

CIBMTR Communication Packet: Resources for potential centers



The CIBMTR collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and other cellular therapy worldwide to increase survival and enrich quality of life for patients. To learn more, please visit our [About CIBMTR page](#).

Join our worldwide network. Center Membership offers many advantages, most notably the opportunity to join a large network of physicians and clinical research professionals around the world who share the goal of advancing the science of cellular therapies. Visit our [Center Membership page](#).

Engage with us

CIBMTR centers have access to many training opportunities. They include [New Data Management Onboarding](#) classes every six-months, online training, and our yearly Clinical Research Professionals/Data Management meeting during the annual [Transplantation & Cellular Therapy Meetings](#).

The CIBMTR's [System Applications](#) provide options for reporting and engaging with CIBMTR. In addition, we recently launched CIBMTR Center Support, a platform to optimize center support. [CIBMTR Center Support](#) delivers a transparent, flexible, and service-oriented experience for Centers.

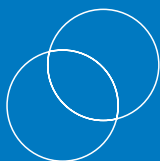
You will also gain access to our Data Operations resources, including our weekly Data Operations eBlast, quarterly Data Management Newsletter, Forms Instruction Manual, Data Management Guide, Audit guide, and much more.

Join now

Becoming a CIBMTR center is simple. The Path to Becoming a Member chart provides a high-level view of the steps and requirements. Additional resources for potential centers include our contracting requirements, and IRB [Protocols and Consent Forms](#). Visit our FAQ Document or contact [cibmtr-centermaintenance](#) for more information.

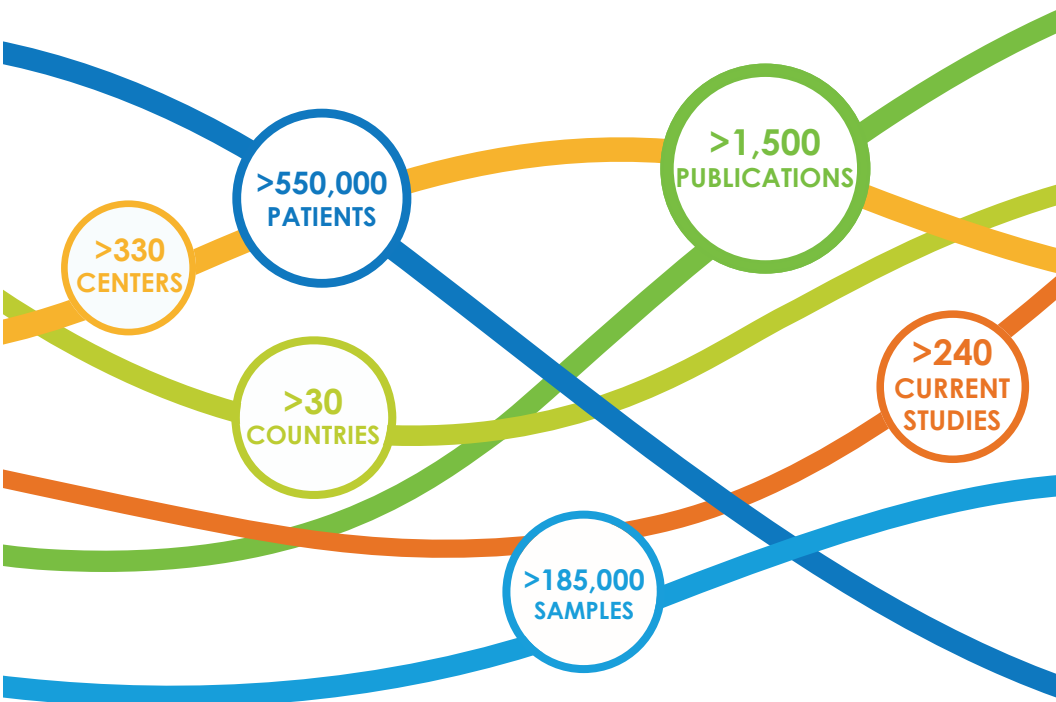
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CIBMTR[®]

