CIBMTR®

CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

2021 ANNUAL REPORT

Sharing Knowledge.
Sharing Hope.

2021 AT-A-GLANCE

OUR NUMBERS

>350 participating centers

>600,000 patients

~26,000 new patients annually

>225 ongoing studies and clinical trials

>195,000 biorepository samples

99 peer-reviewed publications

72 presentations at conferences

LETTER FROM OUR CHIEF SCIENTIFIC DIRECTORS

Dear CIBMTR Community:

We are pleased to share the 2021 Annual Report, a reflection of the combined efforts of the CIBMTR and our community partners. Together, we have accomplished much this year despite our personal and professional challenges inherent to the COVID-19 pandemic.

During the pandemic, the CIBMTR continued our work. We adapted data collection in order to better collect information about patients with COVID-19 and to define the effect that COVID-19 has during transplant, sharing COVID-19 data submitted by participating centers on the CIBMTR COVID-19 webpage. In the spring, the CIBMTR rapidly published several analyses relevant to the pandemic, and the Working Committees continued to complete, present, and publish multiple studies. We truly appreciate the extra efforts all data professionals at the centers made to keep up with reporting to the CIBMTR.

We applaud and recognize your clinical care for cellular therapy patients in the midst of the pandemic, including the sacrifices that you made in so doing. We appreciate these extraordinary efforts while also contributing to the mission of the CIBMTR in improving patient outcomes through impactful research.

We are working hard on a refresh of our strategic priorities – more to come at the 2022 Tandem Meetings. Our cellular therapy community is truly strong! And we are honored to be your CIBMTR leadership.

Bronwen E. Shaw, MD, PhD
Chief Scientific Director, CIBMTR MCW

Jeffery J. Auletta, MD
Chief Scientific Director, CIBMTR NMDP

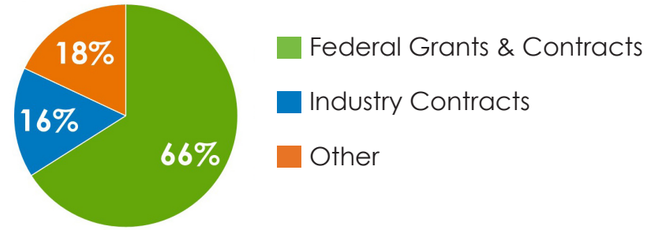
WHO WE ARE

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

FUNDING

The CIBMTR is funded through a variety of sources, including National Institutes of Health (NIH) awards, Navy grants, industry sponsors, NMDP/Be The Match, and MCW.

$57M ANNUAL FUNDING



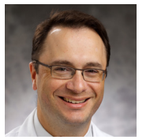
LEADERSHIP

CIBMTR MCW

Bronwen E. Shaw, MD, PhD
Chief Scientific Director, CIBMTR MCW

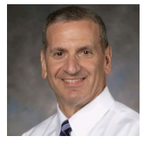
Patricia Steinert, PhD, MBA
Executive Scientific Director, Policy and Governance, CIBMTR MCW

J. Douglas Rizzo, MD, MS
Senior Scientific Director, SCTOD, CIBMTR MCW



Marcelo Pasquini, MD, MS
Senior Scientific Director, CIDR, CIBMTR MCW

CIBMTR NMDP



Jeffery J. Auletta, MD
Chief Scientific Director, CIBMTR NMDP

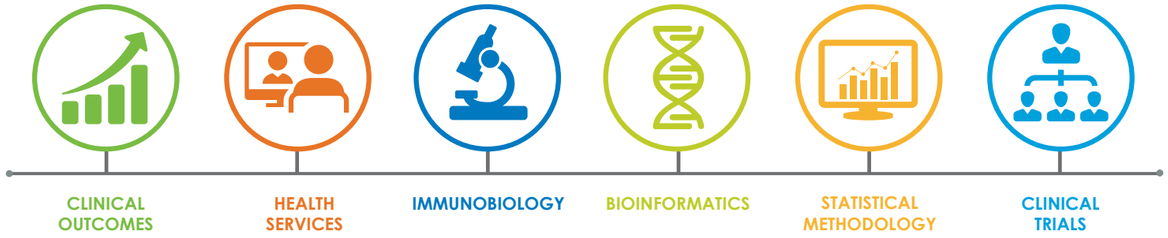
Mary Hengen, MBA
Vice President, Patient Outcomes and Experience, NMDP/Be The Match

Stephen Spellman, MBS
Vice President of Research and Senior Scientific Director, CIBMTR NMDP

Steven Devine, MD
Senior Scientific Director, CIBMTR NMDP; Chief Medical Officer, NMDP/Be The Match

VALUE TO THE COMMUNITY

The CIBMTR has collected health outcomes data worldwide for ~50 years, resulting in a Research Database with information on >600,000 patients. At any given time, the CIBMTR has approximately 200 retrospective research studies and >35 prospective research studies ongoing in 6 major areas of research activity.



