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Happy 40th Birthday!

This year marks the 40th anniversary of the International Bone Marrow Transplant Registry (IBMTR), founded by Dr. Mortimer M. Bortin in 1972. In 1972, just four years after the first successful hematopoietic cell transplantation, pioneers in the field realized the importance of their endeavor. They also understood the importance of a collaborative network contributing to a better understanding of the data.

Dr. Mortimer M. Bortin and several colleagues established the IBMTR at the Medical College of Wisconsin to do just that. Physicians and transplant centers voluntarily contributed their patient data to this outcomes registry. At the time, there were only about 12 transplant centers

and fewer than 50 patients per year worldwide receiving a transplant. Today, approximately 500 transplant centers from around the world contribute data to the CIBMTR Research Database, which now contains information on more than 350,000 transplants and has contributed to more than 700 peer-reviewed publications. The goal of this research is to improve clinical practice, survival, and quality of life for patients. The organization that Dr. Bortin founded has become a respected leader in HCT research.



FORMS REVISION PROCESS: BETTER FORMS, BETTER DATA

by Emilie Meissner, Marie Matlack, and Janet Brunner, PA-C

A new Forms Revision Process officially began in January 2012. The initial Forms Revision Process, which began in May 2011, was postponed until now to coincide with the development of the new FormsNet application. There are many benefits to this revision process. It will simplify the update process for data collection forms, provide better data, and make the forms easier for data management staff to complete. We plan to revise the first set of forms, listed below, by June 1st, 2012.

TED/CRF Forms

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

Disease Specific Forms

- 2010/2110 – Acute Myelogenous Leukemia
- 2011/2111 – Acute Lymphoblastic Leukemia
- 2014/2114 – Myelodysplasia/Myeloproliferative Disorders
- 2015/2115 – Juvenile Myelomonocytic Leukemia
- 2016/2116 – Multiple Myeloma / Plasma Cell Leukemia
- 2017/2117 – Amyloidosis
- 2018/2118 – Hodgkin and Non-Hodgkin Lymphoma
- 2019/2119 – Waldenstrom's Macroglobulinemia

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ABBREVIATIONS USED IN THIS NEWSLETTER:

AGNIS	A Growable Network Information System	IND	Investigational New Drug
ALL	acute lymphoblastic leukemia	MCW	Medical College of Wisconsin
AML	acute myeloid leukemia	MDS	myelodysplastic syndrome
ASBMT	American Society for Blood and Marrow Transplantation	MS	multiple sclerosis
ASH	American Society of Hematology	NCI	National Cancer Institute
BMT	blood and marrow transplantation	NHLBI	National Heart, Lung, and Blood Institute
BMT CTN	Blood and Marrow Transplant Clinical Trials Network	NIAID	National Institute of Allergy and Infectious Disease
CIBMTR	Center for International Blood and Marrow Transplant Research	NMDP	National Marrow Donor Program
CED	Coverage with Evidence Development	PBMTCT	Pediatric Blood and Marrow Transplant Consortium
CMS	Centers for Medicare and Medicaid Services	PBSC	peripheral blood stem cell
CRC	Clinical Research Coordinator	RCI BMT	Resource for Clinical Investigations in Blood and Marrow Transplantation
CTRM	Cellular Therapy for Regenerative Medicine		
CTSI	Clinical and Translational Science Institute	RIC	reduced intensity conditioning
DHHS	Department of Health and Human Services	SCTOD	Stem Cell Therapeutic Outcomes Database
EBMT	European Blood and Marrow Transplantation Group	TBI	total body irradiation
GVHD	graft-versus-host disease	TED	Transplant Essential Data (forms)
HCT	hematopoietic (stem) cell transplant	WBMT	Worldwide Network for Blood & Marrow Transplantation

FORMS REVISION PROCESS: BETTER FORMS, BETTER DATA

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The Forms Revision Process began with an “open comment period.” During this period, Scientific Directors, Working Committee Chairs and members, the Network, and CIBMTR staff were asked to submit suggestions and input regarding possible changes to the forms.

