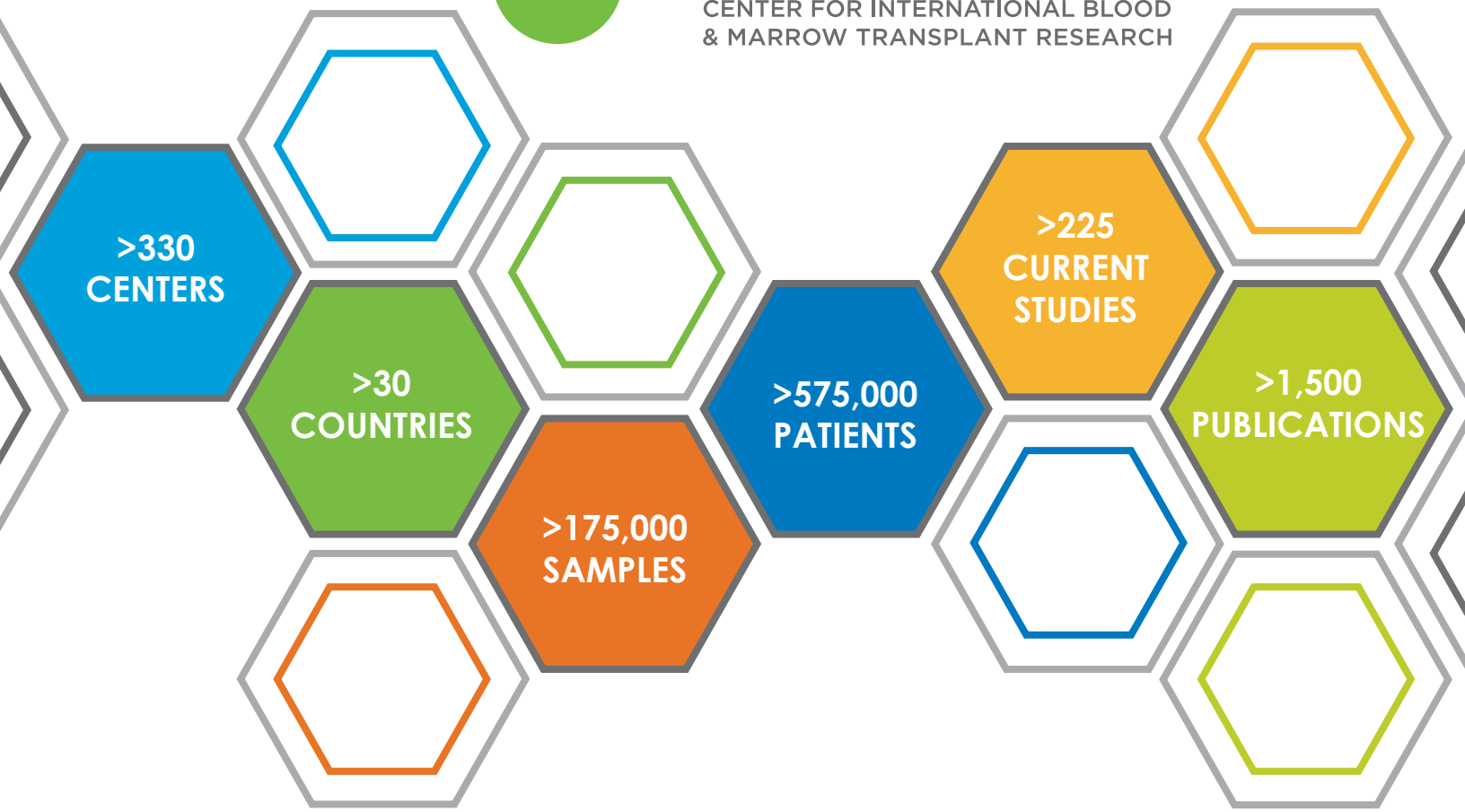
CIBMTR®

CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH



2020 ANNUAL REPORT

Sharing Knowledge.
Sharing Hope.

CIBMTR®
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

2020 Annual Report

January – December

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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.

2020 KEY ACCOMPLISHMENTS

CIBMTR by the Numbers

RESEARCH DATABASE

- >330 participating centers
- >575,000 patients
- ~25,000 new patients annually
- >225 ongoing studies and clinical trials

PUBLICATIONS (Appendix C)

- >1,500 publications since inception
- 89 peer-reviewed publications in 2020
  - 71 from Clinical Outcomes Research Program
  - 10 from Clinical Trials Support Program
  - 3 from Bioinformatics Research Program
  - 3 from Statistical Methodology Research Program
  - 2 from Health Services Research Program

PRESENTATIONS (Appendix D)

- 63 presentations at national and international conferences in 2020 (35 oral and 28 poster)
  - 24 abstracts (9 oral and 15 poster) presented at the American Society of Hematology Annual Meeting
  - 23 abstracts (15 oral and 8 poster) presented at the TCT Meetings of ASTCT and CIBMTR
  - 16 abstracts (11 oral and 5 poster) presented at other national and international conferences

COVID-19 Response

COLLECTING AND MANAGING DATA

- Mechanisms to collect data on COVID-19 infections in place by end of March
- Respiratory Virus Post-Infusion Data Form (2149) launched
- Policies and procedures revised to accommodate centers with limited capacity for data reporting
- Data collection prioritized for consenting patients for research, COVID-19 data, consecutive transplant audit data, and clinical trial data
- Data and specimen collection policies revised to ensure continued collection for BMT CTN and RCI BMT trials
- 29 audits (21 HCT and 8 cellular therapy) conducted remotely

SHARING KNOWLEDGE

- 3 peer-reviewed manuscripts published in Spring 2020 regarding graft cryopreservation and infection risk with tocilizumab
- Expert guidelines published in July 2020 for HCT during the pandemic
- 1,132 COVID-19 infections reported to the CIBMTR; data shared on ***COVID-19 Reported Data*** webpage
- 320 users from 298 centers accessed 1,746 COVID impact reports in new DataOps Dashboard on CIBMTR Portal (Section 3.1.3)
- 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience planned, switching to fully virtual setting

Clinical Outcomes Research Program (Section 2.1)

WORKING COMMITTEES (Section 2.1.1)

- 15 committees with >2,800 researchers worldwide
- 45 global experts voluntarily chair committees
- 153 ongoing studies
- 317 new study proposals
- 36 abstracts (18 oral and 18 poster) presented at national and international conferences
- 60 manuscripts published in peer-reviewed journals

OTHER CELLULAR THERAPY RESEARCH INITIATIVES (Section 2.1.3)

- 4,926 patients
  - 3,453 received CAR T-cell therapy
- 181 participating centers
- 6th Cellular Therapy Registry Forum conducted virtually
- First in-person meeting of the Cellular Immunotherapy for Cancer Working Committee conducted
- Data collection optimized to collect organ toxicities and neurotoxicity
- Form completion time studies conducted to assess burden to sites
- Cellular Therapy Summary Slides published
- First 2 manuscripts published using cellular therapy registry data

Clinical Outcomes Research Program (continued)

Stem Cell Therapeutic Outcomes Database (SCTOD) (Section 2.1.2)

- Center-Specific Survival Report (2016-2018) published
- 22 lay summaries for patients published

GENE THERAPY INITIATIVES (Section 2.1.4)

- Gene therapy data collection initiative launched

MEDICARE CLINICAL TRIALS AND STUDIES (Section 2.1.5)

- 5 Medicare CED studies in progress
- >685 patients received transplants with Medicare reimbursement because of these studies

PATIENT-REPORTED OUTCOMES (Section 2.1.6)

- Protocol launched to centrally and routinely collect patient-reported outcomes data
- 14 patients from 3 centers enrolled

INTERNATIONAL INITIATIVES (Section 2.1.7)

- Data Sharing Agreement and Memorandum of Understanding executed, allowing registry to registry sharing of European Union data
- 53 centers provided with infrastructure support to report outcomes to the Japanese Cellular Therapy Registry
- Data management staff members collaboratively and virtually 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
- 198 centers submitted samples (147 transplant centers, 33 donor centers, and 18 cord blood banks)
- High resolution HLA and KIR typing on 2,666 related and 5,553 unrelated HCT donor / cord and recipient pairs
- 24,974 samples distributed to investigators for various studies
- 12 publications utilized samples from the Research Repository

RESEARCH REPOSITORY (approximate numbers)

- 3,200 new unrelated recipient samples (72,000 overall)
- 1,000 new related recipient samples (12,000 overall)
- 3,000 new adult unrelated donor samples (78,000 overall)
- 1,000 new related donor samples (11,500 overall)
- 300 new unrelated cord blood samples (13,000 overall)

Clinical Trials Support Program (Section 2.3)

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

- 2 trials launched (55 overall)
- >1,600 patients accrued to trials (>13,200 overall)
- 11 open protocols
- 6 new protocols in development
- 47,387 new protocol-related biospecimens (460,200 overall)
- 12 abstracts (7 oral and 5 poster) presented at national and international conferences
- 9 manuscripts published in peer-reviewed journals

Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT) (Section 2.3.2)

- 6 new studies opened to accrual (31 overall)
- >1,400 participants accrued (approximately 45,000 overall)
- 21 active studies supported and 4 upcoming studies in development
- 3 abstracts (2 oral and 1 poster) presented at national and international conferences
- 1 manuscript published in a peer-reviewed journal

Health Services Research Program
(Section 2.4)

- Health care reimbursement, service utilization, and financial responsibility identified among Medicare beneficiaries with multiple myeloma receiving autologous HCT in inpatient and outpatient settings
- State registry and CIBMTR data linked to create a large dataset to address additional novel late effects and health services research questions
- Translating research into practice for survivorship care plans with NMDP/Be The Match
- Qualitative study completed that identified state Medicaid coverage benefits for allogeneic HCT for patients with sickle cell disease
- 3 abstracts (2 oral and 1 poster) presented at national and international conferences
- 2 manuscripts published in peer-reviewed journals

Bioinformatics Research Program
(Section 2.5)

- MDS omics study discovered hypermethylation at *TP53* correlates to higher risk of relapse after HCT for MDS patients
- Service for imputation of *HLA-DPB1* and TCE prediction tool developed and delivered to transplant centers

Bioinformatics Research Program
(continued)

- COVID-19 tool created and released to donor centers to assess need for product cryopreservation based on local COVID-19 infection level
- *HLA-B* Leader Assessment Tool developed for selection of best donors in HLA-mismatched settings
- 3 applications transitioned from research into operations
- 3 abstracts (1 oral and 2 poster) presented at national and international conferences
- 3 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
- 1 oral abstract presented at an international conference
- 3 manuscripts published in peer-reviewed journals

Research Operations

- New CIBMTR Affiliation Agreement executed between NMDP/Be The Match and Medical College of Wisconsin (Section 1.3)
- 459,545 public website unique pageviews (Section 3.1.1)
- *COVID-19 Updates* webpage launched to share up-to-date information about the CIBMTR's response to the COVID-19 pandemic (Section 3.1.1)
- 24 final datasets of published CIBMTR studies shared on the public website for secondary analysis; users downloaded 370 datasets this year (Section 3.1.1)
- 43,029 Secure Portal unique pageviews (Section 3.1.3)
- DBtC extended to include visualization features for non-transplant CAR T patient and infusion data (Section 3.1.3)
- DataOps Dashboard launched on CBMTR Portal, including COVID impact reports, consecutive transplant audit reports, CPI memos, and center-specific analysis reports (Section 3.1.3)
- 17,103 forms for 9,348 patients submitted through AGNIS (Section 3.1.7)
- 17 online data management trainings added / updated (Section 3.3)
- >85 data managers trained through new virtual data manager onboarding (Section 3.3)
- 438 data requests fulfilled (Section 3.4)
- 155,481 patients reported via 344,289 forms in FormsNet (Section 4.2.1)
- 39 forms (34 revised and 5 new) released in FormsNet (Section 4.2.1)

Research Operations (continued)

- 5 pilot partner sites tested a prototype to collect and move data using automation from centers to the CIBMTR as part of the Data Transformation Initiative (Section 4.2.3)
- 31 HCT centers (30 domestic and 1 international) audited (Section 4.3.3)
- 14 cellular therapy audits conducted (Section 4.3.3)
- >100 policies, standard operating procedures, forms, job aides, and plans reviewed for quality assurance and improvement (Section 4.3.4)
- Automated training system identified to connect with the document control system for seamless quality management (Section 4.3.4)

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 **>575,000** 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	The CIBMTR answers clinically important questions using its Research Database, Scientific Working Committees, other cellular and gene therapy initiatives, patient-reported outcomes, and collaboration with research partners.
Immunobiology	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 **>330** participating centers in **>30** countries that submit outcomes-related data for patients. The CIBMTR Coordinating Center, staffed by **>240** employees (**Appendix A**), provides data acquisition and management, information technology (IT), and statistical and scientific support for analyses of these data.

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.

In November 2020, NMDP/Be The Match and MCW executed a revised CIBMTR Affiliation Agreement. The CIBMTR governance and organizational structure are currently under review, based on Affiliation Agreement modifications.

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 includes **>2,800** 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.

The Working Committee structure encourages a collaborative but rigorous methodological approach to CIBMTR clinical outcomes research activities. Each committee is led by 3-4 Chairs, an MD Scientific Director, a PhD Statistical Director, and an MS-level Statistician. Working Committee leadership is listed in **Appendix B5**.

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

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	• Elects members of the Advisory, Nominating, and Clinical Trials Advisory Committees	• Annually during the TCT | Transplantation & Cellular Therapy Meetings of ASBMT and CIBMTR (TCT Meetings of ASTCT and CIBMTR)	Includes representatives from each center that submits CRF-level and / or CTED data
Advisory Committee	• Oversees CIBMTR policies and scientific agenda • Partners with the Working Committees to prioritize scientific studies • Oversees operations of the SCTOD	• In person annually at the TCT Meetings of ASTCT and CIBMTR • By teleconference quarterly and as needed	Appendix B1
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 B2
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 of ASTCT and CIBMTR • By teleconference periodically	Appendix B3

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 B4
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 of ASTCT and CIBMTR • Leadership - by teleconference every 4-8 weeks	Leadership - Appendix B5
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 of ASTCT and CIBMTR • Four times annually by teleconference	Appendix B6
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 of ASTCT and CIBMTR	Appendix B7
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 of ASTCT and CIBMTR • By teleconference as needed	Appendix B8

2.0 WHAT WE DO

The CIBMTR collects data for approximately **25,000** new patients annually in addition to a continually increasing volume of follow-up data for previously reported recipients and donors. Centers submit transplant data at two levels: A Transplant Essential Data (TED) level, which captures key clinical data, and a Comprehensive Report Form (CRF) level, which captures additional detail. Centers submit other cellular therapy data (**Section 2.1.3**) with a suite of Cellular Therapy Essential Data (CTED) forms. The CIBMTR will also soon facilitate data collection for gene therapies (**Section 2.1.4**).

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. Reporting of other cellular therapies is increasing and currently includes about **60%** of patients receiving commercial cell therapy products for hematologic indications. The CIBMTR also receives data for approximately **14,000** non-US patients annually.