OUR STRATEGY

MISSION
The CIBMTR is a collaborative resource of data and experts supporting research in cellular therapies to improve patient outcomes.

VISION
To be the premier data and resource solution for cellular therapy.

STRATEGIC PILLARS

DATA
Acquisition, analysis, sharing, and visualization of diverse data

EQUITY
Elimination of barriers to ensure health equity

RESEARCH
Transformational, interventional, and observational research

INNOVATION
Operational innovation and excellence

NEXT GENERATION
Fostering the next generation of cellular therapy research professionals

STRATEGIC PRIORITIES

1. **Data centralization, including unified data model (data):**
   Centralize CIBMTR data through one system reducing redundancy
2. **Data transformation (innovation):**
   Automate CIBMTR data collection to reduce reliance on manual data entry and facilitate rapid data sharing
3. **Succession planning (next generation):**
   Develop a clear succession plan for CIBMTR leadership positions at multiple levels
4. **Industry collaborations (research):**
   Increase speed, scope, and strategic industry collaborations to facilitate corporate studies and industry-funded clinical trials
5. **Data sharing (data):**
   Create processes to make refreshed data immediately available, which are flexible and can be easily utilized
6. **Access to cellular therapy (equity):**
   Ensure the CIBMTR promotes representative research and processes to improve access to cellular therapies

OUR IMPACT: IMPROVING PATIENTS' LIVES

The CIBMTR is dedicated to improving survival, treatment, and quality of life for patients. With **>1,650 publications**, the CIBMTR conducts practice-changing research that helps patients and physicians:

SELECT DONORS AND GRAFTS
CIBMTR studies help establish the paradigm for selecting the best donor and graft:
- Optimal human leukocyte antigen (HLA) matching
- Impact of donor characteristics
- Cord blood vs bone marrow vs peripheral blood

EVALUATE PATIENT RISK
CIBMTR studies show which patients:
- Have the highest risk of graft-vs-host disease (GVHD) and other complications
- Are most likely to benefit from cellular therapy

IDENTIFY LONG-TERM EFFECTS OF CELLULAR THERAPY
CIBMTR studies provide insight into:
- Long-term impact of cellular therapy on patients and their families, including risk of second cancers and other late complications
- Survivors' quality of life

PROVIDE MEDICAL CARE GUIDANCE FOR SURVIVORS
The CIBMTR works with the medical community to develop guidelines for optimal long-term care of cellular therapy survivors to:
- Decrease the rate of late complications
- Preserve patients' fertility
- Identify post-cellular therapy best practice preventive health behaviors

ADDRESS ACCESS TO CARE
CIBMTR studies address the broad range of issues that influence access to cellular therapy and long-term care after treatment, including:
- Disparities in access and outcomes for specific populations
- Costs of care

CLINICAL OUTCOMES RESEARCH

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 amendable to a randomized research design.

SCIENTIFIC WORKING COMMITTEES

2021 ACCOMPLISHMENTS

104 studies

27 presentations

51 publications

- >2,600 researchers worldwide
- 45 global experts voluntarily chair committees
- 372 new study proposals

Fifteen Scientific Working Committees oversee most of the CIBMTR's clinical outcomes research. Each committee focuses on a specific disease, use of cellular therapy, or complication of therapy. Through these committees, volunteer members propose, design, and implement studies. Working Committee studies are guided by Chairs who are experts in the relevant field, Scientific Directors who are experienced transplant physicians with MS degrees in biostatistics or related fields, and Statistical Directors who are PhD-level faculty experienced in methodological research. MS-level biostatisticians coordinate Working Committee activities and prepare datasets and analyses for the individual studies.

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
- Pediatric Cancer
- Plasma Cell Disorders
- Non-Malignant Diseases
- Regimen-Related Toxicity and Supportive Care

STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD)

The CIBMTR administers the SCTOD contract for the Health Resources and Services Administration (HRSA)-sponsored C.W. Bill Young Cell Transplantation Program (CWBYCTP). For the SCTOD, the CIBMTR tracks and analyzes data for all allogeneic transplants performed in the United States (US) and transplants performed globally with products from the US.

Center-Specific Volumes and Survival Analysis
The CIBMTR provides HRSA with the annual volume of transplants performed at each center, which is posted on the CWBYCTP website. The CIBMTR also performs a center-specific survival analysis evaluating one-year survival rates among US centers for transplants from related and unrelated donors. In September 2021, the CIBMTR released analysis of first allogeneic transplants performed in US centers from 2017 through 2019.

