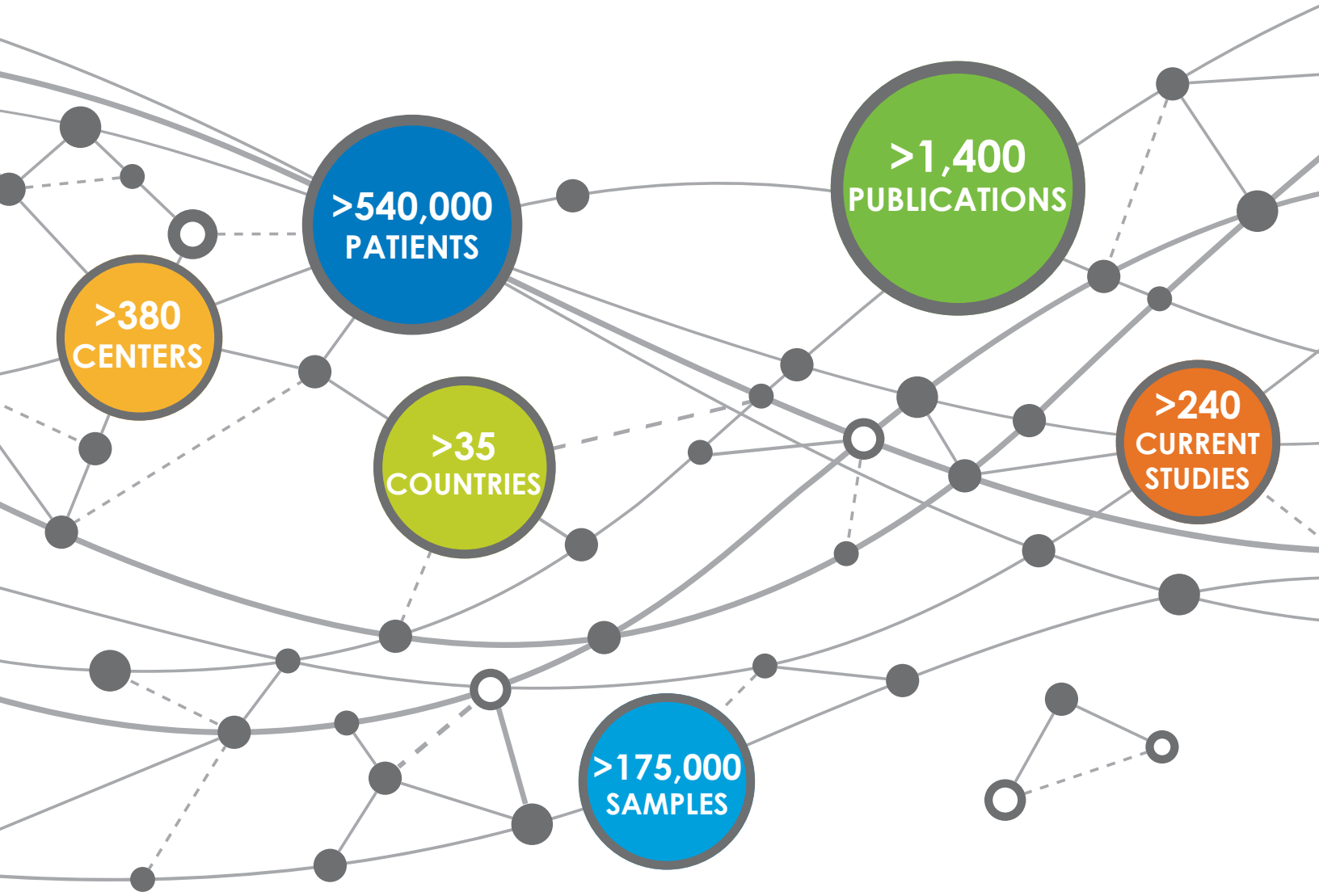
CIBMTR®
CENTER FOR INTERNATIONAL BLOOD
& MARROW TRANSPLANT RESEARCH



2019
ANNUAL REPORT

Sharing Knowledge.
Sharing Hope.

CIBMTR®
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

2019 Annual Report

January – December

Milwaukee Campus
Medical College of Wisconsin
9200 W Wisconsin Ave,
Suite C5500
Milwaukee, WI 53226
(414) 805-0700

Minneapolis Campus
National Marrow Donor Program/
Be The Match
500 N 5th St
Minneapolis, MN 55401
(763) 406-5800

cibmtr.org

TABLE OF CONTENTS

FIGURES AND TABLES

WEB LINKS

Throughout this report, electronic links to webpages and documents are provided; they are underlined and italicized for identification. If you are unable to access items using the links provided, enter the underlined and italicized words into a search engine.

2019 KEY ACCOMPLISHMENTS

CIBMTR by the Numbers

RESEARCH DATABASE

- >380 participating centers
- >540,000 patients
- ~25,000 new patients annually

PUBLICATIONS (Appendix D)

- >1,400 publications since inception
- 95 peer-reviewed publications in 2019
  - 53 from Clinical Outcomes Research Program
  - 18 from Clinical Trials Support Program
  - 10 from Bioinformatics Research Program
  - 8 from Health Services Research Program
  - 6 from Statistical Methodology Research Program

PRESENTATIONS (Appendix E)

- 75 presentations at national and international conferences in 2019 (40 oral and 35 poster)
  - 33 abstracts (14 oral and 19 poster) presented at the ASH Annual Meeting
  - 18 abstracts (9 oral and 9 poster) presented at the TCT Meetings
  - 7 abstracts (5 oral and 2 poster) presented at the European Federation for Immunogenetics Annual Meeting
  - 17 abstracts (12 oral and 5 poster) presented at other national and international conferences

Clinical Outcomes Research Program (Section 2.1)

WORKING COMMITTEES (Section 2.1.1)

- 15 committees with >2,700 researchers worldwide
- 44 global experts voluntarily chair committees
- 173 ongoing studies
- 226 new study proposals
- 36 abstracts (15 oral and 21 poster) presented at national and international conferences
- 44 manuscripts published in peer-reviewed journals

NON-TRANSPLANT CELLULAR THERAPY RESEARCH INITIATIVES (Section 2.1.3)

- 2,908 patients
  - 1,811 received CAR T-cell therapy
- 155 participating centers
- CIDR funded by NIH
- CIDR Executive Committee, CIDR Stakeholder's Council, and Cellular Immunotherapy for Cancer Working Committee established
- 5th Cellular Therapy Registry Forum conducted
- 15-year FDA-mandated follow-up supported for 2 commercial products

More from the Clinical Outcomes Research Program (Section 2.1)

SCTOD (Section 2.1.2)

- Center-Specific Survival Report (2015-2017) and Transplant Center Volumes Data (2014-2018) published
- 17 lay summaries for patients published

CMS CED Studies (Section 2.1.4)

- 5 CMS CED studies conducted
- >900 patients received transplants with Medicare reimbursement because of these studies

PATIENT-REPORTED OUTCOMES (Section 2.1.5)

- Pilot project completed to electronically obtain patient-reported outcomes data using new ePRO system
- 59 patients from 6 centers enrolled in pilot project this year (92 overall)
- Protocol approved to centrally collect patient-reported outcomes data

INTERNATIONAL INITIATIVES (Section 2.1.6)

- WBMT received CIBMTR Distinguished Service Award at TCT Meetings
- FormsNet and CTED forms translated into Japanese to support reporting of non-transplant cellular therapy data to Japanese regulatory agency
- Data management staff members collaboratively trained Brazilian data managers at the Brazilian BMT Annual Meeting

Immunobiology Research Program (Section 2.2)

- Research sample collection and correlative testing for BMT CTN and RCI BMT prospective studies
- 190 centers submitted samples (148 transplant centers, 25 donor centers, and 17 cord blood banks)
- High resolution HLA and KIR typing on 977 related and 3,195 unrelated HCT donor / cord and recipient pairs
- 12,467 samples distributed to investigators for various studies
- 15 publications utilized samples from the Research Repository

RESEARCH REPOSITORY (approximate numbers)

- 4,100 new unrelated recipient samples (68,000 overall)
- 1,300 new related recipient samples (10,900 overall)
- 3,400 new adult unrelated donor samples (71,000 overall)
- 1,200 new related donor samples (10,500 overall)
- 400 new unrelated cord blood samples (12,600 overall)

Clinical Trials Support Program (Section 2.3)

BMT CTN (Section 2.3.1)

- 7 trials launched (53 overall)
- >600 patients accrued to trials (>11,300 overall)
- 11 open protocols
- 6 new protocols in development
- 9,504 new protocol-related biospecimens (416,698 overall)
- 12 abstracts (6 oral and 6 poster) presented at national and international conferences
- 16 manuscripts published in peer-reviewed journals

RCI BMT (Section 2.3.2)

- 6 new studies opened to accrual (24 overall)
- >2,600 participants accrued (approximately 39,000 overall)
- ePRO pilot study complete with data collected on 92 subjects
- 9 active studies supported and 5 upcoming studies in development
- 1 poster abstract presented at international conference
- 2 manuscripts published in peer-reviewed journals

Health Services Research Program (Section 2.4)

- Cost dynamics of HCT versus chemotherapy alone identified for adults with AML; data can be applied for imputing longitudinal costs in randomized controlled trials or dynamic decision-analytic modeling
- CMS Medicare and Medicaid claims data merged with CIBMTR registry data for health economics and outcomes research
- State registry and CIBMTR data linked to evaluate access to HCT at the time of diagnosis of AML
- US donor selection practices clarified and urgent time to HCT defined to support timely access to HCT
- Clinician knowledge gaps addressed regarding the diagnosis, risk stratification, and timing of referral for HCT for patients with AML
- 4 abstracts (2 oral and 2 poster) presented at national and international conferences
- 8 manuscripts published in peer-reviewed journals

Bioinformatics Research Program
(Section 2.5)

- New user platform for demonstration of research-based matching guidelines and tools launched
- Omics data on 1,188 donor and recipient samples received
- Algorithm for multi-racial patient matching developed
- 14 abstracts (9 oral and 5 poster) presented at national and international conferences
- 10 manuscripts published in peer-reviewed journals

Statistical Methodology Research Program
(Section 2.6)

- New statistical models developed
- Statistical integrity of CIBMTR scientific activities ensured
- Articles on cellular therapy-related statistical issues for clinical audiences supported
- Working Committee study investigators supported in developing scientific study protocols using CIBMTR data
- 8 oral abstracts presented at national and international conferences
- 6 manuscripts published in peer-reviewed journals

Research Operations

- 14,344 forms for 7,966 patients submitted through AGNIS (Section 3.1.7)
- Center Support ServiceNow Customer Service Center launched (Section 3.3)
- 6 online data management trainings added / updated (Section 3.3)
- 473 data requests fulfilled (Section 3.4)
- 15 forms (8 revised and 7 new) released in FormsNet (Section 4.2.1)
- Data Transformation Initiative launched (Section 4.2.3)
- 60 centers (50 domestic and 10 international) audited (Section 4.3.3)
- 356 different professional development activities completed by CIBMTR staff members
- 459,212 public website page views (Section 3.1.1)
  - 346,962 Manula (online manuals)
  - 48,717 Training and Reference
  - 47,438 Data Collection Forms
- 37,319 Portal page views (Section 3.1.3)
  - 6,917 DBtC and eDBtC
  - 4,716 Center Volumes Portal
  - 3,610 Survival Calculator for Allogeneic HCT

1.0 WHO WE ARE

The CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin (MCW).

1.1 MISSION

The CIBMTR's mission is to save lives by improving access to and outcomes of cellular therapies worldwide through research and translation.

1.2 VALUE TO THE COMMUNITY

The CIBMTR has collected health outcomes data worldwide for **>45** years, resulting in a Research Database with information on **>540,000** patients. The CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of participating centers, an extensive clinical outcomes database, and a unique biospecimen repository. CIBMTR research involves **6** major programs (**Figure 1.1**).

Figure 1.1 CIBMTR Research Programs

Clinical Outcomes	15 Scientific Working Committees utilize the CIBMTR's Research Database to answer clinically important questions. Each committee focuses on a specific disease or condition, use of specific cell types, or complication of treatment.
Immuno-biology	The CIBMTR manages a repository of tissue samples from donors and recipients, both unrelated and related, to study the genetic, cellular, and immunologic factors that influence transplant outcomes.
Clinical Trials	The BMT CTN conducts multicenter Phase II and III trials with broad national participation while the RCI BMT supports smaller trials that bridge the gap between single-center studies and larger trials.
Health Services	The CIBMTR explores how social factors, financial systems, care processes, and behavior affect access to and outcomes of cellular therapy. Current studies not only improve practice but also address barriers to treatment.
Bioinformatics	The CIBMTR develops and utilizes bioinformatics software tools and analytical methods to facilitate data exchange, interpret information, understand patterns, and predict factors to save and improve lives.
Statistical Methodology	The CIBMTR Coordinating Center provides advice and statistical consultation to researchers developing protocols for cellular therapy studies and investigates new statistical approaches and techniques for analyzing cellular therapy data.

1.3 ORGANIZATIONAL STRUCTURE

The CIBMTR represents a network of **>380** participating centers in **>35** countries (**Appendix A**) that submit outcomes-related data for patients. The CIBMTR Coordinating Center, staffed by approximately **250** employees (**Appendix B**), provides data acquisition and management, information technology (IT), and statistical and scientific support for analyses of these data.

The Chief Scientific Director is responsible for all administrative and scientific operations. The CIBMTR partners scientific and operational leadership within all aspects of its Coordinating Center. The CIBMTR Advisory and Executive Committees (**Table 1.2**), comprised of elected cellular therapy experts, provide input and advice to the internal leadership team, ensuring the continued support of the needs and priorities of the scientific and medical communities.

1.3.1 Scientific Working Committees

To ensure broad input into the research process and efficient use of resources, the CIBMTR looks to **15** Scientific Working Committees focused on specific research areas.

Total Working Committee membership exceeds **2,700** researchers. Membership is open to anyone interested in participating. Many members are clinical researchers, but statisticians, basic scientists, patients, caregivers, and others participate in developing and conducting studies that use CIBMTR data and / or resources. PhD-level statistical faculty and Master's-level statisticians from the CIBMTR Coordinating Center provide unique expertise in data analysis. Basic scientists investigating human leukocyte antigen (HLA), immunogenetics, pharmacogenetics, stem cell biology, and other areas related to cellular therapy provide essential expertise in their respective areas.

Scientific Working Committees

- Acute Leukemia
- Cellular Immunotherapy for Cancer
- Chronic Leukemia
- Donor Health and Safety
- Graft Sources and Manipulation
- Graft-versus-Host Disease
- Health Services and International Studies
- Immunobiology
- Infection and Immune Reconstitution
- Late Effects and Quality of Life
- Lymphoma
- Non-Malignant Diseases
- Pediatric Cancer
- Plasma Cell Disorders and Adult Solid Tumors
- Regimen-Related Toxicity and Supportive Care

The Working Committee structure encourages a collaborative but rigorous methodological approach to CIBMTR clinical outcomes research activities.

Working Committee Leadership

- Chairs (usually 3-4)
- MD Scientific Director
- PhD Statistical Director
- MS-level Statistician

Working Committee leadership is listed in **Appendix C5**.

Table 1.2 Committee Structure

Committee	Function	Meetings	Roster
Joint Affiliation Board	• Reviews and approves the CIBMTR budget and research plan • Amends the terms of the NMDP/Be The Match and MCW affiliation agreement, as necessary • Reviews and approves data access and confidentiality policies	• Annually	Includes representatives from NMDP/ Be The Match and MCW
Assembly	• Includes representatives from each center that submits CRF-level and / or CTED data • Elects members of the Advisory, Nominating, and Clinical Trials Advisory Committees	• Annually during the TCT | Transplanta-tion & Cellular Therapy Meetings of ASBMT and CIBMTR (TCT Meetings)	
Advisory Committee	• Oversees CIBMTR policies and scientific agenda • Partners with the Working Committees to prioritize scientific studies • Oversees operations of the Stem Cell Therapeutic Outcomes Database (SCTOD)	• In person annually at the TCT Meetings • By teleconference quarterly and as needed	Appendix C1
Executive Committee (subcommittee of Advisory Committee)	• Provides scientific and policy advice • Reviews audit results and makes recommendations for improvement	• Four times annually by teleconference	Appendix C2
Consumer Advocacy Committee	• Provides patient and donor perspectives during the development of the CIBMTR research agenda • Communicates CIBMTR research results and data to the non-medical community	• In person annually at the TCT Meetings • By teleconference periodically	Appendix C3

Committee	Function	Meetings	Roster
Nominating Committee	• Prepares a slate of candidates for open positions on the Advisory, Nominating, CIDR Executive, and Clinical Trials Advisory Committees • Makes recommendations to the Advisory Committee for open Working Committee Chair and other leadership appointments	• Three times annually by teleconference	Appendix C4
Scientific Working Committees (Section 1.3.1)	• Design and conduct relevant studies using CIBMTR data, statistical resources, networks, and / or centers • Set priorities for clinical outcomes studies • Assess and revise CIBMTR data collection forms, as needed	• In person annually at the TCT Meetings • Leadership - by teleconference every 4-8 weeks	Leadership - Appendix C5
Cellular Immunotherapy Data Resource (CIDR) Executive Committee	• Provides direction on scientific activities and policy decisions related to CIDR activities • Coordinates activities with other Immuno-Oncology Translational Network (IOTN) programs	• In person twice annually, at the Cellular Therapy Forum and at the TCT Meetings • Four times annually by teleconference	Appendix C6
Immunobiology Steering Committee / NMDP/Be The Match Histocompatibility Advisory Group	• Reviews and approves the use of donor-recipient specimens from the Research Repository in CIBMTR studies	• In person twice annually, in summer and at the TCT Meetings	Appendix C7
Clinical Trials Advisory Committee	• Makes recommendations regarding the RCI BMT's strategy, direction, and alignment with the CIBMTR's scientific agenda	• In person annually at the TCT Meetings • By teleconference as needed	Appendix C8

2.0 WHAT WE DO

The CIBMTR collects data for approximately **25,000** new patients annually as well as a continually increasing volume of follow-up data on previously reported recipients and donors. Centers submit transplant data at two levels: A Transplant Essential Data (TED) level, which captures basic data, and a Comprehensive Report Form (CRF) level, which captures more detail. Centers submit non-transplant cellular therapy data (**Section 2.1.3**) with a suite of Cellular Therapy Essential Data (CTED) forms.

Submission of outcomes data is mandatory for allogeneic transplants in the United States (US); all other submissions are voluntary. The CIBMTR estimates almost **100%** of US allogeneic transplants and **>85%** of US autologous transplants are reported. Since July 2016, the CIBMTR received data for **>2,900** patients who received non-transplant cellular therapy. Data for approximately **12,000** non-US patients are collected annually.

The CIBMTR Research Database contains information on **>540,000** patients. **Figure 2.1** shows the continued growth in the number of patients registered with the CIBMTR. The distribution of patients in the database by type of cellular therapy is displayed in **Figure 2.2.** The distribution of patients by indication is displayed in **Table 2.3** for transplant patients and **Table 2.4** for non-transplant cellular therapy patients.

Research

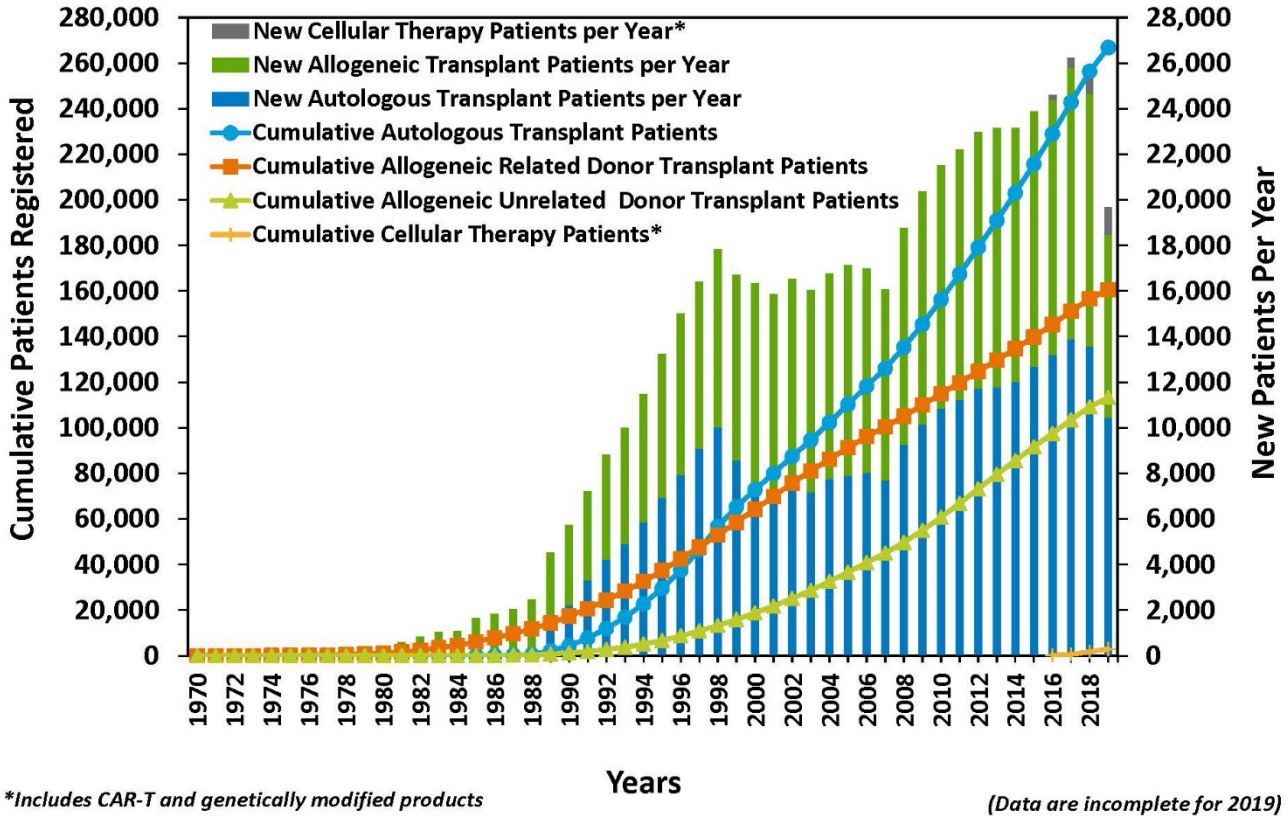
The CIBMTR is dedicated to improving survival, treatment, and quality of life for cellular therapy patients. We provide many opportunities to conduct research utilizing CIBMTR resources, and we encourage both senior and junior investigators to participate.

At any given time, the CIBMTR has **>200** retrospective, correlative, or methodologic studies and **15** prospective trials ongoing in **6** major areas of research activity.

Areas of Research Activity

- **Clinical Outcomes Research (Section 2.1)**
  - Scientific Working Committees (Section 2.1.1)
  - SCTOD (Section 2.1.2)
  - Non-Transplant Cellular Therapy Initiatives (Section 2.1.3)
  - CMS Coverage with Evidence Development Studies (Section 2.1.4)
  - Patient-Reported Outcomes (Section 2.1.5)
  - International Initiatives (Section 2.1.6)
- **Immunobiology Research (Section 2.2)**
- **Clinical Trials Support (Section 2.3)**
  - Blood and Marrow Transplant Clinical Trials Network (BMT CTN) (Section 2.3.1)
  - Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT) (Section 2.3.2)
- **Health Services Research (Section 2.4)**
- **Bioinformatics Research (Section 2.5)**
- **Statistical Methodology Research (Section 2.6)**

Figure 2.1 Continued Growth in the Number of Patients Registered with the CIBMTR



**Includes CAR-T and genetically modified products*

(Data are incomplete for 2019)

Figure 2.2 Distribution of Patients in the CIBMTR Research Database by Type of Cellular Therapy

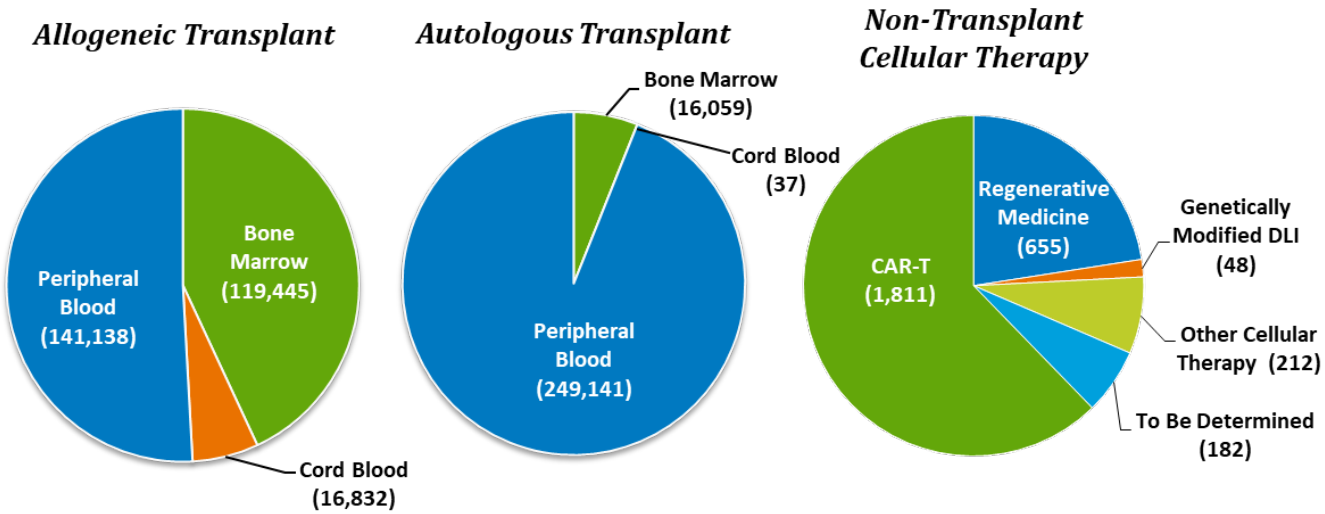


Table 2.3 Distribution of Transplant Patients in the CIBMTR Research Database by Indication

TRANSPLANT DATA	Allogeneic		Autologous		TOTAL
Indication	TED	CRF	TED	CRF	
Lymphoma	18,741	9,164	83,208	15,795	126,908
Plasma cell disorders	4,669	2,391	91,687	16,723	115,470
Acute myeloid leukemia	53,698	32,149	6,253	2,447	94,547
Other malignant diseases[1]	7,090	4,210	33,097	12,533	56,930
Acute lymphoblastic leukemia	26,768	18,494	1,213	479	46,954
Myelodysplastic / myeloproliferative syndromes	17,528	15,030	221	93	32,872
Chronic myelogenous leukemia	14,987	15,423	420	287	31,117
Aplastic anemia / PNH[2]	7,376	8,164	-	-	15,540
Immune disorders[3]	4,167	4,398	-	-	8,565
Hemoglobinopathy[4]	3,296	3,714	-	-	7,010
Inherited bone marrow failure[5]	1,463	1,803	-	-	3,266
Inborn errors of metabolism	1,277	1,647	-	-	2,924
Other nonmalignant disorders[6]	206	169	1,225	169	1,769
Other diseases	382	106	445	17	950
TOTAL	161,648	116,862	217,769	48,542	544,822

1. Includes other leukemia (9,709 allogeneic and 999 autologous) and solid tumors (1,591 allogeneic and 44,631 autologous)
2. Includes severe aplastic anemia (14,872 allogeneic) and paroxysmal nocturnal hemoglobinuria (PNH, 668 allogeneic)
3. Includes immune deficiencies (6,756 allogeneic) and histiocytic disorders (1,809 allogeneic)
4. Includes sickle cell anemia (2,241 allogeneic), sickle cell thalassemia (168 allogeneic), and thalassemia major (4,601 allogeneic)
5. Includes Schwachmann-Diamond (87 allogeneic), Fanconi anemia (2,359 allogeneic), Diamond-Blackfan anemia (460 allogeneic), and other inherited abnormalities of erythrocyte (360 allogeneic)
6. Includes platelet disorders (222 allogeneic and 7 autologous) and autoimmune deficiencies (153 allogeneic and 1,387 autologous)

Table 2.4 Distribution of Non-Transplant Cellular Therapy Patients in the CIBMTR Research Database by Indication

NON-TRANSPLANT CELLULAR THERAPY DATA[1,2] Indication	TOTAL
Non-Hodgkin lymphoma	1,332
Neurologic disorder	651
Acute lymphoblastic leukemia	425
Plasma cell disorder / multiple myeloma	147
Acute myeloid leukemia	78
Solid tumor	30
Myelodysplastic syndrome	21
Immune deficiencies	19
Hodgkin disease	15
Inherited abnormalities of erythrocyte differentiation or function	14
Chronic lymphocytic leukemia / prolymphocytic leukemia	8
Chronic myeloid leukemia	6
Severe aplastic anemia	4
Inherited disorders of metabolism	4
Histiocytic disorder	2
Other acute leukemia	1
Other indication	51
Indication not reported	100
TOTAL	2,908

1. Includes 1,063 therapies that were administered post-transplant
2. Excludes unmanipulated donor leukocyte infusion (DLI)

Publications

In 2019, the CIBMTR published **95** peer-reviewed manuscripts**.** A complete list of 2019 publications is provided in **Appendix D**. **Figure 2.5** displays the number of peer-reviewed CIBMTR publications annually since 2004.

Most Frequent Journals for CIBMTR Publications this Year

Biology of Blood and Marrow Transplantation (25 publications)

Blood Advances (14 publications)

Bone Marrow Transplantation (11 publications)

Haematologica (4 publications)

Presentations

In 2019, CIBMTR study investigators presented **75** abstracts (**40** oral and **35** poster) at national and international conferences. A complete list of presentations is provided in **Appendix E**.

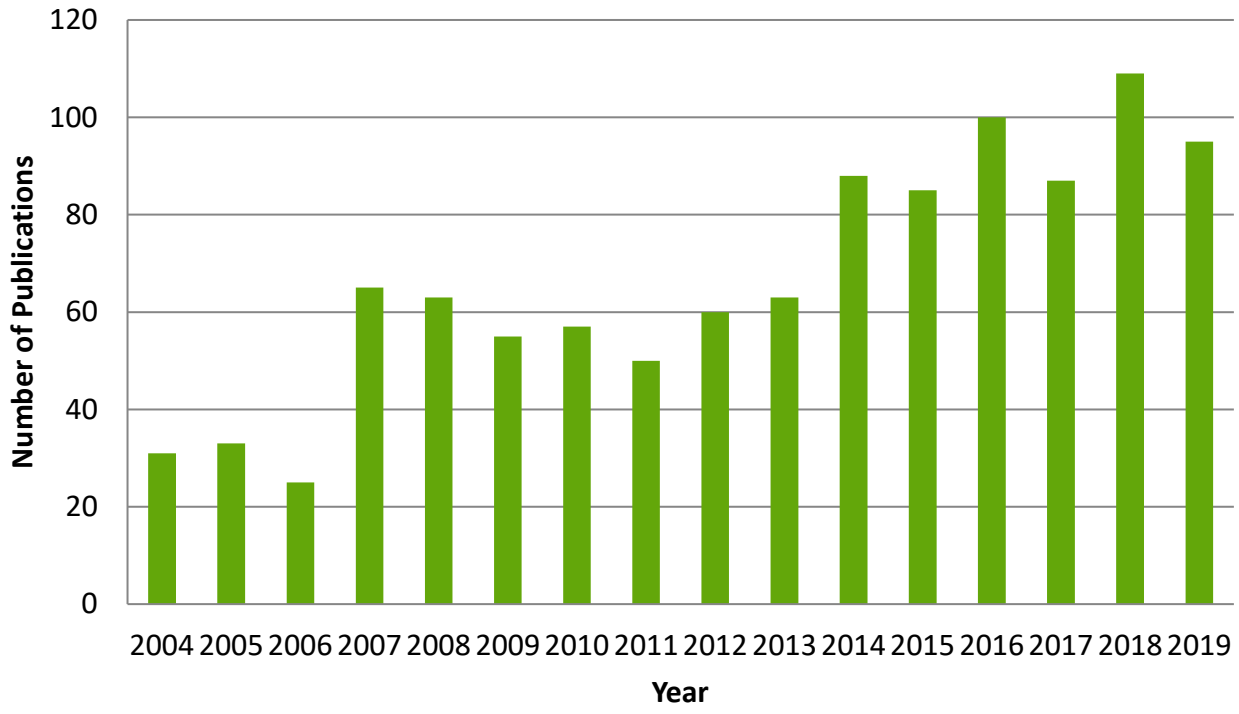
Most Frequent Conferences for CIBMTR Presentations this Year

ASH Annual Meeting
(33 abstracts, 14 oral and 19 poster)

TCT Meetings
(18 abstracts, 9 oral and 9 poster)

European Federation for Immunogenetics
(7 abstracts, 5 oral and 2 poster)

Figure 2.5 CIBMTR Publications by Year



2.1 CLINICAL OUTCOMES RESEARCH PROGRAM

Clinical outcomes research using the CIBMTR Research Database is a core activity of the organization. These studies address a wide range of issues, focusing on questions that are difficult or impossible to address in single-center studies or randomized trials because the diseases studied are uncommon, single centers treat few patients with a given disorder, and not all important questions are amenable to a randomized research design.

2.1.1 Scientific Working Committees

Activities

The **15** Scientific Working Committees oversee most of the CIBMTR's clinical outcomes research with **173** studies in progress (**Table 2.6**). In progress and recently published studies are detailed in the *2019 Working Committee Research Portfolio* linked on the *Working Committee Study Lists* webpage.

The Working Committees reviewed **226** new study proposals before the 2019 TCT Meetings. **102** proposals were presented at the annual meeting, and **39** were approved. The prioritization and selection process ensures the most important issues can be addressed in a timely manner.

Publications

In 2019, Working Committee investigators published **44** peer-reviewed journal articles, **>45%** of CIBMTR publications this year (**Figure 2.7**). A complete list of Clinical Outcomes Research Program publications is provided in **Appendix D1**.

Presentations

In 2019, Working Committee study investigators presented **36** abstracts (**15** oral and **21** poster). A complete list of CIBMTR presentations is provided in **Appendix E**.

Key Working Committee Publications this Year

Kanate AS, Kumar A, Dreger P, et al. **Maintenance therapies for Hodgkin and non-Hodgkin lymphomas after autologous HCT: A consensus project of ASBMT, CIBMTR, and the Lymphoma Working Party of EBMT**. JAMA Oncology. 2019 May 1; 5(5):715-722. Epub 2019 Feb 28.

Parikh SH, Satwani P, Ahn KW, et al. **Survival trends in infants undergoing allogeneic HCT**. JAMA Pediatrics. Epub 2019 Mar 18. PMC6503511.

Ustun C, Le-Rademacher J, Wang H-L, et al. **Allogeneic HCT compared to chemo-therapy consolidation in older AML patients 60-75 years in first complete remission: An alliance (A151509), SWOG, ECOG-ACRIN, and CIBMTR study**. Leukemia. 2019 Nov 1; 33(11):2599-2609. Epub 2019 May 9. PMC6842042.

Eapen M, Brazauskas R, Walters MC, et al. **Effect of donor type and conditioning regimen intensity on allogeneic HCT outcomes in patients with sickle cell disease: A retrospective multicentre, cohort study**. The Lancet Haematology. 2019 Nov 1; 6(11):e585-e596. Epub 2019 Sep 5. PMC6813907.

Atallah E, Logan BR, Chen M, et al. **Comparison of patient age groups in transplantation for myelodysplastic syndrome: The Medicare coverage with evidence development study**. JAMA Oncology. Epub 2019 Dec 12.

Table 2.6 2019 Working Committee Studies

Working Committee	Studies in Progress	Peer-Reviewed Publications	Presentations at International Meetings
Acute Leukemia	**13**	**4**	**5** (2 oral & 3 poster)
Cellular Immunotherapy for Cancer	**7**	**0**	**3** (2 oral & 1 poster)
Chronic Leukemia	**14**	**3**	**2** (1 oral & 1 poster)
Donor Health and Safety	**11**	**2**	**3** (poster)
Graft Sources and Manipulation	**7**	**2**	**1** (poster)
Graft-versus-Host Disease	**9**	**3**	**2** (1 oral & 1 poster)
Health Services and International Studies	**14**	**2**	**1** (oral)
Immunobiology	**34**	**7**	**6** (poster)
Infection and Immune Reconstitution	**6**	**4**	**0**
Late Effects and Quality of Life	**11**	**2**	**2** (oral)
Lymphoma	**10**	**5**	**5** (3 oral & 2 poster)
Non-Malignant Diseases	**14**	**3**	**0**
Pediatric Cancer	**4**	**0**	**0**
Plasma Cell Disorders and Adult Solid Tumors	**10**	**1**	**4** (3 oral & 1 poster)
Regimen-Related Toxicity and Supportive Care	**9**	**6**	**2** (poster)
TOTAL	**173**	**44**	**36** (15 oral & 21 poster)

Funding

Support for the Working Committees is primarily provided by the National Institutes of Health (NIH) grant # U24CA076518 from the National Cancer Institute (NCI); National Heart, Lung, and Blood Institute (NHLBI); and National Institute for Allergy and Infectious Disease (NIAID).

How to Get Involved

Working Committees are collaborative in nature, and all interested individuals are encouraged to participate. Please feel free to attend annual in-person meetings of the Working Committees at the TCT Meetings in February. Additionally, anyone willing to follow the study development and management process (**Appendix F**) is eligible to propose a study to the Working Committees (**Figure 2.8**).

