Not for publication or presentation

A G E N D A
CIBMTR WORKING COMMITTEE FOR LEUKEMIA
Salt Lake City, UT
Thursday, February 5, 2026, 1:00 – 4:00 PM (MT)

Co-Chair:	**Michael Grunwald, MD; Levine Cancer Institute, Charlotte, NC; Phone: 980-442-5125; Email: Michael.grunwald@carolinashealthcare.org**
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Statistician:	**Zhongyu (Nick) Feng, MPH; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Phone: 414-955-3729; E-mail: zfeng@mcw.edu**
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1. **Introduction**
    a. Minutes from February 2025
        i. Acute Leukemia (**Attachment 1a**)
        ii. Chronic Leukemia (**Attachment 1b**)

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

3. Presentations, Publications or Submitted papers

a. **GS19-02** Hickey CL, Zhang M, Allbee-Johnson M, Romee R, Majhail NS, Malki M, Antin JH, Benjamin CL, Bredeson C, Chhabra S, Grunwald MR, Inamoto Y, Kanakry CG, Milano F, Soiffer RJ, Spellman SR, Solomon SR, Brunstein CG, Cutler C. Donor type does not impact late graft failure following reduced-intensity allogeneic hematopoietic cell transplantation with post-transplant cyclophosphamide-based graft-versus-host disease prophylaxis. ***Transplantation and Cellular Therapy. 2025 Mar 1; 31(3):174.e1-174.e12. doi:10.1016/j.jtct.2024.12.021. Epub 2025 Jan 2. PMC11875877.***

b. **LK19-02** Evolving significance of Ph-positive status on ALL post-transplant outcomes in the TKI era (M Krem / R Maziarz). ***Submitted.***

c. **LK20-02** Outcomes of allogeneic hematopoietic cell transplantation among germline RUNX1 mutation carriers with acute myeloid leukemia (P Liu/L Cunningham). ***Submitted.***

d. **LK21-01a** Clinical utility of pre-transplant flow cytometry tests of measurable residual disease in AML patients in first complete remission: A CIBMTR analysis. ***Submitted.***

e. **LK21-01g** Inter-laboratory differences in pre-transplant flow cytometric measurable residual disease in acute myeloid leukemia: A CIBMTR analysis. ***Submitted.***

f. **LK23-01a** Significant Inter-Laboratory Variability in Measurable Residual Disease Multiparameter Flow Cytometry Testing Prior to Allogeneic Transplantation Impedes Outcome Prediction: A CIBMTR Analysis. (A D Law/ T A Moya). ***Poster Presentation, Tandem Meetings 2025.***

g. **CK16-01b** Frequency of Deleterious Pathogenic/Likely Pathogenic Germline Variants in Related and Unrelated Allogeneic Hematopoietic Cell Transplant Donors for Patients with Myelodysplastic Syndrome. (R Stubbins). ***Poster Presentation, Tandem Meetings 2025.***

h. **LK20-01** Acute myeloid leukemia with chromosome 17 abnormalities with or without TP53 abnormalities and outcomes after hematopoietic stem cell transplantation. (A Dias/ J Yared). ***Oral Presentation, ASH 2025.***

i. **CK22-02** Superior long-term outcomes with fludarabine and melphalan reduced intensity regimen in older AML/MDS patients undergoing allogeneic stem cell transplantation: An analysis of CIBMTR data. (P Kongtim/ A Portuguese/ S Ciurea/ B Scott). ***Oral Presentation, ASH 2025.***

4. Studies in progress (Attachment 3)

a. **CK16-01b** Identification of germline predisposition mutations in young myelodysplastic syndrome patients (L Godley). **Analysis.**

b. **LK20-01** Acute myeloid leukemia with chromosome 17 abnormalities with or without TP53 abnormalities and outcomes after hematopoietic stem cell transplantation (A Dias/J Yared). **Data File Preparation.**

c. **LK20-03** Evaluating outcomes of allogeneic hematopoietic cell transplantation in T-cell acute lymphoblastic leukemia (H Murthy/M Iqbal/M Kharfan-Dabaja). **Data File Preparation.**

d. **CK22-01** Impact of somatic mutations on outcomes after allogeneic hematopoietic cell transplantation in patients with myelodysplastic syndrome with ring sideroblasts (MDS-RS) and MDS/myeloproliferative neoplasm with RS and thrombocytosis (MDS/MPN-RS-T) (S Arslan/ R Nakamura). **Protocol Development.**

e. **LK22-01** Impact of pre-allogeneic hematopoietic cell transplantation therapy in acute myeloid leukemia and myelodysplastic syndrome on post-transplant outcomes (Ali N). **Data File Preparation.**

f. **CK22-02** Toxicity and survival of AML/MDS patients receiving allogeneic stem cell transplantation using reduced-intensity conditioning: A propensity score analysis. (P Kongtim/ A Portuguese/ S Ciurea/ B Scott). **Data File Preparation.**

g. **CK23-01** Identifying the Optimal Graft-versus-Host Disease Regimen in Allogeneic Transplantation for Myelofibrosis (S Patel/ D Courier). **Protocol Received.**
h. **LK23-01a** The impact of allogeneic stem cell transplantation on acute myeloid leukemia and myelodysplastic syndrome with chromosome 3 abnormalities (A Datt Law). **Protocol Development.**
i. **LK23-02** Prognostic impact of cytogenetic and molecular risk classification in AML after hematopoietic stem cell transplant in adolescents and young adults (H Lust). **Protocol Development.**
j. **CK23-02** The mutational landscape in Myelodysplastic Syndrome arising from Aplastic Anemia and its impact on Allogeneic Stem Cell Transplantation Outcomes (B Ball/ R Nakamura). **Protocol Received.**
k. **LK23-03** Impact of donor source in second allogeneic hematopoietic cell transplant in patients with acute leukemia/MDS who relapsed after prior allograft during the current era (2014-2020) (A Troullioud Lucas/ A Scaradavou). **Protocol Development.**
l. **CK24-01** Identifying the optimal stem cell dosing for peripheral blood stem cell transplantation with post-transplant cyclophosphamide. (H Elmariah/ A Gandhi/ N Bejanyan/ R Marziarz). **Protocol Development.**
m. **LK24-01a** Safety and efficacy of CAR-T cell therapy in relapsed/refractory acute lymphoblastic leukemia with central nervous system involvement (L F Gonzalez Mosquera/ S Farhan). **Protocol Development.**
n. **LK24-01b** Sequencing of chimeric antigen receptor T-cell therapy and allogeneic transplantation in adult patients with B-cell acute lymphoblastic leukemia (D Eng/ J Fein/ A Arteaga/ M Kharfan-Dabaja/ L Metheny/ R Mohty/ H Sibai/ J Wang). **Protocol Development.**
o. **LK24-01c** Real World Experience (RWE) of adult patients receiving CD19 CAR-T cells for B cell Acute Lymphoblastic Leukemia (B-ALL): A CIBMTR Analysis. (A-S Mirza/ M Bilal Abid/ K Wudhikarn/ L Gowda/ MA Perales/ N Bejanyan). **Protocol Development.**
p. **CK24-02** Outcomes of allogeneic hematopoietic stem cell transplantation in patients with DDX41-mutated myelody splastic syndrome and acute myeloid leukemia. (R Stubbins/ E Wong/ L Fox/ L Gowda/ S Seropian). **Protocol Development**
q. **CK24-03** Comparison of reduced intensity conditioning regimens for haploidentical donor hematopoietic cell transplant with post-transplant cyclophosphamide in patients with acute myeloid leukemia or myelodysplastic syndromes. (H Elmariah/ S Arslan/ M Al Malki/ N Bejanyan). **Protocol Development.**
r. **CK24-04** Comparison of post-transplant cyclophosphamide-based reduced intensity conditioning regimens for older patients with acute myelogenous leukemia and MDS. (L Bachier/ S Solomon). **Protocol Development.**
s. **LK25-01** Comparison of FluFTBI and other myeloablative Conditioning Regimens for Haploidentical and mismatched unrelated Hematopoietic Cell Transplant with Post-Transplant Cyclophosphamide in Patients with Acute Leukemia. (S Arslan/ M Al Malki). **Protocol Pending.**
t. **LK25-02** Myelodysplastic Neoplasms with Hypoplasia (MDS-h) or Fibrosis (MDS-f): Distinct Clinical Entities Compared to Other MDS Subtypes. (A Law/ S Rodriguez). **Protocol Pending.**
u. **LK25-03** Impact of Post-Transplant Cyclophosphamide Based GVHD prophylaxis on Outcomes in Patients with CMML Undergoing Allogeneic Stem Cell Transplant. (Y Berry/ S Farhan/ I Varadarajan/ K Ball). **Protocol Pending.**

5. Future/proposed studies

a. **PROP 2505-02; 2509-43** Allogeneic Stem Cell Transplant Outcomes in Acute Leukemias of Ambiguous Lineage and Prognostic Model in Mixed Phenotype Acute Leukemia (S Cakmak/ A Viswabandya/ X-H Zhang) (**Attachment 4**)

b. **PROP 2508-03** Impact of racial and socio-economic factors on timely referral for Allogeneic stem cell transplant for the treatment of MPNs and MDS: A CIBMTR Report (N Hossain) (**Attachment 5**)
c. **PROP 2509-32** Outcomes of Patients with CLL/SLL Who Receive Allogeneic Hematopoietic Stem Cell Transplant in the Modern Era of Therapies (J Huang/ A Kittai) (**Attachment 6**)
d. **PROP 2509-86** Novel Composite endpoints for outcomes of patients with acute lymphoblastic lymphoma treated with CART therapy (S Tracy/ V Bachanova) (**Attachment 7**)
e. **PROP 2509-119; 2509-183** Outcomes of allogeneic hematopoietic cell transplantation in VEXAS syndrome: A combined EBMT and CIBMTR study (R Stubbins/ T Alexander/ E Ayala) (**Attachment 8**)
f. **PROP 2509-124** Fludarabine exposure and outcome following allogeneic hematopoietic stem cell transplantation for AML and MDS (C Graham/ M Juckett) (**Attachment 9**)
g. **PROP 2509-132** Outcomes of allogeneic hematopoietic stem cell transplant in patients with chronic myelomonocytic leukemia in the contemporary era (X Bi) (**Attachment 10**)
h. **PROP 2509-170** Late-relapse and long-term outcomes in patients with AML/MDS receiving post-transplant cyclophosphamide for GVHD prophylaxis. (A Baranwal/ C Ustun) (**Attachment 11**)
i. **PROP 2509-176** Outcomes of Allogeneic Hematopoietic Cell Transplantation with Post-Transplant Cyclophosphamide Compared to Conventional GVHD Prophylaxis in TP53-Mutated Acute Myeloid Leukemia and Myelodysplastic syndromes (N Sumransub/ M Gooptu) (**Attachment 12**)
j. **PROP 2509-208** Allogeneic Hematopoietic Stem Cell Transplantation Outcomes in Accelerated- and Blast-Phase Chronic Myeloid Leukemia in the Tyrosine Kinase Inhibitor Era (U Gergis) (**Attachment 13**)
k. **PROP 2509-223** Outcomes of Allogeneic Hematopoietic Cell Transplantation for Large Granular Lymphocytic Leukemia (S Park/ V Pullarkat) (**Attachment 14**)
l. **PROP 2509-234** Outcomes of Allogenic HSCT for therapy related myeloid neoplasms arising following treatment with CAR T cell therapy. (S Hamid/ R Faramand) (**Attachment 15**)

Proposed studies; not accepted for consideration at this time

i. **PROP 2501-01** Outcomes after cellular therapy (CAR T-cells, allogeneic stem cell transplantation) in Ph-like acute lymphoblastic leukemia (F Andreozzi). ***Dropped due to small sample size.***
j. **PROP 2507-02** Outcomes of second transplant in myelofibrosis for any indication (H Ali/ S Otoukesh). ***Dropped due to low scientific impact.***
k. **PROP 2509-02** Incidence and Risk Factors for Post-transplant Extramedullary Relapse in Acute Myeloid Leukemia (Post-HSCT EM relapse in AML) (K Poonsombudlert). ***Dropped due to need of supplemental data.***
l. **PROP 2509-25** Outcomes and Predictors of outcomes of adult patients with therapy-related acute lymphoblastic leukemia after allogeneic hematopoietic stem cell transplantation. (R V Nampoothiri). ***Dropped due to need of supplemental data.***
m. **PROP 2509-26** Outcomes of patients undergoing planned allogeneic stem cell transplant after CART cell therapy for Acute lymphoblastic leukemia (R V Nampoothiri). ***Dropped due to overlap with current study/publication.***
n. **PROP 2509-38** Additional Molecular Abnormalities in Relapsed Standard Risk Acute Myeloid Leukemia (AML) and Their Impact on Survival After Allogeneic Bone Marrow Transplantation (BMT) (S P Sudha/ R Kumar). ***Dropped due to small sample size.***
o. **PROP 2509-58** Outcomes of KMT2A rearranged acute leukemias following allogeneic hematopoietic cell transplantation in first complete remission (T Othman/ P Kebriaei). ***Dropped due to overlap with current study/publication.***

p. **PROP 2509-69** Determining optimal consolidation for precursor B-cell Acute lymphoblastic leukemia in CR1. Comparing allogeneic hematopoietic cell transplantation to blinatumomab consolidation. A ECOG/CIBMTR comparative study (H Murthy/ M Litzow). ***Dropped due to need of supplemental data.***
q. **PROP 2509-76** Does Cell Dose Predict Outcomes in Myelofibrosis? (P Smallbone/ U Popat). ***Dropped due to overlap with current study/publication.***
r. **PROP 2509-99** Safety and efficacy of therapeutic donor lymphocyte infusion for AML after post-transplant cyclophosphamide transplant in the mismatched donor setting (L Lekakis). ***Dropped due to low scientific impact.***
s. **PROP 2509-112** Real-World Analysis of CAR-T Cell Therapy in Chronic Lymphocytic Leukemia: Identifying Factors Associated with Clinical Outcomes (T Bahar/ A Aljundi/ S Farhan). ***Dropped due to small sample size.***
t. **PROP 2509-113** Donor Lymphocyte Infusion Versus Second Transplant for Relapse of AML and MDS After Post-Transplant Cyclophosphamide-Based Allogeneic Hematopoietic Cell Transplantation. (E Schulz/ N El Jurdi). ***Dropped due to low scientific impact.***
u. **PROP 2509-116** Effect of total-body irradiation on outcomes of allogeneic hematopoietic cell transplantation with reduced-intensity conditioning in adults with acute lymphoblastic leukemia/lymphoma (J Webster/ J Claiborne). ***Dropped due to low scientific impact.***
v. **PROP 2509-129** Post-transplant maintenance in myelodysplastic syndrome (MDS): impact on relapse and survival outcomes (B Oran/ P Smallbone). ***Dropped due to need of supplemental data.***
w. **PROP 2509-138** Outcomes of ALLO-HCT versus CAR T in patients B ALL in morphological remission (B Dholaria/ O Oluwole). ***Dropped due to low scientific impact.***
x. **PROP 2509-147** Outcomes of secondary graft failure in individuals with hematologic malignancies who have undergone allogeneic transplant. (E Irons/ K van Besien). ***Dropped due to low scientific impact.***
y. **PROP 2509-155** Outcome with Intensive Therapy Combination with Midostaurin or Quizartinib followed by Allogeneic HSCT and Maintenance in Newly Diagnosed FLT3 ITD Mutated AML Patients (A Ladha/ A Kanate). ***Dropped due to low scientific impact.***
z. **PROP 2509-164** Outcomes of Fludarabine–Treosulfan Compared with Fludarabine–Melphalan Conditioning in Allogeneic Transplant for AML and MDS: A CIBMTR Study (C Gates/ A Qasrawi). ***Dropped due to overlap with current study/publication.***
aa. **PROP 2509-180** Impact of Post-transplant Maintenance Strategies on Disease- and Transplant-Related Outcomes in TP53+ Acute Myeloid Leukemia and Myelodysplastic Syndromes (A Mina). ***Dropped due to need of supplemental data.***
bb. **PROP 2509-182** Maintenance therapy after allogeneic HCT in older adults with high-risk myeloid malignancies to reduce relapse rates and improve outcomes. (R Jayani-Kosarzycki/ A Kassim). ***Dropped due to need of supplemental data.***
cc. **PROP 2509-193** Comparative Outcomes of Second Allogeneic Transplantation Versus Donor Lymphocyte Infusion for Relapsed Myeloid Malignancies After Allo-HSCT (X Bi) ***Dropped due to low scientific impact.***
dd. **PROP 2509-196** Outcomes and Prognostic Factors in Adult Acute Lymphoblastic Leukemia Patients Relapsing After Allogeneic Hematopoietic Stem Cell Transplantation: Comparative Effectiveness of Second Transplant versus Donor Lymphocyte Infusion (U Gergis). ***Dropped due to low scientific impact.***
ee. **PROP 2509-199** Optimal reduced intensity conditioning (RIC) regimen with posttransplant cyclophosphamide (PTCy) for adults with AML/MDS undergoing first allogeneic hematopoietic cell transplant (alloHCT) (C Shultz/ T Juranovic). ***Dropped due to overlap with current study/publication.***

ff. **PROP 2509-202** Optimal myeloablative conditioning regimen (MAC) with posttransplant cyclophosphamide (PTCy) for adults with AML/MDS undergoing first allogeneic hematopoetic cell transplant (alloHCT) (C Shultz/ T Juranovic). ***Dropped due to overlap with current study/publication.***

gg. **PROP 2509-203** Allogeneic hematopoietic cell transplantation for systemic mastocytosis (Z Gahvari/ N Callander). ***Dropped due to overlap with current study/publication.***

hh. **PROP 2509-205** Use of TKI maintenance following allogeneic stem cell transplant in the AYA population with Ph+ B-ALL (R Walia/ S Giralt). ***Dropped due to need of supplemental data.***

ii. **PROP 2509-207** Real-World Feasibility, Safety, and Outcomes of Post-Transplant FLT3-Inhibitor Maintenance in AML: A CIBMTR Analysis (A Ambinder). ***Dropped due to need of supplemental data.***

jj. **PROP 2509-211** Outcomes following blinatumomab as a bridge to allogeneic HCT in B-ALL (R Walia/ A Jakubowski). ***Dropped due to overlap with current study/publication.***

kk. **PROP 2509-213** Impact of pre-transplant remission induction strategies for patients with B-cell acute lymphoblastic leukemia undergoing allogeneic hematopoietic cell transplantation (M Hyder/ C Kanakry). ***Dropped due to overlap with current study/publication.***

ll. **PROP 2509-215** Allogeneic Hematopoietic Cell Transplantation Outcomes in Patients with Systemic Mastocytosis Associated with Myeloid Leukemia: An Updated CIBMTR Analysis (M Kulasekaran/ G Hildebrandt). ***Dropped due to small sample size.***

mm. **PROP 2509-228** Impact of conditioning intensity on allogeneic transplant outcomes in acute lymphoblastic leukemia patients who previously received CAR T-cell therapy (S Tsai). ***Dropped due to overlap with current study/publication.***

nn. **PROP 2509-232** Outcomes of cord blood transplant vs. PTCY-based alloHCT among older adults (>60 years of age) with AML who are in complete remission, in the context of MRD status at transplant. (S Manjappa). ***Dropped due to need of supplemental data.***

6. Other business

MINUTES
CIBMTR WORKING COMMITTEE FOR ACUTE LEUKEMIA
Honolulu, HI
Thursday, February 13, 2025, 1:00 – 3:00 PM HST

Co-Chair:	**Filippo Milano, MD, PhD; Fred Hutchinson Cancer Center, Seattle, WA; Telephone: 206-667-5925; E-mail: fmilano@fredhutch.org**
Co-Chair:	**Veronika Bachanova, MD, PhD; University of Minnesota, Minneapolis, MN; Telephone: 612-625-5469; E-mail: bach0713@umn.edu**
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Statistician:	**Wentong Liu, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; E-mail: dylanliu@mcw.edu**

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1. **Introduction**
   a. Minutes from February 2024 (Attachment 1)
   b. Introduction of incoming co-chair:
      Lori Muffly, MD; Stanford Health Care
   c. Acknowledgement of outgoing co-chair:
      Filippo Milano, MD; Fred Hutchinson Cancer Center
   d. Page Scholar participant
      Mariam Nawas, MD; The University of Chicago Medicine

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

3. **Presentations, Publications or Submitted papers**
   a. **LK19-02** Evolving significance of Ph-positive status on ALL post-transplant outcomes in the TKI era (M Krem / R Maziarz). ***Submitted.***

b. **LK20-02** Impact of Germline RUNX1 Mutations on Allogeneic Hematopoietic Stem Cell Transplant Outcomes in AML: A CIBMTR Analysis (L Cunningham). ***Oral Presentation, EBMT 2024.***
c. **LK21-01a** Pre-Allogeneic Transplantation Flow Cytometry Testing For Patients With AML In First CR, As Currently Performed, Has Limited Clinical Utility For Relapse And Survival Prediction: A CIBMTR Analysis (F El Chaer). ***Oral Presentation, EHA 2024.***
d. **LK21-01b** Measurable Residual NPM1 before Allogeneic Transplant for Acute Myeloid Leukemia (L Dillon/ C Hourigan). ***Poster Presentation, ASH 2024.***
e. **LK21-01d** Dillon LW, Gui G, Ravindra N, Andrew G, Mukherjee D, Wong ZC, Huang Y, Gerhold J, Holman M, D'Angelo J, Miller J, Higgins J, Salk JJ, Auletta JJ, El Chaer F, Devine SM, Jimenez-Jimenez AM, De Lima MJG, Litzow MR, Kebriaei P, Saber W, Spellman SR, Zeger SL, Page KM, Hourigan CS. Measurable residual FLT3 internal tandem duplication before allogeneic transplant for acute myeloid leukemia. **JAMA Oncology. doi:10.1001/jamaoncol.2024.0985. Epub 2024 May 2. PMC11066770.**
f. **LK21-01e** Hegde PS, Andrew G, Gui G, Ravindra N, Mukherjee D, Wong ZC, Auletta JJ, El Chaer F, Corner A, Devine SM, Jimenez-Jimenez AM, De Lima MJG, Litzow MR, Kebriaei P, Saber W, Spellman SR, Zeger SL, Page KM, Dillon LW, Hourigan CS. Measurable residual FLT3 tyrosine kinase domain mutations before allogeneic transplant for acute myeloid leukemia. **Bone Marrow Transplantation. doi:10.1038/s41409-024-02444-7. Epub 2024 Oct 18.**
g. **LK21-01f** Gui G, Ravindra N, Hegde PS, Andrew G, Mukherjee D, Wong ZC, Auletta JJ, El Chaer F, Chen EC, Chen Y, Corner A, Devine SM, Iyer SG, Jimenez Jimenez AM, De Lima MJG, Litzow MR, Kebriaei P, Saber W, Spellman SR, Zeger SL, Page KM, Dillon LW, Hourigan CS. Measurable residual mutated IDH2 before allogeneic transplant for acute myeloid leukemia. **Bone Marrow Transplantation. doi:10.1038/s41409-024-02449-2. Epub 2024 Oct 25.**
h. **LK21-01g** Gui G, Ravindra N, Hegde PS, Andrew G, Mukherjee D, Wong ZC, Auletta JJ, El Chaer F, Chen EC, Chen Y, Corner A, Devine SM, Iyer SG, Jimenez Jimenez AM, De Lima MJG, Litzow MR, Kebriaei P, Saber W, Spellman SR, Zeger S, Page KM, Dillon LW, Hourigan CS. Measurable residual mutated IDH1 before allogeneic transplant for acute myeloid leukemia. **Bone Marrow Transplantation. doi:10.1038/s41409-024-02447-4. Epub 2024 Nov 6.**

4. Studies in progress (Attachment 3)

a. **LK20-01** Acute myeloid leukemia with chromosome 17 abnormalities with or without TP53 abnormalities and outcomes after hematopoietic stem cell transplantation (A Dias/J Yared). **Data File Preparation.**
b. **LK20-02** Outcomes of allogeneic hematopoietic cell transplantation among germline RUNX1 mutation carriers with acute myeloid leukemia (P Liu/L Cunningham). **Manuscript Preparation.**
c. **LK20-03** Evaluating outcomes of allogeneic hematopoietic cell transplantation in T-cell acute lymphoblastic leukemia (H Murthy/M Iqbal/M Kharfan-Dabaja). **Data File Preparation.**
d. **LK21-01a** Impact of measurable residual disease status on outcomes of acute myeloid leukemia and patients 18-65 years old in first complete remission undergoing allogeneic hematopoietic cell transplantation (F El Chaer/C Hourigan). **Manuscript Preparation**
e. **LK22-01** Impact of pre-allogeneic hematopoietic cell transplantation therapy in acute myeloid leukemia and myelodysplastic syndrome on post-transplant outcomes (Ali N). **Protocol Development**
f. **LK23-01** The impact of allogeneic stem cell transplantation on acute myeloid leukemia and myelodysplastic syndrome with chromosome 3 abnormalities (A Datt Law). **Protocol Development.**

g. **LK23-02** Prognostic impact of cytogenetic and molecular risk classification in AML after hematopoietic stem cell transplant in adolescents and young adults (H Lust). **Protocol Development.**
h. **LK23-03** Impact of donor source in second allogeneic hematopoietic cell transplant in patients with acute leukemia/MDS who relapsed after prior allograft during the current era (2014-2020) (A Troullioud Lucas/ A Scaradavou). **Protocol Development.**
i. **LK24-01** Sequencing of chimeric antigen receptor T-cell therapy and allogeneic transplantation in adult patients with B-cell acute lymphoblastic leukemia (D Eng/ J Fein/ A Arteaga/ Luis Gonzalez Mosquera/ Kitsada Wudhikarn/ Muhammad Bilal Abid/ Abu-Sayeef Mirza). **Protocol Development**

5. Future/proposed studies

Proposed Studies to be presented for consideration at the Tandem WC Meeting

a. **PROP 2408-04** Outcomes after transplant in acute myeloid leukemia with t(6;9) (p23;q34) translocation (F Andreozzi) (Attachment 4)

Dr. Fabio Andreozzi presented.

- ***Key Points****:*
  - *AML with t(6;9)) is rare, accounting for 1-2% of cases.*
  - *Typically affects younger patients and is often chemotherapy resistant.*
  - *Study aims to assess outcomes post-transplant and correlate with various parameters like remission stage, HLA compatibility, conditioning intensity, FLT3-ITD mutations, and role of pre-transplant as well as post-transplant maintenance with FLT3 inhibitors.*
  - *Inclusion criteria: pediatric AML and MDS patients with t(6;9).*
  - *Classical endpoints: overall survival, incidence of relapse, non-relapse mortality, and graft-versus-host disease.*
  - *219 patients were identified from 2008 to 2019. Median age 37 and most were in CR1.*
  - *Key discussion points: 1) value of having a control arm, for e.g., those with FLT3 mutation but without t(6;9); 2) this question could have been pursued in other studies already and is not clear how the results will impact the practice; 3) EBMT already published on outcomes of these patients; 4) data availability and completeness in CIBMTR regarding FLT3 inhibitors use pre and post HCT*

b. **PROP 2408-06** Efficacy of hypomethylating agent/Venetoclax with or without donor lymphocyte infusion as management of post-transplant relapse acute myeloid leukemia and myelodysplastic syndrome (M Dandwani/ K Poonsombudlert) (Attachment 5)

Dr. Dandwani presented.

- ***Key Points****:*
  - *Study evaluates if adding DLI to hypomethylating agents and Venetoclax improves overall response rate and survival.*
  - *Focus on incidence of graft-versus-host disease, veno-occlusive disease, and hematological toxicity.*

- *Real-world evidence shows mixed results*
- *Key discussion points: 1) Most clinicians would tend to give DLI anyway or proceed to 2nd HCT; 2) concerns regarding selection bias among those chosen to get DLI vs. those who did not get DLI, and given retrospective nature, this will be hard to control; 3) heterogeneity in practice patterns among different centers*

c. **PROP 2410-06** Comparison of FluFTBI and other myeloablative Conditioning Regimens for Haploidentical and mismatched unrelated Hematopoietic Cell Transplant with Post-Transplant Cyclophosphamide in Patients with Acute Leukemia (S Arslan/ M Al Malki) (Attachment 6)

Dr. Arslan presented.

- ***Key Points****:*
  - *Evaluates outcomes of Fludarabine and TBI conditioning versus other myeloablative regimens.*
  - *Hypothesis: Fludarabine and TBI combinations may offer better outcomes.*
  - *Inclusion criteria: AML and ALL patients aged 18-60, undergoing haploidentical or mismatched unrelated transplants.*
  - *Large data set available for analysis.*
  - *Key discussion points: 1) concern whether TBI is mostly used with ALL rather than AML; 2) EBMT already published a similar study; 3) heterogeneity in regimens in control arm*

d. **PROP 2410-08; 2410-214; 2410-222** Survival Outcomes after allogeneic transplantation in Ph-like B-ALL (M Iqbal/ M Kharfan-Dabaja/ L Mendez/ L Gowda/ K V Nadiminti/ C Junge) (Attachment 7)

Dr. Chase Junge presented.

- ***Key Points****:*
  - *Compares outcomes in Ph-like ALL to Philadelphia positive and negative ALL.*
  - *Focus on overall survival, progression-free survival, and impact of novel immunotherapies.*
  - *Large cohort available for analysis, with stratification by age groups.*
  - *Key discussion points: 1) how Ph-like B-cell ALL is actually diagnosed is challenging across centers; 2) MRD data in CIBMTR forms have significant limitations; 3) how will results impact practice; 4) no value of including patients prior to 2014 because they did not have access to blinatumomab*

e. **PROP 2410-28** Comparison of reduced-intensity hematopoietic cell transplantation with CAR T cell therapy in patients age > 60 years with acute lymphoblastic leukemia (J Behman/ R Faramand) (Attachment 8)

Dr. John Behman presented.

- ***Key Points****:*
  - *Hypothesis: CAR T-cell therapy may offer improved survival compared to reduced intensity conditioning.*
  - *Focus on leukemia-free survival, MRD negativity, and treatment-related mortality.*

- *Data set includes patients aged 60 and older.*
- *Key discussion points: 1) the HCT cohort is mostly CR while the CAR-T cohort is mostly relapsed disease patients; 2) median FU among CAR-T patients is short*

f. **PROP 2410-70** Clinical Outcomes of Patients with Acute Lymphoblastic Leukemia with Measurable Residual Disease Who Receive CAR-T Cell Therapy vs Allogeneic Stem Cell Transplantation (G Sanchez-Petitto/ M de Lima) (Attachment 9)

Dr. Sanchez-Petitto presented.

- *Key Points:*
  - *What is the effectiveness of CAR-T cell therapy compared to allogenic stem cell transplant, in treating MRD positive patients with B-cell ALL*
  - *We hypothesize that for those patients who are 35 year old or older, or who have high cytogenetic risk, or advanced disease, allogenic transplant provides better outcomes.*
  - *Key discussion points: 1) CAR-T cohort is mostly comprised of pediatric and AYA patients, while allo-HCT cohort is mostly comprised of adult patients; 2) not clear how to handle post CAR allo-HCT; 3) how complete the data on blinatumomab is in CIBMTR*

g. **PROP 2410-199** Optimal Reduced Intensity Conditioning Regimen for Allogeneic Transplant in Measurable Residual Disease (MRD) Positive Acute Myeloid Leukemia (R Ramlal/ N Bejanyan) (Attachment 10)

Dr. Ramlal presented.

- *Key Points:*
  - *Hypothesis: Fludarabine and Melphalan may offer the best outcomes.*
  - *Focus on overall survival, leukemia-free survival, and relapse rates.*
  - *Large data set available for analysis.*
  - *Key points: 1) heterogeneity in regimens is a concern; 2) MRD definition; 3) completeness of post HCT maintenance therapies is a concern*

h. **PROP 2410-225** Comparison of myeloablative versus reduced intensity conditioning regimens in patients with Acute Lymphoblastic Leukemia achieving an MRD negative remission prior to allogeneic hematopoietic stem cell transplant (X Bi/ U Gergis) (Attachment 11)

Dr. Xia Bi presented.

- *Key Points:*
  - *Hypothesis: Reduced intensity conditioning may offer comparable outcomes with less toxicity.*
  - *Focus on overall survival, leukemia-free survival, and relapse rates.*
  - *Large data set available for analysis.*
  - *Key issues: 1) MRD data quality; 2) for PH +ve ALL, how complete the CIBMTR data on use of post HCT TKI is a concern*

i. **PROP 2410-259** Machine Learning–Based Model Development to Predict Acute Myeloid Leukemia Relapse after Allogeneic Transplantation (N Bejanyan/ G Valdes) (Attachment 12)

Dr. Nelli Bejanyan presented.

- *Key Points:*
  - *Aim to establish and validate a machine learning model using pre- and post-transplant covariates.*
  - *Focus on relapse, overall survival, and leukemia-free survival.*
  - *Large data set available for analysis.*
  - *Key issues: 1) impact on practice*

j. **PROP 2410-52; 2410-227** Determining optimal conosolidation for precursor Bcell Acute lymphoblastic leukemia in CR1. Comparing allogeneic hematopoietic cell transplantation to blinatumumab consolidation. A ECOG/CIBMTR comparitive study. (H S. Murthy/ M Litzow/ L Gowda/ K Chetlapalli) (Attachment 13)

Dr. Murthy presented.

- *Key Points:*
  - *Compares blinatumomab consolidation to allogeneic transplant.*
  - *Focus on overall survival, progression-free survival, and relapse rates.*
  - *Data set includes patients from the E1910 study and CIBMTR*
  - *Key issues: 1) true denominator is different between the two cohorts; 2) MRD data quality; 3) comparing RCT participants to real world evidence can be problematic*

Proposed studies; not accepted for consideration at this time

k. **PROP 2312-02** Do European Leukemia Net (ELN) 2017,2022 add to the Prognostic value of Disease Risk Index in Acute Myeloid Leukemia (AML) Patients in First Complete Remission who undergo Allogeneic Hematopoietic Stem Cell Transplant (A Masurekar). ***Dropped due to overlap with current study/publication.***
l. **PROP 2404-01** Maintenance Therapy after Allogeneic Stem Cell Transplant in Acute Myeloid Leukemia (A Sperotto/ M Gottardi). ***Dropped due to supplemental data needed.***
m. **PROP 2405-01** Real World Utilization Rates of Central Nervous System (CNS) Radiotherapy (RT) in Adult Acute Lymphoid Leukemia (ALL) (L Ballas/ S Zhang). ***Dropped due to small sample size and supplemental data needed.***
n. **PROP 2405-02** Outcomes of Ph+ ALL in CR1 MRD- status in the PostCy/RIC ERA (J Behman). ***Dropped due to overlap with current study/publication.***
o. **PROP 2408-03** Impact of post-transplant blinatumomab maintenance on outcomes of patients with B-cell acute lymphoblastic leukemia (P Vittayawacharin/ S Cirurea). ***Dropped due to low scientific impact.***
p. **PROP 2408-05** Looking beyond the HLA barrier; use of alternative donors for adverse risk acute myeloid leukemia (A Vegel/ K Poonsombudlert). ***Dropped due to overlap with current study/publication.***
q. **PROP 2409-07** Does prophylactic use of defibrotide lead to less incidence of TA-TMA (Y Choi). ***Dropped due to incomplete data in the CIBMTR database and need for supplemental data collection.***

r. **PROP 2409-08** Evaluation of Post-transplant Cyclophosphamide vs Calcineurin + Methotrexate Based Graft Versus Host Disease Prophylaxis in Acute Lymphoblastic Leukemia Patients (J Behman/ T Nishihori). ***Dropped due to overlap with current study/publication.***
s. **PROP 2409-09** Time to Allogeneic Transplant in Acute Myeloid Leukemia: Does it matter? (A Masurekar). ***Dropped due to overlap with current study/publication.***
t. **PROP 2410-05** Early donor chimerism is predictive of relapse and survival following allogeneic hematopoietic stem cell transplantation (P Munshi/ N Hossain). ***Dropped due to overlap with current study/publication.***
u. **PROP 2410-26** Real world data of SCT on TALL in the modern era (S Srikantan/ S Farhan). ***Dropped due to overlap with current study/publication.***
v. **PROP 2410-29** Mixed Donor Chimerism and its Impact on Relapse Rates and Relapse-Free Survival in Patients with Acute Leukemias Receiving PTCy versus Methotrexate-based GVHD Prophylaxis (C Graham/ H Alkhateeb). ***Dropped due to overlap with current study/publication.***
w. **PROP 2410-36** Biological Characteristics and Survival Outcomes in TP53-mutated Myelodysplastic Syndrome and Acute Myeloid Leukemia Patients Undergoing Allogeneic Stem Cell Transplantation: A CIBMTR Study (P Ramadas/ A Ananthaneni). ***Dropped due to overlap with current study/publication.***
x. **PROP 2410-50** Analyzing the Impact of Co-Mutations and Cytogenetics on Transplant Outcomes in NPM1- Mutated AML Using Machine Learning Models (J Wang/ M de Lima). ***Dropped due to overlap with current study/publication.***
y. **PROP 2410-62** Best Donor Type for Allogeneic Hematopoietic Cell Transplantation in High-Risk Acute Leukemia and Myelodysplastic Syndrome: Optimally Selected Haploidentical Donor, Double Unrelated Cord Blood or Matched Unrelated Donor? (G Fatobene/ V Rocha). ***Dropped due to overlap with current study/publication.***
z. **PROP 2410-81** Outcomes of Matched and mismatched unrelated allogeneic stem cell transplantation using posttransplant cyclophosphamide versus tacrolimus and methotrexate in patients with acute myeloid leukemia and myelodysplastic syndrome with TP 53 mutation and/or del(17p)/-17 (F Socola/ B Jonas). ***Dropped due to overlap with current study/publication.***
aa. **PROP 2410-84** Outcomes and Predictors of outcomes of adult patients with therapy-related acute lymphoblastic leukemia after allogeneic hematopoietic stem transplantation (R Nampoothir). ***Dropped due to limited data available in the CIBMTR database.***
bb. **PROP 2410-121** Prophylactic and preemptive donor lymphocyte infusion alone or in combination with hypomethylating agents after allogeneic stem cell transplantation for acute myeloid leukemia and myelodysplastic syndrome (N Tijaro Ovalle/ S Giralt). ***Dropped due small sample size.***
cc. **PROP 2410-130** Comparison of myeloablative versus reduced intensity conditioning regimens in patients with AML achieving an MRD negative remission prior to allogeneic hematopoietic stem cell transplant (X Bi/ U Gergis). ***Dropped due to overlap with current study/publication.***
dd. **PROP 2410-134** Comparison of outcomes between haploidentical, matched sibling, matched unrelated, and mismatched unrelated donor hematopoietic cell transplantation with post-transplant cyclophosphamide, mycophenolate mofetil, and a calcineurin inhibitor graft-versus-host disease prophylaxis in patients with acute myeloid leukemia (A Wofford/ M Wieduwilt). ***Dropped due to overlap with current study/publication.***
ee. **PROP 2410-155** Outcomes of allogeneic hematopoietic cell transplantation for de novo philadelphia chromosome-positive acute myeloid leukemia (N Sumransub/ M Juckett). ***Dropped due to limited availability of data in the CIBMTR database and small sample size.***

ff. **PROP 2410-156** Benefit of planned allogeneic stem cell transplant after CART cell therapy for B cell Acute lymphoblastic leukemia (R Nampoothiri/ N Kekre). ***Dropped due to overlap with current study/publication.***

gg. **PROP 2410-177** Allogeneic hematopoietic cell transplantation for patients with nucleophosmin (NPM1) mutant acute myeloid leukemia (AML) (L Gowda/ V Bhatt). ***Dropped due to overlap with current study/publication.***

hh. **PROP 2410-180** Impact of Clonal Evolution in Post-Transplantation Relapsed Myeloid Neoplasms (L Williams/ C Lai). ***Dropped due to small sample size.***

ii. **PROP 2410-184** Second Allogeneic Stem Cell Transplantation in Relapsed Myeloid Malignancies: Clinical Outcomes and Prognostic Insights (M Alhomoud/ B Shaffer). ***Dropped due to overlap with current study/publication.***

jj. **PROP 2410-190** Outcomes of T-Cell Depleted Allogeneic Stem Cell Transplant in Acute Myeloid Leukemia and High-Risk Myelodysplastic Syndrome (J L Reagan/ M R Christopher). ***Dropped due to overlap with current study/publication.***

kk. **PROP 2410-191** Characteristics and Post-Transplant Outcomes of Patients with Core-Binding Factor Acute Myeloid Leukemia (J L Reagan/ M R Christopher). ***Dropped due to low scientific impact and small sample size.***

ll. **PROP 2410-195** Maintenance Tyrosine Kinase Inhibitors Following Allogeneic Hematopoietic Cell Transplantation in Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia (Ph+ ALL) (J L Reagan/ M R Christopher). ***Dropped due to overlap with current study/publication.***

mm. **PROP 2410-202** Impact of pre-allogeneic stem cell transplantation salvage therapy in adult patients with relapsed and/or refractory (R/R) FLT3 internal tandem duplication (FLT3-ITD) acute myeloid leukemia on post-transplant outcomes (R Mohty/ M Kharfan-Dabaja). ***Dropped due to low scientific impact.***

nn. **PROP 2410-203** Outcomes of T-Cell Depleted Hematopoietic Stem Cell Transplant in Acute Myeloid Leukemia and High-Risk Myelodysplastic Syndrome (J L Reagan/ M R Christopher). ***Dropped due to overlap with current study/publication.***

oo. **PROP 2410-208** Impact of CD19-directed CAR T Dose on Outcomes in Relapsed/Refractory B-acute Lymphoblastic Leukemia (K McNerney/ L Schultz). ***Dropped due to low scientific impact.***

pp. **PROP 2410-220** Impact of Conditioning Intensity and Regimens Across Donor Types and GVHD Prophylactic Platforms in Adults with B-cell ALL Undergoing Allogeneic Hematopoietic Cell Transplantation (M Abid/ M Aljurf). ***Dropped due to overlap with current study/publication.***

qq. **PROP 2410-230** Impact of induction regimen intensity on post- allogeneic hematopoietic cell transplantation (allo-HCT) outcomes in older (age>=60) patients with acute myeloid leukemia (R Mohty/ M Kharfan-Dabaja). ***Dropped due to overlap with current study/publication.***

rr. **PROP 2410-231** Real World Analysis of the use of Maintenance Chemotherapy using Low-Dose HMA Agents in patients with Acute Leukemia and MDS to decrease the Risk of Relapse (C Graham). ***Dropped due to incomplete data in the CIBMTR database and supplemental data needed.***

ss. **PROP 2410-235** Outcomes of Allogeneic Hematopoietic Cell Transplantation for NF1-Mutated Myeloid Neoplasms (MDS and AML) (S Mirza). ***Dropped due to limited data available in the CIBMTR database and supplemental data needed.***

tt. **PROP 2410-247** Early versus late post-transplant maintenance for Patients with high-risk AML (S Mirza/ N Bejanyan). ***Dropped due to incomplete data in the CIBMTR database and supplemental data needed.***

uu. **PROP 2410-255;256** Outcomes of Flu/Bu Vs. Bu/Cy in adults with AML undergoing myeloablative allogeneic HCT for AML in morphologic remission with measurable residual disease (S Manjappa/ R B Walter). ***Dropped due to low scientific impact.***

ww. **PROP 2410-257** To compare the outcomes of different pre-transplant salvage regimens (FLT3i

combination therapy, conventional chemotherapy) in R/R FLT3mut AML (A R Kurup/ H Sibai). ***Dropped due to low scientific impact.***

xx. **PROP 2410**-**262** Evaluating Outcomes in Elderly Patients Undergoing Allogeneic Bone Marrow Transplant (BMT) with Different Pre-Transplant Treatment Regimens (A R Kurup/ H Sibai). ***Dropped due to overlap with current study/publication.***

yy. **PROP 2410**-**265** Impact of Transplant Characteristics on Outcomes in HCT for AML Patients in CRi (E Krieger/ A Toor). ***Dropped due to data available in the CIBMTR database and supplemental data needed.***