Center Outcomes Forums
The CIBMTR has conducted 8 Center Outcomes Forums to engage relevant stakeholders in the center-specific outcomes reporting process.

cibmtr.org/About/WhatWeDo/SCTOD/Pages/index.aspx

2021 ACCOMPLISHMENTS

Transplant Activity Report 2015-2019 published

Center-Specific Survival Report 2017-2019 published

Center Outcomes Forum held in November 2021

MEDICARE CED 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 focused on myelodysplastic syndrome, myelofibrosis, and multiple myeloma for elderly patients and sickle cell disease for adolescents and adults. 6,553 patients received transplants with Medicare reimbursement because of these studies.

2021 ACCOMPLISHMENTS

5 studies

6,553 patients

CLINICAL OUTCOMES RESEARCH

CELLULAR THERAPY INITIATIVES

2021 ACCOMPLISHMENTS

7,519 patients

6,079 CAR T-cell infusions

199 centers

4 publications

- Cellular Therapy 2020 Summary Slides published
- Multiple myeloma form data integrated into the registry in anticipation of myeloma-targeting CAR T-cell products becoming available in 2021

In addition to receiving data on transplant recipients, the CIBMTR received data from 199 centers for 7,519 patients who received other cellular therapies; 4,474 patients were treated for lymphoma, and 983 were treated for acute lymphoblastic leukemia. Most activity is focused on the use of chimeric antigen receptor (CAR) T-cells for hematologic cancers. 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.

AcCELLerate Forum of ASTCT, CIBMTR, and NMDP
In November 2021, the CIBMTR collaborated with the NMDP/Be The Match and the American Society for Transplantation and Cellular Therapy (ASTCT) to hold the inaugural AcCELLerate Forum: Creating a Sustainable Ecosystem of Cell and Gene Therapy, in a virtual format. Four sessions covered topics of cellular immunotherapy treatments as a standard of care, challenges and opportunities in reimbursement, cellular immunotherapy toxicities, and the future of the cellular immunotherapy field.

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 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 (CIDR)
The CIBMTR receives funding from the National Institutes of Health (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 Immuno-Oncology Translational Network (IOTN) supports the Cancer Moonshot℠ initiative to accelerate cancer research to make more therapies available to more patients.

PATIENT-REPORTED OUTCOMES

The CIBMTR collects patient-reported outcomes data using an electronic patient-reported outcomes (ePRO) system. The ePRO system incorporates a user-friendly interface in Qualtrics, automated tracking and alerting functionality, and PROMIS measures via computer logic to focus on questions relevant to each individual patient.

This year, the CIBMTR continued enrollment in the protocol for routine collection of patient-reported outcomes data, enrolling 126 patients at 8 centers and collecting 221 surveys. Core patient-reported outcomes domains include demographics, physical function, fatigue, sleep disturbance, pain interference, anxiety, depression, cognitive function, sexual function, financial toxicity, occupational functioning, and ability to participate in social roles and activities. The CIBMTR collects the core set of patient-reported outcomes data pre-infusion as well as 30 days, 100 days, 180 days, and 1-year post-infusion and annually thereafter. Patient-reported outcomes data are added to the CIBMTR Integrated Data Warehouse and available alongside clinical data for future research and to share back to centers.

2021 ACCOMPLISHMENTS

126 patients

221 patient-reported outcomes surveys

8 centers

GENE THERAPY INITIATIVES

The CIBMTR Gene Therapy Working Group collaborated with an external task force comprised of national gene therapy experts to determine how to best leverage the CIBMTR infrastructure to efficiently collect data for long-term follow-up of patients undergoing an autologous HCT using a genetically modified product. Their recommendations informed a strategic plan for the CIBMTR to seize opportunities in the gene therapy arena focusing on data collection to serve the research community. As part of the plan, the CIBMTR developed a new Gene Therapy Product form and revised the leukodystrophies disease-specific forms; these new and revised forms were released to centers in October. The CIBMTR is developing new thalassemia disease-specific forms, which it plans to release in 2022.

2021 ACCOMPLISHMENTS

Expert national task force informed data collection infrastructure needs

- New Gene Therapy Product form released to centers

CLINICAL OUTCOMES RESEARCH

INTERNATIONAL INITIATIVES

2021 ACCOMPLISHMENTS

8 joint CIBMTR-EBMT studies

68 Japanese centers supported

17 forms translated to Japanese

The CIBMTR continues to strengthen its collaborations with centers around the world as well as national and international registries. A major focus is enhancing data alignment and exchange, particularly through harmonization of data collection. CIBMTR investigators also partner on research studies, and CIBMTR staff members mentor and train colleagues across the globe.

