FACTS AND FIGURES
July 2016 - June 2017
A WORLD OF RESEARCHERS

>5,000 researchers, clinicians, and support personnel
>420 transplant centers
>55 countries

WORKING TOGETHER

>220 studies in progress
>1,200 publications
~150,000 biorepository samples

FOR LIFE

>475,000 patients registered
~23,000 new patients registered annually
WHO WE ARE

MISSION
The CIBMTR® (Center for International Blood and Marrow Transplant Research®) collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy worldwide to increase survival and enrich quality of life for patients. A research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin (MCW), the CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of clinical centers, and a unique and extensive clinical database.

VALUE TO THE COMMUNITY
The CIBMTR has been collecting clinical outcomes data worldwide for 45 years, resulting in a Research Database with information on >475,000 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.

CIBMTR data are freely available to investigators with interest in HCT and treatments for cancer and other life-threatening diseases. The CIBMTR has become a respected leader in clinical research by providing a unique resource of information and expertise to the medical and scientific communities.
The CIBMTR represents an international network of >420 participating clinical centers in >55 countries that submit transplant- and cellular therapy-related data for patients. Almost 100% of US allogeneic transplants and about 90% of US autologous transplants are reported to the CIBMTR. Data for approximately 7,600 non-US patients are collected annually.

This year the CIBMTR continued to strengthen its international collaborations with clinical centers and international registries. With the European Society for BMT, the CIBMTR enhanced data alignment and exchange and formalized a commitment to study collaboration. In January, 6 CIBMTR Scientific Directors spoke at the Workshop and Scientific Symposium hosted by the Worldwide Network for BMT in Riyadh, Saudi Arabia. In February, the CIBMTR hosted the Latin American BMT Society’s meeting at the BMT Tandem Meetings. The CIBMTR also welcomed colleagues from Pakistan and Brazil this year for intensive training in data and study management.
The CIBMTR is dedicated to improving survival, treatment, and quality of life for patients. With >1,200 publications, the CIBMTR conducts practice-changing research that helps patients and physicians:

**SELECT DONORS AND GRAFTS**
CIBMTR studies helped 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 have shown 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 transplant 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 has worked 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 as much as possible
- Identify post-transplant best practice preventive health behaviors

**ADDRESS ACCESS TO CARE AND FUTURE WORKFORCE NEEDS**
CIBMTR studies address the broad range of issues that influence access to transplant and long-term care after transplantation, including:
- Disparities in access and outcomes of specific populations
- Costs of care
- Future workforce capacity

Publications establishing the CIBMTR’s research in each of these areas are listed at [www.cibmtr.org/About/ProceduresProgress/Pages/SummaryPubs.aspx](http://www.cibmtr.org/About/ProceduresProgress/Pages/SummaryPubs.aspx)
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 research 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. >2,600 worldwide researchers participate on the committees, which are chaired by 47 global experts in the field. There are currently >175 studies in progress; 33 new study proposals were approved at the BMT Tandem Meetings. >510 scientific authors at 260 institutions worldwide published 48 manuscripts, including in the New England Journal of Medicine, JAMA, and the Journal of Clinical Oncology. Researchers presented 35 abstracts (26 oral and 9 poster) at national and international conferences, including 22 (16 oral and 6 poster) at the American Society of Hematology Annual Meeting and 12 (9 oral and 3 poster) at the BMT Tandem Meetings.

Cellular Therapy Outcomes Registry
In addition to collecting data on HCT patients, the CIBMTR continues to increase its data collection of cellular and other therapies that may not involve transplantation. This year the CIBMTR continued to enhance its new Cellular Therapy Outcomes Registry. In October 2016, the CIBMTR hosted a second Cellular Therapy Forum for physicians and scientists as well as commercial and government representatives. The CIBMTR also revised cellular therapy data collection forms and collaborated with the European and Asia Pacific BMT groups to harmonize cellular therapy data collection efforts across the globe. Most notably, the CIBMTR conducted a one-year pilot cellular therapy data collection program with 438 patients.
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 is currently engaged in 4 Medicare CED studies:

Myelodysplastic Syndrome (MDS) – Launched in 2010, this study collects data from >130 centers and >3,000 patients with MDS to determine whether outcomes of elderly patients are similar to those in younger patients. The second part of this study opened in 2013 and compares outcomes of HCT to those receiving non-HCT therapies for MDS in patients aged ≥65.

