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May 2020 Newsletter

Volume 26, Issue 2

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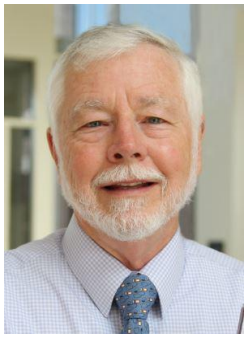
Perspectives: Living through Tough Times

By John Wingard, MD



BMT has never been easy. Not for patients and their loved ones. Not for health care providers. The arrival of COVID-19 has now made it even tougher. The conversations we have about whether to transplant or not have always been tough: How do we best balance the risk and benefits? The decisions are wrenching for all involved. The conversations have now become even tougher.

It is easy to get discouraged. We have seen multiple examples of failure of effective governmental leadership. But, we have also witnessed inspiring examples of decisive action to quell the threat. Sometimes, it seems we are all going together through the five stages of grief that Kubler-Ross described. Some leaders seem to be stuck far too long at the denial and anger stages, while the rest of us are racing on toward acceptance. The paradox can be

maddening. We see crucial community structures and health care resources that we normally count on becoming abruptly disrupted. Yet, we all are called upon to seize the challenges we face in our programs.

We should be mindful of what will get us all through this. First, we can lean on one another for support. Our trusting relationships with each other provide assurance that our fellowship will be there to help us through thick and thin. Second, our communities, forged over the years, are steady help we can count on. Third, our BMT community has a culture of cooperativeness to share lessons learned and mistakes to avoid. Most importantly, we are not in this alone. If ever, we are reminded that the world is one and communication is vitally important.

Already we are seeing our worldwide BMT community responding. The WBMT has compiled measures taken in various countries that offer blueprints that we can draw from. Provisional guidelines have been developed by the EBMT, ASTCT, and other BMT groups worldwide. Individual centers are exchanging guidelines with one another. We have BMT emergency preparedness guidelines published years ago that were developed to face earthquakes, hurricanes, blackouts, and SARS. The CIBMTR launched a COVID-19 webpage to provide updates and urges centers to continue consenting patients (even if data collections are deferred until later). This is essential in order to achieve an accurate reflection of how the crisis is affecting transplant patients so that we can learn as much as possible to help future patients.

I wrote the above in mid-March just as the storm was approaching. Upon review two weeks later, much had changed and when this is published, the world will likely be a very different place. What I now see is that I had underestimated the toll from the free-floating anxiety brought on by this crisis and how important it is for each of us to set aside time each day to take care of our emotional health. And, take care of each other. Exercise and rest are important. Now more than ever, we need to treat each other (and ourselves) with kindness. The courage and compassion of our colleagues and our patients will strengthen us each and every day. I am confident that we all will rise to the difficult challenges for both ourselves and our patients.

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COVID-19 Webpage

By Jessica Gillis-Smith, MPH

The CIBMTR launched a COVID-19 Updates webpage to serve as a single source of information regarding all CIBMTR communications and activities related to the COVID-19 pandemic. The webpage shares COVID-19 data reported to the CIBMTR and breaks down the data by the type of cellular therapy, age at infection, US region, and other factors on the COVID-19 Reported Data subpage. Data are updated every weekday.

The COVID-19 webpage provides updates regarding changes to our data collection, including links to the new Respiratory Virus Post-Infusion Data Form (2149). We strongly encourage all centers to continue reporting to the CIBMTR, particularly COVID-19 infection data. The CIBMTR and ASTCT recently released a position statement urging continued consent and enrollment of patients in the CIBMTR Database Protocol. However, we understand some centers may submit data at a reduced rate, or not at all, during this pandemic. CPI requirements for this trimester are suspended; on-site data audits are on hold, and the requirement that forms be submitted within one year of their due date in order to be reimbursed is suspended for forms due 3/1/20 or later.

The CIBMTR continues to conduct studies and has fast-tracked studies related to COVID-19. The COVID-19 webpage shares COVID-19 published study results and describes the impact of the pandemic on our ongoing studies. The webpage also lists all CIBMTR COVID-19 communications and provides links to additional resources.

