Center for International Blood and Marrow Transplant Research
Progress Report: January – December 2011

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1.0 OVERVIEW OF CIBMTR

The Center for International Blood and Marrow Transplant Research (CIBMTR) collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy research worldwide. CIBMTR is a combined research program of the Medical College of Wisconsin (MCW) and the National Marrow Donor Program (NMDP), facilitating critical observational and interventional research that has led to increased survival and enriched quality of life for thousands of patients.

These research activities are accomplished by the participation of a large network of transplant centers and the expertise of the CIBMTR Statistical Center. Support for this program is provided by the National Institutes of Health (NIH) grant # U24-CA76518 and other supplementary sources. This funding has resulted in the successful completion of hundreds of studies that positively impact clinical practice.

CIBMTR is now in its eighth year of a robust, collaborative, and outcomes-focused research endeavor. The organization spans two campuses and is committed to achieving the highest level of clinical research. This Progress Report covers the period from January 1 through December 31, 2011. Unless otherwise noted, all reported data are effective through December 15, 2011.

1.1 BACKGROUND

In 2004, MCW’s International Bone Marrow Transplant Registry (IBMTR), a voluntary association of more than 400 transplant centers in almost 50 countries, affiliated with the research division of the NMDP to create the CIBMTR, a combined research program with substantial expertise in observational research and clinical trials in HCT.

The IBMTR was founded in 1972 as a voluntary collaboration of HCT clinicians sharing data in order to facilitate research and advance the field. NMDP was established in 1987 to create a registry of unrelated donors for patients in need of HCT and improve outcomes for these patients. The NMDP established a network of U.S. transplant centers, donor centers, collection centers, apheresis centers, and cord blood banks and registries, as well as a Research Sample Repository of unrelated donor-recipient biologic samples.

In September 2006, CIBMTR was awarded a contract with the Health Resources and Services Administration (HRSA) through MCW to administer the Stem Cell Therapeutic Outcomes Database (SCTOD) (Section 2.6) for the C.W. Bill Young Cell Transplantation Program (the Program).

1.2 ORGANIZATIONAL STRUCTURE

CIBMTR now consists of a large network of more than 450 participating transplant centers that submit transplant-related data for patients, diseases, and treatments. Centers submit data at two levels: a Transplant Essential Data (TED) level that captures basic data and a Comprehensive Report Form (CRF) level that captures more detail (see Section 4.2). The CIBMTR Statistical Center, staffed by almost 200 employees, provides statistical support and analysis of these data.

The Chief Scientific Director is responsible for administrative and scientific operations, and the Chief Statistical Director is responsible for the statistical quality of all studies. The Associate Scientific
Directors assist in overseeing operational aspects of the CIBMTR Statistical Center. The various CIBMTR committees (Section 1.3) ensure that the organization meets the needs and priorities of its scientific and medical communities.

As identified in Figure 1.1, CIBMTR research involves four major programs:

- Observational Research
  - Clinical Outcomes
  - Health Services Research (HSR)
  - Survey Research
- Statistical Methodology Research
- Immunobiology Research
- Clinical Trials Support
  - Blood and Marrow Transplant Clinical Trials Network (BMT CTN)
  - Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT)

Figure 1.1: CIBMTR Organizational Structure

* Only centers that report CRF-level data participate in the CIBMTR Assembly
** Chief Medical Officer of NMDP
*** Health Services Research is a collaboration of CIBMTR and the NMDP Office of Patient Advocacy (OPA)
**** BMT CTN Data and Coordinating Center is a collaboration of CIBMTR, NMDP, and the EMMES Corporation (a contract research organization)
1.3 COMMITTEE STRUCTURE

Observational research using the CIBMTR Research Database is a core activity of the organization, and the CIBMTR committee structure ensures that research is conducted responsibly and efficiently. The Joint Affiliation Committee provides executive oversight. The scientific Working Committees (Section 1.3.1) manage research activities and statistical analyses. The Advisory (Section 1.3.3), Executive (Section 1.3.3.1), and Nominating (Section 1.3.5) Committees manage administrative issues, including policy making and organizational development. The Consumer Advocacy Committee (Section 1.3.3.2) provides patient perspective. The Immunobiology Steering Committee (Section 1.3.7) and Clinical Trials Advisory Committee (Section 1.3.7) provide scientific oversight for the Immunobiology Working Committee and RCI BMT, respectively.

1.3.1 Scientific Working Committees (WCs)

Most scientific questions posed by CIBMTR’s scientific and medical communities cannot be answered using data from single centers or addressed adequately in randomized clinical trials. To ensure broad input into the research process and efficient use of resources, CIBMTR created 19 WCs focused on specific research areas. Table 1.1 defines the scientific focus of each CIBMTR WC.

<table>
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<tr>
<th>WC Name</th>
<th>Scientific Focus</th>
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<tr>
<td>Acute Leukemia</td>
<td>HCT for acute leukemia and pre-leukemia</td>
</tr>
<tr>
<td>Autoimmune Diseases</td>
<td>HCT for autoimmune disorders including autoimmune cytopenia</td>
</tr>
<tr>
<td>Cellular Therapies</td>
<td>Non-transplant uses of hematopoietic stem cells</td>
</tr>
<tr>
<td>Chronic Leukemia</td>
<td>HCT for chronic leukemias, myelodysplastic disorders, and myeloproliferative disorders</td>
</tr>
<tr>
<td>Donor Health and Safety</td>
<td>Donor safety and outcomes</td>
</tr>
<tr>
<td>Graft Sources and Manipulation</td>
<td>Graft types, composition, and manipulation techniques</td>
</tr>
<tr>
<td>Graft-Versus-Host Disease</td>
<td>Biology, prevention, and treatment of GVHD and its complications</td>
</tr>
<tr>
<td>Health Policy and Psychosocial Issues</td>
<td>Social and economic barriers to HCT access, including quality of care and the influence of psychosocial factors on transplant outcomes</td>
</tr>
<tr>
<td>Immune Deficiencies/Inborn Errors of Metabolism</td>
<td>HCT for congenital and acquired immune deficiencies and inborn errors of metabolism</td>
</tr>
<tr>
<td>Immunobiology</td>
<td>Histocompatibility and other genetic and immunologic issues related to HCT</td>
</tr>
<tr>
<td>Infection and Immune Reconstitution</td>
<td>Prevention and treatment of post-transplant infections and issues related to recovery of immune function</td>
</tr>
<tr>
<td>International Studies</td>
<td>International issues and differences in HCT, focusing on non-U.S. investigators</td>
</tr>
<tr>
<td>Late Effects and Quality of Life</td>
<td>Long-term survival after HCT, including clinical and psychosocial effects of transplantation</td>
</tr>
<tr>
<td>Lymphoma</td>
<td>HCT for Hodgkin and non-Hodgkin disease</td>
</tr>
<tr>
<td>Non-Malignant Marrow Disorders</td>
<td>HCT for aplastic anemia, congenital disorders of hematopoiesis, autoimmune cytopenias, and other non-malignant hematopoietic disorders</td>
</tr>
<tr>
<td>Pediatric Cancer</td>
<td>HCT for childhood leukemias and other issues related to use of HCT in children</td>
</tr>
<tr>
<td>Plasma Cell Disorders</td>
<td>HCT for multiple myeloma and other plasma cell disorders</td>
</tr>
<tr>
<td>Regimen-Related Toxicity and Supportive Care</td>
<td>Preparative regimens, prevention, and treatment of early non-GVHD toxicities; supportive care in the early post-transplant period</td>
</tr>
<tr>
<td>Solid Tumors</td>
<td>HCT for solid tumors</td>
</tr>
</tbody>
</table>
Total WC membership exceeds 1,600 researchers, most of whom are HCT clinicians but also PhD statistical faculty and Master’s-level Statisticians from the CIBMTR Statistical Center, who provide their unique expertise in data analysis. This collaborative structure encourages a rigorous methodological approach to all CIBMTR projects, including:

- Designing and conducting relevant studies that use CIBMTR data, statistical resources, networks, and/or centers
- Setting priorities for observational studies that use the CIBMTR Research Database, including evaluating study proposals to ensure they are relevant to their subject area and will produce meaningful results
- Assessing and revising CIBMTR data collection forms as needed
- Planning and conducting workshops at CIBMTR meetings

WC membership is open to any researcher willing to take an active role in developing and conducting studies that use CIBMTR data and/or resources. Each WC is staffed by at least one MS-level Statistician, a PhD Statistical Director, and an MD Scientific Director from the CIBMTR Statistical Center. Each also has two to three Co-Chairs who are appointed by the Advisory Committee to non-renewable five-year terms. WC Co-Chair appointments are made each fall, with terms commencing at the close of the BMT Tandem Meetings each year (Section 7.1). Terms are staggered to facilitate succession and maintain continuity. Individuals may serve as Chair more than once, but not consecutively on the same committee. Co-Chairs participate in the nomination process for replacement positions and give special consideration to junior investigators, thus promoting ongoing leadership for the work of CIBMTR. A list of WC leadership can be found in Appendix C.

WC Co-Chairs provide subject matter expertise in autologous and allogeneic transplantation and understand CIBMTR organization and procedures. They must be members of CIBMTR centers that submit Comprehensive Report Forms (CRFs) (Section 4.2) and are compliant with Continuous Process Improvement (CPI) standards for data submission (Section 4.4.1), unless an exception is granted by the Advisory Committee. Co-Chairs are occasionally selected from outside these guidelines for their specific scientific expertise; for example, a scientist who directs a histocompatibility laboratory, apheresis center, or donor registry, who is committed to CIBMTR and the field of HCT.

Co-Chairs monitor and facilitate the progress of studies in their WC’s portfolio. They communicate with Principal Investigators (PIs) to address barriers and/or delays and participate in weekly CIBMTR Statistical Center study critiques. In addition to chairing annual WC meetings, Co-Chairs meet by teleconference every 4-8 weeks with their committee’s Scientific Director and biostatisticians to review the progress of study proposals and ongoing studies. Co-Chairs lead the annual WC meeting and, using input from that meeting, prioritize upcoming studies.

In November 2011, the CIBMTR Nominating Committee submitted recommendations, which the Advisory Committee approved, for the four open WC Co-Chair positions (Cellular Therapies, Graft Sources and Manipulation, International Studies, and Plasma Cell Disorders). As always, experts from international centers, representatives from a broad range of U.S. transplant centers, and mid-level investigators were encouraged to participate, thereby promoting continued growth for the work of CIBMTR. WC leadership also encourages junior investigators to participate by soliciting their
participation in protocol development. Over the past two years, more than one-third of first authors of CIBMTR publications were junior or mid-level PIs.

The CIBMTR Advisory Committee encourages each WC to develop clinically relevant and meaningful studies. To that end, WCs review proposals for CIBMTR observational studies that are solicited each fall. Proposals are reviewed by the Statistical Center and then discussed in-depth at the appropriate WC meeting at the annual BMT Tandem Meetings. The Chief Scientific Director, in consultation with the Chief Statistical Director and the Associate and Assistant Statistical Directors, allocates statistical resources to each WC. See Section 9 for a list of current CIBMTR WC studies. An abbreviated listing, updated biannually, can also be found on the www.cibmtr.org website.

1.3.2 Assembly

The CIBMTR Assembly is the organization’s voting membership. A representative from each CIBMTR center that submits CRF-level data serves on the Assembly, which elects members of the CIBMTR Advisory Committee, Nominating Committee, and Clinical Trials Advisory Committee. The Assembly meets annually during the BMT Tandem Meetings and is regularly updated on CIBMTR activities.

1.3.3 Advisory Committee

The Advisory Committee (Appendix D1) provides oversight for CIBMTR policies and scientific agenda and also partners with the WCs to prioritize scientific studies. Members are elected to three-year terms by the CIBMTR Assembly and must be from qualifying CRF centers. The Advisory Committee also includes appointed representatives from donor centers (1); collection centers (1); the patient community (2); the business community (1); the bioethics field (1); cord blood banks (1); NCI, NHLBI, NIAID, and HRSA (4); and senior members of the CIBMTR Statistical Center (7).

Prior to 2011, the Advisory Committee met biannually to review scientific and other activities of the CIBMTR. To facilitate more meaningful discussion and capitalize on time and resources, a single annual meeting (expanded to four hours) will be held at the BMT Tandem Meetings commencing in 2012. The Advisory Committee also meets as needed by teleconference.

All Advisory, Executive, and Nominating Committee terms begin on March 1.

1.3.3.1 Executive Committee

The CIBMTR Executive Committee (Appendix D2), a subcommittee of the Advisory Committee, ensures that the organization carries out its mission and adheres to CIBMTR policies and procedures; it also provides advice and counsel to the CIBMTR Statistical Center. Members include the Advisory Committee Chair, Chair-Elect or Immediate Past Chair, four Vice-Chairs (each representing a geographic region), appointed members of the Advisory Committee, and senior Statistical Center staff members who also serve on the Advisory Committee. The Executive Committee meets four times annually by teleconference and is responsible for:

- Providing scientific and policy advice to the Chief Scientific Director and Statistical Center
- Reviewing audit results and making recommendations for improvement
- Appointing a CIBMTR BMT Tandem Meetings Scientific Organizing Committee for the annual BMT Tandem Meetings
1.3.3.2 Consumer Advocacy Committee

The Consumer Advocacy Committee (Appendix D3) 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.

Co-Chairs serve on both the Advisory Committee and the Clinical Trials Advisory Committee (Section 1.3.7), and members also participate in Working Committees. A new member, a Hispanic parent of a child who received a cord blood transplant in 2000 at age 18 months, was appointed in 2011. The nine members meet in person at the annual BMT Tandem Meetings and by periodic conference calls.

1.3.3.3 Working Committee Guidelines Task Force

An ad hoc Working Committee Guidelines Task Force was formed by the Advisory Committee in late 2011 to define best practices for CIBMTR WCs and to help prioritize studies. Recommendations from this group will be presented to the CIBMTR Advisory Committee in February 2012 and will include the following goals:

- Disseminating research results to the public
- Collecting metrics to enhance progression of observational studies
- Engaging new clinicians in HCT research
- Defining characteristics of a productive WC in terms of:
  - Number of high-impact studies
  - Number of publications and length of time to publication
  - Efficiency of study development cycle and how to handle studies that are not progressing on schedule
  - Communication among WC members

1.3.5 Nominating Committee

The Nominating Committee (Appendix E) consists of five members elected by the CIBMTR Assembly and meets by teleconference(s) each fall. Following an annual call for nominations, the Nominating Committee prepares a slate of candidates for open positions on the Advisory, Nominating, and Clinical Trials Advisory Committees. Elections are held annually by confidential electronic ballot.

The Nominating Committee also makes recommendations to the Advisory Committee for open WC Chair and other leadership appointments, seeking recommendations from the CIBMTR Assembly, Advisory Committee, and incumbent WC Chairs.

1.3.7 Steering Committees

Two CIBMTR Steering Committees, the Immunobiology Steering Committee (Appendix F) and the Clinical Trials Advisory Committee (Appendix G), provide an additional level of oversight for use of certain resources.

The NMDP Histocompatibility Advisory Group also serves as the CIBMTR Immunobiology Steering Committee, which consists of appointed members and liaisons from CIBMTR, NMDP, the American
Society for Histocompatibility and Immunogenetics (ASHI), the NMDP Cord Blood Advisory Group, HRSA, and the C.W. Bill Young Marrow Donor Recruitment and Research Program. This committee reviews and approves the use of donor-recipient specimens from the NMDP Research Repository in CIBMTR studies and meets in person twice annually, in summer and at the annual BMT Tandem Meetings.

The Clinical Trials Advisory Committee assists in the review, approval, and oversight of proposals and protocols for Phase I and Phase II clinical trials submitted to the CIBMTR Resource for Clinical Investigation for Blood and Marrow Transplantation (RCI BMT) (Section 2.3.2). Its 10 members represent transplant physicians, non-physicians with experience in patient and donor issues, consultants, and representatives from CIBMTR and RCI BMT. The committee meets in-person during the BMT Tandem Meetings and by teleconference as needed.

1.4 CIBMTR STATISTICAL CENTER PERSONNEL

The CIBMTR Statistical Center spans two campuses—one located in Milwaukee, WI and one in Minneapolis, MN—and provides administrative, statistical, data management, clinical trials, information technology (IT), and personnel support for CIBMTR activities. It also benefits from a unique, collegial partnership with the Biostatistics Division of MCW.

CIBMTR Milwaukee has approximately 70 employees and is an academic division of the MCW Department of Medicine, which provides administrative support for the Milwaukee office. CIBMTR Minneapolis has approximately 107 employees and is a research division of NMDP. Several other NMDP departments, either under subcontract or through shared services, provide substantial support for CIBMTR activities, including the Finance, Bioinformatics, and Marketing and Communications departments.

1.4.1 Scientific Directors

CIBMTR Scientific Directors provide scientific and clinical leadership for the organization’s research activities.

1.4.1.1 Milwaukee Campus Scientific Directors

- **Mary M. Horowitz, MD, MS,** is Chief Scientific Director of CIBMTR, the Robert A. Uihlein Professor of Hematologic Research at MCW, Interim Chief of the Division of Hematology and Oncology at MCW, and an attending physician in the MCW HCT program. She received an MD from MCW in 1980 and an MS in biostatistics and clinical epidemiology from MCW in 1991. Dr. Horowitz is Principal Investigator for the Data and Coordinating Center of the BMT CTN and Research Director for the SCTOD program. Dr. Horowitz has been with CIBMTR since 1985.

- **J. Douglas Rizzo, MD, MS,** is Professor of Medicine at MCW, an attending physician in the MCW HCT program, and Associate Scientific Director for CIBMTR Data Operations. Dr. Rizzo received an MD from Johns Hopkins University Hospital in 1990 and an MS in epidemiology from MCW in 2005. He completed a Robert Wood Johnson fellowship in epidemiology and cost-effectiveness research at Johns Hopkins University in 1998. In 2008, he became Project Director for the SCTOD.
• **Mary Eapen, MD, MS**, is Associate Professor of Medicine at MCW and Associate Scientific Director of the CIBMTR. Dr. Eapen received an MD from Ahmadu Bello University, School of Medicine, Zaria, Nigeria in 1986, completed a residency in pediatrics at the University of Minnesota in 1997, and completed a fellowship in pediatric hematology/oncology at the University of Minnesota in 2000. Dr. Eapen is Scientific Director for the Graft Sources and Manipulation, Pediatric Cancer, Non-Malignant Marrow Disorders, and Immune Deficiencies/Inborn Errors of Metabolism Working Committees. Dr. Eapen is also Protocol Officer for several BMT CTN trials.

• **Marcelo Pasquini, MD, MS**, is Assistant Professor at MCW and an attending physician in the MCW HCT program. Dr. Pasquini received an MD from the State University of Londrina, Brazil, in 1997 and an MS degree in epidemiology from MCW in 2006. He completed a residency in internal medicine at the University of Miami/Jackson Memorial Hospital 2001, a fellowship in hematology/oncology fellowship at the University of Utah in 2004, and a blood and marrow transplantation fellowship at MCW in 2005, when he joined CIBMTR, and is Scientific Director of the Autoimmune Diseases, Cellular Therapies, International Studies, and Regimen-Related Toxicity and Supportive Care Working Committees. He is Protocol Officer and Medical Monitor for the BMT CTN and alternate CIBMTR representative to the Worldwide Network for Blood and Marrow Transplantation (WBMT).

• **Parameswaran Hari, MD, MS**, is Associate Professor of Medicine in the Division of Hematology and Oncology and Section Head and Clinical Director of the Adult Bone Marrow Transplant Program at MCW. Dr. Hari received an MD from the Jawaharlal Institute of Post Graduate Medicine, India, in 1995 and an MS in epidemiology from MCW in 2006. He completed fellowships in internal medicine and hematology at the Royal College of Physicians and the Royal College of Pathologists in the United Kingdom in 1997 and medical oncology and transplantation at MCW in 2004. He is Scientific Director of the Plasma Cell Disorders Working Committee. In June 2011, Dr. Hari received the MCW Engstrom Award for Clinical Contribution for promoting clinical efforts in the field of blood and marrow transplantation.

• **Wael Saber, MD, MS**, is Assistant Professor of Hematology/ Oncology at MCW and an attending physician in the MCW HCT program. Dr. Saber received an MD from Ain Shams University, Egypt, in 1995 and an MS in epidemiology from the University of Wisconsin in 2009. He completed a residency and internship in internal medicine in 1999 and 2001, respectively, at the Cleveland Clinic Foundation and a hematology fellowship in 2009, when he joined CIBMTR. He is Scientific Director for the Chronic Leukemia and Lymphoma Working Committees.

### 1.4.1.2 Minneapolis Campus Scientific Directors

• **Dennis Confer, MD**, is Chief Medical Officer of NMDP and Associate Scientific Director of CIBMTR. Dr. Confer received an MD from the University of Nebraska Medical Center and completed a residency and fellowship in hematology/oncology at the University of Minnesota Medical School. He was Principal Investigator (PI) for the development of CIBMTR’s A Growable Network Information System® (AGNIS®) (Section 6.3), Co-PI of the Data and Coordinating Center (DCC) of the BMT CTN, and Scientific Director of the CIBMTR Donor Health and Safety Working Committee. He is Vice President, North and South America, of the
1.0 OVERVIEW OF CIBMTR

World Marrow Donor Association (WMDA) and Secretary/Treasurer of the Executive Board of the Worldwide Network for Blood and Marrow Transplantation (WBMT).

- **Daniel Weisdorf, MD,** is Professor of Medicine and Director of the Adult Blood and Marrow Transplant Program at the University of Minnesota, Scientific Director of NMDP, and President of the American Society for Blood and Marrow Transplantation (ASBMT). Dr. Weisdorf received an MD from Chicago Medical School, completed a residency at Michael Reese Hospital in Chicago, and completed a fellowship at the University of Minnesota. He is Senior Research Advisor to CIBMTR and Scientific Director of the CIBMTR Acute Leukemia Working Committee. He previously chaired the IBMTR Acute Leukemia Working Committee and was a member of the IBMTR Executive Committee.

- **Mukta Arora, MD, MBBS, MS,** is Associate Professor in the Hematology, Oncology, and Transplantation Division at the University of Minnesota. Dr. Arora received an MD from the University of Kanpur, India, in 1997 and completed residency and fellowship training in hematology, oncology, and transplantation at the University of Minnesota in 2002. She is Scientific Director of the Graft-Versus-Host Disease (GVHD) and Solid Tumors Working Committees.

- **Navneet Majhail, MD, MS,** is Medical Director and Director of Health Services Research at NMDP and Adjunct Assistant Professor in the Hematology, Oncology, and Transplantation Division at the University of Minnesota. Dr. Majhail received an MD from All India Institute of Medical Sciences, India, in 2000 and an MS in clinical research from the University of Minnesota School of Public Health in 2006. He completed residencies in Radiation Oncology at the All India Institute of Medical Sciences, India, and Internal Medicine at the Cleveland Clinic Foundation, and a fellowship in Hematology/Oncology at the University of Minnesota. He is Scientific Director for the Health Policy & Psychosocial Issues and the Late Effects & Quality of Life Working Committees, as well as the CIBMTR Health Services Research Program.

- **Willis Navarro, MD,** is Medical Director of Transplant Medical Services at NMDP. Dr. Navarro received an MD from the University of California San Francisco (UCSF) in 1990 and completed a residency in internal medicine at Yale-New Haven Hospital, followed by a hematology/oncology fellowship at UCSF in 1998. He is a Medical Monitor and a Protocol Officer for BMT CTN (Section 2.3.1) and Scientific Director of the Resource for Clinical Investigation in Blood and Marrow Transplantation (RCI BMT) (Section 2.3.2). Before joining NMDP, Dr. Navarro was active in clinical trials development and implementation as Assistant Medical Director in BioOncology Development at Genentech, Inc., in San Francisco, from 2005-2008.

- **Stephen Spellman, MBS,** is a Senior Manager at CIBMTR and is Principal Investigator for the NMDP Research Sample Repository. Mr. Spellman received an MBS in immunobiology and molecular biology in 2000 from the University of Minnesota. He leads the CIBMTR Immunobiology Research (CIR) team (Section 2.4) and oversees the NMDP Research Sample Repository for CIBMTR and the BMT CTN. He is also Co-Scientific Director of the CIBMTR GVHD and Immunobiology Working Committees and staff liaison to the NMDP Cord Blood Advisory Group Research subcommittee and Histocompatibility Advisory Group (also known as the CIBMTR Immunobiology Steering Committee). He is Program Manager for the NMDP Office of Naval Research Grant.
1.4.1.3 Other Scientific Directors

- **Stephanie J. Lee, MD, MPH**, is Professor of Medicine at the University of Washington and a Member at Fred Hutchinson Cancer Research Center in Seattle, Washington. Dr. Lee received an MD from Stanford University in 1990 and an MPH from Harvard School of Public Health in 1996. She completed a fellowship at Brigham Women’s Hospital in hematology/oncology in 1996. Dr. Lee is Scientific Director of the CIBMTR Immunobiology Working Committee.

- **Marcie Tomblyn, MD, MS**, is Associate Member in the Department of Blood and Marrow Transplantation and Director of BMT Clinical Research at Moffitt Cancer Center, Florida. Dr. Tomblyn received an MD from the University of Kentucky in 1997 and an MS in clinical investigation from Northwestern University in 2004. She completed a hematology/oncology fellowship at Northwestern University in 2004 and fellowships in Hematology/Oncology and Hematopoietic Stem Cell Transplantation in 2003 and 2004, respectively. She is Scientific Director of the CIBMTR Infection and Immune Reconstitution Working Committee and a Protocol Officer for several BMT CTN trials.

1.4.2 CIBMTR Biostatistics Staff

CIBMTR Biostatisticians provide statistical support and analyses for all CIBMTR research activities.

- **John P. Klein, PhD**, is Professor and Head of the Division of Biostatistics at MCW, Chief Statistical Director for CIBMTR, and leads the Biostatistics group. Dr. Klein received an MS in 1975 from the University of Wisconsin Milwaukee and a PhD in 1980 from the University of Missouri – Columbia. He has been with MCW and IBMTR since 1993 and is Biostatistician for the Cellular Therapies, Health Policy & Psychosocial Issues, Immunobiology, and International Studies Working Committees. Dr. Klein is an internationally recognized expert in survival analysis.

- **Mei-Jie Zhang, PhD**, is Professor of Biostatistics at MCW. Dr. Zhang received an MS and PhD from Florida State University in 1989 and 1991, respectively. He completed a fellowship at the IBMTR in Milwaukee in 1993. He has been with IBMTR since 1991 and is Biostatistician for the Acute Leukemia, Graft Sources & Manipulation, Lymphoma, Pediatric Cancer, and Plasma Cell Disorders Working Committees. Dr. Zhang’s primary research interest is survival analysis and analysis of HCT transplant data that involve censored and/or truncated time-to-event data and competing risk events.

- **Brent Logan, PhD**, is Professor of Biostatistics at MCW. Dr. Logan received an MS and PhD from Northwestern University in 1998 and 2001, respectively. He has been with CIBMTR since 2001 and is Biostatistician for the Donor Health and Safety and Regimen-Related Toxicity and Supportive Care Working Committees. He is Lead Statistician for the BMT CTN and Statistical Consultant to NMDP. Dr. Logan’s expertise is in clinical trial design and analysis of multiple endpoints. He led the development of the statistical approach for Center-Specific Outcomes Analysis (now the purview of the CIBMTR through the SCTOD).

- **Tao Wang, PhD**, is Associate Professor in the Division of Biostatistics and has a research interest in statistical genetics. Dr. Wang received an MS from Southeastern University, Nanjing, China, in 1990 and a PhD from North Carolina State University in 2001. He has been with
CIBMTR since 2001 and is Biostatistician for the Graft-versus-Host Disease and Immunobiology Working Committees.

- **Ruta Brazauskas, PhD**, is Assistant Professor with the Division of Biostatistics. Dr. Brazauskas received an MS from the University of Texas at Dallas in 1999 and a PhD from MCW in 2003. She has been with CIBMTR since 2008 and is Biostatistician for the Late Effects & Quality of Life Working Committees.

- **Kwang Woo Ahn, PhD**, is Assistant Professor with the Division of Biostatistics. Dr. Ahn received an MS and PhD from the University of Iowa in 2005 and 2008, respectively. He has been with CIBMTR since 2008 and is Biostatistician for the Autoimmune Diseases, Chronic Leukemia, Infection and Immune Reconstitution, and Solid Tumors Working Committees.

- **Jennifer Le-Rademacher, PhD**, is Assistant Professor with the Division of Biostatistics. She received an MS from Georgia State University in 2004 and a PhD from University of Georgia in 2008. She has been with CIBMTR since 2009 and is Biostatistician for the Immune Deficiencies/Inborn Error, and Non-Malignant Marrow Disorders Working Committees. She also provides statistical support to the BMT CTN and the RCI BMT.

The following Master’s-level biostatisticians work in the CIBMTR Statistical Center on the Milwaukee campus:

- **Waleska S. Pérez, MPH** (Associate Statistical Director; joined CIBMTR Statistical Center in 1998)
- **Jeanette Carreras, MPH** (since 2003)
- **Wensheng (Vincent) He, PhD, MS** (since 2006)
- **Zhiwei Wang, MS** (since 2007)
- **Min Chen, MS** (since 2008)
- **Peigang Li, MS** (since 2009)
- **Xiaochun Zhu, MS** (since 2009)
- **Xiaobo (Tony) Zhong, MS** (since 2010)
- **Zhenzuan (Kenny) Hu, MS** (since 2011)
- **Sandra Tomany-Korman, MS** (since 2011)

The following Master’s-level biostatisticians work in the CIBMTR Statistical Center on the Minneapolis campus:

- **Michael Haagenson, MS** (Senior Statistician; since 2000, served in NMDP Research Operations prior to the CIBMTR affiliation)
- **Tanya Pedersen, MPH** (since 2004)
- **Pintip Chitphakdithai, PhD** (since 2004)
- **Anna Hassebroek, MPH** (since 2006)
- **John Bosco Umejiego, MBBS, MPH** (since 2010)
- **Ying Shan, MS** (since 2011)
1.4.3 Other Key CIBMTR Milwaukee Staff

- **Janet Brunner, PA-C**, is Program Director/Data Operations at the CIBMTR Milwaukee campus following many years of clinical experience as a Physician’s Assistant, and she continues to work as a staff Physician Assistant on the MCW inpatient BMT Unit. She is primarily responsible for ensuring that CIBMTR Clinical Research Coordinators (CRCs) and other data management staff adhere to established standard operating procedures and perform effectively; for developing training programs; and for monitoring transplant center Continuous Process Improvement (CPI) (Section 4.4.1). She also assists CRC staff on both campuses with clinical transplant-related questions. She is Six Sigma Green Belt-certified and facilitates process reviews using Six Sigma and Lean techniques (see Section 1.5.3).

- **Michael Collins, MS**, is Director of the Milwaukee-based information technology (IT) team. Mr. Collins received an MS in Medical Informatics from the Milwaukee School of Engineering and MCW. Mr. Collins previously served as Director of Research Informatics at Fox Chase Cancer Center in Philadelphia and has many years of experience consulting as a software engineer and software architect in healthcare settings. His experience with NCI’s cancer Bioinformatics Grid overlaps with CIBMTR’s AGNIS project (Section 6.3).

- **Sue Lorenz** is Business Manager for CIBMTR. She has been with CIBMTR since 2006 and is responsible for overseeing the program’s financial, personnel, and office practices to ensure compliance with institutional and federal policies and procedures.

- **Kathleen (Kitty) Marquardt, RN, MS**, is Associate Director of CIBMTR and joined the organization in January 2011. She has over 20 years of leadership experience in not-for-profit healthcare organizations and has served as the Principal Investigator/Project Director on both federal grants and cooperative agreements. Ms. Marquardt works with Dr. Horowitz on the administrative coordination of CIBMTR.

- **Waleska S. Pérez, MPH**, is Associate Statistical Director of CIBMTR, working under the direction of the Chief Scientific Director and Chief Statistical Director. Ms. Perez oversees the CIBMTR Master’s-level biostatisticians and provides administrative oversight of the CIBMTR Observational Research Program.

- **Paula Watry** is Associate Director-General Operations. She joined CIBMTR in 2004 after 10 years as a Physician’s Assistant in the MCW HCT program. She works closely with Dr. Horowitz on all operational activities.

1.4.4 Other Key CIBMTR Minneapolis Staff

- **Roberta King, MPH**, is Vice President of CIBMTR Minneapolis. She has worked with the National Marrow Donor Program since 1990 and served in its research area since 1993. Ms. King received her Master’s in Public Health in 1988 from the School of Public Health, University of Minnesota. She is responsible for oversight of the administrative, scientific, and statistical support activities on the Minneapolis campus of CIBMTR, which include research administration, human subject protection program, data management, auditing and monitoring, observational research, prospective research, and IT. She also serves as Staff Liaison to the NMDP Donor and Patient Safety Monitoring Committee.
• **Debra Christianson** is Senior Manager of Auditing and Monitoring for CIBMTR Minneapolis. Her group is responsible for monitoring RCI BMT clinical trials and conducting on-site source document audits on data submitted to the research database, as well as writing the FormsNet instruction manual. Ms. Christianson holds Associate and Applied Science degrees, and she is a certified Registered Health Information Technician (RHIT) through the American Health Information Management Association (AHIMA).

• **Rebecca Drexler** is Senior Manager of Prospective Research on the Minneapolis campus. She has more than 25 years of experience in medical research, clinical and regulatory affairs. Ms. Drexler joined the research department at NMDP in 2001 to manage clinical trials, and joined the CIBMTR team in 2004. She is responsible for activities of the RCI BMT including the Survey Research Group, and oversees the administration of the Clinical Trials Advisory Committee.

• **Marie Matlack** is Senior Manager of Data Management for the CIBMTR Minneapolis campus and has been with NMDP since 1990. Ms. Matlack oversees form revision and development, the Continuous Process Improvement (CPI) program (Section 4.4.1), and ensures that the CRCs, Senior Research Programmers, data entry, and imaging staff adhere to established standard operating procedures and perform effectively. She works with the Minneapolis IT staff to develop enhancements to FormsNet. She is Six Sigma Green Belt-certified and facilitates process reviews using Six Sigma and Lean techniques (Section 1.5.3).

• **Colleen O’Neill** is Director of the Minneapolis-based IT staff. Ms. O’Neill joined CIBMTR/NMDP in February 2010, after eight years as a senior consultant. As Program Manager for Bioinformatics at NMDP, she is responsible for improving the processes and quality of FormsNet and AGNIS releases. Ms. O’Neill earned certification as a Project Management Professional (PMP) in 2002.

In addition, **Martin Maiers**, Director of Bioinformatics Research at NMDP, is a key partner with CIBMTR. He collaborates on research with the CIBMTR bioinformatics and immunobiology teams. Mr. Maiers earned an MS in computer science from the University of Minnesota and is a member of the American Society for Histocompatibility & Immunogenetics, European Federation for Immunogenetics, WMDA, and International Immunomics Society. He is also Vice-Chair of the WMDA IT Working Group that develops international informatics standards for cellular therapy.

### 1.5 STAFF TRAINING AND ORGANIZATIONAL DEVELOPMENT

CIBMTR remains committed to staff training and development and continues to promote initiatives that support this goal. New and continuing training initiatives offered in 2011 are described in this section.

#### 1.5.1 Taking CIBMTR to the Next Level

In January 2011, CIBMTR’s Senior Leadership launched an initiative called “Taking CIBMTR to the Next Level” that was designed to create unity and efficiency across the two campuses. This initiative focuses on aligning leadership, improving communication, clarifying relationships to parent organizations (MCW and NMDP), empowering decision-makers, assessing organizational structure, establishing consistent cross-campus staff training, and disseminating CIBMTR’s purpose throughout the CIBMTR organization.
The Senior Leadership team is committed to these initiatives and meets bi-weekly to discuss progress and plan next steps. A new bi-campus management group now meets regularly to ensure progress on the initiatives.

1.5.2 Master’s-Level Statistician Workshop

Each CIBMTR WC is assigned a Master’s-level Statistician who plays a critical role in research project management including data file preparation, performing technical statistical analyses, and coordinating all WC activities. In 2011, the Master’s-level biostatisticians began holding monthly meetings to ensure consistency and coordination of WC management and research studies, as well as compliance with CIBMTR policies, procedures, and report generation.

In November 2011, a two-day Master’s-level Statistical Workshop was held on the Milwaukee campus. All Master’s-level statisticians were in attendance. Based on the positive feedback received from participants, subsequent workshops will be scheduled. Additional topics have already been identified.

The first day of the workshop was hosted by CIBMTR transplant physicians from MCW and CIBMTR Statistical Center and IT management and topics included:

- Overview of HCT
- Conditioning regimens
- Post-transplant complications
- Graft-versus-host disease
- Current and future state of IT tools for statisticians
- Data file preparation: common data checks

The second day of the workshop was hosted by the MCW Division of Biostatistics, a key CIBMTR partner since 1985 and topics included:

- Univariate survival models
- Competing risks
- Basic Cox model
- Pseudo-value regression
- Covariate adjusted survival curves
- Time-dependent covariates and delayed entry

1.5.3 Other Training Initiatives

Internal training initiatives included:

- **CIBMTR staff training.** In 2011, managers from both campuses updated the training syllabi to ensure consistent training for staff in the following functional units: Clinical Research Coordinator (CRC), data entry, imaging, IT, immunobiology, biostatistics, and administrative support.

- **Process review training.** During 2011, two senior data operations staff members (one from each campus) were certified as Green Belts in Six Sigma methodology, a process improvement system. The initial CIBMTR Six Sigma project focused on “data clean-up” and resolved nearly
all issues to allow FormsNet™ data to be added to the CIBMTR Research Database. In 2011, additional staff members were trained in Six Sigma Yellow Belt, Green Belt, and Lean methodologies in an effort to continue these process improvement activities.

- **All-staff meetings.** CIBMTR holds quarterly staff meetings to share information between the two campuses and provide updates on ongoing projects, new initiatives, and departmental objectives and measures. They also highlight staff accomplishments.

External training initiatives included:

- **Clinical Research Professionals/Data Management Conferences.** Two CIBMTR Clinical Research Professionals/Data Management conferences were held in 2011: one during the BMT Tandem Meetings in February and one during the NMDP Council Meeting in November *(Section 7.1).* Combined registration for the conferences was 362 network center personnel. These conferences provide training for upcoming forms, disease-specific reports, health policy updates, and other relevant issues in HCT and ultimately ensure that CIBMTR and SCTOD forms are accurately completed.

### 1.5.4 Staff Continuing Education

During 2011, 88% staff at both the Milwaukee and Minneapolis campuses attended various training programs in operations, continuing medical education, leadership development, and change management. Additionally, staff attended training in the following Microsoft programs to support operational efficiencies in report generation and data management:

- Excel: Linking & Consolidating; Macros Using Visual Basic For Applications (VBA); Advance Formulas, Functions; Pivots tables; Excel 07 Database, Lists, and Pivot Tables
- Word: Managing Large Documents
- PowerPoint
- Outlook

### 1.5.5 Staff Communication

A monthly newsletter called *Campus Connections* is regularly distributed via email to staff. Content is provided by authors from both campuses and includes articles about upcoming major projects, internal activities, local events that feature individual and group accomplishments, and newly-published journal articles.
2.0 CIBMTR RESEARCH ACTIVITIES

CIBMTR’s primary objective is to collect and analyze HCT clinical outcomes data to address important issues in transplantation and cancer treatment. Additionally, CIBMTR remains committed to providing information to clinicians, patients, and the public. Its constantly growing collection of data and peer-reviewed publications can be used for patient care decisions, developing research studies, education, transplant center administrative needs, and CIBMTR research.

2.1 OBSERVATIONAL RESEARCH

In addition to maintaining a high-quality database, the CIBMTR ensures that these data are used for scientifically and clinically relevant research by engaging the scientific community and providing investigators with the statistical and scientific support necessary to perform studies that adhere to rigorous methodological standards.

In 2011, CIBMTR research resulted in the following milestone accomplishments:

- Among the 19 WCs, 236 studies were in progress at the end of December.
- The following proposals were submitted to CIBMTR WCs to be considered for presentation at WC meetings (which occur during the BMT Tandem Meetings):
  - For the 2011 WC meetings, CIBMTR received 97 study proposals; 62 were presented, and 37 were accepted.
  - For the 2012 WC meetings, CIBMTR received 102 study proposals; Statistical Center evaluations are in progress.
- In 2011, 45 CIBMTR scientific and statistical methodology papers were published in peer-reviewed journals, including early electronic publications and printed publications. As of December 27, 2011, an additional 3 publications are in press, and 14 were submitted for publication and are under review.
- CIBMTR PIs presented the following abstracts at annual scientific meetings:
  - At the 2011 BMT Tandem Meetings, 7 abstracts (6 oral and 1 poster) were presented.
  - At the 2011 ASH Annual Meeting, 13 CIBMTR abstracts (10 oral and 3 posters) were presented.

CIBMTR also contributes to the management and completion of Blood and Marrow Transplant Clinical Trials Network (BMT CTN) studies that are accepted for abstract presentation. BMT CTN study PIs presented 5 abstracts (1 Plenary Session, 2 oral presentations, and 2 posters) at the 2011 ASH Annual Meeting in December. See Section 2.3.1 for more information about the BMT CTN and Appendix H for a list of publications.

2.1.1 External Review Panel

In 2009, CIBMTR repeated an initiative first undertaken in 2002 and solicited feedback from an External Review Panel to ensure efficient use of resources and promote high-impact clinical studies and research. The panel included experts in clinical and experimental HCT, cord blood banking, immunology, outcomes and health services research, clinical trials, bioethics, and statistics. The review feedback recognized CIBMTR’s role as a vital community resource and noted that all 2002 review
recommendations were implemented. This progress report describes CIBMTR initiatives undertaken in response to the 2009 External Review Panel recommendations, including:

- Establishing processes for reviewing and modifying the scientific agenda
- Facilitating timely completion of studies
- Enhancing data collection and sharing technologies
- Expanding specific research areas

2.2 CIBMTR STUDIES

CIBMTR provides many opportunities to conduct research in HCT and cellular therapy and encourages both senior and junior investigators to participate. CIBMTR WCs provide an opportunity for investigators to collaborate with leaders in the field and to leverage the unique resources of the CIBMTR Research Database. For a complete list of CIBMTR WC studies and their status updates, see Section 9. The CIBMTR website (www.cibmtr.org) also includes a biannual status update of studies.