6. Other business

MINUTES
CIBMTR WORKING COMMITTEE FOR CHRONIC LEUKEMIA
Honolulu, HI
Saturday, February 15, 2025, 1:00 – 3:00 PM HST

Co-Chair:	**Michael Grunwald, MD; Levine Cancer Institute, Charlotte, NC; Phone: 980-442-5125; Email: Michael.grunwald@carolinashealthcare.org**
Co-Chair:	**Betul Oran, MD, MS; MD Anderson Cancer Center, Houston, TX; Phone: 713-745-2820; Email: boran@mdanderson.org**
Co-Chair:	**Mark Juckett, MD; University of Minnesota, Minneapolis, MN; Phone: 612-625-8942; E-mail: juck0001@umn.edu**
Page Scholar	**Hany Elmariah, MD, MS; Stanford University, Stanford, CA; Email: he3@stanford.edu**
Scientific Director:	**Wael Saber, MD, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Phone: 414-805-0677; Email: wsaber@mcw.edu**
Statistical Director:	**Soyoung Kim, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Phone: 414-955-8271; Email: skim@mcw.edu**
Statistician:	**Charimar Santiago Parrilla, MPH; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; E-mail: csantiago@mcw.edu**

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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. **CK21-01** Jain T, Estrada-Merly N, Queralt Salas M, Kim S, DeVos J, Chen M, Fang X, Kumar R, Andrade Campos M, Elmariah H, Agrawal V, Aljurf M, Ulrike Bacher V, Badar T, Badawy S, Ballen K, Beitinjaneh A, Bhatt V, Bredeson C, DeFilipp Z, Dholaria B, Farhadfar N, Farhan S, Gandhi A, Ganguly S, Gergis U, Grunwald M, Hamad N, Hamilton B, Inamoto Y, Iqbal M, Jamy O, Juckett M, Kharfan-Dabaja MA, Krem M, Lad D, Liesveld J, Al Malki M, Malone AK, Murthy H, Ortí G, Patel S, Pawarode A, Perales M, van der Poel M, Ringden O, Rizzieri D, Rovo A, Savani B, Savoie M, Seo S, Solh M, Ustun C, Verdonck L, Wingard J, Wirk B, Bejanyan N, Jones R, Nishihori T, Oran B, Nakamura R, Scott B, Saber W, Gupta V. Donor types and outcomes of transplantation in myelofibrosis: A CIBMTR study. ***Blood Advances. 2024 Aug 27; 8(16):4281-4293. doi:10.1182/bloodadvances.2024013451. Epub 2024 Jun 25. PMC11372592.***
   b. **GS19-02** Graft failure in MDS and acute leukemia patients after allogeneic stem cell transplantation receiving post transplant cyclophosphamide (M Krem/ R Maziarz). ***Submitted.***

4. **Studies in progress** (Attachment 3)

a. **CK16-01b** Identification of germline predisposition mutations in young myelodysplastic syndrome patients (L Godley). **Analysis.**
b. **CK22-01** Impact of somatic mutations on outcomes after allogeneic hematopoietic cell transplantation in patients with myelodysplastic syndrome with ring sideroblasts (MDS-RS) and MDS/myeloproliferative neoplasm with RS and thrombocytosis (MDS/MPN-RS-T) (S Arslan/ R Nakamura). **Protocol Development.**
c. **CK22-02** Toxicity and survival of AML/MDS patients receiving allogeneic stem cell transplantation using reduced-intensity conditioning: A propensity score analysis. (P Kongtim/ A Portuguese/ S Ciurea/ B Scott). **Data File Preparation.**
d. **CK23-01** Identifying the Optimal Graft-versus-Host Disease Regimen in Allogeneic Transplantation for Myelofibrosis (S Patel/ D Courier). **Protocol Received.**
e. **CK23-02** The mutational landscape in Myelodysplastic Syndrome arising from Aplastic Anemia and its impact on Allogeneic Stem Cell Transplantation Outcomes (B Ball/ R Nakamura). **Protocol Received.**
f. **CK24-01** Identifying the optimal stem cell dosing for peripheral blood stem cell transplantation with post-transplant cyclophosphamide. (H Elmariah/ A Gandhi/ N Bejanyan/ R Marziarz). **Protocol Received.**
g. **CK24-02** Outcomes of allogeneic hematopoietic stem cell transplantation in patients with DDX41-mutated myelody splastic syndrome and acute myeloid leukemia. (R Stubbins/ E Wong/ L Fox/ L Gowda/ S Seropian). **Protocol Received.**
h. **CK24-03** Comparison of reduced intensity conditioning regimens for haploidentical donor hematopoietic cell transplant with post-transplant cyclophosphamide in patients with acute myeloid leukemia or myelodysplastic syndromes. (H Elmariah/ S Arslan/ M Al Malki/ N Bejanyan). **Protocol Development.**
i. **CK24-04** Comparison of post-transplant cyclophosphamide-based reduced intensity conditioning regimens for older patients with acute myelogenous leukemia and MDS. (L Bachier/ S Solomon). **Protocol Development.**

5. Future/proposed studies

a. **PROP 2410-22; 2410-178** Impact of Post-Transplant Cyclophosphamide on Outcomes in Patients with CMML Undergoing Allogeneic Stem Cell Transplant (Y Berry/ S Farhan/ I Varadarajan/ K Ballen) (Attachment 4)

Dr. Farhan's Presentation:

- ***Discussed the role of allogenic stem cell transplant in CMML****.*
- *Highlighted survival outcomes and the impact of GVHD prophylaxis with Ptcy.*
- *Research questions focused on the impact of Ptcy-based GVHD prophylaxis and advances in pre- and post-transplant care.*
- *Cohort includes 940 who got CNI-based GVHD prophylaxis and 458 who got Ptcy-based.*
- *Key issue raised: 1) (which risk stratification system will be used (e.g. CPSS or others)?); 2) impact of splenomegaly needs to be considered; 3) how to handle correlation between donor type (i.e. haploidentical donors) and use of Ptcy; 4) need to include rates of graft failure as an outcome*

b. **PROP 2402-01** Outcomes of Second Transplant for myelofibrosis (H Ali/ S Otoukesh) (Attachment 5)

Dr. Ali's Presentation:

- ***Evaluate outcomes of second transplant for myelofibrosis.***
- *Discussed survival, non-relapse mortality, and relapse risk.*
- *Highlighted the importance of patient selection, donor choices, and timing for second transplant.*
- *Key issue raised: 1) key factor to consider is time from 1st HCT to 2nd HCT; 2) disease phenotype at the time of 2nd HCT; 3) How is this analysis unique when compared to recent EBMT publication*

c. **PROP 2409-16; 2410-261** Propensity score matched analysis comparing survival by pre-transplant treatment in Myeloid Neoplasms in the venetoclax era (H Elmariah/ W Saber/ N Premnath/ M Juckett) (Attachment 6)

Dr. Premnath's Presentation:

- ***Comparison of survival by pre-transplant treatment in myeloid neoplasms in the Venetoclax era.***
- *The main hypothesis is venetoclax-based pre-transplant regimens, leads to superior outcomes in the post-allogeneic stem cell transplant compared to alternative first-line therapy.*
- *Focused on disease-free survival, overall survival, relapse, non-relapse mortality, and mixed donor chimerism.*
- *Propensity score-based matching for various factors.*
- *Key issue raised: 1) impact on practice; 2) what is included in the control arm; 3) heterogeneity is introduced by including AML and MDS and across all disease stages*

d. **PROP 2409-22** Myelodysplastic Neoplasms with Hypoplasia (MDS-h) or Fibrosis (MDS-f): Distinct Clinical Entities Compared to Other MDS Subtypes (A Law/ S Rodriguez Rodriguez) (Attachment 7)

Dr. Sergio Rodriguez's Presentation:

- ***Discussed myelodysplastic neoplasms with hypoplasia or fibrosis.***
- *Highlighted differences in survival and engraftment outcomes compared to other MDS subtypes.*
- *Focused on overall survival, platelet engraftment, graft failure, relapse, non-relapse mortality, and GVHD.*
- *Key issue raised: 1) accuracy of reporting of the histology; 2) availabilities of molecular data; 3) impact of PNH clone size*

e. **PROP 2410-17** Evaluating transplant outcome in high risk chronic phase CML (Z Gong/ Y Lei) (Attachment 8)

Dr. Zimu Gong's Presentation:

- ***Evaluated transplant outcome in high-risk chronic phase CML.***
- *Discussed the impact of cytogenetic aberrations and the timing of transplant.*
- *Focused on overall survival, relapse-free survival, and GRFS.*
- *Key issue raised: Availability of variables needed to define "high risk" disease, is a concern; molecular data; details of TKI therapies; more details are needed on the control arm (non-transplanted patients)*

f. **PROP 2410-46; 2410-167** Outcomes of Patients with CLL/SLL Who Receive Allogeneic Hematopoietic Stem Cell Transplant in the Modern Era of Therapies (A Kittai/ S Jaglowski/ J Huang/ M Shadman) (Attachment 9)

Dr. Jennifer Wong's Presentation:

- ***Discussed outcomes of patients with CLL/SLL who receive allogeneic hematopoietic stem cell transplant in the modern era of therapies****.*
- *Focusing on progression-free survival, overall survival, relapse, non-relapse mortality, and GVHD.*
- *Focused on the impact of targeted therapies and chemoimmunotherapy pre-HCT on HCT outcomes.*
- *Key issue raised: Lead time bias is a concern*

g. **PROP 2410-148** An international study comparing the efficacy and utility of anti-CD19 CAR-T versus allogeneic stem cell transplantation for Richter Transformation (A Kittai/ J Woyach) (Attachment 10)

Dr. Adam Katai's Presentation:

- *Compared the efficacy and utility of anti-CD19 CAR T versus allogeneic transplant for Richter's transformation.*
- *Discussed overall survival, progression-free survival, non-relapse mortality, and safety.*
- *Highlighted the importance of matching patients based on prior treatments and response status.*
- *Questions and Comments:*
    - *Participants raised questions about the impact of pre-transplant treatments, the role of molecular genetics, and the feasibility of including certain patient populations.*
    - *Discussions on the importance of matching patients based on various factors and the potential impact of newer therapies on transplant outcomes.*

Proposed studies; not accepted for consideration at this time

i. **PROP 2409-18** The Impact of Pre-Transplant JAK Inhibition on Outcomes in Allogeneic Stem Cell Transplant for Myelofibrosis (A Ali). ***Dropped due to overlap with current study/publication.***

j. **PROP 2409-25** Outcomes of GATA2+ MDS transplants in the PTCy era (N Hossain/ P Munshi). ***Dropped due to small sample size.***

k. **PROP 2410-04** Factors associated with survival following allogeneic transplant for TP53-mutated myelodysplastic syndrome (M Shah/ G Murthy). ***Dropped due to small sample size.***

l. **PROP 2410-25** Impact of BTKi pre and post CAR T-cell therapy (S Srikantan/ S Farhan). ***Dropped due to small sample size.***
m. **PROP 2410-60** Risk Factors for Graft Failure following Allogeneic Hematopoietic Cell Transplantation in Patients With BCR-ABL negative Myeloproliferative neoplasms (R Mishra/ T Jain). ***Dropped due to overlap with current study/publication.***
n. **PROP 2410-79** Impact of Tacrolimus-Methotrexate Versus Post-transplant Cyclophosphamide on Engraftment, Graft Failure, and GVHD Prevention in Myelofibrosis Patients Undergoing Allogeneic Hematopoietic Cell Transplantation (M Pandey/ A Ashraf). ***Dropped due to overlap with current study/publication.***
o. **PROP 2410-89** The real-world and associated factors of outcomes of relapsed/refractory CLL treated with standard-of-care lisocabtagene maraleucel (E Bezerra/ A Kittai). ***Dropped due to small sample size.***
p. **PROP2410-101** Risk Factors for Treatment Failure Post-Allogeneic Hematopoietic Cell Transplantation for Myelofibrosis (A Trunk/ C Brunstein). ***Dropped due to overlap with current study/publication.***
q. **PROP 2410-123** Impact of post-transplant cyclophosphamide (PT-Cy)-based prophylaxis in matched sibling and matched unrelated donors for patients older than 60-years-old with myelodysplastic syndrome. (W Chai-Ho/ G Schiller). ***Dropped due to overlap with current study/publication.***
r. **PROP 2410-145** Trends in Utilization of Allogeneic Stem Cell Transplant in the Treatment of Myelodysplastic Syndrome (N Punwani). ***Dropped due to overlap with current study/publication.***
s. **PROP 2410-149** Prognostic impact of IPSS-M relative to IPSS-R in the era of modern MDS treatments for allogeneic HCT (P Munshi/ K Pratz). ***Dropped due to supplemental data needed.***
t. **PROP 2410-152** Describing allogeneic transplant outcomes in patients with myelofibrosis who undergo pre-transplant treatment with hypomethylating agents. (A Vartanov). ***Dropped due to small sample size.***
u. **PROP 2410-193** Impact of Ruxolitinib on GVHD and Overall Survival in Patients with Myelofibrosis Following Allogeneic Stem Cell Transplant (J L Reagan, M R Christopher). ***Dropped due to overlap with current study/publication.***
v. **PROP 2410-237** Comprehensive CIBMTR Analysis of Post-Allogeneic Transplant Treatment with Azacitidine in Chronic Myelomonocytic Leukemia (M Kulasekaran/ G Hildebrandt). ***Dropped due to small sample size.***

6. Other business

Accrual Summary for the Acute Leukemia Working Committee

Characteristics of recipients of first allogeneic transplants for AML, ALL, CLL, CML, MDS and MFS reported to the CIBMTR between 2008 and 2025

Characteristic	AML	ALL	CLL	CML	MDS	MFS
Number of patients	59478	25861	2727	5029	20904	5903
No. of centers	416	409	222	337	384	271
Age, by decades, no. (%)						
Median (range)	53.9 (0.3-87.8)	31.1 (0.2-81.8)	57.8 (3.9-76.0)	44.1 (1.3-77.6)	61.4 (0.4-83.4)	61.6 (0.5-80.8)
0-9	2743 (4.6)	3437 (13.3)	1 (0.0)	99 (2.0)	470 (2.2)	18 (0.3)
10-19	3236 (5.4)	4320 (16.7)	1 (0.0)	349 (6.9)	674 (3.2)	18 (0.3)
20-29	4529 (7.6)	4788 (18.5)	21 (0.8)	627 (12.5)	642 (3.1)	33 (0.6)
30-39	5937 (10.0)	3620 (14.0)	74 (2.7)	963 (19.1)	927 (4.4)	146 (2.5)
40-49	8479 (14.3)	3646 (14.1)	424 (15.5)	1201 (23.9)	1904 (9.1)	603 (10.2)
50-59	13619 (22.9)	3537 (13.7)	1153 (42.3)	1164 (23.1)	4841 (23.2)	1715 (29.1)
60-69	16464 (27.7)	2232 (8.6)	958 (35.1)	554 (11.0)	8612 (41.2)	2766 (46.9)
70+	4471 (7.5)	281 (1.1)	95 (3.5)	72 (1.4)	2834 (13.6)	604 (10.2)
TED or RES (RF) track determined for this event, no. (%)						
Ted (registration) patient	47070 (79.1)	20891 (80.8)	1972 (72.3)	4029 (80.1)	14013 (67.0)	2356 (39.9)
cRF (Research) patient	12408 (20.9)	4970 (19.2)	755 (27.7)	1000 (19.9)	6891 (33.0)	3547 (60.1)
Sex, no. (%)						
Male	32043 (53.9)	15380 (59.5)	1972 (72.3)	3037 (60.4)	12999 (62.2)	3451 (58.5)
Female	27435 (46.1)	10481 (40.5)	755 (27.7)	1992 (39.6)	7905 (37.8)	2452 (41.5)
HCT-CI, no. (%)						
0	17937 (30.2)	10473 (40.5)	1013 (37.1)	2028 (40.3)	5423 (25.9)	1575 (26.7)
1	9123 (15.3)	4040 (15.6)	439 (16.1)	746 (14.8)	2826 (13.5)	869 (14.7)
2	7936 (13.3)	3208 (12.4)	373 (13.7)	638 (12.7)	2564 (12.3)	866 (14.7)
3	8992 (15.1)	3280 (12.7)	328 (12.0)	659 (13.1)	3413 (16.3)	1018 (17.2)
4	5879 (9.9)	1965 (7.6)	217 (8.0)	404 (8.0)	2180 (10.4)	647 (11.0)
5+	7413 (12.5)	1976 (7.6)	182 (6.7)	349 (6.9)	3694 (17.7)	773 (13.1)
Not reported	2198 (3.7)	919 (3.6)	175 (6.4)	205 (4.1)	804 (3.8)	155 (2.6)
What was the disease status (AML and ALL)?, no. (%)						
Primary induction failure	5904 (9.9)	621 (2.4)				
1st complete remission	39447 (66.3)	15750 (60.9)				
2nd complete remission	9712 (16.3)	7019 (27.1)				

Characteristic	AML	ALL	CLL	CML	MDS	MFS
1st relapse	2707 (4.6)	585 (2.3)				
>= 3rd complete remission	690 (1.2)	1491 (5.8)				
2nd relapse	511 (0.9)	244 (0.9)				
>= 3rd relapse	110 (0.2)	87 (0.3)				
Never treatment	224 (0.4)	20 (0.1)				
Not answered	0 (0.0)	3 (0.0)				
Not reported	173 (0.3)	41 (0.2)				
What was the disease status (CLL)?, no. (%)						
Never treated			4 (0.1)			
Complete Remission (CR)			455 (16.7)			
nodular Partial Remission (nPR)			47 (1.7)			
Partial Remission (PR)			1313 (48.1)			
No Response / Stable (NR/SD)			510 (18.7)			
Progression			318 (11.7)			
Relapse (untreated)			36 (1.3)			
Not assessed			6 (0.2)			
Not reported			38 (1.4)			
What was the disease status (CML)?, no. (%)						
Hematologic CR				1190 (23.7)		
Chronic phase				1711 (34.0)		
Accelerated phase				513 (10.2)		
Blast crisis				375 (7.5)		
CHR preceded only by chronic phase				247 (4.9)		
CHR preceded by accelerated phase and/or blast phase				533 (10.6)		
First chronic phase				260 (5.2)		
2nd or greater chronic phase				192 (3.8)		
Not reported				8 (0.2)		
What was the disease status (MDS and MFS)?, no. (%)						
Complete remission (CR)					3141 (15.0)	82 (1.4)
Hematologic improvement (HI)					3635 (17.4)	263 (4.5)

Characteristic	AML	ALL	CLL	CML	MDS	MFS
No response / stable disease (NR/SD)					10902 (52.2)	4161 (70.5)
Progression from hematologic improvement (Prog from HI)					838 (4.0)	283 (4.8)
Relapse from complete remission (Rel from CR)					132 (0.6)	12 (0.2)
Not assessed					292 (1.4)	103 (1.7)
Supportive care or treatment without chemotherapy					1663 (8.0)	479 (8.1)
Partial clinical remission(PR)					0 (0.0)	56 (0.9)
Clinical Improvement(CI)					0 (0.0)	204 (3.5)
Progressive disease(PD)					0 (0.0)	173 (2.9)
Not reported					301 (1.4)	87 (1.5)
Time from diagnosis to HCT, no. (%)						
Median (range)	5.4 (-6.4-1207.7)	8.0 (-41.0-542.4)	63.3 (1.1-596.5)	23.1 (0.3-608.1)	7.6 (-4.1-799.1)	31.5 (0.0-630.2)
< 6 months	34223 (57.5)	8668 (33.5)	48 (1.8)	664 (13.2)	7513 (35.9)	738 (12.5)
6-12 months	13838 (23.3)	7819 (30.2)	167 (6.1)	807 (16.0)	7308 (35.0)	1141 (19.3)
> 12 months	11417 (19.2)	9374 (36.2)	2512 (92.1)	3558 (70.7)	6083 (29.1)	4024 (68.2)
Conditioning regimen intensity, no. (%)						
MAC	32949 (55.4)	19659 (76.0)	404 (14.8)	3696 (73.5)	8463 (40.5)	2233 (37.8)
RIC	18280 (30.7)	3729 (14.4)	1136 (41.7)	885 (17.6)	9199 (44.0)	3151 (53.4)
NMA	5452 (9.2)	1484 (5.7)	938 (34.4)	297 (5.9)	2181 (10.4)	335 (5.7)
Not reported	2797 (4.7)	989 (3.8)	249 (9.1)	151 (3.0)	1061 (5.1)	184 (3.1)
Product type, no. (%)						
BM	8607 (14.5)	6631 (25.6)	188 (6.9)	1106 (22.0)	2497 (11.9)	276 (4.7)
PBSC	47282 (79.5)	16989 (65.7)	2441 (89.5)	3712 (73.8)	17626 (84.3)	5574 (94.4)
UCB	3584 (6.0)	2240 (8.7)	98 (3.6)	211 (4.2)	780 (3.7)	52 (0.9)
Other	1 (0.0)	1 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.0)
Not reported	4 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.0)	0 (0.0)
Type of donor, no. (%)						
HLA-identical sibling	16254 (27.3)	8068 (31.2)	891 (32.7)	1647 (32.8)	5071 (24.3)	1512 (25.6)
Identical twin	82 (0.1)	49 (0.2)	6 (0.2)	13 (0.3)	26 (0.1)	11 (0.2)
Other relative	9347 (15.7)	4782 (18.5)	253 (9.3)	745 (14.8)	2852 (13.6)	756 (12.8)
Unrelated	30525 (51.3)	10831 (41.9)	1484 (54.4)	2428 (48.3)	12255 (58.6)	3587 (60.8)
Cord blood	3258 (5.5)	2127 (8.2)	93 (3.4)	196 (3.9)	698 (3.3)	37 (0.6)

Characteristic	AML	ALL	CLL	CML	MDS	MFS
Not reported	12 (0.0)	4 (0.0)	0 (0.0)	0 (0.0)	2 (0.0)	0 (0.0)
Year of HCT, no. (%)						
2008-2009	5207 (8.8)	2401 (9.3)	586 (21.5)	540 (10.7)	1289 (6.2)	321 (5.4)
2010-2011	6267 (10.5)	2673 (10.3)	667 (24.5)	660 (13.1)	1804 (8.6)	362 (6.1)
2012-2013	6599 (11.1)	2814 (10.9)	598 (21.9)	629 (12.5)	2281 (10.9)	401 (6.8)
2014-2015	6552 (11.0)	2784 (10.8)	261 (9.6)	554 (11.0)	2384 (11.4)	476 (8.1)
2016-2017	7199 (12.1)	3096 (12.0)	215 (7.9)	563 (11.2)	2776 (13.3)	680 (11.5)
2018-2019	7521 (12.6)	3232 (12.5)	138 (5.1)	564 (11.2)	3034 (14.5)	902 (15.3)
2020-2021	7213 (12.1)	3220 (12.5)	102 (3.7)	574 (11.4)	2758 (13.2)	884 (15.0)
2022-2023	8122 (13.7)	3527 (13.6)	104 (3.8)	581 (11.6)	3131 (15.0)	1103 (18.7)
2024-2025	4798 (8.1)	2114 (8.2)	56 (2.1)	364 (7.2)	1447 (6.9)	774 (13.1)
Median follow-up of survivors (range), months	53.8 (0.0-206.1)	49.8 (0.0-208.4)	98.2 (0.0-197.2)	59.0 (0.0-197.3)	59.4 (0.0-199.0)	46.7 (0.0-201.7)

Characteristics of recipients of first autologous transplants for AML, ALL, CLL, CML, MDS and MFS reported to the CIBMTR between 2008 and 2025

Characteristic	AML	ALL	CLL	CML	MDS	MFS
Number of patients	1254	207	50	4	12	1
No. of centers	206	82	39	4	11	1
Age, by decades, no. (%)						
Median (range)	45.4 (0.9-80.2)	41.2 (4.7-77.4)	59.1 (34.7-72.8)	56.9 (21.7-64.1)	61.7 (9.0-86.4)	63.3 (63.3-63.3)
0-9	25 (2.0)	1 (0.5)	0 (0.0)	0 (0.0)	1 (8.3)	0 (0.0)
10-19	70 (5.6)	9 (4.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
20-29	166 (13.2)	51 (24.6)	0 (0.0)	1 (25.0)	0 (0.0)	0 (0.0)
30-39	224 (17.9)	39 (18.8)	1 (2.0)	0 (0.0)	2 (16.7)	0 (0.0)
40-49	269 (21.5)	44 (21.3)	5 (10.0)	0 (0.0)	1 (8.3)	0 (0.0)
50-59	274 (21.9)	28 (13.5)	20 (40.0)	1 (25.0)	1 (8.3)	0 (0.0)
60-69	188 (15.0)	28 (13.5)	18 (36.0)	2 (50.0)	5 (41.7)	1 (100)
70+	38 (3.0)	7 (3.4)	6 (12.0)	0 (0.0)	2 (16.7)	0 (0.0)
TED or RES (RF) track determined for this event, no. (%)						
Ted (registration) patient	1052 (83.9)	189 (91.3)	43 (86.0)	4 (100)	11 (91.7)	1 (100)
cRF (Research) patient	202 (16.1)	18 (8.7)	7 (14.0)	0 (0.0)	1 (8.3)	0 (0.0)
Sex, no. (%)						
Male	671 (53.5)	126 (60.9)	35 (70.0)	3 (75.0)	9 (75.0)	0 (0.0)
Female	583 (46.5)	81 (39.1)	15 (30.0)	1 (25.0)	3 (25.0)	1 (100)
HCT-CI, no. (%)						
0	542 (43.2)	88 (42.5)	24 (48.0)	3 (75.0)	5 (41.7)	0 (0.0)
1	204 (16.3)	25 (12.1)	7 (14.0)	0 (0.0)	2 (16.7)	0 (0.0)
2	114 (9.1)	22 (10.6)	7 (14.0)	0 (0.0)	0 (0.0)	1 (100)
3	145 (11.6)	24 (11.6)	4 (8.0)	0 (0.0)	0 (0.0)	0 (0.0)
4	70 (5.6)	15 (7.2)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
5+	68 (5.4)	18 (8.7)	2 (4.0)	0 (0.0)	1 (8.3)	0 (0.0)
Not reported	111 (8.9)	15 (7.2)	5 (10.0)	1 (25.0)	4 (33.3)	0 (0.0)
What was the disease status (AML and ALL)?, no. (%)						
Primary induction failure	12 (1.0)	10 (4.8)				
1st complete remission	732 (58.4)	165 (79.7)				
2nd complete remission	460 (36.7)	21 (10.1)				
1st relapse	14 (1.1)	2 (1.0)				
>= 3rd complete remission	23 (1.8)	2 (1.0)				
2nd relapse	1 (0.1)	0 (0.0)				

Characteristic	AML	ALL	CLL	CML	MDS	MFS
>= 3rd relapse	5 (0.4)	0 (0.0)				
Never treatment	2 (0.2)	2 (1.0)				
Not reported	5 (0.4)	5 (2.4)				
What was the disease status (CLL)?, no. (%)						
Complete Remission (CR)			12 (24.0)			
nodular Partial Remission (nPR)			1 (2.0)			
Partial Remission (PR)			28 (56.0)			
No Response / Stable (NR/SD)			5 (10.0)			
Progression			4 (8.0)			
What was the disease status (CML)?, no. (%)						
Hematologic CR				1 (25.0)		
Chronic phase				2 (50.0)		
CHR preceded only by chronic phase				1 (25.0)		
What was the disease status (MDS and MFS)?, no. (%)						
Complete remission (CR)					5 (41.7)	0 (0.0)
Hematologic improvement (HI)					3 (25.0)	0 (0.0)
No response / stable disease (NR/SD)					1 (8.3)	0 (0.0)
Relapse from complete remission (Rel from CR)					0 (0.0)	1 (100)
Supportive care or treatment without chemotherapy					2 (16.7)	0 (0.0)
Not reported					1 (8.3)	0 (0.0)
Time from diagnosis to HCT, no. (%)						
Median (range)	7.2 (0.0-472.3)	7.4 (2.2-243.3)	39.6 (1.3-338.7)	17.6 (8.1-99.3)	8.7 (2.2-17.7)	86.2 (86.2-86.2)
< 6 months	491 (39.2)	65 (31.4)	2 (4.0)	0 (0.0)	6 (50.0)	0 (0.0)
6-12 months	246 (19.6)	90 (43.5)	12 (24.0)	1 (25.0)	4 (33.3)	0 (0.0)
> 12 months	517 (41.2)	52 (25.1)	36 (72.0)	3 (75.0)	2 (16.7)	1 (100)
Product type, no. (%)						
BM	42 (3.3)	5 (2.4)	0 (0.0)	2 (50.0)	1 (8.3)	0 (0.0)
PBSC	1204 (96.0)	201 (97.1)	49 (98.0)	2 (50.0)	10 (83.3)	1 (100)
UCB	1 (0.1)	1 (0.5)	0 (0.0)	0 (0.0)	1 (8.3)	0 (0.0)

Characteristic	AML	ALL	CLL	CML	MDS	MFS
Not reported	7 (0.6)	0 (0.0)	1 (2.0)	0 (0.0)	0 (0.0)	0 (0.0)
Year of HCT, no. (%)						
2008-2009	394 (31.4)	53 (25.6)	7 (14.0)	0 (0.0)	6 (50.0)	0 (0.0)
2010-2011	281 (22.4)	15 (7.2)	18 (36.0)	1 (25.0)	2 (16.7)	0 (0.0)
2012-2013	164 (13.1)	33 (15.9)	11 (22.0)	0 (0.0)	1 (8.3)	0 (0.0)
2014-2015	106 (8.5)	21 (10.1)	2 (4.0)	0 (0.0)	1 (8.3)	0 (0.0)
2016-2017	88 (7.0)	23 (11.1)	4 (8.0)	0 (0.0)	0 (0.0)	0 (0.0)
2018-2019	78 (6.2)	23 (11.1)	4 (8.0)	0 (0.0)	1 (8.3)	0 (0.0)
2020-2021	69 (5.5)	16 (7.7)	0 (0.0)	1 (25.0)	0 (0.0)	1 (100)
2022-2023	45 (3.6)	18 (8.7)	4 (8.0)	1 (25.0)	1 (8.3)	0 (0.0)
2024-2025	29 (2.3)	5 (2.4)	0 (0.0)	1 (25.0)	0 (0.0)	0 (0.0)
Median follow-up of survivors (range), months	70.1 (0.0-197.3)	48.6 (0.0-159.3)	142.4 (1.2-192.3)	25.9 (3.4-48.3)	24.6 (6.9-123.5)	33.1 (33.1-33.1)

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	26273	14383	6999
Source of data			
CRF	11330 (43)	3861 (27)	2725 (39)
TED	14943 (57)	10522 (73)	4274 (61)
Number of centers	248	226	365
Disease at transplant			
AML	18232 (69)	10649 (74)	4659 (67)
ALL	7447 (28)	3394 (24)	2177 (31)
Other acute leukemia	594 (2)	340 (2)	163 (2)
AML Disease status at transplant			
CR1	10313 (57)	7148 (67)	2436 (52)
CR2	3375 (19)	1683 (16)	904 (19)
CR3+	364 (2)	139 (1)	106 (2)
Advanced or active disease	3996 (22)	1639 (15)	1066 (23)
Missing	184 (1)	40 (<1)	147 (3)
ALL Disease status at transplant			
CR1	3782 (51)	2059 (61)	945 (43)
CR2	2109 (28)	838 (25)	633 (29)
CR3+	614 (8)	214 (6)	201 (9)
Advanced or active disease	860 (12)	259 (8)	277 (13)
Missing	82 (1)	24 (1)	121 (6)
Recipient age at transplant			
0-9 years	1741 (7)	588 (4)	645 (9)
10-17 years	1831 (7)	600 (4)	680 (10)
18-29 years	3625 (14)	1501 (10)	1059 (15)
30-39 years	3039 (12)	1471 (10)	878 (13)
40-49 years	3885 (15)	1864 (13)	969 (14)
50-59 years	5003 (19)	2696 (19)	1161 (17)
60-69 years	5638 (21)	4094 (28)	1277 (18)
70+ years	1511 (6)	1569 (11)	330 (5)
Median (Range)	48 (0-84)	55 (0-84)	43 (0-82)
Recipient race			
White	22973 (91)	12612 (92)	5107 (87)
Black or African American	1071 (4)	517 (4)	300 (5)
Asian	764 (3)	461 (3)	347 (6)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Native Hawaiian or other Pacific Islander	42 (<1)	18 (<1)	31 (1)
American Indian or Alaska Native	108 (<1)	66 (<1)	37 (1)
Other	23 (<1)	12 (<1)	11 (<1)
More than one race	151 (1)	75 (1)	31 (1)
Unknown	1141 (N/A)	622 (N/A)	1135 (N/A)
Recipient ethnicity			
Hispanic or Latino	2554 (11)	1260 (10)	745 (12)
Non Hispanic or non-Latino	20238 (87)	11727 (89)	3713 (62)
Non-resident of the U.S.	445 (2)	134 (1)	1537 (26)
Unknown	3036 (N/A)	1262 (N/A)	1004 (N/A)
Recipient sex			
Male	14462 (55)	7961 (55)	3942 (56)
Female	11811 (45)	6422 (45)	3057 (44)
Karnofsky score			
10-80	9703 (37)	5868 (41)	2300 (33)
90-100	15724 (60)	8200 (57)	4396 (63)
Missing	846 (3)	315 (2)	303 (4)
HLA-A B DRB1 groups - low resolution			
<=3/6	21 (<1)	78 (1)	8 (<1)
4/6	160 (1)	106 (1)	44 (1)
5/6	3705 (14)	1923 (14)	1048 (16)
6/6	22165 (85)	11667 (85)	5511 (83)
Unknown	222 (N/A)	609 (N/A)	388 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	410 (2)	128 (1)	46 (1)
6/8	968 (4)	190 (2)	122 (2)
7/8	4994 (19)	2113 (17)	1146 (22)
8/8	19428 (75)	9783 (80)	3798 (74)
Unknown	473 (N/A)	2169 (N/A)	1887 (N/A)
HLA-DPB1 Match			
Double allele mismatch	6986 (28)	2343 (24)	714 (24)
Single allele mismatch	13200 (53)	5257 (53)	1550 (53)
Full allele matched	4581 (18)	2369 (24)	687 (23)
Unknown	1506 (N/A)	4414 (N/A)	4048 (N/A)
High resolution release score			
No	6575 (25)	14353 (>99)	6778 (97)
Yes	19698 (75)	30 (<1)	221 (3)
KIR typing available			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
No	17660 (67)	14373 (>99)	6960 (99)
Yes	8613 (33)	10 (<1)	39 (1)
Graft type			
Marrow	7815 (30)	2614 (18)	2237 (32)
PBSC	18372 (70)	11612 (81)	4726 (68)
BM+PBSC	15 (<1)	11 (<1)	3 (<1)
PBSC+UCB	25 (<1)	122 (1)	8 (<1)
Others	46 (<1)	24 (<1)	25 (<1)
Conditioning regimen			
Myeloablative	17844 (68)	8086 (56)	4772 (68)
RIC/Nonmyeloablative	8319 (32)	6266 (44)	2143 (31)
TBD	110 (<1)	31 (<1)	84 (1)
Donor age at donation			
To Be Determined/NA	145 (1)	294 (2)	82 (1)
0-9 years	3 (<1)	20 (<1)	0
10-17 years	0	4 (<1)	2 (<1)
18-29 years	13697 (52)	8417 (59)	3182 (45)
30-39 years	7196 (27)	3620 (25)	2067 (30)
40-49 years	3994 (15)	1556 (11)	1265 (18)
50+ years	1238 (5)	472 (3)	401 (6)
Median (Range)	29 (0-61)	28 (0-89)	31 (17-77)
Donor/Recipient CMV serostatus			
+/+	7065 (27)	4204 (29)	1973 (28)
+/-	2863 (11)	1649 (11)	828 (12)
-/+	9299 (35)	4761 (33)	2257 (32)
-/-	6684 (25)	3365 (23)	1690 (24)
CB - recipient +	24 (<1)	104 (1)	7 (<1)
CB - recipient -	1 (<1)	22 (<1)	2 (<1)
CB - recipient CMV unknown	0	1 (<1)	0
Missing	337 (1)	277 (2)	242 (3)
GvHD Prophylaxis			
No GvHD Prophylaxis	101 (<1)	71 (<1)	27 (<1)
TDEPLETION alone	77 (<1)	18 (<1)	37 (1)
TDEPLETION +- other	540 (2)	154 (1)	188 (3)
CD34 select alone	157 (1)	69 (<1)	59 (1)
CD34 select +- other	246 (1)	154 (1)	71 (1)
Cyclophosphamide alone	161 (1)	67 (<1)	39 (1)
Cyclophosphamide +- others	3301 (13)	4381 (30)	807 (12)
FK506 + MMF +- others	2545 (10)	1144 (8)	444 (6)
FK506 + MTX +- others(not MMF)	11708 (45)	5595 (39)	2069 (30)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
FK506 +- others(not MMF,MTX)	1320 (5)	752 (5)	269 (4)
FK506 alone	635 (2)	291 (2)	120 (2)
CSA + MMF +- others(not FK506)	1224 (5)	442 (3)	452 (6)
CSA + MTX +- others(not MMF,FK506)	3275 (12)	908 (6)	1846 (26)
CSA +- others(not FK506,MMF,MTX)	361 (1)	115 (1)	195 (3)
CSA alone	202 (1)	66 (<1)	197 (3)
Other GVHD Prophylaxis	333 (1)	133 (1)	112 (2)
Missing	87 (<1)	23 (<1)	67 (1)
Donor/Recipient sex match			
Male-Male	10024 (38)	5329 (37)	2546 (36)
Male-Female	6955 (26)	3661 (25)	1660 (24)
Female-Male	4371 (17)	2495 (17)	1356 (19)
Female-Female	4786 (18)	2642 (18)	1368 (20)
CB - recipient M	9 (<1)	63 (<1)	2 (<1)
CB - recipient F	16 (<1)	64 (<1)	7 (<1)
Missing	112 (<1)	129 (1)	60 (1)
Year of transplant			
1986-1990	118 (<1)	18 (<1)	37 (1)
1991-1995	727 (3)	186 (1)	299 (4)
1996-2000	1381 (5)	500 (4)	554 (8)
2001-2005	2598 (10)	543 (4)	936 (14)
2006-2010	4788 (19)	996 (8)	980 (14)
2011-2015	6860 (27)	1851 (14)	1405 (21)
2016-2020	5631 (22)	3840 (29)	1441 (21)
2021-2025	4170 (14)	6449 (40)	1347 (17)
Follow-up among survivors, Months			
N Eval	12017	8559	3196
Median (Range)	47 (0-362)	24 (0-362)	28 (0-372)

