

CIBMTR[®]

CENTER FOR INTERNATIONAL BLOOD
& MARROW TRANSPLANT RESEARCH

>330
CENTERS

>550,000
PATIENTS

>1,500
PUBLICATIONS

>30
COUNTRIES

>240
CURRENT
STUDIES

>185,000
SAMPLES

FACTS AND FIGURES

July 2019 - June 2020

SHARING KNOWLEDGE. SHARING HOPE.

Who We Are

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



Medical and Scientific
EXPERTISE



Worldwide **NETWORK**



Clinical **DATABASE**

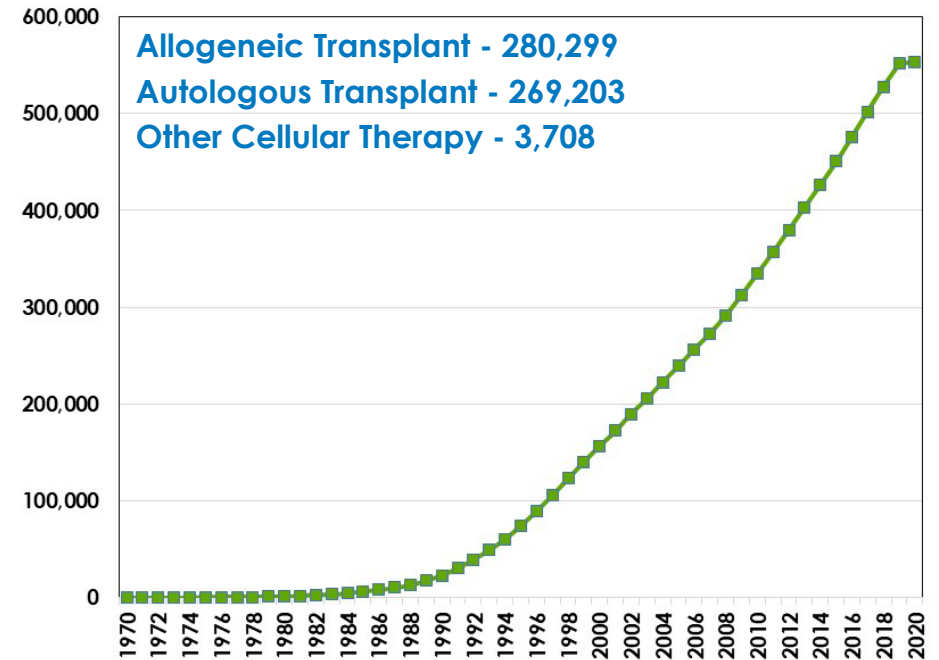


Biospecimen **REPOSITORY**

MISSION

The CIBMTR promotes collaborative research to understand and improve access to and outcomes of cellular therapies for the people we serve.

NUMBER OF PATIENTS REGISTERED WITH THE CIBMTR, 1970-2020



50 Years



>550,000 Patients



Learn about the CIBMTR's response to the pandemic and view the latest COVID-19 data and publications at cibmtr.org/covid19



SCIENTIFIC WORKING COMMITTEES

Answering clinically important questions using the CIBMTR's unique and extensive Research Database

- **15 Working Committees**, each focused on a specific disease or condition, use of specific cell types, or complication of treatment
- **>2,800 worldwide researchers** participate on the committees
- **45 global experts** in the field chair the committees
- **>170 studies** in progress
- **40 abstracts** (22 oral and 18 poster) presented at national and international conferences this year



570 Scientific Authors



300 Institutions



53 Publications this year

PATIENT-REPORTED OUTCOMES (PRO)

Studying long-term survivorship and characterizing the patient experience

- **ePRO system:** Used for routine PRO collection and clinical trials support; incorporates a user-friendly interface in Qualtrics, automated tracking and alerting functionality, and PROMIS measures via computer logic to focus on questions relevant to each individual patient
- **Routine collection:** Core PRO survey instrument developed for routine data collection from adult transplant and CAR-T patients

STEM CELL THERAPEUTIC OUTCOMES DATABASE (SCTOD)

Tracking and analyzing data for all allogeneic transplants performed in the United States (US) and transplants performed globally with products from the US