The CIBMTR Research Database contains information on **>575,000** patients. **Figure 2.1** shows the continued growth in the number of patients registered with the CIBMTR annually. 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 **>25** 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)
- Other Cellular Therapy Initiatives (Section 2.1.3)
- Gene Therapy Initiatives (Section 2.1.4)
- Medicare Clinical Trials and Studies (Section 2.1.5)
- Patient-Reported Outcomes (Section 2.1.6)
- International Initiatives (Section 2.1.7)

Immunobiology Research (Section 2.2)

Clinical Trials Support (Section 2.3)

- BMT CTN (Section 2.3.1)
- 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 Annually

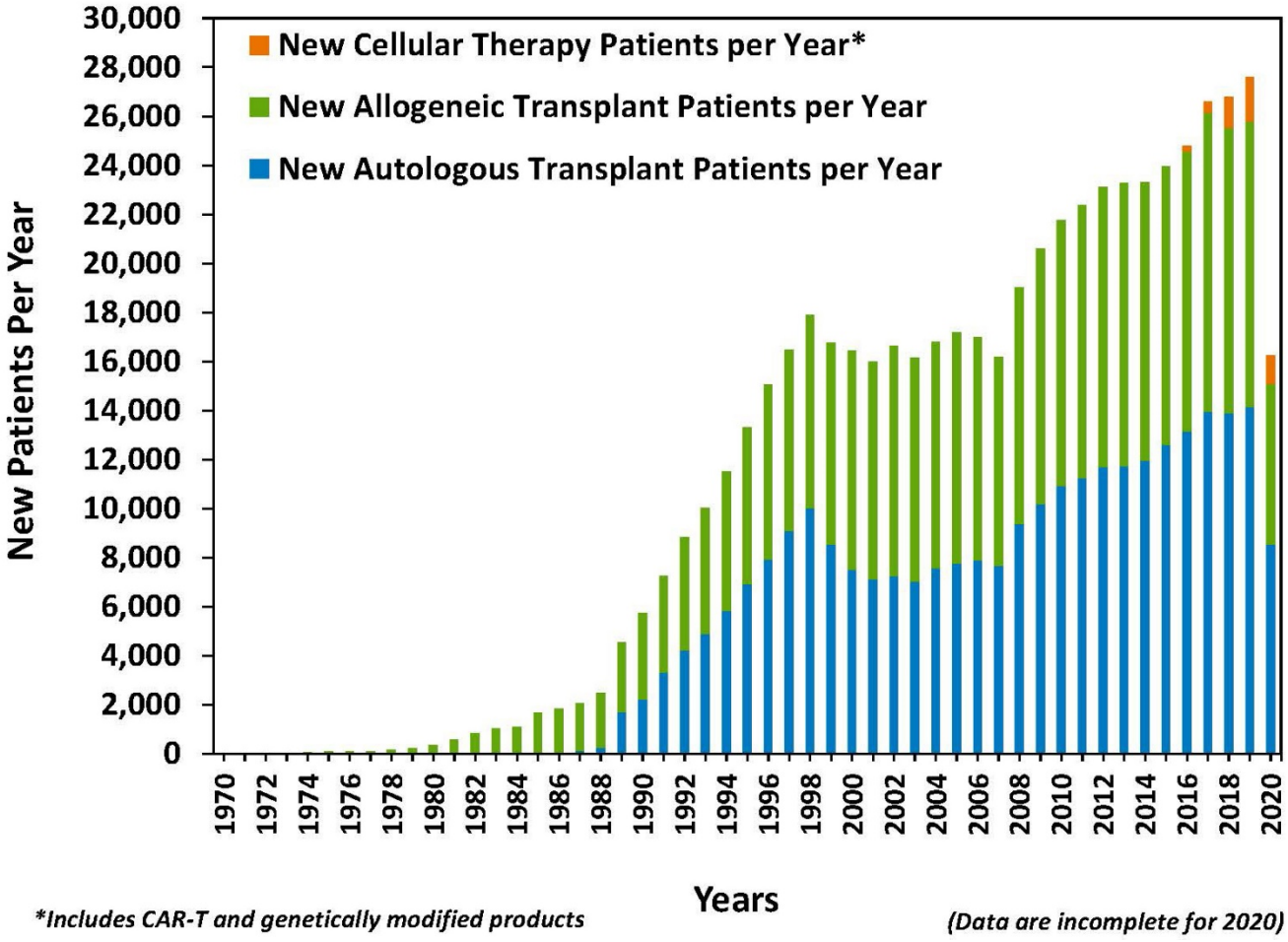


Figure 2.2 Distribution of Patients in the CIBMTR Research Database by Type of Cellular Therapy

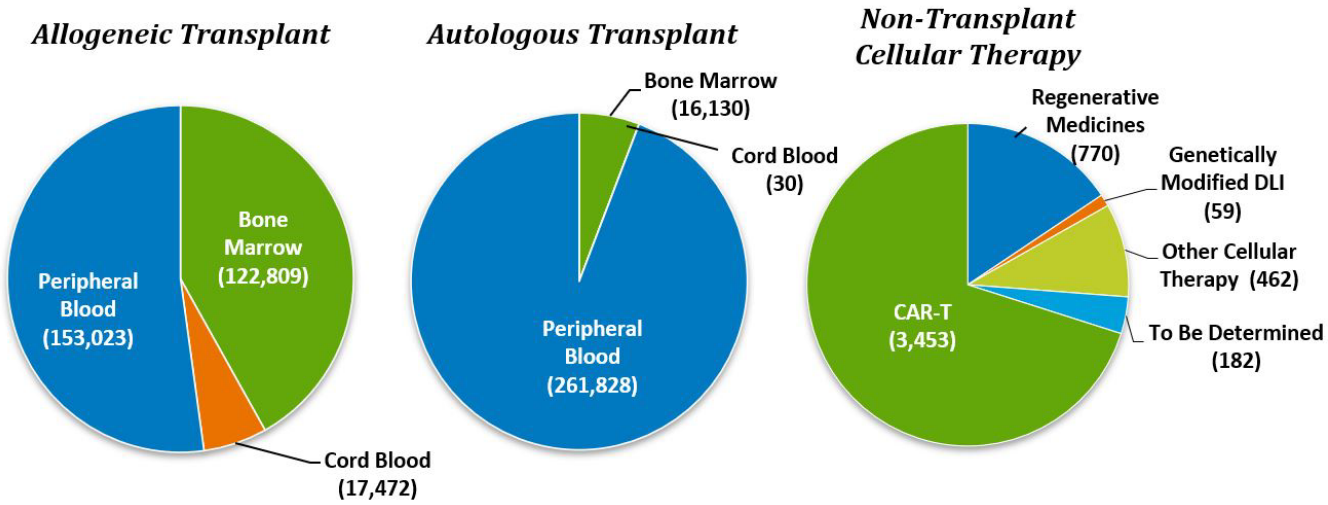


Table 2.3 Distribution of Transplant Patients in the CIBMTR Research Database by Indication

TRANSPLANT DATA	Allogeneic		Autologous		TOTAL
Indication	TED	CRF	TED	CRF	
Lymphoma	19,785	9,701	86,138	16,984	132,608
Plasma cell disorders	5,966	2,782	97,136	17,824	123,708
Acute myeloid leukemia	57,242	32,666	6,180	2,498	98,586
Other malignant diseases[1]	7,401	4,278	33,494	12,842	58,015
Acute lymphoblastic leukemia	28,515	18,771	1,204	492	48,982
Myelodysplastic / myeloproliferative syndromes	19,437	15,963	295	141	35,836
Chronic myelogenous leukemia	15,392	15,481	410	288	31,571
Aplastic anemia / PNH[2]	7,695	8,424	-	-	16,119
Immune disorders[3]	4,491	4,518	-	-	9,009
Hemoglobinopathy[4]	3,559	3,948	-	-	7,507
Inherited bone marrow failure[5]	1,520	1,850	-	-	3,370
Inborn errors of metabolism	1,359	1,660	-	-	3,019
Other nonmalignant disorders[6]	231	174	1,405	180	1,990
Other diseases	389	106	435	42	972
TOTAL	172,982	120,322	226,697	51,291	571,292

1. Includes other leukemia (10,026 allogeneic and 1,005 autologous) and solid tumors (1,653 allogeneic and 45,331 autologous)
2. Includes severe aplastic anemia (15,434 allogeneic) and paroxysmal nocturnal hemoglobinuria (PNH, 685 allogeneic)
3. Includes immune deficiencies (7,113 allogeneic) and histiocytic disorders (1,896 allogeneic)
4. Includes sickle cell anemia (2,501 allogeneic), sickle cell thalassemia (177 allogeneic), and thalassemia major (4,829 allogeneic)
5. Includes Schwachmann-Diamond (92 allogeneic), Fanconi anemia (2,429 allogeneic), Diamond-Blackfan anemia (474 allogeneic), and other inherited abnormalities of erythrocyte (375 allogeneic)
6. Includes platelet disorders (235 allogeneic and 7 autologous) and autoimmune deficiencies (170 allogeneic and 1,578 autologous)

Table 2.4 Distribution of Other Cellular Therapy Patients in the CIBMTR Research Database by Indication

OTHER CELLULAR THERAPY DATA[1,2] Indication	TOTAL
Non-Hodgkin lymphoma	2,635
Neurologic disorder	766
Acute lymphoblastic leukemia	701
Plasma cell disorder / multiple myeloma	276
Acute myeloid leukemia	117
Solid tumor	65
Myelodysplastic syndrome	27
Immune deficiencies	22
Hodgkin disease	22
Inherited abnormalities of erythrocyte differentiation or function	18
Chronic lymphocytic leukemia / prolymphocytic leukemia	12
Chronic myeloid leukemia	6
Severe aplastic anemia	2
Inherited disorders of metabolism	3
Histiocytic disorder	2
Other acute leukemia	2
Other indication	244
Indication not reported	6
TOTAL	4,926

1. Includes 1,773 therapies that were administered post-transplant
2. Excludes unmanipulated donor leukocyte infusion (DLI)

Publications

In 2020, CIBMTR investigators published **89** peer-reviewed manuscripts (**Appendix C**). **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 (32 publications)

Blood Advances (11 publications)

Blood (8 publications)

Cancer (5 publications)

Presentations

In 2020, CIBMTR investigators presented **63** abstracts (**35** oral and **28** poster) at national and international conferences (**Appendix D**).

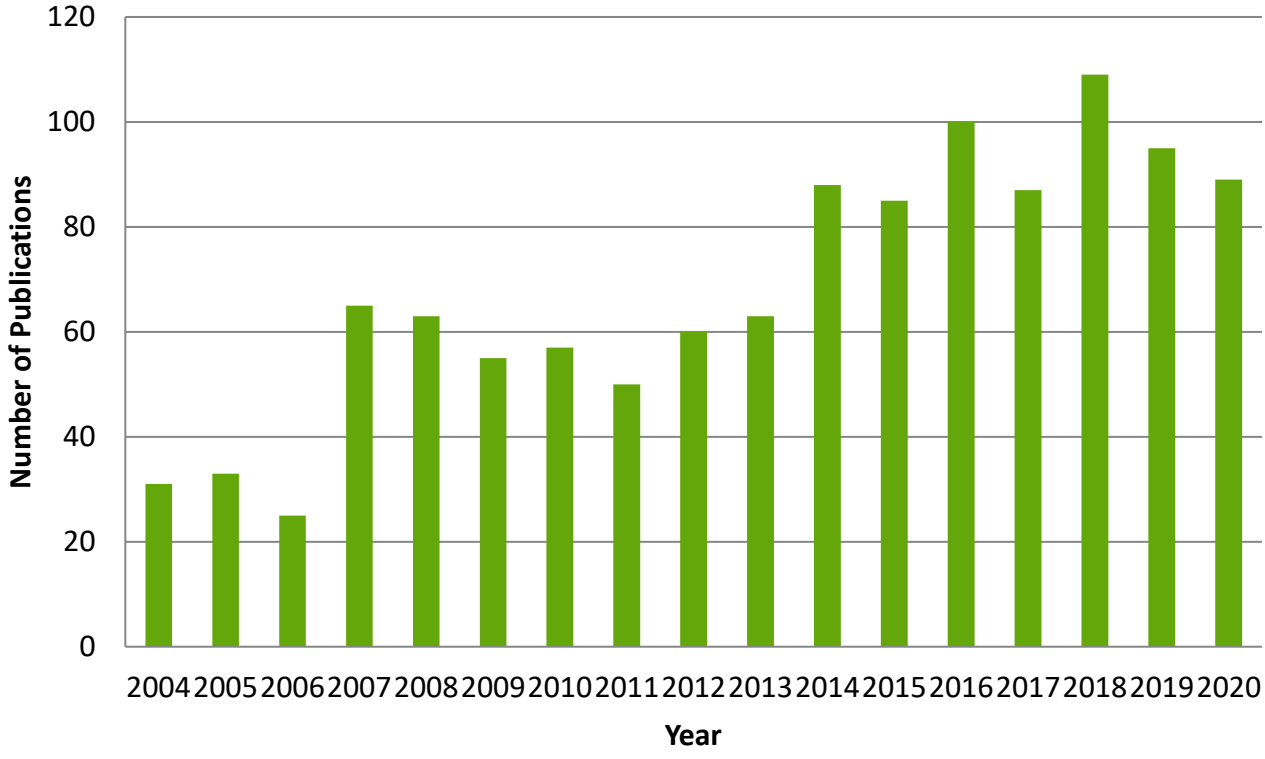
Most Frequent Conferences for CIBMTR Presentations this Year

American Society of Hematology Annual Meeting
(24 abstracts, 9 oral and 15 poster)

TCT Meetings of ASTCT and CIBMTR
(23 abstracts, 15 oral and 8 poster)

American Society of Clinical Oncology Annual Meeting
(4 abstracts, 3 oral and 1 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 **153** studies in progress (**Table 2.6**). In progress and recently published studies are detailed in the Working Committee Research Portfolio linked on the *Working Committee Study Lists* webpage.

The Working Committees reviewed **317** new study proposals before the 2020 TCT Meetings of ASTCT and CIBMTR. **104** proposals were presented at the annual meeting, and **28** were approved. The prioritization and selection process ensures the most important issues can be addressed in a timely manner.

Publications

In 2020, Working Committee investigators published **60** peer-reviewed journal articles, **67%** of CIBMTR publications this year (**Figure 2.7**). A complete list of Clinical Outcomes Research Program publications is provided in **Appendix C1**.

Presentations

In 2020, Working Committee study investigators presented **36** abstracts (**18** oral and **18** poster). A complete list of CIBMTR presentations is provided in **Appendix D**.

Key Working Committee Publications this Year

Fatobene G, Rocha V, St Martin A, et al. **Nonmyeloablative alternative donor transplantation for Hodgkin and non-Hodgkin lymphoma: From the LWP-EBMT, Eurocord, and CIBMTR.** Journal of Clinical Oncology. 2020 May 10; 38(14):1518-1526. Epub 2020 Feb 7. PMC7213591.

Mehta RS, Holtan SG, Wang T, et al. **Composite GRFS and CRFS outcomes after adult alternative donor HCT.** Journal of Clinical Oncology. 2020 Jun 20; 38(18):2062-2076. Epub 2020 May 4. PMC7302955.

Petersdorf EW, Bengtsson M, De Santis D, et al. **Role of HLA-DP expression in GVHD after unrelated donor transplantation.** Journal of Clinical Oncology. 2020 Aug 20; 38(24):2712-2718. Epub 2020 Jun 1. PMC7430213.

Ghosh N, Ahmed S, Ahn KW, et al. **Association of reduced-intensity conditioning regimens with overall survival among patients with non-Hodgkin lymphoma undergoing allogeneic transplant.** JAMA Oncology. 2020 Jul 1; 6(7):1011-1018. Epub 2020 Jun 4. PMC7273311.

Bona K, Brazauskas R, He N, et al. **Neighborhood-poverty and pediatric allogeneic HCT outcomes: A CIBMTR analysis.** Blood. Epub 2020 Oct 26.

Table 2.6 2020 Working Committee Studies

Working Committee	Studies in Progress	Peer-Reviewed Publications	Presentations at International Meetings
Acute Leukemia	**12**	**2**	**4** (2 oral & 2 poster)
Cellular Immunotherapy for Cancer	**10**	**2**	**0**
Chronic Leukemia	**9**	**6**	**3** (1 oral & 2 poster)
Donor Health and Safety	**6**	**6**	**1** (1 oral)
Graft Sources and Manipulation	**4**	**4**	**2** (1 oral & 1 poster)
Graft-versus-Host Disease	**9**	**2**	**1** (1 oral)
Health Services and International Studies	**10**	**6**	**0**
Immunobiology	**34**	**10**	**9** (4 oral & 5 poster)
Infection and Immune Reconstitution	**9**	**1**	**3** (2 oral & 1 poster)
Late Effects and Quality of Life	**12**	**3**	**1** (1 poster)
Lymphoma	**6**	**7**	**4** (4 oral)
Non-Malignant Diseases	**13**	**3**	**1** (1 poster)
Pediatric Cancer	**5**	**2**	**1** (1 oral)
Plasma Cell Disorders and Adult Solid Tumors	**7**	**5**	**2** (2 poster)
Regimen-Related Toxicity and Supportive Care	**7**	**1**	**4** (1 oral & 3 poster)
TOTAL	153	60	36 (18 oral & 18 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). Support for the Cellular Immuno-therapy for Cancer Working Committee is provided by NIH grant # U24CA233032 from the NCI and National Institute on Minority Health and Health Disparities.

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 of ASTCT and CIBMTR in February. Additionally, anyone willing to follow the study development and management process (**Appendix E**) is eligible to propose a study to the Working Committees.

Successful Working Committee Study Proposals are

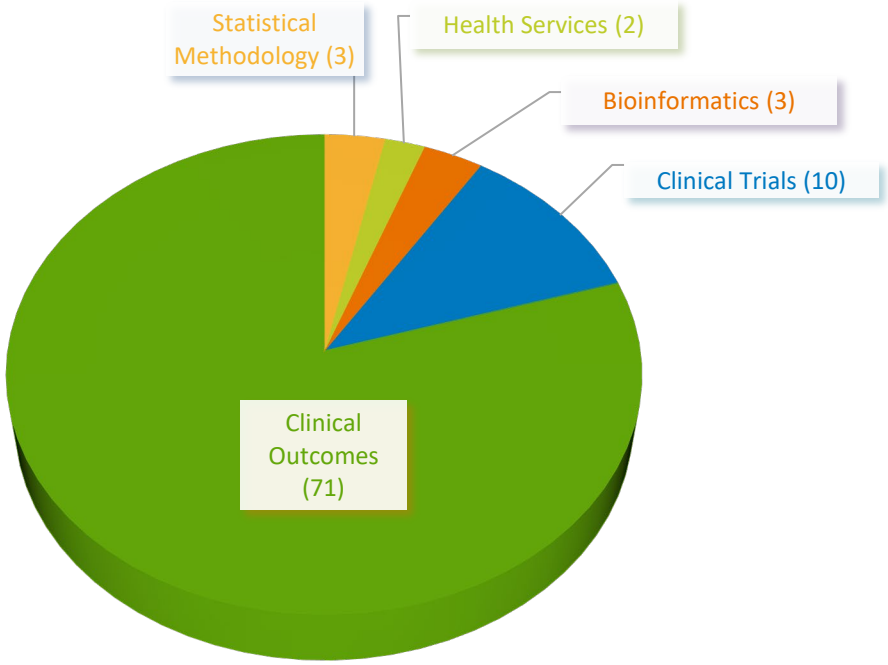
Feasible. Utilize data available in the CIBMTR Research Database.