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

	Samples Available for Recipient and Donor	Samples Available for Recipient Only	Samples Available for Donor Only
Variable	N (%)	N (%)	N (%)
Number of patients	3918	1123	1371
Source of data			
CRF	2674 (68)	657 (59)	604 (44)
TED	1244 (32)	466 (41)	767 (56)
Number of centers	143	125	192
Disease at transplant			
AML	2470 (63)	678 (60)	791 (58)
ALL	1345 (34)	417 (37)	530 (39)
Other acute leukemia	103 (3)	28 (2)	50 (4)
AML Disease status at transplant			
CR1	1311 (53)	398 (59)	410 (52)
CR2	654 (26)	164 (24)	198 (25)
CR3+	69 (3)	11 (2)	30 (4)
Advanced or active disease	428 (17)	102 (15)	147 (19)
Missing	8 (<1)	3 (<1)	6 (1)
ALL Disease status at transplant			
CR1	599 (45)	179 (43)	230 (43)
CR2	515 (38)	154 (37)	189 (36)
CR3+	152 (11)	59 (14)	67 (13)
Advanced or active disease	78 (6)	24 (6)	42 (8)
Missing	1 (<1)	1 (<1)	2 (<1)
Recipient age at transplant			
0-9 years	871 (22)	313 (28)	349 (25)
10-17 years	467 (12)	139 (12)	197 (14)
18-29 years	581 (15)	127 (11)	185 (13)
30-39 years	434 (11)	135 (12)	167 (12)
40-49 years	452 (12)	110 (10)	147 (11)
50-59 years	545 (14)	141 (13)	184 (13)
60-69 years	489 (12)	134 (12)	127 (9)
70+ years	79 (2)	24 (2)	15 (1)
Median (Range)	31 (0-83)	28 (0-84)	26 (0-85)
Recipient race			
White	2770 (75)	787 (76)	844 (73)
Black or African American	510 (14)	137 (13)	152 (13)
Asian	252 (7)	82 (8)	117 (10)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Native Hawaiian or other Pacific Islander	27 (1)	5 (<1)	13 (1)
American Indian or Alaska Native	38 (1)	9 (1)	15 (1)
More than one race	86 (2)	22 (2)	19 (2)
Unknown	235 (N/A)	81 (N/A)	211 (N/A)
Recipient ethnicity			
Hispanic or Latino	876 (23)	240 (22)	248 (19)
Non Hispanic or non-Latino	2926 (76)	833 (77)	792 (60)
Non-resident of the U.S.	25 (1)	15 (1)	289 (22)
Unknown	91 (N/A)	35 (N/A)	42 (N/A)
Recipient sex			
Male	2069 (53)	590 (53)	742 (54)
Female	1849 (47)	533 (47)	629 (46)
Karnofsky score			
10-80	1084 (28)	303 (27)	344 (25)
90-100	2749 (70)	778 (69)	958 (70)
Missing	85 (2)	42 (4)	69 (5)
HLA-A B DRB1 groups - low resolution			
<=3/6	125 (3)	78 (8)	45 (3)
4/6	1716 (45)	445 (43)	584 (45)
5/6	1578 (41)	407 (39)	512 (39)
6/6	409 (11)	108 (10)	163 (13)
Unknown	90 (N/A)	85 (N/A)	67 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	1919 (57)	479 (57)	617 (59)
6/8	801 (24)	206 (25)	230 (22)
7/8	433 (13)	101 (12)	143 (14)
8/8	189 (6)	50 (6)	63 (6)
Unknown	576 (N/A)	287 (N/A)	318 (N/A)
HLA-DPB1 Match			
Double allele mismatch	597 (37)	114 (30)	144 (34)
Single allele mismatch	861 (54)	228 (60)	229 (54)
Full allele matched	151 (9)	41 (11)	49 (12)
Unknown	2309 (N/A)	740 (N/A)	949 (N/A)
High resolution release score			
No	3080 (79)	1091 (97)	1353 (99)
Yes	838 (21)	32 (3)	18 (1)
KIR typing available			
No	3222 (82)	1118 (>99)	1355 (99)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Yes	696 (18)	5 (<1)	16 (1)
Graft type			
UCB	3674 (94)	996 (89)	1271 (93)
PBSC+UCB	225 (6)	122 (11)	91 (7)
Others	19 (<1)	5 (<1)	9 (1)
Number of cord units			
1	3216 (82)	0	1126 (82)
2	701 (18)	0	245 (18)
3	1 (<1)	0	0
Unknown	0 (N/A)	1123 (N/A)	0 (N/A)
Conditioning regimen			
Myeloablative	2742 (70)	789 (70)	929 (68)
RIC/Nonmyeloablative	1167 (30)	332 (30)	436 (32)
TBD	9 (<1)	2 (<1)	6 (<1)
Donor age at donation			
To Be Determined/NA	3067 (78)	455 (41)	1075 (78)
0-9 years	659 (17)	509 (45)	224 (16)
10-17 years	41 (1)	58 (5)	15 (1)
18-29 years	46 (1)	27 (2)	15 (1)
30-39 years	41 (1)	33 (3)	21 (2)
40-49 years	29 (1)	19 (2)	11 (1)
50+ years	35 (1)	22 (2)	10 (1)
Median (Range)	5 (0-72)	5 (0-73)	5 (0-67)
Donor/Recipient CMV serostatus			
+/+	0	0	1 (<1)
-/-	0	0	1 (<1)
CB - recipient +	2681 (68)	747 (67)	917 (67)
CB - recipient -	1190 (30)	347 (31)	426 (31)
CB - recipient CMV unknown	47 (1)	29 (3)	26 (2)
GvHD Prophylaxis			
No GvHD Prophylaxis	18 (<1)	7 (1)	7 (1)
TDEPLETION alone	1 (<1)	0	0
TDEPLETION +- other	20 (1)	6 (1)	7 (1)
CD34 select alone	0	1 (<1)	1 (<1)
CD34 select +- other	193 (5)	97 (9)	58 (4)
Cyclophosphamide +- others	12 (<1)	5 (<1)	7 (1)
FK506 + MMF +- others	1191 (30)	363 (32)	285 (21)
FK506 + MTX +- others(not MMF)	139 (4)	40 (4)	50 (4)
FK506 +- others(not MMF,MTX)	115 (3)	35 (3)	34 (2)
FK506 alone	80 (2)	18 (2)	12 (1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
CSA + MMF +- others(not FK506)	1855 (47)	469 (42)	710 (52)
CSA + MTX +- others(not MMF,FK506)	59 (2)	15 (1)	26 (2)
CSA +- others(not FK506,MMF,MTX)	136 (3)	46 (4)	106 (8)
CSA alone	24 (1)	11 (1)	40 (3)
Other GVHD Prophylaxis	69 (2)	9 (1)	25 (2)
Missing	6 (<1)	1 (<1)	3 (<1)
Donor/Recipient sex match			
Male-Female	0	0	1 (<1)
Female-Male	0	0	1 (<1)
CB - recipient M	2069 (53)	590 (53)	740 (54)
CB - recipient F	1849 (47)	533 (47)	628 (46)
CB - recipient sex unknown	0	0	1 (<1)
Year of transplant			
1996-2000	0	1 (<1)	3 (<1)
2001-2005	56 (1)	53 (5)	17 (1)
2006-2010	1081 (28)	237 (22)	324 (24)
2011-2015	1587 (41)	279 (26)	484 (36)
2016-2020	871 (22)	317 (29)	323 (24)
2021-2025	323 (7)	236 (19)	220 (15)
Follow-up among survivors, Months			
N Eval	1853	601	676
Median (Range)	57 (0-196)	37 (0-213)	37 (0-199)

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, recipient only 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	7000	1297	630
Source of data			
CRF	1585 (23)	219 (17)	130 (21)
TED	5415 (77)	1078 (83)	500 (79)
Number of centers	87	73	63
Disease at transplant			
AML	4487 (64)	768 (59)	409 (65)
ALL	2299 (33)	490 (38)	209 (33)
Other acute leukemia	214 (3)	39 (3)	12 (2)
AML Disease status at transplant			
CR1	3007 (67)	529 (69)	265 (65)
CR2	673 (15)	97 (13)	50 (12)
CR3+	55 (1)	18 (2)	2 (<1)
Advanced or active disease	745 (17)	119 (15)	92 (22)
Missing	7 (<1)	5 (1)	0
ALL Disease status at transplant			
CR1	1355 (59)	298 (61)	131 (63)
CR2	697 (30)	130 (27)	56 (27)
CR3+	150 (7)	35 (7)	10 (5)
Advanced or active disease	97 (4)	27 (6)	12 (6)
Recipient age at transplant			
0-9 years	532 (8)	86 (7)	40 (6)
10-17 years	690 (10)	103 (8)	47 (7)
18-29 years	990 (14)	199 (15)	83 (13)
30-39 years	683 (10)	140 (11)	79 (13)
40-49 years	944 (13)	189 (15)	68 (11)
50-59 years	1372 (20)	270 (21)	119 (19)
60-69 years	1484 (21)	258 (20)	161 (26)
70+ years	305 (4)	52 (4)	33 (5)
Median (Range)	47 (0-82)	47 (1-77)	50 (1-83)
Recipient race			
White	5372 (82)	909 (77)	467 (81)
Black or African American	649 (10)	123 (10)	49 (8)
Asian	338 (5)	113 (10)	46 (8)
Native Hawaiian or other Pacific Islander	24 (<1)	5 (<1)	2 (<1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
American Indian or Alaska Native	57 (1)	10 (1)	4 (1)
More than one race	90 (1)	14 (1)	11 (2)
Unknown	470 (N/A)	123 (N/A)	51 (N/A)
Recipient ethnicity			
Hispanic or Latino	1594 (23)	385 (31)	167 (27)
Non Hispanic or non-Latino	5226 (76)	870 (69)	436 (71)
Non-resident of the U.S.	49 (1)	7 (1)	9 (1)
Unknown	131 (N/A)	35 (N/A)	18 (N/A)
Recipient sex			
Male	3981 (57)	726 (56)	355 (56)
Female	3019 (43)	571 (44)	275 (44)
Karnofsky score			
10-80	2675 (38)	538 (41)	293 (47)
90-100	4125 (59)	732 (56)	311 (49)
Missing	200 (3)	27 (2)	26 (4)
HLA-A B DRB1 groups - low resolution			
<=3/6	1886 (28)	322 (26)	203 (37)
4/6	565 (8)	111 (9)	67 (12)
5/6	165 (2)	33 (3)	14 (3)
6/6	4235 (62)	763 (62)	266 (48)
Unknown	149 (N/A)	68 (N/A)	80 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	2343 (35)	412 (35)	241 (48)
6/8	116 (2)	21 (2)	6 (1)
7/8	105 (2)	23 (2)	11 (2)
8/8	4177 (62)	725 (61)	249 (49)
Unknown	259 (N/A)	116 (N/A)	123 (N/A)
HLA-DPB1 Match			
Double allele mismatch	8 (<1)	1 (<1)	3 (1)
Single allele mismatch	2009 (39)	272 (63)	164 (68)
Full allele matched	3176 (61)	157 (37)	73 (30)
Unknown	1807 (N/A)	867 (N/A)	390 (N/A)
High resolution release score			
No	3171 (45)	1275 (98)	623 (99)
Yes	3829 (55)	22 (2)	7 (1)
Graft type			
Marrow	1703 (24)	226 (17)	145 (23)
PBSC	5247 (75)	1055 (81)	479 (76)
UCB	1 (<1)	7 (1)	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
BM+PBSC	10 (<1)	4 (<1)	1 (<1)
BM+UCB	4 (<1)	1 (<1)	0
PBSC+UCB	0	0	3 (<1)
Others	35 (1)	4 (<1)	2 (<1)
Conditioning regimen			
Myeloablative	4781 (68)	886 (68)	392 (62)
RIC/Nonmyeloablative	2207 (32)	407 (31)	231 (37)
TBD	12 (<1)	4 (<1)	7 (1)
Donor age at donation			
To Be Determined/NA	9 (<1)	3 (<1)	0
0-9 years	333 (5)	49 (4)	18 (3)
10-17 years	492 (7)	101 (8)	38 (6)
18-29 years	1482 (21)	276 (21)	144 (23)
30-39 years	1225 (18)	246 (19)	140 (22)
40-49 years	1150 (16)	215 (17)	96 (15)
50+ years	2309 (33)	407 (31)	194 (31)
Median (Range)	40 (0-80)	39 (0-79)	38 (1-80)
Donor/Recipient CMV serostatus			
+/+	2978 (43)	610 (47)	279 (44)
+/-	648 (9)	103 (8)	49 (8)
-/+	1982 (28)	326 (25)	178 (28)
-/-	1305 (19)	239 (18)	111 (18)
CB - recipient +	4 (<1)	4 (<1)	3 (<1)
CB - recipient -	1 (<1)	4 (<1)	0
Missing	82 (1)	11 (1)	10 (2)
GvHD Prophylaxis			
No GvHD Prophylaxis	75 (1)	10 (1)	2 (<1)
TDEPLETION alone	100 (1)	32 (2)	10 (2)
TDEPLETION +- other	79 (1)	24 (2)	10 (2)
CD34 select alone	47 (1)	15 (1)	7 (1)
CD34 select +- other	38 (1)	9 (1)	6 (1)
Cyclophosphamide alone	48 (1)	8 (1)	7 (1)
Cyclophosphamide +- others	2775 (40)	475 (37)	300 (48)
FK506 + MMF +- others	338 (5)	43 (3)	17 (3)
FK506 + MTX +- others(not MMF)	2454 (35)	362 (28)	190 (30)
FK506 +- others(not MMF,MTX)	511 (7)	233 (18)	41 (7)
FK506 alone	55 (1)	8 (1)	4 (1)
CSA + MMF +- others(not FK506)	65 (1)	11 (1)	5 (1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
CSA + MTX +- others(not MMF,FK506)	334 (5)	43 (3)	21 (3)
CSA +- others(not FK506,MMF,MTX)	0	2 (<1)	0
CSA alone	33 (<1)	7 (1)	0
Other GVHD Prophylaxis	46 (1)	9 (1)	10 (2)
Missing	2 (<1)	6 (<1)	0
Donor/Recipient sex match			
Male-Male	2266 (32)	454 (35)	204 (32)
Male-Female	1556 (22)	303 (23)	144 (23)
Female-Male	1711 (24)	268 (21)	150 (24)
Female-Female	1462 (21)	264 (20)	129 (20)
CB - recipient M	4 (<1)	4 (<1)	1 (<1)
CB - recipient F	1 (<1)	4 (<1)	2 (<1)
Year of transplant			
2006-2010	273 (4)	29 (2)	22 (4)
2011-2015	1784 (26)	285 (23)	107 (18)
2016-2020	2691 (40)	500 (40)	223 (38)
2021-2025	2252 (29)	483 (34)	278 (40)
Follow-up among survivors, Months			
N Eval	4349	832	392
Median (Range)	26 (0-148)	24 (0-122)	24 (0-148)

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	14997	9226	3693
Source of data			
CRF	9015 (60)	3978 (43)	1866 (51)
TED	5982 (40)	5248 (57)	1827 (49)
Number of centers	245	216	319
Disease at transplant			
Other leukemia	1515 (10)	516 (6)	341 (9)
CML	3644 (24)	1331 (14)	1086 (29)
MDS	8027 (54)	5686 (62)	1874 (51)
MPN	1811 (12)	1693 (18)	392 (11)
MDS Disease status at transplant			
Early	1664 (21)	1027 (18)	409 (22)
Advanced	5316 (66)	4226 (74)	1091 (58)
Missing	1047 (13)	433 (8)	374 (20)
Recipient age at transplant			
0-9 years	454 (3)	122 (1)	171 (5)
10-17 years	446 (3)	157 (2)	191 (5)
18-29 years	1027 (7)	336 (4)	337 (9)
30-39 years	1525 (10)	532 (6)	431 (12)
40-49 years	2256 (15)	906 (10)	608 (16)
50-59 years	3451 (23)	1776 (19)	756 (20)
60-69 years	4470 (30)	3655 (40)	913 (25)
70+ years	1368 (9)	1742 (19)	286 (8)
Median (Range)	56 (0-83)	63 (1-83)	52 (1-82)
Recipient race			
White	13385 (92)	8331 (93)	2793 (88)
Black or African American	658 (5)	293 (3)	171 (5)
Asian	326 (2)	209 (2)	154 (5)
Native Hawaiian or other	20 (<1)	13 (<1)	11 (<1)
Pacific Islander			
American Indian or Alaska	45 (<1)	31 (<1)	15 (<1)
Native			
Other	18 (<1)	7 (<1)	8 (<1)
More than one race	68 (<1)	51 (1)	19 (1)
Unknown	477 (N/A)	291 (N/A)	522 (N/A)
Recipient ethnicity			
Hispanic or Latino	834 (7)	467 (6)	202 (7)
Non Hispanic or non-Latino	11166 (91)	7675 (93)	1917 (65)
Non-resident of the U.S.	275 (2)	90 (1)	824 (28)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Unknown	2722 (N/A)	994 (N/A)	750 (N/A)
Recipient sex			
Male	9083 (61)	5769 (63)	2295 (62)
Female	5914 (39)	3457 (37)	1398 (38)
Karnofsky score			
10-80	5505 (37)	4044 (44)	1166 (32)
90-100	8998 (60)	5003 (54)	2358 (64)
Missing	494 (3)	179 (2)	169 (5)
HLA-A B DRB1 groups - low resolution			
<=3/6	8 (<1)	32 (<1)	2 (<1)
4/6	128 (1)	56 (1)	29 (1)
5/6	1947 (13)	1049 (12)	488 (14)
6/6	12774 (86)	7681 (87)	2849 (85)
Unknown	140 (N/A)	408 (N/A)	325 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	334 (2)	51 (1)	29 (1)
6/8	573 (4)	86 (1)	85 (3)
7/8	2571 (18)	1134 (15)	470 (19)
8/8	11125 (76)	6405 (83)	1880 (76)
Unknown	394 (N/A)	1550 (N/A)	1229 (N/A)
HLA-DPB1 Match			
Double allele mismatch	3664 (28)	1475 (22)	373 (24)
Single allele mismatch	7134 (54)	3499 (52)	805 (52)
Full allele matched	2499 (19)	1704 (26)	369 (24)
Unknown	1700 (N/A)	2548 (N/A)	2146 (N/A)
High resolution release score			
No	4609 (31)	9194 (>99)	3527 (96)
Yes	10388 (69)	32 (<1)	166 (4)
KIR typing available			
No	11646 (78)	9215 (>99)	3677 (>99)
Yes	3351 (22)	11 (<1)	16 (<1)
Graft type			
Marrow	4599 (31)	1460 (16)	1295 (35)
PBSC	10362 (69)	7708 (84)	2359 (64)
BM+PBSC	5 (<1)	7 (<1)	2 (<1)
PBSC+UCB	10 (<1)	45 (<1)	2 (<1)
Others	21 (<1)	6 (<1)	35 (1)
Conditioning regimen			
Myeloablative	8285 (55)	3568 (39)	2107 (57)
RIC/Nonmyeloablative	6672 (44)	5635 (61)	1538 (42)
TBD	40 (<1)	23 (<1)	48 (1)
Donor age at donation			
To Be Determined/NA	63 (<1)	155 (2)	55 (1)
0-9 years	0	10 (<1)	0
10-17 years	2 (<1)	5 (<1)	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
18-29 years	7489 (50)	5501 (60)	1586 (43)
30-39 years	4302 (29)	2303 (25)	1135 (31)
40-49 years	2401 (16)	952 (10)	698 (19)
50+ years	740 (5)	300 (3)	219 (6)
Median (Range)	30 (13-62)	28 (1-109)	32 (19-60)
Donor/Recipient CMV serostatus			
+/+	3536 (24)	2342 (25)	962 (26)
+/-	1871 (12)	1294 (14)	415 (11)
-/+	4630 (31)	2525 (27)	1071 (29)
-/-	4817 (32)	2898 (31)	1098 (30)
CB - recipient +	7 (<1)	27 (<1)	2 (<1)
CB - recipient -	3 (<1)	19 (<1)	0
Missing	133 (1)	121 (1)	145 (4)
GvHD Prophylaxis			
No GvHD Prophylaxis	66 (<1)	88 (1)	19 (1)
TDEPLETION alone	25 (<1)	10 (<1)	6 (<1)
TDEPLETION +- other	261 (2)	64 (1)	80 (2)
CD34 select alone	69 (<1)	51 (1)	25 (1)
CD34 select +- other	117 (1)	65 (1)	18 (<1)
Cyclophosphamide alone	59 (<1)	25 (<1)	13 (<1)
Cyclophosphamide +- others	2052 (14)	3233 (35)	483 (13)
FK506 + MMF +- others	1644 (11)	703 (8)	281 (8)
FK506 + MTX +- others(not MMF)	5981 (40)	3225 (35)	981 (27)
FK506 +- others(not MMF,MTX)	705 (5)	497 (5)	115 (3)
FK506 alone	289 (2)	138 (1)	56 (2)
CSA + MMF +- others(not FK506)	776 (5)	287 (3)	260 (7)
CSA + MTX +- others(not MMF,FK506)	2325 (16)	635 (7)	1073 (29)
CSA +- others(not FK506,MMF,MTX)	255 (2)	72 (1)	114 (3)
CSA alone	107 (1)	28 (<1)	92 (2)
Other GVHD Prophylaxis	229 (2)	87 (1)	46 (1)
Missing	37 (<1)	18 (<1)	31 (1)
Donor/Recipient sex match			
Male-Male	6340 (42)	3960 (43)	1511 (41)
Male-Female	3396 (23)	2010 (22)	747 (20)
Female-Male	2710 (18)	1727 (19)	762 (21)
Female-Female	2491 (17)	1393 (15)	637 (17)
CB - recipient M	6 (<1)	32 (<1)	1 (<1)
CB - recipient F	4 (<1)	14 (<1)	1 (<1)
Missing	50 (<1)	90 (1)	34 (1)
Year of transplant			
1986-1990	178 (1)	24 (<1)	40 (1)
1991-1995	863 (6)	185 (2)	313 (9)
1996-2000	1328 (9)	520 (6)	437 (12)
2001-2005	1383 (9)	261 (3)	493 (14)
2006-2010	2307 (16)	466 (6)	414 (12)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
2011-2015	3416 (23)	931 (11)	570 (16)
2016-2020	2958 (20)	2183 (27)	714 (20)
2021-2025	2564 (15)	4656 (44)	712 (16)
Follow-up among survivors, Months			
N Eval	6579	5551	1763
Median (Range)	48 (0-384)	13 (0-334)	36 (0-385)

Unrelated Cord Blood Transplant Research Sample Inventory - Summary for First Allogeneic Transplants in CRF and TED with biospecimens available through the CIBMTR Repository stratified by availability of paired, recipient only and cord blood only samples, Biospecimens include: whole blood, serum/plasma and limited quantities of viable cells and cell lines (collected prior to 2006- recipient only), 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	890	270	313
Source of data			
CRF	626 (70)	178 (66)	127 (41)
TED	264 (30)	92 (34)	186 (59)
Number of centers	124	80	116
Disease at transplant			
Other leukemia	102 (11)	31 (11)	38 (12)
CML	140 (16)	38 (14)	61 (19)
MDS	594 (67)	184 (68)	193 (62)
MPN	54 (6)	17 (6)	21 (7)
MDS Disease status at transplant			
Early	179 (30)	44 (24)	76 (39)
Advanced	358 (60)	123 (67)	92 (48)
Missing	57 (10)	17 (9)	25 (13)
Recipient age at transplant			
0-9 years	131 (15)	37 (14)	56 (18)
10-17 years	64 (7)	16 (6)	28 (9)
18-29 years	76 (9)	13 (5)	22 (7)
30-39 years	85 (10)	24 (9)	33 (11)
40-49 years	123 (14)	36 (13)	41 (13)
50-59 years	185 (21)	57 (21)	68 (22)
60-69 years	186 (21)	70 (26)	61 (19)
70+ years	40 (4)	17 (6)	4 (1)
Median (Range)	48 (0-80)	51 (1-76)	45 (0-74)
Recipient race			
White	625 (72)	199 (76)	196 (74)
Black or African American	155 (18)	38 (14)	40 (15)
Asian	56 (6)	22 (8)	19 (7)
Native Hawaiian or other	9 (1)	0	2 (1)
Pacific Islander			
American Indian or Alaska	5 (1)	1 (<1)	1 (<1)
Native			
Other	0	0	1 (<1)
More than one race	13 (2)	3 (1)	6 (2)
Unknown	27 (N/A)	7 (N/A)	48 (N/A)
Recipient ethnicity			
Hispanic or Latino	126 (15)	32 (12)	27 (9)
Non Hispanic or non-Latino	726 (85)	226 (87)	199 (66)
Non-resident of the U.S.	5 (1)	3 (1)	77 (25)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Unknown	33 (N/A)	9 (N/A)	10 (N/A)
Recipient sex			
Male	529 (59)	160 (59)	187 (60)
Female	361 (41)	110 (41)	126 (40)
Karnofsky score			
10-80	241 (27)	87 (32)	96 (31)
90-100	630 (71)	169 (63)	193 (62)
Missing	19 (2)	14 (5)	24 (8)
HLA-A B DRB1 groups - low resolution			
<=3/6	31 (4)	27 (11)	10 (3)
4/6	392 (45)	126 (51)	154 (53)
5/6	360 (42)	83 (33)	116 (40)
6/6	83 (10)	12 (5)	12 (4)
Unknown	24 (N/A)	22 (N/A)	21 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	459 (61)	135 (71)	147 (61)
6/8	176 (23)	32 (17)	61 (25)
7/8	76 (10)	22 (12)	26 (11)
8/8	40 (5)	2 (1)	6 (3)
Unknown	139 (N/A)	79 (N/A)	73 (N/A)
HLA-DPB1 Match			
Double allele mismatch	140 (39)	28 (38)	29 (32)
Single allele mismatch	180 (50)	40 (54)	52 (57)
Full allele matched	38 (11)	6 (8)	10 (11)
Unknown	532 (N/A)	196 (N/A)	222 (N/A)
High resolution release score			
No	700 (79)	266 (99)	311 (99)
Yes	190 (21)	4 (1)	2 (1)
KIR typing available			
No	732 (82)	270 (100)	311 (99)
Yes	158 (18)	0	2 (1)
Graft type			
UCB	810 (91)	224 (83)	289 (92)
PBSC+UCB	78 (9)	45 (17)	23 (7)
Others	2 (<1)	1 (<1)	1 (<1)
Number of cord units			
1	721 (81)	0	250 (80)
2	168 (19)	0	62 (20)
Unknown	1 (N/A)	270 (N/A)	1 (N/A)
Conditioning regimen			
Myeloablative	486 (55)	135 (50)	162 (52)
RIC/Nonmyeloablative	403 (45)	134 (50)	150 (48)
TBD	1 (<1)	1 (<1)	1 (<1)
Donor age at donation			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
To Be Determined/NA	662 (74)	85 (31)	243 (78)
0-9 years	171 (19)	141 (52)	53 (17)
10-17 years	10 (1)	14 (5)	4 (1)
18-29 years	14 (2)	10 (4)	2 (1)
30-39 years	14 (2)	8 (3)	3 (1)
40-49 years	12 (1)	4 (1)	1 (<1)
50+ years	7 (1)	8 (3)	7 (2)
Median (Range)	5 (0-67)	5 (0-72)	4 (0-65)
Donor/Recipient CMV serostatus			
CB - recipient +	533 (60)	171 (63)	189 (60)
CB - recipient -	350 (39)	90 (33)	114 (36)
CB - recipient CMV unknown	7 (1)	9 (3)	10 (3)
GvHD Prophylaxis			
No GvHD Prophylaxis	2 (<1)	1 (<1)	2 (1)
TDEPLETION +- other	1 (<1)	1 (<1)	0
CD34 select +- other	62 (7)	39 (14)	17 (5)
Cyclophosphamide +- others	1 (<1)	2 (1)	1 (<1)
FK506 + MMF +- others	295 (33)	91 (34)	64 (20)
FK506 + MTX +- others(not MMF)	27 (3)	5 (2)	9 (3)
FK506 +- others(not MMF,MTX)	35 (4)	11 (4)	12 (4)
FK506 alone	25 (3)	9 (3)	4 (1)
CSA + MMF +- others(not FK506)	367 (41)	92 (34)	155 (50)
CSA + MTX +- others(not MMF,FK506)	8 (1)	2 (1)	4 (1)
CSA +- others(not FK506,MMF,MTX)	25 (3)	10 (4)	28 (9)
CSA alone	9 (1)	1 (<1)	9 (3)
Other GVHD Prophylaxis	33 (4)	6 (2)	7 (2)
Missing	0	0	1 (<1)
Donor/Recipient sex match			
CB - recipient M	529 (59)	160 (59)	187 (60)
CB - recipient F	361 (41)	110 (41)	126 (40)
Year of transplant			
1996-2000	0	0	1 (<1)
2001-2005	16 (2)	7 (3)	4 (1)
2006-2010	249 (28)	70 (27)	77 (25)
2011-2015	364 (41)	74 (28)	116 (38)
2016-2020	178 (20)	81 (31)	64 (21)
2021-2025	83 (8)	38 (12)	51 (14)
Follow-up among survivors, Months			
N Eval	359	131	153
Median (Range)	57 (0-170)	43 (0-175)	47 (0-188)

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, recipient only 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	3015	506	247
Source of data			
CRF	1243 (41)	179 (35)	99 (40)
TED	1772 (59)	327 (65)	148 (60)
Number of centers	80	50	39
Disease at transplant			
Other leukemia	232 (8)	46 (9)	19 (8)
CML	396 (13)	59 (12)	28 (11)
MDS	1810 (60)	297 (59)	159 (64)
MPN	577 (19)	104 (21)	41 (17)
MDS Disease status at transplant			
Early	312 (17)	44 (15)	27 (17)
Advanced	1425 (79)	230 (77)	123 (77)
Missing	73 (4)	23 (8)	9 (6)
Recipient age at transplant			
0-9 years	76 (3)	16 (3)	3 (1)
10-17 years	88 (3)	8 (2)	8 (3)
18-29 years	127 (4)	23 (5)	6 (2)
30-39 years	139 (5)	22 (4)	13 (5)
40-49 years	305 (10)	43 (8)	22 (9)
50-59 years	793 (26)	143 (28)	61 (25)
60-69 years	1201 (40)	202 (40)	111 (45)
70+ years	286 (9)	49 (10)	23 (9)
Median (Range)	60 (1-78)	60 (1-81)	61 (2-77)
Recipient race			
White	2414 (83)	368 (76)	195 (84)
Black or African American	312 (11)	68 (14)	23 (10)
Asian	144 (5)	39 (8)	11 (5)
Native Hawaiian or other Pacific Islander	11 (<1)	3 (1)	0
American Indian or Alaska Native	11 (<1)	3 (1)	3 (1)
More than one race	19 (1)	2 (<1)	1 (<1)
Unknown	104 (N/A)	23 (N/A)	14 (N/A)
Recipient ethnicity			
Hispanic or Latino	368 (12)	84 (17)	34 (14)
Non Hispanic or non-Latino	2571 (87)	409 (83)	202 (84)
Non-resident of the U.S.	14 (<1)	1 (<1)	4 (2)
Unknown	62 (N/A)	12 (N/A)	7 (N/A)
Recipient sex			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Male	1834 (61)	316 (62)	165 (67)
Female	1181 (39)	190 (38)	82 (33)
Karnofsky score			
10-80	1295 (43)	238 (47)	118 (48)
90-100	1625 (54)	254 (50)	119 (48)
Missing	95 (3)	14 (3)	10 (4)
HLA-A B DRB1 groups - low resolution			
<=3/6	777 (26)	114 (24)	73 (34)
4/6	219 (7)	46 (10)	20 (9)
5/6	46 (2)	11 (2)	6 (3)
6/6	1900 (65)	312 (65)	118 (54)
Unknown	73 (N/A)	23 (N/A)	30 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	957 (33)	140 (31)	83 (43)
6/8	33 (1)	19 (4)	4 (2)
7/8	34 (1)	4 (1)	2 (1)
8/8	1842 (64)	289 (64)	104 (54)
Unknown	149 (N/A)	54 (N/A)	54 (N/A)
HLA-DPB1 Match			
Double allele mismatch	3 (<1)	0	1 (1)
Single allele mismatch	832 (40)	101 (67)	65 (64)
Full allele matched	1259 (60)	50 (33)	35 (35)
Unknown	921 (N/A)	355 (N/A)	146 (N/A)
High resolution release score			
No	1504 (50)	501 (99)	244 (99)
Yes	1511 (50)	5 (1)	3 (1)
Graft type			
Marrow	458 (15)	57 (11)	37 (15)
PBSC	2539 (84)	444 (88)	210 (85)
UCB	0	2 (<1)	0
BM+PBSC	6 (<1)	0	0
BM+UCB	0	1 (<1)	0
Others	12 (<1)	2 (<1)	0
Conditioning regimen			
Myeloablative	1404 (47)	205 (41)	97 (39)
RIC/Nonmyeloablative	1608 (53)	301 (59)	148 (60)
TBD	3 (<1)	0	2 (1)
Donor age at donation			
To Be Determined/NA	1 (<1)	2 (<1)	0
0-9 years	42 (1)	10 (2)	3 (1)
10-17 years	85 (3)	17 (3)	6 (2)
18-29 years	398 (13)	58 (11)	44 (18)
30-39 years	443 (15)	86 (17)	35 (14)
40-49 years	529 (18)	76 (15)	44 (18)
50+ years	1517 (50)	257 (51)	115 (47)
Median (Range)	50 (0-82)	50 (0-75)	48 (7-73)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Donor/Recipient CMV serostatus			
+/+	1191 (40)	209 (41)	85 (34)
+/-	345 (11)	42 (8)	29 (12)
-/+	727 (24)	141 (28)	69 (28)
-/-	718 (24)	106 (21)	62 (25)
CB - recipient +	0	3 (1)	0
Missing	34 (1)	5 (1)	2 (1)
GvHD Prophylaxis			
No GvHD Prophylaxis	28 (1)	3 (1)	3 (1)
TDEPLETION alone	12 (<1)	4 (1)	2 (1)
TDEPLETION +- other	8 (<1)	4 (1)	3 (1)
CD34 select alone	6 (<1)	7 (1)	0
CD34 select +- other	10 (<1)	3 (1)	0
Cyclophosphamide alone	22 (1)	2 (<1)	3 (1)
Cyclophosphamide +- others	1284 (43)	180 (36)	112 (45)
FK506 + MMF +- others	202 (7)	26 (5)	6 (2)
FK506 + MTX +- others(not MMF)	1025 (34)	167 (33)	93 (38)
FK506 +- others(not MMF,MTX)	214 (7)	81 (16)	19 (8)
FK506 alone	19 (1)	4 (1)	0
CSA + MMF +- others(not FK506)	37 (1)	5 (1)	2 (1)
CSA + MTX +- others(not MMF,FK506)	108 (4)	15 (3)	2 (1)
CSA +- others(not FK506,MMF,MTX)	1 (<1)	1 (<1)	0
CSA alone	9 (<1)	0	1 (<1)
Other GVHD Prophylaxis	27 (1)	2 (<1)	1 (<1)
Missing	3 (<1)	2 (<1)	0
Donor/Recipient sex match			
Male-Male	1047 (35)	182 (36)	97 (39)
Male-Female	617 (20)	93 (18)	43 (17)
Female-Male	784 (26)	131 (26)	68 (28)
Female-Female	564 (19)	96 (19)	39 (16)
CB - recipient M	0	2 (<1)	0
CB - recipient F	0	1 (<1)	0
Missing	3 (<1)	1 (<1)	0
Year of transplant			
2006-2010	149 (5)	21 (4)	14 (6)
2011-2015	821 (28)	101 (21)	41 (18)
2016-2020	1140 (39)	191 (40)	95 (41)
2021-2025	905(27)	193 (35)	97(36)
Follow-up among survivors, Months			
N Eval	1725	293	151
Median (Range)	30 (0-150)	24 (0-124)	24 (0-148)

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A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

TO: Leukemia Working Committee Members

FROM: Wael Saber, MD, MS; Scientific Director for the Leukemia Working Committee

RE: 2025-2026 Studies in Progress Summary

CK16-01b Identification of germline predisposition mutations in young myelodysplastic syndrome patients (L Godley).

