CIBMTR®

CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

2022 ANNUAL REPORT

Sharing Knowledge.
Sharing Hope.

2022 AT-A-GLANCE

OUR NUMBERS

375 participating centers

>630,000 patients

>27,000 new patients annually

~250 ongoing studies and clinical trials

>205,000 biorepository samples

71 peer-reviewed publications

95 presentations at conferences

LETTER FROM OUR CHIEF SCIENTIFIC DIRECTORS

Dear CIBMTR Community:

We are pleased to share the 2022 Annual Report with our cellular therapy community as we celebrate CIBMTR's 50th anniversary (page 4).

As another year of the pandemic passes, we appreciate how its effects continue to cause burden and challenges for centers, patients, and caregivers alike. Despite staff and supply chain shortages, the cellular therapy community remains resilient and determined in its quest to provide the best outcomes for patients based upon rigorous investigation.

For our part, CIBMTR has worked hard to continue to advance our mission. Important successes this year include launching a new strategic plan with significant progress in many of our goals (page 3), renewing the competitive contract to operate the Stem Cell Therapeutic Outcomes Database (page 6), and publishing 71 manuscripts in collaboration with our Working Committees and others in the community to improve patient outcomes (pages 21-22).

Without your individual and center's contributions, our ability to learn from patients would not become a reality. For your continued efforts and determination, we give you our thanks, and in this report, we provide you with all that we accomplished together.

With gratitude,

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

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

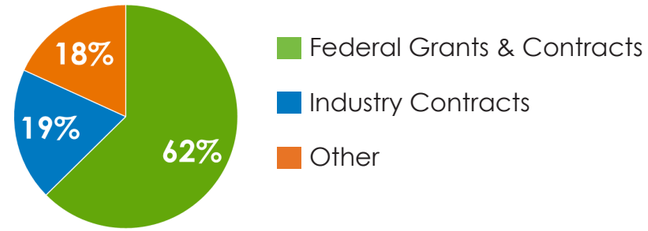
WHO WE ARE

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

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

$59 MILLION ANNUAL FUNDING



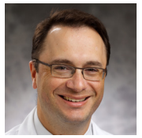
LEADERSHIP

CIBMTR MCW

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

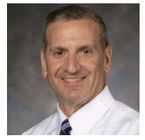
Patricia Steinert, PhD, MBA
Executive Scientific Director, Policy and Governance, CIBMTR MCW

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

OUR RESEARCH

CIBMTR conducts studies in 6 major areas of research activity.

OUR STRATEGY

MISSION
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.

2022 ACHIEVEMENTS

DATA: *Acquisition, analysis, sharing, and visualization of diverse data*
- Made significant progress in centralizing transplant data into the Unified Data Model to remove redundancy and standardize data extracts (page 20)
- Created a roadmap for automating acquisition of various types of data to accelerate progress of the Data Transformation Initiative (page 20)
- Posted 40 new publicly available datasets on the website to promote re-use of CIBMTR data and expand options to access data (page 17)

EQUITY: *Elimination of barriers to ensure health equity*
- Implemented enrollment initiative to increase participation of ethnically diverse patients

RESEARCH: *Transformational, interventional, and observational research*
- Implemented a process for project intake, categorization, prioritization, and tracking

INNOVATION: *Operational innovation and excellence*
- Enhanced speed and scope of strategic industry collaborations to facilitate corporate studies (page 16)
- Implemented a forum for Scientific Directors to standardize Working Committee processes (page 5)
- Created a branded toolkit package for communication use; new website to be launched in early 2023

NEXT GENERATION: *Fostering the next generation of cellular therapy research professionals*
- Developed mentorship strategies and enhanced junior investigators' opportunities for participating in CIBMTR Working Committees (page 5)

CELEBRATING 50 YEARS

HIGHLIGHTS FROM CIBMTR, 1972-2022

1972
Dr. Mortimer M. Bortin and colleagues establish the International Bone Marrow Transplant Registry (IBMTR); Dr. Bortin serves as first Scientific Director (1972-1991)

