



Health Services Research Program

Annual Report

2017

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

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HEALTH SERVICES RESEARCH PROGRAM

Hematopoietic cell transplantation (HCT) is an established treatment for patients with life threatening hematologic cancers and other diseases. At the same time, it is a highly specialized and resource-intensive medical procedure. Approximately 20,000 HCTs are performed in the United States annually, and that number is expected to increase with technological advances, improved outcomes and the introduction of newer indications and donor sources.

To address the need for more research in health services and health policy issues related to HCT, the National Marrow Donor Program®/Be The Match® (NMDP®) operationalized the Health Services Research (HSR) Program in 2006. The program was formally established in 2008, successfully integrating CIBMTR resources and expertise in HCT-related research with NMDP/Be The Match Patient and Health Professional Services expertise in HSR, access to networks, and relationships with patients and health care providers. The CIBMTR HSR Program conducts research using quantitative and qualitative research methods including focus groups, survey research, secondary data analysis, health economics and outcomes research, patient-reported outcomes, and randomized, controlled trials.

What is Health Services Research?

HSR studies how social factors, financing systems, organizational structures and processes, health technologies and personal behaviors affect access to health care, the quality and cost of health care, and ultimately patients' health and well-being (AcademyHealth, 2012). Recent HSR focus has been on patient-centered outcomes research, which involves including patients and caregivers in the research process and emphasizes helping patients and their caregivers communicate and make informed healthcare decisions (PCORI, 2013).

HSR Program Goals

Conduct HSR that contributes knowledge to the field and informs HCT policy, clinical practice, and survivorship issues, while fostering a culture of learning, integrity and excellence:

- Identify and address barriers to access to HCT
- Improve practice of HCT
- Demonstrate the value of cellular therapies (compared to other therapies) and survivorship care through research and dissemination of findings

HSR PROGRAM FOCUS AREAS

The HSR program conducts research on several health services and health policy topics related to HCT, including but not limited to: healthcare disparities in access to and outcomes of HCT; characterizing patient barriers; economic aspects of HCT (e.g. costs, cost-effectiveness); patient and population health status and quality of life; practice patterns and their impact on HCT outcomes; quality of care; and survivorship. Its activities complement research conducted using the CIBMTR database by the CIBMTR Health Services and International Issues Working Committee. The Program's agenda is addressed via six major focus areas (**Figure 1**).