Successful Working Committee Study Proposals are

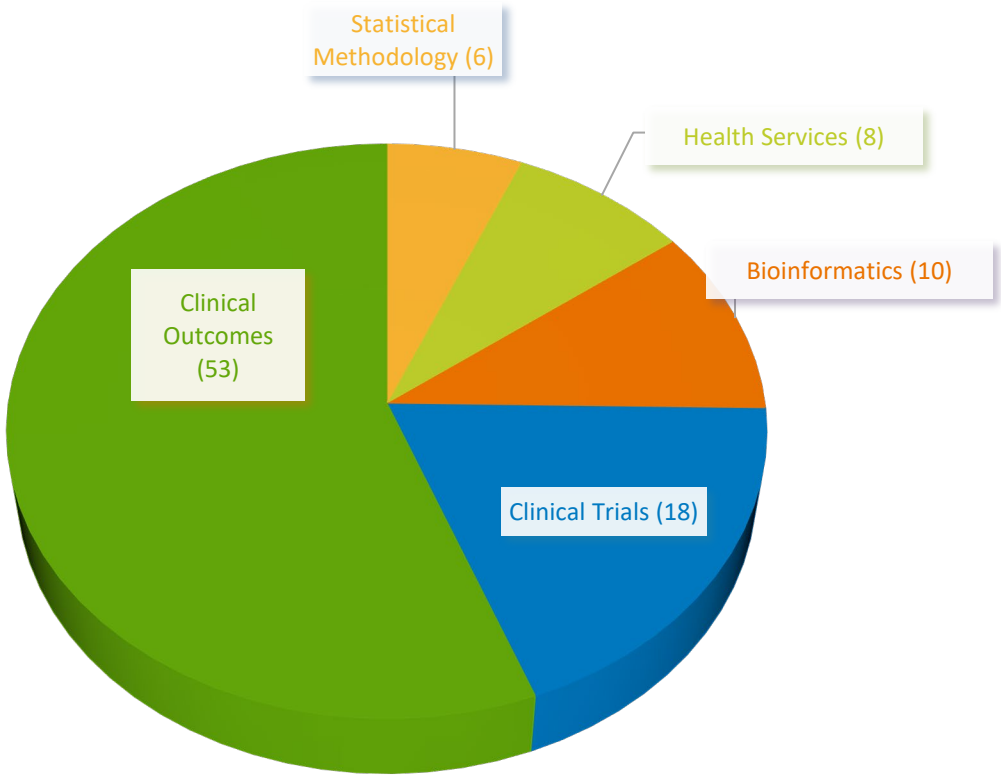
Feasible. Utilize data available in the CIBMTR Research Database.

Unique. Fill a gap not addressed by current studies or publications.

Important. Impact the field by improving cellular therapy procedures or results or increasing access to these therapies.

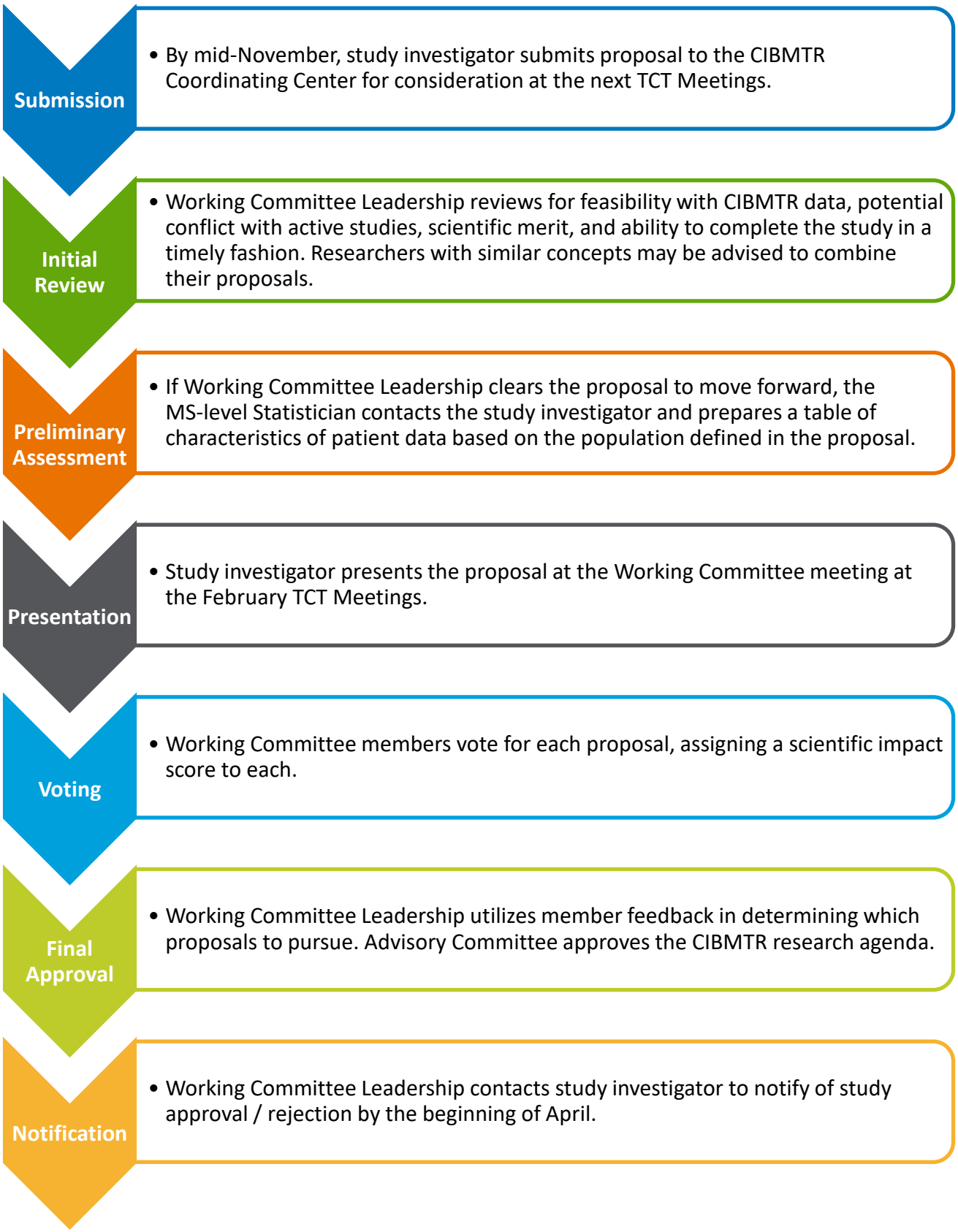
See the *CIBMTR How to Propose a Study* webpage and, specifically, the *Study Proposal Outline* on that webpage for additional guidelines and advice.

Figure 2.7 2019 CIBMTR Publications by Program



Clinical Outcomes includes 44 Working Committee publications and 9 other Clinical Outcomes Research publications. Clinical Trials includes 16 BMT CTN publications and 2 RCI BMT publications.

Figure 2.8 Working Committee Study Proposal Review Process



2.1.2 Stem Cell Therapeutic Outcomes Database (SCTOD)

The CIBMTR administers the SCTOD contract for the Health Resources and Services Administration (HRSA)-sponsored C.W. Bill Young Cell Transplantation Program (CWBYCTP), established by the Stem Cell Therapeutic and Research Act of 2005 and renewed through the Stem Cell Therapeutic and Research Reauthorization Acts of 2010 and 2015.

Activities

For the SCTOD, the CIBMTR tracks and analyzes data for all allogeneic transplants performed in the US and transplants performed globally with products from the US. Each year the CIBMTR publishes hematopoietic cell transplantation (HCT) volumes and performance data by transplant center publicly on the *HRSA CWBYCTP* website.

Center-Specific Volumes and Survival Analysis. As part of the contract to operate the SCTOD, the CIBMTR provides the annual volume of transplants performed at each center, which is posted on the *HRSA CWBYCTP* website. The CIBMTR also performs a center-specific survival analysis evaluating the one-year survival rates among US centers and assesses transplants from both related and unrelated donors. The most recent analysis was completed in September 2019 and contains information on all first allogeneic transplants performed in US centers from January 1, 2015, through December 31, 2017.

Study Summaries for Patients. The CIBMTR published **95** peer-reviewed publications in 2019. In conjunction with NMDP/Be the Match and the Consumer Advocacy Committee, the CIBMTR publishes lay summaries of some of its peer-reviewed publications for patients and their loved ones. This year the CIBMTR published **17** study summaries for patients.

Publicly Available Reports developed by the CIBMTR for the SCTOD

Transplant Outcomes and Data

- ***US Patient Survival Report***
- ***US Transplant Data by Center Report***
- ***US Transplant Data by Disease Report***
- ***Transplant Activity Report and Dataset***

These reports are available on the *HRSA CWBYCTP* website.

Center Outcomes Forums. The CIBMTR has conducted **6** *Center Outcomes Forums* to engage relevant stakeholders in the center-specific outcomes reporting process. The most recent meeting was held in September 2018. Recommendations were generated related to the center-specific analysis modeling, risk adjustment for pediatric non-malignant disease, statistical methodology, and improving collaboration to achieve quality improvement. Many of these recommendations have already been implemented, including evaluation of the impact of new risk adjustment schemes on center performance. The Statistical Methodology Workgroup recommended continued use of logistic regression modeling and will re-evaluate new methods of machine learning on a regular basis. New data elements for pediatric non-malignant disease risk adjustment will be released on forms in January 2020.

Funding

Support for the SCTOD is provided by the HRSA contract # HHSH250201700006C.

How to Get Involved

All US centers performing allogeneic HCTs provide data to the CIBMTR for the SCTOD. These data are used to generate reports, which are distributed to transplant center medical directors and posted on the *HRSA CWBYCTP* website.

2.1.3 Non-Transplant Cellular Therapy Initiatives

Activities

In addition to receiving data on transplant recipients, the CIBMTR received data from **155** centers for **2,908** patients who received other cellular therapies. The CIBMTR receives these data via a suite of CTED forms and continues to work with international registries to review and harmonize data collection globally.

Since 2016, the CIBMTR has collected data for **1,811** patients who received chimeric antigen receptor (CAR) T-cell therapy and **655** patients who received cells for regenerative medicine indications, primarily neurologic disorders. **1,332** patients were treated for non-Hodgkin lymphoma; **651** were treated for neurologic disorders, and **425** were treated for acute lymphoblastic leukemia.

Cellular Therapy Registry Forum. In October 2019, the CIBMTR held a fifth Cellular Therapy Registry Forum (**Section 3.2.2**). Topics included the current status of the Cellular Therapy Registry, CAR T-cell toxicities and risk evaluation and mitigation strategy (REMS) reporting requirements, cellular therapy and real-world experience, center data audits and accreditation, and non-transplant cellular therapy for solid tumors.

Long-Term Follow-Up. The Food and Drug Administration (FDA) requires pharmaceutical companies that commercialize genetically engineered cellular therapies to follow recipients of these therapies for **15** years in order to evaluate their safety and efficacy. The CIBMTR can support this requirement and is currently partnered with several pharmaceutical companies to track these long-term outcome data.

Purpose of the Cellular Immunotherapy Data Resource (CIDR)

Support the IOTN and the biomedical community

Provide an infrastructure to manage data collection and verification for cellular therapies from multiple sources

Facilitate observational cellular therapy research

Cellular Immunotherapy Data Resource. The CIBMTR receives funding from the NIH to serve as the CIDR to collect outcomes of patients receiving non-transplant cellular immunotherapies to support observational studies and inform prospective studies and clinical trials. The IOTN supports the Cancer Moonshot[SM] initiative to accelerate cancer research to make more therapies available to more patients.

In October 2019, the CIBMTR held the first CIDR Stakeholder's Council Meeting. Experts representing academia, centers, professional societies, industry, and payers provided valuable input to shape the activities of the CIDR. This meeting's topics included an updated on CIDR Year 1 achievements, governance, opportunities to interact with the IOTN, data access, uses and intellectual property, and data dissemination and sharing.

Funding

Support for the CIDR is provided by NIH grant # U24CA233032 from the NCI and National Institute on Minority Health and Health Disparities. Support for other non-transplant cellular therapy research initiatives is provided by NIH grant # U24CA076518, HRSA contract # HHSH250201700006C, and Office of Naval Research grant # N00014-17-1-2850.

How to Get Involved

Any center may submit non-transplant cellular therapy data to the CIBMTR. For more information, email *contactus@cibmtr.org*.

2.1.4 CMS Coverage with Evidence Development (CED) Studies

Many patients with specific diseases and / or at certain ages are denied access to HCT therapy in the US due to lack of insurance coverage by the Centers for Medicare and Medicaid Services (CMS).

CMS CED studies allow CMS to provide coverage to patients enrolled on clinical studies that inform policy decisions. The CIBMTR is currently engaged in **5** CMS CED studies (**Table 2.9**). For additional information, visit the *CIBMTR Medicare Clinical Trials* webpage.

Table 2.9 CMS CED Studies

Disease	Patient Population	Enrollment	Dates
Myelodysplastic Syndrome (MDS) (10-CMS-MDS) *NCT# 01166009*	Elderly patients with MDS	**135** centers **4,753** patients **3,099** patients ≥65 years old **1,417** patients 55-64 years old **237** patients <54 years old	Launched in 2010
Sickle Cell Disease (BMT CTN 1503) *NCT# 02766465*	Adolescents and young adults with severe sickle cell disease	**37** centers **112** patients (200 planned)	Launched in October 2016
Myelofibrosis (16-CMS-MF) *NCT# 02934477*	Patients aged ≥55 years with primary myelofibrosis or post-essential thrombocythemia / polycythemia vera	**114** centers **210** patients (650 planned) **41** related (matched 6/6) **146** unrelated (matched 8/8) **23** haploidentical	Launched in December 2016
Multiple Myeloma (17-CMS-MM) *NCT# 03127761*	Elderly patients with Stage II or III multiple myeloma or primary plasma cell leukemia who are eligible to receive allogeneic HCT	**86** centers **10** patients (550 planned)	Launched in July 2017
Sickle Cell Disease (17-CMS-SCD) *NCT# 01166009*	Patients aged 15-50 years with severe sickle cell disease	**73** centers **6** patients (200 planned)	Launched in November 2017

2.1.5 Patient-Reported Outcomes

The CIBMTR collects patient-reported outcomes data using an electronic patient-reported outcomes (ePRO) system. PROMIS® (Patient-Reported Outcomes Measurement Information System) measures form the backbone of the CIBMTR's ePRO system. PROMIS measures are NIH-funded, freely available, and validated in multiple languages. The ePRO system integrates multiple applications: A patient-friendly interface in Qualtrics®; automated tracking and alerting functionality provided via the CIBMTR's customer relationship management system, which communicates directly with FormsNet (**Section 4.2.1**); and computer adaptive testing through the PROMIS application programming interface.

The system is scalable, allowing additional domains, such as financial toxicity and employment outcomes, to be collected to study long-term survivorship issues identified as important by patients and caregivers. Patient-reported outcomes data collected centrally through this system places no additional burden on centers and, with the patient's permission, can be returned to centers.

In 2018, the CIBMTR launched a pilot project to obtain patient-reported outcomes electronically using its new system. The CIBMTR approached patients for the study and obtained consent electronically through the ePRO system. Enrollment for this study closed in November 2019, with **92** patients from **6** centers. The primary objective was to compare quality of life in HCT recipients age 55-64 with recipients age 65 and older. Secondary objectives tested the feasibility of ePRO collection, using the new CIBMTR ePRO system, in recipients age 55 and older. In 2019, the CIBMTR expanded use of the ePRO system to three BMT CTN trials. Analyses are in progress.

Patient-Reported Outcomes PROMIS Domains

- **Physical function**
- **Fatigue**
- **Sleep disturbance**
- **Pain interference**
- **Anxiety**
- **Depression**
- **Cognitive function**
- **Ability to participate in social roles and activities**

This year the CIBMTR developed and obtained Institutional Review Board (IRB) approval for a protocol for the routine collection of patient-reported outcomes data. This protocol allows the CIBMTR to centrally collect patient-reported outcomes data from patients whose clinical data are being collected for the CIBMTR Research Database. The CIBMTR Survey Research Group (**Section 2.3.2**) will consent patients to this protocol and collect a core set of patient-reported outcomes data pre-transplant as well as 100 days, 180 days, and 1-year post-transplant and annually thereafter. Patient-reported outcomes data collected through this protocol will be added to the Integrated Data Warehouse (**Section 4.2.3**) and be available alongside clinical data for future research or to share back to centers. Looking ahead to 2020, the CIBMTR is building the core patient-reported outcomes instruments and data collection structure in the ePRO system and piloting the routine patient-reported outcomes data collection protocol with adult patients at a limited number of US sites.

2.1.6 International Initiatives

This year the CIBMTR continued to strengthen its international collaborations with clinical centers and international registries.

European Society for Blood and Marrow Transplantation. The CIBMTR and EBMT collaborate to collect and exchange data from cellular therapy teams, share data for research studies, and define and revise common data elements and collection instruments. Their combined registry resources have produced many high-quality studies with significant impact that are unlikely to have been possible in single center or single registry studies.

The recent European Union General Data Protection Requirement (GDPR) legislation has made EBMT data sharing challenging, and data shared via AGNIS (A Growable Network Information System) continues to be on hold. However, the CIBMTR remains committed to this collaboration and is working with its EBMT colleagues to determine ways to continue to do joint scientific work.

Worldwide Network for Blood and Marrow Transplantation (WBMT). The CIBMTR is a founding member of the WBMT and, through WBMT, collaborates with other international organizations in a wide range of areas but with a focus on harmonizing data collection forms and specific data elements to share transplant data worldwide. These data are used in reports of international transplant utilization and to support meaningful health services research. The WBMT is utilizing these same processes to define standards for data collection for emerging indications of cellular therapy.

During the 2019 TCT Meetings, the CIBMTR awarded the WBMT the CIBMTR Distinguished Service Award for the WBMT's promotion of cellular therapy research and clinical care in developing countries, wide dissemination of research results, and collaboration with organizations to increase data exchange and research collaboration worldwide.

In September 2019, a CIBMTR Scientific Director supported the WBMT Workshop and Scientific Symposium in Asunción, Paraguay, presenting expert advice regarding the establishment and optimization of an HCT program and an outcomes registry.

World Marrow Donor Association (WMDA). Since its inception, the CIBMTR has worked closely with the WMDA to improve global accessibility to unrelated donors for recipients in need of HCT, standardize global identification systems to match donors and recipients for data reporting, and conduct important donor outcomes research.

Canadian BMT Group and Japan Society for HCT. The CIBMTR partners with the Canadian BMT Group and Japan Society for HCT to collect data from centers in Canada and Japan and return those data to the regional groups to support their own national outcomes registries. Using this approach, the regional groups leverage the CIBMTR's data collection infrastructure and provide the CIBMTR with valuable international data.

The CIBMTR worked with the Japanese Data Center for HCT this year to translate FormsNet and CTED forms into Japanese, allowing Japanese centers to submit non-transplant cellular therapy data, using the CIBMTR infrastructure, to the Japanese Data Center for reporting to the Japanese regulatory agency. Data submission will begin in February 2020.

Data Management. The CIBMTR encourages physicians and data managers of international centers to visit the Milwaukee and Minneapolis campuses to receive focused training on data use, study design, and statistical methods. The CIBMTR also conducts on-site trainings at international sites. In July 2019, CIBMTR staff members and Brazilian data managers provided training at the Brazilian Data Management Conference held at the Brazilian Blood and Marrow Transplant Annual Meeting in Brasilia, Brazil.

2.2 IMMUNOBIOLOGY RESEARCH PROGRAM

The CIBMTR maintains a Research Repository of paired tissue samples from donors and recipients, both unrelated and related. The Immunobiology Research Program manages the Research Repository inventory and immunogenetic testing programs that add critical HLA and killer-cell immunoglobulin-like receptors (KIR) data for use in CIBMTR clinical outcomes studies.

The CIBMTR leverages the NMDP/Be The Match's investment in the Unrelated Donor Research Repository with the NIH's investment in the CIBMTR Research Database. Linking outcomes data to immunologic data available in the Research Repository supports studies that include genetic and immunobiologic data and clinical phenotype data.

The Related Donor Research Repository, supported by HRSA, is a unique opportunity to enhance immunobiologic research. Related donor and recipient samples are better matched than unrelated recipients for HLA, a measure of immunological compatibility, thus reducing the confounding effects of HLA disparity in correlative research.

The combination of the Unrelated and Related Donor Research Repositories facilitates an organized approach to studying transplant biology across the spectrum of allogeneic HCT.

Research Repository

2,801,816 aliquots

17,732 cell lines

74,766 samples from unrelated donors and **10,555** from related donors

68,668 samples from unrelated recipients and **10,978** from related recipients

12,688 samples from unrelated cord blood units

Samples from complete pairs:

- **42,974** from complete unrelated adult donor-recipient pairs
- **9,289** from complete related donor-recipient pairs
- **4,819** from unrelated cord-recipient pairs

Activities

In 2019, **190** centers (**148** transplant centers, **25** donor centers, and **17** cord blood banks) provided samples to the Research Repository. The Immunobiology Research Program enhanced the Research Repository inventory and Immunogenetic Database this year by completing high resolution HLA and KIR typing on **283** related and **475** unrelated HCT donor / cord and recipient pairs, bringing the total to **>35,000** unrelated donor / cord and recipient pairs that have been retrospectively high resolution typed for HLA-A, -B, -C, -DRB1 and -DQB1; **>90%** include -DPB1, and **>18,500** include KIR.

The Immunobiology Research Program distributed **12,467** research samples in support of Working Committee studies this year.

Publications

The Immunobiology Research Program supports investigators' publications by providing research samples. **15** manuscripts published this year by Working Committee and BMT CTN investigators utilized samples from the Research Repository.

Key Publications supported by the Immunobiology Research Program this Year

Tsamadou C, Fürst D, Wang T, et al. **Donor HLA-E status associates with disease free survival and transplant related mortality after non in vivo T-cell depleted HSCT for acute leukemia.** Biology of Blood and Marrow Transplantation. 2019 Dec 1; 25(12):2357-2365. Epub 2019 Aug 16.

Wang Y, McReynolds LJ, Dagnall C, et al. **Pre-transplant short telomeres are associated with high mortality risk after unrelated donor haematopoietic cell transplant for severe aplastic anaemia.** British Journal of Haematology. Epub 2019 Aug 19.

Karaesmen E, Hahn T, Dile AJ, et al. **Multiple functional variants in the IL1RL1 region are pretransplant markers for risk of GVHD and infection deaths.** Blood Advances. 2019 Aug 27; 3(16):2512-2524. Epub 2019 Aug 27. PMC6712530.

Petersdorf EW, Carrington M, O'hUigin C, et al. **Role of HLA-B exon 1 in GVHD after unrelated HCT: A retrospective cohort study.** Haematologica. 2020 Jan 1. 7(1):e50-e60. Epub 2019 Oct 25.

Funding

Support for the Immunobiology Research Program is primarily provided by the Office of Naval Research grant # N00018-1-1-2888, NIH grant # U24CA076518, and HRSA contract # HHSH250201700006C.

The Immunobiology Research Program offers limited research funds supporting immunobiology research studies. The grants are intended to subsidize lab tests, sample collection, or costs associated with the use of research samples. These grants are available to approved CIBMTR studies that support organizational research priorities.

How to Get Involved

All interested parties may attend the annual in-person meeting of the Immunobiology Working Committee at the TCT Meetings in February. Additionally, the Immunobiology Working Committee encourages highly translational, hypothesis-driven proposals through the Working Committee Study Proposal Review Process (**Figure 2.9**).

2.3 CLINICAL TRIALS SUPPORT PROGRAM

The CIBMTR manages a wide array of studies, including multi-center trials, surveys, and correlative studies. Access to the CIBMTR Research Database and use of data from observational studies are important resources to support decisions regarding design of prospective clinical trials.

CIBMTR Coordinating Center Support of Clinical Trials

Study Planning. Oversee study development, including patient population identification, site selection, training, and financial administration.

Data Collection. Collect new data and collaborate to share existing data across systems and centers.

Site Management. Oversee site start-up, enrollment, and protocol compliance.

Study Monitoring. Oversee on-site, centralized, and remote monitoring to ensure data accuracy and mitigate risks.

Statistical Consultation. Provide expert design and review of protocols.

Real-Time Accrual Assessment. Review characteristics of enrolled and non-enrolled patients to address potential accrual barriers.

Trial Interpretation. Evaluate results of clinical trials, including through the provision of matched controls.

Long-Term Follow-Up Data. Capture follow-up data for long-term or secondary analyses, resulting in considerable cost-savings.

2.3.1 Blood and Marrow Transplant Clinical Trials Network

The BMT CTN, sponsored by NHLBI and NCI, is the US network charged with developing and conducting multicenter Phase II and III clinical trials focused on cellular therapy. The CIBMTR is the lead institution for the BMT CTN Data and Coordinating Center, which it runs in collaboration with NMDP/Be The Match and the Emmes Company, a contract research organization based in Rockville, MD.

Activities

The BMT CTN has launched **53** trials (including **7** this year) and completed accrual for **40** of these trials. The Network has accrued **>11,300** patients to its trials from **>100** centers, including **>600** this year. The Network has established a Research Sample Repository that currently houses **>416,000** biospecimens. Additionally, the BMT CTN has conducted **56** ancillary and correlative studies, with another **49** in progress.

More detail regarding Network activities and protocols is provided in the annual *Progress Report* on the BMT CTN website. A list of Network trials open for enrollment is provided in **Appendix G1**.

Publications

In 2019, BMT CTN study investigators published **16** peer-reviewed journal articles. These bring the total number of Network publications to **113**, including **29** primary results papers. A complete list of 2019 BMT CTN publications is provided in **Appendix D2**.

Key BMT CTN Publications this Year

Stadtmauer EA, Pasquini MC, Blackwell B, et al. **Autologous transplantation, consolidation, and maintenance therapy in multiple myeloma: Results of the BMT CTN 0702 trial.** Journal of Clinical Oncology. 2019 Mar 1; 37(7):589-597. Epub 2019 Jan 17. PMC6553842.

Bolaños-Meade J, Reshef R, Fraser R. **Three prophylaxis regimens (tacrolimus, mycophenolate mofetil, and cyclophosphamide; tacrolimus, methotrexate, and bortezomib; or tacrolimus, methotrexate, and maraviroc) versus tacrolimus and methotrexate for prevention of GVHD with HCT with reduced-intensity conditioning: A randomised Phase 2 trial with a non-randomised contemporaneous control group (BMT CTN 1203)**. Lancet Haematology. 2019 Mar 1; 6(3):e132-e143. PMC6503965.

Ambinder RF, Wu J, Logan B, et al. **Allogeneic hematopoietic cell transplant for HIV patients with hematologic malignancies: The BMT CTN-0903/AMC-080 trial.** Biology of Blood and Marrow Transplantation. 2019 Nov 1. 25(11):2160-2166. Epub 2019 Jul 4. PMC6907401.

Pidala J, Hamadani M, Dawson P, et al. **Randomized multicenter trial of sirolimus vs. prednisone as initial therapy for standard risk acute GVHD: BMT CTN 1501.** Blood. Epub 2019 Nov 18.

Presentations

BMT CTN study investigators presented **12** abstracts (**6** oral and **6** poster) at national and international conferences in 2019. These bring the total number of Network presentations to **106**. A complete list of 2019 CIBMTR presentations is provided in **Appendix E**.

Funding

Support for the BMT CTN Data and Coordinating Center is provided by the NIH grant # U24HL138660 from the NHLBI and NCI. Additional support for individual studies is provided by both private and federal contributors.

How to Get Involved

The Network is committed to widespread participation in its trials. If you would like to serve as an Affiliate Center, visit the *BMT CTN* website for more information. Additionally, you may act as a Center Principal Investigator or champion a trial to increase patient accrual at your Center, serve on a Protocol Team or an Endpoint Review Committee, or act as a Medical Monitor. You may also propose an ancillary study to use *data*, *biospecimens*, and / or analyses outside the specific objectives of a primary BMT CTN study.

2.3.2 Resource for Clinical Investigations in Blood and Marrow Transplantation

The RCI BMT provides cellular therapy researchers with infrastructure and expertise in clinical trial conduct and analysis. The program not only helps investigators generate data allowing novel and innovative ideas to move into the larger Phase II or Phase III setting but also supports Phase II/III trials and large survey and cohort studies.

Activities

The RCI BMT has launched **24** studies, including **6** this year. In 2019, the RCI BMT accrued **>2,600** participants, bringing the total number of accrued participants to approximately **39,000**, of which **>21,000** were enrolled in a cohort study examining long-term outcomes of unrelated donors.

The RCI BMT manages **2** FDA investigational new drug (IND) protocols for NMDP/Be The Match. *PBSC Procurement* accrued **>2,000** subjects this year, and *Cord Blood Access* accrued approximately **300**. These protocols allow US centers to access peripheral blood and unlicensed cord blood for transplantation.

The RCI BMT also supported **9** active studies, including **3** BMT CTN protocols (**Section 2.3.1**), and participated in the development of **5** upcoming studies. A list of active RCI BMT studies is provided in **Appendix G2**.

Survey Research Group

The Survey Research Group is a team within the RCI BMT created to assist researchers in developing and conducting research involving questionnaires, direct subject interviews, and patient-reported outcomes. The group is responsible for collecting high quality, scientifically valid data from donors, patients, and their families.

The Survey Research Group launched an ePRO system (**Section 2.1.5**) in 2018 to support clinical trials, long-term follow-up, and other research studies with patients and donors. In 2019, the CIBMTR developed and obtained IRB approval for a new protocol to allow the Survey Research Group to centrally collect patient-reported outcomes data from patients whose clinical data are being collected by the CIBMTR. The Survey Research Group also supported **7** active studies and participated in the development of **3** upcoming studies.

Publications

In 2019, RCI BMT study investigators published **2** peer-reviewed journal articles. A list of RCI BMT publications is provided in **Appendix D3**.

RCI BMT Publications this Year

Chen Y-B, Le-Rademacher J, Brazauskas R, et al. **Plerixafor alone for the mobilization and transplantation of HLA-matched sibling donor hematopoietic stem cells.** Blood Advances. 2019 Mar 26; 3(6):875-883. Epub 2019 Mar 19. PMC6436017.

Ballen K, Logan BR, Chitphakdithai P, et al. **Unlicensed umbilical cord blood units provide a safe and effective graft source for a diverse population: A study of 2456 umbilical cord blood recipients**. Biology of Blood and Marrow Transplantation. Epub 2019 Nov 19.

Presentation

RCI BMT study investigators presented **1** poster abstract at an international conference in 2019. A complete list of 2019 CIBMTR presentations is provided in **Appendix E**.

Funding

Support for RCI BMT studies is provided by NMDP/Be The Match, corporate and private sponsors of specific studies, and grants from federal and non-profit agencies.

The RCI BMT team can work with study investigators to seek funding from a variety of sources, including government agencies, foundations, pharmaceutical companies, and private corporations.

How to Get Involved

Study investigators may partner with the RCI BMT to facilitate clinical trials, including assistance with funding proposals; protocol development and approvals; management of study conduct; data auditing, management, and analysis; and financial administration. Study investigators may also contract for specific services as needed, such as support with surveys, site selection and management, sample management, and more. For additional information, visit the *RCI BMT* webpage.

2.4 HEALTH SERVICES RESEARCH PROGRAM

Health services research is the multi-disciplinary field of scientific investigation that studies how social factors, financial systems, organizational structures and processes, technology, and behavior affect treatment outcomes, quality, and cost.

The Health Services Research Program collaborates with stakeholders, including patients, caregivers, researchers, and clinicians, to conduct and disseminate research that contributes knowledge to the cellular therapy field and informs policy, clinical practice, and survivorship care.

Activities

The Health Services Research Program has **13** studies in progress in **3** research portfolios.

Health Services Research Portfolios

Access to HCT / value and health economics / health care disparities

Survivorship / late effects / patient-reported outcomes

Treatment decision-making

Access to HCT / value and health economics / health care disparities. Health services research investigators study value, quality, and access to care, particularly for patients from disadvantaged backgrounds and racial and ethnic minority populations. Studies completed this year included analysis of cost dynamics, reimbursement, and out-of-pocket costs for HCT in patients with diseases such as AML and multiple myeloma. Investigators are finalizing a study analyzing state Medicaid coverage for patients with sickle cell disease and will present results next year.

Survivorship / late effects / patient-reported outcomes. Quality of life is a key outcome, and patient-reported outcomes provide an essential perspective, particularly for late effects of treatment. Health services research investigators are currently engaged in two survivorship / late effects studies. One will determine the efficacy of an online, self-managed stepped-care program and survivorship care plan intervention in adults. The other will describe pediatric transplant program guidelines for patients returning to school.

Treatment decision-making. Health services research is vital to treatment decision-making. This year investigators completed three studies focused on information needs of patients aged 65 and older, defining urgent time to alloHCT, and referral barriers to transplantation. Ongoing studies focus on patients' perspectives on palliative care, practice preferences regarding candidacy of older patients for HCT, and transplant center organizational factors associated with survival outcomes.

Publications

In 2019, Health Services Research Program investigators published **8** peer-reviewed manuscripts. A list of program publications is provided in **Appendix D4**.

Presentations

In 2019, program investigators presented **4** abstracts (**2** oral and **2** poster) at national and international conferences. A complete list of CIBMTR presentations is provided in **Appendix E**.

Key Health Services Research Publications this Year

Preussler JM, Mau L-W, Majhail NS, et al. **Caregiver availability and patient access to hematopoietic cell transplantation: Social worker perspectives inform practice.** Supportive Care in Cancer. 2019 Nov 1; 27(11):4253-4264. Epub 2019 Mar 9.

Pidala J, Mupfudze TG, Payton T, et al. **Urgent time to allogeneic HCT: A national survey of transplant physicians and unrelated donor search coordinators facilitated by the Histocompatibility Advisory Group to the National Marrow Donor Program.** Biology of Blood and Marrow Transplantation. 2019 Dec 1;25(12):2501-2506. Epub 2019 Aug 13.

Mau L-W, Meyer C, Burns LJ, et al. **Reimbursement, utilization, and one-year survival post-allogeneic HCT for Medicare beneficiaries with AML.** JNCI Cancer Spectrum. 2019 Dec 1;3(4): pkz048. Epub 2019 Aug 28. PMC6845850.

Funding

Health Services Research Program studies are funded via a variety of mechanisms. *Individualized care plans for HCT survivors* was supported by the PCORI award # CD-12-11-4062. *A payer-partnered approach to community-based referral for HCT* was supported by the grant # 11762021 from the National Comprehensive Care Network / Pfizer. *INSPIRE* is funded by the NIH grant # R01CA215134-01 from the NCI. *The Palliative Care Study* receives funding from Caring For A Cure. Additional support for the Health Services Research Program is provided by NMDP/Be The Match.

How to Get Involved

For more information about the Health Services Research Program, contact *hsr@nmdp.org*.

2.5 BIOINFORMATICS RESEARCH PROGRAM

The Bioinformatics Research Program specializes in developing and utilizing software tools and analytical methods to facilitate data exchange, interpret information, understand patterns, and predict factors to save and improve lives. At the intersection of science and technology, this team pursues high-impact and innovative research and produces strategic applications to bridge the transition from research to operations. Bioinformatics research moves in the direction of computational biomedicine with activities in three main areas: Genomics / omics and high-throughput bioanalytics, machine learning and clinical predictions, and cellular therapy matching and donor registry modeling.

Activities

Current Bioinformatics Research Goals

Increase rate of patient survival after transplantation that is free of adverse events through delivery of improved therapy selection tools

Develop flexible algorithms to accommodate missing data, and calculate best donor matches, especially for underserved patient populations

Predict event-free survival outcomes for patients using new and existing models and indexes

Identify novel genomic and epigenomic candidate factors for improving event-free survival outcomes in patients

Model HLA data collections and projects worldwide to improve donor recruitment and biobanking practices and target the unmet need from patients

Key Bioinformatics Publications this Year

Maiers M, Halagan M, Gragert L, et al. **GRIMM: GRaph IMputation and matching for HLA genotypes.** Bioinformatics. 2019 Sep 15; 35(18):3520-3523. Epub 2019 Jan 28.

Bishara A, Halagan M, Brautbar C, et al. **High resolution HLA allele and haplotype frequencies for Arab donors in the Hadassah bone marrow donor registry.** Human Immunology. 2019 Oct 1; 80(10):823-827. Epub 2019 May 21.

Publications

In 2019, Bioinformatics Research Program investigators published **10** peer-reviewed manuscripts. A complete list of program publications is provided in **Appendix D5**.

Presentations

Bioinformatics study investigators presented **14** abstracts (**9** oral and **5** poster) at national and international conferences in 2019. A complete list of CIBMTR presentations is provided in **Appendix E**.

Funding

Support for the Bioinformatics Research Program is primarily provided by the grant # N00014-18-1-2850 from the Office of Naval Research. *Integrated Exchange and Storage of Current and Future-Generation Immunogenomic Data* is supported by the R01 grant # AI128775 from NIAID.

How to Get Involved

For more information about the Bioinformatics Research Program, contact Yung-Tsi Bolon, Senior Manager of Bioinformatics Research, at *ybolon@nmdp.org*.

2.6 STATISTICAL METHODOLOGY RESEARCH PROGRAM

The CIBMTR has enjoyed a positive, collaborative association with the Division of Biostatistics in the MCW Institute for Health and Equity since 1980, an association that is a distinctive asset and crucial to the success of CIBMTR research. Biostatisticians ensure the statistical integrity of CIBMTR scientific activities, contribute to results in articles on cellular therapy-related statistical issues for clinical audiences, and support Working Committee study investigators in developing scientific study protocols using CIBMTR data. CIBMTR biostatisticians have pioneered novel methodologic approaches to analyzing cellular therapy data.

Activities

Transplantation is a complex process with multiple competing risks and dramatic changes in the risks of specific events over time. The CIBMTR has developed and evaluated the statistical models used in cellular therapy research and helped guide the research community in appropriate application and interpretation of these sophisticated models.

Statistical Methodology Research Goals

Develop new statistical models

Compare new statistical models with existing solutions using the CIBMTR Research Database

Publications

In 2019, PhD-level biostatisticians in the CIBMTR published 6 peer-reviewed statistical methodology manuscripts. A list of program publications is provided in **Appendix D6**.

Key Statistical Methodology Publications this Year

Sparapani R, Logan BR, McCulloch RE, et al. **Nonparametric competing risks analysis using Bayesian Additive Regression Trees**. Statistical Methods in Medical Research. Epub 2019 Jan 7.

Hu Z-H, Gale RP, Zhang M-J. **Direct adjusted survival and cumulative incidence curves for observational studies**. Bone Marrow Transplantation. Epub 2019 May 17.

