CIBMTR®

A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

2023 ANNUAL REPORT

LETTER FROM OUR CHIEF SCIENTIFIC DIRECTORS

Dear CIBMTR Community:

We are pleased to share the 2023 Annual Report with our cellular therapy community. In this second year of our 3-year strategy, we are delighted to highlight our accomplishments achieved through the support and involvement of all our stakeholders (pp. 3-4).

CIBMTR's sustainability is extremely important. We are proud this year to have renewed the U24 resource grant, continuing our work in hematopoietic cell transplantation (HCT) and expanding our agenda for adoptive cellular therapies (ACT), including chimeric antigen receptor T cells (CAR-T), gene therapies, and others. We also significantly expanded our partnerships with industry.

To align structure with strategy, we refreshed the roles, responsibilities, and term limits for external leaders elected to CIBMTR committees. These changes provide clarity, ensure diverse representation, enhance efficiencies, expand opportunities, and reflect the future needs of the cellular therapy field.

Lastly, we started planning for the launch of the 2025 strategy. As we reflect on our strategic accomplishments to date, we see further opportunity to enhance data capture to speed research and support the community in all aspects of our mission. We look forward to sharing more information as this develops.

Thank you for your continued partnership as we work together to improve outcomes in cellular therapy patients.

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

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

OUR IMPACT

>360
participating centers

>675,000
patients

~30,000
new patients annually

~200
ongoing studies and clinical trials

>215,000
biorepository samples

97
publications

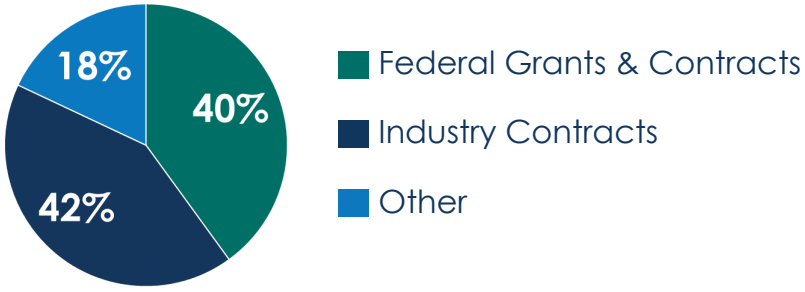
96
presentations at conferences

CIBMTR® (Center for International Blood & Marrow Transplant Research®) is a research collaboration between the Medical College of Wisconsin (MCW) and NMDP.

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, and MCW.

$58.3 MILLION ANNUAL FUNDING



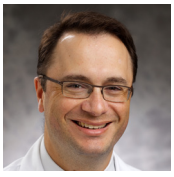
LEADERSHIP

CIBMTR MCW

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

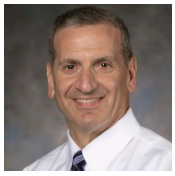
Patricia Steinert, PhD, MBA
Executive Scientific Director, Policy and Governance, CIBMTR MCW

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



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

CIBMTR NMDP



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

Mary Hengen, MBA
Vice President, CIBMTR NMDP

Stephen Spellman, MBS
Vice President, CIBMTR NMDP

Steven Devine, MD
Senior Scientific Director, CIBMTR NMDP

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.

OUR STRATEGY

CIBMTR strategy is organized into **5 pillars:**

2023 ACHIEVEMENTS

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

- Expanded data acquisition to support research in CAR-T and gene therapies (pp. 7-8)
- Distributed **>23,000** research sample aliquots for immunogenetic testing and research studies (p. 11)
- Shared **1,418** publicly available datasets (p. 17)
- Expanded variables available in the Data Back to Centers (DBtC) Download application (p. 18)
- Increased data acquired directly from the electronic health record, including extracting medication data (p. 20)
- Instituted the International System for Human Cytogenomic Nomenclature's parser to simplify CIBMTR's collection of cytogenetics data (p. 20)
- Enhanced data validation and quality in the automated Data Quality Mart, and performed **39** HCT and **13** cellular therapy audits to ensure data quality (p. 20)
- Created **17** plain-language summaries of CIBMTR research (p. 22)