The CIBMTR and **European Society for Blood and Marrow Transplantation** (EBMT) have a Data Sharing Agreement and Memorandum of Understanding in place that allow registry to registry sharing of European Union data compliant with the guidelines of the General Data Protection Regulation (GDPR). Currently the CIBMTR and EBMT are jointly conducting 8 research studies.

With **Cell Therapy Transplant Canada**, the CIBMTR collects data from Canadian centers and returns those data to them to support their own national outcomes registry. With the **Japanese Data Center for HCT**, the CIBMTR translates forms into Japanese, allowing Japanese centers to use the CIBMTR infrastructure to submit non-transplant cellular therapy data to the Japanese Data Center for HCT for reporting to the Japanese regulatory agency.

CURE SICKLE CELL INITIATIVE

2021 ACCOMPLISHMENT

Data set of HCT for sickle cell disease available in the NCBI database of genotypes and phenotypes

Launched in 2018 by the National Heart, Lung, and Blood Institute (NHLBI), the Cure Sickle Cell Initiative builds a community of patients, advocates, researchers, and scientists to accelerate promising genetic therapies to cure sickle cell 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. US HCT data received by the CIBMTR for sickle cell disease are now available in the National Center for Biotechnology Information (NCBI) database of genotypes and phenotypes for public use.

HEALTH SERVICES RESEARCH

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

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 ethnically diverse patient populations. Studies completed this year included analysis of outcomes of allogeneic HCT for patients with sickle cell disease by insurance type, trends in utilization of allogeneic HCT for patients with acute myeloid leukemia, and access to allogeneic HCT for patients in Virginia with a focus on sociodemographic characteristics that may influence receipt of HCT.

Survivorship / late effects / patient-reported outcomes
Patient-reported outcomes provide an essential perspective, particularly for late effects of treatment. A study completed this year described pediatric transplant program guidelines for patients returning to school.

Treatment decision-making
Health services research is vital to treatment decision-making, which can lead to better quality of care and improved outcomes. A study completed this year focused on patients' perspectives regarding palliative care. Investigators also helped merge Medicare administrative claims data with NMDP/Be The Match Search data; this combined dataset will be used to study treatment patterns and barriers to search and transplant.

2021 ACCOMPLISHMENTS

14 studies

5 presentations

5 publications

- Sociodemographic characteristics that impact access to allogeneic HCT for patients in Virginia
- Differences in allogeneic HCT outcomes based on health insurance status for patients with sickle cell disease
- Medicare administrative claims data merged with NMDP/Be The Match Search data

IMMUNOBIOLOGY RESEARCH

RESEARCH REPOSITORY

- **2,984,233** aliquots
- **17,644** cell lines

- **81,425** samples from unrelated donors and **12,702** from related donors
- **76,107** samples from unrelated recipients and **13,246** from related recipients

- **13,401** samples from unrelated cord blood units

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.

In 2021, 188 centers (150 transplant centers, 21 donor centers, and 17 cord blood banks) provided samples to the Research Repository. The Immunobiology Research Program enhanced the Research Repository inventory and Immunogenetic Database this year by completing high resolution HLA and KIR typing on ~500 related and ~2,000 unrelated HCT donor / cord and recipient pairs, bringing the total to ~45,000 unrelated donor / cord and recipient pairs that have been retrospectively high resolution typed for HLA-A, -B, -C, -DRB1 and -DQB1; >90% include -DPB1, and >18,500 include KIR. The program also distributed 38,193 research samples in support of Working Committee studies and 5,080 for the ongoing Donor Recipient Pairs Project this year.

BIOINFORMATICS RESEARCH

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.

This year the Bioinformatics Research group researched and developed three tools to guide donor selection for best patient transplant outcomes.

- **The HLA-DPB1 prediction service** addresses incomplete HLA-DPB1 data in the unrelated donor registry. The service fills missing data gaps to identify more potential matches for patients.
- **The haploidentical donor selection tool** projects disease-free survival for transplant patients with known haploidentical donor profiles, guiding the selection of a donor for best patient outcomes
- **The HLA B-leader assessment tool** optimizes donor selection in mismatched HLA scenarios through B-leader assignment. The tool translates researched B-leader scenarios between the donor and patient into a recommended donor selection sort order for best patient transplant outcomes.

The Bioinformatics Research group also analyzed the whole genome sequencing of 500 patients with myelodysplastic syndrome. Researchers identified novel genetic factors associated with myelodysplastic syndrome disease progression and transplant outcomes. The full models, which included both genomic and clinical data, were 10% better at predicting transplant survival outcomes than clinical models alone. These findings set the stage for larger integrated omics analyses and transplant outcomes prediction models in the coming year.

