Not for publication or presentation

A G E N D A
CIBMTR WORKING COMMITTEE FOR DONOR AND RECIPIENT HEALTH SERVICES WORKING COMMITTEE
Honolulu, HI
Friday, February 14, 2025, 1:00 – 3:00 PM HST

Co-Chair:	**Leslie Lehmann, MD; Dana Farber Cancer Institute, Boston, MA; Telephone: 617-632-4882; Email: leslie_lehmann@dfci.harvard.edu**
Co-Chair:	**Hemalatha Rangarajan, MD; Nationwide Children's Hospital, Columbus, OH; Telephone: 740-953-0602; E-mail: hemalatha.rangarajan@nationwidechildrens.org**
Co-Chair:	**Fotios Michelis, MD, PhD; Princess Margaret Cancer Center, Toronto, ON, Canada; E-mail: fotios.michelis@uhn.ca**
Co-Chair:	**Minoo Battiwalla, MD, MS; Sarah Cannon BMT Program, Nashville, TN; Telephone: 615-342-7644; E-mail: minoo.battiwalla@hcahealthcare.com**
Page Scholar:	**Megan Herr, PhD; Roswell Park, Buffalo, NY; Telephone: (716) 845-3557; Email: megan.herr@roswellpark.org**
Scientific Director:	**Heather Stefanski, MD, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Minneapolis, MN; Telephone: 763-406-8465; E-mail: hstefans@nmdp.org**
Statistical Director:	**Ruta Brazauskas, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-456-8687; E-mail: ruta@mcw.edu**
Statistical Director:	**Brent Logan, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-8849; E-mail: blogan@mcw.edu**
Statistician:	**Gabrielle Schmidt, MPH; CIBMTR® (Center for International Blood and Marrow Transplant Research), Minneapolis, MN; Telephone: 763-406-3841; E-mail: gschmidt@nmdp.org**

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1. **Introduction**
   a. Minutes from February 2024 (Attachment 1)

2. **Accrual summary** (Attachment 2)

3. **Presentations, Publications or Submitted papers**
   a. **HS16-01a** Khera N, Ailawadhi S, Brazauskas R, Patel J, Jacobs B, Ustun C, Ballen K, Abid MB, Diaz Perez MA, Al-Homsi AS, Hashem H, Hong S, Munker R, Schears RM, Lazarus HM, Ciurea S, Badawy SM, Savani BN, Wirk B, LeMaistre CF, Bhatt NS, Beitinjaneh A, Aljurf M, Sharma A, Cerny J, Knight JM, Kelkar AH, Yared JA, Kindwall-Keller T, Winestone LE, Steinberg A, Arnold SD, Seo S, Preussler JM, Hossain NM, Fingrut WB, Agrawal V, Hashmi S, Lehmann LE, Wood WA, Rangarajan HG, Saber W, Hahn T. Trends in volumes and survival after hematopoietic cell transplantation in racial/ethnic minorities. ***Blood Advances. 2024 Jul 9; 8(13):3497-3506. doi:10.1182/bloodadvances.2023012469. Epub 2024 Apr 25. PMC11260842.***
   b. **HS16-01b** Hahn T, Herr MM, Brazauskas R, Patel J, Ailawadhi S, Saber W, Khera N. Use of hematopoietic cell transplant for hematologic cancers by race, ethnicity, and age. ***JAMA***

Network Open. 2024 Sep 3; doi:10.1001/jamanetworkopen.2024.33145. Epub 2024 Sep 18. PMC11411389.

c. **HS16-03** Ballen K, Wang T, He N, Knight JM, Hong S, Frangoul H, Verdonck LF, Steinberg A, Diaz MA, LeMaistre CF, Badaway SM, Pu JJ, Hashem H, Savani B, Sharma A, Lazarus HM, Abid MB, Tay J, Rangarajan HG, Kindwall-Keller T, Freytes CO, Beitinjaneh A, Winestone LE, Gergis U, Farhadfar N, Bhatt NS, Schears R, Gómez-Almaguer D, Aljurf M, Agrawal V, Kuwatsuka Y, Seo S, Marks DI, Lehmann L, Wood WA, Hashmi S, Saber W. Impact of Race/Ethnicity on Outcomes after Umbilical Cord Blood Transplantation. ***Transplantation and Cellular Therapy. 2024 Oct 30; 30(10):1027.el – 1027.e14. doi:10.1016/j.jtct.2024.07.009. Epub 2024 Jul 19.***

d. **HS18-01** Efficacy of Intensified Myeloablative Regimens for Acute Leukemia – An International Collaborative study. (Y Arai/ Y Atsuta/ S Yano). ***Submitted.***

4. Studies in progress (Attachment 3)

a. **DS20-01** Acute toxicities of bone marrow donation in donors with sickle cell trait (N Farhadfar/ J Wingard). **Manuscript Preparation.**

b. **HS18-03** Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia. (L Winestone/ R Aplenc/ K Getz). **Datafile preparation.**

c. **HS19-01** Factors Associated with Clinical Trial Participation among HCT Patients: A CIBMTR Analysis (T F. Gray/ A El-Jawahri). **Analysis.**

d. **HS19-03** Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: a multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group (N Hamerschlak/ M Kerbauy/ A Riberio). **Submitted.**

e. **HS20-01** Resource Intensity of End-of-Life Care in Children After Hematopoietic Stem Cell Transplant for Acute Leukemia: Rates and Disparities (E E Johnston/ C W. Elgarten/ L Winestone/R Aplenc/ K Getz/ V Huang/ Y Li). **Datafile preparation.**

f. **HS22-01** Health care utilization and costs of haploidentical allogeneic stem cell transplants in a contemporary cohort of pediatric patients with acute leukemia and myelodysplastic syndrome. (H Rangarajan/ P Satwani). **Protocol Received.**

g. **HS23-01** Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States. (B Hamilton/ S Hong). **Protocol Received.**

h. **DRS24-01** Outcomes for medicaid beneficiaries following allogeneic hematopoietic cell transplantation: Exploring the impact of variable medicaid eligibility criteria. (P DeMartino/ N Majail). **Protocol Received.**

5. Future/proposed studies

a. **PROP 2410-133** Impact of Race and Ethnicity on Incidence of Primary Graft Failure in Allogeneic Hematopoietic Stem Cell Transplant Recipients (H Rangarajan/ M Kharfan Dabaja) (Attachment 4)

b. **PROP 2409-02; 2410-57** Do housing distance requirements imposed by transplant centers on hematopoietic cell transplantation patients affect non-relapse mortality and other clinical outcomes (C Su/ R Salit) (Attachment 5)

c. **PROP 2410-03** Racial and Ethnic Discrepancies in Clinical Outcomes of Autologous Hematopoietic Cell Transplantation in Multiple Myeloma in Non-Hispanic Black and Hispanic Populations as Compared to Caucasian Patients (P Hagen) (Attachment 6)

d. **PROP 2410-23; 2410-179** The Effect of Social Determinants of Health on Outcomes in Pediatric and Adolescent/Young Adult (AYA) Patients Undergoing Haploidentical Stem Cell

Transplantation for Malignant and Non-Malignant Disease (Y Berry/ S Farhan/ L Davis/ P Satwani) (Attachment 7)

e. **PROP 2410-80** Defibrotide prophylaxis for hepatic sinusoidal obstructive syndrome in pediatric hematopoietic cellular therapy recipients: real-world outcomes and health care utilization implications (M Schoettler/ K Williams) (Attachment 8)

Proposed studies; not accepted for consideration at this time

f. **PROP 2409-11** The impact of race and ethnicity on outcomes of patients treated with B-cell maturation antigen chimeric antigen receptor therapy for relapsed/refractory multiple myeloma (K J Feliciano-Salva/ D K Hansen). ***Dropped due to overlap with current study/publication.***
g. **PROP 2410-09** Racial disparity in outcomes after myeloablative conditioning for hematological malignancies in patients above the age of 45 years undergoing hematopoietic stem cell transplantation (D Lad). ***Dropped due to overlap with current study/publication.***
h. **PROP 2410-41** Disparities in allograft outcomes for patients with acute myeloid leukemia in the era of post-transplant cyclophosphamide-based graft-versus-host-disease prophylaxis (W Fingrut/ K Ballen). ***Dropped due to overlap with current study/publication.***
i. **PROP 2410-107** Social and economic determinants of health in multiple myeloma patients treated with commercial CAR T-cell therapies (M Krem/ N Ahmed). ***Dropped due to small sample size.***
j. **PROP 2410-111** Outcomes of BCMA directed Chimeric Antigen Receptor (BCMA.CAR T-cells) in Multiple Myeloma Patients from Rural America: A CIBMTR analysis (I Muhsen/ S Ganguly). ***Dropped due to supplemental data needed.***
k. **PROP 2410-138** Long term complications and outcomes among the patients treated with CAR-T cell therapy based on racial and demographic differences (E Umyarova/ N Epperla). ***Dropped due to low scientific impact.***
l. **PROP 2410-147** Impact of Race and Ethnicity on Outcomes for AYA patients with B-ALL Receiving CD19 CAR T-cell Therapy (H Lust/ E Burns). ***Dropped due to low scientific impact.***
m. **PROP 2410-157** Racial, ethnicity and socioeconomic disparity in outcomes of adolescent and young adults (15-39yo) undergoing allogeneic hematopoietic cell transplantation (M Daunov/ L Metheny). ***Dropped due to overlap with current study/publication.***
n. **PROP 2410-174** The Impact of Social Determinant of Health and Area of Residence on Outcomes of CD19 directed Chimeric Antigen Receptor (CD19.CAR T-cells) in Indolent Non-Hodgkin Lymphoma Patients: A CIBMTR analysis (I Muhsen/ E Burns). ***Dropped due small sample size.***
o. **PROP 2410-201** Impact of Pre-existing Mental Health Disorders on CAR T-cell Therapy Outcomes in Patients with Large B-cell Lymphoma (S Gouni/ S Ahmed). ***Dropped due to supplemental data needed.***
p. **PROP 2410-224** Social and economic determinants of health and long-term outcomes in acute lymphoblastic leukemia patients treated with commercial CAR T-cell therapies (M Krem/ N Ahmed). ***Dropped due to small sample size.***

6. Other business

MINUTES AND OVERVIEW PLAN
CIBMTR WORKING COMMITTEE FOR DONOR AND RECIPIENT HEALTH SERVICES WORKING COMMITTEE
San Antonio, TX
Wednesday, February 21, 2024, 1:00 – 3:00 PM CT

Co-Chair:	**Jack Hsu, MD; Shands HealthCare and University of Florida, Gainesville, FL; Telephone: 352-273-7539; E-mail: jack.hsu@medicine.ufl.edu**
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Co-Chair:	**Hemalatha Rangarajan, MD; Nationwide Children's Hospital, Columbus, OH; Telephone: 740-953-0602; E-mail: hemalatha.rangarajan@nationwidechildrens.org**
Co-Chair:	**Fotios Michelis, MD, PhD; Princess Margaret Cancer Center, Toronto, ON, Canada; E-mail: fotios.michelis@uhn.ca**
Co-Chair:	**Minocher (Minoo) Battiwalla, MD, MS; Sarah Cannon BMT Program, Nashville, TN; Telephone: 615-342-7644; E-mail: minoo.battiwalla@hcahealthcare.com**
WCTL Participant:	**Megan Herr, PhD; Roswell Park Comprehensive Cancer Center, Buffalo, NY; Telephone: 716-845-3557; Email: megan.herr@roswellpark.org**
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Statistical Director:	**Brent Logan, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Medical College of Wisconsin, Milwaukee, WI; Telephone: 414-955-8849; E-mail: blogan@mcw.edu**
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Statistician:	**Gabrielle Schmidt, MPH; CIBMTR® (Center for International Blood and Marrow Transplant Research), NMDP, Minneapolis, MN; Telephone: 763-406-3841; E-mail: gschmidtnmdp.org**

1. Introduction

Members present: Heather Stefanski, Rafeek Yusuf, Brent Logan, Minoo Battiwalla, Leslie Lehmann, Hemalatha Rangarajan, Fotios Michelis, Megan Herr, Ruta Brazauskas, Gabrielle Schmidt, Jinalben Patel

a. Minutes from February 2023 DSWC and HSWC Tandem meeting sessions

Meeting was commenced by Heather at 1:00 P.M. CST. All present members introduced themselves.

Leslie presented the welcome slides. Previously, we were two committees—donor health/safety and health services—but this year we merged into Donor and Recipient Health Services Working Committee (DRSWC). The merger was completed to ensure the continuation of new collaborations and novel research ideas. The CIBMTR industry funding disclosure was presented. All members of the committee were listed, including those that were unable to attend this year—Jack Hsu and Sandhya Panch. Jack's departure from the committee was announced, and no additional chairs would be added to the committee this year. Conflict of interest (COI) policy was stated, and each chair's COI disclosure was announced. Only Sandhya, Hemalatha, and Minoo had disclosures this year. Leslie revealed the 120 CIBMTR publicly available research datasets, and emphasized the great opportunity was geared towards researchers at various levels—entry through senior. CIBMTR's vast data in the research datasets would propel junior researchers in their career. The utilization of these datasets only required a specific citation to be listed. DRSWC's consistent search for new members was announced. Leslie illuminated the importance of having junior researchers signing up for the opportunity. The goals and expectations of the committee was announced—have high impact studies and provide the status of studies/timelines. A current limitation for the committee was the lack of data for all participating international countries. CIBMTR previously wanted to merge data from other countries, but the difference in data collection processes proved to be operationally and analytically difficult. Since this was also the first year as a merged committee, it was noted there was potential for increased limitations in comparison to prior years.

Leslie presented the scoring process and scoring guide prioritization for the members voting on the proposals. She also noted the components the committee looked for in proposals did reflect the new changes occurring in the field. When members decide to be an author, they were agreeing to engaging and committing substantial contributions, drafting, final approval of the manuscript, and accepting to be accountable for all aspects of the study. Additional information was presented: ongoing studies, working committee information and materials, working committee continuing education credits, and the CIBMTR website.

CIBMTR's source of HCT data was presented. In total, there are two sources of data that come in—pre- and post-transplant. Of those two sources, the data could be on either the TED or CRF track. TED level data is mandatory to report. While the CRF is not mandatory, it does have in-depth questions that are established by CIBMTR leadership. The data collection process only allowed a 3% error rate, and if a center goes above that threshold during an audit, then corrective measures were put in place. Leslie proceeded to describe the flowchart illuminating how cellular therapy data was captured on the forms and put into the database. If members design a project and want to become familiar with that emerging area of research, then they were welcome to look at the information available on the CIBMTR website. There has been a new focus on patient reported outcomes (PRO) data, and the organization has been trying to gather that data in an equally robust way. Social health, mental health, financial toxicity, and other social equity measures are captured at various time points throughout the transplant process. Currently, there are over 1,000 patients enrolled in the PRO protocol that are followed longitudinally with over 2,000 surveys administered. CIBMTR dedicates many resources to engaging early career investigators in the research process. The Working Committee Training and Leadership (WCTL) program was created for individuals three to seven years from fellowship. A two-year commitment is required, and applications are accepted every other year with the next one being in 2025. Participants in this program are introduced to the CIBMTR proposal process through participation in working committee activities.

2. Accrual summary

Hemalatha introduced the accrual summary at 1:13 P.M. CST. The first table on the accrual summary contained data between 1988 and December of 2022 for domestic unrelated NMDP donors. The total number was 69,933. Reported bone marrow transplants were 26,498, while PBSC reported 43,435—almost double the amount of bone marrow transplants. Across all graft types, majority of donors were aged 18-29. Males comprised 60% of all donors. Hispanics comprised about 10% of the donors and 70% of donors were Caucasian. The form completion of the baseline data has been collected for 63.2% of the cohort, with a higher percentage of data collected for PBSC donors. Hemalatha then encouraged the audience to utilize the provided information to inform their decisions while voting.

3. Presentations, Published or Submitted papers

Introduced by Fotios at 1:14 P.M. CST. "The association of ABO mismatch with the outcomes of allogeneic hematopoietic cell transplantation for acute leukemia"—DS13-02—was introduced first. It was published in the American Journal of Hematology. The cohort had almost 5,000 patients diagnosed with AML and ALL for their allogeneic HCT. In the multivariate analysis, major ABO mismatch was associated with worse overall survival, inferior platelet engraftment, and higher graft failure rate.

The next study, HS18-02, was published in the journal of Transplantation and Cellular Therapy the past year. This study investigated racial and socioeconomic disparities in long-term outcomes ≥ 1-year post-allogeneic hematopoietic cell transplantation. The cohort was comprised of almost 5,500 U.S. patients that had survived at least 1 year post transplant. Multivariate analysis results revealed no association between race/ethnicity or poverty level in relation to overall survival, progression free survival, relapse, and non-related mortality.

Fotios then proceeded to list the papers in the submission in-progress category. "Trends in utilization and outcomes of autologous and allogeneic hematopoietic cell transplantation in racial and ethnic minorities" (HS16-01a) was under consideration at Blood Advances. "Relationship of race/ethnicity and survival after single and double umbilical cord blood transplantation" (HS16-03) was submitted to JAMA Oncology. "International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens" (HS18-01) was under review at Transplantation and Cellular Therapy.

a. **DS13-02** Guru Murthy GS, Logan BR, Bo-Subait S, Beitinjaneh A, Devine S, Farhadfar N, Gowda L, Hashmi S, Lazarus H, Nathan S, Sharma A, Yared JA, Stefanski HE, Pulsipher MA, Hsu JW, Switzer GE, Panch SR, Shaw BE. Association of ABO mismatch with the outcomes of allogeneic hematopoietic cell transplantation for acute leukemia. **American Journal of Hematology. 2023 Apr 1; 98(4):608-619. doi:10.1002/ajh.26834. Epub 2023 Jan 6. PMC10290878.** ***Published.***
b. **DS19-02** Farhadfar N, Ahn KW, Bo-Subait S, Logan B, Stefanski H, Hsu J, Panch S, Confer D, Liu H, Badawy S, Beitinjaneh A, Diaz M, Hildebrandt G, Kelkar A, Lazarus H, Murthy H, Preussler JM, Schears R, Sharma A, Poel MV, Bruce J, Pulsipher M, Shaw B, Wingard J, Switzer G. The impact of pre-apheresis Health Related Quality of Life on peripheral blood progenitor cell yield and donor's health and outcome: Secondary analysis of Patient-Reported Outcome Data from the RDSafe and BMT CTN 0201 Clinical Trials **Transplantation and Cellular Therapy. doi:10.1016/j.jtct.2022.05.042. Epub 2022 Jun 7**. ***Published.***
c. **HS18-02** Blue BJ, Brazauskas R, Chen K, Patel J, Zeidan AM, Steinberg A, Ballen K, Kwok J, Rotz SJ, Diaz Perez MAD, Kelkar AH, Ganguly S, Wingard JR, Lad D, Sharma A, Badawy SM, Lazarus HM, Hashem H, Szwajcer D, Knight JM, Bhatt NS, Page K, Beattie S, Arai Y, Liu H, Arnold SD, Freytes CO, Abid MB, Beitinjaneh A, Farhadfar N, Wirk B, Winestone LE, Agrawal V, Preussler JM, Seo S, Hashmi

S, Lehmann L, Wood WA, Rangarajan HG, Saber W, Majhail NS. Racial and socioeconomic disparities in long-term outcomes in ≥1 year allogeneic hematopoietic cell transplantation survivors: A CIBMTR analysis. **Transplantation and Cellular Therapy. 2023 Nov 1; 29(11):709.e1-709.e11. doi:10.1016/j.jtct.2023.07.013. Epub 2023 Jul 22.** ***Published.***

d. **HS16-01a** Nandita Khera, Theresa Hahn, Sikander Ailawadhi, Wael Saber, Jinal Patel, Ruta Brazauskas. Trends in Utilization and Outcomes of Autologous and Allogeneic Hematopoietic Cell Transplantation in Racial and Ethnic Minorities. ***Submission in-progress.***

e. **HS16-03** Karen Ballen, Naya He, Tao Wang. Relationship of Race/Ethnicity and Survival After Single and Double Umbilical Cord Blood Transplantation. ***Submission in-progress.***

f. **HS18-01** Yasuyuki Arai, Yoshiko Atsuta, Shingo Yano, Naya He, Ruta Brazauskas. International Collaborative Study to Compare the Prognosis for Acute Leukemia Patients Transplanted with Intensified Myeloablative Regimens. ***Submitted to BMT.***

4. Studies in-progress

Introduced by Minoo at 1:17 P.M. CST. Proceeded to list all the studies in-progress: DS20-01 was in the data analysis stage, DS23-01 was in the protocol development stage, HS16-01b was in the manuscript preparation stage, HS19-01 was in the datafile preparation stage, HS19-03 was in the data collection stage, HS19-04 was in the data analysis stage, and HS20-01 was in the protocol development stage.

a. **DS20-01** Acute Toxicities of Bone Marrow Donation in Donors with Sickle Cell Trait (Nosha Farhadfar; John Wingard) **Analysis.**

b. **DS23-01** Unrelated Donor Collection Efficiency and Adverse Events During the COVID-19 Pandemic (Mathew Seftel) **Protocol Development.**

c. **HS16-01b** Trends in Utilization and Outcomes of Autologous and Allogeneic Hematopoietic Cell Transplantation in Racial and Ethnic Minorities (N. Khera/ T. Hahn/ S. Ailawadhi / W. Saber) **Manuscript Preparation.**

d. **HS19-01** Factors Associated with Clinical Trial Participation Among HCT Patients: A CIBMTR Analysis (T. F. Gray/ A. El-Jawahri) **Datafile Preparation.**

e. **HS19-03** Haploidentical Stem Cell Transplantation for Malignant and Non-malignant Hematological Diseases in Patients Without Sibling Donor: A Multicenter Prospective and Longitudinal Study of the Brazilian Bone Marrow Transplantation Study Group (SBTMO) (N. Hamerschlak/ M. N. Kerbauy/ A. A. F. Ribeiro) **Data Collection.**

f. **HS19-04** Outcomes After Allogeneic Stem Cell Transplants Performed in Brazil from HLA-matched Siblings, Unrelated and Mismatched Related Donors. Retrospective Study on Behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle Transplant-related complications Consortium), Hospital Israelita Albert Einstein (AmigoH), Associação da Medula Óssea do Estado de São Paulo (Ameo), Programa Nacional de Apoio à Atenção Oncológica (Pronon), and CIBMTR (A. Seber/ N. Hamerschlak/ M. E. Flowers/ M. Pasquini) **Analysis.**

g. **HS20-01** Resource Intensity of End-of-Life Care in Children After Hematopoietic Stem Cell Transplant for Acute Leukemia: Rates and Disparities (E. E. Johnston/ C. W. Elgarten/ L. Winestone/ R. Aplenc/ K. Getz/ V. Huang/ Y. Li) **Protocol Development.**

5. Future/proposed studies

Introduced by Minoo at 1:19 P.M. CST and the future studies below were announced. "Health care utilization and costs of haploidentical allogeneic stem cell transplants in a contemporary cohort of pediatric patients with acute leukemia and myelodysplastic syndrome" (HS22-01) was accepted at Tandem in 2022. "Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States" (HS23-01) was accepted at Tandem in 2023. The proposed studies were then announced: "Determining the barriers leading to inferior survival for Black and Hispanic patients with Hodgkin lymphoma" (2302-01), "Racial and ethnic disparities in safety and efficacy of chimeric antigen receptor T-cell therapies in B-cell acute lymphoblastic leukemia, multiple myeloma or non-Hodgkin's lymphoma" (2310-13; 2310-140; 2310-215; 2310-222; 2310-225; 2310-263), "Impact of social determinants of health on outcomes in pediatric patients undergoing haploidentical stem cell transplantation for acute leukemia" (2310-44), "Outcomes for Medicaid beneficiaries following allogeneic hematopoietic cell transplantation: exploring the impact of variable Medicaid eligibility criteria" (2310-47), and "The effect of social determinants of health on allogeneic transplant outcomes: a study of the impact of social vulnerability index on outcomes for allogeneic transplant for acute myeloid leukemia" (2310-64).

a. **HS22-01** Health Care Utilization and Costs of haploidentical Allogeneic Stem Cell Transplants in a Contemporary Cohort Of Pediatric Patients With Acute Leukemia and Myelodysplastic Syndrome. (H. Rangarajan / P. Satwani)

This is a previously presented study waiting for statistical hours to be assigned; therefore, the PI did not present again.

b. **HS23-01** Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States. (B. Hamilton; S. Hong)

This is a previously presented study waiting for statistical hours to be assigned; therefore, the PI did not present again.

c. **PROP 2302-01** Determining the Barriers Leading to Inferior Survival for Black and Hispanic Patients with Hodgkin lymphoma (E. Mobley; R. Mailhot-Vega)

The study was introduced by Dr. Raymond Mailhot Vega at 1:21 P.M. CST. No COIs were disclosed. Raymond proceeded to discuss how Hodgkin lymphoma (HL) was unique through its impact on individuals of all ages—children, young adults, and older adults. Black and Hispanic patients had worse survival outcomes than White patients amongst all impacted age groups, despite each group receiving distinct therapy. To date, there was no biological data indicating genetic ancestry influenced risk of relapse. Optimum care for these patients was through participation in a trial to receive combined modality therapy and undergoing HSCT at relapse. This plan of care was occurring less frequently for Black and Hispanic patients, which raised concerns about outcomes being associated with race. The proposal proposed a merger between PCORnet and CIBMTR data. PCORnet is a data network of patient level electronic health records, administrative claims, and tumor registry data. The network collected longitudinal encounter data from more than 30 million people across the U.S. from 2010 -2020. The PCORnet CRN site partners for this protocol range in geographical representation. The proposal sought for an overrepresentation of patients and survivors who identify as Black and Hispanic.

Based on the recent census of 2022 in a Brookings Institute analysis, generally Hispanic patients are overrepresented in Florida, Texas, and the Southwest. It was traditionally thought that Black populations are more overrepresented in the southeast like Alabama, Georgia, and North Florida.

