CIBMTR Scientific
Working Committee
Research Portfolio

July 1, 2019

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cibmtr.org
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1.0 OVERVIEW

The CIBMTR® (Center for International Blood and Marrow Transplant Research®) collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy research worldwide to increase survival and enrich quality of life for patients. A research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin, the CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of participating centers, a unique and extensive clinical database, and a robust and comprehensive biospecimen repository.

Fifteen international Scientific Working Committees oversee most of the CIBMTR’s clinical outcomes research. Each committee focuses on a specific disease or condition, use of cellular therapy, or complication of treatment (Table 1). This report details the Scientific Working Committee Research Portfolio as of July 1, 2019.

Table 1. Working Committee Focus Areas

<table>
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<th>Working Committee</th>
<th>Scientific Focus</th>
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<td>Acute Leukemia</td>
<td>HCT for acute leukemia and pre-leukemia</td>
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<tr>
<td>Cellular Immunotherapy for Cancer</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 graft-versus-host disease and its complications</td>
</tr>
<tr>
<td>Health Services and International Studies</td>
<td>Social and economic barriers to HCT access, including quality of care and the influence of psychosocial factors on transplant outcomes, as well as international issues and differences in HCT</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>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 lymphoma</td>
</tr>
<tr>
<td>Non-Malignant Diseases</td>
<td>HCT and cellular therapy for non-malignant diseases, including autoimmune diseases, inherited and acquired marrow failure, hemoglobinopathy, immunodeficiency diseases, and inborn errors of metabolism</td>
</tr>
</tbody>
</table>
## 1.0 Overview

<table>
<thead>
<tr>
<th>Working Committee</th>
<th>Scientific Focus</th>
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<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 and Adult Solid Tumors</td>
<td>HCT for multiple myeloma and other plasma cell disorders as well as solid tumors in adults</td>
</tr>
<tr>
<td>Regimen-Related Toxicity and Supportive Care</td>
<td>Preparative regimens, prevention, and treatment of early non-graft-versus-host disease toxicities; supportive care in the early post-transplant period</td>
</tr>
</tbody>
</table>

### 1.1 Membership

Total Working Committee membership exceeds 2,800 researchers. Membership is open to any researcher willing to take an active role in developing and conducting studies that use CIBMTR data and/or resources. While most of these individuals are HCT clinicians, statisticians and basic scientists also participate. PhD Statistical Directors and Master’s-level statisticians from the CIBMTR Coordinating Center provide their unique expertise in data analysis. Basic scientists investigating HLA, immunogenetics, pharmacogenetics, stem cell biology, and other areas related to HCT provide essential expertise in their respective research areas. The Working Committee structure encourages a collaborative but rigorous methodological approach to all CIBMTR activities.

### 1.2 Leadership

Each Working Committee is staffed by at least one MS-level Statistician, a PhD Statistical Director, and an MD Scientific Director from the CIBMTR Coordinating Center. Each also typically has two to four Chairs who are appointed by the Advisory Committee.

#### 1.2.1 Committee Chairs

Working Committee Chairs are appointed by the Advisory Committee to non-renewable five-year terms. Appointments are made each fall, with terms commencing on March 1 of the following year. Terms are staggered to facilitate succession and maintain continuity. Individuals may serve as Chair more than once but not consecutively for the same committee. Chairs participate in the nomination process for replacement positions and give special consideration to promising junior investigators, thus promoting ongoing leadership for the work of the CIBMTR.

Working Committee Chairs provide subject matter expertise in cellular therapy as well as understanding of CIBMTR organization and procedures. They must be members of CIBMTR centers that submit comprehensive report form (CRF)-level data and are compliant with continuous process improvement standards for data submission, unless an exception is granted by the Advisory Committee. 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 the CIBMTR and to the field of cellular therapy.
Chairs monitor and facilitate the progress of studies in their Working Committee’s portfolio. They communicate with principal investigators (PIs) to address barriers and/or delays and participate in weekly CIBMTR Coordinating Center study critiques when studies in their portfolios are being discussed. In addition to chairing annual Working Committee meetings, Chairs meet by teleconference every four to six weeks with their committee’s Scientific Director and biostatisticians to review the progress of study proposals and ongoing studies. Chairs lead the annual Working Committee meeting, and, using input from that meeting, they prioritize studies and establish the research agenda for the following year.

1.3 Productivity

There are currently 181 studies in progress, 63 of which are collaborations with other organizations (Appendix A). At the 2019 TCT Meetings, 39 new study proposals were approved. The prioritization and selection process (Figure 1) ensures the most important issues can be addressed in a timely manner.

During the past year, Working Committee study investigators published 54 manuscripts in peer-reviewed journals, approximately half of the total number of CIBMTR publications. In each committee’s section of this report, publications since July 1, 2014, are listed (Sections 2.2-16.2). For a complete list of CIBMTR publications, visit the CIBMTR Publication List webpage.

Working Committee study investigators presented 33 abstracts (16 oral and 17 poster) at national and international conferences this year. These presentations include 22 (13 oral and 9 poster) at the 2018 American Society of Hematology Annual Meeting, 9 (2 oral and 7 poster) at the 2019 TCT Meetings, and 2 (1 oral and 1 poster) at other conferences.

1.4 How to Get Involved

Working Committees are collaborative in nature, and all interested individuals are encouraged to participate:

- **Join a Working Committee.** Learn more about each committee on the CIBMTR Working Committee webpage. To join a Working Committee, email contactus@cibmtr.org, contact the Working Committee leadership listed on the individual committee’s webpage, or attend a Working Committee Meeting at the TCT | Transplantation & Cellular Therapy Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) and CIBMTR (TCT Meetings).

- **Attend a Working Committee Meeting at the TCT Meetings.** All TCT Meeting attendees may attend to learn more about the committee, its recent publications and current studies, and have the opportunity to learn about and provide feedback on new study proposals.

- **Participate in a Writing Committee.** When a draft protocol is approved by the Working Committee leadership and Coordinating Center, all Working Committee members on record are invited to participate in the study Writing Committee.

- **Propose a Study.** Anyone willing to follow the study development and management process (Appendix B) is eligible to propose a study to the Working Committees (Figure
1. Guidelines for CIBMTR study PIs, including hints and tips to make the study process as successful as possible, are provided in Appendix C.

For more information regarding participation in a Working Committee, access the "Learn more about how to get involved in a Working Committee" section on the CIBMTR Working Committee webpage.
**Figure 1. Working Committee Study Proposal Review Process**

**Submission**
- By mid-November, study investigator submits proposal to the CIBMTR Coordinating Center for consideration at the next TCT Meetings.

**Initial Review**
- Working Committee Leadership reviews for feasibility with CIBMTR data, potential conflict with active studies, scientific merit, and ability to complete the study in a timely fashion. Researchers with similar concepts may be advised to combine their proposals.

**Preliminary Assessment**
- If Working Committee Leadership clears the proposal to move forward, the MS-level Statistician contacts the study investigator and prepares a table of characteristics of patient data based on the population defined in the proposal.

**Presentation**
- Study investigator presents the proposal at the Working Committee meeting at the February TCT Meetings.

**Voting**
- Working Committee members vote for each proposal, assigning a scientific impact score to each.

**Final Approval**
- Working Committee Leadership utilizes member feedback in determining which proposals to pursue. Advisory Committee approves the CIBMTR research agenda.

**Notification**
- Working Committee Leadership contacts study investigator to notify of study approval / rejection by the beginning of April.
2.0 ACUTE LEUKEMIA WORKING COMMITTEE

2.1 Leadership

Chair: Brenda Sandmaier, MD, Fred Hutchinson Cancer Research Center  
Email: bsandmai@fredhutch.org

Chair: Mark Litzow, MD, Mayo Clinic Rochester  
Email: litzow.mark@mayo.edu

Chair: Partow Kebriaei, MD, MD Anderson Cancer Center  
Email: pkebriaei@mdanderson.org

Scientific Director: Daniel Weisdorf, MD, CIBMTR Minneapolis  
Email: weisd001@umn.edu

Asst Sci Director: Wael Saber, MD, MS, CIBMTR Milwaukee  
Email: wsaber@mcw.edu

Statistical Director: Mei-Jie Zhang, PhD, CIBMTR Milwaukee  
Email: meijie@mcw.edu

MS Statistician: Karen Chen, MS, CIBMTR Milwaukee  
Email: kachen@mcw.edu

2.2 Recent Publications

2019


2016


**2015**


**2014**


**2.3 Current Studies**

**LK13-02**

**Title:** Prognostic significance of cytogenetic abnormalities in patients with Philadelphia-negative acute lymphoblastic leukemia undergoing allogeneic hematopoietic stem cell transplantation in complete remission

**PIs:** Aleksandr Lazaryan (H. Lee Moffitt Cancer Center and Research Institute) Veronika Bachanova (University of Minnesota Blood and Marrow Transplant Program)

**Status:** Submitted (as of July 1, 2019) Published (expected by June 30, 2020)
LK15-03
Title: **Comparison of outcomes of older adolescents and young adults with Philadelphia-chromosome/BCR-ABL1-negative acute lymphoblastic leukemia receiving post-remission consolidation chemotherapy with pediatric-inspired chemotherapy on CALGB 10403 or myeloablative allogeneic hematopoietic cell transplantation**
PIs: Matthew Wieduwilt (University of California, San Diego Medical Center)
Wendy Stock (University of Chicago Medicine)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with CALGB 10403

LK16-01
Title: **Reduced intensity conditioning regimens for acute myeloid leukemia: A comparison of busulfan and melphalan based regimens from the CIBMTR database**
PIs: Zartash Gul (University of Cincinnati Medical Center)
Hassan Alkhateeb (Mayo Clinic Rochester)
Rajneesh Nath (Banner MD Anderson Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LK16-02
Title: **DRI-guided choice of conditioning intensity for allogeneic hematopoietic cell transplantation in adults with acute myeloid leukemia and myelodysplastic syndromes**
PIs: Nelli Behanyan (H. Lee Moffitt Cancer Center and Research Institute)
Erica Warlick (University of Minnesota Blood and Marrow Transplant Program)
Claudio Brunstein (University of Minnesota Blood and Marrow Transplant Program)
Daniel Weisdorf (University of Minnesota Blood and Marrow Transplant Program)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LK16-03
Title: **Allogeneic transplantation to treat therapy related acute myeloid leukemia and myelodysplastic syndromes**
PIs: Natalie Callander (University of Wisconsin Hospital and Clinics)
Leland Metheny (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Marcos De Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Aric Hall (University of Wisconsin Hospital and Clinics)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
LK17-01
Title: Outcomes of acute myeloid leukemia patients who undergo allogeneic transplant stratified by depth of clinical response
PIs: Mary-Elizabeth Percival (University of Washington)
Brenda Sandmaier (Fred Hutchinson Cancer Research Center)
Eli Estey (M.D. Anderson Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LK17-02
Title: Allogeneic hematopoietic transplant outcomes in adult patients with MLL-rearranged acute myeloid leukemia
PIs: Kamal Menghrajani (Memorial Sloan Kettering Cancer Center)
Martin Tallman (Memorial Sloan Kettering Cancer Center)
Status: Analysis (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LK17-03
Title: Impact of post-transplant maintenance therapy with BCR-ABL tyrosine kinase inhibitors on outcomes of Philadelphia chromosome-positive acute lymphoblastic leukemia
PIs: Zack DeFilipp (Massachusetts General Hospital)
Yi-Bin Chen (Massachusetts General Hospital)
Status: Data File Preparation (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

LK18-01
Title: Prognostic impact of the new European LeukemiaNet genetic risk stratification categories in predicting outcomes for adults with acute myeloid leukemia undergoing allogeneic hematopoietic stem cell transplantation
PIs: Antonio Jimenez (University of Miami)
Trent Wang (Fox Chase Temple University Hospital Bone Marrow Transplant Program)
Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Krishna Komanduri (University of Miami)
Status: Data File Preparation (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
LK18-02
Title: **Comparison of outcomes of HCT with matched-related donor or matched-unrelated donor alloHCT for adults with acute lymphoblastic leukemia**
PIs: Matthew Wieduwilt (University of California, San Diego Medical Center)
     Leland Metheny (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
     Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Status: Protocol Development (as of July 1, 2019)
        Manuscript Preparation (expected by June 30, 2020)

LK19-01
Title: **Evaluating outcomes of hematopoietic cell transplantation in blastic plasmacytoid dendritic cell neoplasm**
PI: Hemant Murthy (Mayo Clinic Florida)
Status: Protocol Pending (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

LK19-02
Title: **Evolving significance of Ph-chromosome status on ALL prognosis in the TKI era**
PIs: Maxwell Krem (James Graham Brown Cancer Center, University of Louisville School of Medicine)
     Richard Maziarz (Oregon Health and Science University)
Status: Protocol Pending (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

LK19-03
Title: **Outcomes of alloHCT in AML patients who achieved first complete remission after two or more cycles of induction chemotherapy**
PIs: Michael Boyiadzis (University of Pittsburgh Medical Center - Cancer Center)
     Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Status: Protocol Pending (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)
3.0 CELLULAR IMMUNOTHERAPY FOR CANCER WORKING COMMITTEE

3.1 Leadership

Chair: Sarah Nikiforow, MD, PhD, Dana Farber Cancer Institute  
     Email: snikiforow@partners.org
Chair: Peiman Hematti, MD, University of Wisconsin Hospital and Clinics  
     Email: pxh@medicine.wisc.edu
Scientific Director: Marcelo Pasquini, MD, MS, CIBMTR Milwaukee  
     Email: mpasquini@mcw.edu
Statistical Director: Ruta Brazauskas, PhD, CIBMTR Milwaukee  
     Email: ruta@mcw.edu
MS Statistician: Xianmiao Qiu, MS, CIBMTR Milwaukee  
     Email: xqiu@mcw.edu

3.2 Recent Publications

2018

2017
3.3 Current Studies

**AC16-01**
Title: **Pattern of use and outcomes with donor lymphocyte infusion after HLA-haploidentical allogeneic hematopoietic stem cell transplant**
PIs: Eva Gupta (Mayo Clinic Florida)
     James Foran (Mayo Clinic Florida)
     Vivek Roy (Mayo Clinic Florida)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

**AC17-01**
Title: **CD-19 chimeric antigen receptor T cells with or without hematopoietic cell transplantation for treatment of refractory acute lymphocytic leukemia**
PIs: Miguel-Angel Perales (Memorial Sloan Kettering Cancer Center)
     Jae Park (Memorial Sloan Kettering Cancer Center)
     Sarah Nikiforow (Dana Farber Cancer Institute)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

**AC18-01**
Title: **Effect of stem cell boost and donor lymphocyte infusion on the incidence of GVHD**
PIs: James Yoon (Emory University Hospital)
     Edmund Waller (Emory University)
Status: Draft Protocol Received (as of July 1, 2019)
Protocol Development (expected by June 30, 2020)

**CT13-01**
Title: **Utility of donor leukocyte infusion for the treatment of drug-resistant viral or fungal infections in allogeneic HCT recipients: A CIBMTR analysis**
PI: Gorgun Akpek (Rush University Medical Center)
Status: Analysis (as of July 1, 2019)
Submitted (expected by June 30, 2020)