The forms were then divided among four Forms Revision Review Committee teams led by Marie Matlack, Kay Gardner, Sharon Meiers, Angela Hauck, Janet Brunner, and Shirley Wayne. Each of the Initial Review Committees consists of a management representative, CRC, Metadata representative, FormsNet subject matter expert, and an Auditor. Each team works closely with statisticians, working committee representatives, physicians, data managers, and other subject matter experts.

The committees were given a summary of the suggested changes collected during the “open comment period”, a list of known problem areas encountered by statisticians analyzing the data, and a list of problems identified during audits and validation. They are reviewing this information, along with the forms themselves, during the revision process.

The team leads of the Forms Revision Review Committees have also begun to work on harmonizing the forms. By improving the consistency of the questions and layout, the forms will be easier for data management staff to complete.

When the Initial Review Committees complete their review, revised forms will be drafted and sent to the Secondary Review Committee. The Secondary Review Committee will consist of CIT representatives from the Minneapolis and Milwaukee campuses, a Business System Analyst, CRC III, AGNIS representative, Working Committee Scientific Director(s), Data Managers, and representatives from other departments and organizations such as the Blood and Marrow Clinical Trials Network, the European Blood and Marrow Transplantation Group, and the United States Immunodeficiency Network when applicable. Once the secondary review is complete, additional changes will be added to the form before moving on to the final Tertiary Review Committee.

The Tertiary Review Committee is one of the final steps in the Forms Revision Process. This committee consists of CIT representatives from Minneapolis and Milwaukee, Associate Scientific Director(s), Working Committee Scientific Director(s), a Metadata Analysis Manager, a CRC III, a Senior Business System Analyst, Senior Management, and the Data Operations Program Director. This committee will review the final draft of the form and the list of changes, make any final changes, and provide the final approval.

When the revised form passes all stages of the review process and is approved, it will be prepared for release into the new FormsNet application.

Updates and Highlights

The Forms Revision Process is making progress:

- We received many suggestions for form changes (the Pre-TED form 2400 has 68!). Each one is being reviewed and considered by the Forms Revision Review Committees.
- Pre-TED Form 2400 and Baseline Form 2000 will be changed to match the updated WHO disease classifications.
- Forms 2032/2132 – Chediak-Higashi Syndrome are being retired. They will be replaced with the new Forms 2056/2156 – Pigmentary Dilution Disorder. Diane Knutson worked closely with USIDNET and other subject matter experts to develop this form. The Initial Review Committee completed its revisions and is drafting the updated version.
- Many physicians volunteered to assist on revisions to the Lymphoma and Myeloma forms, and they are working hard to improve them.
- The committee teams are collaborating on ways to harmonize the forms. The goal is to make them more uniform in text and layout and easier to complete, as well as to provide a general template for future forms.

Future Plans

Our goal is to revise all forms by the end of 2013. This will be done in several iterations over the next two years. Subsequently, the forms will be on a 3-year rotation for review and revision.

The following forms will begin the Forms Revision Process starting in July 2012:

CRF/TED Forms

- Form 2450 – Post-TED
- Form 2455 – Selective Post TED
- Form 2100 – 100 Days Post-HSCT Data
- Form 2200 – Six Months to Two Years Post-HSCT Data
- Form 2300 – Yearly Follow-Up for Greater than Two Years Post-HSCT Data
- Form 2451 – Chimerism Studies
- Form 2007 – Cord Blood Unit – SCTOD Requirements
- Form 4000 – Cellular Therapies for Regenerative Medicine

Disease-Specific Forms

- Form 2012/2112 – Chronic Myelogenous Leukemia
- Form 2013/2113 – Chronic Lymphocytic Leukemia

As with any new process, we are making necessary adjustments as we progress. We are gathering suggestions from Forms Revision Review Committee members and other staff working on the project for ways to improve future iterations.

