A WORLD OF RESEARCHERS

>6,500 researchers, clinicians, and support personnel
>420 centers
>50 countries

WORKING TOGETHER

>220 studies in progress
>1,300 publications
>160,000 biorepository samples

FOR LIFE

>495,000 patients registered
~24,000 new patients registered annually
The CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin. The CIBMTR facilitates critical research through medical, scientific, and statistical expertise; a large network of participating centers; a unique and extensive clinical database; and a robust and comprehensive biospecimen repository.

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

**VALUE TO THE COMMUNITY**
The CIBMTR has collected clinical outcomes data worldwide for >45 years, resulting in a Research Database with information on >495,000 patients. At any given time, the CIBMTR has >200 retrospective research studies and >20 prospective research studies ongoing in 6 major areas of research activity.

**GLOBAL COLLABORATION**
The CIBMTR comprises an international network of >420 centers in >50 countries that submit cellular therapy-related data for patients. Data for almost 100% of US allogeneic transplants and >80% of US autologous transplants are reported to the CIBMTR, and data for >7,500 non-US patients are collected annually. The CIBMTR continues to strengthen its collaborations with centers around the world as well as national and international registries. A major focus is enhancing data alignment and exchange, particularly through harmonization of data collection. CIBMTR investigators also partner on research studies, and CIBMTR staff members mentor and train colleagues across the globe.
SELECT DONORS AND GRAFTS
CIBMTR studies help establish the paradigm for selecting the best donor and graft:
- Optimal human leukocyte antigen (HLA) matching
- Impact of donor characteristics
- Cord blood vs bone marrow vs peripheral blood

EVALUATE PATIENT RISK
CIBMTR studies show which patients:
- Have the highest risk of graft-vs-host disease (GVHD) and other complications
- Are most likely to benefit from transplantation

IDENTIFY LONG-TERM EFFECTS OF TRANSPLANTATION
CIBMTR studies provide insight into:
- Long-term impact of transplantation on patients and their families, including risk of second cancers and other late complications
- Survivors’ quality of life

PROVIDE MEDICAL CARE GUIDANCE FOR SURVIVORS
The CIBMTR works with the medical community to develop guidelines for optimal long-term care of transplant survivors to:
- Decrease the rate of late complications
- Preserve patients’ fertility
- Identify post-transplant best practice preventive health behaviors

ADDRESS ACCESS TO CARE
CIBMTR studies address the broad range of issues that influence access to transplantation and long-term care after transplantation, including:
- Disparities in access and outcomes for specific populations
- Costs of care

Publications establishing the CIBMTR’s research in each of these areas are listed at cibmtr.org/About/AdminReports/Pages/SummaryPubs.aspx

The CIBMTR is dedicated to improving survival, treatment, and quality of life for patients. With >1,300 publications, the CIBMTR conducts practice-changing research that helps patients and physicians.
CUMULATIVE PATIENTS REGISTERED WITH THE CIBMTR

NUMBER OF PATIENTS REGISTERED

1 Includes other leukemia and solid tumors
2 Includes myelodysplastic (MDS) and myeloproliferative syndromes (MPS)
3 Includes severe aplastic anemia and paroxysmal nocturnal hemoglobinuria (PNH)
4 Includes immune deficiencies and histiocytic disorders
5 Includes sickle cell anemia, sickle cell thalassemia, and thalassemia major
6 Includes Schwachmann-Diamond, Fanconi anemia, Diamond-Blackfan anemia, and other inherited abnormalities of erythrocyte
7 Includes platelet disorders and autoimmune deficiencies
Answering clinically important questions using the CIBMTR’s unique and extensive Research Database

Clinical Outcomes Research studies address a wide range of issues, focusing on questions that are difficult or impossible to address in single-center studies or randomized trials because diseases treated with HCT and cellular therapy are uncommon, single centers treat few patients with a given disorder, and not all important questions are amenable to a randomized study design.

SCIENTIFIC WORKING COMMITTEES

Fifteen Scientific Working Committees oversee most of the CIBMTR’s clinical outcomes research. Each committee focuses on a specific disease, use of HCT, or complication of therapy. More than 2,800 worldwide researchers participate on the committees, which are chaired by 45 global experts in the field. There are currently >195 studies in progress; 40 new study proposals were approved at the 2018 BMT Tandem Meetings. The CIBMTR encourages new investigators to participate in and lead studies. In each of the past 3 years, almost 50% of proposals were submitted by principal investigators who had not previously submitted a proposal to use CIBMTR data.