**CT19-01**
Title: **Allogeneic hematopoietic cell transplantation vs chimeric antigen receptor t-cell therapy for DLBCL patients with prior autologous transplant failure or refractory disease**
PIs: Mehdi Hamadani (Medical College of Wisconsin)
     Marcelo Pasquini (Medical College of Wisconsin)
     Frederick Locke (H. Lee Moffitt Cancer Center and Research Institute)
     Ajay Gopal (Fred Hutchinson Cancer Research Center)
Status: Protocol Pending (as of July 1, 2019)
Submitted (expected by June 30, 2020)
CT19-02
Title: **Prolonged cytopenia following CD-19 targeted CAR-T therapy for diffuse large B-cell lymphoma**
PI: Mazyar Shadman (Fred Hutchinson Cancer Research Center)
Status: Protocol Pending (as of July 1, 2019)
Submitted (expected by June 30, 2020)

CT19-03
Title: **Patient outcomes after CAR-T cells**
PI: Marcelo Pasquini (Medical College of Wisconsin)
Status: Protocol Development (as of July 1, 2019)
Submitted (expected by June 30, 2020)
4.0 CHRONIC LEUKEMIA WORKING COMMITTEE

4.1 Leadership

Chair: Ronald Sobecks, MD, Cleveland Clinic Foundation  
Email: sobeckr@ccf.org

Chair: Bart Scott, MD, Fred Hutchinson Cancer Research Center  
Email: bscott@fredhutch.org

Chair: Ryotaro Nakamura, MD, City of Hope  
Email: rnakamura@coh.org

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

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

Soyoun Kim, PhD, CIBMTR Milwaukee  
Email: skim@mcw.edu

MS Statistician: Noel Estrada-Merly, MPH, CIBMTR Milwaukee  
Email: nestrada@mcw.edu

4.2 Recent Publications

2019


2018

2017


2016


4.3 Current Studies

CK12-01
Title: **Optimal timing of allogeneic stem cell transplantation for chronic myeloid leukemia patients in the tyrosine kinase inhibitor era**
PI: Hans Lee (M.D. Anderson Cancer Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with M.D. Anderson Cancer Center

CK15-01
Title: **Comparison of transplant versus non-transplant therapies for myelofibrosis**
PIs: Krisstina Gowin (Mayo Clinical Arizona and Phoenix Children’s Hospital)
Ruben Mesa (Mayo Clinical Arizona and Phoenix Children’s Hospital)
Karen Ballen (University of Virginia Health System)
Status: Submitted (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with Mayo Clinic Arizona and Phoenix Children’s Hospital, Duke University Medical Center, Massachusetts General Hospital, M.D. Anderson Cancer Center, Cleveland Clinic Foundation, Dana-Farber Cancer Institute, Medical College of Wisconsin, Memorial Sloan Kettering Cancer Center, H. Lee Moffit Cancer Center and Research Institute, Northwestern University, University of Michigan, Washington University

CK15-03
Title: **Outcome of allogeneic hematopoietic cell transplantation in patients with acute myeloid leukemia with antecedent history of Philadelphia-negative myeloproliferative neoplasm**
PI: Vikas Gupta (Princess Margaret Hospital)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

CK16-01
Title: **Identification of germline predisposition mutations in young myelodysplastic syndrome patients**
PI: Lucy Godley (University of Chicago Medicine)
Status: Sample Typing (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)
* Collaborative study with University of Chicago
CK16-02a
Title: Contemporary role of maintenance tyrosine kinase inhibitors following allogeneic hematopoietic cell transplantation for chronic myeloid leukemia: a CIBMTR analysis
PIs: Zach DeFilipp (Massachusetts General Hospital)
     Richard Ancheta (Scripps Blood & Marrow Transplant Program)
Status: Submitted (as of July 1, 2019)
        Published (expected by June 30, 2020)

CK16-02b
Title: The benefit of donor lymphocyte infusion in the tyrosine kinase inhibitors era in chronic myeloid leukemia post allogeneic hematopoietic cell transplantation
PI: Sarah Schmidt (OU Medical Center and The Children's Hospital at OU Medical Center)
Status: Submitted (as of July 1, 2019)
        Published (expected by June 30, 2020)

CK17-01
Title: Development of a prognostic scoring system predictive of outcomes in patients with myelofibrosis after allogeneic hematopoietic cell transplantation
PI: Roni Tamari (Memorial Sloan Kettering Cancer Center)
Status: Data File Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)
        * Collaborative study with the European Society for Blood and Marrow Transplantation (EBMT)

CK17-02
Title: Reduced-intensity conditioning transplantation in older myelodysplastic syndrome: the effect of specific conditioning regimens on transplant outcomes
PI: Betul Oran (M.D. Anderson Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)

CK18-01
Title: A personalized prediction model for outcomes after allogeneic stem cell transplant in patients with myelodysplastic syndromes
PIs: Aziz Nazha (Cleveland Clinic Foundation)
     Navneet Majhail (Cleveland Clinic Foundation)
     Wael Saber (Medical College of Wisconsin)
     Betty Hamilton (Cleveland Clinic Foundation)
Status: Manuscript Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)
CK18-02
Title: **The impact of somatic mutations on allogeneic transplant in chronic myelomonocytic leukemia**
PIs: Matthew Mei (City of Hope)
Ryotaro Nakamura (City of Hope)
Raju Pillai (City of Hope)
Status: Sample Typing (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

CK18-03
Title: **Impact of donor age on the outcomes of allogeneic hematopoietic stem cell transplantation for myelodysplastic syndrome**
PIs: Guru Murthy (Medical College of Wisconsin)
Wael Saber (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

CK19-01a
Title: **Outcomes after HCT for rare chronic leukemias: Evaluating outcomes of Allogeneic hematopoietic cell transplantation in T-cell prolymphocytic leukemias**
PIs: Hemant Murthy (Mayo Clinic Florida)
Bhagi Dholaria (Vanderbilt University Medical Center)
Mohamed Kharfan (Mayo Clinic Florida)
Susan Bal (University of Alabama at Birmingham)
Craig Sauter (Memorial Sloan Kettering Cancer Center)
Lohith Gowda (Yale New Haven Hospital)
Francine Foss (Yale New Haven Hospital)
Hassan Alkhateeb (Mayo Clinic Rochester)
Deepa Jagadeesh (Cleveland Clinic Foundation)
Bipin Savani (Vanderbilt University Medical Center)
Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)

CK19-01b
Title: **Outcomes after HCT for rare chronic leukemias: Outcomes of chronic neutrophilic leukemia patients who underwent alloHCT**
PIs: Bhagi Dholaria (Vanderbilt University Medical Center)
Bipin Savani (Vanderbilt University Medical Center)
Mohamed Kharfan (Mayo Clinic Florida)
Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)
* Collaborative study with EBMT
SC11-06 / 10-CMSMDS-1
Title: Assessment of allogeneic hematopoietic stem cell transplantation in Medicare beneficiaries with myelodysplastic syndrome and related disorders
PIs: Ehab Atallah (Medical College of Wisconsin)
      JD Rizzo (Medical College of Wisconsin)
Status: Submitted (as of July 1, 2019)
        Published (expected by June 30, 2020)
5.0 DONOR HEALTH AND SAFETY WORKING COMMITTEE

5.1 Leadership

**Chair:** Nirali Shah, MD, MHSc, National Cancer Institute  
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**Chair:** Galen Switzer, PhD, University of Pittsburgh Medical Center  
Email: switzerge@upmc.edu

**Chair:** Jack Hsu, MD, Shands HealthCare & University of Florida  
Email: hsujw@medicine.ufl.edu

**Scientific Director:** Bronwen Shaw, MBChB, MRCP, PhD, CIBMTR Milwaukee  
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**Ex Officio Sr Advisor:** Dennis Confer, MD, CIBMTR Minneapolis  
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**Statistical Director:** Brent Logan, PhD, CIBMTR Milwaukee  
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**MS Statistician:** Stephanie Bo-Subait, MPH, CIBMTR Minneapolis  
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5.2 Recent Publications

2019


2018


2017


2016


2015


2014


### 5.3 Current Studies

**DS05-02d**

**Title:** QoL for related adult donors compared to unrelated adult donors  
**PIs:** Galen Switzer (University of Pittsburgh Medical Center)  
Michael Pulsipher (Children's Hospital of Los Angeles)  
**Status:** Manuscript Preparation (as of July 1, 2019)  
Submitted (expected by June 30, 2020)  
* Collaborative study with University of Utah

**DS05-02g**

**Title:** Late toxicities and SAE for related donors  
**PI:** Michael Pulsipher (Children's Hospital of Los Angeles)  
**Status:** Analysis (as of July 1, 2019)  
Submitted (expected by June 30, 2020)  
* Collaborative study with University of Utah
DS13-02
Title: A retrospective analysis to understand the potential mechanisms underlying the clinical impact of ABO incompatibility on allogeneic transplant outcomes
PI: Guru Murthy (Medical College of Wisconsin)
Status: Protocol Development (as of July 1, 2019)
Analysis (expected by June 30, 2020)

DS16-01
Title: Comparison between one and two day apheresis in unrelated donors
PIs: Jack W. Hsu (Shands HealthCare & University of Florida)
John W. Wingard (Shands HealthCare & University of Florida)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

DS16-S2
Title: Survey of the screening and management for clonal disorders of hematopoiesis in related allogeneic donors
PI: Matthew Seftel (CancerCare Manitoba / University of Manitoba)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

DS17-01
Title: The impact of donor body mass index on collection of G-CSF mobilized peripheral blood progenitor cells from unrelated donors
PIs: Nosha Farhadfar (Shands HealthCare & University of Florida)
Jack Hsu (Shands HealthCare & University of Florida)
John Wingard (Shands HealthCare & University of Florida)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

DS17-02
Title: Impact of pre-operative collection of auto blood for BM harvest on donor health and outcome
PIs: Nosha Farhadfar (Shands HealthCare & University of Florida)
John Wingard (Shands HealthCare & University of Florida)
Hemant Murthy (Mayo Clinic Florida)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

* Collaborative study with American Association of Blood Banks (AABB) / American Society for Transplantation and Cellular Therapy (ASTCT)
DS18-01
Title: To quantify age-related clonal hematopoiesis in healthy marrow and blood donors that may affect the clinical outcome of recipients following HCT
PI: Todd Druley (Washington University School of Medicine)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with Washington University

DS18-02
Title: Factors affecting CD34+ cell yields at subsequent marrow/PBSC collections.
PIs: Sandhya Panch (National Institutes of Health Clinical Center)
David Stronecek (National Heart, Lung and, Blood Institute)
Bipin Savani (Vanderbilt University Medical Center)
Nirali N. Shah (National Institutes of Health)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

DS19-01
Title: Effect of Donor Graft Cryopreservation on Allogeneic Transplant Recipient Outcomes
PIs: Jack Hsu (Shands HealthCare & University of Florida)
Nosha Farhadfar (Shands HealthCare & University of Florida)
Hemant Murthy (Mayo Clinic Florida)
John Wingard (Shands HealthCare & University of Florida)
Status: Draft Protocol Received (as of July 1, 2019)
Analysis (expected by June 30, 2020)

DS19-02
Title: The Impact of pre-apheresis Health related quality of life on peripheral blood progenitor cells yield and donor’s health and outcome
PIs: Nosha Farhadfar (Shands HealthCare & University of Florida)
John Wingard (Shands HealthCare & University of Florida)
Galen Switzer (University of Pittsburgh Medical Center)
Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)
* Collaborative study with University of Pittsburgh
6.0 GRAFT SOURCES AND MANIPULATION WORKING COMMITTEE

6.1 Leadership

**Chair:** Asad Bashey, MD, PhD, The Blood and Marrow Transplant Program at Northside Hospital  
Email: abashey@bmtga.com

**Chair:** Ian McNiece, PhD, University of Miami, CellMED Consulting  
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6.2 Recent Publications

**2019**


**2018**


2017


2016


2015


### 6.3 Current Studies

**GS18-01**

**Title:** Mismatch in the setting of PT-CY based anti-GVHD prophylaxis: Is a matched related, matched unrelated or haploidentical donor still an issue? / Use of post-transplant cyclophosphamide in alloHCT: HLA mismatched donors vs haploidentical donors vs fully matched unrelated donor vs fully matched related donors

**PIs:** Saurabh Chhabra (Medical College of Wisconsin)  
Kehinde Adekola (Northwestern Medicine)  
Mahasweta Gooptu (Thomas Jefferson University Hospital, Inc.)  
Miguel-Angel Perales (Memorial Sloan Kettering Cancer Center)  
Alberto Mussetti (Istituto Nazionale Tumori)  
Rizwan Romee (Dana Farber Cancer Institute at Brigham and Women's Hospital)

**Status:** Draft Protocol Received (as of July 1, 2019)  
Submitted (expected by June 30, 2020)
GS18-02
Title: Impact of racial background on survival following haploidentical donor transplantation with post-transplant cyclophosphamide for adults with hematologic malignancies and comparison with race-specific outcomes following umbilical cord blood transplantation
PIs: Scott Solomon (The Blood and Marrow Transplant Program at Northside Hospital)
Asad Bashey (The Blood and Marrow Transplant Program at Northside Hospital)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

GS18-03
Title: Comparison of outcomes of reduced intensity transplantation in lymphoma patients using haploidentical related donors vs. unrelated cord blood
PIs: Giancarlo Fatobene (Seattle Cancer Care Alliance)
Vanderson Rocha (Churchill Hospital)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with EBMT

GS18-04
Title: Comparison of outcomes with haploidentical and matched unrelated donors for hematopoietic stem cell transplantation in myelodysplastic syndromes / Outcomes of HLA-haploidentical allogeneic blood or marrow transplantation with post-transplant cyclophosphamide for myeloproliferative neoplasms and myelodysplastic syndrome / myeloproliferative neoplasm overlap syndromes
PIs: Auro Viswabandya (Princess Margaret Cancer Center - BMT Program)
Benjamin Tomlinson (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Michael Grunwald (Levine Cancer Institute)
Hany Elmariah (Johns Hopkins University)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

GS19-01
Title: Comparison of myeloablative haploidentical or umbilical cord blood transplantation for pediatric and adult patients with acute leukemia
PIs: John Wagner (University of Minnesota Blood and Marrow Transplant Program)
Karen Ballen (University of Virginia Health System)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
GS19-02
Title: **Graft failure in MDS and acute leukemia patients after allogeneic stem cell transplantation receiving post transplant cyclophosphamide**
PIs: Cindy Lynn Hickey-Gillis (Dana Farber Cancer Institute)
Rizwan Romee (Dana Farber Cancer Institute at Brigham and Women's Hospital)
Corey Cutler (Dana Farber Cancer Institute at Brigham and Women's Hospital)
Navneet Majhail (Cleveland Clinic Foundation)
Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)

GS19-03
Title: **Impact of G-CSF on in-vivo T-cell depleted allogeneic hematopoietic cell transplantation**
PIs: Nina Orfali (Weil Cornell Medicine)
Jaap Jan Boelens (Memorial Sloan Kettering Cancer Center)
Koen Van Besien (New York Presbyterian Hospital at Cornell)
Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)
7.0 GRAFT-VS-HOST DISEASE WORKING COMMITTEE

7.1 Leadership

**Chair:** Joseph Pidala, MD, PhD, H. Lee Moffitt Cancer Center and Research Institute  
Email: joseph.pidala@moffitt.org