Sickle Cell Disease Clinical Trial – Launched in October 2016, this study compares outcomes of 60 adolescents and young adults with severe sickle cell disease who receive HCT with 140 who do not.

Myelofibrosis – Launched in December 2016, this study compares outcomes of 650 patients ≥55 years old with primary myelofibrosis or post-essential thrombocytemia / polycythemia vera who receive HCT with 2,400 historical patients who did not.

Multiple Myeloma – Launched in June 2017, this study compares outcomes of approximately 500 patients with high risk multiple myeloma who receive allogeneic HCT compared to patients treated with autologous HCT.

48 publications this year
>2,600 worldwide researchers
>35 presentations this year
>175 ongoing studies
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 Immunobiology Research group manages the Research Repository inventory and immunogenetic testing programs that add critical HLA and killer-cell immunoglobulin-like receptors (KIR) data for use in CIBMTR clinical outcomes studies. This year the CIBMTR completed HLA and KIR typing on >3,500 donor/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 16,564 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.

149,973 samples

- 65,290 from unrelated donors and 7,251 from related donors
- 58,463 from unrelated recipients and 7,574 from related recipients
- 11,395 from unrelated cord blood units
NUMBERS OF PATIENTS REGISTERED

CUMULATIVE TRANSPLANT PATIENTS REGISTERED WITH THE CIBMTR

- Cumulative Autologous Transplant Patients
- Cumulative Allogeneic Related Donor Transplant Patients
- Cumulative Allogeneic Unrelated Donor Transplant Patients

>475,000 patients registered

~23,000 new patients registered annually

NEW ALLOGENEIC / AUTOLOGOUS TRANSPLANT PATIENTS REGISTERED PER YEAR

- New Autologous Transplant Patients per Year
- New Allogeneic Transplant Patients per Year
Providing expertise in and conducting research on translational and operational bioinformatics

The Bioinformatics Research group studies how to improve the donor recipient matching algorithm, including investigating technologies used to make matching as stable, secure, and fast as possible and considering 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 11 abstracts (3 oral and 8 poster) at national and international conferences and published 13 peer-reviewed manuscripts in scientific journals.

HLA RESOURCES
Exploring how social factors, financial systems, health care processes, and behavior affect access to cellular therapies and treatment outcomes

The Health Services Research Program conducts research on health services topics, such as health economics, survivorship and quality of care, and treatment decision-making support. There are currently 8 studies in progress; investigators completed analysis for 2 this year.

Health Economics - Performed 2 analyses of reimbursement and service utilization associated with transplant or non-transplant therapy for patients with acute myeloid leukemia using datasets: single commercial payer linked with Social Security Death Index, and Medicare data linked with CIBMTR clinical outcomes data.

Survivorship - Investigators, patients, and caregivers completed a randomized, controlled study to evaluate the effectiveness of an individualized treatment summary and survivorship care plan as a communication tool [funded by the Patient Centered Outcomes Research Institute (PCORI)]. Investigators began work on a 5-year National Institutes of Health funded effort, the INSPIRE study, to determine the impact of stepped self-care for survivorship issues.

Treatment Decision-Making Support - Investigators administered a survey to US transplant physicians to identify factors associated with selection of cell source type (unrelated donor bone marrow vs. peripheral blood stem cells) at the clinician and transplant center levels. In addition, an observational cohort study examined the temporal trends in utilization of unrelated donor bone marrow and peripheral blood stem cell graft source for patients who received an HCT in 2002-2014.

