



CIBMTR[®]

CENTER FOR INTERNATIONAL BLOOD
& MARROW TRANSPLANT RESEARCH



FACTS AND FIGURES

July 2020 - June 2021

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 (MCW).



Medical and Scientific
EXPERTISE



Worldwide **NETWORK**



Clinical **DATABASE**

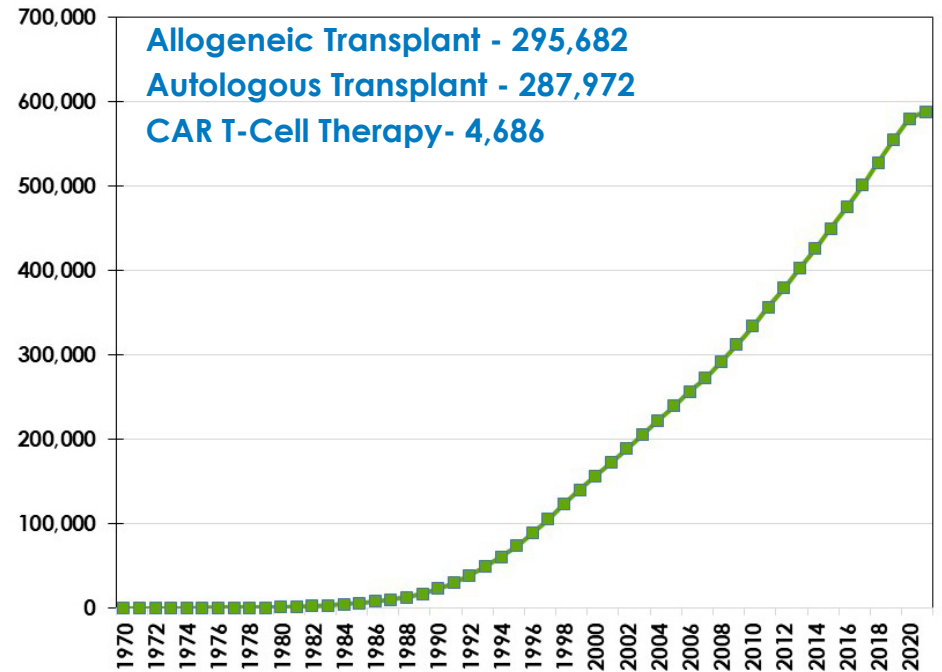


Biospecimen **REPOSITORY**

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, 1970-2021

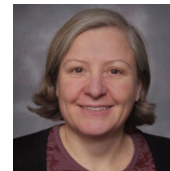


50 Years

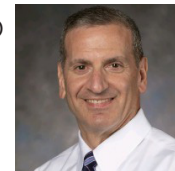


>585,000 Patients

Chief Scientific Directors



Bronwen Shaw, MD, PhD
CIBMTR MCW



Jeffery Auletta, MD
CIBMTR NMDP



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
- **>3,000 worldwide researchers** participate on the committees
- **45 global experts** in the field chair the committees
- **>180 studies** in progress
- **29 abstracts** (11 oral and 18 poster) presented at national and international conferences this year



604 Scientific Authors



238 Institutions



56 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; this year the CIBMTR collected **3,420 PRO surveys** from study subjects across **5 clinical trials**
- **Routine collection:** Launched the Protocol for Collection of PRO Data, which includes routine collection of a core PRO survey instrument from adult transplant and chimeric antigen receptor (CAR) T recipients; this year **38 patients** enrolled in the protocol, providing **66 PRO surveys**

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

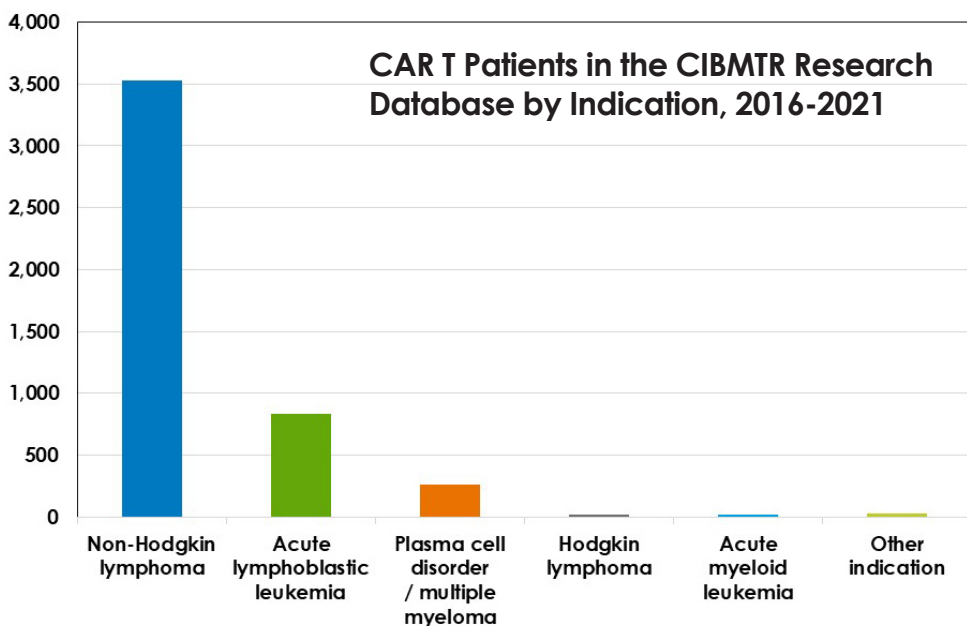
- **Transplant center volumes data:** Includes transplants performed 2014-2018
- **Center-specific survival analysis:** Published the 2020 analysis, which includes first allogeneic transplants performed 2016-2018
- **Center Outcomes Forum:** Discussed impact of the COVID-19 pandemic, minimum residual disease status, and social determinants of health; recommended revised approach for 2021 center-specific survival analysis
- **18 research summaries:** Plain language summaries of research publications created for patients and their loved ones