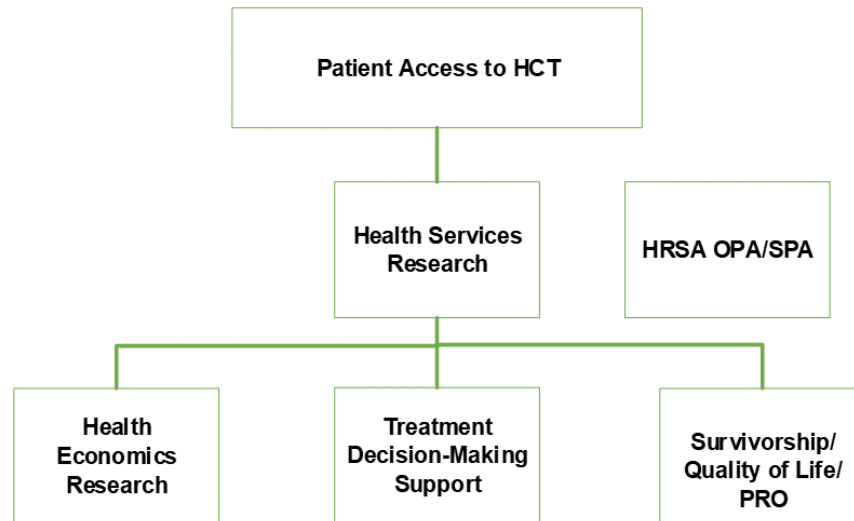


Figure 1. HSR Program Research Portfolio Focus Areas

HEALTH ECONOMICS RESEARCH

- *Reimbursement Analysis of Hematopoietic Cell Transplantation in Older Patients:* This study aims to describe the costs of HCT, billing and coding practice variation among transplant centers, and variation in payment from the Centers for Medicare and Medicaid (manuscript in progress).
- *Cost-Effectiveness Analysis of Allogeneic Hematopoietic Cell Transplantation versus Chemotherapy Alone in Patients with Acute Myeloid Leukemia:* This study will describe the costs of chemotherapy with or without allogeneic HCT in Patients with acute myeloid leukemia and age 20-74 years using Optum Database (data preparation phase).
- *Health Care Utilization and Costs Associated with Patients with Sickle Cell Disease (SCD) Enrolled in Medicaid:* This study will describe health care utilization and costs for patients with SCD enrolled in Medicaid between 2010 and 2014 who were treated with either alloHCT or according to current standard clinical practice (non-HCT) including hydroxyurea and chronic blood transfusions (protocol in development).
- *Reimbursement and Outcomes in Medicare beneficiaries with outpatient and inpatient autologous hematopoietic cell transplantation (HCT) for multiple myeloma:* This study will describe Medicare reimbursement, patient out-of-pocket costs, and clinical outcomes for older patients with multiple myeloma (data analysis).

TREATMENT DECISION-MAKING SUPPORT

- *Payer-Partnered Approach to Community-Based Referral for Hematopoietic Cell Transplantation (HCT):* Phase one of this study aims to identify practice gaps associated with referral to HCT consultation among hematologists/oncologists by conducting three surveys (national, provider with an open-network and provider with a closed-network) as well as focus groups. The goal of the needs assessment is to gain a better understanding of referral practice patterns, clinical-decision making and educational preferences. The results of the needs assessment were used in phase two to create an educational intervention, 3-part webinar series, to address practice gaps (phase 2 data collection in progress).

- *Easy-To-Read Informed Consent For Hematopoietic Cell Transplantation Clinical Trials*: This NHLBI-funded study has two parts:
 - A randomized-controlled trial evaluated the effectiveness of a novel easy-to-read consent form for Blood and Marrow Transplant Clinical Trial Network (BMT CTN) clinical trials. An evaluation study gathered feedback on the perceived helpfulness of the ETRIC template and possible barriers to implementing the template at BMT CTN centers via interviews with network PIs, IRB staff, and research nurses and coordinators at nine centers (manuscript under review).
- *Physician and Patient Perspectives on Palliative Care in HCT*: This study seeks to study access and barriers to palliative care service and identify unmet palliative care needs. This will be done through national web-based survey of physicians and medical directors that provide direct clinical care for HCT patients to meet the study objectives. Data from the Transplant Physicians Perspectives on Palliative Care Survey will be used to generate actionable data that can be used to better understand the role of palliative care in HCT and facilitate more constructive integration. The study will inform the work of the ASBMT Palliative Care Task Force (analysis phase).
- *Translation of clinical research into practice: An impact assessment of the results from the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Protocol 0201 on unrelated graft source utilization*: This study includes 2 phases. Phase 1 describes the trends in graft source utilization before versus after the BMT CTN 0201 clinical trial results were presented at the American Society of Hematology annual meeting. Phase 2 describes assessed clinical decision-making and system level factors for graft selection, knowledge of BMT CTN 0201 results, and opinions regarding translation of research to practice both with respect to the study and generally (manuscript submitted).

SURVIVORSHIP/QoL/PROs

- *Individualized care plans for HCT survivors*: This study evaluated a personalized treatment summary and care plan for HCT survivors. Focus groups of patients/caregivers and transplant center and community clinicians were conducted to optimize care plan content, format and delivery. A randomized study conducted in collaboration with the Resource for Clinical Investigation in Blood and Marrow Transplant (RCI BMT) evaluated whether the care plan enhanced patient knowledge, health behaviors and health care utilization (manuscript preparation in progress; funded by a Patient-Centered Outcomes Research Institute award #CD-12-11-4062).
- *Developing a Patient Centered HCT Outcomes Research Agenda*: This initiative aims to engage a broad community of patients and other key stakeholders to identify, prioritize, communicate, and promote an HCT-related patient centered outcomes research (PCOR) agenda; build PCOR capacity; and leverage partnerships to advance the agenda (manuscript published in BBMT; funded by a Patient-Centered Outcomes Research Institute award #EAIN-2956).
- *INSPIRE: A Multicenter Randomized Controlled Trial Integrating Health Informatics in a Scalable Stepped Care Self-Management Program for Survivors After Hematopoietic Cell Transplantation*: This multi-center clinical trial aims to determine the efficacy of a tailored online, self-management stepped care program and survivorship care plan intervention in adult 2-5 year HCT survivors. Funded by the National Institutes of Health (PI: K Syrjala, Fred Hutchinson Cancer Research Center), the HSR Program and Survey Research Group are collaborating on intervention design, data collection and analysis (qualitative data analysis in progress; trial protocol in development).

ACCESS TO HCT

- *Hematopoietic Cell Transplantation Multidisciplinary Care Teams: Burnout, Moral Distress and Career Satisfaction*: One set of challenges for health professionals in HCT involves adverse consequences of caring: compassion fatigue (secondary traumatic stress), burnout and vicarious traumatization. A web-based survey of HCT health professionals has been conducted to characterize the factors that lead to work-related distress. The study will inform research on strategies and interventions to mitigate these issues in order to overcome some of the barriers of recruitment and retention of workforce in HCT (manuscript published in BBMT).

INFORMATION AND DISSEMINATION ACTIVITIES

Figure 2 illustrates the HSR Program dissemination efforts including publications and abstracts accepted for presentation at national, professional conferences and meetings. In 2017, 3 manuscripts were published in peer-reviewed journals (Appendix A), and 8 abstracts (research and evaluation) were accepted for presentation at professional conferences.

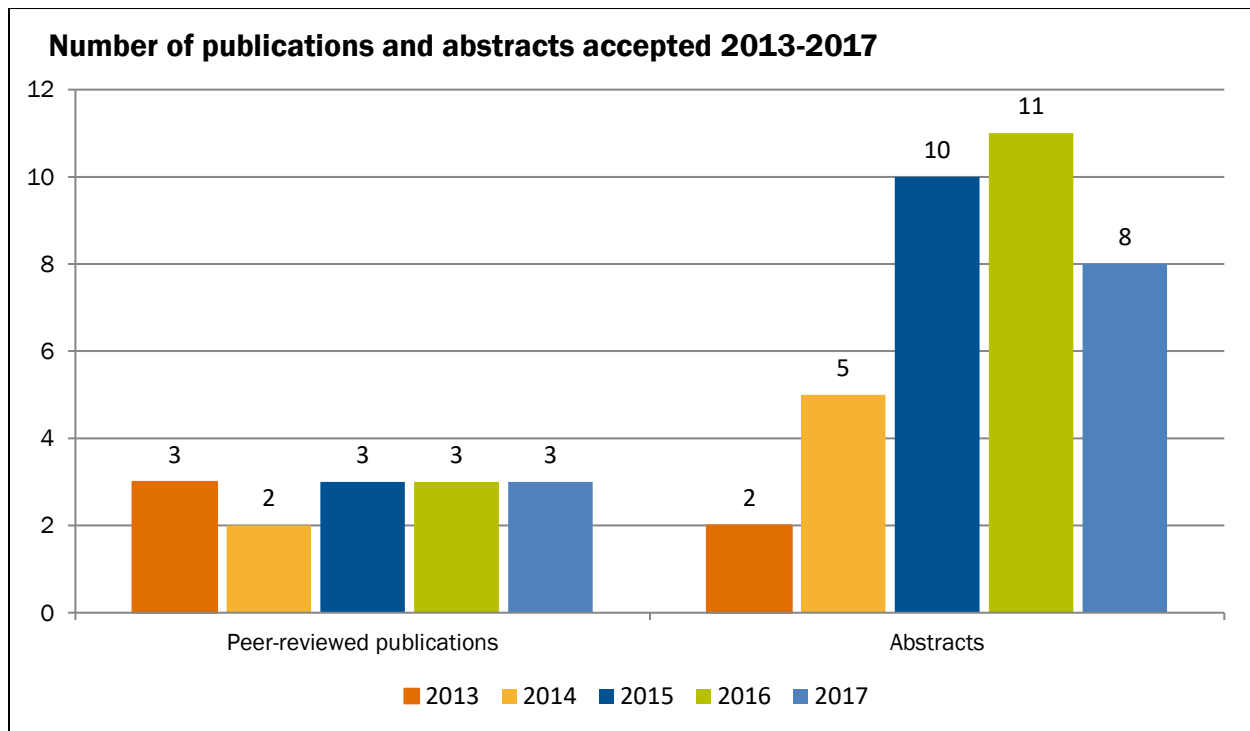


Figure 2. HSR Program publications and abstracts

Appendix A: HSR Program Publications, 2010-2017

*Not peer-reviewed

Health Economics Research

1. Preussler, J., Denzen, E.M., Majhail, N.S. (2012). Costs and cost-effectiveness of hematopoietic cell transplantation. *Biology of Blood and Marrow Transplant*, 18(11):1620-1628.
2. Majhail, N. S., Mau, L.W., Denzen, E. M., Arneson, T. J. (2012). Costs of Autologous and Allogeneic Hematopoietic Cell Transplantation in the United States: A Study Using a Large National Private Claims Database. *Bone Marrow Transplantation*, 48, 294–300.
3. Majhail, N. S., Rizzo, J. D., Hahn, T., Lee, S. J., McCarthy, P. L., Ammi, M., Denzen, E. M., Drexler, R., Flesch, S., James, H., Omondi, N., Pedersen, T. L., Murphy, E. A., Pederson, K. (2013). Pilot Study of Patient and Caregiver Out-of-Pocket Costs of Allogeneic Hematopoietic Cell Transplantation. *Bone Marrow Transplant*. 48(6), 865–871.
4. Preussler, J. M., Mau, L.-W., Majhail, N. S., Meyer, C. L., Denzen, E., Edsall, K. C., ... Vanness, D. J. (2016). Administrative Claims Data for Economic Analyses in Hematopoietic Cell Transplantation: Challenges and Opportunities. *Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation*. <https://doi.org/10.1016/j.bbmt.2016.05.005>
5. Denzen, E. M., Thao, V., Hahn, T., Lee, S. J., McCarthy, P. L., Rizzo, J. D., ... Majhail, N. S. (2016). Financial impact of allogeneic hematopoietic cell transplantation on patients and families over 2 years: results from a multicenter pilot study. *Bone Marrow Transplantation*, 51(9), 1233–1240. <https://doi.org/10.1038/bmt.2016.103>
6. Preussler, J. M., Meyer, C. L., Mau, L.-W., Majhail, N. S., Denzen, E. M., Edsall, K. C., ... Vanness, D. J. (2017). Healthcare Costs and Utilization for Patients Age 50 to 64 Years with Acute Myeloid Leukemia Treated with Chemotherapy or with Chemotherapy and Allogeneic Hematopoietic Cell Transplantation. *Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation*, 23(6), 1021–1028. <https://doi.org/10.1016/j.bbmt.2017.02.017>

Survivorship/QoL/PROs

7. Clauser, S.B., Gayer, C., Murphy, E.A., Majhail, N.S., and Baker, K.S. (2015). Patient Centeredness and Engagement in Quality of Care Oncology Research. *Journal of Oncology Practice*, 11(3):176-179.

Access to HCT

8. Majhail NS, Omondi NA, Denzen E, Murphy, E.A., Rizzo, J.D. (2010) Access to hematopoietic cell transplantation in the United States. *Biology of Blood and Marrow Transplant*, 16(8), 1070-1075.
9. Murphy, E., Ferguson, S.S., Omondi, N.A., Getzendaner, L.C., Gajewski, J.L., Goldstein, G.A., Wingard, J.R., Rizzo, J.D., Mahjail, N.S. (2010). National Marrow Donor Program's symposium on patient advocacy in cellular transplant therapy: addressing barriers to hematopoietic cell transplantation. *Biology of Blood and Marrow Transplant*, 16(2), 147-156.