CENTER FOR INTERNATIONAL BLOOD
& MARROW TRANSPLANT RESEARCH



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**

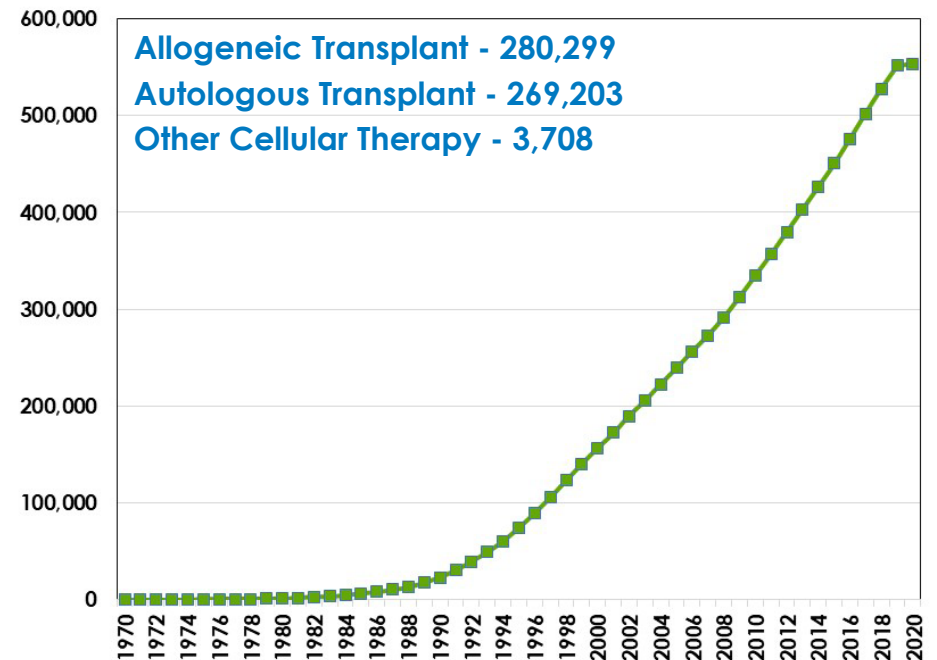


Biospecimen **REPOSITORY**

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



50 Years



>550,000 Patients



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



570 Scientific Authors



300 Institutions



53 Publications 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

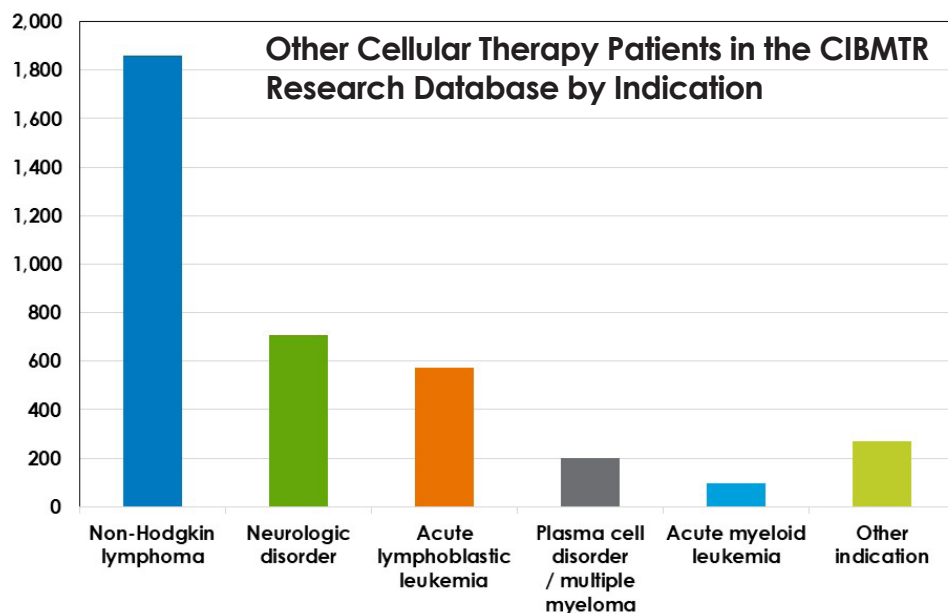
- **Transplant center volumes data:** Includes transplants performed 2013-2017
- **Center-specific survival analysis:** Includes first allogeneic transplants performed 2015-2017
- **18 research summaries:** Plain language summaries of research publications created for patients and their loved ones



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



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 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



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
- **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



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
- **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



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 uncultured 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



24 Studies in progress



3,367 Patients
accrued this year



1 Publication this year

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



13 Trials in progress



>300 Patients
accrued this year



15 Publications this year

Key Publications

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.