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 2020 CIBMTR Publications by Program



Clinical Outcomes includes 60 Working Committee publications and 11 other Clinical Outcomes Research publications. Clinical Trials includes 9 BMT CTN publications and 1 RCI BMT publication.

2.1.2 Stem Cell Therapeutic Outcomes Database

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 that assesses transplants from both related and unrelated donors. The most recent analysis was completed in September 2020 and contains information on all first allogeneic transplants performed in US centers from January 1, 2016, through December 31, 2018.

Study Summaries for Patients. The CIBMTR published **89** peer-reviewed publications in 2020. 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, families, and the lay public. This year the CIBMTR published **22** study summaries for patients.

Publicly Available Reports developed by the CIBMTR for the SCTOD

Transplant Outcomes and Data

- ***Transplant Activity Report and Dataset***

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

Center Outcomes Forums. The CIBMTR has conducted **7** *Center Outcomes Forums* to engage relevant stakeholders in the center-specific outcomes reporting process. The most recent meeting was held in November 2020 (**Section 3.2.2**).

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 Other Cellular Therapy Initiatives

Activities

In addition to receiving data on transplant recipients, the CIBMTR received data from **181** centers for **4,926** 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 **3,453** patients who received chimeric antigen receptor (CAR) T-cell therapy and **770** patients who received cells for regenerative medicine indications. **2,635** patients were treated for non-Hodgkin lymphoma; **766** were treated for neurologic disorders, and **701** were treated for acute lymphoblastic leukemia.

Cellular Therapy Registry Forum. In November 2020, the CIBMTR held a sixth Cellular Therapy Registry Forum in a virtual format (**Section 3.2.3**).

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.

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.

CIDR Achievements this Year

Published first 2 manuscripts using cellular therapy registry data

Performed 14 cellular therapy data audits

Conducted form completion time studies to assess burden to sites

Held the first Cellular Immunotherapy for Cancer Working Committee in-person meeting

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 Gene Therapy Initiatives

The CIBMTR formed an internal Gene Therapy Working Group in early 2020 to design and implement data collection requirements for patients receiving gene therapies. The Gene Therapy Working Group is collaborating with an expert task force comprised of national gene therapy experts to determine how to leverage the CIBMTR infrastructure to efficiently collect data for long-term follow-up of gene therapy recipients. The task force's recommendations will inform the Gene Therapy Working Group's final plan and is expected to be completed in early 2021. The CIBMTR anticipates a data collection mechanism will be in place later next year.

2.1.5 Medicare Clinical Trials and Studies

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

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

Table 2.8 Medicare CED Studies

Disease	Patient Population	Enrollment	Dates
Myelodysplastic Syndrome (MDS) (10-CMS-MDS) *NCT# 01166009*	Elderly patients with MDS	**139** centers **5,346** patients **3,640** patients ≥65 years old **1,466** patients 55-64 years old **240** 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 **126** patients	Launched in October 2016
Myelofibrosis (16-CMS-MF) *NCT# 02934477*	Patients aged ≥55 years with primary myelofibrosis or post-essential thrombocythemia / polycythemia vera	**116** centers **278** patients (650 planned) **49** related (matched 6/6) **195** unrelated (matched 8/8) **34** 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	**91** centers **22** 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	**76** centers **8** patients (200 planned)	Launched in November 2017

2.1.6 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, automated tracking and alerting functionality via the CIBMTR's customer relationship management system, 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 as well as outcomes relevant to clinical trials. 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 2019, 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**) consents patients to this protocol and collects 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 4.2.2**) and be available alongside clinical data for future research or to share back to centers.

Core Patient-Reported Outcomes Domains

- **Demographics**
- **Physical function**
- **Fatigue**
- **Sleep disturbance**
- **Pain interference**
- **Anxiety**
- **Depression**
- **Cognitive function**
- **Sexual function**
- **Financial toxicity**
- **Occupational functioning**
- **Ability to participate in social roles and activities**

This year, the CIBMTR developed the core patient-reported outcomes instrument and launched the protocol for routine collection of patient-reported outcomes data, enrolling **14** patients at **3** centers as of December 1, 2020. In 2021, the CIBMTR will expand routine patient-reported outcomes collection to pediatric and Spanish-speaking patients as well as additional centers.

The CIBMTR continues to work closely with all stakeholders in the area of patient-reported outcomes.

2.1.7 International Initiatives

The CIBMTR continues to strengthen its international collaborations with clinical centers and international registries.

European Society for Blood and Marrow Transplantation (EBMT). 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. This year, the CIBMTR and EBMT continued efforts to achieve cellular therapy form harmonization between the two institutions to foment future collaboration.

The recent European Union General Data Protection Requirement (GDPR) legislation made EBMT data sharing challenging. However, after two years of intensive effort, and with a great deal of support from the legal departments of both MCW and NMDP/Be The Match, the CIBMTR and EBMT established a Data Sharing Agreement this year. The organizations executed a Memorandum of Understanding allowing registry to registry sharing of European Union data compliant with the guidelines of the GDPR, primarily on a project by project basis. This is one of the first arrangements in the US allowing sharing of research data with the European Union since GDPR became effective.

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.

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.

Cell Therapy Transplant Canada. The CIBMTR partners with Cell Therapy Transplant Canada to collect data from Canadian centers and return those data to them to support their own national outcomes registry. Using this approach, the centers leverage the CIBMTR's data collection infrastructure and provide the CIBMTR with valuable international data.

The Japan Society for Hematopoietic Cell Transplantation

Japan Society for HCT. The CIBMTR provides infrastructure support for **53** Japanese centers to report cellular therapy outcomes to the Japanese Cellular Therapy Registry. This year the CIBMTR continued work with the Japanese Data Center for HCT 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. There are currently **15** forms translated in Japanese, **8** of which were added or revised in 2020. Data submission began in February 2020.

Pediatric Transplantation & Cellular Therapy Consortium

Pediatric Transplantation and Cellular Therapy Consortium. This consortium includes **>100** pediatric centers in the US, Cananda, New Zealand, and Australia as well as affiliated members in Europe, Asia, and South America. The CIBMTR provides project management of the consortum through the RCI BMT (**Section 2.3.2**).

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 October 2020, CIBMTR staff members and Brazilian data managers provided virtual training at the Brazilian Data Management Conference held at the Brazilian Blood and Marrow Transplant Annual Meeting.

2.1.8 Cure Sickle Cell Initiative

The Cure Sickle Cell Initiative builds a community of patients, advocates, researchers, and scientists to accelerate promising genetic therapies to cure sickle cell disease. Launched in 2018 by NHLBI, the initiative encourages collaboration among researchers, non-profit organizations, and policy making agencies focused on curing the disease. Since 2019, the CIBMTR has worked with the Cure Sickle Cell Data Consortium to build a research data ecosystem designed to support investigator-initiated collaborative research. Each year the CIBMTR delivers to the data ecosystem a shareable research dataset of sickle cell disease HCT outcomes. The CIBMTR also supports the design and launch of gene therapy sickle cell disease clinical trials through the BMT CTN (**Section 2.3.1**).

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

2,933,783 aliquots

17,675 cell lines

77,979 samples from unrelated donors and **11,566** from related donors

72,053 samples from unrelated recipients and **12,031** from related recipients

13,032 samples from unrelated cord blood units

Complete pairs in inventory:

- **44,454** from unrelated adult donor-recipient pairs
- **10,117** from related donor-recipient pairs
- **5,182** from unrelated cord-recipient pairs

Activities

In 2020, **198** centers (**147** transplant centers, **33** donor centers, and **18** 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 **2,666** related and **5,553** unrelated HCT donor / cord and recipient pairs, bringing the total to **>43,500** 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 **24,974** research samples in support of Working Committee studies this year.

Publications

The Immunobiology Research Program supports investigators' publications by providing research samples. **12** manuscripts published this year by Working Committee and BMT CTN investigators utilized samples from the Research Repository.

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.

How to Get Involved

All interested parties may attend the annual in-person meeting of the Immunobiology Working Committee at the TCT Meetings of ASTCT and CIBMTR in February. Additionally, the Immunobiology Working Committee encourages highly translational, hypothesis-driven proposals through the Working Committee Study Proposal Review Process (*How To Propose a Study* webpage).

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 launched **55** trials (including **2** this year) and completed accrual for **44** of these trials. The Network accrued **>13,200** patients to its trials from **>100** centers, including **>1,600** this year. The BMT CTN's Research Sample Repository currently houses **>460,000** biospecimens. Additionally, the Network completed **65** ancillary and correlative studies, with another **49** in progress.

More detailed information about the Network is shared in the annual *Progress Report* on the BMT CTN website. A list of Network trials open for enrollment is provided in **Appendix F1**.

Publications

In 2020, BMT CTN study investigators published **9** peer-reviewed journal articles. These bring the total number of Network publications to **122**, including **30** primary results papers. A complete list of 2020 BMT CTN publications is provided in **Appendix C2**.

Key BMT CTN Publications this Year

Levine JE, Antin JH, Allen CE, et al. **Priorities for improving outcomes for non-malignant blood diseases: A report from the BMT CTN**. Biology of Blood and Marrow Transplantation. Epub 2020 Jan 24.

Costa LJ, Iacobelli S, Pasquini MC, et al. **Long-term survival of 1,338 MM patients treated with tandem autologous vs. autologous-allogeneic transplantation.** Bone Marrow Transplantation. 2020 Sep 1; 55(9):1810-1816. Epub 2020 Apr 14. PMC7483973.

Fuchs E, O'Donnell P, Eapen M, et al. **Double unrelated umbilical cord blood versus HLA-haploidentical bone marrow transplantation (BMT CTN 1101).** Blood. Epub 2020 Aug 31.

DeFilipp Z, Burns LJ, Jaglowski SM, et al. **A New standard in GVHD prophylaxis? An introduction to BMT CTN 1703.** Biology of Blood and Marrow Transplantation. 2020 Dec 1; 26(12):e305-e308. Epub 2020 Sep 10.

Presentations

BMT CTN study investigators presented **12** abstracts (**7** oral and **5** poster) at national and international conferences in 2020. These bring the total number of Network presentations to **116**. A complete list of 2020 CIBMTR presentations is provided in **Appendix D**.

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 or supported the launch of **31** studies, including **6** this year. In 2020, the RCI BMT accrued **>1,400** participants, bringing the total number of accrued participants to approximately **45,000**, 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 **250**. These protocols allow US centers to access peripheral blood and unlicensed cord blood for transplantation.

In addition to **6** newly launched studies, the RCI BMT supported **15** active studies, including **4** BMT CTN protocols (**Section 2.3.1**), and participated in the development of **4** upcoming studies. A list of active RCI BMT studies is provided in **Appendix F2**.

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 2.1.6**) in 2018 to support clinical trials, long-term follow-up, and other research studies with patients and donors. In 2019, the CIBMTR launched 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 **10** active studies and the development of **4** upcoming studies.

Publications

In 2020, RCI BMT study investigators published **1** peer-reviewed journal article. A list of RCI BMT publications is provided in **Appendix C3**.

RCI BMT Publication this Year

Shah NN, Schneiderman J, Kuruvilla D, et al. **Fatal capillary leak syndrome in a child with ALL treated with moxetumomab pasudotox for pre-transplant minimal residual disease reduction.** Pediatric Blood & Cancer. Epub 2020 May 5.

Presentation

RCI BMT study investigators presented **3** abstracts (**2** oral and **1** poster) at national and international conferences in 2020. A complete list of 2020 CIBMTR presentations is provided in **Appendix D**.

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, project management, site selection, site start-up and site management, study monitoring, data 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 **14** 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. 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 HCT reimbursement and out-of-pocket costs for patients with multiple myeloma and a qualitative analysis of state Medicaid coverage for patients with sickle cell disease. Ongoing studies focus on referral patterns, utilization of allogeneic HCT for patients with acute myeloid leukemia (AML), and outcomes of allogeneic HCT for patients with sickle cell disease.

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. Investigators were also involved this year in merging state registry and hospital discharge data with CIBMTR data; these data will be used to evaluate late effects and outcomes. Investigators will conduct additional research with this large valuable dataset in coming years.

Treatment decision-making. Health services research is vital to treatment decision-making, which can lead to better quality of care and improved outcomes. This year, investigators completed one study focused on practice preferences regarding candidacy of older patients for HCT. Ongoing studies focus on patients' perspectives on palliative care.

Publications

In 2020, Health Services Research Program investigators published **2** peer-reviewed manuscripts. A list of program publications is provided in **Appendix C4**.

Presentations

In 2020, program investigators presented **3** abstracts (**2** oral and **1** poster) at national and international conferences. A complete list of CIBMTR presentations is provided in **Appendix D**.

Health Services Research Publications this Year

Dunavin N, Mau L-W, Meyer CL, et al. **Health care reimbursement, service utilization, and financial responsibility among Medicare beneficiaries with multiple myeloma receiving autologous HCT in inpatient and outpatient settings.** Biology of Blood and Marrow Transplantation. 2020 Apr 1; 26(4):805-813. Epub 2020 Jan 6.

Mau L-W, Preussler JM, Burns LJ, et al. **Healthcare costs of treating privately insured patients with acute myeloid leukemia in the US from 2004 to 2014: A generalized additive modeling approach.** PharmacoEconomics. 2020 May 1; 38(5):515-526. Epub 2020 Mar 4. PMC7194165.

Funding

The Health Service Research Program is supported by the HRSA contract # HHSH250201700005C. Individual health services research studies are funded via a variety of mechanisms. *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 *hsrrequests@nmdp.org*.

2.5 BIOINFORMATICS RESEARCH PROGRAM

The Bioinformatics Research Program specializes in matching patients and cellular therapies. The team saves lives by researching what, where, and how to match patients and cellular therapies. 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

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 projections worldwide to improve donor recruitment and biobanking practices and target the unmet need from patients

Publications

In 2020, Bioinformatics Research Program investigators published **3** peer-reviewed manuscripts. A complete list of program publications is provided in **Appendix C5**.

Key Bioinformatics Publications this Year

Roe D, Williams J, Ivery K, et al. **Efficient sequencing, assembly, and annotation of human KIR haplotypes**. Frontiers in Immunology. 11:582927. Epub 2020 Oct 9. PMC7581912.

Roe D, Vierra-Green C, Pyo C-W, et al**. A detailed view of KIR haplotype structures and gene families as provided by a new motif-based multiple sequence alignment**. Frontiers in Immunology. 11:585731. Epub 2020 Nov 18. PMC7708349.

Presentations

Bioinformatics study investigators presented **3** abstracts (**1** oral and **2** poster) at national and international conferences in 2020. A complete list of CIBMTR presentations is provided in **Appendix D**.

Funding

External support for the Bioinformatics Research Program is primarily provided by grant # N00014-18-1-2850 from the Office of Naval Research.

How to Get Involved

For more information about the Bioinformatics Research Program, contact Yung-Tsi Bolon, Director of Immunobiology and 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 2020, PhD-level biostatisticians in the CIBMTR published **3** peer-reviewed statistical methodology manuscripts. A list of program publications is provided in **Appendix C6**.

Key Statistical Methodology Publications this Year

Kim S, Logan B, Riches M, et al. **Statistical methods for time-dependent variables in HCT studies**. Biology of Blood and Marrow Transplantation. Epub 2020 Oct 2.

DeVogel N, Auer PL, Manansala R, et al. **A unified linear mixed model for familial relatedness and population structure in genetic association studies**. Genetic Epidemiology. Epub 2020 Nov 11.

Presentations

Statistical Methodology Research Program investigators presented **1** oral abstract at an international conference in 2020. A complete list of CIBMTR presentations is provided in **Appendix D**.

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 of ASTCT and CIBMTR 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 COLLABORATION WITH INDUSTRY

The CIBMTR provides critical support to industry, an essential 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 various 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 of ASTCT and CIBMTR**
- **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 2020, the CIBMTR engaged with **22** corporate partners and oversaw a portfolio of **65** active projects. Currently, **23** organizations participate in the CIBMTR Corporate Membership Program.