OUR STRATEGY

EQUITY: *Elimination of barriers to ensure health equity*

- Ensured diverse representation in CIBMTR's Advisory and Nominating Committees

RESEARCH: *Transformational, interventional, and observational research*

- Renewed the U24 resource grant
- Provided critical evidence for the Centers for Medicare and Medicaid Services' (CMS) proposed decision to expand coverage of allogeneic HCT for Medicare patients with myelodysplastic syndromes (p. 6)
- Completed activities under the Cellular Immunotherapy Data Resource (CIDR) grant (p. 7)
- Completed enrollment to the adult cohort of the ACCESS study
- Submitted **3** new external investigator grants with CIBMTR support / collaboration
- Published **97** manuscripts and presented **96** abstracts (pp. 21-22)

INNOVATION: *Operational innovation and excellence*

- Restructured the Working Committees to promote synergies and efficiencies (p. 5)
- Identified novel ways to utilize and share CIBMTR data, including linking with the California Cancer Registry and the Patient Discharge Database (p. 10)
- Signed an agreement with the Childhood Cancer Survivor Study to merge a dataset for study and future use (p. 10)
- Enhanced data sharing among collaborative arrangements between centers, consortia or research partners, and registries
- Launched the new CIBMTR website, which received **>47,000** unique pageviews

NEXT GENERATION: *Fostering the next generation of cellular therapy research professionals*

- Launched the Working Committee Training and Leadership Program with an inaugural cohort of **8** junior faculty (p. 5)
- Added trainings for data managers, including onboarding, cellular therapy, and advanced trainings (p. 19)
- Hosted the 2023 Tandem Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) & CIBMTR with **>5,000** attendees from **>50** countries (p. 19)

CLINICAL OUTCOMES RESEARCH

Clinical outcomes research using CIBMTR's Outcomes 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.

SCIENTIFIC WORKING COMMITTEES

2023 ACCOMPLISHMENTS

112 studies

24 presentations

28 publications

- **>3,000** researchers worldwide
- **43** global experts voluntarily chair committees
- **293** new study proposals this year

CIBMTR's **12** 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.

To optimize the structure and efficiency of its Working Committees, CIBMTR implemented multiple changes this year, starting with the 2023-2024 proposal cycle:

- Merged the Health Services and the Donor Health and Safety Working Committees into the Donor and Recipient Health Services Working Committee
- Phased out the Graft Sources and Manipulation Working Committee
- Started phasing out the Cellular Immunotherapy for Cancer Working Committee

CIBMTR restricted Working Committee leadership terms to **3** years, allowing a greater number of committee members to hold chair positions. CIBMTR Scientific Directors developed and implemented several tools and templates on CIBMTR's public website to assist researchers in proposing studies.

This year CIBMTR also developed and launched the Working Committee Training and Leadership Program with **8** early career investigators. During their 2-year commitment, participants help oversee studies by serving as a liaison between study principal investigators and Working Committee leadership, ushering studies to completion with input from the Scientific Director.

STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD)

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, CIBMTR tracks and analyzes data for all allogeneic transplants performed in the US and transplants performed globally with products from the US.

Center-Specific Volumes and Survival Analysis
CIBMTR provides HRSA with the annual volume of transplants performed at each US center, most recently for transplants in 2017-2021. CIBMTR also performs a Center-Specific Survival Analysis evaluating 1-year survival rates among US centers for transplants from related and unrelated donors, most recently for first allogeneic transplants in 2019-2021.

Center Outcomes Forum
CIBMTR conducted its ninth Center Outcomes Forum this year with **~60** stakeholders providing feedback on the center-specific outcomes reporting process.

2023 ACCOMPLISHMENTS

Center-Specific Survival Analysis 2019-2021 published

Center Outcomes Forum held in October 2023

CMS CED STUDIES

Many US patients with specific diseases and / or at certain ages are denied access to cellular therapy due to lack of insurance coverage by CMS. CMS Coverage with Evidence Development (CED) studies allow CMS to provide coverage to patients enrolled in clinical studies that inform policy decisions.

CIBMTR is currently engaged in **5** CMS CED studies focused on myelodysplastic syndrome, myelofibrosis, and multiple myeloma for older adults and sickle cell disease for adolescents and adults. More than **9,000** patients received transplants with CMS reimbursement because of these studies.