We are confident that the Forms Revision Process will provide the structure we need to continually update our forms and improve the quality of our data.

2012 BMT TANDEM MEETINGS RETURN TO SAN DIEGO

by D'Etta Waldoch

The combined annual meetings of CIBMTR and ASBMT have been North America's largest international gathering of blood and marrow transplant clinicians and investigators, laboratory technicians, transplant nurses, pharmacists, and clinical research associates since 1999.

2012 – Sun !!!

Leading experts convened at the Manchester Grand Hyatt, San Diego during near perfect weather in February to present the latest developments in blood and marrow transplantation during the BMT Tandem Meetings. Scientific Program Chairs for the 2012 meetings were Stella Davies, MBBS, PhD for CIBMTR and John E. Levine, MD, MS for ASBMT. A record set at the 2008 meetings in San Diego of 2501, was surpassed this year with 2,544 attendees. CIBMTR received 553 abstracts submitted by investigators from 31 countries.



Dr. Mary Horowitz
with Distinguished
Service Award recipient,
Dr. Ardeshir Ghavamzadeh

Dr. Mary Horowitz with
Mortimer M. Bortin Lecturer,
Dr. Andrea Bacigalupo



Workshops, Clinical Research Professional Data Management Conference, BMT Center Administrative Director Conference, BMT Pharmacist Conference, Advanced Practice Professional Conference, Transplant Nursing Conference, Pediatric BMT Program and the BMT Center Medical Director Conference.

Detailed information about the 2013 meeting is continuously updated on the CIBMTR (www.cibmtr.org) and ASBMT (www.asbmt.org) websites. Online conference registration, hotel reservations and the abstract submission program (abstract deadline: October 11, 2012) will launch on August 1. Check in periodically for updates to the provisional agenda.

For general information, please e-mail D'Etta Waldoch, CMP, at the conference office at bmtrandem@cs.com. Questions about support opportunities during the 2013 BMT Tandem Meetings may be directed to Sherry Fisher at slfisher@mcw.edu.

2013 Meeting Topics

- Driving CARs after HCT
- Controversies in Myeloma
- Graft Engineering
- AML/MDS
- New Approaches to Alternative Donors for HCT
- Immunotherapy/Immune Reconstitution
- Controversies in BMT for Lymphoma
- Biologic Basis for New GVHD Therapies
- BMT for Non Malignant Disorders
- Paradigm Shifts in the Treatment of GVHD
- Leukemia Stem Cells
- Novel Statistical Approaches for BMT Studies
- Cancer Vaccines and T-cell Therapy in HCT
- Reducing Transplant Related Mortality
- Sessions presented by NMDP and WBMT

2013 – Ski !!!

The 2013 BMT Tandem Meetings will convene for the first time ever in Salt Lake City, Utah at the Salt Palace Convention Center. Many attendees who remember the early days of the BMT Tandem Meetings in Keystone, Colorado, are delighted to return to a "ski" destination, with slopes a quick 30 minutes away from downtown hotels. Topics slated for presentation at the Salt Lake City meetings are listed in the box below.

Peripheral meetings will include the BMT CTN Steering Committee, BMT CTN Coordinator and Investigator Sessions, Foundation for Accreditation of Cellular Therapy Training

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

by Amy Foley, MS

The Blood and Marrow Transplant Clinical Trials Network (BMT CTN), with its 20 core and approximately 100 affiliate centers, has enrolled nearly 4,500 patients since 2003. CIBMTR shares administration of the BMT CTN Data and Coordinating Center (DCC) with NMDP, and The EMMES Corporation. These three organizations together support all BMT CTN activities.

BMT CTN welcomes Ginna Laport from Stanford University, who began a two-year term as BMT CTN Steering Committee Chair on January 1, 2012. We also wish to thank Sergio Giralto from Memorial Sloan-Kettering Cancer Center, who is now the Immediate Past 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: <http://www.bmtctn.net>.