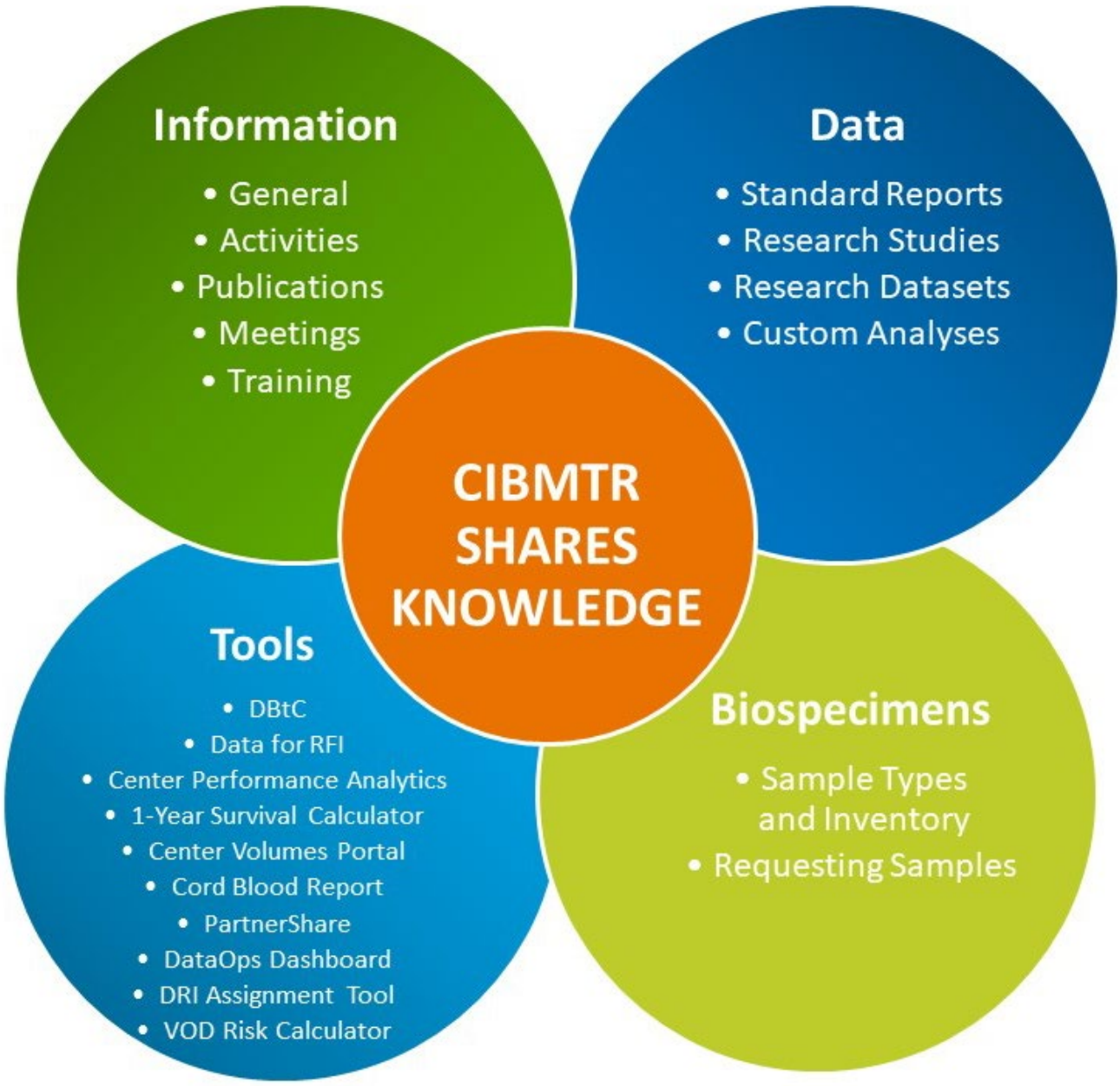
How to Get Involved

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 **>1,200** studies, **>1,500** publications, and **>3,500** authors and their institutions at time of publication. In 2020, the CIBMTR public website had **459,640** unique page views.

COVID-19 Updates

(**6,073** unique page views in 2020)
Provides up-to-date information about the CIBMTR's response to the COVID-19 pandemic, including reported data, data collection, published study results, ongoing studies, communications, and additional resources. This webpage was launched in March 2020.

COVID-19 Reported Data

(**754** unique page views in 2020)
Shares data received by the CIBMTR regarding COVID-19 infections in cellular therapy patients; includes data breakdowns by patient sex, type of cellular therapy, age at infection, US region, and time from infusion. This webpage is updated every weekday.

About CIBMTR

Administrative and Progress Reports

(**1,206** unique page views in 2020)
Provides access to the CIBMTR's Facts and Figures document, Annual Progress Report, and Manual of Operations.

Studies

Working Committee Studies Lists

(**6,182** unique page views in 2020)
A summary of the planned, in-progress, and recently published clinical outcomes studies for each Working Committee.

Meetings

Meeting Materials

(**14,882** unique page views in 2020)
Provides access to agendas, handouts, and educational materials from specific meetings at the TCT Meetings of ASTCT and CIBMTR, 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

(**14,578** unique page views in 2020)
Includes charts and figures summarizing current uses and outcomes of allogeneic and autologous HCT as well as other cellular therapies.

Web-based US Transplant Reports

(**11,395** unique page views in 2020)
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

(**9,211** unique page views in 2020)
Searchable descriptive list of **>1,500** publications resulting from the use of CIBMTR data and statistical resources.

Research Datasets for Secondary Analysis

(**873** unique page views in 2020)
Shares the final datasets of published CIBMTR studies for secondary analysis. The CIBMTR released **24** datasets, and users downloaded **370** datasets this year.

Newsletters

(**5,398** unique page views in 2020)
Published **4** times per year. Articles feature updates on research programs, data management and collection, and noteworthy events in the cellular therapy community.

Patient Resources

(**5,535** unique page views in 2020)
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 stakeholders plan for the specialized care of transplant recipients. The CIBMTR published **22** study summaries for patients this year.

Statistical Resources

(**11,706** unique page views in 2020)
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

(**7,675** unique page views in 2020)
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 Collection Forms

(**47,471** unique page views in 2020)
Provides access to current and retired versions of forms used by the CIBMTR to collect standard data elements for all cellular therapy recipients.

Training

(**10,462** unique page views in 2020)
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.

3.1.3 CIBMTR Portal Site

The CIBMTR Portal site (***portal.cibmtr.org***) delivers applications and data to CIBMTR centers and other partners. In 2020, external visitors accessed **43,029** Portal sessions. **8** applications are currently hosted on this site:

DBtC - Data Back to Centers

DBtC provides centers with self-service access to their most commonly used CRF- and TED-level data, including descriptive statistics and outcomes. DBtC enables centers to view and filter outcomes, including a 5-year overall survival curve as well as acute graft-versus-host disease (GVHD), chronic GVHD, and other outcomes, for the center's population.

This year the CIBMTR extended DBtC to include visualization features for non-transplant CAR T patient and infusion data. These data include patient demographics, indications for therapy, outcomes, and a risk evaluation and mitigation strategy (REMS) report, which allows centers to fulfill sponsor reporting and regulatory compliance needs. The CIBMTR also finalized consolidating the original DBtC and enhanced DBtC features into a single application, known as DBtC.

In 2020, **496** unique users from **328** different centers accessed **3,463** DBtC sessions, and **397** users from **301** centers downloaded data **2,443** times.

A separate application – DBtC Japan – provides Japanese stakeholders with a secure self-service download portal. In 2020, **2** users representing **48** different centers accessed **50** distinct sessions in the DBtC Japan application.

Data for RFI

Data for RFI (Request for Information) 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 DBtC but translates these to the standard RFI format developed by the American Society for Transplantation and Cellular Therapy (ASTCT). In 2020, **171** users representing **177** centers accessed **370** Data for RFI sessions.

Center Performance Analytics

The CIBMTR supports 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. Centers can also view their center's own 1-year survival rate, based on a rolling 3-year period of data included in the Transplant Center Specific Survival dataset. In 2020, **221** users from **186** centers accessed **875** 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 2020, **2,376** users accessed the survival calculator **3,329** times.

Center Volumes Portal

The Center Volumes Portal allows centers to preview, correct, and approve center volume data published annually on the *HRSA CWBYCTP* website. Centers may display and download the previous five years of volume data (2014-2018). In 2020, **3,649** authorized users accessed **5,076** Center Volumes Portal pages.

Cord Blood Report

The Cord Blood Report application 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 2020, **36** users from **53** cord blood banks accessed **231**Cord Blood sessions.

Forms Reimbursement Financial Payments Dashboard

The Forms Reimbursement Financial Payments Dashboard offers transplant centers a self-service application to securely download their monthly financial CIBMTR forms reimbursement reports. This portal provides the most current information to users and also serves as an archive of prior reports . In 2020, **244** users from **193** centers accessed **820** Forms Reimbursement Financial Payment sessions.

PartnerShare

Updated monthly, PartnerShare is a secure, commercial partner facing application that provides partners with access to deidentified data regarding their products from all centers. 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. Since its launch, **3** users from **2** partner organizations accessed **33** PartnerShare sessions.

DataOps Dashboard

Updated weekly, the DataOps Dashboard is a secure center-facing application that provides centers access to files routinely sent via email from CIBMTR to the centers. The files currently included are COVID impact reports, consecutive transplant audit reports, continuous process improvement memos, and center specific analysis reports. Since its launch in May 2020, **396** users from **325** centers accessed **2,259** DataOps Dashboard sessions. With the pandemic, COVID impact reporting was of particular importance to centers this year; **320** users from **298** centers accessed **1,746** COVID impact reports.

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 recipients, 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

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 (A Growable Network Information System) allows participating centers to electronically collect and share data with the CIBMTR. Data are entered once and then distributed and synchronized among databases. In 2020, **17,103** forms for **9,348** patients were submitted through AGNIS by **16** US centers and **3** 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.

Disease Risk Index (DRI) Assignment Tool

Launched in 2015, the DRI Assignment Tool is based on peer-reviewed literature (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 2020, **6,429** users accessed the DRI Assignment Tool **22,402** times.

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.

As part of the Data Transformation Initiative (**Section 4.2.3**), the CIBMTR successfully deployed a custom application to exchange patient demographics, GVHD observations, and laboratory results directly from center electronic health records using FHIR. Currently, this application consumes patient demographic data into FormsNet and assigns a unique recipient ID (CRID) to each patient record. Consumption of GVHD observations and laboratory results into FormsNet will occur next. The CIBMTR also started to receive HLA typing directly from laboratory databases using FHIR. The application consumes these results directly into FormsNet.

This year the CIBMTR expanded the collaborative phase of this project, coordinating with The Ohio State University, Moffitt Cancer Center, Children's Hospital of Colorado, Oregon Health & Science University / Knight Cancer Institute, and Sarah Cannon Research Institute. Over the coming months, the CIBMTR will prioritize additional data resources for electronic exchange and consumption into FormsNet.

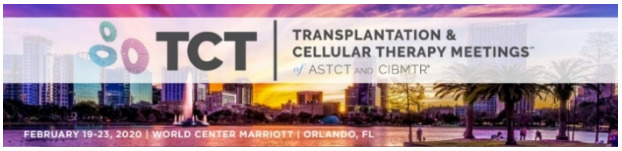
VOD Risk Calculator

Launched in 2017, the Veno-Occlusive Disease (VOD) Risk Calculator is based on peer-reviewed literature (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 2020, **1,864** users accessed the VOD Risk Calculator **6,378** times.

3.2 ANNUAL MEETINGS

3.2.1 TCT Meetings of ASTCT and CIBMTR

The TCT Meetings of ASTCT and CIBMTR, 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.



2020 TCT Meetings of ASTCT and CIBMTR

With **>4,100** attendees from **48** countries, the 2020 TCT Meetings of ASTCT and CIBMTR included **6** plenary sessions, **9** concurrent sessions, **90** oral abstracts, **2** poster sessions, **9**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 **300** attendees, this conference provided forms and subject matter training, which increases the accuracy with which CIBMTR forms are completed. In addition, the CIBMTR offered New Data Manager Onboarding (**Section 3.3**).

BMT CTN Coordinators and Investigators Meetings

With approximately **200** and **>100** attendees, respectively, these meetings focused on study management, including form updates, data quality and data transformation, and specific diseases and conditions.

Administrative Director Conference

With approximately **350** attendees, this conference focused on topics related to leadership, including quality, economics, staffing models, and Medicare coverage.

Pharmacists Conference

With **>350** attendees, this conference presented the latest research and best pharmacy practices with a focus on specific diseases and therapies.

Nursing Conference

With approximately **400** attendees, this conference presented current healthcare trends in cellular therapy for the nurses caring for patients receiving these therapies. Sessions covered new therapies, side effect management, psychosocial issues, abstract presentations, and topic-specific roundtables during which attendees were able to network and learn from each other.

Clinical Education Conference

Designed for advanced practice providers, fellows, and junior faculty, this conference of nearly **400** attendees focused on providing a deeper understanding of the diagnostic evaluation and therapeutic treatments for acute and chronic complications of HCT.

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.

The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience

The 2021 TCT Meetings of ASTCT and CIBMTR will be fully virtual due to the COVID-19 pandemic. Approximately **3,500** attendees are expected to participate virtually. The meetings will include **6** plenary sessions, **9** concurrent sessions, **90** oral abstracts, **1** late breaking abstract session, **2** poster highlight sessions, **7** corporate-supported symposia, and **8** product theaters.

3.2.2 Center Outcomes Forum

In November 2020, the CIBMTR conducted a seventh *Center Outcomes Forum* to engage relevant stakeholders in the center-specific outcomes reporting process. Topics particularly relevant to the center-specific survival analysis included discussion about minimal residual disease for acute leukemia for use as a risk adjustment factor, adjusting for the impact of the COVID-19 pandemic on analyses of HCT outcomes, accounting for social determinants of health, research project proposals related to impacts of public reporting, and consequences of public reporting.

3.2.3 Cellular Therapy Registry Forum

In November 2020, the CIBMTR held a sixth Cellular Therapy Registry Forum. Participants encompassed **>260** attendees representing a variety of industries, including clinical care, research, pharmaceuticals, insurance providers, professional organizations, and government representatives.

Cellular Therapy Registry Forum Topics

CAR T-CELL TOXICITIES

- Converting among different cytokine release syndrome grading criteria
- Evolution of REMS program

CHALLENGING SITUATIONS AT CENTERS

- Numerous accreditation bodies, audits, and data collection and training requirements

LONG-TERM FOLLOW-UP FOR GENE THERAPIES

- Sickle cell disease
- CIBMTR platform for data collection

REAL WORLD DATA EXPERIENCE

- Data transmission prototype
- Innovative surveillance monitoring for biologics
- ePRO data collection

SOLID TUMOR TASK FORCE

- Recommendations

3.3 DATA MANAGEMENT TRAINING

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

CIBMTR Center Support
The CIBMTR implemented a web-based Customer Service Center in 2019. CIBMTR Center Support delivers a transparent, flexible, and service-oriented experience, allowing centers to enter questions and requests in a single location. Job aids, reference guides, and training videos for transplant, apheresis, collection, and donor centers are available on the *CIBMTR Center Support* webpage.

The new system allows CIBMTR Data Operations to unify multiple processes and tools into a consistent, integrated system; simplify centers' experience while expanding support options; and increase internal efficiency, resolve issues more quickly, and drive continuous service improvement with better data and metrics. A recent survey indicated **97%** of respondents had a positive experience with the CIBMTR Center Support application, and **95%** felt their questions, requests, and / or issues were resolved in a timely manner.

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

Data Management Guide
Review detailed information regarding participation in CIBMTR research, center membership, access to FormsNet, data manager education, mentor program, forms submission process, and useful tips and links.

Manuals
Find answers to data submission questions by accessing the *Forms Instructions Manual*, which provides detailed instructions for submitting data based on 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.

New Data Manager Onboarding
Geared towards data managers with **<6** months experience, this training is offered twice per year. The CIBMTR held the first training at the 2020 TCT Meetings of ASTCT and CIBMTR and the second virtually in September 2020; **>85** new data managers participated.

Online Trainings New / Updated this Year

APPLICATION SERIES

- FormsNet3 Recipient Application Training
- Query Functionality

FORM SERIES

- An Overview of Cellular Therapy Forms
- Baseline Form 2000
- Cellular Therapy Forms Submission
- Cellular Therapy Reporting
- CIBMTR Research ID Assignment
- Pre-TED Form 2400
- Submitting Cellular Therapy Data to CIBMTR

HLA SERIES

- HLA Reporting: Confirmation of HLA Typing – Form 2005

DISEASE SPECIFIC FORMS

- Multiple Myeloma 101 – Understanding the Basics & Relevant Assessments
- Multiple Myeloma 101 – Reporting Myeloma Disease Status
- MDS and MDS/MPN Reporting
- MPN Reporting

IRB

- NMDP IRB New Member Orientation – Module 2

GENERAL TOPICS

- Research Sample Submission

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 2020, the CIBMTR fulfilled **438** requests for information and data (**Table 3.2**).