2.2.1 Role of the Principal Investigator (PI)

The study PI is responsible for timely and efficient completion of his or her studies with full contribution and engagement. Once a study proposal is accepted by a CIBMTR WC, the PI is asked to sign a Letter of Commitment that delineates the responsibilities and expectations of both the PI and the CIBMTR Statistical Center at each stage of the WC study development cycle (Appendix K).

2.2.2 Study Prioritization

In response to a recommendation from the 2009 External Review Panel (Section 2.1.1), the CIBMTR Advisory Committee has worked to prioritize CIBMTR studies and to evaluate their impact and the speed in which they are accomplished. The primary purpose is to ensure efficient use of CIBMTR’s limited, yet critical, statistical resources. Numerous improvements were implemented during the WC meetings in February 2011, including standardization of proposal voting practices, use of impact factors and characterization keys, use of complete study titles to better reflect study objectives, and availability of comprehensive study summaries to keep WC members better informed.

2.2.3 Collaborative Studies

CIBMTR WCs continue to grow a strong cooperative research base. In 2011, there were 59 ongoing collaborative studies with numerous organizations, including international and U.S.-based hospitals, research centers, universities, and registries.

2.3 CLINICAL TRIALS SUPPORT AND PROSPECTIVE RESEARCH

The CIBMTR database supports other federally funded and non-federally funded programs and projects, each benefiting from the scientific advances and resources of the others. This is particularly true in the support provided to the BMT CTN and RCI BMT to design and conduct clinical trials.

The CIBMTR Research Database is an important resource to the BMT CTN and RCI BMT, which conduct large and small clinical trials, respectively. These data are used to design, monitor, and analyze clinical trials. Additionally, long-term follow-up data of patients enrolled in BMT CTN and RCI BMT
trials are collected by routine CIBMTR data collection processes. The CIBMTR Statistical Center provides expertise and other resources that support both large and small clinical trials in several ways:

- **Trial planning.** Investigators planning clinical trials in HCT use the CIBMTR database to determine which patient populations may be available for trials. With the aid of CIBMTR Statistical Center staff, they can estimate how changing eligibility criteria will affect patient accrual. The CIBMTR database provides a more precise, less biased estimate of the baseline outcomes of interest than literature reviews, expert opinions, or personal experience at a transplant center. The database can identify the most common supportive care and other practices in potentially eligible patients so that clinical protocols can be written that are acceptable to most transplant centers. CIBMTR routinely makes this information available to BMT CTN and RCI BMT protocol teams.

- **Data collection instruments.** CIBMTR has an open policy for sharing data collection forms and database structures. The forms are freely available on the CIBMTR website and are the basis for data collection in many clinical trials. These forms reflect the knowledge and expertise not only of Statistical Center personnel but also many transplant experts on WCs who evaluate and revise the data collection forms.

- **Statistical consultation.** Statistical Center personnel have provided statistical review of several HCT clinical trial protocols and are considered expert resources. CIBMTR statisticians also serve in that role for the BMT CTN.

- **Trial interpretation.** The CIBMTR database is a valuable tool for evaluating results of clinical trials, especially single-arm studies. Using the database, the Statistical Center can provide matched controls for patients treated in single and multi-institution studies of transplant strategies, thus allowing more accurate analysis of treatment effects after controlling for patient characteristics. The BMT CTN uses this approach to evaluate Phase II data before embarking on large Phase III trials.

### 2.3.1 Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

The BMT CTN conducts large, multi-institutional phase II and III trials focused on HCT. The BMT CTN Data Coordinating Center (DCC) maintains continuity of operations and facilitates effective communications. CIBMTR shares administration of the DCC with NMDP and the EMMES Corporation, a contract research organization. CIBMTR submitted a competitive application for continued administration of the BMT CTN DCC in October 2010, and Cooperative Agreement 2U10HL069294-11 was awarded in August 2011.

During 2011, the BMT CTN surpassed 4,200 total enrollments in its 13 completed and 14 currently open trials. One trial was selected for presentation at the prestigious ASH Plenary Session (see Appendix I). A progress report covering BMT CTN activities and protocols can be found at www.bmtctn.net. For a list of 2011 BMT CTN publications, see Appendix H. For a list of all BMT CTN protocols, see Appendix J.

### 2.3.2 Resource for Clinical Investigation in Blood and Marrow Transplantation (RCI BMT)

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 BMT CTN. The primary focus of this CIBMTR prospective research is interventional research, and their projects are overseen by the Clinical Trials Advisory Committee (Section 1.3.7 and Appendix G).
RCI BMT works with external institutions, pharmaceutical companies, and private corporations to obtain funding for studies. For a list of current RCI BMT projects, see Appendix J.

### 2.4 IMMUNOBIOLOGY RESEARCH ACTIVITIES

CIBMTR leverages the NMDP’s investment in development of an unrelated donor-recipient specimen Research Sample Repository with NIH’s investment in the CIBMTR database. These data are used to perform studies that link genetic and immunobiologic data with clinical phenotype data.

The Related Donor Research Sample Repository, an SCTOD contract requirement (Section 2.6), is a unique opportunity to enhance immunobiologic research. Related donor and recipient samples are better matched than unrelated recipients for human leukocyte antigen (HLA)—a measure of immunological compatibility—thus reducing the confounding effects of HLA disparity in clinical research. The Related Donor Research Repository facilitates an organized approach to studying transplant biology across the full spectrum of allogeneic HCT.

As of December 2011, the number of pilot centers collecting related recipient-donor sample pairs for this new initiative increased to 44 centers. The related pair samples are an important addition to the existing sample repository. There was a 91%, 63%, and 70% increase in related recipient, donor, and pair sample inventory respectively in the last year. In response to a recommendation from the 2009 External Review Panel (Section 2.1.1), CIBMTR has worked to more widely disseminate information about what is available in the Research Sample Repository to individuals with the necessary expertise to do appropriate studies.

As of December 2011, the repository included:

- 1,431,514 million aliquots
- 18,897 cell lines
- 40,197 samples from unrelated donors and 1,247 from related donors
- 39,660 samples from unrelated recipients and 1,359 from related recipients
- 4,977 samples from unrelated cord blood units
- 22,784 samples from complete unrelated adult donor-recipient pairs, 1,135 from related adult donor-recipient pairs, and 1,984 from complete unrelated cord pairs

CIBMTR Immunobiology Research (CIR), delineated as a distinct program in 2010, held a strategic planning session in April 2011 to further define its goals for the coming year and establish the following mission statement: “Improve transplantation outcomes through a better understanding of immunology and genetics.” The goals for 2011 focused on identifying investigators for potential collaboration and disseminating information about the program and resources, e.g., data and samples. Several initiatives were defined and are in various states of completion:

- The first initiative was to develop a survey to solicit feedback on the process, experience, and new proposals from past Immunobiology Working Committee (IBWC) investigators who used the Research Sample Repository. Responses to the survey offered mainly positive feedback on the sample quality and interaction with the IBWC.
- The second initiative was to enhance the presentation of inventory details to clearly define samples available by disease, graft source, transplant year, and other key variables critical to
prospective investigators. Updated tables were developed for the Research Sample Repository and are currently under refinement for public dissemination. The CIBMTR website is regularly updated to ensure that the details of the repository inventory and resources are available for retrospective and prospective investigators.

- The last initiative was to develop and implement a strategy to identify key investigators on the periphery of the HCT field whose research efforts could benefit from the resources available through the CIR program. The ultimate goal is to develop strategic partnerships and increase visibility to the program within and beyond the HCT community. A plan for outreach was completed in 2011, and implementation is planned for 2012.

### 2.5 HEALTH SERVICES RESEARCH (HSR) PROGRAM

The CIBMTR conducts research through the HSR Program in health policy and health services issues involving HCT, such as health care disparities, economic aspects, practice patterns, and quality and accessibility of care. The HSR Program is a collaborative effort between the CIBMTR and the NMDP Patient Services department, and its work complements research using the CIBMTR Research Database that is conducted through the Health Policy and Late Effects & Quality of Life WCs. To meet its scientific goals, the HSR Program continues to collaborate with other investigators and explore opportunities for additional funding. HSR Program publications in 2011 are listed in Appendix H.

The HSR Program is currently involved in the following studies:

- **Rural Health Initiative.** This study investigates barriers to clinical trial participation among two groups: African-American/Black parents of children with sickle cell disease and adolescents with sickle cell disease. Study partners included University of Illinois at Chicago Medical Center, the Children’s Medical Center of Dallas, and Emory University School of Medicine. Patient and parent focus groups were conducted in Chicago, Dallas, and Atlanta. A manuscript based on this project is under peer review.

- **Pilot study of HCT costs.** In collaboration with the Chronic Disease Research Group in Minneapolis, the HSR Program is exploring the feasibility of using a commercial payor database (MarketScan®) to study costs of HCT. This project has been funded through a pilot grant from the University of Minnesota Masonic Cancer Center. Preliminary analysis used ICD-9 procedure and diagnosis codes to identify 3,816 patients receiving allogeneic or autologous HCT from 2007 to 2009. Ongoing analyses will evaluate inpatient and outpatient costs over the first 100 days after transplantation. Results from the pilot study will be used to pursue further research on costs associated with HCT.

- **Transplant provider and center factors and outcomes of allogeneic HCT.** Transplant center and provider characteristics (“center effects”) can influence delivery of care after transplantation and overall patient outcomes. A national survey of U.S. transplant centers will be conducted in early 2012 to obtain information about transplant center personnel, infrastructure, and delivery of care models. This goal of this study is to better understand HCT center effects and help identify center-specific factors that improve patient outcomes.

- **The financial impact of allogeneic HCT on the patient and family.** In collaboration with the Health Policy WC, this pilot study examines the feasibility of collecting patient-reported, out-of-pocket cost information over the first three months after allogeneic HCT. In addition, this study
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2.0 CIBMTR RESEARCH ACTIVITIES

tests the feasibility of studying the financial impact on patients during the first two years after transplantation. The University of Minnesota, Medical College of Wisconsin, and Roswell Park Cancer Institute are participating in this study, and thirty patients are enrolled. The first phase of this study is complete, the patient/caregiver out-of-pocket cost data are being analyzed, and an abstract has been submitted for presentation at the 2012 BMT Tandem Meetings. Data collection for the long-term financial impact of transplant is ongoing.

• Hematopoietic cell transplantation in 2020: A health care resource and infrastructure assessment. The NMDP, in collaboration with other key academic organizations, experts, and stakeholders, is in the third year of multi-year symposia to address system capacity challenges to the future growth of HCT. The goal of the NMDP is to facilitate 10,000 transplants annually by 2015, which is double the current number. Using a deliberative process model, seven working groups have made recommendations for addressing infrastructure and personnel shortages. The HSR program provides expertise and support to the working groups in areas such as survey research, data analysis, literature review, and dissemination of findings.

2.6 STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD)

Since 2006, CIBMTR has administered the SCTOD for the HRSA-sponsored C.W. Bill Young Cell Transplantation Program (“the Program”), which was established by the Stem Cell Therapeutic and Research Act of 2005 (Public Law 109-129). Continued support for the SCTOD is provided the Stem Cell Therapeutic and Research Reauthorization Act of 2010, which was approved by Congress, signed by the President, and became Public Law #111-264 on Oct. 8, 2010.

The Program has several goals:

• Collecting, analyzing, and reporting outcomes data for all allogeneic transplants and other therapeutic uses of blood stem cells
• Publicizing information about HCT available to patients, families, health care professionals and the public
• Defining better processes for identifying unrelated matched marrow donors, peripheral blood stem cell donors, and cord blood units through one electronic system
• Increasing availability of unrelated adult volunteer donors and cord blood units
• Expanding research to improve patient outcomes.

2.6.1 SCTOD Requirements

CIBMTR’s infrastructure and expertise in data collection, management, and analysis provide efficient management of the SCTOD for both the government and participating HCT programs. In 2011, CIBMTR met the following SCTOD requirements:

• Research and data analysis
  • Assessing Quality of Life (QoL) for transplant recipients. A pilot project is in progress to collect QoL data during the first year after HCT and evaluate processes and procedures for direct-to-patient communication for the collection of these data. Five adult transplant centers and three pediatric centers are participating in this pilot study (09-SQOL). Site
initiation visits were held over the summer and accrual began in August. By mid-December 2011, 29 of 270 targeted patients were enrolled.

- **Establishing a related donor-recipient research sample repository.** During 2011, 44 centers participated or received approval to participate in the related donor-recipient transplant research sample collection. In 2011, 30 centers submitted 787 samples, bringing the cumulative total to 2,329 samples.

- **Preparing customized reports**
  - **Center-specific outcomes report.** This report, provided to HRSA in fall 2011, included both related and unrelated HCT outcomes and analyzed three years of data (reduced from five years), as recommended at the September 2010 Center-Specific Outcomes Forum. Centers review and approve these data prior to CIBMTR’s submission to HRSA. Once approved by HRSA, this and previous years’ reports are available on the C.W. Bill Young Cell Transplantation Program website (http://bloodcell.transplant.hrsa.gov).
  
  - **Statistical reports.** An updated aggregate survival analysis was completed and posted on the Program website (http://bloodcell.transplant.hrsa.gov) in early 2011.
  
  - **Scientific reports.** A report providing estimates of availability of matched products for HCT recipients, accounting for different degrees of HLA matching, adult donor recruitment, availability scenarios, cord blood inventory size, recipient age and weight, and total nucleated cell (TNC) requirements was submitted to HRSA in September 2011. Enhancements to the report include better estimation for appropriate unit size based on TNC, benchmarking against historical data, and corrections for donor attrition over time. More than 1 million data points were considered for the analysis. A manuscript for peer-reviewed publication is under development.

- **Collecting data for other therapeutic applications.** The Cellular Therapies for Regenerative Medicine (CTRM) data repository (Section 2.7) is an SCTOD requirement that tracks alternate uses of stem cells.

- **Participating in working groups**
  
  - **Cord blood data working group.** Substantial progress was made in 2011 to provide more useful outcomes reports to cord blood banks. More data fields are included and data received from centers is more complete. Tools and reference guides regarding CIBMTR reporting standards and the SCTOD, were created for banks to use in preparing their FDA-required Biologic License Applications. An electronic event reporting system was developed to systematically collect data on recipient infusion reactions and product complaints. Primary functionality was released in October, with a follow-up release that included the Adverse Event (AE) Follow-up form for unresolved events. This successful release was implemented on schedule. To date, no adverse events have been reported.

  - **Bone marrow and cord blood donation and transplantation working group.** This working group, led by NMDP Patient Services, reviews and makes content suggestions for the C.W. Bill Young Cell Transplantation website (http://bloodcell.transplant.hrsa.gov). CIBMTR provides the HCT data for this website.
2.7 CELLULAR THERAPY FOR REGENERATIVE MEDICINE (CTRM)

CTRM is the use of cells for treatment of diseases without the intent of replacing the recipient’s hematopoietic function. These therapies are not considered blood or marrow transplantation. Examples of CTRM include, but are not limited to, treatment of infectious, cardiovascular, rheumatologic, neurologic, musculoskeletal, and endocrinologic diseases with the intent to improve organ function without resucing or replacing the recipient’s bone marrow function.

As noted above, the SCTOD contract mandates the collection of data on new uses of cells found in bone marrow, peripheral blood, and umbilical cord blood for alternative therapeutic applications including regenerative medicine. CIBMTR anticipates that the expansion of the cellular therapy field with use of not only hematopoietic derived cells, but also cells derived from other tissues, will only increase. A new form and a new, non-transplant database were created in January 2011 to collect these data. Since then, 392 CTRM cases were registered in FormsNet, 287 of which have data available (73%).

The disease indications, data collection, reporting expectations, and cellular therapy center designation are all unique to this new registry/database. Through this process, CIBMTR learned important lessons for establishing a data collection model for future expansion to other types of non-HCT data points.

More information about the CTRM Registry will soon be available on the CIBMTR public website (www.cibmtr.org), and training sessions are planned during the 2012 BMT Tandem Meetings.

2.8 HCT FOR MYELODYSPLASTIC SYNDROME (MDS) FOR MEDICARE RECIPIENTS

MDS is more common in the elderly, many of whom are denied access to HCT therapy in the U.S. due to lack of Medicare insurance coverage by the Centers for Medicare and Medicaid Services (CMS). To help secure Medicare coverage for these patients, CIBMTR, NMDP, ASBMT, and other organizations partnered with CMS to launch a “Coverage with Evidence Determination” (CED) study, using data in the CIBMTR database that are collected to fulfill SCTOD requirements. The CED approach allows CMS to provide coverage for procedures and to advocate for clinical studies that inform policy decisions.

CIBMTR submitted a study plan and protocol, “Assessment of Stem Cell Transplantation in Medicare Beneficiaries with Myelodysplastic Syndrome and Related Disorders,” to CMS in September 2010. CMS approved it, and the study began on December 15, 2010. The plan will progress in several stages:

- Part 1 is the ongoing observational study to compare short-term outcomes (100-day survival) after HCT in MDS patients >65 years with those <65 years. The existing CIBMTR mechanism collects comprehensive outcomes data on all Medicare beneficiaries who receive HCT for the treatment of MDS, allowing eligible patients to receive Medicare coverage for the treatment of MDS under the CED mechanism. The accrual goal is 240 patients ≥ age 65, within 2 years. As of December 2011, 98 centers are participating and 131 patients >65 years old, 155 patients 55-64 years old, and 52 patients <54 years old are enrolled.

- Part 2 will compare outcomes of HCT recipients >65 with outcomes of similar non-HCT patients. Several groups, including CIBMTR and BMT CTN, are evaluating approaches and challenges to this clinical trial protocol.

- Part 3 will focus on collecting quality-of-life data to assess if transplant patients have similar or better quality of life than non-transplant patients.
2.9 DISEASE-BASED REGISTRY FOR SEVERE APLASTIC ANEMIA (SAA)

CIBMTR continues to investigate the implications of establishing a database for a non-transplant reportable population, as evidenced by the CTRM Registry (Section 2.7) and also the recent initiative for a disease-based registry for SAA. CIBMTR leadership partnered with other clinical researchers to define the most important questions in basic science, immunosuppressive therapy, and HCT approaches for managing this rare disease. The following goals were proposed to facilitate clinical and biologic research and improve survival in patients with SAA:

- Obtain accurate, expert diagnosis and early identification of biological risk for new patients
- Establish a population-based SAA outcomes registry in North America to collect data on patients longitudinally from diagnosis to treatment and beyond
- Develop a repository of biologic samples linked to the clinical data in the outcomes registry
- Develop innovative approaches to unrelated donor BMT that decrease GVHD
- Improve alternative donor transplantation approaches for patients without HLA-matched unrelated donors

The overall opinion was that these compelling scientific goals can be achieved by establishing a disease-based registry. CIBMTR was recommended to take the lead in developing a registry due to its established infrastructure, its expertise in collecting data for observational research, and its association with the Research Sample Repository (Section 2.4).

The Advisory Committee supports the SAA endeavor; however, there are system and operational challenges for CIBMTR, including a lack of resources. External funding would be required to successfully manage this project.
3.0 STATISTICAL RESOURCES

CIBMTR has enjoyed a positive, collaborative association with the Division of Biostatistics in the MCW Institute for Health and Society since 1985, an association that is a distinctive asset and crucial to the success of CIBMTR research. The Division of Biostatistics was formed in 1993 as one of three divisions of the Health Policy Institute (known as the Department of Population Health until June 2010). This long-standing relationship has many benefits, including:

- Ensuring the statistical integrity of CIBMTR scientific activities
- Contributing to results in articles on HCT-related statistical issues for clinical audiences
- Supporting WC members and investigators in developing scientific study protocols using CIBMTR data

The Chief Statistical Director of CIBMTR is also the Director of the Division of Biostatistics; Dr. John Klein is an internationally recognized authority in survival analysis and has published methodology research addressing issues in the analysis of post-HCT outcomes. The Division of Biostatistics is a highly-qualified group of seven PhD biostatisticians who actively participate in the development and adaptation of statistical methodologies for optimal analysis of CIBMTR and other transplant-related data.

HCT is a complex process with multiple competing risks and dramatic changes in the risks of specific events over time. CIBMTR has developed and evaluated the statistical models used in HCT research, while also acknowledging that it is important to guide the research community in appropriate application and interpretation of these sophisticated models. Therefore, CIBMTR’s statistical research development goals are two-fold: development of the models themselves and comparison of these models with existing solutions using its clinical database.

The Division of Biostatistics faculty has substantial collective experience in assessing the unique problems associated with HCT research, and the statistical techniques and methodology they develop enable rigorous analysis of these data. In their statistical oversight of CIBMTR studies, this group directly and indirectly trains and mentors the Master’s-level statisticians, positively influencing the overall integrity of CIBMTR data used in its published works. The combined expertise of these statistical partners is a unique asset that continually contributes to the strength of the CIBMTR research portfolio.

For a list of statistical methodology papers published in 2011, see Appendix H.
4.0 DATA COLLECTION, ACCRUAL, AND MANAGEMENT

Due to federal requirements, data are collected on essentially all allogeneic HCTs done in the United States; autologous transplants are reported voluntarily and are estimated to be 80%. CIBMTR also receives data on non-U.S. allogeneic and autologous HCTs from participating international centers who report voluntarily.

4.1 TRANSPLANT DATA COLLECTION

FormsNet™2.0 is CIBMTR’s web-based application for electronic submission of transplant outcomes data. CIBMTR collects data from approximately 18,000 new transplant recipients annually, as well as a continually increasing volume of follow-up data on previously reported recipients and donors. Figure 4.1 shows cumulative accession of transplants since 1970, when IBMTR (Section 1.1) began collecting these data.

Figure 4.1 Accession of patients registered with CIBMTR

Once the data pipeline through AGNIS (Section 6.3) is established, numbers of worldwide allogeneic transplants in the CIBMTR database will increase substantially. Data submission from the European Group for Blood and Marrow Transplantation (EBMT) is expected in January 2012, and full bi-directional data exchange is expected by late 2012.
Table 4.1 shows the distribution of diseases for which these transplants were performed.


<table>
<thead>
<tr>
<th>Disease</th>
<th>Allogeneic Transplants</th>
<th>Autologous Transplants</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>TED-level data</td>
<td>CRF data</td>
</tr>
<tr>
<td>Acute lymphoblastic leukemia (ALL)</td>
<td>30368</td>
<td>14484</td>
</tr>
<tr>
<td>Acute myelogenous leukemia (AML)</td>
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<tr>
<td>Chronic myelogenous leukemia (CML)</td>
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<tr>
<td>Chronic lymphocytic leukemia (CLL)</td>
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</tr>
<tr>
<td>Hodgkin disease (HD)</td>
<td>1295</td>
<td>446</td>
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<tr>
<td>Non-Hodgkin lymphoma (NHL)</td>
<td>12281</td>
<td>4805</td>
</tr>
<tr>
<td>Plasma cell disorders</td>
<td>3208</td>
<td>1318</td>
</tr>
<tr>
<td>Breast cancer</td>
<td>182</td>
<td>90</td>
</tr>
<tr>
<td>Neuroblastoma</td>
<td>188</td>
<td>93</td>
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<tr>
<td>Ovarian cancer</td>
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<td>10</td>
</tr>
<tr>
<td>Melanoma</td>
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<td>17</td>
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<tr>
<td>Lung cancer</td>
<td>10</td>
<td>2</td>
</tr>
<tr>
<td>Sarcoma (soft tissue, bone, and other)</td>
<td>37</td>
<td>11</td>
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<tr>
<td>Ewing sarcoma</td>
<td>76</td>
<td>29</td>
</tr>
<tr>
<td>Wilms tumor</td>
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<td>2</td>
</tr>
<tr>
<td>Myelodysplastic syndrome</td>
<td>16084</td>
<td>6887</td>
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<tr>
<td>Other leukemia</td>
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<td>1023</td>
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<tr>
<td>Medulloblastoma</td>
<td>7</td>
<td>1</td>
</tr>
<tr>
<td>Germ cell tumor</td>
<td>12</td>
<td>3</td>
</tr>
<tr>
<td>Brain tumor</td>
<td>5</td>
<td>4</td>
</tr>
<tr>
<td>Testicular cancer</td>
<td>11</td>
<td>5</td>
</tr>
<tr>
<td>Other malignancies</td>
<td>1116</td>
<td>583</td>
</tr>
<tr>
<td>Autoimmune diseases</td>
<td>76</td>
<td>33</td>
</tr>
<tr>
<td>Severe aplastic anemia (SAA)</td>
<td>10806</td>
<td>6019</td>
</tr>
<tr>
<td>Inherited erythrocyte abnormalities</td>
<td>6296</td>
<td>3555</td>
</tr>
<tr>
<td>Severe combined immune deficiency (SCID) &amp; other immunodeficiencies</td>
<td>4307</td>
<td>2146</td>
</tr>
<tr>
<td>Inherited disorders of metabolism</td>
<td>2100</td>
<td>1235</td>
</tr>
<tr>
<td>Histiocytic disorders</td>
<td>1019</td>
<td>554</td>
</tr>
<tr>
<td>Other non-malignancies</td>
<td>445</td>
<td>157</td>
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<tr>
<td><strong>TOTALS:</strong></td>
<td><strong>173446</strong></td>
<td><strong>82443</strong></td>
</tr>
</tbody>
</table>

* 2011 data incomplete due to reporting delay
1 TED-level data collection began in 1991 and comprehensive data collection in 1992; data for 1989-90 were collected retrospectively
2 Includes retinoblastoma, head and neck tumors, mediastinal neoplasms, GI tract tumors, pancreatic cancer, hepatobiliary, kidney and urinary tract tumors, prostate cancer, cervical, uterine cancer, vaginal cancer, and thymoma
3 Includes multiple sclerosis (n=180), systemic sclerosis (n=108), systemic lupus erythematos (n=76), rheumatoid arthritis (n=15), ITP (N=10), Crohn’s disease (n=17) and other autoimmune disorders (n=111) in registration data
4.2 LEVELS OF DATA COLLECTION

CIBMTR offers participating transplant centers two levels of data collection: a Transplant Essential Data (TED) level and a Comprehensive Report Form (CRF) level. The TED data set is an internationally accepted standard data set that contains a limited collection of data for all consecutive transplant recipients. The Comprehensive Report Form (CRF) captures additional patient, disease, and treatment-related data. TED-level data, with some additional details of donor and graft characteristics, comprise the obligatory data to be submitted to the SCTOD. Centers must comply with all applicable accreditation requirements, including Foundation for Accreditation of Cellular Therapy (FACT) in the U.S. and Joint Accreditation Committee of ISCT and EBMT (JACIE) in Europe. For a list of participating centers worldwide, see Appendices A1 and A2.

About two-thirds of CIBMTR centers submit CRF data for a subset of their patients. Data from the pre-transplant TED forms and a weighted-randomization algorithm (Section 4.3.2) are used to select cases for CRF submission. CRF-level data are critical for observational research conducted by the WCs. This research could not be conducted without these data and the analysis support of the CIBMTR Statistical Center.

Before data collection occurs, CIBMTR assigns a lifelong, unique patient identifier, or CIBMTR Recipient ID (CRID). After CRID assignment, the forms collection process varies, depending on the level of data submitted. Centers must request consent from all HCT recipients for their data to be used in research (Section 5). For more information about the forms submission process, see Appendix L.

TED-only transplant centers submit the following forms:

- CIBMTR Recipient ID Assignment (CRID) (Form 2804), due only for a recipient’s first CIBMTR-reported HCT
- Pre-TED form (Form 2400)
- Post-TED form (Form 2450) at 100 days, 6 months, and annually
- Infectious Disease Markers (Form 2004), Confirmation of HLA Typing (Form 2005), and HCT Infusion form (Form 2006) for any recipients participating in the related specimen repository and for all recipients of non-NMDP cord blood units (including autologous, related, and non-NMDP unrelated cord blood units)
- HCT Infusion form (Form 2006) for all recipients of NMDP products (marrow, PBSC, or cord blood)
- Chimerism Studies (Form 2451) for all recipients of cord blood units

CRF transplant centers must submit the following forms:

- CIBMTR Recipient ID Assignment (CRID) (Form 2804), due for a recipient’s first CIBMTR-reported HCT
- Pre-TED Form (Form 2400), followed by either the Post-TED Form (Form 2450) or a CRF
- Follow-up forms, determined by the CIBMTR’s forms selection algorithm (Section 4.3.2)
- Infectious Disease Markers (Form 2004), Confirmation of HLA Typing (Form 2005), and HCT Infusion form (Form 2006) for any recipients participating in the related specimen repository and for all recipients of non-NMDP cord blood units (including autologous, related, and non-NMDP unrelated cord blood units) assigned to the TED-track
4.0 DATA COLLECTION, ACCRUAL, AND MANAGEMENT

4.2 Data Utilization

Data from the TED series forms are used to evaluate SCTOD operations (Section 2.6), including federally required analyses such as center-specific outcomes, and to evaluate optimal registry and cord blood bank size. Though research studies typically require CRF data, some studies are possible with TED-level data.

Recipient data collected to fulfill the requirements of the SCTOD meet one of the following criteria:

- Allogeneic HCT occurs at a U.S. transplant center
- Stem cell collection (bone marrow or peripheral blood) is performed within the U.S.
- Cord blood unit is obtained from a cord blood bank within the U.S.

Recipient data collected by CIBMTR outside the context of the SCTOD include:

- Recipient receives an autologous HCT
- HCT does not occur in the U.S. and the stem cell donor resides outside the U.S.
- Cord blood unit is obtained from a cord blood bank outside of the U.S. and the transplant occurs outside of the U.S.

4.2.2 Forms Reimbursement

CIBMTR reimburses transplant centers for all completed CRFs. This reimbursement is made possible by a variety of funding sources, including federal funds. When a form is approved and a signed Data Transmission Agreement (DTA) from the center is on record, it can be reimbursed. In general, TED-level data reporting is not reimbursed.

4.3 DATA MANAGEMENT

Data management activities, including data entry and Continuous Process Improvement (CPI) standards for data quality assurance (Section 4.4.1), are shared across the two CIBMTR campuses and supervised by the Associate Scientific Director for Data Operations in Milwaukee and the Vice President of CIBMTR in Minneapolis. Participating centers are assigned a 5-digit CIBMTR center number (CCN) and submit data for autologous, related, and unrelated donor transplants either electronically via FormsNet (Section 6.1) or on paper forms to the Milwaukee or Minneapolis campus, as assigned.

Each transplant center is assigned a Clinical Research Coordinator (CRC) liaison to answer questions and provide assistance with CPI compliance and study queries. Regularly updated data management procedures and manuals are also available on the CIBMTR website (www.cibmtr.org).

4.3.1 Data Administration Mechanisms

The SecurID™ system is one of the important security features built into FormsNet and is used as a means of two-factor identity authentication. The transplant center’s primary contact must set up new
users and request new or replacement SecurID cards online through the CIBMTR website. Centers notify CIBMTR of changes to their staff via a Web-based vehicle called the Center Personnel Change Form. After the request is complete and permission is granted, the primary contact activates the new user in FormsNet.

4.3.2 Forms Selection Algorithm

As noted, a transplant center’s level of participation determines the type of data collection forms that it submits to CIBMTR. Though a center may participate as a TED-only center or a CRF center, all centers submit the pre-TED form for every patient. For an explanation of the CIBMTR forms submission process, see Appendix L.

A selection algorithm applied to the pre-TED form determines whether subsequent patient data are assigned to TED-only or CRF level tracks. The goal of the selection algorithm is to randomly select a sample of recipients for whom a CRF will be requested. The algorithm includes, but is not limited to, type of HCT (e.g., HCTs using a cord blood graft), age of the recipient, disease, graft source, and enrollment on BMT CTN studies) and is weighted to oversample data on rare diseases. The algorithm is periodically reviewed to assess the burden of data submission for transplant centers.

4.3.3 Paper Report Forms

All CIBMTR forms can be found at www.cibmtr.org under the Data Management tab. Most CIBMTR reporting centers submit electronically via FormsNet. This offers substantial benefits to CIBMTR and to centers, including improved data quality. If original signatures are required, forms can be faxed or mailed. All paper forms and attached documents are imaged and electronically stored at CIBMTR for future reference, and the original documents are destroyed using a secure process.

CIBMTR Statistical Center staff review paper forms and enter data into FormsNet. If necessary, errors are corrected by the individual center via Error Correction sheets, which are obtained and submitted in the same manner as the forms.

4.4 DATA QUALITY

4.4.1 Continuous Process Improvement (CPI)

CPI ensures timeliness and completeness of data forms submissions. Transplant centers receive CPI reports three times per year (January, May, and September), listing the number of follow-up forms that were due in the previous trimester, and the number and percentage of each submitted within the trimester. A form is not officially submitted until all errors are resolved and all applicable information is submitted and approved.

To be compliant, centers must submit at least 90% of forms due for the trimester, within the time periods listed below in Table 4.4, for all unrelated donor transplants and for related donor transplants that occurred since December 3, 2007. As of September 1, 2011, 94% of U.S. transplant centers were in compliance. This is a 24% increase from the previous year, when only 70% of U.S. centers were in compliance with CPI at the close of third quarter 2010. The few remaining non-compliant centers enter a forms-due remediation procedure. If a center does not have current institutional review board (IRB) approval information on file with CIBMTR, it will not pass CPI even if the forms submission
requirements are met. Forms-due standards for related donor transplants prior to December 3, 2007 that were reported to the IBMTR/ABMTR will be phased in over time.

<table>
<thead>
<tr>
<th>Table 4.4 CPI Form Submission</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Form and Due Date</strong></td>
</tr>
<tr>
<td>Pre-TED or Form 2000, required disease forms and/or Infection Forms</td>
</tr>
<tr>
<td>Post-TED or Form 2100, required disease forms and/or Infection Forms</td>
</tr>
<tr>
<td>Post-TED or Form 2200, required disease forms and/or Infection Forms</td>
</tr>
<tr>
<td>Post-TED or Form 2300, required disease forms and/or Infection Forms</td>
</tr>
</tbody>
</table>

In addition to the CPI recipient forms program, there are CPI regulations for donor forms. The Donor Data Management Team oversees submission of these forms from NMDP donor, collection, and apheresis centers. Donor CPI reports are generated four times per year (January, April, July, and October). To be compliant, centers must submit 100% of the forms required for that CPI period.

### 4.4.2 On-Site Data Audit Program

Currently, seven clinical research associates (CRAs) perform on-site transplant center audits, spending 1-3 days at each center reviewing original source documents using the following audit processes:

- **Audit cycle.** Each domestic and international center that has submitted data for at least 20 HCTs is audited once within the 4-year audit cycle.
- **Recipient selection and eligibility requirements.** If a center has performed more than 20 HCTs during the audit period, recipient records are randomly selected for audit.
- **Forms and data fields.** All TED and CRF data are subject to data audit, and essential data fields are audited for each recipient.
- **Methodology.** Auditors compare the data submitted to the CIBMTR database with the data in the source documents. Errors are reviewed with the data coordinator. CIBMTR auditors make all data corrections to the database and the transplant center is provided with a record of all changes made to the TED forms and/or CRFs.
- **Audit analysis and reports.** Transplant centers receive a detailed audit report evaluating the results of their audit. Centers may be required to submit a Corrective Action Plan following the audit in response to errors identified during the audit.

In 2011, 70 centers were audited. As of November 30, 2011, CIBMTR completed a complex review of 52 of these audit reports, and 80.8% of centers passed with fewer than 3% critical errors. Of the remaining centers, approximately two-thirds were undergoing their first CIBMTR audit.
In addition to its CPI and audit programs, CIBMTR monitors transplant center regulatory compliance, including submission of Data Transmission Agreements (DTAs). At year end, 98% of active U.S. centers and 73% of international centers had returned a DTA, and 97% of active U.S. centers had IRB approval in place.

4.4.3 Verification and Validation

When a form is entered into FormsNet, either by transplant center or CIBMTR staff, a series of validation checks takes place, ensuring the data are valid. This process allows centers to correct errors immediately while source documents are available. Forms undergo validation checks when entered. If a data field does not pass any of the following validation checks, an error report is generated:

- **Mandatory field validation.** Certain fields on the forms must be completed for all recipients (e.g., primary questions that lead into a box). Other fields must be completed depending on how a primary question is answered (e.g., “yes” to “developed acute GVHD” makes all acute GVHD questions mandatory). The validation process checks that all mandatory fields are completed.

- **Range validation.** The validation process checks all laboratory values, drug doses, heights and weights against established upper and lower limits.

- **Consistency between forms.** The validation process checks for consistency between data reported on the current form and related data reported on a previous form. For example, on all forms, the contact date is validated against the HCT date.

- **Consistency within a form.** The validation process also checks each form for consistency between related data reported on the same form. For example, all dates are validated against the “date of last contact.”
5.0 HUMAN SUBJECTS/HIPAA COMPLIANCE

All CIBMTR Statistical Center personnel, including administrative and data entry staff, are trained in ethical conduct of clinical research, in compliance with policies of the MCW and NMDP IRBs. All Statistical Center personnel are required to complete training through the Collaborative IRB Training Initiative (CITI) program. CITI completion certificates are on file for CIBMTR staff in administrative offices on the respective campuses, and are renewed every two (Minneapolis) to three (Milwaukee) years. New employees are required to complete this training upon hire and existing employees are required to maintain certification.

5.1 INSTITUTIONAL REVIEW BOARD APPROvals

All research supported by federal funding uses patient data that have been continuously reviewed by the MCW IRB since 1987. As of February 2011, a new IRB Authorization Agreement designates the NMDP IRB to oversee all ongoing activities using the CIBMTR Research Database. The current approval cycle is effective through July 2012.

An observational research database protocol and accompanying consent documents are updated and approved annually by the IRB for submission of research data for autologous and allogeneic recipients. The protocol describes the anticipated research uses of the CIBMTR Research Database and is accompanied by a consent form for patients and donors. In late 2007, CIBMTR established a related donor-recipient repository for research purposes that is identical to the repository for unrelated donor-recipient pairs currently maintained by the NMDP. A research protocol that covers submission of allogeneic related and unrelated recipient and donor samples, and its accompanying consent documents, were approved by the IRB and are also reviewed annually. All protocol and consent documents are provided to participating U.S. transplant centers, and centers are required to submit them to their local IRBs for review and approval.

U.S. transplant centers are required to provide written IRB-approved patient consent for research in order to submit research data for new patients. Because of CIBMTR’s role with the SCTOD and its accompanying status as a Public Health Authority (PHA) (Section 5.2), essential patient data are collected for the observational database, regardless of whether the patient provides consent. However, data from non-consenting patients are never used in research studies.

CIBMTR tracks the IRB approval/renewal process, monitors their IRB approval expiration dates, and sends timely reminders to transplant centers regarding their IRB approval. Centers are required to submit new approvals to CIBMTR. International centers are required to provide assurance to CIBMTR, via a Data Transmission Agreement, that data submission from their site is in compliance with all relevant human subject protections and privacy regulations. This approach has been approved by the MCW and NMDP IRBs.

5.2 PRIVACY RULE – HIPAA

The Health Insurance Portability and Accountability Act (HIPAA) Privacy Rule, enacted by the U.S. Congress in 1996, applies to health care providers that transmit any health information in electronic form in connection with certain standard transactions, such as health care claims. It ensures the privacy of protected health care information for all patients.
Historically, CIBMTR has maintained compliance with HIPAA by collecting a limited dataset with a Data Use Agreement with participating centers. However, with receipt of the SCTOD contract, the Office for Civil Rights and the Office of the General Counsel, U.S. Department of Health and Human Services, determined that CIBMTR meets the Privacy Rule’s definition of a PHA. As such, CIBMTR is authorized by law to collect information necessary for the SCTOD to fulfill its statutory purpose and functions. HCT centers, regardless of their status as a “covered entity” are allowed to disclose protected health information to the CIBMTR without an individual’s written consent or authorization so that the CIBMTR can fulfill its statutory obligations as a PHA. In this context, Data Use Agreements are no longer required. All data collected between April 2003 and July 2007 are maintained in compliance with the regulations under which they were collected. Therefore, data submitted by centers during this time period that were not accompanied by a Data Use Agreement are not used by CIBMTR.

5.3 GENDER AND MINORITY INCLUSION

CIBMTR rules require that participating centers register all consecutive HCT recipients. The population available for study, therefore, includes women and minorities in the same proportion as they are found in the general HCT population. No patients are excluded on the basis of race or sex. In fact, the CIBMTR database is a valuable resource for determining whether HCT recipients differ from the general population in race or sex and whether patients enrolled in clinical trials differ from the general HCT population in race, sex, or other characteristics.

5.4 INCLUSION OF CHILDREN

CIBMTR rules require that participating centers register all consecutive HCT recipients, regardless of age. Almost 18% of patients in the CIBMTR database were younger than 18 years of age at time of transplantation. Children are included in most CIBMTR studies, unless the disease of interest (e.g. multiple myeloma) does not occur in the pediatric population or the technology to be studied has substantially different issues to be addressed in children and adults (e.g. comparisons of bone marrow versus peripheral blood grafts; and bone marrow versus cord blood grafts were done separately for children and adults). Additionally, the Pediatric Cancer and Immune Deficiency/Inborn Errors of Metabolism WCs are committed to facilitating studies focused on issues specific to pediatric HCT recipients.
CIBMTR INFORMATION TECHNOLOGY SERVICES

CIBMTR information technology (CIT) services are shared between the Minneapolis and Milwaukee campuses. In 2011, CIT made several enhancements to the IT infrastructure of CIBMTR, including updates to existing data collection tools, planning and development of new tools, and progress toward supporting database users and consolidating legacy databases. Additionally, CIT began development of its Information Management Strategy, which will provide a roadmap for managing and promoting CIBMTR data resources.

CIT supports scientific research objectives by:

- Developing and maintaining the FormsNet™ product suite
  - Developing and maintaining a data pipeline from FormsNet to the research database, which is the final data repository
  - Ensuring the quality of data obtained through the pipeline by processing and validating incoming data from FormsNet
- Developing and maintaining the AGNIS toolkit for electronic exchange of data with centers and networks
- Making data in the research database available to CIBMTR staff and statisticians on both campuses, as well as external research partners
- Enabling team collaboration and information dissemination by maintaining and enhancing the Web Presence websites
- Curation of metadata and promotion of data standards

The CIBMTR research database contains HCT data for recipients and donors submitted by participating centers. Before data are added to the research database, they are validated in several steps, as shown in Figure 6.1.

