CIBMTR®

A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

2024 ANNUAL REPORT

LETTER FROM OUR CHIEF SCIENTIFIC DIRECTORS

Dear CIBMTR Community:

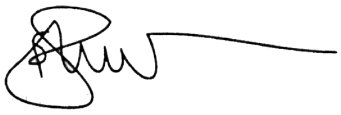
We are pleased to share the 2024 Annual Report with our cell therapy community, highlighting our accomplishments achieved through the support and involvement of all our stakeholders (pp. 3-4).

We have much to celebrate but also want to acknowledge the loss this year of our friend and colleague, Kristin Page, to a serious illness. Her impact will continue to be felt, especially through the 'CIBMTR Page Scholars' (previously Working Committee Training and Leadership) program, which recognizes and supports the importance of growing and diversifying leadership and collaborative opportunities with CIBMTR for junior investigators in our field.

In 2025, we are proud to launch our new 5-year strategic plan focusing on tangible goals to improve our speed and efficiency, to support our ecosystem, and to extend our research impact. We aim to reduce burden both externally and internally through innovation and to ensure sustainability through strong partnerships.

CIBMTR remains committed to improving outcomes through transformative and collaborative research, underpinned by our values of Integrity, Curiosity, Boldness, Accountability, Equity, and Connectivity.

We thank you for your continued partnership and look forward to an exciting and productive year together.

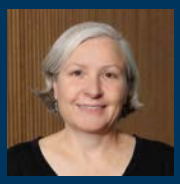


Bronwen E. Shaw, MD, PhD
Chief Scientific Director,
CIBMTR MCW

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

LEADERSHIP

CIBMTR MCW



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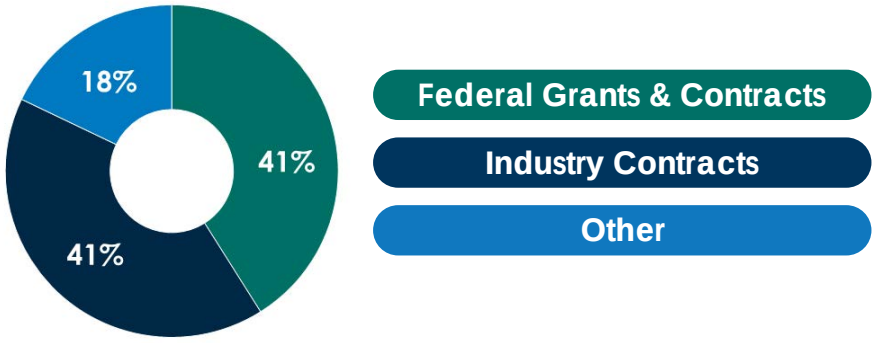
CIBMTR®
A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

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.

$58.4 MILLION ANNUAL FUNDING



MISSION

To lead transformative and collaborative research to improve outcomes in cellular therapy

VISION

To be the premier research organization for cellular therapy

VALUES

INTEGRITY CURIOSITY BOLDNESS ACCOUNTABILITY EQUITY CONNECTIVITY

STRATEGY

CIBMTR strategy is organized into **4 pillars:**

ACCELERATE PRACTICE-CHANGING RESEARCH

BUILD STRATEGIC PARTNERSHIPS

TRANSFORM DATA AND SYSTEMS

PROMOTE A HEALTHY CULTURE

2024 HIGHLIGHTS

ACCELERATE PRACTICE-CHANGING RESEARCH

Obtained 2 new grants
to support long-term follow-up data collection for patients with sickle cell disease receiving gene therapies and the Donor for All initiative

Expanded the research in adoptive cellular therapies (ACT),
including studying risk factors associated with subsequent neoplasms reported following chimeric antigen receptor T cell (CAR-T) therapy as well as modeling risk factors associated with CAR-T toxicities**,** such as cytokine release syndrome, immune effector cell-associated neurotoxicity syndrome (ICANS), and cytopenias

Received the Poster Special Recognition Award at the 2024 Annual Conference of the Society of Clinical Research Associates
for "ACCESS Clinical Trial Eliminates Disparity in Identifying a Donor for All Patients in Need of Stem Cell Transplantation"

Provided critical evidence for the Centers for Medicare and Medicaid Services' (CMS') National Coverage Determination,
expanding allogeneic hematopoietic cell transplantation (HCT) for Medicare patients with myelodysplastic syndromes

Published 86 manuscripts and presented 106 abstracts

BUILD STRATEGIC PARTNERSHIPS

Developed and enhanced tools and calculators to support clinical decision-making at centers,
for example, built a Haploidentical Donor Selection Tool, which projects disease-free survival to guide haploidentical donor selection

Supported external investigators in achieving and delivering grants (1 awarded, 4 submitted, 11 ongoing)

Hosted the 2024 Tandem Meetings I Transplantation & Cellular Therapy Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) & CIBMTR
with 5,000+ attendees from 50+ countries

Collaborated with international centers
to align their reporting obligations with their goals and capabilities

TRANSFORM DATA AND SYSTEMS

Increased data acquired directly from the electronic health record,
including medication (conditioning regimen), height, and weight data