Presentations

Statistical Methodology Research Program investigators presented 8 oral abstracts at national and international conferences in 2019. A complete list of CIBMTR presentations is provided in **Appendix E**.

Funding

Support for the Statistical Methodology Research Program is primarily provided by the NIH grant # U24CA076518 from the NCI, NHLBI, and NIAID and the HRSA contract # HHSH250201700006C.

How to Get Involved

During the TCT Meetings in February, PhD-level biostatisticians plan and present educational sessions related to statistical design and analysis, and they provide 1:1 statistical consultation to researchers writing proposals or developing protocols for CIBMTR studies. Any interested individual may participate in these sessions. Additionally, the *MCW Division of Biostatistics* presents a *seminar series* throughout the year in Milwaukee.

2.7 CORPORATE PROGRAM

The CIBMTR provides critical support to industry, an important partner in achieving our mission. Through these partnerships, the CIBMTR provides statistical support, scientific leadership and expertise, a network of participating centers, and observational datasets of clinical information. The CIBMTR leverages its expertise through Corporate Memberships and cellular therapy related research. Requests are considered based on their alignment with the CIBMTR's mission.

Corporate Membership. The CIBMTR Corporate Membership Program provides a variety of resources to corporations needing access to the most current data. These materials are useful for marketing managers, medical directors, research directors, product managers, case managers, and transplant coordinators. The CIBMTR offers **5** levels of Corporate Membership, each described on the *CIBMTR Corporate Membership Program* webpage.

Corporate Membership Benefits

- **CIBMTR Report on Survival Statistics for BMT**
- **Center Volumes Dataset**
- **US Allogeneic HCT Activity Report**
- **Reduced registration rates at CIBMTR meetings and educational forums, including the TCT Meetings**
- **Access to CIBMTR data and resources**

Corporate Studies and Projects. Studies may be one-time requests or long-term projects.

CIBMTR Services Portfolio

- **Descriptive reports and data analyses**
- **De-identified datasets**
- **Protocol development**
- **Cellular and gene therapy long-term follow-up**
- **Retrospective and prospective studies**
- **Consultation services**
- **Supplemental data collection**

Activities

In 2019, the CIBMTR engaged with **19** corporate partners and oversaw a portfolio of **59** active projects. Currently, **19** organizations participate in the CIBMTR Corporate Membership Program.

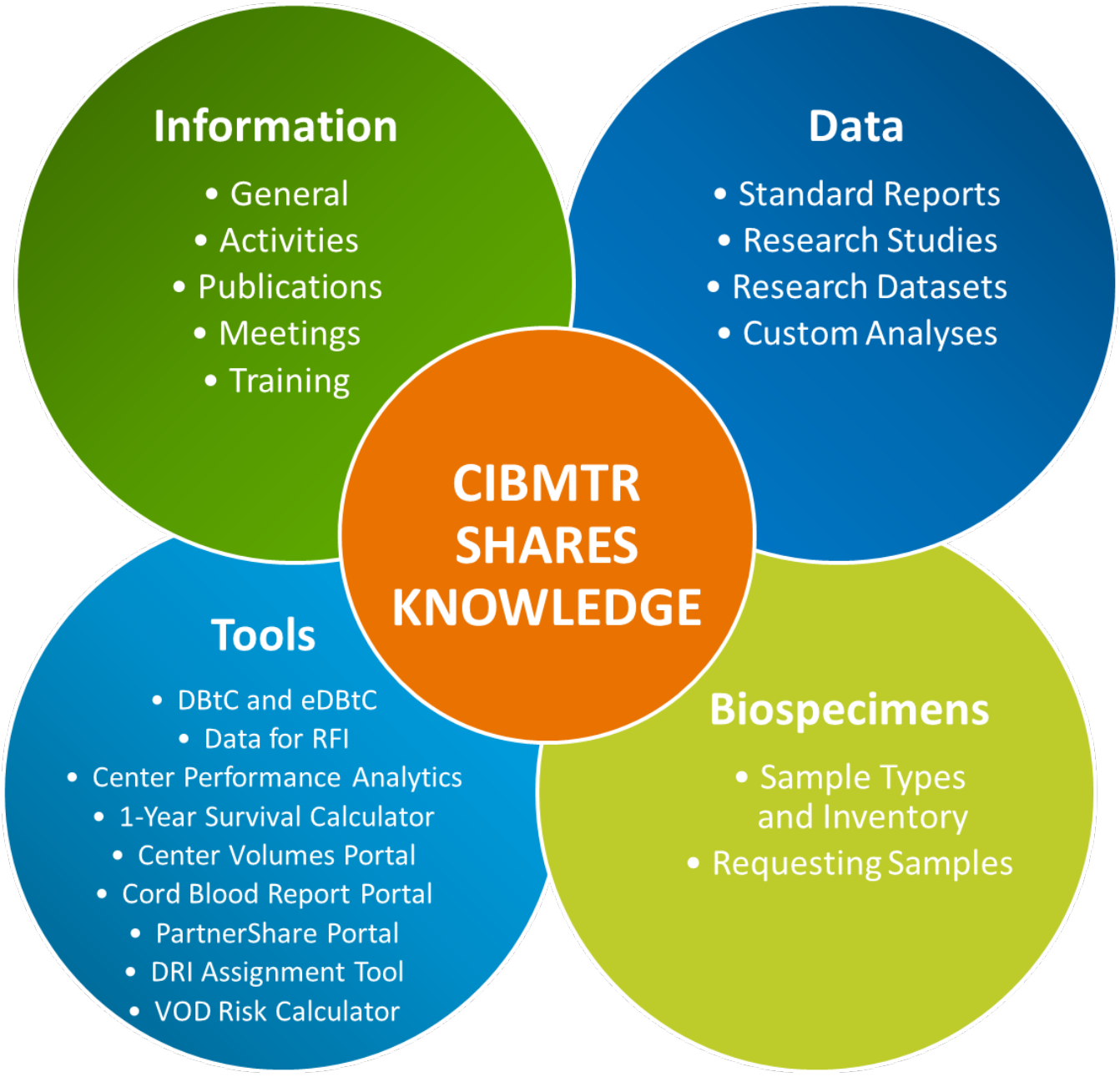
How to Get Involved

If you would like to learn more about the CIBMTR Corporate Program, visit the *CIBMTR Corporate Membership Program* webpage or contact Guillermo Vazquez-Toro, PhD, Director of the Corporate Office, at gvazqueztoro@mcw.edu or 414.805.0675. If you are a Corporate Member requesting analyses, please complete the *Corporate Member Information Request Form* on the CIBMTR website.

3.0 HOW WE SHARE KNOWLEDGE

The CIBMTR is committed to sharing the data we collect as well as the information and knowledge produced from our data and our extensive collaborations with investigators in the cellular therapy field.

Figure 3.1 Knowledge CIBMTR Shares



3.1 WEBSITES AND PORTALS

The CIBMTR Internet presence provides the scientific community and the public with access to cellular therapy information. Current websites include general information about cellular therapy and CIBMTR activities; training and support; a shared communications and collaborative environment for member centers; and secure web access to CIBMTR data for committees, centers, scientific investigators, and CIBMTR staff members.

3.1.1 CIBMTR Public Website

The CIBMTR public website (***cibmtr.org***) is unrestricted and provides information about the CIBMTR and its research. It supports the Working Committees and BMT CTN with information regarding proposal submission, access to a listing and summaries of all studies in process, and access to a summary of all CIBMTR publications. The website facilitates data and information requests, and it provides access to all current and past data collections forms, training manuals, and videos as well as other materials for both investigators and data professionals. The website information is, in part, supported by DISCO (Data and Information for Statistical Center Operations), an application which maintains data on **>1,100** studies, **>1,400** publications, and **>3,200** authors and their institutions at time of publication. In 2019, the CIBMTR public website had **581,448** unique page views.

About CIBMTR

Administrative and Progress Reports
(**2,036** unique page views in 2019)
Provides access to the CIBMTR's Facts and Figures document, Annual Progress Report, and Manual of Operations.

Studies

Working Committee Studies Lists
(**5,492** unique page views in 2019)
A summary of the planned, in-progress, and recently published clinical outcomes studies for each Working Committee.

Meetings

Meeting Materials
(**19,930** unique page views in 2019)
Provides access to agendas, handouts, and educational materials from specific meetings at the TCT Meetings, such as Working Committee Meetings and Clinical Research Professionals / Data Management Conferences, as well as other major events, such as Cellular Therapy and Center Outcomes Forums.

Reference Center

Summary Slides
(**14,573** unique page views in 2019)
Includes charts and figures summarizing current uses and outcomes of allogeneic and autologous HCT.

Web-based US Transplant Reports
(**16,701** unique page views in 2019)
Directs users to the *Be The Match US Center Listing Report* and customizable reports of patient survival and transplant available through the *HRSA CWBYCTP* website.

Publication List
(**7,546** unique page views in 2019)
Searchable descriptive list of **>1,400** publications resulting from the use of CIBMTR data and statistical resources.

Newsletters

(**5,206** unique page views in 2019)

Published **4** times per year. Articles feature updates on Working Committees, the SCTOD, data management and collection, and noteworthy events in the cellular therapy community.

Patient Resources

(**5,861** unique page views in 2019)

Includes *lay summaries of CIBMTR research articles* as well as *post-transplant care recommendations for adult and pediatric autologous and allogeneic HCT recipients* to help patients and clinicians understand and plan for the specialized care of transplant recipients. The CIBMTR published **17** study summaries for patients this year.

Statistical Resources

(**10,508** unique page views in 2019)

Provides access to resources offered through the unique partnership between the CIBMTR and MCW Division of Biostatistics, including biostatistical publications, a series of statistical lectures targeted at basic and clinical investigators, and research tools, such as the *DRI Assignment Tool* and *VOD Risk Calculator*.

Data Management

Data Management Manual

(**28,050** unique page views in 2019)

A comprehensive reference document for completing CIBMTR data collection forms. The manual also details reporting requirements, describes protocols and the consent process, and includes downloadable report forms.

Data Back to Centers Applications

(**740** unique page views in 2019)

Links to the DBtC and eDBtC applications that provide CIBMTR member centers with self-service access to their most commonly used CRF- and TED-level data, including descriptive statistics and outcomes.

Data Collection Forms

(**47,438** unique page views in 2019)

Provides access to current and retired versions of forms used by the CIBMTR to collect standard data elements for all cellular therapy recipients.

Training and Reference

(**48,717** unique page views in 2019)

Provides access to a wide variety of CIBMTR data management training and reference materials.

3.1.2 CIBMTR Collaborate Site

The CIBMTR Collaborate site (***collaborate.cibmtr.org***) uses the SharePoint Enterprise Collaboration platform to promote cooperative work among CIBMTR staff members and provides a communication platform for specific studies and initiatives. This site is secured by username and password, and user-specific security credentials are assigned centrally.

The CIBMTR uses the site for storing and sharing protocol and consent documents, donor / recipient tracking tools, confidential committee information, data, manuscript drafts, and other relevant information. Several Working Committees use the Collaborate site on a regular basis, and all Working Committees are welcome to use the site for sharing information.

3.1.3 CIBMTR Portal Site

The CIBMTR Portal site (***portal.cibmtr.org***) delivers applications and data to CIBMTR centers and other partners. In 2019, external visitors viewed **37,319** Portal pages.

9 applications are currently hosted on this site:

Data Back to Centers

DBtC - Data Back to Centers

The DBtC application provides authorized users the ability to download CIBMTR TED-level data variables for their centers. The data have been validated and processed in the CIBMTR Research Database and are reviewed and refreshed quarterly. Legacy International Bone Marrow Transplant Registry (IBMTR) data from as far back as 1964 and some legacy NMDP/Be The Match data from as far back as 1987 are available. In 2019, authorized users from **151** different centers viewed **2,518** DBtC pages.

eDBtC - Enhanced Data Back to Centers

The CIBMTR deployed eDBtC in Qlikview in 2016 to provide centers with self-service access to their most commonly used CRF- and TED-level data, including descriptive statistics and outcomes. eDBtC enables centers to view and filter outcomes, including a five-year overall survival curve as well as acute graft-versus-host disease (GVHD), chronic GVHD, and other outcomes, for the center's population.

An ad-hoc query tab allows users to create and implement their own custom query of these data. eDBtC data are extracted from the CIBMTR Research Database and validated, reviewed, and refreshed monthly. Users may also export filtered data in Excel file formats.

This year the CIBMTR extended eDBtC to include non-transplant cellular therapy data collected by centers. The CIBMTR also consolidated features of its legacy DBtC into the Qlikview eDBtC to simplify access and usability for centers. The project to combine DBtC and eDBtC features within a single application, which will be known simply as DBtC, will complete next year.

In 2019, **359** unique users from **202** different centers accessed **4,399** eDBtC sessions, and **221** users from **150** centers downloaded data **1,638** times.

Data for RFI

The CIBMTR launched the Data for RFI (Request for Information) application in 2017 to help centers access and use the outcome data they share with the CIBMTR. Data for RFI provides centers with the ability to access, view, reconcile, and format data submitted to the CIBMTR for use in fulfilling submissions to third party payers and other organizations. Data for RFI leverages the same data available in eDBtC but translates these to the standard RFI format developed by the American Society for Transplantation and Cellular Therapy (ASTCT). This application also incorporates the standard ASTCT RFI rules for determining survival and for differentiating between adult and pediatric populations. Centers can export data into the standard format developed by ASTCT and then supplement with additional data not collected by the CIBMTR. In 2019, **272** unique users from **181** different centers accessed **2,573** Data for RFI sessions.

Center Performance Analytics

The CIBMTR deployed Center Performance Analytics in Qlikview in 2016 to support center performance and quality initiatives by allowing authorized users to compare their center’s data to aggregated center data. Predefined filters for this comparison include geographic region, historical performance, volume of transplants as a proxy for size, and patient population served. Centers can also view their center's own one-year survival rate, based on a rolling three-year period of data included in the Transplant Center Specific Survival dataset. Like eDBtC, users can create and implement their own customized, ad hoc query and export a download of the source dataset for their center. In 2019, **114** unique users from **91** different centers accessed **405** Center Performance Analytics sessions.

Patient One-Year Survival Calculator for Allogeneic Transplants

The Patient One-Year Survival Calculator for Allogeneic Transplants provides authorized users with a tool to predict one-year survival for individual allogeneic HCT recipients. The calculator data are updated annually to reflect new information contained in the center outcomes analysis. In 2019, authorized users accessed the survival calculator **3,610** times.

Center Volumes Portal

The Center Volumes Portal allows centers to preview; correct, if necessary; and approve center volume data published annually on the *HRSA CWBYCTP* website. Centers may display and download the previous five years of volume data (2013-2017). In 2019, authorized users accessed **4,716** Center Volumes Portal pages.

Cord Blood Report Portal

The Cord Blood Report Portal provides secure, self-service access to monthly predefined Cord Blood Reports for authorized cord bank users. These reports, updated monthly, include data relevant to the quality and safety of distributed cord blood units to both domestic and international cord blood banks. In 2019, **75** unique users from **55** cord blood banks accessed **1,394** Cord Blood sessions.

Forms Reimbursement Financial Payments Dashboard

Launched in 2019, the Forms Reimbursement Financial Payments Dashboard offers a Qlikview-based, self-service application for transplant centers to securely download their monthly financial CIBMTR forms reimbursement reports. This portal not only provides the most current and up-to-date information to the users, but also serves as an archive of prior reports allowing users to actively monitor the changes of their data from month-to-month in a convenient web-based interface. Since its launch, **90** unique users from **78** different centers accessed **297** Forms Reimbursement Financial Payment sessions.

PartnerShare Portal

Launched in 2019, the PartnerShare portal is a commercial partner facing application built within the Qlikview environment that provides partners with the ability to access deidentified data across all centers. The PartnerShare portal is a secure environment allowing partners to view only those data that involve their products. Predefined filters permit users to isolate and review data subsets of interest, and robust visualization features provide one-click options for viewing their data in a chart or table. Partners can download these data for separate analysis. These data are updated monthly. Since its launch, **12** users accessed **64** PartnerShare sessions.

3.1.4 Be The Match Public Website

The Be The Match public website (***bethematch.org***) is designed for patients and families, donors, and supporters. It incorporates detailed information about transplantation and donation written for the public. The website provides scientific information in lay terms for donors related to the donation process and for patients related to specific diseases, various treatment options, the process of transplantation, and life after transplant. It also addresses concerns related to specific populations, including children and caregivers. The CIBMTR collaborates with NMDP/Be The Match to provide content for several areas of this website, including data for the US Center Listing Report.

US Center Listing Report

Transplant Center Directory
Provides transplant center specific information about facilities, personnel, diseases treated, cost, and transplant experience, including the number of transplants performed and survival rates by age, disease type, and disease stage.

3.1.5 Be The Match Clinical Website

The Be The Match Clinical website (***bethematchclinical.org***) is designed for clinicians, network participants, payors, and bioinformatics professionals. For clinicians, the website provides access to evidence-based tools, clinical guidelines, outcomes data, and education courses. The website also provides information specific to types of network participants: Transplant centers, donor centers, apheresis and collection centers, and cord blood banks. For payors, the website offers information to help individuals understand HCT, determine coverage, and answer employer and patient questions. Related to bioinformatics, the website provides resources for immunogenetic-focused research and operational bioinformatics as well as frequently used HLA tools.

3.1.6 HRSA CWBYCTP Website

The HRSA CWBYCTP website (***bloodstemcell.hrsa.gov***) provides information for the public, physicians, and other constituents. It incorporates information about stem cell donors and the Registry, the need for cord blood donation, resources for potential HCT recipient, and transplant outcomes. CIBMTR data and research findings are incorporated in numerous ways, including through provision of datasets and CIBMTR-created reports:

Transplant Outcomes and Data

US Patient Survival Report
Provides disease-specific post-HCT survival estimates by the length of time after transplant: 100 days, 1 year, and 3 years. Survival estimates are also available by patient age, patient gender, patient race, or cell source.

Transplant Data by US Center Report
Displays the number of adult donor and cord blood transplants performed at a specific US center.

Transplant Data by Disease Report
Displays the number of adult donor and cord blood transplants reported in the US for a specific disease. Totals are also available by patient age, patient gender, patient race, cell source, and the year the transplant was performed.

Transplant Activity Report and Dataset
Displays the number of transplants performed at US centers, including autologous as well as related and unrelated allogeneic. Numbers are also available by patient age, patient gender, patient race, cell source, disease, center location by state, and year in which the transplant was performed. A dataset is available for download.

3.1.7 Other Applications and Data Exchange Standards

The CIBMTR has multiple methods for sharing data. Those hosted on the *CIBMTR Portal site* were described in **Section 3.1.3**. Other methods for sharing data are:

AGNIS

AGNIS allows participating centers to electronically collect and share data with the CIBMTR. Data are entered once and then distributed and synchronized among databases. In 2019, **14,344** forms for **7,966** patients were submitted through AGNIS by **21** US centers and **4** international centers.

BRIDG

The BRIDG (Biomedical Research Integrated Domain Group) Model is an information model, representing a shared view of the concepts of basic, pre-clinical, clinical, and translational research. Common data elements (CDEs) for certain standard CIBMTR forms have been extracted and associated in the BRIDG model to one of three contexts: Recipient, donor, or stem cell product. Adding cellular therapy content is expected to remove barriers centers experience in electronic data transfers.

HL7 FHIR

The CIBMTR continues curating CDEs in the National Cancer Institute's Cancer Data Standards Registry and Repository (caDSR) and mapping to BRIDG, and the CIBMTR continues to develop and sustain additional standards for interoperability. Using *HL7's next-generation standards frame work*, the CIBMTR created a patient profile and successfully initiated demographic data exchange between a CIBMTR Fast Healthcare Interoperability Resources (FHIR) server and an Epic sandbox server. The CIBMTR also successfully deployed a custom operation to evaluate incoming patient demographics and assign a unique recipient ID (CRID) to each patient record.

This year the CIBMTR entered a collaborative phase of this project, coordinating with The Ohio State University and Moffitt Cancer Center. Over the coming months, production-level patient, HLA typing, and acute GVHD data will be exchanged and consumed within FormsNet.

Disease Risk Index Assignment Tool

In 2015, the CIBMTR launched a DRI Assignment Tool developed by investigators at the Dana Farber Cancer Institute and validated in a large CIBMTR study (Armand et al. Blood, 2014). It is intended for use by clinicians and researchers. The tool was developed for the primary outcome of overall survival after HCT and, at present, only applies to adult patients with hematologic malignancies. It is not intended to give an accurate prognosis for individual patients. In 2019, visitors accessed the DRI Assignment Tool **5,095** times.

VOD Risk Calculator

Launched in 2017, the VOD Risk Calculator is based on peer-reviewed publication (Strouse C et al. Biology of Blood and Marrow Transplantation, 2018). It is available on the CIBMTR public website and is intended for use by clinicians and researchers. The aim of the VOD Risk Calculator is to identify patients who have a high-risk score for developing VOD, an uncommon, early complication of HCT associated with significant mortality. In 2019, visitors accessed the VOD Risk Calculator **1,795** times.

3.2 ANNUAL MEETINGS

3.2.1 TCT Meetings

In 2019, the BMT Tandem Meetings became the TCT | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR (TCT Meetings) to better reflect an expanded range of interests and activities. The TCT Meetings, held annually in February, include **5** days of scientific sessions and other meetings targeted to worldwide physicians, scientists, and other professionals interested in cellular therapy.

2019 TCT Meetings

With **>4,000** attendees from **45** countries, the 2019 TCT Meetings included **5** plenary sessions, **9** concurrent sessions, **90** oral abstracts, **2** poster sessions, **7** corporate-supported symposia, and **7** product theaters. Continuing Medical Education (CME) and Continuing Education credits were issued through MCW to physicians and allied health professionals.

Clinical Research Professionals / Data Management Conference

With nearly **250** attendees, this conference provided forms and subject matter training, which increases the accuracy with which CIBMTR forms are completed.

BMT CTN Coordinators and Investigators Meetings

With approximately **100** and **>400** attendees, respectively, these meetings focused on study management, such as promoting studies and reporting adverse events; procedures, such as enrollment; specific clinical trials; and general cellular therapy subject matters.

Administrative Director Conference

With approximately **250** attendees, this conference focused on many topics related to leadership and quality, including methods for improving accuracy and value, staffing models and resilience, and Medicare coverage.

Pharmacists Conference

With **250** attendees, this conference presented the latest research and best pharmacy practices with a focus on specific diseases and therapies.

Nursing Conference

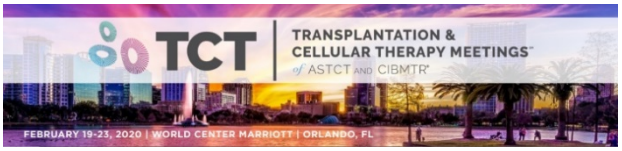
With **400** attendees, this conference presented the latest research and best nursing practices with a focus on communication techniques and quality of life.

Clinical Education Conference

Designed for advanced practice providers, fellows, and junior faculty, and with nearly **300** attendees, this conference focused not only on the latest clinical research but also ethics and resiliency.

Pediatric BMT Program

Held in conjunction with the Pediatric Blood and Marrow Transplant Consortium, this meeting is designed for all health professionals involved in caring for children undergoing cellular therapy. With nearly **700** attendees, the meeting shared cutting-edge science and state-of-the-art advances in treatment.



2020 TCT Meetings

The 2020 TCT Meetings are expected to attract approximately **4,000** attendees. The meetings will include **6** plenary sessions, **9** concurrent sessions, **96** oral abstracts, **1** late breaking abstract session, **2** poster sessions, **7** corporate-supported symposia, and **8** product theaters.

3.2.2 Cellular Therapy Registry Forum

In October 2019, the CIBMTR held a fifth Cellular Therapy Registry Forum. Participants encompassed **115** attendees representing a variety of industries, including clinical care, research, pharmaceuticals, insurance providers, professional organizations, and government representatives. Topics comprised the current status of the Cellular Therapy Registry, CAR T-cell toxicities and REMS reporting requirements, center practice topics, and non-transplant cellular therapy for solid tumors.

CAR T-cell toxicity and REMS reporting requirement topics included presentations regarding the US Food and Drug Administration REMS guidance, industry viewpoints, center reporting, and a CIBMTR proposal for a REMS Assessment Report. In addition, presenters provided updates regarding CIBMTR toxicity reporting along with ASTCT toxicity and ASTCT immune effector cell-associated neurotoxicity syndrome (ICANS) management guidelines.

Cellular therapy center practice topics included presentations regarding services and practices to be considered when establishing a cellular therapy center, immune effector cell center accreditation, the CIBMTR Cellular Therapy Data Audit Program, and the consideration of an RFI for CAR T-cell programs.

Finally, presenters discussed non-transplant cellular therapy for solid tumors and the roadmap for long-term follow-up of patients with solid tumors.

3.3 DATA MANAGEMENT TRAINING

The CIBMTR has comprehensive, secure, and efficient applications to allow centers to electronically submit data to the CIBMTR.

Center Support ServiceNow
The CIBMTR implemented a web-based Customer Service Center in May 2019. The ServiceNow platform allows centers to enter questions and requests in a single location while allowing triage of questions to appropriate Coordinating Center staff. To easily utilize service center functionality, there are job aids, a reference guide for transplant centers and for apheresis / collection / donor centers, and a training video available on the *CIBMTR Center Support* webpage. A recent survey indicated **84%** of respondents felt their questions, requests, and / or issues were resolved in a timely manner using ServiceNow.

Visit the *CIBMTR Data Management* webpage to access online resources:

Data Management Guide
Learn about participation in CIBMTR research, center membership, access to FormsNet, data manager education, mentor program, forms submission process, and many useful tips and links.

Manuals
Find the answers to your data submission questions by accessing the *Forms Instructions Manual*, which includes general instructions and instructions for each form type.

FormsNet
Learn how to submit data to the CIBMTR via the FormsNet application, a secure clinical research management system, which is in compliance with SCTOD requirements.

Conference Materials
Access meeting materials as well as audio and visual presentations from the Clinical Research Professionals / Data Management Conferences.

AGNIS
Learn how to retrieve and transmit form data, extracted directly from your own institution's database, directly to the FormsNet application using AGNIS, a secure, standards-based system.

Legacy Data
Review retired data manuals, forms, and other archived documents for reference purposes and to assist in making changes to legacy data.

Adverse Events
Learn how to report adverse events and product issues through FormsNet.

Newsletters and eBlasts
Read archived issues of the Data Matters Training Newsletter and eBlasts.

Online Training
Review educational modules developed for new and seasoned data managers. Modules are available on the *Online Training* webpage.

Online Trainings
New / Updated this Year

HLA SERIES

- **Introduction to HLA**
- **Basic Biology of HLA**
- **Advanced Biology of HLA**
- **Genetics of HLA**

INSTITUTIONAL REVIEW BOARD (IRB)

- **NMDP IRB New Member Orientation**

GENERAL TOPICS

- **Excel: Filtering and Sorting Recipient CPI Forms Due Reports**

3.4 INFORMATION REQUEST SERVICE

The CIBMTR Information Request Service provides timely access to cellular therapy data to patients, physicians, hospitals, pharmaceutical companies, insurance companies, and others involved in healthcare. Requests range from simple queries of patient, disease, and therapy frequencies to those with greater complexity involving specific data combinations and / or statistical analysis of outcomes.

Potential Reasons for Information Requests

- Self-education and decision making
- Patient counseling or clinical decision making
- Presentation support
- Center assessments
- Clinical trial planning
- Market assessments

Coordinating Center staff members fulfill requests related to clinical decision making within three days and most other requests within five days. If a request will take more than an estimated three weeks to fulfill, a Coordinating Center staff member will contact the requestor to discuss an appropriate timeline.

Accomplishments

In 2019, the CIBMTR fulfilled **473** requests for information and data (**Table 3.2**).

Table 3.2 Data Requests Addressed by the CIBMTR in 2019

Requestor	Number of Requests
Physician / Researcher	285
Commercial Organizations	59
Physician for Patient Care	43
Patient or Relative	21
Student	15
Market Research Firm	12
Federal Government Agency	10
Patient Advocacy Group	2
Cord Blood Bank	2
News Media	2
Insurance Company	1
Be The Match Operations	21
TOTAL	473

How to Access

For more information about requesting data from the Research Database, visit the *CIBMTR How to Request Data* webpage. If you would like a one-time, custom analysis, complete the *Custom Information Request Form* on that webpage. If you have questions about requesting CIBMTR data, please contact *inforequest@mcw.edu*.

3.5 ACCESSING CIBMTR KNOWLEDGE

The CIBMTR shares its knowledge in different ways. To determine the best way to access specific types of CIBMTR knowledge, review **Tables 3.3-3.6**.

Table 3.3 How to Access Information Online at *cibmtr.org**

Information

GENERAL INFORMATION
Access the Facts and Figures report on the *Administrative and Progress Reports* webpage. Read editions of the quarterly newsletter on the *Newsletters* webpage, and email *cibmtr-news@mcw.edu* to be added to the electronic distribution list.

ACTIVITIES
Review Section 2 or visit the *What We Do* webpage. Visit the *Studies* webpage, *SCTOD* webpage, or *Corporate Support* webpage for associated information.

MEETINGS
Visit the *Meetings* webpage for information about annual *TCT Meetings* and other major events, such as Cellular Therapy and *Center Outcomes Forums*.

TRAINING
Review Section 3.3 or visit the *Data Management* webpage to access *training materials*, *manuals, and guides*.

PUBLICATIONS
Review the CIBMTR's >1,400 publications on the *Publication List* webpage. For research summaries written for the lay public, visit the *Study Summaries for Patients* webpage.

OTHER
Email *contactus@cibmtr.org*

Table 3.4 How to Access Data Online at *cibmtr.org**

Data

TYPES OF DATA
Review the baseline and follow-up data available for recipients and donors on the *Types of Data Available for Research or Request* webpage. Assess data fields available in the Research Database by reviewing the *Data Collection Forms for Investigators* webpage.

STANDARD REPORTS (Table 3.7)
Access the *Summary Slides*, *BMT Survival Statistics Report*, *Center Transplant Activity Report*, *Patient Transplant Outcomes Reports*, and *Center-Specific Survival Reports* on the *Slides and Reports* webpage.

RESEARCH STUDIES
Propose a study as explained on the *How to Propose a Study* webpage, or participate in one of the existing studies listed on the *Working Committee Study Lists* webpage.

RESEARCH DATASETS
To request a dataset, propose a study via the *How to Propose a Study* webpage. To access final study analysis datasets, visit the *Publication List*.

CUSTOM ANALYSES
Complete the *Custom Information Request Form* or *Corporate Member Information Request Form*.

OTHER
Email *inforequest@mcw.edu*

Table 3.5 How to Access Tools Online at *cibmtr.org**

Tools

DBtC and eDBtC
On the *Portal site*, authorized users may download their most commonly used CRF- and TED-level variables.

DATA FOR RFI
On the *Portal site*, authorized users may access, view, reconcile, and export into the ASTCT standard RFI format the data their center submitted.

CENTER PERFORMANCE ANALYTICS
On the *Portal site*, authorized users may compare their center's data to aggregated center data filtered by various factors, view their own one-year survival rate, or create and implement ad hoc queries.

PATIENT ONE-YEAR SURVIVAL CALCULATOR FOR ALLOGENEIC TRANSPLANTS
On the *Portal site*, authorized users may predict one-year survival for individual allogeneic HCT recipients.

CENTER VOLUMES PORTAL
On the *Portal site*, authorized users may preview, correct, and approve center volume data published on the HRSA CWBYCTP website.

PARTNERSHARE
On the *Portal site*, authorized commercial research partner users may access deidentified patient data submitted across centers based on therapeutic product used.

More Tools

DISEASE RISK INDEX (DRI) ASSIGNMENT TOOL
Access this tool on the *DRI Assignment Tool* webpage to categorize patients undergoing allogeneic HCT for hemato-logic malignancy by disease risk.

VOD RISK CALCULATOR
Access this tool on the *VOD Risk Calculator* webpage to identify patients at high risk for veno-occlusive disease (VOD).

Table 3.6 How to Access Biospecimens Online at *cibmtr.org**

Biospecimens

SAMPLES TYPES AND INVENTORY
Review the >2 million samples available in the Research Repository via the *Sample Types and Inventory Summary* webpage.

REQUESTING SAMPLES
For studies that include recipient clinical outcome data, propose a study as explained on the *How to Propose a Study* webpage.

For studies that do not include clinical outcome data, review the *How to Request Samples from the Research Sample Repository* webpage.

OTHER
Email *research-repos@nmdp.org*

*If you are unable to access items using the electronic links provided, enter the underlined and italicized words into a search engine.

Table 3.7 Standard Reports Published by the CIBMTR

Report Title	Description	Accessibility*
CIBMTR Annual Report	Information on the CIBMTR's goals and achievements as well as operational details on how the CIBMTR is funded, supported, promoted, and maintained	Published on the *CIBMTR Administrative and Progress Reports* webpage Released: February Format: PDF
CIBMTR Summary Slides	Charts and figures summarizing current uses and outcomes of allogeneic and autologous HCT; developed in conjunction with the TCT Meetings	Published on the *CIBMTR Summary Slides* webpage Released: February Format: PPT
CIBMTR Facts and Figures	High level summary of CIBMTR fiscal year accomplishments and high impact publications	Published on the *CIBMTR Administrative and Progress Reports* webpage Released: September Format: PDF
US Centers Annual Transplant Activity Report and Dataset	Dataset containing center-specific pre-transplant patient-, disease-, and transplant-related characteristics data for nearly all allogeneic and a majority of autologous HCTs performed in the US annually since 2008	Published on the *HRSA CWBYCTP* website Released: September Format: PDF
CIBMTR Report of Survival Statistics for BMT	Highly detailed report on survival statistics that describes use and outcome of autologous and allogeneic HCT in the >500 centers that have participated in the CIBMTR	Via Corporate Membership Program (**Section 2.7**) or by request from physicians for making treatment decisions or clinical investigators planning clinical studies to *inforequest@mcw.edu* Released: November Format: Word
US Patient Center-Specific Survival Report	Comparison of observed to expected one-year survival rates among centers in the HRSA CWBYCTP network; evaluates outcomes for transplants using both related and unrelated donors	Published on the *Be The Match Transplant Center Directory* webpage; available as a Word document upon request to *inforequest@mcw.edu* Released: December Format: Web

Report Title	Description	Accessibility*
US Patient Transplant Outcomes	Disseminated in 3 different reports: • US Patient Survival Report: 100-day, 1-year, and 3-year survival rate estimates for US HCT recipients by disease and donor type • US Transplant Data by Center Report: Number of bone marrow and cord blood transplants performed at a specific center • US Transplant Data by Disease Report: Number of bone marrow and cord blood transplants for a specific disease	Published on the *HRSA CWBYCTP* website Released: December Format: Web
US Allogeneic Transplant Activity Report	Report containing patient, disease, donor HLA match, donor age, and gender match information for allogeneic transplant activity in the US since 2010	Via Corporate Membership Program (**Section 2.7**) Released: January, April, July and October Format: PDF
CIBMTR Newsletter	Articles regarding Working Committees, the SCTOD, data management and collection, and noteworthy events in the cellular therapy community	Published on the *CIBMTR Newsletters* webpage and distributed via email; contact *cibmtr-news@mcw.edu* to be added to the distribution list Released: February, May, August and November Format: Web
Study Summaries for Patients	Summaries of CIBMTR research publications written for patients and others in the lay public	Published on the *CIBMTR Study Summaries for Patients* webpage Released: Ongoing Format: PDF

*If you are unable to access items using the electronic links provided, enter the underlined and italicized words into a search engine.

4.0 HOW WE COLLECT AND MANAGE DATA

4.1 RESEARCH DATA LIFE CYCLE

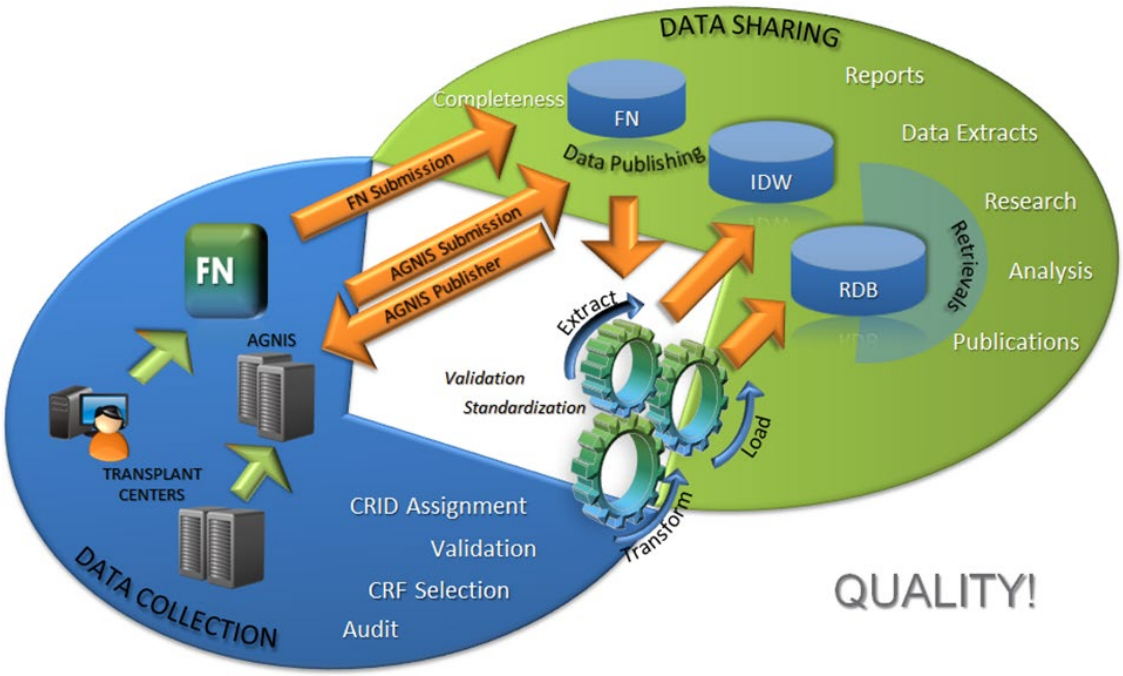
The Research Data Life Cycle (**Figure 4.1**) describes the path of data from the point of submission to its ultimate use in analyses, reports, and publications.

