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February 2021 Newsletter

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Perspectives: Quality Assessment - Yet another COVID-19 victim?

By John Wingard, MD

The casualties from COVID-19 continue to mount. Do we need to add a quality assessment of HCT center outcomes to the list? Can we measure the impact on center outcomes and adjust for that impact in assessing transplant center performance?

Unfortunately, to date, there is a paucity of information. Most of the early reports about COVID-19 infections after HCT involved cases of COVID-19 infection beyond one year after HCT with fewer cases during the first year. Those case series suggest that COVID-19 case fatality rates are substantially higher than in the general population; further, infections occurring early after HCT



seem to fare worse. The direct effects of COVID-19 infection on an individual HCT patient are clearly consequential.

Yet, there are many unanswered questions about the impact of COVID-19 on HCT centers. COVID-19 has affected centers differently and at different times. What is the best metric to assess the COVID-19 burden on a given center: Should we measure rates of infection, hospitalization rates, or numbers of deaths? Should we measure those rates at the center's location or in the patient's community (if different)? How do we factor in the time since rates have varied over time and are different for various parts of the country?

If those considerations are not difficult enough to quantify, what about the indirect effects of COVID-19 on transplant practices? How did the pandemic affect all the quality measures each center put in place? Transplants were delayed, donor options narrowed, cell products were cryopreserved, testing for comorbidities and risk assessment were curtailed, and center-specific protocols altered. Within each center, the staff were stretched or unavailable due to quarantine, transplant bed availability tightened, and ICU capacity became unpredictable. In-person outpatient visits were severely reduced, and initiatives to monitor complications and educate patients disappeared or were severely disrupted. Data managers and study coordinators were sent home and each center's ability to track what was going on was constrained and often changed from hour to hour. For patients, vital social support resources from family and loved ones dissolved, and finances were severely tested. Housing options close to the transplant center closed, and patients were sent home to local non-transplant providers prematurely.

Centers were forced to make changes in transplant practices abruptly, without advance planning, and often without knowing what was best. The list of such indirect effects has only grown over time. How does one measure such indirect effects of COVID-19 on quality measures of transplant performance?

Recognizing the need for additional data to try to understand these practice changes, the CIBMTR revised and developed new data forms. One form collects information about the patients who develop COVID-19 infection. Another contains a series of questions to determine what changes in HCT practices necessitated by the COVID-19 pandemic were taken by centers for their patients. Despite data management staff working remotely, centers have remarkably stepped up to provide this extra information. Capturing this information about both direct and indirect efforts is crucial to gain insight regarding the impact of COVID-19 on center performance.

The CIBMTR routinely takes a step back to ask challenging questions about the way it measures transplant center-specific survival: Are we overlooking important risk factors? Are there new tools to better assess risks? Are there better analysis techniques to capture the complexities of many competing risks? Does the risk adjustment model need adjustment? The underlying premise for these self-assessments is the recognition that no matter how careful we are, we need to examine if we can do better. Topics are identified, committees formed, reports developed, and a forum is held every other year involving diverse stakeholders to forge a plan. In a sense, this is a quality assessment of the CIBMTR's quality assessment tool. This past fall, a major topic for the CIBMTR Center Outcomes Forum was the impact of COVID-19 on analyses of HCT outcomes.

The COVID-19 plan involves evaluating the data gathered in 2020 as each center reports it during the upcoming months. The next challenge is for the CIBMTR's biostatisticians to test new statistical models to measure both the direct and indirect impacts of COVID-19 to determine if a new model is needed to adjust for the COVID-19 effects on center-specific outcomes.

This will not be easy. It may even not be doable. The dilemma is how can one assess quality when a black swan event thwarts all the quality measures one has put in place? Stay tuned; there will be more discussion about this matter.

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[Non-Malignant Diseases Working Committee](#)

Committee Leadership

Co-Chairs:

- [Christopher Dvorak, MD](#), University of California San Francisco Medical Center, San Francisco, CA
- [Andrew Gennery, MD](#), Newcastle General Hospital and The Royal Victoria Infirmary, Newcastle, UK
- [George Georges, MD](#), Fred Hutchinson Cancer Research Center, Seattle, WA

Scientific Director:

- [Mary Eapen, MD, MS](#), CIBMTR Milwaukee

Statistical Director:

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

Statistician:

- [Kyle Hebert, MS](#), CIBMTR Milwaukee

The Non-Malignant Diseases Working Committee conducts clinical research on early and late outcomes following allogeneic HCT for these diseases. Many of the diseases are rare to ultra-rare and the patient population is small, so the best avenue to collect maximum information about HCT outcomes for these diseases is to promote collaborative studies. This working committee collaborates with the EBMT, Eurocord, and individual highly specialized centers to report about transplant-related topics and further the field of knowledge in this area. The disorders covered by the committee may be broadly classified under the following categories: Marrow failure (acquired and inherited), hemoglobinopathies, metabolic disorders, and immune deficiency / dysregulation disorders, and autoimmune diseases. The uniqueness of this working committee is that we manage a wide variety of different diseases. However, these diseases also have much in common. For example, many patients are young, so the toxicities may be different for older children or adults, and the potential to affect their lives in a positive manner is magnified. In the last 4 years (2017-2020), the committee produced 12 peer-reviewed manuscripts.

Three of the manuscripts focused on hemoglobinopathy (one in thalassemia and two in sickle cell disease). They addressed outcomes after transplantation, including matched and mismatched unrelated donors and mismatched related family donors, and they developed and validated a simple risk score for patients considering transplantation for sickle cell disease. We envision that HCT physicians will utilize this tool in their consultations with patients and their families.

Two of the manuscripts concentrated on primary immunodeficiencies. One of the manuscripts was developed in collaboration with the EBMT and described the world's largest dataset on patients with DNA breakage repair disorders, which established the risks of myeloablative conditioning in these patients. Conversely, an ongoing study in patients with hyperinflammatory inborn errors of immunity demonstrates more rejection when reduced-intensity fludarabine-melphalan conditioning is utilized for these diseases.

We are also conducting a study on long-term outcomes of autologous HCT for systemic sclerosis (SSc / scleroderma). After two large, multicenter, randomized clinical trials established the superiority of autologous HCT over the standard of care chemotherapy, we are assessing the long-term status of SSc patients after autologous HCT. In the past 2 decades, more than 123 patients with SSc completed autologous HCT at 10 centers in the US and Canada. Patients were treated with either total body irradiation / cyclophosphamide / ATG or high-dose cyclophosphamide / ATG, with or without CD34+ selection. This study will examine the long-term outcomes of SSc patients followed for up to 20 years after HCT. The project involves contacting patients and completing detailed disease status questionnaires. Currently, in progress, completion of the patient questionnaires is expected in the next few months.

Committee membership draws investigators from diverse backgrounds, each with experience in transplantation for benign, often rare disorders. The uniquely different disorders and the common transplant goals provide a great opportunity for synergy in scientific interactions and for members to bring forth new ideas. A full list of the studies undertaken by this committee, including recent publications, is provided on the Non-Malignant Diseases Working Committee studies [webpage](#). Each year, 1-2 new proposals are selected during the TCT Meetings of ASTCT and CIBMTR. The selection process includes input from the Working Committee membership who score and prioritize studies based on interest and impact factors. As some of the disorders included within the purview of this committee face significant residual disease burden, even after successful transplantation, studying late outcomes has special significance and is often the target of study proposals. We encourage active participation, the submission of new study proposals, and discussion with the chairs, as the success of the committee, is highly dependent upon the scientific community. We also encourage the input of junior investigators. To learn more about our committee or to discuss ideas and new projects, please contact one of the Committee Chairs or the Scientific Director.

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Plasma Cell Disorders and Adult Solid Tumors Working Committee



Back row left to right: Raphael Fraser, Shaji Kumar, Omar Davila, Anita D'Souza, Parameswaran Hari, Muzaffar Qazilbash

Front row left to right: Heather Landau, Noel Estrada-Merly, Nina Shah

Photo credit: Nina Shah

Committee Leadership

Co-Chairs:

- [Shaji Kumar, MD](#), Mayo Clinic Rochester, Rochester, MN
- [Nina Shah, MD](#), University of California San Francisco Medical Center, San Francisco, CA
- [Muzaffar Qazilbash, MD](#), M.D. Anderson Cancer Center, Houston, TX

Scientific Director:

- [Anita D'Souza, MD](#), CIBMTR Milwaukee

Statistical Director:

- [Raphael Fraser, PhD](#), CIBMTR Milwaukee

Statistician:

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

The Plasma Cell Disorders and Adult Solid Tumors Working Committee works with investigators from around the world to define the optimal utilization of transplantation for all plasma cell disorders, including multiple myeloma, as well as rare plasma cell disorders, such as light chain amyloidosis, POEMS syndrome, plasma cell leukemia, etc. Our committee also studies the role of HCT in adult solid tumors. The committee is comprised of a Scientific Director (Dr. Anita D'Souza), 3 Co-Chairs (Dr. Shaji Kumar, Dr. Muzaffar Qazilbash, Dr. Nina Shah), Biostatistician (Mr. Noel Estrada-Merly), and a Statistical Director (Dr. Raphael Fraser). Dr. Parameswaran Hari stepped down from his role as Scientific Director in early 2020. We are grateful for his many successful years of leadership and look forward to his continued presence and guidance as well as mentorship to the committee and investigators across the community. We have multiple advisors who work closely with our committee in providing additional input on our committee work, including Mr. Jack Aiello (Patient Advocate); Dr. Michael Bishop; and Dr. Heather Landau.

Our committee recently completed several studies regarding transplant outcomes of elderly multiple myeloma patients,¹ the role of induction therapy prior to transplant in light-chain amyloidosis,² outcomes in plasma cell leukemia,³ and the largest series of patients with germ cell tumors treated with transplant.⁴ Ongoing studies include the comparison of induction therapies in multiple myeloma, the role of maintenance therapy after a second autologous HCT in multiple myeloma, second autologous HCT as salvage therapy in light-chain amyloidosis patients, outcomes of HCT in POEMS syndrome, second malignancies after autologous HCT in multiple myeloma patients, and a comparison of bortezomib-based versus lenalidomide maintenance therapy in high-risk multiple myeloma patients. In 2019

and 2020, this committee published 7 peer-reviewed manuscripts, including 3 published with editorials; presented 6 abstracts at the ASH Annual Meeting, including 4 oral presentations and one selected for ASH Disparities in Hematologic Malignancies press release; and presented one abstract at the American Society of Clinical Oncology annual meeting.

The Plasma Cell Disorders and Adult Solid Tumors Working Committee provides a platform for consolidating ideas from oncologists across the country to study disease biology as well as management of these diseases in the context of transplant. By serving as a partner for researchers, we leverage the power of big data to undertake projects and answer questions that would be difficult for a single institution to accomplish. Through this collaborative process, the CIBMTR achieves stronger connections and commitment to the overall mission of the organization. The vision of our committee is to keep this loop alive so that we can achieve greater national and international recognition for transplant-related research. We hope to accelerate the discovery of cures for patients afflicted by these diseases and highlight existing disparities in access to transplant to rectify these disparities.

The success of the committee depends on new ideas and testable hypotheses as well as participation by individuals with different perspectives and scientific backgrounds. The Plasma Cell Disorders and Adult Solid Tumors Working Committee encourages all investigators with an interest in our disease focus to propose a study. We seek interesting and novel ideas and encourage the involvement of junior investigators interested in outcomes research. We believe early involvement encourages the long-term participation of early-career investigators in committee activities. Many of our studies are led by senior fellows and junior faculty with Working Committee mentorship. To learn more about study proposals, visit the CIBMTR How to Propose a Study [webpage](#).

To learn more about the committee or discuss your research ideas and proposals, contact one of the members of the Working Committee leadership team. We strongly encourage our 568 current Working Committee members to actively participate in the committee activities and look forward to everyone's participation at The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience.

References

1. Munshi, P, Vesole, D, Jurczyszyn, A, et al. Age no bar: A CIBMTR analysis of elderly patients undergoing autologous hematopoietic cell transplantation for multiple myeloma. *Cancer*. 2020; 126(23):5077-5087.
2. Cornell RF, Fraser, R, Costa L, et al. Bortezomib-based induction is associated with superior outcomes in light-chain amyloidosis patients treated with autologous hematopoietic cell transplantation regardless of plasma cell burden. *Journal of Transplantation and Cellular Therapy* 2020. (In print).
3. Dhakal B, Patel S, Girnius S, et al. Hematopoietic cell transplantation utilization and outcomes for primary plasma cell leukemia in the current era. *Leukemia*. 2020; 34(12):3338-3347.
4. Kilari, D, D'Souza, A, Fraser, R, et al. Autologous hematopoietic stem cell transplantation for male germ cell tumors: Improved outcomes over 3 decades. *Biology of Blood and Marrow Transplantation*. 2019; 25(6):1099-1106.

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CIBMTR 2020 Annual Report

The CIBMTR 2020 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. 2020 accomplishments are highlighted at the beginning of the report and explained in more detail throughout the document.

Thank you for helping us make 2020 a successful year despite all the challenges we faced!



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The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience

By Tia Houseman



The TCT | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR are the combined annual meetings of the ASTCT and the CIBMTR. 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 professional attendees benefit from a full scientific program that addresses the most timely issues in cellular therapy.

Join us for the 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience

The main meeting will take place February 8-12, with the CIBMTR Clinical Research Professionals / Data Management Track and ASTCT Education Sessions taking place February 1-5, and the CIBMTR New Data Manager Onboarding scheduled for February 15-19.

Register now

If you have not done so already, go to the 2021 TCT Meetings of ASTCT and CIBMTR [website](#) TODAY to register and view additional details. Advanced registration is required for access to the digital experience.

A program you will not want to miss

Throughout The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience, leading worldwide experts in the field of transplantation and cellular therapy will present the latest developments through plenary and concurrent sessions, oral abstracts, posters, tracks, and more. Along with these state-of-the-art educational offerings, industry-supported satellite symposia, product theaters, and exhibitors will further broaden the spectrum of presentations.

Scientific Program Topics

- Acute GVHD: Therapies for new targets
- Chronic GVHD: Mechanisms and new therapies
- Creating solutions through international collaboration: Lessons learned during the COVID-19 pandemic
- Data science and machine learning in transplantation and cellular therapy studies
- Deciphering response / resistance mechanisms to instruct next generations of T-cell receptor gene therapy
- Gene therapy for non-malignant diseases
- Immunotherapy in myeloma
- Late effects: Working early to improve long-term outcomes
- Leukemia: Drugs to decrease relapse
- NK cells: Beyond AML
- Oxygen sensing
- T-Cells: Biology to therapeutics
- The microbiome and transplant outcomes
- Transplantation and cell therapy during pandemics
- During a worldwide crisis: Lessons learned across the world
- Pediatric BMT
- ...and more

Highlights

On Tuesday and Thursday evenings, attendees will have a chance to hear from authors of some of the top-rated posters during the Poster Highlights Sessions. While many of us will miss the TCT Meetings of ASTCT and CIBMTR Reception on the beach in Hawaii, we have something special in store for you on Thursday evening as we approach the final day of The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience.

On Friday, we close out The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience with the Mortimer M. Bortin Lecture, E. Donnall Thomas Lecture, Best Abstracts and Late-Breaking Abstracts presentations, and the Awards / Closing,

including a presentation by Anthony Fauci, MD, and a celebration of a very special individual.

View the online agenda by clicking [here](#). View the agenda within your own time zone by clicking the *My Time* button on the navigation panel. Additionally, you can view the agenda by [Target Audience](#), [Date](#), or [Track Overview](#).

Support opportunities and additional information

Questions regarding support opportunities for The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience may be directed to the TCT Meetings of ASTCT and CIBMTR Conference Office: TCTMeetings@mcw.edu.

We look forward to having you join us in the first-ever TCT Meetings of ASTCT and CIBMTR Digital Experience in February 2021!

Join the conversation: #TCTM21

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BMT CTN

By Amy Foley, MA

BMT CTN State of the Science Symposium (SOSS)

The BMT CTN has held three State of the Science Symposia (in 2001, [2007](#), and [2014](#)) to survey the HCT landscape and identify areas in greatest need of multicenter trials. Prioritized concepts served as a road map for studies recommended for implementation by the US transplant community using the resources of the BMT CTN, NCI National Clinical Trials Network, and other programs.

The next SOSS (held virtually) is scheduled for Wednesday, March 10, from 9:00 AM – 5:00 PM CT. Registration is free and open to all: [BMT CTN SOSS website](#)

In preparation for the Symposium, BMT CTN Steering Committee and SOSS Chair, Helen Heslop, MD, DSc, charged 11 committees with identifying 2-3 clinical trials concepts that:

- Represent the most important issues facing the field
- Have the potential to change practice in a significant way
- Require a multicenter network to be done effectively

SOSS committee reports are posted on the [BMT CTN SOSS website](#). The represented committees are:

- Comorbidity & regimen related toxicity
- Graft-versus-host disease
- Hemoglobinopathies
- Infection / immune reconstitution
- Late effects / quality of life / economics
- Lymphoid malignancies
- Myeloid malignancies
- Non-malignant disorders
- Optimal donor and graft source
- Pediatric malignant diseases
- Plasma cell disorders

Two additional committees served in an advisory capacity to all others. Their reports, describing their recommendations, are also available:

- Clinical trial design
- Disparities and access to HCT

During the SOSS, Committee Chairs and assigned external reviewers will present selected proposals, followed by Q&A sessions to get audience feedback. After the meeting, a review panel will prioritize concepts and develop a roadmap for the BMT CTN. The BMT CTN will submit results from the proceedings for publication later this year.

We are seeking participation from a broad spectrum of the cellular therapy community, so we hope you can join us for the virtual event!

BMT CTN Abstracts at The 2021 TCT Meetings of ASTCT and CIBMTR

Digital Experience

Please join us for virtual abstract sessions during The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience, including both a **Late-Breaking Abstract** and **Best Abstract**!

- **LATE-BREAKING:** BMT CTN 1301 primary results
 - LBA1 Friday, February 12, 2:00 PM CST
 - *Calcineurin inhibitor-free graft-versus-host disease (GVHD) prophylaxis in hematopoietic cell transplantation (HCT) with myeloablative conditioning regimens (MAC) and HLA-matched donors: Results of the BMT CTN 1301 Progress II trial*
 - Presenter: Miguel-Angel Perales
- **BEST ABSTRACT:** BMT CTN 1102 primary results
 - Friday, February 12, 12:30 PM CST
 - *A multi-center biologic assignment trial comparing reduced-intensity allogeneic hematopoietic cell transplantation to hypomethylating therapy or best supportive care in patients aged 50-75 with advanced myelodysplastic syndrome: Blood and Marrow Transplant Clinical Trials Network study 1102*
 - Presenter: Ryotaro Nakamura
- **ORAL:** BMT CTN 1202 VOD/SOS analysis
 - Monday, February 8, 3:30 PM CST
 - *Prognostic biomarker signature for hepatic veno-occlusive disease / sinusoidal obstruction syndrome (VOD / SOS) in recipients of myeloablative (MA) allogeneic hematopoietic cell transplantation (HCT)*
 - Presenter: Santosh Putta
- **POSTER:** BMT CTN 1803 Trials in Progress update
 - Poster number: 531 (Note: posters available for access during the entire meeting)
 - *BMT CTN 1803: Haploidentical natural killer cells (K-NK002) to prevent post-transplant relapse in AML and MDS (NK-REALM)*
 - Lead author: Sumithira Vasu

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:



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

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RCI BMT

By Erin Leckrone, MBA

The RCI BMT specializes in the design and execution of a variety of clinical trial programs by providing broad support via protocol design, study and site management, database development, data analysis, and medical and statistical oversight. The RCI BMT currently oversees a clinical portfolio of 21 studies with patients in active recruitment or follow-up and an additional 4 studies in active design or start-up. In 2020, the RCI BMT accrued more than 1,400 participants to studies sponsored by NMDP/Be The Match, industry organizations, consortiums such as BMT CTN and PTCTC, and individual investigators / institutions leading multi-center trials.

Supported by 40 core operational clinical research professionals, the RCI BMT offers full trial execution and can also customize support based on specific study needs. The RCI BMT utilizes Medidata Rave and the CIBMTR's electronic patient-reported outcomes (ePRO) systems as its primary data capture systems and Florence eRegulatory for electronic Trial Master File management and electronic regulatory binders. Studies may also rely on the built-out clinical infrastructure of the NMDP IRB and RCI BMT data and safety monitoring board for data and safety oversight. By using the NMDP/Be The Match and CIBMTR's regulatory and

immunobiology resources, and biostatisticians and physicians, the RCI BMT can oversee on-time and on-budget study execution.

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Health Services Research

By Jaime Preussler, MS; Lin-Wen Mau, PhD, MPH; Christa Meyer, MS; Tatenda Mupfudze, PhD; Jennifer Sees, MPH

The Health Services Research program facilitates studies in multiple focus areas, including health economics, disparities, and barriers to access. Currently, two studies focused on access barriers are in manuscript development, and both will be presented at The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience. Highlights of these studies are presented below:

The study, "Access to transplant for patients with acute myelogenous leukemia in the state of Virginia," compared transplant rates among different regions in the state of Virginia, analyzing the impact of race / ethnicity, urban versus rural location, primary insurance payor, and socioeconomic status on access to allogeneic HCT. This study analyzed a retrospective cohort of patients aged 18-75 years diagnosed with AML in Virginia between 2013-2017 using Virginia Cancer Registry and CIBMTR datasets as well as American Community Survey data. Results showed the likelihood of undergoing HCT was associated with age, percent of African Americans residing in a region, marital status, primary insurance payor, and percent of the population with a bachelor or graduate degree. Future studies will evaluate access to allogeneic HCT after referral to transplant centers and racial disparity in access to allogeneic HCT across the United States.

The study "Hematopoietic cell transplant outcomes among Medicaid and privately insured patients with sickle cell disease," examined sociodemographic characteristics, clinical risk factors, and allogeneic HCT outcomes among patients with sickle cell disease enrolled in Medicaid and private insurance. A retrospective CIBMTR cohort study from 2008-2018, this study found statistically significant differences in lower event-free survival and increased incidence of graft failure among patients with Medicaid compared to private insurance.

Attend The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience for further information about these studies:

1. Mock et al. Geographic and socioeconomic disparities in hematopoietic cell transplantation among acute myeloid leukemia patients in Virginia. Oral presentation; Session C - Health Services; Monday, February 8, 2021, 2:30 PM CST.
2. Mupfudze et al. Hematopoietic cell transplant outcomes among Medicaid and privately insured patients with sickle cell disease. Poster presentation; Poster number 363.

For more information about the Health Services Research Program, visit the Health Services Research [webpage](#) or email [Jennifer Sees, MPH](#), Manager, Strategic Programs & Data Analytics.

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Bioinformatics research: Our science & technology SWAT team with a focus on matchmaking

By Yung-Tsi Boion, PhD

Bioinformatics Research Matchmaking

Strategic Capabilities & Applications



WHY? *We Save Lives by Matching Patients with Cellular Therapies*

What is bioinformatics research? Who are we? Across the CIBMTR and NMDP/Be The Match, the bioinformatics research group serves as our science and technology SWAT team with a unique blend of scientific backgrounds and programming capabilities. Beyond experience and skillsets, each collaborative team member is selected for 1) passion toward the mission of saving lives, 2) demonstrated focus on results as finishers, and 3) high learning agility, a blend of curiosity and drive to continually pursue and exceed our common goals. A new clinical data scientist role on our team is currently open for applications: <https://nmdp.referrals.selectminds.com/jobs/clinical-data-scientist-minneapolis-or-remote-1117>

This team's research focuses on matchmaking and translates findings toward our goal of saving lives by matching patients with cellular therapies. Matchmakers, you say? Yes! We research the what, where, and how of matching. What factors are most important in matching therapies to patients for best outcomes? In an upcoming edition of the CIBMTR newsletter, we will elaborate more about recent findings from omics analyses of candidate molecular factors that may play a role in transplant outcomes.

What features are most important for overall and event-free survival after transplantation? Where along the patient and donor journeys should we be collecting more data to improve the matching of cellular therapies for patients? How can we better predict transplant outcomes and assist in decision-making to positively impact patient survival and quality of life? In future editions, we will also delve more into the details of prediction modeling and machine learning approaches applied to CIBMTR data and our findings in this space.

Where can we find donor and therapy sources most likely to match and bridge patient gaps? Where can we improve our population and demographic data collection to improve matching? We will be providing analyses on donor registries and operational changes in data collection and curation spurred by research to improve matching algorithms and results for patients.

How can we improve matching while optimizing therapy sources to provide the best match for every patient? Stay tuned! In upcoming newsletters, we will report on new tools to select for best therapy sources in potentially mismatched settings. These are a sampling of the research results and translational applications we expect to share with you, our community. Thank you for your support!

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Cellular Therapy

By Carlos Litovich, MPH

Virtual Cellular Therapy Forum

The COVID-19 pandemic threw a giant wrench into everyone's plans for 2020. As expected, the CIBMTR's staff rose to the occasion, adapting, and reinventing the 6th Annual Cellular Therapy Forum for discussion and innovation in the cellular therapy for cancer field attendee benefits included: No airfare costs nor jetlag for anyone, hands-on participation from essential stakeholders, easy and flexible attendance, and the most comfortable venue with *WebEx business casual dress code*.

Forum topics included:

- CAR T-cell toxicity
- Multi-stakeholder initiatives to improve access to CAR T-cells
- Capture of long-term follow-up for gene therapies

- Cellular therapy and real-world data experience
- A report from the Cellular Therapy for Solid Tumors Task Force

The forum had international representation, and nearly 300 attendees engaged in thought-provoking discussions. This record-breaking attendance can be attributed to the easily accessible, virtual format of the forum. Going forward, this year's success will influence how we promote and hold our forum, as it is a pillar of our mission to make it widely accessible.

Year 3 CIDR

What are the plans for the third year of the Cellular Immunotherapy Data Resource? **Expansion, collaboration, and dissemination.** We plan to continue the expansion of patient-reported outcomes data collection, including ramping up collection for cellular therapy recipients. The CIBMTR also plans to expand cellular therapy audits by conducting virtual audits in centers serving cellular therapy recipients. We will implement recommendations from the Solid Tumor Task Force to stay ahead of the curve in this fast-paced field. We will continue our efforts to make our research accessible to the community and collaborate with EHR companies to support centers' ability to submit data directly from EHR. In summary, we will continue to perform in pursuit of our institutional mission of improving outcomes of patients receiving cellular therapies.

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Immunobiology Working Committee Scientific Leadership Joined by Yung-Tsi Bolon, PhD

By Stephen Spellman, MBS, and Stephanie Lee, MD, MPH

We are pleased to announce that Yung-Tsi Bolon, PhD, has joined the leadership of the Immunobiology Working Committee as an assistant Scientific Director. Dr. Bolon has vast experience in omics-related data science and molecular cell mechanisms that underlie cancer biology. She is currently Director of the CIBMTR Immunobiology and Bioinformatics Research team on the CIBMTR Minneapolis campus and serves as adjunct graduate faculty in bioinformatics and computational biology at the University of Minnesota-Rochester. She holds Bachelor's degrees in biochemistry, linguistics, and French from the University of Missouri and a PhD in biochemistry, molecular biology, and biophysics from the University of Minnesota. We are excited to have Dr. Bolon join the Immunobiology Working Committee leadership team and have no doubt she will be extremely productive in her position as assistant Scientific Director! Please join us in congratulating her.

Over the course of the next year, Stephen Spellman, MBS, will step back from his responsibilities as Co-Scientific Director of the working committee to focus on his new role as Vice President of Research on the CIBMTR Minneapolis campus. The Immunobiology Working Committee's scientific leadership team will work closely to ensure a smooth transition of all studies, culminating with Dr. Bolon's promotion to Co-Scientific Director at the end of 2021.

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Data Transformation at The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience

The CIBMTR Data Transformation team is excited to explore data solutions at The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience. Please find us online at the following sessions:

2021 Clinical Research Professionals / Data Management Conference

- *CIBMTR is transforming how data is submitted, what does this mean for data managers?*
 - Thursday, February 4, 12:05 PM – 12:35 PM CST

The 2021 TCT Meetings of ASTCT and CIBMTR Digital Experience

- *Center-specific data reporting: A clinical team's perspective of the CIBMTR DTI*
 - Monday, February 8, 11:00 AM – 11:30 AM CST
- *Transforming data at the CIBMTR*
 - Monday, February 8, 11:30 AM – 11:50 AM CST
- *Data transformation initiative panel*
 - Monday, February 8, 12:30 PM – 1:00 PM CST

The TCT® Trademark belongs to the Cardiovascular Research Foundation. ASTCT, CIBMTR, MCW, NMDP, the 2019, 2020, and 2021 TCT Conferences and materials

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Stem Cell Therapeutic Outcomes Database

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 and Center Outcomes Forum

Outcomes reporting in allogeneic HCT is necessary to provide the 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 useful as a quality improvement tool for transplant centers. The data are available to the public at <http://bethematch.org/tcdirectory/search>. To be included in the analysis, transplant centers must have at least one year of follow-up data for more than 90% of related and unrelated HCT recipients within the reporting period. A description of the methodology used in generating this report can be found on the CIBMTR [website](#).

The 2020 Center-Specific Survival Analysis Report, which includes first allogeneic HCT performed between 2016 and 2018 in the US, was distributed in mid-December to center directors, payers, 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 to these tools is available via the secure CIBMTR Portal. Any questions regarding CIBMTR Portal account credentials should be submitted via CIBMTR Center Support (<https://nmdp.service-now.com/csm>), by selecting CIBMTR Center Maintenance and then CIBMTR Portal Help.

To fairly address the complex issues and maintain a transparent scientific approach to center outcomes reporting, a seventh Center Outcomes Forum was held in November 2020. Participants included representatives of the HCT community, including transplant physicians and center directors, the ASTCT and its Quality Outcomes Committee, FACT, governmental funding agencies, patients, private payers, and statisticians. A summary of the meeting will be distributed to US Medical Directors and posted on the CIBMTR [website](#) when complete. The discussion focused on three essential questions related to the Center-Specific Survival Analysis :

- Is MRD for acute leukemia (AML, ALL) ready for use as a risk adjustment factor in the Center-Specific Survival Analysis?
- Are there new approaches to account for social determinants of health in the Center-Specific Survival Analysis risk adjustment model?
- Can the CIBMTR adjust for the impact of the COVID-19 pandemic in the Center-Specific Survival Analysis?

The CIBMTR is considering the best approach to the Center-Specific Survival Analysis in 2021 to accommodate the impact of the COVID-19 pandemic on center outcomes. Patients who underwent allogeneic HCT in 2019 will be included in the 2021 Center-Specific Survival Analysis Report and may have been affected by COVID-19 during their first year of post-transplant follow-up. We plan to test the effects in a preliminary model in February 2021 before finalizing a plan for consideration with HRSA and other stakeholders. The preliminary analysis plan was discussed as part of the 2020 Center Outcomes Forum. Further information about this plan will be available as part of the meeting summary. US centers are asked to provide necessary data about follow-up on an accelerated schedule this year to make these additional analyses possible.

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DBtC: Accessing REMS (Risk Evaluation and Mitigation Strategies) Related Data

A Risk Evaluation and Mitigation Strategy (REMS) is a program to manage known or potential serious risks associated with a drug product and is required by the FDA to ensure the benefits of the drug outweigh its risks. The FDA requires a REMS program for commercial CAR T products to mitigate the risks of cytokine release syndrome and neurological toxicities to the patients. As a result, centers are asked

to submit REMS-related data to pharmaceutical companies that provide commercial CAR T therapies.

Centers that have submitted CAR T data to the CIBMTR can download their REMS data in an easy-to-use and understand format. These data can be obtained by logging into the DBtC application found within the [CIBMTR Portal](#). The REMS data are available on the Outcomes tab after clicking on the CAR T chart-toggle button and selecting the REMS options from the data payload area. Users will find chart menu items for both REMS Data and REMS Reports in the center of this page.

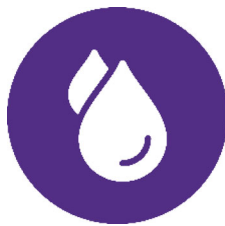
The REMS Data option is a simple data grid containing unformatted raw data that can be sorted, filtered, and downloaded as an excel file. The data presented in the data grid are the same data used to generate the reports found under the REMS Report options. The REMS Report option is a simple download screen allowing users to download formatted Excel reports. A new Excel-formatted REMS report is added to this archive every month.

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Sharing Research In Plain Language

By Jennifer Motl

These four new plain-language summaries may help your patients:



Blood and marrow transplants during COVID-19 pandemic

Experts advise new safety measures for patients and donors

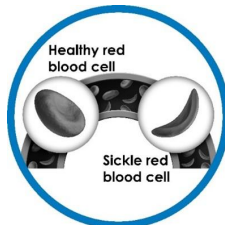
Read more: [accessible](#) or [1-page](#) version



More African Americans can get blood or marrow transplant

Study shows half-matched and cord blood transplants are acceptable

Read more: [accessible](#) or [1-page](#) version



New risk score predicts person's chance that transplant will work to treat sickle cell disease

Children who have matched sibling donors are helped most

Read more: [accessible](#) or [1-page](#) version



New tool shows which people with myelodysplastic syndromes (MDS) will be most helped by transplant

Read more: [accessible](#) or [1-page](#) version

On the [Study Summaries for Patients webpage](#), you will find even more summaries.

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Additional Research Datasets Available for Secondary Analysis

By Liz Siepmann

- ▶ COVID-19 Updates
- ▶ Quick Links
- ▶ Patient Resources
- ▶ Publication List
 - ▶ Publication Dataset Download
- ▶ Newsletters
- ▶ News Releases
- ▶ Slides and Reports
- ▶ Statistical Resources

Research Datasets for Secondary Analysis

The CIBMTR makes its publication analysis datasets freely available to the public for secondary analysis while safeguarding the privacy of participants and protecting confidential and proprietary data.

View the [Terms and Conditions](#).

Year	Publication	Zip Download
2020	Survival outcomes of allogeneic hematopoietic cell transplants with EBV-positive or EBV-negative post-transplant lymphoproliferative disorder: a CIBMTR study.	Download
2020	Impact of cytogenetic abnormalities on outcomes of adult Philadelphia-negative acute lymphoblastic leukemia after allogeneic hematopoietic stem cell transplantation: A study by the Acute Leukemia Working Committee of the Center for International Blood and Marrow Transplant Research.	Download
2020	Comparison of high doses of total body irradiation in myeloablative conditioning before hematopoietic cell transplantation.	Download
2020	Maintenance tyrosine kinase inhibitors following allogeneic hematopoietic stem cell transplantation for chronic myelogenous leukemia: A Center for International Blood and Marrow Transplant Research Study.	Download
2020	Donor HLA-E status associates with disease-free survival and transplant-related mortality after non in vivo T cell-depleted HSCT for acute leukemia.	Download
2020	Weighty choices: Selecting optimal G-CSF doses for stem cell mobilization to optimize yield.	Download
2020	Predictors of loss to follow-up among pediatric and adult hematopoietic cell transplantation survivors: A report from the Center for International Blood and Marrow Transplant Research.	Download
2020	Increased overall and bacterial infections following myeloablative allogeneic HCT for patients with AML in CR1.	Download

In accordance with the [NIH Data Sharing Policy](#) and [NCI Cancer MoonshotSM Public Access and Data Sharing Policy](#), the CIBMTR is making publication analysis datasets publicly available on the [CIBMTR Research Datasets for Secondary Analysis webpage](#).

These publication analysis datasets are freely available to the public for secondary analysis. While providing these data, the CIBMTR is committed to safeguarding the privacy of participants and protecting confidential and proprietary data.

[NEW datasets are now available online.](#)

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Our Supporters

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Abbreviations

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

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