With the identified data, researchers would determine how social determinants of health (SDOH) factors relate with poor HL survival outcomes in Black and Hispanic patients. Raymond claimed identifying SDOH factors that were predictive of worse HL survival among Black and Hispanic patients would provide conceptual framework for the design of targeted interventions. Aim one of the study examined differences in the receipt and quality of HL treatment for initial diagnosis and relapse by race, ethnicity, and social factors (e.g., insurance). Aim two examined the receipt and quality of survivorship care by race, ethnicity, and social factors (e.g., insurance). Aim three evaluated patient beliefs, concerns, and attitudes regarding their upfront, relapse, and survivorship care using qualitative interviews with survivors. Aim one focused on the receding quality of HL treatment, specifically with the other databases previously mentioned. There was a real gap of knowledge in understanding the access patients had to stem cell transplant. Aim two focused on survivorship care and the patients receiving the appropriately recommended survivorship care steps presented in an electronic medical record. Aim three was qualitative, which established the proposal as mixed methods, and focused on how the patients felt about their role in medical decision making.

In the overall design portrayed on the slides, aim one focused on treatment and assessed factors under initial treatment and relapse. The main interest was a collaboration to better understand the aspects of stem cell transplant and how it was captured in the CIBMTR database. Aim two focused on survivorship. Both aims were a mediation analyses; it was thought that race or ethnicity lead towards survival, but it was being mediated by other social factors. Aim three was qualitative. Many partnerships were established to strengthen both the representation of Black and Hispanic patient survivors, as well as adolescents and young adults (AYA): Leukemia and Lymphoma Society, Stupid Cancer, Live like Bella, Elephants and Tea, and Cactus Cancer Society. The proposal was adapted by the identification of key social factors through Anderson's behavioral model—the progress model—and the NIH research framework, which considered predisposing, enabling, and need based factors. The estimated sample size from PCORnet was estimated to be 26,233 across the entire U.S. geography; samples from CIBMTR should be around 9,290. PCORnet would address the gap in knowledge and enhance the understanding around causes that lead to worse outcomes for Black and Hispanic patients. Presenter was notified of reaching time limit at 1:28 P.M. Opened to audience for questions.

First question: How would the proposal account for migration of patients in and out of PCORnet? Patients don't always get their care in a certain setting, and if PCORnet impacts 10% of the population of the U.S. then there were concerns about missing some data. Raymond responded stating the proposed data was prey to that issue. In some health care systems, like Florida, there are not many sites where someone could leave one center and not be captured in the main network. For pediatric cancer survivors, PEDSnet has committed to collecting data from all sites which involved Seattle children, Stanford, and other large organizations. While patients can equally leave, it was less common in the pediatric situation. The proposal was limited to at least two encounters with a certain diagnosis, but they would be susceptible to patients moving away.

Second question: Please elaborate on CIBMTR's role in this study and the data being put together. Raymond then acknowledged the lapse of information being presented on the data linkage was due to time constraints. He elaborated that PCORnet has different participating center sites, and the collected information from centers would get sent out to PCORnet. The centers were interested in

participating because the gap in knowledge has been understated for someone diagnosed with HL; someone may be dying from the disease compared to someone who is getting access to stem cell transplant. The main cohort that met the proposal's specific criteria would be provided from CIBMTR. A de-identified data linkage through PCORnet would occur to enhance the understanding of which patients followed through with stem cell transplant. That data could be available at CIBMTR, but PCORnet contained a stronger depth of data regarding those factors.

Third question: The question holder stated Raymond might miss some critical questions that determine survival. CS data for myeloma makes it look like Blacks and Hispanics reported worse survival outcomes; however, if one was to compare this data to the VA, where insurance and delivery of care is the same, Blacks outpace other races for survival in some instance. Those findings indicate survival was highly dependent on socioeconomic status and the kind of coverage a patient has. The granularity needed to identify what are the true causes for outcomes may not be feasible given the dataset limitations. The results may be an impression of the true outcome but would not be generalizable. Drilling down on the proposal to capture some of these nuances of the U.S. health care system would be best. For example, health care in California was very different than Tennessee where Medicaid was not provided. Raymond responded and agreed with the individual's stated hypothesis being that at no point was race or ethnicity a direct cause of survival. The study was supposed to demonstrate this. Raymond wanted to express the proposed hypothesis is not if Black or Hispanic patients are dying more for any genetic ancestry reason; instead, those disparities are due to social determinants of health with the benefit of this study being that the prior samples are all cross sectional. With PCORnet, a person would gain an understanding of how these factors change across time. They would have access to the datapoints they want to specifically evaluate, like insurance type. By focusing on those key factors via meditation analysis, it is believed that race and ethnicity would be associated with the inferior outcomes as a function of insurance. The data and the findings from the mediation analysis would then inform policy.

Fourth question: What was the ask of CIBMTR here? Was this proposal looking for just the data, or did it also require the resources for statistical support? Raymond responded saying they need CIBMTR to prepare the data. The data would then be merged through Datavant to prevent HIPAA violations from occurring. The actual analysis would be conducted by Dr. Amy Crisp.

Fifth question: How would they identify who didn't get a transplant? Raymond stated the goal would be to have 26,000 patients who had HL, and then identify relapse occurrence from that group. They would also need to determine this from EHR steps, perhaps looking at chemotherapy. If someone was starting on ABVD and they are suddenly receiving ICE, they would determine if a stem cell transplant was ever evident. He acknowledged there was no easy way to determine who didn't get a transplant, but they could also extrapolate data. For example, if those are the patients who had stem cell transplants, and those patients when compared to PCORnet are more likely to have specific factors, then those findings would also be meaningful. The question holder also stated patients get a transplant if they have good disease control. They agree it may not be genetic, but a patient could also not get to transplant because they didn't get the disease control, lacked access to care, or didn't have insurance. Even though the proposal has a lot of patients, probably only 10% of the cohort would relapse—dropping the population rapidly. Raymond did not provide a comment, and the presentation concluded.

d. **PROP 2310-13; 2310-140; 2310-215; 2310-222; 2310-225; 2310-263** Racial and Ethnic Disparities in Safety and Efficacy of Chimeric Antigen Receptor T-cell Therapies in B-cell Acute Lymphoblastic Leukemia, Multiple Myeloma or Non-Hodgkin's Lymphoma (H. Hashmi; S. Usmani; J. Ligon; N. Shah; D. Modi; E. Biltibo; A. Kassim; L. Gowda; A. Mirza)

Dr. Eden Biltibo presented at 1:37 P.M. CST and commenced the presentation by listing the proposal title. Most minority or underrepresented populations in medicine have not participated in clinical trials, which made it difficult for researchers to glean information on those subgroups. The most common solution was merging results from multiple clinical trials to conjure an assessment on those populations. Two different trials were then quoted. The first trial represented on the slide was from NIH and it was five different phase one studies compiled together. Most of the patients in the trial are B-ALL patients, and the survival data in the multivariate analysis (MVA) was completed for the B-ALL patients. There were 139 patients who participated in the trial across nine years. In the five phase one studies, 55.4% of those were White, 28.8% were Hispanic, and 3.6% were Black. The results of this study indicated there were higher CRS amongst Hispanics in the U.S. with an odds ratio of 4.5. The efficacy results had comparable CR rates across all ethnicities. The second and third population groups were relapsed refractory multiple myeloma (RRMM) (n=24) and non-Hodgkin's lymphoma (NHL) (n=23). An MVA could not be completed due to the low sample size. The researchers did observe two cases of severe CRS for Hispanic patients—one NHL and one RRMM. The second study was a retrospective study assessing the U.S. Multiple Myeloma CAR-T Consortium. It was a consortium of 11 higher institutions that brought their patients together. All 207 patients were diagnosed with RRMM, and of that cohort 72% were White, 11% were Hispanic, and 17% were Black. Higher CRS rates and longer median hospital stays were observed in the Black patients of the population. Lower overall response rate among Hispanic patients was also observed—58% vs 86% in the rest of the population—but there was no overall survival difference.

The hypothesis for the proposal was race and ethnicity influenced safety and efficacy outcomes among recipients of CAR-T therapies for B-cell Acute Lymphoblastic Leukemia (B-ALL), NHL, and multiple myeloma (MM). The specific aim was to evaluate racial and ethnic disparities in progression-free survival of B-ALL, MM, and NHL patients treated with CAR-T. The second aim was to explore the racial and ethnic disparities in the CAR T-cell therapy associated outcomes overall response rates (ORR, CR, MRD negativity rate and OS), and side effect profiles (CRS, neurotoxicity, cytopenia's, infection, second primary malignancy, and non-relapse mortality) among B-ALL, MM, and NHL patients. The inclusion criteria consisted of patients of all ages that received CAR-T for B-ALL, MM, and NHL. Patients with missing racial and ethnic information were excluded. The primary and secondary endpoints were a close repetition of the specific aims.

The preliminary data from CIBMTR consisted of 8,413 CAR-T infusions given at 210 centers to 1,143 B-ALL, 6,971 NHL, and 299 MM patients. Kymriah, Yescarta, Tescartus, Breyanzi, Abecma, and other not-specified CAR-T products were used. A large portion of the population (96.6%) had single cell infusions, and 233 patients had two infusions. Some patients had three infusions, and one case reported five infusions. A healthy representation of the elderly population was captured with 21.9% reported being 70+ years old, and 64% were male. There were 1,100 Hispanic or Latino patients across all disease groups, and close to 7,000 reported as not Hispanic or Latino. There were 6,927 White, 520 Black, 384 Asian patients along with a few remaining race groups.

The impact of the study was then presented. To understand the safety and efficacy profiles of CAR-T infusions in racial and ethnic minorities with B-ALL, NHL, and MM patients—a historically underrepresented population in clinical trials. Motivate racial and ethnic minority patients to utilize

CAR T-cell therapies within or outside of clinical trials. Finally, to identify patterns of CAR-T associated adverse effects in the patient population allowing for anticipation, early detection, and treatment of potential side effects in the clinical setting. The presenter then opened the floor for questions.

First question: What was the proposed hypothesis and what was driving the differences? Does the presenter think it was early outcomes, late outcomes, or disparities in care? If that is the case, then the proposal should look at socioeconomic status, distance to center, etc. If they think there are some differences that might lead to different rates of CRS, ICANS, and outcomes then they may have to include some other parameters as well. Eden responded with a reference to the second study she quoted earlier. The identified study looked at baseline Ferritin and CRP levels, and those factors were associated with increased rate of CRS in that patient population. She indicated there were some baseline differences in the patient population attributing to the difference in the toxicity. Patients without initial access to CAR-T would not be included. The researchers were aware they would be excluding a major patient population with the focus on only patients that received CAR-T infusion.

Second question: Are we surprised to see Hispanics did not have any worse outcomes in the referenced studies; an outcome that has been reported with traditional therapy, including allogeneic stem cell transplant and B-ALL? If the patients can access CAR-T it is possible the outcomes are comparable. The question holder proceeded to say the outcome may just be a function of biology—especially with B-ALL—and it is known for Hispanics to have a different molecular characteristics and cytogenetic risk group. The second comment was how the proposal would adjust for attribution of allogeneic transplants, especially in the B-ALL cohort. The limitation of the donor availability or not having a fully matched donor was also raised, and they noted it might be a good idea to censor. Eden responded with CIBMTR data should be able to account for this.

Third question: Look within CIBMTR because Fred Lock already presented a very similar proposal at ASCO with only diffuse large B-cell patients. A look for overlap would be necessary, but MM is the only disease that has not been researched within this question. No comments were made by the presenter.

Fourth question: There were several variables that should be considered in the MVA. The numbers are very small at the different racial groups, and they questioned if there was enough power to look at the outcomes on all these diseases. Presenter stated there are statistical methods that can be used when the sample size is low.

Fifth question: There should be a lot of heterogeneity in outcomes based on the product that was used. For example, the protocol cannot compare Abecma to other products. Stratifying by product would be necessary since it was known to be such a powerful driver for both toxicity and survival. Was there a way to address the primary hypothesis without focusing on something common across all products? The presenter responded stating they will have to divide it among the disease groups since that was a major factor affecting survival and the side effect profile. They noted it was important to take the product into consideration.

e. **PROP 2310-44** Impact of Social Determinants of Health on Outcomes in Pediatric Patients Undergoing Haploidentical Stem Cell Transplantation for Acute Leukemia (L. Davis; P. Satwani)

It was presented by Dr. Davis on behalf of the group with Dr. Satwani attending in-person. This study hypothesizes that pediatric patients with leukemia of non-white ethnicity will have decreased overall survival (OS) and increased transplant related mortality (TRM) compared with patients of white ethnicity undergoing Haploidentical hematopoietic stem cell transplant (HCT). The primary exposure of interest will be to determine the impact of social determinants of health (SDOH) on outcomes in pediatric alloHCT patients for leukemia and the primary outcomes of interest will be overall survival. Covariates of interest will include disease status, disease type, comorbidities, pre-transplant infection, conditioning, year. Treatment related mortality and leukemia free survival stratified by ethnicity will also be investigated as secondary outcomes. Subgroup analysis of patient and transplant related factors will be performed. Study will include survival probability at Day 100 and one-year post transplant using Kaplan Meier analysis which will be stratified by ethnicity and risk factors.

Comments from the audience: Dr. Khera raised her concerns for limiting population to peds only and suggested if study can include both peds and adults or AYA up to 40 years. She asked if SDOH variables would be looked at individually or as a composite variable for multivariate analysis. Dr. Davis confirmed that for the mentioned variables, the study will look individually and mentioned about neighborhood poverty, area deprivation index. Dr. Lehman asked if T cell depletion meaning Alpha cells as PtCY would have center effect with alpha and beta. Dr. Majhail asked if there would be difference in outcome for other donors other than haplo and mentioned similar publication. Presenter answered the question referring to the hypothesis and added that similar publication looked for non-halpo donors so this study will add knowledge. One of the attendees asked how social determinants analytically fit into this study.

f. **PROP 2310-47** Outcomes for Medicaid Beneficiaries Following Allogeneic Hematopoietic Cell Transplantation: Exploring the Impact of Variable Medicaid Eligibility Criteria (P. DeMartino; N. Majhail)

It was presented by Dr. DeMartino on behalf of the group with Dr. Majhail attending in-person. This study hypothesizes that heterogeneity in state Medicaid eligibility criteria influence the association between HCT outcomes and insurance status and that analyses describing inferior outcomes for Medicaid enrollees in aggregate (nationally) are of limited utility. The primary outcomes of interest will be overall survival, treatment related mortality, and relapse. Covariates of interest will be race/ethnicity, neighborhood-level poverty, performance score, disease type, donor age, conditioning intensity, HLA matching, GVHD prophylaxis. Acute-GVHD (2-4) and Chronic-GVHD will also be key secondary outcomes.

Comments from the audience: Dr. Hann asked if study is going to incorporate a cost of living index or adjustment for the heterogeneity and eligibility across states like North Dakota and New York. Dr. DeMartino agreed to the point and confirmed adding that to the study. One of the attendees suggested considering Medicaid as whole instead of expansion or not and look at their survival using an existing sickle cell disease study as an example. Another attendee asked if study is considering duration of Medicaid enrollment. Dr. DeMartino responded by stating that CIBMTR- CRF forms would provide the insurance status by time and added the study would be able to look for dual enrollments.

g. **PROP 2310-64** The Effect of Social Determinants of Health on Allogeneic Transplant Outcomes: A Study of the Impact of Social Vulnerability Index on Outcomes for Allogeneic Transplant for Acute Myeloid Leukemia (K. Ballen; I. Varadarajan)

It was presented by Dr. Ballen on behalf of the group. This study hypothesizes patients who live in counties with high social vulnerability index (above the median) will have lower two-year overall survival compared to patients who live in counties with lower social vulnerability index; and within the Social Vulnerability Index, the household composition and racial/ethnic subgroups will have the most impact on overall survival after HCT. The primary outcome of interest will be two-year overall survival after HCT for AML. 100-day transplant related mortality, Acute GVHD Grades II-IV, Chronic GVHD at one year, GVHD free, and relapse free survival at one and two years after transplant will be key secondary outcomes.

Comments from the audience: Dr. Battiwalla raised a concern for similar publication and asked if proposed study would be different than previous publication. Dr. Ballen mentioned about the small dataset used in prior study and this study will expand to national dataset. Dr. Majhail mentioned similar publication with Community Health Status and outcomes for Leukemia survival completed by CIBMTR. Presenter answered the concern by stating SVI looks at other areas like housing and transportation not only race and ethnicity. One of the attendees asked how study will consider for donor status and caregiver and their financial toxicity. Dr. Ballen agreed with the concern but shared that it's not possible to look at some factors as transplant is already done and added that it is not a part of the index primarily. Chairs raised concern for only pediatric population and one of the scientific directors, Dr. Yusuf, raised concern for interactions between 16 sub variables of SVI.

6. Proposed studies; not accepted for consideration at this time

a. **PROP 2310-74** Disparities in Multiple Myeloma Between Hispanics and non-Hispanics—Real World Outcomes
b. **PROP 2310-133** Impact of Socioeconomic Factors on Allogeneic Stem Cell Transplant Outcomes
c. **PROP 2310-149** Donor Race as a Determinant of Outcomes in Allogeneic Hematopoietic Stem Cell Transplantation for Myeloid Malignancies
d. **PROP 2310-161** Longitudinal Investigation of Financial Toxicity and Association with Health-Related Quality of Life Indicators in Hematopoietic Stem Cell Transplant Recipients
e. **PROP 2310-79** Racial and Ethnic Discrepancies in Clinical Outcomes of Autologous Hematopoietic Cell Transplantation in Multiple Myeloma in non-Hispanic Black and Hispanic Populations as Compared to Caucasian Patients

7. Other business

a. HaploQol donor study

Heather introduced Dr. Galen Switzer's topic at 2:23 P.M. CST. Galen introduced the Donor HRQol and physician decision-making in the context of haplo stem cell transplantation (HaploQol). He noted it was a newly funded study from his team and group of co-investigators. It focused on donor health related quality of life and decision making in the context of haplo transplantation. The study was funded by NHLBI in January with project coordination coming from University of Pittsburgh.

The basic rationale for focusing on haplo donors was because recent medical advances have allowed parent-to-child and child-to-parent donations. It was also known that the number and percentage of

those types of donations have increased dramatically across time. However, health related quality of life among donor populations have not been described or compared to that of other donor groups. It was also unclear how physicians make transplant-related decisions in the context of haploid donation. Health related quality of life among haplo donor population in both parents and kids, and how physicians make decisions about donor choice in the context of haploid donation was the information they sought after.

The goals of the study were to conduct the first systematic, nationwide effort to describe health related quality of life and factors associated with health-related quality of life among haploid donors. Then to compare the haplo donor quality of life to other related and unrelated donors. Quality of life scores on related and unrelated donor cohorts were collected from a prior study Galen conducted a few years ago. With that data they can look at haplo donors in comparison to those two kinds of existing cohorts. The final goal was to describe transplant physician decision making. The first study aim would be qualitative, and it would be conducted with adult and adolescent children or parents-to-child donors. At one year post donation they would conduct six focus groups. The basic goal of that step is to optimize the interview stage—the more quantitative data collection phase. In the second aim, they will finalize the interview script. Phone interviews would then be conducted with a cohort of adult and adolescent parent-to-child haplo donors. At one year post donation there would be 300 adult participants and 50 adolescents from about 30 geographically diverse transplant centers. Part of the goal was to get interest from people going through haplo transplants because they would be reaching out and trying to recruit centers as the next phase in the research study. The third aim is looking at physician decision-making regarding the use of haplo donors. There would be three focus groups with physicians. They would probably try to convene some focus groups at Tandem next year. Otherwise, they may conduct them via video conference. The three focus groups would get a better idea about how the decision making takes place. After the focus groups adjourn, a web-based survey would be sent to all 170 U.S. based centers that perform haplo transplants to gain a more systematic idea from as many centers as possible.

There would be startup and onboarding activities like IRB activation, service agreements, site documentation, and training. Then they would ask centers to identify eligible participants and contact them with a brief study description that can be done by phone, text, or mail. Contact information would then be shared with University of Pittsburgh for donors that have agreed to a focus interview or the interviews themselves. Each site would be paid $2,000 for startup/administrative costs and $150 per haplo donor enrolled in the study. It would be a four-year study, but data collection is expected to take a much shorter time, with the analytic phase at the end.

First question: They would like to get a clear idea of what exactly Galen wanted from the centers. The center gets the study to the IRB, they identify the donor, then does Galen's team complete the rest of the work? Galen responds that these prior assumptions were correct. The question holder proceeds to ask if the study requests only English-speaking donors? Galen responds that they would take English and Spanish-speaking donors. He also asserts that centers can decide internally the best way to contact the haplo donors. The research committee had decided to allow centers to do an opt-out procedure as well.

Second question: If one was to look at haplo transplants demographically, the donors are probably much older than the donors from NMDP. It is already skewed because the comorbidities those patients bring on board is a slightly elevated. Would the study control for that or account for it in the data collection? The second point made was the inability to avoid putting race into the study. Galen stated that it is expected that we know there are differences between related and unrelated donor

populations from the RD Safe study that was published. They have both related and unrelated donor populations, so the related donor population would expect to be like what we would get from the haplo donor population. Galen then addressed the second question that was posed. In all the recent studies, they ensured to have a representation of at least 15% of each ethnic minority groups in the U.S.—five groups in total. That representation provides the ability to look at differences among those categories and the ability then to statistically match it to either related donor samples or the unrelated donors—if desired. The question was then expanded to ask one of the reasons some centers give to doing haplo was due to the easy accessibility to family members. Overall, NMDP's trigger and collection time has shortened, so one can get donors even quicker. The aim three has nothing to do with availability of donors. Galen then states those are the aspects they would certainly explore and how perceptions about time to donor selection would be a factor that is asked about. End of presentation.

Minoo then instructed the audience on voting procedures for Tandem 2024 and thanked everyone for their attendance.

Meeting concluded at 2:34 P.M. CST.

Working Committee Overview Plan 2024-2025		
Study Number and Title	**Current Status**	**Chairs Priority**
HS18-03 Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia (*PI: Lena Winestone/ Richard Aplenc/ Kelly Getz; MS: TBD; PhD: TBD*)	Protocol Received	1
HS19-01 Factors associated with clinical trial participation among HSCT patients: a CIBMTR Analysis (*PI: Tamryn Gray/ Areej El-Jawahri; MS: Jinal Patel; PhD: Ruta Brazauskas*)	Protocol Development	2
HS16-01A Trends in volumes and survival after hematopoietic cell transplantation in racial/ethnic minorities (*PI: Nandita Khera/Theresa Hahn/Sikander Ailawadhi/ Wael Saber; MS: Jinal Patel; PhD: Ruta Brazauskas*)	In Press	1
HS16-01B Trends in utilization and outcomes of autologous and allogeneic hematopoietic cell transplantation in racial and ethnic minorities (*PI: Nandita Khera/Theresa Hahn/Sikander Ailawadhi/ Wael Saber; MS: Jinal Patel; PhD: Ruta Brazauskas*)	Manuscript Preparation	1
HS20-01 Resource Intensity of end-of-life care in children after hematopoietic stem cell transplant for acute leukemia: Rates and disparities (*PI: Emily E Johnston/ Caitlin W. Elgarten/ Lena Winestone/ Richard Aplenc; MS: TBD; PhD: TBD*)	Protocol Received	4
HS22-01 Health Care Utilization and costs of haploidentical allogeneic stem cell transplants in a	Protocol Received	4

contemporary cohort of pediatric patients with acute leukemia and myelodysplastic syndrome. *(PI: Hemalatha / Satwani; MS: TBD; PhD: TBD)*		
HS19-03 Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: a multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group (*PI: Nelson Hamerschlak/ Mariana Kerbauy/ Anrezea Riberio; MS: Jinal Patel; PhD: TBD*)	Analysis	3
HS19-04 Outcomes after allogeneic stem cell transplants performed in Brazil from HLA-matched siblings, unrelated and mismatched related donors. Retrospective study on behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle) (*PI: Adriana Seber/ Nelson Hamerschlak/ Mary Flowers/ Marcelo Pasquini; MS: Naya He; PhD: TBD*)	Manuscript preparation	3
HS23-01 Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States. *(PI*: *Betty Hamilton; Sanghee Hong; MS: TBD; PhD: TBD)*	Protocol received	3
DS20-01: Acute toxicities in Donors with Sickle Cell Trait	Analysis	1
HS16-03 Relationship of race/ethnicity and survival after single and double umbilical cord blood transplantation (*PI: Karen Ballen; MS: Naya He; PhD: Tao Wang*)	Submitted	1
HS18-01 International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens (*PI: Yasuyuki Arai/ Yoshiko Atsuta/ Shingo Yano; MS: Naya He; PhD: Ruta Brazauskas*)	Submitted	1
DRS24-01 Outcomes for Medicaid beneficiaries following allogeneic hematopoietic cell transplantation: Exploring the impact of variable Medicaid eligibility criteria *(PI*: *Patrick DeMartino; Navneet Majail; MS: TBD; PhD: TBD)*	Protocol Pending	4

Working Assignments for Working Committee Leadership (March 2024)	
Fotios Michelis	**DS20-01** Acute toxicities in Donors with Sickle Cell Trait **HS18-01** International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens (*PI: Yasuyuki Arai/ Yoshiko Atsuta/ Shingo Yano; MS: Naya He; PhD: Ruta Brazauskas*)
Sandhya Panch	**HS16-03** Relationship of race/ethnicity and survival after single and double umbilical cord blood transplantation (*PI: Karen Ballen; MS: Naya He; PhD: Tao Wang*)
Hemalatha Rangarajan	**HS18-03** Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia (*PI: Lena Winestone/ Richard Aplenc/ Kelly Getz; MS: Jinalben Patel; PhD: TBD*) **HS19-01** Factors associated with clinical trial participation among HSCT patients: a CIBMTR Analysis (*PI: Tamryn Gray/ Areej El-Jawahri; MS: Jinalben Patel; PhD: Ruta Brazauskas*) **DRS24-01** Outcomes for Medicaid beneficiaries following allogeneic hematopoietic cell transplantation: Exploring the impact of variable Medicaid eligibility criteria
Leslie Lehmann	**HS16-01B** Trends in utilization and outcomes of autologous and allogeneic hematopoietic cell transplantation in racial and ethnic minorities (*PI: Nandita Khera/Theresa Hahn/Sikander Ailawadhi/ Wael Saber; MS: Jinal Patel; PhD: Ruta Brazauskas*) **HS16-01A** Trends in volumes and survival after hematopoietic cell transplantation in racial/ethnic minorities (*PI: Nandita Khera/Theresa Hahn/Sikander Ailawadhi/ Wael Saber; MS: Jinal Patel; PhD: Ruta Brazauskas*) **HS20-01** Resource Intensity of end-of-life care in children after hematopoietic stem cell transplant for acute leukemia: Rates and disparities (*PI: Emily E Johnston/ Caitlin W. Elgarten/ Lena Winestone/ Richard Aplenc; MS: Jinalben Patel; PhD: TBD*) **HS22-01** Health Care Utilization and costs of haploidentical allogeneic stem cell transplants in a contemporary cohort of pediatric patients with acute leukemia and myelodysplastic syndrome. *(PI: Hemalatha / Satwani; MS: TBD; PhD: TBD)*