Ghosh N, Ahmed S, Ahn KW, et al. **Association of reduced-intensity conditioning regimens with overall survival among patients with NHL undergoing allogeneic transplant.** JAMA Oncology. Epub 2020 Jun 4. PMC7273311. [Epub ahead of print].

Petersdorf EW, Bengtsson M, De Santis D, et al. **Role of HLA-DP expression in GVHD after unrelated donor transplantation.** Journal of Clinical Oncology. Epub 2020 Jun 1. [Epub ahead of print].

Petersdorf EW, Stevenson P, Bengtsson M, et al. **HLA-B leader and survivorship after HLA-mismatched unrelated donor transplantation.** Blood. Epub 2020 Jun 1. [Epub ahead of print].

Nikiforow S, Mansour M, Hu Z-H, et al. **Tocilizumab not associated with increased infection risk after CAR T - Implications for COVID-19?** Blood. Epub 2020 May 25. [Epub ahead of print].

Mehta RS, Holtan SG, Wang T, et al. **Composite GRFS and CRFS outcomes after adult alternative donor HCT.** Journal of Clinical Oncology. 2020 Jun 20; 38(18):2062-2076. Epub 2020 May 4. PMC7302955.

Fatobene G, Rocha V, St Martin A, et al. **Nonmyeloablative alternative donor transplantation for Hodgkin and non-Hodgkin lymphoma: From the LWP-EBMT, Eurocord, and CIBMTR.** Journal of Clinical Oncology. 2020 May 10; 38(14):1518-1526. Epub 2020 Feb 7. PMC7213591.

Hourigan CS, Dillon LW, Gui G, et al. **Impact of conditioning intensity of allogeneic transplantation for AML with genomic evidence of residual disease.** Journal of Clinical Oncology. 2020 Apr 20; 38(12):1273-1283. Epub 2019 Dec 20. PMC7164487.

Atallah E, Logan BR, Chen M, et al. **Comparison of patient age groups in transplantation for MDS: The Medicare coverage with evidence development study.** JAMA Oncology. 2019 Dec 12; 6(4):486-493. Epub 2019 Dec 12. PMC6990739.

Meyer C, Mau L-W, Murphy EA, et al. **Addressing knowledge gaps in AML to improve referral for HCT consultation.** Journal of the National Comprehensive Cancer Network. 2019 Dec 1; 17(12):1473-1481. Epub 2019 Dec 1.

Ballen K, Logan BR, Chitphakdithai P, et al. **Unlicensed umbilical cord blood units provide a safe and effective graft source for a diverse population: A study of 2,456 umbilical cord blood recipients.** Biology of Blood and Marrow Transplantation. 2020 Apr 1; 26(4):745-757. Epub 2019 Nov 19.

Pidala J, Hamadani M, Dawson P, et al. **Randomized multicenter trial of sirolimus vs prednisone as initial therapy for standard-risk acute GVHD: The BMT CTN 1501 trial.** Blood. 2020 Jan 1; 135(2):97-107. Epub 2019 Nov 18. PMC6952830.

Sapir-Pichhadze R, Zhang X, Ferradji A, et al. **Epitopes as characterized by antibody-verified eplet mismatches determine risk of kidney transplant loss.** Kidney International. 2020 Apr 1; 97(4):778-785. Epub 2019 Nov 12.

Pidala J, Martens M, Anasetti C, et al. **Factors associated with successful discontinuation of immune suppression after allogeneic HCT.** JAMA Oncology. 2019 Sep 26; 6(1):e192974. Epub 2019 Sep 26. PMC6763979.