Figure 6.1 Data Validation Process
6.1 FORMSNET™2.0

The FormsNet application suite is CIBMTR’s web-based application for data collection, storage, and retrieval for HCT and cellular therapy research. Key functionality includes:

- Electronic data submission from center to CIBMTR for both TED forms and CRFs, including real-time error validation and forms due listings
- Support for all aspects of prospective research, including clinical trials and survey research
- Collection of donor clearance/suitability and follow-up data to ensure donor safety
- Support for data collection, quality control, and clinical studies via the Management Reporting tool

FormsNet improvements in 2011 include:

- **Auditing support.** New auditing applications support auditing of FormsNet data against source documents at recipient and donor centers, clinical trials, and cord blood transplantation being done under an IND.
- **Monitoring support.** A Sample Tracking application was released to monitor samples from time of request to sample testing.
- **Research support.** Support for several research studies was added, including a Minimal Residual Disease study, an MDS study, and a cord blood study.
- **Release of new forms.** Form 4000 (Cellular Therapy for Regenerative Medicine), Form 2007 (cord blood not provided by NMDP), and Form 2518 (Tyrosine Kinase Inhibitors [TKI] in ALL) were released.
- **Updates to several forms.** Form 2804 (CRID creation tool for CTRM, MDS, and others), pre- and post-transplant multiple sclerosis (MS), systemic lupus erythematosus (SLE), ALL, and several other forms were updated.
- **Event reporting support.** Reporting of recipient adverse events (AEs) and follow-ups, product complaints, and cord blood product deviations was added.
- **Additional enhancements.** More than 1,500 enhancements were implemented as part of the above initiatives, plus additional data capture support and management reports.

6.2 FORMSNET™3.0

To accommodate future data collection and management needs, FormsNet will be upgraded based on an “Agile Software Development” model that successively refines software at each development stage to address functionality, usability, and end user requirements. Requirements for FormsNet3 include a more user-friendly design, improved navigation, and customization capability, as well as a new tools and forms with enhanced validation checks. Subsequent releases will include donor and clinical trials functionality. More than 1,100 requirements and enhancements, solicited from various centers and other CIBMTR stakeholders, were added to FormsNet2 in 2011. These improvements will support the upcoming release of FormsNet3.
A GROWABLE NETWORK INFORMATION SYSTEM (AGNIS)

To assist transplant centers in collecting data for internal research, patient care requirements, and reporting purposes, CIBMTR and NMDP BioInformatics created A Growable Network Information System (AGNIS). AGNIS supports secure data sharing across diverse database systems. It is an open-source Web service developed with tools from the NCI caBIG® effort and other well-established projects, such as the Globus Toolkit. AGNIS software, distributed under a public license at www.agnis.net, is available to any interested center.

AGNIS allows participating centers to collect and share data with CIBMTR and others who link to AGNIS. Data are entered once and then distributed and synchronized among databases. Data transmitted via AGNIS are stored in a metadata repository operated by the NCI Center for Biomedical Informatics and Information Technology (NCI CBIIT), known as the Cancer Data Standards Registry and Repository (caDSR). This repository is compliant with government standards for electronic data transmission.

In the caDSR, common data elements (CDEs) for FormsNet database fields are compiled into Form Builder reports, which convert CIBMTR format into caDSR format and which centers use to submit data automatically to FormsNet via AGNIS. To date, CDEs have been created for more than 9,000 FormsNet database fields, representing 99% of the data points. In addition, 24 Form Builder reports were created. Six additional forms for development and production submission were released in 2011. As of November 30, 2011, a total of 3,586 forms were submitted through AGNIS this year.

CIBMTR worked with EBMT and others to standardize data collection for TED and MED-A forms, establishing data collection benchmarks that are now used for most collaborative studies in HCT research. CIBMTR is working toward a more direct connection with the EBMT database using AGNIS, thereby decreasing the workload for European centers who currently report to both organizations. Outcomes data for cord blood recipients can also be exchanged this way. Data already collected by the EBMT, in some cases to fulfill national requirements, will be provided to CIBMTR for inclusion in the research database once the AGNIS connection is completed. Estimated completion of the first phase of this project is mid-2012.

Other AGNIS team accomplishments in 2011 include:

- **Support functionality.** Transplant centers can retrieve data submitted on TED forms in FormsNet.
- **Product development.** Several outside vendors developed and released AGNIS products in 2011.
  - Two separate vendors developed products that use AGNIS to retrieve TED forms and support additional forms; 21 centers currently use these products
  - One vendor released a product that submits and retrieves CIBMTR form data using AGNIS; currently, one form is supported with additional support forthcoming
  - Other vendors are implementing data submission and retrieval systems using AGNIS
- **IT support.** AGNIS support was provided for multiple formats.
- **Forms support.** The AGNIS team continues to support electronic submission forms. Curation of Baseline and Follow-up forms was completed (all follow-up forms were released), CDEs were defined, and individual forms were released.
6.4 RESEARCH DATABASE

The CIBMTR Research Database is an Oracle database containing all historical and current data collected on IBMTR, NMDP, or CIBMTR forms. The data are represented in a domain-based relational model, rather than the forms-based model used in FormsNet, to better support data integration across forms. The database enforces data integrity and constraints to ensure accuracy and consistency.

6.4.1 Extract, Transform, and Load (ETL) program

The Extract, Transform, and Load (ETL) program extracts FormsNet data from AGNIS, then transforms and loads the form-based, horizontal data into the domain-based model in the research database. This program also applies additional data integrity checks to locate and resolve errors.

6.4.2 SAS Retrieval

CIBMTR biostatisticians perform most analyses using third-party SAS software applications for clinical data analysis and reporting. To simplify study datasets, CIT creates SAS datasets of the most commonly analyzed data fields and most commonly used computed variables. The data are extracted from the research database and formatted to be SAS-compatible. Four different datasets are created eight times per year, as shown in Table 6.1.

<table>
<thead>
<tr>
<th>Dataset</th>
<th>Description</th>
<th>Number of Variables</th>
<th>Number of Cases</th>
</tr>
</thead>
<tbody>
<tr>
<td>TED</td>
<td>All patients and transplants</td>
<td>1,082</td>
<td>367,895</td>
</tr>
<tr>
<td>Research: Allogeneic</td>
<td>All patients on CRF track who had an allogeneic transplant and a follow-up form was submitted</td>
<td>11,762</td>
<td>94,469</td>
</tr>
<tr>
<td>Research: Autologous</td>
<td>All patients on CRF track who had an autologous transplant and a follow-up form was submitted</td>
<td>7,658</td>
<td>36,476</td>
</tr>
<tr>
<td>Corporate-sponsored studies</td>
<td>All patients in corporate-sponsored studies not funded by the NIH grant</td>
<td>11,809</td>
<td>9,371</td>
</tr>
</tbody>
</table>
6.4.3 Information Requests

CIBMTR aims to provide maximum access to the data it collects and generally responds to information requests within two days. Information requests can be submitted at www.cibmtr.org and http://bloodcell.transplant.hrsa.gov, as well as by mail, fax, and phone. These requests often come from physicians and patients seeking information about transplant outcomes and from researchers evaluating clinical trials. Table 6.2 summarizes the information requests submitted to CIBMTR.

<table>
<thead>
<tr>
<th>Table 6.2 Information Requests Received by CIBMTR</th>
</tr>
</thead>
<tbody>
<tr>
<td>Type of Organization</td>
</tr>
<tr>
<td>Physician/researcher</td>
</tr>
<tr>
<td>Pharmaceutical/Biotech Company</td>
</tr>
<tr>
<td>Patient or Relative</td>
</tr>
<tr>
<td>Patient Advocacy Group</td>
</tr>
<tr>
<td>Market Research Firm</td>
</tr>
<tr>
<td>Federal Government Agency</td>
</tr>
<tr>
<td>State Government Agency</td>
</tr>
<tr>
<td>Insurance Company</td>
</tr>
<tr>
<td>Medical Society</td>
</tr>
<tr>
<td>Student</td>
</tr>
<tr>
<td>News Media</td>
</tr>
<tr>
<td>Law Firm</td>
</tr>
<tr>
<td>Cord Blood Bank</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
</tr>
</tbody>
</table>

6.5 WEB PRESENCE

The primary goal of the CIBMTR “Web Presence” project is to optimize and enhance CIBMTR websites to provide better access to HCT information for both the scientific community and the public, to provide training and support to transplant centers who provide outcomes data to CIBMTR, to create a shared communications and networking environment for transplant centers and CIBMTR WCs, and to provide web-based access to CIBMTR HCT databases and other resources.

CIBMTR's Web Presence comprises three distinct websites, each with its own purpose and security model. The public website (www.cibmtr.org) is open to everyone, and the two Shared Partner Workspaces (Portal and Collaborative) are password-protected sites for secure information sharing.

Early development and launch of the Web Presence initiative was funded by a supplement to the NIH grant, the American Recovery and Reinvestment Act of 2009 (ARRA) award, which expired on February 28, 2011.

6.5.1 Public Website

The Public website, www.cibmtr.org, is unrestricted and provides information about CIBMTR and its research. The CIBMTR Summary Slides, provided by the Statistical Center, are posted annually on this site in a downloadable PowerPoint format. These frequently requested slides summarize outcomes and current uses of HCT and provide answers to questions posed by the HCT community.
Information from WCs, clinical trials, and publications presented on the Public website comes from the Studies, Publication, and Authors (SPA) database, which maintains data on 414 studies, 717 publications (since 1972), and 763 authors and their institutions at the time of the publication.

A bi-campus Web Advisory Team meets regularly to review the site and plan updates. Web content is assigned to appropriate subject matter experts and content leads to ensure accuracy. The 2011 annual website review was completed in December 2011.

6.5.2 Collaborative Site

The Collaborative site, https://collaborate.cibmtr.org, uses the SharePoint 2010 Collaboration program to promote cooperation among CIBMTR staff and a communication platform for specific studies and initiatives. This secure site requires a username and password and user-specific security credentials are assigned.

CIBMTR is evaluating the Collaborative site for use in a complex, prospective study involving several contributors and with a projected accrual of more than 2,000 participants. Plans include using the site for sharing protocol and consent documents, donor/recipient tracking tools, confidential committee information, data, manuscript drafts, and other relevant information. Two WCs (one small and one large) will pilot this initiative, with the expectation that it will be released to all CIBMTR WCs.

6.5.3 Portal Site

The Portal site (https://portal.cibmtr.org) includes the Global Contact Management (GCM) system, the Data Back to Center (DBtC) application, and Center Volumes Report. These custom applications provide information to CIBMTR staff and partners, including investigators, transplant centers, clinical research coordinators, statisticians and corporate partners.

The Portal site was migrated from an in-house network to the MCW network on October 27, 2011 in order to provide a high-availability site and to leverage available support from MCW. These changes required users to create new accounts. In the first 30 days, 245 transplant center users created new accounts. The following information is available on the Portal site:

- **Data Back to Center (DBtC).** This is an application that permits U.S.-based center staff (generally the Medical Director or primary data contact only) to download data (in either XML or comma-separated value format) that have been validated and processed into the CIBMTR database. These data are updated quarterly. A data dictionary is provided for each field and value in the datasets. Since May 20, 2011, 271 unique, non-CIBMTR visitors viewed 1,100 DBtC pages and downloaded data 194 times. Feedback submitted at the Fall Data Managers conference at the NMDP Council Meeting (Section 7.1) is being analyzed and will be incorporated into future releases.

- **Global Contact Manager (GCM).** This database contains information about organizations and contacts with whom CIBMTR interacts and collaborates, including transplant centers, cord blood banks and registries, committee members, authors, and others. Currently, it can be viewed by users at both CIBMTR campuses, with selected personnel at each campus given editing privileges. GCM maintains contact information on 3,916 personnel at 454 transplant centers worldwide and can accommodate the future inclusion of partners such as the Worldwide Network of Blood and Marrow Transplantation (WBMT) and World Marrow Donor Association (WMDA), in support of a larger cooperative scientific community.
• **Center Volumes Report (CVR).** This report allows centers to review and approve center volume data that is published annually to the Bone Marrow and Cord Blood Donation and Transplantation site (http://bloodcell.transplant.hrsa.gov), to which CIBMTR is required to provide data. This public website, under the HHS banner, is required by the SCTOD contract and includes the center-specific outcomes data collected by the SCTOD (Section 2.6). This year, CIBMTR expanded CVR to display and permit download of the previous two years (2009, 2008) of volume data in addition to the current year. To facilitate review, CIBMTR also added reports to show additional HCTs added since the data were last approved. To date, 181 centers have submitted approval to publish their center volume data. There were 4,005 pages viewed by 436 unique, non-CIBMTR visitors. Of those, 1,406 views were of the center volume reports.

### 6.6 DELIVERING MEANINGFUL DATA

CIBMTR recognizes the need to develop a long-term information management strategy to improve its ability to provide meaningful data in a timely manner. The primary objective of this strategy is to establish a comprehensive program for managing data across the organization. Additional goals include leveraging the research database as a strategic asset to support high-impact sophisticated analyses and enhancing data consolidation, preparation, and validation processes to allow more time for investigation and research.

CIBMTR retained a full-time Business Analyst and part-time Solution Architect in 2011, both with significant data warehousing and business intelligence experience, to lead development of this strategy. These individuals interviewed 30 stakeholders to better understand the current state of data management and to identify strengths, challenges, and opportunities. An Information Management Strategy Charter was written to summarize the team’s findings, make recommendations on best practices, identify the resources required, and provide a roadmap for how to proceed. The results of the analysis and planning for the subsequent phases were incorporated into the charter in December 2011.

To further efforts in the development and adoption of data standards, CIBMTR worked with staff from MD Anderson, NMDP, and the NCI to map curated CDEs to the Biomedical Research Integrated Domain Group (BRIDG) Model. The BRIDG Model is derived from a collaborative effort of the Clinical Data Interchange Standards Consortium (CDISC), Health Level Seven International (HL7), NCI, and the FDA. It is intended to provide a shared domain-based information exchange for protocol-driven research in the HCT community.

The BRIDG initiative involved mapping approximately 1,900 CIBMTR-curated CDEs into the BRIDG model over several years. These CDEs represent all core TED and CRF forms, as well as several disease-specific forms. The next release of the BRIDG Model, version 3.1.0, will incorporate these CDEs and is anticipated for release in February 2012. A pilot project has begun to produce a BRIDG-compatible database model. This project builds on a related effort to develop a database for the Integrated Immunological Integrated Database, which contains data from the NMDP and SCTOD and is being expanded to include infectious disease markers, HLA genetic information, and match grades.

Possible next steps include creation of a data warehouse and the adoption of standardized vocabularies and HL7 messaging standards to allow better communication with Electronic Medical Record (EMR) and other health information systems.
6.7 IT SECURITY

Physical and technical security measures at CIBMTR were substantially enhanced after late 2006 to comply with the federal security standards required by the SCTOD contract. Following an evaluation of confidentiality, integrity, and availability of information sensitivity and the impact of system compromises, the Milwaukee-based and NMDP-based information systems were designated as Moderate and High security categorizations, respectively. CIT Milwaukee was certified by the Chief Information Officer of the HRSA Office of Information Technology in December 2008 and renewed in December 2011. These security standards represent a significant elevation in information-system, security-risk management and are much more robust than the standards established by HIPAA.
7.0 MEETINGS & PRESENTATIONS

CIBMTR collaborates with scientists and clinicians to advance HCT research and outcomes by participating in national scientific meetings and conferences and publishing regular communications for clinicians, patients, and the public. A complete list of CIBMTR presentations is included in Appendix I.

7.1 MEETINGS

CIBMTR cohosts the BMT Tandem Meetings with ASBMT. The meeting was attended by 2,395 participants from 45 countries, and the agenda included 5 plenary sessions, 14 concurrent sessions, 96 oral abstracts, 2 poster sessions and 7 corporate-supported symposia. A record 650 abstracts were received from 33 countries. CIBMTR presented 7 abstracts, including 6 oral sessions and 1 poster session. Meetings of the CIBMTR Working, Advisory, Clinical Trials Advisory, and Consumer Advocacy Committees were held, as well as meetings for FACT, the BMT CTN, Pediatric Blood and Marrow Transplant Consortium, and others. New in 2011 was the Clinical Practice Forum, a session designed to address clinically-relevant topics for allied health professionals. For the upcoming 2012 BMT Tandem Meetings, 12 abstracts from CIBMTR WCs were submitted and are under review.

BMT Tandem abstract presentations are indexed and accessible online at www.cibmtr.org, as well as published in the February 2011 issue of Biology of Blood and Marrow Transplantation (Vol. 17, no. 2, Supplement 2). Audio recordings, slides, and agendas from the meetings are available on the CIBMTR website. Continuing Medical Education (CME) and Continuing Education credits are issued through MCW to physicians and allied health professionals from the United States who attend these meetings. Dr. Mary Horowitz is the CME director.

CIBMTR also hosts a booth at the ASH Annual Meeting. ASH is the world’s largest professional society concerned with the causes and treatments of blood disorders. Annually, the ASH meeting draws more than 20,000 attendees, with 5,000 abstracts submitted. This year, all 18 proposals (13 CIBMTR and 5 BMT CTN) were accepted for submission.

Table 7.1 lists CIBMTR’s presentation at the ASH Annual Meetings.

<table>
<thead>
<tr>
<th>Year</th>
<th>Submitted</th>
<th>Accepted</th>
<th>Oral</th>
<th>Poster</th>
</tr>
</thead>
<tbody>
<tr>
<td>2004</td>
<td>9</td>
<td>9</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>2005</td>
<td>10</td>
<td>9</td>
<td>5</td>
<td>5</td>
</tr>
<tr>
<td>2006</td>
<td>11</td>
<td>10</td>
<td>8</td>
<td>2</td>
</tr>
<tr>
<td>2007</td>
<td>22</td>
<td>21</td>
<td>8</td>
<td>13</td>
</tr>
<tr>
<td>2008</td>
<td>26</td>
<td>24</td>
<td>13</td>
<td>11</td>
</tr>
<tr>
<td>2009</td>
<td>20</td>
<td>20</td>
<td>14</td>
<td>6</td>
</tr>
<tr>
<td>2010</td>
<td>24</td>
<td>24</td>
<td>16</td>
<td>8</td>
</tr>
<tr>
<td>2011</td>
<td>18</td>
<td>18</td>
<td>13</td>
<td>5</td>
</tr>
</tbody>
</table>
Additional meetings included presentation of research results, educational sessions, and communication with various CIBMTR committee members, including:

- The WBMT, of which CIBMTR is a founding member. The WBMT Executive Committee meets by conference call monthly. The Board meets by teleconference three times annually and holds an in-person business meeting at either the BMT Tandem Meetings or the EBMT Meeting. During 2011, CIBMTR provided administrative and planning support for a 2-day Workshop and 1-day Scientific Symposium sponsored by the WBMT in Hanoi, Vietnam, November 10-12, 2011. The purpose of the meetings was to reach out to developing countries who are interested in launching a transplantation program or expanding an existing program. There were approximately 300 attendees from 24 countries. WBMT intends to publish conclusions and recommendations based on feedback from these meetings.

- The annual NMDP Council Meeting in November is an educational conference where members of the NMDP Network and others involved in bone marrow and umbilical cord blood transplantation can share information and ideas. The WMDA holds its annual in-person meeting here.

7.2 NEWSLETTERS

Twice each year, the CIBMTR publishes a newsletter for the HCT community. It is available in printed form, sent out via email to a large mailing list of CIBMTR partners, and posted on the www.cibmtr.org website. The primary audience is transplant center physicians and staff who participate in CIBMTR research by submitting data. Articles feature updates on CIBMTR WCs, research programs, data management and collection, and other newsworthy events in the blood and marrow transplant community. The annual Summary Slides report, included in the newsletter, is a well-regarded tool that reviews transplant-related data submitted to the CIBMTR, focusing on trends, early outcomes, and transplant numbers. This report is also available on the CIBMTR website (www.cibmtr.org).
8.0 SIGNIFICANCE

The CIBMTR has been collecting HCT outcomes data worldwide for almost 40 years, resulting in a research database with information on more than 350,000 transplant recipients. These data are freely available to investigators and others with interest in HCT and treatments for cancer and other life-threatening diseases. More than 700 peer-reviewed publications have resulted from analyses of these data. These studies have changed clinical practice and helped to improve survival and quality of life for patients undergoing this complex procedure. The organization has become a respected leader in HCT research by providing a unique resource of information and expertise to medical and scientific communities.

The CIBMTR leverages its funding to obtain support for a broad array of research programs to the scientific community in a cost-effective manner by virtue of its expert staff of physicians, statisticians, immunologists, and clinical research and information technology professionals.

CIBMTR’s statistical resources and research database facilitate new research initiatives. When the Centers for Medicare and Medicaid Services (CMS) determined that HCT for MDS in the Medicare population would be covered only in the context of a study to determine evidence of efficacy—a Coverage with Evidence Determination (CED)—CIBMTR rapidly assembled a team of experts and designed a study using its existing data collection infrastructure and IRB-approved research protocol. The study met CMS requirements for CED and was approved, thereby minimizing the gap in HCT coverage for this population. This is an excellent example of how CIBMTR can address both scientific and health policy issues in a cost-effective and efficient way.

With the consensus and support of its Advisory Committee, CIBMTR is committed to its newest initiative—collection of data on a non-transplanted patient population. The launch of the Cellular Therapy Regenerative Medicine (CTRM) form in January 2011 was the first of many planned steps towards accumulating data on patients without an HCT landmark. A follow-up study to the CMS study discussed above, which is limited to studying HCT outcomes, will collect comparison data for a cohort of patients receiving non-HCT therapy for MDS.

Finally, CIBMTR is exploring development of a registry for patients with the rare disorder, severe aplastic anemia. This project requires CIBMTR to work with new partners, expert researchers in fields involving the more novel yet heterogeneous cellular therapies, as well as those treating diseases that may not always proceed to HCT.

CIBMTR publications cover a wide range of issues, some with important implications for clinical practice and transplantation biology, such as:

- Studies comparing umbilical cord blood with adult donor transplants
- Studies evaluating the association between donor-recipient HLA-matching and outcomes of unrelated donor transplants
- Late effects of transplantation
- Comparisons with non-transplant therapy
- Rare diseases
- Immunogenetics
The complete portfolio of CIBMTR publications is available at [www.cibmtr.org](http://www.cibmtr.org). CIBMTR publications for 2011 are also listed in Appendix H. For a list of recent publications from CIBMTR contributing biostatisticians, please visit [http://www.mcw.edu/biostatistics/Faculty/Faculty/JohnPKleinPhD.htm](http://www.mcw.edu/biostatistics/Faculty/Faculty/JohnPKleinPhD.htm).

CIBMTR expanded the denominator of patients in its database, through its SCTOD activities, with benefit to new and traditional projects such as:

- Health Services Research, improved quality of life for transplant donors and recipients, and improved access to HCT
- Continued development of new biostatistical methodology for analyzing HCT
- Better access to data, public outreach and education through the CIBMTR Web Presence, to facilitate better communication among participating centers and research collaborators using web-based tools
- Collaboration with domestic and international organizations in data exchange to increase the data available worldwide to study HCT
- Continued involvement as a founding member society of the Worldwide Network for Blood and Marrow Transplantation and its activities (e.g., Workshops in Hanoi, Vietnam; November 2011, see Section 7.1) planned in collaboration with the World Health Organization

Lastly, the studies being conducted through CIBMTR’s WCs are outlined in Section 9. Some of the most important of these include:

- Description of trends in transplant activity, such as the increasing use and success of HCT in older patients and improved outcomes in specific diseases
- Determination of transplant outcomes in rare diseases, in common diseases for which transplants are rarely performed (such as low-grade NHL), and in new indications such as autoimmune diseases
- Identification of patient-related factors like age and performance score, disease-related factors like stage and duration, and treatment-related factors like optimal pre-transplant therapy and conditioning regimens, which affect transplant outcomes
- Long-term effects of HCT on patient quality of life and late complications like second cancers
- Optimal statistical models to study post-transplant events
- Relative efficacy of HLA-identical sibling, alternative allogeneic donor, and autologous transplants for specific diseases
- Relative efficacy of transplant and non-transplant treatments

Thousands of hours of voluntary efforts from physicians and scientists using these data to address important issues in HCT and other cancer treatments validate the need for this unique resource. The inclusive nature of CIBMTR WCs and data access policies make these valuable research data available to many. The Statistical Center provides meaningful access to invaluable data for physicians and, most importantly, for patients.
9.0 WORKING COMMITTEE STUDIES

CIBMTR is founded on the premise of effective collaboration of researchers and clinicians, working together to pursue relevant issues in HCT. To accomplish this goal, CIBMTR’s 19 WCs shape the observational research that leads to publications for CIBMTR. Volunteer members can propose, design, and implement studies. This section summarizes the status of each WC study.

9.1 ACUTE LEUKEMIA WORKING COMMITTEE

Co-Chair: Donald Bunjes, MD, Universitätsklinikum Ulm  
Email: donald.bunjes@uniklinik-ulm.de

Co-Chair: Steven Devine, MD, Ohio State Medical Center, James Cancer Center  
Email: steven.devine@osumc.edu

Co-Chair: John DiPersio, MD, PhD, Barnes Jewish Hospital  
Email: jdipersi@dom.wustl.edu

Scientific Director: Daniel Weisdorf, MD, University of Minnesota Medical Center, Fairview  
Email: weisd001@umn.edu

PhD Statistician: Mei-Jie Zhang, PhD, CIBMTR Milwaukee  
Email: mejie@mcw.edu

MS Statistician: Waleska Perez, MPH, CIBMTR Milwaukee  
Email: wperez@mcw.edu

PUBLISHED STUDIES

Study # LK04-02/GV01-01


Purpose: To compare MA vs. RIC vs. NMA allogeneic transplantation in patients with AML or MDS.

Patients/Methods: We compared disease status, donor, graft, and recipient characteristics with outcomes of 3,731 MA with 1,500 RIC/NMA procedures between 1997 and 2004. Five-year univariate probabilities and multivariate relative risk outcomes of relapse, transplant related mortality (TRM), disease free survival (DFS), and overall survival (OS) are reported.

Results: Adjusted OS at 5 years was 34%, 33%, and 26% for MA, RIC, and NMA transplants, respectively. By 3 years, there was no longer any difference among the groups with respect to TRM. NMA conditioning resulted in slightly inferior DFS and OS, but there was no difference in DFS and OS between RIC and MA regimens.

Conclusion: Similar outcomes with RIC/NMA versus MA regimens. The data indicate that late TRM negates any early advantage offered by RIC and NMA regimens. Prospective
trials are warranted, comparing MA and RIC regimens in patients eligible for either approach.

**Study # LK06-01**


**Purpose:** To compare the outcomes of acute myeloid leukemia (AML) patients aged 60-70 years receiving reduced-intensity allogeneic HCT in first remission (CR1) reported to the CIBMTR with outcomes in patients treated on Cancer and Leukemia Group B (CALGB) protocols.

**Patients/Methods:** Ninety-four patients reported to the CIBMTR and 96 patients were treated with induction and post-remission chemotherapy on CALGB protocols. All patients included had remained in CR1 for at least 4 months.

**Results:** HCT recipients were slightly younger than chemotherapy patients (median ages: 63 v 65 years; p<0.001), with no significant differences in the proportion with therapy-related leukemia or in different cytogenetic risk groups. Time from diagnosis to CR1 was longer for HCT recipients (median: 44 v 38 days; p=0.031). Allogeneic HCT was associated with significantly lower risk of relapse (32% v 81% at 3 years; p<0.001), higher non-relapse mortality (36% v 4% at 3 years; p<0.001), and longer leukemia-free survival (32% v 15% at 3 years; p=0.001). Although overall survival was longer for HCT recipients, this was not statistically significant (37% v 25% at 3 years; p=0.08).

**Conclusion:** RIC allogeneic HCT in CR1 AML patients aged 60-70 years reduces relapse and improves leukemia-free survival. Strategies that reduce non-relapse mortality may yield significant improvements in overall survival.

**Study # LK07-01**


**Purpose:** To determine cytogenetic risk groups for patients with AML or MDS undergoing allogeneic transplantation

**Patients/Methods:** We studied 821 adult patients reported to the CIBMTR who underwent HCT for AML in first or second CR between 1999 and 2004. We compared the ability of the six existing classifications to stratify patients by overall survival (OS). We then
defined a new schema specifically applicable to HCT patients using this patient cohort.

**Results:** Under this CIBMTR schema, inv(16) is favorable, complex karyotype (4+ abnormalities) is adverse, and all other classified abnormalities are intermediate in predicting survival after HCT (5y OS 64%, 18%, and 50%, respectively, p=0.0001). This schema stratified patients into 3 groups with similar non-relapse mortality, but significantly different incidences of relapse, overall, and leukemia-free survival. It applied to patients regardless of their disease status (CR1 or CR2), donor type (MRD or URD), or conditioning intensity (myeloablative or reduced intensity).

**Conclusion:** This transplantation-specific classification could be adopted for prognostication purposes and to stratify patients with AML and karyotypic abnormalities entering HCT clinical trials.

**PRELIMINARY RESULTS**

**Study # LK04-03**

**Title:** Comparison of autologous blood cell and HLA-identical sibling myeloablative transplants for AML in first complete remission

**Study Chairs:** Armand Keating (Princess Margaret Hospital, University of Toronto)

**MS Statistician:** Waleska Pérez (wperez@mcw.edu)

**PhD Statistician:** Gisela da Silva (tunes@ime.usp.br)

**Study Status:** Manuscript preparation

**Purpose:** To study the effect of lower treatment-related mortality after peripheral blood autologous transplantation for AML in CR1.

**Patients/Methods:** We studied 1,133 patients, 19-60 years of age, reported to the CIBMTR in CR1 from 1995-2004. Patients receiving autologous peripheral blood (PB) transplants were compared with those receiving HLA-identical myeloablative bone marrow (BM) or PB allogeneic transplants, for treatment-related mortality (TRM), relapse, leukemia-free survival, and overall survival. Outcomes were evaluated in univariate and multivariate analyses using the pseudo-value technique.

**Results:** Five-year transplant outcomes by univariate analysis for alloBM, alloPB, and autoPB, respectively, were 20% (95% CI17-24%), 26% (21-30%), 45% (38-52%) for relapse (p<0.001); 19% (16-23%), 20% (17-24%), 8% (5-12%) for TRM (p<0.001); 61% (56-65%), 54% (49-59%), 47% (40-54%) for leukemia-free survival (LFS) (p=0.13), and 64% (59-68%), 59% (54-64%), 54% (47-60%) for survival (p=0.19).

**Conclusion:** Autologous transplantation using PB may provide acceptable alternative post-remission therapy for AML CR1 in the absence of a matched sibling donor; hence, its role should be re-investigated prospectively.

**Study # LK07-03b**

**Title:** Assessment of allogeneic HCT in older patients with NHL

**Study Chairs:** Erica Warlick (University of Minnesota Medical Center, Fairview)

Brian McClune (University of Minnesota Medical Center, Fairview)

**MS Statistician:** Tanya Pedersen (tpederse@nmdp.org)

**PhD Statistician:** Kwang Woo Ahn (kwooahn@mcw.edu)
Study Status: Manuscript preparation
Purpose: To analyze transplant outcomes for older patients with CML in the era of Tyrosine Kinase inhibitors
Patients/Methods: We analyzed the post-HCT outcomes of CML patients aged 40 and older undergoing RIC/NMA HCT in 2001-2007. Outcomes were compared between age cohorts of 40-49, 50-59, and >60 years. Overall survival (OS), Day +100 acute graft-versus-host disease (aGVHD) grades II-IV, chronic (cGVHD), transplant-related mortality (TRM), relapse, and disease-free survival (DFS) were analyzed.
Results: A total of 306 CML patients underwent HCT at 125 centers: 38% aged 40-49; 39% aged 50-59; and 23% aged >60. At HCT, 72% of patients age 40-49 were in CP1 versus 31% >60. Three-year OS (54%, 52%, and 41%) and cGVHD (58%, 51%, and 43%), Day +100 grade II-IV acute GVHD (26%, 32%, 32%), and 1-year TRM (18%, 20%, 13%) were similar in all age cohorts (age 40-49, 50-59, and >60 respectively). Three-year relapse incidence increased (36%, 43%, 66%) and DFS (35%, 32%, 16%) decreased with age. Analysis of CP1 patients only showed similar relapse and DFS across age cohorts.
Conclusion: These data indicate that HCT is safe in older patients with CML. Relapse increased in patients receiving NMA conditioning and in those aged 60 and above, most of whom had advanced disease. However, for HCT during CP1, relapse risks and DFS were similar, regardless of age. Allogeneic HCT using RIC conditioning for older patients with CP1 CML can control relapse with acceptable toxicity and survival.

Study # LK07-03b
Title: Assessment of allogeneic HCT in older patients with NHL
Study Chairs: Brian McClune (University of Minnesota Medical Center, Fairview)
Erica Warlick (University of Minnesota Medical Center, Fairview)
MS Statistician: Tanya Pedersen (tpederse@nmdp.org)
PhD Statistician: Kwang Woo Ahn (kwooahn@mcw.edu)
Study Status: Manuscript preparation
Purpose: To study age as a predictor of outcome, we analyzed 1,248 patients receiving RIC HCT from 2001-2007.
Patients/Methods: We analyzed transplant-related mortality (TRM), engraftment, acute and chronic graft-versus-host disease (GVHD), relapse/progression, and progression-free (PFS) and overall survival (OS). Patients were stratified into three age cohorts: 40-54, 55-64, and ≥65 years.
Results: Univariate analysis demonstrated no statistically significant differences in the incidence of relapse/progression or GVHD across age cohorts. TRM at 1 year was lower for the youngest group, but TRM was similar in the two older cohorts. PFS and OS were inferior in the two older cohorts, but no differences between those 55-64 and ≥65 were noted. Multivariate analysis revealed no independently significant impact of age on the incidence of GVHD or on relapse/progression (p=0.06). Older age (>55 years), Karnofsky performance status, and HLA match disparity adversely impacted TRM, PFS, and OS. Disease sensitivity at HCT significantly worsened TRM, relapse/progression, PFS, and OS.
Conclusion: We conclude that patients over age 55 receiving RIC HCT for NHL have modestly worse outcomes that are likely influenced by more aggressive disease, KPS at HCT, and HLA match disparity.

Study # LK08-01
Title: Using landmark analysis to provide updated relapse and leukemia-free or overall survival estimates to patients
Study Chairs: Stephanie Lee (Fred Hutchinson Cancer Research Center)
              Paul Martin (Fred Hutchinson Cancer Research Center)
MS Statistician: JohnBosco Umejiego (jumejieg@nmdp.org)
PhD Statistician: Ted Gooley (tgooley@fhcrc.org)
Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To assess leukemia-free survival (LFS) estimates as patients reach post-transplant milestones
Patients/Methods: Landmark analyses were used. Univariate then multivariate analysis with stepwise backwards selection (p<0.01) identified independent predictors. Analyses were based on Poisson regression with additive risk structure.

Results: A population of 3,339 AML and 1,434 ALL adult patients receiving myeloablative conditioning from 1990-2005 were included. For AML, relapsed/refractory disease and chronic GVHD remained significant risk factors for subsequent LFS through at least 4 years post-transplant. For ALL, only chronic GVHD remained a significant predictor of LFS in the second and subsequent years. Age, donor type, prior acute GVHD, CMV serostatus, and patient race were not significant predictors of LFS in 1-year DFS.

Conclusion: Many factors predictive of LFS early after HCT lose their impact after patients reach 2-year DFS. However, people with chronic GVHD have a lower LFS than those without chronic GVHD, up to 6 years post-HCT. Relapsed/refractory AML also remains an adverse prognostic factor even in 5-year DFS. Using this method, estimates for subsequent LFS can be derived for individual patients or populations.

Study # LK08-02
Title: A decision analysis of reduced intensity conditioning allogeneic HCT for older patients with de-novo MDS
Study Chairs: John Koreth (Dana Farber Cancer Institute - Adults)
              Corey Cutler (Dana Farber Cancer Institute - Pediatrics)
MS Statistician: Waleska Perez (wperez@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To define the benefits of RIC transplantation in older MDS patients
Patients/Methods: Markov modeling of de-novo MDS patients aged 60-70 years to evaluate life expectancy after early RIC transplantation versus non-transplantation approaches, with adjustments for quality-of-life (QoL). Survival evaluated for 92 patients after RIC transplantation, stratified by IPSS risk versus best supportive care for 183 non-anemic low/intermediate-1 IPSS patients; hematopoietic growth factors for 78
anemic low/intermediate-1 IPSS patients; and hypomethylating agents for 160 intermediate-2/high IPSS risk patients.

Results:
For low/intermediate-1 IPSS, early RIC transplantation impaired life expectancy. QoL adjustment narrowed the gap in quality-adjusted life expectancy (QALE), but sensitivity analysis did not support RIC transplantation as the preferred strategy.

For intermediate-2/high IPSS, early RIC transplantation improved life expectancy. However, benefit was apparent only after modeling survival beyond 5 years. QoL adjusted benefit was apparent both at 5 years and beyond. Sensitivity analysis did not change conclusion of QALE benefit.

Conclusion:
For intermediate-2/high IPSS risk, early RIC transplantation offers a life expectancy benefit, with a quality-adjusted survival benefit detectable earlier.

Study # R02-05
Title: Unrelated donor stem cell transplantation in AML and ALL patients who failed an autologous transplant

Study Chairs: James Foran (Mayo Clinic Jacksonville/St. Luke’s Hospital)
Steven Pavletic (NIH-NCI Experimental Transplantation and Immunology Branch)

MS Statistician: Waleska Perez (wperez@mcw.edu)
PhD Statistician: Brent Logan (blogan@mcw.edu)

Study Status: Manuscript preparation

Purpose: To determine the outcome of patients who received an unrelated donor (URD) HCT after failing a previous autologous HCT

Patients/Methods: Patients who received a second URD bone marrow or peripheral blood HCT for AML or ALL after a failed prior autologous transplant for relapse or persistent disease between 1995 and 2005. Multivariate analysis performed for AML patients; univariate analysis for ALL due to small sample size.

Results: ALL patients had an overall survival (OS) of 11% at 3 years (95%CI, 4-20). Donor-recipient cytomegalovirus status -/-(relative risk [RR] 0.64, 0.44-0.94, p<0.001), Karnofsky score >=90 (relative risk (RR) 0.62, 0.47-0.82, p<0.001), and RIC/non-myeloablative HCT (RR 0.62, 0.47-0.82, p<0.001) were associated with better leukemia-free and OS for the AML patients. While there was a significant negative effect of acute GVHD on OS (RR 1.92, 95%CI: 1.36-2.71, p<0.001) and treatment-related mortality (TRM) (RR 2.23, 95% confidence interval (CI): 1.43-3.48 p<0.001), there was no effect of chronic GVHD on OS or relapse.