In December 2023, CMS released a National Coverage Analysis Proposed Decision Memo, which proposes expanding coverage for allogeneic HCT for Medicare patients with high-risk myelodysplastic syndromes. CIBMTR's CMS CED study (10-CMS-MDS) provided critical evidence for this decision.

2023 ACCOMPLISHMENTS

5 studies

>9,000 patients

CMS Proposed Decision to expand HCT coverage

ADOPTIVE CELLULAR THERAPIES

2023 ACCOMPLISHMENTS

21,775 patients

341 centers

8 presentations

8 publications

- Completed accrual for studies of CAR-T for multiple myeloma and pediatric leukemia
- Published study of real-world data on CAR-T for lymphoma; also executed and activated data license contract

CIBMTR received data from **341** centers for **21,775** patients who received other cellular therapies; **10,926** patients were treated for lymphoma. Approximately **70%** of activity is focused on the use of CAR-T for hematologic cancers.

AcCELLerate Forum of ASTCT, CIBMTR, and NMDP
In October 2023, CIBMTR collaborated with NMDP and ASTCT to hold the AcCELLerate Forum: Creating a Sustainable Ecosystem of Cell and Gene Therapy, in a hybrid format in Arlington, VA. Four sessions covered topics regarding the regulatory landscape for new product approval, 80/20 Taskforce updates and logistics for CAR-T, emergent toxicities and their management, and future directions and competing therapies.

Long-Term Follow-Up
The Food and Drug Administration 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.

Transition from CIDR to ACT
CIBMTR operated the CIDR, a Cancer Moonshot Initiative, National Cancer Institute (NCI)-funded program from 2018 to 2023. During that time, CIDR was key to developing a robust infrastructure for cellular immunotherapies. Following the completion of the CIDR in June, all ACT activities fall within the broad structure of the Outcomes Database. CIBMTR will continue to expand and optimize the database to appropriately capture data on new cell and gene therapy products and outcomes. The organization will also continue to oversee research projects and interact with cell and gene therapy manufacturers.

GENE THERAPY

CIBMTR's Gene Therapy Working Group continues to establish how CIBMTR infrastructure can efficiently collect data for long-term follow-up of patients undergoing an autologous HCT using a genetically modified product, and CIBMTR's Corporate Office remains engaged with several biopharmaceutical companies interested in utilizing CIBMTR's Outcomes Database for long-term follow-up of gene therapy recipients.

This year, CIBMTR received data for **21** gene therapy product infusions. In addition to the previously released Gene Therapy Product form, CIBMTR developed a post-transplant Gene Therapy Persistence form and released it in October 2023. CIBMTR is currently developing a Gene Therapy PartnerShare Portal, which will provide commercial partners and centers with access to their data.

2023 ACCOMPLISHMENTS

21 gene therapy product infusions

Gene Therapy Persistence form released

Gene Therapy PartnerShare in development

PATIENT-REPORTED OUTCOMES (PRO)

CIBMTR centrally collects PRO data for addition to CIBMTR's Outcomes Database. In 2023, CIBMTR enrolled **393** patients at **27** centers and collected **1,281** surveys. In total, CIBMTR has enrolled **1,008** patients and collected **2,575** surveys.

This year CIBMTR changed processes for long-term PRO collection to make it easier to recruit participants from clinical trials. These changes will reduce the burden and cost associated with study-specific PRO surveys.

In 2023, CIBMTR also utilized PRO data to answer research questions: What does patient-reported social functioning in the first year after treatment look like, and are there associations with patient or clinical characteristics? Results demonstrated social functioning was significantly associated with Karnofsky Performance Scale score at baseline and 1 year after treatment. Researchers presented these results at **2** conferences.

2023 ACCOMPLISHMENTS

393 patients

27 centers

1,281 surveys

INTERNATIONAL INITIATIVES

2023 ACCOMPLISHMENTS

8 joint CIBMTR-EBMT studies

4,100 patients

Brazilian and Canadian registry agreements signed

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 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). In 2023, CIBMTR and EBMT jointly conducted **8** research studies, **2** of which were published. Using experience with the Center-Specific Survival Analysis, CIBMTR investigators also collaborated with EBMT to develop benchmarking standards for the EBMT registry.