Table 3.2 Data Requests Addressed by the CIBMTR in 2020

Requestor	Number of Requests
Physician / Researcher	268
Physician for Patient Care	55
Patient or Relative	27
Commercial Organizations	27
Student	12
Market Research Firm	7
Federal Government Agency	6
News Media	3
State Government	1
Medical Society	1
Insurance Company	1
Be The Match Operations	30
TOTAL	438

How to Access

For more information about requesting data from the Research Database, visit the *CIBMTR How to Request Information* 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 on the CIBMTR website (**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 of ASTCT and CIBMTR* 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 >1,500 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 Information 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*, 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 *Research Datasets for Secondary Analysis* webpage.

CUSTOM ANALYSES (Section 3.4)
Complete the *Corporate Member Information Request Form* or *Custom Information Request Form* for non-research / publication purposes.

OTHER
Email *inforequest@mcw.edu*

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

Tools
on the *CIBMTR Portal site*

DBtC
Download your CRF- and TED-level as well as CAR T-cell therapy variables.

DATA FOR RFI
Access, view, reconcile, and export into the ASTCT standard RFI format the data your center submitted.

CENTER PERFORMANCE ANALYTICS
Compare your center's data to aggregated center data filtered by various factors, view your own one-year survival rate, or create and implement ad hoc queries.

1-YEAR SURVIVAL CALCULATOR
Predict one-year survival for individual allogeneic HCT recipients.

CENTER VOLUMES PORTAL
Preview, correct, and approve center volume data published on the HRSA CWBYCTP website.

CORD BLOOD REPORT
Access monthly predefined reports regarding the quality and safety of distributed cord blood units.

PARTNERSHARE
Provides robust visualizations for navigating data in commercial partners' data sets.

DATAOPS DASHBOARD
Download data regarding COVID impacts, consecutive transplant audits, and transplant-center specific analysis.

Tools
on other CIBMTR webpages

DRI ASSIGNMENT TOOL
Categorize patients undergoing allogeneic HCT for hematologic malignancy by disease risk on the *DRI Assignment Tool* webpage.

VOD RISK CALCULATOR
Identify patients at high risk for VOD on the *VOD Risk Calculator* webpage.

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

Biospecimens

SAMPLES TYPES AND INVENTORY
Review the >2.9 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 as well as other cellular therapies; developed in conjunction with the TCT Meetings of ASTCT and CIBMTR	Published on the *CIBMTR Slides and Reports* 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 >330 centers that participate 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 Transplant Activity Report	Report containing patient, disease, donor HLA match, donor age, and gender match information for allogeneic and autologous transplant activity in the US since 2010	Via Corporate Membership Program (**Section 2.7**) Released: November Format: PDF

Report Title	Description	Accessibility*
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 Released: December Format: Web
CIBMTR Newsletter	Articles regarding Working Committees, the TCT Meetings of ASTCT and CIBMTR, research programs, 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

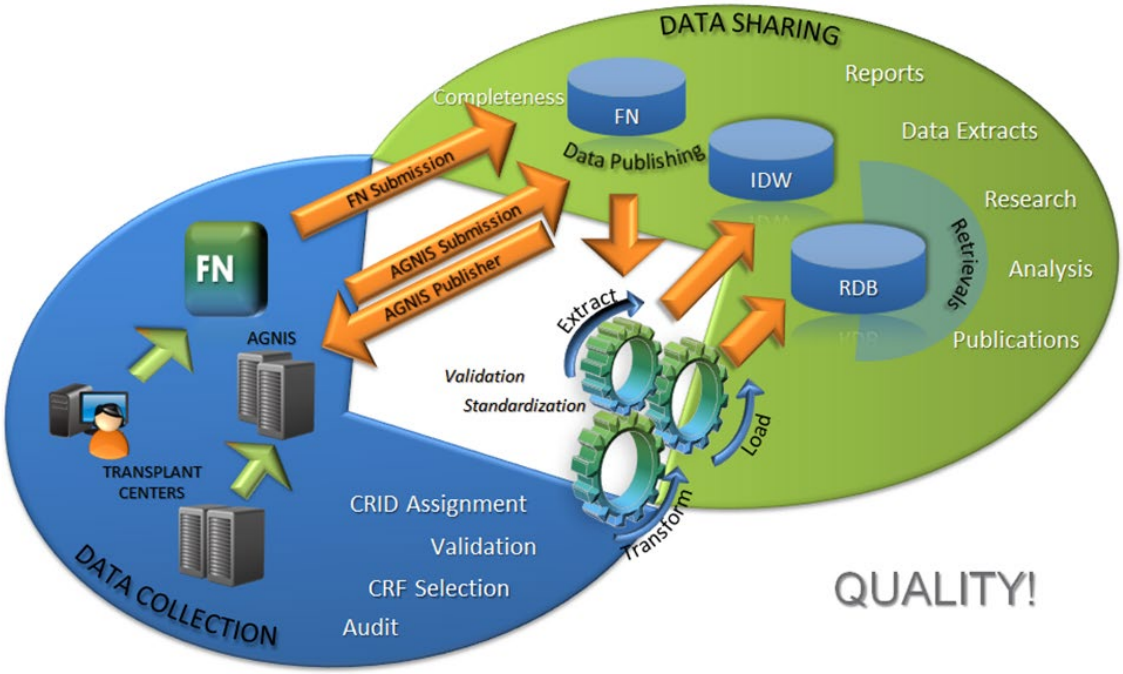
The Research Data Life Cycle (**Figure 4.1**) describes the path of data from the point of submission to its ultimate use in analyses, reports, and publications.

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 were 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 HCT data as well as CAR T infusion data are also directly available to centers through the DBtC 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 **99%** of data collected by the CIBMTR is submitted electronically via FormsNet, a comprehensive electronic data submission system containing **150** forms related to capturing cellular therapy outcomes for donors and recipients. This year, the CIBMTR received data through FormsNet for **155,481** patients via **344,289** forms. The CIBMTR continues to update and enhance the application each year. This year the CIBMTR released several new forms and revised **>25** existing forms in a continuing effort to maintain alignment between data collection and treatment practices as the cellular therapy field changes.

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 enhanced integration of data sources in several ways. The CIBMTR automated transfer of patient-reported outcomes data collected in Qualtrics to the data warehouse. The CIBMTR also continued expanding the newly implemented unified data model. This model consumes, stores, maintains, and extracts cellular therapy outcomes data for CAR T-cell research. This year the CIBMTR created a prototype to incorporate HCT data currently stored in the CIBMTR Research Database, making HCT data out of the unified domain model available to statisticians for research purposes. The CIBMTR also expanded the unified domain model architecture to enable consumption of HLA, ePRO, and Rave data, providing the framework for integrating FormsNet data with data from other sources.

4.2.3 Data Transformation Initiative

The vision of this initiative is to optimize the acquisition and utilization of entrusted data assets to accelerate breakthroughs that transform patient experiences. In 2020, the CIBMTR contracted with a global health technology company, IQVIA, for expertise and resources to accelerate progress. The CIBMTR designed, developed, and tested a prototype to collect and move data using automation from centers to the CIBMTR.

Prototype Design Guiding Principles

Meet the data where it resides

Be minimally disruptive to centers by limiting site-level activities to use the prototype

Enlist data and technical standards wherever possible to future proof the model

Five pilot partner sites tested the prototype. Each site developed a strategy with the CIBMTR based on its own technical environment. After evaluating the results, the CIBMTR found the prototype to be high performing. Pilot centers reported high levels of satisfaction through the stakeholder survey.

Next the CIBMTR will move from a prototype to a finished product and engage more network partners. To learn more about this initiative, please visit the *CIBMTR Data Transformation* webpage. This initiative is made possible with substantial funding support from NMDP/Be The Match and MCW.

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 G**).

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 CIBMTR launched a pilot CPI program to include centers from Australia, Brazil, and New Zealand. Due to reporting impacts related to the COVID-19 pandemic, the CIBMTR put the pilot CPI program on hold temporarily but reinstated it in November 2020. The remaining international centers will begin the CPI program in March 2021.

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 each 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 rate of form rejection due to inconsistently reported data was **<1%** throughout 2020, owing largely to enhancements in center education, direct center queries, and validations built into the FormsNet entry point.

4.3.3 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 center audits, spending 3-4 days reviewing original source documents. The overall audit process is explained in **Table 4.2**.

In 2020, **60** HCT centers were scheduled for audit (**50** domestic, **10** international). However, many audits were canceled between March and December 2020 due to the COVID-19 pandemic. As a result, the total number of audits scheduled in 2020 was reduced to **31** (**29** domestic, **2** international). As of December 1, 2020, **15** centers were notified of their final audit results, including requests of corrective action follow-up. Of the **15** centers sent reports, **67%** passed with **≤3%** critical field errors. Of the **5** centers that did not pass the audit, **4** received approval of their corrective action plans; the remaining center is in the process of completing requested corrective action plans.

The CIBMTR conducted a non-transplant cellular therapy data audit pilot in 2019 as a first step in expanding the standard HCT data audit program to target non-transplant cellular therapy-specific records. This year, the CIBMTR launched the non-transplant cellular therapy data audit program; **14** centers were eligible and audited. As of December 1, 2020, **6** centers were notified of their final audit results, including requests of corrective action follow-up. Of the **6** centers sent reports, **50%** passed within the target accuracy range.

Consolidated FACT-CIBMTR Audits

Since 2017, the CIBMTR has conducted data quality audits and evaluations on behalf of both organizations; the Foundation for the Accreditation of Cellular Therapy (FACT) determines whether the results of a data quality audit are satisfactory for accreditation. CIBMTR audit teams verify the accuracy of data against source data according to their current practices and schedules. At each annual report and application for accreditation renewal, FACT verifies 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.

4.3.4 Quality Assurance Program

The CIBMTR strives to ensure all staff have the education, training, tools, and experience to enable them to perform their assigned functions. To achieve this objective, the CIBMTR focuses on staff training, assessment of learning, and its documentation.

The CIBMTR Quality Assurance Program implemented a centralized document repository that automates the document control and management process through all stages of a document's life. This system houses approximately **300** documents. This year, the Quality Assurance Program reviewed **>100** policies, standard operating procedures, forms, job aides, and plans.

Next the Quality Assurance Program will connect the document control system to an automated training system for seamless quality management. This new system will automatically launch training tasks, ensuring all CIBMTR staff members remain up-to-date and knowledgeable regarding the latest improvements.

Table 4.2 Audit Process

	HCT	Other Cellular Therapies
Audit Cycle	Each domestic and international center that has submitted data for ≥20 HCTs is audited once within the 4-year audit cycle.	Currently, cellular therapy audits are performed on the HCT audit cycle. If centers that are scheduled for an HCT audit have submitted data on CAR T infusions, they are eligible for a cellular therapy audit during the same year as their HCT audit.
Recipient Selection	**16** recipient records are randomly selected for audit.	**8** CAR T infusions are randomly selected for audit.
Forms	All TED and CRF data are subject to audit.	All CRF data are subject to audit.
Data Fields	A core set of critical data fields and a random sample of other data fields are audited for each recipient.	
Methodology	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.	
Audit Reports and Corrective Action	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.	If any consent issues requiring correction or outstanding missing documentation issues are identified.

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 2020. 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 2020. 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 **>1,500** 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

Address patient symptoms and function

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

Address Patient Symptoms and Function

The CIBMTR collects patient-reported outcomes directly from patients to:

- Identify how cellular therapy impacts their symptoms, daily activities, functional status, and mental health
- Understand financial and employment challenges faced by long-term survivors

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.

APPENDIX A: COORDINATING CENTER LEADERSHIP

SENIOR LEADERSHIP

Mary M. Horowitz, MD, MS
Chief Scientific Director

Steven Devine, MD

Associate Scientific Director and Chief Medical Officer for NMDP/Be The Match

Bronwen Shaw MD, PhD
Associate Chief Scientific Director and Associate Center Director

Mei-Jie Zhang, PhD

Chief Statistical Director

Patricia Steinert, PhD, MBA
Executive Director for CIBMTR Milwaukee

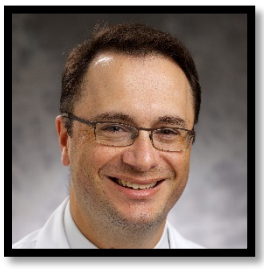
Mary Hengen, MBA

Interim Senior Vice President of Patient Outcomes and Experience for NMDP/Be The Match

Mary Eapen, MBBS, MS
Senior Scientific Director of Research Operations

Kathryn E. Flynn, PhD

Senior Scientific Director of Patient-Reported Outcomes



Marcelo Pasquini, MD, MS
Cellular Therapy Registry Director and Principal Investigator of the CIDR

J. Douglas Rizzo, MD, MS
Senior Scientific Director and Principal Investigator of the SCTOD

Amy Ronneberg, MBA

Chief Executive Officer for NMDP/Be The Match

Stephen Spellman, MBS
Vice President for NMDP/Be The Match Research

SCIENTIFIC DIRECTORS

Mukta Arora, MD, MBBS, MS
Co-Scientific Director of the Graft-versus-Host Disease Working Committee

Yung-Tsi Bolon, PhD

Assistant Scientific Director for the Immunobiology Working Committee

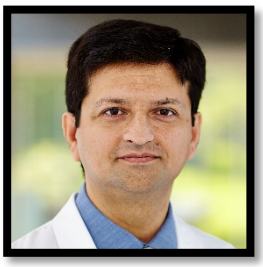
Larisa Broglie, MD, MS

Assistant Scientific Director for the Pediatric Cancer Working Committee

Saurabh Chhabra, MD, MS
Scientific Director for the Regimen-Related Toxicity and Supportive Care Working Committee

Anita D'Souza, MD

Scientific Director for the Plasma Cell Disorders and Adult Solid Tumors Working Committee

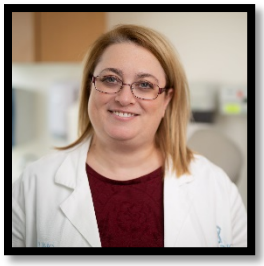


Mehdi Hamadani, MD

Scientific Director for the Lymphoma Working Committee

Stephanie J. Lee, MD, MPH
Senior Scientific Director of Immunobiology Research

Rachel Phelan, MD, MPH
Scientific Director for the Late Effects and Quality of Life Working Committee



Marcie Riches, MD, MS
Scientific Director for the Infection and Immune Reconstitution Working Committee

Wael Saber, MD, MS

Scientific Director for the Chronic Leukemia and Health Services and International Studies Working Committees

Daniel Weisdorf, MD

Senior Research Advisor

STATISTICAL DIRECTORS

Kwang Woo Ahn, PhD
Statistical Director for the Lymphoma, Pediatric Cancer, and Regimen-Related Toxicity and Supportive Care Working Committees

Ruta Brazauskas, PhD
Statistical Director for the Health Services and International Studies, and Late Effects and Quality of Life Working Committees

Raphael Fraser, PhD
Statistical Director for the Plasma Cell Disorders and Adult Solid Tumors Working Committee

Soyoung Kim, PhD
Statistical Director for the Cellular Immunotherapy for Cancer, Chronic Leukemia, and Non-Malignant Diseases Working Committees

Brent Logan, PhD
Statistical Director for the Donor Health and Safety Working Committee

Michael Martens, PhD
Statistical Director for the Infection and Immune Reconstitution Working Committee

Tao Wang, PhD
Statistical Director for the Graft-versus-Host Disease and Immunobiology Working Committees

OTHER LEADERSHIP STAFF

Erik Bergman, MBA, MS
Director of IT for CIBMTR Milwaukee

Janet Brunner-Grady, PA-C
Program Director of Data Operations for CIBMTR Milwaukee

Deb Christianson

Director of Data Operations for CIBMTR Minneapolis

Sharniece Covill

Director of Business Operations for CIBMTR Milwaukee

Sherry Fisher

Director of Advancement for CIBMTR Milwaukee

Erin Leckrone, MBA

Senior Director for NMDP/Be The Match Clinical Trials

Meggan McCann, MPH
Director of Strategic Programs for NMDP/Be The Match

Waleska S. Pérez, MPH
Program Director of Statistical Operations and Clinical Outcomes Research

Matt Prestegaard

Senior Director of Enterprise Architecture and Research Technology for NMDP/Be The Match

Guillermo Vazquez-Toro, PhD
Director of the Corporate Office for CIBMTR Milwaukee

APPENDIX B: 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 B1: 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	John Wingard, MD, Shands HealthCare & University of Florida, Gainesville, FL
Immediate Past Chair	Rob Soiffer, MD, Dana Farber Cancer Institute, Boston, MA

VICE CHAIRS

North America	Marcos de Lima, MD, Seidman 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

Bioethicist	Gregory Abel, MD, MPH, Dana Farber Cancer Institute, Boston, MA
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 Bryce Waldman, Chicago, IL

EX OFFICIO MEMBERS

Chief Executive Officer CIBMTR Minneapolis	Amy Ronneberg
Chief Scientific Director	Mary M. Horowitz, MD, MS, CIBMTR, Milwaukee, WI
Associate Chief Scientific Director CIBMTR Milwaukee	Bronwen Shaw, MD, PhD
Chief Statistical Director	Mei-Jie Zhang, PhD, CIBMTR, Milwaukee, WI
Chief Medical Officer CIBMTR Minneapolis	Steven Devine, MD, CIBMTR, Minneapolis, MN
Interim Senior Vice President, Patient Outcomes and Experience CIBMTR Minneapolis	Mary Hengen, MBA
Executive Director CIBMTR Milwaukee	Patricia Steinert, PhD, MBA, CIBMTR< Milwaukee, WI
Vice President CIBMTR Minneapolis	TBD
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	TBD
MCW / HRSA Contracting Officer Representatives	Marilyn Levi, MD TBD
MCW / NCI Project Officer	Lori Henderson, 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
ASTCT Representative	Pavan Reddy, MD, Ann Arbor, MI
Nominating Committee Chair	Michael Verneris, MD, Aurora, CO

APPENDIX B2: 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 of ASTCT and CIBMTR. All Executive Committee terms begin on March 1.