Surveyed >250 US transplant physicians to better understand their perceptions of palliative care in HCT and to identify the unmet need for palliative care among HCT patients; conducted in partnership with the American Society for Blood and Marrow Transplantation Palliative Care Task Force.

Engaged 160 patients, caregivers, specialty physicians, advanced practice professionals, nurses, social workers, patient advocates, researchers, and decision-makers in prioritizing a patient-centered HCT outcomes research agenda through sponsorship of 2 symposia; funded in part by PCORI Engagement Award.

Conducted a survey of clinicians who provide care for patients with acute myeloid leukemia to identify knowledge gaps and held education regional summits in collaboration with the American Society of Hematology, Oncology Nursing Society, American College of Clinical Pathology, and The France Foundation.
Developing statistical models to use in HCT and 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 published 6 peer-reviewed statistical methodological manuscripts.

1Includes other leukemia and solid tumors
2Includes myelodysplastic (MDS) and myeloproliferative syndromes (MPS)
3Includes severe aplastic anemia and paroxysmal nocturnal hemoglobinuria (PNH)
4Includes immune deficiencies and histiocytic disorders
5Includes Schwachmann-Diamond, Fanconi anemia, Diamond-Blackfan anemia, and other inherited abnormalities of erythrocyte
6Includes sickle cell anemia, sickle cell thalassemia, and thalassemia major
7Includes platelet disorders and autoimmune deficiencies
Conducting multicenter Phase II and III national trials through the BMT CTN and Phase I and II trials through the RCI BMT

The CIBMTR participates in and supports prospective research by providing data management resources, access to the CIBMTR Research Database, and statistical expertise. HCT is a rapidly evolving field, and HCT clinical trials face unique challenges, including the relatively small number of transplantations performed at any single center, the diverse indications for HCT, the complexities of the intervention, and multiple post-transplant complications. The BMT CTN was established to address these challenges and execute multicenter HCT trials with broad national participation. The RCI BMT supports smaller trials that bridge the gap between single-center studies and the larger trials of the BMT CTN.

**Blood and Marrow Transplant Clinical Trials Network (BMT CTN)**

Along with NMDP/Be The Match and The Ememes Corporation, a contract research organization, the CIBMTR serves as the Data and Coordinating Center for the BMT CTN, which has launched 45 trials (3 this year) and accrued >9,600 patients (>600 this year). Over the last 12 months, the BMT CTN managed 13 open protocols with overall accrual for open studies at 100% of projections. Investigators published 13 manuscripts in peer-reviewed journals and presented 15 abstracts of study results at national and international meetings this year.

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

During the past year, the RCI BMT conducted 14 trials (including 4 study activations), which have cumulatively accrued almost 33,500 patients (>3,000 this year). Staff members manage 2 FDA Investigational New Drug protocols for NMDP/Be The Match; the Peripheral Blood Stem Cell Procurement protocol accrued >2,600 patients this year, and the Unlicensed Cord Blood Unit Access protocol accrued >450 patients. Over the last year, the RCI BMT completed accrual on 2 trials and presented 6 abstracts of study data at national and international conferences; 3 manuscripts were accepted for publication in peer-reviewed journals.

**Survey Research Group**

The Survey Research Group of the RCI BMT assists clinical researchers in developing and conducting research involving questionnaires and patient interviews. This year the group supported 5 active studies, contacted >9,000 donors and patients, and participated in the development of one upcoming study.
Tracking and analyzing all allogeneic transplants performed in the US and transplants performed globally with products from the US

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. Each year the CIBMTR publishes transplant center volumes data and center-specific survival rates. This year’s center volumes included transplants performed in 2011-2015. The latest center-specific survival analysis included first allogeneic transplants performed in 2012-2014. The CIBMTR continues to build the related sample repository by collecting pre-HCT biologic specimens of related donor-recipient pairs from 70 centers.