The purpose of this study is to:

1. Determine the frequency of germline variants in candidate genes in paired samples from patients with myelodysplastic syndromes (MDS) and their HLA-matched related donors.
2. Compare clinical and mobilization characteristics between related donors with germline mutations and those without germline mutations.
3. Evaluate engraftment outcomes in MDS patients with germline deleterious mutations who receive HCT from HLA-matched related donors who share the germline variant versus those who do not share the variant.

Status: **Analysis**.

LK20-01 Acute myeloid leukemia with chromosome 17 abnormalities with or without TP53 abnormalities and outcomes after hematopoietic stem cell transplantation (A Dias/J Yared).

The purpose of this study is to:

1. Evaluate overall survival, disease-free survival, relapse, and non-relapse mortality of adult patients with AML with chromosome 17 abnormalities who received allo-HCT.
2. Determine the effect of patient-, disease-, and transplant-related factors on these outcomes.

Status: **Data File Preparation**

LK20-03 Evaluating outcomes of allogeneic hematopoietic cell transplantation in T-cell acute lymphoblastic leukemia (H Murthy/M Iqbal/M Kharfan-Dabaja).
The purpose of this study is to:

1. Describe clinical outcomes of patients with T-cell acute lymphoblastic leukemia (T-ALL) undergoing allo-HCT.
2. Identify the impact of patient-, disease-, and transplant-related factors on overall survival, leukemia-free survival, non-relapse mortality, and relapse after allo-HCT for T-ALL. This will be evaluated in the overall cohort and by age: pediatric (1-14y, AYA and adult ≥15)
3. To compare the clinical outcomes of patients with T-ALL by age group: pediatric, AYA (15-39y), and adult ≥40y.
4. Describe clinical outcomes of patients with early precursor T-cell acute lymphoblastic leukemia (ETP-ALL) undergoing allo-HCT.
5. To propose a prognostic score for T-ALL based on the significant covariates identified above.

Status: **Data File Preparation**

CK22-01 Impact of somatic mutations on outcomes after allogeneic hematopoietic cell transplantation in patients with myelodysplastic syndrome with ring sideroblasts (MDS-RS) and MDS/myeloproliferative neoplasm with RS and thrombocytosis (MDS/MPN-RS-T) (S Arslan/ R Nakamura).

The purpose of this study is to:

1. Evaluate outcomes after allogeneic HCT in patients with MDS-RS or MDS/MPD-RS-T reported to CIBMTR.
2. Characterize the mutational profile and determine the incidence of high-risk somatic mutations in patients with MDS-RS or MDS/MPD-RS-T who undergo allogeneic HCT.
3. Assess the impact of somatic mutations on HCT outcomes after adjustment for other clinical risk factors.

Status: **Protocol development.**

LK22-01 Impact of pre-allogeneic hematopoietic cell transplantation therapy in acute myeloid leukemia and myelodysplastic syndrome on post-transplant outcomes (Ali N).

The purpose of this study is to:

1. Compare clinical outcomes of patients with AML and MDS undergoing alloHCT in first complete remission and receiving low intensity vs. high intensity induction therapies.
2. Compare clinical outcomes of patients with MDS with <5% BM blasts or MDS-EB1 with 5-9% BM blasts undergoing Allo-HCT with low intensity/HMA vs. no pre-HCT therapy.

Status: **Data File Preparation.**

CK22-02 Toxicity and survival of AML/MDS patients receiving allogeneic stem cell transplantation using reduced-intensity conditioning: A propensity score analysis. (P Kongtim/ A Portuguese/ S Ciurea/ B Scott).

The purpose of this study is to:

1. Compare progression-free survival (PFS) among five commonly used RIC/NMA conditioning regimens: FM100 (fludarabine + melphalan 100 mg/m^2), FM140 (fludarabine + melphalan 140 mg/m^2), FB (fludarabine + 2 days of busulfan 4 mg/kg/day PO or 3.2 mg/kg/day IV), FCT

(fludarabine, cyclophosphamide 14.5 mg/kg/day × 2 days, and 2 Gy TBI), and FT (fludarabine and 2 Gy TBI).

2. Compare other clinical outcomes across these five conditioning regimens.

Status: **Data File Preparation.**

CK23-01 Identifying the Optimal Graft-versus-Host Disease Regimen in Allogeneic Transplantation for Myelofibrosis (S Patel/ D Courier).

The purpose of this study is to:

1. Identify the optimal GVHD prophylaxis strategy in allogeneic HCT for primary and secondary myelofibrosis (MF), as assessed by GVHD-free/relapse-free survival (GFRS) and the incidence and severity of acute and chronic GVHD.
2. Evaluate risk factors for engraftment failure in patients receiving ATG versus PTCy, and characterize GFRS and GVHD outcomes in MF patients with impaired renal function.
3. Assess the impact of pre-transplant ruxolitinib use and different GVHD prophylaxis strategies on engraftment and overall HCT outcomes.

Status: **Protocol Received.**

LK23-01a The impact of allogeneic stem cell transplantation on acute myeloid leukemia and myelodysplastic syndrome with chromosome 3 abnormalities (A Datt Law).

The purpose of this study is to:

1. Compare clinical outcomes of patients with AML and MDS with chromosome 3 abnormalities undergoing allogeneic hematopoietic stem cell transplantation (allo-HCT).
2. Assess whether allo-HCT improves survival and reduces relapse risk in this high-risk subgroup, addressing gaps in the current literature.

Status: **Protocol Development**

LK23-02 Prognostic impact of cytogenetic and molecular risk classification in AML after hematopoietic stem cell transplant in adolescents and young adults (H Lust).

The purpose of this study is to:

1. Describe the cytogenetic and molecular signature in AYA patients with AML receiving allogeneic SCT.
2. Describe the prognostic significant of ELN2022 cytogenetic risk stratification in AYA patients with AML receiving allogeneic SCT.

Status: **Protocol Development**

LK23-03 Impact of donor source in second allogeneic hematopoietic cell transplant in patients with acute leukemia/MDS who relapsed after prior allograft during the current era (2014-2020) (A Troullioud Lucas/ A Scaradavou).

The purpose of this study is to:

1. Compare clinical outcomes of patients undergoing second allogeneic hematopoietic stem cell transplantation (HCT-2) for relapsed hematologic malignancies, stratified by donor type.
2. Assess the impact of donor characteristics and transplant-related factors on survival and relapse outcomes in this patient population.

Status: **Protocol Development.**

CK24-01 Identifying the optimal stem cell dosing for peripheral blood stem cell transplantation with post-transplant cyclophosphamide. (H Elmariah/ A Gandhi/ N Bejanyan/ R Marziarz

The purpose of this study is to:

1. Evaluate the impact of infused $CD34^+$ cell dose on overall survival (OS) and other transplant outcomes (engraftment, GVHD, relapse, non-relapse mortality, disease-free survival (DFS), and GVHD-free relapse-free survival (GRFS)) following allogeneic PBSCT with PTCy.
2. Assess the effect of $CD34^+$ cell dose on OS and transplant outcomes in key subgroups, including haploidentical donor PBSCT and patients with myelofibrosis (MF).
3. Test the hypothesis that a $CD34^+$ cell dose $> 5 \times 10^6$ $CD34^+$ cells/kg is associated with improved OS in this setting.

Status: **Protocol Development.**

LK24-01a Safety and efficacy of CAR-T cell therapy in relapsed/refractory acute lymphoblastic leukemia with central nervous system involvement (L F Gonzalez Mosquera/ S Farhan).

The purpose of this study is to:

1. To evaluate Event Free Survival (EFS) in ALL pts with CNS 2/3 : defined as time from CAR-T infusion to leukemia release, failure to achieve remission, or death Patients will be censored at time of last follow up.
2. To identify clinical, disease, and treatment-related factors associated with poor outcomes and toxicity in R/R ALL with CNS involvement.

Status: **Protocol Development.**

LK24-01b Sequencing of chimeric antigen receptor T-cell therapy and allogeneic transplantation in adult patients with B-cell acute lymphoblastic leukemia (D Eng/ J Fein/ A Arteaga/ M Kharfan-Dabaja/ L Metheny/ R Mohty/ H Sibai/ J Wang).

The purpose of this study is to:

1. Evaluate overall survival in patients with relapsed/refractory (R/R) B-ALL who undergo consolidative allo-HCT following CAR-T, compared with CAR-T recipients who do not undergo allo-HCT.
2. Evaluate overall survival in R/R B-ALL patients who receive CAR-T after a prior allo-HCT, compared with CAR-T recipients without prior allo-HCT.

Status: **Protocol Development.**

LK24-01c Real World Experience (RWE) of adult patients receiving CD19 CAR-T cells for B cell Acute Lymphoblastic Leukemia (B-ALL): A CIBMTR Analysis. (A-S Mirza/ M Bilal Abid/ K Wudhikarn/ L Gowda/ MA Perales/ N Bejanyan).

The purpose of this study is to:

1. To estimate PFS of patients with B-ALL receiving CD19+ CAR-T cells (tisa-cel & brexu-cel).
2. To examine the impact of age on efficacy of CAR-T.

Status: **Protocol Development**.

CK24-02 Outcomes of allogeneic hematopoietic stem cell transplantation in patients with DDX41-mutated myelodysplastic syndrome and acute myeloid leukemia. (R Stubbins/ E Wong/ L Fox/ L Gowda/ S Seropian).

The purpose of this study is to:

1. Determine whether patients with DDX41-mutated (mt) MDS/AML undergoing first allo-HSCT have a higher rate of non-relapse mortality (NRM) compared with DDX41–wild-type (wt) patients, and to identify patient- and transplant-specific factors that predict NRM in this group.
2. Describe clinical outcomes in DDX41-mt MDS/AML patients undergoing first allo-HSCT, including risks of GVHD, relapse, duration of remission, causes of death, and survival, and identify biological correlates of these outcomes.

Status: **Protocol Development.**

CK24-03 Comparison of reduced intensity conditioning regimens for haploidentical donor hematopoietic cell transplant with post-transplant cyclophosphamide in patients with acute myeloid leukemia or myelodysplastic syndromes. (H Elmariah/ S Arslan/ M Al Malki/ N Bejanyan).

The purpose of this study is to:

1. Evaluate the impact of haploidentical allogeneic HCT with PTCy-based GVHD prophylaxis on disease-free survival (DFS) and other transplant outcomes, including overall survival (OS), relapse, non-relapse mortality (NRM), GVHD, engraftment, and GVHD-free relapse-free survival (GRFS) in patients with AML or MDS.
2. Assess outcomes in adult patients aged 18–75 years who receive reduced-intensity conditioning (RIC) haploidentical allo-HCT with PTCy.

Status: **Protocol development.**

CK24-04 Comparison of post-transplant cyclophosphamide-based reduced intensity conditioning regimens for older patients with acute myelogenous leukemia and MDS. (L Bachier/ S Solomon).

The purpose of this study is to:

1. Identify the fludarabine-based conditioning regimens that provide the best relapse-free survival (RFS) in older patients (≥50 years) with AML or MDS undergoing first allogeneic HCT with PTCy as GVHD prophylaxis.
2. Evaluate non-relapse mortality (NRM), relapse/progression, overall survival (OS), current RFS (cRFS), GVHD-free relapse-free survival (GRFS), acute GVHD, chronic GVHD, and engraftment associated with these regimens.

Status: **Protocol development.**

LK25-01 Comparison of FluFTBI and other myeloablative Conditioning Regimens for Haploidentical and mismatched unrelated Hematopoietic Cell Transplant with Post-Transplant Cyclophosphamide in Patients with Acute Leukemia. (S Arslan/ M Al Malki).

The purpose of this study is to:

1. Evaluate HCT outcomes in patients with AML and ALL who underwent haploidentical or MMUD HCT with PTCy using myeloablative conditioning (MAC) with either FluFTBI or other MAC regimens and were reported to the CIBMTR.
2. Compare overall survival (OS), non-relapse mortality (NRM), relapse, progression-free survival (PFS), and leukemia-free survival (LFS) between FluFTBI-based MAC and other MAC regimens.

Status: **Protocol Pending.**

LK25-02 Myelodysplastic Neoplasms with Hypoplasia (MDS-h) or Fibrosis (MDS-f): Distinct Clinical Entities Compared to Other MDS Subtypes. (A Law/ S Rodriguez).

The purpose of this study is to:

1. Assess overall survival (primary outcome), measured from transplant to last follow-up or death.
2. Evaluate key secondary outcomes, including:
   - Neutrophil and platelet engraftment, primary and secondary graft failure
   - Cumulative incidence of relapse and non-relapse mortality (with appropriate competing risks)
   - Incidence of acute and chronic GVHD (using competing risk methods)
   - GVHD-free, relapse-free survival from transplant to GVHD, relapse, or last follow-up.

Status: **Protocol Pending.**

LK25-03 Impact of Post-Transplant Cyclophosphamide Based GVHD prophylaxis on Outcomes in Patients with CMML Undergoing Allogeneic Stem Cell Transplant. (Y Berry/ S Farhan/ I Varadarajan/ K Ball).

The purpose of this study is to:

1. Evaluate allogeneic HCT outcomes in patients with CMML in the post–PTCy era, including the impact of treatment-related and disease risk factors, and to provide comparative data that have been lacking in prior (pre-PTCy) analyses.
2. Assess the feasibility and outcomes of using haploidentical and $\leq$7/8 MMUD donors with PTCy in CMML, with a particular focus on GVHD-free, relapse-free survival (GRFS) and the role of allo-HCT as the only potentially curative option.

Status: **Protocol Pending.**

Field	Response
Proposal Number	PROP 2505-02; 2509-43
Proposal Title	**Allogeneic Stem Cell Transplant Outcomes of Acute Leukemias of Ambiguous Lineage and Prognostic Model in Mixed Phenotype Acute Leukemia.**
Key Words	Acute Leukemia of Ambiguous Lineage, Mixed Phenotype Acute Leukemia, Acute Undifferentiated Leukemia, Hematopoietic Stem Cell Transplantation
Principal Investigator #1: - First and last name, degree(s)	Seda Cakmak Xiao-Hui Zhang
Principal Investigator #1: - Email address	seda.cakmak@uhn.ca 1710301242@pku.edu.cn
Principal Investigator #1: - Institution name	Hans Messner Allogeneic Blood and Marrow Transplantation Program Princess Margaret Cancer Centre, University of Toronto Peking University People's Hospital
Principal Investigator #1: - Academic rank	Clinical Fellow Associate Director, Peking University Institute of Hematology, National Clinical Research Center for Hematologic Disease Chair-elect, Chinese Society of Hematology
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes No
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Auro Viswabandya
Principal Investigator #2 (If applicable): - Email address:)	auro.viswabandya@uhn.ca
Principal Investigator #2 (If applicable): - Institution name:	Hans Messner Allogeneic Blood and Marrow Transplantation Program Princess Margaret Cancer Centre, University of Toronto
Principal Investigator #2 (If applicable): - Academic rank:	Associate Professor
Junior investigator status (defined as 㑊、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:	Seda Cakmak Xiao-Hui Zhang

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:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	What are the outcomes of adult patients with subtypes of acute leukemia of ambiguous lineage (ALAL) after allogeneic hematopoietic stem cell transplantation (allo-HSCT)? Can our machine learning model developed from the largest Chinese mixed phenotype acute leukemia (MPAL) transplant cohort (n=254, AUC=0.82) be validated in the CIBMTR international cohort to establish a globally applicable prognostic tool for MPAL patients undergoing allo-HSCT?
RESEARCH HYPOTHESIS:	Adult patients with subtypes of ALAL who undergo allo-HSCT in remission can achieve survival outcomes comparable to those of other poor-risk acute leukemias after transplant. Our prognostic model (presented at EHA 2024), which integrates dynamic measurable residual disease (MRD) trajectories and immunophenotypic plasticity, will demonstrate superior generalizability in the CIBMTR cohort compared to conventional risk scores (ELN/HCT-CI), enabling risk-stratified post-transplant management across diverse populations.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Objective: Evaluate the overall survival (OS) of adult patients with ALAL who undergo allo-HSCT with subgroup analysis of acute undifferentiated leukemia (AUL) and MPAL patients. OS will be assessed at defined time points (1-year, 3-year,5-year post-transplant) and measured from the date of transplant to death from any cause, with surviving patients censored at last follow-up. Validate the performance (AUC, sensitivity, specificity) of the machine learning-based prognostic model for predicting 3-year OS in the CIBMTR cohort. Secondary Objectives: Leukemia-free survival (LFS) of adult ALAL patients after allo-HSCT. Non-relapse mortality (NRM) at 100 days, 1 year, and 3 years post-transplant. Analyze whether patient and disease characteristics (pre-transplant factors) such as cytogenetic abnormalities, molecular mutations, disease and MRD status at transplant, performance status, and comorbidities correlate with OS or leukemia free survival (LFS). We will use both univariate and multivariate approaches to identify significant predictors of poor outcome. Analyze transplant-related factors such as donor type, HLA match, conditioning intensity, graft source, use of T-cell depletion or specific graft-versus-host disease (GVHD) prophylaxis regimens, and development of acute or chronic GVHD. We will assess how these factors impact relapse risk, NRM, and OS. Describe the incidence of clinically significant post-transplant complications, including acute GVHD (grades II-IV and III-IV), chronic GVHD, veno-occlusive disease (VOD)/sinusoidal obstruction syndrome, and opportunistic infections (e.g. CMV reactivation, EBV-associated post-transplant lymphoproliferative disorder). Compare our prognostic model's predictive accuracy against conventional risk indices (ELN 2022, HCT-CI) and develop a risk-adapted algorithm for post-transplant interventions.

SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Despite the clinical severity of ALAL, evidence guiding optimal transplant timing, prognostic stratification, and management remains extremely limited. The proposed CIBMTR analysis will generate a comprehensive dataset for AUL, re-evaluate MPAL outcomes in the modern transplant era and potentially validate a prognostic scoring tool. With far less than 100 total cases reported in the literature, AUL is one of the least understood acute leukemias in terms of optimal therapy and expected outcomes after transplant. This study will be the largest analysis to date focused on AUL patients undergoing allogeneic HSCT. The findings will have several important impacts: - Set **benchmark outcome** data for AUL after transplant by establishing 1-year and 3-year survival rates, relapse rates, and NRM in a sizable cohort, Clinicians will have evidence-based estimates to inform prognosis for AUL patients undergoing HSCT for the first time, rather than relying on anecdotal or extrapolated data. - Identification of key **prognostic factors** (for example, the influence of cytogenetics, remission status at transplant) will help refine patient selection in AUL. If certain high-risk features portend especially poor outcomes even with transplant, patients with those features might be candidates for novel strategies such as experimental therapies or augmented transplant approaches. Conversely, if outcomes are favorable in subsets (e.g. AUL in CR1 with standard-risk cytogenetics), that reinforces the benefit of proceeding to transplant in those cases. - The results will clarify whether allogeneic transplant can overcome the historically poor prognosis of AUL, particularly when performed in CR1. If our hypothesis that AUL transplanted in CR1 can have outcomes comparable to other high-risk leukemias is supported, it validates current practice of pursuing transplant aggressively in AUL. On the other hand, if outcomes are significantly worse than expected even in CR1, it would indicate a need for improved pre- or post-transplant strategies (such as maintenance therapies, novel conditioning, etc.). Additionally, analysis of transplant factors like conditioning intensity or donor source could **inform best practices** specifically for AUL. - This study will **generate hypotheses** for prospective evaluation. For instance, if MRD emerges as a critical predictor of relapse in AUL (similar to findings in AML/ALL), future trials could test interventions in MRD-

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	positive AUL prior to or after transplant. Moreover, our findings could encourage inclusion of AUL patients in clinical trials (perhaps grouped with other high-risk acute leukemias or studied in collaborative efforts due to rarity). Observational studies on MPAL either predates the modern transplant era with increasing post transplant cyclophosphamide (PTCy) use, haploidentical donors, improved HLA typing and modern supportive care or transplant related factors are not described. This study would provide the first large scale evaluation of MPAL transplant outcomes in the contemporary era, help identify transplant related predictors of relapse and clarify whether modern allo-HSCT outcomes now approximate those of AML or ALL. It will also determine whether PTCy mitigates the previously observed elevated risk of GVHD in MPAL compared to other acute leukemias. Prognostication tools in MPAL are limited. Our machine-learning prognostic tool developed from the largest Chinese MPAL transplant cohort (n=254, 2005–2024) demonstrated strong predictive accuracy (AUC 0.82) for 3-year OS. However, external validation is essential before clinical implementation. Using CIBMTR data allows independent validation across ethnically diverse populations. If validated, this model can guide risk-adapted transplant decisions, such as identifying patients who require intensified conditioning or post-transplant maintenance. In conclusion, the proposed CIBMTR study will produce the most comprehensive and clinically relevant analysis of ALAL transplantation outcomes in the modern era. It will fill critical knowledge gaps for AUL, re-define transplant expectations for MPAL in the modern era, and validate a novel prognostic tool capable of transforming risk stratification and individualizing care. The findings will directly impact clinical management, inform guidelines, and lay essential groundwork for future therapeutic innovation in this underserved leukemia population.

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.	Acute leukemias of ambiguous lineage (ALAL) are rare high-risk leukemias defined by the absence of clear lineage commitment. In the WHO classification, ALAL includes acute undifferentiated leukemia (AUL) characterized by blasts lacking myeloid or lymphoid lineage markers and mixed-phenotype acute leukemia (MPAL), which demonstrates co-expression of lineage-defining markers. (1) MPAL accounts for the majority of ALAL and represents approximately 0.6% of all acute leukemias.(2) Both AUL and MPAL exhibit poor outcomes and lack standardized treatment algorithms. A population-based SEER analysis showed significantly inferior survival in MPAL, with the risk of death increased by 59% compared with AML and 26% compared with ALL, though treatment details were unavailable.(2) AUL is similarly aggressive; historical median overall survival in adults is approximately 9 months, worse than de novo AML or ALL.(3) AUL frequently harbors adverse cytogenetic features such as complex karyotypes and 5q deletions.(4) Optimal induction therapy is undefined, though in one analysis of ALAL containing 16 AUL and 26 MPAL cases, ALL-based regimens achieved higher complete remission (CR) rates (40%) than AML-type regimens (22%).(5) Long-term survival in this study was observed only in patients who underwent allogeneic stem cell transplantation, underscoring its potential importance. This aligns with the general view that ALAL, like other high-risk acute leukemias, likely requires consolidation with allogeneic transplantation at CR1 to maximize the chance of cure. (9) Transplant outcomes in AUL remain poorly described, with fewer than ~20 adult cases reported. In the largest series to date (n=10), Kurosawa et al. demonstrated poor overall outcomes but improved survival in patients transplanted in CR1 and without cytogenetic abnormalities.(6)Earlier case series similarly identified allo-HSCT as the only route to durable remission.(5) These data support allo- HSCT as a necessary therapeutic strategy in AUL, yet evidence is insufficient to guide practice. In contrast, allo-HSCT outcomes in MPAL have been better documented. Tian et al. demonstrated markedly superior 3-year OS with transplantation (77% vs. 16%).(7) The largest study, a CIBMTR analysis of 95 MPAL patients (1996–2012), reported 3-year OS 67%, relapse 29%, and NRM 15%, with outcomes comparable to AML/ALL.(8) MPAL recipients had higher rates of acute GVHD and a trend toward more chronic GVHD.

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	However, these data reflect transplant practices prior to widespread use of post-transplant cyclophosphamide (PTCy) and modern donor selection, conditioning, and supportive strategies. More recently, Goulart et al reported outcomes of 42 patients with newly diagnosed MPAL however only 12 underwent allo-HSCT and the transplant details were not described. Also, transplant outcomes of adult MPAL patients on a Japanese registry have reported similar findings including more frequent GVHD compared to AML and ALL patients. (10) Notably, GVHD prophylaxis did not include PTCy. They have also defined an MPAL posttransplant prognostic score system with 6 variables based on their multivariate analysis and evaluated in a validation cohort however it remains unclear if it will be applicable to a more heterogenous population. Overall, prognostic factors for MPAL remain poorly defined, with inconsistent results across small studies. No validated prognostic scoring system exists to stratify risk or inform transplant decisions. We have developed a prognostic model trained on a large Chinese MPAL transplant cohort (n=254, 2005–2024) which showed a strong predictive performance (AUC 0.82 for 3-year OS) and identified novel biomarkers potentially linked to marrow niche remodeling (EHA 2024 Oral Abstract). Independent validation in a diverse CIBMTR cohort is essential to determine the generalizability of this tool which could be utilized to assist in pre transplantation risk assessment and stratification. Given the knowledge gaps in this rare but high risk acute leukemia group, a comprehensive CIBMTR study of all ALAL patients undergoing allo-HSCT will provide a large dataset needed to define transplant outcomes in AUL (an area with minimal existing evidence), reassess MPAL outcomes in the modern GVHD-prophylaxis era, and validate a novel MPAL prognostic model. Such data are critical to improving risk-stratification, guiding transplant decision-making, and informing future clinical trial development.
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Id	
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Name	
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Size	

Field	Response
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Type	

PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	All adult patients (age 18 years) with ALAL (as per WHO 2022 criteria) who underwent allogeneic hematopoietic stem cell transplantation between January 1, 2000 and December 31, 2024 and are reported to the CIBMTR will be included. This will be a retrospective cohort drawn from the CIBMTR database. Inclusion Criteria: Patients 18 years old at time of transplant, diagnosed with ALAL. AUL and MPAL are defined according to WHO 2022 criteria Receipt of an allogeneic hematopoietic stem cell transplant (from any donor source, any graft source) during the period 2000-2024, reported to CIBMTR, with the indication for transplant being treatment of ALAL. This includes transplants in first remission, beyond first remission, or for primary refractory disease. Sufficient data available in the registry on pre-transplant disease characteristics, transplant details, and outcomes (patients must have the necessary CIBMTR forms completed as outlined in Data Requirements). For centers contributing data, a minimum follow-up of 6 months post-transplant is required for surviving patients (to ensure adequate short-term outcome data such as day+100 NRM and engraftment). Exclusion Criteria: Patients with blast crisis of chronic myeloid leukemia or other antecedent hematologic malignancies that transformed, if they were not clearly distinguished as ALAL. Prior solid organ transplantation Inherent germline conditions known to predispose to leukemia (e.g., Fanconi anemia, Li-Fraumeni syndrome, etc.) if reported, will be excluded if the transplant was done in that specific context. (Rationale: such cases may have different biology and transplant risk, and are typically very few;

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	excluding ensures a more homogeneous cohort of sporadic ALAL.) Patients lacking critical outcome data or lost to follow-up immediately post-transplant (e.g. no survival status or relapse information reported). These cases will be excluded from analysis due to incomplete data. Note: Prior history of a different malignancy will not be an exclusion criterion unless the diagnosis was in fact secondary acute leukemia or unless therapy for that prior malignancy directly led to an acute leukemia that is better classified as therapy-related AML/ALL. Patients who have a prior malignancy and then develop true AUL (with no lineage markers) or MPAL will remain eligible, as long as they fit the above inclusion criteria (we will capture prior therapy in patient data for analysis).
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	Paediatric patients were excluded to ensure a more homogenous cohort, as acute leukemias in children differ from adults in their underlying biology,treatment approaches, and post-transplant outcomes. Restricting the study to adults allows for more accurate and clinically meaningful conclusions within this population.

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.	Patients included will have data reported to the CIBMTR via standard reporting forms. We will utilize a combination of TED (Transplant Essential Data) level and comprehensive report forms to capture all relevant information. Only patients from centers that fulfill required follow-up (e.g. 6-month and 1-year follow-up reporting) are included to ensure outcome data availability. Patient and disease variables from; - Recipient Baseline Data (Form 2000): Age at transplant, sex, race/ethnicity, performance status (Karnofsky or ECOG), significant comorbidities (HCT-CI score), date of initial ALAL diagnosis. - Infectious Disease Markers (Form 2004): Serostatus for CMV,EBV, HIV, hepatitis, etc. (to assess baseline risk factors). - AML Pre-HCT Data (Form 2010 - used for ALAL): Disease features at diagnosis and pre-transplant: presenting WBC count; immunophenotype confirming ALAL, cytogenetic results (conventional karyotype and FISH) e.g. presence of complex karyotype, monosomies, 11q23 (KMT2A) rearrangement, etc.; molecular genetics (mutation status if reported, e.g. IDH1/2, FLT3, etc.); any CNS involvement at diagnosis; therapy prior to transplant (induction regimen details, number of cycles, CR achievement); date of CR1 attainment; disease status at transplant (CR1, CR2, refractory, etc.) and MRD status if assessed. - Pre-transplant Essential Data (Form 2400): Interval from diagnosis to transplant; pre-transplant conditioning regimen planned (MAC vs RIC); risk stratification (if center assigned any risk level); donor type intended. (Note: Some of these may overlap with 2402 and 2000.) - Disease Classification(Form 2402): Verification of disease classification as AUL/MPAL (to ensure patients are correctly categorized); this form captures the specific disease code for AUL and MPAL under ALAL. - Recipient Eligibility Form (Form 2500): Verifies that the recipient meets inclusion (e.g. no duplicate registration); also captures if the case was part of any study or trial (for context). Transplant variables from; - Hematopoietic Stem Cell Transplant (Product) Infusion (Form 2006):Date of HSCT; graft source (bone marrow, peripheral blood, or cord blood); total nucleated cell dose (if cord); CD34+ cell dose (if

	reported for PBSC); donor type (HLA-matched sibling,matched unrelated, mismatched unrelated,haploidentical, other); degree of HLA match (8/8, 7/8, etc.); donor recipient sex match; ABO blood type match; donor age. - Confirmation of HLA Typing (Form2005): High-resolution HLA typing results of donor and recipient (to confirm matching and assess any allele-level mismatch not captured on 2006). -Conditioning Regimen (reported in 2400/2006): Conditioning intensity: Myeloablative (MAC) vs Reduced-Intensity (RIC) vs Nonmyeloablative; specific agents used (e.g. Bu/Cy, FLAMSA, Mel/Flu, etc.) and doses. This allows grouping by intensity and agent. -GVHD Prophylaxis (reported in 2006): Agents for GVHD prophylaxis: e.g. calcineurin inhibitor (CNI) based (CSA or tacrolimus)±methotrexate, use of ATG or alemtuzumab (T-cell depletion), post-transplant cyclophosphamide, etc. We will record presence of in vivo T-cell depletion and the general regimen type. -Donor lymphocyte infusion (DLI) indication; although not a standard form, if reported, we will note any planned DLI or use of DLI post-transplant (for pre-emptive or relapse therapy). Post transplant outcomes from; - Post-Transplant Data (Form 2100): Engraftment: Date of neutrophil engraftment; date of platelet engraftment; graft failure (primary or secondary) and date. Acute GVHD: occurrences of grade II−IV acute GVHD by day +100 (and grade III IV subset). Chronic GVHD: occurrence of chronic GVHD by 1 year (limited vs extensive, or NIH mild/moderate/severe if available). Early toxicities: occurrence of hepatic VOD (yes/no, and severity if graded); engraftment syndrome (if captured); other major organ toxicities. - Infectious Disease Post-Tx(Form 2150): Viral reactivations/infections: CMV reactivation (yes/no and date of first reactivation); EBV reactivation or PTLD (yes/no); adenovirus, HHV-6,BK virus reactivation (if reported); fungal infections (invasive aspergillosis etc., if captured). - Post-Transplant Essential Data (Form 2450): Survival status and events: Date of last follow-up; survival status (alive, dead with cause of death); cause of death (CIBMTR coding: relapse, infection, organ failure, GVHD, etc.). Relapse/progression: relapse or progression occurrence (yes/no) and date; site of relapse (marrow, CNS, etc.), and whether based on morphology or molecular criteria. Subsequent

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	therapy: any second hematopoietic cell transplant (yes/no and date); use of DLI for relapse. Disease status at last follow-up: alive in remission, alive with disease, etc. These data will be used to calculate LFS and conditional survival. Note: All data will be obtained through the existing CIBMTR database; no new patient contact or sample collection is involved. The forms listed (with form numbers) correspond to the standardized CIBMTR case report forms in use for the relevant data (e.g., form 2010 for AML covers pre-transplant AML-specific data, which for ALAL cases is the form likely utilized under the ambiguous lineage category). Data completeness will be checked, and any missing critical data elements will be handled via standard CIBMTR queries if possible or those cases may be excluded from specific analyses.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
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	No biological samples are needed for this retrospective registry study.

REFERENCES:	1. Khoury JD, Solary E, Abla O, Akkari Y, Alaggio R, Apperley JF, et al. The 5th edition of the World Health Organization Classification of Haematolymphoid Tumours: Myeloid and Histiocytic/Dendritic Neoplasms. Leukemia. 2022 July;36(7):1703–19. 2. Shi R, Munker R. Survival of patients with mixed phenotype acute leukemias: A large population-based study. Leuk Res. 2015 June;39(6):606–16. 3. Qasrawi A, Gomes V, Chacko CA, Mansour A, Kesler M, Arora R, et al. Acute undifferentiated leukemia: data on incidence and outcomes from a large population-based database. Leuk Res. 2020 Feb;89:106301. 4. Department of Clinical Hematology, National Institute of Blood Disease & Bone Marrow Transplantation, Karachi, Pakistan, Abbas N, Waheed S, Department of Hematology, National Institute of Blood Disease & Bone Marrow Transplantation, Karachi, Pakistan, Jamal A, Department of Clinical Hematology, National Institute of Blood Disease & Bone Marrow Transplantation, Karachi, Pakistan, et al. Acute Leukemia of Ambiguous Lineage with a Rare Abnormality Del17p by FISH Analysis. Natl J Health Sci. 2020 Oct 21;5(2):79–82. 5. Heesch S, Neumann M, Schwartz S, Bartram I, Schlee C, Burmeister T, et al. Acute leukemias of ambiguous lineage in adults: Molecular and clinical characterization. Ann Hematol. 2013 Feb 15;92. 6. Kurosawa S, Toya T, Kishida Y, Nagata A, Yamada Y, Konishi T, et al. Outcome of patients with acute undifferentiated leukemia after allogeneic hematopoietic stem cell transplantation. Leuk Lymphoma. 2018 Dec;59(12):3006–9. 7. Tian H, Xu Y, Liu L, Yan L, Jin Z, Tang X, et al. Comparison of outcomes in mixed phenotype acute leukemia patients treated with chemotherapy and stem cell

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	transplantation versus chemotherapy alone. Leuk Res. 2016 June 1;45:40–6. 8. Munker R, Brazauskas R, Wang HL, de Lima M, Khoury HJ, Gale RP, et al. Allogeneic Hematopoietic Cell Transplantation for Patients with Mixed Phenotype Acute Leukemia. Biol Blood Marrow Transplant. 2016 June 1;22(6):1024–9. 9. Goulart H, Ravandi F, Short NJ, Jain N, Daver N, Kadia TM, et al. Clinical Outcomes of Adult Patients With Newly Diagnosed Mixed Phenotype Acute Leukemia. JCO Precis Oncol. 2025 Dec;(9):e2500494. 10. Jo T, Kondo T, Mizuno S, Kako S, Doki N, Uchida N, et al. Analyses of transplantation outcomes for adult patients with mixed-phenotype acute leukemia. Blood Neoplasia. 2025 Aug 25;2(4):100166. 11. TONG K T, ZHANG X H. Development and validation of a prognostic model for mixed phenotype acute leukemia patients treated with allogeneic hematopoietic stem cell transplantation in a nationwide multicenter study[C]//Proceedings of the 29th Annual Meeting of the European Hematology Association (EHA). Madrid, Spain, 2024 Jun 13-16. Abstract S266.
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

Table 1. Characteristics of adult patients who received first allogenic transplant with acute undifferentiated leukemia, mixed phenotype acute leukemia or acute leukemia of ambiguous lineage at U.S. during 2008-2024

Characteristic	N (%)
No. of patients	581
Patient-related Characteristics	
Age, by decades, no. (%)	
Median (range)	50 (18-77)
18-19	25 (4)
20-29	96 (17)
30-39	86 (15)
40-49	83 (14)
50-59	115 (20)
60-69	131 (23)
70+	45 (8)
Sex, no. (%)	
Male	329 (57)
Female	252 (43)
Karnofsky score prior to HCT, no. (%)	
90-100%	318 (55)
< 90%	253 (44)
Not reported	10 (2)
Race, no. (%)	
White	428 (74)
Black or African American	57 (10)
Asian	35 (6)
Native Hawaiian or other Pacific Islander	4 (1)
American Indian or Alaska Native	5 (1)
More than one race	7 (1)
Not reported	45 (8)
HCT-CI, no. (%)	
0	125 (22)
1	86 (15)
2	90 (15)
3	114 (20)
4	70 (12)
5+	94 (16)
Not reported	2 (<1)
Disease-related Characteristics	

Characteristic	N (%)
Specify ALL classification, no. (%)	
Acute undifferentiated leukemia:	130 (22)
Mixed phenotype acute leukemia (MPAL) with t(9;22)(q34.1;q11.2); BCR-ABL1	80 (14)
Mixed phenotype acute leukemia with t(v; 11q23.3); KMT2A rearranged	18 (3)
Mixed phenotype acute leukemia, B/myeloid, NOS	143 (25)
Mixed phenotype acute leukemia, T/myeloid, NOS	159 (27)
Other acute leukemia of ambiguous lineage or myeloid neoplasm	50 (9)
Mixed-phenotype acute leukemia, rare types	1 (<1)
Disease category, no. (%)	
Acute Undifferentiated Leukemia (AUL)	130 (22)
Mixed Phenotype Acute Leukemia (MPAL)	401 (69)
Acute Leukemia of Ambiguous Lineage	50 (9)
What was the disease status (based on hematological test results)?, no. (%)	
Primary induction failure	41 (7)
1st complete remission	490 (84)
2nd complete remission	31 (5)
1st relapse	10 (2)
>= 3rd complete remission	5 (1)
>= 3rd relapse	1 (<1)
Never treatment	2 (<1)
Not reported	1 (<1)
WBC at diagnosis x 10*9/L, no. (%)	
<10	54 (9)
10-100	25 (4)
>100	9 (2)
Both dose and units not reported	493 (85)
Transplant-related Characteristics	
Donor type, no. (%)	
HLA identical sibling	133 (23)
Haploidentical donor	114 (20)
Other related	3 (1)
Well-matched unrelated (8/8)	234 (40)
Partially-matched unrelated (7/8)	46 (8)
Mismatched unrelated (<= 6/8)	8 (1)
Multi-donor	3 (1)
Unrelated (matching cannot be determined)	19 (3)
Cord blood	21 (4)
Product type, no. (%)	

Characteristic	**N (%)**
BM	81 (14)
PBSC	478 (82)
UCB	22 (4)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)	
MAC	360 (62)
RIC	168 (29)
NMA	32 (6)
Not reported	21 (4)
Conditioning regimen, no. (%)	
TBI/Cy	107 (18)
TBI/Cy/Flu	51 (9)
TBI/Cy/Flu/TT	7 (1)
TBI/Cy/TT	2 (<1)
TBI/Cy/VP	3 (1)
TBI/VP	25 (4)
TBI/Mel	29 (5)
TBI/Flu	83 (14)
TBI/other(s)	8 (1)
Bu/Cy	27 (5)
Bu/Mel	2 (<1)
Flu/Bu/TT	23 (4)
Flu/Bu	111 (19)
Flu/Mel/TT	14 (2)
Flu/Mel	76 (13)
Mel alone	2 (<1)
Mel/other(s)	2 (<1)
Treosulfan	1 (<1)
TLI	1 (<1)
Other(s)	7 (1)
GVHD prophylaxis, no. (%)	
Ex-vivo T-cell depletion	8 (1)
CD34 selection	10 (2)
PtCy + other(s)	231 (40)
PtCy alone	2 (<1)
TAC + MMF +- other(s) (except PtCy)	40 (7)
TAC + MTX +- other(s) (except MMF, PtCy)	227 (39)
TAC + other(s) (except MMF, MTX, PtCy)	20 (3)
TAC alone	9 (2)

Characteristic	N (%)
CSA + MMF +- other(s) (except PtCy,TAC)	17 (3)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	13 (2)
Other(s)	1 (<1)
Missing	3 (1)
Time from diagnosis, no. (%)	
0-6 months	372 (64)
6-12 months	166 (29)
>= 12 months	43 (7)
Year of current transplant, no. (%)	
2008	2 (<1)
2009	4 (1)
2010	4 (1)
2011	5 (1)
2012	10 (2)
2013	11 (2)
2014	10 (2)
2015	9 (2)
2016	8 (1)
2017	42 (7)
2018	41 (7)
2019	73 (13)
2020	65 (11)
2021	68 (12)
2022	73 (13)
2023	81 (14)
2024	75 (13)
Median follow-up of survivors (range), months, median (range), months	37.5 (3.2-189.0)

Field	Response
Proposal Number	2508-03-HOSSAIN
Proposal Title	Impact of racial and socio-economic factors on timely referral for Allogeneic stem cell transplant for the treatment of MPNs and MDS: A CIBMTR Treport
Key Words	Minority Population, Socio-economic Factors, Allogeneic stem cell transplant, MDS, MPN
Principal Investigator #1: - First and last name, degree(s)	Nasheed M. Hossain MD
Principal Investigator #1: - Email address	nasheed.hossain@pennmedicine.upenn.edu
Principal Investigator #1: - Institution name	University of Pennsylvania
Junior investigator status (defined as 㑊、5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
Do you identify as an underrepresented/minority?	No
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	Yes, I am a junior investigator and would like assistance identifying a senior mentor for my project
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	LY22-02 - Co Investigator - protocol development, data analysis, manuscript preparation CT22-02 - Co Investigator - leading in concept design, protocol development and will be involved in data analysis and final manuscript/abstract preparation CT21-01 - protocol development CT20-03 - protocol development, CK21-01 = protocol development GV210-2 - protocol development LK21-01- protocol development and review GV18-01a-protocol development, manuscript review GV18-01b-protocol development, manuscript review MM20-02a - protocol development, data review, manuscript review
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:	Does a patient's racial and socio-economic background impact timely referrals for transplant evaluation in the treatment of MDS and MPNs?