Conclusion: Second URD allogeneic HCT is feasible after failed autologous HCT for AML patients. Excessive TRM and low OS in ALL patients suggest that alternative strategies should be considered in this setting.
## STUDIES IN PROGRESS

**Study # LK04-01**  
**Title:** Comparison of autologous and allogeneic HCT for patients with acute promyelocytic leukemia in second complete remission  
**Study Chairs:** Morel Rubinger (CancerCare Manitoba/University of Manitoba)  
Martin Tallman (Memorial Sloan Kettering Cancer Center)  
**MS Statistician:** Waleska Pérez (wperez@mcw.edu)  
**PhD Statistician:** TBD  
**Study Status:** Data File Preparation

**Study # LK07-02**  
**Title:** CIBMTR scoring system to predict outcomes after allogeneic transplantation for AML  
**Study Chair:** Jorge Sierra (Hospital de la Santa Creu i Sant Pau, Barcelona)  
**MS Statistician:** Zhenhuan (Kenny) Hu (zhu@mcw.edu)  
**PhD Statistician:** TBD  
**Study Status:** Data file preparation

**Study # LK09-02**  
**Title:** Impact of monosomal karyotype in the outcomes of HCT for AML and MDS  
**Study Chairs:** Marcelo Pasquini (CIBMTR Milwaukee)  
Minoo Battiwalla (National Heart Lung and Blood Institute - NIH)  
**MS Statistician:** Zhenhuan (Kenny) Hu (zhu@mcw.edu)  
**PhD Statistician:** TBD  
**Study Status:** Protocol development

**Study # LK10-02**  
**Title:** Effect of pre-transplant consolidation chemotherapy on outcomes of reduced-intensity conditioning allogeneic transplant for adults with AML in first complete remission  
**Study Chairs:** Erica Warlick (University of Minnesota Medical Center, Fairview)  
Mark Litzow (Mayo Clinic Rochester)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Protocol development

**Study # LK10-03**  
**Title:** The outcomes of adults with Philadelphia-positive ALL comparing reduced-intensity conditioning and myeloablative conditioning allogeneic transplants  
**Study Chair:** Veronika Bachanova (University of Minnesota Medical Center, Fairview)  
**MS Statistician:** Waleska Pérez (wperez@mcw.edu)  
**PhD Statistician:** TBD  
**Study Status:** Supplemental form/data collection
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<th>Study #</th>
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<td>LK11-01</td>
<td>Impact of extramedullary disease on the outcome of allogeneic HCT in AML</td>
<td>Sagun Goyal (Barnes Jewish Hospital) Geoffrey Uy (Barnes Jewish Hospital)</td>
<td>TBD</td>
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<td>LK11-02</td>
<td>Development of a prognostic scoring system to predict relapse of ALL after allogeneic HCT</td>
<td>Michael Bishop (Medical College of Wisconsin) David Porter (Abramson Cancer Center University of Pennsylvania Medical Center)</td>
<td>TBD</td>
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<td>R02-09</td>
<td>Evaluation of donor leukocyte infusions to treat relapsed hematologic malignancies after related and unrelated donor myeloablative allogeneic HCT</td>
<td>Alison Loren (Abramson Cancer Center University of Pennsylvania Medical Center)</td>
<td>Zhiwei Wang (<a href="mailto:zwang@mcw.edu">zwang@mcw.edu</a>)</td>
<td>TBD</td>
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### PLANNED STUDIES

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<td>LK08-04/IB08-05</td>
<td>Evaluation of lymphotoxin alpha alleles in relation to relapse in AML and CML</td>
<td>Phillip Posch (Georgetown University Hospital)</td>
<td>TBD</td>
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9.2 AUTOIMMUNE DISEASES WORKING COMMITTEE

Co-Chair: Paolo Muraro, MD, PhD, Imperial College / Hammersmith Hospital  
Email: p.muraro@imperial.ac.uk
Co-Chair: Steven Pavletic, MD, NIH-NCI Experimental Transplantation  
and Immunology Branch)  
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Scientific Director: Marcelo Pasquini, MD, MS, CIBMTR Milwaukee  
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PhD Statistician: Kwang Woo Ahn, PhD, CIBMTR Milwaukee  
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MS Statistician: Xiaobo (Tony) Zhong, MS, CIBMTR Milwaukee  
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PRELIMINARY RESULTS

Study # AI06-03
Title: Overview of HCT for autoimmune diseases performed in North America and South America and reported to the CIBMTR
Study Chair: Richard Nash (Fred Hutchinson Cancer Research Center)  
Peter McSweeney (University of Colorado Hospital)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: Kwang Ahn (kwooahn@mcw.edu)
Study Status: Submitted
Purpose: To assess HCT as a treatment option for autoimmune diseases
Patients/Methods: From 1996 to 2008, 340 patients with AID who underwent HCT were reported to the CIBMTR from 74 North American and South American transplant centers.
Study Summary: The most common disease indications for HCT in AID are multiple sclerosis (MS), systemic sclerosis, and systemic lupus erythematosus. The median age at transplantation is 28 and 40 years for allogeneic and autologous HCT, respectively. Cyclophosphamide-containing regimens either with radiation or antilymphocyte globulin (ATG) are the most common conditioning regimens. Among patients with MS, the mean Expanded Disability Status Scale score was 6.0, and the majority of patients presented with secondary progressive course. Overall survival rates after autologous HCT were 90% and 86%, and after allogeneic HCT were 75% and 52% at 1 and 3 years after transplantation. Fifty patients died, and the most common causes of death were related to AID, infection, and organ failure.
Conclusion: According to data reported to the registry, the activity of transplantation for AID is increasing in these regions. Autologous HCT is a treatment option for AID, however selecting patients in the peak inflammatory phase of the disease prior to end organ failure would maximize the effect of transplantation for these diseases.
STUDIES IN PROGRESS

Study # AI05-02
Title: The effect of allogeneic HCT on the activity and progression of multiple sclerosis
Study Chairs: Richard Nash (Fred Hutchinson Cancer Research Center)
MS Statistician: Marcelo Pasquini (mpasquin@mcw.edu)
PhD Statistician: Brent Logan (blogan@mcw.edu)
Study Status: Analysis in progress

Study # AI06-01
Title: HCT for autoimmune cytopenias
Study Chairs: Harold Atkins (Ottawa General Hospital)
Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Analysis in progress

Study # AI06-02
Title: Effect of allogeneic HCT on systemic lupus erythematosus
Study Chairs: Richard Nash (Fred Hutchinson Cancer Research Center)
Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # AI09-01
Title: Long-term outcomes after autologous HCT for severe multiple sclerosis
Study Chairs: Paolo Muraro (Imperial College / Hammersmith Hospital)
Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # AI11-01
Title: Identification of HLA functional motifs associated with severe Systemic Sclerosis and sclerotic-GVHD: the shared epitopes of fibrosis
Study Chairs: Francesco Del Galdo (St. James's University Hospital)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
9.3 CELLULAR THERAPIES WORKING COMMITTEE

Co-Chair: Mitchell Cairo, MD, New York Medical College
Email: mitchell_cairo@nymc.edu

Co-Chair: Helen Heslop, MD, Baylor College of Medicine
Email: hheslop@bcm.tmc.edu

Co-Chair: Armand Keating, MD, Princess Margaret Hospital
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Scientific Director: Marcelo Pasquini, MD, MS, CIBMTR Milwaukee
Email: mpasquin@mcw.edu

PhD Statistician: John Klein, PhD, CIBMTR Milwaukee
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MS Statistician: Zhenhuan (Kenny) Hu, MS, CIBMTR Milwaukee
Email: zhu@mcw.edu

Sandra Tomany-Korman, MS, CIBMTR Milwaukee
Email: skorman@mcw.edu

STUDIES IN PROGRESS

Study # CT09-01
Title: Follow-up of subjects receiving genetically modified cell products post-transplant
Study Chair: Helen Heslop (Baylor College of Medicine Center for Cell and Gene Therapy)
MS Statistician: Zhenhuan (Kenny) Hu (zhu@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Data collection

Study # CT09-02
Title: Cellular therapy for regenerative medicine: 2008 activity survey in United States and Canada
Study Chair: Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Zhenhuan (Kenny) Hu (zhu@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Protocol development

Study # CT10-01
Title: Donor leukocyte infusion versus second allogeneic hematopoietic cell transplantation (HCT) for disease relapse after first allogeneic HCT
Study Chair: Noelle Frey (Abramson Cancer Center University of Pennsylvania Medical Center)
Alison Loren (Abramson Cancer Center University of Pennsylvania Medical Center)
David Porter (Abramson Cancer Center University of Pennsylvania Medical Center)
MS Statistician: Zhenhuan (Kenny) Hu (zhu@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Protocol development
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<tr>
<td><strong>Title:</strong></td>
<td>Follow-up of subjects receiving ex vivo expanded cord blood and mesenchymal stem cell products</td>
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| **Study Chairs:** | Elizabeth Shpall (MD Anderson Cancer Center)  
Catherine Bollard (Baylor College of Medicine Center for Cell and Gene Therapy) |
| **MS Statistician:** | TBD |
| **PhD Statistician:** | TBD |
| **Study Status:** | Deferred |


**9.4 CHRONIC LEUKEMIA WORKING COMMITTEE**

Co-Chair: Jorge Cortes, MD, MD Anderson Cancer Center  
Email: jcortes@mdanderson.org

Co-Chair: Matt Kalaycio, MD, Cleveland Clinic Foundation  
Email: kalaycm@ccf.org

Co-Chair: Richard Maziarz, MD, Oregon Health and Science University  
Email: maziarzr@ohsu.edu

Scientific Director: Wael Saber, MD, CIBMTR Milwaukee  
Email: wsaber@mcw.edu

PhD Statistician: Kwang Woo Ahn, PhD, CIBMTR Milwaukee  
Email: kwooahn@mcw.edu

PhD Statistician: Mei-Jie Zhang, PhD, CIBMTR Milwaukee  
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MS Statistician: Xiaochun Zhu, MS, CIBMTR Milwaukee  
Email: xzhu@mcw.edu

**PUBLISHED STUDIES**

**Study # CK03-01b**


**Purpose:** To assess the impact of pre-transplant imatinib mesylate (IM) on outcomes of allogeneic HCT in patients in advanced phases of CML

**Patients/Methods:** The cohort included 449 patients in second chronic phase (CP2, n=184), accelerated phase (AP, n=185), and blast phase (BP, n=80) CML who received HLA-identical sibling (27%), related (3%), and matched or mismatched unrelated donor (70%), peripheral blood (47%), or bone marrow (53%) HCT after myeloablative (78%) or non-myeloablative (22%) conditioning. Fifty-two percent of patients in CP2, 49% in AP, and 46% in BP received IM for a median of 7 (1-60), 11 (1-54), and 8 (1-36) months, respectively, prior to a planned transplant (43%) or transplantation for IM intolerance (6%), or failure (51%). Endpoints included overall survival, leukemia-free survival, transplant-related mortality, relapse, and acute and chronic GVHD.

**Results:** Pre-transplant IM was not associated with transplant outcomes. Traditional prognostic factors such as time from diagnosis to HCT, degree of HLA matching, and others were associated with outcomes. CP2 and AP patients had similar outcomes, while BP patients did very poorly.

**Conclusion:** Conventional prognostic indicators remain the major determinants of transplant outcomes in advanced phase CML. The administration of IM prior to HCT was not associated with improved outcomes in patients transplanted for advanced phase CML.
PRELIMINARY RESULTS

Study # CK06-01
Title: Outcomes after allogeneic HCT for polycythemia vera and essential thrombocythemia
Study Chair: Karen Ballen (Massachusetts General Hospital)
PhD Statistician: Kwang Ahn (kwooahn@mcw.edu)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
Study Status: Manuscript preparation
Purpose: To determine overall and disease-free survival, hematopoietic recovery, acute and chronic GVHD, and transplant-related mortality after allogeneic stem cell transplantation in patients with polycythemia vera or essential thrombocythemia
Patients/Methods: From 1990 to 2007, 75 patients with ET (median age 49 years) and 42 patients with PV (median age 53 years) were transplanted at the Fred Hutchinson Cancer Research Center (n=43) or at other centers reporting to the CIBMTR (n=74). Thirty-eight percent of patients had splenomegaly and 28% had a prior splenectomy. The majority of patients (69% for ET and 67% for PV) received a high-intensity ablative conditioning regimen.
Results: Neutrophil and platelet engraftment at 28 days was 88% and 59% for ET patients and 90% and 65% for PV patients, respectively. Acute GVHD grades II-IV occurred in 57% and 50% of ET and PV patients, respectively. The 1-year treatment related mortality (TRM) was 27% for ET and 22% for PV. The 5-year cumulative relapse incidence was 13% for ET and 30% for PV. Five-year overall survival/progression-free survival was 55%/47% and 71%/48% for ET and PV, respectively. Neutrophil and platelet engraftments at day 28 were superior in the patients with normal spleen size and splenectomized patients (p=0.022 and 0.019, respectively) compared to patients with splenomegaly, but there was no difference in TRM, overall survival, or progression-free survival among these groups. Transformation to myelofibrosis (MF) did not affect 28-day engraftment rate or TRM.
Conclusion: More than 45% of patients transplanted for ET and PV enjoy long-term progression free survival; allogeneic HCT offers potentially curative therapy in selected patients with ET and PV.

STUDIES IN PROGRESS

Study # CK06-03/CK07-01
Title: Comparison of conventional myeloablative versus non-myeloablative or reduced-intensity conditioning allogeneic HCT for CLL/small lymphocytic lymphoma
Study Chair: Jose Leis (Mayo Clinic Arizona and Phoenix Children's Hospital)
Mitchell Sabloff (The Ottawa Hospital Blood & Marrow Transplant Program)
Ronald Sobecks (Cleveland Clinic Foundation)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: Kwang Ahn (kwooahn@mcw.edu)
Study Status: Analysis in progress
Study # CK02-01
Title: Comparison of results using busulfan/cyclophosphamide compared to total body irradiation/cyclophosphamide as preparation for allogeneic transplantation in CML and AML
Study Chair: Edward Copelan (Cleveland Clinic Foundation)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # CK06-04
Title: Decision analysis of allogeneic HCT for myelofibrosis: comparison of allogeneic HCT and non-transplantation therapies for myelofibrosis
Study Chairs: Karen Ballen (Massachusetts General Hospital)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # CK08-02
Title: Results of allogeneic HCT in patients with hairy cell leukemia
Study Chairs: Steven Z. Pavletic (NIH-NCI Experimental Transplantation & Immunology)
Robert J. Kreitman (National Cancer Institute)
MS Statistician: Wael Saber (wsaber@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # CK09-01
Title: A comparison of outcomes of minimal intensity and reduced-intensity conditioning regimens for patients with myelofibrosis
Study Chairs: Vikas Gupta (Princess Margaret Hospital)
Adriana Malone (Mount Sinai Medical Center)
MS Statistician: Wael Saber (wsaber@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # CK11-01
Title: Outcomes of allogeneic HCT for chronic myelomonocytic leukemia
Study Chairs: Hien Duong (Cleveland Clinic Foundation)
Mojtaba Akhtari (The Nebraska Medical Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received
Study # CK11-02
Title: Development of a prognostic scoring system to predict relapse of myelodysplastic syndrome after allogeneic HCT
Study Chairs: Brian Shaffer (UTMO da Bahia do Hospital Portugués)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

PLANNED STUDIES

Study # CK08-01
Title: Evaluation of the outcomes of allogeneic HCT for CML in patients with resistance to imatinib or second-generation tyrosine kinase inhibitors either associated with ABL kinase domain mutations or not associated with mutations
Study Chair: Jeffrey Szer (Royal Melbourne Hospital City Campus)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred

Study # CK10-01
Title: An updated assessment of the impact of prior imatinib mesylate on the outcomes of HCT for patients with CML in the era of tyrosine kinase inhibitors
Study Chairs: Richard T. Maziarz (Oregon Health and Science University)
Michael J. Mauro (Oregon Health and Science University)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred

Study # CK10-02
Title: The impact of treatment with second-generation tyrosine kinase inhibitors on the outcomes of HCT for patients with CML
Study Chairs: Richard T. Maziarz (Oregon Health and Science University)
Jeffrey Szer (Royal Melbourne Hospital City Campus)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred
9.5 DONOR HEALTH AND SAFETY WORKING COMMITTEE

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Co-Chair: Paul O'Donnell, MD, PhD, Fred Hutchinson Cancer Research Center  
Email: podonnell@fhcrc.org
Co-Chair: David Stroncek, MD, NIH National Heart Lung and Blood Institute  
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PhD Statistician: Brent Logan, PhD, CIBMTR Milwaukee  
Email: blogan@mcw.edu
MS Statistician: Tanya Pedersen, MPH, CIBMTR Minneapolis  
Email: tpederse@nmdp.org

PRELIMINARY RESULTS

Study # DS09-05
Title: Peripheral blood stem cell (PBSC) versus bone marrow (BM) donor toxicities
Study Chair: Michael Pulsipher (Primary Children’s Medical Center)
MS Statistician: Pintip Chitphakdithai (pchitpha@nmdp.org)
PhD Statistician: Brent Logan (blogan@mcw.edu)
Study Status: Manuscript preparation
Purpose: To compare of contiguous PBSC vs. BM donation experiences
Patients/methods: We report a prospective study of 2,726 BM and 6,768 PBSC donors who underwent collection from 2004-2009. Pain and CTC toxicities were assessed at baseline, during G-CSF administration, on the day of collection, within 48 hours of donation, and weekly until full recovery.

Results: Peak levels of pain and CTC toxicities did not differ between the two donation processes for most donors. Among obese donors, PBSC donors were at increased risk of grade 2-4 pain as well as grade 2-4 CTC toxicities during the peri-collection period. In contrast, BM donors were more likely to experience grade 2-4 toxicities at 1 week and pain at 1 week and 1 month after the procedure. BM donors experienced slower recovery, with 3% still not fully recovered at 24 weeks, while 100% of PBSC donors had recovered. Other factors associated with toxicity included obesity, increasing age, and female gender.

Conclusion: This study provides extensive detail regarding individualized risk patterns of PBSC vs. BM donation toxicity, suggesting donor profiles that can be targeted with interventions to minimize toxicity.
### STUDIES IN PROGRESS

**Study # DS05-02**
- **Title:** RDSafe: A multi-institutional study of HCT donor safety and quality of life
- **Study Chair:** Michael Pulsipher (Primary Children’s Medical Center)
- **MS Statistician:** Tanya Pedersen (tpederse@nmdp.org)
- **PhD Statistician:** Brent Logan (blogan@mcw.edu)
- **Study Status:** Supplemental form/data collection

**Study # DS08-01**
- **Title:** Abnormal cytogenetics in donor-derived stem cells after allogeneic HCT
- **Study Chair:** Noelle Frey (Abramson Cancer Center University of Pennsylvania Medical Center)
- **MS Statistician:** Tanya Pedersen (tpederse@nmdp.org)
- **PhD Statistician:** TBD
- **Study Status:** Supplemental form/data collection

**Study # DS09-03**
- **Title:** Effects of multiple donations on marrow/PBSC donors
- **Study Chair:** David F. Stroncek (National Heart Lung & Blood Institute)
- **MS Statistician:** Tanya Pedersen (tpederse@nmdp.org)
- **PhD Statistician:** Brent Logan (blogan@mcw.edu)
- **Study Status:** Protocol development

**Study # DS09-04**
- **Title:** Race/socioeconomic status and donor center size on BM/PBSC donor experiences
- **Study Chair:** Michael Pulsipher (Primary Children’s Medical Center)
- **MS Statistician:** Tanya Pedersen (tpederse@nmdp.org)
- **PhD Statistician:** Brent Logan (blogan@mcw.edu)
- **Study Status:** Protocol development

**Study # DS10-01**
- **Title:** Effect of demographics on peripheral blood CD34+ counts and CD34+ cell yields in donors undergoing large volume leukapheresis
- **Study Chairs:** John Wingard (Shands HealthCare/University of Florida; LifeSouth Community Blood Centers)
  - Jack Hsu (Shands HealthCare & University of Florida)
- **MS Statistician:** TBD
- **PhD Statistician:** TBD
- **Study Status:** Protocol development

### PLANNED STUDIES

**Study # DS09-02**
- **Title:** Monoclonal B-cell lymphocytosis in matched unrelated donor HCT
- **Study Chair:** Matthew Seftel (CancerCare Manitoba/University of Manitoba)
- **MS Statistician:** TBD
- **PhD Statistician:** TBD
- **Study Status:** Deferred
9.6 GRAFT SOURCES & MANIPULATION WORKING COMMITTEE

Co-Chair: Richard Champlin, MD, MD Anderson Cancer Center
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Co-Chair: Daniel Fowler, MD, NIH-NCI Experimental Transplantation & Immunology Branch
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Co-Chair: Mary Laughlin, MD, University of Virginia Health System
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Scientific Director: Mary Eapen, MBBS, MS, CIBMTR Milwaukee
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PUBLISHED STUDIES

Study # GS05-02

Purpose: To compare outcomes of SAA patients receiving G-CSF stimulated bone marrow graft vs. unstimulated bone marrow vs. G-CSF stimulated peripheral blood stem cell graft

Patients/Methods: Seventy-eight G-CSF simulated bone marrow (G-BM), 547 unstimulated BM (BM), and 134 PBSC transplants from HLA-identical sibling donors for SAA occurred from 1997-2003.

Results: Hematologic recoveries were similar. Rates of grades II-IV acute GVHD were higher for PBSC (p=0.011 and <0.0001 for G-BM and BM). G-BM had similar rates of grades III-IV acute GVHD (p=0.54 and 0.32, to BM and PBSC). Chronic GVHD was higher in PBSC compared to G-BM (p<0.0001). BM had a marginally significant lower rate of overall mortality compared to G-BM (p=0.0502).

Conclusion: G-BM and BM have similar risks of GVHD. PBSC should be avoided in patients with aplastic anemia. A larger prospective study is needed to evaluate if cell dose offered by G-BM compared to BM may be of benefit to patients with high-risk SAA.
Study # GS06-01b


Purpose: To analyze donor characteristics associated with outcome in unrelated donor reduced-intensity conditioning (RIC) transplants. Of particular interest were H-Y effects; e.g., more GVHD if a female donor was used for a male recipient and more graft failure if a male donor was used for female recipients.

Patients/Methods: RIC HCT for malignancy were evaluated for 715 patients who received a transplant from a matched unrelated donor.

Results: Graft failure rates were higher with BM grafts vs. PB grafts. The only donor characteristic associated with survival was HLA disparity. Donor age, parity, donor-recipient sex match, and cytomegalovirus sero-status were not associated with engraftment, risks of acute or chronic GVHD, or survival.

Conclusion: We failed to demonstrate any impact of donor characteristics, other than HLA-matching, on outcome of unrelated RIC transplantation for malignancy.

Study # GS06-02


Purpose: To evaluate the relative importance of matching at the HLA C locus for unrelated umbilical-cord blood transplantation (the selection algorithm for umbilical-cord blood units generally considers intermediate resolution HLA typing at A and B and allele-level typing at DRB1)

Patients/Methods: We used Cox regression to assess retrospectively the effect of donor-recipient HLA matching on outcomes of single umbilical-cord blood transplantations for leukemia and myelodysplastic syndrome. Our primary endpoint was transplant-related mortality. HLA typing was done with molecular techniques with a minimum of intermediate resolution for HLA A, B, and C, and at the allele-level for DRB1.

Results: The median age of our study population was 10 years (range <1—62) and 552 (69%) of 803 patients were aged 16 years or younger at transplantation. Compared with transplantations matched at HLA A, B, C, and DRB1 (n=69), transplant-related mortality risk was higher after transplantations matched at HLA A, B, and DRB1 and mismatched at HLA C (n=23; HR 3.97, 95% CI 1.27—12.40; p=0.018).
Transplant-related mortality risk was also higher after transplantations with a single mismatch at HLA A, B, or DRB1 and mismatched at HLA C (n=234; 1.70, 1.06—2.74; p=0.029) compared with transplantations matched at HLA C with a single mismatch at HLA A, B, or DRB1 (n=127). Assessing the overall effect of HLA disparity on transplant-related mortality, risks were higher with units mismatched at two (n=259; 3.27, 1.42—7.54; p=0.006), three (n=253; 3.34, 1.45—7.71; p=0.005), or four (n=75; 3.51, 1.44—8.58; p=0.006) loci compared with matched units (n=69).

**Conclusion:** Our data suggest that the present strategy for umbilical-cord blood unit selection should be reassessed; matching at HLA C for units that are matched at HLA A, B, or DRB1 or in the presence of a single locus mismatch at HLA A, B, or DRB1 should be included to minimize mortality risks.

**Study # GS08-03**


**Purpose:** To determine whether immune modulation with anti-T cell antibody infusion abrogates some of the therapeutic benefits of transplantation. Anti-T cell antibody infusions anti-thymocyte globulin (ATG) or alemtuzumab are often employed to promote engraftment and reduce GVHD after reduced-intensity conditioning (RIC) transplantation. This is critical since the success of RIC transplantation is largely dependent upon alloimmune effects.

**Patients/Methods:** Using Cox regression, outcomes after RIC transplantation with in vivo T cell depletion (n=584 ATG; n=213 alemtuzumab) were compared to outcomes after T cell-replete (n=879) transplantation for adults with hematologic malignancies. All patients received alkylating agent plus fludarabine for conditioning. 792 patients received allografts from an HLA-matched sibling, 650 from an 8/8 and 234 from a 7/8 HLA-matched unrelated donor.

**Results:** Rates of grade II-IV acute and chronic GVHD were lowest with alemtuzumab regimens, 20% and 27%, respectively, compared to ATG (41% and 43%) and T-replete regimens (42% and 57%). Relapse was more frequent with alemtuzumab and ATG regimens compared to T cell-replete regimens (49%, 51%, and 38% respectively, p<0.001). Corresponding probabilities of non-relapse mortality were 21%, 26%, and 23%, p=0.04. Disease-free survival rates were lower with alemtuzumab and ATG regimens compared to T cell-replete regimens (30%, 25%, and 39%, respectively, p<0.001). Corresponding probabilities of overall survival were 50%, 38%, and 46%, p=0.008.

**Conclusion:** In vivo T cell depletion increased the likelihood of recurrent disease, which had a negative impact on disease-free survival. These results suggest caution in the routine use of in vivo T cell depletion with alkylating RIC regimens.
PRELIMINARY RESULTS

Study # GS08-02
Title: Should an older-aged patient receive a transplant from his older-aged sibling or a young HLA-matched unrelated donor?
Study Chair: Amin Alousi (M.D. Anderson Cancer Center)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Jennifer LeRademacher (jlerade@mcw.edu)
Study Status: Manuscript preparation
Purpose: To determine if receipt of an HLA-matched graft from an older-aged matched related donor (MRD) carries a higher risk for acute and chronic GVHD as well as non-relapse mortality (NRM) when compared to receipt of a graft from a younger-aged matched unrelated donor (MUD)
Patients/Methods: This study compared 1,265 patients with acute or chronic leukemia or non-Hodgkin lymphoma (NHL) who received T cell-replete MRD transplant from a donor <67 years of age to 141 patients who received an MRD transplant from a donor ≥67 years of age or 754 patients who received a transplant from a MUD <50 years of age, adjusting for study group imbalances. Transplants were performed from 1995-2005 and only 8/8 matched transplants were included in this study.
Results: When comparing donor type (older MRD vs. younger MUD), rates of acute GVHD grades II-IV and III-IV were higher for MUD recipients, regardless of donor age (RR 1.60, p<0.001 for grades II-IV acute GVHD), chronic GVHD (RR 1.48, p<0.001), and NRM.
Conclusion: Donor type is associated with lower risk of acute and chronic GVHD after an HLA-MRD when compared to MUD, regardless of donor age. Non-relapse mortality and overall survival are similarly better following MRD when compared to MUD donor, when accounting for recipient’s performance status. Having a younger MUD donor did not appear to protect from GVHD or NRM when compared to older-aged MRD transplant recipients. These results dispute earlier published reports where donor age appeared an important factor for GVHD risk.

Study # GS08-05/R04-88
Title: Umbilical cord blood transplantation for adults with acute leukemia: impact of single vs. double cord blood units on transplantation outcomes
Study Chair: Elizabeth Shpall (MD Anderson Cord Blood Bank)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Jennifer LeRademacher (jlerade@mcw.edu)
Study Status: Manuscript preparation
Purpose: To assess if two partially HLA-matched UCB units is a simple approach for increasing the number of nucleated cells in the UCB graft, if umbilical cord blood (UCB) transplantation is a successful treatment strategy for lymphohematopoietic malignancies, and if cell dose is a major limitation on the use of UCB, because more than two-thirds of adults cannot identify a single UCB unit with a total nucleated cell (TNC) cryopreserved dose >2.5 x 10^7/kg of recipient body weight
**Patients/Methods:** We compared transplantation outcomes observed in patients with acute leukemia who were either transplanted with an adequately dosed single unit (n=106) or two UCB units (n=303).

**Results:** There were no differences in the likelihood of neutrophil recovery (OR 0.83, p=0.59) or risks of transplant-related mortality (HR 0.91, p=0.63), relapse (HR 0.90, p=0.64), and overall mortality (HR 0.93, p=0.62) after transplantation of two UCB units versus 1 unit.

**Conclusion:** Co-infusion of two UCB units is a reasonable alternative when an adequate single unit is not available, extending the access of UCB transplantation to nearly all patients.

**Study # GS09-03**

**Title:** Comparison of outcomes of unrelated donor peripheral blood stem cells and umbilical cord blood after reduced-intensity conditioning

**Study Chair:** Claudio Brunstein (University of Minnesota Medical Center, Fairview)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** Kwang Ahn (kwooahn@mcw.edu)

**Study Status:** Manuscript preparation

**Purpose:** To determine the place of unrelated umbilical cord blood (UCB) relative to unrelated adult donor sources when considering RIC HCT for acute leukemia

**Patients/Methods:** We note the relative efficacy of double CB (dCB, n=161) transplantation vs. PBPC, whether from an 8/8 HLA-matched (MUD, n=313) vs. 1 allele-mismatched (MMUD, n=111) unrelated donor in patients with acute myeloid leukemia (AML, n=523) and lymphoblastic leukemia (ALL, n=62) transplanted from 2000-2009. Patients were aged 21-69 years. While there were no differences in overall survival by conditioning regimen in PBPC recipients, conditioning regimen influenced survival in recipients of dCB with the best survival in those treated with TBI 200 cGy + cyclophosphamide + fludarabine (TCF). Therefore, four groups were created for each multivariate analysis comparing transplant outcomes: dCB after TCF, dCB after other RIC regimens, MUD, and MMUD.

**Results:** Compared to dCB after TCF, grade II-IV but not grade III-IV acute GVHD was lower in those with a MUD, and chronic GVHD was higher in those with either a MUD or MMUD. Compared to dCB after other RIC regimens, transplant-related mortality and overall mortality were similar to those after MMUD and lower after MUD and dCB after TCF transplants.

**Conclusion:** These data support dCB after TCF for adults with acute leukemia where a transplant is indicated but a suitably HLA-matched unrelated adult donor is not available or when transplant is needed urgently.
Study # HC03-01
Title: Prevalence of microbially contaminated hematopoietic stem cell products
Study Chair: Richard Champlin (M.D. Anderson Cancer Center)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To examine the potential of microbially contaminated hematopoietic stem cell products to produce morbidity and mortality in transplant recipients
Patients/Methods: We surveyed 121 US transplant centers, revealing a total of 2,972 transplants reported to the CIBMTR from 2000-2001. Information regarding microbial contamination of infused grafts was obtained from 94 transplant centers for 2,312 patients. Two percent (52) of 2,286 infused grafts tested were culture positive for bacterial or fungal organisms. The microbial isolates included coagulase negative staphylococcus (56%), gram negative organisms (15%), coagulase positive staphylococcus (10%), gram positive rods (10%), streptococcus (8%), and fungus (1%). Prophylactic antibiotics targeted at the contaminant were given to 17 of the 52 recipients of contaminated grafts. Antibiotic regimens included vancomycin alone (76%), aminoglycosides and vancomycin (12%), or cephalosporin and vancomycin (12%). Fifty percent (47) of the centers that participated have existing policies regarding contaminated products.
Results: Patients with non-malignant disorders or who received bone marrow were more likely to have a contaminated graft. No differences in age distribution, sex, race, type of transplant (allogeneic versus autologous), and year of transplant were noted between recipients of contaminated and non-contaminated grafts. The unadjusted 100-day survival of persons receiving contaminated grafts was 86% (72-93) versus 81% (80-83) among those receiving non-contaminated grafts, p=0.35.
Conclusion: Approximately 2% of hematopoietic stem cell products infused for allogeneic or autologous transplantations in U.S. centers will test positive for microbial contamination, but such contamination does not increase post-transplant mortality. The absence of significant 100-day mortality among patients infused with contaminated grafts suggests that stringent regulatory policies regarding the use of contaminated hematopoietic cell products may not be indicated.

STUDIES IN PROGRESS

Study # GS05-01
Title: An audited matched pair analysis of transplants using red cell depleted versus plasma depleted umbilical cord blood stem cells
Study Chair: Karen Ballen (Massachusetts General Hospital)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Supplemental form/data collection
Study # GS07-01
Title: Peripheral blood versus bone marrow for non-myeloablative stem cell transplantation
Study Chair: Richard Champlin (M.D. Anderson Cancer Center)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Analysis in progress

Study # GS08-01
Title: Reassessment of impact of donor age on outcomes after unrelated/related donor HCT
Study Chairs: Craig Kollman (Jaeb Center for Health Research)
Vanderson Rocha (Hôpital Saint Louis)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: Kristin Ellis (kellis@mcw.edu)
John Klein (klein@mcw.edu)
Study Status: Analysis in progress

PLANNED STUDIES

Study # GS09-02
Title: Parameters of banked cord blood units that may be associated with graft failure
Study Chairs: Mary Eapen (CIBMTR Milwaukee)
Vanderson Rocha (Hôpital Saint Louis)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: TBD
Study Status: Protocol pending

Study # GS10-03
Title: Outcomes using a cryopreserved donor graft in unrelated allogeneic HCT
Study Chairs: Noelle Frey (Abramson Cancer Center, University Pennsylvania Medical Center)
Hillard Lazarus (Ireland Cancer Center, University Hospitals Case Medical Center)
MS Statistician: Tanya Pedersen (tpederse@nmdp.org)
PhD Statistician: TBD
Study Status: Protocol pending

Study # GS11-01
Title: The outcome of myeloablative and reduced intensity unrelated donor cord blood transplants for ALL in CR1 and CR2 in adults: a comparison with unrelated donor marrow and peripheral blood transplantation
Study Chairs: David Marks (Bristol Children’s Hospital)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
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9.7 GRAFT-VERSUS-HOST DISEASE WORKING COMMITTEE

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Co-Chair: Mary Flowers, MD, Fred Hutchinson Cancer Research Center
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PUBLISHED STUDIES

Study # GV04-02/GV05-03a


Purpose: To determine factors associated with non-relapse mortality in children diagnosed with chronic graft-versus-host disease (cGVHD) and to use these risk factors to develop an algorithm to identify patients at diagnosis of cGVHD who are at high risk for eventual mortality

Patients/Methods: The study population included all pediatric patients (age 0-20 years) who received an allogeneic transplant from a related or unrelated donor (URD) or umbilical cord blood (UCB) for acute lymphoblastic leukemia (ALL), acute myeloid leukemia (ALL), chronic myeloid leukemia (CML), or myelodysplastic syndrome (MDS) between 1995-2004 who were diagnosed with cGVHD within 1 year of transplant and were registered with CIBMTR. Patients with non-malignancies were not included in this study in order to be able to determine the contribution of cGVHD to mortality (non-relapse) as compared to progression of underlying disease. The study includes a total of 1,117 HCT recipients with cGVHD.

Results: Identification of predictors for mortality in children with cGVHD could permit risk-adapted therapy, help plan for clinical trials, and assist in counseling. A multivariate analysis was performed using data from CIBMTR to identify transplant- and cGVHD-related risk factors for non-relapse mortality (NRM) and survival. Four factors identified to be significantly associated (p<0.01) with higher
NRM: (1) HLA-partially-matched or -mismatched URD; (2) peripheral blood cell graft; (3) Karnofsky/Lansky score (KPS) <80 at cGVHD diagnosis; and (4) platelets <100 x 10⁹/L at cGVHD diagnosis. Factors significantly associated with worse survival were: (1) age >10 years; (2) transplant from an HLA-partially-matched or mismatched URD; (3) advanced disease at transplant; (4) KPS/L <80; and (5) platelets <100 x 10⁹/L. Cumulative incidence of discontinuation of systemic immune suppression (death and relapse treated as competing risks) at 1, 3, and 5 years after diagnosis of cGVHD were 22% (20-25%), 34% (31-37%), and 37% (34-40%).

Conclusion: This is the first large study elucidating factors affecting outcome after diagnosis of cGVHD in children. The results may be useful for risk stratification.

Study # GV04-02/GV05-03a


Purpose: To determine factors associated with non-relapse mortality in all patients diagnosed with chronic graft-versus-host disease (cGVHD) and to use these risk factors to develop an algorithm to identify patients at diagnosis of cGVHD who are at high risk for eventual mortality

Patients/Methods: The study population included all patients who received an allogeneic transplant from a related or unrelated donor (URD) or umbilical cord blood (UCB) for acute leukemia (AML and ALL), CML, or MDS from 1995-2004 and were diagnosed with cGVHD within 1 year of transplant and were registered with CIBMTR. For URD transplants facilitated by NMDP, informed consent was retrospectively obtained from living patients. To adjust for potential bias introduced by exclusion of non-consenting surviving patients, a corrective action plan (CAP)-modeling process randomly excluded approximately the same percentage of deceased patients, using a biased coin randomization with exclusion probabilities based on characteristics associated with not providing consent for use of data in survivors. The study includes a total of 5,343 HCT recipients with cGVHD.

Results: HCT and patient characteristics were evaluated, along with objective variables at diagnosis of cGVHD (thrombocytopenia, serum bilirubin, KPS, time to onset of cGVHD, and type of onset of cGVHD) to develop a simple and parsimonious cGVHD risk score. Ten variables identified as significant in multivariate analysis of overall survival and non-relapse mortality (NRM) were used to build the cGVHD risk score. Variable-specific risk scores were constructed for each variable based on the relative risk of each category of the variable. These scores were summed for each patient to assign an overall risk score (ORS). Risk groups (RG) were assigned based on the overall risk score as follows: RG1: ORS 0-2; RG2: ORS 3-6; RG 3: ORS 7-8; RG 4: ORS 9-10; RG 5: ORS 11; and RG6: ORS>12.
Conclusion: This analysis demonstrates applicability of cGVHD score to a large multi-center cohort of patients with cGVHD. Validation is needed and is planned in a more recent cohort of cGVHD patients registered with CIBMTR.

Study # GV05-02


Purpose: To identify prognostic factors for incidence of aGVHD after allotransplant and evaluate interactions of aGVHD with other factors and outcomes such as age, OS, LFS, TRM, and REL

Patients/Methods: Adult patients (≥20 years of age), undergoing a human leukocyte antigen (HLA) identical sibling donor (SD, n=3191) or unrelated donor (URD, N=2370) HCT, with a non-T cell depleted graft, for acute myeloid leukemia (AML), acute lymphocytic leukemia (ALL), chronic myeloid leukemia (CML), or myelodysplastic syndrome (MDS) from 1995-2005 were included. Six groups were created to evaluate the impact of conditioning [myeloablative (MA) or reduced intensity/ non-myeloablative (RIC)], total body irradiation (TBI) and graft type [(bone marrow (BM) or PBSC]. Analyses were performed separately for SD and URD.

Results: Six treatment categories were evaluated: myeloablative conditioning (MA) with total body irradiation (TBI) + peripheral blood stem cells (PBSC); MA + TBI + bone marrow (BM); MA + nonTBI + PBSC; MA + nonTBI + BM; reduced-intensity conditioning (RIC) + PBSC; RIC + BM. The cumulative incidences of grades B-D AGVHD were 39% (95% confidence interval: 37-41%) in the SD cohort and 59% (95% CI: 57-61%) in the URD cohort. Patients receiving SD transplants with MA + nonTBI + BM and RIC + PBSC had significantly lower risks of grades B-D AGVHD than patients in other treatment categories. Those receiving URD transplants with MA + TBI + BM, MA + nonTBI + BM, RIC + BM or RIC + PBSC had lower risks of grades B-D AGVHD than those in other treatment categories. The 5-year probabilities of survival were 46% (95% CI: 44-49%) with SD transplants and 33% (95% CI: 31-35%) with URD transplants. Conditioning intensity, TBI, and graft source have a combined effect on risk of AGVHD that must be considered in deciding on treatment strategy for individual patients.

Conclusion: This analysis emphasizes the differences in RF that impact aGVHD after SD and URD SCT including the combined effect of conditioning, TBI, and graft type. Modulation of these RF is needed to reduce acute GVHD incidence and associated mortality.
PRELIMINARY RESULTS

Study # GV04-02/GV05-03b

Title: Relapse in patients with chronic GVHD

Study Chairs: Nelson Chao (Duke University Medical Center)
               Madan Jagasia (Vanderbilt University Medical Center)
               Theresa Hahn (Roswell Park Cancer Institute)
               Philip McCarthy (Roswell Park Cancer Institute)

MS Statistician: Mukta Arora (arora005@umn.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
                  John P. Klein (klein@mcw.edu)

Study Status: Manuscript preparation

Purpose: To study the effects of chronic GVHD on late relapse, TRM, and survival after allogeneic HCT for hematologic malignancies

Patients/Methods: The study included patients who were alive and disease-free at 1 year after HCT. We also assessed the impact of cGVHD on treatment-related mortality (TRM), disease-free survival (DFS) and overall survival (OS) for 1 year disease-free survivors. The study included 7,489 recipients of HCT from HLA-identical siblings or unrelated donors conditioned with high-intensity regimens for acute myelogenous leukemia (AML), acute lymphoblastic leukemia (ALL), chronic myelogenous leukemia (CML), or myelodysplastic syndrome (MDS) between 1995 and 2004 and reported to the Center for International Blood and Marrow Transplant Research (CIBMTR).

Results: Within 12 months of HCT, 47% developed cGVHD. The protective effect of cGVHD on late relapse was limited to patients with CML. Other factors included early onset acute GVHD (<29 days of HCT), well-matched or partially-matched URD and GVHD prophylaxis other than T cell depletion. cGVHD was significantly associated with a higher risk of TRM and inferior OS. cGVHD was associated with worse DFS for patients with AML, ALL, and MDS. A separate analysis was performed for CML to evaluate the impact of cGVHD-specific variables on outcomes. A lower risk of late relapse was seen with several cGVHD-specific variables, i.e., severity, type of onset, and involvement of any organ. Moderate or severe cGVHD, platelet count <100 X109/L or KPS <80 had higher TRM and inferior DFS and OS. These results demonstrate that cGVHD was protective towards late relapse only in CML and associated with higher risk of TRM and inferior OS in all patients.

Conclusion: Since the protective effect of cGVHD on late relapse was seen only in CML patients, more aggressive measures to control cGVHD may be beneficial especially in AML, ALL, and MDS patients after high-intensity conditioning regimens.
Study # GV08-01
Title: The role of the HLA DRB1 antigen, DR15 in determining immunobiologic outcomes (graft-versus-host disease and graft-versus-leukemia) in HLA-matched BMT

Study Chairs: Minoo Battiwalla (National Heart Lung and Blood Institute - NIH)
MS Statistician: Peigang Li (peigang@mcw.edu)
PhD Statistician: Kristin Ellis (kellis@mcw.edu) John Klein (klein@mcw.edu)
Study Status: Submitted
Purpose: To determine the impact of DR15 on grades II-IV and III-IV acute GVHD, chronic GVHD, relapse and overall survival (OS), neutrophil engraftment, treatment-related mortality (TRM), and disease-free survival, and in the event of a positive finding in a primary outcome measure: age, disease (“lymphoid” versus “myeloid”), graft source (marrow versus peripheral blood), alleles (*1501 versus *1502).

Patients/Methods: Patient selection criteria are HLA-identical sibling transplantation (first), DNA-based HLA typing, AML, ALL, MDS, CML, marrow or PBSC, T-replete grafts, conventional myeloablative conditioning, and cyclosporine plus methotrexate-based GVHD prophylaxis, where 2,891 eligible transplants were reported to CIBMTR between 1990 and 2007, 732 (25.3%) patients were positive, and 2,159 (74.7%) patients were negative for DRB1*15:01 or *15:02 (DR15). Patient characteristics are AML=1038, ALL=700, CML=948, MDS=205, Caucasians=80%, Graft source: Marrow=56% PBSC=44%. DR15 positive and negative groups were similar in demographics, disease, and transplant characteristics.

Results: There were no significant differences in baseline characteristics between DR15-positive and negative groups. In univariate analysis, HLA-DR15 status had no impact on neutrophil engraftment, acute graft-versus-host disease (GVHD), chronic GVHD, treatment related mortality, relapse, disease-free survival, or overall survival. In multivariate analysis, DR15 status showed no significant differences in acute GVHD, chronic GVHD, overall survival, or relapse. Confining the univariate analysis to myeloid malignancies did not alter these findings. Multivariate analysis was restricted to the primary outcomes defined for the study; overall survival, relapse, grades B-D and C-D acute GVHD, and chronic GVHD, due to lack of significance for any of the secondary outcomes in the univariate analysis.