2021 ACCOMPLISHMENTS

12 studies

7 presentations

3 publications

3 tools

Research Goals:

- Develop flexible algorithms to accommodate missing data
- Calculate best donor matches
- Identify genomic and epigenomic factors to improve outcomes

CLINICAL TRIALS SUPPORT

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.

CLINICAL TRIAL SUPPORT CAPABILITIES

STUDY PLANNING
Oversee protocol development, identify patient population, select and train sites, and facilitate review and monitoring boards

IMMUNOBIOLOGY
Manage central pharmacies, and collect and track research samples

DATA COLLECTION
Collect protocol-specific data, conduct centralized review, and merge data across systems and centers

STATISTICAL CONSULTATION
Provide expert design and review of protocols, including creating analysis plans

PATIENT-REPORTED OUTCOMES
Centrally administer patient-reported outcomes and other surveys

ACCRUAL ASSESSMENT
Review characteristics of patients to address potential accrual barriers

SITE MANAGEMENT
Oversee site start-up, enrollment, and protocol compliance

TRIAL INTERPRETATION
Evaluate results, including through the provision of matched controls

STUDY MONITORING
Oversee on-site and remote monitoring to ensure data accuracy and mitigate risks

LONG-TERM FOLLOW-UP DATA
Capture follow-up data for long-term or secondary analyses

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK (BMT CTN)

The BMT CTN, sponsored by NHLBI and the National Cancer Institute (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.

In 2021, the BMT CTN launched 3 trials, bringing the Network's total to 57 launched trials. Participating centers accrued >1,700 patients to trials this year, increasing the total to >15,500 since inception. In addition to 11 trials in progress, 4 new protocols are in development. The BMT CTN received 60,453 new protocol-related biospecimens this year, bringing the total available biospecimens to 527,116.

cibmtr.org/Studies/ClinicalTrials/BMT_CTN/Pages/index.aspx

2021 ACCOMPLISHMENTS

11 studies

17 presentations

15 publications

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

The RCI BMT provides cellular therapy researchers with infrastructure and expertise in clinical trial design, conduct, and analysis. The program not only helps organizations and 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.

In 2021, the RCI BMT launched 3 new studies to accrual, increasing the total number of opened studies to 34. Investigators accrued >4,500 participants this year, bringing the overall total to approximately 50,000. In addition to 21 active studies in 2021, 5 upcoming studies are in development.

2021 ACCOMPLISHMENTS

26 studies

4 publications

STATISTICAL METHODOLOGY RESEARCH

2021 ACCOMPLISHMENTS

8 presentations

5 publications

Research Goals:

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

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.

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 Support
CIBMTR statisticians support not only observational studies performed through the 15 Scientific Working Committee but also investigators studying HCT, immunobiology, and other uses of hematopoietic stem cells. Support includes assistance with utilizing public databases, determining sample size and power calculations, analyzing and interpreting data, and preparing manuscripts and grants.

Statistical Expertise
The MCW Division of Biostatistics has state-of-the-art statistical software packages and computing facilities. Faculty members have expertise in and publish methodological research regarding analysis of post-HCT outcomes, Bayesian methodology, multiple comparisons, statistical genetics, model selection, high dimensional variable selection, and post-variable selection inference.

COLLABORATION WITH INDUSTRY

The biopharmaceutical industry benefits from the critical support provided by the CIBMTR. Partners gain leverage with the CIBMTR's Corporate Membership Program and unparalleled services portfolio. The CIBMTR's value-adding services include:

- Insightful statistical support
- Top-tier scientific leadership and expertise
- Reliable network of participating centers
- Unparalleled real-world evidence registry
- Value-adding Corporate Membership Program

Corporate Membership Program
Partners benefit from a membership program with updated resources to fulfill strategic, production management, and fundamental scientific objectives. Marketing managers, medical directors, research directors, product managers, case managers, and transplant coordinators leverage their company objectives with the CIBMTR's membership program resources. Program members find the best solution within five membership levels, each described on the CIBMTR Corporate Membership Program webpage.

Members benefit from access to proprietary data and resources:

- BMT survival statistics report
- Center volumes dataset
- US allogeneic HCT activity report
- Reduced registration rates at CIBMTR meetings and educational forums

Corporate Annual Meeting Support
The Tandem Meetings of ASTCT and CIBMTR include scientific plenary sessions, poster sessions, and comprehensive workshops. The meeting provides exhibiting opportunities for corporations with an interest in cellular therapy as well as corporate-supported satellite sessions.