Over the past 12 months, Working Committee researchers presented 40 abstracts (22 oral and 18 poster) at national and international conferences. More than 435 scientific authors at >200 institutions worldwide published 55 Working Committee manuscripts in peer-reviewed journals this year, including in the Journal of Clinical Oncology, Blood, Leukemia, and Haematologica.
MEDICARE CLINICAL TRIALS AND STUDIES

Many patients with specific diseases and/or at certain ages are denied access to HCT 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. The CIBMTR launched 2 new Medicare CED studies this year, increasing the number of studies conducted to 5.

<table>
<thead>
<tr>
<th>Disease</th>
<th>Patient Population</th>
<th>Enrollment</th>
<th>Dates</th>
</tr>
</thead>
<tbody>
<tr>
<td>Myelodysplastic Syndrome (10-CMS-MDS)</td>
<td>Elderly patients with myelodysplastic syndrome</td>
<td>130 centers 3,666 patients 2,327 patients ≥65 years old 1,113 patients 55-64 years old 226 patients &lt;54 years old</td>
<td>Launched in 2010</td>
</tr>
<tr>
<td>Myelofibrosis (16-CMS-MF)</td>
<td>Patients aged ≥55 years with primary myelofibrosis</td>
<td>106 centers 76 patients (650 planned) 19 related (matched 6/6) 47 unrelated (matched 8/8) 10 haploidentical</td>
<td>Launched in December 2016</td>
</tr>
<tr>
<td>Multiple Myeloma (17-CMS-MM)</td>
<td>Elderly patients with Stage II or III multiple myeloma or primary plasma cell leukemia</td>
<td>77 centers 4 patients (550 planned)</td>
<td>Launched in July 2017</td>
</tr>
<tr>
<td>Sickle Cell Disease (BMT CTN 1503)</td>
<td>Adolescents and young adults with severe sickle cell disease</td>
<td>37 centers 55 patients (200 planned)</td>
<td>Launched in October 2016</td>
</tr>
<tr>
<td>Sickle Cell Disease (17-CMS-SCD)</td>
<td>Patients aged 15-50 years with severe sickle cell disease</td>
<td>63 centers 1 patient (200 planned)</td>
<td>Launched in November 2017</td>
</tr>
</tbody>
</table>

Expanded Coverage

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

In addition to receiving data on transplant patients, the CIBMTR received data from >175 centers for approximately 1,500 patients who received cellular therapy. Indications for treatment included malignant hematologic disorders, neurologic diseases, infection and GVHD treatment and prophylaxis, immune reconstitution, cardiovascular disease, and others.

The CIBMTR receives cellular therapy data via a suite of Cellular Therapy Essential Data (CTED) forms. These forms, harmonized with the European and Japanese registries, undergo real time review and revision. In October 2017, the CIBMTR held a third Cellular Therapy Registry Forum focused on long-term follow-up and cytokine release syndrome.

The CIBMTR continues to expand its cellular therapy registry capabilities through fulfillment of the Food and Drug Administration (FDA) long-term follow-up requirement. The FDA requires pharmaceutical companies that commercialize genetically engineered cellular therapies to follow recipients of these therapies for 15 years in order to evaluate their safety and efficacy. The CIBMTR can support this requirement and is currently partnered with several pharmaceutical companies to track these long-term outcome data.

Soon the CIBMTR will also serve as the Cellular Immunotherapy Data Resource (CIDR) for the Immuno-Oncology Transplantation Network (IOTN), funded by the National Cancer Institute and National Institute on Minority Health and Health Disparities. For the CIDR, the CIBMTR’s data registry will collect outcomes of patients receiving cellular immunotherapies to support observational studies and inform subsequent studies and clinical trials. The IOTN supports the Cancer MoonshotSM initiative to accelerate cancer research to make more therapies available to more patients.

The CIBMTR hosted an initial regenerative medicine outcomes registry strategy meeting in August 2017. Collaborative efforts will continue at a second meeting to be held in September 2018 in association with Cord Blood Connect, an international conference hosted by the Cord Blood Association. This meeting will focus not only on sharing the CIBMTR’s registry capabilities, including form development and collection of patient-reported outcomes, but also on determining disease priority and establishing working groups related to the regenerative medicine outcomes registry.
STEM CELL THERAPEUTIC OUTCOMES DATABASE
The CIBMTR operates the Stem Cell Therapeutic Outcomes Database (SCTOD) for the C.W. Bill Young Cell Transplantation Program through a contract with the Health Resources and Services Administration. For the SCTOD, the CIBMTR tracks and analyzes data for all allogeneic transplants performed in the US and transplants performed globally with products from the US. The contract was renewed in 2017.