Field	Response
RESEARCH HYPOTHESIS:	We hypothesize that based on current trends in clinical trials and CIBMTR reports - that patients from minority racial groups and poor socio-economic background face difficulties in being referred for consultation for transplant in the management of their MDS or MPN. This in turn translates into worse outcomes for these patient populations.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary: Compare time from diagnosis to transplant for MDS/MPN patients from minority racial groups and/or low SES background as compared to overall rates in the CIBMTR database Secondary: 1- Compare overall survival, relapse free survival, Graft-versus-host disease (GVHD)-free relapse-free survival (GRFS) between MDS/MPN patients from minority racial groups and/or low SES background as compared to overall rates in the CIBMTR database 2- Compare rates and grade of acute and chronic GVHD for MDS/MPN patients from minority racial groups and/or low SES background as compared to overall rates in the CIBMTR database

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.	In the past decade a number of pivotal studies - including many from the CIBMTR have highlighted the impact racial and SES background have had on outcomes for aggressive malignancies; enrollment on clinical trials and overall long-term outcomes. However, an area that has not been addressed clearly is the approach and outcomes for patients who are diagnosed with potentially less aggressive disorders, such as MPNs and MDS. In many cases these patients are originally diagnosed and managed in community practices and eventually are referred to larger, often academic, centers for an evaluation for an allogeneic stem cell transplant for their disease. However, a certain portion may never be referred or be able to be seen at a transplant center - stemming to a number factor ranging from financial burden to travel logistics. It is postulated that minority and low SES groups would be disproportionately impacted based on referral patterns from community practices in their communities, culture beliefs, language barriers, logistics of travel and finances. Being able to clearly show the objective impact of such inherent biases on disease outcomes within the transplant patient population would have a significant impact in health policy approaches and would underscore the pressing need to reach out to such communities. The ASTCT-NMDP ACCESS Initiative has been an important first step - but further efforts are required to successfully make transplant a viable option for MDS/MPN patients from minority racial groups and/or low SES backgrounds.

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.	Access barriers to stem cell transplant and overall outcomes are closely linked. Barriers that have been previously identified have included issues related to donor, patient, physician, and program/institutional levels (Hematology Am Soc Hematol Educ Program. 2021; 2021:275-280). A previous CIBMTR study focused specifically on pediatric alloSCT patients and the impact of neighborhood poverty on outcomes. This analysis highlighted that for children undergoing transplant for malignant disease, neighborhood poverty conferred an increased risk of TRM specifically that for kids with Medicaid insurance had inferior OS and increased TRM compared with those with private insurance. The authors concluded that targetted interventions are required to overcome these differences in outcomes (Blood (2021) 137 (4): 556 568). More recently [1]. Impeding access delays and even precludes eligible patients from receiving potentially curative therapies and achieving best outcomes. Similarly, patient sociodemographic factors may be associated with therapy-related care differences, resulting in outcome disparities.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	1- Any patient with a diagnosis of MDS or MPN who has undergone an allogeneic stem cell transplant with available ZIP code and day-100 post-HCT data forms 2- Specifically focus on patients from a minority ethnic group (AA/Hispanic/Asian Islander) 3- Specifically focus on patients from low SES communities as defined
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	MDS/MPN diagnosis are rare in the general pediatric population. Furthermore - within a pediatric population there are various confounding factors related to family structure and parental discretion on seeking out treatment that would impact outcome results of the intended analysis in this proposal

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/donor gender Donor/Recipient CMV status: -/+ vs +/- vs +/+ vs -/- Obesity (BMI > 35 kg/m2) at transplant Conditioning Intensity according to CIBTMR definitions Disease: MDS vs MPN IPSS-M, DIPPS Score at time of diagnosis Disease risk index (DRI) Karnofsky score 90 vs < 90 vs unknown or missing Soror Co-morbidity index: 0 vs 1-2 vs 3 Race: White vs African American vs Hispanics vs others Time from diagnosis to HCT Donor Type: MRD vs MUD Graft Type: BM vs PBSC GVHD Prophylaxis: PT-Cy + CNI vs PT-Cy alone vs PT-Cy with ATG Time to ANC recovery Time to ALC recovery Rates of Early vs Late Infection Rates of Viral infections Rates of Fungal Infections Rates of Bacterial Infections Hospitalization for post-transplant infectious complications Acute GVHD: no vs Grade I-II vs Grade III-IV vs unknown or missing (Grade) Chronic GHVD: no vs yes vs unknown or missing Maximum grade of cGVHD: Limited vs Extensive vs unknown or missing Maximum overall severity of cGVHD: mild/moderate/severe/unknown or missing Follow-up of survivors, months, median (range)
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	no
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	no
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	none
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.	Zipcode date for patients will be linked to US Census data to help identify patients who fit into the target analysis population (living in a low SES neighborhood)

REFERENCES:	1. Bona K, Brazauskas R, He N, Lehmann L, Abdel-Azim H, Ahmed IA, Al-Homsi AS, Aljurf M, Arnold SD, Badawy SM, Battiwalla M, Beattie S, Bhatt NS, Dalal J, Dandoy CE, Diaz MA, Frangoul HA, Freytes CO, Ganguly S, George B, Gomez-Almaguer D, Hahn T, Kamble RT, Knight JM, LeMaistre CF, Law J, Lazarus HM, Majhail NS, Olsson RF, Preussler J, Savani BN, Schears R, Seo S, Sharma A, Srivastava A, Steinberg A, Szwajcer D, Wirk B, Yoshimi A, Khera N, Wood WA, Hashmi S, Duncan CN, Saber W. Neighborhood poverty and pediatric allogeneic hematopoietic cell transplantation outcomes: a CIBMTR analysis. Blood. 2021 Jan 28;137(4):556-568. doi: 10.1182/blood.2020006252. Erratum in: Blood. 2021 Dec 2;138(22):2300. doi: 10.1182/blood.2021012809. PMID: 33104215; PMCID: PMC7845011. 2. Auletta JJ, Sandmaier BM, Jensen E, Majhail NS, Knutson J, Nemecek E, Ajayi-Hackworth F, Davies SM; ACCESS Workshop Team. The ASTCT-NMDP ACCESS Initiative: A Collaboration to Address and Sustain Equal Outcomes for All across the Hematopoietic Cell Transplantation and Cellular Therapy Ecosystem. Transplant Cell Ther. 2022 Dec;28(12):802-809. doi: 10.1016/j.jtct.2022.09.020. Epub 2022 Sep 30. PMID: 36184058. 3.Wall SA, Olin R, Bhatt V, Chhabra S, Munshi P, Hacker E, Hashmi S, Hassel H, Howard D, Jayani R, Lin R, McCurdy S, Mishra A, Murthy H, Popat U, Wood W, Rosko AE, Artz A. The Transplantation Ecosystem: A New Concept to Improve Access and Outcomes for Older Allogeneic Hematopoietic Cell Transplantation Patients. Transplant Cell Ther. 2023 Oct;29(10):632.e1-632.e10. doi: 10.1016/j.jtct.2023.04.020. Epub 2023 May 1. PMID: 37137442. 4. Yusuf RA, Preussler JM, Meyer CL, Schoeppner K, Sees Coles JA, Ruffin A, McCann M, Devine SM, Auletta JJ. Reducing barriers of access and care related to hematopoietic cell transplantation and cellular therapy: The mission-driven role of the national marrow donor program. Best Pract Res Clin Haematol. 2023 Jun;36(2):101480. doi: 10.1016/j.beha.2023.101480. Epub 2023 May 25. PMID: 37353289. 5. Blue BJ, Brazauskas R, Chen K, Patel J, Zeidan AM, Steinberg A, Ballen K, Kwok J, Rotz SJ, 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,

Field	Response
	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. Transplant Cell Ther. 2023 Nov;29(11):709.e1-709.e11. doi: 10.1016/j.jtct.2023.07.013. Epub 2023 Jul 22. PMID: 37482244; PMCID: PMC11258715. 6. Blue BJ, Brazauskas R, Chen K, Patel J, Zeidan AM, Steinberg A, Ballen K, Kwok J, Rotz SJ, 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. Transplant Cell Ther. 2023 Nov;29(11):709.e1-709.e11. doi: 10.1016/j.jtct.2023.07.013. Epub 2023 Jul 22. PMID: 37482244; PMCID: PMC11258715. 7. Blue BJ, Brazauskas R, Chen K, Patel J, Zeidan AM, Steinberg A, Ballen K, Kwok J, Rotz SJ, 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. Transplant Cell Ther. 2023 Nov;29(11):709.e1-709.e11. doi: 10.1016/j.jtct.2023.07.013. Epub 2023 Jul 22. PMID: 37482244; PMCID: PMC11258715.
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 patients who received their first allogenic transplant for MDS or MPN with available ZIP and 100-days post forms at U.S. during 2010-2025

Characteristic	N (%)
No. of patients	16247
Patient-related Characteristics	
Age, by decades, no. (%)	
Median (range)	64 (1-83)
0-9	332 (2)
10-19	285 (2)
20-29	304 (2)
30-39	467 (3)
40-49	1101 (7)
50-59	3399 (21)
60-69	7315 (45)
70+	3044 (19)
Sex, no. (%)	
Male	10064 (62)
Female	6183 (38)
Karnofsky score prior to HCT, no. (%)	
90-100%	8158 (50)
< 90%	7835 (48)
Not reported	254 (2)
Race, no. (%)	
White	13966 (86)
Black or African American	895 (6)
Asian	578 (4)
Native Hawaiian or other Pacific Islander	47 (<1)
American Indian or Alaska Native	48 (<1)
More than one race	83 (1)
Not reported	630 (4)
HCT-CI, no. (%)	
0	3002 (18)
1	2189 (13)
2	2130 (13)
3	2935 (18)
4	2067 (13)
5+	3691 (23)
Not reported	233 (1)
Obesity at the time of transplant, no. (%)	

Characteristic	N (%)
No	14082 (87)
Yes	2087 (13)
Not reported	78 (<1)
Disease-related Characteristics	
Primary disease, no. (%)	
MDS	14098 (87)
MPN	2149 (13)
Disease status prior to HCT (MDS), no. (%)	
Complete remission (CR)	2101 (13)
Hematologic improvement (HI)	2310 (14)
No response / stable disease (NR/SD)	10681 (66)
Progression from hematologic improvement (Prog from HI)	547 (3)
Relapse from complete remission (Rel from CR)	52 (<1)
Not assessed	161 (1)
Partial clinical remission(PR)	22 (<1)
Clinical Improvement(CI)	40 (<1)
Progressive disease(PD)	24 (<1)
Not reported	309 (2)
MDS IPSS-R prognostic risk category / score at HCT, no. (%)	
Not MDS	2149 (13)
Very low	1428 (9)
Low	3106 (19)
Intermediate	3518 (22)
High	2260 (14)
Very high	1579 (10)
Not reported	2207 (14)
Transplant-related Characteristics	
Donor type, no. (%)	
HLA identical sibling	2980 (18)
Haploidentical donor	2419 (15)
Other related	198 (1)
Well-matched unrelated (8/8)	8444 (52)
Partially-matched unrelated (7/8)	1214 (7)
Mismatched unrelated (<= 6/8)	67 (<1)
Multi-donor	100 (1)
Unrelated (matching cannot be determined)	434 (3)
Cord blood	391 (2)
Donor/recipient sex match, no. (%)	

Characteristic	N (%)
M-M	6459 (40)
M-F	3405 (21)
F-M	3284 (20)
F-F	2545 (16)
CB - recipient M	243 (1)
CB - recipient F	186 (1)
Not reported	125 (1)
Donor/recipient CMV serostatus, no. (%)	
+/+	4697 (29)
+/-	1950 (12)
-/+	4297 (26)
-/-	4798 (30)
CB - recipient +	249 (2)
CB - recipient -	177 (1)
CB - recipient CMV unknown	3 (<1)
Not reported	76 (<1)
Product type, no. (%)	
BM	1570 (10)
PBSC	14248 (88)
UCB	429 (3)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)	
MAC	5868 (36)
RIC	8144 (50)
NMA	1797 (11)
Not reported	438 (3)
Conditioning regimen, no. (%)	
TBI/Cy	136 (1)
TBI/Cy/Flu	2045 (13)
TBI/Cy/Flu/TT	72 (<1)
TBI/Cy/TT	3 (<1)
TBI/Mel	643 (4)
TBI/Flu	1104 (7)
TBI/other(s)	63 (<1)
Bu/Cy/Mel	88 (1)
Bu/Cy	1408 (9)
Bu/Mel	95 (1)
Flu/Bu/TT	602 (4)
Flu/Bu	5280 (32)

Characteristic	N (%)
Flu/Mel/TT	153 (1)
Flu/Mel	4054 (25)
Cy/Flu	104 (1)
Cy alone	6 (<1)
Mel alone	21 (<1)
Mel/other(s)	45 (<1)
Treosulfan	90 (1)
Carb/other(s)	2 (<1)
TLI	54 (<1)
Other(s)	154 (1)
None	1 (<1)
Missing	24 (<1)
GVHD prophylaxis, no. (%)	
Ex-vivo T-cell depletion	73 (<1)
CD34 selection	220 (1)
PtCy + other(s)	6103 (38)
PtCy alone	51 (<1)
TAC + MMF +- other(s) (except PtCy)	1689 (10)
TAC + MTX +- other(s) (except MMF, PtCy)	5900 (36)
TAC + other(s) (except MMF, MTX, PtCy)	831 (5)
TAC alone	295 (2)
CSA + MMF +- other(s) (except PtCy,TAC)	582 (4)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	251 (2)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	12 (<1)
CSA alone	21 (<1)
Other(s)	125 (1)
Missing	94 (1)
Time from diagnosis, no. (%)	
0-6 months	5360 (33)
6-12 months	5492 (34)
>= 12 months	5395 (33)
Year of current transplant, no. (%)	
2010	1 (<1)
2012	2 (<1)
2013	175 (1)
2014	1097 (7)
2015	1197 (7)
2016	1311 (8)

Characteristic	N (%)
2017	1433 (9)
2018	1558 (10)
2019	1690 (10)
2020	1301 (8)
2021	1420 (9)
2022	1505 (9)
2023	1538 (9)
2024	1745 (11)
2025	274 (2)
Median follow-up of survivors (range), months, median (range), months	53.8 (0.2-144.2)

Field	Response
Proposal Number	2509-32-HUANG
Proposal Title	Outcomes of Patients with CLL/SLL Who Receive Allogeneic Hematopoietic Stem Cell Transplant in the Modern Era of Therapies
Key Words	Chronic lymphocytic leukemia, allogeneic transplant, targeted agents, chemoimmunotherapy
Principal Investigator #1: - First and last name, degree(s)	Jennifer Huang, MD, PhD
Principal Investigator #1: - Email address	jhuang3@fredhutch.org
Principal Investigator #1: - Institution name	Fred Hutch Cancer Center
Principal Investigator #1: - Academic rank	Hematology and Oncology Fellow
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Adam Kittai, MD
Principal Investigator #2 (If applicable): - Email address:)	adam.kittai@mssm.edu
Principal Investigator #2 (If applicable): - Institution name:	Icahn School of Medicine at Mount Sinai
Principal Investigator #2 (If applicable): - Academic rank:	Associate Professor
Junior investigator status (defined as 㑊、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:	Jennifer Huang (jhuang3@fredhutch.org)
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	Adam Kittai - CT 20-03 - Middle author on 3 publications. Helped revise the proposal and edit the manuscripts. Worked with CIBMTR to revise CLL Data collection sheet.
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Lymphoma
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No

Field	Response
RESEARCH QUESTION:	What is the outcome of allogeneic hematopoietic cell transplant (allo-HCT) in patients with CLL/SLL in the era of targeted agents and how often is allo-HCT utilized?
RESEARCH HYPOTHESIS:	a. Allo-HCT will have similar efficacy in patients with relapsed/refractory CLL/SLL no matter if they have a history of receiving targeted agents, chemoimmunotherapy, or both chemoimmunotherapy and targeted agents b. Utilization of allo-HCT has decreased since the approval of chemotherapy-free first line treatment options with targeted agents in 2014
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Objective: - Progression-free survival of patients with CLL/SLL treated with alloHCT analyzed based on whether the patient received targeted agents, targeted agents and chemoimmunotherapy, and chemoimmunotherapy alone prior to alloHCT. - To compare the utilization rate of alloHCT in the pre- and post-target agent eras (2004 2014 vs. 2014 present) Secondary Objective: - To compare the below in patients with CLL/SLL treated with alloHCT analyzed based on whether the patient received targeted agents, targeted agents and chemoimmunotherapy, and chemoimmunotherapy alone prior to alloHCT. o Overall survival (OS) o Cumulative incidence of relapse o Non-relapse mortality o Causes of death o Time to next treatment o Incidence and severity of acute GVHD o Incidence of chronic GVHD o Variables prognostic of 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.	Prior studies evaluating the efficacy of alloHCT for CLL/SLL were conducted in patients who largely received prior chemoimmunotherapy. Standard of care therapy for CLL/SLL has switched to targeted agents, such as BTKi and BCL2i. As such, it is unclear how this change may affect the outcomes of patients receiving alloHCT, given differences in therapeutic mechanism of chemoimmunotherapy and targeted therapies which may affect CLL/SLL disease biology. Furthermore, given the improved efficacy of targeted agents and CLL/SLL being a disease of the elderly, use of alloHCT may be becoming less common. Therefore, this study will be informative in studying the utility of alloHCT in the modern era of therapy for CLL/SLL.

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.	Since ibrutinib was first approved by the FDA as first-line treatment for CLL/SLL in 2014, multiple other targeted agents such as the combination of venetoclax and obinutuzumab have been approved for use as first-line agents for the treatment of CLL/SLL and are also used as later line agents for patients who were treated with chemoimmunotherapy1 5. These targeted agents have excellent efficacy5 and, thus, the number of people who undergo allo-HCT for CLL/SLL has likely decreased over time. However, patients who have relapsed/refractory disease to these modern therapies have few options. The recent approval of pirtobrutinib and lisocabtagene maraleucel in the relapsed/refractory setting has only led to a median PFS of 11.9-19.6 months6,7. Arguably, allo-HCT remains the only curative option for patients with CLL/SLL. Therefore, we believe allo-HCT continues to be a valid treatment option for this group of patients, despite the toxicity of allo-HCT. Further study elucidating the efficacy of allo-HCT after receiving targeted therapy is needed. However, given the toxicity of allo-HCT, the relative safety of targeted agents, and the older population of CLL/SLL, the role of allo-HCT for the treatment of CLL/SLL is unclear. However, patients who are relapsed/refractory to our modern therapies, have few options. In relapsed/refractory patients, recent approval of pirtobrutinib and lisocabtagene maraleucel, only led to a median PFS of 11.9-19.6 months6,7. Therefore, we believe allo-HCT continues to be a valid treatment option for this group of patients, and further study elucidating the efficacy of allo-HCT after patients have received targeted therapy is needed. The CIBMTR database has detailed, high-quality clinical data regarding the real-world outcomes of patients with CLL/SLL who received an allo-HCT. Prior analysis of the data used a date cut off point of only 2-3 years after BTK inhibitors were approved for first-line use for CLL9,10, and since then, multiple other targeted therapies have been approved. The most recent study that evaluated the use of allo-HCT for CLL/SLL was in 2020. 8 It was a retrospective cohort study of 65 patients with CLL/SLL who received allo-HCT after having received at least 1 targeted agent, including ibrutinib (BTKi), venetoclax (BCL2i) or PI3K inhibitor. They found allo-HCT to be effective, after a median follow up of 27 months, the PFS was 63% and OS was 81% at 24 months. Patients had received a median of 3 prior lines of therapy, 71% of the cohort had received

Field	Response
	prior chemotherapy in addition to prior targeted agent. While this is a well-performed study, only 29% of patients (n=19) received novel agents alone. Furthermore, follow up is relatively short for this cohort. As we no longer consider chemoimmunotherapy as the standard of care for patients with CLL/SLL, an updated study evaluating the use of allo-HCT for a larger cohort of patients who only received targeted therapy and no chemoimmunotherapy is a worthy endeavor since this change in treatment history may influence outcomes. The results of this study will help guide the use of allo-HCT for patients with CLL/SLL who have been exposed to targeted therapies.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria - Patients with diagnosis of chronic lymphocytic leukemia or small lymphocytic lymphoma - Patients who have undergone an allogeneic hematopoietic stem cell transplant Exclusion criteria - Patients who have Richter transformation
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	CLL/SLL is not a pediatric disease.

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.	We will use the following patient characteristics for multivariate analysis: Patient-related variables: a. Age b. Sex c. Ethnicity d. ECOG at treatment e. KPS at treatment f. HCT-comorbidity score g. Comorbidity profile Disease-related variables: a. CLL/SLL stage at diagnosis and at alloHCT b. Beta-2 microglobulin elevated at diagnosis c. ALC count at diagnosis and alloHCT d. Presence of cytopenia at diagnosis and at alloHCT i. Anemia ii. Thrombocytopenia iii. Neutropenia e. LDH at diagnosis and at allHCT f. Extranodal disease at diagnosis g. Cytogenetics at diagnosis and at alloHCT i. Cytogenetic abnormalities ii. Del13q abnormality iii. Del11q abnormality iv. Del 17q abnormality v. Trisomy 12 abnormality vi. Presence of NOTCH1 or TP53 mutation vii. IGHV mutational status h. Time from dx to allogeneic HCT: continuous i. Number of prior lines of therapy j. Refractoriness i. Primary refractoriness to last previous therapy k. Prior therapies: i. Cohort 1 (targeted therapy alone): prior therapy only includes BTKi (i.e. ibrutinib, acalabrutinib, zanubrutinib, pirtobrutinib), BCL2i (i.e. Venetoclax), PI3Ki (i.e. idelalisib, duvelisib, copanlisib), or anti-CD20 antibodies (eg. rituximab, obinutuzumab) all as a single agent or in combination ii. Cohort 2 (chemotherapy alone): prior therapy includes bendamustine, chlorambucil, cladribine, cyclophosphamide, cytarabine, doxorubicin, etoposide, fludarabine, gemcitabine, ifosfamide, nelarabine, nitrogen mustard, pentostatin iii. Cohort 3 (both chemoimmunotherapy and targeted therapy): prior therapy includes agents in cohort 1 and cohort 2 l. Time between CLL/SLL diagnosis to alloHCT m. Time from first CLL/SLL treatment to alloHCT Transplant-related variables: a. Disease status at time of allo-HCT b. Allo-HCT year c. Donor type i. HLA type (A, B, C, DRB1) ii. Matched vs. mismatched iii. Unrelated, haploidentical, umbilical cord d. Conditioning intensity e. TBI yes/no f. Graft source g. GVHD prophylaxis h. ATG/alemtuzumab use We will use the CIBMTR database to identify patients with CLL/SLL and who received an allo-HCT. We will compare the outcomes of interest among

Field	Response
	patients who have a history of receiving targeted agent alone with those who have received chemoimmunotherapy alone or both chemoimmunotherapy and targeted agents. Multivariable Cox regression models will be used for time to event outcomes (PFS, OS, etc), and multi-variable competing risk analyses will be applied for GVHD and mortality outcomes.
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	None
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	None
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	None
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.	None

Field	Response
REFERENCES:	1. Fischer K, Al-Sawaf O, Bahlo J, et al. Venetoclax and Obinutuzumab in Patients with CLL and Coexisting Conditions. N Engl J Med. 2019;380(23):2225 2236. 2. Brown JR, Eichhorst B, Hillmen P, et al. Zanubrutinib or Ibrutinib in Relapsed or Refractory Chronic Lymphocytic Leukemia. N Engl J Med. 2023;388(4):319 332. 3. Burger JA, Tedeschi A, Barr PM, et al. Ibrutinib as Initial Therapy for Patients with Chronic Lymphocytic Leukemia. N Engl J Med. 2015;373(25):2425 2437. 4. Byrd JC, Harrington B, O'Brien S, et al. Acalabrutinib (ACP-196) in Relapsed Chronic Lymphocytic Leukemia. N Engl J Med. 2016;374(4):323 332. 5. Shadman M. Diagnosis and Treatment of Chronic Lymphocytic Leukemia: A Review. JAMA. 2023;329(11):918 932. 6. Mato AR, Woyach JA, Brown JR, et al. Pirtobrutinib after a Covalent BTK Inhibitor in Chronic Lymphocytic Leukemia. N Engl J Med. 2023;389(1):33 44. 7. Siddiqi T, Maloney DG, Kenderian SS, et al. Lisocabtagene maraleucel in chronic lymphocytic leukaemia and small lymphocytic lymphoma (TRANSCEND CLL 004): a multicentre, open-label, single-arm, phase 1 2 study. The Lancet. 2023;402(10402):641 654. 8. Roeker LE, Dreger P, Brown JR, et al. Allogeneic stem cell transplantation for chronic lymphocytic leukemia in the era of novel agents. Blood Advances. 2020;4(16):3977 3989. 9. Kim HT, Shaughnessy CJ, Rai SC, et al. Allogeneic hematopoietic cell transplantation after prior targeted therapy for high-risk chronic lymphocytic leukemia. Blood Advances. 2020;4(17):4113 4123. 10. Tournilhac O, Van Gelder M, Eikema D-J, et al. The European landscape on allogeneic haematopoeietic cell transplantation in Chronic Lymphocytic Leukaemia between 2009 and 2019: a perspective from the Chronic Malignancies Working Party of the EBMT. Bone Marrow Transplant. 2023;58(6):621 624.
CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	Yes, I have 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.	Huang: None Kittai: A.S.K has received research funding from AstraZeneca, and BeiGene, has performed speaking engagements for AstraZeneca, BeiGene, and Eli-Lilly, and has participated in advisory boards for Abbvie, AstraZeneca, BeiGene, BMS, and Galapagos.

Table 1. Characteristics of patients who received their first allogenic transplant for CLL or small lymphocytic lymphoma at U.S. during 2008-2022. (CRF only)

Characteristic	2008-2014	2015-2022
No. of patients	447	190
Patient-related Characteristics		
Age, by decades, no. (%)		
Median (range)	58 (29-74)	59 (21-75)
20-29	1 (0)	2 (1)
30-39	10 (2)	3 (2)
40-49	66 (15)	32 (17)
50-59	197 (44)	67 (35)
60-69	158 (35)	77 (41)
70+	15 (3)	9 (5)
Sex, no. (%)		
Male	338 (76)	146 (77)
Female	109 (24)	44 (23)
Race, no. (%)		
White	398 (89)	153 (81)
Black or African American	39 (9)	31 (16)
Asian	4 (1)	1 (1)
Native Hawaiian or other Pacific Islander	1 (0)	0 (0)
American Indian or Alaska Native	0 (0)	1 (1)
Not reported	5 (1)	4 (2)
ECOG prior to HCT, no. (%)		
Asymptomatic	297 (66)	109 (57)
Symptomatic but completely ambulatory	125 (28)	76 (40)
Symptomatic, < 50% in bed during the day	5 (1)	2 (1)
Not reported	20 (4)	3 (2)
Karnofsky score prior to HCT, no. (%)		
90-100%	297 (66)	109 (57)
< 90%	130 (29)	78 (41)
Not reported	20 (4)	3 (2)
HCT-CI, no. (%)		
0	161 (36)	44 (23)
1	63 (14)	36 (19)
2	59 (13)	29 (15)
3	67 (15)	34 (18)
4	49 (11)	22 (12)
5+	40 (9)	24 (13)

Characteristic	2008-2014	2015-2022
Not reported	8 (2)	1 (1)
Disease-related Characteristics		
Specify ALL classification, no. (%)		
CLL Chronic lymphocytic leukemia, NOS:	81 (18)	32 (17)
CLL B-cell/lym small lymphocytic:	366 (82)	158 (83)
CLL pre-HCT disease stage, no. (%)		
CR	52 (12)	36 (19)
PR	225 (50)	109 (57)
Advanced (PIF/Relapse)	169 (38)	44 (23)
Not reported	1 (0)	1 (1)
Prior therapies, no. (%)		
Targeted therapy alone (BTKi, BCL2i, PI3Ki or anti-CD20)	21 (5)	33 (17)
Chemotherapy alone (bendamustine, chlorambucil, cyclophosphamide, cytarabine, doxorubicin, etoposide, fludarabine, gemcitabine, ifosfamide, pentostatin	9 (2)	4 (2)
Both types of therapy	395 (88)	137 (72)
Not reported	22 (5)	16 (8)
Treatment-related Characteristics		
Donor type, no. (%)		
HLA identical sibling	115 (26)	39 (21)
Haploidentical donor	20 (4)	43 (23)
Other related	11 (2)	0 (0)
Well-matched unrelated (8/8)	198 (44)	84 (44)
Partially-matched unrelated (7/8)	31 (7)	12 (6)
Multi-donor	5 (1)	0 (0)
Unrelated (matching cannot be determined)	17 (4)	5 (3)
Cord blood	50 (11)	7 (4)
Donor/recipient sex match, no. (%)		
M-M	192 (43)	98 (52)
M-F	54 (12)	25 (13)
F-M	105 (23)	42 (22)
F-F	43 (10)	18 (9)
CB - recipient M	41 (9)	6 (3)
CB - recipient F	12 (3)	1 (1)
Donor age , by decades, unrelated donor only, no. (%)		
10-19	4 (2)	2 (2)
20-29	121 (49)	50 (50)
30-39	61 (25)	29 (29)
40-49	38 (15)	17 (17)

Characteristic	2008-2014	2015-2022
50-59	14 (6)	3 (3)
Not reported	8 (3)	0 (0)
Product type, no. (%)		
BM	32 (7)	14 (7)
PBSC	362 (81)	169 (89)
UCB	53 (12)	7 (4)
GVHD prophylaxis, no. (%)		
Ex-vivo T-cell depletion	6 (1)	1 (1)
CD34 selection	4 (1)	1 (1)
PtCy + other(s)	15 (3)	48 (25)
TAC + MMF +- other(s) (except PtCy)	104 (23)	32 (17)
TAC + MTX +- other(s) (except MMF, PtCy)	163 (36)	73 (38)
TAC + other(s) (except MMF, MTX, PtCy)	34 (8)	9 (5)
TAC alone	12 (3)	3 (2)
CSA + MMF +- other(s) (except PtCy,TAC)	81 (18)	15 (8)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	4 (1)	4 (2)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	5 (1)	0 (0)
CSA alone	9 (2)	1 (1)
Other(s)	9 (2)	2 (1)
Missing	1 (0)	1 (1)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)		
MAC	69 (15)	22 (12)
RIC	188 (42)	86 (45)
NMA	157 (35)	69 (36)
Not reported	33 (7)	13 (7)
Year of transplant, no. (%)		
2008	127 (28)	0 (0)
2009	83 (19)	0 (0)
2010	18 (4)	0 (0)
2011	27 (6)	0 (0)
2012	24 (5)	0 (0)
2013	102 (23)	0 (0)
2014	66 (15)	0 (0)
2015	0 (0)	47 (25)
2016	0 (0)	41 (22)
2017	0 (0)	50 (26)
2018	0 (0)	30 (16)
2019	0 (0)	10 (5)

Characteristic	2008-2014	2015-2022
2020	0 (0)	6 (3)
2021	0 (0)	3 (2)
2022	0 (0)	3 (2)
Median follow-up of survivors (range), months, median (range), months	128.1 (12.2-197.2)	73.8 (24.2-121.9)

Field	Response
Proposal Number	2509-86-TRACY
Proposal Title	Novel Composite endpoints for outcomes of patients with acute lymphoblastic lymphoma treated with CART therapy
Key Words	CART, obecabtagene, brexucabtagene
Principal Investigator #1: - First and last name, degree(s)	Sean Tracy, MD, PhD
Principal Investigator #1: - Email address	stracy@umn.edu
Principal Investigator #1: - Institution name	University of Minnesota
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 㑊、5 years from fellowship)	No
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Veronika Bachanova
Principal Investigator #2 (If applicable): - Email address:)	bach0173@umn.edu
Principal Investigator #2 (If applicable): - Institution name:	University of Minnesota
Principal Investigator #2 (If applicable): - Academic rank:	Professor
Junior investigator status (defined as 㑊、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:	Sean Tracy
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	VB is chair of leukemia committee
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	Yes

Field	Response
RESEARCH QUESTION:	CAR T therapy can induce prolonged remissions, but is also frequently associated with complications including Cytokine Release Syndrome (CRS) and Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS). An optimal metric for comparing CAR T cell therapies would simultaneously summarize both meaningful disease responses as well as significant toxicity. We propose early composite endpoints which capture both early high-grade toxicity and remission rates. These endpoints may serve as novel outcome measures for comparison of CAR T cell products in clinical trials and real-world studies.
RESEARCH HYPOTHESIS:	1. Novel composite endpoints will determine and compare the net clinical benefit of tisa-cel, brexu-cel and obi-cel therapies. 2. Novel composite endpoints will more accurately identify subsets of patients with favorable and unfavorable long-term outcomes after CAR-T therapy. 3. Novel composite endpoints will describe a benchmark for commercial products which can be applied in clinical testing of novel interventions to lower toxicity without impacting efficacy. We define the novel composite endpoints: toxicity-free complete response/complete response with incomplete hematologic recovery at day 28 (tfCR/i/28), and toxicity-free, progression free survival at day 28 (tfPFS28). Toxicity will be characterized as having experienced grade 3 CRS or grade 3 ICANs. tfCR/i/28 will be defined as the proportion of patients experiencing a complete response (CR) or complete response with incomplete hematologic recovery (Cri) at day 28 post-infusion and without toxicity. tfPFS28 will be defined as the proportion of patients alive, free of leukemia progression at day 28 post-infusion, and without toxicity. We will compare long-term outcomes (PFS, OS) between patients who experience tfCR/i/28 versus CRi28wt (patients in Cr or Cri who experienced gr 3 CRS or gr 3 ICANs) and the group of patients without CR/CRi at day 28 after infusion. We will separately determine PFS and OS for brexucel and obecel.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Objectives: Determine tfCR/i/28 and tfPFS28 in patients with B-ALL treated with commercial CAR T products Tisa-cel, Brexu-cel, and Obe-cel. Evaluate 3-year PFS and OS in tfCR/i/28 patients compared to CR/i/28wt and no CR/Cri separately by each commercial product. Secondary Objectives: 1. Evaluate cumulative incidence of relapse and NRM at 3 years in tfCR/i/28 and CR/i/28wt groups. 2. Evaluate all composite endpoints and objectives with and without censoring for allogeneic HSCT or next line of therapy.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Early post-infusion timepoints have emerged as strong predictors of subsequent long-term outcomes for patients with B-ALL treated with CAR T therapy. Patients who do not experience CR/CRi by day 28 have a high likelihood of subsequent clinical relapse, regardless of CAR T product used1,2. Even among patients in CR/CRi, MRD positivity at any level, at day 28, is a strong predictor of subsequent relapse. Similarly in B-ALL, all high-grade CRS or ICANs has an onset prior to day 28. When it occurs, each of these toxicities frequently causes prolonged hospital or ICU stays, accompanied by considerable cost and morbidity. Therefore, freedom from high-grade CRS or ICANs, and achievement of CR/CRi, by day 28 following commercial CAR T product infusion, are highly meaningful early measures of a CAR T therapy's overall benefit. The novel composite endpoints tfCR/i/28 and tfPFS28 capture the proportion of patients experiencing this optimal early endpoint. Composite early endpoints can also be used as benchmarks for cost effectiveness studies, guiding clinical trials, cross-comparing available CAR T products, and for evaluating novel clinical interventions that prevent or treat toxicity without impacting efficacy.