The process begins with data collection. Most centers enter data in FormsNet, a web application now in its third generation. Centers that have implemented local or third-party systems can also capture and submit data electronically using AGNIS. The goal of these applications is to capture high quality data as efficiently as possible.

Following collection, data undergo quality assessment and validation and are extracted and loaded into the CIBMTR Research Database on a monthly basis.

Data Sharing completes the cycle, providing data for analyses that have been collected and curated to ensure research value. These data are extracted from the Research Database in monthly retrievals to serve a range of research and stakeholder needs. The data retrievals provide the basis for research study data files, reports, and externally-requested datasets. The most commonly used CRF- and TED-level data are also directly available to centers through use of the eDBtC application (**Section 3.1.3**).

Figure 4.1 Research Data Life Cycle



Abbreviations: AGNIS = A Growable Network Information System, CRID = CIBMTR Recipient Identification Number, CRF = Comprehensive Report Form, FN = FormsNet, IDW = Integrated Data Warehouse, RDB = Research Database

4.2 COLLECTING AND STORING DATA

4.2.1 FormsNet

More than **98%** of data collected by the CIBMTR is submitted electronically via FormsNet, a comprehensive electronic data submission system containing **>130** forms related to capturing cellular therapy outcomes for donors and recipients. The application was updated in 2019 to provide key enhancements to support operational efficiencies. The CIBMTR also released several new forms this year in a continuing effort to maintain alignment between data collection and treatment practices as the cellular therapy field changes. Several study forms were also added and revised this year to support specific projects.

4.2.2 Integrated Data Warehouse

The CIBMTR Integrated Data Warehouse accommodates the integration of data coming from multiple sources and spanning multiple domains, such as patient-reported outcomes, product data, HLA, biospecimens, and study data. This year the CIBMTR completed multiple enhancements to support integration of data sources to meet warehouse users' reporting needs. This integration includes form reimbursement data stored in a variety of sources and a consolidated process for external reimbursement. The CIBMTR also completed the design and implementation of a unified data model for consumption, storage, maintenance, and extraction of cellular therapy outcomes data to be used by the CIBMTR for CAR T-cell research. The unified domain model architecture was also expanded to enable consumption of ePRO and Rave data, providing the framework for integrating FormsNet data with data from other sources.

4.2.3 Data Transformation Initiative

In 2019, a CIBMTR Task Force began an assessment of new methods for data acquisition, management, retrieval, merging, and sharing. Task force team members conducted a deep review of current internal processes and consulted with external network partners who interact with the CIBMTR to submit and access data The team explored potential new technologies to address current and future data needs.

The Task Force brought focus to the critical and urgent nature of this work. NMDP/Be The Match identified data transformation as one of its five strategic initiatives beginning in 2019, and MCW included advancing data science in the CIBMTR as an important part of its strategic plan. The Task Force established a formal vision for the initiative and developed a plan that positions the CIBMTR to transform its approach to data acquisition, storage, and analysis, including securing funding. The plan includes a partnership with a globally recognized vendor in human data science that will weave together technologies, resources, and domain expertise. The CIBMTR will continue to own all data and the resulting new data ecosystem and technical solutions developed.

Vision of the Data Transformation Initiative

Optimize the acquisition and utilization of entrusted data assets to accelerate breakthroughs that transform patient experiences

Work is already underway to design, develop, test, and evaluate a prototype for data acquisition that will enable a "source to target" approach using electronic data capture – without the need for manual data entry. The CIBMTR will pilot the prototype with a few initial sites and then begin scaling to its entire network, as appropriate. Further aspects, including enhanced data analytics and visualization, are also in planning phases.

This initiative, made possible with substantial funding form NMDP/Be The Match and MCW, is planned as a 3-year project that will put the CIBMTR in a strong position to support a rapidly changing field with tools that minimize reporting burden and enhance research capabilities.

4.3 ENSURING DATA QUALITY

4.3.1 Continuous Process Improvement

Robust data collection is critical to the success of the CIBMTR. The Continuous Process Improvement (CPI) program ensures timeliness and completeness of data forms submissions (**Appendix H**).

Recipient Forms

Throughout each trimester, US centers receive CPI reports listing the number of follow-up forms that were due in the previous trimester and the number and percentage of each submitted within the trimester. Letters are sent **3** times per year (January, May, and September) to the Medical Director and Primary Data Manager notifying them if their center met CIBMTR CPI criteria for the trimester. A form is not considered officially submitted until errors are resolved and all applicable information is submitted and approved. To be compliant, centers must submit **≥90%** of forms due for the trimester, for all transplants since December 3, 2007.

In 2020, the CPI program will expand to include international centers from Australia, Brazil, and New Zealand. Additional international countries will be phased in over time. To be compliant for the first CPI trimester ending February 29, 2020, centers must submit **>60%** of forms due since January 1, 2016.

Donor Forms

The Donor Data Management Team oversees submission of donor work-up and donation forms from NMDP/Be The Match donor, collection, and apheresis centers. Donor CPI reports are generated **4** times per year (January, April, July, and October) for US centers. To be compliant, centers must submit **100%** of the forms required for that CPI period.

4.3.2 Verification and Validation

FormsNet

When data are entered into FormsNet, a series of entry level validation checks takes place to ensure data consistency. This process flags certain errors at the time of entry so the errors can be corrected immediately while source documents are readily available. If a data field does not pass the FormsNet validation checks, an error message is generated, and the data manager is navigated to an error review page to review, resolve, or override the unresolved errors. Lastly, an error report is generated that lists any unresolved or overridden errors.

The CIBMTR reacts to data quality issues detected downstream by incorporating additional data quality validations in the upstream data capture system (FormsNet). Detection of issues earlier in the process provides real time feedback to the parties most capable of correcting the problem and improves the overall quality of the data.

Research Database

Data extracted from FormsNet and loaded into the Research Database each month undergo comprehensive assessment for quality. These data are rigorously validated for consistency, completeness, and uniqueness using business rules implemented in custom logic. The Data Quality Team reviews errors and works with centers to clarify and correct data.

Rules across various categories are updated and tested as part of ongoing Forms Revision processes. The CIBMTR continues work to reduce the number of data entry errors. The current rate of form rejection due to inconsistently reported data is **<1%**; it decreased from **<2%** because of enhancements in center education, direct center queries, and validations built into the FormsNet entry point.

4.3.3 On-Site Data Audit Program

On-going data audits are performed at all CIBMTR participating centers. The audit compares data in source documents maintained at the center with data contained in the CIBMTR Research Database. Clinical Research Associates perform the on-site center audits, spending 3-4 days at each center reviewing original source documents. The overall audit process is displayed in **Figure 4.2**.

In 2019, **60** centers were scheduled for audit (**50** domestic, **10** international). As of December 1, 2019, **46** centers were notified of their final audit results, including requested corrective action follow-up. Of the centers sent reports, **80%** passed with **≤3%** critical field errors. Of the **9** centers that did not pass the audit, **4** received approval of their corrective action plans; the remaining **5** centers are in the process of completing requested corrective action plans.

The CIBMTR conducted a non-transplant cellular therapy data audit pilot this year as a first step in expanding the standard HCT data audit program to target non-transplant cellular therapy-specific records. **3** high-volume centers participated in this pilot. The results demonstrated that baseline forms had levels of error rates **≤3%**, similar to the rate observed in HCT data audits. Interestingly, follow-up forms, specifically those fields capturing CAR T-cell specific toxicities, had error rates slightly higher, highlighting the need for training focused on toxicity reporting. The results were used to develop a formal non-transplant cellular therapy audit program, which will be implemented next year.

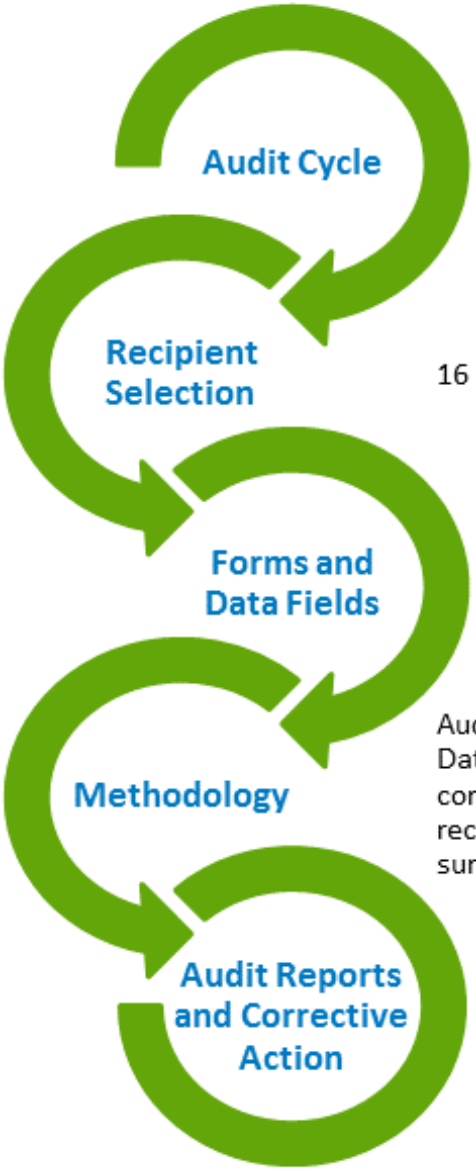
Consolidated FACT-CIBMTR Audits

Since 2017, the CIBMTR has conducted data quality audits and evaluations on behalf of both organizations; FACT determines whether the results of a data quality audit are satisfactory for accreditation.

All verification of the accuracy of data against source data is done by CIBMTR audit teams on site according to their current practices and schedules. FACT verifies, at each annual report and at each application for accreditation renewal, the status of CIBMTR data accuracy by requiring submission of centers' most recent CIBMTR audit results. FACT verifies completeness of data by reviewing each center's most recent CPI report (**Section 4.3.1**). FACT also requires centers to submit results of internal audits and / or update their corrective action plans annually.

Adequate data quality is one component of FACT accreditation. Transplant programs submitting an Annual Report or Renewal Application to FACT are reviewed for data quality and submission during the monthly FACT / CIBMTR Data Audit Committee meeting.

Figure 4.2 Audit Process



Each domestic and international center that has submitted data for ≥20 HCTs is audited once within the 4-year audit cycle.

16 recipient records are randomly selected for audit.

All TED and CRF data are subject to audit, and essential critical data fields are audited for each recipient.

Auditors compare the data submitted to the CIBMTR Research Database with the data in the source documents. They make all data corrections to the database and provide the transplant center with a record of changes made. At the end of the audit, the auditors hold a summary session to review any trends found during the audit.

Transplant centers receive a detailed audit report evaluating the results of their audit. Centers are required to submit a Corrective Action Plan following the audit in response to a critical field error rate above 3%, any systemic errors, consent issues requiring correction, or outstanding missing documentation issues.

4.4 PROTECTING PATIENTS AND DATA

4.4.1 Human Subjects / HIPAA Compliance

The CIBMTR is committed to the ethical conduct of research. All Coordinating Center personnel maintain Collaborative IRB Training Initiative (CITI) certification. The NMDP/Be The Match IRB, which is fully accredited by the Association for the Accreditation of Human Research Protection Programs, reviews all human subject research conducted by the CIBMTR and serves as the single IRB for the BMT CTN (**Section 2.3.1**). The CIBMTR maintains compliance with the Health Insurance Portability and Accountability Act (HIPAA) Privacy Rule, as applicable. CIBMTR rules requiring the registration of all consecutive HCT recipients ensure the inclusion of women, minorities, and children, so the Research Database population includes women and minorities in the same proportion as found in the general HCT population. Children are included in most CIBMTR studies; their inclusion is dependent on the study focus.

4.4.2 Information Security and Data Privacy

The CIBMTR protects the data and information received from centers and patients. The SCTOD contract (**Section 2.1.2**) requires specific protections through security controls, policies, and standards. The CIBMTR's data systems are maintained in accordance with the Federal Information Systems Management Act of 2002, and with information security guidance provided by the National Institute of Standards and Technology (NIST 800-53). In accordance with *National Institute of Standards and Technology Special Publication 800-18*, and in collaboration with HRSA's Office of Information Technology, the CIBMTR maintains a System Security Plan that outlines management, operational, and technical controls.

Since 2008, the CIBMTR has received an Authority to Operate from the Chief Information Officer of HRSA. A third-party auditor completed the CIBMTR's most recent security control assessment of information security programs in September 2019. Controls maintained by both MCW and NMDP/Be The Match protect the data and information in these systems in ways beyond those required by HIPAA.

In response to changing global personal data protection requirements, the CIBMTR reviewed and updated its *Data Use and Processing Policy* in 2018. A *Personal Data Protection Statement* is publicly posted on the CIBMTR website along with additional information regarding *CIBMTR security infrastructure* and how it protects personal data. The CIBMTR understands the importance of its role as a steward of the data it collects and continues to work with domestic and international partners to ensure its systems meet all standards.

5.0 OUR IMPACT

The CIBMTR is dedicated to improving survival, treatment, and quality of life for patients. The CIBMTR conducts practice-changing research, with **>1,400** publications addressing important and timely issues in HCT and non-transplant cellular therapy.

CIBMTR Research Helps Patients and Physicians

Select donors and grafts

Evaluate patient risk

Identify long-term effects of cellular therapy

Provide medical care guidance for survivors

Address access to care

Select Donors and Grafts

CIBMTR studies help establish the paradigm for selecting the best donor and graft:

- Optimal HLA matching
- Impact of non-HLA donor characteristics
- Cord blood vs bone marrow vs peripheral blood

Evaluate Patient Risk

CIBMTR studies show which patients:

- Have the highest risk of GVHD and other complications
- Are most likely to benefit from cellular therapy

Identify Long-Term Effects of Cellular Therapy

CIBMTR studies provide insight into:

- Long-term impact of cellular therapy on patients and their families, including risk of second cancers and other late complications
- Survivors' quality of life

Provide Medical Care Guidance for Survivors

The CIBMTR works with the medical community to develop guidelines for optimal long-term care of cellular therapy survivors to:

- Decrease the rate of late complications
- Preserve patients' fertility
- Identify post-cellular therapy best practice preventive health behaviors

Address Access to Care

CIBMTR studies address the broad range of issues that influence access to cellular therapy and long-term care after treatment, including:

- Disparities in access and outcomes for specific populations
- Costs of care

The CIBMTR has become a respected leader in clinical and translational research by providing a unique resource of clinical data, biospecimens, and scientific and statistical expertise to medical and scientific communities. The importance of this resource is evidenced by the thousands of hours of voluntary efforts from physicians and scientists spent using these data to address important issues in treatment of patients with cancer and life-threatening blood and immune disorders.

APPENDIX A: CENTERS

The CIBMTR represents a network of **>380** participating centers in approximately **>35** countries that submit outcomes-related data for patients.

APPENDIX A1: US CENTERS

The following table lists the US-based centers that submited data to the CIBMTR Research Database for matched unrelated donor (MUD) allogeneic, related donor allogeneic, and autologous transplants in the past three years. Centers submit data at two levels: TED level captures basic data, and CRF level captures more detail.

Participating Center	City	State	MUD	RELATED	AUTO
University of Alabama Birmingham	Birmingham	AL	CRF	CRF	CRF
Huntsville Hospital	Huntsville	AL	N/A	N/A	TED
University of Arkansas for Medical Sciences	Little Rock	AR	CRF	TED	TED
Arkansas Children's Hospital	Little Rock	AR	N/A	N/A	TED
Banner MD Anderson Cancer Center	Gilbert	AZ	N/A	CRF	CRF
Mayo Clinic Arizona and Phoenix Children's Hospital	Phoenix	AZ	CRF	CRF	CRF
University of Arizona Cancer Center at Dignity Health and St. Joseph's Hospital and Medical Center	Phoenix	AZ	N/A	N/A	CRF
Cancer Transplant Institute at Virginia G. Piper Cancer Center	Scottsdale	AZ	CRF	CRF	CRF
Banner University Medical Center - Tucson	Tucson	AZ	CRF	TED	TED
City of Hope National Medical Center	Duarte	CA	CRF	TED	TED
University of California, San Diego Medical Center	La Jolla	CA	CRF	CRF	CRF
Scripps Blood & Marrow Transplant Program	La Jolla	CA	CRF	CRF	CRF
Loma Linda University Cancer Center	Loma Linda	CA	CRF	CRF	CRF
Cedars-Sinai Medical Center	Los Angeles	CA	CRF	TED	N/A
Children's Hospital of Los Angeles	Los Angeles	CA	CRF	TED	TED

Participating Center	City	State	MUD	RELATED	AUTO
USC BMT Program	Los Angeles	CA	CRF	CRF	CRF
UCLA Hematologic Malignancy / Stem Cell Transplantation Program	Los Angeles	CA	TED	TED	TED
UCSF Benioff Children's Hospital - Oakland	Oakland	CA	CRF	CRF	CRF
CHOC Children's	Orange	CA	CRF	CRF	CRF
Lucile Packard Children's Hospital	Palo Alto	CA	CRF	CRF	TED
University of California-Davis Cancer Center	Sacramento	CA	CRF	TED	TED
Sutter Cancer Center	Sacramento	CA	TED	TED	TED
Rady Children's Hospital San Diego	San Diego	CA	CRF	CRF	CRF
University of California - San Francisco - Pediatrics	San Francisco	CA	CRF	CRF	CRF
University of California - San Francisco - Adults	San Francisco	CA	CRF	CRF	CRF
Stanford University Medical Center	Stanford	CA	CRF	CRF	TED
The Children's Hospital of Denver	Aurora	CO	CRF	CRF	CRF
University of Colorado Hospital	Aurora	CO	TED	TED	N/A
Colorado Blood Cancer Institute	Denver	CO	TED	TED	TED
Yale New Haven Hospital	New Haven	CT	CRF	TED	TED
Children's National Medical Center	Washington	DC	CRF	TED	TED
Medstar Georgetown University Hospital	Washington	DC	CRF	CRF	CRF
Christiana Care	Newark	DE	CRF	CRF	CRF
Alfred I. duPont Hospital for Children	Wilmington	DE	CRF	CRF	CRF
Shands HealthCare & University of Florida	Gainesville	FL	CRF	CRF	CRF
Mayo Clinic Florida - Jacksonville	Jacksonville	FL	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
BMT Program of Mayo Clinic / Nemours and Wolfson Children's Hospital	Jacksonville	FL	CRF	CRF	CRF
Nicklaus Children's Hospital	Miami	FL	CRF	TED	TED
University of Miami - Adults	Miami	FL	CRF	CRF	CRF
University of Miami / Jackson Memorial Hospital	Miami	FL	CRF	TED	TED
Baptist Hospital of Miami	Miami	FL	N/A	N/A	CRF
Blood & Marrow Transplant Center, Florida Hospital Medical Group	Orlando	FL	TED	TED	TED
Florida Center for Pediatric Cellular Therapy	Orlando	FL	TED	TED	TED
Memorial Cancer Institute	Pembroke Pines	FL	TED	TED	TED
Johns Hopkins All Children's Hospital	St. Petersburg	FL	CRF	CRF	CRF
H. Lee Moffitt Cancer Center	Tampa	FL	CRF	TED	TED
Children's Healthcare of Atlanta at Egleston	Atlanta	GA	CRF	CRF	CRF
The Blood and Marrow Transplant Program at Northside Hospital	Atlanta	GA	CRF	CRF	CRF
Emory University	Atlanta	GA	CRF	CRF	CRF
Georgia Cancer Center at Augusta University Health	Augusta	GA	CRF	CRF	CRF
Cancer Treatment Centers of America - Southeastern Regional Medical Center	Newnan	GA	N/A	N/A	CRF
Kapi'olani Medical Center for Women and Children	Honolulu	HI	CRF	CRF	CRF
University of Iowa Hospital & Clinics	Iowa City	IA	CRF	CRF	CRF
St. Luke's Mountain States Tumor Institute	Boise	ID	CRF	CRF	CRF
Ann & Robert H. Lurie Children's Hospital of Chicago	Chicago	IL	CRF	CRF	CRF
University of Illinois at Chicago Medical Center	Chicago	IL	CRF	CRF	CRF
Northwestern Memorial Hospital	Chicago	IL	CRF	CRF	TED

Participating Center	City	State	MUD	RELATED	AUTO
The Coleman Foundation Blood and Marrow Transplant Center, Rush University	Chicago	IL	CRF	CRF	CRF
Comer Children's Hospital / University of Chicago Medicine	Chicago	IL	CRF	CRF	CRF
University of Chicago Medicine	Chicago	IL	CRF	CRF	CRF
NorthShore University HealthSystem	Evanston	IL	N/A	N/A	CRF
Loyola University Medical Center	Maywood	IL	CRF	CRF	CRF
Advocate Lutheran General Hospital	Park Ridge	IL	TED	TED	TED
Cancer Treatment Centers of America - Midwestern Regional Medical Center	Zion	IL	CRF	CRF	CRF
Indiana Blood & Marrow Transplantation	Indianapolis	IN	CRF	CRF	CRF
Indiana University Hospital / Riley Hospital for Children	Indianapolis	IN	CRF	CRF	TED
University of Kansas	Kansas City	KS	CRF	CRF	CRF
Ascension Via Christi Hospitals Wichita	Wichita	KS	N/A	CRF	CRF
University of Kentucky Medical Center	Lexington	KY	CRF	TED	TED
University of Louisville Hospital / James Brown Cancer Center	Louisville	KY	CRF	TED	TED
Louisiana State University Children's Hospital	New Orleans	LA	CRF	CRF	CRF
Tulane University Medical Center	New Orleans	LA	CRF	CRF	CRF
Ochsner Medical Center	New Orleans	LA	CRF	CRF	TED
Ochsner LSU Health Shreveport	Shreveport	LA	TED	TED	TED
Beth Israel Deaconess Medical Center	Boston	MA	CRF	TED	TED
Dana Farber Cancer Institute at Brigham and Women's Hospital - Adults	Boston	MA	CRF	CRF	TED
Tufts New England Medical Center	Boston	MA	CRF	TED	TED
Massachusetts General Hospital	Boston	MA	CRF	TED	TED

Participating Center	City	State	MUD	RELATED	AUTO
Dana Farber Cancer Institute & Boston Children's Hospital	Boston	MA	CRF	CRF	TED
Boston Medical Center	Boston	MA	N/A	N/A	TED
Lahey Clinic Medical Center	Burlington	MA	N/A	N/A	TED
UMass Memorial Medical Center	Worcester	MA	CRF	CRF	CRF
University of Maryland School of Medicine	Baltimore	MD	CRF	CRF	CRF
The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins	Baltimore	MD	TED	TED	TED
NIH - Experimental Transplantation and Immunology Branch (Related Donor Program)	Bethesda	MD	N/A	CRF	CRF
National Heart Lung & Blood Institute	Bethesda	MD	N/A	TED	N/A
National Institutes of Health	Bethesda	MD	N/A	CRF	N/A
National Institutes of Allergy & Infectious Disease	Bethesda	MD	N/A	TED	N/A
NIH - NCI Matched Unrelated Donor Program	Bethesda	MD	CRF	N/A	N/A
The University of Michigan	Ann Arbor	MI	CRF	TED	TED
Henry Ford Hospital Bone Marrow Transplant Program	Detroit	MI	CRF	CRF	CRF
Karmanos Cancer Institute	Detroit	MI	CRF	CRF	CRF
Children's Hospital of Michigan	Detroit	MI	CRF	CRF	CRF
Helen DeVos Children's Hospital	Grand Rapids	MI	CRF	CRF	CRF
Spectrum Health	Grand Rapids	MI	CRF	CRF	CRF
Masonic Cancer Center University of Minnesota	Minneapolis	MN	CRF	CRF	CRF
Children’s Hospital and Clinics of Minnesota	Minneapolis	MN	N/A	N/A	CRF
University of Minnesota Blood and Marrow Transplant Program - Pediatrics	Minneapolis	MN	CRF	CRF	CRF
University of Minnesota Blood and Marrow Transplant Program - Adults	Minneapolis	MN	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
Mayo Clinic Rochester	Rochester	MN	CRF	CRF	CRF
Children's Mercy Hospital	Kansas City	MO	CRF	CRF	CRF
Cardinal Glennon Children's Hospital	St. Louis	MO	CRF	CRF	CRF
Barnes Jewish Hospital	St. Louis	MO	CRF	CRF	TED
SSM Health Saint Louis University Hospital	St. Louis	MO	CRF	TED	TED
Washington University / St. Louis Children's Hospital	St. Louis	MO	CRF	CRF	CRF
University of Mississippi Medical Center - Jackson	Jackson	MS	CRF	TED	TED
Billings Clinic Cancer Center	Billings	MT	N/A	N/A	TED
University of North Carolina Hospitals - Chapel Hill	Chapel Hill	NC	CRF	CRF	CRF
BMT Program at Levine Children's Hospital / Carolinas Medical Center	Charlotte	NC	CRF	CRF	CRF
Levine Cancer Institute	Charlotte	NC	CRF	CRF	CRF
Novant Health Transplantation and Cellular Therapy Program	Charlotte	NC	N/A	N/A	CRF
Duke University Medical Center - Pediatrics	Durham	NC	CRF	CRF	CRF
Duke University Medical Center - Adults	Durham	NC	CRF	TED	TED
Wake Forest Baptist Health	Winston-Salem	NC	CRF	TED	TED
Novant Health Oncology Specialists	Winston-Salem	NC	N/A	N/A	N/A
University of Nebraska Medical Center	Omaha	NE	CRF	CRF	CRF
Nebraska Methodist Hospital	Omaha	NE	N/A	N/A	TED
Dartmouth-Hitchcock Medical Center	Lebanon	NH	CRF	CRF	CRF
Hackensack University Medical Center	Hackensack	NJ	CRF	CRF	CRF
Cancer Institute of New Jersey	New Brunswick	NJ	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
UNM Comprehensive Cancer Center	Albuquerque	NM	N/A	N/A	TED
Montefiore Medical Center	Bronx	NY	TED	TED	TED
Children's Hospital at Montefiore	Bronx	NY	CRF	TED	TED
Roswell Park Cancer Institute	Buffalo	NY	CRF	CRF	CRF
Westchester Medical Center	Hawthorne	NY	CRF	CRF	CRF
North Shore University Hospital	Lake Success	NY	CRF	TED	TED
Steven and Alexandra Cohen Children's Medical Center of New York	New Hyde Park	NY	CRF	CRF	CRF
New York Presbyterian Hospital	New York	NY	CRF	CRF	CRF
Morgan Stanley Children's Hospital of New York	New York	NY	CRF	CRF	CRF
Mount Sinai Medical Center - New York	New York	NY	CRF	CRF	TED
New York Presbyterian Hospital / Columbia University Medical Center	New York	NY	CRF	CRF	CRF
New York University Langone Medical Center	New York	NY	TED	TED	TED
Memorial Sloan Kettering Cancer Center - Adults	New York	NY	CRF	TED	TED
Memorial Sloan Kettering Cancer Center - Pediatrics	New York	NY	CRF	TED	TED
University of Rochester Medical Center	Rochester	NY	CRF	CRF	CRF
Stony Brook University Hospital	Stony Brook	NY	TED	TED	CRF
State University of NY Upstate Medical University	Syracuse	NY	N/A	CRF	CRF
Akron Children's Hospital	Akron	OH	CRF	CRF	CRF
Jewish Hospital Blood and Marrow Transplant Center	Cincinnati	OH	CRF	CRF	CRF
Cincinnati Children's Hospital Medical Center	Cincinnati	OH	CRF	CRF	CRF
University of Cincinnati Medical Center	Cincinnati	OH	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
Cleveland Clinic	Cleveland	OH	CRF	CRF	CRF
Seidman Cancer Center - University Hospitals Cleveland Medical Center	Cleveland	OH	CRF	CRF	CRF
Nationwide Children's Hospital	Columbus	OH	CRF	CRF	CRF
The Ohio State University Medical Center	Columbus	OH	CRF	CRF	CRF
Oklahoma University Medical Center	Oklahoma City	OK	CRF	CRF	CRF
Oregon Health and Science University	Portland	OR	CRF	CRF	CRF
Providence Portland Medical Center	Portland	OR	N/A	N/A	CRF
Legacy Good Samaritan Hospital and Medical Center	Portland	OR	N/A	N/A	CRF
Pediatric BMT Program, Doernbecher Children's Hospital (OHSU)	Portland	OR	CRF	CRF	CRF
Geisinger Medical Center	Danville	PA	TED	TED	TED
Penn State Hershey Medical Center	Hershey	PA	CRF	CRF	CRF
Fox Chase Temple University Hospital Bone Marrow Transplant Program	Philadelphia	PA	CRF	TED	TED
Thomas Jefferson University	Philadelphia	PA	TED	TED	TED
Abramson Cancer Center University - Pennsylvania Medical Center	Philadelphia	PA	CRF	CRF	CRF
Hahnemann University Hospitals	Philadelphia	PA	CRF	TED	TED
Philadelphia Children's Hospital	Philadelphia	PA	TED	TED	N/A
St Christopher's Hospital for Children	Philadelphia	PA	TED	TED	TED
Eastern Regional Medical Center	Philadelphia	PA	N/A	CRF	CRF
University of Pittsburgh Medical Center	Pittsburgh	PA	CRF	CRF	N/A
West Penn Hospital	Pittsburgh	PA	CRF	CRF	CRF
UPMC Children's Hospital of Pittsburgh	Pittsburgh	PA	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
Roger Williams Medical Center	Providence	RI	CRF	TED	TED
Charleston Hematology Oncology	Charleston	SC	N/A	CRF	CRF
Medical University of South Carolina	Charleston	SC	CRF	TED	TED
Prisma Health - Upstate	Greenville	SC	CRF	CRF	CRF
Saint Francis Hospital- Greenville	Greenville	SC	CRF	CRF	CRF
Avera McKennan Transplant Institute	Sioux Falls	SD	CRF	CRF	CRF
St Jude Children's Research Hospital	Memphis	TN	CRF	TED	N/A
Baptist Blood and Marrow Transplant	Memphis	TN	TED	TED	TED
West Cancer Center / Methodist Healthcare Blood and Marrow Transplant Center	Memphis	TN	CRF	CRF	CRF
Vanderbilt University	Nashville	TN	CRF	TED	TED
Sarah Cannon BMT Center at Centennial Medical Center	Nashville	TN	CRF	TED	TED
Vanderbilt University Veterans Center	Nashville	TN	TED	TED	N/A
Texas Oncology	Amarillo	TX	CRF	TED	N/A
Children's Medical Center - Dallas	Dallas	TX	CRF	CRF	CRF
Medical City Dallas Hospital	Dallas	TX	CRF	CRF	CRF
UT Southwestern Medical Center - BMT Program	Dallas	TX	CRF	CRF	CRF
Baylor University Medical Center	Dallas	TX	CRF	CRF	CRF
Wilford Hall Medical Center	Fort Sam Houston	TX	TED	TED	TED
Cook Children's Medical Center	Fort Worth	TX	CRF	CRF	CRF
Baylor College of Medicine	Houston	TX	CRF	CRF	CRF
MD Anderson Cancer Center	Houston	TX	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
Texas Tech University Medical Center	Lubbock	TX	TED	TED	N/A
Covenant Health System Hematopoietic Transplant Program	Lubbock	TX	N/A	TED	TED
Texas Transplant Institute	San Antonio	TX	CRF	CRF	CRF
South Texas Veterans Health Care System	San Antonio	TX	N/A	CRF	CRF
The Children's Hospital of San Antonio	San Antonio	TX	CRF	CRF	CRF
Scott and White Memorial Hospital	Temple	TX	N/A	N/A	CRF
Latter Day Saints Hospital	Salt Lake City	UT	CRF	CRF	CRF
Utah Blood and Marrow Transplant Program-Adults	Salt Lake City	UT	CRF	CRF	CRF
Utah Blood and Marrow Transplant Program-Peds	Salt Lake City	UT	CRF	CRF	CRF
University of Virginia Health System	Charlottesville	VA	CRF	CRF	CRF
Fairfax-Northern Virginia Hospital	Falls Church	VA	CRF	CRF	CRF
Virginia Oncology Associates	Norfolk	VA	N/A	N/A	TED
Virginia Commonwealth University Massey Cancer Center BMT Program	Richmond	VA	CRF	CRF	CRF
University of Vermont Cancer Center	Burlington	VT	N/A	N/A	TED
Fred Hutchinson Cancer Research Center	Seattle	WA	CRF	CRF	CRF
VA Puget Sound Healthcare System	Seattle	WA	TED	TED	N/A
The Center for Blood Disorders and Stem Cell Transplantation	Seattle	WA	N/A	N/A	CRF
University of Wisconsin Hospital and Clinics	Madison	WI	CRF	CRF	CRF
Marshfield Clinic	Marshfield	WI	N/A	N/A	CRF
Froedtert & Medical College of Wisconsin	Milwaukee	WI	CRF	CRF	TED
Children's Wisconsin	Milwaukee	WI	CRF	CRF	CRF

Participating Center	City	State	MUD	RELATED	AUTO
Aurora St Luke's Medical Center	Milwaukee	WI	N/A	N/A	CRF
West Virginia University Medicine	Morgantown	WV	CRF	CRF	CRF

APPENDIX A2: INTERNATIONAL CENTERS

The following table lists the international centers that submited data to the CIBMTR Research Database for matched unrelated donor (MUD) allogeneic, related donor allogeneic, and autologous transplants in the past three years. Centers submit data at two levels: Centers submit data at two levels: TED level captures basic data, and CRF level captures more detail. MED-A forms are administered by EBMT and equivalent to CIBMTR TED forms.