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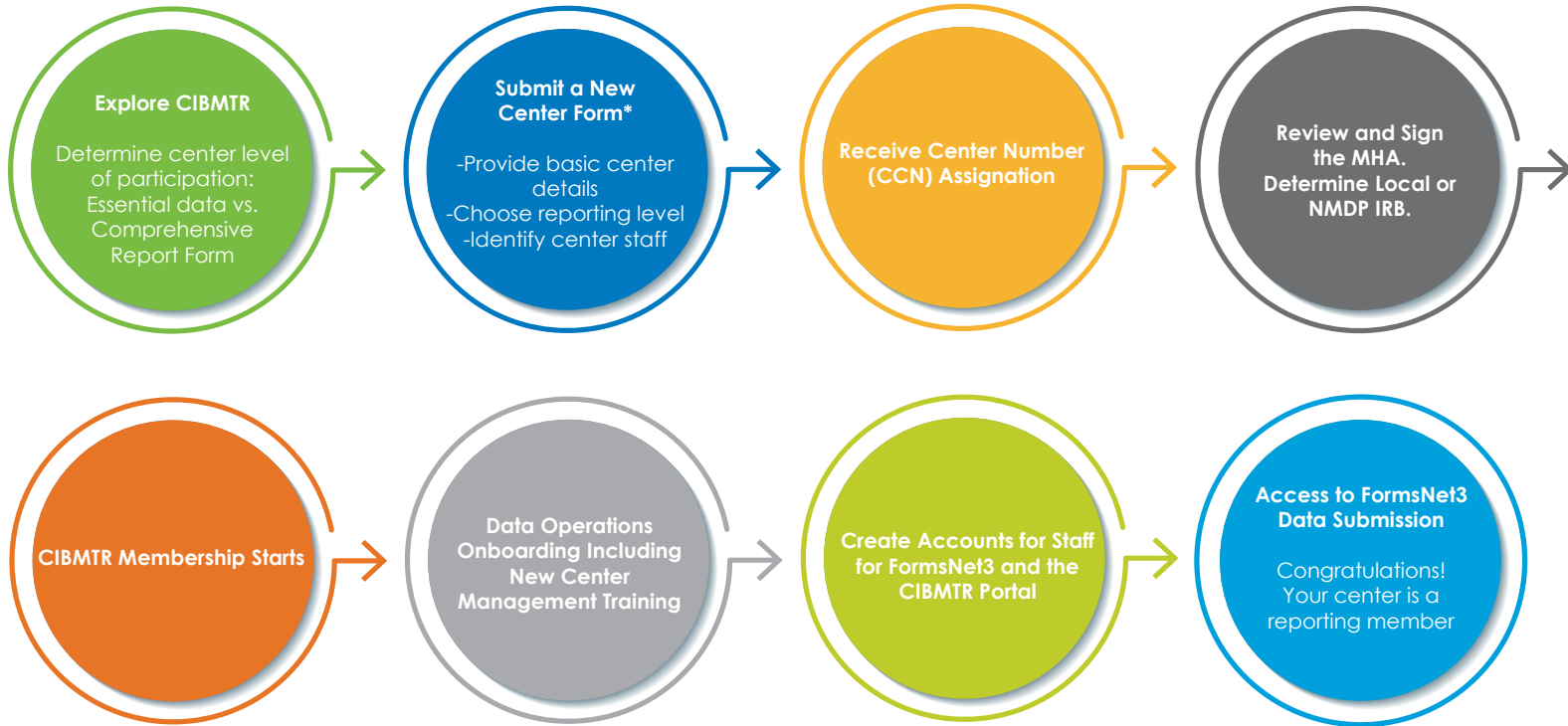
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New Center Onboarding: Roadmap



CIBMTR Membership: New Center FAQ



How does our organization become a CIBMTR center?

There are five basic steps to becoming a CIBMTR center:

- Submit the CIBMTR New Center Information Form
 - Provide basic center details
 - Choose reporting level
 - Identify center staff
- Sign the MHA data and sample sharing agreement
- Obtain IRB approval for the research database and/or sample repository protocols
- Complete new center data management training
- Create accounts for staff for FormsNet3 and CIBMTR Portal

What is an MHA?

The Master Healthcare Data and Sample Submission Agreement (MHA), is an agreement that defines the responsibilities for both the CIBMTR and the center. The MHA is important because it identifies how data and/or samples may be used, informed consent requirements, privacy, confidentiality, and other responsibilities for both parties. The agreement covers the mandated reporting under the C.W. Bill Young Cell Transplantation Program for United States products (SCTOD reporting later defined), as well as the Data and or Samples submitted for participation in the CIBMTR Protocols.