Incorporated 10,000+ data variables into the Integrated Data Warehouse

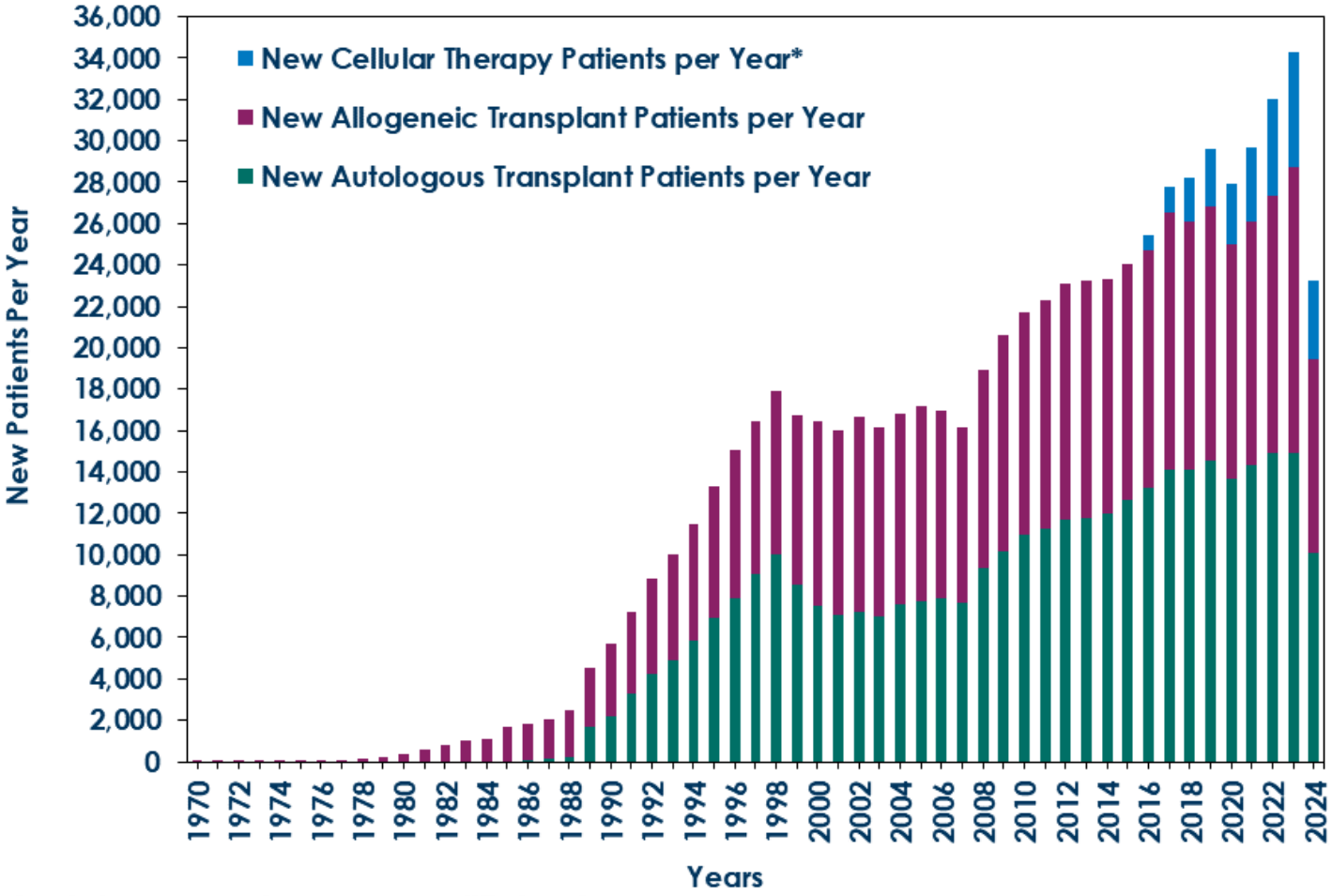
Enhanced new public website, and received Standard of Excellence award from the Web Marketing Association

Added new patient-reported outcomes (PRO) charts, gene therapy data, and transplant essential data (TED)-level variables
to Data Back to Centers (DBtC) and other applications

RESOURCES

DATA

NUMBER OF PATIENTS REGISTERED, 1970-2024



*Includes CAR-T and genetically modified products

(Data are incomplete for 2024)

30,000+
NEW PATIENTS ANNUALLY

Added in 2024:

10,092
New Autologous Transplant Patients**

9,386
New Allogeneic Transplant Patients**

3,840
New Adoptive Cell Therapy Patients**

***Data accurate from 1/1/2024-10/1/2024

CUSTOM ANALYSES

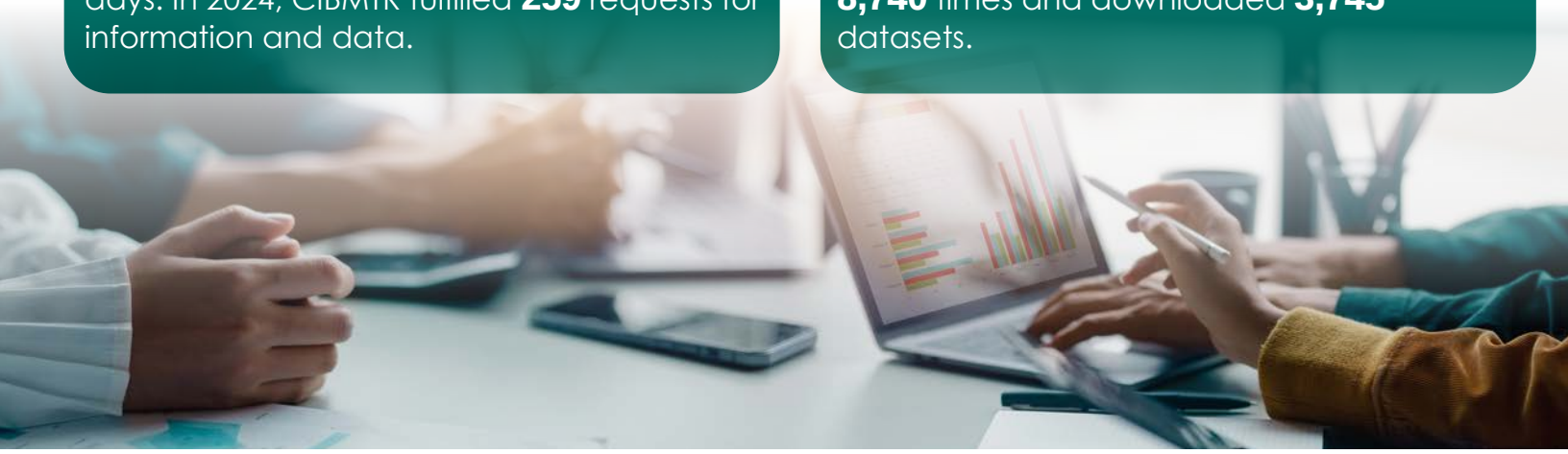
259 REQUESTS FULFILLED

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 2024, CIBMTR fulfilled **259** requests for information and data.

RESEARCH DATASETS

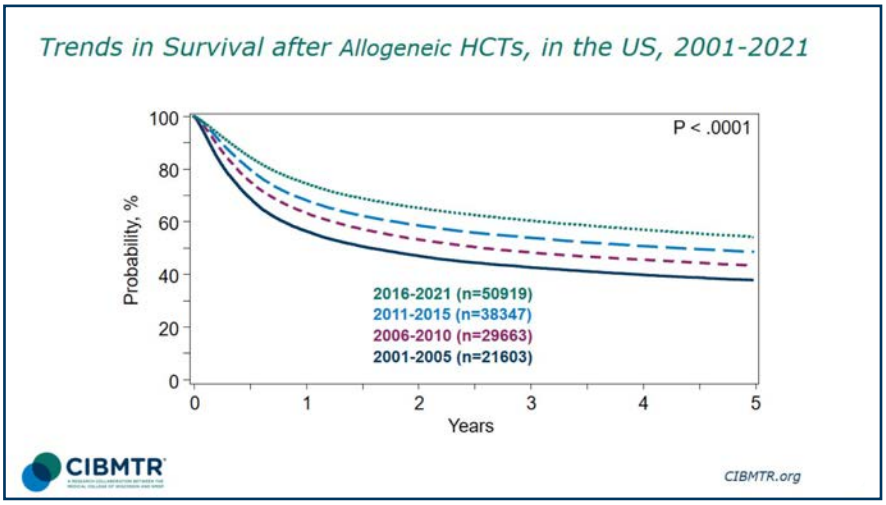
3,745 DATASETS DOWNLOADED

In accordance with the NIH Data Sharing Policy and National Cancer Institute Cancer Moonshot Public Access and Data Sharing Policy, CIBMTR posts the final datasets from published studies on the Publicly Available Datasets webpage within **7** weeks of publication. These datasets are freely available to the public for secondary analysis. Currently there are **128** final datasets from published studies available for download. In 2024, **4,900** users accessed the webpage **8,740** times and downloaded **3,745** datasets.



SUMMARY SLIDES

View charts and figures summarizing use and outcomes of HCT, CAR-T, and PRO. Data include frequency of transplants, overall survival outcomes, and PRO data. Variables include transplant and CAR-T infusion type, donor type, patient age, disease, disease status, graft source, graft-versus-host disease (GVHD) prophylaxis, conditioning regimen, and race and ethnicity.



CENTERS

310+ participating centers

- **198** centers share adoptive cellular therapy (ACT) data
- **170** centers share biospecimens
  - **152** transplant centers
  - **15** cord blood banks
  - **3** donor centers
- **47** centers share PRO data

PATIENTS

700,000+ HCT and ACT patients since inception

- **27,488** ACT patients
- **57** gene therapy patients
- **75,000+** patients participated in clinical trials and studies
- **1,440** patients shared PRO

BIOREPOSITORY

3,110,362 research sample aliquots from 17,846 cell lines

- **91,636** samples from unrelated donors and **15,927** from related donors
- **90,397** samples from unrelated recipients and **16,629** from related recipients
- **14,268** samples from unrelated cord blood units
- **30,000+** samples distributed to research projects

STUDIES

185+ ongoing studies and clinical trials

- **111** Working Committee studies
- **37** Industry studies
- **22** CRO Services studies
- **9** BMT CTN clinical trials
- **5** CMS CED studies
- **4** Implementation Science studies

PUBLICATIONS

1,900+ publications since inception

86 publications in 2024

- **6** published in *Nature Medicine* and *Journal of Clinical Oncology*
- **19** published in journals with impact factor 10+

PRESENTATIONS

106 presentations at national and international conferences in 2024 (**55 oral**, **50 poster**, and **1 publication**)

- **31** abstracts (**12** oral and **19** poster) at the Tandem Meetings
- **26** abstracts (**14** oral and **12** poster) at the American Society of Hematology Annual Meeting