**Chair:** Madan Jagasia, MBBS, MS, Vanderbilt University Medical Center  
Email: madan.jagasia@vanderbilt.edu

**Chair:** Margaret MacMillan, MD, MSc, University of Minnesota Blood and Marrow Transplant Program – Pediatrics  
Email: macmi002@umn.edu

**Scientific Director:** Mukta Arora, MD, MS, CIBMTR Minneapolis  
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**Scientific Director:** Stephen Spellman, MBS, CIBMTR Minneapolis  
Email: sspellma@nmdp.org

**Statistical Director:** Tao Wang, PhD, CIBMTR Milwaukee  
Email: taowang@mcw.edu

**MS Statistician:** Karen Chen, MS, CIBMTR Milwaukee  
Email: kachen@mcw.edu

7.2 Recent Publications

**2019**


2018


2017


2016
2015


2014


7.3 Current Studies

**GV16-01a**
Title: **Graft-versus-host disease-free relapse-free survival in alternative donor hematopoietic cell transplantation**
PIs: Rohtesh S. Mehta (M.D. Anderson Cancer Center)
     Shernan Holtan (University of Minnesota Blood and Marrow Transplant Program)
     Daniel Weisdorf (University of Minnesota Blood and Marrow Transplant Program)
Status: Submitted (as of July 1, 2019)
       Published (expected by June 30, 2020)

**GV17-01**
Title: **Investigating antibiotic exposure and risk of acute graft versus host disease in children undergoing hematopoietic stem cell transplantation for acute leukemia**
PIs: Caitlin Elgarten (Children's Hospital of Philadelphia)
     Brian Fisher (Children's Hospital of Philadelphia)
     Richard Aplenc (Children's Hospital of Philadelphia)
Status: Analysis (as of July 1, 2019)
       Submitted (expected by June 30, 2020)

**GV17-02**
Title: **Risk factors of acute and chronic GVHD in T-replete HLA haploldnetical HCT using post-transplantation cyclophosphamide**
PIs: Annie Im (University of Pittsburgh Medical Center)
     Betty Hamilton (Cleveland Clinic Foundation)
     Armin Rashidi (University of Minnesota)
Status: Manuscript Preparation (as of July 1, 2019)
       Submitted (expected by June 30, 2020)

**GV17-03**
Title: **Alterations in the characteristics and outcomes of acute and chronic GVHD following post-transplant high dose cytoxan prophylaxis for haploldnetal transplantation and in patients over 60 at high risk for GVHD**
PIs: Rima Saliba (M.D. Anderson Cancer Center)
     Stefan Ciurea (M.D. Anderson Cancer Center)
     Jeff Schriber (Cancer Transplant Institute at Virginia G. Piper Cancer Center)
Status: Analysis (as of July 1, 2019)
       Submitted (expected by June 30, 2020)
GV18-01
Title: **Comparison of late effects among allogeneic hematopoietic cell transplantation survivors with and without chronic graft-versus-host disease**
PIs: Catherine Lee (Utah Blood and Marrow Transplant Program)
     Daniel Couriel (Utah Blood and Marrow Transplant Program)
Status: Draft Protocol Received (as of July 1, 2019)
       Analysis (expected by June 30, 2020)

GV18-02
Title: **Comparison of antibacterial prophylaxis strategies and outcomes in allogeneic stem cell transplantation patients with acute graft vs host disease**
PI: Whitney Wallis (Monroe Carrel Jr. Children's Hospital)
Status: Protocol Development (as of July 1, 2019)
       Analysis (expected by June 30, 2020)

GV18-03
Title: **Impact of chronic graft-versus-host disease on non-relapse mortality and disease relapse in transplant recipients**
PIs: Vijaya Bhatt (Nebraska Medicine)
     Stephanie Lee (Fred Hutchinson Cancer Research Center)
Status: Draft Protocol Received (as of July 1, 2019)
       Analysis (expected by June 30, 2020)

GV18-04
Title: **Development of a risk score to predict the incidence of acute GVHD after allogeneic hematopoietic cell transplantation**
PI: Guru Subramanian Guru Murthy (Medical College of Wisconsin)
Status: Data File Preparation (as of July 1, 2019)
       Submitted (expected by June 30, 2020)

GV19-01
Title: **Exploring the link between donor-engrafted clonal hematopoiesis and adverse outcomes in allogeneic HCT recipients**
PIs: Nancy Gillis Johnson (H. Lee Moffitt Cancer Center and Research Institute)
     Eric Padron (H. Lee Moffitt Cancer Center and Research Institute)
     Aleksandr Lazaryan (H. Lee Moffitt Cancer Center and Research Institute)
Status: Protocol Development (as of July 1, 2019)
       Sample Typing (expected by June 30, 2020)
8.0 HEALTH SERVICES AND INTERNATIONAL STUDIES WORKING COMMITTEE

8.1 Leadership

Chair: Nandita Khera, MD, Mayo Clinic Arizona and Phoenix Children’s Hospital
  Email: khera.nandita@mayo.edu

Chair: William Wood, MD, MPH, University of North Carolina Hospitals
  Email: wawood@med.unc.edu

Chair: Shahrukh Hashmi, MD, MPH, King Faisal Specialist Hospital and Research Center
  Email: hashmi.shahrukh@mayo.edu

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  Email: wsaber@mcw.edu

Statistical Director: Ruta Brazauskas, PhD, CIBMTR Milwaukee
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MS Statistician: Naya He, MPH, CIBMTR Milwaukee
  Email: nhe@mcw.edu

8.2 Recent Publications

2019

2018

2017


2016


**2015**


2014


8.3 Current Studies

**HS14-01**
Title: Investigating clinical outcomes and inpatient health care resource utilization of hematopoietic cell transplantation for children with acute leukemia
Pis: Staci Arnold (Emory University Hospital)
Richard Aplenc (Children’s Hospital of Philadelphia)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with Pediatric Health Information System (PHIS)

**HS15-01**
Title: Who is lost to follow-up in the Center for International Blood and Marrow Transplant Research (CIBMTR) registry?
P: David Buchbinder (Children’s Hospital of Orange County)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

**HS15-02**
Title: Impact of socioeconomic status on pediatric stem cell transplant outcomes
P: Kira Bona (Dana Farber Cancer Institute)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
HS16-01  
Title: **Trends in utilization and outcomes of autologous and allogeneic hematopoietic cell transplantation in racial and ethnic minorities**  
PIs: Nandita Khera (Mayo Clinic Arizona and Phoenix Children's Hospital)  
Wael Saber (Medical College of Wisconsin)  
Status: Protocol Development (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)

HS16-02  
Title: **The impact of marital status on hematopoietic stem cell transplant recipient outcomes: A surrogate for consistent caregiver**  
PI: Sara Margaret Beattie (Tom Baker Cancer Centre)  
Status: Submitted (as of July 1, 2019)  
Published (expected by June 30, 2020)

HS16-03  
Title: **Relationship of race / ethnicity and survival after single and double umbilical cord blood transplantation**  
PI: Karen Ballen (University of Virginia Health System)  
Status: Protocol Development (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)

HS17-01  
Title: **Association of community health status and center survival for allogeneic hematopoietic cell transplantation**  
PIs: Sanghee Hong (Cleveland Clinic Foundation)  
Navneet Singh Majhail (Cleveland Clinic Foundation)  
Status: Manuscript Preparation (as of July 1, 2019)  
Submitted (expected by June 30, 2020)  
* Collaborative study with PHIS

HS18-01  
Title: **International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens**  
PIs: Yasuyuki Arai (Kyoto University)  
Yoshiko Atsuta (Nagoya University Graduate School of Medicine)  
Shingo Yano (Jikei University School of Medicine)  
Status: Protocol Development (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)  
* Collaborative study with the Japan Society for Hematopoietic Cell Transplantation
HS18-02
Title: Racial differences in long term survivor outcomes after allogeneic transplants
PIs: Branson Blue (Saint Louis University)
      Navneet Majhail (Cleveland Clinic Foundation)
Status: Protocol Development (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

HS18-03
Title: Racial / ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia
PIs: Lena Winestone (Children's Hospital of Philadelphia)
      Richard Aplenc (Children's Hospital of Philadelphia)
      Kelly Getz (Perelman School of Medicine, University of Pennsylvania)
Status: Protocol Development (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)
* Collaborative study with PHIS

HS19-01
Title: Factors associated with clinical trial participation among HSCT patients: a CIBMTR Analysis
PIs: Tamryn Gray (Dana Farber Cancer Institute)
      Areej El-Jawahri (Massachusetts General Hospital)
Status: Protocol Development (as of July 1, 2019)
        Protocol Development (expected by June 30, 2020)

HS19-02
Title: Comparing outcomes of myeloablative T-replete haploidentical transplantation with PT-CY protocol and ATG+G-CSF Protocol in patients with cytogenetic intermediate / high risk acute myeloid leukemia in first complete remission
PI: Xiao-Jun Huang (Peking University Institute of Hematology)
Status: Data File Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)
* Collaborative study with Peking University

HS19-03
Title: Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: A multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group
PIs: Nelson Hamerschlak (M.D. Anderson Cancer Center)
      Mariana Kerbauy (Hospital Israelita Albert Einstein)
      Andreza Ribeiro (Hospital Israelita Albert Einstein)
Status: Data Collection (as of July 1, 2019)
        Data Collection (expected by June 30, 2020)
* Collaborative study with the Brazilian Transplant Group
HS19-04
Title: Outcomes after allogeneic stem cell transplants performed in Brazil from HLA-matched siblings, unrelated and mismatched related donors: Retrospective study on behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle Transplant-related complications Consortium), Hospital Israelita Albert Einstein (AmigoH), Associação da Medula Óssea do Estado de São Paulo (Ameo), Program Nacional de Apoio à Atenção Oncológica (Pronon), and CIBMTR

PIs: Adriana Seber (Hospital Samaritano)
Nelson Hamerschlak (M.D. Anderson Cancer Center)
Mary Flowers (Fred Hutchinson Cancer Research Center)
Marcelo Pasquini (Medical College of Wisconsin)

Status: Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)

* Collaborative study with the Brazilian Transplant Group
9.0 IMMUNOBIOLOGY WORKING COMMITTEE

9.1 Leadership

Chair: Katharine Hsu, MD, PhD, Memorial Sloan Kettering Cancer Center
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Chair: Steven Marsh, BSc, PhD, ARCS, Anthony Nolan Research Institute
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Email: sjlee@fredhutch.org

Scientific Director: Stephen Spellman, MBS, CIBMTR Minneapolis
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Statistical Director: Tao Wang, PhD, CIBMTR Milwaukee
Email: taowang@mcw.edu

MS Statistician: Michelle Kuxhausen, MS, CIBMTR Minneapolis
Email: mformane@nmdp.org

9.2 Recent Publications

2018


2017


2016


2015


2014


**9.3 Current Studies**

**IB06-05**
**Title:** Use of high-resolution HLA data from the NMDP for the International Histocompatibility Working Group in HCT
**PI:** Effie Petersdorf (Fred Hutchinson Cancer Research Center)
**Status:** Ongoing (as of July 1, 2019)
* Collaborative study with the International Histocompatibility Working Group

**IB09-01**
**Title:** Clinical importance of minor histocompatibility complex haplotypes in umbilical cord blood transplantation
**PI:** Effie Petersdorf (Fred Hutchinson Cancer Research Center)
**Status:** Ongoing (as of July 1, 2019)
* Collaborative study with the International Histocompatibility Working Group
IB09-03
Title: Clinical relevance of cytokine/immune response gene polymorphisms in umbilical cord blood transplantation
PI: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
Status: Ongoing (as of July 1, 2019)
Ongoing (expected by June 30, 2020)
* Collaborative study with the International Histocompatibility Working Group

IB09-05
Title: Identification of functional single nucleotide polymorphisms in umbilical cord blood transplantation
PI: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
Status: Ongoing (as of July 1, 2019)
Ongoing (expected by June 30, 2020)
* Collaborative study with the International Histocompatibility Working Group

IB09-06b
Title: Genetic susceptibility to transplant-related mortality after unrelated donor stem cell transplant
PI: Theresa Hahn (Roswell Park Cancer Institute)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute

IB09-06f
Title: DISCOVeRY-BMT: Case-control study of acute myelogenous leukemia exome and genome-wide association study
PIs: Alyssa Clay-Gilmour (Roswell Park Cancer Institute)
Lara Sucheston-Cambpell (Ohio State Medical Center, James Cancer Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State

IB09-06j
Title: DISCOVeRY-BMT: Additional analysis of major histocompatibility complex single nucleotide polymorphisms
PIs: Steve Spellman (CIBMTR - Minneapolis)
Lara Sucheston-Cambpell (Ohio State Medical Center, James Cancer Center)
Status: Analysis (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State
IB09-06m
Title: **DISCOVeRY-BMT: Analysis of X chromosome single nucleotide polymorphisms**
PIs: Steve Spellman (CIBMTR - Minneapolis)
Lara Sucheston-Cambpell (Ohio State Medical Center, James Cancer Center)
Status: Analysis (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State

IB09-06n
Title: **DISCOVeRY-BMT: Compare unrelated donor to Welcome Trust Case Control Consortium controls**
PIs: Kenan Onel (Comer Children's Hospital / University of Chicago Medicine)
Alyssa Clay-Gilmour (Roswell Park Cancer Institute)
Ezgi Karaesmen (Ohio State Medical Center, James Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State

IB09-06o
Title: **DISCOVeRY-BMT: Genetics and Epidemiology of Myeloid Malignancies candidate gene paper**
PIs: Lara Sucheston-Cambbell (Ohio State Medical Center, James Cancer Center)
Ezgi Karaesmen (Ohio State Medical Center, James Cancer Center)
Alyssa Clay-Gilmour (Roswell Park Cancer Institute)
Theresa Hahn (Roswell Park Cancer Institute)
Status: Analysis (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State

IB09-06p
Title: **DISCOVeRY-BMT: Genetics and Epidemiology of Myeloid Malignancies genome-wide association study**
PIs: Alyssa Clay-Gilmour (Roswell Park Cancer Institute)
Kenan Onel (Comer Children’s Hospital / University of Chicago Medicine)
Theresa Hahn (Roswell Park Cancer Institute)
Status: Analysis (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with Roswell Park Cancer Institute and Ohio State

IB09-07
Title: **Clinical significance of genome-wide variation in unrelated HCT**
PI: Effie Petersdorf (Fred Hutchinson Cancer Research Center)
Status: Ongoing (as of July 1, 2019)
Ongoing (expected by June 30, 2020)
* Collaborative study with International Histocompatibility Working Group
IB10-01f
Title: Epigenetic clock: Can this guide donor selection in HCT
PIs: Shahinaz Gadalla (National Cancer Institute)
     Sharon Savage (National Cancer Institute)
Status: Analysis (as of July 1, 2019)
       Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with the National Cancer Institute

IB14-03a
Title: The prognostic impact of levels of CXC chemokine ligands on post hematopoietic cell transplantation outcomes in patients with myelodysplastic syndromes
PIs: Wael Saber (Medical College of Wisconsin)
     Binod Dhakal (Medical College of Wisconsin)
Status: Submitted (as of July 1, 2019)
       Published (expected by June 30, 2020)

IB14-03c
Title: Impact of telomere length and telomerase gene mutations on allogeneic stem cell transplantation outcomes in myelodysplastic syndrome
PIs: R. Coleman Lindsley (Dana Farber Cancer Institute at Brigham and Women's Hospital)
     Wael Saber (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
       Submitted (expected by June 30, 2020)