Minoo Battiwalla	**HS19-03** Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: a multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group (*PI: Nelson Hamerschlak/ Mariana Kerbauy/ Anrezea Riberio; MS: Naya He, Jinalben; PhD: TBD*) **HS19-04** Outcomes after allogeneic stem cell transplants performed in Brazil from HLA-matched siblings, unrelated and mismatched related donors. Retrospective study on behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle) (*PI: Adriana Seber/ Nelson Hamerschlak/ Mary Flowers/ Marcelo Pasquini; MS: Naya He; PhD: TBD*) **HS23-01** Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States. *(PI*: *Betty Hamilton; Sanghee Hong; MS: TBD; PhD: TBD)*

Table 1. Characteristics of domestic unrelated NMDP donors donating between 1988 and December 2023[a]

Characteristic	Bone marrow	PBSC	Total
No. of patients	26807	46140	72947
Donor age at collection - no. (%)			
Median (min-max)	33.6 (18.3-61.1)	30.2 (18.3-62.3)	31.4 (18.3-62.3)
18-29	10108 (37.7)	22721 (49.2)	32829 (45.0)
30-39	8904 (33.2)	12724 (27.6)	21628 (29.6)
40-49	6091 (22.7)	7645 (16.6)	13736 (18.8)
50+	1704 (6.4)	3050 (6.6)	4754 (6.5)
Donor sex - no. (%)			
Male	16052 (59.9)	28578 (61.9)	44630 (61.2)
Female	10755 (40.1)	17562 (38.1)	28317 (38.8)
Donor race/ethnicity - no. (%)			
Caucasian	18675 (69.7)	30616 (66.4)	49291 (67.6)
African/African-American	1518 (5.7)	1786 (3.9)	3304 (4.5)
Asian/Pacific Islander	1262 (4.7)	2457 (5.3)	3719 (5.1)
Hispanic	2442 (9.1)	3955 (8.6)	6397 (8.8)
Native American	285 (1.1)	287 (0.6)	572 (0.8)
Multiple/Other	1635 (6.1)	3852 (8.3)	5487 (7.5)
Not reported	990 (3.7)	3187 (6.9)	4177 (5.7)
Donor CMV status - no. (%)			
Negative	14708 (54.9)	25758 (55.8)	40466 (55.5)
Positive	11729 (43.8)	20003 (43.4)	31732 (43.5)
Unknown/inconclusive	370 (1.4)	379 (0.8)	749 (1.0)
Year of donation - no. (%)			
1988	78 (0.3)	0 (0.0)	78 (0.1)
1989	176 (0.7)	0 (0.0)	176 (0.2)
1990	280 (1.0)	0 (0.0)	280 (0.4)
1991	433 (1.6)	0 (0.0)	433 (0.6)
1992	540 (2.0)	0 (0.0)	540 (0.7)
1993	640 (2.4)	0 (0.0)	640 (0.9)
1994	794 (3.0)	5 (0.0)	799 (1.1)
1995	867 (3.2)	21 (0.0)	888 (1.2)
1996	1039 (3.9)	14 (0.0)	1053 (1.4)
1997	1164 (4.3)	17 (0.0)	1181 (1.6)
1998	1208 (4.5)	29 (0.1)	1237 (1.7)
1999	1222 (4.6)	71 (0.2)	1293 (1.8)

Characteristic	Bone marrow	PBSC	Total
2000	1185 (4.4)	311 (0.7)	1496 (2.1)
2001	1056 (3.9)	454 (1.0)	1510 (2.1)
2002	1061 (4.0)	749 (1.6)	1810 (2.5)
2003	877 (3.3)	988 (2.1)	1865 (2.6)
2004	796 (3.0)	1085 (2.4)	1881 (2.6)
2005	646 (2.4)	1254 (2.7)	1900 (2.6)
2006	659 (2.5)	1374 (3.0)	2033 (2.8)
2007	643 (2.4)	1470 (3.2)	2113 (2.9)
2008	663 (2.5)	1707 (3.7)	2370 (3.2)
2009	663 (2.5)	1836 (4.0)	2499 (3.4)
2010	710 (2.6)	1938 (4.2)	2648 (3.6)
2011	753 (2.8)	2094 (4.5)	2847 (3.9)
2012	924 (3.4)	2484 (5.4)	3408 (4.7)
2013	907 (3.4)	2697 (5.8)	3604 (4.9)
2014	879 (3.3)	2606 (5.6)	3485 (4.8)
2015	800 (3.0)	2484 (5.4)	3284 (4.5)
2016	810 (3.0)	2268 (4.9)	3078 (4.2)
2017	810 (3.0)	2170 (4.7)	2980 (4.1)
2018	737 (2.7)	2234 (4.8)	2971 (4.1)
2019	655 (2.4)	2232 (4.8)	2887 (4.0)
2020	524 (2.0)	2436 (5.3)	2960 (4.1)
2021	544 (2.0)	2710 (5.9)	3254 (4.5)
2022	547 (2.0)	3089 (6.7)	3636 (5.0)
2023	517 (1.9)	3313 (7.2)	3830 (5.3)
Form completion			
Baseline[b,c] - no./total no. (%)	10780/26807 (40.2)	36582/46140 (79.3)	47362/72947 (64.9)
Day of collection (BM donors)[b,d] - no./total no. (%)	10348/26807 (38.6)	0/46140 (0.0)	10348/72947 (14.2)
Day 1 of collection (PBSC donors)[b,e] - no./total no. (%)	0/26807 (0.0)	45981/46140 (99.7)	45981/72947 (63.0)
Product (BM donors)[b,f] - no./total no. (%)	24670/26807 (92.0)	0/46140 (0.0)	24670/72947 (33.8)
First product (PBSC donors)[b,g] - no./total no. (%)	0/26807 (0.0)	35278/46140 (76.5)	35278/72947 (48.4)

[a] There have been 6710 bone marrow and 27034 PBSC international donors during this time frame.
[b] Completed with FormsNet1 or FormsNet2 (approximately 2004 and forward).
[c] Form 700 collects information related to vital signs, hematology, MTC, infection, pain, and venous access.
[d] Form 732 collects information related to MTC, infection, pain, vital signs, pre-collection hematology, post-collection hematology, and ABO typing.

[e] Form 730 collects information related to MTC, infection, pain, vital signs, pre-apheresis hematology, post-apheresis hematology, and ABO typing.
[f] Form 772 collects information related to marrow product analysis.
[g] Form 770 collects information related to PBSC product analysis.
Abbreviations: NMDP – National Marrow Donor Program; PBSC – Peripheral blood stem cell; CMV – Cytomegalovirus; MTC – Modified toxicity criteria.

Table 2. Characteristics of domestic related NMDP donors donating between 1988 and December 2023[a]

Characteristic	Bone marrow	PBSC	Total
No. of patients	112	1147	1259
Donor age at collection - no. (%)			
Median (min-max)	38.0 (18.0-60.5)	47.8 (18.2-63.0)	46.2 (18.0-63.0)
18-29	32 (28.6)	144 (12.6)	176 (14.0)
30-39	36 (32.1)	246 (21.4)	282 (22.4)
40-49	25 (22.3)	239 (20.8)	264 (21.0)
50+	19 (17.0)	518 (45.2)	537 (42.7)
Donor sex - no. (%)			
Male	64 (57.1)	694 (60.5)	758 (60.2)
Female	48 (42.9)	453 (39.5)	501 (39.8)
Donor race/ethnicity - no. (%)			
Caucasian	64 (57.1)	855 (74.5)	919 (73.0)
African/African-American	24 (21.4)	73 (6.4)	97 (7.7)
Asian/Pacific Islander	2 (1.8)	47 (4.1)	49 (3.9)
Hispanic	13 (11.6)	92 (8.0)	105 (8.3)
Native American	0 (0.0)	1 (0.1)	1 (0.1)
Multiple/Other	6 (5.4)	40 (3.5)	46 (3.7)
Not reported	3 (2.7)	39 (3.4)	42 (3.3)
Donor CMV status - no. (%)			
Negative	59 (52.7)	610 (53.2)	669 (53.1)
Positive	53 (47.3)	532 (46.4)	585 (46.5)
Unknown/inconclusive	0 (0.0)	5 (0.4)	5 (0.4)
Year of donation - no. (%)			
2009	0 (0.0)	1 (0.1)	1 (0.1)
2012	0 (0.0)	1 (0.1)	1 (0.1)
2013	0 (0.0)	5 (0.4)	5 (0.4)
2014	1 (0.9)	2 (0.2)	3 (0.2)
2015	1 (0.9)	6 (0.5)	7 (0.6)
2016	4 (3.6)	11 (1.0)	15 (1.2)
2017	15 (13.4)	37 (3.2)	52 (4.1)
2018	13 (11.6)	58 (5.1)	71 (5.6)
2019	9 (8.0)	72 (6.3)	81 (6.4)
2020	25 (22.3)	240 (20.9)	265 (21.0)
2021	13 (11.6)	285 (24.8)	298 (23.7)
2022	15 (13.4)	237 (20.7)	252 (20.0)

Characteristic	Bone marrow	PBSC	Total
2023	16 (14.3)	192 (16.7)	208 (16.5)
Form completion			
Baseline[b,c] - no./total no. (%)	112/112 (100)	1147/1147 (100)	1259/1259 (100)
Day of collection (BM donors) [b,d] - no./total no. (%)	111/112 (99.1)	0/1147 (0.0)	111/1259 (8.8)
Day 1 of collection (PBSC donors) [b,d] - no./total no. (%)	0/112 (0.0)	1146/1147 (99.9)	1146/1259 (91.0)
Product form (BM donors) [b,d] - no./total no. (%)	112/112 (100)	0/1147 (0.0)	112/1259 (8.9)
First product form (PBSC donors) [b,d] - no./total no. (%)	0/112 (0.0)	1144/1147 (99.7)	1144/1259 (90.9)

[a] There have been 32 bone marrow and 161 PBSC international donors during this time frame.
[b] Completed with FormsNet2 (approximately 2009 and forward). Similar data are collected prior to 2009.
[c] Form 700 collects information related to vital signs, hematology, MTC, infection, pain, and venous access.
[d] Form 732 collects information related to MTC, infection, pain, vital signs, pre-collection hematology, post-collection hematology, and ABO typing.
[e] Form 730 collects information related to MTC, infection, pain, vital signs, pre-apheresis hematology, post-apheresis hematology, and ABO typing.
[f] Form 772 collects information related to marrow product analysis.
[g] Form 770 collects information related to PBSC product analysis.
Abbreviations: NMDP – National Marrow Donor Program; PBSC – Peripheral blood stem cell; CMV – Cytomegalovirus; MTC – Modified toxicity criteria.

Table 3. Unrelated Donor HCT Research Sample Inventory - Summary for First Allogeneic Transplants in CRF and TED with biospecimens available through the CIBMTR Repository stratified by availability of paired samples, recipient only samples and donor only samples, Biospecimens include: whole blood, serum/plasma and limited quantities of viable cells and cell lines (collected prior to 2006), Specific inventory queries available upon request through the CIBMTR Immunobiology Research Program

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	50520	24709	13268
Source of data			
CRF	25730 (51)	9079 (37)	5980 (45)
TED	24790 (49)	15630 (63)	7288 (55)
Number of centers	265	245	396
Disease at transplant			
AML	17660 (35)	9401 (38)	4437 (33)
ALL	7264 (14)	3073 (12)	2113 (16)
Other leukemia	1493 (3)	488 (2)	335 (3)
CML	3610 (7)	1233 (5)	1063 (8)
MDS	7617 (15)	4675 (19)	1739 (13)
Other acute leukemia	565 (1)	301 (1)	151 (1)
NHL	4389 (9)	1657 (7)	983 (7)
Hodgkin Lymphoma	970 (2)	305 (1)	224 (2)
Plasma Cell Disorders, MM	952 (2)	300 (1)	211 (2)
Other malignancies	61 (<1)	15 (<1)	22 (<1)
Breast cancer	7 (<1)	3 (<1)	1 (<1)
SAA	1644 (3)	746 (3)	582 (4)
Inherited abnormalities erythrocyte diff fxn	733 (1)	256 (1)	226 (2)
Inherited bone marrow failure syndromes	63 (<1)	67 (<1)	39 (<1)
Hemoglobinopathies	40 (<1)	44 (<1)	25 (<1)
Paroxysmal nocturnal hemoglobinuria	6 (<1)	11 (<1)	5 (<1)
SCIDs	893 (2)	408 (2)	401 (3)
Inherited abnormalities of platelets	43 (<1)	18 (<1)	13 (<1)
Inherited disorders of metabolism	311 (1)	100 (<1)	163 (1)
Histiocytic disorders	408 (1)	148 (1)	140 (1)
Autoimmune disorders	32 (<1)	28 (<1)	15 (<1)
MPN	1705 (3)	1406 (6)	354 (3)
Others	54 (<1)	26 (<1)	26 (<1)
AML Disease status at transplant			
CR1	9875 (56)	6142 (65)	2275 (51)
CR2	3296 (19)	1521 (16)	868 (20)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
CR3+	354 (2)	128 (1)	102 (2)
Advanced or active disease	3951 (22)	1571 (17)	1045 (24)
Missing	184 (1)	39 (<1)	147 (3)
ALL Disease status at transplant			
CR1	3664 (50)	1842 (60)	906 (43)
CR2	2067 (28)	760 (25)	616 (29)
CR3+	599 (8)	202 (7)	196 (9)
Advanced or active disease	852 (12)	244 (8)	274 (13)
Missing	82 (1)	25 (1)	121 (6)
MDS Disease status at transplant			
Early	1612 (21)	864 (18)	388 (22)
Advanced	5026 (66)	3561 (76)	1002 (58)
Missing	979 (13)	250 (5)	349 (20)
NHL Disease status at transplant			
CR1	638 (15)	349 (21)	143 (15)
CR2	826 (19)	337 (20)	160 (16)
CR3+	383 (9)	149 (9)	90 (9)
PR	446 (10)	111 (7)	99 (10)
Advanced	2003 (46)	685 (42)	457 (47)
Missing	73 (2)	18 (1)	31 (3)
Recipient age at transplant			
0-9 years	4138 (8)	1425 (6)	1714 (13)
10-17 years	3290 (7)	1118 (5)	1202 (9)
18-29 years	5989 (12)	2237 (9)	1733 (13)
30-39 years	5608 (11)	2226 (9)	1529 (12)
40-49 years	7457 (15)	3016 (12)	1884 (14)
50-59 years	10282 (20)	4730 (19)	2288 (17)
60-69 years	10984 (22)	7231 (29)	2356 (18)
70+ years	2772 (5)	2726 (11)	562 (4)
Median (Range)	49 (0-84)	56 (0-83)	43 (0-84)
Recipient race			
White	44161 (91)	21667 (91)	9821 (87)
Black or African American	2423 (5)	1007 (4)	651 (6)
Asian	1334 (3)	750 (3)	604 (5)
Native Hawaiian or other Pacific Islander	75 (<1)	36 (<1)	41 (<1)
American Indian or Alaska Native	208 (<1)	112 (<1)	64 (1)
Other	49 (<1)	27 (<1)	28 (<1)
More than one race	304 (1)	146 (1)	67 (1)
Unknown	1966 (N/A)	964 (N/A)	1992 (N/A)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Recipient ethnicity			
Hispanic or Latino	4313 (10)	1880 (8)	1238 (11)
Non Hispanic or non-Latino	38348 (88)	20014 (90)	7032 (64)
Non-resident of the U.S.	889 (2)	302 (1)	2769 (25)
Unknown	6970 (N/A)	2513 (N/A)	2229 (N/A)
Recipient sex			
Male	29289 (58)	14449 (58)	7866 (59)
Female	21231 (42)	10260 (42)	5402 (41)
Karnofsky score			
10-80	17764 (35)	9943 (40)	4214 (32)
90-100	30913 (61)	14082 (57)	8398 (63)
Missing	1843 (4)	684 (3)	656 (5)
HLA-A B DRB1 groups - low resolution			
<=3/6	30 (<1)	111 (<1)	12 (<1)
4/6	298 (1)	130 (1)	70 (1)
5/6	6900 (14)	2920 (13)	1821 (15)
6/6	42187 (85)	19862 (86)	10249 (84)
Unknown	1105 (N/A)	1686 (N/A)	1116 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	908 (2)	167 (1)	86 (1)
6/8	1853 (4)	237 (1)	253 (3)
7/8	9291 (19)	3166 (16)	1966 (22)
8/8	36454 (75)	16187 (82)	6834 (75)
Unknown	2014 (N/A)	4952 (N/A)	4129 (N/A)
HLA-DPB1 Match			
Double allele mismatch	12440 (28)	3578 (23)	1263 (24)
Single allele mismatch	23579 (54)	8083 (52)	2693 (52)
Full allele matched	7901 (18)	3896 (25)	1211 (23)
Unknown	6600 (N/A)	9152 (N/A)	8101 (N/A)
High resolution release score			
No	15052 (30)	24646 (>99)	12759 (96)
Yes	35468 (70)	63 (<1)	509 (4)
KIR typing available			
No	36672 (73)	24684 (>99)	13197 (99)
Yes	13848 (27)	25 (<1)	71 (1)
Graft type			
Marrow	16860 (33)	5544 (22)	5029 (38)
PBSC	33538 (66)	18919 (77)	8167 (62)
BM+PBSC	22 (<1)	29 (<1)	6 (<1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
PBSC+UCB	40 (<1)	191 (1)	11 (<1)
Others	60 (<1)	26 (<1)	55 (<1)
Conditioning regimen			
Myeloablative	30248 (60)	12189 (49)	8090 (61)
RIC/Nonmyeloablative	20037 (40)	12441 (50)	4995 (38)
TBD	235 (<1)	79 (<1)	183 (1)
Donor age at donation			
To Be Determined/NA	778 (2)	952 (4)	373 (3)
0-9 years	4 (<1)	30 (<1)	1 (<1)
10-17 years	1 (<1)	11 (<1)	1 (<1)
18-29 years	25102 (50)	13722 (56)	5750 (43)
30-39 years	14053 (28)	6255 (25)	3900 (29)
40-49 years	8127 (16)	2853 (12)	2459 (19)
50+ years	2455 (5)	886 (4)	784 (6)
Median (Range)	30 (0-69)	28 (0-89)	32 (4-77)
Donor/Recipient CMV serostatus			
+/+	12758 (25)	6857 (28)	3524 (27)
+/-	5937 (12)	3179 (13)	1622 (12)
-/+	16579 (33)	7438 (30)	4066 (31)
-/-	14540 (29)	6518 (26)	3533 (27)
CB - recipient +	36 (<1)	151 (1)	10 (<1)
CB - recipient -	4 (<1)	47 (<1)	2 (<1)
CB - recipient CMV unknown	0	1 (<1)	0
Missing	666 (1)	518 (2)	511 (4)
GvHD Prophylaxis			
No GvHD Prophylaxis	208 (<1)	162 (1)	70 (1)
Ex vivo T-cell depletion alone	127 (<1)	45 (<1)	63 (<1)
Ex vivo T-cell depletion +- other	1122 (2)	305 (1)	382 (3)
CD34 select alone	309 (1)	182 (1)	117 (1)
CD34 select +- other	537 (1)	291 (1)	147 (1)
Cyclophosphamide alone	233 (<1)	94 (<1)	58 (<1)
Cyclophosphamide +- others	5003 (10)	6053 (24)	1208 (9)
FK506 + MMF +- others	5513 (11)	2213 (9)	1006 (8)
FK506 + MTX +- others(not MMF)	21115 (42)	9671 (39)	3662 (28)
FK506 +- others(not MMF,MTX)	2501 (5)	1377 (6)	493 (4)
FK506 alone	1202 (2)	532 (2)	226 (2)
CSA + MMF +- others(not FK506)	3118 (6)	1008 (4)	1067 (8)
CSA + MTX +- others(not MMF,FK506)	7022 (14)	1956 (8)	3542 (27)
CSA +- others(not FK506,MMF,MTX)	1089 (2)	337 (1)	467 (4)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
CSA alone	467 (1)	132 (1)	401 (3)
Other GVHD Prophylaxis	772 (2)	296 (1)	225 (2)
Missing	182 (<1)	55 (<1)	134 (1)
Donor/Recipient sex match			
Male-Male	20319 (40)	9586 (39)	5081 (38)
Male-Female	12469 (25)	5846 (24)	2904 (22)
Female-Male	8717 (17)	4479 (18)	2664 (20)
Female-Female	8572 (17)	4094 (17)	2399 (18)
CB - recipient M	18 (<1)	108 (<1)	3 (<1)
CB - recipient F	22 (<1)	91 (<1)	9 (<1)
Missing	403 (1)	505 (2)	208 (2)
Year of transplant			
1986-1990	347 (1)	47 (<1)	103 (1)
1991-1995	1837 (4)	439 (2)	745 (6)
1996-2000	3305 (7)	1184 (5)	1213 (9)
2001-2005	5347 (11)	1070 (4)	1880 (14)
2006-2010	9591 (19)	1921 (8)	1878 (14)
2011-2015	13348 (26)	3589 (15)	2650 (20)
2016-2020	10394 (21)	7186 (29)	2816 (21)
2021-2024	6351 (13)	9273 (38)	1983 (15)
Follow-up among survivors, Months			
N Eval	23098	14565	6370
Median (Range)	49 (0-384)	20 (0-362)	36 (0-385)

Table 4. Unrelated Cord Blood HCT Research Sample Inventory - Summary for First Allogeneic Transplants in CRF and TED with biospecimens available through the CIBMTR Repository stratified by availability of paired samples, recipient only samples and donor only samples, Biospecimens include: whole blood, serum/plasma and limited quantities of viable cells and cell lines (collected prior to 2006), Specific inventory queries available upon request through the CIBMTR Immunobiology Research Program