ACCELERATE PRACTICE-CHANGING
RESEARCH

Expedite practice change through dissemination of high-impact research

OBSERVATIONAL RESEARCH

CIBMTR continues to manage a large, observational research portfolio with significant activity in its scientific working committees, strategic and grant-funded studies, and industry relations program. In 2024, CIBMTR published a number of high-impact and practice-changing studies based on the registry data. A few important examples include:

- Published in *JAMA Oncology*, a working committee study explored the important question of the impact of minimum residual disease prior to transplant on outcomes in acute myeloid leukemia (Dillon, *JAMA Oncol*, 2024, PMC11066770). This study already has **2,000+** views and **7** citations.
- A large study showed that, in the era of post-transplant cyclophosphamide, patient outcomes using a 7/8 human leukocyte antigen (HLA) matched donor were the same as outcomes using an 8/8 HLA-matched donor (Shaffer, *J Clin Oncol*, 2024, PMC11421565). This encouraging finding supports the expansion of donor sources, making transplant safer for many more patients. This paper has been downloaded **8,000+** times and has an Altmetric score of **206**, putting it in the top **5%** of all papers ever tracked by Altmetric.
- CIBMTR data also confirmed that the addition of abatacept to standard GVHD prophylaxis resulted in improved outcomes, both in the matched and mismatched HLA donor setting (Kean, *Blood*, 2024, PMC11530361).

CIBMTR PAGE SCHOLARS: WORKING COMMITTEE TRAINING AND LEADERSHIP PROGRAM

CIBMTR launched a new training and leadership program in June 2023 that engages early career investigators. This year, leaders renamed the program CIBMTR Page Scholars, in honor of one of the program's leaders, Kristin Page, MD, who passed away in September. The first cohort of **8** Page Scholars participants are in their second year of the program, overseeing studies, acting as liaisons between study principal investigators and working committee leadership, and helping to shepherd studies to completion with input from Scientific Directors.

Kristin Page, MD

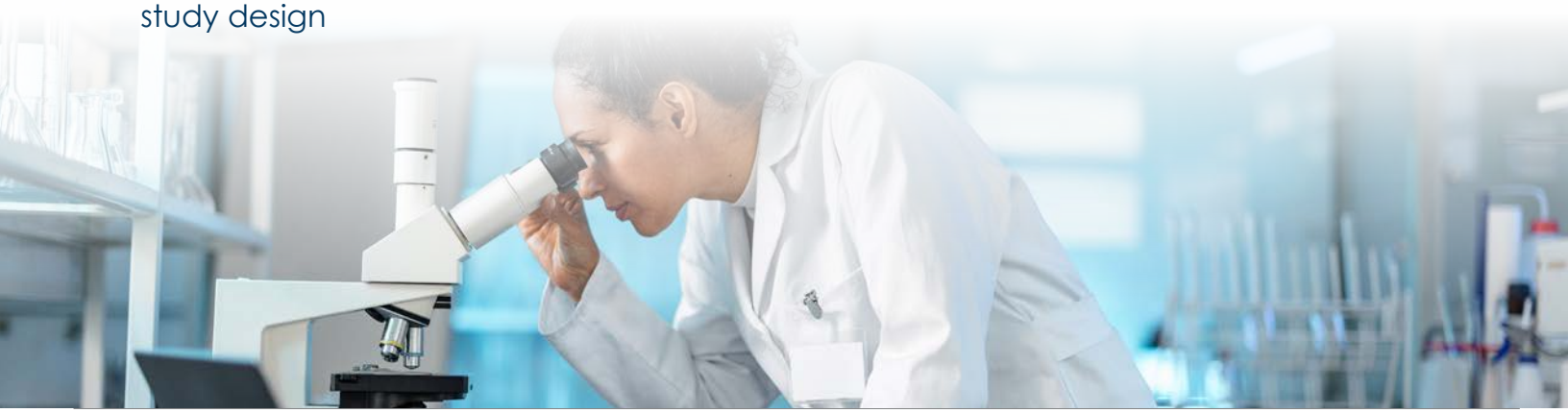
PROSPECTIVE CLINICAL TRIALS

CIBMTR CRO Services provides cellular therapy researchers and organizational partners with infrastructure and expertise in clinical trial design, conduct, and analysis. In 2024, the CRO continued to work on numerous projects, including (highlighted below) the Donor for All initiative.

DONOR FOR ALL

CIBMTR is leading a comprehensive prospective clinical trial strategy to expand access to all patients in need of a transplant through improving outcomes for patients who underwent transplant with a mismatched unrelated donor. Significant achievements in 2024 include:

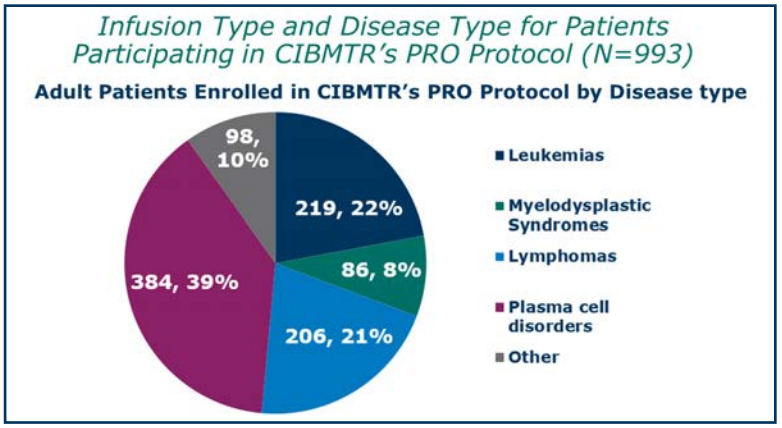
- Presented and published clinical results from the initial cohorts of the ACCESS study, showing excellent outcomes in both the initial reduced-intensity and myeloablative conditioning adult patient cohorts. The study also found that:
  - **50%+** patients enrolled are from a racially / ethnically diverse background
  - Most patients had a genotype frequency that is rare (i.e., unlikely to find a matched donor in the registry)
- Launched the OPTIMIZE study of reduced dose post-transplant cyclophosphamide to improve infection-free survival, and enrolled **114** patients at **17** clinical sites
- Received US Food and Drug Administration approval for the ACCELERATE platform study design



PATIENT-CENTERED OUTCOMES

CIBMTR centrally collects PRO data for addition to CIBMTR's Outcomes Database. In 2024, CIBMTR enrolled **370** patients from **47** sites, bringing the total number of patients enrolled to **1,440**. The PRO team reported on the current state of PRO data collection through the CIBMTR central electronic system in a peer-reviewed manuscript and published summary slides on CIBMTR's website.

Infusion Type and Disease Type for Patients Participating in CIBMTR's PRO Protocol (N=993)



CIBMTR researchers also conducted a survey and follow-up qualitative interview to understand how patients, caregivers, and care team members understand graphic visualizations of PRO data and how they prefer to receive and see these data. Other researchers determined that a single, health-related quality-of-life question asked of patients before transplant was significantly predictive of patient survival one year after transplant.

IMPLEMENTATION SCIENCE

CIBMTR implementation science systematically closes the gap between what we know and what we do by:

- **Disseminating Research.** Sharing research findings with specific audiences to improve awareness of the results.
- **Understanding Current Practice.** Conducting studies and analyses to understand the current state of evidence-based practices and potential barriers to adoption.
- **Supporting Implementation.** Designing and deploying implementation strategies to enhance the uptake of evidence-based practices.

This year, the implementation science team:

- Disseminated tailored materials for **15** studies
- Conducted mixed methods research on donor and cord blood selection practices
- Designed and evaluated multidisciplinary workshops on reimagining caregiving
- Piloted implementation interventions, including survivorship care plans and a health equity toolkit

BIOINFORMATICS

CIBMTR's bioinformatics research specializes in matching patients and cellular therapies, saving lives by researching what, where, and how to match patients and cellular therapies. 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
- Donor registry modeling

This year, bioinformatics researchers:

- Developed annotation pipelines for processing whole genome sequencing into annotation of genes and identification of structural variants
- Developed the QLASSy tool described below

TOOLS

Class I HLA Peptide Binding Motif (PBM) Tool

Map Class I HLA typing to PBM groups and calculate the direction of the group match or mismatch

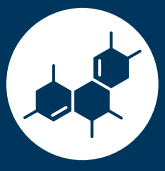
QLASSy: HLA-DQ Heterodimers Tool

Optimize transplant matches by considering HLA-DQ1 to lower disease relapse risk, based on HLA-DQ heterodimer research

HLA-B Leader Assessment Tool (BLEAT)

Sort 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

Haploidentical Donor Selection Tool



Project disease-free survival for transplant patients, guiding donor selection for best patient outcomes

IMMUNOBIOLOGY

CIBMTR maintains a Research Sample Repository of paired tissue samples from first allogeneic related and unrelated transplant recipient / donor or cord blood pairs. The Research Sample Repository inventory and CIBMTR's immunogenetic testing programs add critical HLA, killer cell immunoglobulin-like receptors (KIR), and other data for use in CIBMTR studies.

In 2024, investigators published **5** studies resulting from analysis of CIBMTR Research Repository samples, **2** studies using CIBMTR sample-derived data, and **3** other Immunobiology Working Committee manuscripts. Investigators researched:

- Transplant outcomes for patients with severe aplastic anemia and specific gene variants
- Risk of relapse and death for patients with acute myeloid leukemia and specific mutations
- Transplant outcomes for patients with myelodysplastic syndromes based on donor variants, which could help physicians choose the best donors
- Risk of relapse for patients with myelodysplastic neoplasms by analyzing proteomics