1985
IBMTR receives first major NIH grant funding

1986
Dr. Bob Graves, Adm. Elmo Zumwalt, and colleagues establish the NMDP with funds appropriated by the US government

1991
Dr. Mary Horowitz begins her 30-year tenure as Chief Scientific Director of IBMTR / CIBMTR

CELEBRATING 50 YEARS 1972-2022 CIBMTR® CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

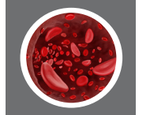
1995
First BMT Tandem Meetings in Keystone, Colorado

2001
IBMTR, NMDP, and Emmes establish the Blood and Marrow Transplant Clinical Trials Network (BMT CTN), which receives grant funding

2004
IBMTR at MCW and NMDP enter a research affiliation to form CIBMTR, integrating the research activities of the organizations

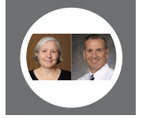
2007
CIBMTR is awarded the contract for the Stem Cell Therapeutic Outcomes Database (SCTOD), part of the C.W. Bill Young (pictured) Cell Transplantation Program, from the Health Resources and Services Administration (HRSA)

2016
CIBMTR launches cellular therapy data collection and research with initial funding from the National Cancer Institute (NCI)



2019
The National Heart, Lung, and Blood Institute (NHLBI) awarded CIBMTR an Other Transactional Agreement to build a research data ecosystem with the Cure Sickle Cell Initiative

2020
CIBMTR launches the gene therapy data collection initiative



2021
Drs. Jeffery Auletta and Bronwen Shaw named co-Chief Scientific Directors of CIBMTR

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

2022 ACCOMPLISHMENTS

113 studies

38 presentations

32 publications

- >2,800 researchers worldwide
- 45 global experts voluntarily chair committees
- 330 new study proposals this year

Fourteen Scientific Working Committees oversee most of 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, prepare datasets, and perform 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
- Lymphoma
- Morbidity, Recovery, and Survivorship
- Non-Malignant Diseases
- Pediatric Cancer
- Plasma Cell Disorders

STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD)

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

Renewal of SCTOD Contract
CIBMTR successfully renewed the SCTOD contract for another 5 years: September 27, 2022, through September 26, 2027.

Center-Specific Volumes and Survival Analysis
CIBMTR provides HRSA with the annual volume of transplants performed at each center, which was updated on the CWBYCTP website in April 2022 for transplants performed in US centers from 2016 through 2020. CIBMTR also performs a Center-Specific Survival Analysis evaluating one-year survival rates among US centers for transplants from related and unrelated donors. In December 2022, CIBMTR released analysis of first allogeneic transplants performed in US centers from 2018 through 2020.

2022 ACCOMPLISHMENTS

- SCTOD contract renewed in September
- Transplant Activity Report 2016-2020 published
- Center-Specific Survival Analysis 2018-2020 published

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 in clinical studies that inform policy decisions. 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. Nearly 8,000 patients received transplants with Medicare reimbursement because of these studies.

2022 ACCOMPLISHMENTS

5 studies

7,970 patients

CELLULAR THERAPY INITIATIVES

2022 ACCOMPLISHMENTS

10,976 patients

214 centers

4 publications

- Cellular Therapy 2021 Summary Slides published
- Follicular lymphoma and mantle cell lymphoma post-authorization safety study completed accrual

In addition to receiving data on transplant recipients, CIBMTR received data from 214 centers for 10,976 patients who received other cellular therapies; 6,646 patients were treated for lymphoma, and 1,401 were treated for acute lymphoblastic leukemia. Most activity is focused on the use of chimeric antigen receptor T cells (CAR-T) for hematologic cancers. CIBMTR receives these data via a suite of Cellular Therapy Essential Data (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 2022, CIBMTR collaborated with the NMDP/Be The Match and the American Society for Transplantation and Cellular Therapy (ASTCT) to hold the AcCELLerate Forum: Creating a Sustainable Ecosystem of Cell and Gene Therapy, in a virtual format. Four sessions covered topics of Center-Specific Survival Analysis for CAR-T immunotherapy, updates on the 80/20 Initiative, updates in immune effector cell-associated toxicity, and the future of cellular therapies.