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	6426	1862	2353
Source of data			
CRF	4570 (71)	1180 (63)	1105 (47)
TED	1856 (29)	682 (37)	1248 (53)
Number of centers	155	146	230
Disease at transplant			
AML	2441 (38)	651 (35)	768 (33)
ALL	1319 (21)	404 (22)	520 (22)
Other leukemia	102 (2)	30 (2)	37 (2)
CML	137 (2)	37 (2)	57 (2)
MDS	579 (9)	180 (10)	188 (8)
Other acute leukemia	101 (2)	26 (1)	49 (2)
NHL	414 (6)	109 (6)	140 (6)
Hodgkin Lymphoma	103 (2)	27 (1)	35 (1)
Plasma Cell Disorders, MM	38 (1)	12 (1)	13 (1)
Other malignancies	12 (<1)	1 (<1)	3 (<1)
SAA	96 (1)	36 (2)	52 (2)
Inherited abnormalities erythrocyte diff fxn	171 (3)	51 (3)	45 (2)
Inherited bone marrow failure syndromes	8 (<1)	5 (<1)	6 (<1)
Hemoglobinopathies	3 (<1)	1 (<1)	1 (<1)
SCIDs	294 (5)	97 (5)	183 (8)
Inherited abnormalities of platelets	21 (<1)	6 (<1)	10 (<1)
Inherited disorders of metabolism	404 (6)	138 (7)	156 (7)
Histiocytic disorders	111 (2)	31 (2)	54 (2)
Autoimmune disorders	8 (<1)	0	6 (<1)
MPN	54 (1)	17 (1)	20 (1)
Others	10 (<1)	3 (<1)	10 (<1)
AML Disease status at transplant			
CR1	1292 (53)	377 (58)	393 (51)
CR2	647 (27)	159 (24)	198 (26)
CR3+	67 (3)	11 (2)	28 (4)
Advanced or active disease	427 (17)	101 (16)	143 (19)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Missing	8 (<1)	3 (<1)	6 (1)
ALL Disease status at transplant			
CR1	591 (45)	173 (43)	225 (43)
CR2	500 (38)	150 (37)	189 (36)
CR3+	151 (11)	56 (14)	64 (12)
Advanced or active disease	76 (6)	24 (6)	40 (8)
Missing	1 (<1)	1 (<1)	2 (<1)
MDS Disease status at transplant			
Early	177 (31)	43 (24)	75 (40)
Advanced	349 (60)	122 (68)	90 (48)
Missing	53 (9)	15 (8)	23 (12)
NHL Disease status at transplant			
CR1	66 (16)	12 (11)	26 (19)
CR2	77 (19)	26 (24)	35 (25)
CR3+	47 (11)	11 (10)	12 (9)
PR	68 (17)	12 (11)	16 (12)
Advanced	153 (37)	47 (43)	47 (34)
Missing	0	1 (1)	3 (2)
Recipient age at transplant			
0-9 years	1939 (30)	672 (36)	848 (36)
10-17 years	675 (11)	174 (9)	270 (11)
18-29 years	765 (12)	167 (9)	252 (11)
30-39 years	617 (10)	172 (9)	233 (10)
40-49 years	681 (11)	179 (10)	223 (9)
50-59 years	877 (14)	226 (12)	296 (13)
60-69 years	750 (12)	232 (12)	212 (9)
70+ years	122 (2)	40 (2)	19 (1)
Median (Range)	27 (0-85)	23 (0-84)	20 (0-78)
Recipient race			
White	4512 (74)	1293 (74)	1426 (72)
Black or African American	952 (16)	257 (15)	293 (15)
Asian	383 (6)	137 (8)	179 (9)
Native Hawaiian or other Pacific Islander	36 (1)	4 (<1)	21 (1)
American Indian or Alaska Native	61 (1)	18 (1)	24 (1)
Other	1 (<1)	1 (<1)	1 (<1)
More than one race	133 (2)	41 (2)	39 (2)
Unknown	348 (N/A)	111 (N/A)	370 (N/A)
Recipient ethnicity			
Hispanic or Latino	1352 (22)	349 (19)	401 (18)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Non Hispanic or non-Latino	4863 (78)	1416 (79)	1395 (61)
Non-resident of the U.S.	54 (1)	27 (2)	494 (22)
Unknown	157 (N/A)	70 (N/A)	63 (N/A)
Recipient sex			
Male	3569 (56)	1060 (57)	1338 (57)
Female	2857 (44)	802 (43)	1015 (43)
Karnofsky score			
10-80	1714 (27)	477 (26)	584 (25)
90-100	4483 (70)	1261 (68)	1553 (66)
Missing	229 (4)	124 (7)	216 (9)
HLA-A B DRB1 groups - low resolution			
<=3/6	181 (3)	103 (6)	65 (3)
4/6	2414 (41)	642 (39)	822 (40)
5/6	2526 (43)	657 (40)	858 (42)
6/6	764 (13)	230 (14)	310 (15)
Unknown	541 (N/A)	230 (N/A)	298 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	2802 (55)	669 (54)	881 (53)
6/8	1233 (24)	299 (24)	387 (23)
7/8	734 (14)	181 (14)	246 (15)
8/8	370 (7)	101 (8)	133 (8)
Unknown	1287 (N/A)	612 (N/A)	706 (N/A)
HLA-DPB1 Match			
Double allele mismatch	896 (37)	163 (32)	225 (37)
Single allele mismatch	1289 (53)	294 (58)	320 (53)
Full allele matched	240 (10)	50 (10)	63 (10)
Unknown	4001 (N/A)	1355 (N/A)	1745 (N/A)
High resolution release score			
No	4954 (77)	1812 (97)	2320 (99)
Yes	1472 (23)	50 (3)	33 (1)
KIR typing available			
No	5153 (80)	1856 (>99)	2325 (99)
Yes	1273 (20)	6 (<1)	28 (1)
Graft type			
UCB	6025 (94)	1663 (89)	2210 (94)
BM+UCB	1 (<1)	0	0
PBSC+UCB	369 (6)	191 (10)	129 (5)
Others	31 (<1)	8 (<1)	14 (1)
Number of cord units			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
1	5387 (84)	0	1968 (84)
2	1037 (16)	0	384 (16)
3	1 (<1)	0	0
Unknown	1 (N/A)	1862 (N/A)	1 (N/A)
Conditioning regimen			
Myeloablative	4181 (65)	1192 (64)	1481 (63)
RIC/Nonmyeloablative	2229 (35)	664 (36)	850 (36)
TBD	16 (<1)	6 (<1)	22 (1)
Donor/Recipient CMV serostatus			
+/+	0	0	1 (<1)
-/-	0	0	1 (<1)
CB - recipient +	4036 (63)	1133 (61)	1432 (61)
CB - recipient -	2288 (36)	662 (36)	843 (36)
CB - recipient CMV unknown	102 (2)	67 (4)	76 (3)
GvHD Prophylaxis			
No GvHD Prophylaxis	25 (<1)	10 (1)	17 (1)
Ex vivo T-cell depletion alone	1 (<1)	0	0
Ex vivo T-cell depletion +- other	27 (<1)	9 (<1)	9 (<1)
CD34 select alone	0	2 (<1)	1 (<1)
CD34 select +- other	286 (4)	147 (8)	85 (4)
Cyclophosphamide alone	0	0	1 (<1)
Cyclophosphamide +- others	18 (<1)	11 (1)	11 (<1)
FK506 + MMF +- others	1913 (30)	595 (32)	486 (21)
FK506 + MTX +- others(not MMF)	218 (3)	57 (3)	77 (3)
FK506 +- others(not MMF,MTX)	235 (4)	69 (4)	93 (4)
FK506 alone	147 (2)	44 (2)	27 (1)
CSA + MMF +- others(not FK506)	2914 (45)	730 (39)	1132 (48)
CSA + MTX +- others(not MMF,FK506)	100 (2)	30 (2)	50 (2)
CSA +- others(not FK506,MMF,MTX)	342 (5)	116 (6)	236 (10)
CSA alone	51 (1)	18 (1)	73 (3)
Other GVHD Prophylaxis	137 (2)	21 (1)	45 (2)
Missing	12 (<1)	3 (<1)	10 (<1)
Donor/Recipient sex match			
Male-Female	0	0	1 (<1)
Female-Male	0	0	1 (<1)
CB - recipient M	3569 (56)	1060 (57)	1336 (57)
CB - recipient F	2857 (44)	802 (43)	1014 (43)
CB - recipient sex unknown	0	0	1 (<1)
Year of transplant			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
1996-2000	1 (<1)	2 (<1)	5 (<1)
2001-2005	112 (2)	85 (5)	34 (1)
2006-2010	1850 (29)	427 (23)	618 (26)
2011-2015	2678 (42)	513 (28)	840 (36)
2016-2020	1340 (21)	529 (28)	552 (23)
2021-2024	445 (7)	306 (16)	304 (13)
Follow-up among survivors, Months			
N Eval	3180	1050	1246
Median (Range)	61 (0-196)	38 (0-213)	37 (0-240)

Table 5. Related Donor HCT Research Sample Inventory - Summary for First Allogeneic Transplants in CRF and TED with biospecimens available through the CIBMTR Repository stratified by availability of paired samples, recipient only samples and donor only samples, Biospecimens include: whole blood, serum/plasma and limited quantities of viable cells and cell lines (collected prior to 2006), Specific inventory queries available upon request through the CIBMTR Immunobiology Research Program

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	12809	2225	1080
Source of data			
CRF	4126 (32)	601 (27)	337 (31)
TED	8683 (68)	1624 (73)	743 (69)
Number of centers	96	81	68
Disease at transplant			
AML	4215 (33)	718 (32)	375 (35)
ALL	2158 (17)	447 (20)	202 (19)
Other leukemia	230 (2)	43 (2)	19 (2)
CML	375 (3)	54 (2)	32 (3)
MDS	1702 (13)	272 (12)	148 (14)
Other acute leukemia	200 (2)	37 (2)	11 (1)
NHL	1041 (8)	192 (9)	91 (8)
Hodgkin Lymphoma	229 (2)	43 (2)	31 (3)
Plasma Cell Disorders, MM	265 (2)	41 (2)	22 (2)
Other malignancies	24 (<1)	1 (<1)	1 (<1)
Breast cancer	1 (<1)	0	0
SAA	621 (5)	94 (4)	40 (4)
Inherited abnormalities erythrocyte diff fxn	493 (4)	71 (3)	18 (2)
Inherited bone marrow failure syndromes	43 (<1)	3 (<1)	5 (<1)
Hemoglobinopathies	247 (2)	47 (2)	19 (2)
Paroxysmal nocturnal hemoglobinuria	3 (<1)	1 (<1)	0
SCIDs	279 (2)	46 (2)	18 (2)
Inherited abnormalities of platelets	11 (<1)	0	0
Inherited disorders of metabolism	24 (<1)	7 (<1)	2 (<1)
Histiocytic disorders	73 (1)	9 (<1)	6 (1)
Autoimmune disorders	12 (<1)	0	1 (<1)
MPN	540 (4)	97 (4)	39 (4)
Others	23 (<1)	2 (<1)	0
AML Disease status at transplant			
CR1	2813 (67)	494 (69)	242 (65)
CR2	632 (15)	94 (13)	46 (12)
CR3+	50 (1)	16 (2)	2 (1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Advanced or active disease	713 (17)	109 (15)	85 (23)
Missing	7 (<1)	5 (1)	0
ALL Disease status at transplant			
CR1	1275 (59)	268 (60)	131 (65)
CR2	649 (30)	120 (27)	50 (25)
CR3+	140 (6)	31 (7)	9 (4)
Advanced or active disease	94 (4)	28 (6)	12 (6)
MDS Disease status at transplant			
Early	300 (18)	39 (14)	25 (17)
Advanced	1349 (79)	220 (81)	116 (78)
Missing	53 (3)	13 (5)	7 (5)
NHL Disease status at transplant			
CR1	207 (20)	46 (24)	19 (21)
CR2	197 (19)	37 (19)	14 (15)
CR3+	107 (10)	24 (13)	7 (8)
PR	68 (7)	13 (7)	7 (8)
Advanced	453 (44)	71 (37)	44 (48)
Missing	5 (<1)	0	0
Recipient age at transplant			
0-9 years	1386 (11)	209 (9)	86 (8)
10-17 years	1313 (10)	179 (8)	77 (7)
18-29 years	1508 (12)	298 (13)	121 (11)
30-39 years	980 (8)	192 (9)	114 (11)
40-49 years	1503 (12)	277 (12)	122 (11)
50-59 years	2584 (20)	471 (21)	228 (21)
60-69 years	2950 (23)	498 (22)	275 (25)
70+ years	585 (5)	101 (5)	57 (5)
Median (Range)	49 (0-82)	49 (0-77)	51 (0-83)
Recipient race			
White	9481 (78)	1519 (74)	791 (79)
Black or African American	1717 (14)	311 (15)	121 (12)
Asian	616 (5)	172 (8)	65 (6)
Native Hawaiian or other Pacific Islander	48 (<1)	8 (<1)	3 (<1)
American Indian or Alaska Native	87 (1)	11 (1)	9 (1)
More than one race	162 (1)	19 (1)	13 (1)
Unknown	698 (N/A)	185 (N/A)	78 (N/A)
Recipient ethnicity			
Hispanic or Latino	2441 (19)	543 (25)	231 (22)
Non Hispanic or non-Latino	9996 (80)	1598 (74)	805 (76)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Non-resident of the U.S.	128 (1)	26 (1)	18 (2)
Unknown	244 (N/A)	58 (N/A)	26 (N/A)
Recipient sex			
Male	7507 (59)	1306 (59)	629 (58)
Female	5302 (41)	919 (41)	451 (42)
Karnofsky score			
10-80	4626 (36)	880 (40)	471 (44)
90-100	7728 (60)	1270 (57)	552 (51)
Missing	455 (4)	75 (3)	57 (5)
HLA-A B DRB1 groups - low resolution			
<=3/6	2995 (25)	510 (25)	280 (32)
4/6	889 (7)	170 (8)	92 (11)
5/6	272 (2)	50 (2)	25 (3)
6/6	7882 (65)	1287 (64)	471 (54)
Unknown	771 (N/A)	208 (N/A)	212 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	3713 (32)	629 (33)	327 (42)
6/8	171 (1)	46 (2)	13 (2)
7/8	186 (2)	31 (2)	18 (2)
8/8	7594 (65)	1203 (63)	420 (54)
Unknown	1145 (N/A)	316 (N/A)	302 (N/A)
HLA-DPB1 Match			
Double allele mismatch	14 (<1)	0	1 (<1)
Single allele mismatch	3168 (30)	410 (32)	226 (42)
Full allele matched	7462 (70)	887 (68)	316 (58)
Unknown	2165 (N/A)	928 (N/A)	537 (N/A)
High resolution release score			
No	6504 (51)	2196 (99)	1069 (99)
Yes	6305 (49)	29 (1)	11 (1)
Graft type			
Marrow	3705 (29)	490 (22)	282 (26)
PBSC	8988 (70)	1694 (76)	789 (73)
UCB	2 (<1)	14 (1)	0
BM+PBSC	19 (<1)	6 (<1)	1 (<1)
BM+UCB	46 (<1)	13 (1)	2 (<1)
PBSC+UCB	1 (<1)	2 (<1)	5 (<1)
Others	48 (<1)	6 (<1)	1 (<1)
Conditioning regimen			
Myeloablative	7117 (56)	1224 (55)	553 (51)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
RIC/Nonmyeloablative	5628 (44)	984 (44)	510 (47)
TBD	64 (<1)	17 (1)	17 (2)
Donor age at donation			
To Be Determined/NA	17 (<1)	7 (<1)	2 (<1)
0-9 years	897 (7)	132 (6)	39 (4)
10-17 years	1030 (8)	171 (8)	62 (6)
18-29 years	2369 (18)	408 (18)	233 (22)
30-39 years	2002 (16)	389 (17)	208 (19)
40-49 years	2042 (16)	360 (16)	168 (16)
50+ years	4452 (35)	758 (34)	368 (34)
Median (Range)	40 (0-82)	40 (0-79)	40 (0-80)
Donor/Recipient CMV serostatus			
+/+	5191 (41)	995 (45)	442 (41)
+/-	1374 (11)	190 (9)	108 (10)
-/+	3232 (25)	540 (24)	284 (26)
-/-	2788 (22)	439 (20)	214 (20)
CB - recipient +	31 (<1)	17 (1)	6 (1)
CB - recipient -	18 (<1)	12 (1)	1 (<1)
Missing	175 (1)	32 (1)	25 (2)
GvHD Prophylaxis			
No GvHD Prophylaxis	191 (1)	26 (1)	17 (2)
Ex vivo T-cell depletion alone	122 (1)	37 (2)	12 (1)
Ex vivo T-cell depletion +- other	119 (1)	30 (1)	12 (1)
CD34 select alone	83 (1)	26 (1)	11 (1)
CD34 select +- other	96 (1)	28 (1)	9 (1)
Cyclophosphamide alone	80 (1)	12 (1)	10 (1)
Cyclophosphamide +- others	4566 (36)	754 (34)	452 (42)
FK506 + MMF +- others	851 (7)	105 (5)	35 (3)
FK506 + MTX +- others(not MMF)	4357 (34)	659 (30)	359 (33)
FK506 +- others(not MMF,MTX)	867 (7)	339 (15)	72 (7)
FK506 alone	118 (1)	19 (1)	5 (<1)
CSA + MMF +- others(not FK506)	246 (2)	41 (2)	18 (2)
CSA + MTX +- others(not MMF,FK506)	756 (6)	96 (4)	44 (4)
CSA +- others(not FK506,MMF,MTX)	81 (1)	10 (<1)	3 (<1)
CSA alone	83 (1)	12 (1)	4 (<1)
Other GVHD Prophylaxis	181 (1)	23 (1)	17 (2)
Missing	12 (<1)	8 (<1)	0
Donor/Recipient sex match			
Male-Male	4269 (33)	790 (36)	365 (34)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Male-Female	2718 (21)	451 (20)	234 (22)
Female-Male	3203 (25)	496 (22)	261 (24)
Female-Female	2566 (20)	457 (21)	213 (20)
CB - recipient M	31 (<1)	18 (1)	3 (<1)
CB - recipient F	18 (<1)	11 (<1)	4 (<1)
Missing	4 (<1)	2 (<1)	0
Year of transplant			
2006-2010	604 (5)	71 (3)	58 (5)
2011-2015	3685 (29)	504 (23)	215 (20)
2016-2020	5016 (39)	902 (41)	407 (38)
2021-2024	3504 (27)	748 (34)	400 (37)
Follow-up among survivors, Months			
N Eval	8318	1467	694
Median (Range)	25 (0-150)	24 (0-147)	24 (0-148)

Table 6. Characteristics of TED/CRF track recipients who underwent a first allogeneic transplant registered with the CIBMTR between 1968-2022

Characteristic	TED N (%)	CRF N (%)
No. of patients	273533	112940
No. of centers	676	589
Age at transplant, years - no. (%)		
Median (min-max)	37.7 (0.0-87.8)	33.1 (0.0-87.8)
0-9	38364 (14.0)	19173 (17.0)
10-19	34240 (12.5)	16364 (14.5)
20-29	35100 (12.8)	15831 (14.0)
30-39	38654 (14.1)	16653 (14.7)
40-49	43891 (16.0)	16878 (14.9)
50-59	44469 (16.3)	14797 (13.1)
60-69	33138 (12.1)	11079 (9.8)
70+	5677 (2.1)	2165 (1.9)
Recipient gender - no. (%)		
Male	160088 (58.5)	66394 (58.8)
Female	113445 (41.5)	46546 (41.2)
Recipient race - no. (%)		
Caucasian	184171 (67.3)	89060 (78.9)
African-American	13923 (5.1)	6933 (6.1)
Asian	20088 (7.3)	8821 (7.8)
Pacific islander	588 (0.2)	277 (0.2)
Native American	912 (0.3)	440 (0.4)
Other	8543 (3.1)	4031 (3.6)
Unknown	45308 (16.6)	3378 (3.0)
Disease - no. (%)		
Acute myelogenous leukemia	87769 (32.1)	31402 (27.8)
Acute lymphoblastic leukemia	46736 (17.1)	18445 (16.3)
Other leukemia	6703 (2.5)	2422 (2.1)
Chronic myelogenous leukemia	30427 (11.1)	15360 (13.6)
Myelodysplastic/myeloproliferative disorders	34402 (12.6)	15123 (13.4)
Other acute leukemia	3115 (1.1)	1069 (0.9)
Non-Hodgkin lymphoma	18339 (6.7)	6512 (5.8)
Hodgkin lymphoma	1767 (0.6)	660 (0.6)
Plasma cell disorder/Multiple Myeloma	3453 (1.3)	1421 (1.3)
Other Malignancies	1229 (0.4)	510 (0.5)

Characteristic	TED N (%)	CRF N (%)
Breast Cancer	183 (0.1)	93 (0.1)
Severe aplastic anemia	15327 (5.6)	7855 (7.0)
Inherited abnormalities erythrocyte differentiation or function	11455 (4.2)	5945 (5.3)
SCID and other immune system disorders	6982 (2.6)	3424 (3.0)
Inherited abnormalities of platelets	234 (0.1)	112 (0.1)
Inherited disorders of metabolism	2960 (1.1)	1650 (1.5)
Histiocytic disorders	1880 (0.7)	800 (0.7)
Autoimmune Diseases	164 (0.1)	50 (0.0)
Other diseases	408 (0.1)	87 (0.1)
Year of transplant - no. (%)		
<1985	4899 (1.8)	4507 (4.0)
1985-1989	10639 (3.9)	9507 (8.4)
1990-1994	23038 (8.4)	14871 (13.2)
1995-1999	36471 (13.3)	17192 (15.2)
2000-2004	41758 (15.3)	17722 (15.7)
2005-2009	42700 (15.6)	18974 (16.8)
2010-2014	51642 (18.9)	12042 (10.7)
2015-2019	54605 (20.0)	16893 (15.0)
2020-2022	7781 (2.8)	1232 (1.1)
Education - no. (%)	NA	
No primary education		69 (0.1)
Less than primary or elementary education		90 (0.1)
Primary of elementary education		767 (0.7)
Lower secondary education		876 (0.8)
Upper secondary education		11678 (10.3)
Post-secondary , non-tertiary education		4321 (3.8)
Tertiary education, Type A		8690 (7.7)
Tertiary education, Type B		1870 (1.7)
Advance research qualification		2236 (2.0)
Age<18 years old		32500 (28.8)
Missing		49843 (44.1)
Health insurance - no. (%)	NA	
No insurance		4587 (4.1)
Medicaid		9844 (8.7)
Medicare		6544 (5.8)
Disability insurance		772 (0.7)
HMO		2539 (2.2)
Private health insurance		26161 (23.2)

Characteristic	TED N (%)	CRF N (%)
National health insurance		16065 (14.2)
VA/Military		792 (0.7)
Other		3669 (3.2)
Missing		41967 (37.2)
Heath insurance - no. (%)	NA	
No insurance		3600 (3.2)
Disability insurance +/-others		881 (0.8)
Private health insurance +/- others		31927 (28.3)
Medicaid +/-others		8718 (7.7)
Medicare +/-others		4035 (3.6)
Others		21812 (19.3)
Missing		41967 (37.2)
Occupation - no. (%)	NA	
Professional, technical, or related occupation		19906 (17.6)
Manager, administrator or proprietor		4105 (3.6)
Clerical or related occupation		2841 (2.5)
Sales occupation		2135 (1.9)
Service occupation		3476 (3.1)
Skilled crafts or related occupation		3438 (3.0)
Equipment/vehicle operator or related occupation		1617 (1.4)
Laborer		2225 (2.0)
Farmer		423 (0.4)
Member of military		362 (0.3)
Homemaker		1642 (1.5)
Student		11489 (10.2)
Under school age		2724 (2.4)
Not previously employed		2153 (1.9)
Other, specify		8090 (7.2)
Missing		46314 (41.0)

Table 7. Characteristics of TED/CRF track recipients who underwent a first autologous transplant registered with the CIBMTR between 1968-2022

Characteristic	TED N (%)	CRF N (%)
No. of patients	272409	49244
No. of centers	635	465
Age at transplant, years - no. (%)		
Median (min-max)	53.6 (0.0-86.4)	50.3 (0.0-83.2)
0-9	11519 (4.2)	2373 (4.8)
10-19	8270 (3.0)	1838 (3.7)
20-29	17700 (6.5)	3389 (6.9)
30-39	26827 (9.8)	6048 (12.3)
40-49	47864 (17.6)	10630 (21.6)
50-59	73941 (27.1)	13009 (26.4)
60-69	69942 (25.7)	10129 (20.6)
70+	16346 (6.0)	1828 (3.7)
Recipient gender - no. (%)		
Male	147757 (54.2)	24370 (49.5)
Female	124652 (45.8)	24874 (50.5)
Recipient race - no. (%)		
Caucasian	189096 (69.4)	38393 (78.0)
African-American	25695 (9.4)	6287 (12.8)
Asian	7095 (2.6)	1450 (2.9)
Pacific islander	356 (0.1)	60 (0.1)
Native American	823 (0.3)	232 (0.5)
Other	5620 (2.1)	1443 (2.9)
Unknown	43724 (16.1)	1379 (2.8)
Disease - no. (%)		
Acute myelogenous leukemia	8477 (3.1)	2504 (5.1)
Acute lymphoblastic leukemia	1657 (0.6)	485 (1.0)
Other leukemia	809 (0.3)	258 (0.5)
Chronic myelogenous leukemia	715 (0.3)	297 (0.6)
Myelodysplastic/myeloproliferative disorders	291 (0.1)	95 (0.2)
Other acute leukemia	165 (0.1)	31 (0.1)
Non-Hodgkin lymphoma	73249 (26.9)	11819 (24.0)
Hodgkin lymphoma	27495 (10.1)	4417 (9.0)
Plasma cell disorder/Multiple Myeloma	113141 (41.5)	16848 (34.2)
Other Malignancies	21262 (7.8)	4486 (9.1)
Breast Cancer	22948 (8.4)	7773 (15.8)

Characteristic	TED N (%)	CRF N (%)
Autoimmune Diseases	1560 (0.6)	142 (0.3)
Other diseases	640 (0.2)	89 (0.2)
Year of transplant - no. (%)		
<1985	31 (0.0)	5 (0.0)
1985-1989	2089 (0.8)	673 (1.4)
1990-1994	20048 (7.4)	7587 (15.4)
1995-1999	42023 (15.4)	13099 (26.6)
2000-2004	36630 (13.4)	6300 (12.8)
2005-2009	39539 (14.5)	8366 (17.0)
2010-2014	53702 (19.7)	4702 (9.5)
2015-2019	68088 (25.0)	8238 (16.7)
2020-2022	10259 (3.8)	274 (0.6)
Education - no. (%)	NA	
No primary education		18 (0.0)
Less than primary or elementary education		55 (0.1)
Primary of elementary education		356 (0.7)
Lower secondary education		429 (0.9)
Upper secondary education		7008 (14.2)
Post-secondary , non-tertiary education		2872 (5.8)
Tertiary education, Type A		5807 (11.8)
Tertiary education, Type B		1274 (2.6)
Advance research qualification		1783 (3.6)
Age<18 years old		3741 (7.6)
Missing		25901 (52.6)
Health insurance - no. (%)	NA	
No insurance		886 (1.8)
Medicaid		3823 (7.8)
Medicare		4788 (9.7)
Missing		39747 (80.7)
Heath insurance - no. (%)	NA	
No insurance		886 (1.8)
Medicaid +/-others		3823 (7.8)
Medicare +/-others		4788 (9.7)
Missing		39747 (80.7)
Occupation - no. (%)	NA	
Professional, technical, or related occupation		17402 (35.3)
Manager, administrator or proprietor		1908 (3.9)
Clerical or related occupation		1353 (2.7)

Characteristic	TED N (%)	CRF N (%)
Sales occupation		912 (1.9)
Service occupation		1787 (3.6)
Skilled crafts or related occupation		1622 (3.3)
Equipment/vehicle operator or related occupation		912 (1.9)
Laborer		1076 (2.2)
Farmer		232 (0.5)
Member of military		182 (0.4)
Homemaker		664 (1.3)
Student		1190 (2.4)
Under school age		381 (0.8)
Not previously employed		1100 (2.2)
Other, specify		3560 (7.2)
Missing		14963 (30.4)

Table 8. Characteristics of CRF track recipients who received a first transplant from US centers reported to the CIBMTR, 2008 – 2023