ELECTED MEMBERS

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

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

Bioethicist	Gregory Abel, MD, MPH, Dana Farber Cancer Institute, Boston, MA
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
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Chief Medical Officer CIBMTR Minneapolis	Steven Devine, MD, CIBMTR, Minneapolis, MN
Interim Senior Vice President, Patient Outcomes and Experience CIBMTR Minneapolis	Mary Hengen, MBA
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	TBD
MCW / HRSA Contracting Officer Representatives	Marilyn Levi, MD TBD
MCW / NCI Project Officer	Lori Henderson, 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
ASTCT Representative	Pavan Reddy, MD, Ann Arbor, MI
Nominating Committee Chair	Michael Verneris, MD, Aurora, CO

APPENDIX B3: 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

Bryce Waldman, Chicago, IL

MEMBERS

Nicole Adams, San Angelo, TX

Johnathan (Reid) Besch, Franklin, TN

Jeffery Haertling, Tierra Verde, FL

Hilary Hall, Sanofi Genzyme, Cambridge, MA

Brandon Nuechterlein, University of Colorado, Aurora, CO

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

Jennifer Wilder, National Institutes of Health, Bethesda, MD

SCIENTIFIC DIRECTOR

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

EX OFFICIO MEMBERS

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

Jackie Foster, MPH, RN, OCN, (NMDP liaison) NMDP/Be The Match, Minneapolis, MN

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

Marilyn Levi, MD, HRSA, Rockville, MD

Jennifer Motl, CIBMTR, Milwaukee, WI

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

Nawraz Shawir, MBBS, HRSA, Rockville, MD

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

APPENDIX B4: 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

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

MEMBERS

Edward Copelan, MD, Levine Cancer Institute, NC

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

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

Kirk Schultz, MD, British Columbia's Children's Hospital, UBC, Vancouver, BC

APPOINTED MEMBER

CIDR Executive Committee Representative

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

APPENDIX B5: 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	Mark Litzow, MD, Mayo Clinic Rochester
	Partow Kebriaei, MD, MD Anderson Cancer Center
	Christopher Hourigan, MD, D Phil, National Heart, Lung, and Blood Institute – NIH, Bethesda, MD
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
	Cameron Turtle, MBBS, PhD, Fred Hutchinson Cancer Research Center
Scientific Director	Marcelo Pasquini, MD, MS, CIBMTR
Statistical Director	Soyoung Kim, PhD, CIBMTR
Statistician	Kelley Qiu, MPH, CIBMTR

CHRONIC LEUKEMIA WORKING COMMITTEE

Chairs	Bart Scott, MD, Fred Hutchinson Cancer Research Center
	Ryotaro Nakamura, MD, City of Hope
	Betul Oran, MD, MS, MD Anderson Cancer Center
Scientific Director	Wael Saber, MD, MS, CIBMTR
Statistical Director	Soyoung Kim, 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
Statistical Director	Brent Logan, PhD, CIBMTR
Statistician	Stephanie Bo-Subait, MPH, CIBMTR

GRAFT SOURCES AND MANIPULATION WORKING COMMITTEE

Chairs	Ian McNiece, PhD, MD Anderson Cancer Center
	Claudio Brunstein, MD, PhD, University of Minnesota
	Filippo Milano, MD, PhD, Fred Hutchinson Cancer Research Center
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
	Margaret MacMillan, MD, MSc, University of Minnesota
	Carrie Kitko, MD, Vanderbilt University Medical Center
Scientific Directors	Mukta Arora, MD, MS, CIBMTR
	Stephen Spellman, MBS, CIBMTR
Statistical Director	Tao Wang, PhD, CIBMTR
Statistician	Karen Chen, MS, CIBMTR

HEALTH SERVICES AND INTERNATIONAL STUDIES WORKING COMMITTEE

Chairs	William Wood, MD, MPH, University of North Carolina Hospitals
	Shahrukh Hashmi, MD, MPH, King Faisal Specialist Hospital and Research Center
	Leslie Lehmann, MD, Dana Farber Cancer Institute
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	Sophie Paczesny, MD, PhD, Indiana University Hospital / Riley Hospital for Children
	Steven Marsh, BSc, PhD, ARCS, Anthony Nolan Research Institute
	Shahinaz Gadalla, MD, PhD, National Cancer Institute
Scientific Directors	Stephanie J. Lee, MD, MPH, CIBMTR, Fred Hutchinson Cancer Research Center
	Stephen Spellman, MBS, CIBMTR
Asst Scientific Dir	Yung-Tsi Bolon, PhD, CIBMTR
Statistical Director	Tao Wang, PhD, CIBMTR
Statistician	Meilun He, MPH, CIBMTR

INFECTION AND IMMUNE RECONSTITUTION WORKING COMMITTEE

Chairs	Miguel-Angel Perales, MD, Memorial Sloan Kettering Cancer Center
	Roy Chemaly, MD, MPH, MD Anderson Cancer Center
	Christopher Dandoy, MD, Cincinnati Children's Hospital Medical Center
Scientific Director	Marcie Riches, MD, MS, CIBMTR, University of North Carolina Hospitals
Statistical Director	Michael Martens, PhD, CIBMTR
Statistician	Naya He, MPH, CIBMTR

LATE EFFECTS AND QUALITY OF LIFE WORKING COMMITTEE

Chairs	David Buchbinder, MD, Children's Hospital of Orange County
	Betty Hamilton, MD, Cleveland Clinic Foundation
	Helene Schoemans, MD, University Hospitals Leuven and KU Leuven
Scientific Director	Rachel Phelan, MD, CIBMTR
Statistical Director	Ruta Brazauskas, PhD, CIBMTR
Statistician	Stephanie Bo-Subait, MPH, CIBMTR

LYMPHOMA WORKING COMMITTEE

Chairs	Mohamed Kharfan-Dabaja, MD, MBA, Mayo Clinic Florida
	Craig Sauter, MD, Memorial Sloan Kettering Cancer Center
	Alex Herrera, MD, City of Hope
Scientific Director	Mehdi Hamadani, MD, CIBMTR
Statistical Director	Kwang Woo Ahn, PhD, CIBMTR
Statistician	Stella Chen, MS, CIBMTR

NON-MALIGNANT DISEASES WORKING COMMITTEE

Chairs	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	Gregory Yanik, MD, The University of Michigan
	Muna Qayed, MD, MSc, Children's Healthcare of Atlanta at Egleston
	Kirk Schultz, MD, British Columbia's Children's Hospital, The University of British Columbia
Scientific Director	Mary Eapen, MD, MS, CIBMTR
asst Scientific Dir	Larisa Broglie, MD, MS, CIBMTR
Statistical Director	Kwang Woo Ahn, 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	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	Edward Stadtmauer, MD, Abramson Cancer Center University of Pennsylvania Medical Center
	Bipin Savani, MD, Vanderbilt University Medical Center
	Mohamed Sorror, MD, MSc, Fred Hutchinson Cancer Research Center
Scientific Director	Saurabh Chhabra, MD, CIBMTR
Statistical Director	Kwang Woo Ahn, PhD, CIBMTR
Statistician	Mariam Johnson, MPH, CIBMTR

APPENDIX B6: CIDR EXECUTIVE COMMITTEE MEMBERSHIP

The CIDR Executive Committee provides direction on scientific activities and policy decisions related to CIDR activities (**Section 2.1.3**) 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	John Wingard, MD
Associate Chief Scientific Director CIBMTR Milwaukee	Bronwen Shaw, MD, PhD
Chief Scientific Director	Mary M. Horowitz, MD, MS
Chief Statistical Director	Mei-Jie Zhang, PhD
Chief Medical Officer CIBMTR Minneapolis	Steven Devine, MD
Executive Director CIBMTR Milwaukee	Patricia Steinert, PhD, MBA
Principal Investigator	Marcelo Pasquini, MS, MS
Senior Scientific Director CIBMTR Milwaukee	Kathryn Flynn, PhD
IT Director CIBMTR Milwaukee	Erik Bergman, MBA, MS
IT Director CIBMTR Minneapolis	Matt Prestegaard, BA

APPENDIX B7: 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 (**Section 2.2**).

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 HCT 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

NMDP/BE THE MATCH AND CIBMTR STAFF

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 B8: 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

Shernan Holtan, MD, University of Minnesota BMT Program, Minneapolis, MN

Carrie Kitko, MD, Vanderbilt University Medical Center, Nashville, TN

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 BMT Program, Doernbecher Children's Hospital, Portland, OR

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

APPOINTED MEMBERS

Jack Aiello, MS, CIBMTR Consumer Advocacy Committee

Bryce Waldman, CIBMTR Consumer Advocacy Committee

EX OFFICIO MEMBERS

Mary Hengen, MBA, NMDP/Be The Match, Minneapolis MN

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

Steven Devine, MD, CIBMTR, Minneapolis, MN

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

Bronwen Shaw, MD, PhD, CIBMTR Milwaukee

Erin Leckrone, MBA, 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

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

APPENDIX C: PUBLICATIONS

In 2020, the CIBMTR published **89** 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 C.1 2020 CIBMTR Publications by Journal

Journal	Number of Publications
Biology of Blood and Marrow Transplantation	32
Blood Advances	11
Blood	8
Cancer	5
Bone Marrow Transplantation	3
British Journal of Haematology	3
Journal of Clinical Oncology	3
Leukemia & Lymphoma	3
Leukemia	3
Frontiers in Immunology	2
Other Journals*	16
TOTAL	89

*One publication each in American Journal of Human Genetics, Current Oncology, Genetic Epidemiology, Haematologica, Journal of Immunology, JAMA Network Open, JAMA Oncology, JCO Oncology Practice, The Lancet Haematology, Pediatric Blood & Cancer, PharmacoEconomics, PLoS One, Scandinavian Journal of Statistics Theory and Applications, Science Translational Medicine, Statistics in Medicine, and Statistical Methods in Medical Research.

APPENDIX C1: 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**.

- Dreger P, Fenske TS, Montoto S, et al. **Cellular immunotherapy for refractory DLBCL in the CART era: Still a role for allogeneic transplantation?** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.12.771. Epub 2020 Jan 6.
- Dandoy CE, Kim S, Chen M, et al. **Incidence, risk factors, and outcomes of patients who develop mucosal barrier injury-laboratory confirmed bloodstream infections in the first 100 days after allogeneic hematopoietic stem cell transplant.** JAMA Network Open. 2020 Jan 3; 3(1):e1918668. doi:10.1001/jamanetworkopen.2019.18668. Epub 2020 Jan 8. PMC6991246.
- Wong WH, Bhatt S, Trinkaus K, et al. **Engraftment of rare, pathogenic donor hematopoietic mutations in unrelated hematopoietic stem cell transplantation.** Science Translational Medicine. 2020 Jan 15; 12(526):1-9. doi:10.1126/scitranslmed.aax6249. Epub 2020 Jan 15.
- McReynolds LJ, Way Y, Thompson AS, et al. **Population frequency of Fanconi pathway gene variants and their association with survival after hematopoietic cell transplantation for severe aplastic anemia.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 May 1; 26(5):817-822. doi:10.1016/j.bbmt.2020.01.011. Epub 2020 Jan 23. PMC7243455.
- Pagel JM, Othus M, Garcia-Manero G, et al. **Rapid donor identification improves survival in high-risk first-remission patients with acute myeloid leukemia.** JCO Oncology Practice. 2020 Jun 1; 16(6):e464-e475. doi:10.1200/JOP.19.00133. Epub 2020 Jan 27. PMC7291544.
- Savage SA, Viard M, O'hUigin C, et al. **Genome-wide association study identifies HLA-DPB1 as a significant risk factor for severe aplastic anemia.** American Journal of Human Genetics. 2020 Feb 6; 106(2):264-271. doi:10.1016/j.ajhg.2020.01.004. Epub 2020 Jan 30. PMC7010969.
- Fatobene G, Rocha V, St Martin A, et al. **Nonmyeloablative alternative donor transplantation for Hodgkin and non-Hodgkin lymphoma: From the LWP-EBMT, Eurocord, and CIBMTR.** Journal of Clinical Oncology. 2020 May 10; 38(14):1518-1526. doi:10.1200/JCO.19.02408. Epub 2020 Feb 7. PMC7213591.
- Jagadeesh D, Majhail NS, He Y, et al. **Outcomes of rituximab-BEAM versus BEAM conditioning regimen in patients with diffuse large B cell lymphoma undergoing autologous transplantation.** Cancer. 2020 May 15; 126(10):2279-2287. doi:10.1002/cncr.32752. Epub 2020 Feb 12. PMC7190439.
- Schmidt S, Liu Y, Hu Z-H, et al. **The role of donor lymphocyte infusion (DLI) in post-hematopoietic cell transplant (HCT) relapse for chronic myeloid leukemia (CML) in the tyrosine kinase inhibitor (TKI) era.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jun 1; 26(6):1137-1143. doi:10.1016/j.bbmt.2020.02.006. Epub 2020 Feb 14. PMC7367282.
- Hsu JW, Shaw BE, Kim S, et al. **Collection of peripheral blood progenitor cells in 1 day is associated with decreased donor toxicity compared to 2 days in unrelated donors.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jun 1; 26(6):1210-1217. doi:10.1016/j.bbmt.2020.02.011. Epub 2020 Feb 20. PMC7347029.

- Farhadfar N, Hsu JW, Logan BR, et al. **Weighty choices: Selecting optimal G-CSF doses for stem cell mobilization to optimize yield.** Blood Advances. 2020 Feb 25; 4(4):706-716. doi:10.1182/bloodadvances.2019000923. Epub 2020 Feb 25. PMC7042992.
- Weisdorf D, Cooley S, Wang T, et al. **KIR B donors improve the outcome for AML patients given reduced intensity conditioning and unrelated donor transplantation.** Blood Advances. 2020 Feb 25; 4(4):750-754. doi:10.1182/bloodadvances.2019001053. Epub 2020 Feb 25. PMC7042994.
- Epperla N, Li A, Logan B, et al. **Incidence, risk factors for and outcomes of transplant-associated thrombotic microangiopathy.** British Journal of Haematology. 2020 Jun 1; 189(6):1171-1181. doi:10.1111/bjh.16457. Epub 2020 Mar 2.
- Seftel MD, Kuxhausen M, Burns L, et al. **Clonal hematopoiesis in related allogeneic transplant donors: Implications for screening and management.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jun 1; 26(6):e142-e144. doi:10.1016/j.bbmt.2020.02.022. Epub 2020 Mar 5. PMC7440392.
- Hamadani M, Khanal M, Ahn KW, et al. **Higher total body irradiation dose intensity in fludarabine/TBI-based reduced-intensity conditioning regimen is associated with inferior survival in non-Hodgkin lymphoma patients undergoing allogeneic transplantation.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jun 1; 26(6):1099-1105. doi:10.1016/j.bbmt.2020.02.025. Epub 2020 Mar 9. PMC7255948.
- Cusatis RN, Tecca HR, D'Souza A, et al. **Prevalence of decisional regret among patients who underwent allogeneic hematopoietic stem cell transplantation and associations with quality of life and clinical outcomes.** Cancer. 2020 Jun 1; 126(11):2679-2686. doi:10.1002/cncr.32808. Epub 2020 Mar 10. PMC7220834.
- Lee CJ, Kim S, Tecca HR, et al. **Late effects after ablative allogeneic stem cell transplantation for adolescent and young adult acute myeloid leukemia.** Blood Advances. 2020 Mar 24; 4(6):983-992. doi:10.1182/bloodadvances.2019001126. Epub 2020 Mar 13. PMC7094022.
- Whiteside S, Chin A, Tripathi G, et al. **Curability and transferability of atopy with allogeneic hematopoietic cell transplantation.** Bone Marrow Transplantation. 2020 Jul 1; 55(7):1282-1289. doi:10.1038/s41409-020-0876-7. Epub 2020 Mar 30.
- Dhakal B, Patel S, Girnius S, et al. **Hematopoietic cell transplantation utilization and outcomes for primary plasma cell leukemia in the current era.** Leukemia. doi:10.1038/s41375-020-0830-0. Epub 2020 Apr 2. PMC7572530.
- Hamadani M, Zhang M-J, Tang X-Y, et al. **Graft cryopreservation does not impact overall survival after allogeneic hematopoietic cell transplantation using post-transplantation cyclophosphamide for graft-versus-host disease prophylaxis**. Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jul 1; 26(7):1312-1317. doi:10.1016/j.bbmt.2020.04.001. Epub 2020 Apr 10. PMC7194895.
- Verneris MR, Miller JS, Hsu KC, et al. **Investigation of donor KIR content and matching in children undergoing hematopoietic cell transplantation for acute leukemia**. Blood Advances. 2020 Apr 14; 4(7):1350-1356. doi:10.1182/bloodadvances.2019001284. Epub 2020 Apr 14. PMC7160272.
- Ahmed S, Ghosh N, Ahn KW, et al. **Impact of type of reduced-intensity conditioning regimen on the outcomes of allogeneic haematopoietic cell transplantation in classical Hodgkin lymphoma.** British Journal of Haematology. doi:10.1111/bjh.16664. Epub 2020 Apr 21.