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BMT CTN COVID-19 Response

By Amy Foley, MA

The BMT CTN team, like all of you, has been focused on the COVID-19 pandemic. Our primary concern is patient safety and the safety of BMT CTN investigators and research staff. We are working with each protocol team to determine how COVID-19 impacts each BMT CTN trial, from accrual to feasibility of required evaluations and sample collection. The protocol teams considered whether accrual should be temporarily suspended and treatments and evaluations should be revised during

the pandemic. The deliberations, some still ongoing, include study drug distribution, safety reporting, protocol variances, study lab assessment requirements, patient visit schedules, monitoring plans, and effects on study endpoints. Just when we all think we've considered every area of impact, another is brought to light. Protocol teams have commendably addressed, and continue to address, all issues related to the crisis.

As of April 10, accrual to all BMT CTN studies but one is temporarily suspended. The sole exception is the BMT CTN 1502 (Haploidentical BMT for Aplastic Anemia) trial, which remains open to accrual given the acute need for transplantation in some of these patients. For already-enrolled patients, sites continue to collect data to the best of their ability. Protocol teams are evaluating protocols to determine if telehealth visits are feasible and which assessments are critical.

Thus far, we have distributed more than two dozen COVID-19-related communications to keep BMT CTN sites apprised of protocol and Network updates. Additionally, there have been multiple communications with the Institutional Review Board, the Data and Safety Monitoring Boards, and NIH. As a reminder, all BMT CTN documents related to COVID-19 are posted on a single designated page on the password-protected BMT CTN SharePoint website in addition to being distributed via email. While BMT CTN Data and Coordinating Center (DCC) staff members are all working remotely from home, there has been no impact to DCC operations, and we do not anticipate there will be.

We're hopeful the next newsletter article is about BMT CTN studies being reopened to accrual and "lessons learned" from the COVID-19 crisis. In the meantime, we hope that you, your families, and your patients stay safe and healthy.

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)

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Lymphoma Working Committee

Co-Chairs:

- Mohamed Kharfan-Dabaja, MD, MBA, Mayo Clinic, Jacksonville, FL
- Craig Sauter, MD, Memorial Sloan Kettering Cancer Center, New York, NY
- Alex Herrera, MD, City of Hope, Duarte, CA

Scientific Director:

- Mehdi Hamadani, MD, Froedtert Hospital, Medical College of Wisconsin, CIBMTR Milwaukee

Statistical Director:

- Kwang Woo Ahn, PhD, CIBMTR Milwaukee

Statistician:

- Andrew St. Martin, MS, CIBMTR Milwaukee

The Lymphoma Working Committee, one of the first established within the CIBMTR, focuses on cellular therapy for both Hodgkin (HL) and non-Hodgkin (NHL) lymphomas and has conducted numerous studies addressing a wide range of issues in the field for these diseases. Although the number of autologous and allogeneic HCTs have been steadily increasing over time, for both HL and NHL, the role and optimal timing of HCT is evolving with new pharmacologic and immunologic therapies. In addition, advances in molecular profiling / subtyping and the discovery of new biologic risk factors, combined with the increasing utilization of metabolic imaging in lymphomas, have shed light into the vast heterogeneity of lymphomas in general and led to identification of multiple subsets with different clinical behavior.

Since 2008, the disease-specific research-level forms have collected basic PET scan information, such as whether a PET scan was performed or not and whether it identified presence of disease at any site prior to the start of the conditioning regimen. Since 2013, the updated forms collected additional PET information, for

instance, whether there was nodal versus organ involvement, and not just pre-transplant but also at the time of disease transformation. In 2018, an updated version of lymphoma disease-specific forms was implemented to capture molecular testing information, such as identifying the presence of chromosomal translocations and other molecular subtyping information, such as germinal center versus non-germinal center "cell of origin" in the case of diffuse large B-cell lymphoma. This additional information will allow the Lymphoma Working Committee to conduct more up-to-date and clinically relevant analyses in the future. At the same time, the increasing use of new cellular therapy strategies (e.g. CAR T-cell therapies) will result in additional important data for the Lymphoma Working Committee to capture and analyze.

Given the considerable number of lymphoma patients treated with HCT whose data are in the CIBMTR Research Database, the Lymphoma Working Committee is able to provide information with the capacity to influence clinical practice in various transplant-related matters.

The Lymphoma Working Committee has been extremely active over the last few years, in large part due to the extensive data available in the CIBMTR Research Database. The number of transplants for lymphoma added to the Research Database from 2000 through 2019 are listed in the table below. During the annual committee meeting at the 2020 TCT Meetings of ASTCT and CIBMTR, 10 new proposals were presented, and 2 were approved to be further developed and analyzed. More than 150 people attended the Lymphoma Working Committee meeting, and the great involvement from the transplant community helps the committee to produce timely and substantive research.

The Lymphoma Working Committee was also quite productive with presentations and publications in 2019. Committee investigators presented 5 oral abstracts at national and international conferences, including 3 at the ASH Annual Meeting and 2 at the EBMT Annual Meeting. They published 4 manuscripts in peer-reviewed journals, including Journal of Hematology and Oncology, Blood Cancer, Biology of Blood and Marrow Transplantation, and Blood Advances.

Number of Cases Added to the CIBMTR Research Database 2000-2019		
	TED-Level Data	CRF-Level Data
Non-Hodgkin Lymphoma		
Allogeneic HCT	14,298	4,616
Autologous HCT	47,915	5,479
Hodgkin Lymphoma		
Allogeneic HCT	3,407	669
Autologous HCT	17,724	1,722

View planned, in-progress, and completed studies and publications on the Lymphoma Working Committee webpage.

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Graft-versus-Host Disease Working Committee

Co-Chairs:

- Margaret MacMillan, MD, MSc, University of Minnesota, Minneapolis, MN
- Madan Jagasia, MD, MS, MMHC, Vanderbilt University Medical Center, Nashville, TN
- Carrie Kitko, MD, Vanderbilt University Medical Center, Nashville, TN

Scientific Directors:

- Mukta Arora, MD, MS, University of Minnesota Blood and Marrow Transplant Program, CIBMTR Minneapolis
- Stephen Spellman, MS, CIBMTR Minneapolis

Statistical Director:

- Tao Wang, PhD, CIBMTR Milwaukee

Statistician:

- Karen Chen, MS, CIBMTR Milwaukee

GVHD is the most critical complication of allogeneic HCT, and its occurrence prevents favorable outcomes in a large proportion of affected patients. GVHD is an entirely iatrogenic complication of allogeneic HCT, and its prevention and treatment are of paramount importance to the transplantation community. In the last few years, the prevention and treatment of GVHD has received increased attention as alternative HLA mismatched donors are increasingly used in transplantation and as novel immunosuppressive agents are developed for the treatment of the autoimmune and rheumatologic conditions. The GVHD Working Committee examines both acute and chronic GVHD outcomes across all diseases treated by allogeneic HCT.

Under the committee leadership (listed above), the GVHD Working Committee focuses on:

- Comparing GVHD incidence, presentation, GVHD-free and relapse-free survival, and other outcomes among various:
  - GVHD prophylaxis regimens, including calcineurin inhibitor and mycophenolate mofetil based regimens as well as post HCT cyclophosphamide-based regimens
  - Types of donors, including HLA haploidentical donors, matched sibling donors, and matched and mismatched unrelated donors
  - Stem cell sources, including bone marrow, PBSC, and umbilical cord blood.
- Evaluating the impact of T-cell dose on GVHD risk after allogeneic HLA-matched PBSC transplantation
- Evaluating the impact of early broad-spectrum antibiotics and risk of acute GVHD in children
- Investigating the impact of donor clonal hematopoiesis on GVHD

The GVHD Working Committee has 8 ongoing studies (6 in progress and 2 newly accepted proposals) with plans to complete at least 3 studies each academic year, making it a very productive committee. This committee benefits from broad enrollment to the CIBMTR Research Database. The number of allogeneic HCTs in 2000-2019 total >50,000 for leukemic diseases (AML, ALL, MDS, CML, and other leukemias) and >20,000 for non-leukemia malignancies.

The GVHD Working Committee always seeks interesting and novel ideas for study, and it encourages the involvement of junior investigators and those wanting to break into the field of BMT and outcomes research. Join the GVHD Working Committee for their annual in-person meeting during the TCT Meetings of ASTCT and CIBMTR in Honolulu, Hawai'i, Feb 10-14, 2021.

View planned, in-progress, and completed studies and publications on the GVHD Working Committee webpage.

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2020 TCT Meetings of ASTCT and CIBMTR

By Tia Houseman

TCT | TRANSPLANTATION & CELLULAR THERAPY MEETINGS of ASTCT and CIBMTR

2020 marked the 25th anniversary of ASTCT and CIBMTR co-hosting the TCT Meetings of ASTCT and CIBMTR, previously known as the BMT Tandem Meetings. 4,195 registered attendees (150 more than the 2019 TCT Meetings of ASTCT and CIBMTR) attended the 2020 TCT Meetings of ASTCT and CIBMTR at the World Center Marriott in Orlando, Florida, Feb. 19-23. Attendees came from 48 countries (4 more than 2019). Countries with the highest attendance included the US, Canada, Brazil, Japan, Germany, Australia, and the United Kingdom. Just ahead of the COVID-19 pandemic in the US, the meetings experienced fewer than 20 cancellations, most from China due to travel restrictions.

2020 Scientific Organization Chairs, Mary Flowers, MD, and Katy Rezvani, MD, PhD, along with the scientific organizing committee assembled an excellent program consisting of 6* plenary sessions and 9 concurrent sessions. The meeting

also included 4 breakfast symposia and 5 luncheon symposia, 13 oral abstract sessions with 90 abstracts presented, 7 Product Theaters, 2 poster sessions, 15 CIBMTR Working Committee meetings, 10 ASTCT Special Interest Group afternoon sessions, 8 Meet-the-Professor sessions, and a Pediatric Day. In addition to an outstanding scientific program, parallel sessions were held for administrative directors, BMT CTN coordinators, BMT CTN investigators, clinical research professionals / data managers, information technologists, nurse practitioners, pharmacists, and transplant nurses.

**This year ASTCT, CIBMTR, and EBMT partnered to offer an additional plenary session related to bridging the gap in BMT and cellular therapy between North America and Europe.*

Awards

CIBMTR Distinguished Service Award: Jong Wook Lee, MD, PhD

The Distinguished Service Award recognizes an individual who has made outstanding contributions to the CIBMTR's research mission in one or more of the following areas: Promoting HCT research and clinical care in developing countries, advancing the field despite unique challenges, expanding the availability of transplantation, disseminating research results to clinicians and patients to improve outcomes and quality of life, and collaboration with organizations to increase data exchange and research collaboration worldwide.

ASTCT Lifetime Achievement Award: Keith Sullivan, MD

The ASTCT Lifetime Achievement Award recognizes an individual who has made continuing contributions to the field of blood and marrow transplantation, either in basic biology or clinical application.

ASTCT Public Service Award: Meredith Cowden Foundation

Lectures

Mortimer M. Bortin Lecture: Shinichiro Okamoto, MD, PhD: "Challenges of Hematopoietic Cell Transplantation in Asia-Pacific Countries / Regions"

The Mortimer M. Bortin Lecture commemorates the Founding Scientific Director of the International Bone Marrow Transplant Registry (IBMTR, forerunner of the CIBMTR), whose foresight and dedication were critical to the development of the CIBMTR as a global resource of HCT research. Lecturers are chosen on the basis of their contributions to our understanding of graft-versus-tumor effects and/or the advancement of clinical HCT research.

E. Donnall Thomas Lecture: Effie W. Petersdorf, MD: "Immunogenetics of Hematopoietic Cell Transplantation"

In honor of Dr. Thomas, the E. Donnall Thomas Lecture recognizes an eminent physician or scientist, either a clinician or investigator, who has contributed meritoriously to the advancement of knowledge in HCT.

Networking

Attendees had the opportunity to network with colleagues during several dedicated events, including 2 poster sessions, the TCT Meetings of ASTCT and

CIBMTR Networking Reception in the Exhibit Hall, the TCT Meetings of ASTCT and CIBMTR 25th Anniversary Celebration Saturday evening, and more.

See you in 2021!

Watch for details on the 2021 TCT | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR in the coming months. Contact TCTMeetings@mcw.edu for information regarding support opportunities for next year's meeting.

On behalf of the TCT Meetings of ASTCT and CIBMTR Core Team we wish to **thank you** for all of your time, hard work, and dedication to the field, especially during the COVID-19 pandemic. We look forward to seeing you at the Hawai'i Convention Center in Honolulu, Hawai'i, Feb. 10-14, 2021.

Join the conversation: #TCTM21

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Congratulations, Roberta King!

Roberta King was recognized for her 30 years of service to NMDP/Be The Match and CIBMTR. Congratulations, Roberta!

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2020 TCT Meetings of ASTCT and CIBMTR: Clinical Research Professionals / Data Management Conference

By Sharon Meiers

Best Oral Abstract recipient, Kayla Woodring, poses for a photo with Janet Brunner-Grady, PA-C, Program Director- Data Operations.

We had a great turn out for the Clinical Research Professionals / Data Management meeting at the 2020 TCT Meetings of ASTCT and CIBMTR in sunny Orlando, Florida. Our meetings took place on February 18 and 19 with an average of 275 data professionals each day.

Speaker highlights included cytogenetics, data transformation, cellular therapy, and updates from Center Support and the audit team. New this year: 3 pediatric breakout sessions on SCIDS, Fanconi anemia, and neuroblastoma. These new sessions were both well attended and a big hit.

Kayla Woodring received this year's award for best oral abstract for her presentation titled, "Rebuilding a Data Management Team to Improve Program Efficiency and Clinical Compliance."

Additional oral abstracts included:

- "A Diamond in the Rough!" – presented by Nicolette Minas and Kathleen Ruehle
- "Strategies to Increase the Number of Centers Reporting Data to CIBMTR" – presented by Heliz Regina Neves and Cinthya Correa da Silva

On Feb. 20, we held a new data manager onboarding session for approximately 35 registered data managers. The workshop included interactive activities, such as registering a new CRID, understanding how forms come due, and how to use CIBMTR Portal applications like Enhanced Data Back to Centers. Important topics for new data mangers included CPI, annual reports such as transplant center specific analysis and center volumes data report, and information about how to respond to queries. We ended the day with disease overviews and hands on activities using disease trackers. It was a very productive and educational day for all.

Presentations from the Clinical Research Professionals / Data Management meeting are available on the CIBMTR Data Management CRP / DM Conference webpage.

Special thanks to all who completed their evaluations! We use these data to improve our meetings from year to year. Please SAVE the DATE for 2021. Our meeting will take place in Honolulu, Hawai'i, on February 9 and 10.

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CIDR Solid Tumor Task Force, Cellular Therapy Slides, and Upcoming Year

By Carlos Litovich, MPH

Since its inception, the CIDR has been a resource for the community, built by partnering with the community. The CIDR is launching a Solid Tumor Task Force to obtain guidance on how to prepare for the future of cellular therapy. The task force will benefit from the input of experts in the solid tumor arena and members of the CIBMTR.

The main goals are to forecast the future of cellular therapy for solid tumors and to inform the CIBMTR on the path to proactive adaptation including prioritizing revamping relevant forms. The task force will guide the CIBMTR's form development process and provide continuing advice to best position the CIDR for the future. More updates will come soon. We plan to provide a task force summary near the end of summer.

The CIDR is also pleased to announce the release of the Cellular Therapy Slides, complementing the CIBMTR Summary Slides. The Cellular Therapy Slides include a summary of the timeline of our milestones, annual number of recipients since 2016, and additional interesting information.

In other news, the Cellular Immunotherapy for Cancer Working Committee's meeting during the 2020 TCT Meetings of ASTCT and CIBMTR was groundbreaking. With more than 300 attendees, the community is greatly engaged in this newly formed committee, which received 52 proposals in its kick-off year! Four studies were accepted, so we are positive the committee will have an extremely productive year and will continue to be a great resource for a rapidly growing community.

Finally, the Cellular Therapy Forum preparations are underway, aiming for early October. We already have a packed agenda and many thought-provoking meetings planned. The Cellular Therapy Forum is quickly becoming the premier cellular therapy meeting for the community, giving an opportunity to a myriad of stakeholders to share thoughts on this evolving field and shape the future of the resource.

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Deep Dive Into Clinical Trial Monitoring

By Jennifer Larsen, Justin Peterson, and Abby Hendrickson

Clinical trial monitoring is an essential component of trial quality assurance. The sponsor of a clinical trial is responsible for ensuring the trial is adequately monitored. Clinical trial monitoring is conducted by the sponsor or delegated appropriately to a contract research organization (CRO). The purposes of trial monitoring are to verify:

1. Rights and well-being of human subjects are protected.
2. Reported trial data are accurate, complete, and verifiable from source documents.
3. Conduct of the trial is in compliance with currently approved protocols / amendments.

Clinical research monitors, also known as clinical research associates (CRAs), have several responsibilities that warrant their role as a critical function in clinical research. Per the International Conference on Harmonization Guidelines for Good Clinical Practice (ICH GCP), monitors must receive appropriate training and have the scientific and / or clinical knowledge needed to monitor the trial adequately. Responsibilities include acting as a primary line of communication between the sponsor and the investigators, verifying investigational product accountability, verifying appropriate receipt of written informed consent, and ensuring the investigator follows the approved protocol and all approved amendments.

The CIBMTR monitoring program provides monitoring services for several RCI BMT, BMT CTN, PTCTC, investigator-initiated, industry, and internally sponsored studies. There are three strategies utilized for monitoring clinical studies. The first is onsite visits in which a CRA travels to the site to conduct the visit. Visit types may include a site qualification visit, site initiation visit, interim monitoring visit, or site closeout visit. Another monitoring strategy is remote visits. Some clinical trials may fully rely on remote monitoring while others may use remote monitoring for targeted source documents, such as eligibility review. Lastly, the monitoring team is involved in centralized monitoring. Centralized monitoring is the remote review of data to identify unreliable, non-conformant data and assess for protocol compliance. The monitoring plan for a clinical trial will describe the scope and monitoring strategies. The sponsor and CRO will consider the disease area, study population, study design, the number of sites, and other critical areas when determining the extent of monitoring required.

Due to the COVID-19 pandemic, CIBMTR CRAs are not currently traveling to conduct on-site monitoring visits. However, team members have adjusted their workflow to continue adequately monitoring clinical trials. The team is currently exploring expanded remote monitoring procedures and have even implemented these procedures on several studies.

The RCI BMT / Prospective Research Monitoring Team includes Ashley Birch, Abby Hendrickson, Amanda Castellon, Catherine Calistro, Chew Vang, Cynthia Kunakom, Jennifer Larsen, Justin Peterson, Kailey Zaffran, and Anna Yost.

References:

1. *International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, Guideline for Good Clinical Practice E6(R1), [https://ichgcp.net/518-monitoring/]*

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Health Services Research Team Uses Administrative Claims Data

By Jaime Preussler, MS; Christa Meyer, MS; Tatenda Mupfudze, PhD; Lih-Wen Mau, PhD, MPH

The Health Services Research team utilizes multiple research methods, including but not limited to retrospective and prospective research, administrative claims data analysis, survey design and administration, data management, and dataset linkage.

Administrative claims data are generally available (at a cost) from payers, and the data represent "real-world" experiences of patients and health care providers. This type of data provides a way to study and estimate patterns of costs and utilization of cellular therapy. The Health Services Research team currently has administrative claims data from Medicare from 2010 to 2016 and Medicaid from 2010 to 2014 and is requesting more recent CMS data. The available CMS data have been linked with CIBMTR clinical outcomes data (for patients who received transplants); this linked CIBMTR-CMS dataset allows inclusion of variables from the CIBMTR that are not available in claims data (e.g., transplant-related and outcomes data).

The Health Services Research team is currently working on protocols that use these data to study the following:

- Sickle cell disease
- Referral and treatment patterns for patients with AML
- Access to allogeneic HCT for patients with AML, MDS, and multiple myeloma

For more information about the Health Services Research Program, visit the Health Services Research webpage or email HSRrequests@nmdp.org.

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Team Spotlight: Immunobiology Research

By Steve Spellman, MBS

The CIBMTR's Immunobiology Research Program is led by Assistant Scientific Director Stephen Spellman, MBS, while Senior Scientific Director Stephanie Lee, MD, MPH, provides scientific oversight. The team is based on the Minneapolis campus and comprised of eight members: Alan Howard, Allison Maclean, Alyssa Carlson, Anu Elayaperumal, Ashley Spahn, Cynthia Vierra-Green, Maria Brown, and Stephanie Waldvogel.

Established in 2010, the Immunobiology Research Program supports the CIBMTR Working Committees, mainly the GVHD and Immunobiology committees, conducting correlative sample testing studies. The program is responsible for managing all CIBMTR research sample collection and sample utilization. This includes the unrelated and related donor / cord blood pre-transplant sample collections and prospective clinical trial sample collection supporting the RCI BMT and BMT CTN. The CIBMTR related and unrelated donor transplant repository contains samples from nearly 180,000 individuals, and the program distributed >12,500 samples to investigators last year. The Immunobiology Research team works closely with the Repository Steering Committee comprised of the NMDP Histocompatibility Advisory Group to monitor sample submission compliance, sample processing protocols, and approval of CIBMTR studies that use the stored samples. Details on the research sample inventory are available on the CIBMTR and BMT CTN websites.

The Immunobiology Research team manages immunogenetic testing programs that perform retrospective high-resolution HLA and KIR typing on stored donor and cord-recipient pairs. This testing program not only provides key immunogenetic data for histocompatibility research but also confirms sample identity in the repository for future analyses. In a typical year, the program tests >4,000 donor / recipient pairs. The immunogenetic database includes >35,000 pairs with retrospectively validated HLA data, >18,500 pairs with KIR presence / absence data. The CIBMTR immunogenetics database incorporates HLA and KIR data for future studies. In addition, the team provides HLA expertise for validation of all typing submitted on CIBMTR data collection forms prior to inclusion in research studies. The team also provides scientific expertise and logistical support to establish laboratory contracting and sample testing services for the CIBMTR, BMT CTN, and RCI BMT studies.

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Bioinformatics Research

By Yung-Tsi Bolon, PhD

Pradeep Bashyal -
Bioinformatics Software Architect

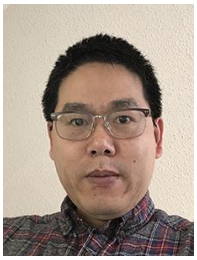
Stephanie Fingerson
Bioinformatics Scientist

Abeer Madbouly
Principal Bioinformatics Scientist

Tolutola Oyetunde
Bioinformatics Scientist

Ray Sajulga, Jr.
Bioinformatics Scientist

Wei Wang
Bioinformatics Scientist



Tao Zhang
Senior Bioinformatics Scientist

Yung-Tsi Bolon
Director, Bioinformatics Research

The Bioinformatics Research team drives high-impact research innovations and produces strategic applications for the business to bridge the transition from research to operations. At the intersection of science and technology, this team moves in the direction of computational biomedicine with three main pursuits:

1. Analyze molecular profiles, integrate clinical and other data sources, and improve patient outcome predictions:

- *What unknown molecular factors play a role in disease risk and progression?*
- *Which factors associate with patient survival and quality of life?*
- *How can we improve prediction accuracy for better transplant outcomes?*

The team utilizes and develops data science / machine learning and genomics / omics high-throughput bioanalytics platforms and merges data collected in business and research operations at NMDP/Be The Match and the CIBMTR. We identify novel molecular factors that impact transplant outcomes, discover prognostic factors, and build prediction models that contribute to increased event-free survival for patients.

2. Understand donor registry, cord, biotherapies, and biobank potential to best serve diverse patient populations in the US and internationally:

- *What genetic diversity is reflected in patient populations in the US and internationally?*
- *Do therapy sources exist that meet the need?*
- *How can we close the gap?*

The team characterizes HLA haplotype frequencies and models the genetic composition and match likelihoods from donor registry, cord, biotherapies, and biobank sources to optimize access to cellular therapies for all patients in need. These and related projects help to increase the likelihood of finding a match for all patient populations and seek to prepare a ready source of cellular therapy as needed.

3. Find best cellular therapy matches for patients:

- *What helpful but missing information can we infer?*
- *Which cell source or therapy best fulfills the patient need?*
- *How can we translate research for the provider to improve patient outcomes in practice?*

The Bioinformatics Research team investigates algorithms to improve the prediction of missing data and the selection of cellular therapies for patients toward best survival and quality of life outcomes, especially for underserved populations. The translation of research results and evidence-based guidelines to user interfaces by the Bioinformatics Research team helps to optimize cellular therapy matching and donor selection processes.

We interpret molecular data, build prediction models from integrated data sources, characterize diverse genetic population needs and potential, drive new technology applications, and translate research findings into practice to save lives.

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Research Datasets Available for Secondary Analysis

By Liz Siepmann

In accordance with the NIH Data Sharing Policy and NCI Cancer Moonshot℠ 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.

Datasets are now available online.

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CIBMTR Data: Enhanced and even more secure!

By Amna Zafar, Erik Bergman, Tad Degen

To continue our commitment to information security and data protection, the CIBMTR applied Okta Multifactor Authentication to the CIBMTR Portal. The login experience to the Portal is now both more convenient and more secure for centers, blood banks, and external partners by applying the same multifactor solution and credentials as FormsNet3. The CIBMTR also took this opportunity to introduce a new look and feel to the Portal. New icon-driven, card-design architecture is utilized to offer a more modern aesthetic and streamline the data access, requiring fewer clicks to view the desired data set.

DBtC (Data Back to Centers) expanded the variables that are available to our centers. Within DBtC Data Download, MyTED now provides nearly 2,000 Transplant Essential Data (TED) variables to download. Similarly, MyCTED provides nearly 7,500 CAR-T data points available for download for the first time.

Keeping in theme with these enhancements, the CIBMTR is looking forward to future enhancements of DBtC this summer, including updates to the user interface, improved organization of HCT and CAR-T data, and new visualizations.

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Sharing Research in Plain Language

By Jennifer Motl

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

More radiation not necessarily better

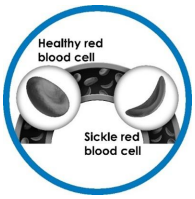
Irradiation therapy helps most people with leukemia before blood or marrow transplant

Read more

Transplant can help people aged 65 and older with myelodysplastic syndromes (MDS)

Experts recommend that Medicare pay for transplant

Read more



Blood or marrow transplant can help children with severe sickle cell disease

Matched, sibling donor is best

Read more

Certain medicines used for bone marrow transplant (BMT) offer better cure for severe aplastic anemia (SAA)

For people who got BMT from a matched brother or sister, the best medicines were cyclophosphamide together with anti-thymocyte globulin (ATG), given with or without fludarabine

Read more

Unlicensed cord blood transplants can help people of color

Umbilical cord blood is safe, effective treatment for many diseases

Read more

Freezing cells for blood and marrow transplant saves lives

Study helps people with cancer get life-saving transplants during pandemic

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 as well as Twitter, Facebook, and LinkedIn.

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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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