There are 11 trials open, one released to sites, and three in development. The following BMT CTN trials are open or will soon be opened for enrollment:

- BMT CTN 0301—Phase I/II dose optimization study of Fludarabine-based conditioning for matched unrelated donor (MUD) transplant in patients with **aplastic anemia**
- BMT CTN 0601—Phase II unrelated donor HCT for patients with **sickle cell** using reduced-intensity conditioning
- BMT CTN 0701—Phase II allogeneic transplant using reduced-intensity conditioning in patients with relapsed follicular **Non-Hodgkin's Lymphoma** using related or unrelated donors
- BMT CTN 0702—Phase III single autologous transplant with or without consolidation versus tandem autologous transplant with lenalidomide maintenance; also known as Stem Cell Transplantation for **Multiple Myeloma** Incorporating Novel Agents (STaMINA)
- 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 0805/SWOG 0805 –Phase II trial of chemotherapy plus dasatinib regimen for newly-diagnosed **Ph + ALL patients**
- BMT CTN 0901 – Phase III study comparing **myeloablative vs. reduced-intensity conditioning** regimens (MAvRIC) in MS or AML
- BMT CTN 0902 – Phase III trial testing whether peri-transplant **exercise or stress management** improves functional status and symptoms of autologous and allogeneic HCT recipients
- BMT CTN 0803 / 0903 – Phase II studies for autologous / 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)

Presentations

EBMT— *Don't miss:*

Ned Waller's abstract presentation: Grade III - IV acute GvHD and treatment-related mortality are reduced among recipients of larger numbers of donor naïve CD8+ T-cells and plasmacytoid dendritic cell precursors in allogeneic BM grafts from unrelated donors: results from BMT CTN 0201 (PBSC vs. Marrow) Paolo Anderlini's poster presentation: Fludarabine-based conditioning for allogeneic marrow transplantation from unrelated donors in severe aplastic anaemia: serious and unexpected adverse events in pre-defined cyclophosphamide dose levels (BMT CTN 0301)

Tandem 2012 — More than 350 people attended the BMT CTN Investigator's Meeting this year—an amazing turnout! Certainly Claudio Anasetti and Julie Vose were a draw with their 0201 (PB vs. BM)

and 0401 (Bexxar BEAM) results presentations. But Paul Carpenter and Paul O'Donnell were hopefully able to encourage sites to participate on the 0801 (cGVHD) and 1101 (Haplo vs. Double Cord) studies as well.

BMT CTN Publications

The following manuscripts were published thus far in 2012:

- Kamani NR, Walters MC, Carter S, Aquino V, Brochstein JA, Chaudhury S, Eapen M, Freed BM, Grimley M, Levine JE, Logan B, Moore T, Panepinto J, Parikh S, Pulsipher MA, Sande J, Schultz KR, Spellman S, Shenoy S, Unrelated donor cord blood transplantation for Children with severe Sickle Cell Disease: Results of one cohort from the Phase II study from the Blood and Marrow Transplant Clinical Trials Network (BMT CTN). *Biology of Blood and Marrow Transplantation*. 2012 Feb 16. [Epub ahead of print] PMID: PMC Journal - In Process
- Levine J, Logan BR, Wu J, Alousi AM, Bolaños-Meade J, Ferrara JLM, Ho VT, Weisdorf DJ, Paczesny S. Acute Graft-Versus-Host Disease Biomarkers Measured During Therapy Can Predict Treatment Outcomes: A Blood and Marrow Transplant Clinical Trials Network Study. *Blood*. 2012 Mar 1. [Epub ahead of print] PMID: PMC Journal - In Process
- Pasquini M, Devine S, Mendizabal A, Baden L, Wingard J, Lazarus H, Appelbaum F, Keever-Taylor C, Horowitz M, Carter S, O'Reilly R, Soiffer R. Comparative Outcomes of Donor Graft CD34+ Selection and Immune Suppressive Therapy as Graft-vs-Host Disease Prophylaxis for Patients with Acute Myeloid Leukemia in Complete Remission undergoing HLA-Matched Sibling Allogeneic Hematopoietic Cell Transplantation. *Journal of Clinical Oncology*. In Press, 2012