Conclusion: Our study used carefully selected criteria to avoid HLA-disparity, excluded low-resolution DRB1 typing, and defined a homogenous cohort of patients (myeloablative, T-lymphocyte replete transplants with uniform cyclosporine plus methotrexate-based GVHD prophylaxis). In this setting, DR15 status did not account for significant differences in clinical outcomes such as survival, relapse, or GVHD. This was noted on univariate analysis and confirmed on multivariate analysis. Alternative explanations for immunobiologic outcomes related to DR15 need to be considered. DR15 is associated with the DRB1*1501-DRB5*0101-DQB1*0602 haplotype. Linkage disequilibrium with other non-HLA immunogenetic factors, such as tumor necrosis factor-alpha (TNF-α) could also
account for immunological outcomes attributed to DR15. While our findings reduce the prospect for DR15-based therapeutic intervention in the allogeneic transplant setting, they do not diminish the influence of this unique HLA Class II antigen in the non-transplant setting for marrow failure states.

STUDIES IN PROGRESS

Study # GV06-01
Title: Comparison of GVHD prophylaxis regimens with and without methotrexate in matched, related, and unrelated donor transplantation
Study Chairs: Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Marcelo Pasquini (mpasquin@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # GV06-04
Title: Current trends in chronic GVHD - updated report from the CIBMTR/NMDP
Study Chairs: Sally Arai (Stanford Hospital & Clinics)
MS Statistician: Peigang Li (peigang@mcw.edu)
PhD Statistician: Tao Wang (taowang@mcw.edu)
Study Status: Data file preparation

Study # GV08-02
Title: Balancing acute GVHD and survival after allogeneic hematopoietic progenitor cell transplantation in hematological malignancies: a potential for graft engineering by T cell dose control
Study Chair: Ayman Saad (Medical College of Wisconsin)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # GV11-01
Title: Analysis of graft versus Lymphoma effect after allogeneic HCT
Study Chairs: Alvaro Urbano-Ispizua (Hospital Clinic of Barcelona)
Steven Pavletic (NIH-NCI Experimental Transplantation and Immunology Branch)
Mary Flowers (Fred Hutchinson Cancer Research Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # GV11-02
Title: Acute and chronic GVHD after unrelated umbilical cord blood transplantation: analysis of risk factors and outcomes
Study Chairs: Yi-Bin Chen (Massachusetts General Hospital)
Mary Flowers (Fred Hutchinson Cancer Research Center)
MS Statistician: Peigang Li (peigang@mcw.edu)
PhD Statistician: TBD
Study Status: Draft protocol received
Study # GV11-03
Title: A retrospective comparison of tacrolimus versus cyclosporine with methotrexate for immunosuppression after allogeneic HCT according to graft and donor type
Study Chairs: Yoshihiro Inamoto (APBMT)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # GV11-04
Title: Validation of chronic GVHD CIBMTR Risk Score
Study Chairs: Mary Flowers (Fred Hutchinson Cancer Research Center)
MS Statistician: Peigang Li (peigang@mcw.edu)
PhD Statistician: TBD
Study Status: Draft protocol received
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9.8 HEALTH POLICY & PSYCHOSOCIAL ISSUES WORKING COMMITTEE

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PUBLISHED STUDIES

Study # HS06-02


Patients/Methods: Our retrospective cohort study consisted of 885 adults and children (612 Whites, 145 Blacks, and 128 Hispanics) who received unrelated single UCBT for leukemia and myelodysplastic syndromes between 1995 and 2006 and were reported to the Center for International Blood and Marrow Transplant Research.

Results: A 5/6 HLA-matched unit with a total nucleated cell count infused of ≥2.5x10^7/kg was given to 40% of White and 42% of Hispanic, but only 21% of Black patients. Overall survival (OS) at 2 years was 44% for Whites, 34% for Blacks, and 46% for Hispanics (P=0.008). In multivariate analysis adjusting for patient, disease, and treatment factors (including HLA-match and cell dose), Blacks had inferior OS (RR of death=1.31, P=0.02) while OS of Hispanics was similar (RR=1.03, P=0.81) to that of Whites. For all patients, younger age, early-stage disease, use of units with higher cell dose, and performance status ≥80 were independent predictors of improved survival. Black patients and White patients infused with well-matched cords had comparable survival; similarly, Black and White patients receiving units with adequate cell dose had similar survival.

Conclusion: These results suggest that Blacks have inferior survival to Whites after single UCBT, but outcomes are improved when units with higher cell dose are used.
**Study # HS06-03**

**Journal Citation:** Majhail NS, Brazauskas R, Hassebroek A, Bredeson CN, Hahn T, Hale GA, Horowitz MM, Lazarus HM, Maziarz RT, Wood WA, Parsons SK, Joffe S, Rizzo JD, Lee SJ, Hayes-Lattin BM. *Outcomes of Allogeneic Hematopoietic Cell Transplantation for Adolescent and Young Adults Compared with Children and Older Adults with Acute Myeloid Leukemia.* 2011 Oct 29. [Epub ahead of print]. [PMC Journal - In Process]

**Purpose:** To compare outcomes among children (<15 years old), AYAs (15-40 years old) and older adults (>40 years old) receiving allogeneic HCT for acute myeloid leukemia (AML). Adolescents and young adults (AYAs) with cancer have not experienced improvements in survival to the same extent as children and older adults.

**Patients/Methods:** Our cohort consisted of 900 children, 2,708 AYA and 2,728 older adult recipients of HLA-identical sibling or unrelated donor (URD) transplant using myeloablative or reduced-intensity/non-myeloablative conditioning. Outcomes were assessed over three time periods (1980-1988, 1989-1997, 1998-2005) for sibling and two time periods (1989-1997, 1998-2005) for URD HCT. Analyses were stratified by donor type.

**Results:** Overall survival for AYAs using either siblings or URD improved over time. Although children had better and older adults had worse survival compared to AYAs, improvements in survival for AYAs did not lag behind those for children and older adults. Improvements in survival occurred due to a reduction in risk of treatment-related mortality. The risk of relapse did not change over time.

**Conclusion:** Improvements in survival among AYAs undergoing allogeneic HCT for AML have paralleled those among children and older adults.

**Study # HS08-04**


**Purpose:** To examine disadoption of high-dose chemotherapy followed by autologous blood and marrow transplantation (HDC/BMT) as a treatment for breast cancer following the release of negative clinical trials results in early 1999

**Patients/Methods:** In this study, we use patient-level data from CIBMTR to examine disadoption of high-dose chemotherapy followed by autologous blood and marrow transplantation (HDC/BMT) as a treatment for breast cancer following the release of negative clinical trials results in early 1999.

**Results:** There was rapid disadoption; one year after the results became public, procedure volume was 20% of the early 1998 peak. Hospitals that participated in the trials, teaching hospitals, and comprehensive cancer centers were no slower to disadopt HDC/BMT compared to other hospitals. Further examination suggests that hospitals passively abandoned HDC/BMT as demand declined rather than actively deciding to discontinue offering HDC/BMT to patients.
Conclusion: The case of HDC/BMT suggests that randomized controlled trials of questionable treatments, though costly, can yield long-run savings.

PRELIMINARY RESULTS

Study # HS05-02
Title: Consolidation, volume, and outcomes in leukemia treatment
Study Chair: Frank Schneider (previously at Harvard University)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Submitted
Purpose: To study the effect of consolidation on survival by empirically analyzing the causal nature of the volume-outcome relationship

Patients/Methods: The focus of the study was on the market of matched unrelated donor bone marrow transplants for leukemia treatment. Controlling for average hospital quality during the study period, we find that a one standard deviation increase in hospital volume during the 6 months preceding an unrelated transplant is associated with a 12% increase in the patient's survival probability 3 years after the transplant. We address the potential endogeneity due to time variant hospital quality with instrumental variables, using changes in the number of competitors adjusted by their distance to a hospital as exogenous predictor of changes in volume. Predicted volume was then used to establish a causal relationship between volume and outcome.

Results: Evidence showed a strong and causal relationship between volume and survival. A one standard deviation increase in volume is associated with a 36% increase in survival. Finally, we find evidence for perfect forgetting. This implies that high volume is not sufficient, but rather that regular performance of the procedure and hence high and constant average volume is essential.

Conclusion: This evidence suggests that some consolidation would be beneficial for patient survival, even at the cost of lower competition.

Study # HS07-01
Title: Consolidation, volume and outcomes in leukemia treatment
Study Chair: Philip McCarthy (Roswell Park Cancer Institute)
Theresa Hahn (Roswell Park Cancer Institute)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Preliminary results
Purpose: To assess if recent advances in HCT techniques and supportive care, new HCT indications, and improvements in survival outcomes may have increased access to and use of HCT

Patients/Methods: Autologous (Auto) and allogeneic (Allo) HCT have been used to cure malignant and non-malignant conditions for more than 40 years. We describe the HCT population, utilization, and procedure patterns in U.S. and Canadian HCT centers from 1994-2005. All data exclude donor lymphocyte infusions.
Results: Because of a decrease in auto HCT for breast cancer, the number of auto HCTs increased by >30% from the mid to late 1990s, then declined by >30% in the early 2000s, but rose again in 2004-05. In this same period, auto HCT increased for multiple myeloma (MM) and non-Hodgkin lymphoma (NHL)/Hodgkin lymphoma (HL), whereas acute or chronic leukemia decreased, and solid tumors remained stable. The number of allo HCTs increased over this 12-year period, mostly due to an increase in unrelated donor allo HCTs. From 1994-95 to 2004-05, Allo HCT increased for acute myelogenous leukemia (AML)/acute lymphoblastic leukemia (ALL), MDS/MPS, and NHL/HL, but decreased for chronic myelogenous leukemia (CML) and MM. Allo HCT conditioning regimen intensity was 100% myeloablative in 1994-95, whereas reduced-intensity/non-myeloablative regimens were used in 28% of Allo HCTs in 2004-05. The use of peripheral blood and cord blood has dramatically increased. The median age and upper age limit have increased for both auto HCT and allo HCT. The percent of non-White race recipients increased from 1994-95 to 2004-05 for both auto HCT and allo HCT.

Conclusion: The HCT population has changed dramatically over time. Further analyses are ongoing to determine the impact of these changes on the success of HCT.

STUDIES IN PROGRESS

Study # HS07-02
Title: The financial impact of allogeneic HCT on patient and family
Study Chairs: Navneet Majhail (National Marrow Donor Program)
              Kate Pederson (National Marrow Donor Program)
MS Statistician: Tanya Pedersen (tpederse@nmdp.org)
PhD Statistician: TBD
Study Status: Analysis in progress

Study # HS08-01
Title: Accreditation deficiencies and survival outcomes post HCT
Study Chairs: Fausto Jr. Loberiza (The Nebraska Medical Center)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: Brent Logan (blogan@mcw.edu)
Study Status: Analysis in progress

Study # HS08-03
Title: Estimating the current burden and future trends in the number of HCT survivors in the U.S.
Study Chair: Navneet Majhail (National Marrow Donor Program)
MS Statistician: TBD
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Analysis in progress
Study # HS09-01
Title: Practice patterns in HCT for AML
Study Chair: Navneet Majhail (National Marrow Donor Program)
MS Statistician: Navneet Majhail (nmajhail@nmdp.org)
PhD Statistician: Anna Hassebroek (ahassebr@nmdp.org)
Study Status: Protocol development

Study # HS10-01
Title: Outcomes of HCT for adolescents and young adults with ALL
Study Chairs: William Wood (University of North Carolina Hospitals)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: TBD
Study Status: Data file preparation

Study # HS11-01
Title: Generalizability of BMT CTN 0201 results: prognostic and outcome differences between participants and nonparticipants
Study Chairs: Nandita Khera (Fred Hutchinson Cancer Research Center)
Stephanie Lee (Fred Hutchinson Cancer Research Center)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: TBD
Study Status: Protocol development

Study # HS11-02
Title: Comparison of length of stay among alternative graft sources: single and double cord blood, matched unrelated donor, and other related donor
Study Chairs: Karen Ballen (Massachusetts General Hospital)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: TBD
Study Status: Protocol development
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9.9 IMMUNE DEFICIENCIES & INBORN ERRORS OF METABOLISM WORKING COMMITTEE

Co-Chair: Edwin Horwitz, MD, PhD, Children’s Hospital of Philadelphia
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Co-Chair: Harry Malech, MD, National Institute for Allergy & Infectious Diseases, NIH
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Co-Chair: Paul Veys, MD, Great Ormond Street Hospital for Children
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PUBLISHED STUDIES

Study # ID00-01

Purpose: To assess the incidence of malignancies in children with PIDD following allogeneic HCT and analyze risk factors for the development of malignancies

Patients/Methods: Our retrospective cohort study consisted of 885 adults and children (612 Whites, 145 Blacks, and 128 Hispanics) who received unrelated single UCBT for leukemia and myelodysplastic syndromes between 1995 and 2006 and were reported to the Center for International Blood and Marrow Transplant Research.

Results: The incidence of malignancy was highest for severe combined immunodeficiency (SCID) patients (3.7%), with an overall incidence of 1.65% for PIDD. Out of 27 patients, 24 patients were confirmed as having developed a malignancy post-HCT and had a lymphoproliferative disorder (PTLD) at a median of 2 months post-HCT. Of these, 16 had received T cell depleted (TCD) bone marrow. Three patients developed myelodysplastic disorder MDS (median 44 months). All had undergone total body irradiation (TBI) and had received TCD marrows.

Conclusion: Patients with PIDD who undergo HCT appear to be at a relatively low risk of developing malignancies compared to the historical risk of cancer in these patients. The most frequent malignancy seen is early-onset PTLD and, as has been noted for other HCT recipients, the use of TCD appears to correlate with the development of PTLD post-transplantation.
PRELIMINARY RESULTS

Study # ID06-03
Title: Outcomes of transplantation using various cell sources for Hurler’s Syndrome: a Eurocord/EBMT/CIBMTR collaborative study

Study Chairs: Jaap-Jan Boelen (Wihelmina Children’s Hospital/UMCU)
Joanne Kurtzberg (CORD:USE Cord Blood Bank; Duke University Medical Center; Pediatric Blood and Marrow Transplant; Carolinas Cord Blood Bank)
Paul Orchard (University of Minnesota Medical Center, Fairview)

MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: Jennifer Le-Rademacher (jlerade@mcw.edu)

Study Status: Manuscript preparation

Purpose: To assess the impact of various cell sources (Matched Sibling donor [MSD], unrelated donor [URD], unrelated cord blood [UCB] and T cell depleted URD [TCD-URD]) on outcomes of allogeneic HCT in Hurler’s Syndrome. Allogeneic HCT, if performed early, halts disease progression and prolongs life for patients with Hurler’s Syndrome, an otherwise lethal disease.

Patients/Methods: We analyzed 258 patients with Hurler’s Syndrome transplanted from 1995 to 2007. Median age at transplantation was 17 months, and median follow-up at 5 years. The donors were MSD (n= 37; 14%), URD (n=52; 21%), UCB (n=116; 44%), and TCD-URD (n=53; 21%). 97% of patients received busulfan-containing myeloablative.

Results: Overall survival was higher in patients aged <20 months and after MSD transplant, compared to the other sources URD, UCB, and TCD-URD. 21% of surviving patients (median follow-up, 5 years) had mixed donor chimerism (<5% donor) and the remaining 69% patients had donor chimerism >=95%. 93% of UCB transplant recipients had >=95% donor chimerism compared to 73% after MSD, 64% after URD and 75% after TCD-URD.

Conclusion: Early referral for transplantation with a suitably matched related or unrelated donor prior to 20 months of age is recommended.

Study # ID09-01
Title: Long-term survival and late deaths after HCT for primary immunodeficiency diseases and inborn errors of metabolism

Study Chair: Mary Eapen (CIBMTR Milwaukee)

MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: Kwang Woo Ahn (kwooahn@mcw.edu)

Study Status: Manuscript submitted

Purpose: To study late mortality after HCT for severe combined immunodeficiency (SCID), non-SCID immune diseases, and inborn errors of metabolism (IEM)

Patients/Methods: 960 patients (SCID, n=201; non-SCID, n=352; IEM, n=352) who survived at least 2 years after HCT and were disease free were eligible. 70% of SCID patients received grafts from a related donor and 56% of non-SCID and IEM patients received grafts from an unrelated donor.
Results: The 7-year probabilities of overall survival were 93%, 96%, and 90% for SCID, non-SCID immune diseases and IEM, respectively. No factor was associated with late deaths for SCID. For non-SCID, late deaths included more recipients of T cell depleted grafts. For IEM, there were more late deaths after unrelated donor and mismatched related donor transplant than matched sibling donor transplant. There were 69 late deaths, with 52 occurring 2-6 years after transplantation and 17 after 6 years.

Conclusion: Though the risk of late deaths in this population is in excess of that for the general population for several years after transplantation, with extended follow-up, the risk appears to decrease towards normal rates for patients with SCID and non-SCID surviving beyond 6 years after HCT, but not for patients with IEM.

Study # ID99-02
Title: The role of HCT in Langerhans cell histiocytosis
Study Chair: Paul Veys (Great Ormond Street Hospital for Children)
MS Statistician: Anna Hassebroek (ahassebr@nmdp.org)
PhD Statistician: Jennifer Le-Rademacher (jlerade@mcw.edu)
Study Status: Preliminary results
Purpose: To assess the feasibility of HCT in patients with Langerhans cell histiocytosis (LCH), a poorly understood and occasionally aggressive disorder that features lesional cells akin to Langerhans cells

Patients/Methods: The results of HCT for LCH were studied through the collaborative use of three large observational databases. The study included 22 allogeneic transplantations (HLA-identical and non-identical related as unrelated donors) for LCH reported to the CIBMTR, the EBMT, and the general Japanese Registry. Twenty of the 22 patients (91%) in this cohort were younger than 2 years old at transplantation. All patients received front-line therapy for LCH, but failed to achieve remission. All patients had multi-organ involvement and 20 of 22 (91%) had bone marrow involvement prior to or at transplantation. All but 1 patient had at least one poor prognosis organ involvement (bone marrow, liver or lung). Six had stable disease at transplantation and 16 had progressive disease. With a median follow-up of more than 4 years, 8 of 22 patients are alive.

Results: The 1- and 2-year probabilities of overall survival were 45% (range 25-66) and 35% (range 16-56), respectively. Causes of mortality included recurrent/progressive disease (n=2), veno-occlusive disease (n=2), infections (n=7), and diffuse alveolar hemorrhage (n=1).

Conclusion: While HCT in LCH is feasible for patients who fail conventional therapy, transplant-related mortality (TRM) is high. It is uncertain whether newer approaches in transplantation such as reduced-intensity conditioning regimens may lower TRM.
STUDIES IN PROGRESS

**Study # ID11-02**

**Title:** Outcomes after HCT in patients with I Cell Disease  
**Study Chair:** Troy Lund (lundx072@umn.edu)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Draft protocol received

**Study # ID98-05**

**Title:** HCT for infantile osteopetrosis  
**Study Chairs:** Anders Fasth (Sahlgrenska University Hospital; Tobias Registry of Swedish Bone Marrow Donors; Tobias Registry of Swedish Bone Marrow Donors - Cord Blood)  
Paul Orchard (University of Minnesota Medical Center, Fairview)  
**MS Statistician:** Anna Hassebroek (ahassebr@nmdp.org)  
**PhD Statistician:** Mei-Jie Zhang (meijie@mcw.edu)  
**Study Status:** Analysis in progress

PLANNED STUDIES

**Study # ID10-01**

**Title:** Follow-up of patients undergoing HCT for meta-chromatic leukodystrophy  
**Study Chair:** Peter J. Shaw (Children’s Hospital at Westmead)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Protocol pending

**Study # ID10-02**

**Title:** Outcomes of HCT for DNA repair disorders  
**Study Chair:** Andrew R. Gennery (Haematology & Cancer Services - Newcastle)  
**MS Statistician:** Anna Hassebroek (ahassebr@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Protocol pending

**Study # ID11-01**

**Title:** Outcomes in allogeneic stem cell transplantation for adrenoleukodystrophy  
**Study Chair:** Paul Orchard (University of Minnesota Medical Center, Fairview)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Protocol pending
9.10 IMMUNOBIOLOGY WORKING COMMITTEE

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PUBLISHED STUDIES

Study # IB06-04  

Purpose: To compare outcomes of haploidentical HCT between adults and children

Patients/Methods: Outcomes of adults and children aged 1-65 years old receiving myeloablative conditioning regimens followed by family 2 to 3 antigen HLA-mismatched HCT and reported to the Center for International Blood and Marrow Transplant Research (CIBMTR, n=137) or performed in Dao-Pei Hospital in Beijing, China (n=181) were compared

Results: Overall survival (OS) and outcomes were similar between adults and children. In the CIBMTR cohort receiving ex vivo T cell depletion (TCD), adults had higher TRM (RR 2.71, 95% CI 1.29-5.69, p=0.008) and lower overall survival (RR 1.75, 95%CI 1.08-2.84, p=0.023) than children. In the CIBMTR subset that did not receive ex vivo TCD, relapse was lower in adults compared to children (RR 0.24, 95% CI 0.07-0.80, p=0.020) but TRM, LFS and OS were similar.

Conclusion: Outcomes in adults and children are similar overall, although children have better survival than adults if ex vivo TCD is used.
Study # IB07-02

Purpose: To examine the effect of HLA mismatches at the amino acid sequence level on day 100 survival (D100S).

Patients/Methods: Patients received an HCT between 1988 and 2003 for acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, or myelodysplastic syndromes (early or intermediate stage) and were HLA-class I and DRB1 matched (n=1,507) or had a single HLA-class I mismatch at HLA-A (n=179), -B (n=88), or -C (n=333) and were DRB1 matched. Random forest as well as traditional univariate and multivariate analyses were performed.

Results: Four patient variables (age, disease stage, disease type, gender match) and 33 amino acid substitutions were identified as predictors of death at D100 post-transplant using Random Forest techniques. Thirteen of these amino acid substitutions had been previously reported by other investigators using classical multivariate analysis. Multivariate analyses using this dataset identified 5 amino acid substitutions associated with mortality at day 100.

Conclusion: Although additional studies need to be performed to confirm these findings, based on the weight of the literature, it may be prudent to avoid donors, if possible, that are mismatched with the recipient at positions 116 or 156 at either of the HLA class I loci, at position 9 at HLA-A or HLA-C, and at position 99 at HLA-C, since these mismatches are consistently associated with higher risks of early death and other adverse outcomes.

Study # IB10-05

Purpose: To test the hypothesis that a scoring system, HistoCheck, based on the functional similarity of amino acids and their position within the HLA molecule (i.e., DSS score), could predict the outcomes of unrelated HCT using 7/8 class I mismatched donors.

Patients/Methods: We evaluated the ability of a mismatch (MM) ranking system, HistoCheck, to predict the risk associated with HLA class I disparity in a population of 744 single allele or antigen HLA-A, B, or C MM myeloablative URD HCT recipients with AML, ALL, CML, or MDS, facilitated through the NMDP from 1988-2003. Multivariate models were used to adjust for other significant clinical risk factors. HLA MMs were scored using the HistoCheck web-based tool and the patients divided into 4 quartiles: Dissimilarity Score (DSS) 1.04-2.84 (allele MM), >2.84-13.75 (allele and antigen MM), >13.75-19.39 (antigen MM) and >19.39-36.62 (antigen MM).
Results: Using the lowest scoring quartile as the reference, the DSS groups were evaluated for associations with relapse, treatment-related mortality (TRM), acute and chronic graft-versus-host disease (GVHD), leukemia-free survival (LFS), and overall survival (OS) in the entire cohort, and in subset analyses by disease and disease stage. No significant associations were found between DSS and any outcomes in the overall cohort using the quartile categories or treating DSS as a continuous variable. Higher DSS scores were associated with decreased engraftment in early stage disease (p=0.0003) but not in other disease stages.

Conclusion: DSS does not correlate with transplantation outcomes, and the HistoCheck scoring system does not provide an effective strategy to rank HLA class I MM.

PRELIMINARY RESULTS

Study # IB05-03s
Title: Genetic polymorphisms in the genes encoding human interleukin-7 receptor-alpha: prognostic significance in allogeneic HCT

Study Chair: TBD
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Tao Wang (taowang@mcw.edu)
Study Status: Submitted

Purpose: To validate previous results suggesting that donor +1237G in the alpha chain for IL-7R is associated with higher TRM and more acute GVHD.

Patients/Methods: 590 Caucasian adults with AML, ALL, CML or MDS with early stage disease transplanted with myeloablative conditioning and 10/10 HLA matched unrelated donors were studied. All transplants were T-replete.

Results: Neither donor nor recipient SNP genotypes for IL-7R alpha were associated with overall survival or TRM. Some univariate associations were seen between donor SNP genotypes, relapse and acute and chronic GVHD but these were not confirmed in multivariate analysis.

Conclusion: Results do not support an association between the 3 examined IL-7R alpha SNPs and adverse transplant outcomes.

Study # IB06-02
Title: The impact of mismatches in low expression HLA loci on the outcomes of unrelated donor transplants

Study Chair: Marcelo Fernandez Vina (Stanford Hospital & Clinics)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Manuscript preparation

Purpose: To investigate the role of incompatibilities in the HLA DRB3/4/5, DQ and DP (low expression) loci (LEL) on the outcome of unrelated HCT. We hypothesized that the effects of these loci are weak, cumulative and only demonstrable in combination with mismatches in other loci.

Patients/Methods: Results of 3,853 U.S. transplants facilitated by NMDP between 1988 and 2003 were evaluated and compared to retrospective HLA high-resolution typing. Patients
had acute myelogenous leukemia (AML), acute lymphoblastic leukemia (ALL), chronic myelogenous leukemia (CML), or MDS.

Results: In the 7/8 HLA-A, B, C, DRB1 matched group, patients with 3 or more mismatches (MM) in LEL in the GVHD vector had significantly higher risk for mortality and transplant-related mortality than the subgroups with 0MM and 1MM in LEL. No single LEL appeared to have a more pronounced effect on clinical outcome, when mismatched, versus the other LEL.

Conclusion: Three or more mismatches at the HLA class II LEL are associated with poor clinical outcome after 7/8 matched transplantation. Prospective evaluation of matching for the LEL may be warranted to reduce post-transplant risks in 7/8 HLA-A, B, C, DRB1 matched donor recipient pairs.

Study # IB06-09s
Title: Detection of HLA antibody to the mismatched antigen in single antigen HLA-mismatched unrelated donor HCTs: is it associated with GVHD outcome?
Study Chair: Sally Arai (Stanford Hospital & Clinics)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: TBD
Study Status: Manuscript preparation
Purpose: To determine whether alloantibody to HLA is made in patients with acute GVHD

Patients/Methods: Day 100 serum samples of 40 recipient-donor pairs of single antigen/allele HLA-mismatched unrelated donor transplants who developed acute GVHD were tested. Class I and II antibody levels to the mismatched alleles were tested using single antigen beads covering 96 different class I (A,B,C) and 56 different class II (DR,DQ,DP) alleles on a Luminex platform. Since the threshold antibody response to HLA mismatch in marrow transplantation is unknown, we applied the established scale from solid organ transplantation where >=1,000 is positive, 500-999 is possible, and <500 is negative. The limitation of the analysis is that the beads contain every antigen but not every allele.

Results: In the 40 pairs, no anti-A, B, or C allele-specific antibodies were detected at day 100 in either GVH or HVG directions. Class II antibodies against DRB1, DQB1, DPA1 and DPB1 were detected, with the highest frequency antibody seen against DPA1.

Conclusion: Day 100 Ab to class II rather than class I allele mismatch were observed. Current studies to determine if these are de novo or preformed antibodies are being performed using pre-transplant sera from these same pairs. Future studies will look at antibody association with GVHD and disease relapse with attention to class II on B cells as a meaningful GVL target.
Study # IB06-11s
Title: The effect of non-inherited maternal antigens in cord blood transplantation
Study Chair: Lee Baxter-Lowe (University of California San Francisco Medical Center)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: TBD
Study Status: Manuscript preparation
Purpose: To determine whether in utero exposure to non-inherited maternal antigens (NIMA) induces partial tolerance to the haplotype in the fetus and umbilical cord blood (UCB) stem cells.
Patients/Methods: Matched case-control analysis of patients receiving a single UCB unit for treatment of hematological malignancy. NIMA matched cases (N=48) and NIMA mismatched controls (N=116) were matched on age, disease, disease status, conditioning regimen, HLA-match (4/6 or 5/6) and cell dose.
Results: TRM was lower after NIMA-matched compared to NIMA-mismatched transplantations (RR=0.48, p=0.05; 18% vs. 32% at 5 years after transplantation). Consequently, overall survival was higher after NIMA-matched transplantations. The 5-year probabilities of overall survival after NIMA-matched and NIMA-mismatched transplantations were 55% and 38%, respectively (p=0.04).
Conclusion: When faced with the choice of multiple HLA-mismatched UCB units containing adequate cell dose, selecting a NIMA-matched unit offers a novel strategy to lower TRM and consequently, improve survival.

Study # IB07-06
Title: Impact of HLA-DP matching and epitopes on transplant outcomes
Study Chair: Katharina Fleischhauer (San Raffaele Telethon Institute for Gene Therapy) Bronwen Shaw (Anthony Nolan)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Ted Gooley (tgooley@fhcrc.org)
Study Status: Manuscript preparation
Purpose: To define the clinical importance of HLA-DP matching and epitopes on transplant outcome. This is an IHWG study.
Patients/Methods: 5,838 HLA-A,-B,-C,-DRB1,-DQB1 allele matched pairs included 4,490 DPB1 mismatched and 1,348 12/12 matched pairs. HLA-DP mismatched pairs were defined as T cell epitope disparate (TCED) or matched (TCEM). Using the TCEM HLA-DP mismatched pairs as the reference population, the risks of GVHD, relapse, TRM and mortality were defined for the TCED pairs.
Results: Compared to the DP mismatched by TCEM group, 10/10 TCED transplants had statistically significantly higher hazards of TRM, grades III - IV acute GVHD, and mortality, but lower relapse (ns). The 12/12 transplants had lower treatment-related mortality and grades III - IV GVHD, but higher relapse, and comparable mortality.
Conclusion: Among 10/10 matched pairs, knowledge of the TCE match status provides new information on transplant risks. TCED patients represent a high risk transplant group, whereas TCEM patients have comparable survival compared to 12/12
matches. These data provide impetus for future exploration of the impact of TCEs in HLA (9/10) mismatched transplants.

**Study # IB07-09**

**Title:** To develop and test a prognostic index for survival in CML matched unrelated donor cohorts

**Study Chair:** Anne Dickinson (University of Newcastle)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** Kim Pearce (k.f.pearce@newcastle.ac.uk)

**Study Status:** Submitted

**Purpose:** To validate previous work in HLA-matched sibling transplantation showing that cytokine SNPs were associated with decreased survival and improved prognostic accuracy when combined with the European Group for Blood and Marrow Transplantation (EBMT) risk score in CML patients.

**Patients/Methods:** We studied 383 adults with CML receiving 10/10 HLA-matched URD transplants. After calculation of the EBMT score (determined by clinical factors), we tested whether absence of recipient TNFRII 196R, absence of donor IL-10 ATA/ACC and presence of donor IL-1Ra allele 2 were associated with transplant outcomes.

**Results:** None of the polymorphisms were associated with overall survival, non-relapse mortality, relapse, or acute graft-versus-host disease in the URD cohort. Comparison of the URD and HLA-identical sibling cohorts showed differences in survival and clinical characteristics.

**Conclusion:** We did not confirm that non-HLA polymorphisms were associated with outcomes in myeloablative URD HCT for CML, possibly due to the strong association between clinical variables and outcome that masked more subtle genetic effects.

**Study # IB09-02**

**Title:** Non-permissive HLA-DPB1 disparities based on T cell alloreactivity: validation of Italian Registry findings and definition of the role of the DP-alpha chain

**Study Chair:** Katharina Fleischhauer (San Raffaele Telethon Institute for Gene Therapy)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** Tao Wang (taowang@mcw.edu)

**Study Status:** Manuscript preparation

**Purpose:** To test whether HLA-DPA1 should be considered when determining permissive and non-permissive HLA-DP mismatches

**Patients/Methods:** First, the HLA-DPB1-TCE algorithm was validated in an independent cohort of 1281 HCT from 10/10 HLA-matched UD, facilitated through the National Marrow Donor Program for hematologic malignancies. Second, the relative contribution of HLA-DPA1 was tested in this cohort.

**Results:** Non-permissive HLA-DPB1 TCE disparities were associated with a significant increase in non-relapse mortality (NRM) both in GvH (HR 1.29, 95% CI 1.03-1.60, P=0.026) and HvG (HR 1.26, 95% CI 1.01-1.58, P=0.04) direction, as compared to permissive disparities. In contrast, non-permissive GvH but not HvG mismatches were significantly protective from disease relapse (HR 0.55, 95% CI 0.37-0.80, P=0.002 GvH and HR 1.08, 95% CI 0.79-1.47, P=0.60 HvG). There was a trend for association of non-permissive GVH or HVG with an increased risk of GVHD.
(P=0.11) or overall mortality (P=0.11), however, this did not reach statistical significance. A post-hoc power analysis suggests a 5 times larger cohort is required to evaluate overall mortality with 80% power. When HLA-DPA1 was also considered, no significant correlations with any clinical endpoint were observed.

Conclusion: Consideration of HLA-DPA1 did not improve identification of permissive and non-permissive TCE disparities. This is in line with data from molecular modeling suggesting that the polymorphic HLA-DPA1 residues do not appear to be in close contact with residues deemed important for the HLA-DPB1-TCE.

STUDIES IN PROGRESS

Study # IB05-02s
Title: The effect of a single major histocompatibility complex class I mismatch with numerous sequence differences on the clinical outcomes of unrelated HCT
Study Chair: Martin Heemskerk (Dutch Transplant Foundation)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Ted Gooley (tgooley@fhcrc.org)
Study Status: Ongoing

Study # IB06-05
Title: Use of high-resolution HLA data from the NMDP for the International Histocompatibility Working Group in HCT
Study Chair: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Ted Gooley (tgooley@fhcrc.org)
Study Status: Ongoing

Study # IB07-03
Title: Analysis of killer immunoglobulin-like receptor ligands in reduced intensity conditioning allogeneic HCT
Study Chair: Ronald Sobecks (Cleveland Clinic Foundation)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: TBD
Study Status: Analysis in progress

Study # IB07-04
Title: Employing advanced bioinformatic methods for predicting peptide specificities of HLA molecules in the characterization of permissible mismatches in hematopoietic cell transfer
Study Chair: Soren Buus (University of Copenhagen)
MS Statistician: Mike Haagenson (mhaagens@nmdp.org)
PhD Statistician: Ted Gooley (tgooley@fhcrc.org)
Study Status: Ongoing
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<thead>
<tr>
<th>Study #</th>
<th>Title</th>
<th>Study Chair</th>
<th>MS Statistician</th>
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<tr>
<td>IB07-05</td>
<td>Impact of donor-recipient ethnicity on risk of acute GVHD among HLA-A, B, C, DRB1, DQB1, DPB1 matched unrelated transplants</td>
<td>Yasuo Morishima (Aichi Cancer Center)</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
<td>Ted Gooley (<a href="mailto:tgooley@fhcrc.org">tgooley@fhcrc.org</a>)</td>
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<td>IB07-07</td>
<td>Impact of HLA-DR15 on clinical outcomes after unrelated HCT</td>
<td>TBA</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
<td>Ted Gooley (<a href="mailto:tgooley@fhcrc.org">tgooley@fhcrc.org</a>)</td>
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<td>IB07-08</td>
<td>Single nucleotide polymorphisms in the P53 pathway (P53, MDM2, ATM AND P21/WAF1) and transplant outcomes after unrelated HCT</td>
<td>Bo Dupont (Memorial Sloan-Kettering Cancer Center)</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
<td>Ted Gooley (<a href="mailto:tgooley@fhcrc.org">tgooley@fhcrc.org</a>)</td>
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<td>IB08-02</td>
<td>Evaluation of HLA matching requirements in unrelated HCT for nonmalignant disorders</td>
<td>Ann Woolfrey (Fred Hutchinson Cancer Research Center) John Horan (Children’s Healthcare of Atlanta at Egleston)</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
<td>Tao Wang (<a href="mailto:taowang@mcw.edu">taowang@mcw.edu</a>)</td>
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<td>IB08-08</td>
<td>Genome-wide association in unrelated donor transplant recipients and donors: a pilot study</td>
<td>Rakesh Goyal (Children’s Hospital of Pittsburgh of UPMC)</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
<td>TBD</td>
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<td>IB09-01s</td>
<td>Clinical importance of minor histocompatibility complex haplotypes in umbilical cord blood transplantation</td>
<td>Effie Petersdorf (Fred Hutchinson Cancer Research Center)</td>
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Study # IB09-03s
Title: Clinical relevance of cytokine/immune response gene polymorphisms in umbilical cord blood transplantation
Study Chair: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Ongoing

Study # IB09-04s
Title: D/R gene polymorphisms of drug metabolisms and inate immune response post allele matched matched unrelated donor HCT
Study Chair: Vanderson Rocha (Hopital Saint Louis)
MS Statistician: JohnBosco Umejiego (jumejieg@nmdp.org)
PhD Statistician: TBD
Study Status: Sample typing

Study # IB09-05s
Title: Identification of functional single nucleotide polymorphisms in umbilical cord blood transplantation
Study Chair: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Ongoing

Study # IB09-06s/RT09-04
Title: Genetic polymorphisms and HCT related mortality re: pre-HCT conditioning in matched unrelated donor HCT
Study Chair: Theresa Hahn (Roswell Park Cancer Institute)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # IB09-07s
Title: Clinical significance of genome-wide variation in unrelated HCT
Study Chair: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Ongoing

Study # IB10-01
Title: Donor and recipient telomere length as predictors of outcomes after HCT in patients with acquired severe aplastic anemia
Study Chair: Shahinaz Gadalla (National Cancer Institute)
MS Statistician: TBD
PhD Statistician: David Savage (NYPH/ Columbia University Medical Center)
Study Status: Sample typing
### Study # IB10-02
**Title:** Development of GVHD prevention diagnostic test  
**Study Chair:** Larry Greller (Predictive BioDiagnostics)  
Roland Somogyi (Predictive BioDiagnostics)  
**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Sample typing

### Study # IB10-03
**Title:** TLR and HMGB1 gene polymorphisms in unrelated HCT  
**Study Chair:** Klaus Müller (Juliane Marie Center Pediatric Clinic, Denmark)  
**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Sample typing

### Study # IB10-04s
**Title:** A validation study of the role of base excision repair pathway as a predictor of outcomes after HCT  
**Study Chair:** Mukta Arora (University of Minnesota Medical Center, Fairview)  
**MS Statistician:** JohnBosco Umejiego (jumejieg@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Analysis in progress

### Study # IB10-07
**Title:** Use of HLA structure and function parameters to understand the relationship between HLA disparity and transplant outcomes  
**Study Chair:** Lee Baxter-Lowe (University of California San Francisco Medical Center)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Protocol development

### Study # IB11-01
**Title:** Analysis of the NIMA effect on the outcome of unrelated PBSC/BM transplantation  
**Study Chair:** Gerhard Ehninger (Universitätsklinikum Dresden)  
**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Protocol development

### Study # IB11-02s
**Title:** Impact of ctla4 single nucleotide polymorphisms on outcome after unrelated donor transplant  
**Study Chair:** Madan Jagasia (Vanderbilt University Medical Center)  
**MS Statistician:** JohnBosco Umejiego (jumejieg@nmdp.org)  
**PhD Statistician:** TBD  
**Study Status:** Protocol development
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<th>Study # IB11-03</th>
<th>Title: Evaluation of the impact of allele homozygosity at HLA loci on outcome</th>
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<tr>
<td>Study Chair:</td>
<td>Carolyn Hurley (Georgetown University Hospital)</td>
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<td>MS Statistician:</td>
<td>John Bosco Umejiego (<a href="mailto:jumejieg@nmdp.org">jumejieg@nmdp.org</a>)</td>
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<th>Title: Impact of amino acid substitution at peptide binding pockets of HLA class I molecules on HCT outcome</th>
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<td>Study Chair:</td>
<td>Joseph Pidala (H. Lee Moffitt Cancer Center and Research Institute)</td>
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<td>MS Statistician:</td>
<td>Mike Haagenson (<a href="mailto:mhaagens@nmdp.org">mhaagens@nmdp.org</a>)</td>
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<th>Study # IB11-05</th>
<th>Title: KIR genotyping and Immune function in MDS patients prior to unrelated donor transplantation</th>
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<tr>
<td>Study Chair:</td>
<td>Erica Warlick (University of Minnesota Medical Center, Fairview)</td>
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<td></td>
<td>Jeffrey Miller (University of Minnesota Medical Center, Fairview)</td>
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<th>Title: Evaluation of the impact of potentially non-immunogenic HLA-C allele level mismatch</th>
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<td>Study Chair:</td>
<td>Marcelo Fernandez Vina (Stanford Hospital &amp; Clinics)</td>
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<td>Michelle Setterholm (National Marrow Donor Program)</td>
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<td>MS Statistician:</td>
<td>John Bosco Umejiego (<a href="mailto:jumejieg@nmdp.org">jumejieg@nmdp.org</a>)</td>
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<th>Title: Effect of Rituximab and ABO mismatch</th>
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<td>David Miklos (Stanford Hospital &amp; Clinics)</td>
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<th>Study # IB11-08</th>
<th>Title: Synergism between minor and major histocompatibility antigens</th>
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<td>Study Chair:</td>
<td>Eric Spierings (University Medical Center)</td>
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**Study # R02-40s/R03-63s**

**Title:** The effect of a single major histocompatibility complex class I mismatch with numerous sequence differences on the clinical outcomes of unrelated HCT

**Study Chair:** Jeffrey S. Miller (University of Minnesota)
Elizabeth Trachtenberg (Children's Hospital of Oakland Research Institute)
Sarah Cooley (University of Minnesota)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** John Klein (klein@mcw.edu)
Chap Le (University of Minnesota)

**Study Status:** Ongoing

**Study # R04-75s**

**Title:** Functional significance of cytokine gene polymorphisms in modulating risk of post-transplant complications

**Study Chair:** Effie Petersdorf (Fred Hutchinson Cancer Research Center)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** Ted Gooley (tgooley@fhcrc.org)

**Study Status:** Ongoing

**Study # R04-76s**

**Title:** Identification of functional single nucleotide polymorphisms in unrelated HCT (IHWG)

**Study Chair:** Effie Petersdorf (Fred Hutchinson Cancer Research Center)

**MS Statistician:** Mike Haagenson (mhaagens@nmdp.org)

**PhD Statistician:** Ted Gooley (tgooley@fhcrc.org)

**Study Status:** Ongoing

---

**PLANNED STUDIES**

**Study # IB06-10**

**Title:** Evaluation of the impact of the exposure to non-inherited maternal antigens during fetal life and breast feeding and to the inherited paternal antigens during pregnancy on the clinical outcomes of HCT from haploidentical family members

**Study Chair:** Jon Van Rood (Leiden University Medical Centre)

**MS Statistician:** TBD

**PhD Statistician:** TBD

**Study Status:** Deferred

**Study # IB06-13**

**Title:** HLA disparity in unrelated cord blood transplantation: delineation of factors contributing to transplant outcomes

**Study Chair:** Lee Baxter-Lowe (University of California San Francisco Medical Center)

**MS Statistician:** TBD

**PhD Statistician:** TBD

**Study Status:** Deferred
Study # IB08-04
Title: Immune response gene polymorphisms in unrelated donor HCT in children
Study Chair: Klaus Müller (Juliane Marie Center Pediatric Clinic, Denmark)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending

Study # IB08-05/LK08-04
Title: Evaluation of lymphotoxin alpha alleles in relation to relapse in AML and CML
Study Chair: Phillip Posch (Georgetown University Hospital)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred

Study # IB08-06
Title: Analysis of killer immunoglobulin-like receptor ligands in umbilical cord blood transplantation
Study Chair: Ronald Sobecks (Cleveland Clinic Foundation)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending

Study # IB10-06
Title: Identification of common, clinically significant, minor histocompatibility antigens through HCT donor/patient polymorphism disparities
Study Chair: Paul Armistead (University of North Carolina Hospitals)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred

Study # R04-80s
Title: Impact of HLA matching on outcomes in pediatric patients undergoing unrelated umbilical cord blood transplantation
Study Chair: Susana Marino (University of Chicago Hospitals)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred
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9.11 INFECTION & IMMUNE RECONSTITUTION WORKING COMMITTEE

Co-Chair: Juan Gea-Banacloche, MD, NIH-NCI Experimental Transplantation & Immunology Branch
Email: banacloj@mail.nih.gov

Co-Chair: Michael Boeckh, MD, Fred Hutchinson Cancer Research Center
Email: mboeckh@fhcrc.org

Co-Chair: Paul Szabolcs, MD, Children’s Hospital of Pittsburgh
Email: paul.szabolcs@chp.edu

Scientific Director: Marcie Tomblyn, MD, MS, H. Lee Moffitt Cancer Center and Research Institute
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PUBLISHED STUDIES

Study # LE04-01b

Purpose: To evaluate herpes virus (cytomegalovirus [CMV], herpes simplex virus, varicella zoster virus) and invasive fungal infections as a cause of significant morbidity and mortality in allogeneic HCT recipients despite the availability of effective therapies

Patients/Methods: In this study, we developed an Internet-based survey, which was distributed to all hematopoietic cell transplant centers participating in CIBMTR, to gather information on strategies utilized for the prevention of disease caused by herpes viruses and fungal infections between 1999 and 2003.