Characteristic	Allogeneic	Autologous
No. of patients	41256	18797
No. of centers	193	198
Age at transplant, years - no. (%)		
Median (min-max)	51.4 (0.0-87.8)	57.7 (0.2-82.8)
0-9	5230 (12.7)	1096 (5.8)
10-19	3475 (8.4)	423 (2.3)
20-29	3304 (8.0)	868 (4.6)
30-39	3199 (7.8)	1092 (5.8)
40-49	4535 (11.0)	2308 (12.3)
50-59	7869 (19.1)	5070 (27.0)
60-69	10779 (26.1)	6375 (33.9)
70+	2863 (6.9)	1565 (8.3)
Not reported	2 (0.0)	0 (0.0)
Recipient gender - no. (%)		
Male	24334 (59.0)	10997 (58.5)
Female	16922 (41.0)	7792 (41.5)
Not reported	0 (0.0)	8 (0.0)
Recipient race - no. (%)		
White	32127 (77.9)	12869 (68.5)
Black or African American	4715 (11.4)	4401 (23.4)
Asian	2051 (5.0)	709 (3.8)
Native Hawaiian or other Pacific Islander	140 (0.3)	44 (0.2)
American Indian or Alaska Native	269 (0.7)	162 (0.9)
More than one race	614 (1.5)	216 (1.1)
Not reported	1340 (3.2)	396 (2.1)
Disease - no. (%)		
AML or ANLL	12618 (30.6)	189 (1.0)
ALL	4719 (11.4)	17 (0.1)
Other Leukemia	844 (2.0)	15 (0.1)
CML	922 (2.2)	0 (0.0)
MDS	7779 (18.9)	2 (0.0)
Acute Leukemia	368 (0.9)	2 (0.0)
NHL	2619 (6.3)	3788 (20.2)
HD	857 (2.1)	1512 (8.0)
Plasma cell disorder	637 (1.5)	11409 (60.7)

Characteristic	Allogeneic	Autologous
Solid Tumor	32 (0.1)	1672 (8.9)
Aplastic anemia	2232 (5.4)	2 (0.0)
Inherited abnormal of erythrocyte differ.	37 (0.1)	0 (0.0)
Inherited bone marrow failure syndromes	519 (1.3)	2 (0.0)
Hemoglobinopathies	1334 (3.2)	47 (0.3)
Paroxysmal nocturnal hemoglobinuria (PNH)	80 (0.2)	0 (0.0)
Immune Deficiencies (ID)	1444 (3.5)	68 (0.4)
Inherited platelet abnormality	43 (0.1)	0 (0.0)
Inherited disorders of metabolism	537 (1.3)	14 (0.1)
Histiocytic disorder	294 (0.7)	2 (0.0)
Autoimmune Disease	22 (0.1)	48 (0.3)
Other non-malignant disorder	5 (0.0)	3 (0.0)
Other disease	35 (0.1)	5 (0.0)
Tolerance induction associated with solid organ transplant	1 (0.0)	0 (0.0)
Myeloproliferative neoplasms	3278 (7.9)	0 (0.0)
Education - no. (%)		
No primary education	39 (0.1)	16 (0.1)
Less than primary or elementary education	62 (0.2)	34 (0.2)
Primary or elementary education	154 (0.4)	103 (0.5)
Lower secondary education	727 (1.8)	398 (2.1)
Upper secondary education	8169 (19.8)	4331 (23.0)
Post-secondary, non-tertiary education	2759 (6.7)	1472 (7.8)
Tertiary education, Type A	8104 (19.6)	3720 (19.8)
Tertiary education, Type B	1463 (3.5)	973 (5.2)
Advanced research qualification	1494 (3.6)	677 (3.6)
Less than 18 years old	8005 (19.4)	1398 (7.4)
Not reported	10280 (24.9)	5675 (30.2)
Health insurance - no. (%)		
No insurance	425 (1.0)	203 (1.1)
Private health insurance	21060 (51.0)	9688 (51.5)
Private health insurance + others	4246 (10.3)	2089 (11.1)
National health insurance	225 (0.5)	12 (0.1)
National health insurance + others	9 (0.0)	1 (0.0)
Medicare	4597 (11.1)	2174 (11.6)
Medicare + others	1012 (2.5)	506 (2.7)
Medigap	1 (0.0)	1 (0.0)
Medicaid	6414 (15.5)	1983 (10.5)
Medicaid + others	207 (0.5)	50 (0.3)

Characteristic	Allogeneic	Autologous
CHIP	24 (0.1)	0 (0.0)
VA/Military	537 (1.3)	262 (1.4)
VA/Military + others	5 (0.0)	3 (0.0)
Indian health service	2 (0.0)	1 (0.0)
State-sponsored health plan	94 (0.2)	25 (0.1)
State-sponsored health plan + others	2 (0.0)	1 (0.0)
Other government program	15 (0.0)	11 (0.1)
Other health insurance coverage	691 (1.7)	218 (1.2)
Not reported	1690 (4.1)	1569 (8.3)
Occupation - no. (%)		
Professional, technical, or related occupation	7684 (18.6)	3640 (19.4)
Manager, administrator or proprietor	3621 (8.8)	1741 (9.3)
Clerical or related occupation	2142 (5.2)	1182 (6.3)
Sales occupation	1672 (4.1)	747 (4.0)
Service occupation	2615 (6.3)	1484 (7.9)
Skilled crafts or related occupation	2844 (6.9)	1484 (7.9)
Equipment/vehicle operator or related occupation	1236 (3.0)	756 (4.0)
Laborer	1725 (4.2)	985 (5.2)
Farmer	278 (0.7)	174 (0.9)
Member of military	330 (0.8)	143 (0.8)
Homemaker	725 (1.8)	359 (1.9)
Student	5473 (13.3)	741 (3.9)
Under school age	3476 (8.4)	805 (4.3)
Not previously employed	609 (1.5)	334 (1.8)
Other, specify	1815 (4.4)	801 (4.3)
Not reported	5011 (12.1)	3421 (18.2)
Recipient zip code - no. (%)		
Not Available	17021 (41.3)	7752 (41.2)
Available	24235 (58.7)	11045 (58.8)
Year of transplant - no. (%)		
2008	3474 (8.4)	2441 (13.0)
2009	3254 (7.9)	1215 (6.5)
2010	2188 (5.3)	621 (3.3)
2011	1631 (4.0)	728 (3.9)
2012	1611 (3.9)	783 (4.2)
2013	2766 (6.7)	1453 (7.7)
2014	3510 (8.5)	1447 (7.7)
2015	3517 (8.5)	1661 (8.8)

Characteristic	Allogeneic	Autologous
2016	3213 (7.8)	1701 (9.0)
2017	3070 (7.4)	1565 (8.3)
2018	3033 (7.4)	2177 (11.6)
2019	2777 (6.7)	1285 (6.8)
2020	1947 (4.7)	360 (1.9)
2021	1965 (4.8)	298 (1.6)
2022	1789 (4.3)	589 (3.1)
2023	1511 (3.7)	473 (2.5)

Table 9. Characteristics of recipients who received allogeneic transplants registered with the CIBMTR by WHO region, 2008 – 2023

Characteristic	Africa	Latin Americas	US / Canada	Eastern Mediterranean	Europe	Southeastern Asia	Western Pacific
No. of patients	64	10116	145074	6895	19417	4151	12915
No. of centers	3	66	237	19	117	23	29
Age, in years - no. (%)							
<10	1 (1.6)	1941 (19.2)	14624 (10.1)	2754 (39.9)	1510 (7.8)	1528 (36.8)	1498 (11.6)
10-19	12 (18.8)	1883 (18.6)	12139 (8.4)	1610 (23.4)	1304 (6.7)	1034 (24.9)	1399 (10.8)
20-29	8 (12.5)	1478 (14.6)	12416 (8.6)	1184 (17.2)	1895 (9.8)	531 (12.8)	1340 (10.4)
30-39	4 (6.3)	1458 (14.4)	12758 (8.8)	699 (10.1)	2034 (10.5)	461 (11.1)	1488 (11.5)
40-49	14 (21.9)	1315 (13.0)	18080 (12.5)	378 (5.5)	3243 (16.7)	339 (8.2)	2138 (16.6)
50-59	14 (21.9)	1195 (11.8)	30690 (21.2)	211 (3.1)	4541 (23.4)	224 (5.4)	2820 (21.8)
60-69	11 (17.2)	713 (7.0)	35536 (24.5)	58 (0.8)	4233 (21.8)	32 (0.8)	2083 (16.1)
>=70	0 (0.0)	133 (1.3)	8831 (6.1)	1 (0.0)	657 (3.4)	2 (0.0)	149 (1.2)
Gender - no. (%)							
Male	38 (59.4)	5942 (58.7)	84547 (58.3)	4042 (58.6)	11570 (59.6)	2701 (65.1)	7583 (58.7)
Female	26 (40.6)	4174 (41.3)	60527 (41.7)	2853 (41.4)	7847 (40.4)	1450 (34.9)	5332 (41.3)
Primary disease - no. (%)							
Acute myelogenous leukemia	20 (31.3)	2737 (27.1)	53144 (36.6)	1182 (17.1)	7408 (38.2)	694 (16.7)	4876 (37.8)
Acute lymphoblastic leukemia	4 (6.3)	2537 (25.1)	21300 (14.7)	980 (14.2)	2751 (14.2)	459 (11.1)	2352 (18.2)
Chronic myelogenous leukemia	3 (4.7)	555 (5.5)	4130 (2.8)	182 (2.6)	600 (3.1)	136 (3.3)	302 (2.3)
Myelodysplastic disorders	14 (21.9)	1148 (11.3)	26869 (18.5)	257 (3.7)	3433 (17.7)	268 (6.5)	2087 (16.2)
Non-Hodgkin lymphoma	5 (7.8)	374 (3.7)	12196 (8.4)	71 (1.0)	1407 (7.2)	61 (1.5)	883 (6.8)

Characteristic	Africa	Latin Americas	US / Canada	Eastern Mediterranean	Europe	Southeastern Asia	Western Pacific
Hodgkin lymphoma	1 (1.6)	302 (3.0)	2565 (1.8)	76 (1.1)	297 (1.5)	44 (1.1)	189 (1.5)
Multiple myeloma	2 (3.1)	69 (0.7)	2615 (1.8)	18 (0.3)	747 (3.8)	11 (0.3)	267 (2.1)
Other malignancies	1 (1.6)	234 (2.3)	5560 (3.8)	82 (1.2)	950 (4.9)	42 (1.0)	425 (3.3)
Severe aplastic anemia	6 (9.4)	1057 (10.4)	5387 (3.7)	795 (11.5)	630 (3.2)	569 (13.7)	802 (6.2)
Other non-malignancies	8 (12.5)	1103 (10.9)	11285 (7.8)	3252 (47.2)	1194 (6.1)	1867 (45.0)	732 (5.7)
Tolerance induction associated with solid organ transplant	0 (0.0)	0 (0.0)	22 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Recessive Dystrophic Epidermolysis Bullosa	0 (0.0)	0 (0.0)	1 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Donor type - no. (%)							
HLA-identical sibling	29 (45.3)	4281 (42.3)	39304 (27.1)	5090 (73.8)	5729 (29.5)	2425 (58.4)	4377 (33.9)
Other Related donor	2 (3.1)	3143 (31.1)	24033 (16.6)	1271 (18.4)	1414 (7.3)	1259 (30.3)	1998 (15.5)
Unrelated donor	33 (51.6)	2690 (26.6)	81644 (56.3)	532 (7.7)	11471 (59.1)	467 (11.3)	6537 (50.6)
Missing	0 (0.0)	2 (0.0)	93 (0.1)	2 (0.0)	803 (4.1)	0 (0.0)	3 (0.0)
Graft type - no. (%)							
Bone Marrow	6 (9.4)	4545 (44.9)	28755 (19.8)	3580 (51.9)	3544 (18.3)	891 (21.5)	1861 (14.4)
Peripheral Blood	57 (89.1)	5275 (52.1)	105189 (72.5)	2991 (43.4)	15056 (77.5)	3230 (77.8)	10180 (78.8)
Cord Blood	1 (1.6)	290 (2.9)	11097 (7.6)	321 (4.7)	809 (4.2)	28 (0.7)	856 (6.6)
Missing	0 (0.0)	6 (0.1)	33 (0.0)	3 (0.0)	8 (0.0)	2 (0.0)	18 (0.1)
Year of transplant - no. (%)							
2008	8 (12.5)	220 (2.2)	6885 (4.7)	455 (6.6)	2004 (10.3)	90 (2.2)	594 (4.6)

Characteristic	Africa	Latin Americas	US / Canada	Eastern Mediterranean	Europe	Southeastern Asia	Western Pacific
2009	12 (18.8)	378 (3.7)	7292 (5.0)	486 (7.0)	2090 (10.8)	115 (2.8)	814 (6.3)
2010	9 (14.1)	463 (4.6)	7457 (5.1)	477 (6.9)	2160 (11.1)	127 (3.1)	942 (7.3)
2011	16 (25.0)	432 (4.3)	7975 (5.5)	239 (3.5)	2016 (10.4)	164 (4.0)	1027 (8.0)
2012	8 (12.5)	493 (4.9)	8234 (5.7)	270 (3.9)	2021 (10.4)	168 (4.0)	984 (7.6)
2013	6 (9.4)	471 (4.7)	8753 (6.0)	284 (4.1)	1853 (9.5)	167 (4.0)	874 (6.8)
2014	0 (0.0)	489 (4.8)	8936 (6.2)	299 (4.3)	1277 (6.6)	206 (5.0)	881 (6.8)
2015	0 (0.0)	482 (4.8)	9184 (6.3)	290 (4.2)	1207 (6.2)	247 (6.0)	729 (5.6)
2016	0 (0.0)	480 (4.7)	9165 (6.3)	316 (4.6)	1030 (5.3)	308 (7.4)	941 (7.3)
2017	1 (1.6)	629 (6.2)	9570 (6.6)	394 (5.7)	1518 (7.8)	342 (8.2)	712 (5.5)
2018	3 (4.7)	783 (7.7)	9978 (6.9)	411 (6.0)	608 (3.1)	366 (8.8)	662 (5.1)
2019	1 (1.6)	869 (8.6)	10220 (7.0)	494 (7.2)	447 (2.3)	405 (9.8)	733 (5.7)
2020	0 (0.0)	753 (7.4)	9827 (6.8)	388 (5.6)	348 (1.8)	227 (5.5)	677 (5.2)
2021	0 (0.0)	824 (8.1)	10154 (7.0)	587 (8.5)	269 (1.4)	309 (7.4)	722 (5.6)
2022	0 (0.0)	1129 (11.2)	10222 (7.0)	611 (8.9)	270 (1.4)	440 (10.6)	731 (5.7)
2023	0 (0.0)	1221 (12.1)	11222 (7.7)	894 (13.0)	299 (1.5)	470 (11.3)	892 (6.9)

Table 10. Allogeneic transplant recipients and centers by country registered with the CIBMTR, 2008-2023

Regions	N	Centers
Africa		
South Africa	59	2
Nigeria	5	1
Americas		
USA	136E3	224
Argentina	871	8
Brazil	7893	40
Canada	9395	25
Chile	21	2
Venezuela	53	2
Mexico	770	5
Uruguay	102	3
Peru	125	2
Colombia	227	3
Ecuador	47	1
Paraguay	7	1
Eastern Mediterranean		
Saudi Arabia	4668	8
Egypt	215	2
Iran	679	1
Kuwait	29	1
Pakistan	1304	7
Europe		
Austria	101	2
Belgium	1701	6
Denmark	1753	1
United Kingdom	2557	18
Finland	411	2

Regions	N	Centers
France	1199	10
Germany	3804	20
Ireland	169	1
Israel	1315	8
Italy	603	7
Netherlands	860	10
Norway	154	2
Poland	392	4
Portugal	136	2
Spain	669	9
Sweden	983	4
Switzerland	1068	3
Russia	93	1
Turkey	692	3
Greece	3	1
Czech Republic	637	3
Slovak Republic	117	1
Southeastern Asia		
India	4130	22
Thailand	21	1
Western Pacific		
Australia	6721	17
South Korea	3393	3
New Zealand	1450	6
Taiwan	79	1
Hong Kong	125	1
Singapore	1147	5

Table 11. Number of patients who received a first allogeneic transplant registered with the CIBMTR between 2008 and 2023 by country

	CRF		TED	
Country	**Malignant disease**	**Non-malignant disease**	**Malignant disease**	**Non-malignant disease**
Argentina	<100	<100	100-500	<100
Australia	501-999	100-500	>=1000	501-999
Austria	NA	NA	<100	<100
Belgium	<100	<100	>=1000	100-500
Brazil	501-999	501-999	>=1000	>=1000
Canada	100-500	100-500	>=1000	>=1000
Chile	<100	<100	<100	<100
Colombia	<100	<100	100-500	<100
Czech Republic	<100	<100	100-500	<100
Denmark	100-500	<100	>=1000	100-500
Ecuador	<100	<100	<100	<100
Egypt	NA	NA	<100	<100
Finland	NA	NA	100-500	<100
France	<100	<100	501-999	100-500
Germany	100-500	<100	>=1000	100-500
Greece	NA	NA	<100	<100
Hong Kong	<100	<100	<100	<100
India	100-500	>=1000	>=1000	>=1000
Iran	<100	100-500	100-500	100-500
Ireland	<100	<100	100-500	<100
Israel	<100	<100	501-999	100-500
Italy	NA	NA	100-500	<100
Kuwait	NA	NA	NA	<100
Mexico	<100	<100	501-999	100-500
Netherlands	<100	<100	501-999	100-500
New Zealand	100-500	100-500	501-999	100-500
Nigeria	NA	NA	NA	<100

	CRF		TED	
Country	**Malignant disease**	**Non-malignant disease**	**Malignant disease**	**Non-malignant disease**
Norway	<100	<100	<100	<100
Pakistan	<100	501-999	100-500	100-500
Paraguay	NA	NA	<100	<100
Peru	<100	<100	<100	<100
Poland	<100	<100	100-500	<100
Portugal	NA	NA	<100	<100
Russia	NA	NA	<100	<100
Saudi Arabia	100-500	501-999	>=1000	>=1000
Singapore	100-500	<100	501-999	<100
Slovak Republic	NA	NA	<100	<100
South Africa	<100	<100	<100	<100
South Korea	501-999	100-500	>=1000	100-500
Spain	<100	<100	100-500	<100
Sweden	<100	<100	501-999	100-500
Switzerland	<100	<100	501-999	<100
Taiwan	<100	<100	<100	<100
Thailand	NA	NA	<100	NA
Turkey	<100	<100	100-500	<100
USA	>=1000	>=1000	>=1000	>=1000
United Kingdom	100-500	100-500	>=1000	100-500
Uruguay	<100	<100	<100	<100
Venezuela	<100	NA	<100	<100

Table 12. Number of patients who received a first autologous transplant registered to the CIBMTR between 2008 and 2023 by country

	CRF		TED	
Country	**Malignant disease**	**Non-malignant disease**	**Malignant disease**	**Non-malignant disease**
Argentina	100-500	NA	>=1000	<100
Australia	<100	<100	501-999	<100
Austria	NA	NA	<100	NA
Belgium	NA	NA	100-500	<100
Brazil	100-500	<100	>=1000	<100
Canada	100-500	<100	>=1000	100-500
Colombia	<100	NA	100-500	<100
Czech Republic	<100	NA	100-500	<100
Ecuador	<100	NA	<100	NA
Egypt	NA	NA	<100	NA
Finland	NA	NA	100-500	NA
France	NA	NA	501-999	<100
Germany	<100	NA	501-999	<100
Greece	NA	NA	<100	NA
Hong Kong	NA	NA	<100	NA
India	100-500	<100	>=1000	<100
Iran	<100	<100	100-500	NA
Israel	<100	<100	501-999	<100
Italy	NA	NA	501-999	<100
Kuwait	NA	NA	<100	NA
Mexico	<100	NA	100-500	501-999
Netherlands	NA	NA	100-500	<100
New Zealand	<100	NA	100-500	NA
Pakistan	<100	<100	100-500	<100

	CRF		TED	
Country	**Malignant disease**	**Non-malignant disease**	**Malignant disease**	**Non-malignant disease**
Paraguay	NA	NA	<100	NA
Peru	<100	NA	<100	<100
Poland	NA	NA	100-500	<100
Portugal	NA	NA	<100	NA
Russia	NA	NA	100-500	<100
Saudi Arabia	<100	NA	>=1000	<100
Singapore	100-500	<100	501-999	<100
Slovak Republic	NA	NA	<100	NA
South Africa	<100	NA	<100	NA
South Korea	100-500	NA	>=1000	<100
Spain	<100	NA	100-500	<100
Switzerland	NA	NA	100-500	<100
Taiwan	NA	NA	<100	NA
Thailand	NA	NA	<100	NA
Turkey	<100	NA	501-999	NA
USA	>=1000	100-500	>=1000	100-500
United Kingdom	NA	NA	100-500	NA
Uruguay	<100	NA	501-999	NA
Venezuela	<100	NA	100-500	NA

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A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

TO: Donor and Recipient Health Services Working Committee Members

FROM: Heather Stefanski, MD, PhD; Scientific Director for the Donor and Recipient Health Services Working Committee

RE: 2024-2025 Studies in Progress Summary

DS20-01 Acute toxicities of bone marrow donation in donors with sickle cell trait (N Farhadfar/ J Wingard). This study primarily aims to evaluate the impact of present of sickle cell trait on per-donation toxicity experienced by unrelated bone marrow donors. Secondary aims are to evaluate the impact of sickle cell rail on time to complete recovery from donation-associated symptoms and to compare the BM collected yield between unrelated donors with and without sickle cell trait.

Status: **Manuscript Preparation**. **We aim to submit the manuscript by April 2025.**

DRS18-03 Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia. (L Winestone/ R Aplenc/ K Getz). The primary aims of this study are 1.) Compare the prevalence of hematopoietic cell transplant by donor source between racial/ethnic minorities compared to the White non-Hispanic populations among a cohort of relapsed pediatric acute leukemia patients. 2.) Among those who received HCT, compare resource utilization by race and ethnicity. 3.) Among those who received HCT, evaluate the role of pre-transplant organ failure in mediating the relationship between race and ethnicity and post-transplant resource utilization.

Status: **Datafile Preparation**. **We aim to submit the manuscript by December 2025.**

HS19-01 Factors Associated with Clinical Trial Participation among HCT Patients: A CIBMTR Analysis (T F. Gray/ A El-Jawahri). The primary aims of this study are: 1) To describe rates of clinical trial participation based on HCT type; 2) To explore factors that are associated with clinical trial participation in patients with undergoing HCT; 3) To assess the impact of clinical trial participation on overall survival (OS) and non-relapse mortality (NRM) in autologous and allogeneic HCT recipients.

Status: **Analysis. We aim to submit the manuscript by July 2025.**

HS19-03 Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: a multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group (N Hamerschlak/ M Kerbauy/ A Riberio). The primary aims of this study are: 1.) Determine if the 1 year overall survival after HCT plus post-Cy from Haploidentical related donor (Haplo-HCT) for acute leukemia and MDS is not inferior compared to matched related or unrelated allogeneic HCT donor with 10/10 and 9/10 compatibility. 2.) Compare the

incidences of acute gvhd (grades II-IV, and III-IV), chronic gvhd (NIH moderate to severe), non-relapse related mortality, disease relapse (only Arm 1), time to neutrophil engraftment, time to platelet engraftment and event-free survival between recipients of Haplo plus Post-CY and of matched related and unrelated donors.

Status: **Submitted.**

DRS20-01 Resource Intensity of End-of-Life Care in Children After Hematopoietic Stem Cell Transplant for Acute Leukemia: Rates and Disparities (E E Johnston/ C W. Elgarten/ L Winestone/ R Aplenc/ K Getz/ V Huang/ Y Li). The primary aims of this study are: 1) Describe the resource utilization during the 30 days before death among children who received a HSCT for a hematologic malignancy and then died within 5 years at the same PHIS hospital. 2) Determine the prevalence of patients with a resource intense phenotype in the last 30 days of life among children who received a HSCT for a hematologic malignancy and then died within 5 years at the same PHIS hospital. 3) Determine the clinical and sociodemographic characteristics associated with a resource intense phenotype among children who received a HSCT for a hematologic malignancy and then died within 5 years at the same PHIS hospital.

Status: **Datafile Preparation. We aim to submit the manuscript by December 2025.**

DRS22-01 Health care utilization and costs of haploidentical allogeneic stem cell transplants in a contemporary cohort of pediatric patients with acute leukemia and myelodysplastic syndrome. (H Rangarajan/ P Satwani). The primary aims of this study are 1.) Determine the cost and HCU associated with HaploHCT for pediatric patients (≤ 21 years) patients with acute leukemia (ALL,AML) and MDS from 2010-2020. 2.) To compare the costs and health care utilization of HaploHCT with that of MSD, MUD, MMURD, and UCB transplants.

Status: **Protocol Received. We aim to submit the manuscript by December 2026.**

DRS23-01 Community health status and long-term outcomes in 1-year survivors of autologous and allogeneic hematopoietic cell transplantation in the United States. (B Hamilton/ S Hong). The primary aims of this study are 1.) Investigate the association between community health status based on patient's residence (Patient Community Score [PCS] from CHRR data associated with patient zip code/county) and both continuous and 5-year overall survival (OS) in long-term survivors (>1 year) of allogeneic and autologous HCT. 2.) Investigate the association between PCS and the following long-term transplant outcomes: non-relapse mortality (NRM), relapse, and chronic graft-versus-host disease (cGVHD). 3.) Investigate the association between PCS and other late effects (5.2.6-5.2.14) of transplant. 4.) Identify associations between long-term outcomes (2.2)/ late effects (2.3) and the four PCS subcategories: physical environment, social and economic factors, clinical care, and health behaviors.

Status: **Protocol Received. We aim to submit the manuscript by December 2026.**

DRS24-01 Outcomes for Medicaid beneficiaries following allogeneic hematopoietic cell transplantation: Exploring the impact of variable medicaid eligibility criteria. (P DeMartino/ N Majhail). The primary aims of this study are 1.) Assess the association between insurance type and HCT outcomes for adult recipients from states with expansion of Medicaid compared to non-expansion states. 2.) Assess the association between insurance type and HCT outcomes for pediatric recipients from states with higher versus lower Medicaid income eligibility thresholds.

Status: **Protocol Received. We aim to submit the manuscript by December 2026.**

Field	Response
Proposal Number	2410-133-RANGARAJAN
Proposal Title	Impact of Race and Ethnicity on Incidence of Primary Graft Failure in Allogeneic Hematopoietic Stem Cell Transplant Recipients
Key Words	Primary Graft Failure, Trends, Allogeneic Transplant recipients, Disparities
Principal Investigator #1: - First and last name, degree(s)	Hemalatha Rangarajan
Principal Investigator #1: - Email address	hemalatha.rangarajan@nationwidechildrens.org
Principal Investigator #1: - Institution name	Nationwide Children's Hospital
Principal Investigator #1: - Academic rank	Clinical Associate Professor of Pediatrics
Junior investigator status (defined as ≤5 years from fellowship)	No
Do you identify as an underrepresented/minority?	Yes
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Mohamed A Kharfan Dabaja MD MBA
Principal Investigator #2 (If applicable): - Email address:)	KharfanDabaja.Mohamed@mayo.edu
Principal Investigator #2 (If applicable): - Institution name:	Mayo Clinic, Jacksonville, Florida
Principal Investigator #2 (If applicable): - Academic rank:	Professor of Medicine
Junior investigator status (defined as ≤5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
We encourage a maximum of two Principal Investigators per study. If more than one author is listed, please indicate who will be identified as the corresponding PI below:	Hemalatha Rangarajan is the corresponding PI. Please note there is a 3rd PI: Dr. Prakash Satwani, . Professor of Pediatrics, Columbia University Medical Center, ps2087@cumc.columbia.edu
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	Hemalatha Rangarajan MD The following proposals that I have submitted have been accepted and are at varying stages of development. I am one of the co-principal investigators on all these protocols. 1. IN20-01: Incidence, Risk Factors, and Outcomes of Infections post CD19 CAR T therapies. February 2020. Data analysis is ongoing. 2. CT20-02: Resource utilization in patients receiving CAR-T Therapy. February 2020. Data analysis ongoing 3. PC19-03: Outcomes of allogeneic hematopoietic cell transplantation in pediatric patients with AML and CNS involvement. February 2019. Manuscript writing stage. 4. NM22-01:Outcomes after second or greater allogeneic stem cell transplants in patients with severe aplastic anemia: A contemporary analysis: Protocol development 5. RRT: 2110-80:Incidence, risk factors and outcomes of acute cardiac complications after post-transplant cyclophosphamide based GVHD prophylaxis; A Retrospective Analysis from CIBMTR Database: Protocol Development

Field	Response
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Donor and Recipient Health Services
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	Has there been a disproportionate increase in incidence of primary graft failure (PGF) post allogeneic HCT in certain racial/ethnic minorities over time?
RESEARCH HYPOTHESIS:	There has been an increase in the use of mismatched unrelated donors and haploidentical donors in the recent era. This has led to improved access to allogeneic HCT in historically, underserved populations (Hispanics and Blacks). However these “alternative mismatched donor sources” can be associated with an increased risk of GF. We therefore, hypothesize that there has been an increased incidence of primary graft failure (PGF) in racial and ethnic minority HCT recipients over time.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Objective To assess the impact of race and ethnicity on trends of PGF over last 3 decades following allogeneic hematopoietic stem cell transplant performed at CIBMTR reporting centers. Secondary objectives We will compare the following outcomes stratified by race and ethnicity Trends in 1, 3 and 5 -year OS in patients who had PGF Trends in 100-day and 1-year TRM in patients with PGF Determine the proportion of patients with PGF who underwent secondary therapies and the trends of individual therapies categorized as follows a. CD34 Stem cell boost b. Second allogeneic hematopoietic cell transplants c. Donor lymphocyte infusion Exploratory objective: Trends in secondary graft failure in our study defined cohort

Field	Response
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Significant advances in hematopoietic cell transplantation (HCT) have transformed the field over the past four decades. These include improvements in supportive care, the adoption of less toxic conditioning regimens (such as reduced-intensity conditioning (RIC) and non-myeloablative (NMA) approaches), and the broader use of alternative donor sources, including umbilical cord blood, matched unrelated donors, mismatched unrelated donors, and haploidentical (mismatched related) donors. Additionally, newer graft-versus-host disease (GVHD) prevention strategies, such as post-transplant cyclophosphamide (PTCY), abatacept, TCR αβ depletion, and CD45RA selection, have further enhanced transplant outcomes. Advances in supportive care, such as the use of Letermovir and cytotoxic T lymphocytes, have also played a critical role in improving patient outcomes. These innovations have expanded access to HCT for historically underserved populations, including Black and Hispanic among others. However, it remains unclear whether these advancements have influenced the incidence of PGF in HCT recipients over time, or whether underserved populations have been disproportionately affected by this complication. Accordingly, we propose analyzing the incidence of PGF following HCT performed at U.S. transplant centers from 1990 to the 2020s, stratified by race and ethnicity. In addition, we aim to assess trends in the utilization of secondary therapies in patients who experience PGF. It is possible that the increased use of mismatched unrelated (MMUD) and haploidentical donors has heightened the risk of PGF in minority populations over time. This study will provide valuable insights into whether transplant physicians are increasingly considering secondary interventions, likely facilitated by improvements in supportive care practices. Moreover, our work is timely, given emerging data on the risk of malignancy following graft failure or mixed chimerism status in non-malignant diseases, particularly in sickle cell disease (SCD) [1]. By offering a comprehensive analysis of temporal trends in PGF and identifying which groups—such as pediatric patients with non-malignant diseases (NMDs)—are most affected, this study will contribute important knowledge to the HCT community. Our findings could help identify healthcare disparities, guide resource allocation, and pave the way for future prospective studies to improve outcomes for all HCT recipients.

SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	Allogeneic hematopoietic cell transplantation (HCT) is an established curative therapy for a range of both malignant and non-malignant diseases (NMDs). Over recent decades, the HCT landscape has evolved, marked by expanded indications, the use of alternative donors, advancements in graft-versus-host disease (GVHD) prophylaxis, and improved supportive care strategies. A study by the Center for International Blood and Marrow Transplant Research (CIBMTR) [2] highlighted significant trends from the 1980s to the 2000s, including an increase in unrelated donor HCTs, greater utilization of cord blood transplants in pediatric settings, and a rise in haploidentical transplants, which grew from 6% in 2008 to 11% by 2015. Notably, according to a more recent CIBMTR activity report, haploidentical transplants have since surpassed both cord blood and matched sibling donor transplants in frequency [3]. In parallel with the rising number of HCTs performed annually [2, 4], improvements in transplant-related outcomes have been observed. For example, D'Souza et al. [2] reported a positive trend in 100-day survival rates between 2000 and 2015, with autologous transplant survival increasing from 95% to 98%, and allogeneic transplant survival improving from 80% to 90%. A more recent study by Auletta et al [5]. further corroborated these findings. This study demonstrated that, from 2009 to 2020, a more ethnically diverse patient population received mismatched unrelated donor, haploidentical donor, and cord blood transplants compared to matched related or unrelated donor HCT. Importantly, survival outcomes in the recent era have shown improvement, with no significant impact of patient race or ethnicity on overall survival. Disease relapse remained the leading cause of mortality across all racial and ethnic groups. Neither report, however, shed light on trends related to primary graft failure (PGF). PGF occurs in approximately 3% of autologous transplants and 2-20% of allogeneic transplants [6]. However, no comprehensive studies have evaluated global trends in PGF incidence or the influence of race and ethnicity on its occurrence. This is particularly relevant as emerging evidence suggests an elevated risk of PGF with the use of haploidentical donors. For instance, a recent CIBMTR study involving 938 adults who underwent haploidentical HCT with post-transplant cyclophosphamide [7] found PGF rates as high as 14% when grafts were from parents, compared to 6-7% when grafts were from siblings or offspring. Similarly, PGF occurred in 30% of pediatric patients with NMDs following TCRαβ/CD19-depleted haploidentical transplants [8]. In a study conducted at our center [9] involving pediatric allogeneic hematopoietic cell transplantation (alloHCT) recipients (n=290) with both malignant and non-malignant disorders, we examined outcomes, risk factors,

Field	Response
	healthcare utilization, and costs among patients with primary graft failure (PGF) across two time periods: Period 1 (2005–2010) and Period 2 (2011–2018). PGF occurred in 10.4% (n=30) of the overall cohort. The overall survival rate for the entire HCT group was 69%, while the survival rate among patients with PGF was 50%. Notably, survival outcomes for PGF patients significantly improved between the two periods, with survival rates increasing from 20% (3/15 survivors) in Period 1 to 80% (12/15 survivors) in Period 2 (p=0.001). Due to the relatively small sample size, we were unable to assess the impact of race and ethnicity on PGF incidence. Physicians managing patients with PGF may opt for secondary interventions such as CD34+ stem cell boosts, donor lymphocyte infusions, or second transplants. Through our proposed study, we also aim to capture trends in the use of secondary procedures following PGF in alloHCT recipients. We hypothesize that secondary interventions have become more frequent in recent years, likely due to advancements in supportive care, graft manipulation techniques, and improved viral prevention strategies. In summary, several changes in transplant practices could influence the incidence of graft failure. Of particular relevance are the increasing number of transplants for non-malignant diseases, the growing use of haploidentical donors, ex vivo T-cell depletion for GVHD prophylaxis, and the use of ex vivo expanded cord blood products [10], all of which may impact PGF rates. A better understanding of these trends could help identify areas of healthcare and resource disparities, leading to future prospective studies. Our findings may also shed light on whether there is an increasing incidence of PGF over time, particularly among racial and ethnic minority patients who are more likely to receive grafts from mismatched donors (e.g., cord blood, haploidentical, or unrelated donors). This could help predict future trends and inform resource allocation to improve patient outcomes.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion Criteria: • Recipients of allogeneic HCT from 1990 to 2022 • Any age • Both malignant and non-malignant disorders • Any donor type: matched sibling, vs matched unrelated vs mismatched unrelated vs haploidentical) • Any graft type: Bone Marrow, Cord blood, Peripheral blood stem cells Exclusion criteria • Non consented patients. • Patients with incomplete forms • Patients who received more than 1 graft source
Does this study include pediatric patients?	Yes

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Outline any supplementary data required.	Patient related. Age at transplant: continuous and </=; 18 years and ≥ 18 years Race/ethnicity: Non-Hispanic white vs. non-Hispanic black vs. Hispanic vs. Asian Sex: male vs. female HCT CI index 0-2, &>/=; 2 CMV serologic status CD34 cell dose Detectable donor specific antibodies (yes vs. no vs. unknown) Disease will be classified into malignant and non- malignant disease: Malignant diseases will be classified as: ALL, AML, MDS, CML, CLL, Myelofibrosis, other MPN, HD, NHL Nonmalignant diseases (likely mostly alloHCTs) will be broadly categorized as follows: Hemoglobinopathies (Sickle cell and thalassemia) Bone marrow failure syndromes (FA, DKC, SD, DBA, other constitutional anemia, severe congenital neutropenia, aplastic anemia) Metabolic disorders (ALD, MLD, Hurlers syndrome e.tc.). Inborn Errors of Immunity Donor related Donor gender Donor age CMV serologic status Treatment-related Conditioning regimen intensity: MAC vs. RIC/NMA according to CIBMTR definition [11] Graft source: bone marrow vs. peripheral blood vs. cord blood Donor: HLA-identical sibling vs 8/8 unrelated donor vs 7/8 mismatched unrelated vs Umbilical cord (6/6 vs others), vs haploidentical donor (≥ 2 antigen mismatched and related) GVHD prophylaxis: No GVHD prophylaxis vs Ex-vivo T-cell depletion vs Tacrolimus/Cyclosporine ± others (not PTCY) vs PTCY based± others vs others Year of alloHCT:, 1990-2000, 2001-2010, 2011-2022 Graft failure details Interval between HCT and Graft failure Did patient proceed to second therapy if Y, specify CD34 boost/DLI/2nd Transplant Interval between Graft failure and second therapy Follow up Last follow up in months Alive or Dead at Last follow up Cause of Death
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A desc	Hematopoietic Cell Transplantation (HCT)
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	Not applicable
SAMPLE REQUIREMENTS: If the study requires biologic samples from the CIBMTR Repository, the proposal should also include: 1) A detailed description of the proposed testing methodology and sample requirements; 2) A summary o	Not applicable
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	Not applicable

Field	Response
REFERENCES:	Not applicable
CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	REFERENCES: 1. Ghannam, J.Y., et al., Baseline TP53 mutations in adults with SCD developing myeloid malignancy following hematopoietic cell transplantation. Blood, 2020. 135(14): p. 1185-1188. 2. D'Souza, A., et al., Current Use of and Trends in Hematopoietic Cell Transplantation in the United States. Biol Blood Marrow Transplant, 2020. 26(8): p. e177-e182. 3. Bolon YT, Atshan R, Allbee-Johnson M, Estrada-Merly N, Lee SJ. Current use and outcome of hematopoietic stem cell transplantation: CIBMTR summary slides, 2022. 4. Khandelwal, P., et al., Hematopoietic Stem Cell Transplantation Activity in Pediatric Cancer between 2008 and 2014 in the United States: A Center for International Blood and Marrow Transplant Research Report. Biol Blood Marrow Transplant, 2017. 23(8): p. 1342-1349. 5. Auletta JJ, Kou J, Chen M, Bolon YT, Broglie L, Bupp C, Christianson D, Cusatis RN, Devine SM, Eapen M, Hamadani M, Hengen M, Lee SJ, Moskop A, Page KM, Pasquini MC, Perez WS, Phelan R, Riches ML, Rizzo JD, Saber W, Spellman SR, Stefanski HE, Steinert P, Tuschl E, Yusuf R, Zhang MJ, Shaw BE. Real-World Data Showing Trends and Outcomes by Race and Ethnicity in Allogeneic Hematopoietic Cell Transplantation: A Report from the Center for International Blood and Marrow Transplant Research. Transplant Cell Ther. 2023 Jun;29(6):346.e1-346.e10. PMID: 36924931; PMCID: PMC10239334. 6. Hutt, D., Engraftment, Graft Failure, and Rejection, in The European Blood and Marrow Transplantation Textbook for Nurses: Under the Auspices of EBMT, M. Kenyon and A. Babic, Editors. 2018: Cham (CH). p. 259-270. 7. McCurdy, S.R., et al., Effect of donor characteristics on haploidentical transplantation with post transplantation cyclophosphamide. Blood Adv, 2018. 2(3): p. 299-307. 8. Merli, P., et al., TCRαβ/CD19 depleted HSCT from an HLA-haploidentical relative to treat children with different nonmalignant disorders. Blood Adv, 2022. 6(1): p. 281-292. 9. Wobma, H., et al., Risk Factors, Clinical Outcomes, and Cost-of-Care Related to Graft Failure in Pediatric Allogeneic Hematopoietic Cell Transplant Recipients. Biology of Blood and Marrow Transplantation, 2020. 26(7): p. 1318-1325. 10. Parikh, S., et al., Allogeneic stem cell transplantation with omidubicel in sickle cell disease. Blood Adv, 2021. 5(3): p. 843-852.
If yes, provide detail on the nature of employment, name of organization, role, entity, ownership, type of financial transaction or legal proceeding and whether renumeration is >$5000 annually.	No, I do not have any conflicts of interest pertinent to this proposal

Table 1. Characteristics of allogeneic HCT for recipients that only received one graft source between 2008 to 2022

Characteristic	N (%)
No. of patients	159665
No. of centers	441
Recipient age at collection, grouped - no. (%)	
Median (min-max)	47.8 (0.0-87.8)
0-10	20442 (12.8)
11-17	10200 (6.4)
18-29	18582 (11.6)
30-39	15351 (9.6)
40-49	20920 (13.1)
50-59	32714 (20.5)
60+	41456 (26.0)
Recipient sex - no. (%)	
Male	93464 (58.5)
Female	66201 (41.5)
Recipient race - no. (%)	
White	108922 (68.2)
Black or African American	10068 (6.3)
Asian	11570 (7.2)
Native Hawaiian or other Pacific Islander	608 (0.4)
American Indian or Alaska Native	799 (0.5)
More than one race	1510 (0.9)
Not reported	26188 (16.4)
Recipient ethnicity - no. (%)	
Hispanic or Latino	16237 (10.2)
Non-Hispanic or non-Latino	104786 (65.6)
Non-resident of the U.S.	36541 (22.9)
Not reported	2101 (1.3)
Disease - no. (%)	
AML or ANLL	56638 (35.5)
ALL	24509 (15.4)
Other Leukemia	3763 (2.4)
CML	4866 (3.0)
MDS	21531 (13.5)
Acute Leukemia	2124 (1.3)
NHL	12266 (7.7)
HD	2840 (1.8)
Plasma cell disorder	3022 (1.9)

Characteristic	N (%)
Solid Tumor	178 (0.1)
Aplastic anemia	7238 (4.5)
Inherited abnormal of erythrocyte differ.	54 (0.0)
Inherited bone marrow failure syndromes	1945 (1.2)
Hemoglobinopathies	5038 (3.2)
Paroxysmal nocturnal hemoglobinuria (PNH)	327 (0.2)
Immune Deficiencies (ID)	4270 (2.7)
Inherited platelet abnormalities	155 (0.1)
Inherited disorders of metabolism	1412 (0.9)
Histiocytic disorder	1402 (0.9)
Autoimmune Disease	130 (0.1)
Other non-malignant disorder	5 (0.0)
Other disease	179 (0.1)
Tolerance induction associated with solid organ transplant	7 (0.0)
Recessive dystrophic epidermolysis Bullosa	1 (0.0)
Myeloproliferative neoplasms	5765 (3.6)
Graft type - no. (%)	
Bone marrow	35846 (22.5)
Peripheral blood stem cells	113644 (71.2)
Umbilical cord blood	10175 (6.4)
Donor type - no. (%)	
HLA-identical sibling	49269 (30.9)
Twin	23 (0.0)
Other related	24370 (15.3)
8/8 matched URD	48841 (30.6)
7/8 mismatched URD	9626 (6.0)
<= 6/8 mismatched URD;	545 (0.3)
Multi-donor	1275 (0.8)
Unrelated (matching TBD)	14782 (9.3)
Cord blood	10175 (6.4)
Not reported	759 (0.5)
Conditioning intensity- center reported - no. (%)	
MAC	86014 (53.9)
NST/RIC	48792 (30.6)
Not reported	24859 (15.6)
GVHD prophylaxis - no. (%)	
None	2813 (1.8)
Ex-vivo T-cell depletion	2045 (1.3)
CD34 selection	2414 (1.5)
PtCy	26954 (16.9)
TAC based	69995 (43.8)
CSA based	51676 (32.4)

Characteristic	N (%)
Other	3194 (2.0)
Not reported	574 (0.4)
Year of transplant - no. (%)	
2008	8500 (5.3)
2009	9648 (6.0)
2010	10194 (6.4)
2011	10453 (6.5)
2012	10693 (6.7)
2013	10737 (6.7)
2014	10276 (6.4)
2015	10200 (6.4)
2016	10490 (6.6)
2017	11472 (7.2)
2018	11283 (7.1)
2019	11749 (7.4)
2020	10645 (6.7)
2021	11403 (7.1)
2022	11922 (7.5)
Any timepoint the 2100 form was completed - no. (%)	
No	2927 (1.8)
Yes	43951 (27.5)
N/A- TED level patient	112787 (70.6)
Any timepoint the 2450 form was completed - no. (%)	
No	7325 (4.6)
Yes	109048 (68.3)
N/A- CRF level patient	43292 (27.1)
Follow-up - median (range)	57.7 (0.0-2211.5)

Field	Response
Proposal Number	2409-02-SU/2410-57-SALIT
Proposal Title	Do housing distance requirements imposed by transplant centers on hematopoietic cell transplantation patients affect non-relapse mortality and other clinical outcomes
Key Words	stem cell transplant, housing, financial toxicity, non-relapse mortality,
Principal Investigator #1: - First and last name, degree(s)	Rachel Salit
Principal Investigator #1: - Email address	rsalit@fredhutch.org
Principal Investigator #1: - Institution name	Fred Hutchinson Cancer Center
Principal Investigator #1: - Academic rank	Associate Professor
Junior investigator status (defined as ≤5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Chris Su
Principal Investigator #2 (If applicable): - Email address:)	chrissu@uw.edu
Principal Investigator #2 (If applicable): - Institution name:	University of Washington Medical Center
Principal Investigator #2 (If applicable): - Academic rank:	Assistant Professor
Junior investigator status (defined as ≤5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	Yes
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Donor and Recipient Health Services
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	Are housing distance requirements imposed by transplant centers on allogeneic hematopoietic cell transplant patients associated with early non-relapse mortality?
RESEARCH HYPOTHESIS:	There is no association between housing distance requirements for patients undergoing allogeneic hematopoietic cell transplantation and non-relapse mortality in the first 100 days post-transplant.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary outcome: Non-relapse mortality in first 100 days (Form 2100 Question 2 and form 2900) Secondary outcomes: 1. Unplanned admission (Form 2100, Questions 385, 386) 2.Hospitalization days (Form 2100, Question 387, 388) 3. Acute graft versus host disease (Form 2100 Questions 95-11) 4. Incidence of infection (Form 2100, Questions 228-235) 5. Organ Impairment/Disorder (liver, renal) (Form 2100, Questions 273-278, 292-305) 6. Overall survival (Form 2100, Question 2 and form 2900) 7. Disease relapse, yes/no (collected via disease specific forms e.g., 2110 for AML)
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	All transplant centers have housing requirements for patients undergoing hematopoietic cell transplantation in the first 100 days to ensure timely provision of follow-up care should complications (e.g. GVHD, infection, organ dysfunction) develop. However, it is unclear whether these housing requirements are effective in reducing non-relapse mortality, and hospital readmissions, or improving clinical outcomes. Unfortunately, many patients are not eligible or suffer considerable financial hardship because of a primary residence that lies outside of housing radius and the inability of patients to afford temporary residence closer to the transplant center. This clearly presents an access-to-care barrier for these patients that is externally imposed by the transplant center and introduces inequity in the provision of life-saving therapy for patients with advanced hematologic malignancies. Therefore, our proposed study will be the first to rigorously examine the construct of these early housing requirements imposed by transplant centers on patients receiving allogeneic hematopoietic cell transplantation, within the first 100 days after transplant. Given that there have been no studies examining outcomes from these early housing requirements and that different centers have slightly different residency requirements, we will be able to design a study that exploits these differences for analysis.

Field	Response
SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	Based on our preliminary Pubmed literature search, we have identified a few key studies regarding the impact of geography on transplant outcomes (for example, PMID 21906576, 27013013). However, these single center studies examined the distance of the patients' primary residence to the transplant center on 1 and 2 year outcomes rather than in the acute period directly following transplant. We believe that our research proposal will provide a novel lens for transplant centers to re-examine their policies regarding their patient residency requirements in the immediate post-transplant period. Ultimately, this is likely to mitigate significant patient-level financial toxicity that is well-known to result following transplant and cellular therapies. Previous studies (such as patient-reported cost diaries collected as part of BMT CTN 1102 – presented at ASH 2023 and currently under journal review) have shown that accommodations and travel costs are one of the largest contributors to financial toxicity among transplant patients. These requirements also have implications for patients receiving cellular therapy (e.g., CAR-T), given similar restrictions on housing location, and could form the basis of another future study examining the same issue among patients receiving cellular therapy.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Patients > 18 years Allogeneic hematopoietic cell transplantation Years 2014-2024 Donor Source: HLA-matched related, HLA-matched unrelated, Mismatched HLA Unrelated, Haplo-identical or cord blood Conditioning to include myeloablative or reduced intensity Inpatient or outpatient transplant planned Diseases include patients with hematologic malignancy or aplastic anemia Patient has at least one form 2100 collected, and other forms such as 2900 for mortality data and disease-specific forms to evaluate for relapse. Exclusion: Second transplant patients Non-malignant conditions other than aplastic anemia
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	Adult transplant centers have different requirements and housing options than pediatric transplant patients.

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Outline any supplementary data required.	Data should be able to be compiled from the following forms: Form 2000-recipient baseline data Form 2100-post transplant data Form 2900-recipient death data Patient variables: Age Sex Race Ethnicity Health insurance Y/N Health insurance type Combined household income Patient's address during the first 100 days post HCT Disease: Disease Type Remission Status Transplant related variables: Year of transplant Conditioning intensity: ie myeloablative (MAC), reduced intensity (RIC), or nonmyeloablative (NMA) Conditioning regimen Donor source: (ie) HLA matched sibling, HLA-matched and mismatched unrelated donor (URD), umbilical cord blood (UCB), haploidentical Graft source: bone marrow (BM), peripheral blood (PBSCT), UCB GVHD prophylaxis regimen Inpatient or outpatient transplant planned Transplant center housing distance requirement
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A desc	Hematopoietic Cell Transplantation (HCT)
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	Not applicable
SAMPLE REQUIREMENTS: If the study requires biologic samples from the CIBMTR Repository, the proposal should also include: 1) A detailed description of the proposed testing methodology and sample requirements; 2) A summary o	Not applicable
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	not applicable
REFERENCES:	Specific transplant center housing distance requirements if unknown by CIBMTR will be collected via hospital websites or direct inquiry to providers at the different transplant centers.

Field	Response
CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	Abou-Nassar KE, Kim HT, Blossom J, Ho VT, Soiffer RJ, Cutler CS, Alyea EP, Koreth J, Antin JH, Armand P. The impact of geographic proximity to transplant center on outcomes after allogeneic hematopoietic stem cell transplantation. Biol Blood Marrow Transplant. 2012 May;18(5):708-15. doi: 10.1016/j.bbmt.2011.08.022. Epub 2011 Sep 8. PMID: 21906576; PMCID: PMC3245811. Khera N, Gooley T, Flowers MED, Sandmaier BM, Loberiza F, Lee SJ, Appelbaum F. Association of Distance from Transplantation Center and Place of Residence on Outcomes after Allogeneic Hematopoietic Cell Transplantation. Biol Blood Marrow Transplant. 2016 Jul;22(7):1319-1323. doi: 10.1016/j.bbmt.2016.03.019. Epub 2016 Mar 22. PMID: 27013013; PMCID: PMC4905774.
If yes, provide detail on the nature of employment, name of organization, role, entity, ownership, type of financial transaction or legal proceeding and whether renumeration is >$5000 annually.	No, I do not have any conflicts of interest pertinent to this proposal

Table 1. Characteristics of allogeneic transplants amongst adult recipient's first HCT on the CRF track for non-malignant conditions or aplastic anemia between 2014 to 2022 with at least one follow-up form completed.

Characteristic	N (%)
No. of patients	13551
No. of centers	231
Recipient age at collection, grouped - no. (%)	
Median (min-max)	58.1 (18.0-87.8)
18-29	1640 (12.1)
30-39	1214 (9.0)
40-49	1674 (12.4)
50-59	2937 (21.7)
60+	6086 (44.9)
Recipient sex - no. (%)	
Male	8008 (59.1)
Female	5543 (40.9)
Recipient race - no. (%)	
White	10410 (76.8)
Black or African American	1160 (8.6)
Asian	975 (7.2)
Native Hawaiian or other Pacific Islander	87 (0.6)
American Indian or Alaska Native	77 (0.6)
More than one race	105 (0.8)
Not reported	737 (5.4)
Recipient ethnicity - no. (%)	
Hispanic or Latino	1236 (9.1)
Non-Hispanic or non-Latino	10772 (79.5)
Non-resident of the U.S.	1291 (9.5)
Not reported	252 (1.9)
Disease - no. (%)	
AML or ANLL	4397 (32.4)
ALL	1674 (12.4)
Other Leukemia	253 (1.9)
CML	312 (2.3)
MDS	3181 (23.5)
Acute Leukemia	132 (1.0)
NHL	673 (5.0)
HD	147 (1.1)
Plasma cell disorder	31 (0.2)
Solid Tumor	4 (0.0)
Aplastic anemia	795 (5.9)
Myeloproliferative neoplasms	1952 (14.4)
Graft type - no. (%)	

Characteristic	N (%)
Bone marrow	2195 (16.2)
Peripheral blood stem cells	10539 (77.8)
Umbilical cord blood	817 (6.0)
Donor type - no. (%)	
HLA-identical sibling	3187 (23.5)
Other related	2478 (18.3)
8/8 matched URD	6000 (44.3)
7/8 mismatched URD	988 (7.3)
<= 6/8 mismatched URD;	60 (0.4)
Multi-donor	21 (0.2)
Cord blood	817 (6.0)
Haploidentical donor - no. (%)	
Yes	2499 (18.4)
No	11052 (81.6)
Conditioning intensity- center reported - no. (%)	
MAC	6680 (49.3)
NST/RIC	6871 (50.7)
GVHD prophylaxis - no. (%)	
None	208 (1.5)
Ex-vivo T-cell depletion	60 (0.4)
CD34 selection	154 (1.1)
PtCy	3492 (25.8)
TAC based	7919 (58.4)
CSA based	1601 (11.8)
Other	113 (0.8)
Not reported	4 (0.0)
Patient zip code available - no. (%)	
No	2947 (21.7)
Yes	10604 (78.3)
Year of transplant - no. (%)	
2014	1861 (13.7)
2015	1836 (13.5)
2016	1822 (13.4)
2017	1759 (13.0)
2018	1661 (12.3)
2019	1590 (11.7)
2020	984 (7.3)
2021	1034 (7.6)
2022	1004 (7.4)
Follow-up - median (range)	60.7 (1.3-127.2)

Field	Response
Proposal Number	2410-03-HAGEN
Proposal Title	Racial and Ethnic Discrepancies in Clinical Outcomes of Autologous Hematopoietic Cell Transplantation in Multiple Myeloma in Non-Hispanic Black and Hispanic Populations as Compared to Caucasian Patients
Key Words	Ethnicity, Race, Autologous Stem Cell transplantation, Multiple Myeloma
Principal Investigator #1: - First and last name, degree(s)	Patrick Hagen, MD
Principal Investigator #1: - Email address	patrick.hagen@lumc.edu
Principal Investigator #1: - Institution name	Loyola University Medical Center
Principal Investigator #1: - Academic rank	Associate Professor
Junior investigator status (defined as ≤5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	-
Principal Investigator #2 (If applicable): - Email address:)	-
Principal Investigator #2 (If applicable): - Institution name:	-
Principal Investigator #2 (If applicable): - Academic rank:	-
Junior investigator status (defined as ≤5 years from fellowship)	-
Do you identify as an underrepresented/minority?	-
We encourage a maximum of two Principal Investigators per study. If more than one author is listed, please indicate who will be identified as the corresponding PI below:	-
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	-
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	N/A
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Plasma Cell Disorders
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
If you have already spoken with a scientific director or working committee chair regarding this study, then please specify who:	-

Field	Response
RESEARCH QUESTION:	Are outcomes inferior for both Black/African American as well as Hispanic/Latino as compared to their Caucasian or non-Hispanic counterparts after undergoing up front Autologous Stem Cell Transplantation (ASCT) for Multiple Myeloma (MM)?
RESEARCH HYPOTHESIS:	When controlled for disease risk factors and induction therapy, both Black/African American as well as Hispanic/Latino patients will show no difference in progression free survival (PFS) as compared to their white or non-Hispanic counterparts after undergoing up front ASCT for newly diagnosed MM (NDMM).
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	1. Primary outcome: progression free survival 2. Secondary outcome: overall survival
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	In 2020, the rate of MM occurring within the non-Hispanic black population was 14.3 per 100,000 compared to 6.6 in the Hispanic population, 6.4 in the non-Hispanic white population and 5.5 in American Indian/Alaskan native populations.1 Despite most of the cases of MM occurring in non-white patients, a pooled analysis of all trials submitted to the FDA for MM therapies from 2006 to 2019 found that only 4% of patients in these trials were black and only 4% were Hispanic.2 Thus, treatment patterns and consequently outcomes are likely to be inferior in non-Hispanic black and Hispanic populations as compare to their Caucasian counterparts. Up front consolidative ASCT remains the standard of care in NDMM patients and remains a Category 1 NCCN recommendation.3 Of note, of the nine studies referenced for this recommendation, only one mentioned race in baseline characteristics of patients, and in that study, the only race addressed was African American. Therefore, there is large population of non-white patients, in particular African American and Hispanics, who would benefit from validated data on whether MM ASCT outcomes vary by race.

SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	Research on the disparities in outcomes of ASCT for MM across different racial groups has yielded inconclusive results. The SEER-Medicare database study showed no difference between whites, blacks and Hispanics for OS and myeloma-specific survival (time to death caused by MM).4 For overall multiple myeloma survival, recent data has shown that with multivariable adjustment, real world PFS is slightly inferior and real world OS is similar between non-Hispanic African Americans and non-Hispanic whites.5 However, novel induction therapy utilization and time from diagnosed to transplant patterns remain concerning for non-white patients and inconsistencies persist in the literature. Specifically, in regards to AA patients, various small scale studies have been done comparing African American (AA) ASCT recipients to white ASCT recipients but consensus on outcomes remains elusive. In an 80-patient study, overall survival (OS) was similar, but AA patients showed greater 26-month event-free survival, while non-AA patients had a higher relapse rate within 6 months.6 Some single center studies have shown superior survival in AA patients.7,8 In one of these studies, the AA population specifically had better post-relapse survival and therefore potentially better response to salvage therapy.8 Other single center studies have shown equal OS between AA and white patients receiving ASCT.9 Larger and registry studies show similarly conflicting outcomes. The Connect MM Registry study found that (11;14) negatively affects OS in AA patients10, a finding that was not supported by 2021 CIBMT report11 that showed neither race (AA v Whites) nor t(11;14) impacted OS while OS was superior among African Americans with t(11;14) compared to Whites with t(11;14). A now outdated CIBMTR report (as it predates novel induction therapy) demonstrated that black and white recipients from 1995-2005 had no difference in 5 year OS, progression free survival, cumulative incidence of disease progression, or non-relapse mortality from time of transplant.12 Therefore, no consensus has been made on outcomes in racial discrepancy of outcomes in ASCT for MM. The final large scale study of note done at the VA showed superiority of OS in AA patients, particularly in the younger population, although in this study only ten percent of patients received transplantation.13 Data in regards to outcomes among the Hispanic population is scarce. A recent systematic review found that 59% of published studies evaluated found worse outcomes for Hispanic Americans with MM when compared to other ethnic groups.14 One recent report did show that non-Hispanic white race has been found to be associated with higher risk of developing secondary primary malignancy after adjusting for sex and year of ASCT.15 Additionally, Pulte et al. found that while there

Field	Response
	is lower Hispanic participation in clinical studies, minority populations respond similarly to newer drugs in MM treatment.16 Therefore, further investigation is needed to explore differences in outcomes between Hispanic and non-Hispanic populations. The reason for continued differences in study outcomes is unclear, but some data suggest it may be due to genetic variation or socioeconomic differences. Genetic variation as a cause was suggested by Peres et al., who found initially superior OS for AA patients but no significant difference after adjusting for clinical features, including tumor mutations and expression.17 Racial discrepancies in the likelihood of receiving HCT have also been observed in previous studies.18 AA patients experience longer delays in receiving ASCT, but Pan et al. showed that these disparities were no longer significant when controlling for functional status, socioeconomic status, and age.19 Some other studies have also indicated that low socioeconomic status is an independent risk factor for poor outcomes in MM treatment.20,21 Yet one Canadian study showed that patients living in more marginalized areas actually had significantly longer survival and marginalization was not associated with inferior survival.22 Further, a study of the National Cancer Database showed that although socioeconomic factors did negatively impact OS with a univariate analysis, when adjusting for biologic risk factors, OS was not significantly different.23 Therefore, there is still debate whether biological risk factors or socioeconomic status are the root cause of differences in outcomes between racial and ethnic differences in outcomes. The question of true differences in outcomes for MM patients treated with ASCT based on race and ethnicity remains unanswered but is vital to address. As the majority of MM patients receiving treatment each year are non-white, conducting an updated study with a large MM dataset to validate outcomes for ASCT in Hispanic and AA patients is warranted and may in the lead to improved access to ASCT in these communities.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion Criteria: 1. Multiple myeloma 2. Time for diagnosis to transplant 1 year or less 3. Cytogenetics tested by FISH available 4. Ethnicity and Race available Exclusion Criteria: 1. Amyloidosis 2. Plasma Cell Leukemia
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	-

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Outline any supplementary data required.	Main effect variable: Comparing outcomes between ethnicity and race namely: 1. Hispanic or Latino vs Not Hispanic or Latino 2. White v Black or African American Patient related variables: 1. Age <50 vs. 50-64 vs. >65 2. Gender: Male vs Female 3. Zipcode 4. Marital status 5. Type of health insurance 6. Combined household gross annual income Diagnostics Studies 7. MM subtype: IgG vs. IgA vs. Light chain vs. other 8. Plasma cells in the bone marrow 9. PET-CT scan results 10. ISS stage 11. R-ISS stage 12. Cytogenetics by FISH Pre-Infusion Therapy 13. Pre-transplant Systemic therapy 14. Best hematologic response to line of therapy 15. Minimal residual disease negativity Post-infusion Data 1. Best response post-transplant 2. Minimal residual disease negativity 3. Maintenance therapy: yes v no Outcome variables 1. Progression free survival 2. Overall survival
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A description of the hypothesis speci	N/A
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	N/A
SAMPLE REQUIREMENTS: If the study requires biologic samples from the CIBMTR Repository, the proposal should also include: 1) A detailed description of the proposed testing methodology and sample requirements; 2) A summary of the investigator's previous e	N/A
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	N/A

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	Myeloma in the Connect MM Registry. Clin Lymphoma Myeloma Leuk. 2022 Mar;22(3):149-157. doi: 10.1016/j.clml.2021.08.007. Epub 2021 Aug 29. PMID: 34588149. 11. Badar T, Hari P, Dávila O, et al. African Americans with translocation t(11;14) have superior survival after autologous hematopoietic cell transplantation for multiple myeloma in comparison with Whites in the United States. Cancer. 2021 Jan 1;127(1):82-92. doi: 10.1002/cncr.33208. Epub 2020 Sep 23. Erratum in: Cancer. 2021 Jul 10;: PMID: 32966625; PMCID: PMC7736245. 12. Hari PN, Majhail NS, Zhang MJ, et al. Race and outcomes of autologous hematopoietic cell transplantation for multiple myeloma. Biol Blood Marrow Transplant. 2010 Mar;16(3):395-402. doi: 10.1016/j.bbmt.2009.11.007. Epub 2009 Nov 14. PMID: 19922808; PMCID: PMC2833344. 13. Fillmore NR, Yellapragada SV, Ifeorah C, et al. With equal access, African American patients have superior survival compared to white patients with multiple myeloma: a VA study. Blood. 2019 Jun 13;133(24):2615-2618. doi: 10.1182/blood.2019000406. Epub 2019 Apr 19. PMID: 31003998; PMCID: PMC6566591. 14. Anampa-Guzmán A, Alam ST, Abuali I, Al Hadidi S. Health Disparities Experienced by Hispanic Americans with Multiple Myeloma: A Systematic Review. Clin Hematol Int. 2023 Mar;5(1):29-37. doi: 10.1007/s44228-022-00026-2. Epub 2022 Dec 31. PMID: 36586086; PMCID: PMC10063691. 15. Krishnan AY, Mei M, Sun CL, et al. Second primary malignancies after autologous hematopoietic cell transplantation for multiple myeloma. Biol Blood Marrow Transplant. 2013 Feb;19(2):260-5. doi: 10.1016/j.bbmt.2012.09.023. Epub 2012 Oct 13. PMID: 23073267; PMCID: PMC3553262. 16. Pulte ED, Nie L, Gormley N, et al. Survival of ethnic and racial minority patients with multiple myeloma treated with newer medications. Blood Adv. 2018 Jan 23;2(2):116-119. doi: 10.1182/bloodadvances.2017010512. PMID: 29365319; PMCID: PMC5786427. 17. Peres LC, Colin-Leitzinger CM, Teng M, et al. Racial and ethnic differences in clonal hematopoiesis, tumor markers, and outcomes of patients with multiple myeloma. Blood Adv. 2022 Jun 28;6(12):3767-3778. doi: 10.1182/bloodadvances.2021006652. PMID: 35500227; PMCID: PMC9631567. 18. Joshua TV, Rizzo JD, Zhang MJ, et al. Access to hematopoietic stem cell transplantation: effect of race and sex. Cancer. 2010 Jul 15;116(14):3469-76. doi: 10.1002/cncr.25297. PMID: 20564154; PMCID: PMC3153958. 19. Pan D, Coltoff A, Ozbek U, et al. Evaluating Race and Time to Transplantation in Multiple Myeloma: The Mount Sinai Hospital Experience. Clin Lymphoma Myeloma Leuk. 2021 Jul;21(7):439-443. doi: 10.1016/j.clml.2021.02.003.

Field	Response
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CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	No, I do not have any conflicts of interest pertinent to this proposal
If yes, provide detail on the nature of employment, name of organization, role, entity, ownership, type of financial transaction or legal proceeding and whether renumeration is >$5000 annually.	N/A

Table 1. Characteristics of Autologous Transplants with Available Race/Ethnicity Data for Multiple Myeloma Patients (Excluding Amyloidosis and Plasma Cell Leukemia), Age 18+, Diagnosed Within One Year Prior to Transplant

Characteristic	TED	CRF
No. of patients	13229	370
No. of centers	190	80
Recipient age groups - no. (%)		
Median (min-max)	63.1 (22.9-83.3)	63.2 (33.4-78.3)
<50	1404 (10.6)	33 (8.9)
50-54	1380 (10.4)	28 (7.6)
55-59	2151 (16.3)	71 (19.2)
60-64	2808 (21.2)	73 (19.7)
65-69	3018 (22.8)	106 (28.6)
70+	2468 (18.7)	59 (15.9)
Recipient race - no. (%)		
White	10175 (76.9)	225 (60.8)
Black or African American	2244 (17.0)	101 (27.3)
Asian	642 (4.9)	40 (10.8)
Native Hawaiian or other Pacific Islander	31 (0.2)	1 (0.3)
American Indian or Alaska Native	79 (0.6)	3 (0.8)
More than one race	58 (0.4)	0 (0.0)
Recipient ethnicity - no. (%)		
Hispanic or Latino	991 (7.5)	25 (6.8)
Not Hispanic or Latino	11622 (87.9)	322 (87.0)
Non-resident of the U.S.	616 (4.7)	23 (6.2)
Recipient sex - no. (%)		
Male	7635 (57.7)	217 (58.6)
Female	5594 (42.3)	153 (41.4)
Year of Transplant - no. (%)		
2012	1 (0.0)	0 (0.0)
2015	2 (0.0)	0 (0.0)
2016	7 (0.1)	0 (0.0)
2017	30 (0.2)	3 (0.8)
2018	41 (0.3)	4 (1.1)
2019	431 (3.3)	19 (5.1)
2020	3765 (28.5)	65 (17.6)
2021	4390 (33.2)	73 (19.7)
2022	4562 (34.5)	206 (55.7)
Country of Transplant Center - no. (%)		
USA	12458 (94.2)	339 (91.6)
Argentina	17 (0.1)	2 (0.5)
Australia	9 (0.1)	0 (0.0)
Brazil	93 (0.7)	1 (0.3)
Canada	195 (1.5)	2 (0.5)
India	30 (0.2)	3 (0.8)

Characteristic	TED	CRF
South Korea	159 (1.2)	17 (4.6)
New Zealand	83 (0.6)	0 (0.0)
Saudi Arabia	30 (0.2)	0 (0.0)
Spain	6 (0.0)	0 (0.0)
Mexico	2 (0.0)	0 (0.0)
Turkey	4 (0.0)	1 (0.3)
Uruguay	70 (0.5)	4 (1.1)
Singapore	68 (0.5)	1 (0.3)
Pakistan	5 (0.0)	0 (0.0)
Subdisease - no. (%)		
Multiple myeloma, no subdisease specified	10428 (78.8)	274 (74.1)
Solitary plasmacytoma	11 (0.1)	1 (0.3)
Osteosclerotic myeloma/POEMS syndrome	57 (0.4)	0 (0.0)
Light chain deposition disease	14 (0.1)	2 (0.5)
Other plasma cell disorder	23 (0.2)	0 (0.0)
Smoldering myeloma - asymptomatic	22 (0.2)	0 (0.0)
Mult myeloma-light chain	2538 (19.2)	89 (24.1)
Mult myeloma-non-secretory	131 (1.0)	3 (0.8)
MGRS - Monoclonal gammopathy of renal significance	5 (0.0)	1 (0.3)
Graft type - no. (%)		
Bone marrow	7 (0.1)	0 (0.0)
Peripheral blood stem cells	13218 (99.9)	370 (100)
BM + PBSC	3 (0.0)	0 (0.0)
Other	1 (0.0)	0 (0.0)
Conditioning regimen - no. (%)		
TBI/Mel	3 (0.0)	0 (0.0)
Bu/Mel	85 (0.6)	0 (0.0)
BEAM like	22 (0.2)	2 (0.5)
Mel alone	12936 (97.8)	363 (98.1)
Mel/other(s)	96 (0.7)	3 (0.8)
Other(s)	23 (0.2)	0 (0.0)
None	43 (0.3)	2 (0.5)
Not Reported	21 (0.2)	0 (0.0)
Karnofsky score prior to HCT - no. (%)		
90-100%	6920 (52.3)	179 (48.4)
< 90%	6094 (46.1)	184 (49.7)
Not reported	215 (1.6)	7 (1.9)
Any timepoint of the 2100 form was completed - no. (%)		
No	0 (0.0)	6 (1.6)
Yes	0 (0.0)	364 (98.4)
Not Applicable – TED level patient	13229 (100)	0 (0.0)
Any timepoint of the 2450 form was completed - no. (%)		

Characteristic	TED	CRF
No	132 (1.0)	0 (0.0)
Yes	13097 (99.0)	0 (0.0)
Not Applicable – CRF level patient	0 (0.0)	370 (100)
Time from diagnosis to transplant (months)		
N	13229	370
Not Reported	0	0
Median (min-max)	7.0 (0.0-12.0)	7.0 (0.0-12.0)
Follow-up - median (range)	24.9 (0.0-108.5)	24.1 (0.0-72.1)

CIBMTR Study Proposal

Study Title:
The Effect of Social Determinants of Health on Outcomes in Pediatric and Adolescent/Young Adult (AYA) Patients Undergoing Haploidentical Stem Cell Transplantation for Malignant and Non-Malignant Disease

Co-1st PI Information:
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Junior investigator status (defined as <40 years of age and/or ≤5 years from fellowship)
Yes

Do you identify as an underrepresented/minority?
Yes

Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?
Yes (Satwani)

Research Hypothesis:
Pediatric/AYA patients of non-white ethnicity and/or lower socioeconomic status (SES) will have decreased OS and increased treatment related mortality (TRM) compared with patients of white ethnicity and higher socioeconomic status undergoing Haploidentical HCT (HaploHCT).

Specific Aims:
Primary Aim: Determine the impact of social determinants of health (SDOH) including race/ethnicity and SES on outcomes in pediatric and adolescent/young adult (AYA) patients who undergo HaploHCT.

Scientific Impact:

As the relative population of minority groups within the United States continues to grow, so will the need for pediatric/AYA patients of these minority groups to undergo HCT. Unfortunately, patients from diverse ethnic backgrounds without a fully HLA matched sibling are at a disadvantage when trying to find a suitable donor within the bone marrow registries and are thus more reliant on alternative donor sources such as HLA matched/mismatched unrelated donor, cord blood and haploidentical family donors [1]. The number of patients undergoing HaploHCT has increased in recent years due to wide applicability of post-transplant cyclophosphamide. Currently, HaploHCT is a viable alternative donor source through randomized controlled trials which indicate that HaploHCT have similar OS compared to other donor types [2]. Unfortunately it has also been shown that patients of minority backgrounds have disparate outcomes post HCT [3]. Minority patients have also been shown to have disparate outcomes within the non-HCT patient population in the setting of the COVID-19 pandemic, which may indicate that there are potential intrinsic and extrinsic patient factors that could affect HCT outcomes in patients of minority ethnicity/race [4, 5]. Previous studies did not specifically analyze pediatric/AYA patients, which were often grouped with adult data, limiting the ability to apply results to pediatric/AYA populations. Therefore, we propose that the analysis of pediatric and AYA HaploHCT patient outcomes in relation to SDOH factors would elucidate any potential modifiable risk factors that could lead to changes in practice within this patient population.

Scientific Justification:

It has been stated that in order to address disparities within any group they must first be identified to exist within at-risk patient populations [6]. Analysis of studies performed over the last 2 decades have shown disparity in outcomes in ethnic minority patients as well as those of lower SES who underwent HCT in terms of overall survival, treatment related mortality, relapse and other measures [3, 7, 8]. A meta-analysis of available studies in adults and pediatrics indicated an increase in overall mortality in Hispanic and African American patients compared to White patients, however this study did not elucidate the cause of this disparity in outcomes [3]. A recent study of adult HCT patients using the CIBMTR database indicated that SES was associated with outcome in patients who develop GVHD, and in those patients Non-Hispanic Black race was associated with increased TRM and overall mortality [9]. This study also showed that Non-Hispanic Black race was associated with the development of more severe GVHD. It is plausible that ethnicity may increase the social and financial burden on patients and families going through HCT due to increased incidence of post-transplant complications due to requiring more frequent and prolonged treatment courses. They would also require more frequent follow up appointments due to the use of a longer course of immunosuppression to treat GVHD, which could cause issues with medical compliance and affect overall outcomes in these patients. The identification of pediatric/AYA patients who have an SES or ethnicity-based risk factor for worse outcomes could be an opportunity to provide more nuanced interventions and support to improve clinical outcomes.

It is known that patients with diverse ethnic backgrounds have been shown to have a disparity in the availability of unrelated donors via donor registries [1]. In recent years patients with ethnically diverse backgrounds are increasingly receiving allogeneic HCT, which has been accompanied by an increase in the use of post-transplant Cytoxan (ptCy) as GVHD prophylaxis [10]. Phase 3 trials in patients older than 16 yo has shown that OS is not significantly different when compared to historical CIBMTR data, indicating that HaploHCT with ptCy could be a viable alternative for patients without sufficient HLA match in unrelated donor registries [2]. Bona et al utilized the CIBMTR database to analyze the impact of social determinants of health on outcomes in pediatric malignant alloHCT, showing that decreased OS and increased treatment related mortality were associated with high poverty zip codes, Medicaid insurance status and Black Race [7]. However, this analysis did not include AYA patients and the number of potential pediatric HaploHCT patients available at the time of analysis was limited compared to those currently available for analysis today (86 patients vs >900 patients) (Table 1). This analysis also did not specify the degree of HLA match in the population that was described as “Other Related Bone Marrow” and “Other Related Peripheral Blood,” limiting the ability to determine the effect of HLA matching within this population. The isolation of pediatric/AYA HaploHCT patients for analysis could allow for a more granular analysis of their risk factors contributing to post-transplant outcome, which was not possible in the previous cohort due to the relatively small number of HaploHCT patients (Table 1) [7].

The COVID-19 pandemic was a significant world event that further highlighted disparities in the outcomes of patients with minority ethnicity and race. It has been reported that there were striking differences in mortality based on patient ethnicity, with Hispanic and Black patients having increased mortality [4, 5]. There are several studies that indicate COVID-19 infection is detrimental to patients both peri-transplant as well as during long term survivorship [11, 12]. However, there is a paucity of studies looking at the disparity in outcomes of HSCT patients who contracted COVID-19 based on their ethnicity. Due to the fact that there is a known ethnic disparity in the general population of non-HCT patients who contracted COVID-19, we would expect that ethnicity would have an impact on HCT patients with this infectious complication. Analysis of patients during the era pre- and post-COVID-19 pandemic would allow us to determine the effect of this infection within the pediatric/AYA HCT population and identify any opportunities for patient specific care interventions.

There are a limited number of studies that have assessed the effect of SDOH on HCT outcomes compared to other patient related factors, and even fewer in pediatric/AYA patient populations. These studies can vary in terms of finding significant differences between ethnic groups post-transplant [3, 13], which may highlight the limitations of smaller cohort studies. Ethnicity specific data can often be limited to adult patient populations, limiting the applicability of findings to pediatric HCT patient populations. Therefore, a larger study of pediatric/AYA patients including more recent data would allow for a full evaluation of outcomes. Also, inclusion of the additional pediatric and AYA patient data to the analysis of specifically HaploHCT patients would allow for the evaluation of the impact of SDOH within this patient population. The analysis of pre- and post-COVID-19 era data may provide insights what factors affect outcome post HCT based on SDOH. The identification of disparities among pediatric and AYA patients is essential to developing patient specific interventions to attempt to ameliorate transplant related complications and give patients the best possible outcome post HCT.