CIBMTR partners with national outcomes registries in **Brazil**, **Canada**, and **Japan**. The registries use CIBMTR's infrastructure to collect data from centers in their respective countries, and CIBMTR returns those data to the national registries. In 2023, CIBMTR updated agreements with the Brazilian and Canadian registries and received data from **>1,000** patients each.

CURE SICKLE CELL SUPPORT

2023 ACCOMPLISHMENT

BMT CTN 2001 clinical trial is open to enrollment

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, 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. BMT CTN 2001, a Phase II multicenter gene transfer study opened by the initiative, continues to enroll patients nationwide.

HEALTH SERVICES RESEARCH

The Health Services Research Program, in partnership with CIBMTR Working Committees, provides scientific oversight for studies associated with the social determinants of health and how these factors impact overall survival, quality of life, and life expectancy.

Social determinants of health / health inequities / health disparities
Investigators study access, utilization, value (quality and cost), delivery, organization, and financing of care, particularly for patients from disadvantaged backgrounds and ethnically diverse patient populations. Researchers completed multiple studies this year using linked CIBMTR and California Cancer Registry data, including 1 study comparing cause of death and follow-up between the 2 datasets.

Survivorship / late effects / PRO
Survivorship is an important part of care for patients, and PRO provide an essential perspective, particularly for late effects of treatment. Researchers completed multiple studies this year using a merged database from long-term transplant survivors to assess PRO, including health-related quality of life and distress.

Treatment decision-making
Health services research is vital to treatment decision-making and care guidelines, which can lead to better quality of care and improved outcomes. Researchers are currently studying caregiver requirements and the experiences and perspectives of social workers, patients, and caregivers on those requirements. Additionally, researchers wrote a manuscript explaining survey results regarding variation in return-to-school guidelines among physicians and transplant centers.

2023 ACCOMPLISHMENTS

16 studies

9 presentations

5 publications

- Published a study comparing cause of death and follow-up between merged CIBMTR and California Cancer Registry datasets
- Characterized trends in utilization of and unmet need for allogeneic HCT for patients with acute myeloid leukemia
- Characterized pediatric physician perspectives on palliative care

IMMUNOBIOLOGY RESEARCH

RESEARCH SAMPLE REPOSITORY

(as of Nov. 1, 2023)

- **1,795,914** aliquots
- **17,592** cell lines

- **87,794** samples from unrelated donors and **14,918** from related donors
- **84,838** samples from unrelated recipients and **15,552** from related recipients

- **13,997** samples from unrelated cord blood units

CIBMTR maintains a Research Sample Repository of paired tissue samples from first allogeneic related and unrelated transplant recipient / donor or cord blood pairs. 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 NMDP's establishment of the Unrelated Donor Research Sample Repository and NIH's investment in CIBMTR's Outcomes Database to provide the community with a unique resource to assess the impact of immunogenetic determinants alone or as a variable in transplant outcomes. Linkage of clinical outcomes with high-quality immunogenetic data from the Research Sample Repository supports a variety of research studies that require genetic, immunogenetic, and clinical phenotypic data to address important questions in transplantation.

The scientific potential of the Research Sample Repository is augmented through inclusion of the HRSA-supported Related Donor Research Sample Repository. The Research Sample Repository now boasts paired samples collected from **~3,250** transplants involving haploidentical donors, allowing further exploration of donor optimization. Together, the Unrelated and Related Donor Research Sample Repositories provide an organized approach to studying transplant biology across the spectrum of allogeneic HCT.

In 2023, **182** centers (**152** transplant centers, **12** donor centers, and **18** cord blood banks) provided samples to the Research Sample Repository. CIBMTR enhanced the Research Sample Repository inventory and Immunogenetic Database this year by completing high-resolution HLA typing on **~800** related and **~1,200** unrelated donor / cord and recipient pairs, bringing the total number of donor / cord recipient pairs retrospectively typed through the program to nearly **52,000.** The program also distributed **>19,000** research sample aliquots to support research projects, including Working Committee studies, and **4,000** samples for the ongoing Donor-Recipient Pairs Project retrospective typing initiative.