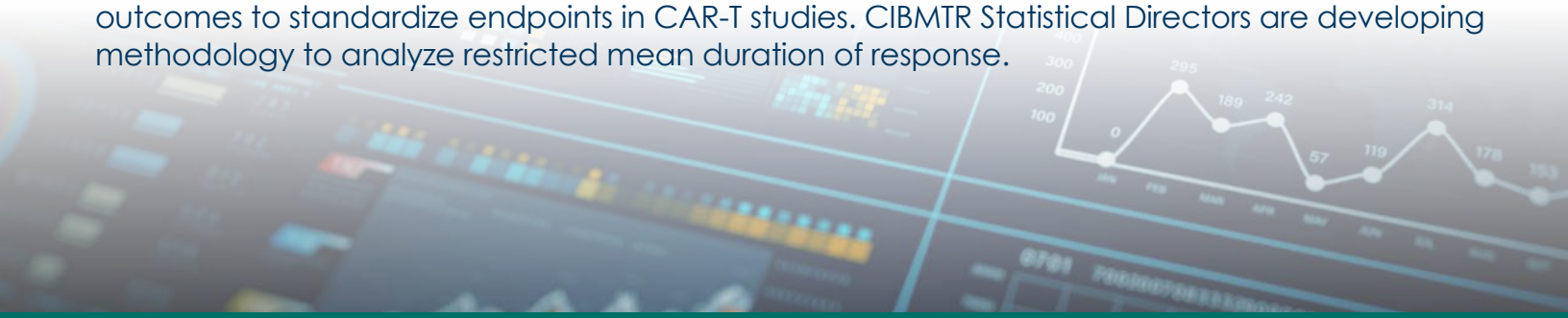
STATISTICAL METHODOLOGY

Transplantation is a complex process with multiple competing risks and dramatic changes in the risks of specific events over time. CIBMTR biostatisticians develop and evaluate the statistical models used in cellular therapy research and help guide the research community in appropriate application and interpretation of these sophisticated models.

In 2024, biostatisticians published **4** manuscripts focused on:

- Machine learning
- Right-censored data analysis
- Case-cohort study design

CIBMTR Statistical and Scientific Directors continue to discuss definitions of CAR-T therapy outcomes to standardize endpoints in CAR-T studies. CIBMTR Statistical Directors are developing methodology to analyze restricted mean duration of response.



BUILD STRATEGIC

PARTNERSHIPS

Leverage our strengths to ensure sustainability of CIBMTR

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. 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 2018-2022. 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 2020-2022.

CMS COVERAGE WITH EVIDENCE DEVELOPMENT (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 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, and **10,000** patients received transplants with CMS reimbursement because of these studies.

On March 6, 2024, CMS released its National Coverage Determination for allogeneic HCT for patients with myelodysplastic syndromes. The CED study CIBMTR performed generated important evidence supporting CMS' decision, which represents a substantial improvement in payor policy and an important increase in access to therapy for Medicare patients.

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK (BMT CTN)

The BMT CTN, sponsored by the National Heart, Lung, and Blood Institute and National Cancer Institute, is the US network charged with developing and conducting multicenter phase 2 and 3 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 2024, the BMT CTN:

- Enrolled patients to **7** open trials, and planned **7** new protocols in development
- Released **2** new trials to participating centers, bringing the total to **65** launched trials
- Approved **11** ancillary studies, bringing the total to **186** ancillary studies
- Accrued **240+** patients to trials, increasing the total to **17,100+** patients from **100+** centers since inception
- Received **3,390** new protocol-related biospecimen aliquots this year, bringing the total available biospecimens to **515,033**

65
clinical trials

17,100+
patients

515,000+
biospecimens

GENE THERAPY FOR SICKLE CELL AND OTHER DISEASES

CIBMTR collaborated with various stakeholders, including an expert task force as well as industry and federal partners, to identify key clinical data elements that will support observational gene therapy research and long-term follow-up studies to meet regulatory requirements. In September, CIBMTR was awarded additional NIH funding for 2 sickle cell initiatives. One, under the Cure Sickle Cell Initiative, supports planning for a long-term follow-up program for patients with sickle cell disease who are treated with either HCT or gene therapy on NIH-funded clinical trials. The other, funded by a supplement to CIBMTR's NIH U24 resource grant, coordinates with the Center for Medicare and Medicaid Innovation (CMMI) to share CIBMTR clinical and PRO outcomes data to support a CMS Cell and Gene Therapy Access Model for Medicaid-eligible patients who receive gene therapy for sickle cell disease. Also this year, CIBMTR updated the thalassemic, adrenoleukodystrophy, and sickle cell disease-specific forms in line with commercial approval of gene therapies for these indications, and the organization launched new forms to collect transfusions and bone marrow evaluations following gene therapy.

ANNUAL TANDEM MEETINGS OF ASTCT AND CIBMTR

Held annually in February, the Tandem Meetings I Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR (Tandem Meetings) include 4 days of scientific sessions and other meetings.

2024 TANDEM MEETINGS

The 2024 Tandem Meetings, held at the Henry B. González Convention Center in San Antonio, TX, February 21-24, 2024, offered in-person and virtual programming. With **5,300+** attendees from **58** countries, the 2024 Tandem Meetings included **6** plenary, **9** concurrent, and **12** oral abstract sessions; **7** corporate-supported symposia, **8** Meet-the-Professor, **8** ASTCT Spotlight, and **12** CIBMTR Working Committee Sessions; and **10** product and innovation theaters.

TANDEM MEETINGS
Transplantation & Cellular Therapy Meetings
of ASTCT® and CIBMTR®

2025 TANDEM MEETINGS

The 2025 Tandem Meetings will be held at the Hawai'i Convention Center, in Honolulu, HI, February 12-15, 2025, and will offer both in-person programming and a digital access experience.

INTERNATIONAL INITIATIVES

CIBMTR is committed to not only including high-quality international data in its research studies but also to supporting centers and regions in their own data quality and regulatory initiatives.

Individual Centers. This year, CIBMTR surveyed all participating international centers to evaluate if their reporting commitments align with their reasons for sharing data. If applicable, CIBMTR collaborated with centers to reduce or remove reporting obligations to better align with center capabilities. Throughout 2024, CIBMTR worked with international centers to improve the timeliness and overall completeness of their reporting to meet continuous process improvement requirements implemented in July 2023.