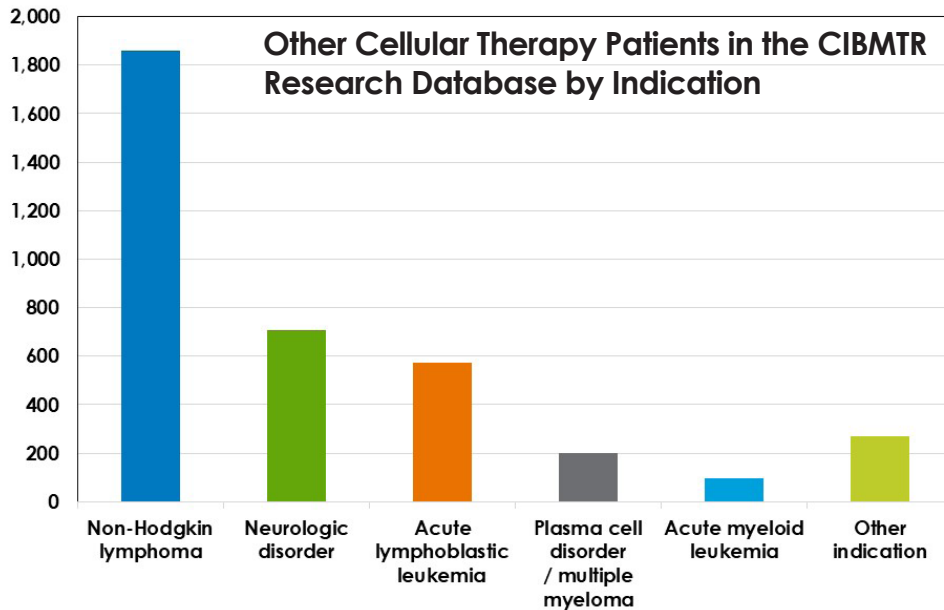
- **Transplant center volumes data:** Includes transplants performed 2013-2017
- **Center-specific survival analysis:** Includes first allogeneic transplants performed 2015-2017
- **18 research summaries:** Plain language summaries of research publications created for patients and their loved ones



OTHER CELLULAR THERAPIES

Capturing the nature, sequence, and effects of modern cellular therapies

- **>3,700 patients** at >160 centers
- **Cellular Therapy Registry Forum:** Held annually, the meeting brings together experts to discuss real-world experience
- **Long-term follow-up for CAR-T patients:** The CIBMTR partners with pharmaceutical companies to track long-term outcome data for Food and Drug Administration (FDA) 15-year follow-up requirements
- **Cellular Immunotherapy Data Resource (CIDR):** The CIDR provides the framework for collection of outcomes data to support observational studies and clinical trials through innovation and collaboration



MEDICARE CLINICAL TRIALS AND STUDIES

Conducting national clinical studies that allow providers to offer coverage and patients to receive treatment

- Many patients with specific diseases and/or at certain ages are denied access to cellular therapy in the US due to lack of insurance coverage by the Centers for Medicare and Medicaid Services (Medicare)
- Medicare Coverage with Evidence Development (CED) studies allow Medicare to provide coverage to patients enrolled on clinical studies that inform policy decisions
- **5 Medicare CED studies** in progress



For elderly patients:

Myelodysplastic syndrome
 Myelofibrosis
 Multiple myeloma



For adolescents and young adults:

2 for sickle cell disease



Health Services Research

Exploring how socioeconomic factors, health behaviors, financial systems, and health care processes affect access to and outcomes of cellular therapies

- Quantitative and qualitative research methods identify and address barriers to treatment, improve practice, and demonstrate the value of treatment and survivorship care
- **13 studies** in progress
- **5 abstracts** (4 oral and 1 poster) presented at national and international conferences this year
- **7 manuscripts** published in peer-reviewed journals this year



Access to Treatment
Value and Health Economics
Health Disparities



Survivorship
Late Effects
Patient-Reported Outcomes



Treatment Decision Making



Immunobiology Research

Managing a repository of paired tissue samples from donors and recipients, both unrelated and related, to study the genetic, cellular, and immunologic factors that influence cellular therapy outcomes

- Research Repository inventory and immunogenetic testing programs add critical HLA and KIR data for use in clinical outcomes studies
- **>10,500 samples** received this year
- **>15,700 samples** distributed to investigators for research this year

186,569 Samples in Inventory

Multiple aliquots available for each sample



76,976 from unrelated donors
11,095 from related donors



74,104 from unrelated recipients
11,508 from related recipients

12,884 from unrelated cord blood units



Bioinformatics Research

Interpreting molecular data, building prediction models from integrated data sources, characterizing diverse genetic population needs, driving new technology applications, and translating research findings into practice to save lives

- **4 new tools / pipelines** developed this year
- **3 services / methods** transitioned into practice this year
- **12 studies** in progress
- **5 abstracts** (3 oral and 2 poster) presented at national and international conferences this year
- **4 manuscripts** published in peer-reviewed journals this year



Genomics / omics and high-throughput bioanalytics



Machine learning and clinical predictions



Cellular therapy matching and donor registry modeling



Statistical Methodology Research

Developing statistical models to use in cellular therapy research and comparing new models to existing solutions using the CIBMTR Research Database