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.

The Bioinformatics Research group researched and continued to develop **4** tools to guide donor selection for best patient transplant outcomes. This year **>1,000** users across **60** countries utilized these tools.

- **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.
- **The HLA-B Leader Assessment Tool (BLEAT)** sorts single HLA-B mismatches between the patient and potential unrelated and haploidentical donors to categorize donors from lowest risk to highest risk, based on research from prior studies.
- **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 haploidentical donor selection tool** projects disease-free survival for transplant patients with known haploidentical donor profiles, guiding donor selection for best patient outcomes.

The Bioinformatics Research 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.

2023 ACCOMPLISHMENTS

17 presentations

14 publications

4 tools

Goals Achieved:

- Automate generation of population haplotype frequencies
- Produce tools to complement operational decisions in donor selection for best patient outcomes
- Identify impactful genomic biomarkers

CLINICAL TRIALS SUPPORT

CIBMTR manages a wide array of studies, including multicenter trials, surveys, and correlative studies. Access to CIBMTR's Outcomes 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 / SAMPLE RESEARCH
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 PRO 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. The BMT CTN Data and Coordinating Center is managed by **3** organizations: MCW, NMDP, and the Emmes Company, a contract research organization based in Rockville, MD. Together, MCW and NMDP operate CIBMTR, a rich data source for the BMT CTN.

In 2023, the BMT CTN launched **1** trial, bringing the total to **62** launched trials, and it approved **11** ancillary studies, bringing the total to **175** ancillary studies. Participating centers accrued **>200** patients to trials this year, increasing the total to **>16,800** patients from **>100** centers since inception. In addition to **10** trials in progress, **9** new protocols are in development. The BMT CTN received **5,557** new protocol-related biospecimen aliquots this year, bringing the total available biospecimens to **524,260**.

2023 ACCOMPLISHMENTS

10 trials

17 presentations

17 publications

CIBMTR CLINICAL RESOURCE ORGANIZATION (CRO) SERVICES

CRO Services, formerly known as the Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT), provides cellular therapy researchers and organizational partners 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 it also supports Phase II/III trials and large survey and cohort studies.

In 2023, CRO Services launched **3** new studies to accrual, increasing the total number of opened studies to **39**. Investigators accrued **~4,400** participants this year, bringing the overall total to **~58,400**. In addition to **21** active studies in 2023, **2** upcoming studies are in development.

2023 ACCOMPLISHMENTS

21 studies

3 presentations

STATISTICAL METHODOLOGY RESEARCH

2023 ACCOMPLISHMENTS

4 presentations

10 publications

Research Goals:

- Develop new statistical models
- Compare new statistical models with existing solutions using CIBMTR's Outcomes Database

CIBMTR has enjoyed a positive, collaborative association since 1985 with the Division of Biostatistics in the MCW Institute for Health and Equity, 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 **12** 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, post-variable selection inference, and missing data.

COLLABORATION WITH INDUSTRY

CIBMTR offers biopharmaceutical industry partners critical resources and mutually beneficial collaborations. CIBMTR's service portfolio includes:

Real-World Data
- Retrospective data collection
- Prospective data collection
- Custom de-identified datasets and reports
- Patient-reported outcomes

Real-World Evidence
- Post-authorization studies
- Control arms
- Cross-sectional and longitudinal studies
- Landmark analyses
- Retrospective and prospective studies

Planning and Optimization
- Protocol design or review
- Statistical support
- Experimental designs

Regulatory Consulting
- Support services for managing regulatory obligations

Corporate Membership Program
Corporate Members receive updated resources that fulfill strategic, production management, and fundamental scientific objectives. Members find the best solution within **5** membership levels, each described on CIBMTR's Corporate Membership Program webpage.

Corporate Annual Meeting Support
The Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR include scientific plenary sessions, poster sessions, and comprehensive workshops. The meetings provide exhibit and marketing opportunities for corporations as well as corporate-supported satellite symposia.