RESOURCE FOR CLINICAL INVESTIGATIONS IN BLOOD AND MARROW TRANSPLANTATION (RCI BMT) UPDATE

by Becky Drexler

RCI BMT is a CIBMTR initiative that offers infrastructure and support services, including survey research, for prospective, multi-center trials on a smaller scale than the larger, multicenter trials of the BMT CTN. The RCI BMT continues to develop new and ongoing projects and has accomplished several program goals.

Trials that Recently Completed Accrual

The RCI BMT completed accrual on two of its first trials.

- In October 2011 accrual completed for the 05-DCB trial. This study investigates double cord blood HCT after myeloablative conditioning for AML, ALL, and MDS. Over the course of this trial, 12 sites participated and a total of 56 patients were accrued. Follow-up and site monitoring continues.
- In January 2012 the accrual goal of 30 was met for the 07-REV trial. This study evaluates the safety and tolerability of lenalidomide after allogeneic HCT for myeloma. Sites continue to perform follow-up on patients and site monitoring is occurring.

New and Ongoing Projects

Collaboration with the Pediatric Blood and Marrow Transplant Consortium (PBMTTC). RCI BMT partnered with PBMTTC on two recent trials. The first protocol, 09-MRD, is a multi-center study to determine the role of minimal residual disease testing before and after HCT for pediatric acute myeloid leukemia. It opened to accrual in October 2011. The second protocol, 11-TREO, is a multi-center study evaluating a fixed regimen of treosulfan, fludarabine, and low-dose total body irradiation (TBI) in children with AML or MDS undergoing HCT from allogeneic donors. It is anticipated to be open for accrual at the end of 2012.

Providing access to unlicensed cord blood units. During this year, RCI BMT staff worked closely with the NMDP to develop and implement the 10-CBA protocol, a multi-center access and distribution protocol for unlicensed cryopreserved cord blood units for transplantation in pediatric and adult patients with hematologic malignancies and other indications. This protocol provides distribution and access to unlicensed cord blood units. There is no accrual maximum, and the accrual period is open-ended. RCI BMT has a number of other new and ongoing projects:

- A pilot study to assess the feasibility of collecting quality-of-life data in collaboration with the Stem Cell Therapeutic Outcomes Database
- A multi-institutional study of hematopoietic stem cell donor safety and quality of life (RDSafe)
- Two projects investigating KIR receptors and their effect on recipient outcomes
- A long-term follow-up study evaluating the incidence of hematologic and non-hematologic malignancies, thrombotic events, and autoimmune disorders in unrelated normal donors undergoing bone marrow harvest versus peripheral blood stem cell mobilization with recombinant human granulocyte colony-stimulating factor
- A study assessing effect of donor statin use on GVHD after unrelated donor HCT
- A study assessing allogeneic HCT in Medicare beneficiaries with MDS and related disorders

Also, the 09-PLEX study that was delayed is now back on track. This is a phase II study evaluating the safety and efficacy of intravenous Plerixafor for the mobilization and transplantation of HLA-matched sibling donor hematopoietic stem cells in recipients with hematological malignancies. This study was delayed during the past year due to organizational changes at the pharmaceutical company that is funding this project. The trial is now back on track and is expected to open at sites in the fall of this year.

HEALTH SERVICES RESEARCH PROGRAM

Jaime Preussler, MS, Ellen Denzen, MS, Tammy Payton, Heather Moore, MPH, CHES, and Navneet Majhail, MD, MS

The CIBMTR, in collaboration with the NMDP's Patient Services department, established the Health Services Research (HSR) program in 2009 to complement activities of the Health Policy and Psychosocial Issues Working Committee. The HSR program was designed to conduct health policy and services research that requires more resources than typical CIBMTR committee studies. The program focuses on three areas of research related to HCT: (1) access and health care disparities, (2) quality of care, and (3) economic aspects of transplantation.

