Mission • To improve the outcomes of hematopoietic stem cell transplantation (HCT) through clinical research. The CIBMTR gathers data on patient, disease and transplant characteristics and on transplant outcomes to support studies to determine the most effective patient treatments. Analyzing and providing access to these data are fundamental to the purpose of the organization.

History • The CIBMTR was formed through an affiliation of the International Bone Marrow Transplant Registry (IBMTR) of the Medical College of Wisconsin and the Research Department of the National Marrow Donor Program (NMDP). The purpose of the affiliation was to establish a formal working relationship for collaborative research to advance the HCT field and related research areas, including cellular therapies.

Value to the HCT Community • The CIBMTR has been collecting HCT outcomes data worldwide for 40 years, resulting in a research database with information on more than 350,000 transplant recipients. These data are freely available to investigators with interest in HCT and treatments for cancer and other life-threatening diseases. More than 800 peer-reviewed publications have resulted from analyses of these data. The study findings have changed clinical practice and helped to improve survival and quality of life for patients. The CIBMTR has become a respected leader in HCT research by providing a unique resource of information and expertise to the medical and scientific communities.

The CIBMTR network comprises more than 300 centers across the globe.
OBSESRVATIONAL RESEARCH PROGRAM

Observational research using the CIBMTR’s database is the core activity of the organization.

Outcomes Research: Working Committees • Nineteen scientific Working Committees oversee most of the CIBMTR’s outcomes research. Their accomplishments this year include the following.

- Conducted the 228 studies that were in progress as of July 1, 2013.
- Received 101 new study proposals, 62 of which were presented and 46 of which were accepted at the 2013 BMT Tandem Meetings in February.
- Presented 16 abstracts (13 oral and 3 poster) at the 2012 American Society of Hematology Annual Meeting in December.
- Presented 4 abstracts at the 2013 BMT Tandem Meetings and 5 abstracts at the 2013 European Group for Blood and Marrow Transplantation Annual Meeting.
- Published 33 papers in peer-reviewed journals.
- Implemented many of the recommendations of the Advisory Committee’s Task Force for Best Practices, including Working Committee Leadership formally evaluating each study three times per year.

Health Services Research • The CIBMTR conducts research in program evaluation, health policy and system capacity issues through its Health Policy Working Committee and the Health Services Research Program. This year the Program’s accomplishments include the following.

- Completed a pilot study of the costs of hematopoietic cell transplantation.
- Received extramural funding for 2 studies, one from the National Heart, Lung and Blood Institute and the other from the Patient Centered Outcomes Research Institute.
- Conducted more than 35 evaluation projects within NMDP’s Department of Patient Services.
- Hosted the System Capacity Initiative Year III Symposium to provide a resource to understand the current national capacity for performing HCT and address growing needs for the future.
- Provided technical writing support and developed easy-to-read consent forms for 3 clinical trial protocols, bringing the total number of supported clinical trials to 10.
- Published 5 papers in peer-reviewed journals and 1 in a lay news magazine.

![Distribution of Patients in the CIBMTR Database](chart.png)
Survey Research • The Survey Research Group was created to assist HCT researchers in developing and conducting research involving questionnaires and patient interviews. This year the Group’s accomplishments include the following.

- Supported 8 active studies.
- Participated in the development of 2 upcoming studies.

Additional Outcomes Research Initiatives •

- Continued collecting data for the Myelodysplastic Syndrome study, which qualifies for Coverage with Evidence Development under the Centers for Medicare and Medicaid Services; this mechanism allows eligible patients on the study to receive Medicare or Medicaid reimbursement for transplantation.
- Collaborated with industry partners on various studies, such as long-term follow-up of new therapies, drug comparisons, and assessment of target populations for new therapies.

PROSPECTIVE RESEARCH PROGRAM

The CIBMTR participates in and supports a number of clinical trial activities in various ways, including providing data management resources, access to its research database and statistical expertise.

Blood and Marrow Transplant Clinical Trials Network • Along with the NMDP and The EMMES Corporation, a contract research organization, the CIBMTR serves as the Data and Coordinating Center for the Blood and Marrow Transplant Clinical Trials Network. The Network’s accomplishments over the past year include the following.