2021 ACCOMPLISHMENTS

22 corporate partners

65 studies

- 10 corporate-supported symposia at the annual meetings

Services Portfolio:

- Descriptive reports
- Real-world data analyses
- De-identified datasets
- Protocol development and study design
- Post-authorization safety or efficacy studies
- Retrospective and prospective cohort studies
- Consultation services

PUBLICLY AVAILABLE DATA RESOURCES

STANDARD REPORTS

- **Summary Slides.** Charts and figures summarizing current uses and outcomes of cellular therapies: allogeneic and autologous HCT as well as other cellular therapies.
- **BMT Survival Statistics Report.** Detailed report on survival statistics describing the use and outcome of autologous and allogeneic HCT.
- **Transplant Data by US Center and Disease Reports.** Statistics of autologous as well as related and unrelated allogeneic transplants performed by US transplant centers.
- **Center-Specific Survival Report.** Overall risk-adjusted analysis indicating whether one-year survival for first allogeneic transplants is as expected for each specific transplant center compared to an average transplant center in the US.

RESEARCH STUDIES

To access CIBMTR data through research studies:

- **Propose a Study.** Anyone willing to follow the Study Development and Management Process is eligible to propose a study to the Working Committees.
- **Participate in a Writing Committee.** When a draft protocol is approved by the Working Committee leadership and Coordinating Center, all Working Committee members on record are invited to participate in the study Writing Committee.

CUSTOM ANALYSES

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. Coordinating Center staff members fulfill requests related to clinical decision making within 3 days and most other requests within 5 days. In 2021, the CIBMTR fulfilled 343 requests for information and data.

RESEARCH DATASETS

In accordance with the NIH Data Sharing Policy and NCI Cancer Moonshot℠ Public Access and Data Sharing Policy, the CIBMTR makes the final datasets from published studies publicly available on the CIBMTR Research Datasets for Secondary Analysis webpage. These datasets are freely available to the public for secondary analysis. Currently there are 67 final datasets from published studies available for download. In 2021, 2,492 users accessed the webpage 6,967 times and downloaded 731 datasets.

TOOLS

The CIBMTR shares its data with participating centers and corporate partners as well as other stakeholders using a variety of tools.

Data Back to Centers (DBtC)
Interact with visualizations of selected variables and download CRF- and TED-level as well as CAR T-cell therapy variables for your center.

Data for RFI
Access, view, reconcile, and export the data your center submitted into the ASTCT standard Request for Information (RFI) format.

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.

Center Volumes Portal
Preview, correct, and approve center volume data published on the HRSA CWBYCTP website.

DataOps Dashboard
Download data regarding COVID impacts, consecutive transplant audits, continuous process improvement memos, data requests, and transplant-center specific analysis.

Financial Payments Dashboard
Download your center's financial reimbursement reports related to SCTOD form payment, BMT CTN quarterly enrollment, and other study-related activities.

1-Year Survival Calculator
Predict one-year survival for individual allogeneic HCT recipients.

Disease Risk Index Assignment Tool
Categorize patients undergoing allogeneic HCT for hematologic malignancy by disease risk.

VOD Risk Calculator
Identify patients at high risk for veno-occlusive disease (VOD).

Cord Blood Report
Access monthly predefined reports regarding the quality and safety of distributed cord blood units.

PartnerShare
Visualize data in commercial partners' data sets.

Standard Reports: cibmtr.org/ReferenceCenter/SlidesReports/Pages/index.aspx
Research Datasets: cibmtr.org/ReferenceCenter/PubList/PubDsDownload/Pages/default.aspx

MEETINGS

Annual Meetings of ASTCT and CIBMTR
Held annually in February, these meetings include 5 days of scientific sessions and other meetings targeted to worldwide physicians, scientists, and other professionals interested in cellular therapy.

- **The 2021 Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR Digital Experience**
  Due to the COVID-19 pandemic, the 2021 meetings took place as a digital experience rather than an in-person event. With >4,600 attendees from 47 countries, the 2021 Digital Experience included 6 plenary sessions, 9 concurrent sessions, 13 oral abstract sessions, 4 poster highlight sessions, 9 corporate-supported symposia, 17 Meet-the-Professor sessions, 12 ASTCT Education Sessions, a CIBMTR Working Committee Session, and 12 product theaters.
- **The 2022 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR**
  The 2022 Tandem Meetings will be held at the Salt Palace Convention Center in Salt Lake City, UT, April 23-26, 2022, with some virtual components.

AcCELLerate Forum: Creating a Sustainable Ecosystem of Cell and Gene Therapy
The CIBMTR joined with the ASTCT and NMDP/Be The Match to host the AcCELLerate Forum in November 2021. The two-day, virtual workshop offered 401 attendees increased educational and advocacy opportunities. This workshop featured educational sessions bridging the gap among stakeholders to identify ongoing needs and opportunities in the field for advocacy, measurement of value and impact, and sustainability.

