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

- Allogeneic Transplant - 280,299
- Autologous Transplant - 269,203
- Other Cellular Therapy - 3,708

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

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

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

Access to Treatment
Value and Health Economics
Health Disparities

Survivorship
Late Effects
Patient-Reported Outcomes

Treatment Decision Making

cibmtr.org/Studies/HealthServices/Pages/index.aspx
cibmtr.org/Samples/Inventory/Pages/index.aspx
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

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

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

[Statistical Methodology Research](http://cibmtr.org/StatisticalSupport/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
- >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

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
- 2 poster abstracts (1 oral and 1 poster) presented at an international conference this year

Clinical Trials Support

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<thead>
<tr>
<th>RCI BMT</th>
<th>BMT CTN</th>
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<tbody>
<tr>
<td>24 Studies in progress</td>
<td>13 Trials in progress</td>
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<tr>
<td>3,367 Patients accrued this year</td>
<td>&gt;300 Patients accrued this year</td>
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<tr>
<td>1 Publication this year</td>
<td>15 Publications this year</td>
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cibmtr.org/Studies/ClinicalTrials/RCI_BMT/Pages/index.aspx
cibmtr.org/Studies/ClinicalTrials/BMT_CTN/Pages/index.aspx
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.