CELLULAR THERAPY REGISTRY

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

- **>4,600 CAR T patients** at >170 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
- **5 post-FDA approval CAR T-cell safety studies** currently supported by the CIDR



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

- **1 sickle cell study** completed enrollment this year, and investigators **published primary results**



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
- **3 abstracts** (1 oral and 2 poster) presented at national and international conferences this year
- **4 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 human leukocyte antigen (HLA) and killer immunoglobulin like receptor (KIR) data for use in clinical outcomes studies
- **3 oral abstracts** presented at national and international conference this year
- **>10,000 samples** received this year
- **>36,500 samples** distributed to investigators this year

195,026 Samples in Inventory

Multiple aliquots available for each sample



80,507 from unrelated donors
12,372 from related donors



75,795 from unrelated recipients
12,866 from related recipients

13,486 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
- **4 abstracts** (3 oral and 1 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
- **2 oral abstracts** presented at an international conference this year
- **5 manuscripts** published in peer-reviewed journals 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 unlicensed 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
- **1 oral abstract** presented at an international conference this year



26 Studies in progress



4,873 Patients
accrued this year



2 Publications 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
- **>490,000 biospecimens** in the Research Sample Repository
- **48 ancillary and correlative studies** in progress in addition to primary trials
- **13 abstracts** (9 oral and 4 poster) presented at national and international conferences this year



13 Trials in progress



>2,200 Patients
accrued this year



10 Publications this year

Key Publications

The CIBMTR published **94 peer-reviewed manuscripts** in scientific journals this year. Some of the CIBMTR's key findings were published in the following articles.

Kim S, Logan B, Riches M, et al. **Statistical methods for time-dependent variables in HCT.** *Biology of Blood and Marrow Transplantation.* 2021 Feb 1; 27(2):125-132. doi:10.1016/j.bbmt.2020.09.034. Epub 2020 Oct 2. PMC8015678.

Roe D, Williams J, Ivery K, et al. **Efficient sequencing, assembly, and annotation of human KIR haplotypes.** *Frontiers in Immunology.* 11:582927. doi:10.3389/fimmu.2020.582927. Epub 2020 Oct 9. PMC7581912.

Qayed M, Ahn KW, Kitko CL, et al. **A validated pediatric disease risk index for allogeneic HCT.** *Blood.* 2021 Feb 18; 137(7):983-993. doi:10.1182/blood.2020009342. Epub 2020 Nov 18. PMC7918183.

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

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

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

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

Mayor NP, Wang T, Lee SJ, et al. **Impact of previously unrecognized HLA mismatches using ultrahigh resolution typing in unrelated donor HCT.** *Journal of Clinical Oncology.* doi:10.1200/JCO.20.03643. Epub 2021 Apr 9.

Gooptu M, Romee R, St Martin A, et al. **HLA haploidentical vs matched unrelated donor transplants with PTCy based prophylaxis.** *Blood.* doi:10.1182/blood.2021011281. Epub 2021 Apr 13.

Mupfudze TG, Meyer C, Preussler JM, et al. **HCT outcomes among Medicaid and privately insured patients with sickle cell disease.** *Transplantation and Cellular Therapy.* doi:10.1016/j.jtct.2021.04.009. Epub 2021 Apr 22.

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

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

Nakamura R, Saber W, Martens M J, et al. **Biologic assignment trial of reduced-intensity HCT based on donor availability in patients 50-75 years of age with advanced MDS.** *Journal of Clinical Oncology.* doi:/10.1200/jco.20.03380. Epub 2021 Jun 9.



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