Each year the CIBMTR publishes transplant center volumes data and center-specific survival rates. This year’s center volumes report includes transplants performed in 2012-2016. The latest center-specific survival analysis includes first allogeneic transplants performed in 2013-2015. The center-specific survival analysis being prepared for issue in 2018 will contain enhanced risk adjustment variables. Working with NMDP/Be The Match Patient and Health Professional Services, the CIBMTR published 16 lay summaries of research publications for patients and their loved ones.

PATIENT-REPORTED OUTCOMES
This year the CIBMTR launched a pilot project to obtain patient-reported outcomes (PRO) electronically using a new ePRO system. This system incorporates a user-friendly interface in Qualtrics, automated tracking and alerting functionality, and PROMIS measures, using computer logic to focus on questions relevant to the particular patient. The pilot project will enroll as many as 220 patients and conclude in 2019. The primary objective is to compare quality of life in transplant recipients age 55-64 with recipients age 65 and older.

In the future, the CIBMTR plans to use the ePRO system to routinely collect data on a subset of US patients. With these data, the CIBMTR will study long-term survivorship issues and characterize the patient experience, overall and in specific patient cohorts defined by disease, ethnicity, race, region, age, and other demographics.
SHARING KNOWLEDGE.

INFORMATION

- >1,300 Publications
- Annual Report
- TCT Meetings (formerly BMT Tandem Meetings)
- Center Reference Guide
- Forms Instruction Manual
- Online Trainings
- Quarterly Newsletter

TOOLS

- Disease Risk Index Assignment Tool
- Center Performance Analytics
- Veno-Occlusive Disease of the Liver Risk Calculator
- Patient One-Year Survival Calculator for Allogeneic Transplants
SHARING KNOWLEDGE. SHARING HOPE.

DATA RESOURCES

- BMT Survival Statistics Report
- Center-Specific Survival Reports
- Patient Transplant Outcomes Report
- Propose a Working Committee Study
- eDBtC
- Custom Information Request Form

BIOSPECIMENS

- >2 Million Samples
- Review Sample Types and Inventory
- Request Samples from the Research Sample Repository
Exploring how social factors, financial systems, health care processes, and behavior affect access to and outcomes of cellular therapies

The Health Services Research Program uses both quantitative and qualitative research methods to identify and address barriers to treatment, improve practice, and demonstrate the value of cellular therapies and survivorship care. This year investigators presented 9 abstracts (3 oral and 6 poster) at national and international conferences and published 4 manuscripts in peer-reviewed journals.

Access – The CIBMTR is conducting several studies to identify and remove barriers to transplant, particularly for patients from disadvantaged backgrounds and racial and ethnic minority populations. For example, the CIBMTR, in partnership with the NMDP/Be The Match Health and Public Policy department, is exploring differences among states’ Medicaid coverage of transplantation for patients with sickle cell disease.

Health Economics – Value, quality, and access are critical aspects of care for patients who may receive transplant. The CIBMTR continues work on 3 analyses of reimbursement and service utilization associated with transplant or non-transplant therapy for patients with acute myeloid leukemia or multiple myeloma using both a commercial payer dataset and a novel Medicare dataset linked with CIBMTR clinical outcomes data.

Survivorship - Quality of life is a very important outcome of therapy. CIBMTR investigators continue work with investigators from the Fred Hutchinson Cancer Center, University of Washington, and Cleveland Clinic on a 5-year National Institutes of Health (NIH)-funded effort, the INSPIRE study, to determine the impact of stepped self-care in improving quality of life for transplant survivors. Patient reported outcomes are an important aspect of this trial as well as several others.

Treatment Decision-Making Support - Translation of research results into clinical practice is critical to advancing patient care. Investigators analyzed the impact of a prospective, randomized study of unrelated donor cell source type (BMT CTN 0201) at the clinician and transplant center levels. Researchers also studied the perspectives of transplant physicians and coordinators with regard to donor identification and urgency of transplantation, and, in collaboration with other organizations, the CIBMTR shared knowledge with >3,000 health care providers regarding optimal transplant timing for patients with acute myeloid leukemia.

In a survey of >250 transplant physicians, 76% stated they trust palliative care physicians to care for their patients; nearly half felt the service name ‘palliative care’ is a barrier to utilization of services by transplant recipients.
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 transplant outcomes

The CIBMTR’s Immunobiology Research group manages the Research Repository inventory and immunogenetic testing programs that add critical HLA and killer-cell immunoglobulin-like receptor (KIR) data for use in CIBMTR clinical outcomes studies. This year the CIBMTR received >11,500 samples, including >4,000 from unrelated donors and >1,400 from related donors, almost 4,000 from unrelated recipients and >1,400 from related recipients, and >500 from unrelated cord blood units. Over the last 12 months, the CIBMTR completed HLA and KIR typing on >4,000 donor and recipient research sample pairs.