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. 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)
CIBMTR receives funding from 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

CIBMTR's electronic patient-reported outcomes system incorporates a user-friendly interface in Qualtrics, automated tracking and alerting functionality, and Patient-Reported Outcomes Measurement Information System (PROMIS) computer adaptive measures that use computer logic to focus on questions relevant to each individual patient.

In 2022, CIBMTR enrolled 390 patients at 19 centers and collected 1,065 surveys. Core outcome domains include fatigue, sleep disturbance, pain interference, anxiety, depression, sexual function, financial toxicity, occupational functioning, ability to participate in social roles and activities, and others. CIBMTR collects these data pre-infusion as well as 30 days (for CAR-T patients), 100 days, 180 days, and 1-year post-infusion and annually thereafter.

This year CIBMTR began sharing patient-reported outcomes data, with patient consent, in the Data Back to Centers platform. Centers can view PROMIS scores by time point for individuals or groups of patients, filtered by standard CIBMTR variables.

2022 ACCOMPLISHMENTS

390 patients

19 centers

1,065 surveys collected

GENE THERAPY INITIATIVES

In 2020, CIBMTR began developing capabilities to address the emerging field of gene therapies for multiple diseases. CIBMTR established a Gene Therapy Working Group, which collaborated with a task force, and engaged with several biopharmaceutical companies interested in utilizing the CIBMTR Research Database for long-term follow-up of gene therapy recipients.

Task Force recommendations informed a gene therapy strategic plan for CIBMTR to seize opportunities in data collection to serve the research community. In 2022, CIBMTR developed and released new thalassemia disease-specific forms and developed an eLearning module to provide comprehensive instructions to centers on submitting gene therapy data to CIBMTR. CIBMTR received infusion data for 14 gene therapy products this year.

2022 ACCOMPLISHMENTS

14 gene therapy product infusions reported

New thalassemia disease-specific forms released

- Data submission education offered

CLINICAL OUTCOMES RESEARCH

INTERNATIONAL INITIATIVES

2022 ACCOMPLISHMENTS

8 joint CIBMTR-EBMT studies

88 Japanese centers supported

17 forms translated to Japanese

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 harmonizing data collection. CIBMTR investigators also partner on research studies, and CIBMTR staff members mentor and train colleagues across the globe.

CIBMTR and **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 CIBMTR and EBMT are jointly conducting 8 research studies.

CIBMTR collects data from Canadian centers and returns those data to **Cell Therapy Transplant Canada** to support their national outcomes registry. With the **Japanese Data Center for HCT**, CIBMTR translates forms into Japanese, allowing Japanese centers to use CIBMTR's 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

2022 ACCOMPLISHMENT

BMT CTN 2001 clinical trial opened to enrollment

Launched in 2018 by 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, CIBMTR has worked with the Cure Sickle Cell Data Consortium to build a research data ecosystem designed to support investigator-initiated collaborative research. US transplant data received by CIBMTR for sickle cell disease are now available for public use in the National Center for Biotechnology Information database of genotypes and phenotypes. In 2022, the initiative opened BMT CTN 2001, a Phase II multicenter gene transfer study that is currently enrolling patients nationwide.

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 access, utilization, delivery, 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 linking CIBMTR and California Cancer Registry data; this dataset will be used to further study access and healthcare disparities.

Survivorship / late effects / patient-reported outcomes
Patient-reported outcomes provide an essential perspective, particularly for late effects of treatment. Multiple completed studies this year used a merged database of long-term transplant survivors to assess patient-reported outcomes.

Treatment decision-making
Health services research is vital to treatment decision-making, which can lead to better quality of care and improved outcomes. Researchers are currently using merged Medicare administrative claims data and NMDP/Be The Match Search data to study referral and treatment patterns as well as barriers to search and transplant.