National / Regional Support. 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 2024, CIBMTR and EBMT jointly conducted 7 research studies, 1 of which was published. CIBMTR also 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 2024, CIBMTR received data for **5,000+** international patients.

INDUSTRY COLLABORATION

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

REAL-WORLD DATA

- 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 access to the most current and comprehensive data on blood and bone marrow transplantation and other cellular therapies as well as an opportunity to participate in CIBMTR's annual meetings. Choose membership benefits from the 4 membership levels described at cibmtr.org/CorporateMembership.

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.

TRANSFORM
DATA AND SYSTEMS

Make usable data available faster through innovation

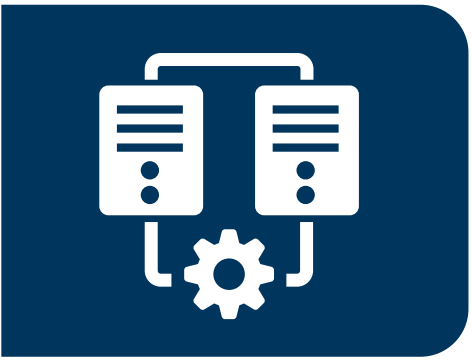
Data are one of the 4 pillars underpinning CIBMTR's strategy, with the overall goal to make enhancements over the entire data life cycle, from acquisition through data sharing, to increase the speed of data use, reduce burden at sites, and increase the value of CIBMTR as a resource to the community.

ACQUISITION STANDARDS AND MECHANISMS

CIBMTR is committed to optimizing the acquisition and utilization of high-quality data to accelerate research through expanding data collection using more automated methods, which eliminate manual data capture, and engaging more centers to partner with CIBMTR to implement these methods. By the end of 2024, **34** partner sites implemented technology introduced by CIBMTR. Important improvements this year include:

Data Collection using FormsNet

In 2024, CIBMTR introduced FormsNet functionality to prevent the need for diagnosis information to be collected again for repeat infusions for the same diagnosis, and the organization introduced a new process for reporting subsequent infusions so that infusion information is collected on the same form for all infusion types. Finally this year, CIBMTR introduced a tool for lost to follow-up status at the recipient level to reduce center reporting burden.



Automated Data Exchange using Fast Healthcare Interoperability Resources (FHIR) Standards

In 2024, CIBMTR expanded its capabilities for electronic data submission by integrating new functionality into its SMART on FHIR app, enabling centers using Epic electronic health records to electronically submit medication-related data. Leveraging HL7 FHIR standards, this automated process allows data to be seamlessly selected and processed into CIBMTR's Outcomes Database, facilitating the capture of pre-transplant planned therapy data. Additionally this year, CIBMTR introduced functionality for transferring height and weight data from electronic health records into CIBMTR's FHIR server.

DATA INTEGRATION, STORAGE, AND MANAGEMENT

CIBMTR achieved significant progress integrating transplant data into the Integrated Data Warehouse by successfully incorporating **10,000+** data variables, including **100%** of critical data in TED forms and **50%+** of commonly used data in the comprehensive report forms, representing the most common diseases treated with transplant. These additions greatly simplify and speed up dataset preparation. CIBMTR's IT team also mapped **3,000+** new variables collected for current indications of gene therapy, incorporating CIBMTR's newest therapeutic modality into the Integrated Data Warehouse.

DATA ACCESS, EXTRACTION, AND SHARING

CIBMTR continues to enhance data accessibility in ways that support the core principles of simplicity and flexibility and expand the footprint of availability and usefulness of the data.

DBtC and Other Applications

In 2024, CIBMTR updated branding and readability, included new PRO charts and gene therapy data, and added new TED-level variables.

PartnerShare

This year, CIBMTR enhanced PartnerShare to include deidentified gene therapy data and added new partners for CAR-T products.

Public Website

In 2024, CIBMTR improved the layout and search features for publicly available datasets and introduced new features for downloading and browsing data dictionaries.

Data Standards

This year, CIBMTR established a strategic approach to metadata and terminology services that moves to more open-source technology frameworks.

DATA SHARING TOOLS

Data Back to Centers
Visualize and download your center's TED-level and CAR-T data, including thousands of variables.

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

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

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

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, and access research partners' deidentified data using clinical study-specific criteria.