Linking outcomes data to immunologic data available in the Research Repository supports studies that include genetic and immunobiologic data and clinical phenotype data. The Immunobiology Research group distributed >12,700 samples to investigators this year. The combination of the Unrelated and Related Donor Research Repositories facilitates an organized approach to studying transplant biology across the spectrum of allogeneic HCT.

161,521 Samples

| 69,422 | from unrelated donors and 8,670 from related donors |
| 62,433 | from unrelated recipients and 9,025 from related recipients |
| 11,971 | from unrelated cord blood units |
Providing expertise in and conducting research on translational and operational bioinformatics

The Bioinformatics Research group studies how to improve the donor recipient matching algorithm, investigating technologies used to make matching as stable, secure, and fast as possible and examining the role of genetic ancestry in transplantation. They develop pipelines to analyze next generation sequencing typing data, including full-gene HLA, KIR, and genome-wide sequencing, to refine our understanding of genetic matching, and they create data standards and tools for making immunogenetic data portable for research and clinical use. This year investigators with the Bioinformatics Research Program presented 10 abstracts (3 oral and 7 poster) at national and international conferences and published 11 manuscripts in peer-reviewed journals.

**HLA Resources**

- **HaploStats**
  Frequency information for haplotypes and haplotype pairs relative to specific HLA types found in the US population.

- **Allele Codes**
  Allele code lists and tools, including a description of how allele codes are affected by the World Health Organization’s version 3 nomenclature change.

- **Haplotype Frequencies**
  Haplotype associations and allele frequency data for various race and ethnic populations, including those in the US and Jerusalem.

- **Histoimmunogenetics Markup Language (HML)**
  Software reporting format, developed by the Bioinformatics Research group, to facilitate exchange of genetic typing data to reduce donor search times.
Developing statistical models to use in cellular therapy research and comparing new models to existing solutions using the CIBMTR Research Database

HCT is a complex process with multiple competing risks and dramatic changes in the risks of specific events over time. The CIBMTR has developed and evaluated many of the statistical models used in HCT and cellular therapy research and works to provide guidance for appropriate application and interpretation of these sophisticated models.

Biostatisticians with the Statistical Methodology Research Program ensure the statistical integrity of CIBMTR scientific activities, contribute to results in articles on statistical issues for clinical audiences, and support Working Committee study investigators in developing scientific study protocols using CIBMTR data. This year biostatisticians presented 2 oral abstracts at an international conference and published 7 peer-reviewed manuscripts focused on methodology.

Biostatistical Resources

At the TCT Meetings

Educational Sessions
related to statistical design and analysis

1:1 Statistical Consultation
for researchers writing proposals or developing protocols for CIBMTR studies

>20 Online Lectures
• ANOVA: Comparing >2 Treatments
• Basic Concepts of Bayesian Statistics
• Common Errors in Linear Regression
• Designing Clinical Trials
• Introduction to Survival Analysis
• Matched Studies in Medical Research
• Uses and Abuses of Non-Parametric Statistics
• Web-Based Sample Size Calculation
• Writing a Protocol
CLINICAL TRIALS SUPPORT

Conducting multicenter Phase I-III national trials through the BMT CTN and RCI BMT

The CIBMTR provides skilled administrative leadership, a vast network of participating centers, and substantial infrastructure support for prospective research. Cellular therapy is a rapidly evolving field, and clinical trials face unique challenges, including the relatively small number of treatments performed at any single center, the diverse indications for cellular therapy, the complexities of the intervention, and multiple post-treatment complications. The BMT CTN addresses these challenges and executes multicenter Phase II and III trials with broad national participation. The RCI BMT supports smaller trials that bridge the gap between single-center studies and larger trials.

Blood and Marrow Transplant Clinical Trials Network (BMT CTN)
The CIBMTR, along with NMDP/Be The Match and The Emmes Corporation, serves as the BMT CTN Data and Coordinating Center, which is supported by an NIH grant from the National Heart, Lung, and Blood Institute and the National Cancer Institute. The BMT CTN has launched 46 trials (one this year) and accrued >10,300 patients (>600 this year). Over the last 12 months, the BMT CTN managed 11 open protocols with overall accrual for open studies at 100% of projections. Investigators presented 8 abstracts (5 oral and 3 poster) of study results at national and international meetings, and they published 11 manuscripts in peer-reviewed journals this year.