- Salit RB, Lee SJ, Burns LJ, et al. **Return-to-work guidelines and programs for post-hematopoietic cell transplantation survivors: An initial survey.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Aug 1; 26(8):1520-1526. doi:10.1016/j.bbmt.2020.04.022. Epub 2020 Apr 29. PMC7546432.
- Farhadfar N, Murthy HS, Logan BR, et al. **Impact of autologous blood transfusion after bone marrow harvest on unrelated donor's health and outcome: A CIBMTR analysis.** Bone Marrow Transplantation. 2020 Nov 1; 55(11):2121-2131. doi:10.1038/s41409-020-0911-8. Epub 2020 Apr 30. PMC7606543.
- Mehta RS, Holtan SG, Wang T, et al. **Composite GRFS and CRFS outcomes after adult alternative donor HCT.** Journal of Clinical Oncology. 2020 Jun 20; 38(18):2062-2076. doi:10.1200/JCO.19.00396. Epub 2020 May 4. PMC7302955.
- Eapen M, Zhang M-J, Tang X-Y, et al. **Hematopoietic cell transplantation with cryopreserved grafts for severe aplastic anemia.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jul 1; 26(7):e161-e166. doi:10.1016/j.bbmt.2020.04.027. Epub 2020 May 7. PMC7206419.
- Gowin K, Ballen K, Ahn KW, et al. **Survival following allogeneic transplant in patients with myelofibrosis.** Blood Advances. 2020 May 12; 4(9):1965-1973. doi:10.1182/bloodadvances.2019001084. Epub 2020 May 8. PMC7218417.
- D'Souza A, Fretham C, Lee SJ, et al. **Current use of and trends in hematopoietic cell transplantation in the United States.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Aug 1; 26(8):e177-182. doi:10.1016/j.bbmt.2020.04.013. Epub 2020 May 11. PMC7404814.
- Kahn JM, Brazauskas R, Tecca HR, et al. **Subsequent neoplasms and late mortality in children undergoing allogeneic transplantation for nonmalignant diseases.** Blood Advances. 2020 May 12; 4(9):2084-2094. doi:10.1182/bloodadvances.2019000839. Epub 2020 May 12. PMC7218429.
- Myers K, Hebert K, Antin J, et al. **Hematopoietic stem cell transplantation for Shwachman-Diamond syndrome.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Aug 1; 26(8):1446-1451. doi:10.1016/j.bbmt.2020.04.029. Epub 2020 May 16. PMC7371524.
- Im A, Rashidi A, Wang T, et al. **Risk factors for graft-versus-host disease in haploidentical hematopoietic cell transplantation using post-transplant cyclophosphamide.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Aug 1; 26(8):1459-1468. doi:10.1016/j.bbmt.2020.05.001. Epub 2020 May 17. PMC7391266.
- Nikiforow S, Mansour M, Hu Z-H, et al. **Tocilizumab not associated with increased infection risk after CAR T-cell therapy: Implications for COVID-19?** Blood. 2020 Jul 2; 136(1):137-139. doi:10.1182/blood.2020006216. Epub 2020 May 25. PMC7332891.
- Arnold SD, Brazauskas R, He N, et al. **The impact of donor type on outcomes and cost of allogeneic hematopoietic cell transplantation for pediatric leukemia: A merged Center for International Blood and Marrow Transplant Research and Pediatric Health Information System analysis.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Sep 1; 26(9):1747-1756. doi:10.1016/j.bbmt.2020.05.016. Epub 2020 May 25. PMC7518194.

- Petersdorf EW, Bengtsson M, De Santis D, et al. **Role of HLA-DP expression in graft-versus-host disease after unrelated donor transplantation.** Journal of Clinical Oncology. 2020 Aug 20; 38(24):2712-2718. doi:10.1200/JCO.20.00265. Epub 2020 Jun 1. PMC7430213.
- Petersdorf EW, Stevenson P, Bengtsson M, et al. **HLA-B leader and survivorship after HLA-mismatched unrelated donor transplantation.** Blood. 2020 Jul 16; 136(3):362-369. doi:10.1182/blood.2020005743. Epub 2020 Jun 1. PMC7365916.
- Ghosh N, Ahmed S, Ahn KW, et al. **Association of reduced-intensity conditioning regimens with overall survival among patients with non-Hodgkin lymphoma undergoing allogeneic transplant.** JAMA Oncology. 2020 Jul 1; 6(7):1011-1018. doi:10.1001/jamaoncol.2020.1278. Epub 2020 Jun 4. PMC7273311.
- Brazauskas R, Scigliuolo GM, Wang HL, et al. **Risk score to predict event-free survival after hematopoietic cell transplant for sickle cell disease.** Blood. 2020 Jul 30; 136(5):623-626. doi:10.1182/blood.2020005687. Epub 2020 Jun 9. PMC7393258.
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- Tay J, Beattie S, Bredeson C, et al. **Pre-transplant marital status and hematopoietic cell transplantation outcome.** Current Oncology. 2020 Dec 1; 27(6):e596-e606. doi:10.3747/co.27.6327. Epub 2020 Dec 1. PMC7755447.

APPENDIX C2: 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**.

- Levine JE, Antin JH, Allen CE, et al. **Priorities for improving outcomes for non-malignant blood diseases: A report from the Blood and Marrow Transplant Clinical Trials Network.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2020.01.024. Epub 2020 Jan 24.
- Shah O, Tamaresis JS, Kenyon LJ, et al. **Analysis of the whole CDR3 T cell receptor repertoire after hematopoietic stem cell transplantation in 2 clinical cohorts.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jun 1; 26(6):1050-1070. doi:10.1016/j.bbmt.2020.01.020. Epub 2020 Feb 18. PMC7503214.
- Costa LJ, Iacobelli S, Pasquini MC, et al. **Long-term survival of 1338 MM patients treated with tandem autologous vs. autologous-allogeneic transplantation.** Bone Marrow Transplantation. 2020 Sep 1; 55(9):1810-1816. doi:10.1038/s41409-020-0887-4. Epub 2020 Apr 14. PMC7483973.
- Holstein SA, Howard A, Avigan D, et al. **Summary of the 2019 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. 2020 Oct 1; 26(10):e247-e255. doi:10.1016/j.bbmt.2020.06.011. Epub 2020 Jun 24. PMC7529908.
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- Fuchs E, O'Donnell P, Eapen M, et al. **Double unrelated umbilical cord blood versus HLA-haploidentical bone marrow transplantation (BMT CTN 1101).** Blood. doi:10.1182/blood.2020007535. Epub 2020 Aug 31.
- DeFilipp Z, Burns LJ, Jaglowski SM, et al. **A new standard in graft-versus-host disease prophylaxis? An introduction to Blood and Marrow Transplant Clinical Trials Network 1703.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Dec 1; 26(12):e305-e308. doi:10.1016/j.bbmt.2020.08.029. Epub 2020 Sep 10.
- Farhadfar N, Burns LJ, Mupfudze T, et al. **Hematopoietic cell transplantation: Practice predictions for the year 2023.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2020.10.006. Epub 2020 Oct 9.
- Martens MJ, Logan B. **A unified approach to sample size and power determination for testing parameters in generalized linear and time-to-event regression models.** Statistics in Medicine. doi:10.1002/sim.8823. Epub 2020 Nov 18.

APPENDIX C3: RCI BMT PUBLICATION

The following publication was 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**.

- Shah NN, Schneiderman J, Kuruvilla D, et al. **Fatal capillary leak syndrome in a child with acute lymphoblastic leukemia treated with moxetumomab pasudotox for pre-transplant minimal residual disease reduction.** Pediatric Blood & Cancer. doi:10.1002/pbc.28574. Epub 2020 May 5.

APPENDIX C4: 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**.

- Dunavin N, Mau L-W, Meyer CL, et al. **Health care reimbursement, service utilization, and financial responsibility among Medicare beneficiaries with multiple myeloma receiving autologous hematopoietic cell transplantation in inpatient and outpatient settings.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2019.12.772. Epub 2020 Jan 6.
- Mau L-W, Preussler JM, Burns LJ, et al. **Healthcare costs of treating privately insured patients with acute myeloid leukemia in the United States from 2004 to 2014: A generalized additive modeling approach.** PharmacoEconomics. 2020 May 1; 38(5):515-526. doi:10.1007/s40273-020-00891-w. Epub 2020 Mar 4. PMC7194165.

APPENDIX C5: BIOINFORMATICS RESEARCH PROGRAM PUBLICATIONS

The following publications were generated by the Bioinformatics Research Program, which specializes in matching patients and cellular therapies. For more information, see **Section 2.5**.

- Roe D, Williams J, Ivery K, et al. **Efficient sequencing, assembly, and annotation of human KIR haplotypes.** Frontiers in Immunology. 11:582927. doi:10.3389/fimmu.2020.582927. Epub 2020 Oct 9. PMC7581912.
- Single RM, Meyer D, Nunes K, et al. **Demographic history and selection at HLA loci in Native Americans.** PLoS One. 15(11):e0241282. doi:10.1371/journal.pone.0241282. Epub 2020 Nov 4. PMC7641399.
- Roe D, Vierra-Green C, Pyo C-W, et al. **A detailed view of KIR haplotype structures and gene families as provided by a new motif-based multiple sequence alignment.** Frontiers in Immunology. 11:585731. doi:10.3389/fimmu.2020.585731. Epub 2020 Nov 18. PMC7708349.

APPENDIX C6: 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**.

- Kim S, Xu Y, Zhang MJ, et al. **Stratified proportional subdistribution hazards model with covariate-adjusted censoring weight for case- cohort studies.** Scandinavian Journal of Statistics. doi:10.1111/sjos.12461. Epub 2020 May 7.
- Kim S, Logan B, Riches M, et al. **Statistical methods for time-dependent variables in hematopoietic cell transplantation studies.** Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. doi:10.1016/j.bbmt.2020.09.034. Epub 2020 Oct 2.
- DeVogel N, Auer PL, Manansala R, et al. **A unified linear mixed model for familial relatedness and population structure in genetic association studies.** Genetic Epidemiology. doi:10.1002/gepi.22371. Epub 2020 Nov 11.

APPENDIX D: PRESENTATIONS

In 2020, CIBMTR study investigators presented **63** abstracts (**35** oral and **28** poster) at the national and international conferences listed in **Table E.1**. A complete list of presentations is provided by meeting.

Table E.1 2020 CIBMTR Presentations by Meeting

Conference	Oral	Poster	Total
American Society of Hematology (ASH) Annual Meeting	9	15	**24**
TCT Meetings of ASTCT and CIBMTR	15	8	**23**
American Society of Clinical Oncology Annual Meeting	3	1	**4**
European Society for Blood and Marrow Transplantation Annual Meeting	2	1	**3**
European Hematology Association Annual Meeting	1	1	**2**
The ONE Forum	2	0	**2**
Other Meetings and Conferences*	3	2	**5**
TOTAL	**35**	**28**	**63**

* One oral presentation each at ASH Workshop on Developing Biomarkers for CAR T Cells and BiTES® Toxicity and Efficacy, E0542: New Advances in Biomedical Data Analysis, and the HRSA Advisory Council on Blood Stem Cell Transplantation. One poster presentation each at the American Society of Histocompatibility and Immunogenetics and the European Congress of Mathematics.

2020 AMERICAN SOCIETY OF HEMATOLOGY ANNUAL MEETING

Study	Title	Type	PI
15-MMUD	Bridging the gap in access to transplant for underserved minority patients using mismatched unrelated donors and post-transplant cyclophosphamide: A National Marrow Donor Program/Be The Match (NMDP/BTM) initiative	Oral	B Shaw
BMT CTN 1102	A multi-center biologic assignment trial comparing reduced intensity allogeneic hematopoietic cell transplantation to hypomethylating therapy or best supportive care in patients aged 50-75 with advanced myelodysplastic syndrome: Blood and Marrow Transplant Clinical Trials Network study 1102	Oral	C Cutler
BMT CTN 1803	BMT CTN 1803: Haploidentical natural killer cells (K-NK002) to prevent post-transplant relapse in AML and MDS (NK-REALM)	Poster	S Vasu
CK18-03	Younger HLA-matched unrelated donor allogeneic hematopoietic cell transplantation (allo-HCT) for myelodysplastic syndromes (MDS) is associated with superior disease-free survival compared to older HLA-identical sibling donors: CIBMTR analysis	Poster	G Murthy W Saber
CK19-01a	Allogeneic hematopoietic cell transplantation (allo-HCT) in T-cell prolymphocytic leukemia (T-PLL): An analysis from the CIBMTR	Poster	H Murthy B Dholaria M Kharfan *et al.*
DS19-01	Impact of cryopreservation of donor grafts on outcomes of allogeneic hematopoietic cell transplant (HCT)	Oral	J Hsu N Farhadfar H Murthy J Wingard
GS18-01	Comparison of outcomes after haploidentical relative and HLA matched unrelated donor transplantation with post-transplant cyclophosphamide containing GVHD prophylaxis regimens	Oral	M Gooptu R Romee
HSR18-05	Geographic and socioeconomic disparities in hematopoietic cell transplantation among acute myeloid leukemia patients in Virginia	Poster	K Ballen
IB09-06a	Population distribution of GvL and GvH minor histocompatibility antigens	Oral	K Olsen

2020 AMERICAN SOCIETY OF HEMATOLOGY ANNUAL MEETING

Study	Title	Type	PI
IB09-06b	Associations of clinical outcomes after allogeneic hematopoietic cell transplantation with number of predicted class II restricted mHA	Poster	O Jadi
IB09-06c	Meta-analysis of genome-wide association studies of acute myeloid leukemia (AML) patients identifies variants associated with risk of 11q23/KMT2A-translocated and core-binding factor (CBF) AML and suggests a role for transcription elongation in leukemogenesis	Poster	L Sucheston-Campbell
IB09-06d	Pre-transplant clonal mosaicism is associated with increased relapse and lower survival in acute lymphoblastic leukemia patients undergoing allogeneic hematopoietic cell transplant	Poster	Y Wang
IB17-02	Outcomes of pediatric patients with JMML following unrelated donor transplant: The impact of donor KIR gene content and KIR ligand matching	Poster	D Lee H Rangarahan
IB17-03	Chromosomal aberrations in pre-HCT blood samples and outcomes after transplantation in patients with myelofibrosis	Oral	Y Wang
IB19-02 combined abstract	Improving donor selection for haploidentical stem cell transplantation with post-transplant cyclophosphamide through selective HLA-mis/matching	Poster	S McCurdy S Solomon Y Kasamon *et al.*
LK18-01	Prognostic impact of a modified European LeukemiaNet (ELN) genetic risk stratification in predicting outcomes for adults with acute myeloid leukemia (AML) undergoing allogeneic hematopoietic stem cell transplantation (HCT). A Center for International Blood and Marrow Transplant Research (CIBMTR) analysis	Poster	A Jimenez T Wang M de Lima K Komanduri
LK18-02	Comparison of haploidentical donor hematopoietic cell transplantation using post-transplant cyclophosphamide to matched-sibling, matched-unrelated, mismatched-unrelated, and umbilical cord blood donor transplantation in adults with acute lymphoblastic leukemia: A CIBMTR study	Oral	M Wieduwilt L Metheny M de Lima
LK20-04	Impact of age on the outcomes of HCT for AML in CR1: Promising therapy for older adults	Poster	J Maakaron