- Opened one new trial to accrual, bringing the total number of launched trials to 29.
- Accrued 510 patients to trials, increasing the total number of accrued patients to 5,351.
- Managed 10 open protocols with overall accrual at 104% of projections.
- Published 6 peer-reviewed manuscripts.
- Presented 7 abstracts of study results at national and international meetings.

Resource for Clinical Investigations in Blood and Marrow Transplantation • The Resource for Clinical Investigations in Blood and Marrow Transplantation was developed to provide a multi-center clinical trials and prospective observational research mechanism complementary to the Blood and Marrow Transplant Clinical Trials Network. The Resource's accomplishments over the past year include the following.

- Managed 5 open protocols, which accrued 4,187 patients.
- Released 2 new protocols to sites; these protocols focus on a novel mobilizing agent for related donors and the use of a novel preparative regimen drug for children.
- Continued the development of 2 other protocols.
- Supported 4 studies involving unrelated donor data or sample collection for investigators.

Transplant Graft Types

- **Aliogeneic**
  - Peripheral Blood (77,116)
  - Bone Marrow (98,723)
  - Cord Blood (11,028)

- **Autologous**
  - Bone Marrow (15,866)
  - Cord Blood (26)

- **Combined**
  - Peripheral Blood (114,589)
  - Bone Marrow (231,569)
  - Cord Blood (11,054)
IMMUNOBIOLOGY RESEARCH PROGRAM
The CIBMTR’s Immunobiology Program is facilitated by its Immunobiology Working Committee, functioning in collaboration with the NMDP Histocompatibility Advisory Group. This year the Program accomplished the following activities.

- Published 9 manuscripts in peer-reviewed journals.
- Presented 7 abstracts at scientific meetings.
- Reviewed and accepted 9 new project proposals.
- Distributed 9,882 samples to investigators for 14 different studies.
- Curated 12,482 samples in the NMDP Repository (106,788 overall):
  - 4,353 unrelated recipient samples (46,228 overall)
  - 760 related recipient samples (2,509 overall)
  - 4,901 adult unrelated donor samples (48,199 overall)
  - 745 related donor samples (2,362 overall)
  - 1,723 unrelated cord blood samples (7,490 overall).

STATISTICAL METHODOLOGY RESEARCH PROGRAM
The Statistical Methodology Research Program’s development goals are two-fold: develop statistical models to use in HCT research and compare the models with existing solutions using the research database. This year the researchers published 3 manuscripts in peer-reviewed journals.

STEM CELL THERAPEUTIC OUTCOMES DATABASE
Since 2006, the CIBMTR has held a Health Resources and Services Administration contract, authorized by the C.W. Bill Young Cell Transplantation Program. Through this contract, the CIBMTR created and manages the Stem Cell Therapeutic Outcomes Database, a national outcomes registry for allogeneic transplants performed in the US. The contract was renewed in 2012, and this year the CIBMTR accomplished the following related to the contract.

- Coordinated the third Center-Specific Outcomes Analysis Forum, which generated recommendations for improved clinical and statistical approaches to center-specific analysis.
- Published the first center-specific analysis to include 3 full years of data on both related and unrelated donor transplantation in the US.
- Continued to build the related sample repository by collecting pre-HCT biologic specimens of related donor recipient pairs from 37 centers.
- Doubled accrual on a quality of life pilot project, which is unique in that CIBMTR collects data directly from patients after HCT.

DATA MANAGEMENT
The CIBMTR collects data for ~18,000 new HCT recipients annually as well as follow-up data on previously reported transplants. To collect and share data in an efficient manner while ensuring data quality, the CIBMTR accomplished the following over the past year.

- Audited 61 centers with an overall error rate of 2.5%, compared to the CIBMTR standard of 3%.
- Continued the Continuous Process Improvement program with both US and international centers to ensure quality data is submitted within specified time frames. As of the May 2013 report, 90% of US centers met CPI standards.
- Maintained Institutional Review Board approval of the research database by 97% of active US transplant centers. Currently, there are only two centers that do not have Institutional Review Board approval for the research database.
- Published a monthly newsletter for data managers as another means of communicating timely information to the network.
INFORMATION TECHNOLOGY SERVICES
The CIBMTR Information Technology team manages CIBMTR data and information, adapts commercial software products, develops custom software and provides technical support.