IB14-04
Title: Assessing the similarity of the T cell receptor repertoire in allogeneic hematopoietic stem cell recipients with the same single human leukocyte mismatches
PI: Everett Meyer (Stanford Health Care)
Status: Submitted (as of July 1, 2019)
       Published (expected by June 30, 2020)

IB14-05
Title: mtDNA haplotypes and unrelated donor transplant outcomes
PIs: Michael Verneris (University of Colorado - Children's Hospital)
     Logan Spector (University of Minnesota)
Status: Manuscript Preparation (as of July 1, 2019)
       Submitted (expected by June 30, 2020)
* Collaborative study with University of Minnesota
IB14-07
Title: Indirectly recognizable HLA epitopes (PIRCHES): A retrospective validation study on the role of indirect recognition of mismatched HLA in hematopoietic stem cell transplantation outcome
PI: Eric Spierings (University Medical Center Utrecht)
Status: Manuscript Preparation (as of July 1, 2019)
         Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with International Histocompatibility Working Group

IB15-03
Title: Killer immunoglobulin receptor (KIR) gene content and pediatric acute leukemia transplant outcomes
PIs: Michael R. Verneris (University of Colorado - Children’s Hospital)
        Jeffrey Miller (University of Minnesota Blood and Marrow Transplant Program)
        Sarah Cooley (University of Minnesota Blood and Marrow Transplant Program)
Status: Manuscript Preparation (as of July 1, 2019)
         Submitted (expected by June 30, 2020)
* Collaborative study with University of Minnesota

IB15-04
Title: Clinical outcomes among hematopoietic stem cell transplant recipients as a function of socioeconomic status and related transcriptome differences
PIs: Jennifer Knight (Medical College of Wisconsin)
        J. Douglas Rizzo (Medical College of Wisconsin)
        Steve Cole (UCLA School of Medicine)
Status: Submitted (as of July 1, 2019)
         Published (expected by June 30, 2020)
* Collaborative study with the Medical College of Wisconsin

IB16-01
Title: The role of HLA-E compatibility in the prognosis of acute leukemia patients undergoing 10/10 HLA matched unrelated HSCT
PIs: Chrysanthi Tsamadou (IKT Ulm)
        Daniel Furst (IKT Ulm)
        Joannis Mytilineos (IKT Ulm)
Status: Submitted (as of July 1, 2019)
         Published (expected by June 30, 2020)

IB16-02
Title: Use of HLA structure and function parameters to understand the relationship between HLA disparity and transplant outcomes
PI: Lee Ann Baxter Lowe (Children’s Hospital of Los Angeles)
Status: Manuscript Preparation (as of July 1, 2019)
         Submitted (expected by June 30, 2020)
**IB16-03**

**Title:** Role of recipient and donor genetic polymorphisms in interferon lambda 4 (IFNL4) on outcomes after unrelated allogeneic cell transplant  
**PIs:** Shahinaz M. Gadalla (National Cancer Institute)  
Ludmila Prokunina-Olsson (National Cancer Institute)  
**Status:** Submitted (as of July 1, 2019)  
Published (expected by June 30, 2020)  
* Collaborative study with the National Cancer Institute

**IB17-02**

**Title:** Donor-recipient NK cell determinants associated with survival in JMML after hematopoietic stem cell transplantation  
**PIs:** Dean Lee (Nationwide Children’s Hospital)  
Hemalatha Rangarahan (Nationwide Children’s Hospital)  
**Status:** Sample Typing (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)

**IB17-03**

**Title:** Identification of genomic markers of post hematopoietic cell transplantation (HCT) outcomes in patients with myelofibrosis: A pilot study  
**PIs:** Wael Saber (Medical College of Wisconsin)  
Shahinaz Gadalla (National Cancer Institute)  
**Status:** Data File Preparation (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)  
* Collaborative study with the National Cancer Institute

**IB17-04**

**Title:** Epigenetic profiling of unrelated donor-recipient pairs to improve donor selection during HCT transplants  
**PIs:** Stephan Beck (University College London)  
Karl Peggs (University College London)  
Vardhman Rakyvan (Barts and The London School of Medicine, Blizard Institute)  
Amy Webster (University College London)  
**Status:** Analysis (as of July 1, 2019)  
Analysis (expected by June 30, 2020)

**IB18-01**

**Title:** Effect of HLA phenotypes on long term GVHD risk  
**PIs:** Charlotte Story (University of North Carolina School of Medicine)  
Marcie Riches (University of North Carolina Hospitals)  
Paul Armistead (University of North Carolina Hospitals)  
**Status:** Data File Preparation (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)
**IB18-02**
Title: **The impact of HLA class I risk alleles associated with AA immune pathogenesis on allogeneic transplant outcomes in patients with severe acquired aplastic anemia**
PIs: Daria Babushok (Children's Hospital of Philadelphia)  
Timothy Olson (Children's Hospital of Philadelphia)
Status: Sample Typing (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)

**IB18-03**
Title: **The effect of HLA Class I heterozygosity and HLA supertypes on outcomes following allogeneic hematopoietic cell transplant for myeloid and lymphoid malignancies**
PIs: Christine Camacho-Bydume (Memorial Sloan Kettering Cancer Center)  
Katharine Hsu (Memorial Sloan Kettering Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)  
Submitted (expected by June 30, 2020)

**IB18-04**
Title: **Evaluation of the impact of donor KIR genotype on outcome after unrelated donor transplantation in patients with myelodysplastic syndromes or secondary acute myeloid leukemia**
PIs: Johannes Schetelig (Universitaetsklinikum Carl Gustav Carus)  
Nicolas Kröger (Universitaetsklinikum Hamburg, Eppendorf)  
Marie Robin (Hopital Saint Louis)
Status: Manuscript Preparation (as of July 1, 2019)  
Submitted (expected by June 30, 2020)
* Collaborative study with Deutsche Knochenmarkspenderdatei GmbH (DKMS, German Bone Marrow Donor Center)

**IB18-05**
Title: **Imputation of KIR in genome-wide association study and the association of KIR-HLA with outcomes following alloHCT In AML and MDS**
PIs: Christine Camacho-Bydume (Memorial Sloan Kettering Cancer Center)  
Lara Sucheston-Campbell (Ohio State Medical Center, James Cancer Center)  
Stephen Leslie (University of Melbourne)  
Katharine Hsu (Memorial Sloan Kettering Cancer Center)
Status: Analysis (as of July 1, 2019)  
Analysis (expected by June 30, 2020)
* Collaborative study with International Histocompatibility Working Group
IB18-06
Title: **Clonal mosaicism and HCT outcomes in patients with acute leukemia and myelodysplastic syndromes**
PIs: Shahinaz Gadalla (National Cancer Institute)  
Theresa Hahn (Roswell Park Cancer Institute)  
Lara Sucheston-Campbell (Ohio State Medical Center, James Cancer Center)
Status: Analysis (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with the National Cancer Institute

IB18-07
Title: **Donor and recipient genomic associations with acute GVHD**
PI: Vahid Afshar-Khargan (M.D. Anderson Cancer Center)
Status: Sample Typing (as of July 1, 2019)  
Analysis (expected by June 30, 2020)
* Collaborative study with the National Cancer Institute

IB19-01
Title: **The impact of ultra-high resolution HLA matching on the outcome of unrelated donor hematopoietic cell transplantation**
PIs: Neema Mayor (Anthony Nolan Research Institute)  
Stephen Spellman (CIBMTR - Minneapolis)  
Steven Marsh (Anthony Nolan Research Institute)
Status: Protocol Development (as of July 1, 2019)  
Manuscript Preparation (expected by June 30, 2020)

IB19-02
Title: **Effect of class II HLA mismatching on the outcome of HLA-haploidentical hematopoietic cell transplantation with high dose, post-transplantation cyclophosphamide: a combined CIBMTR/EBMT analysis**
PIs: Shannon McCurdy (Hospital of the University of Pennsylvania)  
Scott Solomon (The Blood and Marrow Transplant Program at Northside Hospital)  
Yvette Kasamon (Johns Hopkins Hospital)  
Asad Bashey (The Blood and Marrow Transplant Program at Northside Hospital)  
Ephraim Fuchs (The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins)
Status: Draft Protocol Received (as of July 1, 2019)  
Analysis (expected by June 30, 2020)
**IB19-03**

Title: **Impact of the direction of NK cell alloreactivity predicted by KIR ligand mismatch on engraftment in umbilical cord blood and haploidentical stem cell transplantation**

PIs: Folashade Otegbeye (Abramson Cancer Center University of Pennsylvania Medical Center)
     Marcelo Fernandez-Vina (Stanford Health Care)
     Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)

Status: Draft Protocol Received (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

**IB19-04**

Title: **Impact of donor HLA on transplant outcomes in NPM1 mutated AML**

PIs: Rupa Narayan (Massachusetts General Hospital)
     Everett Meyer (Stanford Health Care)
     Yi-Bin Chen (Massachusetts General Hospital)

Status: Protocol Pending (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

**IB19-05**

Title: **Impact of donor signal-regulatory protein alpha polymorphism on outcomes of allogeneic hematopoietic stem cell transplantation**

PIs: Jayne Danska (The Hospital for Sick Children)
     Fadi Lakkis (University of Pittsburgh)

Status: Draft Protocol Received (as of July 1, 2019)
        Sample Typing (expected by June 30, 2020)

* Collaborative study with The Hospital for Sick Children

**R02-40/R03-63d**

Title: **Acquisition of natural killer cell receptors in recipients of unrelated transplant**

PI: Jeff Miller (University of Minnesota Blood and Marrow Transplant Program)

Status: Ongoing (as of July 1, 2019)
        Ongoing (expected by June 30, 2020)

* Collaborative study with University of Minnesota

**R04-74d**

Title: **Functional significance of killer cell immunoglobulin-like receptor genes in HLA-matched and mismatched unrelated HCT**

PI: Katharine Hsu (Memorial Sloan Kettering Cancer Center)

Status: Ongoing (as of July 1, 2019)
        Ongoing (expected by June 30, 2020)

* Collaborative study with International Histocompatibility Working Group
RT09-04 / IB09-06i

Title: DISCOVeRY-BMT: Recip, donor genome-wide association study interaction with conditioning intensity (myeloablative / reduced intensity conditioning), total body irradiation, disease status

PIs: Ezgi Karaesmen (Ohio State Medical Center, James Cancer Center)
     Lara Sucheston-Camppell (Ohio State Medical Center, James Cancer Center)
     Theresa Hahn (Roswell Park Cancer Institute)

Status: Analysis (as of July 1, 2019)
        Manuscript Preparation (expected by June 30, 2020)

* Collaborative study with Roswell Park Cancer Institute and Ohio State
10.0 INFECTION AND IMMUNE RECONSTITUTION WORKING COMMITTEE

10.1 Leadership

Chair: Krishna Komanduri, MD, University of Miami  
Email: kkomanduri@med.miami.edu

Chair: Miguel-Angel Perales, MD, Memorial Sloan Kettering Cancer Center  
Email: peralesm@mskcc.org

Chair: Roy Chemaly, MD, MPH, MD Anderson Cancer Center  
Email: rfchemaly@mdanderson.org

Scientific Director: Marcie Riches, MD, MS, University of North Carolina Hospitals  
Email: marcie_riches@med.unc.edu

Statistical Director: Soyoung Kim, PhD, CIBMTR Milwaukee  
Email: skim@mcw.edu

MS Statistician: Naya He, MPH, CIBMTR Milwaukee  
Email: nhe@mcw.edu

10.2 Recent Publications

2019


2018


2016


10.3 Current Studies

IN16-02
Title: Determination of the burden of mucosal barrier injury-laboratory confirmed bloodstream infections in the first 100 days after stem cell transplant
PIs: Christopher Dandoy (Cincinnati Children's Hospital Medical Center)
Paulina Daniels (Cincinnati Children's Hospital Medical Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

IN17-01
Title: Incidence and impact of cytomegalovirus infection in haploidentical and matched-related donors receiving post-transplant cyclophosphamide: A CIBMTR analysis
PI: Scott Goldsmith (Washington University)
Status: Analysis (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)
**IN18-01**

**Title:** Comparison of early (By D+100) infections between posttransplantation cyclophosphamide and other GVHD prophylaxis

**PIs:** Genovefa Papanicolaou (Memorial Sloan Kettering Cancer Center)
Celalettin Ustun (Rush University Medical Center)

**Status:** Data File Preparation (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

**IN18-02**

**Title:** Study the incidence and impact of C diff infection within 100 days on transplant outcomes after allogeneic stem cell transplant

**PIs:** Muthalagu Ramanthan (UMass Memorial Medical Center)
Bipin Savani (Vanderbilt University Medical Center)

**Status:** Protocol Development (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

**IN19-01**

**Title:** Immune recovery predicts post-transplant outcomes

**PI:** Miguel-Angel Perales (Memorial Sloan Kettering Cancer Center)

**Status:** Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)

**IN19-02**

**Title:** Impact of antibacterial prophylaxis on outcomes after allogeneic hematopoietic stem cell transplant

**PIs:** C Dandoy (Cincinnati Children’s Hospital Medical Center)
Priscila Alonso (Cincinnati Children’s Hospital Medical Center)
Z El Boghdady (The Ohio State University)

**Status:** Draft Protocol Received (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)
11.0 LATE EFFECTS AND QUALITY OF LIFE WORKING COMMITTEE

11.1 Leadership

Chair: Minoo Battiwalla, MD, MS, Sarah Cannon Research Institute  
Email: minoo.battiwalla@hcahealthcare.com

Chair: David Buchbinder, MD, Children's Hospital of Orange County  
Email: dbuchbinder@choc.org

Chair: Betty Hamilton, MD, Cleveland Clinic Taussig Cancer Institute  
Email: hamiltb2@ccf.org

Scientific Director: Bronwen Shaw, MBChB, MRCP, PhD, CIBMTR Milwaukee  
Email: beshaw@mwc.edu

Asst Sci Director: Rachel Phelan, MD, CIBMTR Milwaukee  
Email: rphelan@mwc.edu

Statistical Director: Ruta Brazauskas, PhD, CIBMTR Milwaukee  
Email: ruta@mwc.edu

MS Statistician: Stephanie Bo-Subait, MPH, CIBMTR Minneapolis  
Email: sbosuba2@nmdp.org

11.2 Recent Publications

2018

Inamoto Y, Valdés-Sanz N, Ogawa Y, Alves M, Berchicci L, Galvin J, Greinin H, Hale GA, Horn B,  
Bhatt N, Byrne M, Chhabra S, DeFilipp Z, Fahnehjelm K, Farhadfar N, Horn E, Lee C, Nathan S,  
Penack O, Prasad P, Rotz S, Rovó A, Yared J, Prasad P, Pulanic D, Rotz S,  
Shreenivas A, Steinberg A, Tabbara K, Tichelli A, Wirk B, Yared J, Basak GW, Battiwalla M, Duarte R,  


2017


2016


2015


2014


11.3 Current Studies

LE12-03
Title: Solid organ transplantation and hematopoietic cell transplantation
PIs: Meera Gupta (Abramson Cancer Center University of Pennsylvania Medical Center)
      Peter L Abt (Abramson Cancer Center University of Pennsylvania Medical Center)
      Matthew Levine (Abramson Cancer Center University of Pennsylvania Medical Center)
Status: Analysis (as of July 1, 2019)
      Submitted (expected by June 30, 2020)
* Collaborative study with United Network for Organ Sharing

LE13-02
Title: Risk factors for melanoma following allogeneic hematopoietic stem cell transplantation
PIs: Megan Herr (Roswell Park Cancer Institute)
      Lindsay Morton (National Cancer Institute)
      Eric Engels (National Cancer Institute)
      Margaret Tucker (National Cancer Institute)
      Rochelle Curtis (NIH-Experimental Transplantation and Immunology Branch)
      Ruth Pfeiffer (National Cancer Institute)
      David A Jacobson (Vanderbilt Univeristy Medical Center)
Status: Submitted (as of July 1, 2019)
      Published (expected by June 30, 2020)
* Collaborative study with National Institutes of Health
LE16-02
Title: An investigation of new malignant neoplasms in pediatric patients undergoing allogeneic hematopoietic stem cell transplantation for non-malignant diseases
PIs: Justine Kahn (Morgan Stanley Children’s Hospital of New York Presbyterian – Columbia University Medical Center)
Prakash Satwani (New York Presbyterian – Columbia University Medical Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

LE17-01
Title: Long-term follow up after hematopoietic stem cell transplantation for sickle cell disease
PIs: Elizabeth Stenger (Children’s Healthcare of Atlanta at Egleston)
Rachel Phelan (Medical College of Wisconsin)
Shalini Shenoy (Washington University / St. Louis Children’s Hospital)
Lakshmanan Krishnamurti (Children’s Healthcare of Atlanta at Egleston)
Status: Analysis (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with Emory University

LE17-02
Title: Comparison of late effects among allogeneic hematopoietic cell transplantation survivors conditioned with high dose total body irradiation (TBI) versus non-TBI based ablative regimens in adolescents and young adults (15-39yo) with acute leukemia
PIs: Catherine Lee (Utah Blood and Marrow Transplant Program)
Lori Muffly (Stanford Health Care)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LE18-01
Title: Survival trends amongst two-year survivors of alloHCT
PIs: Prakash Satwani (New York Presbyterian Hospital/Columbia University Medical Center)
Larisa Brogile (Columbia University)
Status: Protocol Development (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LE18-02
Title: Return to work or school status in survivors of adolescent and young adult alloHCT
PIs: Neel Bhatt (Fred Hutchinson Cancer Research Center)
Rachel Salit (Fred Hutchinson Cancer Research Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
LE18-03
Title: Incorporating patient reported outcomes into individualized prognostication tools for survival and quality of life in transplant patients
PI: Bronwen Shaw (Medical College of Wisconsin)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with The Emmes Company

LE19-01
Title: Long-term survival and late effects in critically ill pediatric hematopoietic cell transplant patients
PIs: Matt Zinter (University of California San Francisco Medical Center)
     Chris Dvorak (University of California San Francisco Medical Center)
     Christy Duncan (Dana Farber Cancer Institute & Boston Children's Hospital)
Status: Draft Protocol Received (as of July 1, 2019)
        Manuscript Preparation (expected by June 30, 2020)
* Collaborative study with Virtual Pediatric Systems

LE19-02
Title: Incidence and predictors of Long term toxicities and late side effects in elderly patients (>=60 years) receiving allogeneic hematopoietic cell transplantation for hematological malignancies.
PIs: Muthu Veeraputhiran (University of Arkansas for Medical Sciences)
     Sai Ravi Pingali (M.D. Anderson Cancer Center)
     Akash Mukherjee (University of Arkansas for Medical Sciences)
     Lori Muffly (Stanford Health Care)
Status: Draft Protocol Received (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)

LE99-01
Title: Quality of life in late HCT survivors
PI: John Wingard (Shands HealthCare & University of Florida)
Status: Ongoing (as of July 1, 2019)
        Ongoing (expected by June 30, 2020)
* Collaborative study with Univeristy of Florida

SC09-05d
Title: Regret having bone marrow transplant
PI: Rachel Cusatis (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)
* Collaborative study with University of Minnesota
12.0 LYMPHOMA WORKING COMMITTEE

12.1 Leadership

Chair: Timothy Fenske, MD, MS, Froedtert Hospital
Email: tfenske@mcw.edu

Chair: Mohamed Kharfan-Dabaja, MD, MBA, Mayo Clinic Florida
Email: kharfandabaja.mohamed@mayo.edu

Chair: Craig Sauter, MD, Memorial Sloan Kettering Cancer Center - Adults
Email: sauterc@mskcc.org

Scientific Director: Mehdi Hamadani, MD, CIBMTR Milwaukee
Email: mhamadani@mcw.edu

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

MS Statistician: Andrew St. Martin, MS, CIBMTR Milwaukee
Email: astmartin@mcw.edu

12.2 Recent Publications

2019


2018


2017


2016


2015


2014


12.3 Current Studies

LY17-01b
Title: Clinical outcomes of patients age ≥65 undergoing allogeneic hematopoietic cell transplant for non-Hodgkin lymphoma
PI: Nirav Shah (Medical College of Wisconsin)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

LY17-02a
Title: Allografts following reduced intensity conditioning for Hodgkin's lymphoma
PI: Nilanjan Ghosh (Levine Cancer Institute)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LY17-02b
Title: Allografts following reduced intensity conditioning for non-Hodgkin disease
PI: Sairah Ahmed (M.D. Anderson Cancer Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
LY17-02c
Title: Conditioning regimen in allografts for diffuse large B cell lymphoma
PI: Mehdi Hamadani (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LY17-02d
Title: 2 versus 4 centigray fludarabine / total body irradiation in allografts for non-Hodgkin lymphoma
PI: Mehdi Hamadani (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LY18-01
Title: Outcomes in B cell non-Hodgkin’s lymphoma patients who underwent autologous stem cell transplantation following rituximab containing conditioning regimens
PIs: Deepa Jagadeesh (Cleveland Clinic Foundation)
Navneet Majhail (Cleveland Clinic Foundation)
Brian Hill (Cleveland Clinic Foundation)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LY18-02
Title: Effect of time to relapse on overall survival in mantle cell lymphoma patients following frontline autologous stem cell transplant
PIs: Peter Riedell (University of Chicago Medicine)
Sonali Smith (University of Chicago Medicine)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

LY18-03
Title: Transplantation for CLL undergoing Richter’s transformation arising in the setting of indolent lymphoma
PI: Alex Herrera (City of Hope)
Status: Data File Preparation (as of July 1, 2019)
Analysis (expected by June 30, 2020)
**LY19-01**

**Title:** Post-transplant cyclophosphamide-based haploidentical transplantation versus matched sibling or well matched unrelated donor transplantation for peripheral T-cell lymphoma: A CIBMTR Lymphoma Working Committee and EBMT Lymphoma Working Party analysis

**PIs:**
- Peter Dreger (Universitaetsklinikum Heidelberg)
- Mehdi Hamadani (Medical College of Wisconsin)

**Status:** Protocol Pending (as of July 1, 2019)
- Manuscript Preparation (expected by June 30, 2020)

* Collaborative study with EBMT

**LY19-02**

**Title:** Determining the optimal conditioning regimen for patients with primary central nervous system lymphoma undergoing autologous hematopoietic cell transplantation

**PIs:**
- Michael Scordo (Memorial Sloan Kettering Cancer Center)
- Craig Sauter (Memorial Sloan Kettering Cancer Center)
- Antonio Jimenez (University of Miami)

**Status:** Draft Protocol Received (as of July 1, 2019)
- Data File Preparation (expected by June 30, 2020)
13.0 NON-MALIGNANT DISEASES WORKING COMMITTEE

13.1 Leadership

Chair: Vikram Mathews, MD, Christian Medical College Hospital
      Email: vikram@cmcvellore.ac.in
Chair: Christopher Dvorak, MD, University of California San Francisco Medical Center
      Email: christopher.dvorak@ucsf.edu
Chair: Andrew Gennery, MD, Newcastle General Hospital and The Royal Victoria Infirmary
      Email: a.r.gennery@ncl.ac.uk
Chair: George Georges, MD, Fred Hutchinson Cancer Research Center
      Email: ggeorges@fredhutch.org
Scientific Director: Mary Eapen, MD, MS, CIBMTR Milwaukee
      Email: meapen@mcw.edu
Statistical Director: Soyoung Kim, PhD, CIBMTR Milwaukee
      Email: skim@mcw.edu
MS Statistician: Kyle Hebert, MS, CIBMTR Milwaukee
      Email: khebert@mcw.edu

13.2 Recent Publications

2018


2017


2016


2015


2014


13.3 Current Studies

**AA13-02**
Title: **Malignancies in patients with Fanconi anemia**
PI: John Wagner (University of Minnesota Blood and Marrow Transplant Program)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with the National Cancer Institute

**AC18-02**
Title: **Prospective cohort study of recipients of autologous hematopoietic cell transplant for systemic sclerosis**
Pis: George Georges (Fred Hutchinson Cancer Research Center)
Keith Sullivan (Duke University Medical Center)
Status: Protocol Development (as of July 1, 2019)
Data File Preparation (expected by June 30, 2020)

**ID13-01**
Title: **Haematopoietic stem cell transplants for congenital neutropenia / Kostmann agranulocytosis**
Pis: Cornelia Zeidler (Medizinische Hochschule Hannover)
Steven Keogh (The Children's Hospital at Westmead)
James Connelly (Vanderbilt University Medical Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with Severe Chronic Neutropenia International Registry

**NM14-02**
Title: **Outcomes of allogeneic hematopoietic cell transplant in patients with Shwachman Diamond syndrome**
PI: Kasiani Myers (Cincinnati Children’s Hospital Medical Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

**NM15-01**
Title: **Outcome of allogeneic hematopoietic cell transplant in erythropoietic porphyria**
Pis: Ayman Saad (Ohio State Medical Center, James Cancer Center)
Hisham Abdel-Azim (Children's Hospital of Los Angeles)
Joseph Bloomer (University of Alabama at Birmingham)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with EBMT
NM16-03
Title: **Results of transplants from genetically-identical twin donors in persons with aplastic anemia**
PI: Robert Peter Gale (LifebankUSA-Celularity)
Status: Analysis (as of July 1, 2019)
Submitted (expected by June 30, 2020)

NM16-04
Title: **The effect of conditioning regimen on clinical outcomes of allogeneic hematopoietic cell transplantation in severe aplastic anemia**
PIs: Nelli Bejanyan (H. Lee Moffitt Cancer Center and Research Institute)
Natasha Kekre (The Ottawa Hospital Blood & Marrow Transplant Program)
Daniel Weisdorf (University of Minnesota Blood and Marrow Transplant Program)
Joseph Antin (Dana Farber Cancer Institute)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

NM17-01
Title: **Late effects after hematopoietic stem cell transplantation in patients with hemophagocytic lymphohistiocytosis**
PIs: AnnaCarin Horne (Karolinska Institutet)
K Scott Baker (Fred Hutchinson Cancer Center)
Karin Beutel (Histiocyte Society)
Status: Protocol Development (as of July 1, 2019)
Analysis (expected by June 30, 2020)
* Collaborative study with EBMT

NM17-03b
Title: **Risk factor scoring for allogeneic hematopoietic stem cell transplantation for sickle cell disease**
PI: Ruta Brazauskas (Medical College of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

NM18-01
Title: **Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease**
PIs: Anand Prakash (Hospital for Sick Children, Toronto)
Donna Wall (Hospital for Sick Children)
Kristjan Paulson (CancerCare Manitoba/University of Manitoba)
Status: Protocol Development (as of July 1, 2019)
Submitted (expected by June 30, 2020)
NM19-01
Title: **Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after alloHCT or immunosuppressive therapy**
PIs: Ryotaro Nakamura (City of Hope)
     F. Lennie Wong (City of Hope)
     Saro Armenian (City of Hope)
Status: Protocol Pending (as of July 1, 2019)
     Data File Preparation (expected by June 30, 2020)
* Collaborative study with National Heart, Lung, and Blood Institute

NM19-02
Title: **Impact of reduced intensity conditioning on allogeneic HCT outcomes for HLH**
PI: Rebecca Marsh (Cincinnati Children’s Hospital Medical Center)
Status: Draft Protocol Received (as of July 1, 2019)
     Data File Preparation (expected by June 30, 2020)

NM19-03
Title: **Hematopoietic stem cell transplantation for congenital amegakaryocytic thrombocytopenia**
PIs: Farid Boulad (Memorial Sloan Kettering Cancer Center)
     Maria Cancio (Memorial Sloan Kettering Cancer Center)
     Jaap Jan Boelens (Memorial Sloan Kettering Cancer Center)
Status: Draft Protocol Received (as of July 1, 2019)
     Data File Preparation (expected by June 30, 2020)

SC17-09/17-CMS-SCD
Title: **Prospective assessment of allogeneic hematopoietic cell transplantation in adolescents and young adults with severe sickle cell disease**
PI: Mary Eapen (Medical College of Wisconsin)
Status: Data Collection/Data File Preparation (as of July 1, 2019)
     Data Collection/Data File Preparation (expected by June 30, 2020)
14.0 PEDIATRIC CANCER WORKING COMMITTEE

14.1 Leadership

Chair: Angela Smith, MD, MS, University of Minnesota Medical Center, Fairview  
Email: smith719@umn.edu  
Chair: Gregory Yanik, MD, The University of Michigan  
Email: gyanik@med.umich.edu  
Chair: Muna Qayed, Children’s Healthcare of Atlanta at Egleston  
Email: muna.qayed@choa.org  
Scientific Director: Mary Eapen, MD, MS, CIBMTR Milwaukee  
Email: meapen@mcw.edu  
Statistical Director: Kwang Woo Ahn, PhD, CIBMTR Milwaukee  
Email: kwooahn@mcw.edu  
MS Statistician: Kyle Hebert, MS, CIBMTR Milwaukee  
Email: khebert@mcw.edu

14.2 Recent Publications

2018
Lund TC, Ahn KW, Tecca HR, Hilgers MV, Abdel-Azim H, Abraham A, Diaz MA, Badawy SM,  
Broglie L, Brown V, Dvorak CC, Gonzalez-Vicent M, Hashem H, Hayashi RJ, Jacobsohn DA, Kent  
MW, Li C-K, Margossian SP, Martin PL, Mehta P, Myers K, Olsson R, Page K, Pulsipher MA, Shaw  
PJ, Smith AR, Triplett BM, Verneris MR, Eapen M. Outcomes after second hematopoietic cell  
transplantation in children and young adults with relapsed acute leukemia. Biology of  
Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow  
19. PMC6339844. ncbi.nlm.nih.gov/pubmed/30244103

2017
Malogolowkin MH, Hemmer MT, Le-Rademacher J, Hale GA, Mehta PA, Smith AR, Kitko C,  
Abraham A, Abdel-Azim H, Dandoy C, Angel Diaz M, Gale RP, Guilcher G, Hayashi R, Jodele S,  
Kasow KA, MacMillian ML, Thakar M, Wirk BM, Woolfrey A, Thiel EL. Outcomes following  
autologous hematopoietic stem cell transplant for patients with relapsed Wilms' tumor: A  
CIBMTR retrospective analysis. Bone Marrow Transplantation. 2017 Nov 1; 52(11):1549- 
28869618


2015


14.3 Current Studies

**PC18-01**

**Title:** Comparison of TBI vs non-TBI based regimens for pediatric AML in the modern era

**PI:** Christopher Dandoy (Cincinnati Children's Hospital Medical Center)

**Status:** Manuscript Preparation (as of July 1, 2019)

Submitted (expected by June 30, 2020)
PC19-01
Title: **Variation of the disease risk index in children undergoing alloHCT**
PIs: M Qayed (Children’s Healthcare of Atlanta at Egleston)
     C Kitko (Vanderbilt University Medical Center)
Status: Protocol Pending (as of July 1, 2019)
       Manuscript Preparation (expected by June 30, 2020)

PC19-02
Title: **Does mixed peripheral blood T cell chimerism predict relapse?**
PIs: S Prockop (Memorial Sloan Kettering Cancer Center)
     J Boelens (Memorial Sloan Kettering Cancer Center)
     K Peggs (University College London)
Status: Protocol Pending (as of July 1, 2019)
       Analysis (expected by June 30, 2020)

PC19-03
Title: **The impact of pre-transplant extramedullary disease on the outcome of allogeneic hematopoietic cell transplantation for acute myeloid leukemia in children- A combined CIBMTR and EBMT analysis**
PIs: H Rangarajan (Nationwide Children’s Hospital)
     P Satwani (NYPH/ Columbia University Medical Center)
     K Rao (University of North Carolina Hospitals)
     D Chellapandian (Johns Hopkins All Children’s Hospital)
     B Savani (Vanderbilt University Medical Center)
     Juliana Silva (Great Ormond Street Hospital for Children NHS Foundation Trust)
Status: Data File Preparation (as of July 1, 2019)
       Analysis (expected by June 30, 2020)
* Collaborative study with EBMT
15.0 PLASMA CELL DISORDERS AND ADULT SOLID TUMORS WORKING COMMITTEE

15.1 Leadership

Chair: Shaji Kumar, Mayo Clinic Rochester
Email: kumar.shaji@mayo.edu
Chair: Nina Shah, MD, University of California San Francisco Medical Center
Email: nina.shah@ucsf.edu
Chair: Muzaffar Qazilbash, MD, MD Anderson Cancer Center
Email: mqazilba@mdanderson.org
Scientific Director: Parameswaran Hari, MD, MS, CIBMTR Milwaukee
Email: phari@mcw.edu
Asst Sci Director: Anita D'Souza, MD, CIBMTR Milwaukee
Email: anitadsouza@mcw.edu
Statistical Director: Raphael Fraser, PhD, CIBMTR Milwaukee
Email: rfraser@mcw.edu
MS Statistician: Noel Estrada-Merly, MS, CIBMTR Milwaukee
Email: nestrada@mcw.edu

15.2 Recent Publications

2019

2018


2017


2016


2015


15.3 Current Studies

**MM17-01**

**Title:** Hematopoietic cell transplantation for primary plasma cell leukemia in the era of novel agents

**PIs:** Saulius Girnius (TriHealth Cancer Institute)
Sagar S. Patel (University of Utah)
Lohith S. Bachedgowda (M.D. Anderson Cancer Center)
Binod Dhakal (Medical College of Wisconsin)

**Status:** Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
MM17-02
Title: The impact of bortezomib based induction therapy vs no induction therapy on outcomes for light chain amyloidosis
PIs: Robert F. Cornell (Vanderbilt University Medical Center)
Luciano Costa (University of Alabama at Birmingham)
Stacey A. Goodman (Vanderbilt University Medical Center)
Status: Protocol Development (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

MM17-03
Title: Assessment of allogeneic hematopoietic cell transplantation in Medicare beneficiaries with multiple myeloma: A study to develop evidence of effectiveness for the Centers for Medicare and Medicaid Services (CMS)
PIs: Anita D'Souza (Medical College of Wisconsin)
Parameswaran Hari (Medical College of Wisconsin)
Mary Horowitz (Medical College of Wisconsin)
Sergio Giralt (Memorial Sloan Kettering Cancer Center)
Gunjun Shah (Memorial Sloan Kettering Cancer Center)
Status: Data Collection/Data File Preparation (as of July 1, 2019)
Data Collection/Data File Preparation (expected by June 30, 2020)

MM18-01
Title: Racial discrepancy in clinical outcomes of multiple myeloma patients harboring t(11;14) genetic abnormality
PIs: Dharshan Sivaraj (Duke University Medical Center)
Amrita Krishnan (City of Hope)
Cristina Gasparetto (Duke University Medical Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

MM18-02
Title: Prognostic scoring system of outcomes in patients undergoing autologous stem cell transplantation for multiple myeloma
PIs: Binod Dhakal (Medical College of Wisconsin)
Saurabh Chhabra (Medical College of Wisconsin)
Natalie Callander (University of Wisconsin Hospital and Clinics)
Aric Hall (University of Wisconsin Hospital and Clinics)
Zhubin Gahvari (University of Wisconsin)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
* Collaborative study with EBMT
MM18-03
Title: To compare the outcomes in young patients with multiple myeloma at diagnosis (<50 years) undergoing autologous or allogeneic hematopoietic stem cell transplant with older patients (≥50): progression-free and overall survival in a case match
PIs: Pashna Munshi (Georgetown University Hospital)
     Artur Jurczyszyn (Jagellonian University Medical College)
     Jan Maciej Zaucha (Medical University of Gdansk)
     David Vesole (Hackensack University Medical Center)
Status: Manuscript Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)

MM18-04
Title: Busulfan, melphalan, and bortezomib versus high-dose melphalan as a conditioning regimen for autoHCT in MM: Long term follow up of a novel conditioning regimen
PIs: Patrick Hagen (Loyola University Medical Center)
     Patrick Stiff (Loyola University Medical Center)
Status: Manuscript Preparation (as of July 1, 2019)
        Submitted (expected by June 30, 2020)
* Collaborative study with Loyola University

MM19-01
Title: Impact of induction therapy with VRD vs. VCD on outcomes in patients with multiple myeloma undergoing stem cell transplantation
PIs: Surbhi Sidana (Mayo Clinic)
     Maxim Norkin (Shands HealthCare & University of Florida)
     Shaji K. Kumar (Mayo Clinic Rochester)
     Sergio Giralt (Memorial Sloan Kettering Cancer Center)
Status: Draft Protocol Received (as of July 1, 2019)
        Analysis (expected by June 30, 2020)

MM19-02
Title: Maintenance therapy after second autologous hematopoietic cell transplantation for multiple myeloma
PIs: Oren Pasvolsky (Rabin Medical Center)
     Moshe Yeshurun (Davidoff Cancer Center, Rabin Medical Center, Beilinson Hospital)
     Uri Rozovski (Rabin Medical Center)
     Liat Shargian-Alon (Rabin Medical Center)
Status: Protocol Pending (as of July 1, 2019)
        Analysis (expected by June 30, 2020)
Title: Second autologous stem cell transplantation as salvage therapy for relapsed or refractory AL amyloidosis

PIs: Carlyn Tan (Fox Chase Cancer Center)
     Henry Fung (Fox Chase Temple University Hospital Bone Marrow Transplant Program)

Status: Draft Protocol Received (as of July 1, 2019)
        Data File Preparation (expected by June 30, 2020)
16.0 REGIMEN-RELATED TOXICITY AND SUPPORTIVE CARE WORKING COMMITTEE

16.1 Leadership

**Chair:** Shin Mineishi, MD, Penn State Hershey Medical Center
Email: smineishi@pennstatehealth.psu.edu

**Chair:** Edward Stadtmauer, MD, Abramson Cancer Center University of Pennsylvania Medical Center
Email: edward.stadtmauer@uphs.upenn.edu

**Chair:** Bipin Savani, MD, Vanderbilt University Medical Center
Email: bipin.savani@vumc.org

**Scientific Director:** Marcelo Pasquini, MD, MS, CIBMTR Milwaukee
Email: mpasquini@mcw.edu

**Asst Sci Director:** Saurabh Chhabra, MD, CIBMTR Milwaukee
Email: schhabra@mcw.edu

**Statistical Director:** Brent Logan, PhD, CIBMTR Milwaukee
Email: blogan@mcw.edu

**MS Statistician:** Mariam Johnson, MPH, CIBMTR Milwaukee
Email: mhjohnson@mcw.edu

16.2 Recent Publications

**2019**


2018


2017


2016


2015


2014


16.3 Current Studies

**RT13-02**
Title: Safety of high-dose TBI followed by an allogeneic stem cell transplant for hematologic malignancies
PI: Mitchell Sabloff (The Ottawa Hospital Blood & Marrow Transplant Program)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)

**RT14-02**
Title: Endothelial injury complications after allogeneic hematopoietic cell transplantation
PIs: Narendranath Epperla (Ohio State Medical Center, James Cancer Center)
Ang Li (University of Washington)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

**RT14-03**
Title: Multicenter cohort identification of transplant-related risk-factors for infection, organ failure, and mortality among pediatric hematopoietic stem cell transplant patients requiring intensive care unit admission
PIs: Matt Zinter (University of California San Francisco Medical Center)
Chris Dvorak (University of California San Francisco Medical Center)
Anil Sapru (University of California San Francisco Medical Center)
Status: Submitted (as of July 1, 2019)
Published (expected by June 30, 2020)
* Collaborative study with Virtual Pediatric Systems

**RT17-01**
Title: Allogeneic hematopoietic stem cell transplant outcome for patients with end stage renal disease on dialysis
PIs: Nosha Farhadfar (Shands HealthCare & University of Florida)
Ajoy Dias (University of Kansas Medical Center)
John Reid Wingard (Shands HealthCare & University of Florida)
Hemant Murthy (Mayo Clinic Florida)
Sid Ganguly (University of Kansas Medical Center)
Status: Manuscript Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)
RT18-01
Title: Developing a modified hematopoietic stem cell transplantation-comorbidity index for adolescents and young adults / A modified hematopoietic cell transplantation-comorbidity index (HCT-CI) for pediatric recipients of allogeneic transplantation
PIs: Brian Friend (University of California Los Angeles)
      Gary Schiller (UCLA Health)
      Larisa Broglie (Columbia University)
      Monica Thakar (Fred Hutchinson Cancer Research Center)
      Mohamed Sorror (Fred Hutchinson Cancer Research Center)
Status: Data File Preparation (as of July 1, 2019)
Submitted (expected by June 30, 2020)

RT18-02
Title: The effect of obesity on outcomes after alternative donor stem cell transplants
PIs: Mouhamed Yazan-Abou Ismail (Rochester General Hospital)
      Gayathri Ravi (Seidman Cancer Center - University Hospitals Cleveland Medical Center)
      Leland Metheny (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
      Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)
Status: Data File Preparation (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

RT18-03
Title: An analysis of non-infectious pulmonary toxicities in myeloablative total body irradiation vs chemotherapy-based conditioning regimens after alloHCT for hematologic malignancies / Diffuse alveolar hemorrhage (DAH) is a result of complex interaction between conditioning regimen (MAC>RIC and TBI>noTBI), graft source (UCB>others), and engraftment (delayed>early).
PIs: Sagar Patel (Cleveland Clinic Foundation)
      Betty Hamilton (Cleveland Clinic Foundation)
      Navneet Majhail (Cleveland Clinic Foundation)
      Celattin Ustun (The Coleman Foundation Blood and Marrow Transplant Center, Rush University)
Status: Data File Preparation (as of July 1, 2019)
Manuscript Preparation (expected by June 30, 2020)

RT19-01
Title: Analysis of comorbidity-associated toxicity at the regimen level.
PIs: Roni Shouval (Sheba Medical Center)
      Bipin Savani (Vanderbilt University Medical Center)
      Aarnon Nagler (Sheba Medical Center)
Status: Draft Protocol Received (as of July 1, 2019)
Analysis (expected by June 30, 2020)
RT19-02
Title: Hemorrhagic cystitis as a complication of HCT in the Pt-Cy GVHD prophylaxis era compared to other alloHCTs.
PIs: Kehinde Adekola (Northwestern Medicine)  
     Naveed Ali (Federal Institute for Material Research)  
     Olga Frankfurt (Northwestern Medicine)  
     Leland Metheny (Seidman Cancer Center-University Hospitals Cleveland Medical Center)  
     Jonathan Moreira (Northwestern Medicine)  
     Marcos de Lima (Seidman Cancer Center-University Hospitals Cleveland Medical Center)  
Status: Protocol Pending (as of July 1, 2019)  
     Data File Preparation (expected by June 30, 2020)
APPENDIX A: COLLABORATIVE STUDIES

AABB

• DS17-02: Impact of pre-operative collection of auto blood for BM harvest on donor health and outcome
  o Also in collaboration with ASTCT

ASTCT

• DS17-02: Impact of pre-operative collection of auto blood for BM harvest on donor health and outcome
  o Also in collaboration with AABB

Brazilian Transplant Group

• HS19-03: Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: A multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group
• HS19-04: Outcomes after allogeneic stem cell transplants performed in Brazil from HLA-matched siblings, unrelated and mismatched related donors: Retrospective study on behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle Transplant-related complications Consortium), Hospital Israelita Albert Einstein (AmigoH), Associação da Medula Óssea do Estado de São Paulo (Ameo), Program Nacional de Apoio à Atenção Oncológica (Pronon), and CIBMTR

CALGB 10403

• LK15-03: Comparison of outcomes of older adolescents and young adults with Philadelphia-chromosome / BCR-ABL1-negative acute lymphoblastic leukemia receiving post-remission consolidation chemotherapy with pediatric-inspired chemotherapy on CALGB 10403 or myeloablative allogeneic hematopoietic cell transplantation

City of Hope

• CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  o Also in collaboration with 12 other organizations

Cleveland Clinic Foundation

• CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  o Also in collaboration with 12 other organizations

Dana-Farber Cancer Institute

• CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  o Also in collaboration with 12 other organizations
Deutsche Knochenmarkspenderdatei GmbH (DKMS, German Bone Marrow Donor Center)

- IB18-04: Evaluation of the impact of donor KIR genotype on outcome after unrelated donor transplantation in patients with myelodysplastic syndromes or secondary acute myeloid leukemia

Duke University Medical Center

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

EBMT

- CK17-01: Development of a prognostic scoring system predictive of outcomes in patients with myelofibrosis after allogeneic hematopoietic cell transplantation
- CK19-01b: Outcomes after HCT for rare chronic leukemias: Outcomes of chronic neutrophilic leukemia patients who underwent alloHCT
- GS18-03: Comparison of outcomes of reduced intensity transplantation in lymphoma patients using haploidentical related donors vs. unrelated cord blood
- LY19-01: Post-transplant cyclophosphamide-based haploidentical transplantation versus matched sibling or well-matched unrelated donor transplantation for peripheral T-cell lymphoma: A CIBMTR Lymphoma Working Committee and EBMT Lymphoma Working Party analysis
- NM15-01: Outcome of allogeneic hematopoietic cell transplant in erythropoietic porphyria
- NM17-01: Late effects after hematopoietic stem cell transplantation in patients with hemophagocytic lymphohistiocytosis
- PC19-03: The impact of pre-transplant extramedullary disease on the outcome of allogeneic hematopoietic cell transplantation for acute myeloid leukemia in children: A combined CIBMTR and EBMT analysis
- MM18-02: Prognostic scoring system of outcomes in patients undergoing autologous stem cell transplantation for multiple myeloma

The Emmes Company

- LE18-03: Incorporating patient reported outcomes into individualized prognostication tools for survival and quality of life in transplant patients

Emory University

- LE17-01: Long-term follow up after hematopoietic stem cell transplantation for sickle cell disease

H. Lee Moffitt Cancer Center and Research Institute

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

The Hospital for Sick Children

- IB19-05: Impact of donor signal-regulatory protein alpha polymorphism on outcomes of allogeneic hematopoietic stem cell transplantation
International Histocompatibility Working Group

- IB06-05: Use of high-resolution HLA data from the NMDP for the International Histocompatibility Working Group in HCT
- IB09-01: Clinical importance of minor histocompatibility complex haplotypes in umbilical cord blood transplantation
- IB09-03: Clinical relevance of cytokine / immune response gene polymorphisms in umbilical cord blood transplantation
- IB09-05: Identification of functional single nucleotide polymorphisms in umbilical cord blood transplantation
- IB09-07: Clinical significance of genome-wide variation in unrelated HCT
- IB14-07: Indirectly recognizable HLA epitopes (PIRCHES): A retrospective validation study on the role of indirect recognition of mismatched HLA in hematopoietic stem cell transplantation outcome
- IB18-05: Imputation of KIR in genome-wide association study and the association of KIR-HLA with outcomes following alloHCT in AML and MDS
- R04-74d: Functional significance of killer cell immunoglobulin-like receptor genes in HLA-matched and mismatched unrelated HCT

Japan Society for Hematopoietic Cell Transplantation

- HS18-01: International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens

Loyola University

- MM18-04: Busulfan, melphalan, and bortezomib versus high-dose melphalan as a conditioning regimen for AutoHCT in MM: Long term follow up of a novel conditioning regimen

M.D. Anderson Cancer Center

- CK12-01: Optimal timing of allogeneic stem cell transplantation for chronic myeloid leukemia patients in the tyrosine kinase inhibitor era
- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

Massachusetts General Hospital

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

Mayo Clinic Arizona and Phoenix Children’s Hospital

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations
**Medical College of Wisconsin**

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations
- IB15-04: Clinical outcomes among hematopoietic stem cell transplant recipients as a function of socioeconomic status and related transcriptome differences

**Memorial Sloan Kettering Cancer Center**

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

**National Cancer Institute**

- IB10-01f: Epigenetic clock: Can this guide donor selection in HCT?
- IB16-03: Role of recipient and donor genetic polymorphisms in interferon lambda 4 (INFL4) on outcomes after unrelated allogeneic cell transplant
- IB17-03: Identification of genomic markers of post hematopoietic cell transplantation (HCT) outcomes in patients with myelofibrosis: A pilot study
- IB18-06: Clonal mosaicism and HCT outcomes in patients with acute leukemia and myelodysplastic syndromes
- IB18-07: Donor and recipient genomic associations with acute GVHD
- AA13-02: Malignancies in patients with Fanconi anemia

**National Heart, Lung, and Blood Institute**

- NM19-01: Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after alloHCT or immunosuppressive therapy

**National Institutes of Health**

- LE13-02: Risk factors for melanoma following allogeneic hematopoietic stem cell transplantation

**Northwestern University**

- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

**The Ohio State University**

- IB09-06f: DISCOVeRY-BMT: Case-control study of acute myelogenous leukemia exome and genome-wide association study
- IB09-06j: DISCOVeRY-BMT: Additional analysis of major histocompatibility complex single nucleotide polymorphisms
- IB09-06m: DISCOVeRY-BMT: Analysis of X chromosome single nucleotide polymorphisms
- IB09-06n: DISCOVeRY-BMT: Compare unrelated donor to Welcome Trust Case Control Consortium controls
- IB09-06o: DISCOVeRY-BMT: Genetics and epidemiology of myeloid malignancies candidate gene paper
• IB09-06p: DISCOVeRY-BMT: Genetics and epidemiology of myeloid malignancies genome-wide association study
• RT09-04 / IB09-06i: DISCOVeRY-BMT: Recip, donor genome-wide association study interaction with conditioning intensity (myeloablative / reduced intensity conditioning), total body irradiation, disease status

Peking University
• HS19-02: Comparing outcomes of myeloablative T-replete haploidentical transplantation with PT-CY protocol and ATG+G-CSF protocol in patients with cytogenetic intermediate / high risk acute myeloid leukemia in first complete remission

Pediatric Health Information System (PHIS)
• HS14-01: Investigating clinical outcomes and inpatient health care resource utilization of hematopoietic cell transplantation for children with acute leukemia
• HS17-01: Association of community health status and center survival for allogeneic hematopoietic cell transplantation
• HS18-03: Racial / ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia

Roswell Park Cancer Institute
• IB09-06b: Genetic susceptibility to transplant-related mortality after unrelated donor stem cell transplant
• IB09-06f: DISCOVeRY-BMT: Case-control study of acute myelogenous leukemia exome and genome-wide association study
• IB09-06j: DISCOVeRY-BMT: Additional analysis of major histocompatibility complex single nucleotide polymorphisms
• IB09-06m: DISCOVeRY-BMT: Analysis of X chromosome single nucleotide polymorphisms
• IB09-06n: DISCOVeRY-BMT: Compare unrelated donor to Welcome Trust Case Control Consortium controls
• IB09-06o: DISCOVeRY-BMT: Genetics and epidemiology of myeloid malignancies candidate gene paper
• IB09-06p: DISCOVeRY-BMT: Genetics and epidemiology of myeloid malignancies genome-wide association study
• RT09-04 / IB09-06i: DISCOVeRY-BMT: Recip, donor genome-wide association study interaction with conditioning intensity (myeloablative / reduced intensity conditioning), total body irradiation, disease status

Severe Chronic Neutropenia International Registry
• ID13-01: Haematopoietic stem cell transplants for congenital neutropenia / Kostmann agranulocytosis

University of Florida
• LE99-01: Quality of life in late HCT survivors
University of Chicago
- CK16-01: Identification of germline predisposition mutations in young myelodysplastic syndrome patients

University of Michigan
- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations

University of Minnesota
- IB14-05: mtDNA haplotypes and unrelated donor transplant outcomes
- IB15-03: Killer immunoglobulin receptor (KIR) gene content and pediatric acute leukemia transplant outcomes
- R02-40 / R03-63d: Acquisition of natural killer cell receptors in recipients of unrelated transplant
- SC09-05d: Regret having bone marrow transplant

University of Pittsburgh
- DS19-02: The impact of pre-apheresis health related quality of life on peripheral blood progenitor cells yield and donor's health and outcome

University of Utah
- DS05-02d: QoL for related adult donors compared to unrelated adult donors
- DS05-02g: Late toxicities and SAE for related donors

United Network for Organ Sharing
- LE12-03: Solid organ transplantation and hematopoietic cell transplantation

Virtual Pediatric Systems
- RT14-03: Multicenter cohort identification of transplant-related risk-factors for infection, organ failure, and mortality among pediatric hematopoietic stem cell transplant patients requiring intensive care unit admission
- LE19-01: Long-term survival and late effects in critically ill pediatric hematopoietic cell transplant patients

Washington University
- CK15-01: Comparison of transplant vs non transplant therapies for myelofibrosis
  - Also in collaboration with 12 other organizations
- DS18-01: To quantify age-related clonal hematopoiesis in healthy marrow and blood donors that may affect the clinical outcome of recipients following HCT
# APPENDIX B: STUDY DEVELOPMENT AND MANAGEMENT PROCESS

This study development cycle pertains to studies for which CIBMTR provides data, scientific, 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 the CIBMTR prior to journal submission.

## STUDY DEVELOPMENT AND MANAGEMENT PROCESS

<table>
<thead>
<tr>
<th>Planned</th>
<th>Protocol pending. Proposals remain in this preliminary stage until the principal investigator (PI) creates a draft protocol.</th>
</tr>
</thead>
<tbody>
<tr>
<td>Draft protocol received. When a PI submits a draft protocol, Coordinating Center staff review it.</td>
<td></td>
</tr>
<tr>
<td>Protocol development. During the development process, the Working Committee biostatisticians, Scientific Director, and Chairs refine the submission into a comprehensive study protocol. They add a table with a preliminary description of the proposed study population and present the draft protocol for discussion at a weekly Coordinating Center statistical meeting. When a protocol is approved, Coordinating Center personnel invite Working Committee members to participate in a Writing Committee.</td>
<td></td>
</tr>
<tr>
<td>In Progress</td>
<td>Sample typing. If applicable, the PIs perform laboratory tests (e.g., genotyping) on samples from the CIBMTR Research Repository. The testing data will be used in the analysis to determine any correlation with clinical outcome.</td>
</tr>
<tr>
<td>Supplemental forms / data collection. Most studies use routinely-collected data. If necessary, Coordinating Center staff, in collaboration with the PI and relevant Working Committee Chairs, develop a supplemental form, which is approved prior to soliciting centers for additional data. Use of supplemental data (e.g., data not collected on standard CIBMTR data collection forms) is discouraged unless it will result in a particularly meaningful publication and/or external funding can support the extra burden placed on centers and supplement forms reimbursement costs.</td>
<td></td>
</tr>
<tr>
<td>STUDY DEVELOPMENT AND MANAGEMENT PROCESS</td>
<td></td>
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<tr>
<td>------------------------------------------</td>
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</tr>
<tr>
<td><strong>Data file preparation.</strong> 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-level statistician, working with the Scientific Director, PI(s), and sometimes the Clinical Research Coordinator, to ensure data quality:</td>
<td></td>
</tr>
<tr>
<td>• Verifying selection criteria</td>
<td></td>
</tr>
<tr>
<td>• Including and excluding patients so that the investigators can determine whether the final study population is representative of the target population</td>
<td></td>
</tr>
<tr>
<td>• Assessing follow-up</td>
<td></td>
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<tr>
<td>• Determining the extent and nature of missing values and their potential effect on the study</td>
<td></td>
</tr>
<tr>
<td>• Resolving and reconciling data discrepancies / outliers by examining data collection forms and communicating with centers and the PI</td>
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</tbody>
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<table>
<thead>
<tr>
<th>In Progress (continued)</th>
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<tbody>
<tr>
<td><strong>Analysis in progress.</strong> Analysis proceeds in several phases. The first generally includes a detailed description of the patient population and univariate and multivariate analyses of study endpoints. Study PI(s) and associated Working Committee Chairs present these data for discussion at a weekly Coordinating Center statistical meeting and then distribute them to Writing Committee members for suggestions and comments. The PI works with Coordinating Center staff in an iterative process to review comments from the Writing Committee. The process repeats until final analysis, which serves as the basis for the manuscript.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Ongoing</th>
</tr>
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<tbody>
<tr>
<td>A study in ongoing status is long-term and often involves multiple grants and/or renewals outside of the CIBMTR in order to reach its objectives. The study typically has its own Statistical Director for analysis, but it requires data from the CIBMTR, usually each year.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Preliminary Results</th>
</tr>
</thead>
<tbody>
<tr>
<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. Study Leadership reviews and revises the document, ensuring that the description and interpretation of the statistical analyses are accurate and contribute to the fundamental message of the manuscript. The Coordinating Center then distributes the approved first draft to the Writing Committee and solicits feedback. The PI incorporates comments from the Writing Committee and creates a revised draft, which is reviewed in an iterative process by the Writing Committee until reaching a reasonable consensus on a final manuscript.</td>
</tr>
<tr>
<td>STUDY DEVELOPMENT AND MANAGEMENT PROCESS</td>
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<tr>
<td>------------------------------------------</td>
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<tr>
<td><strong>Preliminary Results (continued)</strong></td>
</tr>
<tr>
<td><strong>Submitted.</strong> The Coordinating Center staff is responsible for submitting the manuscript and corresponding with the chosen journal. The Working Committee Scientific Director often serves as corresponding author, and the study statistician forwards all editor and reviewer comments to the PI and Statistical Director. The PI is expected to prepare a response, working with Study Leadership who provide additional analyses of data, as needed. Coordinating Center personnel communicate with the journal, including re-submissions, in most cases.</td>
</tr>
<tr>
<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><strong>Completed</strong></td>
</tr>
<tr>
<td><strong>Published.</strong> A manuscript is considered published when a citation is available, including a PMCID number, if applicable.</td>
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</table>
APPENDIX C: GUIDELINES FOR STUDY PRINCIPAL INVESTIGATORS

The role of a Study Chair / PI is simple: always behave ethically and do whatever it takes to complete the study that answers your research question. It is easier to accomplish this task if you have an understanding of the CIBMTR study process, specifically where and when your efforts are most needed. The following document will explain the life-cycle of a CIBMTR observational study and review the responsibilities of a PI. Hints and tips to make the study process as successful as possible are noted with an arrow (→).

STUDY PROPOSAL

The PI is usually the first person who suggests the study and who prepares the Study Proposal (cibmtr.org/Studies/Observational/ProposeStudy/Pages/index.aspx). Ideally the PI presents his or her proposal in person to the appropriate Working Committee at the TCT Meetings. Some PIs view the CIBMTR presentation as a formality but, in reality, it is an important opportunity to convince the other Working Committee members that your study is more important, more feasible, and more likely to advance the field and be published in a high-profile journal than other studies being proposed. CIBMTR Working Committee hours and resources are limited, and not all good studies proposed can be supported.

→ Review the data collection forms (cibmtr.org/DataManagement/DataCollectionForms/Pages/Investigators.aspx) to ensure the data you wish to study are available for the timeframe you wish to study. Many people propose studies that require data not collected routinely by the CIBMTR. Studies that require additional data collection usually get greater scrutiny because of the extra time and effort required whenever centers have to be contacted for additional data. Additionally, the response rate to requests for supplemental data is often disappointing; ask your own data managers how difficult it is to go back and find data for patients transplanted years ago. The ability to collect supplemental data successfully depends on how complex and/or extensive the data are, the size of the study population, how far back in time the transplants were done, and whether you have resources (people or funds) to assist in the process.

→ Examine the latest accrual tables for your proposed Working Committee to ensure there are sufficient data in the CIBMTR Research Database to answer your study questions. Accrual tables are provided in the meeting agenda for each committee’s annual in person meeting at the TCT Meetings under Attachment 2.

→ Review this report and/or the CIBMTR Publication List and Working Committee Study Lists of planned, in progress, and recently published studies to ensure someone else has not already conducted the study you are proposing.

→ Note that study proposals may be submitted throughout the year. The vast majority are submitted just before the deadline, three months before the TCT Meetings. If you want your proposal to benefit from greater CIBMTR statistical and scientific input, then submitting your proposal far in advance is helpful. Proposals submitted throughout the year will be reviewed by the Working Committee Chairs and Scientific Director. They have the authority to approve a proposal based on the importance of the scientific question or they may elect to defer it until presentation at the Working Committee meeting.
PRIORITIZATION AND DISTRIBUTION OF STATISTICAL HOURS

After the TCT Meetings, the Working Committee Chairs, Scientific Directors, Statistical Director, and MS Statistician meet to discuss the results of the meeting and prioritize new and ongoing studies. Studies are assigned Coordinating Center hours according to their need and priority. In general, a study needs 100 hours of Statistical Staff time to finish the protocol document, 100-160 hours to prepare the data file (depending on whether additional data collection, follow-up, or excessive data cleaning is necessary), 60 hours for the analysis phase, and 70 hours for manuscript preparation. PIs are generally notified about Committee decisions (i.e., approval, prioritization, and assigned hours) regarding their proposals within one month after the meeting. At that time, PIs also learn which MS-level Statistician is assigned to their studies; this MS-level Statistician subsequently serves as the point person for communications regarding study issues.