HSR studies

Some examples of recent HSR program studies include:

- **Financial impact study.** This pilot study examines the feasibility of collecting patient-reported, out-of-pocket cost information over the first three months after transplant and the long-term financial impact of allogeneic HCT. Thirty patients were enrolled at three sites (University of Minnesota, Medical College of Wisconsin, and

Roswell Park Cancer Institute). Analysis of the first phase of the study showed that patients and caregivers can incur substantial out-of-pocket costs, especially if they need to relocate temporarily to be closer to the transplant center. The long-term phase of the study is ongoing and will collect information on financial impact through two years post-transplantation. A manuscript based on the first phase results is under review.

- **Costs of hematopoietic cell transplantation.** In collaboration with the Chronic Disease Research Group in Minneapolis and funded through a pilot grant from the University of Minnesota Masonic Cancer Center, the HSR Program conducted a feasibility study of using a commercial payor database (MarketScan®) to study the costs of HCT. Analyses evaluated inpatient and outpatient costs over the first 100 days after transplantation for 3,365 patients who received HCT from 2007 to 2009. A manuscript is in development, and results from this pilot study will be used to pursue more in-depth research on costs of HCT.

HEALTH SERVICES RESEARCH PROGRAM

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- **Transplant provider and center factors and outcomes of allogeneic hematopoietic cell transplantation.** HCT center and provider characteristics (“center effects”) can impact the organization and delivery of care, and can potentially impact overall patient outcomes. A national survey of U.S. transplant centers is in progress to obtain information about transplant center personnel, infrastructure, and models of care delivery. This study will use CIBMTR observational data and primary data to improve understanding of HCT center effects and help identify center-specific factors that could be modified to improve outcomes.

HSR Publications

Representative publications that have resulted from the HSR program over the past year include:

- NS Majhail, S Nayyar, ME Burton Santibanez, EA Murphy, EM Denzen. Racial disparities in hematopoietic cell transplantation in the United States. *Bone Marrow Transplantation*. [Bone Marrow Transplantation](#). 2011 Nov 7. doi: 10.1038/bmt.2011.214. [Epub ahead of print] *PMC Journal – In Process*

- NS Majhail, EA Murphy, EM Denzen, SS Ferguson, C Anasetti, A Bracey, L Burns, R Champlin, N Hubbard, R Maziarz, E Medoff, J Neumann, K Schmit-Pokorny, DJ Weisdorf, DS Yolin

Raley, J Chell, EL Snyder. The National Marrow Donor Program’s Symposium on hematopoietic cell transplantation in 2020: A health care resource and infrastructure assessment. *Biology of Blood and Marrow Transplantation*. 2012 Feb;18(2):172-82. Epub 2011 Dec 14. *PMC Journal – In Process*

- NS Majhail, EA Murphy, NA Omondi, P Robinett, JL Gajewski, CF LeMaistre, D Confer, JD Rizzo. Allogeneic transplant physician and center capacity in the United States. *Biology of Blood and Marrow Transplantation*. 2011 Jul;17(7):956-61. Epub 2011 Apr 12. PMID: PMC3114271 [Available on 2012/7/1]

- NA Omondi, E Denzen, D Jacobson, T Payton, K Pederson, EA Murphy. Evaluating patient satisfaction with the Office of Patient Advocacy. *Journal of Cancer Education*. 2011; 26, 44-50. *PMC Journal – In process*

The HSR program is presently in its foundational phase. As it gets established, the program will continue to expand its portfolio of patient-oriented research—and research using the CIBMTR and other secondary databases—to address disparities, quality, and costs of transplantation. It also looks forward to broader participation of the transplant community and health services researchers. For more information about the program or to discuss potential opportunities for collaboration, please contact Navneet Majhail, MD, MS, at nmajhail@nmdp.org.

STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD) UPDATE

by J Douglas Rizzo, MD, MS and Carol Doleys, BS, CPA

The SCTOD is part of the HRSA-funded C. W. Bill Young Cell Transplantation Program. Its purpose is to collect data on all allogeneic transplants performed in the United States and on transplants done elsewhere using cellular products that originated in the U.S. Several activities of the SCTOD, including the center-specific outcome analyses and a quality-of-life pilot project, are highlighted below.

Center Outcomes

The SCTOD contract requires that CIBMTR conduct an analysis of one-year survival rates at each transplant center in the United States. 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.

The 2011 Center-Specific Outcomes Report was completed by CIBMTR and approved by HRSA in December 2011. In January 2012 CIBMTR provided Center Directors with a redacted version of the report along with national normative data, in order to provide a context for individual center results. The public version of the report is available at http://marrow.org/Patient/Transplant_Planning/Choosing_a_Transplant_Center/U_S_Transplant_Centers.aspx.

In order to be included in the 2011 report, transplant centers were required to have at least one year of follow-up on more than 80% of their HCT recipient survivors. Nearly all US transplant centers were included in the 2011 report, a total of 156 centers. Because of the inclusion of related and unrelated HCT recipients, the 2011 report uses a three-year time span, rather than the five-year span used in previous reports. This is a significant advantage, as the report contains HCT results that are more representative of “current” practices and outcomes at the centers. The 2011 report contains outcomes of related HCT performed between 2008 and 2009, as well as unrelated HCT between 2007 and 2009 in U.S. centers.

Preparation for the 2012 Center Outcomes Report has been underway since January 2012. Participating centers are expected to have complete follow-up on 90% of related and unrelated HCT recipients to be included in the analysis, which will include transplants performed between 2008 and 2010. Improvements for 2012 include a new process to make pre-HCT data elements that are used in the analysis available to centers for review and correction. This new process includes more communication between CIBMTR and transplant centers, and it will provide time to collect comments from center directors prior to publishing the outcomes on the public website.

In order to address the complex issues and maintain a transparent scientific approach to center outcomes reporting, a third Center-Specific Outcomes Analysis Forum will be held in September 2012 in Milwaukee. Invited participants (including transplant physicians, payors, statisticians, patients, and center outcomes reporting experts) will review the current process and generate recommendations for the best approach to produce and publish the center-specific analysis.

Quality of Life (QOL) Pilot Project

The 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. More information about this study can be found in the August 2011 CIBMTR Newsletter.

(SCTOD) UPDATE>> *continued from page 6*

All eight interested centers have been activated to participate in the pilot study. The pilot group has a broad representation, including large and small, adult and pediatric centers. Accrual, which began in August 2011, has reached 95 patients as of the end of February 2012. CIBMTR is currently contacting patients who reach the 100-day post-transplant survey time point, a key objective of the study. Participating patients will be followed for at least six months after transplant. CIBMTR holds monthly calls with center coordinators to review study status and address questions regarding accrual and follow-up.

MDS Study for Centers for Medicare and Medicaid Services (CMS) CED

Coverage with Evidence Development (CED) is currently necessary for Medicare coverage of HCT for myelodysplastic syndrome (MDS). Through CED, the Centers for Medicare and Medicaid Services (CMS) provides conditional payment for items and services while generating clinical data to demonstrate their impact on health outcomes. CIBMTR is conducting a study to satisfy CED requirements. The goals of this study are to prospectively examine outcomes of allogeneic HCT in adults 65 years of age or older with MDS and to determine whether outcomes for these patients are similar to those in younger patients. In part I of this observational study, up to 240 patients over 65 years of age may be enrolled. This part of the study was activated in December 2010, and 180 participants over the age of 65 have been enrolled through the end of February 2012. CIBMTR and NMDP provide assistance to centers with questions regarding enrollment and reimbursement.