Participating Center	City	Country	MUD	RELATED	AUTO
Institutos Medicos Antartidica	Buenos Aires	Argentina	CRF	CRF	CRF
Sanatorio Anchorena	Buenos Aires	Argentina	N/A	CRF	CRF
Hospital Privado de Cordoba	Cordoba	Argentina	CRF	CRF	CRF
Royal Adelaide Hospital	Adelaide	Australia	CRF	CRF	N/A
Royal Prince Alfred Hospital	Camperdown	Australia	CRF	CRF	TED
St Vincent's Hospital	Darlinghurst	Australia	TED	TED	N/A
Austin Health	Heidelberg	Australia	CRF	CRF	N/A
Royal Brisbane & Women's Hospital	Herston	Australia	CRF	CRF	N/A
Royal Children's Hospital	Melbourne	Australia	CRF	CRF	N/A
Alfred Hospital	Melbourne	Australia	TED	TED	N/A
Fiona Stanley Hospital	Perth	Australia	CRF	CRF	N/A
Princess Margaret Hospital for Children	Perth	Australia	CRF	CRF	TED
Sydney Children's Hospital	Randwick	Australia	CRF	CRF	TED
Queensland Children's Hospital	South Brisbane	Australia	CRF	CRF	CRF
Royal North Shore Hospital	St. Leonards	Australia	TED	TED	N/A
Royal Melbourne Hospital	Victoria	Australia	CRF	CRF	N/A
Children's Hospital at Westmead	Westmead	Australia	CRF	CRF	TED

Participating Center	City	Country	MUD	RELATED	AUTO
Medical University of Vienna	Vienna	Austria	TED	TED	N/A
Hospital Az Sint-Jan	Brugge	Belgium	TED	TED	N/A
Children's University Hospital	Bruxelles	Belgium	TED	TED	TED
University Hospital Antwerp	Edegem	Belgium	TED	TED	N/A
University Hospital Gasthuisberg	Leuven	Belgium	TED	TED	MED-A
Department of Hematology - Centre Hospitalier Universitaire - Liege	Liege	Belgium	MED-A	MED-A	MED-A
Hospital De Barretos	Barretos	Brazil	CRF	CRF	CRF
Instituto de Cardiologia do Distrito Federal - Unidade de TMO Pietro Albuquerque	Brasilia	Brazil	CRF	CRF	CRF
University Estadual De Campinas	Campinas	Brazil	CRF	CRF	CRF
Hospital De Clínicas Curitiba	Curitiba	Brazil	CRF	CRF	CRF
Centro de Pesquisas Oncológicas Dr. Alfredo Daura Jorge (CEPON)	Florianopolis	Brazil	CRF	CRF	TED
Federal University of Ceara	Fortaleza	Brazil	N/A	N/A	CRF
Hospital Amaral Carvalho	Jau	Brazil	CRF	CRF	CRF
Natal Hospital Center	Natal	Brazil	TED	TED	TED
Complexo Hospitalar de Niterói	Niterói	Brazil	TED	TED	TED
Hospital de Porto Alegre	Porto Alegre	Brazil	TED	TED	TED
Associação Hospitalar Moinhos de Vento	Porto Alegre	Brazil	N/A	TED	TED
Instituto Nacional de Câncer	Rio de Janeiro	Brazil	CRF	CRF	CRF
Universidad do São Paulo	São Paolo	Brazil	CRF	CRF	CRF
Instituto De Oncologia Pediatrica	São Paolo	Brazil	TED	TED	TED

Participating Center	City	Country	MUD	RELATED	AUTO
Hospital Sírio Libanês	São Paolo	Brazil	CRF	CRF	CRF
Instituto da Criança - Universidade de São Paulo	São Paolo	Brazil	N/A	TED	TED
Albert Einstein Hospital	São Paulo	Brazil	CRF	CRF	CRF
Hospital Samaritano	São Paulo	Brazil	CRF	CRF	CRF
IBCC - Instituto Brasileiro de Controle do Cancer	São Paulo	Brazil	CRF	CRF	CRF
Real e Benemérita Sociedade de Beneficiência Portuguesa de São Paulo	São Paulo	Brazil	TED	TED	TED
Hospital São Camilo	São Paulo	Brazil	TED	TED	TED
Bio Sana's São Camilo	São Paulo	Brazil	CRF	CRF	CRF
Hospital Leforte Liberdade	São Paulo	Brazil	CRF	CRF	CRF
Universidade Federal de São Paulo - Hospital São Paulo	São Paulo	Brazil	CRF	CRF	CRF
Tom Baker Cancer Centre	Calgary	Canada	TED	TED	TED
Alberta Children's Hospital	Calgary	Canada	CRF	CRF	CRF
Queen Elizabeth II Health Sciences Center	Halifax	Canada	TED	TED	TED
Hamilton Health Sciences - Juravinski Hospital and Cancer Centre	Hamilton	Canada	CRF	CRF	CRF
Kingston Health Sciences Centre	Kingston	Canada	N/A	N/A	TED
Montreal Children's Hospital	Montreal	Canada	TED	TED	TED
Centre Hospitalier Universitaire Sainte-Justine	Montreal	Canada	CRF	CRF	CRF
McGill University Health Centre-Royal Victoria Hospital-Glen Campus	Montreal	Canada	TED	TED	N/A
Maisonneuve-Rosemont Hospital	Montreal	Canada	TED	TED	N/A
Ottawa General Hospital	Ottawa	Canada	TED	TED	TED
Hotel-Dieu De Quebec	Quebec	Canada	TED	TED	TED

Participating Center	City	Country	MUD	RELATED	AUTO
CHA-Enfant-Jesus Hospital	Quebec City	Canada	TED	TED	TED
Saint John Regional Hospital	Saint John	Canada	N/A	N/A	TED
Health Sciences North	Sudbury	Canada	N/A	N/A	TED
Hospital for Sick Children	Toronto	Canada	TED	TED	TED
Princess Margaret Cancer Center - BMT Program	Toronto	Canada	TED	TED	TED
British Columbia Children's Hospital	Vancouver	Canada	CRF	CRF	CRF
Vancouver General Hospital	Vancouver	Canada	TED	TED	TED
CancerCare Manitoba/University of Manitoba	Winnipeg	Canada	CRF	CRF	CRF
Pontifica Universidad Catolica de Chile	Santiago	Chile	CRF	CRF	N/A
Instituto de Trasplante de Médula Ósea de la Costa	Barranquilla	Colombia	TED	TED	TED
Clinica de Marly	Bogotá	Colombia	CRF	CRF	CRF
Charles University Hospital - Pilsen	Pilsen	Czech Republic	CRF	CRF	CRF
Institute of Hem-Blood Transfusion	Praha	Czech Republic	TED	TED	N/A
University Hospital Motol	Praha	Czech Republic	TED	TED	N/A
University Hospital, Rigshospitalet	Copenhagen	Denmark	CRF	CRF	N/A
Children's Cancer Hospital - Egypt	Cairo	Egypt	N/A	TED	TED
Turku University Hospital	Turku	Finland	TED	TED	N/A
Centre Hospitalier Regional University D'Angers	Angers	France	TED	TED	N/A
Hopital Jean Minjoz	Besançon	France	CRF	CRF	N/A
Hospital A Michallon, CHU de Grenoble	Grenoble	France	MED-A	MED-A	N/A
Hopital Claude Huriez, Lille	Lille	France	MED-A	MED-A	N/A

Participating Center	City	Country	MUD	RELATED	AUTO
Centre Hospitalier Universitaire ARCHET 1	Nice	France	TED	TED	MED-A
Hospital Saint Louis	Paris	France	CRF	CRF	N/A
Centre Lyon Sud	Pierre Bénite Cedex	France	MED-A	MED-A	MED-A
Universitaetsklinikum Carl Gustav Carus	Dresden	Germany	CRF	CRF	CRF
University Hospital of Essen	Essen	Germany	CRF	CRF	CRF
Albert-Ludwig University - Freiburg	Freiburg	Germany	MED-A	MED-A	N/A
UKE Hamburg, Klinik und Poliklinik fur Stammzelltransplantation	Hamburg	Germany	CRF	CRF	N/A
Hannover Medical School	Hannover	Germany	MED-A	MED-A	N/A
Heidelberg University Clinic	Heidelberg	Germany	TED	TED	N/A
Klinikum Nuernberg, Einheit für Knochenmarktransplantation	Nuernberg	Germany	MED-A	MED-A	MED-A
Klinikum der Universitaet Regensburg	Regensburg	Germany	MED-A	MED-A	N/A
Universitats Klinikum Tubingen	Tubingen	Germany	TED	TED	N/A
Universitat Ulm - Adults	Ulm	Germany	CRF	CRF	TED
University Hospital of Patras, Patras University Medical Center	Rio Patras	Greece	TED	TED	TED
Chinese University of Hong Kong	Shatin	Hong Kong	TED	TED	N/A
Gujrat Cancer & Research Institute	Ahmedabad	India	CRF	CRF	CRF
SANKALP-CIMS Centre for Paediatric Bone Marrow Transplantation	Ahmedabad	India	N/A	CRF	CRF
People Tree Centre for Paediatric Bone Marrow Transplantation	Bangalore	India	N/A	CRF	N/A
Rajiv Gandhi Cancer Institute and Research Centre	Delhi	India	CRF	CRF	CRF
Asian Institute of Medical Sciences, Faridabad	Faridabad	India	CRF	CRF	CRF
Medanta - The Medicity Hospital	Gurgaon	India	CRF	CRF	CRF

Participating Center	City	Country	MUD	RELATED	AUTO
Sri Aurobindo Medical College	Indore	India	N/A	CRF	CRF
South East Asia Institute for Thalassemia	Jaipur	India	N/A	CRF	N/A
Tata Medical Center - Kolkata	Kolkata	India	CRF	CRF	CRF
Bone Marrow (Stem Cell) Transplant Unit, Christian Medical College, Ludhiana	Ludhiana	India	TED	TED	TED
Sahyadri Specialty Hospital	Pune	India	CRF	CRF	CRF
Deenanath Mangeshkar Hospital	Pune	India	CRF	CRF	CRF
Christian Medical College Hospital	Vellore	India	CRF	CRF	N/A
Rambam - Health Care Campus	Haifa	Israel	CRF	CRF	CRF
Haddasah University Hospital	Jerusalem	Israel	CRF	CRF	N/A
Davidoff Cancer Center, Rabin Medical Center, Beilinson Hospital	Petah Tikva	Israel	CRF	CRF	CRF
Tel-Aviv Sourasky Medical Center	Tel-Aviv	Israel	CRF	CRF	CRF
Sheba Medical Center	Tel-Hashomer	Israel	CRF	CRF	N/A
Sant' Orsola-Malpighi Hospital	Bologna	Italy	MED-A	MED-A	N/A
University Bologna - Pediatrics	Bologna	Italy	TED	TED	TED
Spedali Civili di Brescia	Brescia	Italy	N/A	N/A	MED-A
Ospedale Ferrarotto	Catania	Italy	CRF	CRF	TED
Policlinic Tor Vergata University - Rome Transplant Network	Rome	Italy	CRF	CRF	N/A
Universita Cattolica Sacro Cuore	Rome	Italy	TED	TED	TED
Ospedale Molinette	Torino	Italy	MED-A	MED-A	N/A
Samsung Medical Center	Seoul	Korea (South)	CRF	CRF	CRF
Instituto Nacional de Ciencias Medicas y Nutricion Salvador Zubiran	Ciudad de Mexico	Mexico	CRF	CRF	CRF

Participating Center	City	Country	MUD	RELATED	AUTO
Institute Nacional de Pediatria	Coyoacan	Mexico	CRF	CRF	CRF
Hospital Universitario Dr. José Eleuterio González, Universidad Autónoma de Nuevo León	Monterrey	Mexico	CRF	CRF	CRF
Centro de Hematologia y Medicina Interna Clinica RUIZ de Puebla	Puebla	Mexico	TED	TED	TED
VU Medical Center - Amsterdam	Amsterdam	Netherlands	TED	TED	TED
Leiden University Medical Center	Leiden	Netherlands	CRF	TED	N/A
Academic Hospital Maastricht	Maastricht	Netherlands	CRF	TED	N/A
Radboud University Medical Center	Nijmegen	Netherlands	TED	TED	N/A
Erasmus MC - Daniel den Hoed Cancer Center	Rotterdam	Netherlands	TED	TED	N/A
University Hospital Utrecht	Utrecht	Netherlands	TED	N/A	N/A
University Medical Center Utrecht - Pediatrics	Utrecht	Netherlands	CRF	N/A	N/A
Auckland City Hospital	Auckland	New Zealand	CRF	CRF	N/A
Starship Children's Hospital	Auckland	New Zealand	CRF	CRF	N/A
Christchurch Hospital	Christchurch	New Zealand	CRF	CRF	TED
Wellington Blood and Cancer Centre	Wellington	New Zealand	CRF	CRF	N/A
Rikshospitalet - The National Hospital	Oslo	Norway	CRF	N/A	N/A
Shifa International Hospitals	Islamabad	Pakistan	N/A	CRF	CRF
Armed Forces Bone Marrow Transplant Center	Rawalpindi	Pakistan	N/A	CRF	CRF
Hospital Rebagliati	Lima	Peru	CRF	CRF	CRF
Silesian Medical Academy	Katowice	Poland	CRF	CRF	N/A
Poznan University of Medical Sciences	Poznan	Poland	MED-A	MED-A	N/A

Participating Center	City	Country	MUD	RELATED	AUTO
Lower-Silesian Center for Cellular Transplantation and National Bone Marrow Donor Registry	Wroclaw	Poland	CRF	CRF	N/A
Institute Portugues de Oncologia - Porto	Porto	Portugal	MED-A	MED-A	MED-A
King Faisal Specialist Hospital & Research Center - Adults	Riyadh	Saudi Arabia	CRF	CRF	N/A
King Faisal Specialist Hospital - Peds	Riyadh	Saudi Arabia	CRF	CRF	N/A
King Abdulaziz Medical City-Riyadh	Riyadh	Saudi Arabia	N/A	CRF	CRF
King Abdullah Specialist Children's Hospital (KASCH)	Riyadh	Saudi Arabia	CRF	CRF	CRF
King Fahad Medical City	Riyadh	Saudi Arabia	CRF	CRF	CRF
National University Health System - Adults	Singapore	Singapore	CRF	CRF	CRF
Singapore General Hospital	Singapore	Singapore	CRF	CRF	CRF
National University Health System - Peds	Singapore	Singapore	CRF	CRF	CRF
Slovak Medical University	Bratislava	Slovak Republic	TED	TED	N/A
University Barcelona	Barcelona	Spain	TED	TED	N/A
Hospital Infantil Vall d'Hebron	Barcelona	Spain	MED-A	MED-A	MED-A
Institut Català d'Oncologia-IDIBELL	L'Hospitalet	Spain	CRF	CRF	MED-A
Hospital Puerta Hierro	Madrid	Spain	MED-A	MED-A	N/A
Gregorio Maranon University General Hospital	Madrid	Spain	CRF	CRF	CRF
Hospital Universitario La Fe	Valencia	Spain	CRF	CRF	N/A
Sahlgrenska University Hospital	Gothenborg	Sweden	CRF	CRF	N/A
University Hospital of Lund	Lund	Sweden	CRF	MED-A	N/A
Karolinska University Hospital, Dept. of Cellular Therapy and Allogeneic Stem Cell Transplantation	Stockholm	Sweden	CRF	CRF	N/A

Participating Center	City	Country	MUD	RELATED	AUTO
University Hospital - Uppsala	Uppsala	Sweden	CRF	CRF	N/A
Basel Kantonsspital	Basel	Switzerland	CRF	CRF	N/A
Geneva University Hospitals	Geneva	Switzerland	MED-A	MED-A	N/A
University Hospital-Zurich	Zurich	Switzerland	MED-A	MED-A	N/A
Medical Park Hospital - Antalya	Antalya	Turkey	CRF	CRF	CRF
Queen Elizabeth Hospital-Birmingham	Birmingham	United Kingdom	CRF	CRF	N/A
Birmingham Heartlands Hospital	Birmingham	United Kingdom	TED	TED	TED
Birmingham Children's Hospital	Birmingham	United Kingdom	TED	TED	N/A
Bristol Children's Hospital	Bristol	United Kingdom	CRF	CRF	N/A
Western General Hospitals	Edinburgh	United Kingdom	TED	TED	TED
Beatson West of Scotland Cancer Centre	Glasgow	United Kingdom	MED-A	MED-A	N/A
Royal Hospital for Sick Children	Glasgow	United Kingdom	CRF	CRF	N/A
St James University Hospital	Leeds	United Kingdom	TED	TED	N/A
Imperial College- St Mary's Hospital	London	United Kingdom	CRF	CRF	N/A
Haematology & Cancer Services - Newcastle	Newcastle	United Kingdom	CRF	CRF	TED
British Hospital	Montevideo	Uruguay	N/A	CRF	CRF
Centro de Trasplante del Servicio Medico Integral (SMI)	Montevideo	Uruguay	CRF	CRF	CRF
Hospital Maciel	Montevideo	Uruguay	CRF	CRF	CRF
Ciudad Hospitalaraia Dr Enrique Tejera	Valencia	Venezuela	TED	TED	TED

APPENDIX B: COORDINATING CENTER LEADERSHIP

SENIOR LEADERSHIP

Mary M. Horowitz, MD, MS

- Chief Scientific Director
- Principal Investigator for the Data and Coordinating Center of the BMT CTN
- Research Director for the SCTOD
- Robert A. Uihlein Professor of Hematologic Research at MCW

C. Randy Mills, PhD

- Chief Executive Officer for NMDP/Be The Match
- Executive Director for the CIBMTR Affiliation Board

Steven Devine, MD

- Associate Scientific Director
- Chief Medical Officer, NMDP/Be The Match
- Co-Scientific Director of the RCI BMT

India Hook-Barnard, PhD

- Senior Vice President, Patient Outcomes and Experience, NMDP/Be The Match

Mei-Jie Zhang, PhD

- Chief Statistical Director
- Biostatistician for the Acute Leukemia and Graft Sources and Manipulation Working Committees
- Professor of Biostatistics at MCW

Mary Eapen, MBBS, MS

- Senior Scientific Director of Research Operations
- Scientific Director for the Graft Sources and Manipulation, Pediatric Cancer, and Non-Malignant Diseases Working Committees
- Protocol Officer for several BMT CTN trials
- Professor of Medicine at MCW

Kathryn E. Flynn, PhD

- Senior Scientific Director of Patient-Reported Outcomes
- Associate Professor of Medicine at MCW

J. Douglas Rizzo, MD, MS

- Senior Scientific Director and Principal Investigator of the SCTOD
- Professor of Medicine at MCW
- Associate Director of Clinical Operations for the Froedtert and MCW Cancer Center

Bronwen Shaw MD, PhD

- Senior Scientific Director of Data Operations
- Co-Scientific Director for the RCI BMT
- Scientific Director for the Late Effects and Quality of Life Working Committee as well as the Donor Health and Safety Working Committee
- Professor of Medicine at MCW

Marcelo Pasquini, MD, MS

- Cellular Therapy Registry Director and Principal Investigator of the CIDR
- Scientific Director for the Cellular Immunotherapy for Cancer and Regimen-Related Toxicity and Supportive Care Working Committees
- Protocol Officer and Director of Medical Monitors for the BMT CTN
- CIBMTR Representative to the WBMT
- Associate Professor of Medicine at MCW

Martin Maiers, MS

- Vice President of Biomedical Informatics
- Staff liaison to the Immunobiology Steering Committee
- Co-Chair of Informatics: International Histocompatibility and Immunogenetics Workshop
- Member of World Health Organization HLA Nomenclature Committee

Roberta King, MPH

- Vice President for CIBMTR Minneapolis
- Staff Liaison to the NMDP/Be The Match Donor and Patient Safety Monitoring Advisory Group

Patricia Steinert, PhD, MBA

- Executive Director for CIBMTR Milwaukee

SCIENTIFIC DIRECTORS

Mukta Arora, MD, MBBS, MS

- Co-Scientific Director of the Graft-versus-Host Disease Working Committee
- Associate Professor of Medicine at the University of Minnesota

Saurabh Chhabra, MD, MS

- Assistant Scientific Director for the Regimen-Related Toxicity and Supportive Care Working Committee
- Associate Professor of Medicine at MCW

Anita D'Souza, MD

- Assistant Scientific Director for the Plasma Cell Disorders and Adult Solid Tumors Working Committee
- Associate Professor of Medicine at MCW

Parameswaran Hari, MD, MS

- Scientific Director for the Plasma Cell Disorders and Adult Solid Tumors Working Committee
- Armand J. Quick – William F. Stapp Professor of Hematology at MCW
- Division Chief of Hematology and Oncology at MCW

Mehdi Hamadani, MD

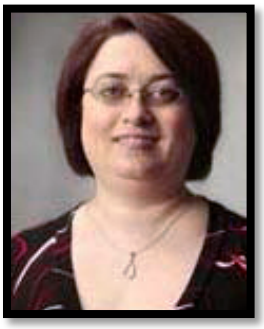
- Scientific Director for the Lymphoma Working Committee
- Director of Adult Blood and Marrow Transplantation Program at MCW
- Professor of Internal Medicine at MCW

Stephanie J. Lee, MD, MPH

- Senior Scientific Director of Immunobiology Research
- Co-Scientific Director for the Immunobiology Working Committee
- Professor of Medicine at the University of Washington

Rachel Phelan, MD, MPH

- Assistant Scientific Director for the Late Effects and Quality of Life Working Committee
- Assistant Professor of Pediatrics in the Division of Hematology / Oncology / BMT at MCW
- Director of the Next Steps Survivorship Program at Children's Wisconsin

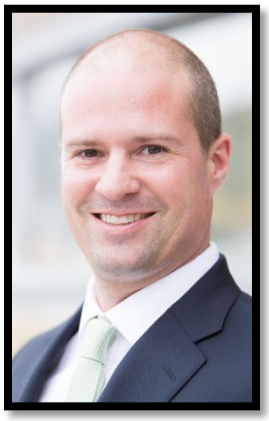


Marcie Riches, MD, MS

- Scientific Director for the Infection and Immune Reconstitution Working Committee
- Protocol Officer and Medical Monitor for several BMT CTN trials
- Clinical Associate Chief of the Division of Hematology and Oncology at the University of North Carolina at Chapel Hill
- Clinical Medical Director of the BMT Clinic at the University of North Carolina at Chapel Hill
- Clinical Associate Professor Director of Clinical Research and Data Quality, BMT at the University of North Carolina at Chapel Hill

Wael Saber, MD, MS

- Scientific Director for the Chronic Leukemia and Health Services and International Studies Working Committees
- Assistant Scientific Director for the Acute Leukemia Working Committee
- Associate Professor of Medicine at MCW



Stephen Spellman, MBS

- Co-Scientific Director for the Graft-versus-Host Disease and Immunobiology Working Committees
- Director of Immunobiology Research
- Principal Investigator for the Research Repository
- Staff liaison to the Immunobiology Steering Committee
- Program Manager for the NMDP/Be The Match Office of Naval Research Grant
- Graduate faculty in the University of Minnesota Bioinformatics and Computational Biology program

Daniel Weisdorf, MD

- Senior Research Advisor
- Scientific Director for the Acute Leukemia Working Committee
- President of the WBMT
- Professor of Medicine at the University of Minnesota
- Program Co-Leader of Transplant Biology and Therapy at the Masonic Cancer Center at the University of Minnesota
- Deputy Director of the Clinical and Translational Science Institute at the University of Minnesota

STATISTICAL DIRECTORS

Kwang Woo Ahn, PhD

- Statistical Director for the Chronic Leukemia, Lymphoma, and Pediatric Cancer Working Committees
- Associate Professor of Biostatistics at MCW
- Graduate Program Director at MCW

Ruta Brazauskas, PhD

- Statistical Director for the Cellular Immunotherapy for Cancer, Health Services and International Studies, and Late Effects and Quality of Life Working Committees
- Associate Professor of Biostatistics at MCW

Raphael Fraser, PhD

- Statistical Director for the Plasma Cell Disorders and Adult Solid Tumors Working Committee and the BMT CTN
- Assistant Professor of Biostatistics at MCW

Soyoung Kim, PhD

- Statistical Director for the Chronic Leukemia, Infection and Immune Reconstitution, and Non-Malignant Diseases Working Committees
- Assistant Professor of Biostatistics at MCW

Brent Logan, PhD

- Statistical Director for the Donor Health and Safety and the Regimen-Related Toxicity and Supportive Care Working Committees
- Lead Statistician for the BMT CTN and Statistical Consultant to the NMDP/Be The Match
- Professor of Biostatistics at MCW
- Director of the Division of Biostatistics at MCW

Tao Wang, PhD

- Statistical Director for the Graft-versus-Host Disease and Immunobiology Working Committees
- Associate Professor of Biostatistics at MCW

OTHER LEADERSHIP STAFF

Erik Bergman, MBA, MS

- Director of IT for CIBMTR Milwaukee
- Oversees management of the Research Database, including extraction of data from source systems as well as their transformation and loading to the database
- Responsible for data retrievals from the Research Database as well as key solutions for sharing data with stakeholders

Janet Brunner-Grady, PA-C

- Program Director of Data Operations
- Manages the clinical research coordinators, develops training programs, and monitors center CPI
- Assists clinical research coordinators on both campuses with clinical transplant-related questions

Sharniece Covill

- Director of Business Operations for CIBMTR Milwaukee
- Oversees the deliverables and milestones associated with the full portfolio of CIBMTR projects

Sherry Fisher

- Director of Advancement for CIBMTR Milwaukee
- Manages the advancement activities to generate continued non-federal financial support
- Oversees meetings and communications activities, including the annual TCT Meetings

Erin Leckrone

- Director of Prospective Research
- Manages the activities of the RCI BMT, including the Survey Research Group
- Oversees the administration of the Clinical Trials Advisory Committee

Waleska S. Pérez, MPH

- Program Director of Statistical Operations and Clinical Outcomes Research
- Oversees Master's-level statisticians
- Provides administrative oversight of the Clinical Outcomes Research Program

Matt Prestegaard

- Director of IT for CIBMTR Minneapolis
- Develops IT systems in the areas of searching and matching, case management, international communications, and research

Guillermo Vazquez-Toro, PhD

- Director of the Corporate Office for CIBMTR Milwaukee
- Directs acquisition of corporate opportunities
- Provides oversight of the Corporate Project portfolio

APPENDIX C: COMMITTEE MEMBERSHIP

CIBMTR committees provide input and advice to the leadership team, ensuring the continued support of both the needs and priorities of its scientific and medical communities. All committees and their functions are listed in **Table 1.2**.

APPENDIX C1: ADVISORY COMMITTEE MEMBERSHIP

The Advisory Committee provides oversight for CIBMTR policies and scientific agenda and also partners with the Working Committees to prioritize scientific studies. Members are elected to three-year terms by the CIBMTR Assembly and must be from qualifying CRF centers. All Advisory Committee terms begin on March 1.

ELECTED MEMBERS

Chair	Rob Soiffer, MD, Dana Farber Cancer Institute, Boston, MA
Chair Elect	John Wingard, MD, Shands HealthCare & University of Florida, Gainesville, FL

VICE CHAIRS

North America	Marcos de Lima, MD, Sideman Cancer Center-University Hospitals Cleveland Medical Center, Cleveland, OH
Europe	Katarina Le Blanc, MD, PhD, Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation, Stockholm, Sweden
Central / South America	Gregorio Jaimovich, MD, Sanatorio Anchorena, Caba, Argentina, and Hospital Universitario Fundacion Favaloro, Buenos Aires, Argentina
Asia / Africa / Australia	Biju George, MBBS, MD, Christian Medical College Hospital, Vellore, India

MEMBERS AT LARGE

North America	Edwin Alyea, MD, Dana Farber Cancer Institute, Boston, MA
	Michael Bishop, MD, University of Chicago Medicine, Chicago, IL
	Claudio Brunstein, MD, PhD, University of Minnesota Blood and Marrow Transplant Program, Minneapolis, MN
	Heather Landau, MD, Memorial Sloan Kettering Cancer Center, New York, N
	Steven Pavletic, MD, MS, NIH-NCI Experimental Transplantation and Immunology Branch, Bethesda, MD
	Bart Scott, MD, Fred Hutchinson Cancer Research Center, Seattle, WA
Non-North America	Mahmoud Aljurf, MD, King Faisal Specialist Hospital & Research Center, Riyadh, Saudi Arabia
	Carmem Sales Bonfim, MD, PhD, Hospital de Clinicas – UFPR, Curitiba, Brazil

Shahrukh Hashmi, MD, MPH, King Faisal Specialist Hospital & Research Center, Riyadh, Saudi Arabia

Steven Marsh, BSc, PhD, ARCS, Anthony Nolan Research Institute, London, England

Tracey O'Brien, MBA, LLM, MBChB, BSc, Sydney Children's Hospital, Sydney, Australia

Anna Sureda, MD, PhD, Institut Català d'Oncologia-Hospital Duran I Reynals, Barcelona, Spain

APPOINTED MEMBERS

ASTCT Representative	Navneet Majhail, MD, MS, Cleveland Clinic Foundation, Cleveland, OH
Bioethicist	Steven Joffe, MD, MPH, Abramson Cancer Center University of Pennsylvania Medical Center, Philadelphia, PA
Business Representative	Jeff Chell, MD, NMDP/Be The Match, Minneapolis, MN
CIDR Executive Committee Representative	Miguel-Angel Perales, Memorial Sloan Kettering Cancer Center, New York, NY
Collection Center Representative	James Mason, MD, Scripps Blood & Marrow Transplant Program, La Jolla, CA
Cord Blood Bank Representative	Andromachi Scaradavou, MD, New York Blood Center, Long Island City, NY
Donor Center Representative	Susan Rossman, MD, PhD, Gulf Coast Marrow Donor Center, Houston, TX
Patient / Family Representatives (Co-Chairs, Consumer Advocacy Committee)	Jack Aiello, MS, San Jose, CA Hilary Hall, Cambridge, MA

EX OFFICIO MEMBERS

Executive Director	Randy Mills, PhD, NMDP/Be The Match, Minneapolis, MN
Chief Scientific Director	Mary M. Horowitz, MD, MS, CIBMTR, Milwaukee, WI
Chief Statistical Director	Mei-Jie Zhang, PhD, CIBMTR, Milwaukee, WI
Senior Scientific Director SCTOD	J. Douglas Rizzo, MD, MS, CIBMTR, Milwaukee, WI
Senior Vice President and Associate Scientific Director CIBMTR Minneapolis	Steven Devine, MD, CIBMTR, Minneapolis, MN
Vice President CIBMTR Minneapolis	Roberta King, MPH, CIBMTR, Minneapolis, MN
Senior Administrator CIBMTR Milwaukee	Patricia Steinert, PhD, MBA, CIBMTR, Milwaukee, WI

Senior Manager, Data and Program Evaluation, Patient Services	Meggan McCann, MPH, NMDP/Be The Match, Minneapolis, MN
Senior Research Advisor	Daniel Weisdorf, MD, CIBMTR, Minneapolis, MN
NMDP/Be The Match / MCW / HRSA Contracting Officer Representative	Nawraz Shawir, MBBS
NMDP/Be The Match / Navy Project Officer	Robert Hartzman, MD, Capt. MC, USN (ret)
MCW / HRSA Contracting Officer Representatives	Janet Kuramoto-Crawford, PhD, MHS Marilyn Levi, MD
MCW / NCI Project Officer	William Merritt, PhD, Bethesda, MD
MCW / NHLBI Project Officers	Nancy DiFronzo, PhD, Bethesda, MD Nahed El Kassar, MD, PhD, Bethesda, MD
MCW / NIAID Project Officer	Linda Griffith, MD, PhD, Bethesda, MD
Nominating Committee Chair	Corey Cutler, MD, MPH, Dana Farber Cancer Institute, Boston, MA

APPENDIX C2: EXECUTIVE COMMITTEE MEMBERSHIP

The Executive Committee, a subcommittee of the Advisory Committee, ensures that the organization carries out its mission and adheres to CIBMTR policies and procedures. It also provides advice and counsel to the Coordinating Center between meetings of the Advisory Committee. Specifically, the Executive Committee is responsible for providing scientific and policy advice to the Chief Scientific Director and Coordinating Center, reviewing audit results and making recommendations for improvement, and appointing a CIBMTR Co-Chair and additional CIBMTR representatives to the scientific organizing committee for the annual TCT Meetings. All Executive Committee terms begin on March 1.

ELECTED MEMBERS

Chair	Rob Soiffer, MD, Dana Farber Cancer Institute, Boston, MA
Chair Elect	John Wingard, MD, Shands HealthCare & University of Florida, Gainesville, FL

VICE CHAIRS

North America	Marcos de Lima, MD, Seidman Cancer Center-University Hospitals Cleveland Medical Center
Europe	Katarina Le Blanc, MD, PhD, Karolinska University Hospital, Centre for Allogeneic Stem Cell Transplantation, Stockholm, Sweden
Central / South America	Gregorio Jaimovich, MD, Sanatorio Anchorena, Caba, Argentina and Hospital Universitario Fundacion Favaloro, Buenos Aires, Argentina
Asia / Africa / Australia	Biju George, MBBS, MD, Christian Medical College Hospital, Vellore, India

APPOINTED MEMBERS

ASTCT Representative	Navneet Majhail, MD, MS, Cleveland Clinic Foundation, Cleveland, OH
Bioethicist	Steven Joffe, MD, MPH, Abramson Cancer Center University of Pennsylvania Medical Center, Philadelphia, PA
Business Representative	Jeff Chell, MD, NMDP/Be The Match, Minneapolis, MN
CIDR Executive Committee Representative	Miguel-Angel Perales, MD, Memorial Sloan Kettering Cancer Center, New York, NY
Collection Center Representative	James Mason, MD, Scripps Blood and Marrow Transplant Program, San Antonio, TX
Cord Blood Bank Representative	Andromachi Scaradavou, MD, New York Blood Center, Long Island City, NY
Donor Center Representative	Susan Rossmann, MD, PhD, Gulf Coast Marrow Donor Center, Houston, TX

Patient / Family Representatives (Co-Chairs, Consumer Advocacy Committee)	Jack Aiello, MS, San Jose, CA Hilary Hall, Cambridge, MA

EX OFFICIO MEMBERS

Executive Director	Randy Mills, PhD, NMDP/Be The Match, Minneapolis, MN
Chief Scientific Director	Mary M. Horowitz, MD, MS, CIBMTR, Milwaukee, WI
Chief Statistical Director	Mei-Jie Zhang, PhD, CIBMTR, Milwaukee, WI
Senior Scientific Director SCTOD	J. Douglas Rizzo, MD, MS, CIBMTR, Milwaukee, WI
Senior Vice President and Associate Scientific Director CIBMTR Minneapolis	Steven Devine, MD, CIBMTR, Minneapolis, MN
Vice President CIBMTR Minneapolis	Roberta King, MPH, CIBMTR, Minneapolis, MN
Executive Director CIBMTR Milwaukee	Patricia Steinert, PhD, MBA, CIBMTR, Milwaukee, WI
Senior Manager, Data and Program Evaluation, Patient Services	Meggan McCann, MPH, NMDP/Be The Match, Minneapolis, MN
Senior Research Advisor	Daniel Weisdorf, MD, CIBMTR, Minneapolis, MN
NMDP/Be The Match / MCW / HRSA Contracting Officer Representative	Nawraz Shawir, MBBS
NMDP/Be The Match / Navy Project Officer	Robert Hartzman, MD, Capt. MC, USN (ret)
MCW / HRSA Contracting Officer Representatives	Janet Kuramoto-Crawford, PhD, MHS Marilyn Levi, MD
MCW / NCI Project Officer	William Merritt, PhD, Bethesda, MD
MCW / NHLBI Project Officers	Nancy DiFronzo, PhD, Bethesda, MD Nahed El Kassar, MD, PhD, Bethesda, MD
MCW / NIAID Project Officer	Linda Griffith, MD, PhD, Bethesda, MD
Nominating Committee Chair	Corey Cutler, MD, MPH, Dana Farber Cancer Institute, Boston, MA

APPENDIX C3: CONSUMER ADVOCACY COMMITTEE MEMBERSHIP

The Consumer Advocacy Committee communicates research results and data to the non-medical community and provides patient and donor perspectives during the development of the CIBMTR research agenda. Many committee members have personal experience as a donor, recipient, or family member of a recipient.

CHAIRS

Jack Aiello, MS, San Jose, CA

Hilary Hall, Cambridge, MA

MEMBERS

Nicole Adams, San Angelo, TX

Maureen Beaman, MBA, Milwaukee, WI

Jeffery Haertling, Tierra Verde, FL

Kristin Scheeler, Leukemia and Lymphoma Society, White Plains, NY

Bryce Waldman, Chicago, IL

Jennifer Wilder, National Institutes of Health, Bethesda, MD

SCIENTIFIC DIRECTOR

J. Douglas Rizzo, MD, MS, CIBMTR, Milwaukee, WI

EX OFFICIO MEMBERS

Dennis Confer, MD, NMDP/Be The Math, Minneapolis, MN

Carol Doleysh, (CIBMTR liaison) CIBMTR, Milwaukee, WI

Mary Horowitz, MD, MS, CIBMTR, Milwaukee, WI

Janet Kuramoto-Crawford, PhD, MHS, Health Resources and Services Administration, Rockville, MD

Marilyn Levi, MD, Health Resources and Services Administration, Rockville, MD

Jennifer Motl, CIBMTR, Milwaukee, WI

Meggan McCann, MPH, (NMDP/Be The Match liaison) NMDP/Be The Match, Minneapolis, MN

Bronwen Shaw, MD, PhD, CIBMTR, Milwaukee, WI

Nawraz Shawir, MBBS, Health Resources and Services Administration, Rockville, MD

Patricia Steinert, PhD, MBA, CIBMTR, Milwaukee, WI

APPENDIX C4: NOMINATING COMMITTEE MEMBERSHIP

The Nominating Committee consists of six members elected by the CIBMTR Assembly. Following an annual call for nominations, the Nominating Committee prepares a slate of candidates for open positions on the Advisory, Nominating, and Clinical Trials Advisory Committees. Elections are held annually by confidential electronic ballot. The Nominating Committee also makes recommendations to the Advisory Committee for open Working Committee Chair and other leadership appointments after seeking recommendations from the CIBMTR Assembly, Advisory Committee, and incumbent Working Committee Chairs. All terms begin on March 1.

CHAIR

Corey Cutler, MD, MPH, Dana Farber Cancer Institute, Boston, MA

MEMBERS

Mitchell Horwitz, MD, Duke University Medical Center, Durham, NC

Richard Maziarz, MD, Oregon Health and Science University, Portland, OR

Steven Pavletic, MD, MS, NIH - NCI Experimental Transplantation and Immunology Branch, Bethesda, MD

Carlos Ramos, MD, Baylor College of Medicine – Center for Cell & Gene Therapy, Houston, TX

Michael Verneris, MD, University of Colorado – Children's Hospital, Aurora, CO

APPENDIX C5: SCIENTIFIC WORKING COMMITTEE LEADERSHIP

For information on Scientific Working Committee structure and organization, see **Section 1.3.1**. For information on Working Committee studies and their accomplishments, see **Section 2.1.1**.

ACUTE LEUKEMIA WORKING COMMITTEE

Chairs	Brenda Sandmaier, MD, Fred Hutchinson Cancer Research Center
	Mark Litzow, MD, Mayo Clinic Rochester
	Partow Kebriaei, MD, MD Anderson Cancer Center
Scientific Director	Daniel Weisdorf, MD, CIBMTR
Asst Scientific Dir	Wael Saber, MD, MS, CIBMTR
Statistical Director	Mei-Jie Zhang, PhD, CIBMTR
Statistician	Karen Chen, MS, CIBMTR

CELLULAR IMMUNOTHERAPY FOR CANCER WORKING COMMITTEE

Chairs	Sarah Nikiforow, MD, PhD, Dana Farber Cancer Institute
	Peiman Hematti, MD, University of Wisconsin Hospital and Clinics
Scientific Director	Marcelo Pasquini, MD, MS, CIBMTR
Statistical Director	Ruta Brazauskas, PhD, CIBMTR
Consumer Adv Rep	Hilary Hall
Statistician	Daniel Klaver, MPH, CIBMTR

CHRONIC LEUKEMIA WORKING COMMITTEE

Chairs	Ronald Sobecks, MD, Cleveland Clinic Foundation
	Bart Scott, MD, Fred Hutchinson Cancer Research Center
	Ryotaro Nakamura, MD, City of Hope
Scientific Director	Wael Saber, MD, MS, CIBMTR
Statistical Director	Kwang Woo Ahn, PhD, CIBMTR
Statistician	Noel Estrada-Merly, MPH, CIBMTR

DONOR HEALTH AND SAFETY WORKING COMMITTEE

Chairs	Nirali Shah, MD, MHSc, National Cancer Institute
	Galen Switzer, PhD, University of Pittsburgh Medical Center
	Jack Hsu, MD, Shands HealthCare & University of Florida
Scientific Director	Bronwen Shaw, MD, PhD, CIBMTR
Ex Officio Sr Advisor	Dennis Confer, MD, CIBMTR
Statistical Director	Brent Logan, PhD, CIBMTR
Consumer Adv Reps	Jeffrey Haertling
	Maureen Beaman
Statistician	Stephanie Bo-Subait, MPH, CIBMTR

GRAFT SOURCES AND MANIPULATION WORKING COMMITTEE

Chairs	Asad Bashey, MD, PhD, The Blood and Marrow Transplant Program at Northside Hospital
	Ian McNiece, PhD, MD Anderson Cancer Center
	Claudio Brunstein, MD, PhD, University of Minnesota
Scientific Director	Mary Eapen, MD, MS, CIBMTR
Statistical Director	Mei-Jie Zhang, PhD, CIBMTR
Statistician	Mariam Johnson, MPH, CIBMTR

GRAFT-VERSUS-HOST DISEASE WORKING COMMITTEE

Chairs	Joseph Pidala, MD, PhD, H. Lee Moffitt Cancer Center and Research Institute
	Madan Jagasia, MBBS, MS, Vanderbilt University Medical Center
	Margaret MacMillan, MD, MSc, University of Minnesota
Scientific Directors	Mukta Arora, MD, MS, CIBMTR
	Stephen Spellman, MBS, CIBMTR
Statistical Director	Tao Wang, PhD, CIBMTR
Consumer Adv Reps	Kristin Scheeler, Leukemia & Lymphoma Society
	Jennifer Wilder, BSN, RN, National Institutes of Health
Statistician	Karen Chen, MS, CIBMTR

HEALTH SERVICES AND INTERNATIONAL STUDIES WORKING COMMITTEE

Chairs	Nandita Khera, MD, Mayo Clinic Arizona and Phoenix Children's Hospital
	William Wood, MD, MPH, University of North Carolina Hospitals
	Shahrukh Hashmi, MD, MPH, King Faisal Specialist Hospital and Research Center
Scientific Director	Wael Saber, MD, MS, CIBMTR
Statistical Director	Ruta Brazauskas, PhD, CIBMTR
Consumer Adv Rep	Jack Aiello, MS
Statistician	Naya He, MPH, CIBMTR

IMMUNOBIOLOGY WORKING COMMITTEE

Chairs	Katharine Hsu, MD, PhD, Memorial Sloan Kettering Cancer Center
	Sophie Paczesny, MD, PhD, Indiana University Hospital / Riley Hospital for Children
	Steven Marsh, BSc, PhD, ARCS, Anthony Nolan Research Institute
Scientific Directors	Stephanie J. Lee, MD, MPH, CIBMTR, Fred Hutchinson Cancer Research Center
	Stephen Spellman, MBS, CIBMTR
Statistical Director	Tao Wang, PhD, CIBMTR
Statistician	Michelle Kuxhausen, MS, CIBMTR

INFECTION AND IMMUNE RECONSTITUTION WORKING COMMITTEE

Chairs	Krishna Komanduri, MD, University of Miami
	Miguel-Angel Perales, MD, Memorial Sloan Kettering Cancer Center
	Roy Chemaly, MD, MPH, MD Anderson Cancer Center
Scientific Director	Marcie Riches, MD, MS, CIBMTR, University of North Carolina Hospitals
Statistical Director	Soyoung Kim, PhD, CIBMTR
Statistician	Naya He, MPH, CIBMTR

LATE EFFECTS AND QUALITY OF LIFE WORKING COMMITTEE

Chairs	Minoo Battiwalla, MD, MS, Sarah Cannon Research Institute
	David Buchbinder, MD, Children's Hospital of Orange County
	Betty Hamilton, MD, Cleveland Clinic Foundation
Scientific Director	Bronwen Shaw, MD, PhD
Asst Scientific Dir	Rachel Phelan, MD, CIBMTR
Statistical Director	Ruta Brazauskas, PhD, CIBMTR
Consumer Adv Reps	Hilary Hall
	Jim Omel
Statistician	Stephanie Bo-Subait, MPH, CIBMTR

LYMPHOMA WORKING COMMITTEE

Chairs	Timothy Fenske, MD, MS, Froedtert Memorial Lutheran Hospital
	Mohamed Kharfan-Dabaja, MD, MBA, Mayo Clinic Florida
	Craig Sauter, MD, Memorial Sloan Kettering Cancer Center
Scientific Director	Mehdi Hamadani, MD, CIBMTR
Statistical Director	Kwang Woo Ahn, PhD, CIBMTR
Statistician	Carlos Litovich, MPH, CIBMTR

NON-MALIGNANT DISEASES WORKING COMMITTEE

Chairs	Vikram Mathews, MD, Christian Medical College Hospital
	Christopher Dvorak, MD, University of California San Francisco Medical Center
	Andrew Gennery, MD, Newcastle General Hospital / The Royal Victoria Infirmary
	George Georges, MD, Fred Hutchinson Cancer Research Center
Scientific Director	Mary Eapen, MD, MS, CIBMTR
Statistical Director	Soyoung Kim, PhD, CIBMTR
Statistician	Kyle Hebert, MS, CIBMTR

PEDIATRIC CANCER WORKING COMMITTEE

Chairs	Angela Smith, MD, MS, University of Minnesota Medical Center
	Gregory Yanik, MD, The University of Michigan
	Muna Qayed, MD, MSc, Children's Healthcare of Atlanta at Egleston
Scientific Director	Mary Eapen, MD, MS, CIBMTR
Statistical Director	Soyoung Kim, PhD, CIBMTR
Statistician	Kyle Hebert, MS, CIBMTR

PLASMA CELL DISORDERS AND ADULT SOLID TUMORS WORKING COMMITTEE

Chairs	Shaji Kumar, MD, Mayo Clinic Rochester
	Nina Shah, MD, University of California San Francisco Medical Center
	Muzaffar Qazilbash, MD, MD Anderson Cancer Center
Scientific Director	Parameswaran Hari, MD, MS, CIBMTR
Asst Scientific Dir	Anita D'Souza, MD, CIBMTR
Statistical Director	Raphael Fraser, PhD, CIBMTR
Statistician	Noel Estrada-Merly, MPH, CIBMTR

REGIMEN-RELATED TOXICITY AND SUPPORTIVE CARE WORKING COMMITTEE

Chairs	Shin Mineishi, MD, Penn State Hershey Medical Center
	Edward Stadtmauer, MD, Abramson Cancer Center University of Pennsylvania Medical Center
	Bipin Savani, MD, Vanderbilt University Medical Center
Scientific Director	Marcelo Pasquini, MD, MS, CIBMTR
Asst Scientific Dir	Saurabh Chhabra, MD, CIBMTR
Statistical Director	Brent Logan, PhD, CIBMTR
Statistician	Mariam Johnson, MPH, CIBMTR

APPENDIX C6: CIDR EXECUTIVE COMMITTEE MEMBERSHIP

The CIDR Executive Committee provides direction on scientific activities and policy decisions related to CIDR activities and provides input for selection of Cellular Immunotherapy for Cancer Working Committee Leadership. The Executive Committee coordinates activities with other IOTN programs and facilitates compliance with the terms of the CIDR grant and progress toward its specific goals.

CHAIR

Miguel-Angel Perales, MD, Memorial Sloan Kettering Cancer Center, New York, NY

MEMBERS

Catherine Bollard, MBChB, MD, Children's National Medical Center, Washington, DC

Partow Kebriaei, MD, M.D. Anderson Cancer Center, Houston, TX

David Porter, MD, Abramson Cancer Center University of Pennsylvania Medical Center, Philadelphia, PA

Susan Prockop, MD, Memorial Sloan Kettering Cancer Center, New York, NY

Carlos Ramos, MD, Baylor College of Medicine - Center for Cell & Gene Therapy, Houston, TX

EX OFFICIO MEMBERS

IOTN Steering Committee Chair	Alan Hutson, PhD, MA
CIDR Project Officer	Lori Henderson, PhD
CIBMTR Advisory Committee Chair	Rob Soiffer, MD
Chief Scientific Director	Mary M. Horowitz, MD, MS
Principal Investigator	Marcelo Pasquini, MS, MS
Chief Statistical Director	Mei-Jie Zhang, PhD
Senior Vice President and Associate Scientific Director, CIBMTR Minneapolis	Steven Devine, MD
Executive Director CIBMTR Milwaukee	Patricia Steinert, PhD, MBA
Senior Scientific Director CIBMTR Milwaukee	Bronwen Shaw, MD PhD
Senior Scientific Director CIBMTR Milwaukee	Kathryn Flynn, PhD
IT Director CIBMTR Milwaukee	Erik Bergman, MBA, MS
IT Director CIBMTR Minneapolis	Matt Prestegaard, BA

APPENDIX C7: IMMUNOBIOLOGY STEERING COMMITTEE MEMBERSHIP

The NMDP/Be The Match Histocompatibility Advisory Group also serves as the CIBMTR Immunobiology Steering Committee. This committee reviews and approves the use of donor-recipient specimens from the Research Repository in CIBMTR studies.

CHAIR

Joseph Pidala, MD, PhD, H. Lee Moffitt Cancer Center, Tampa, FL

ADVISORY GROUP MEMBERS

Janet Kuramoto-Crawford, PhD, HRSA Division of Transplantation, Rockville, MD

Marilyn Levi, MD, HRSA Division of Transplantation, Rockville, MD

Juliet Barker, MBBS, Memorial Sloan Kettering Cancer Center, New York, NY

Marcelo Fernandez-Viña, PhD, D (ABHI), Stanford Hospital and Clinics, Palo Alto, CA

Michael D. Gautreaux, PhD, D (ABHI), Wake Forest School of Medicine, Winston-Salem, NC

Marie Bleakley, MD, PhD, Fred Hutchinson Cancer Research Center, Seattle, WA

Ketevan (Ketty) Gendzekhadze, MD, PhD, D (ABHI), City of Hope Medical Center, Duarte, CA

Jennifer S. Wilder, RN, BSN, OCN, CHTC, NIH Unrelated Donor Hematopoietic Stem Cell Transplant Program, Bethesda, MD

Brent Logan, PhD, Medical College of Wisconsin, Milwaukee, WI

Carlheinz Mueller, MD, PhD, German National Bone Marrow Donor Registry (ZKRD), Ulm, Germany

Miguel Angel Perales, MD, Memorial Sloan Kettering Cancer Center

Bronwen Shaw, MD, PhD, Medical College of Wisconsin, Milwaukee, WI

NATIONAL MARROW DONOR PROGRAM (NMDP) STAFF

C. Randal Mills, PhD, NMDP

Steven Devine, MD, NMDP / CIBMTR

Neng Yu, MD, NMDP

Tim Tripp, NMDP

Jason Dehn, MPH, NMDP

Martin Maiers, MS, CIBMTR

Stephen Spellman, MBS, CIBMTR

APPENDIX C8: CLINICAL TRIALS ADVISORY COMMITTEE MEMBERSHIP

The Clinical Trials Advisory Committee make recommendations regarding the RCI BMT's strategy, direction, and alignment with the CIBMTR's scientific agenda (**Section 2.3.2**).

CHAIR

John Koreth, MBBS, DPhil, Dana Farber Cancer Institute, Boston, MA

MEMBERS

Andrew Artz, MD, MS, University of Chicago Medicine, Chicago, IL

Amrita Krishnan, MD, City of Hope, Duarte, CA

Shaji Kumar, MD, Mayo Clinic Rochester, Rochester, MN

John Levine, MD, MS, Mount Sinai Medical Center, New York, NY

David Maloney, MD, PhD, Fred Hutchinson Cancer research Center, Seattle, WA

Eneida Nemecek, MD, Pediatric Blood & Marrow Transplant Program, Doernbecher Children's Hospital, OHSU, Portland, OR

Marco Mielcarek, MD, Fred Hutchinson Cancer Research Center, Seattle, WA

Bipin Savani, MD, Vanderbilt University Medical Center, Nashville, TN

APPOINTED MEMBERS

Jack Aiello, EE, MS, CIBMTR Consumer Advocacy Committee

Hilary Hall, CIBMTR Consumer Advocacy Committee

EX OFFICIO MEMBERS

Julie Clark, JD, NMDP/Be The Match, Minneapolis, MN

Dennis Confer, MD, CIBMTR, Minneapolis, MN

Steven Devine, MD, CIBMTR, Minneapolis, MN

Mary Horowitz, MD, MS, CIBMTR, Milwaukee, WI

Roberta King, MPH, CIBMTR, Minneapolis, MN

Erin Leckrone, CIBMTR, Minneapolis, MN

Brent R. Logan, PhD, CIBMTR, Milwaukee, WI

Marcie Riches, MD, MS, CIBMTR Scientific Director, University of North Carolina Hospitals, Chapel Hill, NC

Bronwen Shaw, MD, PhD, CIBMTR, Milwaukee, WI

Daniel Weisdorf, MD, CIBMTR Senior Research Advisor, Minneapolis, MN

APPENDIX D: PUBLICATIONS

In 2019, the CIBMTR published **95** peer-reviewed manuscripts in the journals listed in **Table D.1**. Full lists of publications by program are provided in the following appendices. The PMCID number is assigned by PubMed Central, the NIH's free digital archive of biomedical and life sciences journal literature, and is in compliance with the NIH policy on public access.

Table D.1 2019 CIBMTR Publications by Journal

Journal	Number of Publications
Biology of Blood and Marrow Transplantation	25
Blood Advances	14
Bone Marrow Transplantation	11
Haematologica	4
Journal of Clinical Oncology	3
Journal of the American Medical Association (JAMA) Oncology	3
Blood	2
Hematology/Oncology and Stem Cell Therapy	2
Human Immunology	2
Journal of the National Cancer Institute (JNCI) Cancer Spectrum	2
The Lancet	2
Proceedings of the National Academy of Sciences of the United States of America	2
Supportive Care in Cancer	2
Other Journals*	21
TOTAL	95

*One publication each in Bioinformatics, Blood Cancer Journal, British Journal of Haematology, Clinical Cancer Research, Clinical Infectious Diseases, Communications in Statistics, Immunogenetics, Journal of the American Academy of Dermatology, Journal of Cancer Education, Journal of Hematology & Oncology, Journal of the National Comprehensive Cancer Network, JAMA Pediatrics, Kidney International, Leukemia & Lymphoma, Leukemia, Lifetime Data Analysis, New Genetics and Society, Scientific Reports, Statistical Methods in Medical Research, Transfusion, and Transplant Infectious Diseases.

APPENDIX D1: CLINICAL OUTCOMES RESEARCH PUBLICATIONS

The following publications were generated by the Clinical Outcomes Research Program. For more information about the Clinical Outcomes Research Program, see **Section 2.1**.

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Epperla N, Ahn KW, Litovich C, Ahmed S, Battiwalla M, Cohen JB, Dahi P, Farhadfar N, Farooq U, Freytes CO, Ghosh N, Haverkos B, Herrera A, Hertzberg M, Hildebrandt G, Inwards D, Kharfan-Dabaja MA, Khimani F, Lazarus H, Lazaryan A, Lekakis L, Murthy H, Nathan S, Nishihori T, Pawarode A, Prestidge T, Ramakrishnan P, Rezvani AR, Romee R, Shah NN, Sureda A, Fenske TS, Hamadani M	Allogeneic hematopoietic cell transplantation provides effective salvage despite refractory disease or failed prior autologous transplant in angioimmunoblastic T-cell lymphoma: A CIBMTR analysis	Journal of Hematology & Oncology. 12(1):6. doi:10.1186/s13045-018-0696-z. Epub 2019 Jan 10.	PMC6329157
Papanicolauo GA, Ustun C, Young JH, Chen M, Kim S, Ahn KW, Komanduri K, Lindemans C, Auletta JJ, Riches ML	Bloodstream infection due to vancomycin-resistant enterococcus is associated with increased mortality after hematopoietic cell transplantation for acute leukemia and myelodysplastic syndrome: A multicenter, retrospective cohort study	Clinical Infectious Diseases. 2019 Nov 15; 69(10):1771-1779. doi:10.1093/cid/ciz03. Epub 2019 Jan 14.	PMC6821199
Abidi MZ, Hari P, Chen M, Kim S, Battiwala M, Dahi PB, Diaz MA, Gale RP, Ganguly S, Gergis U, Green J, Hildebrandt G, Hill JA, Komanduri K, Lazarus H, Marks D, Nishihori T, Olsson R, Seo S, Ustun C, Yared J, Yin D, Wingard J, Wirk BM, Auletta J, Lindemans C, Riches M	Virus detection in the cerebrospinal fluid of hematopoietic stem cell transplant recipients is associated with poor patient outcomes: A CIBMTR contemporary longitudinal study	Bone Marrow Transplantation. doi:10.1038/s41409-019-0457-9. Epub 2019 Jan 29.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Prokopishyn NL, Logan BR, Kiefer DM, Sees JA, Chitphakdithai P, Ahmed IA, Anderlini PN, Beitinjaneh AM, Bredeson C, Cerny J, Chhabra S, Daly A, Diaz MA, Farhadfar N, Frangoul HA, Ganguly S, Gastineau DA, Gergis U, Hale GA, Hematti P, Kamble RT, Kasow KA, Lazarus HM, Liesveld JL, Murthy HS, Norkin M, Olsson RF, Papari M, Savani BN, Szer J, Waller EK, Wirk B, Yared JA, Pulsipher MA, Shah NN, Switzer GE, O'Donnell PV, Confer DL, Shaw BE	The concentration of total nucleated cells in harvested bone marrow for transplantation has decreased over time	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2019 Jul 1; 25(7):1325-1330. doi:10.1016/j.bbmt.2019.01.034. Epub 2019 Feb 2.	PMC6615955
Dreger P, Sureda A, Ahn KW, Eapen M, Litovich C, Finel H, Boumendil A, Gopal A, Herrera AF, Schmid C, Diez-Martin JL, Fuchs E, Bolaños-Meade J, Gooptu M, Al Malki MM, Castagna L, Ciurea SO, Dominietto A, Blaise D, Ciceri F, Tischer J, Corradini P, Montoto S, Robinson S, Gülbas Z, Hamadani M	PTCy-based haploidentical vs matched related or unrelated donor reduced-intensity conditioning transplant for DLBCL	Blood Advances. 2019 Feb 12; 3(3):360-369. doi:10.1182/bloodadvances.2018027748. Epub 2019 Feb 5.	PMC6373757
Aljurf M, Weisdorf D, Alfraih F, Szer J, Müller C, Confer D, Hashmi S, Kröger N, Shaw BE, Greinix H, Kharfan-Dabaja MA, Foeken L, Seber A, Ahmed S, El-Jawahri A, Al-Awwami M, Atsuta Y, Pasquini M, Hanbali A, Alzahrani H, Okamoto S, Gluckman E, Mohty M, Kodera Y, Horowitz M, Niederwieser D, El Fakih R	Worldwide Network for Blood & Marrow Transplantation (WBMT) special article, challenges facing emerging alternate donor registries	Bone Marrow Transplantation. 2019 Aug 1; 54(8):1179-1188. doi:10.1038/s41409-019-0476-6. Epub 2019 Feb 18.	N/A
Kilari D, D'Souza A, Fraser R, Qayed M, Davila O, Agrawal V, Diaz MA, Chhabra S, Cerny J, Copelan E, Farhadfar N, Freytes CO, Gale RP, Ganguly S, Hildebrandt GC, Holmberg L, Kamble RT, Kapoor P, Lazarus H, Lee C, Murthy HS, Naik S, Nishihori T, Saad A, Savani BN, Seo S, Warwick A, Wirk B, Yared JA, Nieto Y, Hari P	Autologous transplantation for male germ cell tumors: Improved outcomes over 3 decades	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2019 Jun 1; 25(6):1099-1106. doi:10.1016/j.bbmt.2019.02.015. Epub 2019 Feb 20.	PMC6559839

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Yeshurun M, Weisdorf D, Rowe JM, Tallman MS, Zhang M-J, Wang HL, Saber W, de Lima M, Sandmaier BM, Uy G, Kamble RT, Cairo MS, Cooper BW, Cahn J-Y, Ganguly S, Camitta B, Verdonck LF, Dandoy C, Diaz MA, Savani BN, George B, Liesveld J, McGuirk J, Byrne M, Grunwald MR, Drobyski WR, Pulsipher MA, Abdel-Azim H, Prestidge T, Wieduwilt MJ, Martino R, Norkin M, Beitinjaneh A, Seo S, Nishihori T, Wirk B, Frangoul H, Bashey A, Mori S, Marks DI, Bachanova V	The impact of the graft-versus-leukemia effect on survival in acute lymphoblastic leukemia	Blood Advances. 2019 Feb 26; 3(4):670-680. doi:10.1182/bloodadvances.2018027003. Epub 2019 Feb 26.	PMC6391668
Inamoto Y, Petricek I, Burns L, Chhabra S, DeFilipp Z, Hematti P, Rovó A, Schears R, Shah A, Agrawal V, Al-Khinji A, Ahmed I, Ali A, Aljurf M, Alkhateeb H, Beitinjaneh A, Bhatt N, Buchbinder D, Byrne M, Callander N, Fahnehjelm K, Farhadfar N, Gale RP, Ganguly S, Hildebrandt GC, Horn E, Jakubowski A, Kamble RT, Law J, Lee C, Nathan S, Penack O, Pingali R, Prasad P, Pulanic D, Rotz S, Shreenivas A, Steinberg A, Tabbara K, Tichelli A, Wirk B, Yared J, Basak GW, Battiwalla M, Duarte R, Savani BN, Flowers MED, Shaw BE, Valdés-Sanz N	Non-GVHD ocular complications after hematopoietic cell transplantation: Expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT	Bone Marrow Transplantation. 2019 May 1; 54(5):648-661. doi:10.1038/s41409-018-0339-6. Epub 2019 Feb 27.	PMC6497536
Kanate AS, Kumar A, Dreger P, Dreyling M, Le Gouill S, Corradini, Bredeson C, Fenske TS, Smith SM, Sureda A, Moskowitz A, Friedberg JW, Inwards DJ, Herrera AF, Kharfan-Dabaja MA, Reddy N, Montoto S, Robinson S, Abutalib SA, Gisselbre C, Vose J, Gopal A, Shadman M, Perales M-A, Carpenter P, Savani BN, Hamadani M	Maintenance therapies for Hodgkin and non-Hodgkin lymphomas after autologous transplantation: A consensus project of ASBMT, CIBMTR, and the Lymphoma Working Party of EBMT.CIBMTR and LWP-EBMT	JAMA Oncology. doi:10.1001/jamaoncol.2018.6278. Epub 2019 Feb 28.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
McCune JS, Wang T, Bo-Subait K, Aljurf M, Beitinjaneh A, Bubalo J, Cahn J-Y, Cerny J, Chhabra S, Cumpston A, Dupuis LL, Lazarus HM, Marks DI, Maziarz RT, Norkin M, Prestidge T, Mineishi S, Krem MM, Pasquini M, Martin PJ	Association of antiepileptic medications with outcomes after allogeneic hematopoietic cell transplantation with busulfan / cyclophosphamide conditioning	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2019 Jul 1; 25(7):1424-1431. doi:10.1016/j.bbmt. 2019.03.001. Epub 2019 Mar 11.	PMC6615968
Parikh SH, Satwani P, Ahn KW, Sahr NA, Fretham C, Abraham AA, Agrawal V, Auletta JJ, Abdel-Azim H, Copelan E, Diaz M-A, Dvorak CC, Frangoul HA, Freytes CO, Gadalla SM, Gale RP, George B, Gergis U, Hashmi S, Hematti P, Hildebrandt GC, Keating AK, Lazarus HM, Myers K, Olsson RF, Prestidge T, Rotz S, Savani BN, Shereck E, Williams KM, Wirk B, Pasquini MC, Loren AW	Survival trends in infants undergoing allogeneic hematopoietic cell transplant	JAMA Pediatrics. doi:10.1001/jamape diatrics.2019.0081. Epub 2019 Mar 18.	PMC6503511
Wiener L, Hoag JA, Pelletier W, Shah NN, Shaw BE, Pulsipher MA, Bruce J, Bader P, Willasch AM, Dalissier A, Guilcher G, Anthias C, Confer DL, Sees JA, Logan B, Switzer GE	Transplant center practices for psychosocial assessment and management of pediatric hematopoietic stem cell donors	Bone Marrow Transplantation. doi:10.1038/s41409 -019-0515-3. Epub 2019 Apr 10.	N/A
Aljurf M, Weisdorf D, Hashmi SK, Nassar A, Gluckman E, Mohty M, Rizzo D, Pasquini M, Hamadani M, Saber W, Hari P, Kharfan-Dabaja MA, Majhail N, Gerges U, Hamidieh AA, Hussain F, Elhaddad A, Mahmoud HK, Tbakhi A, Othman TB, Hamladji RM, Bekadja MA, Ahmed P, Bazarbachi A, Adil S, Alkindi S, Ladeb S, Dennison D, Patel M, Lu P, Quessar AE, Okamoto S, Atsuta Y, Alhejazi A, Ayas M, Ahmed SO, Novitzky N, Srivastava A, Seber A, Elsolh H, Ghavamzadeh A, Confer D, Kodera Y, Greinix H, Szer J, Horowitz M, Niederwieser D	Worldwide Network for Blood and Marrow Transplantation (WBMT) recommendations for establishing a hematopoietic stem cell transplantation program in countries with limited resources (Part II): Clinical, technical and socio-economic considerations	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt. 2019.04.012. Epub 2019 Apr 17.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Mehta RS, Holtan SG, Wang T, Hemmer MT, Spellman SR, Arora M, Couriel DR, Alousi AM, Pidala J, Abdel-Azim H, Ahmed I, Aljurf M, Askar M, Auletta JJ, Bhatt V, Bredeson C, Chhabra S, Gadalla S, Gajewski J, Gale RP, Gergis U, Hematti P, Hildebrandt GC, Inamoto Y, Kitko C, Khandelwal P, MacMillan ML, Majhail N, Marks DI, Mehta P, Nishihori T, Olsson RF, Pawarode A, Diaz MA, Prestidge T, Qayed M, Rangarajan H, Ringden O, Saad A, Savani BN, Seo S, Shah A, Shah N, Schultz KR, Solh M, Spitzer T, Szer J, Teshima T, Verdonck LF, Williams KM, Wirk B, Wagner J, Yared JA, Weisdorf DJ	GRFS and CRFS in alternative donor hematopoietic cell transplantation for pediatric patients with acute leukemia	Blood Advances. 2019 May 14; 3(9):1441-1449. doi:10.1182/ bloodadvances.2018 030171. Epub 2019 May 3.	PMC6517657
Pasquini MC, Srivastava A, Ahmed SO, Aljurf M, Atsuta Y, Doleysh C, Galeano S, Gluckman E, Greinix H, Hale G, Hari P, Hashmi SK, Kamani N, Laughlin MJ, Niederwieser D, Seber A, Szer J, Snowden JA, Van Biesen K, Watry P, Weisdorf DJ, Apperley J	Worldwide Network for Blood and Marrow Transplantation (WBMT) recommendations for establishing a hematopoietic cell transplantation program (Part I): Minimum requirements and beyond	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt. 2019.05.002. Epub 2019 May 6.	N/A
Ustun C, Le-Rademacher J, Wang H-L, Othus M, Sun Z, Major B, Zhang M-J, Storrick E, Lafky JM, Chow S, Mrózek K, Attar EC, Nand S, Bloomfield CD, Cripe LD, Tallman MS, Appelbaum F, Larson RA, Marcucci G, Roboz GJ, Uy GL, Stone RM, Jatoi A, Shea TC, de Lima M, Foran JM, Sandmaier BM, Litzow MR, Erba HP, Hurria A, Weisdorf DJ, Artz AS	Allogeneic hematopoietic cell transplantation compared to chemotherapy consolidation in older acute myeloid leukemia (AML) patients 60-75 years in first complete remission (CR1): An alliance (A151509), SWOG, ECOG-ACRIN, and CIBMTR study	Leukemia. 2019 Nov 1; 33(11):2599-2609. doi:10.1038/s41375 -019-0477-x. Epub 2019 May 9.	PMC6842042

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Saad A, Lamb L, Wang T, Hemmer MT, Spellman S, Couriel D, Alousi A, Pidala J, Abdel-Azim H, Agrawal V, Aljurf M, Beitinjaneh AM, Bhatt VR, Buchbinder D, Byrne M, Cahn J-Y, Cairo M, Castillo P, Chhabra S, Diaz MA, Farhan S, Floisand Y, Frangoul HA, Gadalla SM, Gajewski J, Gale RP, Gandhi M, Gergis U, Hamilton BK, Hematti P, Hildebrandt GC, Kamble RT, Kanate AS, Khandelwal P, Lazaryan A, MacMillan M, Marks DI, Martino R, Mehta PA, Nishihori T, Olsson RF, Patel SS, Qayed M, Rangarajan H, Reshef R, Ringden O, Savani BN, Schouten HC, Schultz KR, Seo S, Shaffer BC, Solh M, Teshima T, Urbano-Ispizua A, Verdonck LF, Vij R, Waller EK, William B, Wirk B, Yared J, Yu LC, Arora M, Hashmi S	Impact of T-cell dose on the outcome of T-cell replete HLA matched allogeneic peripheral blood stem cell transplantation	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.05.007. Epub 2019 May 11.	N/A
Perales M-A, Tomlinson B, Zhang M-J, St. Martin A, Beitinjaneh A, Gibson J, Hogan W, Kekre N, Lazarus H, Marks D, McGuirk J, Romee R, Solh M, Wagner JE, Weisdorf DJ, de Lima M, Eapen M	Alternative donor transplantation for acute myeloid leukemia in patients aged ≥50 years: Young HLA-matched unrelated or haploidentical donor?	Haematologica. doi:10.3324/haematol.2018.215202. Epub 2019 May 17.	N/A
Ahmed S, Kanakry JA, Ahn KW, Litovich C, Abdel-Azim H, Aljurf M, Bacher VU, Bejanyan N, Cohen JB, Farooq U, Fuchs EJ, Bolaños-Meade J, Ghosh N, Herrera AF, Hossain NM, Inwards D, Kanate AS, Martino R, Munshi PN, Murthy H, Mussetti A, Nieto Y, Perales M-A, Romee R, Savani BN, Seo S, Wirk B, Yared JA, Sureda A, Fenske TS, Hamadani M	Lower GVHD and relapse risk in PTCy-based haploidentical vs matched sibling donor RIC transplant for Hodgkin lymphoma	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.05.025. Epub 2019 May 24.	PMC6553842

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Hamilton BK, Liu Y, Hemmer MT, Majhail N, Ringden O, Kim D, Costa L, Stuart R, Alousi A, Pidala JA, Couriel DR, Aljurf M, Antin JH, Bredeson C, Cahn J-Y, Cairo M, Choi SW, Dandoy C, Gale RP, Gergis U, Hematti P, Inamoto Y, Kamble RT, MacMillan M, Marks DI, Nemecek E, Nishihori T, Saad A, Savani BN, Schriber J, Seo S, Socié G, Teshima T, Verdonck LF, Waller EK, Wirk M, Spellman SR, Arora M, Chhabra S	Inferior outcomes with cyclosporine and mycophenolate mofetil after myeloablative allogeneic hematopoietic cell transplantation	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.05.019. Epub 2019 May 31.	N/A
Broglie L, Fretham C, Al-Seraihy, George B, Kurtzberg J, Loren A, MacMillan M, Martinez C, Davies SM, Pasquini M	Pulmonary complications in pediatric and adolescent patients following allogeneic hematopoietic cell transplantation	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.06.004. Epub 2019 Jun 12.	N/A
Rashidi A, Hamadani M, Zhang M-J, Wang H-L, Abdel-Azim H, Aljurf M, Assal A, Bajel A, Bashey A, Battiwalla M, Beitinjaneh AM, Bejanyan N, Bhatt VR, Bolaños-Meade J, Byrne M, Cahn J-Y, Cairo M, Ciurea S, Copelan E, Cutler C, Daly A, Diaz M-A, Farhadfar N, Gale RP, Ganguly S, Grunwald MR, Hahn T, Hashmi S, Hildebrandt GC, Holland HK, Hossain N, Kanakry CG, Kharfan-Dabaja MA, Khera N, Koc Y, Lazarus HM, Lee JW, Maertens J, Martino R, McGuirk J, Munker R, Murthy HS, Nakamura R, Nathan S, Nishihori T, Palmisiano N, Patel S, Pidala J, Olin R, Olsson RF, Oran B, Ringden O, Rizzieri D, Rowe J, Savoie ML, Schultz KR, Seo S, Shaffer BC, Singh A, Solh M, Stockerl-Goldstein K, Verdonck LF, Wagner J, Waller EK, De Lima M, Sandmaier BM, Litzow M, Weisdorf D, Romee R, Saber W	Outcomes of haploidentical vs matched sibling transplantation for acute myeloid leukemia in first complete remission	Blood Advances. 2019 Jun 25; 3(12):1826-1836. doi:10.1182/bloodadvances.2019000050. Epub 2019 Jun 14.	PMC6595262

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Paulson K, Brazauskas R, Khera N, He N, Majhail N, Akpek G, Aljurf M, Buchbinder D, Burns L, Beattie S, Freytes C, Garcia A, Gajewski J, Hahn T, Knight J, LeMaistre C, Lazarus H, Szwajcer D, Seftel M, Wirk B, Wood W, Saber W	Inferior access to allogeneic transplant in disadvantaged populations: A CIBMTR analysis	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.06.012. Epub 2019 Jun 19.	N/A
Kim HT, Ahn KW, Hu Z-H, Davids MS, Volpe VO, Antin JH, Sorror ML, Shadman M, Press O, Pidala J, Hogan W, Negrin R, Devine S, Uberti J, Agura E, Nash R, Mehta J, McGuirk J, Forman S, Langston A, Giralt SA, Perales M-A, Battiwalla M, Hale GA, Gale RP, Marks DI, Hamadani M, Ganguly S, Bacher U, Lazarus H, Reshef R, Hildebrandt GC, Inamoto Y, Cahn J-Y, Solh M, Kharfan-Dabaja MA, Ghosh N, Saad A, Aljurf M, Schouten HC, Hill BT, Pawarode A, Kindwall-Keller T, Saba N, Copelan EA, Nathan S, Beitinjaneh A, Savani BN, Cerny J, Grunwald MR, Yared J, Wirk BM, Nishihori T, Chhabra S, Olsson RF, Bashey A, Gergis U, Popat U, Sobecks R, Alyea E, Saber W, Brown JR	Prognostic score and cytogenetic risk classification for chronic lymphocytic leukemia patients: Center for International Blood and Marrow Transplant Research report	Clinical Cancer Research. 2019 Aug 15; 26(16):5143-5155. doi:10.1158/1078-0432.CCR-18-3988. Epub 2019 Jun 28.	PMC6697588
Dehn J, Spellman S, Hurley CK, Shaw BE, Barker JN, Burns LJ, Confer DL, Eapen M, Fernandez-Vina M, Hartzman R, Maiers M, Marino SR, Mueller C, Perales M-A, Rajalingam R, Pidala J	Selection of unrelated donors and cord blood units for hematopoietic cell transplantation: Guidelines from the NMDP/CIBMTR	Blood. 2019 Aug 19; 134(12):924-934. doi:10.1182/BLOOD.2019001212. Epub 2019 Jul 10.	PMC6753623
Naik S, Riches M, Soyoung K, Chen M, Bachier C, Shaughnessy P, Hill J, Ljungman P, Battiwalla M, Chhabra S, Daly A, Storek J, Ustun C, Diaz MA, Cerny J, Beitinjaneh A, Yared J, Brown V, Page K, Dahi PB, Ganguly S, Seo S, Chao N, Freytes CO, Saad A, Savani BN, Ahn KW, Boeckh M, Heslop HE, Lazarus HM, Auletta JJ, Kamble RT	Survival outcomes of allogeneic hematopoietic cell transplants with EBV positive or EBV negative post-transplant Lymphoproliferative Disorder (PTLD), a CIBMTR study	Transplant Infectious Disease. doi:10.1111/tid.13145. Epub 2019 Jul 12.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Tang H, Hahn T, Karaesmen E, Rizvi AA, Wang J, Paczesny S, Wang T, Preus L, Zhu Q, Wang Y, Haiman CA, Stram D, Pooler L, Sheng X, Van Den Berg D, Brock G, Webb A, Pasquini MC, McCarthy PL, Spellman SR, Sucheston-Campbell LE	Validation of genetic associations with acute GVHD and nonrelapse mortality in DISCOVeRY-BMT	Blood Advances. 2019 Aug 13; 3(15):2337-2341. doi:10.1182/ bloodadvances.2019 000052. Epub 2019 Aug 7.	PMC6693017
Tsamadou C, Fürst D, Wang T, He N, Lee SJ, Spellman SR, Fleischhauer K, Hsu KC, Paczesny S, Verneris MR, Schrezenmeier H, Mytilineos J	Donor HLA-E status associates with disease free survival and transplant related mortality after non in vivo T-cell depleted HSCT for acute leukemia	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt. 2019.08.007. Epub 2019 Aug 16.	N/A
Wang Y, McReynolds LJ, Dagnall C, Katki HA, Spellman SR, Wang T, Hicks B, Freedman ND, Jones K, Lee SJ, Savage SA, Gadalla SM	Pre-transplant short telomeres are associated with high mortality risk after unrelated donor haematopoietic cell transplant for severe aplastic anaemia	British Journal of Haematology. doi:10.1111/bjh.161 5. Epub 2019 Aug 19.	N/A
Aljurf M, Weisdorf D, Hashmi SK, Nassar A, Gluckman E, Mohty M, Rizzo D, Pasquini M, Hamadani M, Saber W, Hari P, Kharfan-Dabaja MA, Majhail N, Gergis U, Ali Hamidieh A, Hussain F, Elhaddad A, Mahmoud HK, Tbakhi A, Othman TB, Hamladji RM, Bekadja MA, Ahmed P, Bazarbachi A, Adil S, Alkindi S, Ladeb S, Dennison D, Patel M, Lu P, Quessar AE, Okamoto S, Atsuta Y, Alhejazi A, Ayas M, Ahmed SO, Novitzky N, Srivastava A, Seber A, Elsolh H, Ghavamzadeh A, Confer D, Kodera Y, Greinix H, Szer J, Horowitz M, Niederwieser D	Worldwide Network for Blood and Marrow Transplantation (WBMT) recommendations for establishing a hematopoietic stem cell transplantation program in countries with limited resources (Part II): Clinical, technical and socio-economic considerations	Hematology/Oncolo gy and Stem Cell Therapy. doi:10.1016/j.hemo nc.2019.08.002. Epub 2019 Aug 20.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Pasquini MC, Srivastava A, Ahmed SO, Aljurf M, Atsuta Y, Doleysh C, Galeano S, Gluckman E, Greinix H, Hale G, Hari P, Hashmi SK, Kamani N, Laughlin MJ, Niederwieser D, Seber A, Szer J, Snowden JA, Van Biesen K, Watry P, Weisdorf DJ, Apperley J	Worldwide Network for Blood and Marrow Transplantation (WBMT) recommendations for establishing a hematopoietic cell transplantation program (Part I): Minimum requirements and beyond	Hematology/Oncology and Stem Cell Therapy. doi:10.1016/j.hemonc.2019.08.001. Epub 2019 Aug 20.	N/A
Karaesmen E, Hahn T, Dile AJ, Rizvi AA, Wang J, Wang T, Haagenson MD, Preus L, Zhu Q, Liu Q, Yan L, Liu S, Haiman CA, Stram D, Pooler L, Sheng X, Van Den Berg D, Brock G, Webb A, McCarthy PL, Pasquini MC, Spellman SR, Lee SJ, Paczesny S, Sucheston-Campbell LE	Multiple functional variants in the IL1RL1 region are pretransplant markers for risk of GVHD and infection deaths	Blood Advances. 2019 Aug 27; 3(16):2512-2524. doi:10.1182/bloodadvances.2019000075. Epub 2019 Aug 27.	PMC6712530
Sabloff M, Chhabra S, Wang T, Fretham C, Kekre N, Abraham A, Adekola K, Auletta JJ, Barker C, Beitinjaneh AM, Bredeson C, Cahn J-Y, Diaz MA, Freytes C, Gale RP, Ganguly S, Gergis U, Guinan E, Hamilton B, Hashmi S, Hematti P, Hildebrandt G, Holmberg L, Hong S, Lazarus HM, Martino R, Muffly L, Nishihori T, Perales M-A, Yared J, Mineishi S, Stadtmauer EA, Pasquini MC, Loren AW	Comparison of high doses of total body irradiation in myeloablative conditioning prior to hematopoietic cell transplantation	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.08.012. Epub 2019 Aug 29.	N/A
Ustun C, Kim S, Chen M, Beitinjaneh AM, Brown VI, Dahi PB, Daly A, Diaz MA, Freytes CO, Ganguly S, Hashmi S, Hildebrandt GC, Lazarus HM, Nishihori T, Olsson RF, Page KM, Papanicolaou G, Saad A, Seo S, William BM, Wingard JR, Wirk B, Yared JA, Perales M-A, Auletta JJ, Komanduri KV, Lindemans CA, Riches ML	Increased overall and bacterial infections following myeloablative allogeneic HCT for patients with AML in CR1	Blood Advances. 2019 Sep 10; 3(17):2525-2536. doi:10.1182/bloodadvances.2019000226. Epub 2019 Aug 30.	PMC6737406
Li C, Mathews V, Kim S, George B, Hebert K, Jiang H, Li C, Zhu Y, Keesler DA, Boelens JJ, Dvorak CC, Argarwal R, Auletta JJ, Goyal RK, Hanna R, Kasow K, Shenoy S, Smith AR, Walters MC, Eapen M	Related and unrelated donor transplantation for β-thalassemia major: Results of an international survey	Blood Advances. 2019 Sep 10; 3(17):2562-2570. doi:10.1182/bloodadvances.2019000291. Epub 2019 Aug 30.	PMC6737407