CIBMTR Center Outcomes Forum
In November 2021, the CIBMTR hosted the Center Outcomes Forum in a virtual format for 49 attendees. This forum offered physicians, transplant center administrators, insurance industry representatives, quality experts, patient advocates, and HCT nationwide leadership an opportunity to openly discuss the 2021 analysis, adjustment for COVID-19, and COVID-19 Working Group recommendations for the 2022 Center-Specific Survival Analysis.

TRAINING

CENTER SUPPORT
19,466 tickets addressed this year

MANUALS AND GUIDES
>2,000 pages to answer your questions

ONLINE TRAININGS
36 offered, **7** added this year

DATA MANAGER ONBOARDING
>75 new data managers this year

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. The CIBMTR's new tools collect and move data from centers to the CIBMTR electronically.

DATA TRANSFORMATION
Data. Knowledge. Lives Saved.

In 2021, the CIBMTR began implementing its data acquisition suite of products with all interested centers. Twelve centers have onboarded and report high levels of satisfaction. Most of the other US centers who share data with the CIBMTR are in discussions and on path to onboard.

The CIBMTR already has roadmaps to progressively increase functionality of the new tools, providing exponential value back to centers.

In November 2021, the CIBMTR launched Phase 2 of the Data Transformation Initiative. This phase focuses on data analysis while work continues in data acquisition (Phase 1). Within Phase 2, teams will design, build, and test 2 focused areas of development that make data become more actionable for research.

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 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 research.

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. In 2021, CIBMTR Clinical Research Associates audited HCT data at 53 domestic centers and cellular therapy data at 19 domestic centers. To assist centers that are at-risk of failing a second consecutive audit resulting in the application of audit consequences, the CIBMTR Clinical Data Validation team sent center-specific reports to 9 centers as an additional data quality check. These Centralized Data Reports identified approximately 600 potential data quality issues for centers to investigate and correct prior to their next audit.

KEY PUBLICATIONS

The CIBMTR published **99 peer-reviewed manuscripts** in scientific journals this year. Some of the CIBMTR's key findings were published in the following articles.

Devine SM, Horowitz MM. **Building a fit for purpose clinical trials infrastructure to accelerate the assessment of novel HCT strategies and cellular immunotherapies.** Journal of Clinical Oncology. 2021 Feb 10; 39(5):534-544. doi:10.1200/JCO.20.01623. Epub 2021 Jan 12. PMC8443822.

Watkins B, Qayed M, McCracken C, et al. **Phase II trial of costimulation blockade with abatacept for prevention of acute GVHD.** Journal of Clinical Oncology. 2021 Jun 1; 39(17):1865-1877. doi:10.1200/JCO.20.01086. Epub 2021 Jan 15. PMC8260909.

Sharma A, Bhatt NS, St. Martin A, et al. **Clinical characteristics and outcomes of COVID-19 in HCT recipients: An observational cohort study.** The Lancet Haematology. 2021 Mar 1; 8(3)e185-e193. doi:10.1016/S2352-3026(20)30429-4. Epub 2021 Jan 19. PMC7816949.

Shalabi H, Gust J, Taraseviciute A, et al. **Beyond the storm - subacute toxicities and late effects in children receiving CAR T cells.** Nature Reviews Clinical Oncology. 2021 Jun 1; 18(6):363-378. doi:10.1038/s41571-020-00456-y. Epub 2021 Jan 25. PMC8257494.

Reshef R, Saber W, Bolaños-Meade J, et al. **Acute GVHD diagnosis and adjudication in a multicenter trial: A report from the BMT CTN 1202 biorepository study.** Journal of Clinical Oncology. 2021 Jun 1; 39(17):1878-1887. doi:10.1200/JCO.20.00619. Epub 2021 Jan 28. PMC8260916.

Goldsmith SR, Abid MB, Auletta JJ, et al. **Posttransplant cyclophosphamide is associated with increased cytomegalovirus infection: A CIBMTR analysis.** Blood. 2021 Jun 10; 137(23):3291-3305. doi:10.1182/blood.2020009362. Epub 2021 Mar 3. PMC8351903.

Wieduwilt MJ, Stock W, Advani A, et al. **Superior survival with pediatric-style chemotherapy compared to myeloablative allogeneic HCT in older adolescents and young adults with Ph-negative ALL in first complete remission: Analysis from CALGB 10403 and the CIBMTR.** Leukemia. 2021 Jul 1; 35(7):2076-2085. doi:10.1038/s41375-021-01213-5. Epub 2021 Mar 30. PMC8257494.