Table 1.

	Bona et al 2021	**Proposed Analysis**
Patient Age Range	**≤ 18 yo**	**0-39 yo**
Years Analyzed	**2005 - 2015**	**2000 - 2024**
Donor Source	**"Other Related Bone Marrow and Peripheral Blood" Degree of Match Not Specified**	**Haploidentical**
Number of Patients in Analysis	**86**	**>929**
Number of Patient Centers	**90**	**94**
Follow up of Survivors (Median)	**74 (2 - 126)**	**At least 35.4 (2.5 – 120.9)**
COVID19+ Patient Population	**No**	**Yes**

Patient Eligibility Population:

Inclusion Criteria:

- Haploidentical Allogeneic Transplant
- Year-2010-2024
- Age 0-39 years at time of alloHCT
- Malignant and Non-Malignant Disease
- Stem Cell Source: Peripheral blood stem cell and Bone Marrow

Exclusion Criteria:

- Embargoed centers and centers with 5-year completion index of <85%

Data Requirements: Variables to be described:

Patient Characteristics

- Age
- Gender
- Ethnicity (Caucasian vs. Hispanic vs. African American vs. Other/Missing)
- Indications: malignant vs non-malignant
- HCT-CI score
  Performance Status (<90 vs 90-100)
- Disease Status (CR1 and CR2)
- Insurance type (Private vs Public vs Uninsured)
- Household Income (Low/Median/High – Based on National Income Quartiles)
- Zip code of residence at the time of AlloHCT
- Distance of residence from the transplant center
- Setting of residence (Urban vs Suburban vs Rural)

Donor Characteristics

- Age
- Sex (M/F)

Transplant Characteristics

- Conditioning Intensity: Myeloablative vs Reduced intensity
- Era of transplant: 2010 - 2017 v 2018 – 2024
- Pre and post covid: 2015-2019 vs. 2020-2024
- Graft Source (Bone Marrow vs PBSC)
- Graft Manipulation (yes v no)
- CD34 cell dose infused
- GCSF (yes v no)
- GVHD prophylaxis used: “ptCy + other” vs “Ex Vivo T cell Depletion”
- Donor-recipient CMV status: +/+ vs +/- vs -/+ vs -/- vs Missing
- Donor-recipient sex match: M-M vs M-F vs F-M vs F-F

Outcomes

- Days to neutrophil engraftment
- Days to platelet engraftment
- Incidence of graft failure
- One- and 2-year overall survival
  Transplant related mortality at day 100, 1 and 2 years
- Relapse incidence and 1 and 2 years among patients with malignant diseases
- Incidence of Acute GVHD (II-IV and III-IV)
- Incidence of Chronic GVHD
- GVHD-free Relapse Free Survival (GRFS) – Malignant Patients
- Disease Free Survival (DFS) – Malignant Patients
- Incidence of VOD
- Incidence of respiratory failure/mechanical ventilation
- Incidence of dialysis/CRRT
- Incidence of TA-TMA
- Incidence of bacterial/viral/fungal infections
- Post-HaploHCT organ dysfunction - Liver, Cardiac, Lung, CNS, Renal, Other

Study Design:

The CIBMTR database will be utilized as part of this retrospective analysis of children undergoing alloHCT. Patient and transplant variables will be described using frequencies for categorical variables and median (range) for continuous variables.

The primary outcome of this study will be the overall survival of patients based on race/ethnicity (Hispanic vs. African American vs White vs. Other) and SES (Low/Median/High). Using the cumulative incidence method, this data will then be assessed for risk factors (disease status, disease type, comorbidities, pre-transplant infection status, conditioning intensity, and year of transplant). Death due to relapse will be a competing risk. Secondary outcomes will include outcomes based on SDOH patient background post alloHCT including relapse, transplant related mortality, and overall survival.

In order to assess the effect of patient and transplant related risk factors within our ethnic groups, we will use multivariable logistic regression after adjusting for recipient gender, and year of transplant. Subgroup analyses will be performed by disease type (malignant vs non-malignant), disease status (CR 1 vs >CR1), Ethnicity, Income Level, and insurance type.

Using Kaplan-Meier estimates the probability of survival at 1 and 2 years after alloHCT will be assessed, which will then be stratified by ethnicity/income and compared using log-rank testing. Within each ethnic group Kaplan-Meier estimates will be used to compare patients with risk factors of interest and compared using log-rank testing. If patients undergo a second alloHCT they will be censored at that time. Causes of death will be described.

References:

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2. Shaw, B.E., et al., *National Marrow Donor Program-Sponsored Multicenter, Phase II Trial of HLA-Mismatched Unrelated Donor Bone Marrow Transplantation Using Post-Transplant Cyclophosphamide.* J Clin Oncol, 2021. **39**(18): p. 1971-1982.
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7. Bona, K., et al., *Neighborhood poverty and pediatric allogeneic hematopoietic cell transplantation outcomes: a CIBMTR analysis.* Blood, 2021. **137**(4): p. 556-568.
8. Shoag, J., et al., *Disparities in access to hematopoietic cell transplant persist at a transplant center.* Bone Marrow Transplant, 2024. **59**(9): p. 1258-1264.
9. Farhadfar, N., et al., *Racial, ethnic, and socioeconomic diversity and outcomes of patients with graft-versus-host disease: a CIBMTR analysis.* Blood Adv, 2024. **8**(18): p. 4963-4976.
10. Auletta, J.J., et al., *Real-World Data Showing Trends and Outcomes by Race and Ethnicity in Allogeneic Hematopoietic Cell Transplantation: A Report from the Center for International Blood and Marrow Transplant Research.* Transplant Cell Ther, 2023. **29**(6): p. 346 e1-346 e10.
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12. Johnston, E.E., et al., *Risk of COVID-19 infection in long-term survivors of blood or marrow transplantation: a BMTSS report.* Blood Adv, 2023. **7**(12): p. 2843-2854.
13. Harney, S.M., et al., *Race and socioeconomic status in pediatric allogeneic hematopoietic cell transplantation for nonmalignant conditions.* Pediatr Blood Cancer, 2020. **67**(9): p. e28367.

Conflicts of Interest: None

Table 1. Characteristics of CRF track recipients less than 40 years old that received a PBSC or BM haploidentical HCT between 2010 to 2022

Characteristic	N (%)
No. of patients	2591
No. of centers	209
Recipient age at collection, grouped - no. (%)	
Median (min-max)	18.4 (0.0-40.0)
0-5	508 (19.6)
6-10	341 (13.2)
11-17	430 (16.6)
18-24	483 (18.6)
25-29	346 (13.4)
30-39	483 (18.6)
Recipient sex - no. (%)	
Male	1563 (60.3)
Female	1028 (39.7)
Recipient race - no. (%)	
White	1105 (42.6)
Black or African American	557 (21.5)
Asian	294 (11.3)
Native Hawaiian or other Pacific Islander	17 (0.7)
American Indian or Alaska Native	43 (1.7)
More than one race	72 (2.8)
Not reported	503 (19.4)
Recipient ethnicity - no. (%)	
Hispanic or Latino	483 (18.6)
Non Hispanic or non-Latino	1190 (45.9)
Non-resident of the U.S.	880 (34.0)
Not reported	38 (1.5)
Disease - no. (%)	
AML or ANLL	518 (20.0)
ALL	473 (18.3)
Other Leukemia	5 (0.2)
CML	55 (2.1)
MDS	107 (4.1)
Acute Leukemia	31 (1.2)
NHL	78 (3.0)

Characteristic	N (%)
HD	211 (8.1)
Plasma cell disorder	3 (0.1)
Solid Tumor	12 (0.5)
Aplastic anemia	342 (13.2)
Inherited abnormal of erythrocyte differ.	1 (0.0)
Inherited bone marrow failure syndromes	98 (3.8)
Hemoglobinopathies	314 (12.1)
Paroxysmal nocturnal hemoglobinuria (PNH)	10 (0.4)
Immune Deficiencies (ID)	231 (8.9)
Inherited platelet abnormalities	2 (0.1)
Inherited disorders of metabolism	36 (1.4)
Histiocytic disorder	49 (1.9)
Other disease	5 (0.2)
Myeloproliferative neoplasms	10 (0.4)
Graft type - no. (%)	
Bone marrow	1142 (44.1)
Peripheral blood stem cells	1449 (55.9)
Conditioning intensity- center reported - no. (%)	
MAC	1458 (56.3)
NST/RIC	1018 (39.3)
Not reported	115 (4.4)
GVHD prophylaxis - no. (%)	
None	73 (2.8)
Ex-vivo T-cell depletion	229 (8.8)
CD34 selection	135 (5.2)
PtCy	1861 (71.8)
TAC based	181 (7.0)
CSA based	78 (3.0)
Other	27 (1.0)
Not reported	7 (0.3)
Patient zip code available - no. (%)	
No	1221 (47.1)
Yes	1370 (52.9)
Year of transplant - no. (%)	
2010	21 (0.8)
2011	17 (0.7)
2012	26 (1.0)
2013	94 (3.6)

Characteristic	N (%)
2014	172 (6.6)
2015	236 (9.1)
2016	278 (10.7)
2017	327 (12.6)
2018	374 (14.4)
2019	405 (15.6)
2020	197 (7.6)
2021	206 (8.0)
2022	238 (9.2)
Follow-up - median (range)	48.6 (0.0-155.8)

Field	Response
Proposal Number	2410-80-SCHOETTLER
Proposal Title	Defibrotide prophylaxis for hepatic sinusoidal obstructive syndrome in pediatric hematopoietic cellular therapy recipients: real-world outcomes and health care utilization implications
Key Words	Sinusoidal obstructive syndrome (SOS), defibrotide prophylaxis
Principal Investigator #1: - First and last name, degree(s)	Michelle L Schoettler, MD, MS
Principal Investigator #1: - Email address	Michelle.Schoettler@emory.edu
Principal Investigator #1: - Institution name	Children's Healthcare of Atlanta
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as ≤5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	Yes
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Kirsten M Williams, MD
Principal Investigator #2 (If applicable): - Email address:)	kirsten.marie.williams@emory.edu
Principal Investigator #2 (If applicable): - Institution name:	Children's Healthcare of Atlanta
Principal Investigator #2 (If applicable): - Academic rank:	Professor
Junior investigator status (defined as ≤5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
We encourage a maximum of two Principal Investigators per study. If more than one author is listed, please indicate who will be identified as the corresponding PI below:	-
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	-
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	CIBMTR Leadership Committee with the morbidity and survivorship group
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Morbidity, Recovery and Survivorship
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
If you have already spoken with a scientific director or working committee chair regarding this study, then please specify who:	-

Field	Response
RESEARCH QUESTION:	Outside of a clinical trial setting, does defibrotide prophylaxis in children result in significant differences in sinusoidal obstructive syndrome (SOS) incidence, severe SOS(as measured by organ failure), and non-relapse related mortality and what are the early post HCT health care utilization (HCU) implications of defibrotide prophylaxis.
RESEARCH HYPOTHESIS:	Patients who received defibrotide prophylaxis compared to matched cohort will have a significantly lower cumulative incidence of SOS and severe SOS as defined by multiorgan dysfunction. We hypothesize there will be no differences in health care utilization (HCU) in those who received defibrotide prophylaxis and the matched cohort, cost differences in the defibrotide cohort will be defrayed by critical care and other costs in the cohort who did not receive prophylaxis.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Objective 1: Determine the difference in cumulative incidence of severe SOS (defined as multi-organ dysfunction) by day 100 post HCT in patients who received defibrotide prophylaxis compared to matched controls. Objective 2: Determine the difference in cumulative incidence of SOS by day 100 in patients who received defibrotide prophylaxis compared to matched controls. Objective 3: Determine the difference in health care utilization as measured by standardized inpatient costs from time HCT admission day 100 post HCT in patients who received defibrotide prophylaxis compared to matched controls. Objective 4: Determine the subcategory of costs from time HCT admission day 100 post HCT in patients who received defibrotide prophylaxis compared to matched controls.

Field	Response
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Reporting real-world outcomes of defibrotide prophylaxis in children can immediately inform clinical practice for practitioners caring for these patients. In addition to clinical outcomes, reporting HCU of this expensive therapy can help hospitals and programs evaluate the value of this therapy. SCIENTIFIC JUSTIFICATION: A randomized phase 3 clinical trial in children demonstrated a significant difference in cumulative incidence of SOS on day 30 post HCT (Corbacioglu et al, 2016). However, the study was not powered to detect differences in multi-organ failure or non-relapse related mortality. A follow up randomized, open-label phase 3 multicenter trial evaluating the efficacy of SOS-free survival at day 30 in children and adults was closed early after an interim analysis determined that the study met protocol-specified futility (Grupp et al, 2023). Given these recent results, there is unlikely to be a follow up clinical trial. However, it remains unclear whether defibrotide prophylaxis impacts SOS incidence or the severity of disease, particularly in very high-risk children for whom a prior study demonstrated some benefit. Further, the recent clinical trial, HARMONY, had a primary endpoint of day 30 SOS free- survival; given that death particularly this early post HCT from SOS is quite uncommon. While death is uncommon, significant morbidity Another important endpoint of morbidity, as defined as multi-organ dysfunction, remains very relevant and there are scant data of defibrotide prophylaxis and the impact of severe SOS. In addition to concerns about efficacy, concerns for costs dictate the use of defibrotide prophylaxis. Several analyses have demonstrated that VOD is cost effective for the treatment of VOD. However, there are no data on the impact of costs for VOD prophylaxis. There are ~650 children who have received defibrotide prophylaxis in the CIBMTR database; this is no other way to answer this question. The number of patients and ability to merge data from the Pediatric Health Information System (PHIS) and CIBMTR database will allow us to answer these questions and is highly clinically impactful.

Field	Response
SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	A randomized phase 3 clinical trial in children demonstrated a significant difference in cumulative incidence of SOS on day 30 post HCT (Corbacioglu et al, 2016). However, the study was not powered to detect differences in multi-organ failure or non-relapse related mortality. A follow up randomized, open-label phase 3 multicenter trial evaluating the efficacy of SOS-free survival at day 30 in children and adults was closed early after an interim analysis determined that the study met protocol-specified futility (Grupp et al, 2023). Given these recent results, there is unlikely to be a follow up clinical trial. However, it remains unclear whether defibrotide prophylaxis impacts SOS incidence or the severity of disease, particularly in very high-risk children for whom a prior study demonstrated some benefit. Further, the recent clinical trial, HARMONY, had a primary endpoint of day 30 SOS free- survival; given that death particularly this early post HCT from SOS is quite uncommon. While death is uncommon, significant morbidity Another important endpoint of morbidity, as defined as multi-organ dysfunction, remains very relevant and there are scant data of defibrotide prophylaxis and the impact of severe SOS. In addition to concerns about efficacy, concerns for costs dictate the use of defibrotide prophylaxis. Several analyses have demonstrated that VOD is cost effective for the treatment of VOD. However, there are no data on the impact of costs for VOD prophylaxis. There are ~650 children who have received defibrotide prophylaxis in the CIBMTR database; this is no other way to answer this question. The number of patients and ability to merge data from the Pediatric Health Information System (PHIS) and CIBMTR database will allow us to answer these questions and is highly clinically impactful.

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	CASES (defibrotide prophylaxis) Inclusion: - Including all children (aged <= 18 years old) who underwent allogeneic or autologous HCT and received defibrotide prophylaxis from 2009-2022. Exclusion: - Defibrotide administration for treatment of VOD - Of note, years 2006 to 2009 are not included as this would potentially capture patients who received defibrotide prophylaxis on the previously reported clinical trial. CONTROLS (no defibrotide prophylaxis) Inclusion: - Including all children (aged <= 18 years old) who underwent allogeneic or autologous HCT who did not receive defibrotide prophylaxis from 2009-2022 and will be matched to the SOS group on the following characteristics: o Underlying disease o Prior gemtuzumab/inotuzumab exposure o Known prior liver injury o Age o Myeloablative busulfan We intend on matching cases and controls (1:3) to improve power.
Does this study include pediatric patients?	Yes
If this study does not include pediatric patients, please provide justification:	-

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Outline any supplementary data required.	After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Data collection forms available at: http://www.cibmtr.org/DataManagement/DataCollectionForms/Pages/index.aspx Outline any supplementary data required. Additional data collection is extremely difficult and will make your proposal less feasible. No additional data collection is necessary. Variables needed for the analysis are available on CRF forms. Patient Related: - HCT indication - Age - Sex - Prior HCT (yes/no) - Receipt of gemtuzumab or inotuzumab prior to HCT - Prior liver disease - DRI (hematologic malignancy only) Transplant Related - Transplant type (autologous/allogeneic) - HLA mismatch - Stem Cell Source - Donor related/unrelated - Preparative regimen o Myeloablative/ non and TBI vs busulfan o Busulfan and AUC - Acute GVHD prophylaxis Transplant complications - Sinusoidal obstructive syndrome (SOS) o Date SOS o Maximum severity (maximum bilirubin, organ function as below) o Management of late sequelae required (variceal banding, TIPS, paracentesis, thoracentesis) - TA-TMA o Date TA-TMA - Defibrotide prophylaxis (yes/no) - Acute GVHD, maximum stage and grade - Relapsed disease (yes/no) o Date relapse - Dead/Alive, date, o NRM or relapse o Primary points of interest: day 100, 180 and 1 year Cost Data (PHIS) - Significant organ impairment in the first 100 days: o Acute renal failure requiring dialysis o Intubation/Mechanical Ventilation o Diffuse alveolar hemorrhage o Intensive care admission and days (PHIS data) - Standardized costs from day of HCT admission to day 100 - Cost subcategory costs
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A description of the hypothesis speci	NA
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	NA

Field	Response
SAMPLE REQUIREMENTS: If the study requires biologic samples from the CIBMTR Repository, the proposal should also include: 1) A detailed description of the proposed testing methodology and sample requirements; 2) A summary of the investigator's previous e	NA
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	Pediatric Health Information System (PHIS) data will be linked to obtain a standardized unit cost and determine severity of illness. The standardized cost accounts for differences in geographic areas, inflation, and hospital cost- it is not what patients are charged, but is a measure of health care utilization. Linkage is required because CIBMTR does not collect cost data. Further, CRF level data are available on only a small subset of patients. Linkage with PHIS allows for assessment of severe SOS, which is the primary endpoint of interest, while using the most data available. Linkage with PHIS is feasible and has been done in a prior CIBMTR study (Arnold, et al). Between data from the CIBMTR and PHIS data systems, we will have all the data necessary to answer the study questions. RATIONALE FOR LIMITING TO A PEDIATRIC COHORT: The end point of interest in this study is severe SOS. Linkage to the PHIS database allows assessment of severe SOS without relying on CRF data, which we anticipate will be available only in a small proportion of pateints. Further, the second endpoint of interest in this study is costs, which are only available using the PHIS linkage system.
REFERENCES:	-
CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	No, I do not have any conflicts of interest pertinent to this proposal
If yes, provide detail on the nature of employment, name of organization, role, entity, ownership, type of financial transaction or legal proceeding and whether renumeration is >$5000 annually.	-

Table 1. Characteristics of case/control matching (1:3) for allogeneic and autologous transplants amongst pediatric recipients between 2020 to 2022

Characteristic	Cases: received defibrotide prophylaxis	Controls: did not receive defibrotide prophylaxis
No. of patients	477	7629
No. of centers	63	163
Recipient age at collection, grouped - no. (%)		
Median (min-max)	5.0 (0.1-18.0)	6.5 (0.0-18.0)
0-2	166 (34.8)	1894 (24.8)
3-5	103 (21.6)	1688 (22.1)
6-9	80 (16.8)	1575 (20.6)
10-17	128 (26.8)	2472 (32.4)
Recipient sex - no. (%)		
Male	272 (57.0)	4564 (59.8)
Female	205 (43.0)	3065 (40.2)
Recipient race - no. (%)		
White	317 (66.5)	4381 (57.4)
Black or African American	61 (12.8)	878 (11.5)
Asian	29 (6.1)	809 (10.6)
Native Hawaiian or other Pacific Islander	5 (1.0)	36 (0.5)
American Indian or Alaska Native	9 (1.9)	61 (0.8)
More than one race	11 (2.3)	287 (3.8)
Not reported	45 (9.4)	1177 (15.4)
Recipient ethnicity - no. (%)		
Hispanic or Latino	112 (23.5)	1180 (15.5)
Non-Hispanic or non-Latino	191 (40.0)	3478 (45.6)
Non-resident of the U.S.	168 (35.2)	2803 (36.7)
Not reported	6 (1.3)	168 (2.2)
Disease - no. (%)		
AML or ANLL	105 (22.0)	848 (11.1)
ALL	84 (17.6)	1092 (14.3)
Other Leukemia	0 (0.0)	4 (0.1)
CML	3 (0.6)	57 (0.7)
MDS	28 (5.9)	264 (3.5)
Acute Leukemia	6 (1.3)	77 (1.0)
NHL	8 (1.7)	114 (1.5)
HD	1 (0.2)	156 (2.0)
Solid Tumor	86 (18.0)	1913 (25.1)
Sub-diseases of Solid Tumor		
Solid tumor	1 (1.2)	4 (0.2)
Head and neck	0 (0)	2 (0.1)

Characteristic	Cases: received defibrotide prophylaxis	Controls: did not receive defibrotide prophylaxis
Testicular	0 (0)	15 (0.8)
Ovary (epithelial)	1 (1.2)	10 (0.5)
Central nervous system	1 (1.2)	340 (17.8)
Wilm tumor	0 (0)	27 (1.4)
Neuroblastoma	67 (77.9)	1018 (53.2)
Retinoblastoma	0 (0)	20 (1.1)
Germ cell tumor	1 (1.2)	46 (2.4)
Medulloblastoma	5 (5.8)	280 (14.6)
Lung, NOS	0 (0)	1 (0.1)
Rhabdomyosarcoma	2 (2.3)	6 (0.3)
Leiomyosarcoma	0 (0)	1 (0.1)
Other malignancy, specify	7 (8.1)	126 (6.6)
Soft tissure sarcoma	1 (1.2)	2 (1.0)
Ewing family tumors of bone	0 (0)	12 (0.6)
Ewing family tumors, extraosseous	0 (0)	3 (0.2)
Aplastic anemia	14 (2.9)	653 (8.6)
Inherited bone marrow failure syndromes	7 (1.5)	316 (4.1)
Hemoglobinopathies	44 (9.2)	954 (12.5)
Paroxysmal nocturnal hemoglobinuria (PNH)	1 (0.2)	10 (0.1)
Immune Deficiencies (ID)	30 (6.3)	707 (9.3)
Inherited platelet abnormality	0 (0.0)	28 (0.4)
Inherited disorders of metabolism	27 (5.7)	205 (2.7)
Histiocytic disorder	31 (6.5)	171 (2.2)
Autoimmune Disease	0 (0.0)	20 (0.3)
Other disease	2 (0.4)	28 (0.4)
Recessive Dystrophic Epidermolysis Bullosa	0 (0.0)	1 (0.0)
Myeloproliferative neoplasms	0 (0.0)	11 (0.1)
Graft type - no. (%)		
Bone marrow	191 (40.0)	3404 (44.6)
Peripheral blood stem cells	230 (48.2)	3751 (49.2)
Umbilical cord blood	56 (11.7)	471 (6.2)
Other	0 (0.0)	3 (0.0)
Donor type - no. (%)		
Autologous	88 (18.4)	2095 (27.5)
HLA-identical sibling	89 (18.7)	1596 (20.9)
Other related	156 (32.7)	1781 (23.3)
8/8 matched URD	62 (13.0)	1193 (15.6)
7/8 mismatched URD	20 (4.2)	297 (3.9)
<= 6/8 mismatched URD;	0 (0.0)	9 (0.1)
Multi-donor	0 (0.0)	43 (0.6)
Unrelated (matching TBD)	5 (1.0)	97 (1.3)

Characteristic	Cases: received defibrotide prophylaxis	Controls: did not receive defibrotide prophylaxis
Cord blood	57 (11.9)	518 (6.8)
Conditioning intensity- center reported - no. (%)		
MAC	321 (67.3)	3870 (50.7)
NST/RIC	64 (13.4)	1584 (20.8)
N/A- autologous	88 (18.4)	2096 (27.5)
Not reported	4 (0.8)	79 (1.0)
GVHD prophylaxis - no. (%)		
None	99 (20.8)	2260 (29.6)
Ex-vivo T-cell depletion	39 (8.2)	369 (4.8)
CD34 selection	17 (3.6)	121 (1.6)
PtCy	95 (19.9)	1316 (17.2)
TAC based	134 (28.1)	1559 (20.4)
CSA based	86 (18.0)	1793 (23.5)
Other	4 (0.8)	200 (2.6)
Not reported	3 (0.6)	11 (0.1)
Prior Gemtuzumab exposure - no. (%)		
No	390 (81.8)	6780 (88.9)
Yes	64 (13.4)	292 (3.8)
Not reported	23 (4.8)	557 (7.3)
Prior Inotuzumab exposure - no. (%)		
No	421 (88.3)	6990 (91.6)
Yes	34 (7.1)	82 (1.1)
Not reported	22 (4.6)	557 (7.3)
Prior liver injury - no. (%)		
No	476 (99.8)	7562 (99.1)
Yes	1 (0.2)	33 (0.4)
Not reported	0 (0.0)	34 (0.4)
Myeloablative busulfan - no. (%)		
No	182 (38.2)	3387 (44.4)
Yes	203 (42.6)	2067 (27.1)
N/A-autologous	88 (18.4)	2096 (27.5)
Not reported	4 (0.8)	79 (1.0)
Year of transplant - no. (%)		
2020	115 (24.1)	2156 (28.3)
2021	181 (37.9)	2606 (34.2)
2022	181 (37.9)	2867 (37.6)
Follow-up - median (range)	24.3 (0.0-51.8)	24.4 (0.0-56.7)