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Meybodi MA, Cao W, Luznik L, Bashey A, Zhang X, Romee R, Saber W, Hamadani M, Weisdorf DJ, Chu H, Rashidi A	HLA-haploidentical vs matched-sibling hematopoietic cell transplantation: A systematic review and meta-analysis	Blood Advances. 2019 Sep 10; 3(17):2581-2585. doi:10.1182/ bloodadvances.2019 000614. Epub 2019 Sep 4.	PMC6737418
Muhsen IN, Hashmi SK, Niederwieser D, Kroeger N, Agrawal S, Pasquini MC, Atsuta Y, Ballen KK, Seber A, Saber W, Kharfan-Dabaja MA, Rasheed W, Okamoto S, Khera N, Wood WA, Koh MBC, Greinix H, Kodera Y, Szer J, Horowitz MM, Weisdorf DJ, Aljurf M	Worldwide Network for Blood and Marrow Transplantation (WBMT) perspective: the role of biosimilars in hematopoietic cell transplant: current opportunities and challenges in low- and lower-middle income countries	Bone Marrow Transplantation. doi:10.1038/s41409 -019-0658-2. Epub 2019 Sep 4.	N/A
Eapen M, Brazauskas R, Walters MC, Bernaudin F, Bo-Subait K, Fitzhugh CD, Hankins JS, Kanter J, Meerpohl JJ, Bolaños-Meade J, Panepinto JA, Rondelli D, Shenoy S, Williamson J, Woolford TL, Gluckman E, Wagner JE, Tisdale JF	Effect of donor type and conditioning regimen intensity on allogeneic transplantation outcomes in patients with sickle cell disease: A retrospective multicentre, cohort study	The Lancet Haematology. 2019 Nov 1; 6(11):e585-e596. doi:10.1016/S2352-3026(19)30154-1. Epub 2019 Sep 5.	PMC6813907
Knight JM, Rizzo JD, Wang T, He N, Logan BR, Spellman SR, Lee SJ, Verneris MR, Arevalo JMG, Cole SW	Molecular correlates of socioeconomic status and clinical outcomes following hematopoietic cell transplantation for leukemia	JNCI Cancer Spectrum. 2019 Dec 1; 3(4): pkz073. doi:10.1093/jncics/p kz073. Epub 2019 Sep 12.	PMC6859844

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Lazaryan A, Dolan M, Zhang MJ, Wang HL, Kharfan-Dabaja MA, Marks DI, Bejanyan N, Copelan E, Majhail N, Waller EK, Chao N, Prestidge T, Nishihori T, Kebriaei P, Inamoto Y, Hamilton B, Hashmi SK, Kamble RT, Bacher U, Hildebrandt GC, Stiff PJ, McGuirk J, Aldoss I, Beitinjaneh AM, Muffly L, Vij R, Olsson RF, Byrne M, Schultz KR, Aljurf M, Seftel M, Savoie ML, Savani BN, Verdonck LF, Cairo MS, Hossain N, Bhatt VR, Frangoul HA, Abdel-Azim H, Al Malki M, Munker R, Rizzieri D, Khera N, Nakamura R, Ringdén O, van der Poel M, Murthy HS, Liu H, Mori S, De Oliveira S, Bolaños-Meade J, Elsawy M, Barba P, Nathan S, George B, Pawarode A, Grunwald MR, Agrawal V, Wang Y, Assal A, Castillo Caro P, Kuwatsuka Y, Seo S, Ustun C, Politikos I, Lazarus HM, Saber W, Sandmaier BM, De Lima M, Litzow M, Bachanova V, Weisdorf D	Impact of cytogenetic abnormalities on outcomes of adult Philadelphia-negative acute lymphoblastic leukemia after allogeneic hematopoietic stem cell transplantation: A study by the Acute Leukemia Working Committee of the Center for International Blood and Marrow Transplant Research	Haematologica. doi:10.3324/haematol.2019.220756. Epub 2019 Sep 26.	N/A
Zinter MS, Logan BR, Fretham C, Sapru A, Abraham A, Aljurf MD, Arnold SD, Artz A, Auletta JJ, Chhabra S, Copelan E, Duncan C, Gale RP, Guinan E, Hematti P, Keating AK, Marks DI, Olsson R, Savani BN, Ustun C, Williams K, Pasquini MC, Dvorak CC	Comprehensive prognostication in critically ill pediatric hematopoietic cell transplant patients: Results from merging the Center for International Blood and Marrow Transplant Research (CIBMTR) and Virtual Pediatric Systems (VPS) Registries	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.09.027. Epub 2019 Sep 26.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Mohty M, Malard F, Abecasis M, Aerts E, Alaskar AS, Aljurf M, Arat M, Bader P, Baron F, Basak G, Bazarbachi A, Blaise D, Ciceri F, Corbacioglu S, Dalle J-H, Dignan F, Fukuda T, Huynh A, Kuball J, Lachance S, Lazarus H, Masszi T, Michallet M, Nagler A, NiChonghaile M, Okamoto S, Pagliuca A, Peters C, Petersen FB, Richardson PG, Ruutu T, Saber W, Savani BN, Soiffer R, Styczynski J, Wallhult E, Yakoub-Agha I, Duarte RF, Carreras E	Prophylactic, preemptive, and curative treatment for sinusoidal obstruction syndrome/veno-occlusive disease in adult patients: a position statement from an international expert group	Bone Marrow Transplantation. doi:10.1038/s41409-019-0705-z. Epub 2019 Oct 1.	N/A
Solomon SR, St. Martin A, Shah NN, Fatobene G, Al Malki MM, Ballen KK, Bashey A, Bejanyan N, Bolaños Meade J, Brunstein CG, DeFilipp Z, Champlin RE, Fuchs EJ, Hamadani M, Hematti P, Kanakry CG, McGuirk JP, McNiece IK, Ciurea SO, Pasquini MC, Rocha V, Romee R, Patel SS, Vasu S, Waller EK, Wingard JR, Zhang M-J, Eapen M	Myeloablative vs reduced intensity T-cell-replete haploidentical transplantation for hematologic malignancy	Blood Advances. 2019 Oct 8; 3(19):2836-2844. doi:10.1182/bloodadvances.2019000627. Epub 2019 Oct 3.	PMC6784523
Snowden JA, Saccardi R, Orchard K, Ljungman P, Duarte RF, Labopin M, McGrath E, Brook N, de Elvira CR, Gordon D, Poirel HA, Ayuk F, Beguin Y, Bonifazi F, Gratwohl A, Milpied N, Moore J, Passweg J, Rizzo JD, Spellman SR, Sierra J, Solano C, Sanchez-Guijo F, Worel N, Gusi A, Adams G, Balan T, Baldomero H, Macq G, Marry E, Mesnil F, Oldani E, Pearce R, Perry J, Raus N, Schanz U, Tran S, Wilcox L, Basak G, Chabannon C, Corbacioglu S, Dolstra H, Kuball J, Mohty M, Lankester A, Montoto S, Nagler A, Styczinski J, Yakoub-Agha I, de la Tour RP, Kroeger N, Brand R, de Wreede LC, van Zwet E, Putter H	Benchmarking of survival outcomes following haematopoietic stem cell transplantation: A review of existing processes and the introduction of an international system from the European Society for Blood and Marrow Transplantation (EBMT) and the Joint Accreditation Committee of ISCT and EBMT (JACIE)	Bone Marrow Transplantation. doi:10.1038/s41409-019-0718-7. Epub 2019 Oct 21.	N/A
Herr MM, Curtis RE, Tucker MA, Tecca HR, Engels EA, Cahoon EK, Battiwalla M, Buchbinder D, Flowers ME, Brazauskas R, Shaw BE, Morton LM	Risk factors for the development of cutaneous melanoma after allogeneic hematopoietic cell transplantation	Journal of the American Academy of Dermatology. doi:10.1016/j.jaad.2019.10.034. Epub 2019 Oct 22.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Bejanyan N, Kim S, Hebert KM, Kekre N, Abdel-Azim H, Ahmed I, Aljurf M, Badawy SM, Beitinjaneh A, Boelens JJ, Diaz MA, Dvorak CC, Gadalla S, Gajewski J, Gale RP, Ganguly S, Gennery AR, George B, Gergis U, Gómez-Almaguer D, Vicent MG, Hashem H, Kamble RT, Kasow KA, Lazarus HM, Mathews V, Orchard PJ, Pulsipher M, Ringden O, Schultz K, Teira P, Woolfrey AE, Saldaña BD, Savani B, Winiarski J, Yared J, Weisdorf DJ, Antin JH, Eapen M	Choice of conditioning regimens for bone marrow transplantation in severe aplastic anemia	Blood Advances. 2019 Oct 22; 3(2):3123-3131. doi:10.1182/bloodadvances.2019000722. Epub 2019 Oct 22.	PMC6849938
DeFilipp Z, Ancheta R, Liu Y, Hu Z-H, Gale RP, Snyder D, Schouten HC, Kalaycio M, Hildebrandt GC, Ustun C, Daly A, Ganguly S, Inamoto Y, Litzow M, Szer J, Savoie ML, Hossain N, Kharfan-Dabaja MA, Hamadani M, Reshef R, Bajel A, Schultz KR, Gadalla S, Gerds A, Liesveld J, Juckett MB, Kamble R, Hashmi S, Abdel-Azim H, Solh M, Bacher U, Lazarus HM, Olsson R, Cahn J-Y, Grunwald MR, Savani BN, Yared J, Rowe JM, Cerny J, Chaudhri NA, Aljurf M, Beitinjaneh A, Seo S, Nishihori T, Hsu JW, Ramanathan M, Alyea E, Popat U, Sobecks R, Saber W	Maintenance tyrosine kinase inhibitors following allo-HCT for chronic myeloid leukemia: A CIBMTR study	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.10.017. Epub 2019 Oct 25.	N/A
Petersdorf EW, Carrington M, O'hUigin C, Bengtsson M, De Santis D, Dubois V, Gooley T, Horowitz M, Hsu K, Madrigal JA, Maiers MJ, Malkki M, McKallor C, Morishima Y, Oudshoorn M, Spellman SR, Villard J, Stevenson P	Role of HLA-B exon 1 in graft-versus-host disease after unrelated haemopoietic cell transplantation: A retrospective cohort study	Haematologica. doi:10.1016/S2352-3026(19)30208-X. Epub 2019 Oct 25.	N/A

CLINICAL OUTCOMES RESEARCH PUBLICATIONS

Authors	Title	Citation	PMCID
Buchbinder D, Brazauskas R, Bo-Subait K, Ballen K, Parsons S, John T, Hahn T, Sharma A, Steinberg A, D'Souza A, Kumar A, Yoshimi A, Wirk B, Shaw B, Freytes C, LeMaistre C, Bredeson C, Dandoy C, Almaguer D, Marks DI, Szwajcer D, Hale G, Schouten H, Hashem H, Schoemans H, Murthy HS, Lazarus HM, Cerny J, Tay J, Yared JA, Adekola K, Schultz KR, Lehmann L, Burns L, Aljurf M, Diaz MA, Majhail N, Farhadfar N, Kamble R, Olsson R, Schears R, Seo S, Beattie S, Chhabra S, Savani BN, Badawy SM, Ganguly S, Ciurea S, Marino S, Gergis U, Kuwatsuka Y, Inamoto Y, Khera N, Hashmi S, Wood W, Saber W	Predictors of lost to follow-up among pediatric and adult hematopoietic cell transplant survivors: A report from the Center for International Blood and Marrow Transplant Research	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.11.003. Epub 2019 Nov 11.	N/A
Ahn KW, Litovich C, Sureda A, Kharfan-Dabaja MA, Awan FT, Ganguly S, Gergis U, Inwards D, Karmali R, Shah NN, Lazaryan A, Lekakis L, Munshi P, Nathan S, Saad AA, Solh M, Steinberg A, Vij R, Wood WA, Fenske TS, Smith S, Hamadani M	Allogeneic transplantation in elderly patients ≥65 years with non-Hodgkin lymphoma: A time-trend analysis	Blood Cancer Journal. 9(12):97. doi:10.1038/s41408-019-0261-1. Epub 2019 Dec 3.	PMC6890709
Atallah E, Logan BR, Chen M, Cutler C, Deeg J, Jacoby E, Champlin R, Nishihori T, Confer D, Gajewski J, Farnia S, Greenberg P, Warlick E, Weisdorf D, Saber W, Horowitz MM, Rizzo JD	Comparison of patient age groups in transplantation for myelodysplastic syndrome: The Medicare coverage with evidence development study	JAMA Oncology. doi:10.1001/jamaoncol.2019.5140. Epub 2019 Dec 12.	N/A
Ho VT, Martin AS, Pérez WS, Steinert P, Zhang MJ, Chirnomas D, Hoang CJ, Loberiza FR Jr, Saber W	Prior gemtuzumab ozogamicin exposure in adults with acute myeloid leukemia does not increase hepatic veno-occlusive disease risk after allogeneic hematopoietic cell transplantation: A CIBMTR analysis	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.12.763. Epub 2019 Dec 28.	N/A

APPENDIX D2: BMT CTN PUBLICATIONS

The following publications were generated by the BMT CTN, a component of the Clinical Trials Support Program, which conducts multi-institutional Phase II and III trials focused on HCT. The BMT CTN Data and Coordinating Center maintains continuity of operations and facilitates effective communications. The Data and Coordinating Center effort is a collaboration of the CIBMTR, NMDP/Be The Match, and the Emmes Company. For more information, see **Section 2.3.1**.

BMT CTN PUBLICATIONS

Authors	Title	Citation	PMCID
King AA, McKinstry RC, Wu J, Eapen M, Abel R, Varughese T, Kamani N, Shenoy S	Functional and radiologic assessment of the brain after reduced-intensity unrelated donor transplantation for severe sickle cell disease: Blood and Marrow Transplant Clinical Trials Network study 0601	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2019 May 1; 25(5):e174-e178. doi:10.1016/j.bbmt.2019.01.008. Epub 2019 Jan 10.	PMC6511327
Paul J, Nakasone H, Sahaf B, Wu F, Wang K, Ho V, Wu J, Kim H, Blazar B, Ritz J, Howard A, Cutler C, Miklos D	A confirmation of chronic graft-versus-host disease prediction using allogeneic HY antibodies following sex-mismatched hematopoietic cell transplantation	Haematologica. 2019 Jul 1; 104(7):e314-e317. doi:DOI:10.324. Epub 2019 Jan 17.	PMC6601098
Stadtmauer EA, Pasquini MC, Blackwell B, Hari P, Bashey A, Devine S, Efebera Y, Ganguly S, Gasparetto C, Geller N, Horowitz MM, Koreth J, Knust K, Landau H, Brunstein C, McCarthy P, Nelson C, Qazilbash MH, Shah N, Vesole DH, Vij R, Vogl DT, Giralt S, Somlo G, Krishnan A	Autologous transplantation, consolidation, and maintenance therapy in multiple myeloma: Results of the BMT CTN 0702 trial	Journal of Clinical Oncology. 2019 Mar 1; 37(7):589-597. doi:10.1200/JCO.18.0068. Epub 2019 Jan 17.	PMC6553842

BMT CTN PUBLICATIONS

Authors	Title	Citation	PMCID
Bolaños-Meade J, Reshef R, Fraser R, Fei M, Abhyankar S, Al-Kadhimi Z, Alousi AM, Antin J, Arai S, Bickett, K, Chen Y-B, Damon LE, Efebera YA, Geller N, Giralt SA, Parameswaran H, Holtan SG, Horowitz MM, Jacobsohn DA, Jones RJ, Liesveld JL, Logan BR, MacMillan Ml, Mielcarek M, Noel P, Pidala J, Porter D, Pusic I, Sobecks R, Solomon SR, Weisdorf DJ, Wu J, Pasquini MC, Koreth J	Three prophylaxis regimens (tacrolimus, mycophenolate mofetil, and cyclophosphamide; tacrolimus, methotrexate, and bortezomib; or tacrolimus, methotrexate, and maraviroc) versus tacrolimus and methotrexate for prevention of graft-versus-host disease with haemopoietic cell transplantation with reduced-intensity conditioning: A randomised phase 2 trial with a non-randomised contemporaneous control group (BMT CTN 1203)	The Lancet Haematology. 2019 Mar 6; 6(3):PE132-E142. doi:10.1016/S2352-3026(18)30221-7. Epub 2019 Feb 26.	PMC6503965
Levis, Mark, Chen, Yi Bin, Hamadani, Mehdi, Horowitz, Mary, Jones, Richard	FLT3 inhibitor maintenance after allogeneic transplantation: Is a placebo-controlled, randomized trial ethical?	Journal of Clinical Oncology. 2019 Jul 1; 37(19):1604-1607. doi:10.1200/JCO.19.0032. Epub 2019 Apr 29.	N/A
Gooptu M, Kim HT, Howard A, Choi SW, Soiffer RJ, Antin JH, Ritz J, Cutler CS	Effect of sirolimus on immune reconstitution following myeloablative allogeneic stem cell transplantation: An ancillary analysis of a randomized controlled trial comparing tacrolimus / sirolimus and tacrolimus / methotrexate (Blood and Marrow Transplant Clinical Trials Network / BMT CTN 0402)	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.06.029. Epub 2019 Jul 2.	N/A
Ambinder RF, Wu J, Logan B, Durand CM, Shields R, Popat UR, Little RF, McMahon DK, Cyktor J, Mellors JW, Ayala E, Kaplan LD, Noy A, Jones RJ, Howard A, Forman SJ, Porter D, Arce-Lara C, Shaughnessy P, Sproat L, Hashmi SK, Mendizabal AM, Horowitz MM, Navarro WH, Alvarnas JC	Allogeneic hematopoietic cell transplant for HIV patients with hematologic malignancies: The BMT CTN 0903 / AMC-080 trial	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.06.033. Epub 2019 Jul 4.	PMC6907401

BMT CTN PUBLICATIONS

Authors	Title	Citation	PMCID
Waller EK, Logan BR, Fei M, Lee SJ, Confer D, Howard A, Chandrakasan S, Anasetti C, Fernando SM, Giver CR	Kinetics of immune cell reconstitution predict survival in allogeneic bone marrow and G-CSF-mobilized stem cell transplantation	Blood Advances. 2019 Aug 13; 3(15):2250-2263. doi:10.1182/bloodadvances.2018029892. Epub 2019 Jul 25.	PMC6693008
Rashidi A, Luo X, Cooley S, Anasetti C, Waller EK, Brunstein CG, Cichocki F, Weisdorf DJ, Miller JS	The association of CMV with NK-cell reconstitution depends on graft source: Results from BMT CTN-0201 samples	Blood Advances. 2019 Aug 27; 3(16):2465-2469. doi:10.1182/bloodadvances.2019000298. Epub 2019 Aug 19.	PMC6712525
Pidala J, Martens M, Anasetti C, Carreras J, Horowitz M, Lee SJ, Antin J, Cutler C, Logan B	Factors associated with successful discontinuation of immune suppression after allogeneic hematopoietic cell transplantation	JAMA Oncology. doi:10.1001/jamaoncol.2019.2974. Epub 2019 Sep 26.	PMC6763979
Holstein SA, Al-Kadhimi Z, Costa LJ, Hahn T, Hari P, Hillengrass J, Jacob A, Munshi NC, Oliva S, Pasquini MC, Shi Q, Stadtmauer EA, Waldvogel SL, McCarthy PL	Summary of the third annual Blood and Marrow Transplant Clinical Trials Network myeloma intergroup workshop on minimal residual disease and immune profiling	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.09.015. Epub 2019 Sep 29.	N/A
Newell LF, DeFor TE, Cutler C, Verneris MR, Blazar BR, Miller JS, Antin JH, Howard A, Wu J, MacMillan ML, Panaskaltsis-Mortari A, Weisdorf DJ, Holtan SG	Follistatin and soluble endoglin predict 1-year nonrelapse mortality after allogeneic hematopoietic cell transplantation	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.11.006. Epub 2019 Nov 10.	N/A
Martens, MJ, Logan BR	Group sequential tests for treatment effect on survival and cumulative incidence at a fixed time point	Lifetime Data Analysis. doi:10.1007/s10985-019-09491-z. Epub 2019 Nov 15.	N/A

BMT CTN PUBLICATIONS

Authors	Title	Citation	PMCID
Pidala J, Hamadani M, Dawson P, Martens M, Alousi A, Jagasia MH, Efebera YA, Chhabra S, Pusic I, Holtan SG, Ferrara JL, Levine JE, Mielcarek M, Anasetti C, Antin J, Bolaños-Meade J, Howard A, Logan BR, Leifer ES, Pritchard TS, Horowitz MM, MacMillan ML	Randomized multicenter trial of sirolimus vs. prednisone as initial therapy for standard risk acute GVHD: BMT CTN 1501	Blood. doi:10.1182/blood.2019003125. Epub 2019 Nov 18.	N/A
Giralt S, Costa LJ, Maloney D, Krishnan A, Fei M, Antin JH, Brunstein C, Geller N, Goodman S, Hari P, Logan B, Lowsky R, Qazilbash MH, Sahebi F, Somlo G, Rowley S, Vogl DT, Vesole DH, Pasquini M, Stadtmauer E	Tandem autologous-autologous versus autologous-allogeneic hematopoietic stem cell transplantation for patients with multiple myeloma: Long-term follow-up results from the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0102 trial	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.11.018. Epub 2019 Nov 19.	N/A
Hourigan CS, Dillon LW, Gui G, Logan BR, Fei M, Ghannam J, Li Y, Licon A, Alyea EP, Bashey A, Deeg HJ, Devine SM, Fernandez HF, Giralt S, Hamadani M, Howard A, Maziarz RT, Porter DL, Scott BL, Warlick ED, Pasquini MC, Horwitz ME	Impact of conditioning intensity of allogeneic transplantation for acute myeloid leukemia with genomic evidence of residual disease	Journal of Clinical Oncology. doi:10.1200/JCO.19.0301. Epub 2019 Dec 20.	N/A

APPENDIX D3: RCI BMT PUBLICATIONS

The following publications were generated by the RCI BMT, a component of the Clinical Trials Support Program, which provides cellular therapy researchers with infrastructure and expertise in clinical trial conduct and analysis. For more information, see **Section 2.3.2**.

RCI BMT PUBLICATIONS

Authors	Title	Citation	PMCID
Chen Y-B, Le-Rademacher J, Brazauskas R, Kiefer DM, Hamadani M, DiPersio JF, Litzow MR, Craig M, Horwitz ME, Artz AS, McClune BL, Fernandez HF, Duong HK, Kobusingye H, Proue M, Drexler RJ, Horowitz MM, Shaw BE, Miller JP, Hosoba S, Waller EK, Devine SM	Plerixafor alone for the mobilization and transplantation of HLA-matched sibling donor hematopoietic stem cells	Blood Advances. 2019 Mar 26; 3(6):875-883. doi:10.1182/bloodadvances.2018027599. Epub 2019 Mar 19.	PMC6436017
Ballen K, Logan BR, Chitphakdithai P, Spellman SR, Adams A, Drexler RJ, Duffy M, Kemp A, King R, Babic A, Delaney C, Karanes C, Kurtzberg J, Petz L, Scaradavou A, Shpall E, Smith C, Confer DL, Miller JP, Kuxhausen M	Unlicensed umbilical cord blood units provide a safe and effective graft source for a diverse population: A study of 2456 umbilical cord blood recipients	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.11.016. Epub 2019 Nov 19.	N/A

APPENDIX D4: HEALTH SERVICES RESEARCH PROGRAM PUBLICATIONS

The following publications were generated by the Health Services Research program, through which the CIBMTR identifies and addresses barriers to treatment, improves practice, and demonstrates the value of cellular therapy and survivorship care. For more information, see **Section 2.4**.

HEALTH SERVICES RESEARCH PROGRAM PUBLICATIONS

Authors	Title	Citation	PMCID
Schoemans, HM, Finn L, Foster J, Roche-Green A, Bevans M, Kullberg S, Lee E, Sargeant C, Schatz BA, Scheeler K, Shaw BE, Shereck E, Murphy EA, Burns LJ, Schmit-Pokorny K	A conceptual framework and key research questions in educational needs of blood and marrow transplant patients, caregivers and families	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019. 02.017. Epub 2019 Feb 20.	N/A
Preussler JM, Mau L-W, Majhail NS, Bevans M, Clancy E, Messner C, Parran L, Pederson KA, Stickney Ferguson S, Walters K, Murphy EA, Denzen EM	Caregiver availability and patient access to hematopoietic cell transplantation: social worker perspectives inform practice	Supportive Care in Cancer. doi:10.1007/s00520-019-04696-2. Epub 2019 Mar 9.	N/A
Foster J, Moore, Preussler JM, Burns LJ, Umar JH, Glotzbecker B, Johnson S, MacDougall H, Mau LW, Murphy EA, Ustun C, Ferguson SS, Denzen E	Information needs for treatment decision-making of hematopoietic cell transplant patients 65 years or older and caregivers	Journal of Cancer Education. doi:10.1007/ s13187-019-01506-5. Epub 2019 Mar 15.	N/A
Pidala J, Mupfudze TG, Payton T, Barker J, Perales M-A, Shaw BE, Fernandez-Vina M, Burns LJ, Dehn J	Urgent time to allogeneic hematopoietic cell transplantation: A national survey of transplant physicians and unrelated donor search coordinators facilitated by the Histocompatibility Advisory Group to the National Marrow Donor Program	Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt. 2019.08.002. Epub 2019 Aug 13.	N/A
Mau L-W, Meyer C, Burns LJ, Saber W, Steinert P, Vanness DJ, Preussler JM, Silver A, Leppke S, Murphy EA, Denzen E	Reimbursement, utilization, and one-year survival post-allogeneic transplantation for Medicare beneficiaries with acute myeloid leukemia	JNCI Cancer Spectrum. doi:10.1093/jncics/pkz04. Epub 2019 Aug 28.	N/A

HEALTH SERVICES RESEARCH PROGRAM PUBLICATIONS

Authors	Title	Citation	PMCID
Preussler JM, Denzen EM, Majhail NS, Baker KS, McCann M, Burns LJ, Yi J, Syrjala KL	Engaging hematopoietic cell transplantation patients and caregivers in the design of print and mobile application individualized survivorship care plan tools	Supportive Care in Cancer. doi:10.1007/s00520-019-05114-3. Epub 2019 Nov 15.	N/A
Majhail NS, Mau L-W, Chitphakdithai P, Denzen E, Joffe S, Lee SJ, LeMaistre CF, Loberiza F, Parsons SK, Repaczki-Jones R, Robinett P, Rizzo JD, Murphy E, Logan B, Le-Rademacher J	Transplant center characteristics and survival after allogeneic hematopoietic cell transplantation in adults	Bone Marrow Transplantation. doi:10.1038/s41409-019-0748-1. Epub 2019 Nov 18.	N/A
Meyer C, Mau L-W, Murphy E, Denzen E, Hayes E, Haven D, Moore H, Foster J, Preussler JM, Burns LJ	Addressing knowledge gaps to improve referral for hematopoietic cell transplantation (HCT) consultation for acute myeloid leukemia (AML)	Journal of the National Comprehensive Cancer Network: JNCCN. 2019 Dec 1; 17(12):1473-1481. doi:10.6004/jnccn.2019.7327. Epub 2019 Dec 1.	N/A

APPENDIX D5: BIOINFORMATICS RESEARCH PROGRAM PUBLICATIONS

The following publications were generated by the Bioinformatics Research Program, which conducts bioinformatics for high impact research and translation of research into practice. For more information, see **Section 2.5**.

BIOINFORMATICS RESEARCH PROGRAM PUBLICATIONS

Authors	Title	Citation	PMCID
Horowitz AL, Saperstein A, Little J, Maiers M, Hollenbach JA	Consumer (dis-)interest in genetic ancestry testing: The roles of race, immigration, and ancestral certainty	New Genetics and Society. 2019 Apr 1; 38(2):165-194. doi:10.1080/14636778.2018.1562327. Epub 2019 Jan 20.	PMC6897494
Maiers M, Halagan M, Gragert L, Bashyal P, Brelsford J, Schneider J, Lutsker P, Louzoun Y	GRIMM: GRaph IMputation and matching for HLA genotypes	Bioinformatics. 2019 Sep 15; 35(18):3520-3523. doi:10.1093/bioinformatics/btz050. Epub 2019 Jan 28.	N/A
Allan D, Kiernan J, Gragert L, Dibdin N, Bartlett D, Campbell T, Mostert K, Halpenny M, Ganz K, Maiers M, Petraszko T, Elmoazzen H.	Reducing ethnic disparity in access to high-quality HLA-matched cord blood units for transplantation: analysis of the Canadian Blood Services' Cord Blood Bank inventory	Transfusion. 2019 Jul 1; 59(7):2382-2388. Epub 2019 Apr 19.	N/A
Bishara A, Halagan M, Brautbar C, Israel S, Maiers M	High resolution HLA allele and haplotype frequencies for Arab donors in the Hadassah Bone Marrow Donor Registry	Human Immunology. doi:10.1016/j.humimm.2019.05.003. Epub 2019 May 21.	N/A
Zhong C, Gragert L, Maiers M, Hill BT, Garcia-Gomez J, Gendzekhadze K, Senitzer D, Song J, Weisenburger D, Goldstein L, Wang SS	The association between HLA and non-Hodgkin lymphoma subtypes, among a transplant-indicated population	Leukemia & Lymphoma. 2019 Dec 1; 60(12):2899-2908. doi:10.1080/10428194.2019.1617858. Epub 2019 Jun 19.	PMC6858537
Lobkovsky AE, Levi L, Wolf YI, Maiers M, Gragert L, Alter I, Louzoun Y, Koonin EV	Multiplicative fitness, rapid haplotype discovery, and fitness decay explain evolution of human MHC	Proceedings of the National Academy of Sciences of the United States of America. doi:10.1073/pnas.1714436116. Epub 2019 Jun 21.	PMC6628782

BIOINFORMATICS RESEARCH PROGRAM PUBLICATIONS

Authors	Title	Citation	PMCID
Askar M, Madbouly A, Zhrebker L, Willis A, Kennedy S, Padros K, Rodriguez MB, Bach C, Spriewald B, Ameen R, Shemmari SA, Tarassi K, Tsirogianni A, Hamdy N, Mossallam G, Hönger G, Spinnler R, Fischer G, Fae I, Charlton R, Dunk A, Vayntrub TA, Halagan M, Osoegawa K, Fernández-Viña M	HLA haplotypes in 250 families: The Baylor Laboratory results and a perspective on a core NGS testing model for the 17th International HLA And Immunogenetics Workshop	Human Immunology. doi:10.1016/j.humimm.2019.07.298. Epub 2019 Oct 23.	N/A
Louzoun Y, Lobkovsky AE, Levi L, Wolf YI, Maiers M, Gragert L, Alter I, Koonin EV	Reply to Hedrick and Klitz: High haplotype discovery rate in the HLA locus	Proceedings of the National Academy of Sciences of the United States of America. 2019 Nov 19; 116(47):23388-23389. doi:10.1073/pnas.1916124116. Epub 2019 Oct 29.	PMC6876207
Sapir-Pichhadze R, Zhang X, Ferradji A, Madbouly A, Tinckam K, Gebel H, Blum D, Marrari M, Joseph Kim S, Fingerson S, Bashyal P, Cardinal H, Foster B	Epitopes as characterized by antibody-verified eplet mismatches determine risk of kidney transplant loss	Kidney International. doi:10.1016/j.kint.2019.10.028. Epub 2019 Nov 12.	N/A
Simanovsky AL, Madbouly A, Halagan M, Maiers M, Louzoun Y	Single haplotype admixture models using large scale HLA genotype frequencies to reproduce human admixture	Immunogenetics. 2019 Nov 1; 71(10):589-604. doi:10.1007/s00251-019-01144-7. Epub 2019 Nov 18.	N/A

APPENDIX D6: STATISTICAL METHODOLOGY RESEARCH PROGRAM PUBLICATIONS

The following publications were generated by the Statistical Methodology Research Program, which develops and evaluates the statistical models used in cellular therapy. For more information, see **Section 2.6**.

STATISTICAL METHODOLOGY RESEARCH PROGRAM PUBLICATIONS

Authors	Title	Citation	PMCID
Sparapani R, Logan BR, McCulloch RE, Laud PW	Nonparametric competing risks analysis using Bayesian Additive Regression Trees	Statistical Methods in Medical Research. doi:10.1177/0962280218822140. Epub 2019 Jan 7.	N/A
Liu N, Liu Y, Logan B, Xu Z, Tang J, Wang Y	Learning the dynamic treatment regimes from medical registry data through deep Q-network	Scientific Reports. 9(1):1495. doi:10.1038/s41598-018-37142-0. Epub 2019 Feb 6. PMC6365640.	PMC6365640
Zheng C, Dai R, Gale RP, Zhang M-J	Causal inference in randomized clinical trials	Bone Marrow Transplantation. doi:10.1038/s41409-018-0424-x. Epub 2019 Mar 26.	N/A
Gale RP, Zhang MJ	Statistical analyses of clinical trials in haematopoietic cell transplantation or why there is a strong correlation between people drowning after falling out of a fishing boat and marriage rate in Kentucky	Bone Marrow Transplantation. doi:10.1038/s41409-019-0431-6. Epub 2019 Mar 26.	N/A
Wang T, DeVogel N	A revisit to two-way factorial ANOVA with mixed effects and interactions	Communications in Statistics – Theory and Methods. doi:10.1080/03610926.2019.1604961. Epub 2019 Apr 26.	N/A
Hu Z-H, Gale RP, Zhang M-J	Direct adjusted survival and cumulative incidence curves for observational studies	Bone Marrow Transplantation. doi:10.1038/s41409-019-0552-y. Epub 2019 May 17.	N/A

APPENDIX D7: 2018 PUBLICATION NOT PREVIOUSLY REPORTED

The following publication was not presented in the 2018 CIBMTR Annual Report because it was published in the latter part of the year.

2018 PUBLICATION NOT PREVIOUSLY REPORTED

Authors	Title	Citation	PMCID
(Statistical Methodology Research Program) Kim S, Woo Ahn K	Bi-level variable selection for case-cohort studies with group variables	Statistical Methods in Medical Research. 2019 Nov 1; 28(10-11):3404-3414. doi:10.1177/0962280218803654. Epub 2018 Oct 11.	PMC6748310

APPENDIX E: PRESENTATIONS

In 2019, CIBMTR study investigators presented **75** abstracts (**40** oral and **35** poster) at the national and international conferences listed in **Table E.1**. A complete list of presentations is provided by meeting.

Table E.1 2019 CIBMTR Presentations by Meeting

Conference	Oral	Poster	Total
American Society of Hematology (ASH)	14	19	**33**
TCT Meetings	9	9	**18**
European Federation for Immunogenetics	5	2	**7**
European Society for Blood and Marrow Transplantation (EBMT)	1	2	**3**
Data Standards and Symposium Hackathon	3	0	**3**
Eastern North American Region International Biometric Society	2	0	**2**
CIBMTR MS Workshop	2	0	**2**
Other Meetings and Conferences*	4	3	**7**
TOTAL	**40**	**35**	**75**

* One oral presentation each at the European Hematology Association Congress, Joint Statistical Meetings of the American Statistical Association, Conference on Lifetime Data Science: Foundations and Frontiers, and the International Chinese Statistical Association Applied Statistics Symposium. One poster presentation each at the University of Virginia Scholar Research Day, American Society of Histocompatibility and Immunogenetics, and the KIR Workshop.