2023 ACCOMPLISHMENTS

58 corporate partners

23 studies

9 satellite symposia at the annual meetings

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

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.
- **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 1-year survival for first allogeneic transplants is as expected for each specific transplant center compared to an average US transplant center.

RESEARCH STUDIES

To access CIBMTR data through research studies:

- **Propose a Working Committee 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

249 REQUESTS FULFILLED

CIBMTR's 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 2023, CIBMTR fulfilled **249** requests for information and data.

RESEARCH DATASETS

1,418 DATASETS DOWNLOADED

In accordance with the NIH Data Sharing Policy and NCI Cancer Moonshot Public Access and Data Sharing Policy, CIBMTR posts the final datasets from published studies on the Publicly Available Datasets webpage. These datasets are freely available to the public for secondary analysis. Currently there are **120** final datasets from published studies available for download. In 2023, **3,459** users accessed the webpage **5,741** times and downloaded **1,418** 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)
Visualize selected variables and download your center's TED-level HCT, CAR-T, and PRO data.

Data Back to Centers Download (DBtC Download)
Download your center's TED-level and CAR-T data, including thousands of variables.

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

Center Performance Analytics
Compare your center's data to aggregated national averages.

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

DataOps Dashboard
Download audit, continuous process improvement, center-specific survival analysis, and other reports.

Financial Payments Dashboard
Download your center's financial payment reports.

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

Center Audit Reporting (Audit Results Dashboard)
Access your center's audit results, change summaries, completion certificates, and other reports.

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

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

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

PartnerShare
Visualize data in commercial partners' own data sets.

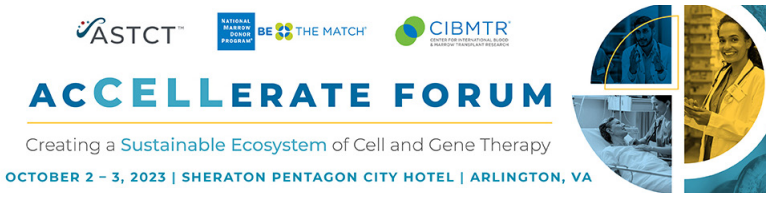
PartnerShare-StudyLink BMT CTN
Access research partners' deidentified data using criteria specific to BMT CTN studies.

MEETINGS

Annual Tandem Meetings of ASTCT and CIBMTR
Held annually in February, these meetings include **4** days of scientific sessions and other meetings targeted to worldwide physicians, scientists, and other professionals interested in cellular therapy.

- **The 2023 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR** The 2023 Tandem Meetings, held at the World Center Marriott in Orlando, FL, February 15-19, 2023, offered in-person and virtual programming. With **>5,200** attendees from **52** countries, the 2023 Tandem Meetings included **6** plenary, **9** concurrent, and **15** oral abstract sessions; **9** corporate-supported symposia; **9** Meet-the-Professor, **10** ASTCT Spotlight, and **15** CIBMTR Working Committee Sessions; and **10** product and innovation theaters.
- **The 2024 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR** The 2024 Tandem Meetings will be held at the Henry B. González Convention Center in San Antonio, TX, February 21-24, 2024, and will offer both in-person and virtual programming.

2023 AcCELLerate Forum
CIBMTR joined with the ASTCT and NMDP to host the third annual AcCELLerate Forum in October 2023. The **2**-day, hybrid workshop offered **344** attendees in the field of cell and gene therapy increased educational and advocacy opportunities. This workshop featured sessions bridging the gap among stakeholders and identifying ongoing needs and opportunities in the field for advocacy, measurement of value and impact, and sustainability.



TRAINING

CENTER SUPPORT
>14,500 tickets addressed this year

MANUALS AND GUIDES
>2,500 pages to answer your questions

ONLINE TRAININGS
45 available, **6** added/updated

DATA MANAGER ONBOARDING
>80 new data managers trained this year

DATA TRANSFORMATION INITIATIVE (DTI)

This year the DTI team continued to accelerate expansion of data capture. As CIBMTR onboards and engages transplant centers and partners with DTI, interest continues to grow; **>25** transplant centers and partners are currently onboarded with DTI.

