Hope for their participation in this testing. We received great insights from our site visits, and we are using their ideas to enhance communication, training, applications, and overall customer service. The pilot processes for site visits and user acceptance testing have had great benefits, and we are considering doing more of both in the future.

RESOURCE FOR CLINICAL INVESTIGATIONS IN BLOOD AND MARROW TRANSPLANTATION

by Becky Drexler

The Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT) is a CIBMTR program that offers infrastructure and support for a wide array of clinical studies including multi-center trials, survey and quality of life assessments. Dr. Willis Navarro, a CIBMTR Senior Scientific Director, oversees this program with the assistance of the Senior Manager, Rebecca Drexler, and her team. Together with guidance from the Senior Leadership of CIBMTR, this program continues to develop new projects and support ongoing studies and projects.

Highlights of Recent Accomplishments

Two manuscripts have been developed, one submitted for publication while the second is in final author review. The first manuscript describes the results of our Revlimid (07-REV) trial titled **Revlimid as maintenance therapy post-transplant: evaluating the safety and tolerability of lenalidomide after allogeneic HCT.** The second manuscript is for our Adult Double Cord (05-DCB) phase II multi-center trial of myeloablative double unit umbilical cord blood transplantation in adults with hematologic malignancy. We are excited to have our first two studies so close to publication.

Our collaboration with the Pediatric Blood and Marrow Transplant Consortium (PBMTC) has continued to be active and productive. The initial study, 09-MRD, continues to enroll with 95 enrolled of the targeted 150 or 63% of goal. In September 2013, the second PBMTC trial was opened, 11-TREO, a multi-center study evaluating a fixed regimen of treosulfan, fludarabine, and low dose total body irradiation in children with AML or MDS undergoing HCT from allogeneic donors. Accrual is at 9 of the targeted 40 or 23% to goal. Protocol development continues on our third project with the PBMTC, 12-MOXE, with an anticipated activation of sites late summer or early fall 2014.

We closed the adult accrual to the RDSafe, our multi-institutional study of hematopoietic stem cell donor safety and quality of life of related donors. Pediatric accrual continues. A total of 1,507 adults were accrued and to date 250 pediatrics. Data revew in ongoing. An abstract accepted at the 2013 ASH meetings was submitted on data from a sub-study of this trial. Dr. Galen Switzer led the team and will present a poster titled **Physical and psychosocial donation experiences of older adult related HSC donors (>60 yrs) compared to those of younger adult donors.**

The RCI BMT continues to support a number of other studies and projects including collaboration on a project with the NMDP Bioinformatics team and the Health Services Research program.

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

by Amy Foley

The Blood and Marrow Transplant Clinical Trials Network (BMT CTN), with its 20 core and approximately 100 affiliate centers, has enrolled over 5,800 patients since 2003. The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with NMDP and The EMMES Corporation. These three organizations together support all BMT CTN activities.

The BMT CTN Steering Committee is currently under the leadership of Chair Ginna Laport (Stanford University) whose two year term will end December 31st. Fred Appelbaum (Fred Hutchinson Cancer Research Center) will serve as Chair starting on January 1st. Steve Devine (Ohio State University Medical University) will continue to serve as Vice Chair.

Clinical Trials: Open Enrollment

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

There are 10 trials open, 1 released to sites, and 6 in development. The following BMT CTN trials are open or will soon be opened for enrollment:

- BMT CTN 0601—Phase II unrelated donor HCT for patients with **sickle cell** using reduced-intensity conditioning
- BMT CTN 0801—Phase II/Phase III trial comparing sirolimus plus prednisone vs. sirolimus/calcineurin inhibitor plus prednisone for **chronic GHVD treatment**
- BMT CTN 0804/CALGB 100701 – Phase II study comparing reduced-intensity allogeneic HCT in **high-risk CLL** patients
- BMT CTN 0901 – Phase III study comparing **myeloablative vs. reduced-intensity conditioning regimens** (MAvRIC) in MDS or AML
- 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 (released to sites)
- 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 1204 - Reduced-Intensity conditioning for children and adults with **hemophagocytic syndromes or selected primary immune deficiencies**
- 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
- BMT CTN 1205 – **Easy-to-read informed consent** for HCT clinical trials

Presentations

American Society of Hematology, December 2013:

- **BMT CTN 0902: Stephanie Lee** - Exercise and Stress Management Training for Patients Undergoing Autologous or Allogeneic Hematopoietic Cell Transplantation. Results from Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0902

International Myeloma Workshop, April 2013:

- **BMT CTN 0704/CALGB 100104: Philip McCarthy** - Analysis of Overall Survival (OS) in the Context of Cross-Over from Placebo to Lenalidomide and the Incidence of Second Primary Malignancies (SPM) in the Phase III Study of Lenalidomide Versus Placebo Maintenance Therapy Following Autologous Stem Cell Transplant (ASCT) for Multiple Myeloma (MM) CALGB (Alliance) ECOG BMT CTN 100104