The CIBMTR recently launched a new application, the Request for Information (RFI) tool, which provides data reported to the CIBMTR in a format centers can verify and use to complete the outcomes data section of the American Society of Blood and Marrow Transplantation’s RFI form, a standard form used by payers to collect US transplant center information. The CIBMTR also submitted a manuscript on a quality of life pilot project, which demonstrated the CIBMTR can assist centers by collecting data directly from patients. Working with NMDP/Be The Match Patient and Health Professional Services, the CIBMTR published 14 lay summaries of research publications for patients and their loved ones.
Collaborating with corporate partners to provide access to high quality data and conduct scientifically sound studies

The CIBMTR Corporate Program 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:

**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 18 organizations participated in the CIBMTR Corporate Membership program, including 15 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 13 studies with corporate partners.

To help make life-saving research possible, the CIBMTR provides opportunities for foundations, companies, and other industry collaborators to support its work. Learn more about the CIBMTR’s membership program and opportunities for specific studies and events at cibmtr.org/support.
In addition to its Research Programs, the CIBMTR is committed to sharing the data it collects as well as the information and knowledge produced from these data and its extensive collaborations with investigators in the field. The CIBMTR shares information, data, tools, and biospecimens in various ways.

INFORMATION REQUEST SERVICE
The CIBMTR provides timely access to data to patients, physicians, hospitals, pharmaceutical companies, insurance companies, and others involved in healthcare. This year the CIBMTR fulfilled >470 requests for information and data. For questions about requesting CIBMTR data, contact inforequest@mcw.edu.

PUBLIC WEBSITE
The CIBMTR public website (cibmtr.org) provides visitors with information about the CIBMTR and its research, including standard reports, publication and study lists, annual meeting information, and data management training materials. This year visitors accessed pages on the CIBMTR public website >1 million times.

DATA SHARING APPLICATIONS
AGNIS allows participating centers to electronically collect and share data with the CIBMTR. This year 30,512 forms for 19,435 patients were submitted through AGNIS by 25 US transplant centers and by the European Society for Blood and Marrow Transplantation for 65 of their affiliated centers.

In 2017, the CIBMTR released new features for the enhanced Data Back to Center (eDBtC) application, which allows users to download their own data, view and filter outcomes, and even create their own queries. The CIBMTR also launched the RFI tool, which allows centers to leverage data they send to the CIBMTR to fulfill reporting obligations to third party payers and other organizations.

This year, eDBtC was accessed 3,601 times by 280 users from 176 centers. Data was downloaded 456 times. Center Performance Analytics was accessed 555 times by 123 users from 106 centers.

DATA MANAGEMENT TRAINING
The CIBMTR provides detailed information and training to help centers submit high quality data in an efficient manner. Examples on the CIBMTR Training and Reference webpage include the Forms Instruction Manual, FormsNet and AGNIS trainings, and Clinical Research Professional/Data Manager Conference materials. This year the CIBMTR added a Data Management Guide, which provides detailed information in a searchable indexed style. The CIBMTR also added 17 new online courses, increasing the total number of educational materials to 31. The 17 courses include 9 donor, 6 recipient, and 2 clinical trial modules.

BMT TANDEM MEETINGS
The CIBMTR and American Society for Blood and Marrow Transplantation host the BMT Tandem Meetings annually in February. With >3,500 attendees from >40 countries, the 2017 BMT Tandem Meetings included 5 plenary sessions, 9 concurrent sessions, 96 oral abstracts, 2 poster sessions, 9 corporate-sponsored symposia, and 7 product theaters.

UPCOMING MEETINGS
February 21-25, 2018 Salt Lake City, Utah
February 20-24, 2019 Houston, Texas
February 19-23, 2020 Orlando, Florida
February 11-15, 2021 Honolulu, Hawaii
The CIBMTR published 85 peer-reviewed manuscripts and 2 other articles in scientific journals and books this year. Some of the CIBMTR’s key findings were published in the following articles:


