

[COVID-19 Updates](#)

[Quick Links](#)

[Patient Resources](#)

[Publication List](#)

[Newsletters](#)

[News Releases](#)

[Slides and Reports](#)

[Statistical Resources](#)

[CIBMTR 50th Anniversary](#)

[Get Involved](#)



February 2020 Newsletter

Volume 26, Issue 1

Table of Contents:

[Perspectives](#)

[Acute Leukemia Working Committee](#)

[Chronic Leukemia Working Committee](#)

[2020 TCT Meetings of ASTCT and CIBMTR in Orlando, FL](#)

[CIBMTR 2019 Annual Report](#)

[RCI BMT Announces Further Collaboration with the PTCTC](#)

[BMT CTN Meetings at the TCT Meetings of ASTCT and CIBMTR](#)

[State Medicaid Coverage for Allogeneic HCT for Patients with Sickle Cell Disease](#)

[Stem Cell Therapeutic Outcomes Database](#)

[Presentations and Form Revisions for the Cellular Immunotherapy for Cancer Working Committee](#)

[3 New Summaries for Patients](#)

[CIBMTR Trivia](#)

[CIBMTR on Social Media](#)

[Our Supporters](#)

[Abbreviations](#)

Perspectives

By Robert Soiffer, MD

Should a 55-year-old with AML receive myeloablative or reduced-intensity conditioning? Should all unrelated donor recipients receive ATG to prevent chronic GVHD? Should DLI be administered for falling donor chimerism? For how long should patients receive lenalidomide after autologous HCT for myeloma? Which agents should be paired with cyclophosphamide for haploidentical HCT? When should post-transplant vaccinations begin? Should patients receive prophylactic defibrotide for veno-occlusive disease prevention? Should azacytidine be administered to prevent relapse after allogeneic HCT for AML? Should we transplant patients with p53 mutations? How should we approach patients with evidence of minimal residual disease prior to transplant? What is the optimal therapy for steroid refractory GVHD?

The answers to these and many other fundamental questions are not set in stone. Opinions differ locally, and there are even more striking variations globally.



Sometimes this can be attributed to availability and affordability of new drugs, but even taking that out of the equation, practice patterns have clearly evolved in divergent directions across the continents. Treatment algorithms in Japan, Europe, and the US are distinct. Both retrospective registry analyses and prospective controlled studies often yield conflicting results and leave many of us scratching our heads.

Are different treatment pathways a consequence of genetic polymorphisms among ethnic populations? Do regional academic training philosophies dictate therapeutic choices? Does the local microbiome impact transplant outcomes and influence response to interventions? To address these issues, it is important to carefully catalogue practice variations among different geographic regions so we comprehensively study why approaches have evolved as they have and perhaps determine if global best practices can be adopted. The Worldwide Network for Blood and Marrow Transplantation (WBMT) has made strides towards this end, but we need to do more.

At both February's TCT Meetings of ASTCT and CIBMTR and March's EBMT Congress, the CIBMTR, ASTCT, and EBMT will jointly present a session aimed at benchmarking variations in both transplantation and immune effector cell therapies in North America and Europe. Hopefully this session will lay out a blueprint for collaborative international studies for the future. We should not blindly continue along parallel paths of care, and we must find a way, if appropriate, to merge our strategies to optimize patient outcomes.

With these parting comments, my tenure as the Chair of the CIBMTR Advisory Committee draws to a close. I will hand over my responsibilities to John Wingard, MD, who has unfailingly exhibited remarkably wise, even-handed leadership at ASTCT, NMDP/Be The Match, and BMT CTN over the years. I have no doubt he will continue to help the CIBMTR promote critical collaborations with our international registry partners that will lead to scientific advancement and optimization of clinical care across the globe. I also want to thank the remarkable team at the CIBMTR and, of course, its driving force, Mary Horowitz, MD, MS, for their dedication to our mission.

See you in Orlando.

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

[Acute Leukemia Working Committee](#)

Committee Leadership

Co-Chairs



[Mark R. Litzow, MD](#), Mayo Clinic, Rochester, MN



[Partow Kebriaei, MD, MD](#), Anderson Cancer Center, Houston, TX



[Brenda M. Sandmaier, MD](#), Fred Hutchinson Cancer Research Center and University of Washington, Seattle, WA

Scientific Directors

Statistical Director

Statistician



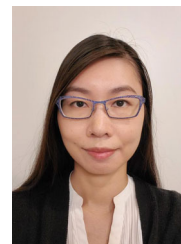
[Daniel Weisdorf, MD](#), CIBMTR
Minneapolis



[Wael Saber, MD, MS](#), CIBMTR
Milwaukee



[Mei-Jie Zhang, PhD](#), CIBMTR Milwaukee



[Karen Chen, MS](#), CIBMTR Milwaukee

Acute leukemia remains the most common indication for allogeneic HCT. With increasing use of molecular profiling to help inform therapy and risk prognostication, the CIBMTR added questions to the 2013 Pre-TED forms regarding cytogenetics and molecular markers to allow for more informative and relevant transplant registry studies. Furthermore, the CIBMTR added minimal residual disease (MRD) test details including flow cytometry, cytogenetics, and fluorescence in situ hybridization (FISH) to the 2017 CRFs. Discussions are ongoing as to the best format for incorporating newer markers of pre- and post-HCT MRD, including next-generation sequencing and polymerase chain reaction. Finally, the emergence of post-HCT therapies to prevent relapse has added another setting for data collection. We expect many debates and analyses proposed on these and other topics in the Acute Leukemia Working Committee with the collection of data about post-HCT maintenance on 2017 post-TED forms. HCT indications, approaches, and comparison with other therapies are all valid topics for study.

The success of the Acute Leukemia Working Committee derives predominantly from a dedicated team with collaborative input. Committee leadership works closely with committee members, data managers, and transplant groups in both the US and abroad. Committee leadership develops and promotes the scientific agenda established with input from committee members; determines priorities in the selection of high-impact studies; and ensures timely progress in protocol development, statistical analyses, manuscript development, and publications. The committee strives to improve quality and efficiency and is guided by the three principles established by the CIBMTR Advisory Committee: Publish peer-reviewed papers of high scientific impact, complete studies within a reasonable time period, and ensure inclusiveness and fairness within the study process.

The Acute Leukemia Working Committee's recent academic activity includes four presentations at the 2019 ASH Annual Meeting and five submitted / accepted manuscripts in 2019. In addition, the committee received 47 proposals in anticipation of the 2020 TCT Meetings of ASTCT and CIBMTR. These numbers reflect a rapidly evolving field and a high-level of interest in the rich data on acute leukemia within the CIBMTR Research Database. As a committee, we are eager to review and discuss many new proposals from centers worldwide.

Number of Cases in the CIBMTR Research Database

	TED-Level Data	CRF-Level Data
AML Autologous HCT	6,160	2,525
AML Allogeneic HCT	56,960	35,027
ALL Autologous HCT	1,216	507
ALL Allogeneic HCT	28,323	19,721

View planned, in-progress, and completed studies and publications on the [Acute Leukemia Working Committee webpage](#).

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

Co-Chairs



[Ryotaro Nakamura, MD](#),
City of Hope, Duarte, CA



[Bart Scott, MD](#), Fred
Hutchinson, Seattle, WA



[Ronald Sobeks, MD](#),
Cleveland Clinic,
Cleveland, OH

Scientific Director



[Wael Saber, MD,
MS](#), CIBMTR
Milwaukee

Statistical Directors



[Kwang Woo Ahn, PhD](#),
CIBMTR Milwaukee



[Soyoung Kim, PhD](#),
CIBMTR Milwaukee

Statistician



[Noel Estrada-Merly,
MS](#), CIBMTR Milwaukee

The main goals of the Chronic Leukemia Working Committee are to help establish the optimal timing of HCT for patients with MDS, CML, CLL, and myeloproliferative neoplasms (MPN) and to improve HCT outcomes for such patients. During the last several years, the efforts of the committee resulted in several published manuscripts as well as oral and poster presentations. The committee recently revised the MDS data collection forms to better capture molecular and disease specific data of prognostic importance. We also created a new separate series of forms for MPN disorders. In addition, these forms now incorporate more recent novel therapies, which will help generate important future studies for the committee to pursue. To help foster new study proposals, the committee not only provides our members with disease-specific lists of accepted studies but also with prior proposals that were not accepted and the rationale for the decision. This information allows investigators to focus their new study proposals on concepts that are feasible and unique. A few of the recently published studies are described below.

Saurabh Chhabra, MD, MS, led an important study (CK15-02) to determine whether reduced-intensity / nonmyeloablative conditioning (RIC) allogeneic-HCT and myeloablative conditioning (MAC) result in similar outcomes in CML patients in the era of TKI. Multivariable analysis showed no significant difference in overall survival, leukemia-free survival, or non-relapse mortality between MAC and RIC groups. Compared with MAC, the RIC group had a higher risk of early relapse after allogeneic HCT (HR, 1.85; $P = .001$). The cumulative incidence of chronic GVHD was lower with RIC than with MAC (HR, 0.77; $P = .02$).

It remains unknown whether the administration of TKIs targeting BCR-ABL1 after allogeneic HCT is associated with improved outcomes for patients with CML. Zachariah DeFilipp, MD, led a study (CK16-02a), which analyzed clinical outcomes of adult patients with CML who underwent HCT and received maintenance TKI following HCT compared with no TKI maintenance. As measured from day +100 (landmark analysis), the adjusted estimates for 5-year relapse (maintenance, 35% versus no maintenance, 26%; $P = .11$), leukemia-free survival (maintenance, 42% versus no maintenance, 44%; $P = .65$), or overall survival (maintenance, 61% versus no maintenance, 57%; $P = .61$) did not differ significantly between patients receiving

TKI maintenance or no maintenance. These results remained unchanged in multivariate analysis.

Sonali Chaudhury, MD, led another important study of CML in children and young adults as they have long life expectancies and low morbidity with HCT (CK13-01). Prolonged TKI use may cause significant morbidity. In addition, indication for HCT in patients in first chronic phase is not established. This study found that in the current era of TKI therapy, HCT outcomes are similar in young patients and children with early CML, and best outcomes are achieved with bone marrow grafts and matched sibling donors.

For patients with hematologic malignancies undergoing allogeneic HCT, UCBT has become an acceptable alternative donor source in the absence of a matched sibling or unrelated donor. However, there have been few published series describing the outcomes of adult patients with MDS who have undergone UCBT. Aaron T. Gerds, MD, MS, thus led a study (CK14-01) to evaluate the outcome of UCBT in MDS. The probability of relapse and transplant-related mortality at 3 years was 32% and 40%, respectively, leading to a 3-year, disease-free survival of 28%, and overall survival of 31%.

Given prior reports of specific HLA alleles impacting the incidence of CLL and clinical outcomes of allogeneic HCT for CLL, Brian T. Hill, MD, PhD, and the protocol team sought to study the overall survival and progression free survival of a large cohort of CLL patients who underwent HCT from fully HLA matched related and unrelated donors (CK12-02b). The study found no statistically significant association of allogeneic HCT outcomes for CLL based on previously reported HLA combinations.

Additionally, there are many ongoing studies. For CML, our committee is evaluating the optimal timing of HCT in the era of TKI (CK12-01) and benefit of DLI (CK16-02b). For MDS, our studies include identification of germline predisposition mutations in young MDS patients (CK16-01), effect of specific conditioning regimens on RIC HCT outcomes (CK17-02), development of personalized prediction model for HCT outcomes (CK18-01), impact of donor age (CK18-03), and alternative donor vs. matched donor HCT (CK19-02). We are also actively studying MPN / myelofibrosis, including outcomes of HCT in AML arising from MPN (CK15-03), comparison of HCT vs. non-HCT therapies in myelofibrosis (CK15-01), development of a prognostic scoring system to predict HCT outcomes for myelofibrosis (CK17-01), and impact of somatic mutations in chronic myelomonocytic leukemia (CK18-02). Lastly, CK19-01 is evaluating outcomes of HCT in patients with rare chronic leukemias, such as chronic neutrophilic leukemia and T-cell prolymphocytic leukemia.

The committee welcomes new participants as well as new proposals. We also encourage collaboration with other committees and the use of outside data sets, which can better define the role and timing of transplantation as new non-transplant strategies emerge. We encourage young investigators to take part in this committee, which provides an excellent opportunity to become familiar with novel study designs for observational research and the statistical methodologies utilized. The next in-person meeting of the Chronic Leukemia Working Committee is at the TCT Meetings of ASTCT and CIBMTR in Orlando, FL, this month. We look forward to seeing you there and welcoming new proposals.

View planned, in-progress, and completed studies and publications on the [Chronic Leukemia Working Committee webpage](#).

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

2020 TCT | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR in Orlando, FL

By Tia Houseman



The TCT | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR are the combined annual meetings of the ASTCT and CIBMTR. This has been North

America's largest international gathering of administrators, clinicians, data manager / clinical research professionals, fellows-in-training, investigators, laboratory technicians, MD / PhDs, nurses, nurse practitioners, pharmacists, physician assistants, and other allied health professionals in HCT and cellular therapy since 1995.

Join us February 19-23, 2020, at the World Center Marriott in Orlando, Florida, for the TCT Meetings of ASTCT and CIBMTR. This year we celebrate the 25th anniversary of ASTCT and CIBMTR joining together to hold their meetings, previously known as the BMT Tandem Meetings.

Last Call: Register Today

Go to the [2020 TCT Meetings of ASTCT and CIBMTR Home Page](#) to register and view additional details.

A Program you Won't Want to Miss

Leading cellular therapy experts from around the world will present the latest developments in the field during 6 plenary sessions, 9 concurrent sessions, 13 oral abstract sessions, a pediatric BMT program, and more.

We invite you to join us for the Best Oral Abstract Session in the afternoon of Friday, February 21, in Cypress 3.

Please join us after the Best Abstracts Session for the ASTCT & CIBMTR Awards Ceremony as the CIBMTR Distinguished Service Award is presented to *Jong Wook Lee, MD, PhD*. Following the awards, the Mortimer M. Bortin Lecture will be presented by *Shinichiro Okamoto, MD, PhD*, and the E. Donnal Thomas Lecture will be presented by *Effie W. Petersdorf, MD*.

In addition to an outstanding scientific program, the 2020 TCT Meetings of ASTCT and CIBMTR will offer parallel sessions for pharmacists, center administrators, coordinators, investigators, medical directors, clinical research professionals / data managers, transplant nurses, and advanced practice professionals. Along with state-of-the-art educational offerings, industry-supported satellite sessions and product theaters will broaden the spectrum of presentations.

Networking Opportunities

Attend several different networking opportunities during the TCT Meetings of ASTCT and CIBMTR, including poster sessions on Wednesday and Saturday evenings, the TCT Meetings of ASTCT and CIBMTR Networking Reception on Thursday evening in the Exhibit Hall - Cypress 2, the TCT Meetings of ASTCT and CIBMTR 25th Anniversary Celebration Saturday evening, and more.



Saturday, Feb. 22 @ 7:45 PM

Don't miss the TCT Meetings of ASTCT and CIBMTR 25th Anniversary Celebration Saturday evening beginning at 7:45 PM. Grab your feathers, fedoras, roaring 20s attire, and dancing shoes to join in the celebration with colleagues and friends! Tickets go fast, so reserve your ticket now through registration or purchase one on-site at the TCT Meetings of ASTCT and CIBMTR registration desk located within the World Center Marriott – Palms.

Plan Ahead

The TCT Meetings of ASTCT and CIBMTR agenda can be found on the TCT Meetings of ASTCT and CIBMTR home page [Agenda Tab](#).

On the right side, in the middle of the page, click on the tab that says *To view the full 2020 TCT Meetings of ASTCT and CIBMTR Agenda "click here"* to view the agenda by day, by track, or to search specific topics.

Sign in to build your personal agenda, search to find topics of interest, and more.

TCT Meetings of ASTCT and CIBMTR Mobile App

You will soon be able to download the official TCT Meetings of ASTCT and CIBMTR mobile app for quick and easy access to the current schedule, venue information, and more! If you already created a personalized schedule within the online app, it will transfer to your mobile app. Watch for additional details coming

via e-mail. The app is free for all attendees, and with wi-fi available throughout all TCT Meetings of ASTCT and CIBMTR rooms, users can:

- View and search the meeting program schedule
- Vote / participate in interactive sessions
- Complete session surveys
- Search for speakers
- Check out who is exhibiting and find their booth on a map
- Create a personal schedule
- Message other attendees
- Access other meeting information

Support Opportunities and Additional Information

Questions regarding support opportunities at the 2020 TCT Meetings of ASTCT and CIBMTR may be directed to the TCT Meetings of ASTCT and CIBMTR Conference Office: TCTMeetings@mcw.edu.

In celebration of the 25th Anniversary, if you have previous year's meeting photos that you would like to display and share, please email them to TCTMeetings@mcw.edu or share them online using the hashtags #TCTM20 and #CelebrateTCTM25

We look forward to seeing you in Orlando, Florida!

Join the conversation: #TCTM20

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

CIBMTR 2019 Annual Report

The [CIBMTR 2019 Annual Report](#) is now available to view on the [Administrative and Progress Reports webpage](#).

This document explains who we are, what we do, how we share knowledge, how we collect and manage data, and the impact we make on the field.

Review the electronic version to access links directly, or pick up a hard copy at the CIBMTR booth at the TCT Meetings of ASTCT and CIBMTR.

Email contactus@cibmtr.org if you would like a printed copy or copies mailed to you.



The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

RCI BMT Announces Further Collaboration with the PTCTC

By Erin Leckrone

The RCI BMT is pleased to announce further collaboration with the Pediatric Transplant and Cellular Therapy Consortium (PTCTC), formerly known as the Pediatric Blood and Marrow Transplant Consortium (PBMTC). The RCI BMT will continue to operationally oversee certain individual PTCTC trials (currently 5 active studies) while now also providing overall program management support to the consortium.

The RCI BMT's PTCTC program manager will work closely with the PTCTC Executive Committee Chair, Leslie Kean, MD, PhD. In collaboration with CIBMTR IRB staff and NMDP/Be The Match contracts and finance teams, the RCI BMT will oversee strategy, process oversight, data and safety and monitoring assistance, and contracting and budgeting. The RCI BMT's collaboration with PTCTC also includes administrative oversight of the steering committee and overall meeting organization of various PTCTC committees and strategy groups.

To date, the PTCTC includes more than 100 pediatric BMT centers in the US, Canada, New Zealand, and Australia. It also has affiliated members located in

Europe, Asia, and South America. The PTCTC is a core member of the NIH-funded BMT CTN and has a close relationship with the Children's Oncology Group. The PTCTC collaborates with the ASTCT and CIBMTR by hosting a session at the annual TCT Meetings of ASTCT and CIBMTR focused on cutting-edge science and advances in treatment related to the unique and specific needs of children and adolescents in HCT.

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

BMT CTN Meetings at the TCT Meetings of ASTCT and CIBMTR

By Amy Faley

New BMT CTN Leadership

The BMT CTN Steering Committee is now under the leadership of Helen Heslop, MD, DSc(Hon), (Baylor College of Medicine) who started her two-year Chair term on January 1. Edward Stadtmauer, MD, (University of Pennsylvania) is Chair-Elect, and Richard Jones, MD, (Johns Hopkins) is now Immediate Past-Chair.

See you at the TCT Meetings of ASTCT and CIBMTR!

Mark your meeting calendars for the **BMT CTN Investigators Meeting**:

- Thursday, February 20
- 7:00 – 8:30 AM ET
- World Center Marriott – Palms: Canary 3

Dr. Heslop is moderating this session, which is open to all attendees. She will highlight BMT CTN cellular therapies, including haplo NK cells (BMT CTN 1803; Sumithira Vasu, MD, MBBS); dendritic cell / myeloma fusions (BMT CTN 1401; David Avigan, MD); anti-B cell maturation antigen CAR T-cells (BMT CTN 1901 and 1902; Alfred Garfall, MD, MS, and Natalie Callander, MD); and HIV-specific T-cells (BMT CTN 1903; Kieron Dunleavy, MD). We hope you are able to join us!

The **BMT CTN Coordinators Meeting** will be held on Wednesday, February 19, from 8:30 – 5:30 PM ET in Grand: Salon 14. The meeting will cover BMT CTN processes and study overviews and, as always, will feature presentations from several BMT CTN investigators. We hope to see all of the BMT CTN study coordinators there!

Don't miss the BMT CTN 1101 (double cord vs. haplo) study results presented in the **Late Breaking Abstracts** session:

- Sunday, February 23 at 12:00 PM ET
- World Center Marriott – Palms: Royal
- Claudio Brunstein, MD, PhD
- *Results of Blood and Marrow Transplant Clinical Trials Network protocol 1101 a multicenter Phase III randomized trial of transplantation of double umbilical cord blood vs. HLA-haploidentical related bone marrow for hematologic malignancy.*

For the full schedule of BMT CTN abstracts being presented, follow us on Twitter: @BMTCTN

Also, please stop by the **BMT CTN booth**. BMT CTN investigators and data and coordinating center staff will be there during breaks to answer your questions and provide ideas for how your center can get involved.

BMT CTN Publications

There are 112 BMT CTN published articles, including 29 primary analyses. The following manuscripts were recently published:

- Holstein et al. **Summary of the third annual Blood and Marrow Transplant Clinical Trials Network Myeloma Intergroup Workshop on minimal residual disease and immune profiling.** *Biology of Blood Marrow Transplantation.* 2019 Sep 14. [ncbi.nlm.nih.gov/pubmed/31526843](https://pubmed.ncbi.nlm.nih.gov/31526843)
- Newell et al. **Follistatin and soluble endoglin predict 1-year non relapse mortality after allogeneic hematopoietic cell transplantation.** *Biology of Blood Marrow Transplantation.* 2019 Nov 10. [ncbi.nlm.nih.gov/pubmed/31715306](https://pubmed.ncbi.nlm.nih.gov/31715306)
- Martens et al. **Group sequential tests for treatment effect on survival and cumulative incidence at a fixed time point.** *Lifetime Data Analysis.* 2019 Nov 15. [ncbi.nlm.nih.gov/pubmed/31729633](https://pubmed.ncbi.nlm.nih.gov/31729633)

- Pidala et al. **Randomized multicenter trial of sirolimus vs. prednisone as Initial therapy for standard risk acute GVHD: BMT CTN 1501.** Blood. 2019 Nov 18. [ncbi.nlm.nih.gov/pubmed/31738834](https://pubmed.ncbi.nlm.nih.gov/31738834)
- Giralt et al. **Tandem autologous-autologous versus autologous-allogeneic Hematopoietic stem cell transplantation for patients with multiple myeloma: Long-term follow-up results from the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 0102 Trial.** Biology of Blood Marrow Transplantation. 2019 Nov 19. [ncbi.nlm.nih.gov/pubmed/31756536](https://pubmed.ncbi.nlm.nih.gov/31756536)
- Hourigan et al. **Impact of conditioning Intensity of allogeneic transplantation for acute myeloid leukemia with genomic evidence of residual disease.** Journal of Clinical Oncology. 2019 Dec 20. [ncbi.nlm.nih.gov/pubmed/31860405](https://pubmed.ncbi.nlm.nih.gov/31860405)

About the BMT CTN

The CIBMTR shares administration of the BMT CTN Data and Coordinating Center with NMDP/Be The Match and The Emmes Company. Together, these three organizations support all BMT CTN activities.

To receive up-to-date information about BMT CTN studies, meetings, and news:



Like us on Facebook: facebook.com/BMTCTN



Follow us on Twitter: twitter.com/BMTCTN (@BMTCTN)

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

State Medicaid Coverage for Allogeneic HCT for Patients with Sickle Cell Disease

By Tatenda Mupfudze

Sickle cell disease is the most common inherited hemoglobin disorder affecting approximately 100,000 people in the US. Allogeneic HCT is currently the only established curative option for sickle cell disease. However, allogeneic HCT is an optional benefit under Medicaid. This means that individual state Medicaid programs can choose to cover allogeneic HCT, define the indications, and determine the scope of key health benefits, including donor search, cell procurement, medication, travel and lodging, and clinical trial coverage.

There is limited publicly available information on Medicaid benefits for allogeneic HCT for patients with sickle cell disease. To address this knowledge gap, the Health Services Research team collaborated with the NMDP/Be The Match Health Policy and Reimbursement team to design a qualitative study aimed at understanding the scope of state Medicaid coverage benefits for allogeneic HCT for patients with sickle cell disease and the transplant center experience working with state Medicaid programs.

Between May and October 2019, 10 transplant center coordinators, representing eight states (FL, GA, IL, MI, NY, PA, TX, and VA), participated in semi-structured interviews via teleconference. Results showed that travel and lodging was the benefit that most often had limited or no coverage for allogeneic HCT patients with sickle cell disease, but there was wide variation by state. Clinical trials were inconsistently covered with some states following Medicare guidelines for covering allogeneic HCT as part of a clinical trial but not as standard of care while other states covered allogeneic HCT as a part of standard care but not as part of a clinical trial.

NMDP/Be The Match is using the results of this study to identify potential areas to influence changes in policy in order to enhance access to life altering curative therapy for SCD.

Abstracts at the 2020 TCT Meetings of ASTCT and CIBMTR

Join attendees at the upcoming TCT Meetings of ASTCT and CIBMTR in Orlando to see results of studies conducted by the Health Services Research Program:

1. Mupfudze et al. **A qualitative study of state Medicaid coverage for allogeneic HCT for patients with sickle cell disease.** Oral presentation.
2. Mishra et al. **Transplant physicians' attitudes on candidacy for allogeneic HCT in older patients: The need for a standardized geriatric assessment tool.** Oral presentation.

3. Farhadfar et al. **HCT predictions for the year 2023**. Poster presentation.

For more information about the Health Services Research Program, visit the [Health Services Research webpage](#) or email [India Hook-Barnard, PhD](#).

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials are NOT affiliated with or sponsored by Cardiovascular Research Foundation.

[Return to Top](#)

Stem Cell Therapeutic Outcomes Database

By Carol Doleysh

The SCTOD is part of the US Health Resources and Services Administration (HRSA)-funded C.W. Bill Young Cell Transplantation Program that collects data on all allogeneic HCT performed in the US and on transplants done elsewhere using cellular products that originated in the US.

Center-Specific Survival Analysis

Outcomes reporting in allogeneic HCT is necessary to provide information requested by patients, insurers, and government agencies, and to comply with current laws. The SCTOD contract requires the CIBMTR to annually conduct an analysis of one-year survival rates at each transplant center in the US. The report generated by the CIBMTR is meant to be used as a quality improvement tool for transplant centers. The data are also made available to the public at <http://bethematch.org/tcdirectory/search>.

The 2019 Center-Specific Survival Report, which includes first allogeneic HCT performed between 2015 and 2017 in the US, was distributed in mid-December to center directors, payors, and FACT. The data were also updated on the Be The Match website. Additional tools accessible to transplant centers for quality improvement work include Center Performance Analytics and the Survival Calculator. Access these tools through the secure CIBMTR Portal. For additional information, contact cibmtr-portalhelp@mcw.edu.

To be included in the analysis, transplant centers were required to have at least one year of follow-up on more than 90% of related and unrelated HCT recipients. [A description of the methodology used in generating this report](#) can be found on the CIBMTR website.

Public Website

The HRSA Blood Stem Cell website (<https://bloodstemcell.hrsa.gov/>) was recently updated to feature a new design. The new site continues to feature basic transplant, cord blood, and donor information as well as a description of the C. W. Bill Young Cell Transplantation Program and its contractors. We anticipate the website will evolve to include more query functionality than in the past. At this time, center volumes data are available through the [Transplant Activity Reports](#).

OMB Approval

Re-approval for SCTOD data collection forms was received from the Office of Management and Budget (OMB) on October 16, 2019. The new expiration date is October 31, 2022.

[Return to Top](#)

Presentations and Form Revisions for the Cellular Immunotherapy for Cancer Working Committee

By Carlos Litovich

First Presentations of New Cellular Immunotherapy for Cancer Working Committee

Marcelo Pasquini, MD, is spreading the word about the preliminary cellular therapy data collected by the CIBMTR with support from the NIH for the CIDR.

At the Society of Hematologic Oncology Seventh Annual Meeting in September, the Cellular Immunotherapy for Cancer Working Committee presented the study, *Real-world experience of tisagenlecleucel CAR T-cells targeting CD19 in patients with ALL and DLBCL using the CIBMTR Cellular Therapy Registry*.

Why is this important? Tisagenlecleucel is showing similar outcomes in the real-world setting as it did in the pivotal trials. For the CIBMTR, this presentation also signifies a successful implementation of our outcomes database for recipients of commercial CAR T-cells. And we are just getting started!

At the 61st Annual ASH Meeting in December, the committee presented three studies:

- *Tisagenlecleucel CAR T-cell therapy for adults with DLBCL: Real-world experience from the CIBMTR Cellular Therapy Registry*
- *Tisagenlecleucel CAR T-cell therapy for relapsed / refractory children and young adults with ALL: Real-world experience from the CIBMTR Cellular Therapy Registry*
- *Outcomes of post-marketing use of an anti-CD19 CAR T-cell therapy, axicabtagene ciloleucel (Axi-Cel), for the treatment of large B cell lymphoma in the US*

The Axi-Cel presentation demonstrated the utilization of this CD19 CAR T-cell product for treatment of lymphoma, and it was the largest CAR T-cell series presented to date! Both tisagenlecleucel presentations were unique, as they compared important CAR T-cell manufacturing parameters to clinical outcomes. These analyses were performed in collaboration with Novartis.