10. Majhail, N.S., Murphy, E.A., Omondi, N.A., Robinett, P., Gajewski, J.L., LeMaistre, C.F., Confer, D., Rizzo, J.D. (2011). Allogeneic transplant physician and center capacity in the United States. *Biology of Blood and Marrow Transplant*, 17(7), 956-961.
11. *Majhail, N.S., Chell, J.W., Snyder, E.L. (2012). Addressing workforce and infrastructure challenges to the growth of hematopoietic cell transplantation: The System Capacity Initiative. *The Hematologist*, 9(3):14.
12. Majhail, N.S., Murphy, E.A., Denzen, E., Stickney Ferguson, S., Anasetti, C., Bracey, A., Burns, L., Champlin, R., Hubbard, N., Markowitz, M., Maziarz, R.T., Medoff, E., Neumann, J., Schmit-Pokorny, K., Weisdorf, D.J., Yolin Raley, D.S., Chell, J., Snyder, E.L. (2012). The National Marrow Donor Program's symposium on hematopoietic cell transplantation in 2020: A health care resource and infrastructure assessment. *Biology of Blood and Marrow Transplant*, 18(2), 172-182.
13. *Murphy, E.A., Sylvanus, T., Tierney, D.K., Denzen, E.M., De Kesel Lofthus, A., Neumann, J. (2012). Addressing workforce and infrastructure challenges to the growth of blood and marrow transplantation: The System Capacity Initiative. *ONS Connect*, November, 20.
14. Denzen, E.M., Majhail, N.S., Stickney Ferguson, S., Anasetti, C., Bracey, A., Burns, L., Champlin, R., Chell, J., Leather, H., Lill, M., Maziarz, R.T., Medoff, E., Neumann, J., Schmit-Pokorny, K., Snyder, E.L., Wiggins, L., Yolin Raley, D.S., Murphy, E.A. (2013). Hematopoietic cell transplantation in 2020: Summary of Year II recommendations from the National Marrow Donor Program's System Capacity Initiative. *Biology of Blood and Marrow Transplant*, 19(1):4-11.
15. Majhail NS, Mau L-W, Chitphakdithai P, Payton T, ..., Murphy EA, and Denzen E. (2015). National Survey of Hematopoietic Cell Transplantation Center Personnel, Infrastructure, and Models of Care Delivery. *Biol Blood Marrow Transplant*, 21(7):1308-1314. doi:10.1016/j.bbmt.2015.03.020.
16. Neumann, J. L., Mau, L.-W., Virani, S., Denzen, E. M., Boyle, D. A., Boyle, N. J., ... Burns, L. J. (2018). Burnout, Moral Distress, Work-Life Balance, and Career Satisfaction among Hematopoietic Cell Transplantation Professionals. *Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation*, 24(4), 849–860. <https://doi.org/10.1016/j.bbmt.2017.11.015>
17. Stickney Ferguson, S., Randall, J., Dabney, J., Kalbacker, M. E., Boyle, N., Thao, V., ... Denzen, E. M. (2017). Perceived Workforce Challenges among Clinical Social Workers in Hematopoietic Cell Transplantation Programs. *Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation*. <https://doi.org/10.1016/j.bbmt.2017.12.793>

Program Evaluation

18. Omondi, N.A., Denzen, E., Jacobson, D., Payton, T., Pederson, K., Murphy, E.A. (2010). Evaluating patient satisfaction with the Office of Patient Advocacy. *Journal of Cancer Education*, 26(1), 44-50.
19. Moore, H.K., Preussler, J., Denzen, E.M., Payton, T.J., Thao, V., Murphy, E.A., Harwood, E. (2014). Designing and operationalizing a customized internal evaluation model. *Journal of Cancer Education*, 29(3), 463-472.