How long does it take to become a CIBMTR center?

From the time the New Center Information Form is submitted, it takes up to five business days for the MHA to be prepared for the new center. Once the MHA is sent to the center, the center has an opportunity to ask questions and propose edits to the MHA Agreement. That can take as little or as much time as needed. It is difficult to predict that portion of the timeline. Training for new centers is self-guided and takes approximately one week. Training must be completed before the accounts are created for center staff to begin data entry. Creating the accounts takes only a day or two. The first day the primary data manager's account is created and then CIBMTR staff will do a brief training with that person and walk them through creating the accounts for other people at the center. An account is created for every person who will need access to FormsNet3, the CIBMTR Portal, or CIBMTR Center Support.

What training is offered for new centers?

We have an online, self-paced data management course for new users that must be completed prior to beginning to input data. Additional training is available as needed with our CIBMTR Center Support team.

What are the CIBMTR levels of participation?

CIBMTR offers two levels of participation:

Transplant Essential Data (TED) only Centers receive all CIBMTR general mailings, including our newsletter and summary slides, and pay member rates for attending CIBMTR's annual meeting. These centers report only TED-level forms.

Comprehensive Report Form Centers receive all the benefits of TED only centers, plus members may chair CIBMTR Working Committees, may be members of the Executive Committee, and have voting privileges. CRF-level forms collect in-depth data for research purposes.

TED and CRF reporting tracks are primarily for hematopoietic cell transplant and gene therapy data collection. For other cellular therapy data collection, CIBMTR has developed the Cellular Therapy Essential Data (CTED) suite of forms.

CIBMTR holds the contract for the Stem Cell Therapeutic Outcomes Database (SCTOD), a component of the C.W. Bill Young Transplantation Program, awarded by the Health Resources and Services Administration of the U.S. Department of Health and Human Services.

As the contract holder, the CIBMTR is charged with collecting data on all allogeneic hematopoietic cell transplantations (HCTs) performed in the United States, and on all HCTs done with products procured through the Program but performed outside of the United States.

TED level data reporting is mandatory for all recipients and donors of allogeneic HCTs in the US. For more information on the SCTOD, please visit the [Health Resources and Service Administration site](#).

What is the difference between TED forms and CRFs?

TED stands for "Transplant Essential Data" and those forms capture just the basic information on the transplants. There is only one follow up form for each post-transplant time point.

CRF stands for "Comprehensive Report Form" and there is an algorithm in our FormsNet3 application that runs to select either TED forms or CRFs. Centers electing to do CRF reporting do not do CRFs for each case rather only for those for which CRFs are selected by the algorithm. Some studies also require that CRFs are completed. The CRFs have more detailed information than the TED forms and have disease-specific forms that are completed at each post-infusion time point.

Will selecting CRF-level reporting mean that I will do CRFs for all patients?

No, the algorithm that selects either TED track or CRF track will determine which type of forms will be required for each patient if the center has chosen CRF-level reporting. Some studies require completion of CRFs and all patients on the study would need CRFs to be completed even if the center has elected to do TED forms only.

Why do I need to complete finance paperwork if I selected TED-level reporting?

Centers are encouraged to complete and return their financial paperwork at the same time as their MHA review to ensure payment is not delayed. The financial paperwork includes but is not limited to submitting a copy of the center's applicable W8 or W9 and a Supplier Information Form. Centers are reimbursed for CRF and cellular therapy (CTED) forms as indicated on the [Fee Schedule](#).

When do centers get paid for submitting the forms?

Centers are reimbursed for forms completed within one year of the due date of the form. The due date is shown in the FormsNet3 application. Payments to U.S. centers are made quarterly by ACH (Automatic Clearing House, a type of bank transfer) and backup documentation for those payments is available on the CIBMTR Portal. Non-U.S. centers are paid one time per year between August and October.

What type of data management staff do we need to have?

The data management team at a center depends upon many variables. Some centers have specific individuals working as data managers at the centers. Many centers have their nursing staff, transplant coordinators or even attending physicians completing the data forms. This is especially true outside of the U.S.

If I want to be the chair of a committee what are the requirements?

To be eligible to chair a Working Committee it is necessary for the individual's center to be submitting CRF forms for, at minimum, one type of transplant.