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.	We have conducted several studies with the Real-World Collaborative of CAR-T in Adult ALL (ROCCA) consortium. Our initial manuscript, focused on Brexu-cel, demonstrated similar frequencies of high-grade CRS and ICANs, as well as CR/CRi rates as the pivotal Zuma-3 trial1,3. Obe-cel was approved in 2024 for adults with R/R B-ALL primarily on the basis of the FELIX trial2, which suggested favorable rates of high-grade CRS and ICANs. Here, we propose a previously untested uniquely novel way to combine the events of toxicity and efficacy after CAR-T to understand the joined impact of clinically significant events and their treatment on survival. Given this analysis requires solely basic events already collected, it is ideally suited for this international registry and will likely reveal novel insight.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	18+, with a diagnosis of B-ALL, who underwent collection for commercial CAR T manufacture.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	The studied products are approved only for 18+ populations.
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 variables: Age, sex. Disease variables: Ph+ vs. Ph- disease, prior allogeneic HSCT, prior # of lines of therapy, prior Blinatumomab exposure, prior Inotuzumab exposure, BM Blast % at time of apheresis, CNS positivity Infusion variables: Toxicity prophylaxis, total serum ferritin, C-reactive protein, CAR T total cell dose.
Types of cellular therapy data this proposal includes:	Chimeric Antigen Receptor (CAR) T-Cell Therapy (CAR-T)
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

Field	Response
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
REFERENCES:	1 Roloff, G. W. et al. Outcomes After Brexucabtagene Autoleucel Administered as a Standard Therapy for Adults With Relapsed/Refractory B-Cell ALL. J Clin Oncol, JCO2400321 (2024). https://doi.org/10.1200/jco.24.00321 2 Roddie, C. et al. Obecabtagene Autoleucel in Adults with B-Cell Acute Lymphoblastic Leukemia. N Engl J Med 391, 2219 2230 (2024). https://doi.org/10.1056/NEJMoa2406526 3 Shah, B. D. et al. KTE-X19 for relapsed or refractory adult B-cell acute lymphoblastic leukaemia: phase 2 results of the single-arm, open-label, multicentre ZUMA-3 study. Lancet 398, 491 502 (2021). https://doi.org/10.1016/s0140-6736(21)01222-8
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

Table 1. Characteristics of consented adult patients who received their first CAR-T during 2008 to 2022 in U.S. for B-ALL

Characteristic	No (%)
No. of patients	432
Patient-related Characteristics	
Age, by decades, no. (%)	
Median (range)	24 (18-79)
10-19	79 (18)
20-29	214 (50)
30-39	43 (10)
40-49	23 (5)
50-59	38 (9)
60-69	28 (6)
70+	7 (2)
Recipient Sex, no. (%)	
Male	248 (57)
Female	184 (43)
Karnofsky performance score prior to CT, no. (%)	
90-100	207 (48)
80	113 (26)
< 80	82 (19)
Not reported	30 (7)
HCT comorbidity score, no. (%)	
0	108 (25)
1	94 (22)
2	73 (17)
3	67 (16)
4	43 (10)
5+	46 (11)
Not reported	1 (0)
Disease-related Characteristics	
Specify ALL classification, no. (%)	
t(5;14) (q31;q32); IL3-IGH:	1 (0)
B-lymphoblastic leukemia / lymphoma with Hyperdiploidy (51-65 chromosomes)	20 (5)
B-lymphoblastic leukemia / lymphoma with Hypodiploidy (<46 chromosomes)	5 (1)
B-lymphoblastic leukemia / lymphoma, BCR-ABL1-like	50 (12)
B-lymphoblastic leukemia / lymphoma, with iAMP21	7 (2)
precursor B-cell ALL:	260 (60)
t(9;22)(q34;q11); BCR/ABL+:	60 (14)

Characteristic	No (%)
t(v;11q23); MLL rearranged:	17 (4)
t(1;19)(q23;p13) E2A/PBX1:	5 (1)
t(12;21)(p12;q22) ETV/CBFa:	7 (2)
Disease status prior to CT for leukemia, no. (%)	
CR1	43 (10)
CR2	65 (15)
CR3+	52 (12)
Relapse, 1st	104 (24)
Relapse, other	116 (27)
PIF/Untreated	52 (12)
No. of lines of prior therapies (including CT and HCT), no. (%)	
1-3	152 (35)
4-6	184 (43)
7-9	56 (13)
10+	16 (4)
No lines reported/not reported	24 (6)
Prior Blinatumomab, no. (%)	
No	231 (53)
Yes	183 (42)
Not reported	18 (4)
Prior Inotuzumab, no. (%)	
No	286 (66)
Yes	128 (30)
Not reported	18 (4)
Prior HCT, no. (%)	
No	295 (68)
Yes	133 (31)
Not reported	4 (1)
Treatment-related Characteristics	
Product, no. (%)	
Kymriah	260 (60)
Tecartus	172 (40)
Lymphodepleting regimen, no. (%)	
Fludarabine + Cyclophosphamide	417 (97)
Bendamustine only	3 (1)
Others	12 (3)
Year of infusion, no. (%)	
2017	5 (1)

Characteristic	No (%)
2018	31 (7)
2019	64 (15)
2020	58 (13)
2021	56 (13)
2022	218 (50)
CRS grade (ASTCT consensus) (at 100-day reporting), no. (%)	
No CRS	127 (29)
Grade 1	141 (33)
Grade 2	96 (22)
Grade 3	39 (9)
Grade 4	24 (6)
Grade 5	1 (0)
TBD	4 (1)
Neurotoxicity grade (at 100-day reporting), no. (%)	
No neurologic impairment	281 (65)
Grade 1	29 (7)
Grade 2	22 (5)
Grade 3	47 (11)
Grade 4	19 (4)
Grade 5	2 (0)
TBD	32 (7)
Median follow-up of survivors (range), months, median (range), months	34.8 (2.2-85.0)

Study Title: Outcomes of allogeneic hematopoietic cell transplantation in VEXAS syndrome: A combined EBMT and CIBMTR study

Keywords: VEXAS syndrome, myelodysplastic syndrome, autoinflammatory disorders, non-malignant transplantation

Principal Investigators:
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Academic rank: Group leader: Autoimmunology

Junior Investigator Status: RJS [Yes]

Underrepresented minority: No

Would you like assistance in identifying a senior mentor? No

Current ongoing work with CIBMTR: Multiple CIBMTR projects as co-PI

PI(s) with a CIBMTR WC study in manuscript preparation >6 months? No

Proposed working committee: Non-malignant

Please indicate if you have spoken with a scientific director or chair: No

Research Questions:

Patients with VEXAS undergoing allogeneic hematopoietic cell transplantation (allo-HCT), with or without concomitant MDS, have distinct risk factors for treatment-related toxicity and non-relapse mortality (NRM). The optimal transplant strategy and patient selection criteria remain uknown. This study will identify predictors of efficacy and toxicity by comparing patients with VEXAS to a propensity-score matched (PSM) cohort of MDS patients without VEXAS.

Research Hypothesis:

We hypothesize that patients with VEXAS will have a higher risk of non-relapse mortality (NRM) as compared to matched patients with MDS undergoing allo-HCT and will have unique risk factors that predict for transplant-related toxicity.

Specific Objectives/Outcomes to be Investigated:

Primary outcome:

- Rate and predictors of NRM for patients with VEXAS undergoing allo-HCT as compared to a PSM matched control cohort of MDS patients.

Secondary outcomes:

- Cumulative incidence and predictors of infection (bacterial, fungal, or viral) related complications in VEXAS versus matched MDS patients undergoing allo-HCT.
- Cumulative incidence and predictors of severe acute or chronic graft-versus-host disease (GVHD) in VEXAS versus matched MDS patients undergoing allo-HCT.
- Cumulative incidence and predictors of relapse in VEXAS versus matched MDS patients undergoing allo-HCT.
- All cause mortality for VEXAS versus matched MDS patients undergoing allo-HCT.
- GVHD-free relapse-free survival (GRFS), relapse-free survival (RFS), and overall survival (OS) for VEXAS versus matched MDS patients undergoing allo-HCT.

Covariates to be included: Age, WHO subtype, IPSS-R, GVHD prophylaxis, conditioning intensity, donor type, and year of transplant, disease type inflammatory vs. MDS vs. both.

Scientific Impact:

This study will provide the most comprehensive assessment to date of allo-HCT outcomes in VEXAS. By combining EBMT and CIBMTR registry data, we will generate a sufficiently large cohort to evaluate predictors of NRM and other outcomes. The findings will guide patient selection, inform transplant strategies, and is likely to be practice-changing by defining the role of allo-HCT in VEXAS.

Scientific Justification:

Vacuoles, E1 enzyme, X-linked, Autoinflammatory, Somatic (VEXAS) is a clonal myeloid disorder caused by somatic mutations in UBA1 in myeloid and erythroid progenitors. (1) The dominant clinical manifestations of VEXAS are autoinflammatory in nature and can produce a diverse set of symptoms such as polychondritis, neutrophilic dermatosis or vasculitis, inflammatory arthritis, pneumonitis, or recurrent fevers amongst

others. (2) Hematologic manifestations can include plasma cell disorders, myelodysplastic syndrome (MDS), and bone marrow failure, often with clonal evolution. (2) While transformation to acute myeloid leukemia is rare, some patients with VEXAS will develop advanced marrow failure and become transfusion dependent, which is associated with a worse prognosis. (3) The long-term prognosis of patients with VEXAS is generally thought to be poor due to the cumulative effects of recurrent autoinflammation and steroid exposure, marrow failure, and infections, with larger cohorts reporting a 5-year survival of 63%. (4)

The optimal treatment approach in VEXAS is yet to be fully defined. Although steroids are effective at controlling autoinflammatory symptoms, they result in substantial long-term morbidity. Currently accepted steroid sparing agents include ruxolitinib (5, 6), tocilizumab (6), and azacitidine for those with MDS. (7) While effective, none of these treatments are ultimately curative and, except for azacitidine, do not improve marrow function. Given this, there is interest in the application of allogeneic hematopoietic cell transplantation (allo-HCT) to patients with VEXAS. (8-11) Outcomes of allo-HCT in patients with VEXAS have been reported in the literature but, given the relative rarity of the disease, is largely limited to small cohorts or case studies. One of the largest series to date examined 19 patients with VEXAS who underwent allo-HCT. The majority of these patients had an established diagnosis of intermediate- or lower-risk MDS and received transplant with a variety of donor sources and conditioning regimens. They observed allo-HCT was consistently able to resolve the autoinflammatory phenotype, although the rate of non-relapse mortality (NRM) was high at 25.8%. (10) The high rate of NRM has also been observed in other series. (8)

Despite this data, there does not yet exist a definitive study that establishes the outcomes and predictive factors for allo-HCT in VEXAS. With the early signals of high NRM in this group, data to guide the patient selection and allo-HCT platform is particularly crucial for patients with VEXAS. Given the relative rarity of VEXAS and the small overall numbers of patients at any one institution, we propose a combined EBMT and CIBMTR registry study to definitely assess the outcomes of allo-HCT in VEXAS. Specifically, given their biological and clinical overlap, we propose to perform an analysis of patients with VEXAS versus a propensity score matched control cohort of patients with MDS undergoing allo-HCT.

Participant Selection Criteria:

Inclusion criteria:

- Adult patients (≥18 years old)
- A reported diagnosis of VEXAS, with or without concomitant MDS
- Presence of a pathogenic variant in UBA1.
- Receipt of first allo-HCT including any type of conditioning, donor, and GVHD prophylaxis
- Receipt of first allo-HCT between 2018 through 2025

Exclusion criteria:

- Lack of available data on conditioning regimen, GVHD prophylaxis, or follow-up (lost to follow-up with unknown status before day 100)
- Unclear reported diagnosis of VEXAS syndrome

Patients included in the control cohort will be MDS patients that are propensity score matched for: Recipient age at transplantation, Karnofsky performance status (KPS), Hematopoietic cell transplantation comorbidity index (HSCT-CI), Sex, and Disease risk index (DRI) for patients with VEXAS and MDS.

Does this study include pediatric patients? No

Data Requirements:
- Recipient baseline data: Age, gender, ethnicity, conditioning regimen, use of in-vivo T-cell depletion
- Hematopoietic cellular transplant infusion: Product type, CD34 cell count, produce processing/manipulation, date of product infusion
- MDS pre-infusion data: Disease assessment at diagnosis, diagnostic studies including molecular markers performed, IPSS-R prognosis score, cytogenetics, receipt of therapy prior to allo-HSCT (Y/N) and response
- Inflammatory conditions pre-infusion data: Affected organs skin, lung, vascular, cartilage, other.
- Post HSCT status: Hematopoietic recovery, chimerism, acute GVHD (onset, severity), chronic GVHD (onset, severity), subsequent cellular infusions (donor lymphocyte infusion or second transplant)
- MDS post infusion: Best response to allo-HSCT, post-transplant therapy, current disease status (relapse)
- Disease Classification (2402): VEXAS diagnosis, diagnosis date
- Pre-transplant essential data: Recipient information (age, sex), receipt of prior allo-HSCT, donor information, product type, related donor type, unrelated donor type, degree of match, donor age and sex, donor cytomegalovirus antibodies, clinical status of recipient prior to conditioning (functional status, recipient cytomegalovirus antibodies), pre-HSCT preparative regimen – intensity, use of radiation, drugs used, use of T-cell depleting agents or alemtuzumab, GVHD prophylaxis regimen
- Pre-TED disease classification: Primary diagnosis, AML classification, transformation from prior MDS/MPN, therapy related, predisposing conditions, cytogenetics, molecular features, status at transplantation including minimal residual disease, MDS subtype, cytogenetics, transformation to AML, status at transplantation
- Post-transplant essential data: Alive/dead, subsequent allo-HSCT, donor lymphocyte infusion, hematopoietic recovery, acute GVHD onset and severity, veno-occlusive disease incidence, chimerism, disease response, relapse or progression post infusion, incidence and characterization of infections, persistent or new inflammatory conditions.
- Recipient death data: Date of death, primary cause of death, contributing cause of death

- Subsequent neoplasms: Hematologic malignancy, solid tumors, date of diagnosis, donor derived

Patient reported outcome requirements:
Not required

Machine learning:
Not applicable

Sample requirements:
Not applicable

Non-CIBMTR Data Source:
Not applicable

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Conflicts of Interest:
No COI relevant to this study

Characteristics of US Adult VEXAS Patients with First Allo-HCT during 2018-2025

Characteristic	Total
Number of patients	27
No. of centers	19
TED or RES (RF) track determined for this event, no. (%)	
TED	19 (70)
CRF (RES)	8 (30)
Patient-related	
Age, by decades, no. (%)	
Median (range)	66 (36-78)
30-39	1 (4)
40-49	1 (4)
50-59	4 (15)
60-69	15 (56)
70+	6 (22)
Sex, no. (%)	
Male	22 (81)
Female	5 (19)
Race, no. (%)	
White	23 (85)
Black or African American	1 (4)
Asian	1 (4)
More than one race	1 (4)
Not reported	1 (4)
Ethnicity, no. (%)	
Hispanic or Latino	4 (15)
Non-Hispanic or Latino	23 (85)
Karnofsky score prior to HCT, no. (%)	
90-100%	9 (33)
< 90%	18 (67)
HCT-CI, no. (%)	
0	5 (19)
1	5 (19)
2	2 (7)
3	5 (19)
4	2 (7)
5+	8 (30)
Disease-related	

Characteristic	Total
Primary disease, no. (%)	
AML	10 (37)
MDS	13 (48)
MPN	4 (15)
MDS IPSS-R prognostic risk category / score at HCT, no. (%)	
Not MDS	14 (52)
Very low	5 (19)
Low	4 (15)
Intermediate	3 (11)
Very high	1 (4)
MDS pre-HCT disease stage, no. (%)	
Disease is not MDS/MPN	10 (37)
Early	1 (4)
Advanced	16 (59)
ELN 2022 (AML), no. (%)	
Not AML	17 (63)
Normal	2 (7)
Favorable	1 (4)
Intermediate	1 (4)
Poor	6 (22)
AML pre-HCT disease stage, no. (%)	
Disease is not AML	17 (63)
CR1	7 (26)
CR2	1 (4)
Advanced or active disease	2 (7)
Transplant-related	
Interval from diagnosis to HCT, months	
Mean (SD)	20.0 (27.52)
Median (25-75 percentile)	6.7 (4.5-22.8)
Range	2.3-111.8
Donor type, no. (%)	
HLA identical sibling	3 (11)
Haploidentical donor	3 (11)
Other related	1 (4)
Well-matched unrelated (8/8)	17 (63)
Partially-matched unrelated (7/8)	3 (11)
Donor/recipient sex match, no. (%)	
M-M	12 (44)

Characteristic	Total
M-F	2 (7)
F-M	10 (37)
F-F	3 (11)
Donor age, by decades, no. (%)	
10-19	1 (4)
20-29	14 (52)
30-39	8 (30)
40-49	2 (7)
50-59	1 (4)
60-69	1 (4)
Product type, no. (%)	
PBSC	27 (100)
Serotherapy-ATG/Campath, no. (%)	
ATG alone	5 (19)
CAMPATH alone	1 (4)
No ATG or CAMPATH	21 (78)
Conditioning regimen intensity, no. (%)	
MAC	2 (7)
RIC	23 (85)
NMA	1 (4)
Under review	1 (4)
Conditioning regimen, no. (%)	
TBI/Cy/Flu	1 (4)
TBI/Mel	3 (11)
TBI/Flu	2 (7)
Flu/Bu/TT	2 (7)
Flu/Bu	9 (33)
Flu/Mel	9 (33)
Other(s)	1 (4)
GVHD prophylaxis, no. (%)	
PtCy + other(s)	16 (59)
TAC + MMF +- other(s) (except PtCy)	4 (15)
TAC + MTX +- other(s) (except MMF, PtCy)	6 (22)
TAC alone	1 (4)
Year of current transplant, no. (%)	
2021	2 (7)
2022	2 (7)
2023	11 (41)

Characteristic	Total
2024	10 (37)
2025	2 (7)
Follow-up of survivors, median (range), months	17.3 (3.3-47.2)

Field	Response
Proposal Number	2509-124-GRAHAM
Proposal Title	Fludarabine exposure and outcome following allogeneic hematopoietic stem cell transplantation for AML and MDS
Key Words	Fludarabine, pharmacokinetic model, allogeneic transplant, AML, MDS
Principal Investigator #1: - First and last name, degree(s)	Chris Graham
Principal Investigator #1: - Email address	grah0329@umn.edu
Principal Investigator #1: - Institution name	University of Minnesota
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 欧、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Mark Juckett
Principal Investigator #2 (If applicable): - Email address:)	juck0001@umn.edu
Principal Investigator #2 (If applicable): - Institution name:	University of Minnesota
Principal Investigator #2 (If applicable): - Academic rank:	Professor
Junior investigator status (defined as 欧、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:	Chris Graham
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	No active projects
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	Yes
If you have already spoken with a scientific director or working committee chair regarding this study, then please specify who:	Mark Juckett

Field	Response
RESEARCH QUESTION:	Does fludarabine exposure impact outcomes following allogeneic PBSCT?
RESEARCH HYPOTHESIS:	The exposure to fludarabine, as estimated by a population-based pharmacokinetic model, influences overall survival at one year following myeloablative, reduced-intensity, or non-myeloablative conditioning regimens using PTCy-based GVHD prophylaxis.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Objective: Determine whether the predicted fludarabine exposure as measured by the area under the curve (AUC) calculated according to a population-based pharmacokinetic model, influences overall survival at 1-year following allogeneic hematopoietic stem cell transplantation. Secondary Objective: Determine the fludarabine AUC range that is associated with the best overall survival at 1-yr following a myeloablative, reduced-intensity, and non-myeloablative HSCT, respectively. Determine whether the predicted fludarabine AUC impacts: o Incidence of grade III/IV graft-versus-host disease, o Relapse at 1 year, o Disease-free survival at 1 year o Non-relapse survival at 100 days and 1 year o Graft failure at 100 days o Incidence of moderate, severe chronic GVHD
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Dosing based on using fludarabine AUC pharmacokinetic (PK)-guided information could reduce exposure variability, allowing for more predictable toxicity and efficacy as measured by less graft failure, relapse, and non-relapse mortality leading to improved overall survival.

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.	The nucleoside analog fludarabine has become a cornerstone of modern conditioning regimens, particularly in non-myeloablative (NMA) and reduced-intensity conditioning (RIC) protocols, due to its potent immunosuppressive effects. Optimal fludarabine exposure will enhance early engraftment and full T-cell chimerism while minimizing significant non-hematopoietic toxicity. Inadequate fludarabine dosing may increase risk of non-engraftment and relapse. The dosing of fludarabine in the allo-HSCT setting remains largely based on body surface area (BSA). This method is imprecise and fails to account for significant interpatient variability in drug metabolism and clearance, with some patients receiving a potentially sub-therapeutic or toxic dose, placing them at risk of adverse clinical outcomes(1)(3). A pharmacokinetic (PK)-guided dosing strategy, which integrates patient-specific factors most importantly, renal function as measured by glomerular filtration rate (GFR) will significantly reduce exposure variability (2). By achieving a more consistent and predictable systemic exposure, this precision medicine approach is hypothesized to improve key clinical outcomes, including reduced non-relapse mortality (NRM), lower risk of disease relapse, and enhanced overall and disease-free survival (OS/DFS)(6). Fludarabine is administered intravenously as a monophosphate prodrug (F-ara-AMP), which is then rapidly dephosphorylated into the principal circulating metabolite, 2-fluoro-ara-A (F-ara-A). It is this metabolite, and its subsequent intracellular phosphorylation to the active triphosphate (F-ara-ATP), that is responsible for the drug's therapeutic effects. The decreasing number of target cells during conditioning limit the practicality of measuring intracellular F-ara-ATP. Consequently, clinical pharmacology studies have focused on the systemic kinetics of F-ara-A as it correlates with F-ara-ATP formation (1). The single most critical physiological factor influencing F-ara-A clearance is renal function. The kidney clears approximately 60% of fludarabine's active metabolite and there is a strong correlation between F-ara-A clearance and creatinine clearance (CrCl) or estimated glomerular filtration rate (eGFR)(1). This means that even a moderate decrease in renal function can lead to a significant increase in systemic exposure to the drug. Current clinical practice for adjusting fludarabine dosing based on renal function is inadequate. Manufacturer guidelines

	suggest a non-specific 20% dose reduction for patients with a CrCl between 30 and 79 mL/min and state that the drug is not recommended for patients with a CrCl below 30 mL/min due to insufficient data (fluarabine product insert - accessdata.fda.gov). A substantial body of evidence, though primarily retrospective and correlational, demonstrates a clear relationship between fludarabine's systemic exposure and clinical outcomes. This relationship suggests a narrow therapeutic window, where exposure must be carefully balanced to maximize efficacy while minimizing toxicity(1). High systemic exposure to fludarabine is strongly correlated with an increased risk of toxicity and treatment-related mortality (TRM). One study found a strong association between high plasma concentrations of F-ara-A and an increased risk of TRM and reduced overall survival (OS)(3). Specifically, patients with a first dose F-ara-A area-under-the-curve (AUC) greater than 6.5 µg*h/mL experienced a 4.56 times greater risk of TRM and significantly lower OS. In a different analysis, a predictive model for F-ara-A clearance showed that a lower predicted clearance (<8.50 L/h) and a higher predicted first dose AUC (>6.00 µg×h/mL) were significantly associated with a higher hazard ratio of non-relapse mortality (NRM) at day 100(2). Fludarabine exposure may also influence the development of GVHD. Population pharmacokinetic studies have found that high fludarabine AUC was a significant factor associated with the development of acute GVHD(4). Another study noted that a lower fludarabine clearance trended toward a higher risk of acute GVHD, consistent with the hypothesis that higher exposure may lead to greater toxicity and/or GVHD(2). There is an exposure-response relationship for fludarabine that suggests a shift toward a more precise dosing strategy will improve outcomes. Population pharmacokinetic (PopPK) models for fludarabine are available that successfully predict drug clearance by integrating actual body weight, height, and eGFR(1)(see figure below for the published model). This approach overcomes the difficulty with therapeutic drug monitoring, which isn't feasible due to lack of F-ara-A measurement outside of a research setting. These models have been prospectively validated and have been shown to achieve a more precise overall exposure of fludarabine compared to standard dosing(5). A previous study of patients receiving fludarabine, busulfan, and ATG found an optimal AUC of 20 mg*h/L(6). Patients with higher

Field	Response
	exposure experienced more NRM and infection, while those with lower exposure had more graft failure and NRM. Further testing of this approach will occur in an upcoming BMT CTN protocol focused on children with non-malignant diseases. Most patients undergoing allogeneic HSCT receive fludarabine, typically combined with busulfan, melphalan, or cyclophosphamide and low-dose TBI in myeloablative, reduced-intensity, or non-myeloablative conditioning regimens respectively. Our proposal assumes most patients receive fludarabine dosed according to their BSA. We propose to calculate the fludarabine exposure based on the Langenhorst pK model(1) and study the impact of the fludarabine exposure on HSCT relevant outcomes. To help clarify the impact in different regimen intensities, we will restrict the population of patients to those receiving a fludarabine/busulfan (myeloablative), fludarabine/melphalan (reduced-intensity), or fludarabine/cyclophosphamide/2 Gy TBI (non-myeloablative). All patients eligible will have received PTCy/MMF with sirolimus or tacrolimus.
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Id	F_211BoOHF0exMkTc
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Name	Model1.png
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Size	33543
SCIENTIFIC JUSTIFICATION: If applicable, upload graphic as a single file (JPG, PNG, GIF) - Type	image/png

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion Criteria Adult recipients (> 18 years) of allogeneic HSCT Diagnosis of either AML and MDS Receiving a first allogeneic transplant from a MSD, MUD, MMUD, or haploidentical donor Conditioning regimen contains fludarabine combined with one of the following agents: o Busulfan (myeloablative dosing) o Melphalan (100-140 mg/m2) (RIC) o Cyclophosphamide, total body irradiation 2 Gy (NMA) Received PTCy, MMF, and CNI or PTCy, MMF and sirolimus for GVHD prophylaxis. Stem cell source restricted to unmanipulated peripheral blood stem cells Exclusion Criteria Ex vivo T cell depletion In vivo T cell depletion with ATG or Campath Use of thiotepa AML with > 5% marrow blasts pre-transplant MDS with > 10% marrow blasts pre-transplant Diagnosis of myelofibrosis PTCy alone without CNI or sirolimus
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	Usage of PTCy is lower in children and AML/MDS less common in this group.

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.	Fludarabine related: Calculated fluarabine AUC based on the Langerhorst model (depicted above)(1) Patient-Related: Age at transplant Sex: Male, Female Actual Body weight Height Serum creatinine at the time of conditioning Karnofsky/Lansky performance score: 90 100 versus < 90 HCT CI: 0-2 versus 3 Recipient CMV serostatus: seropositive versus seronegative Donor-Related: HLA: (4/8, 5/8, 6/8, 7/8, 8/8) HLA-matched sibling (MRD) versus Mismatched relative versus Matched Unrelated donor versus Mismatched unrelated donor Disease-Related: Primary diagnosis: MDS, AML Disease Risk Index Disease status: CR1 versus CR2 for AML IPSS-R at time of HSCT for MDS Graft versus host disease-Related: GVHD prophylaxis: PTCy/CNI/MMF OR PTCy/Sirolimus/MMF Acute GVHD, maximum grade, and date of onset Chronic GVHD, presence or absence; mild, moderate, severe Transplant-related: Conditioning intensity: o Myeloablative (restricted to fludarabine/busulfan) o Reduced-intensity (restricted to fludarabine/melphalan (100-140 mg/m2) o Non-myeloablative (restricted to flu/cy/2Gy TBI) o Serotherapy (yes/no) o Graft type: unmanipulated peripheral blood stem cell graft only Endpoints Primary endpoint o Overall Survival Secondary endpoints o NRM o Relapse/progression o Acute GVHD o Chronic GVHD o Engraftment
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)

REFERENCES:	1. Langenhorst JB, Dorlo TPC, van Maarseveen EM, Nierkens S, Kuball J, Boelens JJ, van Kesteren C, Huitema ADR. Population Pharmacokinetics of Fludarabine in Children and Adults during Conditioning Prior to Allogeneic Hematopoietic Cell Transplantation. Clin Pharmacokinet. 2019 May;58(5):627-637. doi: 10.1007/s40262-018-0715-9. PMID: 30327943; PMCID: PMC6451721. 2. Sanghavi K, Wiseman A, Kirstein MN, Cao Q, Brundage R, Jensen K, Rogosheske J, Kurtzweil A, Long-Boyle J, Wagner J, Warlick ED, Brunstein CG, Weisdorf DJ, Jacobson PA. Personalized fludarabine dosing to reduce nonrelapse mortality in hematopoietic stem-cell transplant recipients receiving reduced intensity conditioning. Transl Res. 2016 Sep;175:103-115.e4. doi: 10.1016/j.trsl.2016.03.017. Epub 2016 Mar 31. PMID: 27094990; PMCID: PMC5003687. 3. Long-Boyle JR, Green KG, Brunstein CG, Cao Q, Rogosheske J, Weisdorf DJ, Miller JS, Wagner JE, McGlave PB, Jacobson PA. High fludarabine exposure and relationship with treatment-related mortality after nonmyeloablative hematopoietic cell transplantation. Bone Marrow Transplant. 2011 Jan;46(1):20-6. doi: 10.1038/bmt.2010.53. Epub 2010 Apr 12. PMID: 20383215; PMCID: PMC2904846. 4. Mohanan E, Panetta JC, Lakshmi KM, Edison ES, Korula A, Fouzia NA, Abraham A, Viswabandya A, Mathews V, George B, Srivastava A, Balasubramanian P. Population pharmacokinetics of fludarabine in patients with aplastic anemia and Fanconi anemia undergoing allogeneic hematopoietic stem cell transplantation. Bone Marrow Transplant. 2017 Jul;52(7):977-983. doi: 10.1038/bmt.2017.79. Epub 2017 May 8. Erratum in: Bone Marrow Transplant. 2018 Nov;53(11):1490. doi: 10.1038/s41409-018-0276-4. PMID: 28481355; PMCID: PMC5584518. 5. Brooks JT, Solans BP, Lu Y, Kharbanda S, Dvorak CC, Lalefar N, Long S, Gupta AO, Horn B, Lamba JK, Huang L, Apsel-Winger B, Keizer RJ, Savic R, Long-Boyle J. Prospective Validation and Refinement of a Population Pharmacokinetic Model of Fludarabine in Children and Young Adults Undergoing Hematopoietic Cell Transplantation. Pharmaceutics. 2022 Nov 15;14(11):2462. doi: 10.3390/pharmaceutics14112462. PMID: 36432661; PMCID: PMC9694406. 6. Langenhorst JB, van Kesteren C, van Maarseveen EM, Dorlo TPC, Nierkens S, Lindemans CA, de Witte MA, van Rhenen A,

Field	Response
	Raijmakers R, Bierings M, Kuball J, Huitema ADR, Boelens JJ. Fludarabine exposure in the conditioning prior to allogeneic hematopoietic cell transplantation predicts outcomes. Blood Adv. 2019 Jul 23;3(14):2179-2187. doi: 10.1182/bloodadvances.2018029421. PMID: 31324638; PMCID: PMC6650734.
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

References

- Langenhorst JB et al. Clin Pharmacokinet 2018

Model

$$\frac{dA_1}{dt} = -(CL_i/V_i) \cdot A_1 - (Q/V_i) \cdot A_1 + (Q/V_{2,i}) \cdot A_2 - (Q_2/V_i) \cdot A_1 + (Q_2/V_{3,i}) \cdot A_3$$

$$\frac{dA_2}{dt} = (Q/V_i) \cdot A_1 - (Q/V_{2,i}) \cdot A_2$$

$$\frac{dA_3}{dt} = (Q_2/V_i) \cdot A_1 - (Q_2/V_{3,i}) \cdot A_3$$

Characteristic	Details

Table 1. Characteristics of US CRF only Adult non-Myelofibrosis AML or MDS Patients with First Allo-HCT during 2008-2022 with MSD, MUD, MMUD or Haploidentical Donor, Conditioning Regimen containing Flu and one of the following: Bu, Mel, Cy, TBI, PTCy/MMF/Sirolimus or PTCy/MMF/CNI, peripheral blood stem cell product

Characteristic	Total
Number of patients	1496
Patient-related	
Age, by decades, no. (%)	
Median (range)	64 (19-82)
10-19	6 (0)
20-29	58 (4)
30-39	69 (5)
40-49	121 (8)
50-59	239 (16)
60-69	709 (47)
70+	294 (20)
Sex, no. (%)	
Male	878 (59)
Female	618 (41)
Race, no. (%)	
White	1164 (78)
Black or African American	205 (14)
Asian	77 (5)
Native Hawaiian or other Pacific Islander	5 (0)
American Indian or Alaska Native	5 (0)
More than one race	8 (1)
Not reported	32 (2)
Karnofsky score prior to HCT, no. (%)	
90-100%	710 (47)
< 90%	774 (52)
Not reported	12 (1)
HCT-CI, no. (%)	
0	222 (15)
1	207 (14)
2	225 (15)
3	256 (17)
4	200 (13)
5+	369 (25)
Not reported	17 (1)

Characteristic	Total
Serum creatinine, no. (%)	
Known	1460 (98)
Unknown	12 (1)
Not reported	24 (2)
GFR, no. (%)	
Known	1430 (96)
Unknown	66 (4)
Disease-related	
Primary disease, no. (%)	
AML	918 (61)
MDS	578 (39)
MDS IPSS-R prognostic risk category / score at HCT, no. (%)	
Not MDS	918 (61)
Very low	49 (3)
Low	132 (9)
Intermediate	157 (10)
High	106 (7)
Very high	59 (4)
Not reported	75 (5)
AML pre-HCT disease stage, no. (%)	
Disease is not AML	578 (39)
CR1	638 (43)
CR2	126 (8)
CR3+	8 (1)
Advanced or active disease	143 (10)
Not reported	3 (0)
ELN 2022 (AML), no. (%)	
Not AML	578 (39)
Normal	197 (13)
Favorable	154 (10)
Intermediate	217 (15)
Poor	345 (23)
Not tested	3 (0)
Not reported	2 (0)
MDS pre-HCT disease stage, no. (%)	
Disease is not MDS/MPN	918 (61)
Early	81 (5)
Advanced	485 (32)

Characteristic	Total
Not reported	12 (1)
Transplant related	
Interval from diagnosis to HCT, months	
Mean (SD)	11.4 (16.69)
Median (25-75 percentile)	6.3 (4.4-10.9)
Range	0.2-257.2
Donor type, no. (%)	
HLA identical sibling	89 (6)
Haploidentical donor	894 (60)
Well-matched unrelated (8/8)	325 (22)
Partially-matched unrelated (7/8)	173 (12)
Mismatched unrelated (<= 6/8)	15 (1)
Donor/recipient CMV serostatus, no. (%)	
+/+	563 (38)
+/-	133 (9)
-/+	442 (30)
-/-	348 (23)
Not reported	10 (1)
Conditioning regimen intensity, no. (%)	
MAC	287 (19)
RIC	596 (40)
NMA	601 (40)
Under review	12 (1)
Conditioning regimen, no. (%)	
TBI/Cy/Flu	631 (42)
TBI/Flu	282 (19)
Flu/Bu	276 (18)
Flu/Mel	290 (19)
Cy/Flu	17 (1)
PTCy/Sirolimus/MMF vs PTCy/CNI/MMF, no. (%)	
PTCy/Sirolimus/MMF	90 (6)
PTCy/CNI/MMF	1406 (94)
Year of current transplant, no. (%)	
2011	1 (0)
2012	5 (0)
2013	14 (1)
2014	69 (5)
2015	132 (9)

Characteristic	Total
2016	146 (10)
2017	143 (10)
2018	197 (13)
2019	254 (17)
2020	194 (13)
2021	181 (12)
2022	160 (11)
Follow-up of survivors, median (range), months	60.0 (3.3-123.7)

Field	Response
Proposal Number	2509-132-BI
Proposal Title	Outcomes of allogeneic hematopoietic stem cell transplant in patients with chronic myelomonocytic leukemia in the contemporary era
Key Words	CMML; allogeneic stem cell transplant; allo-SCT; post-transplant cyclophosphamide
Principal Investigator #1: - First and last name, degree(s)	Xia Bi, MD MS
Principal Investigator #1: - Email address	xia.bi@jefferson.edu
Principal Investigator #1: - Institution name	Thomas Jefferson University
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	Yes
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	I am a CIBMTR Page Scholar in the leukemia committee.
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	In patients with chronic myelomonocytic leukemia (CMML) undergoing allogeneic hematopoietic stem cell transplantation (allo-HSCT), what are the outcomes of modern graft-versus-host disease (GVHD) prophylaxis with post-transplant cyclophosphamide (PTCY), and how do donor type, conditioning intensity, and disease risk influence non-relapse mortality, relapse, and overall survival in the contemporary era?
RESEARCH HYPOTHESIS:	In adults with CMML undergoing allo-HSCT, the use of PTCY-based GVHD prophylaxis is associated with lower non-relapse mortality and reduced chronic GVHD and, as a result, improved overall survival at 2 years compared with non-PTCY prophylaxis, independent of donor type, conditioning intensity, and baseline disease risk after multivariable adjustment.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary objectives 1. Overall survival Secondary objectives 1. Progression-free survival 2. GVHD-free relapse-free survival 3. Cumulative incidence of non-relapse mortality (NRM) 4. Cumulative incidence of disease relapse 5. Cumulative incidence and severity of acute and chronic GVHD 6. Primary causes of death
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	The proposed study will have a direct impact on both participant care and the advancement of clinical practice in CMML. By generating contemporary outcome data for patients undergoing allo-HSCT with modern GVHD prophylaxis, including PTCY, this research will provide clinicians with accurate and up-to-date benchmarks for survival, relapse, and treatment-related morbidity. Such information will allow for more precise counseling of patients and families regarding the risks and benefits of transplantation, leading to more informed decision-making. Additionally, identifying prognostic factors relevant in the current era, such as donor type, conditioning regimen, and molecular risk features, will enable physicians to better individualize transplant strategies, refine patient selection, and optimize post-transplant monitoring. The study findings will fill a critical gap in the literature by replacing outdated outcome data with analyses that reflect contemporary standards of care. This will inform future clinical trials, guide updates to transplantation guidelines, and support the development of risk-adapted treatment strategies. Ultimately, completion of these aims will improve the safety, effectiveness, and personalization of allo-HSCT for CMML, advancing both patient outcomes and the field of transplant medicine.

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 stem cell transplantation (allo-HSCT) is the only potentially curative option for patients with CMML, but it carries high risks of morbidity and mortality. Previous large registry-based studies have provided important insights into transplant outcomes, yet these data largely reflect older transplant practices that may not be representative of current standards. For example, the 2023 CIBMTR analysis by Mei et al. evaluated 313 CMML patients who underwent allo-HSCT between 2001 and 2017, all of whom received matched related or unrelated donor grafts (1). Importantly, none of these patients were treated with post-transplant cyclophosphamide (PTCY) for graft-versus-host disease (GVHD) prophylaxis, which has since become the contemporary standard of care based on the CTN 1703 trial (2). Additionally, more than half of the CIBMTR cohort had low or intermediate-1 CPSS risk scores, representing a population with relatively low likelihood of progression to acute myeloid leukemia and not necessarily reflective of today's higher-risk transplant candidates. Similarly, the largest retrospective series published by the European Group for Blood and Marrow Transplantation (EBMT) included 513 patients and reported a 4-year non-relapse mortality (NRM) of 41%, relapse incidence of 32%, and overall survival of 33% (3). However, 60% of these patients were transplanted before 2006, an era when supportive care, conditioning approaches, and GVHD prophylaxis were significantly different from current practice. These prior studies have notable strengths, including large sample sizes, multi-center participation, and long-term follow-up, all of which provide robust outcome estimates for their respective eras. However, their weaknesses are equally clear: limited donor diversity (haploidentical donors were excluded), outdated GVHD prophylaxis regimens, and patient risk profiles that do not align with modern transplant indications. As a result, the prognostic models and outcome benchmarks derived from these cohorts may no longer be applicable in the PTCY era. Updated research is urgently needed to evaluate outcomes of CMML patients undergoing allo-HSCT with contemporary GVHD prophylaxis and broader donor availability, including haploidentical transplantation. Such data would provide more accurate estimates of survival, relapse, and treatment-related toxicity, while also identifying relevant prognostic factors in today's clinical context.