Publications

There are 33 BMT CTN published articles, including 9 primary analyses. The following manuscripts were accepted/published since the last CIBMTR newsletter:

- Bolanos-Meade J, Wu J, Logan BR, Levine JE, Ho VT, Alousi AM, Weisdorf DJ, Luznik L. Lymphocyte phenotype during therapy for acute graft versus host disease: a brief report from BMT-CTN 0302. Biology of Blood and Marrow Transplantation. 2013 Mar 1; 19(3): 481-485. Epub 2012 Dec 11.
- Vose JM, Carter S, Burns LJ, Ayala E, Press O, Moskowitz CH, Stadtmauer EA, Mineshi S, Ambinder RF, Fenske TS, Horowitz MM, Fisher RI, Tomblyn M. Phase III Randomized study of rituximab/carmustine, etoposide, cytarabine, melphalan (BEAM) compared with 131-Iodine tositumomab/BEAM with autologous stem cell transplantation for relapsed diffuse large B-cell lymphoma: Results from the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0401 trial. Journal of Clinical Oncology. 2013 May 1;31(13):1662-1668. Epub 2013 Mar 11.
- Switzer GE, Bruce JG, Harrington D, Haagenson M, Drexler R, Foley A, Confer D, Bishop M, Anderlini P, Rowley S, Leitman SF, Anasetti C, Wingard JR. Health-related quality of life of bone marrow versus peripheral blood stem cell donors: a pre-specified subgroup analysis from a phase III RCT BMT CTN protocol 0201. Biology of Blood and Marrow Transplantation. Epub 2013 Nov 1.

STEM CELL THERAPEUTIC OUTCOMES DATABASE

by J. Douglas Rizzo, MD, MS, and Carol Doleysh, BS, CPA

The Stem Cell Therapeutic Outcomes Database (SCTOD) is part of the US Health Resources and Services Administration (HRSA)-funded C. W. Bill Young Cell Transplantation Program, which collects data on all allogeneic hematopoietic cell transplants performed in the United States as well as data on transplants performed elsewhere using cellular products that originated in the US. Several activities of the SCTOD, including the center-specific outcomes analysis and a quality-of-life pilot project, are highlighted below.

Center Outcomes

The SCTOD contract requires that the CIBMTR conduct an analysis of one-year survival rates at each transplant center in the US. The report generated by the CIBMTR is meant to be useful as a quality improvement tool for transplant centers. The data are also made available to the public at bethematch.org/access.

An un-blinded version of the 2012 Center-Specific Outcomes Report, which includes first allogeneic HCTs performed between 2008 and 2010, was recently distributed to Center Directors and US Payors. This is consistent with the goal of the CIBMTR to increase transparency of the Center Outcomes Report. This approach will reduce requests from payors directly to transplant center staff to provide data about their center-specific survival report and reduce instances of transcription errors using data taken from the current website.

The 2013 Center-Specific Outcomes Report, which includes first allogeneic HCTs performed between 2009 and 2011, has been completed by the CIBMTR and approved by HRSA. In order to be included in the analysis, transplant centers were required to have at least one year of follow-up on more than 90% of related and unrelated HCT recipients. Nearly all US transplant centers were included in the 2013 report, a total of 168 centers. Preparations are underway to distribute the 2013 report to Center Directors and US Payors and to update the information on the website.

Quality of Life Pilot Project

The quality of life (QOL) project is being conducted to determine the feasibility and acceptability of collecting QOL data directly from patients after HCT, with the goal of minimizing the burden on transplant centers to collect this information. The eight centers that participated in the pilot study included a broad representation of large and small as well as adult and pediatric centers. Accrual began in August 2011 and concluded in September 2013; pediatric accrual was completed in May 2013. Participating patients will be followed for at least six months after transplant. Analysis plans are in progress.

BMT TANDEM MEETINGS

by D'Etta Waldoch, CMP

The 2014 BMT Tandem Meetings will be held February 26th through March 2nd at the Gaylord Texan Hotel and Convention Center in Grapevine, Texas, a few short minutes from the Dallas (DFW) airport. Industry-supported satellite sessions and product theaters will broaden the spectrum of state-of-the-art offerings. In addition to an outstanding scientific program, the 2014 Meetings will again offer peripheral sessions for BMT pharmacists, center administrators, coordinators, investigators, medical directors, clinical research professionals/data managers, transplant nurses, and advanced practitioners.

Keep an eye on www.cibmtr.org or www.asbmt.org as the program firms up over the next few months. Make sure to register and make housing reservations before the on-site rates take effect in January. Also, don't forget to reserve your ticket to the Saturday evening Tandem Reception to end a memorable week on a high note!

We're looking forward to seeing you at the Gaylord Texan!