Mayor NP, Wang T, Lee SJ, et al. **Impact of previously unrecognized HLA mismatches using ultrahigh resolution typing in unrelated donor HCT.** Journal of Clinical Oncology. 2021 Jul 20; 39(21):2397-2409. doi:10.1200/JCO.20.03643. Epub 2021 Apr 9. PMC8280068.

Gooptu M, Romee R, St Martin A, et al. **HLA-haploidentical vs matched unrelated donor transplants with posttransplant cyclophosphamide-based prophylaxis.** Blood. 2021 Jul 22; 138(3):273-282. doi:10.1182/blood.2021011281. Epub 2021 Apr 13. PMC8310426.

Mupfudze TG, Meyer C, Preussler JM, et al. **HCT outcomes among Medicaid and privately insured patients with sickle cell disease.** Transplantation and Cellular Therapy. 2021 Aug 1; 27(8):685.e1-685.e8. doi:10.1016/j.jtct.2021.04.009. Epub 2021 Apr 13.

Shaw BE, Jimenez-Jimenez AM, Burns LJ, et al. **NMDP-sponsored multicenter, Phase II trial of HLA-mismatched unrelated donor bone marrow transplantation using post-transplant cyclophosphamide.** Journal of Clinical Oncology. 2021 Jun 20; 39(18):1971-1982. doi:10.1200/JCO.20.03502. Epub 2021 Apr 27. PMC8260905.

Scordo M, Wang TP, Ahn KW, et al. **Outcomes associated with thiotepa-based conditioning in patients with primary central nervous system lymphoma after autologous HCT.** JAMA Oncology. 2021 Jul 1; 7(7):993-1003. doi:10.1001/jamaoncol.2021.1074. Epub 2021 May 6. PMC8283558.

Logan BR, Maiers MJ, Sparapani RA, et al. **Optimal donor selection for HCT using Bayesian machine learning.** JCO Clinical Cancer Informatics. 2021 May 1; 5:494-507. doi:10.1200/CCI.20.00185. Epub 2021 May 1. PMC8443829.

Reilly CR, Myllymäki M, Redd R, et al. **The clinical and functional effects of TERT variants in MDS.** Blood. 2021 Sep 9; 138(10):898-911. doi:10.1182/blood.2021011075. Epub 2021 May 21. PMC8432045.

Nakamura R, Saber W, Martens MJ, et al. **Biologic assignment trial of reduced-intensity HCT based on donor availability in patients 50-75 years of age with advanced MDS.** Journal of Clinical Oncology. 2021 Oct 20; 39(30):3328-3339. doi:10.1200/JCO.20.03380. Epub 2021 Jun 9.

Ciurea SO, Kongtim P, Soebbing D, et al. **Decrease post-transplant relapse using donor-derived expanded NK-cells.** Leukemia. 2022 Jan 1; 36(1):155-164. doi:10.1038/s41375-021-01349-4. Epub 2021 Jul 26.

Marsh RA, Hebert K, Kim S, et al. **A comparison of HCT conditioning regimens for hemophagocytic lymphohistiocytosis disorders.** Journal of Allergy and Clinical Immunology. doi:10.1016/j.jaci.2021.07.031. Epub 2021 Aug 7.

Shindiapina P, Pietrzak M, Seweryn M, et al. **Immune recovery following autologous HCT in HIV-related lymphoma patients on the BMT CTN 0803/AMC 071 trial.** Frontiers in Immunology. doi:10.3389/fimmu.2021.700045. Epub 2021 Sep 3. PMC8446430.

Sajulga R, Bolon Y-T, Maiers M, Petersdorf EW. **Assessment of HLA-B genetic variation with an HLA-B leader tool and implications in clinical transplantation.** Blood Advances. doi:10.1182/bloodadvances.2021004561. Epub 2021 Sep 16.

Shadman M, Pasquini MC, Ahn KW, et al. **Autologous transplant versus CAR T-cell therapy for relapsed DLBCL in partial remission.** Blood. doi:10.1182/blood.2021013289. Epub 2021 Sep 27.

Fuchs EJ, McCurdy SR, Solomon SR, et al. **HLA informs risk predictions after haploidentical HCT with post-transplantation cyclophosphamide.** Blood. doi:10.1182/blood.2021013443. Epub 2021 Nov 1.

Luznik L, Pasquini M, Logan B, et al. **Randomized Phase III BMT CTN trial of calcineurin inhibitor-free chronic GVHD interventions in myeloablative HCT for hematologic malignancies.** Journal of Clinical Oncology. doi:10.1200/JCO.21.02293. Epub 2021 Dec 2.

RESEARCH SUMMARIES FOR PATIENTS

The CIBMTR creates plain language summaries of some of its research articles. These easy-to-read summaries help patients and their loved ones learn about the latest research and treatment options. In 2021, the CIBMTR published **18 research summaries**.

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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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