Results: The survey response rate was 72%, representing 175 programs from 32 countries. Generally, reported center strategies were in accord with the Center for Disease Control and Prevention guidelines published in 2000, with 81% of programs using low-dose acyclovir prophylaxis for herpes simplex virus seropositive patients, 99% of programs reporting use of a CMV prevention strategy during the first 100 days post transplant for all patients at risk of CMV disease, and 90% of programs using antifungal prophylaxis. Seventy percent of programs reported routine use of a CMV prevention strategy in high-risk patients after day 100. The greatest departure from published guidelines was the use of acyclovir prophylaxis for varicella zoster virus seropositive recipients in 75% of programs. There were very few reported changes within centers in practices over the study time period. Significant regional variations were found with regard to surveillance procedures.
and treatment durations. There were no significant differences in treatment practices by center size and very few differences found between those centers that reported treating primarily pediatric patients versus primarily adult patients.

**Conclusion:** In summary, our survey demonstrates overall agreement with published guidelines for the prevention of disease because of herpes viruses and fungal infections with significant regional differences found in duration of antiviral prophylaxis, duration of preemptive therapy, and duration and dosing of antifungal prophylaxis. Center size and age of primary patient population were not associated with many reported differences in strategies.

### PRELIMINARY RESULTS

**Study # GV02-02**

**Title:** No adverse effect of recipient or donor hepatitis B and/or hepatitis C serostatus on the outcomes of related donor allogeneic HCT

**Study Chairs:** Karen K. Ballen (Massachusetts General Hospital)  
Marcie Tomblyn (H Lee Moffitt Cancer Center)

**MS Statistician:** Min Chen (minchen@mcw.edu)  
**PhD Statistician:** Sergey Tarima (starima@mcw.edu)

**Study Status:** Manuscript submitted

**Purpose:** To evaluate available information, though limited, on allogeneic HCT in patients with prior or current hepatitis B (HBV) or C (HCV) infection as well as for recipients receiving hematopoietic cells from seropositive donors.

**Patients/Methods:** Transplant outcomes in 416 recipients from 121 centers who received a related-donor allogeneic HCT for either AML, ALL, CML, or NHL from 1995-2003 were evaluated. Of these, 161 cases had markers of ongoing or previous viral hepatitis in the recipient (n=89), the donor (n=35), or both (n=37), compared to 256 matched seronegative controls. Cases were positive for either HBV (n=100), HCV (n=46), or both (n=15).

**Results:** With a median follow-up of 5.9 years for cases and 6.7 years for controls, there were no significant differences in the incidence of treatment-related mortality, survival, and GVHD, whether comparing recipient and/or donor positivity for infections with HBV and HCV. The frequencies of hepatic toxicities as well as causes of death between cases and controls were similar.

**Conclusion:** Infection with HBV or HCV in either the recipient or the donor should not be considered a contraindication to transplant.

**Study # IN05-02**

**Title:** Atypical mold infections in HCT patients

**Study Chairs:** Steven Trifilio (Northwestern Memorial Hospital)  
Marcie Tomblyn (H Lee Moffitt Cancer Center)

**MS Statistician:** Min Chen (minchen@mcw.edu)  
**PhD Statistician:** John Klein (klein@mcw.edu)

**Study Status:** Manuscript preparation
Purpose: To assess the impact of atypical mold infection (AMI) on transplant outcome and risk factors for AMI which have not been well assessed in the current era of antifungal agents.

Patients/Methods: All patients receiving allogeneic HCT from 1995 - 2008 for any indication are included. Cases (n=124) had a Zygomycetes [n=72 (58%)] or Fusarium [n=52 (42%)] AMI between day 0 - 365 after HCT. A control cohort (n=11856) included all patients from the same 66 centers as cases. HCT indication was malignancy in 106 (85%) cases and 9603 (81%) controls.

Results: Cases were older [41 yrs (1–68) versus 34 years (1 – 79); p=0.004] and more likely to have a KPS <90% at HCT [cases=50 (40%); ctl=3330 (28%); p=0.009]. Similar numbers of cases occurred before and after May 2002 [1995 – Apr 2002=60 (48%); May 2002 – 2008=64 (52%)]. Systemic antifungal prophylaxis was similar between cases and ctl (p=0.64). The median time from HCT to AMI was 48 days (0 – 363) with most occurring between day 0–100 [day 0 – 30=49 (40%); day 31–60=18 (15%); day 61 – 100=22 (18%)]. AMI diagnosis significantly impacted 1-year survival (OS) with a probability (95% CI) for cases of only 22% (15 – 29) compared to 65% (64 – 65) for ctl [p<0.001]

Conclusion: Atypical mold infection occurs infrequently but appears similar in the current antifungal era and remains associated with high morbidity.

STUDIES IN PROGRESS

Study # IN06-01
Title: The rate of breakthrough of pneumocystis jiroveci pneumonia as a function of prophylaxis regimens
Study Chairs: Kirsten Williams (NIH-NCI Experimental Transplantation & Immunology Branch – Related Donor Program)
MS Statistician: Tanya Pedersen (tpederse@nmdp.org)
PhD Statistician: TBD
Study Status: Supplemental form/data collection

Study # IN07-01
Title: Bloodstream infection by vancomycin-resistant enterococcus in patients undergoing allogeneic HCT
Study Chairs: Mark Robien (Masonic Cancer Center University of Minnesota) Genovefa A. Papanicolaou (Memorial Sloan-Kettering Cancer Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Supplemental form/data collection

Study # IN07-02
Title: Epidemiology of infections after HCT
Study Chair: Randall T. Hayden (St. Jude Children’s Research Hospital)
MS Statistician: Min Chen (minchen@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
Study # IN08-01
Title: Comparison of nonmyeloablative allogeneic HCT and myeloablative transplantation for early bacterial, fungal, and viral infections in patients with AML in first complete remission
Study Chair: Celalettin Ustun (Medical College of Georgia)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # IN09-01
Title: Outcomes of allogeneic HCT for patients with hematologic malignancies with and without pre-existing fungal infections
Study Chair: Richard T. Maziarz (Oregon Health and Science University)
MS Statistician: Min Chen (minchen@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # IN10-01
Title: Comparison of infectious rates among alternative graft sources: single and double cord blood, matched unrelated donor, and mismatched unrelated donor
Study Chair: Karen Ballen (Massachusetts General Hospital)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # LE04-01a
Title: CMV infection and mortality after HCTs
Study Chair: Michael Boeckh (Fred Hutchinson Cancer Center)
MS Statistician: Min Chen (minchen@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

PLANNED STUDIES

Study # IN11-01
Title: Bacterial infections within 45 days after HCT and their effect on long-term survival
Study Chair: Celalettin Ustun (University of Minnesota Medical Center, Fairview) Jo-Anne Young (University of Minnesota Medical Center, Fairview)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
9.12 INTERNATIONAL STUDIES WORKING COMMITTEE

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PRELIMINARY RESULTS

Study # IS09-04
Title: Progress of hematopoietic cell transplantation (HCT) in Latin America according to data reported to the Center for International Blood and Marrow Transplant Research (CIBMTR)

Study Chairs: Michael Eckrich, MD (CIBMTR)

MS Statistician: Marcelo Pasquini (mpasquin@mcw.edu)

PhD Statistician: John Klein (klein@mcw.edu)

Study Status: Manuscript preparation

Purpose: To describe HCT activity in Latin America, compare transplant activity in Latin America with the U.S., and evaluate survival outcomes after transplantation performed in Latin America.

Patients/Methods: Continuous and categorical variables were compared using Kruskal-Wallis test and Chi-square test, respectively. Univariate probability of overall survival was calculated using the Kaplan-Meier estimator and long-rank test for survival curve comparisons. One-year survival per transplant year was plotted to assess changes in overall outcomes.

Results: The main indications for allogeneic HCT were leukemias, severe aplastic anemia, myelodysplasia, and red cell disorders. The most frequent malignant and non-malignant diseases for an allogeneic HCT were chronic myelogenous leukemia and bone marrow failure syndrome, respectively. The most frequent indication for an autologous HCT was lymphoma. The annual number of transplants has generally remained constant, with the exception of chronic myeloid leukemia which decreased. Autologous HCT was more common than allogeneic, except for younger patients. The number of alternative donor transplants increased. The most common graft type for unrelated donor allogeneic transplants in younger patients.
is cord blood and for adults it is peripheral blood. One-year survival rates have generally improved, especially in the last 5 years.

**Conclusion:** Despite a lack of financial resources, HCT in Latin America continues to grow and is doing well with survival rates approaching their North American counterparts. The number of transplant centers reporting transplant activity to the Center for International Blood and Marrow Transplant Research demonstrates an increase over time. As more centers report, it is important to note that no center that reported in a particular decade failed to do so in the subsequent decade. Continued voluntary reporting is critical to establishing the relationship of trends in transplant use for this generally low to average socio-economic set of countries.

**Study # IS10-02**

**Title:** Total body irradiation (TBI) prior to hematopoietic stem cell transplantation (HCT): a pattern of care study

**Study Chairs:** Christopher Barker (Memorial Sloan-Kettering Cancer Center)

**MS Statistician:** Zhenhuan (Kenny) Hu (zhu@mcw.edu)

**PhD Statistician:** John Klein (klein@mcw.edu)

**Study Status:** Manuscript preparation

**Purpose:** To summarize the patterns of TBI as part of HCT conditioning over time and compare them with non-TBI based conditioning, and to describe characteristics of TBI techniques used and whether they changed in the last 15 years.

**Patients/Methods:** Chi-square test and Wilcoxon test were used for the analysis of categorical and continuous variables, respectively. A non-continuity corrected binomial test was used for the subgroup analysis. Frequencies of TBI-based conditioning regimen, shielding usage, total dosage, and dose per fraction were displayed as histograms and scatter-plots.

**Results:** TBI was used in 10% of autologous and 46% of allogeneic HCTs. Among autologous HCT, TBI was most frequently used in patients 10 to 29 years old, chronic lymphocytic leukemia indication, Middle East/Africa region, and countries with a medium Human Development Index (HDI). Among allogeneic HCT, TBI was most frequently used in patients 10 to 39 years old, acute lymphocytic leukemia indication, recipients of unrelated donor grafts, European region and in countries with a very high HDI. Use of TBI-based conditioning decreased in both autologous and allogeneic HCTs. Decrease in TBI use was observed in all continents except in Latin America, and all diseases except for ALL in allogeneic HCTs.

**Conclusion:** Variations in use of TBI were mainly observed by age, regions, HDI, cell sources, and disease indications. Use of TBI changed significantly over time with the general trend of using regimens without TBI with exception of ALL in allogeneic HCTs. This trend is likely a result of availability of less toxic non-TBI conditioning regimens.
STUDIES IN PROGRESS

Study # IS08-01
Title: Hematopoietic cell transplantation activity survey in the Americas: Worldwide Network for Blood & Marrow Transplantation (WBMT) global survey
Study Chairs: Marcelo Pasquini (CIBMTR Milwaukee)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Analysis in progress

Study # IS09-01/MM08-04
Title: Outcomes of autologous hematopoietic cell transplantation in patients with multiple myeloma, comparison of patients from North versus South America
Study Chair: Giuseppe Milone (Ospedale Ferrarotto)
MS Statistician: Baldeep Wirk (Shands HealthCare & University of Florida)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Protocol development

Study # IS09-02
Title: Outcomes comparison of the effect of HLA-matched sibling donor transplantation for severe aplastic anemia across different regions
Study Chair: Rajat Kumar (CancerCare Manitoba/University of Manitoba)
MS Statistician: Zhenhuan (Kenny) Hu (zhu@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Protocol development

Study # IS10-01
Title: Global variation in stem cell transplantation techniques for acute lymphoblastic leukemia and acute myeloid leukemia: a comparative analysis
Study Chair: William A. Wood (University of North Carolina Hospitals)
MS Statistician: Zhenhuan (Kenny) Hu (zhu@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Protocol development
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9.13 LATE EFFECTS & QUALITY OF LIFE WORKING COMMITTEE

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PUBLISHED STUDIES

Study # LE07-03/LE09-02


Purpose: To examine long-term survival in 2-year survivors in the largest cohort ever studied. Allogeneic HCT is curative but is associated with life-threatening complications. Most deaths occur within the first 2 years after transplantation.

Patients/Methods: Records of 10,632 patients worldwide reported to the Center for International Blood and Marrow Transplant Research who were alive and disease free 2 years after receiving a myeloablative allogeneic HCT before 2004 for acute myelogenous or lymphoblastic leukemia, myelodysplastic syndrome, lymphoma, or severe aplastic anemia were reviewed.

Results: Median follow-up was 9 years, and 3,788 patients had been observed for 10 or more years. The probability of being alive 10 years after HCT was 85%. The chief risk factors for late death included older age and chronic graft-versus-host disease (GVHD). For patients who underwent transplantation for malignancy, relapse was the most common cause of death. The greatest risk factor for late relapse was advanced disease at transplantation. Principal risk factors for non-relapse deaths were older age and GVHD. When compared with age, sex, and nationality-matched general population, late deaths remained higher than expected for each disease, with the possible exception of lymphoma, although the relative risk generally receded over time.

Conclusion: The prospect for long-term survival is excellent for 2-year survivors of allogeneic HCT. However, life expectancy remains lower than expected. Performance of HCT
earlier in the course of disease, control of GVHD, enhancement of immune reconstitution, less toxic regimens, and prevention and early treatment of late complications are needed.

PRELIMINARY RESULTS

Study # LE09-01
Title: Second Malignancies after Autologous Hematopoietic Stem Cell Transplantation in Children
Study Chair: Karina Danner-Koptik (The Children’s Memorial Medical Center)
David Jacobsohn (Children’s National Medical Center)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: Ruta Brazauskas (ruta@mcw.edu)
Study Status: Manuscript submitted
Purpose: To evaluate the incidence and risk factors for SMNs (secondary malignant neoplasms) following autologous hematopoietic cell transplantation (AHCT) in children (not well known). AHCT is curative for selected cancers in children. Childhood AHCT survivors can be at risk for late complications, including SMNs.
Patients/Methods: We assembled a cohort of 1487 pediatric AHCT recipients transplanted between 1987 and 2003 to investigate the incidence and risk factors for SMNs. The median age at AHCT was 8 years and median follow-up of survivors was 8 years from AHCT. Primary diagnoses included neuroblastoma (39%), lymphoma (26%), sarcoma (18%), CNS tumors (14%) and Wilms tumor (2%).
Results: SMNs were reported in 35 patients (AML/MDS=13, solid cancers=20, subtype missing=2). The overall cumulative incidence of SMNs at 10-years from AHCT was 2.60%. The corresponding cumulative incidence of secondary AML/MDS was 1.06% and for secondary solid tumors was 1.30%. We found no association between SMNs risk and age, gender, diagnosis, disease status, time since diagnosis, or use of total body irradiation or etoposide as part of conditioning. Overall survival at 5-years from the time of diagnosis of SMNs was 33% (95% CI, 16-52%). When compared to age- and gender-matched general population, AHCT recipients had 25 times higher risks of developing SMNs (95% CI, 17.0-35.0). Risks of SMNs increased with longer follow-up from AHCT.
Conclusion: Although the overall incidence of SMNs among pediatric AHCT recipients is low, they are at considerably increased risk for SMNs compared to the general population and need life-long surveillance for secondary cancers.

STUDIES IN PROGRESS

Study # LE05-01
Title: Incidence of bronchiolitis obliterans after reduced intensity HCT
Study Chair: Shin Mineishi (The University of Michigan)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Supplemental form/data collection
Study # LE06-01
Title: Genetic susceptibility to transformed MDS/AML after autologous HCT in NHL
Study Chair: Timothy Fenske (Medical College of Wisconsin)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: Ruta Brazauskas (ruta@mcw.edu)
Study Status: Analysis in progress

Study # LE07-01
Title: Second cancers after allogeneic non-myeloablative HCT
Study Chairs: Navneet S. Majhail (Masonic Cancer Center University of Minnesota) Olle Ringden (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation) Jonas Mattsson (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # LE09-03a
Title: National survey of transplant physician practice patterns regarding fertility preservation
Study Chair: Alison W. Loren (Abramson Cancer Center University at Pennsylvania Medical Center)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: TBA
Study Status: Analysis in progress

Study # LE09-03b
Title: Review of fertility preservation practice in HSCT patients
Study Chair: Sarita Joshi (Cincinnati Children's Hospital Medical Center)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: TBD
Study Status: Analysis in progress

Study # LE10-01
Title: Avascular necrosis of bone in children and adolescents after HCT
Study Chair: Xiaxin Li (Akron Children's Hospital)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation
Study # LE10-02
Title: Late effects in pediatric HCT recipients with severe aplastic anemia
Study Chairs: David Buchbinder (Children’s Hospital of Orange County)
              Diane J. Nugent (Children’s Hospital of Orange County)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: TBD
Study Status: Analysis in progress

Study # LE11-02
Title: Risk factors for development of secondary central nervous system tumors in survivors of pediatric HCT
Study Chair: Melissa Gabriel (The Children’s Hospital at Westmead)
            Peter Shaw (The Children’s Hospital at Westmead)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # LE99-01
Title: Quality of life in late HCT survivors
Study Chair: John Wingard (Shands HealthCare & University of Florida)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Ongoing

PLANNED STUDIES

Study # LE11-01
Title: Long-term survival following second allogeneic HCT for hematologic malignancies
Study Chair: Christine Duncan (Dana Farber Cancer Institute - Pediatrics)
             Mohamed Sorror (Fred Hutchinson Cancer Research Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
9.14 LYMPHOMA WORKING COMMITTEE

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MS Statistician: Jeanette Carreras, MPH, CIBMTR Milwaukee  
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PRELIMINARY RESULTS

Study # LY04-01
Title: Alternative donor transplantation for non-Hodgkin lymphoma (NHL)
Study Chair: Gregory A. Hale (All Children’s Hospital)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Jennifer Le-Rademacher (jlerade@mcw.edu)
Study Status: Submitted
Purpose: To investigate alternative donor (unrelated or mismatched family) options for adult allogeneic HCT candidates lacking a matched family donor
Patients/Methods: We analyzed the outcomes of 248 (61% male) adult recipients of HCT for NHL from alternative donors after RIC/NMA conditioning reported to the CIBMTR from 1997 to 2004.
Results: High-grade histology, the use of anti-thymocyte globulin (ATG), and chemotherapy resistant disease at HCT were associated with higher risk of treatment failure or lower PFS. Older age, shorter interval from diagnosis to HCT, non-total body irradiation conditioning regimens or T cell depletion and HLA mismatched unrelated donors were associated with lower risk of survival.
Conclusion: Higher grade NHL, use of ATG or T cell depletion and HLA mismatch were associated with inferior outcomes. Lack of an available sibling donor should not be a barrier to allogeneic HCT in the appropriate patient.

Study # LY04-03
Title: Outcomes of autologous versus allogeneic HCT for patients with NHL with pre-existing central nervous system involvement
Study Chair: Richard T. Maziarz (Oregon Health and Science University)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To study the effects of pre-existing CNS involvement as a contraindication for allogeneic HCT for patients with NHL, as shown in other retrospective single institutional and registry analyses.

Patients/Methods: We analyzed the outcomes of 151 (64% male) adult recipients of allogeneic HCT with NHL and CNS involvement identified at any time prior to transplant and compared them to 4,688 patients undergoing allogeneic HCT with no CNS lymphoma involvement during the years 1990-2005.

Results: No statistically significant differences in outcomes non-relapse mortality, relapse, disease-free survival or overall survival were identified between the two groups. Patients with CNS remission (96/151) at time of allogeneic HCT had significantly superior survival compared with those with active CNS disease (55/151) at transplant.

Conclusion: Excellent long-term survival is possible after allogeneic HCT in NHL patients with prior CNS involvement despite adverse baseline prognostic factors. However, best outcomes are in patients who achieve a CNS remission prior to transplant.

Study # LY06-02
Title: Nonmyeloablative allogeneic HCT in patients who experience relapse after autologous HCT for lymphoma
Study Chair: Cesar Freytes (University of Texas Health Science Center)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Submitted
Purpose: To determine the outcome of patients with lymphoma who undergo non-myeloablative (NST) allogeneic HCT after a previous autologous HCT failure.
Patients/Methods: We analyzed 267 patients who underwent NST HCT after an autologous HCT failure performed between 1997 and 2006. Excluded were patients with planned second transplants, patients younger than 21 and cord blood transplants.
Results: Transplant-related mortality at 1 year was 39% and 45%, respectively. Overall survival at 1 and 5 years was 44% and 28%. Progression-free survival was 30% and 19% at 1 and 5 years, respectively. Factors associated with a higher risk of progression or death included poor performance status, time interval between transplants of two years or less, not receiving a total body irradiation-containing regimen and primary induction failure.
Conclusion: NST after autologous HCT failure is associated with substantial TRM but selected patients can attain prolonged remissions.

Study # LY06-03
Title: HLA identical sibling transplantation versus HLA-matched unrelated donor transplantation in patients with follicular lymphoma (FL)
Study Chairs: Harry C. Schouten (Academic Hospital Maastricht)
Anna Sureda (Hospital Santa Creu i Sant Pau)
MS Statistician: Marcelo Pasquini (mpasquin@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To study allogeneic HCT as a potential cure in FL, though wide adoption of this treatment is limited by its toxicity and donor availability and improvements in HLA-matching have improved the safety of unrelated donor (URD) HCT.

Patients/Methods: We compared the outcomes of 702 recipients of allogeneic HCT for FL (198 URD and 504 sibling donors) from 171 centers worldwide reporting to the CIBMTR or EBMT between 1997 and 2005.

Results: Significant risk factors associated with worse outcomes included poor performance status at transplantation and extensive pre-transplant therapy (> 4 lines of therapy or prior autologous HCT). Additionally, in vivo T cell depletion was associated with a higher risk of relapse/progression and treatment failure.

Conclusion: This study shows that URD HCTs are performed later in the treatment course for FL, in higher risk patients, most commonly with RIC, and are associated with worse PFS and OS compared to HLA identical sibling HCT.

Study # LY06-05
Title: Comparison of autologous versus allogeneic HCT for T cell non-Hodgkin lymphoma (NHL)

Study Chairs: Sonali Smith (University of Chicago)
Linda J. Burns (Masonic Cancer Center University of Minnesota)
Koen van Besien (Weill Cornell Medical College)

MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: Jennifer Le-Rademacher (jlerade@mcw.edu)
Study Status: Manuscript preparation

Purpose: To analyze post-transplant outcomes for T cell lymphomas (T-NHL). T-NHLs are aggressive, heterogeneous malignancies, marked by chemoresistance with fewer than one-third of patients achieving disease control with non-transplant therapies. There are no large comparative studies of autologous HCT and allogeneic HCT in T-NHL.

Patients/Methods: We analyzed post-transplant outcomes of 241 recipients (≤60 yrs) of autologous HCT (n=115) and allogeneic HCT (n=126, 76 matched siblings) for T-NHL from 1996-2006 reported to the CIBMTR.

Results: In multivariate analysis, allogeneic HCT and more pre-transplant chemotherapy regimens were associated with a higher risk of transplant-related mortality (TRM). Progression-free and overall survival rates were similar after autologous HCT or allogeneic HCT, with a greater number of prior chemotherapy regimens and chemotherapy resistance impacting survival outcomes adversely.

Conclusion: Allogeneic HCT is an effective strategy for high-risk patients, albeit with significant TRM. Greater numbers of chemotherapy regimens prior to transplant adversely impacted both TRM and survival, suggesting that HCT should be considered earlier in the disease course.

Study # LY06-06
Title: A prognostic model for prolonged event-free survival after autologous or allogeneic transplantation for relapsed and refractory Hodgkin lymphoma (HL)

Study Chairs: Philip L. McCarthy (Roswell Park Cancer Institute)
Theresa Hahn (Roswell Park Cancer Institute)

**MS Statistician:** Jeanette Carreras (jcarrera@mcw.edu)

**PhD Statistician:** Mei-Jie Zhang (meijie@mcw.edu)

**Study Status:** Manuscript preparation

**Purpose:** To compare prognostic risk score models for HL patients receiving allogeneic HCT, with the aim to predict post-transplant outcomes based on factors measured at the time of HCT.

**Patients/Methods:** We analyzed a cohort of 728 relapsed/refractory HL patients receiving an AHCT between 1996 and 2007 and reported to CIBMTR by 162 centers, to develop a prognostic model for PFS based on pre-AHCT factors. A random subset of patients was used for model development (n=337) and validation (n= 391).

**Results:** Multivariate analysis determined the following significant factors and scores: >3 prior chemotherapy regimens (score=2); KPS<90 (score=2); chemo-resistant disease at AHCT (score=1); extranodal involvement at AHCT (score=1). Risk groups based on the prognostic score sum were: High (score = 4-6); Intermediate (score = 1-3); and Low, (score = 0) risk for progression or death.

**Conclusion:** These risk groups discriminate patients with good post-AHCT outcomes and those who may benefit from other therapies, such as allogeneic HCT. Prospective evaluation is needed.

**Study # LY07-02**

**Title:** Transplantation outcomes in the mycosis fungoides and Sézary syndrome patients

**Study Chair:** Mary Jo Lechowicz (Emory University)

**MS Statistician:** Jeanette Carreras (jcarrera@mcw.edu)

**PhD Statistician:** Mei-Jie Zhang (meijie@mcw.edu)

**Study Status:** Manuscript preparation

**Purpose:** To report the outcomes for patients with advanced stage cutaneous T cell lymphoma (CTCL), who have a poor prognosis with standard therapy. Allogeneic hematopoietic cell transplantation (AHCT) possibly is curative. Published experience with allogeneic HCT for CTCL is modest.

**Patients/Methods:** We report the outcomes of a large cohort of CTCL patients receiving AHCT (N=133) reported to the CIBMTR from 2000-2009. The majority of patients 129 (97%) were diagnosed with Mycosis Fungoides or Sezary Syndrome.

**Results:** Overall mortality at 30 and 100 days was 7% and 16%, respectively. Overall survival at 6 months and 2 years was 75% (95% CI 67-82%) and 44% (95% CI 34-53%), respectively. Treatment related mortality (TRM) at 6 months and 2 years was 10% (95% CI 5-17%). Progression free survival at 6 months was 46% (95% CI 36-57%) and at 2 years 32% (95% CI 23-42%).

**Conclusion:** This very large series of allogeneic HCT demonstrates feasibility in patients with advanced CTCL with a low treatment-related mortality and long-term progression-free survival in approximately one-third of patients.

**Study # LY08-01**

**Title:** Outcomes of allogeneic and autologous HCT for Burkitt and Burkitt-like lymphoma

**Study Chair:** James L. Gajewski (Oregon Health and Science University)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To report the outcomes of 241 recipients of HCT for Burkitt lymphoma (BL). BL is an aggressive B cell lymphoma primarily affecting children and young adults, and is characterized by the highest doubling time of any tumor.

Patients/Methods: We report the outcomes of 241 recipients of HCT for BL from 1985-2007 reported to the CIBMTR. Five patients received syngeneic twin grafts, 113 patients received an autologous HCT, 80 patients received HLA identical sibling allogeneic HCT (SIB), and 48 patients received mismatched related or unrelated donor (UNR/MM) allogeneic HCT.

Results: Treatment-related mortality was higher in allogeneic HCT recipients. For autologous HCT, 5-year progression-free survival (PFS) was 48% (range 39-58, 78% for those in first complete remission (CR1), versus 27% for disease beyond CR1 (p<0.001). For allogeneic HCT, 5-year PFS was 50% for those in CR1 versus 19% for disease beyond CR1 (p=0.001). Five-year PFS was 30% (range 20-41 for SIB and 22% (range 12-35) for UNR/MM.

Conclusion: Approximately one-fifth of advanced BL patients receiving allogeneic HCT beyond CR1 had long term disease free survival.

Study # LY08-02
Title: Outcomes of patients with mantle cell lymphoma treated with autologous versus allogeneic HCT
Study Chair: Timothy Fenske (Medical College of Wisconsin)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To analyze the lack of consensus regarding the optimal transplant strategy for mantle cell lymphoma.

Patients/Methods: We studied the outcome of 640 patients with MCL who underwent a first autologous (Auto, n=433) or reduced-intensity allogeneic (Allo, n=207) HCT between 1996 and 2007. We classified patients as “Early” MCL in first complete or partial remission.

Results: For Early MCL, 1 year treatment-related mortality (TRM) was significantly higher in the Allo group (25% versus 4%, p=0.001), while 5 year progression/relapse was lower in the Allo group (16% versus 32%, p=0.012). Five-year overall survival (OS) was similar (62% Allo versus 61% Auto, p=0.941). For Late MCL, 1-year TRM was again significantly higher in the Allo group (18% versus 9%, p=0.036), with progression/relapse similar at 5 years for Allo and Auto (38% versus 49%, p=0.148). Five-year OS was also similar (32% in Allo versus 44% in Auto, p=0.193).

Conclusion: We conclude that, when applied early in the disease course, both autologous and allogeneic HCT result in favorable long-term survival. For more heavily pre-treated patients, long-term survival is less favorable for both allogeneic and autologous HCT, although a subset of patients does appear to benefit from transplant even in this setting.
Study # LY08-03
Title: Comparison of reduced and standard conditioning in allogeneic transplantation in patients with B cell non-Hodgkin lymphoma

Study Chair: Ulrike Bacher (UKE Hamburg, Klinik und Poliklinik für Stammzelltransplantation)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Jennifer LeRademacher (jlerade@mcw.edu)
Study Status: Manuscript preparation

Purpose: To investigate allogeneic HCT in patients with refractory/relapsed diffuse large cell lymphoma (DLBCL). The optimal conditioning strategies for these patients are unknown.

Patients/Methods: We analyzed outcomes for 396 adults receiving related/unrelated alloHCT for DLBCL following myeloablative (MAC, n=165), reduced intensity (RIC, n=143) or non-myeloablative conditioning (NMC, n=88) between 2000-2009 and reported to the CIBMTR.

Results: Day+100 TRM was higher for MAC than RIC and NMC (32% vs. 24% vs. 17%; p=0.029). Lymphoma relapse/progression at 5 years was lower for MAC vs. RIC and NMC (p=0.031). PFS at 5-years (MAC: 18%; RIC: 15%; NMC: 25%) and OS (MAC: 18%; RIC 20%; NMC: 26%) did not differ significantly. In multivariate analysis NMC was associated with lower TRM, relapse/progression with NMC.

Conclusion: TRM was lower with reduced-intensity/non-myeloablative regimens, but relapse/progression in these settings was concordantly higher. Further studies should clarify the optimal conditioning strategies for advanced DLBCL aiming to further reduce TRM.

STUDIES IN PROGRESS

Study # LY09-01
Title: Clinical outcomes of HCT in patients with diffuse large B cell lymphoma transformed from CLL, follicular lymphoma or Waldenstrom Macroglobulinemia

Study Chair: Baldeep Wirk (Shands HealthCare & University of Florida)
Cesar Freytes (UTHSC Veterans Health Care System)
Linda Burns (University of Minnesota Medical Center, Fairview)

MS Statistician: Wael Saber (wsaber@mcw.edu)
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Study Status: Protocol development

Study # LY10-01
Title: Outcomes of allogeneic HCT for patients with chemorefractory aggressive NHL

Study Chair: Mehdi Hamadani (West Virginia University Hospitals, Inc.)
Hillard Lazarus (University Hospitals Case Medical Center)

MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
Study # LY10-02
Title: Umbilical cord blood allogeneic HCT for patients with lymphoma
Study Chair: Veronika Bachanova (University of Minnesota Medical Center, Fairview)
Claudio Brunstein (University of Minnesota Medical Center, Fairview)
Linda Burns (University of Minnesota Medical Center, Fairview)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # LY10-03
Title: Allogeneic hematopoietic stem cell transplant for lymphoblastic lymphoma
Study Chair: Allen Chen (Johns Hopkins University Sidney Kimmel Cancer Center)
Richard Maziarz (Oregon Health and Science University)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # LY11-01
Title: High-dose therapy followed by autologous stem cell transplant for patients with Primary Central Nervous System Lymphoma: Impact of the conditioning regimen
Study Chair: Silvia Montoto (Royal London Hospital Whitechapel, St. Bartholomew’s)
Nishitha Reddy (Vanderbilt University Medical Center)
Galit Perets Avraham (Oregon Health and Science University)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received

Study # LY11-02
Title: Development of a prognostic scoring system to predict relapse of diffuse large B cell lymphoma after allogeneic HCT
Study Chair: Rachel Salit (National Institutes of Health - MUD Program)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Draft protocol received
9.15 NON-MALIGNANT MARROW DISORDERS WORKING COMMITTEE

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PUBLISHED STUDIES

Study #AA07-03

Purpose: To compare outcomes of PBPC and BM transplantation for severe aplastic anemia (SAA). Outcome after unrelated donor bone marrow (BM) transplantation for (SAA) has improved, with survival rates now approximately 75%. There is increasing use of peripheral blood stem and progenitor cells (PBPCs) instead of BM as a graft source.

Patients/Methods: We studied 296 patients receiving either BM (n=225) or PBPC (n=71) from unrelated donors matched at human leukocyte antigen-A, -B, -C, -DRB1.

Results: Hematopoietic recovery was similar after PBPC and BM transplantation. Grade 2 to 4 acute graft-versus-host disease risks were higher after transplantation of PBPC compared with BM (hazard ratio= 1.68, P=.02; 48% vs 31%). Chronic graft-versus-host disease risks were not significantly different after adjusting for age at transplantation (hazard ratio= 1.39, P=.14). Mortality risks, independent of age, were higher after PBPC compared with BM transplantation (hazard ratio = 1.62, P=.04; 76% vs 61%).

Conclusion: These data indicate that BM is the preferred graft source for unrelated donor transplantation in SAA.

Study # AA08-02
Purpose: To assess the efficacy of unrelated cord blood (CB) transplantation for thalassemia and sickle cell disease.

Patients/Methods: Thirty-five patients had thalassemia and 16, sickle cell disease. Donor-recipient pairs were matched at HLA-A and -B (antigen level) and DRB1 (allele level) in 7 or mismatched at 1 (n=18), 2 (n= 25), or 3 loci (n=1). Transplant conditioning was myeloablate (n=39) or reduced intensity (n=12).

Results: Overall survival and disease-free survival (DFS) were 62% and 21% for thalassemia and 94% and 50% for sickle cell disease (SCD), respectively. In multivariate analysis, engraftment rate (hazard ratio [HR] 2.2, \( p=0.05 \)) and DFS (HR 0.4, \( p=0.01 \)) were higher with CB cell dose >5 x 10^7/kg. The 2-year probability of DFS was 45% in patients who received grafts with cell dose >5 x 10^7/kg and 13% with lower cell dose. Primary graft failure was the predominant cause of treatment failure.

Conclusion: These results suggest that CB transplantation is associated with high rates of graft failure; infused cell dose >5x10^7/kg improves DFS.

PRELIMINARY RESULTS

Study # AA08-01
Title: The role of the HLA DRB1 antigen, DR15, in patients with severe aplastic anemia undergoing HLA-matched HCT

Study Chair: Minoo Battiwalla (National Heart Lung and Blood Institute - NIH)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Tao Wang (taowang@mcw.edu)
Study Status: Submitted

Purpose: To investigate the hypothesis that the presence of DR15 could impact clinical outcomes after allogeneic stem cell transplant in severe aplastic anemia

Patients/Methods: We studied 1,422 patients with SAA (30% were DR15+) who were transplanted in 1990-2006 to determine whether the presence of HLA DR15 affected clinical outcomes.

Results: Proportions of patients who had received prior immunosuppressive therapy were similar among those with and without DR15. In multivariate analysis, after adjustment for significant factors, neutrophil recovery at day 28 (odds ratio [OR] 1.02, \( p=0.905 \)), platelet recovery at day 100 (OR 0.95, \( p=0.776 \)), secondary graft failure at 2 years (OR 0.66, \( p=0.123 \)), acute (hazard ratio [HR] 0.88, \( p=0.345 \)) and chronic (HR 1.09, \( p=0.519 \)) graft-versus-host disease and overall mortality (HR 1.22, \( p=0.102 \)) risks were similar in patients with and without DR15.

Conclusion: The presence of DR15 did not impact clinical outcomes after HLA-identical sibling transplantation for SAA.

Study # AA09-01
Title: Outcomes of allogeneic stem cell transplantation in patients with Fanconi anemia who present with MDS, clonal abnormalities, and/or leukemia

Study Chair: Mary Eapen (CIBMTR Milwaukee)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: Jennifer Le-Rademacher (jlerade@mcw.edu)
Study Status: Manuscript preparation

Purpose: To study outcomes after allogeneic HCT in persons with Fanconi anemia with pretransplant cytogenetic abnormalities (CA), myelodysplastic syndrome (MDS) or acute leukemia (AL)

Patients/Methods: We studied 113 FA patients with CA (n=54), MDS (n=45), or AL (n=14) who received allogeneic HCT. Pretransplant conditioning regimens were radiation-based in 67 patients and chemotherapy-based in 46 patients. The donor source was bone marrow/peripheral blood (matched related, n=82; unrelated, n=16) or cord blood (related, n=2; unrelated, n=13). Univariate analyses were performed.

Results: Survival probabilities were 64%, 58%, and 55% at 1, 3, and 5 years, respectively. Age at transplant was the only variable significantly correlated with 5-year OS (≤14 years vs. >14 years, 69% vs. 39%, respectively; p=0.001). When analysis was restricted to recipients of related donor HCT (n=82), age again correlated with 5-year survival (≤14 years vs. >14 years, 78% vs. 34%, respectively; p<0.001); additionally, patients with CA pre-transplant had better survival than those with MDS or AL (5-year OS: 67% vs. 43%, p=0.03).

Conclusion: FA patients transplanted with CA, MDS, or AL have a reasonable survival rates. Survival rates are better in patients aged 14 years or younger.

STUDIES IN PROGRESS

Study # AA09-02
Title: Outcomes of HCT in patients with dyskeratosis congenita
Study Chair: Shahinaz Gadalla (National Cancer Institute)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # AA10-02
Title: Outcomes after allogeneic HCT for patients who developed AML/MDS after immunosuppressive therapy for severe aplastic anemia
Study Chair: Joachim Deeg (Fred Hutchinson Cancer Research Center)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: TBD
Study Status: Supplemental form/data collection

Study # AA11-02
Title: Chronic blood transfusions therapy compared to HCT in children with Sickle Cell Disease: a cost-utility analysis
Study Chair: Robert Sidonio (Vanderbilt University Medical Center)
MS Statistician: Jeanette Carreras (jcarrera@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
PLANNED STUDIES

Study # AA11-01
Title: Durable engraftment and correction of hematological defects in children with congenital amegakaryocytic thrombocytopenia following myeloablative umbilical cord blood transplantation
Study Chair: Kris Mahadeo (Duke University Medical Center; Pediatric Blood and Marrow Transplant)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
9.16 PEDIATRIC CANCER WORKING COMMITTEE

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PUBLISHED STUDIES

Study # PC05-02

Purpose: To describe the outcomes and to determine the prognostic factors that are associated with the outcomes of children who received unrelated donor HCT for ALL in third remission

Patients/Methods: We observed 155 patients aged 1-18 years old who received an unrelated donor HCT for ALL in third complete remission. Patient, disease, transplant-related characteristics, and the outcomes were described. Cox proportional hazard regression models were fit to identify the risk factors that are associated with the outcomes.