2022 ACCOMPLISHMENTS

10 studies

9 presentations

5 publications

- Merged CIBMTR and California Cancer Registry datasets
- Characterized trends in utilization of and unmet need for allogeneic HCT for patients with acute myeloid leukemia
- Identified factors associated with receipt of allogeneic HCT using linked Medicare administrative claims and NMDP/Be The Match Search data

IMMUNOBIOLOGY RESEARCH

RESEARCH SAMPLE REPOSITORY

(as of Dec. 1, 2022)

- **2,678,873** aliquots
- **17,627** cell lines

- **84,626** samples from unrelated donors and **13,849** from related donors
- **80,453** samples from unrelated recipients and **14,467** from related recipients

- **13,730** samples from unrelated cord blood units

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

CIBMTR leverages both the NMDP/Be The Match's investment in the Unrelated Donor Research Sample Repository and NIH's investment in the CIBMTR Research Database. Linking clinical outcomes data with high-quality immunogenetic data from the Research Sample Repository supports research studies involving genetic, immunobiology, and clinical phenotype data. Together the Unrelated and Related Donor Research Sample Repositories provide an organized approach to studying transplant biology across the spectrum of allogeneic HCT.

The Related Donor Research Sample Repository, supported by HRSA, offers a unique opportunity to enhance immunobiology research. Related donors often have greater HLA match levels. With advancements in graft-versus-host disease prevention, haploidentical relatives have emerged as a viable donor option, expanding access to HCT. The Repository now boasts paired samples collected from ~3,000 transplants with haploidentical donors, allowing researchers to further explore donor optimization.

In 2022, 184 centers (152 transplant centers, 17 donor centers, and 15 cord blood banks) provided samples to the Repository. This year, CIBMTR enhanced the Repository inventory and Immunogenetic Database by completing high resolution HLA typing on ~450 related and ~1,450 unrelated donor / cord and recipient pairs, bringing the total number of donor / cord recipient pairs retrospectively typed through the program to nearly 50,000. All paired samples are typed at high resolution at HLA-A, -B, -C, -DRB1, and -DQB1; >90% of paired samples have typing at -DPB1, and >18,500 include KIR. The program also distributed 28,444 research samples in support of Working Committee studies and 3,758 samples for the ongoing Donor-Recipient Pairs Project.

BIOINFORMATICS RESEARCH

The Bioinformatics Research Program specializes in matching patients and cellular therapies, saving 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 4 main areas: Genomics / omics and high-throughput bioanalytics, machine learning and clinical predictions, cellular therapy matching, and donor registry modeling.

This year the Bioinformatics Research group researched and developed 3 tools to guide donor selection for best patient transplant outcomes.

- **The Class I HLA peptide binding motif (PBM) tool** maps Class I HLA typing to PBM groups and calculates the direction of the group match or mismatch. Mismatches across PBM groups in the graft-versus-host vector are predicted to be less well tolerated than mismatches within the same PBM group or in the host-versus-graft vector.
- **The HLA-DP tool "ExPAT"** aids users in assessing HLA-DPB1 sequence data, produces allele and model assignments, and sorts from potential donor lists. The tool automates complex allele and model assignments to match donor sources to patients. It can also be leveraged to research population diversity associated with HLA variation and clinical impacts.
- **The haploidentical donor selection tool** projects disease-free survival for transplant patients with known haploidentical donor profiles, guiding donor selection for best patient outcomes, based on prior outcomes with the use of post-transplant cyclophosphamide.

The group also analyzed whole genome sequencing, methylation, and proteomic data from patient samples pre-transplant. These analyses identified novel factors associated with disease progression and transplant outcomes. The team will use these factors to develop prediction models and assessment tools.

2022 ACCOMPLISHMENTS

- **12** studies
- **15** presentations
- **3** publications
- **3** tools

Research Goals:

- Automate generation of population haplotype frequencies
- Complement operational decisions in donor selection for best patient outcomes
- Identify impactful genomic and epigenomic biomarkers

CLINICAL TRIALS SUPPORT

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

In 2022, the BMT CTN launched 3 trials, bringing the Network's total to 60 launched trials. Participating centers accrued >600 patients to trials this year, increasing the total to >16,400 since inception. In addition to 10 trials in progress, 8 new protocols are in development. The BMT CTN received 17,776 new protocol-related biospecimens this year, bringing the total available biospecimens to 544,825.