There is considerable interest in implementing a study to compare HCT to non-HCT therapy for MDS in patients aged 65 and older. However, despite the high level of enthusiasm for such a study, it has proven very difficult to design a methodologically sound study which will equitably compare transplant and non-transplant therapy. CIBMTR is working closely with transplant and non-transplant therapy disease experts, members of the Chronic Leukemia Working Committee, and the BMT CTN Steering Committee to design an appropriate study.

This study is explained in depth in the October 2010 CIBMTR Newsletter. Additional information can be found at <http://www.cibmtr.org/studies/clinicaltrials/hct-mds/pages/index.aspx>.

FormsNet™3

FormsNet™3, the latest version of the CIBMTR web-based data collection tool, is currently in development. A demonstration was held at the BMT Tandem Meetings, and there is considerable enthusiasm. This version will contain a more user-friendly design with improved navigation and customization capability. Some of the highest priority improvements requested by our stakeholders (especially transplant center data professionals) include auto-population of key fields such as HCT date, enabling or disabling of fields based on answers to prior questions, faster navigation, real-time validation, automatic saving after each field, and a new user interface. Look for this substantially improved version in November 2012.

AGNIS (A Growable Network Information System)

AGNIS is the system CIBMTR uses to exchange data electronically with transplant centers and other registries. As of the end of January

2012, one transplant center was directly submitting forms to CIBMTR through AGNIS, one center was submitting test data, and one center was ready to begin testing. Several other centers have expressed interest in connecting directly to CIBMTR through AGNIS. Additionally, several vendors have expressed interest in using AGNIS to develop third-party applications for transplant centers. One transplant center is currently submitting data through AGNIS, and twenty sites are receiving data from AGNIS, using these vendor software packages. At least two additional vendors are working with client centers.

As the use of cord blood as a source of hematopoietic cell transplantation expands, the AGNIS team continues to support efforts of the European Blood and Marrow Transplantation Group (EBMT) and Eurocord to facilitate data exchange with European registries and cord blood banks, particularly for cord blood outcomes data. These data are critical for cord blood banks to meet their regulatory reporting requirements. CIBMTR has received pre-transplant data from the EBMT as a first step in the development of this important data exchange. More information regarding AGNIS can be found at <http://agnis.net>.

Cord Blood/Adverse Event System

Effective October 20th, 2011, all cord blood units (domestic and international) to be used for transplants at U.S. centers must be licensed or distributed under an FDA-accepted Investigational New Drug (IND) protocol. NMDP's IND protocol (10-CBA) provides a mechanism to allow access to unlicensed cords for FDA-specified indications on and after 10/20/11.

An Adverse Events system, integrated with the FormsNet application, was developed in conjunction with the implementation of the 10-CBA protocol to collect real-time reporting of serious adverse events caused by or likely caused by the product, as well as other product issues. The system has been in place since October 2011. Transplant centers can report product complaints (e.g., thawed on arrival, incomplete documentation), and cord blood banks can report deviations from their manufacturing processes (e.g., specific tests not done correctly). This system is not limited to NMDP-facilitated or 10-CBA products, so transplant centers are encouraged to report all product-related events. As events are reported, FormsNet will triage notifications to the NMDP, cord blood bank, or IND sponsor as appropriate to facilitate their regulatory investigation.

Coming in April 2012, this mechanism will also allow for reporting of marrow and peripheral blood products. For transplant center users familiar with the FormsNet application, accessing these forms is as simple as selecting the form from the Create Unscheduled Form dropdown on the Recipient Forms page.

Other SCTOD news: Cellular Therapies

Hematopoietic cells are increasingly used for indications other than hematologic malignancies and hematopoietic reconstitution, such as regenerative medicine. To better characterize this activity, the Cellular Therapies for Regenerative Medicine (CTRM) Form was released on January 18, 2011. This form collects registration data at one time point. This approach to data collection is similar to the EBMT process, and it facilitates an ongoing partnership between the two registries.

The form can be found at:

<http://www.cibmtr.org/DataManagement/DataCollectionForms/Documents/4000/Rev1.0/combine-4000%20CTRM%20r1.pdf>.



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