Utilizing tools centers are already using, the DTI team added functionality this year that allows data to be extracted more efficiently. The team instituted the International System for Human Cytogenomic Nomenclature's parser to significantly simplify CIBMTR's collection of cytogenetics data. Additionally, medication and laboratory data can now be automatically extracted from the electronic health record. The DTI team also improved the CIBMTR Reporting App's filtering and standards for data exchange this year.

INTEGRATED DATA WAREHOUSE

CIBMTR's Integrated Data Warehouse consolidates data coming from multiple sources and spanning various domains, such as PRO, product data, HLA, biospecimens, and study data. This year CIBMTR's Information Technology (IT) team automated transfer of clinical trials study data collected in RAVE to the Data Warehouse. The IT team 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 CIBMTR's Outcomes Database. In 2023, CIBMTR clinical research associates audited HCT data at **39** centers and cellular therapy data at **13** centers.

CIBMTR's Clinical Data Validation team sent center-specific reports to **10** 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 **~400** potential data quality issues for centers to investigate and correct prior to their next audit.

KEY PUBLICATIONS

CIBMTR published **97 manuscripts** in scientific journals this year. Some of CIBMTR's key findings were published in the following articles.

Turcotte LM, Whitton JA, Leisenring WM, et al. **Chronic conditions, late mortality, and health status after childhood AML: A Childhood Cancer Survivor Study report.** Blood. 2023 Jan 5; 141(1):90-101. doi:10.1182/blood.2022016487. Epub 2023 Jan 5. PMC9837436.

Guru Murthy GS, Logan BR, Bo-Subait S, et al. **Association of ABO mismatch with the outcomes of allogeneic hematopoietic cell transplantation for acute leukemia.** American Journal of Hematology. 2023 Apr 1; 98(4):608-619. doi:10.1002/ajh.26834. Epub 2023 Jan 6. PMC10290878.

Eapen M, Brazauskas R, Williams DA, et al. **Secondary neoplasms after hematopoietic cell transplant for sickle cell disease.** Journal of Clinical Oncology. 2023 Apr 20; 41(12):2227-2237. doi:10.1200/JCO.22.01203. Epub 2023 Jan 9. PMC10448940.

Crivello P, Arrieta-Bolaños E, He M, et al. **Impact of the HLA immunopeptidome on survival of leukemia patients after unrelated donor transplantation.** Journal of Clinical Oncology. 2023 May 1; 41(13):2416-2427. doi:10.1200/JCO.22.01229. Epub 2023 Jan 20. PMC10150892.

Dillon LW, Gui G, Page KM, et al. **DNA sequencing to detect residual disease in adults with acute myeloid leukemia prior to hematopoietic cell transplant.** JAMA: The Journal of the American Medical Association. 2023 Jan 28; 329(9):745-755. doi:10.1001/jama.2023.1363. Epub 2023 Mar 7. PMC9993183.

Dong J, Buradagunta CS, Zhang T, et al. **Prognostic landscape of mitochondrial genome in myelodysplastic syndrome after stem-cell transplantation.** Journal of Hematology & Oncology. 16(1):21. doi:10.1186/s13045-023-01418-4. Epub 2023 Mar 10. PMC9999628.

Auletta JJ, Kou J, Chen M, et al. **Real-world data showing trends and outcomes by race and ethnicity in allogeneic hematopoietic cell transplantation: A report from the Center for International Blood and Marrow Transplant Research.** Transplantation and Cellular Therapy. 2023 Jun 1; 29(6):346.e1-346.e10. doi:10.1016/j.jtct.2023.03.007. Epub 2023 Mar 15. PMC10239334.

Devine SM, Bo-Subait S, Kuxhausen M, et al. **Clinical impact of cryopreservation of allogeneic hematopoietic cell grafts during the onset of the COVID-19 pandemic.** Blood Advances. 2023 Oct 10; 7(19):5982-5993. doi:10.1182/bloodadvances.2023009786. Epub 2023 Apr 10. PMC10580174.

Zhang T, Auer P, Dong J, et. al. **Whole-genome sequencing identifies novel predictors for hematopoietic cell transplant outcomes for patients with myelodysplastic syndrome: A CIBMTR study.** Journal of Hematology & Oncology. 2023 Apr 11; 16(1):37. doi:10.1186/s13045-023-01431-7. Epub 2023 Apr 11. PMC10088148.