2022 ACCOMPLISHMENTS

10 studies

9 presentations

11 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 2022, the RCI BMT launched 2 new studies to accrual, increasing the total number of opened studies to 36. Investigators accrued ~4,000 participants this year, bringing the overall total to ~54,000. In addition to 21 active studies in 2022, 3 upcoming studies are in development.

2022 ACCOMPLISHMENTS

21 studies

3 presentations

STATISTICAL METHODOLOGY RESEARCH

2022 ACCOMPLISHMENTS

4 presentations

5 publications

Research Goals:

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

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. 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 14 Scientific Working Committees 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 garners critical resources from mutually beneficial collaborations with CIBMTR. Benefits include significant leverage through CIBMTR's Corporate Membership Program and an exceptional services portfolio. CIBMTR's noteworthy services include:

- Insightful statistical support
- Top-tier scientific leadership and expertise
- Reliable network of participating centers
- Unparalleled real-world data registry
- Value-adding real-world evidence to fulfill project requirements

Corporate Membership Program
Partners benefit from a membership program with updated resources that 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 CIBMTR's membership program resources. Members find the best solution within 5 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 I Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR include scientific plenary sessions, poster sessions, and comprehensive workshops. The meetings provide exhibiting opportunities for corporations with an interest in cellular therapy as well as corporate-supported satellite symposia.

2022 ACCOMPLISHMENTS

44 corporate partners

84 studies

- 11 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 Analysis.** 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 2022, CIBMTR fulfilled 295 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, 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 107 final datasets from published studies available for download. In 2022, 3,325 users accessed the webpage 9,396 times and downloaded 811 datasets.

TOOLS

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 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.

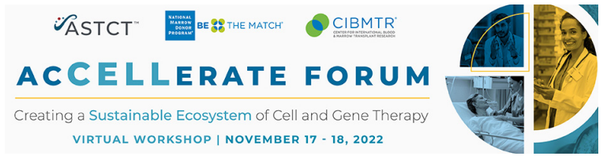
MEETINGS

Annual Tandem 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 2022 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR** The 2022 Tandem Meetings took place at the Salt Palace Convention Center in Salt Lake City, UT, April 23-26, 2022, offering in-person and virtual programming. With >4,600 attendees from 51 countries, the 2022 Tandem Meetings included 6 plenary sessions, 9 concurrent sessions, 15 oral abstract sessions, 11 corporate-supported symposia, 11 Meet-the-Professor Sessions, 14 ASTCT Spotlight Sessions, 16 CIBMTR Working Committee Sessions, and 15 product theaters.

- **The 2023 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR** The 2023 Tandem Meetings will be held at the World Center Marriott in Orlando, FL, February 15-19, 2023, and will offer both in-person and virtual programming.

2022 AcCELLerate Forum: Creating a Sustainable Ecosystem of Cell and Gene Therapy
CIBMTR joined with the ASTCT and NMDP/Be The Match to host the AcCELLerate Forum in November 2022. The 2-day, virtual workshop offered 360 attendees in the field of cell and gene therapy 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.



TRAINING

DATA TRANSFORMATION INITIATIVE

Data Transformation Initiative (DTI) team members connected 20 new centers to the DTI data acquisition suite of products this year. This connection allows centers to reduce the burden of data entry by taking advantage of tools and solutions that streamline the data capture process.

DATA TRANSFORMATION
Data. Knowledge. Lives Saved.

In addition, the DTI team upgraded the CIBMTR Reporting Application, deployed within the EPIC Electronic Health Record App Orchard, from Fast Healthcare Interoperability Resources (FHIR) Revision 3 to FHIR Revision 4. This change benefits centers using the application by supporting further data capture automation via FHIR.

Finally, the DTI team worked in parallel to expand FHIR Implementation Guides for non-EPIC partners, and they launched an effort to move towards modular data acquisition. Together these accomplishments help expand data acquisition automation, streamline the data capture experience, and advance research supported by CIBMTR data capture.