What is CPI?

CPI stands for "Continuous Process Improvement" and is a program that requires centers to complete a pre-determined percentage of forms error free within a specific timeframe relative to the due dates of the forms. Additionally, it requires that centers have an MHA in place and, for U.S. centers, that their IRB approval is current. The program has been ongoing for U.S. centers for years and is being rolled out to other countries a few at a time. Currently Australia, Brazil and New Zealand participate in the pilot of international CPI.

What is CTA?

CTA stands for "Consecutive Transplant Audit" and is a list that is submitted each year to ensure that all transplants done at the center have been reported. It contains basic data about the transplant in order to know that the center has reported the data accurately on all transplants, including but not limited to date of birth, sex, transplant date, and transplant type. The information is submitted according to a template that is provided to the center as a guideline.

Why does my site need IRB approval?

The CIBMTR Research Database and Research Sample Repository protocols are human subjects research. To be compliant with United States Federal Regulations for human research subject protection, all participating transplant centers must have IRB approval and obtain IRB-approved informed consent from recipients, regardless of the level of data the center submits to CIBMTR.

How do I get IRB approval?

You may obtain approval through your institution's local IRB, or you may enroll as a site with the NMDP IRB. The CIBMTR has a single IRB (sIRB) through the National Marrow Donor Program (NMDP) that may review and approve this research for your center. If you have a local IRB, your local IRB may cede its oversight to the NMDP IRB. While enrolling in the sIRB takes some additional work up front, utilizing the NMDP IRB will likely reduce the time your center spends on study administration, since CIBMTR staff are responsible for most NMDP IRB submissions for this research.

Why might I want to use the NMDP single IRB?

Utilizing the NMDP IRB will likely reduce the time your center spends on study administration. After enrollment in sIRB, initial approval on the protocol typically takes less than two weeks. CIBMTR staff is responsible for submitting protocol amendments, consent form template updates, and annual updates to the NMDP IRB. If your center chooses to use your local IRB, your center will be responsible for all IRB submissions to your local IRB.

What is involved in getting NMDP IRB approval at my site?

To obtain initial approval for a study from the NMDP sIRB, your center must first enroll in the sIRB following. This only needs to occur once per institution. After sIRB enrollment is complete, your center may be added as a research site to the database and repository studies under the NMDP IRB.

The sIRB forms and instructions for enrollment are available on the NMDP IRB website at the following link (no special username/password required): <https://network.bethematchclinical.org/research/institutional-review-board/study-site-hrpp/irb-staff-forms/>

The NMDP Single IRB Manual for Local Institutions contains helpful information and step-by-step instructions regarding using the NMDP single IRB. This manual can be also be found at the above link.

How do I get started and who can I contact with questions about the IRB approval process?

Regardless of whether you would like to use your local IRB, the NMDP sIRB, or are unsure and would like more information, you may contact the study protocol coordinators at the emails addresses below.

- Research Database: DatabaseIRB@nmdp.org
- Sample Repository: RepositoryIRB@nmdp.org

Information for both protocols is also available on the CIBMTR website at the links below.

| Study | Website URL |
|------------|---|
| Database | https://www.cibmtr.org/DataManagement/Protocol-Consent/ObservationalData/pages/index.aspx |
| Repository | https://www.cibmtr.org/DataManagement/Protocol-Consent/ResearchSamples/pages/index.aspx |

If you have specific questions about the NMDP single IRB, you may contact the IRB directly at NMDPSIRB@nmdp.org.

The NMDP IRB's website also has useful information, including the NMDP Single IRB Manual for Local Institutions that contains step-by-step instructions for using the NMDP IRB.

NMDP IRB website: <https://network.bethematchclinical.org/research/institutional-review-board/study-site-hrpp/irb-staff-forms/>

How do I get involved in CIBMTR research?

Working Committees shape the observational research that leads to publications for CIBMTR. Through 15 focused committees, volunteer members can propose, design and implement studies. Everyone can be a member of a committee although leadership roles are limited to those centers reporting CRF-level data. You can find a list of our Working Committees on our website at our [CIBMTR Working Committees page](#).

Our center is ready to become a CIBMTR member. How do I get started?

Please submit a New Center Form to get started. Visit the [Center Membership](#) site to get started.