→ PIs can increase the chance of their proposal being approved by carefully preparing the Proposal Form that is presented to the Working Committee. Discussion with Working Committee Leadership in advance of the Working Committee meeting may help clarify the study and address study design questions. Many great concepts fail because PIs do not consider available data, size of the available study population, power calculations, and other statistical issues. The Working Committee is much less receptive to studies that appear to have multiple unresolved issues at the meeting.

STUDY PROTOCOL

The next step in the study’s life is generation of the study protocol. This is an important document that is first drafted by the PI and submitted to the MS-level Statistician. The draft study protocol should be completed within two months of concept approval notification. In preparing this document, it is crucial to carefully review the applied study selection criteria and description of patient, disease, and transplant characteristics. The PI must also carefully consider the variables to be included in the analysis because the MS-level Statisticians, Statistical Directors, and Scientific Directors use these documents to guide data collection and cleaning. Common pitfalls include failure to include important variables to address study hypotheses and failure to consider potentially confounding variables. After the initial draft is reviewed and approved by the Coordinating Center, it is circulated to the Working Committee for comment; at that time, Committee members may request to participate in the study and a Writing Committee is formed (see below). Individuals wishing to serve on the Writing Committee provide substantive comments on the study protocol. It is the PI’s responsibility to collate and address these comments by either modifying the protocol or providing an explanation for not incorporating suggested changes. Since Writing Committee members earn their authorship by reviewing the study protocol, analyses, and manuscripts, the CIBMTR also keeps track of comments and contributions.

→ Each study protocol is reviewed at the weekly Coordinating Center conference call / meeting (held on Tuesdays, 9:00-10:00 am US Central Time) before distribution to the Working Committee; it is very helpful for the PI to join that meeting by phone and to participate in the discussion of the study’s design and implementation. (Studies are again discussed at a Coordinating Center weekly meeting as they reach significant milestones. PI participation in each of these discussions is strongly encouraged.)
The most successful PIs respond to Writing Committee critiques as they do journal reviews — by carefully organizing them and responding to each. If a Writing Committee member brought up an issue, it is likely that a reviewer will also bring up the same points. It is expected that the PI will summarize and respond to these critiques within three weeks after the deadline for comments has passed.

PIs have a great deal of control over the interval between study proposal approval and the completion of a final study protocol. Timely submission of the draft protocol and response to Writing Committee comments can vault your study ahead of others in terms of Coordinating Center priority. If yours is ready to go and another is not, yours may be given priority, even if initially it was planned for the other study to be done first.

DATA COLLECTION

If supplemental data collection is needed for the study, approval from the Chief Scientific Director is required. The PI needs to provide the following information for the approval: 1) number of questions, 2) types of questions, 3) number of cases and 4) the study calendar. Once the request has been approved, the Forms Development Clinical Research Coordinator will prepare a supplemental form for review within one week. This draft form will be a Word document listing all the supplemental questions that are relevant, as well as the most frequent response options. This form will have input from the Scientific Director, PI, Study Statistician, Metadata and Data Operations Staff for clarity, length, internal consistency of response options, and feasibility of data retrieval. The form will be formatted to be consistent with other CIBMTR forms, and a table will be created in the database to receive the data. This step is very important for any study collecting additional data. If the form is long or leaves out critical variables, the ultimate study results could be compromised by missing data. The supplemental form will go to the Chief Scientific Director, Scientific Director, and PI for final approval. The Scientific Director and PI will prepare a letter detailing the importance of the data needed for the study with a copy to the Medical Director. This letter will be sent with the study request. If terms or concepts on the supplemental data collection form are unfamiliar to the data management teams, an instruction manual that describes the variable and provides examples of how data managers should interpret primary data will have to be written. Each study is assigned a Clinical Research Coordinator who communicates with centers to facilitate data submission. Most, but not all, centers are very responsive to these requests. If some centers are lagging behind in submitting extra forms, PIs may need to make personal email or phone appeals.

Providing the initial draft form and content for the instruction manual is the responsibility of the PI. Delay in putting it together can significantly delay initiating the data collection process. If the process is inordinately delayed so that the data needed for a study is not available in a timely manner, the study may be deferred to the next year.

For smaller studies, where every patient counts, personal appeals from the PI to the Transplant Center Director can sometimes be very effective.

DATA FILE PREPARATION

In this step, the MS-level Statistician prepares a data file using the finalized study protocol as guidance. Data interpretation issues may arise here, especially if uncommon variables are necessary for the study. Values for common variables have probably already been reviewed and, if missing or out of range or inconsistent, already clarified (data “cleaning”) for other studies. If
your study is the first to examine a particular variable or study population, then expect to do a lot of data cleaning.

→ The PI can accelerate this process by being available to the MS-level Statistician and Scientific Director as questions come up. The PI should also carefully review the frequencies of study variables for outliers and other clinical inconsistencies.

**UNIVARIATE ANALYSIS**

Once the data file is prepared, the MS-level Statistician performs as much of the analysis as possible before handing the data set to the Statistical Director assigned to the project. First, a table of study population characteristics and preliminary univariate analysis is prepared. This is reviewed by the PI and Scientific Director. When they are satisfied with the population, the study is scheduled for another Coordinating Center weekly meeting / conference call to confirm final composition of the population and study design and review the univariate analysis before multivariate analyses are performed. Relevant comments from the Coordinating Center review will be summarized by the MS-level Statistician or the Scientific Director and relayed back to the PI for comment if the PI cannot participate personally in the meeting.

→ As noted above, the PI is invited to participate in the CIBMTR Coordinating Center Meeting (Tuesdays) when his or her study is discussed. It is worth repeating that it is very helpful for the PI to participate since they can often address questions as they arise so that the statistical input is most helpful.

**MULTIVARIATE ANALYSIS**

Once the population characteristics and univariate analyses are approved, the data file is transmitted to the Statistical Directors for multivariate and more complex modeling. When completed, results are sent to the PI and Scientific Director who present them on a weekly Coordinating Center conference call. The PI and Scientific Director address comments provided at the meeting and then prepare a memo for circulation to the Writing Committee for comments. The comment period usually lasts two to three weeks. The PI summarizes the comments and prepares another memo for the Writing Committee within three weeks of the close of the comment period. If substantive issues arise, especially related to the study population or analyses, then a conference call involving the PI, Scientific Director, Statistical Director, and MS-level Statistician may need to be convened to plan an approach for addressing the comments.

→ The most successful PIs take advantage of the MS-level Statisticians’ and Statistical Directors’ familiarity with the project and the data to finish their analyses quickly. If extended time passes between each phase of the analysis, the Statisticians will have to re-familiarize themselves with the project and coding. A task that could take a couple of hours immediately after the initial results are completed may take much longer a month or two later (and the Statisticians understandably will be less excited about picking up the project again).

**ABSTRACTS**

Many PIs hope to submit abstracts to national and international meetings. Multivariate analyses must be complete with enough time to allow generation of an abstract. These abstracts must be circulated to the Writing Committee and reviewed by the Coordinating Center faculty and staff prior to submission. Please allow enough time to complete these steps before the abstract
deadline. If the abstract is accepted for oral presentation, the Coordinating Center staff will also need to review the slides, primarily for accuracy but sometimes also to make suggestions for clarity. The CIBMTR has a template for format and background that is required for all presentations.

→ Planning for meeting abstracts for the American Society of Hematology and other meetings happens immediately after the TCT Meetings. If you would like to submit your abstract to one of these meetings, an early declaration of your intentions and demonstrable effort in moving towards that goal will result in your study getting higher priority.

→ In general, studies are only submitted to one meeting; once submitted in abstract form, priority should be placed on writing and submitting the manuscript.

MANUSCRIPT

Once the analysis is completed, drafting the manuscript is the responsibility of the PI. A draft manuscript is expected within 30 days of the final analysis. The draft is circulated to the Writing Committee and comments are again summarized and incorporated. At least one round and sometimes up to three or more rounds are necessary to create a final manuscript. The CIBMTR will do the final formatting for journal submission, attach all the co-authors’ information (such as institution and contact information), collect any necessary signatures, and submit the paper. The CIBMTR has a long list of acknowledgment for funding sources that are attached to the paper.

→ The initial manuscript draft usually causes the greatest delay in study progress and is the step most directly under control of the PI. The most successful PIs recognize that publishing their study results is a critical measure of success for all involved parties - themselves, the CIBMTR and all the collaborators involved in the study. Working Committee Chairs have the authority to re-assign a study to a different PI if the delay in manuscript preparation is too long (>60 days).

ACCEPTANCE

Unless the paper is accepted on the first submission, it will need to be revised or resubmitted. If comments are straightforward, the PI can prepare a response to reviewers for circulation, along with the revised version. Some comments from reviewers require additional analyses or discussion at a Coordinating Center meeting prior to resubmission. The CIBMTR will assist with manuscript resubmission. Once the paper is accepted, the PI also handles proof review.

→ Unless a study is completed in record time, it will be “in progress” at the next TCT Meetings. PIs should plan to present a study update at the CIBMTR Working Committee meetings or designate another person on the Writing Committee to do this, as long as the study is active.

→ Any expected or unexpected deviations from the above timetable should be discussed between the PI and Working Committee Leadership. Sometimes unavoidable delays are due to either the CIBMTR or the PI. A proactive plan designed to keep the study moving forward should be devised. Generally, the CIBMTR expects studies to be completed within 18-24 months.
<table>
<thead>
<tr>
<th>Abbreviation/Acronym</th>
<th>Meaning</th>
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<tbody>
<tr>
<td>AA</td>
<td>aplastic anemia</td>
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<tr>
<td>AABB</td>
<td>American Association of Blood Banks</td>
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<tr>
<td>ABO</td>
<td>Landsteiner’s blood grouping system</td>
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<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>APL</td>
<td>acute promyelocytic leukemia</td>
</tr>
<tr>
<td>ASTCT</td>
<td>American Society for Transplantation and Cellular Therapy (formerly ASBMT – American Society for Blood and Marrow Transplantation)</td>
</tr>
<tr>
<td>autoHCT</td>
<td>autologous hematopoietic cell transplantation</td>
</tr>
<tr>
<td>BM</td>
<td>bone marrow</td>
</tr>
<tr>
<td>BMT</td>
<td>bone or blood marrow transplant</td>
</tr>
<tr>
<td>BMT CTN</td>
<td>Blood and Marrow Transplant Clinical Trials Network</td>
</tr>
<tr>
<td>BSI</td>
<td>bacterial bloodstream infections</td>
</tr>
<tr>
<td>CALGB</td>
<td>Cancer and Leukemia Group B (member Alliance for Clinical Trials in Oncology)</td>
</tr>
<tr>
<td>CIBMTR</td>
<td>Center for International Blood and Marrow Transplant Research</td>
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<tr>
<td>CMV</td>
<td>cytomegalovirus</td>
</tr>
<tr>
<td>CR2</td>
<td>second complete remission</td>
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<tr>
<td>CRF</td>
<td>Comprehensive Report Form</td>
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<tr>
<td>DLBCL</td>
<td>diffuse large B-cell lymphoma</td>
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<tr>
<td>DNA</td>
<td>deoxyribonucleic acid</td>
</tr>
<tr>
<td>DRI</td>
<td>disease risk index</td>
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<tr>
<td>EBMT</td>
<td>European Society for Blood and Marrow Transplantation</td>
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<tr>
<td>ECOG</td>
<td>Eastern Cooperative Oncology Group</td>
</tr>
<tr>
<td>FA</td>
<td>Fanconi anemia</td>
</tr>
<tr>
<td>FACT</td>
<td>Foundation for the Accreditation of Cellular Therapy</td>
</tr>
<tr>
<td>FL</td>
<td>follicular lymphoma</td>
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<tr>
<td>FLT3</td>
<td>FMS like tyrosine kinase 3</td>
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<tr>
<td>G-CSF</td>
<td>subcutaneous filgrastim</td>
</tr>
<tr>
<td>GVHD</td>
<td>graft-versus-host disease</td>
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<tr>
<td>HCT or HSCT</td>
<td>hematopoietic stem cell transplantation</td>
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<tr>
<td>HLH</td>
<td>hemophagocytic lymphohistiocytosis</td>
</tr>
<tr>
<td>JACIE</td>
<td>Joint Accreditation Committee – International Society for Cellular Therapy &amp; European Society for Blood and Marrow Transplantation</td>
</tr>
<tr>
<td>JMML</td>
<td>Juvenile Myelomonocytic Leukemia</td>
</tr>
<tr>
<td>KIR</td>
<td>killer-cell immunoglobulin-like receptors</td>
</tr>
<tr>
<td>KIR3DL1</td>
<td>killer cell immunoglobulin like receptor, three Ig domains and long cytoplasmic tail 1</td>
</tr>
<tr>
<td>MDS</td>
<td>myelodysplastic syndrome</td>
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<tr>
<td>mtDNA</td>
<td>mitochondrial DNA</td>
</tr>
<tr>
<td>Abbreviation/Acronym</td>
<td>Meaning</td>
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<tr>
<td>----------------------</td>
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<tr>
<td>MHC</td>
<td>major histocompatibility complex</td>
</tr>
<tr>
<td>MLL</td>
<td>mixed lineage leukemia</td>
</tr>
<tr>
<td>MS</td>
<td>Master of Science (level statistician)</td>
</tr>
<tr>
<td>NK</td>
<td>natural killer (cell)</td>
</tr>
<tr>
<td>NIH</td>
<td>National Institutes of Health</td>
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<tr>
<td>NIMA</td>
<td>National Integrated Medical Association</td>
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<tr>
<td>NMDP</td>
<td>National Marrow Donor Program</td>
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<tr>
<td>PBSC</td>
<td>peripheral blood stem cell</td>
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<tr>
<td>PBSCT</td>
<td>peripheral blood stem cell transplantation</td>
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<tr>
<td>PHIS</td>
<td>Pediatric Health Information System®</td>
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<tr>
<td>PI</td>
<td>principal investigator</td>
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<tr>
<td>PICU</td>
<td>pediatric intensive care unit</td>
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<tr>
<td>PIRCHES</td>
<td>predicted indirectly recognizable HLA epitopes</td>
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<tr>
<td>PMCID</td>
<td>PubMed Central unique identifier</td>
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<tr>
<td>QoL</td>
<td>quality of life</td>
</tr>
<tr>
<td>SAE</td>
<td>severe adverse event</td>
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<tr>
<td>SWOG</td>
<td>Southwest Oncology Group</td>
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<tr>
<td>SWOT</td>
<td>strengths, weaknesses, opportunities, threats</td>
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<tr>
<td>TBI</td>
<td>total body irradiation</td>
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<tr>
<td>TNFSF4</td>
<td>tumor necrosis factor superfamily member 4</td>
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<tr>
<td>TRM</td>
<td>transplantation-related mortality</td>
</tr>
<tr>
<td>US</td>
<td>United States</td>
</tr>
<tr>
<td>VRE</td>
<td>vancomycin-resistant enterococcus</td>
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<td>vs</td>
<td>versus</td>
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</tbody>
</table>