INTEGRATED DATA WAREHOUSE

The CIBMTR Integrated Data Warehouse consolidates data coming from multiple sources and spanning multiple domains, such as patient-reported outcomes, product data, HLA, biospecimens, and study data. This year CIBMTR automated transfer of clinical trials study data collected in RAVE to the Data Warehouse. CIBMTR also continued expanding the newly implemented Unified Data Model and completed integration of all required FormsNet data. Staff members designed and implemented a patient safety update report template for cellular therapy partners, designed an audit report for the Japanese Data Center for HCT, and developed a design approach for new gene therapy extracts.

DATA AUDIT PROGRAM

Ongoing 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 2022, CIBMTR clinical research associates audited HCT data at 55 domestic centers and cellular therapy data at 13 domestic centers.

The CIBMTR Clinical Data Validation team sent center-specific reports to 29 centers as an additional data quality check to assist centers that are at risk of failing a second consecutive audit, resulting in the application of audit consequences; centers that currently have consequences applied; and select centers that have never had a CIBMTR audit. These Centralized Data Reports identified ~3,600 potential data quality issues for centers to investigate and correct prior to their next audit.

KEY PUBLICATIONS

CIBMTR published **71 peer-reviewed manuscripts** in scientific journals this year. Some of CIBMTR's key findings were published in the following articles.

Guru Murthy GS, Kim S, Hu Z-H, et al. **Relapse and disease-free survival in patients with MDS undergoing allogeneic HCT using older matched sibling donors vs younger matched unrelated donors.** JAMA Oncology. 2022 Mar 1; 8(3):404-411. doi:10.1001/jamaoncol.2021.6846. Epub 2022 Jan 13. PMC8759031.

Savani M, Ahn KW, Chen Y, et al. **Impact of conditioning regimen intensity on the outcomes of peripheral T-cell lymphoma, anaplastic large cell lymphoma and angioimmunoblastic T-cell lymphoma patients undergoing allogeneic transplant.** British Journal of Haematology. 2022 Apr 1; 197(2):212-222. doi:10.1111/bjh.18052. Epub 2022 Feb 2. PMC9018546.

Mei M, Hamadani M, Ahn KW, et al. **Autologous HCT in diffuse large B-cell lymphoma after three or more lines of prior therapy: Evidence of durable benefit.** Haematologica. 2022 May 1; 107(5):1214-1217. doi:10.3324/haematol.2021.279999. Epub 2022 Feb 3. PMC9052914.

Brazauskas R, Jatoi A, Le-Rademacher J. **A cautionary tale: Grouping patients on late events.** Mayo Clinic Proceedings. 2022 Mar 1; 97(3):449-453. doi: 10.1016/j.mayocp.2021.11.003. Epub 2022 Feb 5.

Dispenzieri A, Krishnan A, Arendt B, et al. **Mass-Fix better predicts for PFS and OS than standard methods among multiple myeloma patients participating on the STAMINA trial (BMT CTN 0702 / 07LT).** Blood Cancer Journal. 2022 Feb 10; 12(2):27. doi:10.1038/s41408-022-00624-6. Epub 2022 Feb 10. PMC8831597.

Petersdorf EW, Bengtsson M, Horowitz MM, et al. **HLA-DQ heterodimers in HCT.** Blood. 2022 May 19; 139(20):3009-3017. doi:10.1182/blood.2022015860. Epub 2022 Mar 10. PMC9121842.

Abou-Ismail MY, Fraser R, Allbee-Johnson M, et al. **Does recipient body mass index inform donor selection for allogeneic HCT?** British Journal of Haematology. 2022 May 1; 197(3):326-338. doi:10.1111/bjh.18108. Epub 2022 Mar 14. PMC9675037.

Baccarani M, Bonifazi F, Soverini S, et al. **Questions concerning tyrosine kinase-inhibitor therapy and transplants in chronic phase chronic myeloid leukaemia.** Leukemia. 2022 May 1; 36(5):1227-1236. doi:10.1038/s41375-022-01522-3. Epub 2022 Mar 25. PMC9061294.