The preliminary conclusions are promising for the future of cellular therapies to treat cancer, as all three studies found similar safety and efficacy in the real world as in the pivotal trials. Pivotal trials are studies performed before the product is commercially available. The data we collect represents how these products perform after commercial approval in the community we serve. We are all excited to see how outcomes unfold, as we may be closer than ever to achieving our mission of improving outcomes and access for patients receiving cellular therapies.

Latest Revisions to CTED Forms

The latest revisions of the Pre-Cellular Therapy Essential Data, Product, Infusion and Essential Data Follow-Up Forms were released on January 24, 2020. The largest revisions were to the Essential Data Follow-Up Form, in which many changes were made to the collection of cellular therapy toxicities. A change made to the Pre-Cellular Therapy Essential Data will enable the CIBMTR to contact the patient directly to collect quality of life data through patient-reported outcomes in the future.

[Return to Top](#)

3 New Summaries for Patients

Share the 3 new patient-friendly summaries of CIBMTR publications with your patients. Watch for new summaries on the [Study Summaries for Patients webpage](#):



New treatment for GVHD has fewer side effects

Sirolimus treats graft-versus-host disease

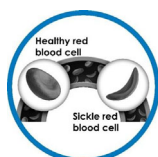
[Read more](#)



Intense treatment before transplant linked to infections

More people with leukemia who had intense treatment just before blood or marrow transplant (BMT) got a bacterial infection, compared to people who had less intense treatment.

[Read more](#)



New tool helps doctors decide when to stop medicines

After BMT, many people need immune suppression for more than a year.

[Read more](#)

Each quarter, the [Consumer Advocacy Committee](#) chooses a few studies of particular interest to patients. CIBMTR staff members create the plain-language summaries, which are reviewed by scientific directors.

Watch for new summaries on the [Study Summaries for Patients webpage](#) and social media.

[Return to Top](#)

CIBMTR Trivia

In December 2019, the CIBMTR presented both oral and poster abstracts at the ASH Annual Meeting. How many abstracts did CIBMTR investigators present?

- A. 28
- B. 33
- C. 24
- D. 32

[Enter your answer online.](#) If you answer correctly, you will be entered into a drawing to win a CIBMTR prize.

[Return to Top](#)

CIBMTR on Social Media

Like us on [Facebook](#) and follow us on [Twitter](#) and [LinkedIn](#) to stay up-to-date with important news and events. We promote our publications, share important content from other organizations, and publicize our key meetings and events. Follow us today!

facebook.com/theCIBMTR
[@CIBMTR](https://twitter.com/CIBMTR)
linkedin.com/company/thecibmtr/

[Return to Top](#)

Our Supporters

The CIBMTR is supported primarily by Public Health Service grant / cooperative agreement U24CA076518 with the National Cancer Institute (NCI), the National Heart, Lung and Blood Institute (NHLBI), and the National Institute of Allergy and Infectious Diseases (NIAID); grant / cooperative agreement U24HL138660 with the NHLBI and NCI; grant U24CA233032 from the NCI; grants OT3HL147741, R21HL140314, and U01HL128568 from the NHLBI; contract HSH250201700006C with the Health Resources and Services Administration (HRSA); grants N00014-18-1-2888 and N00014-17-1-2850 from the Office of Naval Research; subaward from prime contract award SC1MC31881-01-00 with HRSA; subawards from prime grant awards R01HL131731 and R01HL126589 from the NHLBI; subawards from prime grant awards 5P01CA111412, 5R01HL129472, R01CA152108, 1R01HL131731, 1U01AI126612, and 1R01CA231141 from the NIH; and grants from our [corporate and private contributors](#).

[Return to Top](#)

Abbreviations

Need an acronym defined? Review our [list of common abbreviations](#).

[Return to Top](#)

Last Updated: 9/22/2021 11:27 AM

CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program®/Be The Match® and the Medical College of Wisconsin