Results: The 5-year estimates of leukemia-free survival (LFS), relapse, and non-relapse mortality were 30%, 25%, and 45%, respectively. In multivariate analysis, the only risk factor associated with relapse was interval between first and second relapse. Second relapses that occurred late, >26 months from first relapse, were associated with lower risk for post-HCT relapse compared to second relapses ≤26 months (RR 0.4; p=0.01). Relapse risks were lowest when late second relapse was preceded by late first relapse (>36 months from diagnosis) as shown by a 3-year relapse rate of 9%, p=0.0009.

Conclusion: Long-term LFS can be achieved for children with ALL in third remission using unrelated donor HCT, especially when the second relapse occurred late.
PRELIMINARY RESULTS

Study # PC10-05
Title: Determine the impact of anti-T cell antibody in conditioning regimens in patients aged less than 18 years with ALL
Study Chair: Paul Veys (Great Ormond Street Hospital for Children)
             Robert Wynn (Royal Manchester Children’s Hospital)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: Kwang Ahn (kwooahn@mcw.edu)
Study Status: Manuscript preparation
Purpose: To determine whether anti-T cell antibody infusion (ATG or alemtuzumab), which lowers GVHD, abrogates any of the therapeutic benefits of unrelated donor transplantation
Patients/Methods: We analyzed 715 patients with ALL, aged <18 years. Using Cox regression analysis, we compared outcomes after transplantation of T cell replete grafts (n=392) to that after infusions of ATG (n=191) or alemtuzumab (n=132).
Results: Compared to transplantation of T cell replete grafts, grade 2-4 acute and chronic GVHD rates were lower with ATG-containing (RR 0.66, p=0.005; RR 0.55, p<0.0001,) and alemtuzumab-containing (RR 0.09, p<0.003; RR 0.21, p<0.0001) regimens. In all patients, acute GVHD was higher after mismatched transplants (RR 1.35, p=0.007) and chronic GVHD higher in patients aged >10 years (RR 1.29, p=0.04) and PBPC transplants (RR 2.08, p<0.0001). Higher chronic GVHD after PBPC grafts led to higher mortality. Mortality was also higher after mismatched transplants. However, there were no differences in risks of transplant-related mortality, relapse, leukemia-free and overall survival after ATG-containing, alemtuzumab-containing and T cell replete transplants.
Conclusion: These results suggest that in children: 1) outcomes after in vivo T cell depletion are similar to that after T cell replete transplants, 2) BM is the preferred graft and 3) transplantations matched at HLA-A, -B, -C, -DRB1 and in CR have the best outcome.

STUDIES IN PROGRESS

Study # PC09-01
Title: Outcomes in children with T cell ALL following allogeneic HCT
Study Chair: Michael Burke (University of Minnesota Medical Center, Fairview)
             Robert Wynn (Royal Manchester Children’s Hospital)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
Study # PC10-01
Title: HCT for children with AML using reduced intensity conditioning
Study Chair: Menachem Bitan (Tel-Aviv Sourasky Medical Center)
Parinda Mehta (Cincinnati Children’s Hospital Medical Center)
Sonata Jodele (Cincinnati Children’s Hospital Medical Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # PC10-02
Title: Outcomes of HCT for the treatment of leukemia in children with Down syndrome
Study Chair: Johann (Hans) Hitzler (Hospital for Sick Children)
John J. Doyle (Hospital for Sick Children)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # PC10-03
Title: Transplantation in children with hypodiploid ALL
Study Chair: Parinda Mehta (Cincinnati Children’s Hospital Medical Center)
Sonata Jodele (Cincinnati Children’s Hospital Medical Center)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # PC10-04
Title: Treatment outcomes for children with poor risk AML
Study Chair: Michael Kelly (The Floating Hospital for Children, Tufts Medical Center)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: Todd Alonzo (talonzo@childrensoncologygroup.org)
Study Status: Analysis in progress

Study # PC11-01
Title: Development of a prognostic scoring system to predict relapse of pediatric ALL after allogeneic HCT
Study Chair: Nirali Shah (National Cancer Institute - NIH)
Michael Pulsipher (Primary Children’s Medical Center)
Peter Bader (University Children’s Hospital, Germany)
Alan S. Wayne (National Cancer Institute - NIH)
MS Statistician: Wensheng (Vincent) He (vhe@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
Study # PC11-02
Title: Development of a prognostic scoring system to predict relapse of pediatric AML and MDS after allogeneic HCT
Study Chair: Nirali Shah (National Cancer Institute - NIH)
MS Statistician: David Jacobsohn (Children’s National Medical Center)
Charlote Niemeyer (University of Freiberg, Germany)
Terry Fry (National Cancer Institute - NIH)
PhD Statistician: TBD
Study Status: Draft protocol received

PLANNED STUDIES

Study # PC09-02
Title: Does the intensity of prior chemotherapy affect transplant outcomes for children with ALL who receive an allogeneic HCT in second complete remission?
Study Chair: Bruce Camitta (Children’s Hospital of Wisconsin)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
9.17 PLASMA CELL DISORDERS WORKING COMMITTEE

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PUBLISHED STUDIES

Study # MM02-03/MM03-01

Purpose: To study non-myeloablative (NMA) conditioning and reduced-intensity conditioning (RIC) to reduce the risk of transplant-related mortality (TRM). These conditioning regimens depend on a graft-versus-myeloma effect.

Patients/Methods: We analyzed the outcomes of 177 HLA-identical sibling HCT between 1997 and 2005 following NMA (n=120) or RIC (n=57). Median age was 50 years and 62% were Durie-Salmon Stage III.

Results: Rate of acute GVHD (grades I-IV) was 42% at 100 days and of chronic GVHD at 3 years was 55%. At 3 years, TRM, relapse, PFS and OS were 21%, 44%, 35% and 56%, respectively. Multivariate analysis showed that acute GVHD was associated with an increased risk of TRM (relative risk [RR]=2.38, p=0.018). Chronic GVHD significantly decreased relapse in the non-immunoglobulin G myeloma subgroup (RR=0.11, p=0.004). PFS was improved in patients with chronic GVHD (RR=0.60, p=0.049).

Conclusion: Chronic GVHD decreased relapse and improved PFS, suggesting a beneficial graft-versus-myeloma effect following NMA and RIC.

Study # MM05-02

**Purpose:**
To determine whether obesity affects outcomes of high-dose autologous HCT using a melphalan-based conditioning for patients with MM.

**Patients/Methods:**
1,087 patients were identified who received high-dose melphalan, with or without total body irradiation (TBI), as part of initial therapy. Patients were categorized by body mass index (BMI) as normal, overweight, obese, or severely obese. Overall survival and progression-free survival were analyzed.

**Results:**
There was no clear effect of BMI on outcomes among patients receiving melphalan alone. In the subgroup of patients who received TBI as part of conditioning, higher BMI was associated with longer progression-free survival.

**Conclusion:**
The reason for restricting this effect to TBI-containing conditioning regimens requires further investigation. The current common strategy of reducing melphalan doses (i.e. calculating based on ideal or adjusted body weight) does not appear to impair outcomes for obese patients. Obesity should not exclude patients from consideration of autologous HCT.

**Study # MM08-01**

**Journal Citation:**

**Purpose:**
To describe the demographics of patients undergoing allogeneic stem cell transplantation for myeloma and the trends over time and to analyze the changes over time in the disease characteristics among patients undergoing allogeneic stem cell transplantation for myeloma

**Patients/Methods:**
We observed 1,211 patients, including 346 during 1989-1994, 285 during 1995-2000, and 580 during 2001-2005. Outcomes of patients who underwent alloHCT in difference period were analyzed and compared.

**Results:**
There was decreasing use of myeloablative regimens and marrow grafts over time (96%, 87%, and 24% for myeloablative regimens and 99%, 62%, and 13% for marrow grafts, respectively). Increasing number of patients in the later cohorts received an auto-HCT prior to alloHCT and the proportion of unrelated alloHCT increased over time (5% vs. 21% vs. 33%). Median survival increased over the time periods and the TRM decreased in the last period.

**Conclusion:**
A clear trend towards reduced intensity conditioning, unrelated donor HCT and use of PBSC grafts was noted. While the TRM has decreased in the last cohort, this did not translate into improvement in overall survival because of increased risk of relapse.
Study # MM09-01


Purpose: To determine the potential for long-term disease control using HCT in primary plasma cell leukemia (PPCL).

Patients/Methods: A retrospective review of 160 patients with PPCL who received autologous (n=107), allogeneic (n=52) or syngeneic (n=1) HCT and were reported to the CIBMTR between 1995 and 2006 was performed.

Results: The relapse rate at 3 years was 29% (95% confidence interval [CI], 14%-48%) in the allogeneic group and 24% (95% CI, 4%-54%) in the autologous group. Non-relapse mortality at 3 years was 40% (95% CI, 23%-59%) in the allogeneic group and 11% (95% CI, 0-36%) in the autologous group. Progression-free survival at 3 years was 31% (95% CI, 14%-50%) in the allogeneic group and 65% (95% CI, 32%-91%) in the autologous group; overall survival was 46% (95% CI, 27%-65%) in the allogeneic group and 70% (95% CI, 40%-93%) in the autologous group.

Conclusion: The remarkable overall survival of patients after autologous HCT in this analysis is noteworthy and indicates that autologous HCT is a safe and acceptable treatment option for patients with PPCL.

PRELIMINARY RESULTS

Study # MM02-01

Title: Comparison of second autologous transplant versus related or unrelated non-myoeloblative allogeneic HCT in patients with multiple myeloma who relapse after autologous transplantation

Study Chair: Cesar Freytes (University of Texas Health Science Center at San Antonio)
David Vesole (Hackensack University Medical Center)

MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: Jennifer LeRademacher (jlerade@mcw.edu)

Study Status: Manuscript preparation

Purpose: To compare the outcome of second autologous transplant and related/unrelated non-myoeloblative allogeneic HCT for patients with relapse MM after first auto transplant.

Patients/Methods: We studied 289 patients, including 137 patients with autologous HCT and 152 patients with allogeneic HCT. The outcomes between these two groups were compared by proportional hazards models.

Results: AlloHCT recipients were significantly younger. The groups were similar in Karnofsky performance score and gender. Conditioning regimens differed between two groups. Time from 1st to 2nd transplant was significantly shorter for the alloHCT cohort. Acute graft-versus-host disease was 35% at 60 days while chronic GVHD was 44% at 36 months for alloHCT group.
Conclusion: Patients with MM who underwent NST/RIC alloHCT after AHCT failure experienced higher TRM and lower probability of survival compared with those who received second AHCT. Because genetic risk data were not available for these patients, we cannot exclude the possibility that the alloHCT population was a higher risk population. Despite this limitation, our data demonstrate that the value of alloHCT after relapse from prior AHCT is limited.

Study # MM05-01
Title: Outcome of patients with Immunoglobulin D and Immunoglobulin M Multiple Myeloma undergoing autologous HCTs: A retrospective CIBMTR study
Study Chair: Donna Reece (Princess Margaret Hospital, University of Toronto)
David Vesole (Hackensack University Medical Center)
MS Statistician: Smriti Shrestha (sshresth@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: In press
Purpose: To analyze the outcomes of patients who underwent autologous transplant for IgD and IgM multiple myeloma
Patients/Methods: We identified 47 patients with IgD and IgM Multiple Myeloma. Due to small sample size for this rare disease, descriptive statistics and univariate analysis were used to summarize the patients-, disease-, transplant-related characteristics.
Results: The relapse rate at 1 year is 79% for IgD and 71% for IgM; at 3 years, it is 59% for IgD and 32% for IgM. The non-relapse mortality at 3 years was 3% for IgD and 21% for IgM. Progression-free survival at 1 year is 79% for IgD and 71% for IgM; at 3 years, it is 38% for IgD and 47% for IgM. Overall survival rates are similar for both groups. At 1 year, overall survival for IgD is 87% and IgM is 91%. At 3 years, overall survival is 69% for IgD and 68% for IgM.
Conclusion: Although formal statistical analysis was limited by the small sample size, results were similar to those for IgG and IgM patients undergoing autologous stem cell transplant (ASCT) during the same time period. Similarly, the incidence of transplant-related mortality and relapse/progression of myeloma were comparable for all these subtypes. This study supports the use of ASCT in eligible patients with these uncommon immunological subtypes.

Study # MM08-02
Title: HLA specificities and predisposition to the development of multiple myeloma
Study Chair: Meral Beksac (Ankara University Faculty of Medicine)
MS Statistician: Peigang Li (peigang@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Manuscript preparation
Purpose: To compare the HLA-A,-B and -DRB1 2-digit allele and haplotype frequencies of patients with MM to the normal population.
Patients/Methods: HLA-A, B and DRB1 allele frequencies among 1,803 U.S. Caucasian patients were compared to A-B-DRB1 frequencies in HLA-typed healthy U.S. Caucasian controls (N= 433838) from the NMDP database.
Results: B*44 was observed less frequently among the patients (a protective effect) frequency (f): 0.126 versus 0.145 (p= 0.005, Odds Ratio (OR): 0.848). HLA-B*07 was
more common (a predisposing effect) among patients (OR: 1.135, p=0.047). Similar predisposing and protective haplotype associations were observed with A*02-B*07-DRB1*04 and A*02-B*44-DRB1*04 respectively.

Conclusion: A protective effect was observed on the risk of developing MM with B*44 and a predisposing effect with B*07. Similar predisposing and protective haplotype associations were observed with A*02-B*07-DRB1*04 and A*02-B*44-DRB1*04 respectively. A revised analysis using a gender-matched control group repeated the previous findings confirming the impact of B*44.

Study # MM09-02
Title: Outcomes of salvage second autologous HCT in relapsed multiple myeloma after first autologous transplant

Study Chairs: Ayman Saad (Medical College of Wisconsin)
David Vesole (Hackensack University Medical Center)
Laura Michaelis (Loyola University Medical Center)
Jorge Vela (Hospital Especialidades CMN La RAZA IMSS)

MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: Jennifer LeRademacher (jlerade@mcw.edu)

Study Status: Manuscript preparation

Purpose: To analyze the outcomes of second salvage autologous HCT for patients who relapse from first autologous HCT for Multiple Myeloma.

Patients/Methods: We identified 187 patients with second AHCT as salvage for MM relapsing after a prior AHCT. Planned tandem AHCT and AHCT2 within 6 months after AHCT1 were excluded. Outcomes were analyzed and multivariate analyses were performed using proportional hazards models.

Results: Median follow up after AHCT2 was 47 months (range: 3-97). The proportion of IgG, IgA and free light chain subtypes was 48, 20 and 18% respectively. Durie Salmon stage at diagnosis was stage I (6%), II (21%), III (59%) respectively. Conditioning regimen was single agent melphalan was in 78% and 84% for AHCT1 and AHCT2 respectively. At the time of AHCT2, 60% were chemotherapy resistant compared with 80% patients with sensitive disease prior to AHCT1.

Conclusion: AHCT2 can be safely performed in relapsed MM with the best outcomes observed in later relapses (>36 months from AHCT1). OS has improved since 2004, but NRM, relapse and PFS after AHCT2 were not impacted by year of AHCT2. Thus the improved OS likely reflects greater availability of novel agents for post AHCT progression.
### STUDIES IN PROGRESS

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<th>Study #</th>
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<td>Impact of pre-transplant therapy and depth of disease response prior to autologous peripheral blood stem cell transplantation in multiple myeloma</td>
<td>Ravi Vij (Washington University School of Medicine)</td>
<td>Peigang Li (<a href="mailto:peigang@mcw.edu">peigang@mcw.edu</a>)</td>
<td>Mei-Jie Zhang (<a href="mailto:meijie@mcw.edu">meijie@mcw.edu</a>)</td>
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<td>Comparison of outcomes after initial upfront autologous PBSC transplant with delayed initial autologous PBSC transplant in multiple myeloma patients</td>
<td>Leona Holmberg (Fred Hutchinson Cancer Center)</td>
<td>Xiaobo (Tony) Zhong (<a href="mailto:xzhong@mcw.edu">xzhong@mcw.edu</a>)</td>
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<td>Long term complications after HCT for multiple myeloma</td>
<td>Paulette Mehta (University of Arkansas for Medical Sciences/CAVHS)</td>
<td>Xiaobo (Tony) Zhong (<a href="mailto:xzhong@mcw.edu">xzhong@mcw.edu</a>)</td>
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<td>The impact of pre-transplant co-morbidities on the outcomes of autologous HCT in multiple myeloma</td>
<td>Anuj Mahindra (Massachusetts General Hospital)</td>
<td>Xiaobo (Tony) Zhong (<a href="mailto:xzhong@mcw.edu">xzhong@mcw.edu</a>)</td>
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<td>Do patients above 65 years old attain the same benefit from an autologous progenitor cell transplant as younger patients?</td>
<td>Manisha Kukreja (Temple University Medical Center)</td>
<td>Xiaobo (Tony) Zhong (<a href="mailto:xzhong@mcw.edu">xzhong@mcw.edu</a>)</td>
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Study # MM11-02
Title: Waldenstrom's Macroglobulinemia: Retrospective analysis with HCT
Study Chair: Robert Cornell (Medical College of Wisconsin)
           Veronika Bachanova (University of Minnesota Medical Center, Fairview)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

Study # MM11-03
Title: Outcomes of HCT for Amyloidosis
Study Chair: Leena Maramattom (Medical College of Wisconsin)
           Baldeep Wirk (Shands HealthCare & University of Florida)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # MM11-04
Title: High Dose Intravenous Busulfan and Melphalan Followed by Bortezomib vs High Dose Melphalan as Conditioning Regimen for Autologous HCT for Multiple Myeloma patients
Study Chair: Tulio Rodriguez (Loyola University Medical Center)
MS Statistician: Xiaobo (Tony) Zhong (xzhong@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
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9.18 REGIMEN-RELATED TOXICITY & SUPPORTIVE CARE WORKING COMMITTEE

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Sandra Tomany-Korman, MS, CIBMTR Milwaukee
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PUBLISHED STUDIES

Study # D98-70


Purpose: To compare the clinical outcomes and toxicities of patients who received Cy/TBI regimens with those receiving BuCy regimens for unrelated HCT in patients with AML, CML, and MDS.

Patients/methods: Clinical outcomes were compared in 1,593 patients with AML, MDS, and CML who received myeloablative regimens of either BuCy, standard dose Cy/TBI (1,000-1,260 cGy) or high-dose Cy/TBI (1,320-1,500 cGy) prior to a T cell-replete unrelated donor HCT facilitated by the NMDP between 1991 and 1999.

Results: Patients who received high-dose Cy/TBI regimens were slightly younger, more likely to receive a mismatched transplant, and more likely to have intermediate or advanced disease compared to patients in the BuCy or standard dose TBI group. The rate of neutrophil and platelet engraftment was significantly higher in the standard dose CY/TBI group, compared to the high dose Cy/TBI or Bu/Cy group. The risk of developing grade II-IV aGVHD was also significantly higher among patients who received TBI-based regimens compared to the BuCy regimen (P=0.003). Overall survival, disease-free survival, TRM, and relapses were not significantly different between any of the regimens.
Conclusion: BuCy, standard-dose Cy/TBI and high dose Cy/TBI regimens have equivalent efficacy profiles in terms of the major outcomes overall survival, disease-free survival, TRM, and relapse in patients undergoing T-replete unrelated donor marrow transplantation for AML, CML, and MDS.

Study # RT06-02


Purpose: To estimate the combined effect of the many advances in transplant care since the 1980s on the incidence of TRM.

Patients/methods: Patients younger than 50 years who received a matched related or an unrelated transplant, using a bone marrow or peripheral blood graft, for AML in first (CR1) or second (CR2) complete remission from 1985 through 2004. Only patients who received myeloablative conditioning with a BuCy or TBI-Cy based regimen were eligible. The incidence of TRM was determined for four consecutive 5-year periods for HLA-matched sibling donors, and the later 3 for unrelated donor (URD) separately by donor type and disease status at transplant.

Results: Adjusting for changes in patient and disease characteristics over time in the multivariate analysis, there was a significant drop in TRM over time in three of the four groups. In the matched related donor patients, the relative risk for TRM in 2000-2004 (compared to 1985-1989) was 0.5 (95% CI, 0.37-0.66, p<0.001) and 0.25 (0.15-0.44, p<0.001) for the CR1 and CR2 groups, respectively. In the unrelated donor patients, the relative risk for TRM in 2000-2004 (compared to 1990-1994) was 0.73 (95% CI, 0.5-1.06 p=0.095) for the CR1 group and was 0.58 (95% CI, 0.42-0.79, p<0.001) for the CR2 group.

Conclusion: For patients with AML, there has been a marked reduction in the incidence of TRM over time in matched related transplants performed in a first or second CR. There has also been a significant reduction in TRM in unrelated donor transplants for AML performed in a second CR.

PRELIMINARY RESULTS

Study # RT06-01s

Title: Evaluation of TGF-β1 promoter and signal peptide polymorphisms as risk factors for renal dysfunction in HCT patients treated with cyclosporine A

Study Chair: Riddhishkumar Shah (Georgetown University Hospital)
Meral Bek sac (Ankara Cord Blood Bank; Ankara University Medical School / Ibni Sina Hospital)

MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: Brent Logan (blogan@mcw.edu)

Study Status: Manuscript preparation
Purpose: To evaluate the impact of single nucleotide polymorphisms (SNPs) associated with increased TGF-ß1 production on the risk of cyclosporine A (CsA)-induced renal dysfunction.

Patients/methods: The study included cytomegalovirus (CMV)-negative recipients who received their CMV-negative allogeneic HCT from 2000 to 2004 and who were treated with CsA. Patients with aplastic anemia and CsA exposure of more than 30 days before transplant were excluded. Patients were genotyped for -1550, -509 and +869 SNPs. Renal dysfunction was measured by serum creatinine collected at 5 time points: preconditioning, day 30, day 100, day 180 and day 365 following transplant.

Results: There was no clear association between the three TGF-ß1 SNPs and the development of renal insufficiency post-transplant, regardless of the definition of this event.

Conclusion: The frequency of TGF-ß1 high-expression SNPs was low compared to the other polymorphisms, which could have reduced our ability to detect a small impact on renal function post-transplant.

Study # RT08-02

Title: The effect of prior splenectomy or splenic irradiation on myeloid engraftment after myeloablative allogeneic HCT: a CIBMTR analysis

Study Chair: Gorgun Akpek (Greenebaum Cancer Center U of MD)
MS Statistician: Marcelo Pasquini (mpasquin@mcw.edu)
PhD Statistician: Brent Logan (blogan@mcw.edu)
Study Status: Submitted

Purpose: To compare neutrophil engraftment at day +21 and 100-day survival between transplant recipients with or without splenectomy/splenic irradiation/splenomegaly prior to stem cell transplantation; to compare the cumulative incidence of acute GVHD, chronic GVHD, and overall survival 1 year after transplant.

Patients/Methods: We analyzed 9,683 HCT recipients from 1990 to 2006: 472 had prior splenectomy (ST); 300 splenic irradiation (SI); 1,471 had splenomegaly (SM); and 7,440 had a normal spleen (NS).

Results: Median times to neutrophil and platelet recovery were 15 vs. 18 days and 22 vs. 24 days for the ST and NS groups, respectively (p<0.001). Hematopoietic recovery at day +100 was not different across all groups, but the odds of neutrophil recovery at day +14 and +21 and platelet engraftment at day +28 were 3.26, 2.25, and 1.28 for the ST and 0.56, 0.55, and 0.82 for SM groups compared to NS (p<0.001), respectively. Among patients with SM, use of peripheral blood grafts improved neutrophil recovery at day +21, and a CD34+ cell dose of >5.7x106/kg improved platelet recovery at day +28.

Conclusion: After adjusting variables by Cox regression, the incidences of graft-versus-host disease and overall survival were not different among groups. Recipients of mismatched grafts with SM had a two-fold higher incidence of chronic graft-versus-host disease. SM is associated with delayed engraftment, while ST prior to...
HCT facilitates engraftment without impact on treatment-related mortality or survival.

Study # RT09-01

Title: Primary graft failure following allogeneic HCT for the treatment of hematological malignancies

Study Chairs: Sonali Chaudhury (Children’s Memorial Hospital)
Jeffrey Schriber (City of Hope Samaritan)
Olle Ringden (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation)
Richard Olsson (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation)

MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: Brent Logan (blogan@mcw.edu)

Study Status: Manuscript preparation

Purpose: To determine prognostic factors for primary graft failure (PGF) in alloHCT which may assist identifying patients with highest risk for PGF and developing interventions to minimize this complication.

Patients/Methods: Both children (n=4 846) and adults (n=18 428) with leukemia, myelodysplasia (MDS), or myeloproliferative disorders (MPD), who underwent first alloHCT, and reported to the Center for International Blood and Marrow Transplant Research (CIBMTR) between 1995-2008 (n=23 274), were included in the study. Myeloablative conditioning regimens were defined by the CIBMTR criteria, and primary graft failure was defined as alive without progressive disease and < 500/µL in absolute neutrophil count by day +28 or received second transplant within 28 days post-transplant for treatment of PGF. Donors were HLA-id siblings (n=10 059) or unrelated (n=13 215), and cell sources were bone marrow (BM, n=14 367), or peripheral blood (PB; n=8 907).

Results: Primary graft failure occurred in 1,278 (5.5%) patients. Patients with chronic myeloid leukemia (CML; OR 1.78; 95% CI 1.54-2.06; p<0.001), MDS (OR 1.65; 95% CI 1.31-2.07; p<0.001), and MPD (OR 4.41; 95% CI 3.02-6.43; p<0.001) experienced higher PGFs than patients with acute leukemia. Other factors associated with higher incidence of PGF include recipients of HLA-mismatched unrelated donor grafts (OR=1.50; 95% CI 1.32-1.70; p≤0.001), male donors to female recipients (OR=1.30; 95% CI 1.14-1.48; p<0.001), major ABO incompatibility (OR=1.22; 95% CI 1.05-1.41; p=0.008), age <30 years old (OR 1.37; 95% CI 1.21-1.56; p<0.001), and recipients of cryopreserved products (OR 1.40; 95% CI 1.11-1.75; p=0.004). Among recipients of BM grafts, total nucleated cell doses ≤2.4 x10^8/kg were associated with higher rates of PGF (OR 1.41; 95% CI 1.18-1.68, p=0.001). Factors decreasing PGF incidence were: PB (OR 0.39; 95% CI 0.32-0.48; p<0.001), tacrolimus-based immunosuppression (OR 0.61; 95% CI 0.51-0.75; p<0.001), cyclophosphamide/total body irradiation (Cy/TBI; OR 0.75; 95% CI 0.66-0.85; p<0.001), or planned granulocyte colony-stimulating factor (G-CSF; OR 0.36; 95% CI 0.31-0.43; p<0.001) initiated within 1 week post-transplant.
Conclusion: Patients with CML, MDS, or MPD, male donor to female recipient sex mismatch transplants, major ABO-mismatch, age younger than 30, product cryopreservation, and low BM cell dose were associated with higher rates of PGF. In patients with risk factors for PGF, use of Cy/TBI conditioning, tacrolimus-based immunosuppression, PB grafts, or planned G-CSF may diminish the risk of primary graft failure.

STUDIES IN PROGRESS

Study # RT07-01
Title: Prospective validation of the impacts of the HCT comorbidity index, alone and combined with aging on HCT outcomes
Study Chair: Mohamed Sorror (Fred Hutchinson Cancer Center)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # RT08-01
Title: End-stage renal disease in HCT recipients
Study Chairs: Parameswaran Hari (Medical College of Wisconsin)
Hariprasad Trivedi (Medical College of Wisconsin)
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: John Klein (klein@mcw.edu)
Study Status: Data file preparation

Study # RT09-02
Title: Effects of body mass in children with leukemias undergoing allogeneic HCT
Study Chairs: Nancy Bunin (Philadelphia Children’s Hospital)
Richard Aplenc (Philadelphia Children’s Hospital)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: Mei-Jie Zhang (meijie@mcw.edu)
Study Status: Analysis in progress

Study # RT09-03
Title: Comparison of clinical outcomes between myeloablative and reduced-intensity conditioning for haploidentical HCT
Study Chair: Stefan Ciurea (MD Anderson Cancer Center)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development
Study # RT09-04/IB09-06s
Title: Assessment of genetic polymorphisms and transplant-related mortality in relation to conditioning regimen before HLA-matched unrelated donor allogeneic HCT
Study Chair: Theresa Hahn (Roswell Park Cancer Institute)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Data file preparation

Study # RT10-01
Title: C-reactive protein to predict non-relapse mortality after allogeneic HCT
Study Chair: Andrew S. Artz (University of Chicago Medical Center)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol development

Study # RT10-04
Title: Pre-transplant risk factors for relapse after reduced intensity conditioning allogeneic HCT
Study Chair: Frederic Baron (Department of hematology - CHU - Liege)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol development

PLANNED STUDIES

Study # RT10-02
Title: Survival after second allografting following amyoablative conditioning in patients with relapsed hematologic malignancies: a CIBMTR report
Study Chair: Gorgun Akpek (Greenebaum Cancer Center U of MD)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending

Study # RT10-03
Title: Prognostic Scoring System for pulmonary complications post-transplant (impact of A1B8DR3 haplotype)
Study Chair: Hong Liu (Roswell Park Cancer Institute)
Gorgun Akpek (Greenebaum Cancer Center U of MD)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Deferred
Study # RT11-01
Title: Risk factors for the development of idiopathic pneumonitis after autologous HCT for lymphoma using BCNU-containing regimens
Study Chair: Andrew Lane (Dana Farber Cancer Institute - Adults)
            Yi-Bin Chen (Massachusetts General Hospital)
MS Statistician: Xiaochun Zhu (xzhu@mcw.edu)
PhD Statistician: TBD
Study Status: Protocol pending

Study # RT11-02
Title: Chimerism as predictor of secondary graft failure or relapse following allogeneic HCT using reduced intensity conditioning regimens in acute leukemia
Study Chair: Richard Olsson (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation, CAST)
MS Statistician: TBD
PhD Statistician: TBD
Study Status: Protocol pending
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9.19 SOLID TUMORS WORKING COMMITTEE

Co-Chair: Michael Bishop, MD, Medical College of Wisconsin
Email: mbishop@mcw.edu
Co-Chair: Edward Stadtmauer, MD, Abramson Cancer Center, University of Pennsylvania Medical Center
Email: stadtmae@uphs.upenn.edu
Co-Chair: Naoto Ueno, MD, PhD, MD Anderson Cancer Center
Email: nueno@mdanderson.org
Scientific Director: Mukta Arora, MD, MS, University of Minnesota Medical Center
Email: arora005@umn.edu
PhD Statistician: Kwang Woo Ahn, PhD, CIBMTR Milwaukee
Email: kwooahn@mcw.edu
MS Statistician: Xiaochun Zhu, MS, CIBMTR Milwaukee
Email: xzhu@mcw.edu
MS Statistician: John Bosco Umejiego, MBBS, MPH, CIBMTR Milwaukee
Email: jumejieg@nmdp.org

PRELIMINARY RESULTS

Study # ST06-01
Title: Clinical outcomes of patients with desmoplastic small round cell tumor of the peritoneum undergoing autologous HCT: a CIBMTR retrospective analysis
Study Chair: Edward Stadtmauer (Abramson Cancer Center University, Pennsylvania Medical Center)
Rachel Cook, University of Wisconsin School of Medicine and Public Health
MS Statistician: Zhiwei Wang (zwang@mcw.edu)
PhD Statistician: Kwang Woo Ahn (kwooahn@mcw.edu)
Study Status: Manuscript submitted
Purpose: To describe clinical outcomes of autologous transplant for desmoplastic small round cell tumors
Patients/Methods: We identified 36 patients with DSRCTP reported to the CIBMTR between 1999 and 2007 who received autologous HCT for desmoplastic SRCT. Disease and transplant-related characteristics were obtained from the CIBMTR database and pathology reports and additional information were obtained directly from the centers. Descriptive statistics to characterize the patients and outcomes was performed.
Results: The probability of disease-free survival (DFS) at 1 year for those patients in CR and not in CR was 75% (95% CI: 48-94%) and 35% (15-59%), respectively. For the entire cohort, 1 year DFS was 53% (39-69%). DFS at 3 years was 40% (15-69%) and 9% (0-29%) in the two groups, respectively. The probability of overall survival at 3 years was 57% (29-83%) for patients in CR and 28% (9-51%) for patients not in CR. Median survival for the entire cohort was 31 months (36 months for those in CR, 21 months for those not in CR). Treatment-related mortality was low with only 1 death in the first 100 days. Engraftment at 42 days was 97% (88-100%).
Conclusion: Autologous stem cell transplant (ASCT) appears to be a tolerable approach in patients with DSCRTP with the greatest benefit seen in those patients who obtain CR. Overall, with low treatment-related mortality and prolonged survival compared to historical controls, ASCT is a reasonable option for patients with DSRCTP though its role should be confirmed in larger studies.

Study # ST07-01
Title: Clinical outcomes of patients with desmoplastic small round cell tumor of the peritoneum undergoing autologous HCT: a CIBMTR retrospective analysis
Study Chair: Gregory A. Hale (All Children’s Hospital, St. Petersburg, Florida)
Stephan Grupp (Children’s Hospital of Philadelphia)
MS Statistician: Vincent He (vhe@mcw.edu)
PhD Statistician: Kwang Woo Ahn (kwooahn@mcw.edu)
Study Status: Manuscript preparation
Purpose: To describe outcome after allogeneic HCT in a modern treatment era in patients with neuroblastoma and to identify variables associated with improved disease-free survival

Patients/Methods: We studies 143 transplants at 61 centers reported to the CIBMTR who underwent HCT for neuroblastoma. For this analysis, patients were categorized into 2 groups for comparison: those without and those with a prior auto HCT. Five-year univariate probabilities of relapse, transplant related mortality (TRM), event free survival (EFS) and overall survival (OS) are reported.

Results: For this analysis, patients were categorized into 2 groups for comparison: those without (Group 1; n=97) and those with a prior auto HCT (Group 2; n=46). There was no difference in the prevalence of acute (p=.15) or chronic GVHD (p=.24) between the two groups. OS rates at 1 year and 5 years were 59% and 29% for Group 1 and 50% and 7% for Group 2. Relapse rates were higher after unrelated donor transplants and lower after HLA-mismatched related transplants, compared with HLA-identical related donor transplants (p<0.0001). EFS and survival were significantly lower for unrelated donor transplants at 1 year and 3 years but not 5 years post-HCT. The most common causes of death in Group 1 vs. Group 2 were relapse (64%/75%), GVHD (5%/0%), infections (11%/5%), and organ toxicity (11%/13%).

Conclusion: Allogeneic HCT can cure some NB patients, with lower relapse rates and improved survival in patients without a history of autologous HCT compared with those patients who had undergone auto HCT first. Future studies comparing OS after autologous HCT and allogeneic HCT for patients in CR1 should be considered.
## STUDIES IN PROGRESS

### Study # ST00-02
**Title:** Allogeneic HCT for renal cell cancer  
**Study Chair:** A. John Barrett (National Heart Lung & Blood Institute)  
Olle Ringdén (Karolinska University Hospital, Sweden)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Supplemental form/data collection

### Study # ST02-02
**Title:** Allogeneic HCT in colorectal cancer  
**Study Chair:** Lisbeth Barkholt (Karolinska University Hospital Huddinge, Sweden)  
Olle Ringden (Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation)  
Massimo Aglietta (Istituto per la Ricerca e la Cure del Cancro, Italy)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Supplemental form/data collection

### Study # ST09-01
**Title:** Transplant trends for metastatic solid tumors in North America and Europe  
**Study Chairs:** Richard Childs (National Heart Lung & Blood Institute)  
Didier Blaise (Institut Paoli Calmettes, France)  
**MS Statistician:** TBD  
**PhD Statistician:** TBD  
**Study Status:** Protocol development

### Study # ST10-01
**Title:** High dose chemotherapy for autologous HCT in patients with high risk or metastatic breast cancer  
**Study Chairs:** Naoto Ueno (MD Anderson Cancer Center)  
Yee C. Cheng (Medical College of Wisconsin)  
**MS Statistician:** John Bosco Umejiego (jumejieg@nmdp.org)  
**PhD Statistician:** Kwang Woo Ahn (kwooahn@mcw.edu)  
**Study Status:** Protocol development
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APPENDIX A1: CIBMTR INTERNATIONAL CENTERS

The following tables list the international and U.S.-based transplant centers that submit data to the CIBMTR research database for matched unrelated donor (MUD), related donor, and autologous transplants. Centers submit data at two levels: a Transplant Essential Data (TED) level and a Comprehensive Report Form (CRF) level (Section 4.2).

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APPENDIX B1: CIBMTR STATISTICAL CENTER ORGANIZATIONAL STRUCTURE – MILWAUKEE CAMPUS
APPENDIX C: WORKING COMMITTEE LEADERSHIP

For information on WC studies and associated contact information, see Section 9. The bolded name below identifies the coordinating statistician for that committee.

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Shalini Shenoy, MD, Washington University/St. Louis Children's Hospital

Scientific Director
Mary Eapen, MD, MS, Medical College of Wisconsin

Statisticians
Jeanette Carreras, MPH, CIBMTR Statistical Center Milwaukee
Jennifer Le-Rademacher, PhD, CIBMTR Statistical Center Milwaukee

PEDIATRIC CANCER WORKING COMMITTEE

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Carrie Kitko, MD, University of Michigan
Adriana Seber, MD, Instituto de Oncologia Pediatrica, Sao Paolo, Brazil

Scientific Director
Mary Eapen, MD, MS, Medical College of Wisconsin

Statisticians
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Mei-Jie Zhang, PhD, CIBMTR Statistical Center Milwaukee

PLASMA CELL DISORDERS WORKING COMMITTEE

Co-chairs
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Sagar Lonial, MD, Emory University Hospital
Gustavo Milone, MD, Angelica Ocampo-Fundaleu

Scientific Director
Parameswaran Hari, MD, MS, Medical College of Wisconsin

Statisticians
Tony X. Zhong, MS, CIBMTR Statistical Center Milwaukee
Mei-Jie Zhang, PhD, CIBMTR Statistical Center Milwaukee

Consumer Advocacy Committee Representative
Jim Omel, MD, retired
APPENDIX C: WORKING COMMITTEE LEADERSHIP

REGIMEN-RELATED TOXICITY/SUPPORTIVE CARE WORKING COMMITTEE

Co-Chairs
Kenneth Cooke, MD, University Hospitals Case Medical Center  
Vincent T. Ho, MD, Dana-Faber Cancer Institute  
Philip McCarthy, MD, Roswell Park Cancer Institute

Scientific Director
Marcelo Pasquini, MD, MS, Medical College of Wisconsin

Statisticians
Xiaochun Zhu, MS, CIBMTR Statistical Center Milwaukee  
Sandra Tomany-Korman, MS, CIBMTR Statistical Center Milwaukee  
Brent Logan, PhD, CIBMTR Statistical Center Milwaukee

SOLID TUMORS WORKING COMMITTEE

Co-Chairs
Michael Bishop, MD, NCI Experimental Transplantation & Immunology Branch  
Edward Stadtmauer, MD, Hospital of the University of Pennsylvania  
Naoto Ueno, MD, MD Anderson Cancer Center

Scientific Director
Mukta Arora, MD, MS, University of Minnesota

Statisticians
John Bosco, MBBS, MPH, CIBMTR Statistical Center Milwaukee  
Kwang Woo Ahn, PhD, CIBMTR Statistical Center Milwaukee
APPENDIX D1: ADVISORY COMMITTEE

All Advisory, Executive, and Nominating Committee terms begin on March 1.

ELECTED MEMBERS

Chair
Thomas Shea, MD, University of North Carolina at Chapel Hill, NC

Immediate Past Chair
Stella Davies, MBBS, PhD, Cincinnati Children’s Hospital, Cincinnati, OH

VICE CHAIRS

North America
Paul Martin, MD, Fred Hutchinson Cancer Research Center, Seattle, WA

Europe
Jane Apperley, MD, Imperial College School of Medicine, London, England

Central America/ South America
Ricardo Pasquini, MD, Hospital de Clinicas, Universidade Federal do Parana, Curitiba, Brazil

Asia/Africa/Australia
Mammen Chandy, MD, Tata Medical Center, Kolkata, India

MEMBERS AT LARGE

North America
Marcos De Lima, MD, MD Anderson Cancer Center, Houston, TX
Hillard Lazarus, MD, University Hospitals Case Medical Center, Cleveland, OH
Brenda Sandmaier, MD, Fred Hutchinson Cancer Research Center, Seattle, WA
Bart Scott, MD, Fred Hutchinson Cancer Research Center, Seattle, WA
Robert Soiffer, MD, Dana Farber Cancer Institute, Boston, MA
Koen van Besien, MD, Weill Cornell Medical College, New York, NY

Non-North America
Yoshiko Atsuta, MD, PhD, Nagoya University, Graduate School of Medicine, Nagoya, Japan
Peter Dreger, MD, University of Heidelberg, Heidelberg, Germany
Jürgen Finke, MD, Freiburg University Medical Center, Freiburg, Germany
Jonas Mattsson, MD, Karolinska University Hospital, Stockholm, Sweden
Judith Marsh, MD, St. George’s Hospital, London, England
Tracey O’Brien, MD, Sydney Children’s Hospital, Sydney, Australia
APPOINTED MEMBERS

Patient/Family Representatives
Richard Boyajian, RN, MS, ANP, Co-Chair, Consumer Advocacy Committee
Barry Schatz, Co-Chair, Consumer Advocacy Committee,
Donor Center Representative
Corina Gonzalez, MD, Georgetown University Hospital, Washington, DC

Collection Center Representative
Edward Snyder, MD, Yale New Haven Hospital, New Haven, CT

Bioethicist
Raquel Schears, MD, MPH, FACEP, Mayo Clinic, Rochester, MN

Business Representative
Alan Leahigh, Executive Director, ASBMT, Chicago, IL (retired)

Cord Blood Bank Representative
Elizabeth Shpall, MD, MD Anderson Cord Blood Bank, Houston, TX

EX OFFICIO MEMBERS

Executive Director
Jeffrey Chell, MD, NMDP, Minneapolis, MN

Chief Scientific Director
Mary M. Horowitz, MD, MS, CIBMTR, Milwaukee, WI

Chief Statistical Director
John P. Klein, PhD, CIBMTR, Milwaukee, WI

Associate Scientific Director Data Operations
J. Douglas Rizzo, MD, MS, CIBMTR, Milwaukee, WI

Associate Scientific Director CIBMTR Minneapolis
Dennis Confer, MD, NMDP, Minneapolis, MN

Vice President
CIBMTR-Minneapolis
Roberta King, CIBMTR, Minneapolis, MN

Senior Research Advisor
Daniel Weisdorf, MD, University of Minnesota, Minneapolis, MN

NMDP/HRSA Project Officer
Shelley Grant, MPH

NMDP/Navy Project Officer
Robert Hartzman, MD, Capt. MC, USN (ret)

MCW/HRSA Project Officers
Robert Baitty, MPP
Nawraz Shawir, MBBS

MCWINCI Project Officer
Roy Wu, PhD

MCW/NHLBI Project Officer
Nancy DiFronzo, PhD

MCW/NIAID Project Officer
Linda Griffith, MD, PhD

Nominating Committee Chair
David Porter, MD, Hospital of the University of Pennsylvania, Philadelphia, PA
APPENDIX D2: EXECUTIVE COMMITTEE

All Advisory, Executive, and Nominating Committee terms begin on March 1. The CIBMTR Executive Committee is a subset of the Advisory Committee.

