FACTS AND FIGURES
July 2020 - June 2021
SHARING KNOWLEDGE. SHARING HOPE.
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).

Who We Are

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

- Allogeneic Transplant - 295,682
- Autologous Transplant - 287,972
- CAR T-Cell Therapy - 4,686

50 Years >585,000 Patients

Chief Scientific Directors

Bronwen Shaw, MD, PhD
CIBMTR MCW

Jeffery Auletta, MD
CIBMTR NMDP
PATIENT-REPORTED OUTCOMES (PRO)

Studying long-term survivorhip 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

[www.cibmtr.org/Studies/HealthServices/Pages/index.aspx](www.cibmtr.org/Studies/HealthServices/Pages/index.aspx)
[www.cibmtr.org/Samples/Inventory/Pages/index.aspx](www.cibmtr.org/Samples/Inventory/Pages/index.aspx)
### 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

[cibmtr.org/Studies/Bioinformatics/Pages/Bioinformatics-Research-index.aspx](cibmtr.org/Studies/Bioinformatics/Pages/Bioinformatics-Research-index.aspx)

### 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

[cibmtr.org/StatisticalSupport/Pages/index.aspx](cibmtr.org/StatisticalSupport/Pages/index.aspx)
## Clinical Trials Support

### 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

#### Stats:

- **26 Studies** in progress
- **4,873 Patients** accrued this year
- **2 Publications** this year

[cibmtr.org/Studies/ClinicalTrials/RCI_BMT/Pages/index.aspx](cibmtr.org/Studies/ClinicalTrials/RCI_BMT/Pages/index.aspx)

### 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

#### Stats:

- **13 Trials** in progress
- **>2,200 Patients** accrued this year
- **10 Publications** this year

[cibmtr.org/Studies/ClinicalTrials/BMT_CTN/Pages/index.aspx](cibmtr.org/Studies/ClinicalTrials/BMT_CTN/Pages/index.aspx)
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.