2019 AMERICAN SOCIETY OF HEMATOLOGY (ASH) ANNUAL MEETING

Study	Title	Type	PI
Bioinformatics	Epigenomic signatures in myelodysplastic syndrome patients as predictors of donor compatibility and transplant outcome	Poster	W Wang
BMT CTN 0102	Tandem autologous-autologous vs. autologous-allogeneic transplantation for newly diagnosed multiple myeloma: Pooled analysis of 1,338 patients from four trials with long-term follow-up	Oral	L Costa
BMT CTN 0803/0903	Comparative analysis of immune reconstitution in HIV-positive recipients of allogeneic and autologous stem cell transplant on the BMT CTN 0903/AMC-080 and BMT CTN 0803/AMC-071 trial	Poster	P Shindiapina
BMT CTN 1503	A phase II trial to compare allogeneic transplant vs. standard of care for severe sickle cell disease: Blood and Marrow Transplant Clinical Trials Network (BMT CTN) protocol 1503	Poster	M Eapen
BMT CTN 1506	A phase 3 trial of Gilteritinib as maintenance therapy after allogeneic hematopoietic stem cell transplantation in patients with FLT3-ITD+ AML	Poster	M Levis
BMT CTN 1507	Reduced-intensity conditioning for haploidentical bone marrow transplantation in patients with symptomatic sickle cell disease: BMT CTN protocol 1507	Oral	A Kassim
BMT CTN 1703/1801	Making progress in graft-versus-host disease prophylaxis and microbiome analysis in the Blood and Marrow Transplant Clinical Trials Network: Progress III (1703)/MI-Immune (1801)	Poster	S Holtan
BMT CTN 1803	BMT CTN 1803: Haploidentical natural killer cells (CSTD002) to prevent post-transplant relapse in AML and MDS (NK-REALM)	Poster	S Vasu
CK17-02	Fludarabine and melphalan compared with reduced doses of busulfan and fludarabine improves transplant outcomes in older MDS patients	Oral	B Oran
DS18-02	Lower hematopoietic progenitor cell counts and yields at subsequent donations is influenced by a shorter inter-donation interval between the first and subsequent mobilizations	Poster	S Panch D Stronecek B Savani Nirali Shah
GS18-04	Allogeneic transplantation for myelodysplastic syndrome in adults over 50 years old using reduced intensity/non-myeloablative conditioning: Haploidentical relative versus matched unrelated donor	Poster	A Viswabandya B Tomlinson M Grunwald H Elmariah

2019 AMERICAN SOCIETY OF HEMATOLOGY (ASH) ANNUAL MEETING

Study	Title	Type	PI
GV17-01	Early broad-spectrum antibiotics and risk of acute graft-versus-host disease in children: An analysis from the Center for International Blood and Marrow Transplantation Research (CIBMTR) and the Pediatric Health Information System (PHIS)	Oral	C Elgarten B Fisher R Aplenc
IB09-06	Genome wide interaction analysis identifies expression quantitative trait loci associated with reduced survival after reduced intensity conditioning HLA-matched unrelated donor allogeneic hematopoietic cell transplant	Poster	T Hahn
IB10-01	Genome-wide association study identifies an immune-related etiology for severe aplastic anemia	Poster	S Gadalla S Savage
IB18-06	De novo and therapy-related acute myeloid leukemia and myelodysplastic syndrome: Similarities and differences in SNP-array detected chromosomal aberrations in pre-transplant blood samples	Poster	S Gadalla T Hahn L Sucheston-Campbell
LE17-01	Excellent overall survival and low incidence of late effects in patients undergoing allogeneic hematopoietic cell transplant for sickle cell disease: A report from the Center for International Blood and Marrow Transplant Research (CIBMTR)	Oral	E Stenger R Phelan S Shenoy L Krishnamurti
LE18-02	Post-transplant work status of young adult survivors of allogeneic hematopoietic cell transplant: A report from the Center for International Blood and Marrow Transplant Research (CIBMTR)	Oral	N Bhatt R Salit
LK15-03	Superior survival with post-remission pediatric-inspired chemotherapy compared to myeloablative allogeneic hematopoietic cell transplantation in adolescents and young adults with Ph-negative acute lymphoblastic leukemia in first complete remission: Comparison of CALGB 10403 to patients reported to the CIBMTR	Oral	M Wieduwilt W Stock
LK16-02	Myeloablative conditioning is preferred for allogeneic transplantation of acute myeloid leukemia and myelodysplastic syndromes with low/intermediate but not high disease risk index	Poster	N Bejanyan E Warlick C Brunstein D Weisdorf
LK16-03	Allogeneic hematopoietic stem cell transplantation for therapy-related myelodysplastic syndromes and acute myeloid leukemia	Poster	N Callander L Metheny M De Lima A Hall
LK17-01	Impact of depth of pretransplant clinical response on outcomes of acute myeloid leukemia patients in first complete remission (AML-CR1) who undergo allogeneic hematopoietic cell transplantation (alloHCT)	Poster	M Percival B Sandmaier E Estey

2019 AMERICAN SOCIETY OF HEMATOLOGY (ASH) ANNUAL MEETING

Study	Title	Type	PI
LY17-02a	Comparison of reduced-intensity conditioning (RIC) regimens for allogeneic hematopoietic cell transplantation (alloHCT) for classical Hodgkin lymphoma (cHL): A Center for International Blood & Marrow Transplant Research (CIBMTR) analysis.	Poster	N Ghosh
LY17-02b	Comparison of reduced-intensity conditioning (RIC) regimens for allogeneic hematopoietic cell transplantation (alloHCT) in non-Hodgkin lymphomas (NHL)- A Center for International Blood & Marrow Transplant Research (CIBMTR) analysis	Oral	S Ahmed
LY18-01	Does addition of rituximab (R) to BEAM conditioning improve outcomes of patients with diffuse large b-cell lymphoma (DLBCL) undergoing autologous hematopoietic cell transplantation (auto-HCT)?	Oral	D Jagadeesh N Majhail B Hill
MM17-01	Primary plasma cell leukemia outcomes remain dismal despite novel agents and hematopoietic cell transplantation	Oral	S Girnius S Patel L Bachegowda B Dhakal
MM18-02	Novel prognostic scoring system for autologous hematopoietic cell transplantation (AHCT) in multiple myeloma (MM)	Oral	B Dhakal S Chhabra N Callander A Hall Z Gahvari
MM18-03	Breaking the glass ceiling of age in transplant in multiple myeloma	Oral	P Munshi A Jrczyszyn J Zaucha D Vesole
MM18-04	Busulfan, melphalan, and bortezomib compared to single agent high- dose melphalan as a conditioning regimen for autologous hematopoietic stem cell transplantation in multiple myeloma: Long term follow up of a novel conditioning regimen	Poster	P Hagen P Stiff
RT17-01	Impact of renal dysfunction measured by estimated glomerular filtration rate (eGFR) on outcomes after allogeneic hematopoietic cell transplantation (HCT)	Poster	N Farhadfar J Wingard H Murthy
SC17-07	Post-marketing use outcomes of an Anti-CD19 chimeric antigen receptor (CAR) T cell therapy, axicabtagene ciloleucel (Axi-Cel), for the treatment of large B cell lymphoma (LBCL) in the United States (US)	Oral	M Pasquini

2019 AMERICAN SOCIETY OF HEMATOLOGY (ASH) ANNUAL MEETING

Study	Title	Type	PI
SC17-08a	Tisagenlecleucel chimeric antigen receptor (CAR) t-cell therapy for relapsed/refractory children and young adults with acute lymphoblastic leukemia (ALL): Real world experience from the Center for International Blood and Marrow Transplant Research (CIBMTR) and Cellular Therapy (CT) Registry	Poster	M Pasquini
SC17-08b	Tisagenlecleucel chimeric antigen receptor (CAR) t-cell therapy for adults with diffuse large b-cell lymphoma (DLBCL): Real world experience from the Center for International Blood & Marrow Transplant Research (CIBMTR) Cellular Therapy (CT) Registry	Oral	M Pasquini
SC18-07	Cognitive impairment is associated with inferior survival and increased non-relapse mortality in older allogeneic hematopoietic cell transplant (alloHCT) recipients: A multicenter retrospective study	Poster	R Olin A Artz

2019 TCT MEETINGS

Study	Title	Type	PI
10-CBA	Use of unlicensed unrelated umbilical cord blood expands access to underserved patients: report of 2466 transplants in a racially/ethnically diverse population	Poster	J Miller
Bioinformatics	Efforts of the donor registry to eliminate disparities in access to transplant for underserved patients	Oral	M Maiers
Statistical Methodology	Sensitivity Analysis: E-value technique for time-to-event data research	Oral	MJ Zhang C Zheng ZH Hu
BMT CTN 0702	Minimal residual disease (MRD) assessment before and after autologous hematopoietic cell transplantation (AutoHCT) and maintenance for multiple myeloma (MM): Results of the prognostic immunophenotyping for myeloma response (PRIMeR) study	Oral	T Hahn
BMT CTN 1202	Acute GVHD diagnosis and adjudication in a multicenter trial – a report from the BMT CTN 1202 biorepository study	Oral	R Reshef
BMT CTN 1204	Alemtuzumab controls acute GVHD and mixed chimerism risks in pediatric patients with HLH	Poster	M Krupski

2019 TCT MEETINGS

Study	Title	Type	PI
BMT CTN 1501	Sirolimus vs. prednisone as initial systemic therapy for Minnesota Standard Risk (MN-SR), Ann Arbor 1/2 acute graft-vs-host disease (GVHD): Primary results of the multi-center randomized Phase II BMT CTN 1501 trial	Oral	J Pidala
CK15-03	Comparison of outcomes of allogeneic hematopoietic cell transplantation in patients with acute myeloid leukemia with antecedent history of Philadelphia negative myeloproliferative neoplasm with de novo AML and with AML arising from myelodysplastic syndrome: A study from CIBMTR	Poster	G Murthy
DS16-01	Peripheral blood stem cell collection in one day is preferable to two days in unrelated donors	Poster	J Hsu J Wingard
DS17-02	Impact of autologous blood transfusion after bone marrow harvest on donor health and outcome	Poster	N Farhadfar J Wingard H Murthy
GV17-02	Risk factors for graft-vs-host disease in haploidentical hematopoietic cell transplantation using post transplant cyclophosphamide	Poster	A Im B Hamilton A Rashidi N Majhail S Pavletic D Weisdorf
HS17-01	Association of community health status and center survival for allogeneic hematopoietic cell transplantation	Oral	S Hong H Hong
HSR18-01	What defines and urgent time to transplant? A NMDP survey of transplant physicians and search coordinators unrelated donor selection practices	Oral	K Ballen
HSR18-05	Access to allogeneic hematopoietic cell transplantation for patients with acute myeloid leukemia in the state of Virginia	Oral	K Ballen
IB10-01c	Pre-HCT telomere abnormalities and mortality after unrelated donor HCT for severe aplastic anemia	Poster	Y Wang
IB18-03	Effect of heterozygosity of human leukocyte antigen on outcomes following allogeneic hematopoietic cell transplant for myeloid and lymphoid malignancies	Poster	C Camacho-Bydume K Hsu
LK16-04	HLA-matched sibling versus haploidentical hematopoietic cell transplantation in patients with acute leukemia in first complete remission	Oral	R Romee A Rashidi W Saber M Hamadani

2019 TCT MEETINGS

Study	Title	Type	PI
LY16-02	Post-transplant cyclophosphamide based haploidentical donor vs calcineurin inhibitor based matched related donor reduced intensity conditioning allogeneic hematopoietic cell transplantation for classical Hodgkin lymphoma	Poster	J Kanakry S Ahmed

2019 EUROPEAN FEDERATION FOR IMMUNOGENETICS ANNUAL CONFERENCE

Program	Title	Type	PI
Bioinformatics	Developed of a whole genome QC pipeline for HSCT donor / recipient pairs	Poster	A Madbouly
Bioinformatics	Five locus high resolution HLA allele and haplotype frequencies for Arab donors in the Hadassah Bone Marrow Donor Registry, clinical impact on local and worldwide patients	Poster	A Amal Bishara
Bioinformatics	HLA haplotypes in 250 families: A core NGS testing model for the 17th International HLA and Immunogenetics Workshop	Oral	M Aksar
Bioinformatics	Multiplicative fitness, rapid haplotype discovery, and fitness decay explain evolution of human major histocompatibility complex	Oral	Y Louzoun
Bioinformatics	Efficient sequencing and assembly of diploid KIR haplotypes	Oral	D Roe
Bioinformatics	GRIMM: GRaph IMputation and Matching for HLA Genotypes	Oral	M Maiers
Bioinformatics	Revealing novel complete KIR haplotypes in African Americans using long-read sequencing technology	Oral	C Vierra-Green

2019 AMERICAN SOCIETY OF HISTOCOMPATIBILITY AND IMMUNOGENETICS ANNUAL MEETING

Program	Title	Type	PI
Bioinformatics	HLA genotypes in ethnically diverse sub-Saharan African populations	Poster	M Maiers

2019 CIBMTR MS WORKSHOP

Program	Title	Type	PI
Statistical Methodology	On haplotype association for identification of candidate genes	Oral	T Wang
Statistical Methodology	Confidence band for adjusted survival curves with left-truncated and right-censored time-to-event data and efficient new SAS macro	Oral	MJ Zhang Z Hu

2019 CONFERENCE ON LIFETIME DATA SCIENCE: FOUNDATIONS AND FRONTIERS

Program	Title	Type	PI
Statistical Methodology	Proportional subdistribution hazards model for competing risks in case-cohort studies	Oral	S Kim Y Xu MJ Zhang KW Ahn

2019 DATA STANDARDS AND SYMPOSIUM HACKATHON

Program	Title	Type	PI
Bioinformatics	Introduction to FHIR	Oral	B Milius
Bioinformatics	Clinical Genomic Implementation Guide	Oral	B Milius
Bioinformatics	CIBMTR on FHIR… HML2FHIR	Oral	B Milius

2019 EASTERN NORTH AMERICAN REGION INTERNATIONAL BIOMETRIC SOCIETY SPRING MEETING

Program	Title	Type	PI
Statistical Methodology	Comparison of hypothesis testing methods on random genetic effects in family data	Oral	N DeVogel T Wang
Statistical Methodology	Doubly robust outcome weighted learning estimators for competing risk data with group variable selection	Oral	Y He MO Kim S Kim KW Ahn

2019 EUROPEAN HEMATOLOGY ASSOCIATION CONGRESS

Program	Title	Type	PI
BMT CTN	Impact of conditioning intensity of allogeneic transplantation for acute myeloid leukemia with genomic evidence of residual disease	Oral	C Hourigan

2019 EUROPEAN SOCIETY FOR BLOOD AND MARROW TRANSPLANTATION ANNUAL MEETING

Study	Title	Type	PI
HSR17-01	Patients and caregivers inform the design of a mobile app platform for hematopoietic cell transplantation survivorship care	Poster	K Syrjala
IB18-04	Does donor KIR-genotype impact outcome after unrelated hematopoietic stem cell transplantation for myelodysplastic syndromes or secondary acute myeloid leukemia?	Poster	J Schetelig
LY17-01b	Allogeneic hematopoietic cell transplantation (allo-HCT) in elderly (≥65 years) non-Hodgkin lymphoma (NHL) Patients: a time trends analysis from the Center for International Blood and Marrow Transplant Research (CIBMTR)	Oral	M Hamadani

2019 INTERNATIONAL CHINESE STATISTICAL ASSOCIATION APPLIED STATISTICS SYMPOSIUM

Program	Title	Type	PI
Statistical Methodology	Proportional subdistribution hazards regression for competing risks data under case-cohort studies	Oral	S Kim Y Xu MJ Zhang KW Ahn

2019 JOINT STATISTICAL MEETINGS OF THE AMERICAN STATISTICAL ASSOCIATION

Program	Title	Type	PI
Statistical Methodology	Model checking for subdistribution hazards model under case-cohort design	Oral	S Kim Y Xu MJ Zhang KW Ahn

2019 KIR WORKSHOP

Program	Title	Type	PI
Bioinformatics	A machine learning model that predicts binding between KIR3DL1 and HLA class I allotypes	Poster	M Maiers

2019 UNIVERISTY OF VIRGINA SCHOLAR RESEARCH DAY

Study	Title	Type	PI
HSR18-05	Access to allogeneic hematopoietic cell transplantation for patients with acute myeloid leukemia in the state of Virginia	Poster	K Ballen

APPENDIX F: STUDY DEVELOPMENT AND MANAGEMENT PROCESS

This study development and management process pertains to studies for which the CIBMTR provides data, scientific, and statistical support. Data sets are also made available to investigators who have their own statistical resources. Final analyses and manuscripts resulting from these analyses are reviewed and approved by the CIBMTR prior to journal submission.

STUDY DEVELOPMENT AND MANAGEMENT PROCESS

Planned	**Protocol pending**. Proposals remain in this preliminary stage until the principal investigator (PI) creates a draft protocol.
In Progress	**Draft protocol received**. When a PI submits a draft protocol, Coordinating Center staff review it.
	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.
	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.
	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.

STUDY DEVELOPMENT AND MANAGEMENT PROCESS

In Progress (continued)	**Data file preparation**. The objective of data file preparation is to create a file of eligible subjects who are consecutively treated at participating centers with adequate follow-up, with minimal missing data fields, and in large enough numbers to give the analysis sufficient statistical power to meet the stated study objectives. This process involves a series of steps by the MS-level statistician, working with the Scientific Director, PI(s), and sometimes the Clinical Research Coordinator, to ensure data quality: • Verifying selection criteria • Including and excluding patients so that the investigators can determine whether the final study population is representative of the target population • Assessing follow-up • Determining the extent and nature of missing values and their potential effect on the study • Resolving and reconciling data discrepancies / outliers by examining data collection forms and communicating with centers and the PI
	Analysis in progress. 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.
	Ongoing. 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.
Preliminary Results	**Manuscript preparation**. 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.

STUDY DEVELOPMENT AND MANAGEMENT PROCESS

Preliminary Results (continued)	**Submitted**. 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.
	In press. A publication is in press when it has been approved but does not yet have a citation.
Completed	**Published**. A manuscript is considered published when a citation is available, including a PMCID number, if applicable. For a list of this year's publications, see **Appendix D**.

APPENDIX G: CLINICAL STUDIES AND TRIALS

Through the Clinical Trials Support Program, the Coordinating Center supports clinical trial planning and interpretation; data collection, including long-term follow-up data; and real-time accrual assessment. See **Section 2.3** for more information.

APPENDIX G1: BMT CTN CLINICAL TRIALS OPEN FOR ENROLLMENT

The BMT CTN (**Section 2.3.1**) is the US national trials group charged with developing and conducting multicenter Phase II and III clinical trials focused on HCT. The CIBMTR is the lead institution for the BMT CTN Data and Coordinating Center, which it runs in collaboration with NMDP/Be The Match and the Emmes Company. A status of BMT CTN trials open for enrollment is included in this appendix and is available on the BMT CTN website. For additional information on completed Network trials, see the *annual progress report on the BMT CTN* website.

BMT CTN CLINICAL TRIALS OPEN FOR ENROLLMENT

Protocol Number	Title	Status to Date
BMT CTN 1201 (Alliance A051301)	A randomized double-blind Phase III study of ibrutinib during and following autologous stem cell transplantation versus placebo in patients with relapsed or refractory diffuse large B-cell lymphoma of the activated B-cell subtype	• Opened to accrual Jul 2016 • 38 of 296 patients enrolled • Anticipated accrual completion in mid 2022
BMT CTN 1502	Optimizing cord blood and haploidentical aplastic anemia transplantation (CHAMP)	• Opened to accrual May 2017 • 22 of 30 patients enrolled • Anticipated accrual completion in mid 2020
BMT CTN 1503	A study to compare bone marrow transplantation to standard care in adolescents and young adults with severe sickle cell disease	• Opened to accrual Nov 2016 • 105 of 200 patients enrolled • Anticipated accrual completion in mid 2022
BMT CTN 1506	A multi-center, randomized, double-blind, placebo-controlled Phase III Trial of the FLT3 inhibitor gilteritinib administered as maintenance therapy following allogeneic transplant for patients with FLT3/ITD AML	• Opened to accrual May 2017 • 311 of 346 patients enrolled • Anticipated accrual completion in early 2020
BMT CTN 1507	Reduced intensity conditioning for haploidentical bone marrow transplantation in patients with symptomatic sickle cell disease	• Opened to accrual Oct 2017 • 55 of 80 patients enrolled • Anticipated accrual completion in late 2021

BMT CTN CLINICAL TRIALS OPEN FOR ENROLLMENT

Protocol Number	Title	Status to Date
BMT CTN 1601 (ECOG-ACRIN EA4151)	A randomized Phase III trial of consolidation with autologous HCT followed by maintenance rituximab vs. maintenance rituximab alone for patients with mantle cell lymphoma in minimal residual disease-negative first complete remission	• Opened to accrual Aug 2017 • 204 of 689 patients enrolled • Anticipated accrual completion in mid 2022
BMT CTN 1702	Clinical transplant-related long-term outcomes of alternative donor allogeneic transplantation	• Opened to accrual June 2019 • 150 of 1,732 patients enrolled • Anticipated accrual completion in early 2022
BMT CTN 1703 and 1801 companion study	A randomized, multicenter, Phase III trial of tacrolimus / methotrexate versus post-transplant cyclophosphamide / tacrolimus / mycophenolate mofetil in non-myeloablative / reduced intensity conditioning allogeneic peripheral blood stem cell transplantation Companion Study: Microbiome and immune reconstitution in cellular therapies and hematopoietic stem cell transplantation	• Opened to accrual June 2019 • 51 of 428 patients enrolled • Anticipated accrual completion in early 2022
BMT CTN 1704	Composite health assessment risk model (CHARM) for older adults: Applying pre-transplant comorbidity, geriatric assessment, and biomarkers on non-relapse mortality after allogeneic transplant	• Opened to accrual July 2019 • 70 of 1,100 patients enrolled • Anticipated accrual completion in early 2021
BMT CTN 1706 (SWOG S1803)	Phase III study of daratumumab / rHuPH20 (NSC- 810307) + lenalidomide or lenalidomide as post-autologous stem cell transplant maintenance therapy in patients with multiple myeloma using minimal residual disease to direct therapy duration	• Opened to accrual June 2019 • 13 of 1,100 patients enrolled • Anticipated accrual completion mid 2029

APPENDIX G2: RCI BMT CLINICAL STUDIES

The RCI BMT (**Section 2.3.2**) provides researchers in the field of cellular therapy with infrastructure and expertise in clinical trial conduct and analysis. The program's goal is to help investigators generate data allowing novel and innovative ideas to move into the larger Phase II or Phase III setting into such groups as the BMT CTN or the national cancer cooperative groups. It also facilitates large survey and cohort studies. A status of its projects is included in this appendix.

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
ABA2	Abatacept combined with a calcineurin inhibitor and methotrexate for graft versus host disease prophylaxis	• Closed to accrual Nov 2017 • 186 subjects enrolled • RCI BMT responsible for retrospective adverse event collection / review and ongoing study monitoring
13-TLEC	Prospective non-therapeutic study, assessing the long-term toxicity of HCT for childhood leukemia	• Closed to accrual Mar 2018 • 340 recipients enrolled • Study follow-up complete in Mar 2020
BMT CTN 1102-QOL	A study comparing reduced intensity allogeneic hematopoietic cell transplant to hypomethylating therapy or best supportive care in patients aged 50-75 with intermediate-2 and high risk myelodysplastic syndrome	• Closed to accrual Nov 2018 • 384 subjects enrolled • Survey Research Group performing quality of life assessments
BMT CTN 1102-Ancillary CEA study	A cost effectiveness ancillary study to parent study 1102-QOL above	• Closed to accrual Nov 2018 • 258 subjects enrolled • Collaboration with Fred Hutchinson Cancer Research Center • Survey Research Group performing cost effectiveness analysis (CEA) survey collection
15-MMUD	HLA-mismatched unrelated donor bone marrow transplantation with post-transplantation cyclophosphamide for patients with hematologic malignancies	• Closed to accrual Mar 2019 • 80 subjects enrolled; 3 in 2019 • Study follow-up complete Mar 2020
State Street	Generation of an induced pluripotent stem cell bank immune matched to a majority of the US population	• Study closed Nov 2019 • 8 of 12 whole blood products collected

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
17-PRO	Investigate quality of life in older vs younger patients receiving transplants for myelodysplasia	• Closed to accrual Nov 2019 • 92 subjects enrolled; 62 in 2019 • Companion study to CIBMTR CMS-approved expanded access study • Pilot study for ePRO system
PBSC	Filgrastim-mobilized peripheral blood stem cells for allogeneic transplantation with unrelated donors	• Opened to accrual Apr 1996 • >40,000 unrelated donors enrolled
10-CBA	A multi-center access and distribution protocol for unlicensed cryopreserved cord blood units for transplantation in pediatric and adult patients with hematologic malignancies and other indications	• Opened to accrual Oct 2011 • 4,437 recipients enrolled; 290 enrolled in 2019 • Open indefinitely to allow access and distribution of unlicensed cord blood units
MMP	Millennial member project: Survey Research Group supporting Be The Match Operations project	• First subject contacted by the Survey Research Group in July 2016 • 1,957 registry members / donors completed the study • Phase 2 of the study launching to collect approximately 40 additional interviews
17-CSIDE	Busulfan exposure-finding for SCID	• Opened to accrual Oct 2018 • 6 of 64 subjects enrolled; 5 in 2019
OrcaGraft	Phase 1, dose escalation / expansion study of engineered donor grafts derived from mobilized peripheral blood (OrcaGraft), with single agent GVHD prophylaxis	• Opened to accrual Feb 2019 • RCI BMT overseeing site management and study monitoring
17-SIBS	Identifying predictors of poor health-related quality-of-life among pediatric hematopoietic stem cell donors	• Opened to accrual June 2019 • 32 of 1,876 subjects enrolled
BMT CTN 1702	Clinical transplant-related long-term outcomes of alternative donor allogeneic transplantation (CTRL-ALT-D)	• Opened to accrual June 2019 • 150 of 1,732 evaluable subjects enrolled

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
BMT CTN 1703 / 1801	A randomized, multicenter Phase III trial of tacrolimus / methotrexate versus post-transplant cyclophosphamide / tacrolimus / mycophenolate mofetil in non-myeloablative / reduced intensity conditioning allogeneic peripheral blood stem cell transplantation	• Opened to accrual June 2019 • 56 of 728 subjects enrolled • RCI BMT overseeing site activation and regulatory document management
BMT CTN 1704	Composite health assessment model for older adults: Applying pre-transplant comorbidity, geriatric assessment and biomarkers to predict non-relapse mortality after allogeneic transplantation	• Opened to accrual Jul 2019 • 70 of 1,100 subjects enrolled
16-NTCD	Naïve T cell depletion for prevention of chronic GVHD in pediatric population	• Opened to accrual Sep 2019 • 1 of 66 subjects enrolled
Stanford Kidney Tolerance Arm2	Phase I study of total lymphoid irradiation, anti-thymocyte globulin and purified donor CD34+ T-cell and recipient T regulatory cell transfusion in leukocyte antigen mismatched living donor kidney transplantation	• Opened to accrual Nov 2019 • Pending first enrollment • RCI BMT responsible for data management and monitoring
17-CD33-CAR-T	Phase 1 study of CD33-redirected chimeric antigen receptor T cell immunotherapy in children and young adults with relapsed or refractory acute myeloid leukemia	• Protocol team established June 2017 • IRB approval May 2019 • FDA notification that study may proceed Jul 2019 • First site activation 2019
INSPIRE-SCP	Integrating health informatics in a scalable stepped care self-management program for HCT survivors	• Collaboration with Fred Hutchinson Cancer Research Center • Initial protocol team meeting Feb 2017 • RCI BMT will prepare individualized care plans and contact patients for electronic surveys • Study pending first enrollments in 2019

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
PRO Data Collection	Protocol for collection of patient reported outcomes (PRO) data	• NMDP IRB approval Jul 2019 • PRO instrument build and operational process development underway
Biobank	NMDP biobank cryopreserved bone marrow graft source	• Initial protocol team meeting Oct 2019

APPENDIX H: FORMS SUBMISSION PROCESS

- Center submits CRID Assignment Form (Form 2804), and CRID is generated
- Indication for CRID Assignment Form (Form 2814) is added to Forms Due list
- Center completes Indication Form and reports indication as HCT
- Pre-TED (Form 2400) and Disease Classification (Form 2402) are added to Forms Due list
- Center completes and submits Pre-TED and Disease Classification Forms
- Pre-TED and Disease Classification data are processed through the selection algorithm resulting in CRF or TED track
    - If autologous recipient declines consent for research, **stop here**. Otherwise, follow the appropriate track below

	CRF Track	**TED Track**
1	Forms 2004, 2005, and 2006 are added, depending on donor type and if the donor has been used for a prior transplant.*	Forms 2004, 2005, and 2006 are added, depending on donor type, consent for sample repository, and if the donor has been used for a prior transplant.*
2	Baseline form 2000, disease specific inserts, and Follow-up Forms are added to Forms Due list.	Post-TED Follow-up Form 2450 is added to Forms Due list.
3	Center completes Baseline form after infusion.	Center completes designated Post-TED Forms at appropriate time points.
4	Center completes designated CRF Follow-up Forms at appropriate time points.	Is recipient alive? If yes, go to Step 5. If no, report the death on the follow-up form, and go to Reporting Recipient Death.
5	Is recipient alive? If yes, go to Step 6. If no, report the death on the follow-up form, and go to Reporting Recipient Death.	Did recipient have a subsequent transplant? If yes, go to Step 6. If no, continue reporting at next time point (Step 3).
6	Did recipient have subsequent transplant? If yes, go to Step 7. If no, continue reporting at next time point (Step 4).	Subsequent transplant is reported on the next available follow-up form.
7	When the form reporting the subsequent transplant is in complete status, future forms for the prior transplant will be automatically deleted from FormsNet.	When the form reporting the subsequent transplant is in complete status, future forms for the prior transplant will be automatically deleted from FormsNet.
8	Center completes and submits Pre-TED (Form 2400) and Disease Classification (Form 2402) for subsequent transplant. Go to Step 2 for subsequent transplant.	Center completes and submits Pre-TED (Form 2400) and Disease Classification (Form 2402) for subsequent transplant. Go to Step 2 for subsequent transplant.
Reporting Recipient Death		
Death Form 2900 is completed to report the recipient's death.**		The recipient's death is reported on the Post TED. **A 2900 Death Form should not be completed for patients on the TED track.**

* For more details regarding when Forms 2004, 2005, and 2006 are required, see "How Forms Come Due (2004, 2005, and 2006)".

**Complete Death Form 2900 even if autopsy is pending. Another death form will be requested to confirm cause of death if autopsy was pending.

APPENDIX I: WEBSITES

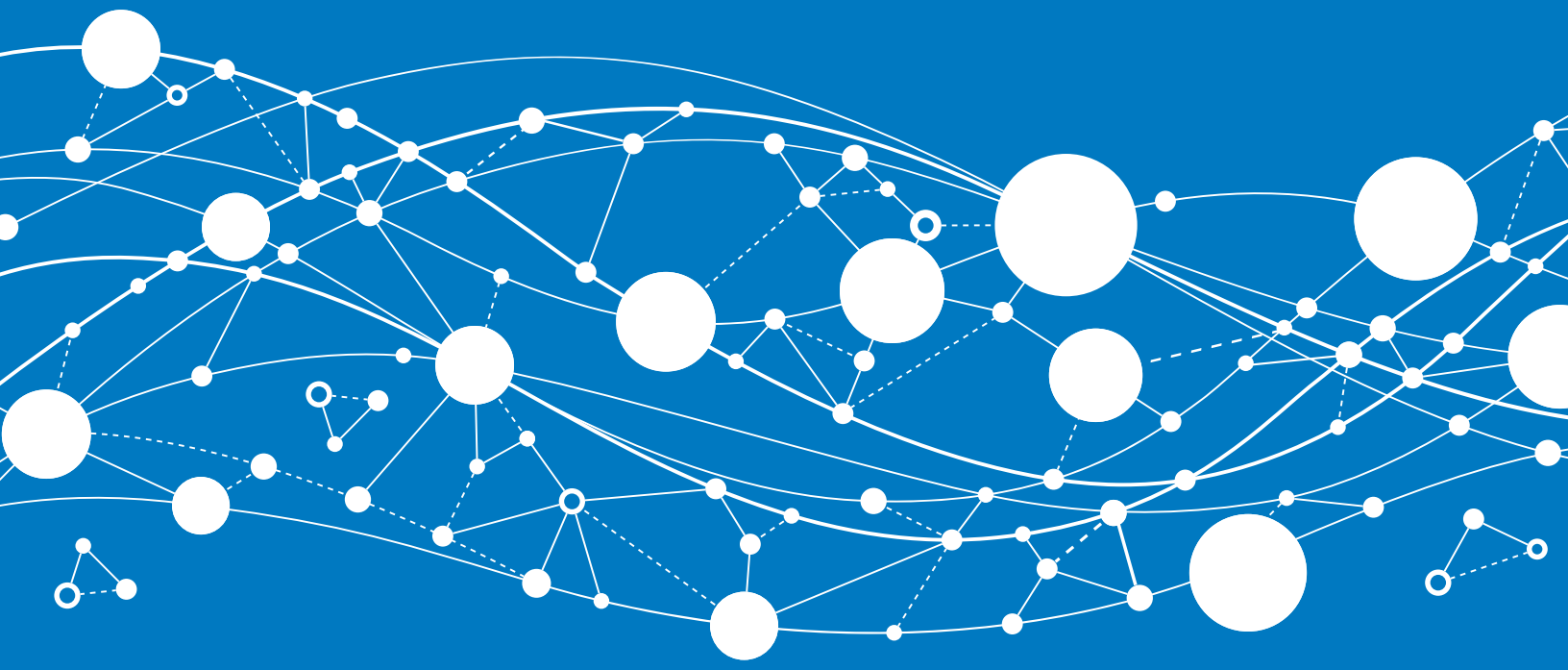
Throughout this report, electronic links to webpages and documents are provided; they are underlined and italicized for identification. If you are unable to access items using the links provided, enter the underlined and italicized words into a search engine. URLs for the websites mentioned in this report are provided here.

Name	URL
Be The Match	bethematch.org
Be The Match Clinical	bethematchclinical.org
BMT CTN	bmtctn.net
CIBMTR	cibmtr.org
CIBMTR Collaborate	collaborate.cibmtr.org
CIBMTR Portal	portal.cibmtr.org
HRSA CWBYCTP	bloodcell.transplant.hrsa.gov

APPENDIX J: GLOSSARY

Abbreviation/ Acronym	Meaning
AGNIS	A Growable Network Information System
alloHCT	allogeneic hematopoietic cell transplantation
AML	acute myeloid (myelogenous) leukemia
ASH	American Society of Hematology
ASTCT	American Society for Transplantation and Cellular Therapy
BMT CTN	Blood and Marrow Transplant Clinical Trials Network
BRIDG	Biomedical Research Integrated Domain Group
BSI	bloodstream infection
CAR	chimeric antigen receptor
CDE	common data elements
CDISC	Clinical Data Interchange Standards Consortium
CED	Coverage with Evidence Determination
CIBMTR	Center for International Blood and Marrow Transplant Research
CIDR	Cellular Immunotherapy Data Resource
CITI	Collaborative IRB Training Initiative
CMS	Centers for Medicare and Medicaid Services
CPI	Continuous Process Improvement
CRF	Comprehensive Report Form
CRID	CIBMTR Recipient Identification Number
CTED	Cellular Therapy Essential Data
CWBYCTP	C.W. Bill Young Cell Transplantation Program
DBtC	Data Back to Centers application
DISCO	Data and Information for Statistical Center Operations
DRI	Disease Risk Index
EAIN	Engagement Award Initiative Notice
EBMT	European Society for Blood and Marrow Transplantation
eDBtC	enhanced Data Back to Centers application
ePRO	electronic patient-reported outcomes
FACT	Foundation for the Accreditation of Cellular Therapy
FDA	Food and Drug Administration
FHIR	Fast Healthcare Interoperability Resources
GDPR	General Data Protection Requirement of the European Union
GVHD	graft-versus-host disease
HCT	hematopoietic cell transplantation
HIPAA	Health Insurance Portability and Accountability Act
HRSA	Health Resources and Services Administration
IBMTR	International Bone Marrow Transplant Registry
IND	investigational new drug
IOTN	Immuno-Oncology Translational Network
IRB	Institutional Review Board

Abbreviation/ Acronym	Meaning
IT	Information Technology
JAMA	Journal of the American Medical Association
JNCI	Journal of the National Cancer Institute
KIR	killer-cell immunoglobulin-like receptors
MCW	Medical College of Wisconsin
MDS	myelodysplastic syndrome
MUD	matched unrelated donor
N/A	not applicable
NCI	National Cancer Institute
NHLBI	National Heart, Lung, and Blood Institute
NIAID	National Institute of Allergy and Infectious Disease
NIH	National Institutes of Health
NMDP	National Marrow Donor Program
PBMTC	Pediatric Blood and Marrow Transplant Consortium
PBSC	peripheral blood stem cell
PCORI	Patient-Centered Outcomes Research Institute
PI	principal investigator
PMCID	PubMed Central unique identifier
PNH	paroxysmal nocturnal hemoglobinuria
PROMIS	Patient Reported Outcome Measurement Information System
QOL	quality of life
RCI BMT	Resource for Clinical Investigations in Blood and Marrow Transplantation
REMS	risk evaluation and mitigation strategy
SCTOD	Stem Cell Therapeutic Outcomes Database
SNP	single nucleotide polymorphisms
TED	Transplant Essential Data
US	United States
VOD	veno-occlusive disease
vs	versus
WBMT	Worldwide Network for Blood and Marrow Transplantation
WMDA	World Marrow Donor Association



The CIBMTR is supported primarily by Public Health Service grant / cooperative agreement U24CA076518 with the National Cancer Institute (NCI), the National Heart, Lung and Blood Institute (NHLBI), and the National Institute of Allergy and Infectious Diseases (NIAID); grant / cooperative agreement U24HL138660 with NHLBI and NCI; grant U24CA233032 from the NCI; grants OT3HL147741, R21HL140314, and U01HL128568 from the NHLBI; contract HHSH250201700006C with Health Resources and Services Administration (HRSA); grants N00014-18-1-2888 and N00014-17-1-2850 from the Office of Naval Research; subaward from prime contract award SC1MC31881-01-00 with HRSA; subawards from prime grant awards R01HL131731 and R01HL126589 from NHLBI; subawards from prime grant awards 5P01CA111412, 5R01HL129472, R01CA152108, 1R01HL131731, 1U01AI126612, and 1R01CA231141 from the NIH; and grants from our corporate and private contributors.

CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and Medical College of Wisconsin.

CIBMTR®
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

Milwaukee Campus
Medical College of Wisconsin
9200 W Wisconsin Ave, Suite C5500
Milwaukee, WI 53226 USA
(414) 805-0700
cibmtr.org

Minneapolis Campus
National Marrow Donor Program/
Be The Match
500 N 5th Street
Minneapolis, MN 55401 USA
(763) 406-5800
cibmtr.org