ELECTED MEMBERS
Chair Thomas Shea, MD, University of North Carolina at Chapel Hill, Chapel Hill, NC
Immediate Past Chair Stella Davies, MBBS, PhD, Cincinnati Children’s Hospital, Cincinnati, OH

VICE CHAIRS
North America Paul Martin, MD, Fred Hutchinson Cancer Research Center, Seattle, WA
Europe Jane Apperley, MD, Imperial College School of Medicine, London, England
Central/South America Ricardo Pasquini, MD, Hospital de Clinicas, Universidade Federal do Parana, Curitiba, Brazil
Asia/Africa/Australia Mammen Chandy, MD, Tata Medical Center, Kolkata, India

APPOINTED MEMBERS
Patient/Family Representatives Richard Boyajian, RN, MS, ANP, Co-Chair, Consumer Advocacy Committee
Barry Shatz, Co-Chair, Consumer Advocacy Committee
Donor Center Representative Corina Gonzalez, MD, Georgetown University Hospital, Washington, DC
Collection Center Representative Edward Snyder, MD, Yale New Haven Hospital, New Haven, CT
Bioethicist Raquel Schears, MD, MPH, FACEP, Mayo Clinic, Rochester, MN
Business Representative Alan Leahigh, Executive Director, ASBMT, Chicago, IL (retired)
Cord Blood Bank Representative Elizabeth Shpall, MD, MD Anderson Cord Blood Bank, Houston, TX

EX OFFICIO MEMBERS
Executive Director Jeffrey Chell, MD, NMDP, Minneapolis, MN
Chief Scientific Director Mary M. Horowitz, MD, MS, CIBMTR, Milwaukee, WI
Chief Statistical Director John P. Klein, PhD, CIBMTR, Milwaukee, WI
Associate Scientific Director Data Operations J. Douglas Rizzo, MD, MS, CIBMTR, Milwaukee, WI
Associate Scientific Director CIBMTR Minneapolis Dennis Confer, MD, NMDP, Minneapolis, MN
Vice President CIBMTR Minneapolis Roberta King, CIBMTR, Minneapolis, MN

Senior Research Advisor Daniel Weisdorf, MD, University of Minnesota, Minneapolis, MN
NMDP/HRSA Project Office Shelly Grant, MPH
NMDP/Navy Project Office Robert Hartzman, MD, Capt. MC, USN (ret)
MCW/HRSA Project Officers Robert Baitty, MPP
Nawraz Shawir, MBBS
MCW/NCI Project Officer Roy Wu, PhD
MCW/NHLBI Project Officer Nancy DiFronzo, PhD
MCW/NIAID Project Officer Linda Griffith, MD, PhD
Nominating Committee Chair David Porter, MD, Hospital of the University of Pennsylvania, Philadelphia, PA
APPENDIX D3: CONSUMER ADVOCACY COMMITTEE

CO-CHAIRS

Richard Boyajian, RN, Dana-Farber Cancer Institute
Barry Schatz, Cardinal Bernardin Cancer Center, Loyola University Medical Center

MEMBERS

Maureen Beaman
Gerardo Camarillo, MD Anderson Cancer Center Family Advisory Council
Arthur Flatau, PhD, Association of Cancer Online Resources
Winona Hollins-Hauge, MSW, LICSW, Washington State Commission on African American Affairs and Governor’s Interagency Council on Health Disparities
Myra Jacobs, MA, CFRE, National Bone Marrow Transplant Link
James Omel, MD
Susan Stewart, Blood & Marrow Transplant Information Network

SCIENTIFIC DIRECTOR

J. Douglas Rizzo, MD, MS, CIBMTR Milwaukee

EX OFFICIO MEMBERS

Robyn Ashton, RN, MSN, Health Resources and Services Administration
Robert Baitty, MPH, Health Resources and Services Administration
Jeffrey Chell, MD, National Marrow Donor Program
Dennis Confer, MD, CIBMTR Minneapolis
Carol Doleysh, BS, CPA, (CIBMTR liaison) CIBMTR Milwaukee
Rebecca Drexler, BS, AAS, CIBMTR Minneapolis
Shelley Grant, MPH, Health Resources and Services Administration
Anna Hassebroek, MPH, CIBMTR Minneapolis
Darlene Haven, BS, National Marrow Donor Program
Mary Horowitz, MD, MS, CIBMTR Milwaukee
Stephanie Lee, MD, MPH, Fred Hutchinson Cancer Center
Navneet Majhail, MBBS, MD, MS, CIBMTR Minneapolis
Elizabeth Murphy, EdD, RN, (NMDP liaison) National Marrow Donor Program
Tanya Pedersen, MS, CIBMTR Minneapolis
Jerry Wang, MS, CIBMTR Milwaukee
APPENDIX E: NOMINATING COMMITTEE

All Advisory, Executive, and Nominating Committee terms begin on March 1.

CHAIR

David Porter, MD, Hospital of the University of Pennsylvania, PA

MEMBERS

Steven Devine, MD, Ohio State University Medical Center, Columbus, OH
Neena Kapoor, MD, Children’s Hospital of Los Angeles, Los Angeles, CA
Ginna Laport, MD, Stanford University, Stanford, CA
Jeffrey Szer, MD, Royal Melbourne Hospital City Campus, Victoria, Australia
APPENDIX F: IMMUNOBIOLOGY STEERING COMMITTEE/
NMDP HISTOCOMPATIBILITY ADVISORY GROUP

MEMBERS

Robert Baitty, MPP, Health Resources & Services Administration, HHS
James Bowman, MD, Health Resources & Services Administration, HHS
Robert J. Hartzman, MD, Capt, MC, USN (Ret.) Navy Representative,
          C.W. Bill Young Marrow Donor Recruitment and Research Program
Carolyn K. Hurley, PhD, Diplomat ABHI, Georgetown University Medical Center
Craig Kollman, PhD, Jaeb Center for Health Research
Carlheinz Mueller, MD, PhD, German National Bone Marrow Donor Registry
Harriet Noreen, CHS, University of Minnesota-Fairview
Pablo Rubinstein, MD, New York Blood Center
Ann Woolfrey, MD, Fred Hutchinson Cancer Center
Neng Yu, MD, American Red Cross Blood Services, NE Division

CIBMTR STAFF

Brent Logan, PhD, CIBMTR Milwaukee
Mary Eapen, MD, MS, CIBMTR Milwaukee
Stephen Spellman, MBS, CIBMTR Minneapolis

NMDP STAFF

Dennis Confer, MD, National Marrow Donor Program
Karen Dodson, National Marrow Donor Program
Martin Maiers, MS, National Marrow Donor Program
Willis Navarro, MD, National Marrow Donor Program
Michelle Setterholm, BS, CHS, National Marrow Donor Program
APPENDIX G: CLINICAL TRIALS ADVISORY COMMITTEE

CHAIR

Steven Devine, MD, Ohio State Medical Center, James Cancer Center

MEMBERS

Mitchell Horwitz, MD, Duke University Medical Center
Neena Kapoor, MD, Children’s Hospital of Los Angeles
Hillard Lazarus, MD, University Hospitals Case Medical Center
John Levine, MD, MS University of Michigan
Richard Maziarz, MD, Oregon Health and Science University
Scott Rowley, MD, Hackensack University Medical Center
Edward Stadtmauer, MD, Abramson Cancer Center University of Pennsylvania Medical Center
Kirsten Williams, MD, NIH-NCI Experimental Transplantation and Immunology Branch

EX OFFICIO MEMBERS

Richard Boyajian, ANP, RN, MS, Dana Farber Cancer Institute - Pediatrics
Dennis Confer, MD, CIBMTR Minneapolis
Becky Drexler, BS, AAS, CIBMTR Minneapolis
Robert Hartzman, MD, Organization/Center information not available
Mary Horowitz, MD, MS, CIBMTR Milwaukee
Roberta King, MPH, CIBMTR Minneapolis
Jennifer LeRademacher, PhD, CIBMTR Milwaukee
Brent Logan, PhD, CIBMTR Milwaukee
Willis Navarro, MD, National Marrow Donor Program Operations
Nancy Poland, MA, National Marrow Donor Program Operations
Barry Schatz, Loyola University Medical Center
Marcie Tomblyn, MD, MS, H. Lee Moffitt Cancer Center and Research Institute
Kevin Weber, National Marrow Donor Program Operations
Daniel Weisdorf, MD, CIBMTR Minneapolis
## APPENDIX H: CIBMTR PUBLICATIONS IN 2011

### CIBMTR WORKING COMMITTEE PUBLICATIONS

The following publications were generated by WCs and related research programs, all of which use the CIBMTR Research Database. The PMCID number is assigned by PubMed Central, the NIH’s free digital archive of biomedical and life sciences journal literature.

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<td>Chaves W, Walters MC, Wagner J, Gluckman E, Rocha V; Eurocord Registry;</td>
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<td>Center for International Blood and Marrow Transplant Research; New</td>
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## BMT CTN PUBLICATIONS

The following publications were generated by the BMT CTN, which conducts multi-institutional phase II and III trials focused on HCT. The BMT CTN Data Coordinating Center (DCC) maintains continuity of operations and facilitates effective communications. The DCC effort is a collaboration of CIBMTR, NMDP, and the EMMES Corporation. For more information, see Section 2.3.1.

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## HSR PUBLICATIONS

The following publications were generated by the HSR program, through which CIBMTR conducts health policy and health services issues involving HCT. For more information, see Section 2.5.

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<td>Recommended screening and preventive practices for long-term survivors after hematopoietic cell transplantation</td>
<td>Biol Blood Marrow Transplant. 2011 Dec 13. [Epub ahead of print]</td>
<td>[PMC Journal – In Process]</td>
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STATISTICAL METHODOLOGY PUBLICATIONS

The following publications were generated by the CIBMTR Statistical Center, which provides administrative, statistical, data management, clinical trials, information technology (IT), and personnel support for CIBMTR activities. For more information, see Sections 1.4 and 3.0.

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<th>Statistical Methodology Publications</th>
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<td>Le-Rademacher J, Billard L</td>
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### 2011 BMT Tandem Meetings

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<td>IB08-02</td>
<td>Evaluation of HLA matching requirements in unrelated hematopoietic stem cell transplantation for nonmalignant disorders</td>
<td>Oral</td>
<td>A Woolfrey</td>
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<td>IB10-05</td>
<td>Scoring HLA mismatches by Histocheck does not predict clinical outcome in HCT</td>
<td>Oral</td>
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<td>LE09-03</td>
<td>Transplant physician perceptions and practice patterns regarding fertility preservation in hematopoietic-cell transplant (HCT) recipients</td>
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<td>BMT CTN 0603</td>
<td>Donor KIR2DS1 and KIR3DS1 are associated with improved outcomes following unrelated allogeneic stem cell transplantation for acute myeloid leukemia</td>
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<td>BMT CTN 0603</td>
<td>Phase II Trial of non-myeloablative conditioning (NST) double umbilical cord blood transplantation (DUCBT) from unrelated donors in patients with hematologic malignancies: results of Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Protocol 0604</td>
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### 2011 ASH Annual Meeting

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<td>Graft-versus-host disease (GVHD) induced graft-versus-leukemia (GVL) effect: more impact on later relapse and disease-free survival following reduced intensity conditioning</td>
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<td>AA08-01</td>
<td>HLA DR15 antigen status does not impact graft-versus-host disease or disease-free survival in HLA-matched sibling transplantation for hematologic malignancies</td>
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<td>AA09-01</td>
<td>Results of allogeneic hematopoietic cell transplantation in persons with Fanconi anemia and pretransplant cytogenetic abnormalities, myelodysplastic syndrome, or acute leukemia</td>
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<td>IS09-04</td>
<td>Effect of race on outcomes after allogeneic hematopoietic cell transplantation for severe Aplastic Anemia</td>
<td>Oral</td>
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<td>LK07-03b</td>
<td>Older adults with chronic myelogenous leukemia (CML), during the tyrosine kinase inhibitor (TKI) era, can be successfully treated with reduced intensity conditioning (RIC) hematopoietic cell transplant (HCT) using sibling or unrelated donors: a Center for International Blood and Marrow Transplant Research (CIBMTR) analysis</td>
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<td>Reduced intensity or non-ablative hematopoietic cell transplantation in older patients with non-Hodgkin lymphoma (NHL): encouraging survival for patients ≥55 years</td>
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<td>LK08-02</td>
<td>A decision analysis of reduced-intensity conditioning allogeneic hematopoietic stem cell transplantation for older patients with de-novo myelodysplastic syndrome (MDS): early transplantation offers survival benefit in higher-risk MDS</td>
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<td>LY06-06</td>
<td>A CIBMTR prognostic model for progression-free survival (PFS) after autologous hematopoietic cell transplantation (AHCT) for relapsed or refractory Hodgkin lymphoma (HL)</td>
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<td>Conditioning intensity in allogeneic hematopoietic cell transplantation (alloHCT) for diffuse large B-cell lymphoma (DLBCL)</td>
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<td>MM02-01</td>
<td>Second autologous transplants for multiple myeloma (MM) relapse after a prior autologous transplant (AHCT) – a report from the Center for International Blood and Marrow Transplant Research (CIBMTR)</td>
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<td>Second transplants in relapsed multiple myeloma (MM): autologous (AHCT) versus non-myeloablative/reduced intensity (NST/RIC) allogeneic transplantation (alloHCT)</td>
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<td>ST06-01</td>
<td>Is there a role for ASCT in patients with desmoplastic small round cell tumor of the peritoneum?</td>
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<td>Allogeneic hematopoietic cell transplantation (HCT) for neuroblastoma (NB): the CIBMTR experience</td>
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<td>BMT CTN</td>
<td>Immunoglobulin free light chain (FLC) and heavy chain/light chain (HLC) assays – comparison with electrophoretic responses in multiple myeloma (MM)</td>
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<td>Increased incidence of chronic graft-versus-host disease (GVHD) and no survival advantage with filgrastim-mobilized peripheral blood stem cells (PBSC) compared to bone marrow (BM) transplants from unrelated donors: results of Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Protocol 0201, a phase III prospective randomized trial</td>
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<td>Larger numbers of donor naïve CD8+ T-cells and plasmacytoid dendritic cell precursors in allogeneic BM grafts from unrelated donors are associated with improved survival: results from BMT CTN 0201</td>
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<td>Fludarabine-based conditioning for allogeneic marrow transplantation from unrelated donors in severe aplastic anemia (SAA): serious and unexpected adverse events in pre-defined cyclophosphamide (CY) dose levels</td>
<td>Poster</td>
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<tr>
<td>BMT CTN</td>
<td>Randomized phase III trial of ¹³¹Iodine-Tositumomab (Bexxar)/Carmustine, Etoposide, Cytarabine, Melphalan (BEAM) vs. Rituximab/BEAM and autologous stem cell transplantation for relapsed diffuse large B-cell lymphoma (DLBCL): no difference in progression-free (PFS) or overall survival (OS)</td>
<td>Oral</td>
<td>J Vose</td>
</tr>
<tr>
<td>0401</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
## 2011 International Conference on Malignant Lymphoma (ICML)

<table>
<thead>
<tr>
<th>Study #</th>
<th>Title</th>
<th>Type</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>LY08-02</td>
<td>Allogeneic transplantation (ALLOHCT) for patients with mantle cell lymphoma (MCL) progressing after autologous transplantation (AUTOHCT)</td>
<td>Oral</td>
<td>P Hari</td>
</tr>
<tr>
<td>LY08-02</td>
<td>Outcomes of patients with mantle cell lymphoma undergoing autologous versus reduced-intensity allogeneic transplantation</td>
<td>Oral</td>
<td>T Fenske</td>
</tr>
</tbody>
</table>

## 2011 European Hematology Association (EHA) Annual Meeting

<table>
<thead>
<tr>
<th>Study #</th>
<th>Title</th>
<th>Type</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>IB09-08</td>
<td>Birth Order is not a major factor influencing transplant outcome in HLA-identical sibling SCT - an analysis on behalf of the CIBMTR</td>
<td>Oral</td>
<td>C Dobblestein</td>
</tr>
</tbody>
</table>

## 2011 European Federation for Immunogenetics (EFI) Annual Meeting

<table>
<thead>
<tr>
<th>Study #</th>
<th>Title</th>
<th>Type</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>IB09-02</td>
<td>Non-permissive HLA-DPB1 T-cell epitope disparities are associated with non-relapse mortality after unrelated stem cell transplantation and are not dependent on HLA-DPA1</td>
<td>Poster</td>
<td>K Fleischhauer</td>
</tr>
</tbody>
</table>

## 2011 European Group for Blood and Marrow Transplantation (EBMT)

<table>
<thead>
<tr>
<th>Study #</th>
<th>Title</th>
<th>Type</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>LE09-01</td>
<td>Second malignancies after autologous hematopoietic stem cell transplantation in children</td>
<td>Oral</td>
<td>N Majhail</td>
</tr>
<tr>
<td>PC10-05</td>
<td>Impact of immune modulation with anti-T cell antibodies on outcomes after unrelated donor transplantation for acute lymphoblastic leukemia (ALL) in children and adolescents</td>
<td>Oral</td>
<td>P Veys</td>
</tr>
</tbody>
</table>
The BMT CTN (Section 2.3.1) and the RCI BMT (Section 2.3.2) use the CIBMTR research database to design, monitor, and analyze clinical trials. A status of their projects is included in this appendix. Support for BMT CTN clinical trials is provided by the NHLBI in partnership with the NCI. Support for RCI BMT projects is provided by a variety of external support sources.

### BMT CTN Clinical Trials - Open for Enrollment

<table>
<thead>
<tr>
<th>Protocol Number</th>
<th>Title</th>
<th>Accrual Dates</th>
</tr>
</thead>
<tbody>
<tr>
<td>BMT CTN 0301</td>
<td>Fludarabine-based conditioning for allogeneic marrow transplantation from HLA-compatible unrelated donors in severe aplastic anemia</td>
<td>• Opened January 24, 2006</td>
</tr>
<tr>
<td>BMT CTN 0501</td>
<td>Multi-center, open label, randomized trial comparing single versus double cord blood transplantation in pediatric patients with leukemia and myelodysplastic syndrome (MDS)</td>
<td>• Opened October 16, 2006</td>
</tr>
<tr>
<td>BMT CTN 0601</td>
<td>Unrelated donor hematopoietic cell transplantation for children with severe sickle cell anemia using a reduced-intensity conditioning regimen</td>
<td>• Opened June 27, 2008</td>
</tr>
<tr>
<td>BMT CTN 0701</td>
<td>Allogeneic HCT using reduced-intensity conditioning for relapsed follicular cell non-Hodgkin lymphoma using related or unrelated donors</td>
<td>• Opened April 27, 2009</td>
</tr>
<tr>
<td>BMT CTN 0702</td>
<td>Single autologous transplant with or without consolidation versus tandem autologous transplant with lenalidomide maintenance</td>
<td>• Opened June 1, 2010</td>
</tr>
<tr>
<td>BMT CTN 0801</td>
<td>Phase II/III randomized, multi-center trial comparing sirolimus plus prednisone, sirolimus/extracorporeal photopheresis plus prednisone, and sirolimus/calcineurin inhibitor plus prednisone for the treatment of chronic graft-versus-host disease</td>
<td>• Opened April 15, 2010</td>
</tr>
<tr>
<td>BMT CTN 0803</td>
<td>High-dose chemotherapy with autologous stem cell rescue for aggressive B cell lymphoma and Hodgkin lymphoma in HIV-infected patients</td>
<td>• Opened July 12, 2010</td>
</tr>
<tr>
<td>BMT CTN 0804</td>
<td>Phase II study of reduced-intensity allogeneic stem cell transplantation for high-risk chronic lymphocytic leukemia</td>
<td>• Opened March 16, 2011</td>
</tr>
<tr>
<td>(CALGB 100701)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>BMT CTN 0901</td>
<td>Phase III study comparing myeloablative to non-myeloablative conditioning for transplantation in patients with MDS or AML</td>
<td>• Opened June 2, 2011</td>
</tr>
<tr>
<td>BMT CTN 0902</td>
<td>A phase III randomized, multi-center trial testing whether exercise or stress management improves functional status and symptoms of autologous and allogeneic HCT recipients</td>
<td>• Opened January 3, 2011</td>
</tr>
<tr>
<td>BMT CTN 0903</td>
<td>Allogeneic blood stem cell transplant for hematological cancers and bone marrow failure in HIV-infected individuals</td>
<td>• Opened September 12, 2011</td>
</tr>
<tr>
<td>BMT CTN0805</td>
<td>Randomized phase II trial of chemotherapy + dasatinib versus allogeneic HCT for adults with ph+ acute lymphoblastic leukemia</td>
<td>• Open to accrual through SWOG BMT CTN anticipates release in 2012</td>
</tr>
<tr>
<td>(SWOG 0805)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>BMT CTN 1101</td>
<td>Phase III study comparing HLA-haploidentical related donor bone marrow versus double umbilical cord blood with reduced-intensity conditioning for patients with hematologic malignancies</td>
<td>• Anticipated release in 2012</td>
</tr>
<tr>
<td>Protocol Number</td>
<td>Title</td>
<td>Accrual Dates</td>
</tr>
<tr>
<td>-----------------</td>
<td>--------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------</td>
<td>---------------------------------------------------</td>
</tr>
</tbody>
</table>
| BMT CTN 0101    | A randomized, double-blind trial of fluconazole versus voriconazole for prevention of invasive fungal infections in allogeneic blood and marrow transplant recipients                                              | • Opened December 1, 2003  
• Closed September 21, 2006                     |
| BMT CTN 0102    | A trial of tandem autologous stem cell transplants with or without post-second autologous transplant maintenance therapy versus single autologous stem cell transplant followed by matched sibling non-myeloablative allogeneic stem cell transplant for patients with multiple myeloma | • Opened November 15, 2003  
• Closed March 30, 2007                           |
| BMT CTN 0201    | A phase III, randomized, multi-center trial comparing G-CSF mobilized peripheral blood stem cell with marrow transplantation from HLA-compatible, unrelated donors                                | • Opened January 20, 2004  
• Closed October 16, 2009                          |
| BMT CTN 0202    | Autologous versus non-myeloablative allogeneic hematopoietic stem cell transplantation for patients with chemosensitive follicular non-Hodgkin lymphoma beyond first complete response of first partial response | • Opened July 28, 2004  
• Closed March 2, 2006                            |
| BMT CTN 0302    | Initial systemic treatment of acute GVHD: a phase II, randomized trial evaluating etanercept, mycophenolate mofetil, denileukin diftitox (ONTAK), and pentostatin                                      | • Opened August 25, 2005  
• Closed March 24, 2008                            |
| BMT CTN 0303    | A single-arm, multi-center phase II trial of transplants of HLA-matched, CD34+ enriched, T cell depleted peripheral blood stem cells isolated by the CliniMACS system in the treatment of patients with AML in first or second morphologic complete remission | • Opened June 5, 2005  
• Closed December 24, 2008                         |
| BMT CTN 0401    | Phase III Rituxan/BEAM versus Bexxar/BEAM with autologous hematopoietic stem cell transplantation for persistent or relapsed chemotherapy-sensitive diffuse large B cell non-Hodgkin lymphoma            | • Opened December 5, 2007  
• Closed July 17, 2009                             |
| BMT CTN 0603    | A multi-center, phase II trial of non-myeloablative conditioning and transplantation of partially matched HLA-mismatched bone marrow for patients with hematologic malignancies                                      | • Opened October 17, 2008  
• Closed May 17, 2010                              |
| BMT CTN 0604    | A multi-center, phase II trial of non-myeloablative conditioning and transplantation of umbilical cord blood from unrelated donors in patients with hematologic malignancies                                   | • Opened December 23, 2008  
• Closed March 31, 2010                            |
| BMT CTN 0703    | Tandem autologous stem cell transplantation for patients with primary progressive or recurrent Hodgkin disease (a BMT study), phase I                                                                 | • Opened March 18, 2008  
• SWOG 0410 opened August 15, 2005  
• Closed February 1, 2009                          |
| BMT CTN 0704    | A phase III, randomized, double-blind study of maintenance therapy with CC-55103 or placebo following autologous stem cell transplantation for multiple myeloma                                                                 | • Opened May 15, 2007  
• CALGB 100104 opened December 15, 2004  
• Closed July 2, 2009                              |
| BMT CTN 0402    | A phase III randomized, multi-center trial comparing sirolimus/tacrolimus with tacrolimus/methotrexate as GVHD prophylaxis after HLA-matched, related peripheral blood stem cell transplantation                                                 | • Opened November 20, 2006  
• Closed October 28, 2011                           |
<table>
<thead>
<tr>
<th>Protocol Number</th>
<th>Long Title</th>
<th>Accrual Dates</th>
</tr>
</thead>
</table>
| BMT CTN 0403    | A randomized, double-blind, placebo-controlled trial of soluble tumor necrosis factor receptor Enbrel (etanercept) for the treatment of acute non-infectious pulmonary dysfunction (idiopathic pneumonia syndrome, [IPS]) following allogeneic stem cell transplantation                                                                                                                                                                                                                      | • Opened August 27, 2007  
• Closed September 14, 2011                                                                                                                                                                                                                                                                       |
| BMT CTN 0802    | A multi-center, randomized, double blind, phase III trial evaluating corticosteroids with mycophenolate mofetil versus corticosteroids with placebo as initial systemic treatment of acute GVHD                                                                                                                                                                                                                                                                  | • Opened February 1, 2010  
• Closed November 14, 2011                                                                                                                                                                                                                                                                                                              |
| BMT CTN 0502    | A phase II study of allogeneic transplantation for older patients with acute myeloid leukemia in first morphologic complete remission using a non-myeloablative preparative regimen                                                                                                                                                                                                                                                                                                                   | • CALGB opened August 29, 2006  
• BMT CTN opened January 29, 2007  
• Closed December 29, 2008  
• Re-opened November 10, 2009 to centers with IRB approval of NCI-approved amendment to increase accrual  
• Closed December 29, 2011                                                                                                                                                                                                                                                      |
### RCI BMT Clinical Trials

<table>
<thead>
<tr>
<th>Protocol Number</th>
<th>Long Title</th>
<th>Status To Date</th>
</tr>
</thead>
</table>
| 10-CBA          | A multi-center access and distribution protocol for unlicensed cryopreserved cord blood units (CBUs) for transplantation in pediatric and adult patients with hematologic malignancies and other indications                                                                                                                                                                                                                             | • Opened to accrual October 2010  
• 141 enrolled  
• Open indefinitely to allow for distribution and access to unlicensed cord units                                                                                                                                                                                                                                                                  |
| 11-TREO         | A multi-center study evaluating a fixed regimen of treosulfan, fludarabine, and low-dose TBI in children with AML or MDS undergoing HCT from allogeneic donors                                                                                                                                                                                                                          | • Protocol in development  
• Submission to PBMTC Data Safety Monitoring Committee (DSMC) anticipated in January 2012                                                                                                                                                                                                                                                              |
| COG-KIR         | A multi-center study examining donor NK-cell receptors and patient outcomes                                                                                                                                                                                                                                                                                                                                                                                                               | • Opened to accrual January 2008  
• 239 out of 1200 targeted samples collected  
• 66 out of 400 targeted enrollment                                                                                                                                                                                                                                                                                                                             |
| KIR-DS          | A multi-center study looking at the selection of a favorable KIR donor                                                                                                                                                                                                                                                                                                                                                                                                                 | • Opened to accrual June 2011  
• 15 out of 1800 donor samples collected  
• 10 of 600 targeted enrollment                                                                                                                                                                                                                                                                                                                              |
| 10 CMS-MDS-1    | Assessment of allogeneic HCT in Medicare beneficiaries with MDS and related disorders                                                                                                                                                                                                                                                                                                                                         | • Opened to accrual December 2010  
• 136 of 240 targeted enrollment                                                                                                                                                                                                                                                                                                                                            |
| 05-DCB          | A phase II multi-center trial of myeloablative double unit umbilical cord blood transplantation (UCBT) in adults with hematologic malignancy                                                                                                                                                                                                                                                                                  | • Completed accrual October 2011  
• 56 out of 55 targeted enrollment  
• Follow-up and site monitoring continues                                                                                                                                                                                                                                                                                                                                  |
| PBMTTC ONC 1001 / 09-MRD | A multi-center study to determine the role of minimal residual disease testing before and after HCT for pediatric acute myeloid leukemia                                                                                                                                                                                                                                                                                        | • Opened to accrual October 2011  
• 16 of 25 targeted sites open  
• 6 of 150 targeted enrollment                                                                                                                                                                                                                                                                                                                                           |
| 09-PLEX         | 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                                                                                                                                                                                         | • Protocol development completed July 2011  
• In renegotiation since spring 2011 due to Genzyme Corporation merging with Sanofi-aventis  
• Expected to open to accrual mid-2012                                                                                                                                                                                                                                                                 |
| PBSC            | Filgrastim-mobilized peripheral blood stem cells for allogeneic transplantation with unrelated donors                                                                                                                                                                                                                                                                                                                                                                 | • Opened to accrual April 1996  
• More than 14,500 unrelated donors enrolled  
• Will close to accrual upon FDA license and then another protocol will open for all unlicensed product                                                                                                                                                                                                                                                     |
| Statin          | Impact of donor statin use on graft-versus-host disease (GVHD) after unrelated donor HCT                                                                                                                                                                                                                                                                                                                                            | • Opened to accrual October 2011  
• 20 of 5,000 targeted donors enrolled                                                                                                                                                                                                                                                                                                                                         |
<table>
<thead>
<tr>
<th>Protocol Number</th>
<th>Long Title</th>
<th>Status To Date</th>
</tr>
</thead>
</table>
| rHuG-CSF        | 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 | • Opened to accrual October 2010  
• More than 9,500 donors out of 32,128 targeted enrollment |
| BMT CTN 0201    | Peripheral blood versus bone marrow grafts from unrelated donors            | • Opened to accrual January 2004  
• 551 out of 550 targeted enrollment |
| 09-SQOL         | Pilot study to assess the feasibility of collecting quality of life data in collaboration with the Stem Cell Therapeutic Outcomes Database | • Opened to accrual July 2011  
• 6 sites out of 8 activated  
• 50 of 270 patients enrolled |
| DS05-02, 06-DON | RDSafe: A multi-institutional study of hematopoietic stem cell donor safety and quality of life | • Opened to accrual January 2010  
• 754 of 2,300 patients enrolled |
| 07-REV          | Revlimid as maintenance therapy post-transplant: evaluating the safety and tolerability of lenalidomide after allogeneic HCT | • Opened to accrual February 2009  
• 28 of 30 patients enrolled |
This study development cycle pertains to studies for which CIBMTR provides data and statistical support. Data sets are also made available to investigators who have their own statistical resources. Manuscripts resulting from these analyses are reviewed and approved by CIBMTR prior to journal submission.

### Study Development Cycle

<table>
<thead>
<tr>
<th>Stage</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Planned</td>
<td>Proposal pending. Proposals remain in this preliminary stage until a draft protocol is created.</td>
</tr>
<tr>
<td>Protocol pending</td>
<td>Protocol development. The PI and invited WC members develop a study proposal into a comprehensive study protocol. Statistical Center staff use the protocols to prepare data files for analysis and define the detailed study design. This crucial document defines the study and how it progresses.</td>
</tr>
<tr>
<td>Draft protocol received</td>
<td>When a draft protocol is received by the Statistical Center, it is refined in collaboration with the WC biostatisticians, Scientific Director, and Chairs. A table with a preliminary description of the proposed study population is added, and the draft protocol is presented for discussion at a weekly Statistical Center meeting. When a protocol is approved, WC members are invited to participate in a Writing Committee.</td>
</tr>
<tr>
<td>Ongoing</td>
<td>Ongoing. When a study is ongoing, there is a grant funding source outside of CIBMTR that is supporting the study. The study has its own PhD biostatisticians for analysis, but it requires data from CIBMTR, usually each year. The study is long-term and often involves multiple grants and/or renewals in order to reach its objectives.</td>
</tr>
<tr>
<td>Sample Typing</td>
<td>Sample Typing. During sample typing, the PIs perform laboratory tests (e.g., genotyping) on samples from the CIBMTR Research Sample Repository. The testing data will later be used in the analysis to determine any correlation with clinical outcome.</td>
</tr>
<tr>
<td>Supplemental forms/data collection</td>
<td>Supplemental forms/data collection. Most studies use routinely-collected data. Use of supplemental data (e.g., data not collected on CIBMTR CRFs) is discouraged unless it will result in a particularly meaningful publication and/or external funding can support the extra burden placed on transplant centers and supplement forms reimbursement costs. If necessary, a supplemental form must be developed and approved prior to soliciting centers for additional data. These forms are prepared by Statistical Center staff in collaboration with the PI and relevant WC Co-Chairs.</td>
</tr>
</tbody>
</table>
| Data file preparation | Data file preparation. The objective of data file preparation is to create a file of eligible subjects who are consecutively treated at participating centers with adequate follow-up, with minimal missing data fields, and in large enough numbers to give the analysis sufficient statistical power to meet the stated study objectives. This process involves a series of steps by the MS Statistician, sometimes working with the CRC, to ensure data quality:  
  - Verifying selection criteria  
  - Assessing follow-up Determining the extent and nature of missing values and their potential effect on the study  
  - Resolving and reconciling data discrepancies/outliers by examining data collection forms and communicating with centers and the PI  
  - Including and excluding patients so that the investigators can determine whether the final study population is representative of the target population (unbiased sample) |
| Analysis in progress | Analysis in progress. Analysis proceeds in several phases. The first generally includes a detailed description of the patient population and univariate analyses of study endpoints. These data are distributed to Writing Committee members for suggestions and comments. An iterative process then ensues, in which the PI works with Statistical Center staff to review comments from the Writing Committee. The process is repeated until final analysis, which serves as the basis for the manuscript. |
### Study Development Cycle (continued)

<table>
<thead>
<tr>
<th>Stage</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Preliminary Results</td>
<td><strong>Manuscript preparation.</strong> The PI is primarily responsible for manuscript preparation and is expected to prepare a draft manuscript within 30 days of receiving analysis results. After review and revision, it is approved by WC leadership, who ensure that the description and interpretation of the statistical analyses are accurate and contribute to the fundamental message of the manuscript.</td>
</tr>
<tr>
<td></td>
<td><strong>Submitted.</strong> The Statistical Center staff is responsible for submitting the manuscript and corresponding with the chosen journal. The WC Scientific Director often serves as corresponding author. The MS Statistician forwards all editor and reviewer comments to the PI and PhD statisticians. The PI is expected to prepare a response, if necessary. Communication with the journal, including re-submissions, is done by Statistical Center personnel in most cases.</td>
</tr>
<tr>
<td></td>
<td><strong>In press.</strong> A publication is in press when it has been approved but does not yet have a citation.</td>
</tr>
<tr>
<td>Completed</td>
<td><strong>Published.</strong> A manuscript is considered published when a citation is available, including a PMCID number, if applicable. For a list of 2011 publications, see Appendix H.</td>
</tr>
</tbody>
</table>
The following table describes the forms submission processes for the TED and CRF tracks. For more information about data collection, see Section 4.

<table>
<thead>
<tr>
<th>CIBMTR Forms Submission Process</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Center submits CRID Assignment Form (Form 2804)</td>
</tr>
<tr>
<td>• CRID is generated – Stop here if autologous recipient declines consent for research participation¹</td>
</tr>
<tr>
<td>• Pre-TED (Form 2400) is added to Forms Due list</td>
</tr>
<tr>
<td>• Center completes and submits Pre-TED</td>
</tr>
<tr>
<td>• Pre-TED data are processed through the selection algorithm resulting in CRF or TED track</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>CRF Track²</th>
<th>TED Track</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 Forms 2004 and 2005 are due for each non-NMDP allogeneic donor. Form 2005 is due for each non-NMDP allogeneic recipient. Form 2006 is added for all products.</td>
<td>Forms 2004 and 2005 are added if participating in related specimen repository and all non-NMDP cord blood units Form 2006 due for all NMDP products and all cord blood units.</td>
</tr>
<tr>
<td>2 Baseline and Follow-up Forms are added to the Forms Due list.</td>
<td>Post-TED Follow-up Form 2450 are added to Forms Due list.</td>
</tr>
<tr>
<td>3 Center completes Baseline form after infusion.</td>
<td>Center completes designated Post-TED Forms.³</td>
</tr>
<tr>
<td>4 Center completes designated CRF Follow-up Forms.³</td>
<td>Is recipient alive? If yes, go to Step 5. If no, go to Recipient Death Table.</td>
</tr>
<tr>
<td>5 Follow-up Form entered.</td>
<td>Did recipient have a subsequent transplant? If yes, go to Step 6. If no, go to Step 3.</td>
</tr>
<tr>
<td>6 Is recipient alive? If yes, go to Step 7. If no, go to Recipient Death Table.</td>
<td>Contact Center Liaison with date of subsequent transplant.</td>
</tr>
<tr>
<td>7 Did recipient have subsequent transplant? If yes, go to Step 8. If no, continue reporting at next time point (Step 4).</td>
<td>Center completes and submits Pre-TED (Form 2400) for subsequent transplant. Go to Step 2.</td>
</tr>
<tr>
<td>8 Future time points will be deleted for prior transplant from FormsNet when the form reporting the subsequent transplant is error free. Go to Step 2 for subsequent transplant.</td>
<td>Future time points will be deleted for prior transplant from FormsNet when the form reporting the subsequent transplant is error free.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Recipient Death Table</th>
</tr>
</thead>
<tbody>
<tr>
<td>Death Form 2900 is completed to report the recipient’s death.* If a follow-up form is received and reports the recipient’s death, and a form 2900 has not been submitted, one will be made due on the Forms Due list.</td>
</tr>
</tbody>
</table>

¹ If center has chosen to submit autologous forms without consent, follow the TED track.
² Recipient has the option to withdraw consent at any time. If this happens, the Center Liaison moves this transplant record to the TED Track.
³ Designated time points are 100 days, 6 months, and annually post-transplant.
<table>
<thead>
<tr>
<th>Abbreviation/Acronym</th>
<th>Meaning</th>
</tr>
</thead>
<tbody>
<tr>
<td>ABMTR</td>
<td>Autologous Bone Marrow Transplant Registry</td>
</tr>
<tr>
<td>AE</td>
<td>adverse event</td>
</tr>
<tr>
<td>AGNIS</td>
<td>A Growable Network Information System</td>
</tr>
<tr>
<td>aGVHD</td>
<td>acute graft-versus-host disease</td>
</tr>
<tr>
<td>AID</td>
<td>autoimmune disease</td>
</tr>
<tr>
<td>ALL</td>
<td>acute lymphoblastic leukemia</td>
</tr>
<tr>
<td>AlloHCT</td>
<td>allogeneic hematopoietic cell transplantation</td>
</tr>
<tr>
<td>AML</td>
<td>acute myeloid (myelogenous) leukemia</td>
</tr>
<tr>
<td>AP</td>
<td>accelerated phase</td>
</tr>
<tr>
<td>APBMT</td>
<td>Asia Pacific Blood and Marrow Transplantation Group</td>
</tr>
<tr>
<td>ARRA</td>
<td>American Recovery and Reinvestment Act of 2009</td>
</tr>
<tr>
<td>ASBMT</td>
<td>American Society of Blood and Marrow Transplantation</td>
</tr>
<tr>
<td>ASH</td>
<td>American Society of Hematology</td>
</tr>
<tr>
<td>ATG</td>
<td>anti-thymocyte globulin</td>
</tr>
<tr>
<td>ATO</td>
<td>Authorization to Operate</td>
</tr>
<tr>
<td>AutoHCT</td>
<td>autologous hematopoietic cell transplantation</td>
</tr>
<tr>
<td>BM</td>
<td>bone marrow</td>
</tr>
<tr>
<td>BMI</td>
<td>Body Mass Index</td>
</tr>
<tr>
<td>BMT</td>
<td>bone marrow transplant</td>
</tr>
<tr>
<td>BMT CTN</td>
<td>Blood and Marrow Transplant Clinical Trials Network</td>
</tr>
<tr>
<td>BP</td>
<td>blast phase</td>
</tr>
<tr>
<td>BRIDG</td>
<td>Biomedical Research Integrated Domain Group</td>
</tr>
<tr>
<td>Bu</td>
<td>busulfan</td>
</tr>
<tr>
<td>BuCy</td>
<td>busulfan/cyclophosphamide</td>
</tr>
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<td>Center for International Blood and Marrow Transplant Research</td>
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<tr>
<td>Abbreviation/Acronym</td>
<td>Meaning</td>
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