Field	Response
	Ultimately, this information is critical to guide clinical decision-making, refine patient selection, and inform future clinical trials and guidelines.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria: Adult patients 18 years old with CMML who underwent allo-HSCT on or after 2010 will be included in this analysis. Exclusion criteria: Patients who experienced disease transformation to secondary acute myeloid leukemia at any time prior to allo-HSCT will be excluded.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	This study does not include pediatric patients because CMML is an overwhelmingly adult disease, with a median age at diagnosis in the mid-60s.
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: 1. Age at transplant 2. Gender 3. Ethnicity 4. Karnofsky performance score 5. HCT-CI Disease related: 1. CMML status according to WHO criteria at transplant: CMML-0 vs CMML-1 vs CMML-2 2. CPSS score 3. CPSS-mol score 4. Disease status at transplant 5. Relapse 6. Time from HCT to relapse Treatment related: 1. Therapy prior to transplant 2. Graft source: bone marrow vs peripheral blood 3. Donor type: matched related donor vs haploidentical vs matched unrelated vs mismatched unrelated 4. Donor/recipient CMV serostatus 5. Conditioning intensity 6. Type of GVHD prophylaxis 7. Incidence and severity of acute GVHD 8. Incidence and severity of chronic GVHD 9. Year of HCT 10. Date and cause of death
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.	NA
REFERENCES:	1. Mei M, Pillai R, Kim S, Estrada-Merly N, Afkhami M, Yang L, et al. The mutational landscape in chronic myelomonocytic leukemia and its impact on allogeneic hematopoietic cell transplantation outcomes: a Center for Blood and Marrow Transplantation Research (CIBMTR) analysis. Haematologica. 2023;108(1):150-60. 2. Bolanos-Meade J, Hamadani M, Wu J, Al Malki MM, Martens MJ, Runaas L, et al. Post-Transplantation Cyclophosphamide-Based Graft-versus-Host Disease Prophylaxis. N Engl J Med. 2023;388(25):2338-48. 3. Symeonidis A, van Biezen A, de Wreede L, Piciocchi A, Finke J, Beelen D, et al. Achievement of complete remission predicts outcome of allogeneic haematopoietic stem cell transplantation in patients with chronic myelomonocytic leukaemia. A study of the Chronic Malignancies Working Party of the European Group for Blood and Marrow Transplantation. Br J Haematol. 2015;171(2):239-46.
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

Table 1. Characteristics of adult patients who received first allogenic transplant with CMML at U.S. during 2010-2024

Characteristic	N (%)
No. of patients	1812
Patient-related Characteristics	
Age, by decades, no. (%)	
Median (range)	65 (18-80)
18-19	2 (<1)
20-29	8 (<1)
30-39	41 (2)
40-49	113 (6)
50-59	366 (20)
60-69	921 (51)
70+	361 (20)
Sex, no. (%)	
Male	1233 (68)
Female	579 (32)
Karnofsky score prior to HCT, no. (%)	
90-100%	972 (54)
< 90%	818 (45)
Not reported	22 (1)
Race, no. (%)	
White	1585 (87)
Black or African American	87 (5)
Asian	57 (3)
Native Hawaiian or other Pacific Islander	8 (<1)
American Indian or Alaska Native	3 (<1)
More than one race	5 (<1)
Not reported	67 (4)
HCT-CI, no. (%)	
0	340 (19)
1	275 (15)
2	288 (16)
3	326 (18)
4	243 (13)
5+	323 (18)
Not reported	17 (1)
Disease-related Characteristics	
Specify disease classification, no. (%)	

Characteristic	N (%)
CMMoL Chronic myelomonocytic leukemia	1735 (96)
Chronic myelomonocytic leukemia (CMML), Myeloproliferative	77 (4)
Disease status prior to HCT (MDS), no. (%)	
Complete remission (CR)	245 (14)
Hematologic improvement (HI)	319 (18)
No response / stable disease (NR/SD)	1153 (64)
Progression from hematologic improvement (Prog from HI)	50 (3)
Relapse from complete remission (Rel from CR)	5 (<1)
Not assessed	5 (<1)
Supportive care or treatment without chemotherapy (2400v2 Q230)	33 (2)
Not reported	2 (<1)
Transplant-related Characteristics	
Donor type, no. (%)	
HLA identical sibling	341 (19)
Haploidentical donor	247 (14)
Other related	14 (1)
Well-matched unrelated (8/8)	961 (53)
Partially-matched unrelated (7/8)	145 (8)
Mismatched unrelated (<= 6/8)	8 (<1)
Multi-donor	13 (1)
Unrelated (matching cannot be determined)	47 (3)
Cord blood	36 (2)
Donor/recipient CMV serostatus, no. (%)	
+/+	524 (29)
+/-	237 (13)
-/+	489 (27)
-/-	518 (29)
CB - recipient +	25 (1)
CB - recipient -	12 (1)
Not reported	7 (<1)
Product type, no. (%)	
BM	142 (8)
PBSC	1633 (90)
UCB	37 (2)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)	
MAC	637 (35)
RIC	940 (52)
NMA	195 (11)

Characteristic	N (%)
Not reported	40 (2)
Conditioning regimen, no. (%)	
TBI/Cy	24 (1)
TBI/Cy/Flu	224 (12)
TBI/Cy/Flu/TT	4 (<1)
TBI/Cy/TT	1 (<1)
TBI/Mel	67 (4)
TBI/Flu	131 (7)
TBI/other(s)	4 (<1)
Bu/Cy	140 (8)
Bu/Mel	3 (<1)
Flu/Bu/TT	73 (4)
Flu/Bu	607 (33)
Flu/Mel/TT	13 (1)
Flu/Mel	478 (26)
Cy/Flu	8 (<1)
Mel alone	2 (<1)
Mel/other(s)	6 (<1)
Treosulfan	3 (<1)
Carb/other(s)	1 (<1)
TLI	6 (<1)
Other(s)	14 (1)
Missing	3 (<1)
GVHD prophylaxis, no. (%)	
CD34 selection	17 (1)
PtCy + other(s)	701 (39)
PtCy alone	6 (<1)
TAC + MMF +- other(s) (except PtCy)	165 (9)
TAC + MTX +- other(s) (except MMF, PtCy)	661 (36)
TAC + other(s) (except MMF, MTX, PtCy)	111 (6)
TAC alone	31 (2)
CSA + MMF +- other(s) (except PtCy,TAC)	77 (4)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	19 (1)
CSA alone	2 (<1)
Other(s)	13 (1)
Missing	9 (<1)
Time from diagnosis, no. (%)	
0-6 months	525 (29)

Characteristic	N (%)
6-12 months	727 (40)
>= 12 months	560 (31)
Year of current transplant, no. (%)	
2010	48 (3)
2011	56 (3)
2012	57 (3)
2013	72 (4)
2014	80 (4)
2015	91 (5)
2016	106 (6)
2017	121 (7)
2018	111 (6)
2019	158 (9)
2020	135 (7)
2021	156 (9)
2022	160 (9)
2023	198 (11)
2024	229 (13)
2025	34 (2)
Median follow-up of survivors (range), months, median (range), months	48.5 (1.0-171.7)

Field	Response
Proposal Number	2509-170-BARANWAL
Proposal Title	Late-relapse and long-term outcomes in patients with AML/MDS receiving post-transplant cyclophosphamide for GVHD prophylaxis.
Key Words	AML, MDS, methotrexate, post-transplant cyclophosphamide, relapse
Principal Investigator #1: - First and last name, degree(s)	Anmol Baranwal, MD
Principal Investigator #1: - Email address	Anmol_Baranwal@rush.edu
Principal Investigator #1: - Institution name	Rush University Medical Center
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Celalettin Ustun
Principal Investigator #2 (If applicable): - Email address:)	Celalettin_Ustun@rush.edu
Principal Investigator #2 (If applicable): - Institution name:	Rush University Medical Center
Principal Investigator #2 (If applicable): - Academic rank:	Professor
Junior investigator status (defined as 㑊、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:	Anmol Baranwal
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	NA
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	Is post-transplant cyclophosphamide (PT-Cy) associated with late relapses, beyond 12 months after transplant.

Field	Response
RESEARCH HYPOTHESIS:	We hypothesize that among patients with MDS/AML, PT-Cy is associated with an increased risk of late relapse.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary objective: Overall survival Secondary objectives: Cumulative incidence of relapse and disease-free survival. Landmark analysis for relapse incidence and disease-free survival with the landmark timepoint of 1 year.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	While PT-Cy is associated with overall similar 1-year outcomes, compared to methotrexate-based GVHD prophylaxis, outcomes beyond 1-year are limited. This study will help evaluate long-term outcomes and will help to determine the most optimal GVHD prophylaxis.

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.	Allogeneic hematopoietic stem cell transplantation (alloHCT) is a potentially curable treatment strategy for patients with hematologic malignancies. The BMT-CTN 1703 trial showed that the 1-year GVHD-free, relapse-free survival (GRFS) was significantly better among patients receiving post-transplant cyclophosphamide (PT-Cy), compared to those receiving methotrexate (MTX) for GVHD prophylaxis (52.7% vs. 34.9%).(1) However, the disease-free survival was similar between the PT-Cy and MTX groups (67% vs. 62.4%). In an updated follow-up of the BMT-CTN 1703 study, including patients 70 years of age, the adjusted GRFS was 67.1% in the PT-Cy group and 29.5% in the MTX group.(2) Al Malki et al. recently showed that PT-Cy was safe and effective, regardless of conditioning intensity, for patients proceeding for alloHCT with a mismatched unrelated donor (MMUD).(3) While the risk of relapse after alloHCT has significantly decreased with post-transplant maintenance therapies,(4 7) most of the post-transplant maintenance treatment strategies are recommended for a duration of 1 year.(4,6,7) Hassan et al. recently published outcomes of patients with high-risk myeloid malignancies receiving PT-Cy for GVHD prophylaxis.(8) The authors showed that, compared to MTX, PT-Cy was associated with an increased risk of 2-year relapse (2-year CIR: 50.2% vs. 17.3%, P < 0.001), suggesting that patients receiving PT-Cy may be having late relapses. However, the study was limited by a small sample size with only 36 patients receiving PT-Cy. Therefore, there currently exists a need to evaluate outcomes of patients receiving PT-Cy with a longer follow-up and assess incidence of late-relapses in comparison to the, commonly used, methotrexate-based prophylaxis.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Adult patients with MDS or AML receiving their first alloHCT and receiving either PT-Cy or MTX for GVHD prophylaxis will be included in the study.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	The study evaluates adult patients with AML and MDS. Therefore, pediatric patient population was excluded.
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 will be required on the patient's baseline demographics, disease characteristics, GVHD prophylaxis, post-transplant relapse and death. All the data needed are available in the CIBMTR forms.

Field	Response
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
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.	NA

Field	Response
REFERENCES:	1. Bola os-Meade J, Hamadani M, Wu J, Al Malki MM, Martens MJ, Runaas L, et al. Post-Transplantation Cyclophosphamide-Based Graft-versus-Host Disease Prophylaxis. New England Journal of Medicine. 2023 Jun 22;388(25):2338 48. 2. Abedin S, Martens MJ, Bola os-Meade J, Al Malki MM, Lian Q, Runaas L, et al. Impact of posttransplant cyclophosphamide-based GVHD prophylaxis in patients 70 years and older: an update from BMT CTN 1703. Blood Adv. 2025 Jul 22;9(14):3495 501. 3. Al Malki MM, Bo-Subait S, Logan B, Olson J, Kou J, Smith S, et al. Post-Transplant Cyclophosphamide-Based Graft-Versus-Host Disease Prophylaxis After Mismatched Unrelated Donor Peripheral Blood Stem Cell Transplantation. Journal of Clinical Oncology. 2025 Sep;43(25):2772 81. 4. Xuan L, Wang Y, Yang K, Shao R, Huang F, Fan Z, et al. Sorafenib maintenance after allogeneic haemopoietic stem-cell transplantation in patients with FLT3-ITD acute myeloid leukaemia: long-term follow-up of an open-label, multicentre, randomised, phase 3 trial. Lancet Haematol. 2023 Aug;10(8):e600 11. 5. Levis MJ, Hamadani M, Logan B, Jones RJ, Singh AK, Litzow M, et al. Gilteritinib as Post-Transplant Maintenance for AML With Internal Tandem Duplication Mutation of FLT3. Journal of Clinical Oncology. 2024 May 20;42(15):1766 75. 6. Gao L, Zhang Y, Wang S, Kong P, Su Y, Hu J, et al. Effect of rhG-CSF Combined With Decitabine Prophylaxis on Relapse of Patients With High-Risk MRD-Negative AML After HSCT: An Open-Label, Multicenter, Randomized Controlled Trial. Journal of Clinical Oncology. 2020 Dec 20;38(36):4249 59. 7. Garcia JS, Kim HT, Murdock HM, Ansuinelli M, Brock J, Cutler CS, et al. Prophylactic maintenance with venetoclax/azacitidine after reduced-intensity conditioning allogeneic transplant for high-risk MDS and AML. Blood Adv. 2024 Feb 27;8(4):978 90. 8. Hassan K, Baranwal A, Kassis AR, Braun J, Bartoo G, Wolf R, et al. Risk of Relapse Post Reduced Intensity Conditioning Allogeneic Stem Cell Transplant in Patients With High-Risk Myeloid Neoplasms Based on GvHD Prophylaxis: PTCy Vs. TAC/MTX. Am J Hematol. 2025 Sep 4; DOI: 10.1002/ajh.70059

Field	Response
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.	NA

Table 1. Characteristics of adult patients who received first allogenic transplant for AML or MDS with PT-Cy or MTX for GVHD prophylaxis at U.S. during 2008-2025

Characteristic	N (%)
No. of patients	42370
Patient-related Characteristics	
Age, by decades, no. (%)	
Median (range)	60 (18-88)
18-19	332 (1)
20-29	2241 (5)
30-39	3121 (7)
40-49	5132 (12)
50-59	10454 (25)
60-69	15811 (37)
70+	5279 (12)
Sex, no. (%)	
Male	24084 (57)
Female	18286 (43)
Karnofsky score prior to HCT, no. (%)	
90-100%	22129 (52)
< 90%	19495 (46)
Not reported	746 (2)
Race, no. (%)	
White	36074 (85)
Black or African American	2684 (6)
Asian	1627 (4)
Native Hawaiian or other Pacific Islander	83 (<1)
American Indian or Alaska Native	128 (<1)
More than one race	226 (1)
Not reported	1548 (4)
HCT-CI, no. (%)	
0	8685 (20)
1	6386 (15)
2	6303 (15)
3	7651 (18)
4	5330 (13)
5+	7685 (18)
Not reported	330 (1)
Disease-related Characteristics	
What was the disease status (AML)?, no. (%)	

Characteristic	N (%)
Primary induction failure	2764 (10)
1st complete remission	18342 (69)
2nd complete remission	3897 (15)
1st relapse	1087 (4)
>= 3rd complete remission	297 (1)
2nd relapse	179 (1)
>= 3rd relapse	31 (<1)
Never treatment	50 (<1)
Not reported	8 (<1)
Disease status prior to HCT (MDS), no. (%)	
Complete remission (CR)	1778 (11)
Hematologic improvement (HI)	2396 (15)
No response / stable disease (NR/SD)	9770 (62)
Progression from hematologic improvement (Prog from HI)	536 (3)
Relapse from complete remission (Rel from CR)	55 (<1)
Not assessed	103 (1)
Supportive care or treatment without chemotherapy (2400v2 Q230)	576 (4)
Partial clinical remission(PR)	46 (<1)
Clinical Improvement(CI)	150 (1)
Progressive disease(PD)	72 (<1)
Treated with chemotherapy (2400v2 Q230)	1 (<1)
Not reported	232 (1)
Transplant-related Characteristics	
Donor type, no. (%)	
HLA identical sibling	8996 (21)
Haploidentical donor	7242 (17)
Other related	615 (1)
Well-matched unrelated (8/8)	20163 (48)
Partially-matched unrelated (7/8)	3629 (9)
Mismatched unrelated (<= 6/8)	271 (1)
Multi-donor	203 (<1)
Unrelated (matching cannot be determined)	1209 (3)
Cord blood	42 (<1)
Product type, no. (%)	
BM	5009 (12)
PBSC	37319 (88)
UCB	42 (<1)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)	

Characteristic	N (%)
MAC	20799 (49)
RIC	16109 (38)
NMA	4234 (10)
Not reported	1228 (3)
Conditioning regimen, no. (%)	
TBI/Cy	1379 (3)
TBI/Cy/Flu	4975 (12)
TBI/Cy/Flu/TT	3 (<1)
TBI/Cy/TT	4 (<1)
TBI/Cy/VP	46 (<1)
TBI/VP	49 (<1)
TBI/Mel	1277 (3)
TBI/Flu	2068 (5)
TBI/other(s)	221 (1)
Bu/Cy/Mel	1 (<1)
Bu/Cy	5883 (14)
Bu/Mel	48 (<1)
Flu/Bu/TT	1286 (3)
Flu/Bu	15883 (37)
Flu/Mel/TT	387 (1)
Flu/Mel	7875 (19)
Cy/Flu	240 (1)
Cy alone	6 (<1)
BEAM	1 (<1)
Mel alone	36 (<1)
Mel/other(s)	50 (<1)
Treosulfan	129 (<1)
Carb/other(s)	3 (<1)
TLI	4 (<1)
Other(s)	441 (1)
Missing	75 (<1)
GVHD prophylaxis, no. (%)	
PtCy + other(s)	18129 (43)
PtCy alone	375 (1)
TAC + MTX +- other(s) (except MMF, PtCy)	23866 (56)
Time from diagnosis, no. (%)	
0-6 months	20554 (49)
6-12 months	11394 (27)

Characteristic	N (%)
>= 12 months	10422 (25)
Year of current transplant, no. (%)	
2008	943 (2)
2009	1065 (3)
2010	1193 (3)
2011	1437 (3)
2012	1507 (4)
2013	1825 (4)
2014	2051 (5)
2015	2239 (5)
2016	2358 (6)
2017	2531 (6)
2018	2983 (7)
2019	3296 (8)
2020	3096 (7)
2021	3288 (8)
2022	3408 (8)
2023	4150 (10)
2024	4368 (10)
2025	632 (1)
Median follow-up of survivors (range), months, median (range), months	57.0 (0.8-201.7)

Field	Response
Proposal Number	2509-176-SUMRANSUB
Proposal Title	Outcomes of Allogeneic Hematopoietic Cell Transplantation with Post-Transplant Cyclophosphamide Compared to Conventional GVHD Prophylaxis in TP53-Mutated Acute Myeloid Leukemia and Myelodysplastic syndromes
Key Words	Allogeneic hematopoietic cell transplantation, TP53 mutation, 17p deletion, Acute myeloid leukemia, myelodysplastic syndromes, post-transplant cyclophosphamide
Principal Investigator #1: - First and last name, degree(s)	Nuttavut Sumransub, MD
Principal Investigator #1: - Email address	Nuttavut_Sumransub@dfci.harvard.edu
Principal Investigator #1: - Institution name	Dana-Farber Cancer Institute
Principal Investigator #1: - Academic rank	-
Junior investigator status (defined as 年、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Mahasweta Gooptu, MD
Principal Investigator #2 (If applicable): - Email address:)	Mahasweta_Gooptu@dfci.harvard.edu
Principal Investigator #2 (If applicable): - Institution name:	Dana-Farber Cancer Institute
Principal Investigator #2 (If applicable): - Academic rank:	Assistant Professor of Medicine
Junior investigator status (defined as 年、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:	Nuttavut Sumransub, MD
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	None
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	Yes

Field	Response
If you have already spoken with a scientific director or working committee chair regarding this study, then please specify who:	This study has been discussed with Dr. Najla El Jurdi, Scientific Director of the GVHD Working Group; Dr. Wael Saber, Scientific Director of the Leukemia Working Group; Dr. Lori Muffly, Chair of the Leukemia Working Group; and Dr. Bronwen Shaw, Chief Scientific Director of CIBMTR.
RESEARCH QUESTION:	Does the use of post-transplant cyclophosphamide (PTCy)-based graft-versus-host disease (GVHD) prophylaxis adversely affect relapse risk and relapse-free survival (RFS) in patients with TP53-mutated acute myeloid leukemia (AML) and myelodysplastic syndromes (MDS) treated with allogeneic hematopoietic cell transplantation (HCT) compared to conventional GVHD prophylaxis regimen?
RESEARCH HYPOTHESIS:	Patients with TP53-mutated AML/MDS treated with allogeneic HCT with PTCy-based GVHD prophylaxis have a higher risk of relapse and inferior RFS compared to those receiving conventional GVHD prophylaxis regimens, such as calcineurin inhibitor (CNI) and methotrexate (MTX). We hypothesize that PTCy may impair immune reconstitution early after transplant and lead to increased relapse rates and decreased RFS in this population, who are at particularly high risk for early relapse post-transplantation.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary end point: - Relapse-free survival (RFS): Time from allogeneic HCT to death or relapse. Secondary end points: - Cumulative incidence of relapse: Development of relapse/progression with or without post-transplant maintenance therapy. Events will be summarized by the cumulative incidence estimate. - Overall survival (OS): Time from allogeneic HCT to death from any cause. - Non-relapse mortality (NRM): Death due to conditions other than disease relapse or progression beyond 28 days. - Acute GVHD: Incidence, severity, and time to development of acute GVHD using the standardized definition and grading system (1) - Chronic GVHD: Incidence, severity, and time to the development of chronic GVHD requiring systemic immunosuppression using standardized definition and grading system (2) - GVHD-free relapse-free survival (GRFS): Survival without acute grade III-IV GVHD or chronic GVHD requiring immunosuppression or disease relapse or progression or death - Graft dysfunction (GD) rate: Include primary graft failure, secondary graft failure, and poor graft function as defined per ASTCT/EBMT standard criteria (3) - Cumulative incidence of infection (viral or fungal infection) at 1-year post-HCT Subgroup analysis based: Type of AML (De Novo vs s-AML including t-AML) Pre-transplant disease status (CR vs no-CR) (MRD+ vs MRD-) Conditioning intensity (MAC vs RIC) TP53 mutation status (single-hit vs multi-hit). Single-hit defined as single TP53 point mutation. Multi-hit defined as 2 TP53 mutations or 1 point mutation in combination with TP53 deletion, or chromosome 17/17p deletion by karyotype (4)

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.	This study aims to advance the understanding of the interplay between GVHD prophylaxis strategies and relapse risk in TP53-mutated AML/MDS, a disease subtype with limited therapeutic options and poor outcomes. While PTCy has become the standard of care for GVHD prophylaxis in HLA-matched HCT following the results of BMT CTN 1703, where relapse rates were similar between the PTCy/Tac/MMF and Tac/MTX groups, the impact of genomic status on relapse outcomes was not specifically examined in this study. Hence the potential impact of GVHD prophylaxis on outcomes in TP53-mutated AML/MDS remains unexplored. By evaluating the effect of PTCy-based GVHD prophylaxis on relapse risk and other HCT outcomes in this high-risk population, this research aims to provide critical insights that can inform clinical decision-making and optimize GVHD prophylaxis strategies in TP53 mutated AML/MDS.

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.	TP53-mutated AML/MDS represents the most treatment refractory of all myeloid neoplasm subtypes, with dismal outcomes despite advancements in therapeutic strategies (5, 6). Mutant TP53 AML and MDS-EB do not differ with respect to molecular characteristics and survival and were suggested to be considered as a single molecular disease entity (6). The 2022 ELN classification categorizes "AML/MDS with mutated TP53" as a separate entity, characterized by highly aggressive disease biology and poor prognosis, underscoring the urgent need for improved therapeutic approaches in this population (7). Allogeneic HCT still remains the only potentially curative option for TP53-mutated AML/MDS. However, outcomes following allogeneic HCT are significantly impaired, with relapse being the predominant cause of treatment failure (8, 9). There is currently debate regarding consensus on the optimal transplant strategy for this high-risk subgroup (10). Recently published data indicated that TP53-mutated AML/MDS patients undergoing allogeneic HCT have 2-years overall survival in the range of 20-30% (4, 11, 12), with relapse rates as high as 74% at 12 months post-HCT (9). The prognosis is especially poor in the subgroup with TP53 mutation variant allele frequency (VAF) 50% and those with complex/5q/7q cytogenetic abnormalities (13). These findings highlight the critical need to optimize transplant protocols to improve disease control and survival outcomes in TP53-mutated AML/MDS. Emerging evidence suggests that, in the pre-transplant setting, the microenvironment in TP53-mutated AML/MDS may have an immune privileged evasive phenotype, with notably, significantly increased PDL1 expression in stem cells of patients with TP53 mutations. The disease is also associated with MYC upregulation and marked downregulation of MYC's negative regulator miR-34a, a p53 transcription target, significantly reduced numbers of bone marrow-infiltrating OX40+ cytotoxic T cells and helper T cells, as well as decreased ICOS+ and 4-1BB+ natural killer cells (14). Preliminary data from our institution further suggests a link between defects in immune reconstitution early after transplant and outcomes in TP53 mutated AML. Using single-cell sequencing strategies on longitudinal bone marrow samples post-transplant in TP53 mutated AML patients, we found that reduced TCR

	diversity 2-6 months post-transplant was associated with eventual relapse (15). Interestingly these findings were not seen in non-TP53 mutated disease (Gooptu et al. Abstract submitted to ASH conference 2025). This further suggests that TP53 mutated myeloid disease may have a distinctive effect on immune environment before and after transplantation, which may affect relapse risk. PTCy has emerged as the new standard for GVHD prophylaxis irrespective of donor type due to its ability to effectively control GVHD. PTCy spares regulatory T cells, which are critical for GVHD control, but induces depletion and persistent dysfunction of alloreactive effector T cells and natural killer (NK) cells, both of which are essential for GVL (16). While this theoretically raises concerns about the potential impact of PTCy on relapse risk in TP53-mutated AML/MDS, across numerous studies, increased relapse rates have not been found with PTCy based GVHD prophylaxis (17-19). Immune reconstitution analyses from Kean et al from BMT CTN 1801, the companion study to BMT CTN 1703, has elegantly shown markedly constrained TCR diversity early after transplant in the PTCy arm when compared to the Tac/MTX arm (20), which may be relevant in the beneficial effects of PTCy on GVHD prevention but may also be relevant in particularly aggressive genomic sub-groups in terms of relapse risk. Our hypothesis was supported by recent observational study in high-risk myeloid neoplasms from Hassan et al which demonstrated higher 1-year cumulative incidence of relapse (CIR) in PTCy compared to TAC/MTX (37.6% vs 11.1%, p=0.01) and CIR of 34.7% vs 10.2% (p=0.007) when limiting the analysis to MRD/MUD subgroup. (High-risk AML was defined as AML with 1 of the following: complex or monosomal karyotype, TP53, WT1, FLT3 ITD+/NPM1-, active disease, MRD+, secondary AML. High-risk MDS was defined as MDS with 1 of the following: complex karyotype, monosomal karyotype, TP53, RAS pathway mutation, marrow blast of 10% or more or chronic myelomonocytic leukemia.) PTCy and high disease risk index were independent predictive factors of post-transplant relapse in multivariate analysis in this study (21). In conclusion it is important to critically examine the effect of PTCy based prophylaxis on relapse outcomes specifically in sub-groups such as TP53 mutated disease which are already predisposed to early relapse due to the aggressive nature of the disease and where the immune environment early

Field	Response
	post-transplant may be critical to prevent relapse. While PTCy has become the standard of care for GVHD prophylaxis following the results of BMT CTN 1703 (22), its impact on outcomes in TP53-mutated AML/MDS remains unexplored. This study aims to address this critical gap by evaluating the impact of PTCy, focusing on relapse risk, RFS, and OS in TP53-mutated AML patients compared to conventional GVHD prophylaxis regimens, such as CNI/MTX.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria: - Adult (18 years of age) patients with AML or MDS with pathogenic TP53 mutation by next-generation sequencing (NGS) or with loss of the TP53 gene locus (chromosome 17/17p deletion) demonstrated by cytogenetic testing - Received treatment with allogeneic HCT between 2014-2022 from HLA-matched related donor or HLA-matched unrelated donor (MRD/MUD) - Received GVHD prophylaxis with PTCy-based or CNI/MTX regimens Exclusion criteria: - Patients with a history of prior allogeneic HCT - Patients who received GVHD prophylaxis regimen containing ATG
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	Pediatric patients are not included in this study due to significant biological, clinical, and therapeutic differences between pediatric and adult populations in the context of TP53-mutated AML and allogeneic HCT. Although we foresee that knowledge obtained from this study can be a foundation for further study in pediatric population in the future.

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 variables 1. Age 2. Gender 3. Karnofsky performance score (KPS) 4. Hematopoietic cell transplant comorbidity index (HCT-CI) Disease variables 5. Cytogenetics abnormalities at diagnosis per ELN2022 classification (conventional cytogenetic or FISH): Chromosome 17p and other cytogenetic abnormalities 6. Molecular profile (NGS) at diagnosis: Any TP53 mutation by NGS regardless of VAF or number of mutations and other genetic abnormalities (co-mutations) 7. Baseline hemoglobin, WBC, absolute neutrophil, and platelet counts at diagnosis 8. Baseline peripheral blast count 9. Baseline bone marrow blast count 10. Extramedullary disease (Y/N) 11. Secondary AML (therapy-related AML or AML evolving from a pre-existing hematologic disorder) 12. Treatments prior to HCT a. Intensive induction (7+3 or CPX-351 or FLAG-Ida-Ven) (Y/N) b. Hypomethylating therapy (Y/N) c. BCL2 inhibitor therapy (Y/N) d. Other therapies (Y/N) 13. Number of induction chemotherapies to achieve CR1 14. Number of lines of therapy prior to HCT Transplant-related variables 15. Time from diagnosis to HCT 16. Disease status at transplantation (including MRD status) 17. Conditioning regimen (MAC, RIC, NMA) 18. Donor type (sibling, related, unrelated) 19. HLA status (matched donor only) 20. Graft source 21. GVHD prophylaxis regimen (PTCy-based vs CNI/MTX) 22. Post-transplant maintenance treatment (Y/N) 23. Post-transplant salvage treatment (Y/N) a. Intensive induction (7+3 or CPX-351 or FLAG-Ida-Ven) (Y/N) b. Hypomethylating therapy (Y/N) c. BCL2 inhibitor therapy (Y/N) d. Cellular therapy (DLI or second transplant) (Y/N) Outcome variables 24. Time to neutrophil engraftment 25. Time to platelet engraftment 26. Cumulative incidence of viral infection at 1-year post-HCT (CMV, EBV, Adenovirus,

Field	Response
	HHV-6, BK virus, and respiratory virus infection). 27. Cumulative incidence of fungal infection at 1-year post-HCT (Candida, Aspergillus, Blastomyces, Cryptococcus, Fusarium, Histoplasma, Mucorales, Rhizopus, Scedosporium, Zygomycetes) 28. Graft dysfunction (primary graft failure, secondary graft failure, poor graft function) 29. Cumulative incidence of relapse Overt relapse, MRD (MFC, FISH, NGS) 30. Acute GVHD Onset, most severe grade, organ involved, systemic immunosuppression (IS) 31. Chronic GVHD Onset, most severe grade, organ involved, systemic IS 32. Time from HCT to last follow-up 33. Time from HCT to relapse 34. Time from HCT to death and cause of death 35. NRM, GRFS, RFS, and OS (median and percentage survival at 2-year)
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	Not applicable
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 of the investigator's previous e	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:	1. Harris AC, Young R, Devine S, et al. International, Multicenter Standardization of Acute Graft-versus-Host Disease Clinical Data Collection: A Report from the Mount Sinai Acute GVHD International Consortium. Biol Blood Marrow Transplant. 2016;22(1):4-10. 2. Jagasia MH, Greinix HT, Arora M, et al. National Institutes of Health Consensus Development Project on Criteria for Clinical Trials in Chronic Graft-versus-Host Disease: I. The 2014 Diagnosis and Staging Working Group report. Biol Blood Marrow Transplant. 2015;21(3):389-401.e1. 3. Sureda A, Carpenter PA, Bacigalupo A, et al. Harmonizing definitions for hematopoietic recovery, graft rejection, graft failure, poor graft function, and donor chimerism in allogeneic hematopoietic cell transplantation: a report on behalf of the EBMT, ASTCT, CIBMTR, and APBMT. Bone Marrow Transplant. 2024;59(6):832-7. 4. Versluis J, Saber W, Tsai HK, et al. Allogeneic Hematopoietic Cell Transplantation Improves Outcome in Myelodysplastic Syndrome Across High-Risk Genetic Subgroups: Genetic Analysis of the Blood and Marrow Transplant Clinical Trials Network 1102 Study. J Clin Oncol. 2023;41(28):4497-510. 5. Metzeler KH, Herold T, Rothenberg-Thurley M, et al. Spectrum and prognostic relevance of driver gene mutations in acute myeloid leukemia. Blood. 2016;128(5):686-98. 6. Grob T, Al Hinai ASA, Sanders MA, et al. Molecular characterization of mutant TP53 acute myeloid leukemia and high-risk myelodysplastic syndrome. Blood. 2022;139(15):2347-54. 7. D hner H, Wei AH, Appelbaum FR, et al. Diagnosis and management of AML in adults: 2022 recommendations from an international expert panel on behalf of the ELN. Blood. 2022;140(12):1345-77. 8. Lindsley RC, Saber W, Mar BG, et al. Prognostic Mutations in Myelodysplastic Syndrome after Stem-Cell Transplantation. N Engl J Med. 2017;376(6):536-47. 9. Masuda Y, Sadato D, Toya T, et al. Transplantation outcomes of TP53-mutant AML and MDS: a single transplantation center experience of 63 patients. Int J Hematol. 2025;121(6):820-32. 10. Nawas MT, Kosuri S. Utility or futility? A contemporary approach to allogeneic hematopoietic cell transplantation for TP53-mutated MDS/AML. Blood Adv. 2024;8(3):553-61. 11. Badar T, Atallah E, Shallis R, et al. Survival of TP53-mutated acute myeloid leukemia patients receiving allogeneic

	stem cell transplantation after first induction or salvage therapy: results from the Consortium on Myeloid Malignancies and Neoplastic Diseases (COMMAND). Leukemia. 2023;37(4):799-806. 12. Esteve J, Nagler A, Labopin M, et al. Allogeneic Hematopoietic Cell Transplantation in Patients With Acute Myeloid Leukemia With Myelodysplasia-Related Genetic Features: Relevance of the Genetic Underlying Category. A Retrospective Analysis on Behalf of the Acute Leukemia Working Party of the EBMT. Am J Hematol. 2025;100(6):954-62. 13. Lontos K, Saliba RM, Kanagal-Shamanna R, et al. TP53-mutant variant allele frequency and cytogenetics determine prognostic groups in MDS/AML for transplantation. Blood Adv. 2025;9(11):2845-54. 14. Sallman DA, McLemore AF, Aldrich AL, et al. TP53 mutations in myelodysplastic syndromes and secondary AML confer an immunosuppressive phenotype. Blood. 2020;136(24):2812-23. 15. Sariipek N, Safina KR, Cutler C, et al. Post-Transplant T Cell Clonotype Diversity Is Associated with Survival in Patients with TP53-Mutated Acute Myeloid Leukemia. Blood. 2023;142:2176. 16. Wachsmuth LP, Patterson MT, Eckhaus MA, et al. Post-transplantation cyclophosphamide prevents graft-versus-host disease by inducing alloreactive T cell dysfunction and suppression. J Clin Invest. 2019;129(6):2357-73. 17. Luznik L, Pasquini MC, Logan B, et al. Randomized Phase III BMT CTN Trial of Calcineurin Inhibitor-Free Chronic Graft-Versus-Host Disease Interventions in Myeloablative Hematopoietic Cell Transplantation for Hematologic Malignancies. J Clin Oncol. 2022;40(4):356-68. 18. Shimoni A, Peczynski C, Labopin M, et al. Post-transplant cyclophosphamide separates graft-versus host disease and graft versus leukemia effects after HLA-matched stem-cell transplantation for acute myeloid leukemia. Leukemia. 2025;39(1):222-8. 19. McCurdy SR, Luznik L. Relapse after allogeneic transplantation with post-transplant cyclophosphamide: Shattering myths and evolving insight. Blood Rev. 2023;62:101093. 20. Siegel SJ, DeWolf S, Schmalz J, et al. Graft-versus-host disease prophylaxis shapes T cell biology and immune reconstitution after hematopoietic cell transplant. medRxiv. 2025. 21. Hassan K, Baranwal A, Mangaonkar AA, et al. Risk of Relapse Post Reduced Intensity Conditioning Allogeneic Stem Cell Transplant in Patients with

Field	Response
	High-Risk Myeloid Neoplasms Based on Ptcy Vs TAC/MTX Gvhd Prophylaxis. Blood. 2024;144(Supplement 1):4918-. 22. Bola os-Meade J, Hamadani M, Wu J, et al. Post-Transplantation Cyclophosphamide-Based Graft-versus-Host Disease Prophylaxis. N Engl J Med. 2023;388(25):2338-48.
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.	None

Table 1. Characteristics of US adult patients who received their first Allo-HCT for AML and MDS during 2018-2022, with HLA-matched related and unrelated donor, Received GVHD prophylaxis with PTCy-based or CNI/MTX regimens (ATG excluded), having TP53/P53/17P/-17 detected.