Mei M, Pillai R, Kim S, et al. **The mutational landscape in chronic myelomonocytic leukemia and its impact on allogeneic HCT outcomes: A CIBMTR analysis.** Haematologica. doi:10.3324/haematol.2021.280203. Epub 2022 Apr 21.

Thanarajasingam G, Minasian LM, Bhatnagar V, et al. **Reaching beyond maximum grade: Progress and future directions for modernising the assessment and reporting of adverse events in haematological malignancies.** The Lancet Haematology. 2022 May 1; 9(5):e374-e384. doi:10.1016/S2352-3026(22)00045-X. Epub 2022 Apr 29. PMC9241484.

Osoegawa K, Marsh SGE, Holdsworth R, et al. **A new strategy for systematically classifying HLA alleles into serological specificities.** HLA. 2022 Sep 1; 100(3):193-231. doi: 10.1111/tan.14662. Epub 2022 May 10.

Arrieta-Bolaños E, Crivello P, He M, et al. **A core group of structurally similar HLA-DPB1 alleles drives permissiveness after HCT.** Blood. 2022 Aug 11; 140(6):659-663. doi:10.1182/blood.2022015708. Epub 2022 May 24. PMC9373015.

Dholaria B, Radujkovic A, Estrada-Merly N, et al. **Outcomes of allogeneic HCT for chronic neutrophilic leukaemia: A combined CIBMTR/CMWP of EBMT analysis.** British Journal of Haematology. 2022 Aug 1; 198(4):785-789. doi:10.1111/bjh.18297. Epub 2022 Jun 3. PMC9750039.

Richardson PG, Jacobus SJ, Weller EA, et al. **Triplet therapy, transplantation, and maintenance until progression in myeloma.** New England Journal of Medicine. 2022 Jul 14; 387(2):132-147. doi:10.1056/NEJMoa2204925. Epub 2022 Jun 5.

McReynolds LJ, Rafati M, Wang Y, et al. **Genetic testing in severe aplastic anemia is required for optimal HCT outcomes.** Blood. 2022 Aug 25; 140(8):909-921. doi:10.1182/blood.2022016508. Epub 2022 Jul 1.

Rotz SJ, Yi JC, Hamilton BK, et al. **Health related quality of life in young adult survivors of HCT.** Transplantation and Cellular Therapy. 2022 Oct 1; 28(10):701.e1-701.e7. doi:10.1016/j.jtct.2022.07.018. Epub 2022 Jul 22. PMC9547939.

DeZern AE, Eapen M, Wu J, et al. **Haploidentical bone marrow transplantation in patients with relapsed or refractory severe aplastic anaemia in the USA (BMT CTN 1502): A multicentre, single-arm, Phase 2 trial.** The Lancet Haematology. 2022 Sep 1; 9(9):e660-e669. doi:10.1016/S2352-3026(22)00206-X. Epub 2022 Jul 27. PMC9444987.

Worel N, Aljurf M, Anthias C, et al. **Suitability of haematopoietic cell donors: Updated consensus recommendations from the WBMT standing committee on donor issues.** The Lancet Haematology. 2022 Aug 1; 9(8):e605-e614. doi:10.1016/S2352-3026(22)00184-3. Epub 2022 Jul 29.

Feurstein SK, Trottier AM, Estrada-Merly N, et al. **Germline predisposition variants occur in MDS patients of all ages.** Blood. 2022 Dec 15; 140(24):2533-2548. doi:10.1182/blood.2022015790. Epub 2022 Aug 19.

Brown DW, Zhou W, Wang Y, et al. **Germline-somatic JAK2 interactions are associated with clonal expansion in myelofibrosis.** Nature Communications. 2022 Sep 8; 13(1):5284. doi:10.1038/s41467-022-32986-7. Epub 2022 Sep 8. PMC9458655.

Furqan F, Ahn KW, Chen Y, et al. **Allogeneic HCT in patients with relapsed/refractory anaplastic large cell lymphoma.** British Journal of Haematology. 2023 Jan 1; 200(1):54-63. doi:10.1111/bjh.18467. Epub 2022 Sep 19.

RESEARCH SUMMARIES FOR PATIENTS

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 2022, CIBMTR published **14 research summaries**.

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

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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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