- Guide the research community in appropriate application and interpretation of sophisticated statistical models
- Develop and evaluate statistical models addressing the complex processes of cellular therapy, including multiple competing risks and dramatic changes in the risks of specific events over time
- **1 oral abstract** presented at an international conference this year



Ensure statistical integrity of CIBMTR scientific activities



Collaboratively publish statistical articles for clinical audiences



Support investigators in developing studies using CIBMTR data



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

Providing researchers with infrastructure and expertise in clinical trial conduct and analysis

- Manage FDA investigational new drug protocols, allowing US centers to access peripheral blood and uncultured cord blood for transplantation
- Provide full-scale and a la carte research operations for multi-center studies
- Assist researchers in developing and conducting research involving questionnaires and patient interviews
- **2 poster abstracts** (1 oral and 1 poster) presented at an international conference this year



24 Studies in progress



3,367 Patients accrued this year



1 Publication this year

Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

Conducting well-designed, multi-institutional Phase II and III trials available to patients in all regions of the US

- Manage the BMT CTN Data Coordinating Center in collaboration with NMDP/Be The Match and The Emmes Company
- **>100 centers** in network
- **>410,000 biospecimens** in the Research Sample Repository
- **49 ancillary and correlative studies** in progress in addition to primary trials
- **16 abstracts** (7 oral and 9 poster) presented at national and international conferences this year



13 Trials in progress



>300 Patients accrued this year



15 Publications this year

Key Publications

The CIBMTR published **89 peer-reviewed manuscripts** in scientific journals this year. Some of the CIBMTR's key findings were published in the following articles.

Ghosh N, Ahmed S, Ahn KW, et al. **Association of reduced-intensity conditioning regimens with overall survival among patients with NHL undergoing allogeneic transplant.** JAMA Oncology. Epub 2020 Jun 4. PMC7273311. [Epub ahead of print].

Petersdorf EW, Bengtsson M, De Santis D, et al. **Role of HLA-DP expression in GVHD after unrelated donor transplantation.** Journal of Clinical Oncology. Epub 2020 Jun 1. [Epub ahead of print].

Petersdorf EW, Stevenson P, Bengtsson M, et al. **HLA-B leader and survivorship after HLA-mismatched unrelated donor transplantation.** Blood. Epub 2020 Jun 1. [Epub ahead of print].

Nikiforow S, Mansour M, Hu Z-H, et al. **Tocilizumab not associated with increased infection risk after CAR T - Implications for COVID-19?** Blood. Epub 2020 May 25. [Epub ahead of print].

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

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

Hourigan CS, Dillon LW, Gui G, et al. **Impact of conditioning intensity of allogeneic transplantation for AML with genomic evidence of residual disease.** Journal of Clinical Oncology. 2020 Apr 20; 38(12):1273-1283. Epub 2019 Dec 20. PMC7164487.

Atallah E, Logan BR, Chen M, et al. **Comparison of patient age groups in transplantation for MDS: The Medicare coverage with evidence development study.** JAMA Oncology. 2019 Dec 12; 6(4):486-493. Epub 2019 Dec 12. PMC6990739.

Meyer C, Mau L-W, Murphy EA, et al. **Addressing knowledge gaps in AML to improve referral for HCT consultation.** Journal of the National Comprehensive Cancer Network. 2019 Dec 1; 17(12):1473-1481. Epub 2019 Dec 1.

Ballen K, Logan BR, Chitphakdithai P, et al. **Unlicensed umbilical cord blood units provide a safe and effective graft source for a diverse population: A study of 2,456 umbilical cord blood recipients.** Biology of Blood and Marrow Transplantation. 2020 Apr 1; 26(4):745-757. Epub 2019 Nov 19.

Pidala J, Hamadani M, Dawson P, et al. **Randomized multicenter trial of sirolimus vs prednisone as initial therapy for standard-risk acute GVHD: The BMT CTN 1501 trial.** Blood. 2020 Jan 1; 135(2):97-107. Epub 2019 Nov 18. PMC6952830.

Sapir-Pichhadze R, Zhang X, Ferradji A, et al. **Epitopes as characterized by antibody-verified eplet mismatches determine risk of kidney transplant loss.** Kidney International. 2020 Apr 1; 97(4):778-785. Epub 2019 Nov 12.

Pidala J, Martens M, Anasetti C, et al. **Factors associated with successful discontinuation of immune suppression after allogeneic HCT.** JAMA Oncology. 2019 Sep 26; 6(1):e192974. Epub 2019 Sep 26. PMC6763979.



Milwaukee Campus

Medical College of Wisconsin
9200 W. Wisconsin Ave, Suite C5500
Milwaukee, WI 53226 USA
(414) 805-0700

Minneapolis Campus

National Marrow Donor Program/
Be The Match
500 N. 5th Street
Minneapolis, MN 55401-1206 USA

CIBMTR.ORG

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