Jadi O, Tang H, Olsen K, et al. **Associations of minor histocompatibility antigens with outcomes following allogeneic hematopoietic cell transplantation.** American Journal of Hematology. 2023 Jun 1; 98(6):940-950. doi:10.1002/ajh.26925. Epub 2023 Apr 13. PMC10368187.

Hill JA, Martens MJ, Young JH, et al. **SARS-CoV-2 vaccination in the first year after allogeneic hematopoietic cell transplant: A prospective, multicentre, observational study.** EClinicalMedicine. 2023 May 1; 59:101983. doi:10.1016/j.eclinm.2023.101983. Epub 2023 Apr 27. PMC10133891.

Sajulga R, Bolon YT, Maiers MJ, et al. **Assessment of HLA-DPB1 genetic variation using an HLA-DP tool and its implications in clinical transplantation.** Blood Advances. 2023 Sep 12; 7(17):4809-4821. doi:10.1182/bloodadvances.2022009554. Epub 2023 May 1. PMC10469530.

Thakar MS, Logan BR, Puck JM, et al. **Measuring the effect of newborn screening on survival after haematopoietic cell transplantation for severe combined immunodeficiency: A 36-year longitudinal study from the Primary Immune Deficiency Treatment Consortium.** Lancet. 2023 Jul 8; 402(10396):129-140. doi:10.1016/S0140-6736(23)00731-6. Epub 2023 Jun 20. PMC10386791.

Bolaños-Meade J, Hamadani M, Wu J, et al. **Post-transplantation cyclophosphamide-based graft-versus-host disease prophylaxis.** New England Journal of Medicine. 2023 Jun 22; 388(25):2338-2348. doi:10.1056/NEJMoa2215943. Epub 2023 Jun 22. PMC10575613.

Chung DJ, Shah N, Wu J, et al. **Randomized trial of a personalized dendritic cell vaccine after autologous stem cell transplant for multiple myeloma.** Clinical Cancer Research. 2023 Dec 1; 29(23):4784-4796. doi:10.1158/1078-0432.CCR-23-0235. Epub 2023 Jul 18. PMC10690096.

Augusto DG, Murdolo LD, Chatzileontiadou DSM, et al. **A common allele of HLA is associated with asymptomatic SARS-CoV-2 infection.** Nature. 2023 Aug 1; 620(7972):128-136. doi:10.1038/s41586-023-06331-x. Epub 2023 Jul 19. PMC10396966.

Logan BR, Fu D, Howard A, et al. **Validated graft-specific biomarkers identify patients at risk for chronic graft-versus-host disease and death.** The Journal of Clinical Investigation. 2023 Aug 1; 133(15):e168575. doi:10.1172/JCI168575. PMC10378149.

Versluis J, Saber W, Tsai HK, et al. **Allogeneic hematopoietic cell transplantation improves outcome in myelodysplastic syndrome across high-risk genetic subgroups: Genetic analysis of the Blood and Marrow Transplant Clinical Trials Network 1102 study.** Journal of Clinical Oncology. 2023 Oct 1; 41(28):4497-4510. doi:10.1200/JCO.23.00866. Epub 2023 Aug 22. PMC10552956.

Magenau J, Jaglowski S, Uberti J, et al. **A Phase 2 trial of CD24Fc for prevention of graft-versus-host disease.** Blood. 2024 Jan 4; 143(1):21-31. doi:10.1182/blood.2023020250. Epub 2023 Aug 30.

Petersdorf EW, McKallor C, Malkki M, et al. **HLA haplotypes and relapse after hematopoietic cell transplantation.** Journal of Clinical Oncology. doi:10.1200/JCO.23.01264. Epub 2023 Dec 5.

Zinter M, Brazauskas R, Strom J, et al. **Intensive care risk and long-term outcomes in pediatric allogeneic hematopoietic cell transplant recipients.** Blood Advances. doi:10.1182/bloodadvances.2023011002. Epub 2023 Dec 21.

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 2023, CIBMTR published **17** 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 Medical College of Wisconsin and NMDP.

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