2020 AMERICAN SOCIETY OF HEMATOLOGY ANNUAL MEETING

Study	Title	Type	PI
LY19-02	Superiority of thiotepa-containing conditioning regimens in patients with primary diffuse large b-cell lymphoma (DLBCL) of the central nervous system (CNS) undergoing autologous hematopoietic cell transplantation (autoHCT)	Oral	T Peng Wang M Scordo C Sauter
MM19-01	Maintenance use is more important than the choice of bortezomib-based triplet induction in newly diagnosed multiple myeloma patients undergoing upfront autologous stem cell transplantation	Poster	S Sidana M Norkin S K Kumar S Giralt
NM19-02	Conditioning regimens and outcomes after allogeneic hematopoietic cell transplant for hyperinflammatory inborn errors of immunity	Poster	R Marsh
RT18-01a	Expanded comorbidity definitions improve applicability of the hematopoietic stem cell transplantation-comorbidity index for children, adolescents, and young adults with hematologic malignancies undergoing allogeneic stem cell transplantation	Poster	L Broglie B Friend G Schiller M Thakar M Sorror
RT18-01b	Expanded comorbidity definitions improve application of the hematopoietic cell transplantation comorbidity index (HCT-CI) for children and young adults with non-malignant diseases receiving allogeneic hematopoietic cell transplantation	Oral	B Friend G Schiller L Broglie M Thakar M Sorror
RT18-03	Non-infectious pulmonary toxicity after allogeneic hematopoietic cell transplantation (HCT): A Center for International Blood and Marrow Transplant Research (CIBMTR) study	Poster	S Patel B Hamilton N Majhail C Ustun

2020 TCT MEETINGS OF ASTCT AND CIBMTR

Study	Title	Type	PI
15-MMUD	Transplantation using bone marrow from a (very) HLA mismatched unrelated donor in the setting of post-transplant cyclophosphamide is feasible and expands access to underserved minorities	Poster	B Shaw
17ePRO	Feasibility of centralized electronic patient-reported outcome (ePRO) collection by an outcome registry, a CIBMTR study of patients on the centers for Medicaid & Medicare coverage with evidence development (CMS CED) myelodysplasia protocol	Oral	B Shaw
BMT CTN	Hematopoietic cell transplantation (HCT) predictions for the year 2023	Poster	N Farhadfar
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 trials	Poster	P Shindiapina
BMT CTN 0901	Long-term follow up of BMT CTN 0901, a randomized Phase III trial comparing myeloablative (MAC) to reduced intensity conditioning (RIC) prior to hematopoietic cell transplantation (HCT) for acute myeloid leukemia (AML) or myelodysplasia (MDS) (MAvRIC Trial)	Oral	B Scott
BMT CTN 1101	Results of Blood and Marrow Transplant Clinical Trials Network protocol 1101 a multicenter Phase III randomized trial of transplantation of double umbilical cord blood vs. HLA-haploidentical -related bone marrow for hematologic malignancy	Oral	C Brunstein
BMT CTN 1401	Evaluation of tumor vaccine generation in a Phase II multicenter trial of single autologous hematopoietic cell transplant (autoHCT) followed by lenalidomide maintenance for multiple myeloma (MM) with or without vaccination with dendritic cell/ myeloma fusions (DC/MM fusion vaccine): Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 1401	Oral	D Avigan
BMT CTN 1803	BMT CTN 1803: Trial to investigate if haploidentical natural killer cells (CSTD002) prevent post-transplant relapse in AML and MDS (NK-REALM)	Poster	S Vasu

2020 TCT MEETINGS OF ASTCT AND CIBMTR

Study	Title	Type	PI
CK15-03	Impact of genetic mutations on the outcomes of allogeneic hematopoietic cell transplantation in patients with acute myeloid leukemia with antecedent myeloproliferative neoplasm	Oral	S Vasu
GS19-01	Comparison of haploidentical related donor with post-transplant cyclophosphamide (PTCy) and umbilical cord blood (UCB) transplantation after myeloablative conditioning for hematological malignancy	Poster	J Wagner K Ballen
GV17-03	Acute graft-versus-host disease is less severe and associated with lower non-relapse mortality after haploidentical transplantation with post-cyclophosphamide prophylaxis	Oral	R Saliba S Ciurea J Schriber
HSR18-10	A qualitative study of state Medicaid coverage benefits for allogeneic hematopoietic cell transplantation (alloHCT) for patients with sickle cell disease (SCD)	Oral	T Mupfudze
HSR19-02	Transplant physicians' attitudes on candidacy for allogeneic hematopoietic cell transplantation (HCT) in older patients: The need for a standardized geriatric assessment (GA) tool	Oral	A Mishra
IB18-01	HLA genotyping does not predict outcomes in hematopoietic cell transplantation (alloHCT)	Oral	C Story M Riches P Armistead
IB18-06	Prognostic impact of pre-transplant chromosomal aberrations detected by SNP-array in patients undergoing unrelated donor hematopoietic cell transplant for acute myeloid leukemia	Oral	S Gadalla T Hah L Sucheston-Campbell
Immunobiology	Evaluating the role for mismatched unrelated donor transplantation	Oral	S Spellman
IN17-01a	Incidence and impact of cytomegalovirus infection in haploidentical and matched-related donors receiving post-transplant cyclophosphamide (PTCy): A CIBMTR analysis	Oral	R Romee A Singh R Allsion Taplitz
IN17-01b	Incidence and impact of non-CMV herpes viral infection in haploidentical and matched sibling donors receiving post-transplant cyclophosphamide (PTCy): A CIBMTR analysis	Poster	R Romee A Singh R Allsion Taplitz

2020 TCT MEETINGS OF ASTCT AND CIBMTR

Study	Title	Type	PI
IN17-01c	Incidence and impact of community respiratory viral infection (CRV) in haploidentical and matched sibling donors receiving post-transplant cyclophosphamide (PTCy): A CIBMTR analysis	Oral	R Romee A Singh R Allsion Taplitz
LE12-03	Hematopoietic cell transplantation (HCT) followed by solid organ transplantation (SOT) and SOT followed by HCT: A descriptive analysis of patients undergoing sequential transplantation in the United States	Poster	M Gupta P L Abt M Levine
LK17-02	MLL-rearranged AML is associated with poor outcomes as compared to patients with intermediate- and adverse-risk disease: A CIBMTR study of 3779 adult patients	Oral	K Menghrajani M Tallman
LY17-02c	Higher total body irradiation (TBI) dose-intensity in fludarabine (Flu)/TBI-based reduced-intensity conditioning (RIC) regimen is associated with inferior survival in non-Hodgkin lymphoma (NHL) patients undergoing allogeneic hematopoietic cell transplantation (alloHCT)	Oral	M Hamadani
RT17-01	Development of the renal adjusted hematopoietic cell transplant comorbidity index (RA-HCT-CI) using different levels of renal dysfunction according to estimated glomerular filtration rate (eGFR)	Poster	N Farhadfar A Dias J R Wingard H Murthy S Ganguly

2020 AMERICAN SOCIETY OF CLINICAL ONCOLOGY ANNUAL MEETING

Program	Title	Type	PI
BMT CTN 07LT (0702)	Long-term follow-up of BMT CTN 0702 (STaMINA) of post autologous hematopoietic cell transplantation (autoHCT) strategies in the upfront treatment of multiple myeloma (MM)	Oral	P Hari
LY18-01b	Is autologous transplantation (autoHCT) in relapsed diffuse large B-cell lymphoma (DLBCL) patients achieving only a PET/CT positive partial remission (PR) appropriate in the CAR-T cell era?	Oral	N Shah K Woo Ahn C Litovich T Fenske M Hamadani

2020 AMERICAN SOCIETY OF CLINICAL ONCOLOGY ANNUAL MEETING

MM17-02	Bortezomib induction prior to autologous hematopoietic cell transplantation (AHCT) for newly diagnosed light chain amyloidosis (AL): A study of 426 patients	Poster	R F Cornell P Hari L Costa *et al.*
PC19-01	Development and validation of a pediatric disease risk index for allogeneic hematopoietic cell transplantation	Oral	M Qayed CL Kitko KW Ahn *et al.*

2020 EUROPEAN SOCIETY FOR BLOOD AND MARROW TRANSPLANTATION ANNUAL MEETING

Program	Title	Type	PI
BMT CTN 1101	Results of Blood and Marrow Transplant Clinical Trials Network protocol 1101 a multicenter Phase III randomized trial of transplantation of double umbilical cord blood vs. HLA-haploidentical -related bone marrow for hematologic malignancy	Oral	C Brunstein
LY17-02d	Comparison of reduced-intensity conditioning (RIC) regimes for allogeneic hematopoietic cell transplantation (allo-HCT) for diffuse large B-cell lymphoma (DLBCL): A CIBMTR analysis	Oral	M Hamadani
SC06-02b	A prospective cohort study comparing long-term outcomes with and without palifermin in patients receiving hematopoietic cell transplantation for hematologic malignancies	Poster	M Horowitz W Saber

2020 EUROPEAN HEMATOLOGY ASSOCIATION ANNUAL MEETING

Program	Title	Type	PI
BMT CTN 0901	Impact of conditioning intensity and genomics on relapse after allogeneic transplantation for patients with myelodysplastic syndrome	Oral	L Dillon
BMT CTN 1803	Trial to investigate if haploidentical natural killer cells (CSTD002) prevent post-transplant relapse in AML and MDS (NK-REALM)	Poster	S Vasu

2020 NMDP/BE THE MATCH ONE FORUM

Program	Title	Type	PI
Bioinformatics	Donor optimization for transplant success (DOTS) breakout session	Oral	R Sajulga Y Bolon M Maiers
Immunobiology	Transplant across HLA barriers: Novel strategies for use of mismatched unrelated donors	Oral	S Spellman

2020 ASH WORKSHOP ON DEVELOPING BIOMARKERS FOR CAR T CELLS AND BiTES® TOXICITY AND EFFICACY

Program	Title	Type	PI
Immunobiology	Clinical data and sample collection	Oral	M Perales S Spellman

2020 E0542: NEW ADVANCES IN BIOMEDICAL DATA ANALYSIS

Program	Title	Type	PI
Statistical Methodology	Sensitivity analysis: E-value based on direct adjusted survival probabilities	Oral	M Zhang

2020 HRSA ADVISORY COUNCIL ON BLOOD STEM CELL TRANSPLANTATION

Program	Title	Type	PI
Immunobiology	Mismatched bone marrow transplantation	Oral	S Spellman

2020 AMERICAN SOCIETY OF HISTOCOMPATIBILITY AND IMMUNOGENETICS ANNUAL MEETING

Program	Title	Type	PI
Bioinformatics	How better collection of race and ethnicity information can improve matching for HSCT	Poster	A Madbouly E Williams M Maiers Y Bolon

2020 EUROPEAN CONGRESS OF MATHEMATICS ANNUAL MEETING

Program	Title	Type	PI
Bioinformatics	SU-QMI: A feature selection method based on graph theory for prediction of antimicrobial resistance in gram-negative bacteria	Poster	A Chowdhury S Broschat

APPENDIX E: 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 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 Leadership 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 F: 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 F1: 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 • 67 of 296 patients enrolled • Anticipated accrual completion in 2024
BMT CTN 1507	Reduced intensity conditioning for haploidentical bone marrow transplantation in patients with symptomatic sickle cell disease	• Opened to accrual Oct 2017 • 53 of 80 patients enrolled and transplanted • Adult cohort accrual completed October 2020; anticipated pediatric accrual completion in late 2022
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 • 308 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 • 691 of 1,732 patients enrolled • Anticipated accrual completion in early 2022

BMT CTN CLINICAL TRIALS OPEN FOR ENROLLMENT

Protocol Number	Title	Status to Date
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 • 269 of 428 patients enrolled (1703) • 172 of 300 patients enrolled (1801) • 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 • 628 of 1,100 patients enrolled • Anticipated accrual completion in early 2021
BMT CTN 1705	A randomized, double-blind, placebo-controlled multicenter Phase III trial of alpha 1 – antitrypsin combined with corticosteroids vs corticosteroids alone for the treatment of high risk acute graft-versus-host disease following allogeneic hematopoietic stem cell transplant	• Opened to accrual January 2020 • 21 of 122 patients enrolled • Anticipated accrual completion in late 2022
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 • 174 of 1,100 patients enrolled • Anticipated accrual completion mid 2025
BMT CTN 1803	Haplo-identical natural killer cells to prevent post-transplant relapse in AML and MDS (NK-REALM)	• Opened to accrual November 2020 • No patients enrolled yet • Anticipated accrual completion late 2022
BMT CTN 1905 (ITN077AI)	A multicenter randomized controlled trial of best available therapy versus autologous hematopoietic stem cell transplant for treatment-resistant relapsing multiple sclerosis	• Opened to accrual December 2019 • 1 of 156 patients enrolled • Anticipated completion late 2023

APPENDIX F2: 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 November 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 March 2018 • 340 recipients enrolled • Study follow-up complete March 2020 • Data analysis in progress
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 November 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 November 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 March 2019 • 80 subjects enrolled; 3 in 2019 • Study follow-up complete March 2020 • Manuscript in development

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 November 2019 • 92 subjects enrolled • Companion study to CIBMTR CMS-approved expanded access study • Pilot study for ePRO system • Manuscript in development
PBSC	Filgrastim-mobilized peripheral blood stem cells for allogeneic transplantation with unrelated donors	• Opened to accrual April 1996 • >37,000 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 October 2011 • 4,671 recipients enrolled • 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 • Manuscript in development
17-CSIDE	Busulfan exposure-finding for SCID	• Opened to accrual October 2018 • 13 of 64 subjects enrolled
17-SIBS	Identifying predictors of poor health-related quality-of-life among pediatric hematopoietic stem cell donors	• Opened to accrual June 2019 • 187 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 • 641 of 1,732 evaluable subjects enrolled
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 • 234 of 728 subjects enrolled • RCI BMT overseeing site activation and regulatory document management

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
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 July 2019 • 557 of 1,100 subjects enrolled
16-NTCD	Naïve T cell depletion for prevention of chronic GVHD in pediatric population	• Opened to accrual September 2019 • 3 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 November 2019 • 1 subject enrolled • 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	• Open to accrual December 2019 • First subject enrolled December 2020
Orca Treg Graft	Multicenter Phase Ib trial for patients with advanced hematologic malignancies undergoing allogeneic hematopoietic cell transplantation with TregGraft, a T-cell depleted graft with additional infusion of conventional T cells and regulatory T cells	• Opened to accrual December 2019 • RCI BMT overseeing site management and study monitoring
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 February 2020 • RCI BMT overseeing site management and study monitoring

RCI BMT CLINICAL STUDIES

Protocol Number	Title	Status to Date
INSPIRE-SCP	Integrating health informatics in a scalable stepped care self-management program for HCT survivors	• Collaboration with Fred Hutchinson Cancer Research Center • RCI BMT will prepare individualized care plans and contact patients for electronic surveys • Study pending first enrollments in 2020
PRO Data Collection	Protocol for collection of patient reported outcomes (PRO) data	• Broad data collection protocol • First subject enrolled August 2020
Palliative Care Survey	Patients / perspectives on palliative care	• NMDP IRB approval / exemption September 2020 • Patient contact initiated November 2020
145-ADS-202	Phase II study evaluating the safety and efficacy of MGTA-145 in combination with plerixafor for the mobilization and transplantation of HLA-matched hematopoietic stem cells in recipients with hematological malignancies	• Collaboration with Magenta Therapeutics • IRB approval August 2020 • FDA notification to proceed October 2020 • Pending first enrollment
Be The Match Survivorship Protocol	A randomized study of virtual survivorship support for allogeneic hematopoietic stem cell transplant survivors	• Initial protocol team meetings August 2020
Donor For All	A multi-center, Phase II trial of HLA-mismatched unrelated donor hematopoietic cell transplantation with post-transplantation cyclophosphamide for patients with hematologic malignancies	• Initial protocol team meetings August 2020

APPENDIX G: 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 H: 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	bloodstemcell.hrsa.gov

APPENDIX I: GLOSSARY

Abbreviation/ Acronym	Meaning
AGNIS	A Growable Network Information System
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
CAR	chimeric antigen receptor
CDE	common data elements
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
DLI	donor leukocyte infusion
DRI	Disease Risk Index
EBMT	European Society for Blood and Marrow Transplantation
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
HLA	human leukocyte antigen
HRSA	Health Resources and Services Administration
IND	investigational new drug
IOTN	Immuno-Oncology Translational Network
IRB	Institutional Review Board
IT	Information Technology
JAMA	Journal of the American Medical Association
KIR	killer-cell immunoglobulin-like receptors
MCW	Medical College of Wisconsin

Abbreviation/ Acronym	Meaning
MDS	myelodysplastic syndrome
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
PBSC	peripheral blood stem cell
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
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

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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.

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