Characteristic	PTCy based	Conventional
No. of patients	306	718
TED or RES (RF) track determined for this event, no. (%)		
TED	260 (85)	576 (80)
CRF (RES)	46 (15)	142 (20)
Patient-related Characteristics		
Age, by decades, no. (%)		
Median (range)	62 (18-77)	62 (19-80)
10-19	1 (0)	3 (0)
20-29	11 (4)	13 (2)
30-39	21 (7)	33 (5)
40-49	31 (10)	82 (11)
50-59	59 (19)	158 (22)
60-69	130 (42)	329 (46)
70+	53 (17)	100 (14)
Sex, no. (%)		
Male	166 (54)	414 (58)
Female	140 (46)	304 (42)
Race, no. (%)		
White	265 (87)	641 (89)
Black or African American	11 (4)	33 (5)
Asian	15 (5)	16 (2)
Native Hawaiian or other Pacific Islander	0 (0)	1 (0)
American Indian or Alaska Native	0 (0)	2 (0)
More than one race	2 (1)	4 (1)
Not reported	13 (4)	21 (3)
Ethnicity, no. (%)		
Hispanic or Latino	16 (5)	33 (5)
Non-Hispanic or Latino	282 (92)	668 (93)
Non-resident of the U.S.	1 (0)	1 (0)
Not reported	7 (2)	16 (2)
ECOG prior to HCT, no. (%)		
Asymptomatic	159 (52)	345 (48)
Symptomatic but completely ambulatory	131 (43)	351 (49)
Symptomatic, < 50% in bed during the day	7 (2)	15 (2)
Not reported	9 (3)	7 (1)

Characteristic	PTCy based	Conventional
Karnofsky score prior to HCT, no. (%)		
90-100%	159 (52)	345 (48)
< 90%	138 (45)	366 (51)
Not reported	9 (3)	7 (1)
HCT-CI, no. (%)		
0	46 (15)	98 (14)
1	50 (16)	117 (16)
2	38 (12)	98 (14)
3	50 (16)	131 (18)
4	51 (17)	98 (14)
5+	68 (22)	170 (24)
Not reported	3 (1)	6 (1)
Disease-related Characteristics		
Was extramedullary disease present, no. (%)		
No	35 (11)	96 (13)
Yes	0 (0)	6 (1)
Not reported	268 (88)	613 (85)
Uknown	3 (1)	3 (0)
ELN 2022 (AML), no. (%)		
MDS	31 (10)	67 (9)
Favorable	28 (9)	65 (9)
Intermediate	1 (0)	17 (2)
Poor	246 (80)	569 (79)
TP53 mutation, details, no. (%)		
TP53	276 (90)	607 (85)
P53	3 (1)	22 (3)
17p	2 (1)	6 (1)
-17	25 (8)	83 (12)
MDS IPSS-R prognostic risk category / score at HCT, no. (%)		
Not MDS	275 (90)	651 (91)
Very low	1 (0)	2 (0)
Low	1 (0)	3 (0)
Intermediate	10 (3)	15 (2)
High	8 (3)	21 (3)
Very high	7 (2)	20 (3)
Not reported	4 (1)	6 (1)
AML pre-HCT disease stage, no. (%)		
Disease is not AML	31 (10)	67 (9)

Characteristic	PTCy based	Conventional
CR1	212 (69)	466 (65)
CR2	23 (8)	54 (8)
CR3+	1 (0)	6 (1)
Advanced or active disease	39 (13)	124 (17)
Not reported	0 (0)	1 (0)
Treatment-related Characteristics		
Time from diagnosis to HCT, months		
n / N	306/306	718/718
Mean (SD)	7.2 (7.67)	7.6 (10.57)
Median (25-75 percentile)	5.3 (4.1-7.4)	5.2 (4.0-7.3)
Range	1.5-88.3	0.5-127.2
Donor type, no. (%)		
HLA identical sibling	69 (23)	192 (27)
Well-matched unrelated (8/8)	237 (77)	526 (73)
Product type, no. (%)		
BM	11 (4)	60 (8)
PBSC	295 (96)	658 (92)
GVHD prophylaxis, no. (%)		
PtCy + other(s)	303 (99)	0 (0)
PtCy alone	3 (1)	0 (0)
TAC + MMF +- other(s) (except PtCy)	0 (0)	139 (19)
TAC + MTX +- other(s) (except MMF, PtCy)	0 (0)	579 (81)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)		
MAC	138 (45)	310 (43)
RIC	131 (43)	334 (47)
NMA	35 (11)	35 (5)
Not reported	2 (1)	39 (5)
Year of transplant, no. (%)		
2018	31 (10)	162 (23)
2019	49 (16)	149 (21)
2020	69 (23)	140 (19)
2021	68 (22)	136 (19)
2022	89 (29)	131 (18)
Median follow-up of survivors (range), months, median (range), months	43.6 (11.9-82.0)	51.5 (3.5-79.6)

Field	Response
Proposal Number	2509-208-GERGIS
Proposal Title	Allogeneic Hematopoietic Stem Cell Transplantation Outcomes in Accelerated- and Blast-Phase Chronic Myeloid Leukemia in the Tyrosine Kinase Inhibitor Era
Key Words	Chronic myeloid leukemia; accelerated phase; blast phase; allogeneic stem cell transplant; allo-SCT; tyrosine kinase inhibitor
Principal Investigator #1: - First and last name, degree(s)	Usama Gergis, MD
Principal Investigator #1: - Email address	usama.gergis@jefferson.edu
Principal Investigator #1: - Institution name	Thomas Jefferson University
Principal Investigator #1: - Academic rank	Professor
Junior investigator status (defined as 坋、5 years from fellowship)	No
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:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	What are the contemporary outcomes of allogeneic hematopoietic stem cell transplantation (allo-HSCT) in patients with accelerated- or blast-phase chronic myeloid leukemia (CML) in the tyrosine kinase inhibitor (TKI) era, and which pretransplant factors are associated with improved survival and reduced relapse?
RESEARCH HYPOTHESIS:	In patients with accelerated- or blast-phase CML, allo-HSCT performed in the TKI era is associated with improved survival outcomes compared to historical cohorts, and specific pretransplant factors such as disease status at transplant, donor type, and conditioning regimen are independently predictive of post-transplant survival and relapse.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary objectives 1. Overall survival Secondary objectives 1. Progression-free survival 2. GVHD-free relapse-free survival 3. Cumulative incidence of non-relapse mortality (NRM) 4. Cumulative incidence of disease relapse 5. Cumulative incidence and severity of acute and chronic GVHD 6. Primary causes of death
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	The proposed study will directly improve patient care by providing contemporary, real-world evidence on allo-HSCT outcomes in patients with accelerated- and blast-phase CML. By identifying prognostic factors and clarifying the role of transplant in the modern TKI era, this work will support evidence-based decision-making, optimize patient selection, and refine timing of transplantation, ultimately improving survival and quality of care for affected individuals. Scientifically, it will fill a critical knowledge gap, generate the largest and most comprehensive dataset in this population, and inform future clinical trial design and therapeutic strategies, thereby advancing both the science and clinical management of advanced-phase CML.

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.	The introduction of tyrosine kinase inhibitors (TKIs) has fundamentally transformed the management of chronic myeloid leukemia (CML), leading to markedly improved survival for patients diagnosed in the chronic phase. However, once the disease progresses to accelerated phase (AP) or blast phase (BP), outcomes remain poor, with median survival of less than 12 months despite available therapies (1, 2). While TKIs and allogeneic hematopoietic stem cell transplantation (allo-HSCT) are the main treatment strategies in this setting, there is no clear consensus regarding optimal management, and long-term survival outcomes are unsatisfactory. Allo-HSCT represents the only potentially curative treatment for patients with AP or BP CML, yet contemporary data on transplant outcomes in the TKI era are limited. The previous CIBMTR study addressing this question, published by Khoury et al in 2012, analyzed 449 patients with advanced-phase CML previously treated with imatinib who underwent transplantation between 1999 and 2004 (3). These results, while valuable, are not generalizable to the modern era, as they reflect a period when second- and third-generation TKIs were not available, haploidentical transplantation was rarely performed, and current graft-versus-host-disease (GVHD) prophylaxis strategies such as post-transplantation cyclophosphamide (PTCY) (4) were not in use. More recently, the largest retrospective analysis, conducted by the European Society for Blood and Marrow Transplantation (EBMT), evaluated 170 adults who underwent allo-HSCT for BP CML between 2004 and 2016 (5). The study showed a 3-year cumulative incidence of relapse of 51%, non-relapse mortality of 23%, and an overall survival of only 38%. Importantly, multivariate analysis demonstrated that active disease at the time of transplant was the strongest predictor of poor survival, while unrelated donor transplantation was associated with improved leukemia-free survival in patients transplanted with active disease. While this study provides important insights, it was geographically limited, focuses exclusively on BP CML, and highlights the continued need for contemporary, comprehensive analyses. Currently, no data from the CIBMTR have been published regarding allo-HSCT outcomes in patients with AP or BP CML in the modern TKI era, leaving a critical gap in knowledge. Given the persistently poor survival rates, lack of consensus on treatment strategies, and absence of large-scale

Field	Response
	contemporary data, further research is urgently needed. A robust analysis using the CIBMTR database would provide the largest and most diverse assessment of transplant outcomes in this population, enabling the identification of prognostic factors and clarifying the role of allo-HSCT in the TKI era. The results of such research will provide clinicians with evidence-based guidance for patient selection, pretransplant management, and timing of transplant, ultimately improving outcomes and informing future therapeutic strategies for patients with advanced-phase CML.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria: Adult patients 18 years old with accelerated phase or blast phase CML at transplant (before the start of conditioning) and who received their first allo-HSCT on or after 2004 and with prior exposure to at least one TKI will be included in this analysis. Exclusion criteria: Patients with chronic-phase only CML will be excluded.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	CML is primarily a disease of adults.
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: 1. Age at transplant 2. Gender 3. Ethnicity 4. Karnofsky performance score 5. HCT-CI Disease related: 1. Accelerated phase vs blast phase 2. Disease status at transplant 3. Time from diagnosis of AP or BP to transplant (<=12 months vs >12 months) 4. Presence of BCR-ABL1 mutations (T315I, Other than T315I) Treatment related: 1. Stem cell source: bone marrow vs peripheral blood 2. Sex of donor 3. Donor source: MRD vs MUD vs haploidentical vs MMUD 4. Donor/recipient CMV serostatus 5. Conditioning intensity 6. Type of GVHD prophylaxis 7. Year of transplant
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

Field	Response
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	NA
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.	NA
REFERENCES:	1. Mukherjee S, Kalaycio M. Accelerated Phase CML: Outcomes in Newly Diagnosed vs. Progression From Chronic Phase. Curr Hematol Malig Rep. 2016;11(2):86-93. 2. Jabbour E, Cortes J, Santos FP, Jones D, O'Brien S, Rondon G, et al. Results of allogeneic hematopoietic stem cell transplantation for chronic myelogenous leukemia patients who failed tyrosine kinase inhibitors after developing BCR-ABL1 kinase domain mutations. Blood. 2011;117(13):3641-7. 3. Khoury HJ, Kukreja M, Goldman JM, Wang T, Halter J, Arora M, et al. Prognostic factors for outcomes in allogeneic transplantation for CML in the imatinib era: a CIBMTR analysis. Bone Marrow Transplant. 2012;47(6):810-6. 4. Bolanos-Meade J, Hamadani M, Wu J, Al Malki MM, Martens MJ, Runaas L, et al. Post-Transplantation Cyclophosphamide-Based Graft-versus-Host Disease Prophylaxis. N Engl J Med. 2023;388(25):2338-48. 5. Radujkovic A, Dietrich S, Blok HJ, Nagler A, Ayuk F, Finke J, et al. Allogeneic Stem Cell Transplantation for Blast Crisis Chronic Myeloid Leukemia in the Era of Tyrosine Kinase Inhibitors: A Retrospective Study by the EBMT Chronic Malignancies Working Party. Biol Blood Marrow Transplant. 2019;25(10):2008-16.
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

Table 1. Characteristics of US adult patients who received their first Allo-HCT for CML during 2008 to 2024, with TKI prior to transplant

Characteristic	Accelerated phase	Blast phase
No. of patients	305	157
TED or RES (RF) track determined for this event, no. (%)		
TED	217 (71)	95 (61)
CRF (RES)	88 (29)	62 (39)
Patient-related Characteristics		
Age, by decades, no. (%)		
Median (range)	47 (19-77)	44 (18-75)
10-19	2 (1)	1 (1)
20-29	35 (11)	27 (17)
30-39	59 (19)	33 (21)
40-49	79 (26)	40 (25)
50-59	82 (27)	32 (20)
60-69	44 (14)	19 (12)
70+	4 (1)	5 (3)
Sex, no. (%)		
Male	176 (58)	106 (68)
Female	129 (42)	51 (32)
Race, no. (%)		
White	224 (73)	114 (73)
Black or African American	55 (18)	21 (13)
Asian	11 (4)	7 (4)
Native Hawaiian or other Pacific Islander	0 (0)	1 (1)
American Indian or Alaska Native	2 (1)	0 (0)
More than one race	0 (0)	4 (3)
Not reported	13 (4)	10 (6)
Ethnicity, no. (%)		
Hispanic or Latino	45 (15)	24 (15)
Non-Hispanic or Latino	253 (83)	131 (83)
Non-resident of the U.S.	1 (0)	0 (0)
Not reported	6 (2)	2 (1)
ECOG prior to HCT, no. (%)		
Asymptomatic	161 (53)	72 (46)
Symptomatic but completely ambulatory	127 (42)	64 (41)
Symptomatic, < 50% in bed during the day	8 (3)	13 (8)
Symptomatic, > 50% in bed, but not bedbound	1 (0)	1 (1)

Characteristic	Accelerated phase	Blast phase
Bedbound	0 (0)	1 (1)
Not reported	8 (3)	6 (4)
Karnofsky score prior to HCT, no. (%)		
90-100%	161 (53)	72 (46)
< 90%	136 (45)	79 (50)
Not reported	8 (3)	6 (4)
HCT-CI, no. (%)		
0	109 (36)	40 (25)
1	38 (12)	27 (17)
2	42 (14)	22 (14)
3	55 (18)	35 (22)
4	40 (13)	16 (10)
5+	19 (6)	15 (10)
Not reported	2 (1)	2 (1)
Disease-related Characteristics		
Time from DX of accelerated or blast phase to TX, no. (%)		
<= 12 month	43 (14)	37 (24)
> 12 month	192 (63)	81 (52)
Not reported	70 (23)	39 (25)
Was BCR / ABL kinase domain mutation analysis performed?, no. (%)		
No	8 (3)	0 (0)
Yes	1 (0)	0 (0)
Not reported	294 (96)	157 (100)
Uknown	2 (1)	0 (0)
T315I at Diagnosis, no. (%)		
Not Done	1 (0)	0 (0)
Not reported	304 (100)	157 (100)
Treatment-related Characteristics		
Donor type, no. (%)		
HLA identical sibling	85 (28)	40 (25)
Haploidentical donor	41 (13)	21 (13)
Other related	8 (3)	3 (2)
Well-matched unrelated (8/8)	110 (36)	58 (37)
Partially-matched unrelated (7/8)	28 (9)	15 (10)
Mismatched unrelated (<= 6/8)	2 (1)	2 (1)
Multi-donor	1 (0)	0 (0)
Unrelated (matching cannot be determined)	10 (3)	10 (6)

Characteristic	Accelerated phase	Blast phase
Cord blood	20 (7)	8 (5)
Donor/recipient sex match, no. (%)		
M-M	101 (33)	58 (37)
M-F	63 (21)	29 (18)
F-M	62 (20)	42 (27)
F-F	56 (18)	19 (12)
CB - recipient M	13 (4)	6 (4)
CB - recipient F	9 (3)	3 (2)
Not reported	1 (0)	0 (0)
Donor/recipient CMV serostatus, no. (%)		
+/+	103 (34)	59 (38)
+/-	30 (10)	14 (9)
-/+	80 (26)	47 (30)
-/-	70 (23)	27 (17)
CB - recipient +	15 (5)	9 (6)
CB - recipient -	7 (2)	0 (0)
Not reported	0 (0)	1 (1)
Product type, no. (%)		
BM	40 (13)	12 (8)
PBSC	243 (80)	136 (87)
UCB	22 (7)	9 (6)
GVHD prophylaxis, no. (%)		
CD34 selection	5 (2)	1 (1)
PtCy + other(s)	75 (25)	38 (24)
PtCy alone	3 (1)	2 (1)
TAC + MMF +- other(s) (except PtCy)	38 (12)	21 (13)
TAC + MTX +- other(s) (except MMF, PtCy)	128 (42)	62 (39)
TAC + other(s) (except MMF, MTX, PtCy)	16 (5)	14 (9)
TAC alone	7 (2)	3 (2)
CSA + MMF +- other(s) (except PtCy,TAC)	15 (5)	10 (6)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	11 (4)	5 (3)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	1 (0)	0 (0)
Other(s)	4 (1)	0 (0)
Missing	2 (1)	1 (1)
Conditioning regimen intensity (F2400 pre-TED data), no. (%)		
MAC	208 (68)	116 (74)
RIC	73 (24)	25 (16)

Characteristic	Accelerated phase	Blast phase
NMA	19 (6)	12 (8)
Not reported	5 (2)	4 (3)
Year of transplant, no. (%)		
2008	18 (6)	14 (9)
2009	21 (7)	18 (11)
2010	24 (8)	19 (12)
2011	18 (6)	11 (7)
2012	24 (8)	12 (8)
2013	22 (7)	7 (4)
2014	21 (7)	8 (5)
2015	15 (5)	6 (4)
2016	24 (8)	13 (8)
2017	14 (5)	7 (4)
2018	19 (6)	6 (4)
2019	12 (4)	7 (4)
2020	23 (8)	8 (5)
2021	13 (4)	3 (2)
2022	10 (3)	5 (3)
2023	15 (5)	6 (4)
2024	12 (4)	7 (4)
Median follow-up of survivors (range), months, median (range), months	72.3 (3.4-192.5)	73.2 (3.3-194.1)

Field	Response
Proposal Number	2509-223-PARK
Proposal Title	Outcomes of Allogeneic Hematopoietic Cell Transplantation for Large Granular Lymphocytic Leukemia
Key Words	LGLL, Large granular lymphocytic leukemia, hematopoietic cell transplantation
Principal Investigator #1: - First and last name, degree(s)	Sunmin Park MD, PhD
Principal Investigator #1: - Email address	supark@coh.org
Principal Investigator #1: - Institution name	City of Hope Medical Center
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 玆、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Vinod Pullarkat MD
Principal Investigator #2 (If applicable): - Email address:)	vpullarkat@coh.org
Principal Investigator #2 (If applicable): - Institution name:	of
Principal Investigator #2 (If applicable): - Academic rank:	Professor
Junior investigator status (defined as 玆、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:	Vinod Pullarkat
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	None
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Non-Malignant Diseases
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	What are the outcomes of allogeneic hematopoietic cell transplantation (HCT) in patients with large granular lymphocytic leukemia (LGLL)?

Field	Response
RESEARCH HYPOTHESIS:	1. Allogeneic HCT is feasible and can provide long-term disease control in patients with refractory LGLL. 2. The presence of STAT3 and other co-occurring mutations and/or high clonal TCR burden prior to HCT may predict GVHD risk, graft failure or disease relapse.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	1. Characterize patient and transplant features of individuals undergoing HCT for LGLL 2. Estimate the overall survival (OS), transplant-related mortality (TRM), relapse or progression, engraftment, graft-versus-host-disease (GVHD) after HCT 3. Evaluate the impact of somatic mutations and clonal TCR burden with transplant outcomes using pre-HCT biospecimens

SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	LGLL is a rare hematologic condition that is part of a spectrum of acquired immune dysregulation syndrome, characterized by clonal expansion of lymphocytes following chronic antigen stimulation. It is often accompanied by clinical autoimmune disorders. Recurrent mutations, particularly in STAT3,1 STAT5b,2,3 and CCL224 drive resistance to apoptosis and uncontrolled proliferation of clonal T cells, leading to bone marrow, splenic and hepatic infiltration. This results in cytopenia and autoimmune manifestations.5,6 Approximately 85% cases are T-cell LGLL, and 15% are NK cell LGLL. Activating STAT3 mutations, identified in up to 50-70% of T-LGLL cases, are associated with neutropenia, increased autoimmune manifestations, and potentially survival compared to wild-type cases.7 Smaller subsets of patients harbor STAT5b or CCL22 mutations, with its clinical significance to be further elucidated. Additional NGS studies have identified co-occurring alterations in epigenetic regulators (e.g. TET2, KMT2D, IDH1/2, DNMT3A), but their roles in LGLL pathogenesis and prognosis are not yet established.8 10 Although often indolent, LGLL can cause severe cytopenias, transfusion dependence, and autoimmune complications requiring therapy.5 The standard treatment is immunosuppressive therapy (IST), most commonly with methotrexate, cyclophosphamide or cyclosporine A, with overall response rates of 30-60%.6,11 Patients who fail IST may receive other agents such as alemtuzumab, ATG, and more recently ruxolitinib,5,12,13 but no curative therapy exists outside of HCT for refractory LGLL. Published data on HCT in refractory LGLL are limited to case reports and very small series.14 16 The largest experience to date is an EBMT study of 10 T-LGLL patients receiving various conditioning and GVHD regimens, which reported high mortality due to infection, especially in those who received prior alemtuzumab.14 Beyond this report, no registry-level analyses have been conducted, and the indications, patterns of use, and long-term outcomes of HCT in LGLL remain undefined. Although agents such as ruxolitinib, monoclonal antibodies and STAT3 degraders are under investigation in refractory LGLL, the role of HCT versus non-transplant approaches remains unclear. A CIBMTR analysis linking outcomes with biospecimens would provide the first large-scale assessment of HCT in LGLL and the first correlative investigation of somatic mutations and TCR clonality in this context. Such

Field	Response
	findings would clarify the feasibility and risks of HCT, identify biological predictors of outcome, and inform referral practices and transplant strategies for refractory LGLL.
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.	LGLL is defined by clonal lymphocyte expansion, often associated with somatic mutations in STAT3, STAT5b, and CCL22. While STAT3 mutations are most frequent, mutations in regulators of the JAK-STAT pathway have been reported, though their clinical impact is unclear. Furthermore, patients refractory to IST may progress to transfusion dependence or life-threatening cytopenias without curative options and die of infection. Allogeneic HCT has shown anecdotal success but systematic evidence is lacking. This study will use the CIBMTR registry to 1) define outcomes of HCT in the largest LGLL cohort reported to date, 2) identify clinical and potentially modifiable prognostic factors and 3) explore the contribution of somatic mutations and clonal TCR burden to transplant outcomes. Challenges include the rarity of LGLL with the potential for diagnostic misclassification, as it may present alongside conditions such as aplastic anemia, MDS, CLL, PNH or PRCA. Despite these limitations, this analysis would represent the largest contemporary dataset of LGLL patients undergoing HCT and provide much-needed evidence for guiding clinical decision-making.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion: Patients with LGLL who underwent HCT between 2001-2023. Primary LGLL or LGLL associated with other hematologic disorders (e.g. PRCA, SAA, PNH, MDS, B-cell lymphomas, multiple lymphoma) will be included. Exclusion: Patients with post-HCT LGL Biologic sample availability is not required for inclusion; patients with available samples in the NMDP Biobank will undergo correlative testing. Outcomes: Primary: overall survival (OS) at 1-year post HCT Secondary: GVHD (acute GVHD grade 2-4, chronic GVHD at 1, 2, and 5 years post-HCT), graft failure, engraftment of neutrophils and platelets, treatment-related mortality (TRM), Event-free Survival (EFS), Relapse, OS (100 days, 2 years), transfusion-independence (8 weeks without RBC transfusion), and cause of death Exploratory: association of somatic mutations and clonal TCR burden with OS, NRM, GVHD, graft failure.

Field	Response
Does this study include pediatric patients?	Yes
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 variables: age, sex, KPS, HCT-CI Disease-specific variables: associated conditions, prior-treatment including IST, pre-HCT CBC HCT-related variables: conditioning (RIC vs MAC), GVHD prophylaxis, donor type, graft source, sex match, CMV serostatus, ABO compatibility, and year of HCT
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	None
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	None
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	For patients with pre-HCT biospecimens (peripheral blood or marrow) in the NMDP Biobank, we will perform TCR gene rearrangement analysis to assess clonal expansion and Hopeseq mutation assay. This platform includes DNA full exon sequencing of up to 523 genes and RNA fusion detection of up to 165 genes, and the methods have been described and published in detail in other diseases characterizing mutational landscapes.17 19 Institutional funds will support these assays.
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.	Given the rarity of LGLL, collaboration with EBMT or other international registries may be considered to enhance sample size and statistical power.

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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

Characteristics of US LGLL Patients with First Allo-HCT during 2008-2022[a]

Characteristic	Total
Number of patients	41
No. of centers	30
TED or RES (RF) track determined for this event, no. (%)	
TED	30 (73)
CRF (RES)	11 (27)
Patient-related	
Age, by decades, no. (%)	
Median (range)	45 (15-73)
10-19	2 (5)
20-29	8 (20)
30-39	5 (12)
40-49	10 (24)
50-59	7 (17)
60-69	8 (20)
70+	1 (2)
Sex, no. (%)	
Male	26 (63)
Female	15 (37)
Karnofsky score prior to HCT, no. (%)	
90-100%	23 (56)
< 90%	18 (44)
HCT-CI, no. (%)	
0	5 (12)
1	3 (7)
2	5 (12)
3	8 (20)
4	6 (15)
5+	14 (34)
Co-existing disease/organ impairment, no. (%)	
Arrhythmia	1 (2)
Cardiac	1 (2)
Cerebrovascular disease	1 (2)
Diabetes	5 (12)
Heart valve disease	1 (2)
Hepatic, moderate/severe	6 (15)
Infection	3 (7)

Characteristic	Total
Obesity	2 (5)
Psychiatric disturbance	3 (7)
Pulmonary, moderate	2 (5)
Pulmonary, severe	7 (17)
Renal, moderate/severe	1 (2)
Rheumatologic	2 (5)
Prior malignancy	1 (2)
Not reported	5 (12)
Disease-related	
NHL pre-HCT disease stage, no. (%)	
CR1	7 (17)
CR2	2 (5)
Advanced	31 (76)
Not reported	1 (2)
Transplant-related	
Interval from diagnosis to HCT, months	
Mean (SD)	44.9 (38.57)
Median (25-75 percentile)	38.9 (13.2-57.7)
Range	3.3-167.6
Donor type, no. (%)	
HLA identical sibling	10 (24)
Haploidentical donor	7 (17)
Well-matched unrelated (8/8)	15 (37)
Partially-matched unrelated (7/8)	2 (5)
Mismatched unrelated (<= 6/8)	1 (2)
Multi-donor	1 (2)
Unrelated (matching cannot be determined)	1 (2)
Cord blood	4 (10)
Donor/recipient ABO match, no. (%)	
Matched	7 (17)
Minor mismatch	2 (5)
Major mismatch	3 (7)
Bi-directional	2 (5)
CB - recipient A	1 (2)
CB - recipient O	3 (7)
Not reported	23 (56)
Donor/recipient CMV serostatus, no. (%)	
+/+	12 (29)

Characteristic	Total
+/-	2 (5)
-/+	11 (27)
-/-	12 (29)
CB - recipient +	3 (7)
CB - recipient -	1 (2)
Donor/recipient sex match, no. (%)	
M-M	17 (41)
M-F	9 (22)
F-M	6 (15)
F-F	5 (12)
CB - recipient M	3 (7)
CB - recipient F	1 (2)
Product type, no. (%)	
BM	7 (17)
PBSC	30 (73)
UCB	4 (10)
Conditioning regimen intensity, no. (%)	
MAC	9 (22)
RIC	16 (39)
NMA	13 (32)
Under review	3 (7)
Conditioning regimen, no. (%)	
TBI/Cy	2 (5)
TBI/Cy/Flu	10 (24)
TBI/Cy/VP	1 (2)
TBI/VP	1 (2)
TBI/Mel	1 (2)
TBI/Flu	5 (12)
Flu/Bu/TT	2 (5)
Flu/Bu	7 (17)
Flu/Mel	9 (22)
BEAM	1 (2)
TLI	1 (2)
Other(s)	1 (2)
GVHD prophylaxis, no. (%)	
Ex-vivo T-cell depletion	1 (2)
CD34 selection	1 (2)
PtCy + other(s)	10 (24)

Characteristic	Total
TAC + MMF +- other(s) (except PtCy)	9 (22)
TAC + MTX +- other(s) (except MMF, PtCy)	12 (29)
TAC + other(s) (except MMF, MTX, PtCy)	2 (5)
TAC alone	2 (5)
CSA + MMF +- other(s) (except PtCy,TAC)	4 (10)
Year of current transplant, no. (%)	
2009	1 (2)
2010	2 (5)
2011	1 (2)
2012	4 (10)
2013	5 (12)
2014	2 (5)
2015	2 (5)
2016	4 (10)
2017	4 (10)
2018	4 (10)
2019	4 (10)
2020	3 (7)
2021	2 (5)
2022	3 (7)
Follow-up of survivors, median (range), months	96.9 (29.2-169.8)

[a] No STAT3 information available

Field	Response
Proposal Number	2509-234-HAMID
Proposal Title	Outcomes of Allogenic HSCT for therapy related myeloid neoplasms arising following treatment with CAR T cell therapy.
Key Words	ACR T , Allogenic HSCT, Therapy related Myeloid neoplasm, Post CAR T myeloid neoplasm.
Principal Investigator #1: - First and last name, degree(s)	Showkat Hamid
Principal Investigator #1: - Email address	showkat.hamid@moffitt.org
Principal Investigator #1: - Institution name	Moffitt Cancer Center
Principal Investigator #1: - Academic rank	BMT-CI Fellow
Junior investigator status (defined as 玆、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Principal Investigator #2 (If applicable): - First and last name, degree(s):	Dr. Rawan Faramand
Principal Investigator #2 (If applicable): - Email address:)	rawan.faramand@moffitt.org
Principal Investigator #2 (If applicable): - Institution name:	Moffitt Cancer Center
Principal Investigator #2 (If applicable): - Academic rank:	Associate Professor
Junior investigator status (defined as 玆、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:	Dr. Rawan Faramand MD
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	SC2301. Incidence, risk factors, and characteristics of subsequent neoplasms in CAR-T recipients and its impact on survival.
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	Yes
PROPOSED WORKING COMMITTEE:	Leukemia
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	What are the outcomes of allogeneic transplant for patients who have developed secondary myeloid neoplasms after receiving CAR T cell therapy ?

Field	Response
RESEARCH HYPOTHESIS:	Patients undergoing allogenic stem cell transplantation following CAR-T therapy experience inferior overall survival, primarily driven by increased non-relapse mortality and adverse genomic features.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary: Estimate 2-year overall survival (OS) after Allo-HSCT for therapy related myeloid neoplasms post CAR T cell therapy (t-MN post CAR) arising following treatment with CAR-T cell therapy. Secondary: Estimate 2-year cumulative incidence of TRM after Allogenic HSCT following CAR T cell therapy. Estimate cumulative incidence of grade II IV aGVHD and moderate to severe cGVHD and cumulative incidence of relapse of t-MN post CAR in patients undergoing allo HSCT after CAR T cell therapy. Estimate 2-year leukemia free survival (LFS) l and GVHD free survival (GRFS) in patients undergoing Allogenic transplant for t-MN post CAR. Estimate cumulative incidence of relapse of primary malignancy for which CAR T cell therapy was indicated. Estimate the time to engraftment (days) and estimate rates of graft failure. Examine the impact of cytogenetics and molecular risk features on OS, LFS and TRM Evaluate the effect of the target antigen CAR T/ product used on outcomes of Allo HSCT

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.	The rapid growth of CAR T-cell therapy has created a new and expanding cohort of survivors who remain at risk for developing t-MN. For patients who develop these high-risk secondary malignancies, allo-HSCT remains the only potentially curative therapy. EBMT recently reported that in t-MN following treatment for multiple myeloma overall survival (OS) and progression-free survival (PFS) estimates after allo-HSCT at 1 and 5 years were 55% (95% CI: 47-63%) and 27% (95% CI: 19-35%) and 45% (95% CI 36-53%) and 24% (95% CI 16-32%). For lymphoma cohort, 5-year OS, and t-MN PFS, relapse incidence and NRM were 32%, 28%, 35% and 37%, respectively. Yet, outcomes of allo-HCST in this population of patients following CAR T are essentially undefined existing literature is limited to anecdotal reports and small single-center experiences, with no systematic assessment of transplant-specific metrics such as engraftment, graft failure, graft-versus-host disease, relapse, and non-relapse mortality. Robust evaluation of these endpoints requires large-scale, harmonized datasets that can capture both pre CAR T exposures and transplant-related variables. The CIBMTR represents the only resource capable of addressing this critical gap, with detailed longitudinal data on transplant related variables and outcomes across centers. Leveraging CIBMTR data is therefore essential to characterize the risks and benefits of allo-HSCT for post CAR T t-MN, inform clinical decision-making, and guide the design of future interventional strategies as the development of prospective trials that standardize conditioning, and GVHD prophylaxis and maintenance strategies in this growing population.

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.	CAR T cell therapy has transformed the treatment paradigm for hematologic malignancies leading to FDA approval of several products. While early studies highlighted acute toxicities such as cytokine release syndrome (CRS), immune effector cell-associated neurotoxicity syndrome (ICANS) longer-term follow-up has brought attention to the emergence of secondary neoplasms (SN) as an area of concern. Cordeiro et al. (Transplant and Cellular Therapy, 2020) reported a 5% myelodysplastic syndrome (MDS), and 1% multiple myeloma (MM) along with other secondary malignancies. In a separate cohort of 189 patients with R/R non-Hodgkin lymphoma (NHL) treated with commercial CAR-T therapy, Alkhateeb et al. (Blood Cancer Journal, 2022) reported that 10 patients (5.3%) developed myeloid neoplasms post cytotoxic therapy (MN-pCT). The median time to therapy-related myeloid neoplasm (t-MN) onset was 9.1 months, with 60% occurring within the first year. At diagnosis, 40% had complex karyotypes and TP53 mutations. These findings highlight a concerning incidence of secondary and therapy-related myeloid neoplasms following CAR-T therapy, often marked by early onset and adverse genomic features. However, despite increased understanding of the risk of t-MN following CAR T cell therapy, the optimal treatment strategy remains undefined and largely extrapolated from t-MN who are CAR T cell therapy naive. This knowledge gap is particularly critical for allogeneic transplantation the only curative option where outcomes are unknown and transplant-specific risks may be magnified by prior CAR T therapy. A comprehensive understanding of transplant outcomes in this setting is essential to inform clinical decision-making and to guide the development of future strategies aimed at improving survival. Center for International Blood and Marrow Transplant Research (CIBMTR) analyzed 1531 allo-HCT for adults with t-MDS (n = 759) or t-AML (n = 772) performed from 2000 to 2014. t-AML patients tend to be older, have more comorbidities, and often present with adverse cytogenetics due to prior chemo/radiation exposure. De novo AML patients, especially those transplanted in CR1, show significantly better long-term 5year OS (25 vs 40-45%) and lower relapse rates (23 vs 30-35%). Disease status at transplant (CR1 vs. relapse/PIF) is a major determinant of outcome in both groups, but especially critical in t-AML, where 5-year DFS drops to 8% in non-CR1 settings.

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	All patients who underwent allogenic HSCT for t-MN after receiving CAR T cell therapy for the lymphoma or multiple myeloma. Patients who had prior allogenic HSCT or those who received investigational CAR T products will be excluded.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	Very low incidence of t-MN post CAR in pediatric population.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)

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Field	Response
	severe neurotoxicity in axicabtagene ciloleucel therapy of large B-cell lymphoma." Blood Cancer Discov. 7. Shouse, G. P., T. Xue, A. Herrera, T. Siddiqi, J. Zain, L. Popplewell, S. Forman and E. Budde (2019). "Mds as a Cause for Prolonged Hematologic Toxicity after Treatment with Cd19 Targeted Car-T Cell Therapy in Patients with Relapsed Refractory Lymphoma." Hematological Oncology 37(S2): 507-508. 8. (Shouse, Xue et al. 2019, Cordeiro, Bezerra et al. 2020, Alkhateeb, Mohty et al. 2022, Martin, Usmani et al. 2022, Saini, Swoboda et al. 2022, Rejeski, Subklewe et al. 2023, Gurney, Baranwal et al. 2024) 9. Raj K, Eikema DJ, Lawless S, Koster L, Kunadt D, Kr ger N, Platzbecker U, Stelljes M, Bethge W, Holderried T, Fanin R, Zeiser R, Kuball J, Leblond V, Nicholson E, Passweg J, Potter V, Bay JO, Bazarbachi A, Corral LL, Gurnari C, Scheid C, Drozd-Sokolowska J, Morris TC, Hayden P, Yakoub-Agha I, Robin M, McLornan DP. Allogeneic hematopoietic cell transplantation for therapy-related myeloid neoplasms arising following treatment for multiple myeloma: a retrospective study on behalf of the Chronic Malignancies Working Party of the EBMT. Bone Marrow Transplant. 2025 Feb;60(2):220-226. doi: 10.1038/s41409-024-02462-5. Epub 2024 Nov 12. PMID: 39533014. 10. Nabergoj, M., Eikema, DJ., Koster, L. et al. Allogeneic haematopoietic cell transplantation for therapy-related myeloid neoplasms arising following treatment for lymphoma: a retrospective study on behalf of the Chronic Malignancies Working Party of the EBMT. Bone Marrow Transplant 59, 395 402 (2024). https://doi.org/10.1038/s41409-023-02193-z
CONFLICTS OF INTEREST: Do you have any conflicts of interest pertinent to this proposal concerning?	Yes, I have 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.	Dr. Faramand serves as an advisor to Sanofi, Kite/Gilead and Autlous. She receives institutional research funding from Novartis

Table 1. Characteristics of Adult US Patients with first Allo-HCT for therapy related myeloid neoplasm during 2008-2022 after Car-T for MM or Lymphoma

Characteristic	Total
Number of patients	30
TED or RES (RF) track determined for this event, no. (%)	
TED	25 (83)
CRF (RES)	5 (17)
Patient-related	
Age, by decades, no. (%)	
Median (range)	63 (33-77)
30-39	1 (3)
40-49	1 (3)
50-59	9 (30)
60-69	16 (53)
70+	3 (10)
Sex, no. (%)	
Male	15 (50)
Female	15 (50)
Race, no. (%)	
White	27 (90)
Black or African American	1 (3)
Not reported	2 (7)
Karnofsky score prior to HCT, no. (%)	
90-100%	7 (23)
< 90%	22 (73)
Not reported	1 (3)
HCT-CI, no. (%)	
2	1 (3)
3	3 (10)
4	5 (17)
5+	21 (70)
Disease-related	
Primary disease, no. (%)	
AML	3 (10)
MDS	26 (87)
MPN	1 (3)
MDS IPSS-R prognostic risk category / score at HCT, no. (%)	
Not MDS	4 (13)
Low	4 (13)

Characteristic	Total
Intermediate	4 (13)
High	6 (20)
Very high	8 (27)
Not reported	4 (13)
ELN 2022 (AML), no. (%)	
Not AML	27 (90)
Intermediate	1 (3)
Poor	2 (7)
MDS pre-HCT disease stage, no. (%)	
Disease is not MDS/MPN	3 (10)
Advanced	27 (90)
AML pre-HCT disease stage, no. (%)	
Disease is not AML	27 (90)
CR1	3 (10)
Transplant related	
Interval from diagnosis to HCT, months	
Mean (SD)	4.6 (2.04)
Median (25-75 percentile)	4.4 (3.6-5.9)
Range	0.3-9.6
Donor type, no. (%)	
HLA identical sibling	7 (23)
Haploidentical donor	2 (7)
Well-matched unrelated (8/8)	16 (53)
Partially-matched unrelated (7/8)	3 (10)
Unrelated (matching cannot be determined)	2 (7)
Product type, no. (%)	
BM	1 (3)
PBSC	29 (97)
Conditioning regimen intensity, no. (%)	
MAC	4 (13)
RIC	22 (73)
NMA	4 (13)
Conditioning regimen, no. (%)	
TBI/Cy/Flu	6 (20)
TBI/Mel	1 (3)
TBI/Flu	1 (3)
Bu/Cy	1 (3)
Flu/Bu/TT	2 (7)

Characteristic	Total
Flu/Bu	9 (30)
Flu/Mel	10 (33)
GVHD prophylaxis, no. (%)	
PtCy + other(s)	12 (40)
TAC + MMF +- other(s) (except PtCy)	1 (3)
TAC + MTX +- other(s) (except MMF, PtCy)	12 (40)
TAC + other(s) (except MMF, MTX, PtCy)	3 (10)
TAC alone	1 (3)
Other(s)	1 (3)
Year of current transplant, no. (%)	
2019	1 (3)
2020	6 (20)
2021	5 (17)
2022	18 (60)
Follow-up of survivors, median (range), months	31.3 (17.0-53.0)