FormsNet • More than 95% of data collected by the CIBMTR is submitted electronically via FormsNet™, a comprehensive electronic data submission system launched in 2007. FormsNet contains >240 forms related to capturing HCT outcomes for donors and recipients. This year the Information Technology team accomplished the following.
• Released a major upgrade, FormsNet3, in December. This upgrade improved user experience, data capturing and validation.
• Enhanced the FormsNet3 application in March with additional functionality and data quality improvements.
• Received 186,266 HCT recipient forms.
• Documented, produced and released 15 new reports to the Management Reporting Website to assist management oversight and clinical trials support.

AGNIS • A Growable Network Information System (AGNIS) is a critically important complimentary application that allows data to flow from other databases into FormsNet as well as back to centers. This functionality allows centers to submit data from a local database without manual re-entry. This year the Information Technology team accomplished the following.
• Increased the number of AGNIS production users from 4 to 6. Two of the production users are vendors who support multiple clients. One production user is a registry that supports 48 centers.
• Added 2 forms to the list of forms currently available for production use via AGNIS.
• Mapped all AGNIS-supported forms from the European Group for Blood and Marrow Transplantation, facilitating exchange of data with European registries and cord blood banks.

DISCO • The CIBMTR migrated to a new customer relationship system, called DISCO (Data and Information for Statistical Center Operations), in December. This new system improves accuracy, collaboration with the research community and process efficiencies surrounding the CIBMTR research activities by accomplishing the following.
• Organizes and tracks contacts, communications, surveys, mass mailings and newsletters.
• Manages information for studies, publications, authors, working committees and clinical trials.

CIBMTR Data Warehouse • The CIBMTR Data Warehouse strategy will result in a new, enterprise-wide solution for managing data that will dramatically improve access to data, improve data quality and confidence and reduce the time and effort to complete studies.
• Began implementing the first Data Warehouse release, which will deliver key reports and dashboards that will provide process improvement and automation, eliminate manual processes and improve data quality.
• Continued the migration of legacy data from the NMDP and IBMTR databases into the CIBMTR research database in order to improve the quality and completeness of data available to statisticians.

SOURCES OF REVENUE
Fiscal Year ending June 30, 2013
Total = $23.9 Million

- Health Resources and Services Administration 7%
- National Institutes of Health 18%
- National Marrow Donor Program 34%
- US Navy 25%
- Other 6%
- Other Grants / Contracts 10%

Contributions 1%
BMT Tandem Meetings 3%
Medical College of Wisconsin 2%
OTHER ACCOMPLISHMENTS

BMT Tandem Meetings • The 2013 BMT Tandem Meetings, the CIBMTR’s joint annual meeting with the American Society for Blood and Marrow Transplantation, were held Feb. 12-16 in Salt Lake City, UT. The CIBMTR accomplished the following at this year’s meetings.
• Hosted 2,823 attendees at this first-time venue, almost 300 more attendees than the previous record high.
• Received 553 abstracts from investigators representing 31 countries.

Data Management Meetings • The CIBMTR held 2 educational meetings over the past year for Clinical Research Professionals and Data Managers who complete data forms for submission to the CIBMTR database. The goals were to educate data professionals and create opportunities for interaction with the CIBMTR.

Consumer Advocacy Committee • The Consumer Advocacy Committee provides valuable patient and donor perspectives during the development of the CIBMTR’s research agenda and helps present CIBMTR research outcomes to the public. This year the committee met in person at the BMT Tandem Meetings and helped develop 11 patient-friendly synopses of published research studies, which were posted on the CIBMTR website.

Worldwide Network for Blood and Marrow Transplantation • The CIBMTR is a founding society member of the Worldwide Network for Blood and Marrow Transplantation. In the past year, the CIBMTR has supported the organization’s following accomplishments.
• Developed a Latin American Blood and Marrow Transplant Society.
• Published guidelines for minimal datasets regarding donor data and outcomes.