CALCULATORS

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

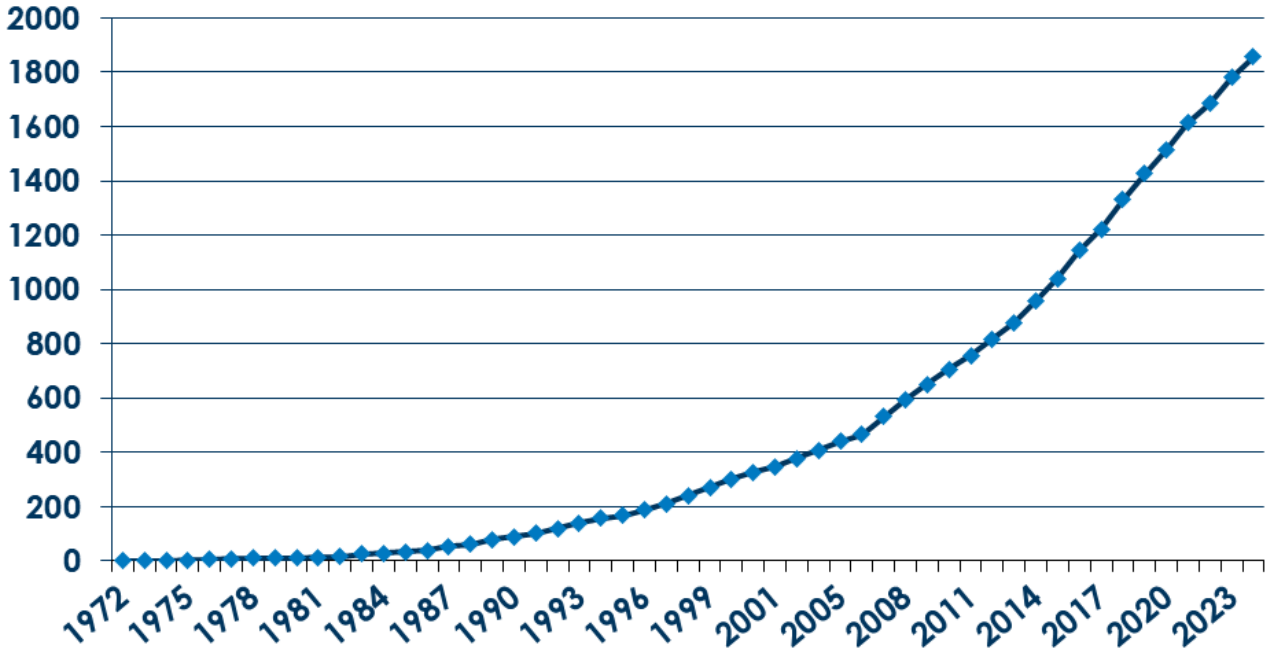
1-Year Survival Calculator

Predict 1-year survival for individual allogeneic HCT recipients

KEY PUBLICATIONS

1,900+

cumulative publications, 1972-2024



*CIBMTR published **86** manuscripts in scientific journals this year.*
Some of CIBMTR's key findings were published in the following articles.

- Levine BL, Pasquini MC, Connolly JE, et al. **Unanswered questions following reports of secondary malignancies after CAR-T cell therapy.** *Nature Medicine*. 2024 Feb 1; 30(2):338-341. doi:10.1038/s41591-023-02767-w. Epub 2024 Jan 10. PMC11688691.

- Levis MJ, Hamadani M, Logan B, et al. **Gilteritinib as post-transplant maintenance for AML with internal tandem duplication mutation of FLT3.** *Journal of Clinical Oncology*. 2024 May 20; 42(15):1766-1775. doi:10.1200/JCO.23.02474. Epub 2024 Mar 12. PMC11095884.

- Pasquini M, Wallace PK, Logan B, et al. **Minimal residual disease status in multiple myeloma 1 year after autologous hematopoietic cell transplantation and lenalidomide maintenance are associated with long-term overall survival.** *Journal of Clinical Oncology*. 2024 Aug 10; 42(23):2757-2768. doi:10.1200/JCO.23.00934. Epub 2024 May 3. PMC11634105.

- Shaffer BC, Gooptu M, DeFor TE, et al. **Post-transplant cyclophosphamide-based graft-versus-host disease prophylaxis attenuates disparity in outcomes between use of matched or mismatched unrelated donors.** *Journal of Clinical Oncology*. 2024 Oct 1; 42(28):3277-3286. doi:10.1200/JCO.24.00184. Epub 2024 Jul 17. PMC11421565.

KEY PUBLICATIONS

- Dong J, Arsang-Jang S, Zhang T, et al. **Prognostic impact of donor mitochondrial genomic variants in myelodysplastic neoplasms after stem-cell transplantation.** *Journal of Hematology & Oncology*. 2024 Nov 4; 17(1):104. doi:10.1186/s13045-024-01622-w. Epub 2024 Nov 4. PMC11533675.

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- Shadman M, Ahn KW, Kaur M, et al. **Autologous transplant vs. CAR-T therapy in patients with DLBCL treated while in complete remission.** *Blood Cancer Journal*. 2024 Jul 8; 14(1):108. doi:10.1038/s41408-024-01084-w. Epub 2024 Jul 8. PMC11231252.

- Wang TP, Ahn KW, Shadman M, et al. **Chimeric antigen receptor T-cell infusion for large B-cell lymphoma in complete remission: A Center for International Blood and Marrow Transplant Research analysis.** *Leukemia*. 2024 Jul 1; 38(7):1564-1569. doi:10.1038/s41375-024-02242-6. Epub 2024 May 15. PMC11271761.

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- Guru Murthy GS, Zhang T, Bolon YT, et al. **Proteomics to predict relapse in patients with myelodysplastic neoplasms undergoing allogeneic hematopoietic cell transplantation.** *Biomarker Research.* 2024 Jan 25; 12(1):10. doi:10.1186/s40364-023-00550-0. Epub 2024 Jan 25. PMC10809608.

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- Cusatis R, Litovich C, Feng Z, et al. **Current trends and outcomes in cellular therapy activity in the United States, including prospective patient-reported outcomes data collection in the Center for International Blood and Marrow Transplant Research registry.** *Transplantation and Cellular Therapy.* 2024 Sep 1; 30(9):917.e1-917.e12. doi:10.1016/j.jtct.2024.06.021. Epub 2024 Jun 27. PMC11587342.

RESEARCH SUMMARIES FOR PATIENTS

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

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