HEALTH SERVICES RESEARCH PROGRAM RECEIVES $1.3 MILLION GRANT TO CONDUCT SURVIVORSHIP RESEARCH

Navneet Majhail, MD, MS, and Ellen Denzen, MS

The CIBMTR Health Services Research Program, which is conducted in partnership with Be The Match Patient and Health Professional Services, received a $1.3 million contract from the Patient Centered Outcomes Research Institute (PCORI) in August 2013 to conduct research on transplant survivorship. The project is titled **Individualized Care Plans for Hematopoietic Cell Transplant Survivors** and will develop a treatment summary and survivor care plan (SCP) using data collected by the CIBMTR. The principal investigators on the project are Elizabeth Murphy, EdD, RN (Be The Match Patient and Health Professional Services), Navneet Majhail, MD, MS (Cleveland Clinic and NMDP/CIBMTR), and K Scott Baker, MD, MS (Fred Hutchinson Cancer Research Center). Ellen Denzen, MS, Senior Manager Health Services Research Program at Be The Match, is the Project Manager for this study.

It all started around two years ago when Be The Match's Patient Services Advisory Group, including patients, caregivers and clinical providers, decided to take on the issue of care of transplant survivors, care which can be fragmented and inadequate. After some brainstorming sessions, the group came up with the idea of developing a SCP that was specific to transplant survivors. Very quickly, the group realized that we have several resources on which we could capitalize to develop the SCP. First, there were the guidelines for screening for late complications and preventive practices for transplant survivors that were developed by an international panel of experts and published in March 2012. The guidelines lay out the evaluations that patients need at periodic time intervals post-transplant to prevent late complications based on their specific exposures (age, gender, transplant type, steroids, total body irradiation and graft-versus-host disease). Second, the CIBMTR routinely collects data on these exposures through its registration (TED) forms, so we could use CIBMTR data and generate a SCP that was specific to a given patient's treatment exposures. This would be a win-win-win situation: a win for patients who could be empowered to participate in their own long-term care, a win for centers who could generate the care plans without too much extra burden, and a win for the CIBMTR whose data could be used for directly helping patients.

We had a great idea, but we wondered: how do we make it work? This led to the next issue – "Show me the money!" Fortuitously, the PCORI was formed around the same time as part of the Patient Protection and Affordable Care Act of 2010 with the mission of stimulating research that is patient-centered and helps people make informed, evidence-based, individualized healthcare decisions. Our project nicely fit a PCORI Funding Announcement related to Communication and Dissemination Research. In addition to the Patient Services Advisory Group team, which had been developing this project, we brought in additional experts to the drawing board. We submitted the grant in December of 2012 and, after an anxious wait, were informed in May 2013 that we were one of the few projects selected for funding from more than 400 applications.

We were thrilled, but the celebration was short lived as it quickly dawned on us that we would have to really buckle down to accomplish this large, complicated project within three years.

The study itself has two parts. The first phase would involve conducting phone focus groups of (1) patients, (2) caregivers, (3) transplant center clinical providers (physicians and NP/PA's), (4) transplant center nurses and social workers who are involved in transitioning patients back to the community, and (5) referring clinical providers (hematology-oncology and primary care physicians as well as NP/PA's). This phase has already been completed and involved 12 focus groups consisting of 10-12 participants each. Feedback was obtained on what the SCP instrument should look like, what should it contain, and how patient and providers would best use it. Thanks to Stacey Stickney Ferguson and Darlene Haven from Be The Match for expertly facilitating the focus groups. The results from this qualitative phase of the study are presently being analyzed.

The second phase of this project, whose protocol is presently being developed, will involve a randomized trial of our individualized SCP versus routine follow-up care in adult autologous and allogeneic transplant survivors who are between one and five years post-transplant and who have been registered with the CIBMTR. Approximately 500 survivors will be enrolled on this study and will complete several instruments at baseline and at six months to assess their confidence in survivorship information, knowledge of required preventive care, health care utilization and preventive health practices. The RCI BMT will manage this second phase of the project. Sixteen transplant centers from all over the country have committed to participate in the randomized study, and we are sincerely grateful for the support we have received from them thus far. This is the biggest project for the Health Services Research Program to date. However, we know we will be able to complete this project successfully with the support of our colleagues at the CIBMTR, Be The Match, and transplant centers, and we are confident that the SCP will be a step forward for the care of transplant survivors.

Footnote: We would like to acknowledge the efforts of the project team (in addition to the PI's and Ellen Denzen) that wrote and submitted the grant: Barry Schatz (patient, Chicago, IL), Lizzette Salazar (caregiver, Haledon, NJ), Hildy Dillon (Leukemia and Lymphoma Society), Doug Rizzo (CIBMTR), John Wingard (University of Florida), Naynesh Kamani (AABB), Prakash Laud (Medical College of Wisconsin), Karen Syrjala (Fred Hutchinson Cancer Research Center), Balkrishna Jahagirdar (HealthPartners, Minneapolis), Rebecca Drexler (CIBMTR), and Heather Moore (Be The Match). We also want to thank Kevin Weber and Kiila Lee for toiling and persevering through the budget and contractual process. A big THANK YOU to all the transplant centers who have agreed to support this project. For more information, contact Ellen Denzen (edenzen@nmdp.org.)

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Our Supporters

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