Resource for Clinical Investigation in Blood and Marrow Transplantation (RCI BMT)
Supported primarily by NMDP/Be The Match and corporate and private sponsors, the RCI BMT operates without NIH funding. This year the RCI BMT managed 9 active ongoing protocols and opened one new protocol, which have cumulatively accrued almost 3,400 patients. The RCI BMT also initiated development of 4 new protocols. Over the last 12 months, the RCI BMT completed accrual on one trial and presented an oral abstract at an international conference. Investigators published 2 manuscripts in peer-reviewed journals this year.

The Survey Research Group of the RCI BMT assists clinical researchers in developing and conducting research involving questionnaires and patient interviews. The group also manages the CIBMTR’s new ePRO system. This year the Survey Research Group supported 5 active studies, including activation of a new study; contacted >7,000 donors and patients; and participated in the development of 4 upcoming studies.
CORPORATE SUPPORT

Collaborating with corporate partners to provide access to high quality data and conduct scientifically sound studies

The CIBMTR provides opportunities for industry collaborators to access CIBMTR data and statistical support to address questions specific to their business needs through Corporate Membership as well as Corporate Studies and Projects. The CIBMTR also offers access to scientific and statistical expertise through consultation services, such as protocol design and development and long-term outcomes registry development.

**Corporate Membership** - The CIBMTR Corporate Membership program provides a variety of resource materials to corporations seeking access to the most current and comprehensive data on HCT. These materials are useful for marketing managers, medical directors, research directors, product managers, case managers, and transplant coordinators. This year 17 organizations participated in the CIBMTR Corporate Membership program, including 14 that joined or renewed this year.

**Corporate Studies and Projects** - Corporate partners may contract with the CIBMTR to conduct a study, support a project involving more complex analyses, or license a specified data set. Organizations interested in funding a study, such as one comparing HCT with one or more other therapies, or using historical controls, can negotiate with the CIBMTR for data and/or expert statistical analyses. This year the CIBMTR engaged in >20 studies with corporate partners.

Making life-saving research possible

Learn more about the CIBMTR’s membership program and opportunities for specific studies and events at cibmtr.org/support.

Together, the CIBMTR and Be The Match BioTherapies®, a subsidiary of NMDP/Be The Match, offer end-to-end solutions (E2ES) for developers of cellular therapies, including cell sourcing and collection, clinical trial services, supply chain logistics, manufacturing and commercialization support, and outcomes management.
## ANNUAL MEETINGS

### 2018 BMT TANDEM MEETINGS
- >3,800 attendees
- >45 countries
- 5 plenary sessions
- 9 concurrent sessions
- 96 oral abstracts
- 2 poster sessions
- 7 corporate-sponsored symposia
- 9 product theaters

### 2019 TCT MEETINGS
- Cell and gene therapy
- GVHD
- CAR T-cell therapy
- Haploidentical and cord blood transplants
- Post-transplant relapse
- Hemoglobinopathies
- Many other topics

As of 2019, the BMT Tandem Meetings are called the **TCT I Transplantation & Cellular Therapy Meetings of ASBMT and CIBMTR** (TCT Meetings) to better reflect an expanded range of interests and activities.

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**SUPPORT THE TCT MEETINGS**

The TCT Meetings provide exhibiting opportunities as well as corporate-supported satellite sessions, offering exposure to the world’s top HCT and cellular therapy physicians and allied health professionals. Educational support could focus on a particular session or consist of a mealtime continuing medical education session unopposed by other scientific meetings. Product theaters are also available as well as marketing / promotional support and advertising opportunities.

### UPCOMING MEETINGS

<table>
<thead>
<tr>
<th>Location</th>
<th>Dates</th>
</tr>
</thead>
<tbody>
<tr>
<td>Orlando, FL</td>
<td>Feb. 19-23, 2020</td>
</tr>
<tr>
<td>Feb. 15-19, 2023</td>
<td></td>
</tr>
<tr>
<td>Salt Lake City, UT</td>
<td>Feb. 2-6, 2022</td>
</tr>
<tr>
<td>Houston, TX</td>
<td>Feb. 20-24, 2019</td>
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<tr>
<td>Feb. 2-6, 2022</td>
<td></td>
</tr>
<tr>
<td>Honolulu, HI</td>
<td>Feb. 11-15, 2021</td>
</tr>
</tbody>
</table>

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![Map of TCT Meetings Locations]

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The CIBMTR published **90 manuscripts in peer-reviewed journals** this year. Some of the CIBMTR’s key findings were published in the following articles.


