Not for publication or presentation

A G E N D A
CIBMTR WORKING COMMITTEE FOR PEDIATRIC CANCER WORKING COMMITTEE
Salt Lake City, UT
Saturday, February 7, 2026, 1:00 – 3:00 PM (MT)

Co-Chair:	**Hemalatha Rangarajan, MD; Nationwide Children's Hospital, Columbus, OH; Telephone: 614-355-1689; Email: Hemalatha.rangarajan@nationwidechildrens.org**
Co-Chair:	**Akshay Sharma, MBBS; St. Jude Children's Research Hospital, Memphis, TN; Telephone: 901-595-2238; E-mail: Akshay.sharma@stjude.org**
Co-Chair:	**Parinda Mehta, MD; Cincinnati Children's Hospital, Cincinnati, OH; Telephone: 513-636-5917; E-mail: Parinda.mehta@cchmc.org**
Co-Chair:	**Christine Phillips, MD; Cincinnati Children's Hospital, Cincinnati, OH; Telephone: 513- 803-3216; E-mail: christine.phillips@cchmc.org**
Scientific Director:	**Larisa Broglie, MD, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-4268; Email: lbroglie@mcw.edu**
Statistical Director:	**Kwang Woo Ahn, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414- 456-7387; Email: kwooahn@mcw.edu**
Statistician:	**Sarthak Kumar, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-3756; E-mail: sarkumar@mcw.edu**
Page Scholar:	**Takuto Takahashi, MD, PhD; University of Minnesota, Minneapolis, MN; E-mail: takah033@umn.edu**

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1. **Introduction**
   a. Minutes from February 2025 (**Attachment 1**)

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

3. **Presentations, Publications or Submitted papers**
   a. **PC19-03** Rangarajan HG, Chellapandian D, Atshan R, Ahn KW, Kumar S, Knight TE, Leung W, Ganguly S, Williams KM, Shah NN, Bhatt NS, Lust H, Prestidge T, Brown VI, Hayashi RJ, Choe M, Saad A, Bidgoli A, Thakar MS, Wirk B, MacMillan ML, Lalefar NR, Hematti P, Schultz KR, Phillips CL, Mehta PA, Qayed M, Sharma A, Broglie L, Satwani P. Impact of extramedullary disease at diagnosis on outcomes post allogeneic hematopoietic cell transplant in children and young adults with acute myeloid leukemia: A CIBMTR report. **Transplantation and Cellular Therapy. doi:10.1016/j.jtct.2025.12.990. Epub 2025 Dec 28.**
   b. **AC17-01** CD-19 chimeric antigen receptor T-cells with or without hematopoietic cell transplantation for treatment of refractory acute lymphocytic leukemia (M Perales/ J Park/ S Nikiforow). ***Submitted.***

c. **PC22-01** Impact of Graft Versus Host Disease Following Allogeneic Hematopoietic Cell Transplantation on Leukemia Free Survival in Hematologic Malignancies: A CIBMTR Analysis (M Qayed/ A Bauchat). ***Oral Presentation, Tandem Meetings 2025.***

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

a. **PC19-02** Does mixed peripheral blood T Cell Chimerism predict relapse? (S Prockop/ J Boelens/K Peggs). **Demographic Table Preparation.**
b. **CT20-02** Resource utilization with chimeric antigen receptor T cells (M Battiwalla/ H Rangarajan/ C Scheckel). **Protocol Development.**
c. **PC20-02** Germline genetics of pediatric Myelodysplastic Syndromes (J Poynter/ L Spector). **Analysis.**
d. **PC22-01** Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). **Manuscript Preparation.**
e. **PC22-02** Evaluating predictors of access and outcomes with hematopoietic cell transplantation in pediatric and adolescent patients with relapsed/refractory classical Hodgkin lymphoma after treatment on an initial cooperative group clinical trial (S Castellino/ J Kahn). **Protocol Development.**
f. **PC23-01** post-transplant cyclophosphamide vs. TCR αβ/CD19+ deplete approaches for haploidentical transplant in pediatric patients with acute leukemias and myelodysplastic syndrome: A CIBMTR/EBMT collaborative study (A Li/ H Rangarajan/ P Satwani). **Analysis**
g. **PC23-02** Comparison of bone marrow and peripheral blood stem cells as graft source in children undergoing allogeneic hematopoietic stem cell transplantation for hematological malignancies with unmanipulated haploidentical grafts utilizing post-transplant cyclophosphamide as GvHD prophylaxis (A Srinivasan/ J Krueger). **Abstract Submitted to EBMT 2025, Manuscript in Preparation.**
h. **PC24-01** Transplantation and cellular therapy for children and young adults with down's syndrome and acute leukemia (L Appell/ S Rotz). **Protocol Development.**
i. **PC25-01** Impact of Planned Post-Transplant Granulocyte Colony Stimulating Factor (G-CSF) on Transplant-Related Outcomes in Pediatric Patients with Malignant Disease Undergoing Haploidentical Hematopoietic Cell Transplant (HCT) with Post-Transplant Cyclophosphamide (ptCy) for Graft vs. Host Disease (GVHD) Prophylaxis (L Davis/ P Satwani). **Protocol Development.**

5. Future/proposed studies

a. **PROP 2411-01** The Role of Maintenance Tyrosine Kinase Inhibitors Following Allogenic Hematopoietic Stem Cell Transplant for Pediatric Patients with Chronic Myelogenous Leukemia (R Hans/ A Harris) (**Attachment 4**)
b. **PROP 2508-09** Outcomes Following Post Transplant Tyrosine Kinase Inhibitor (TKI) Maintenance Therapy in Pediatric Patients with Ph+ Acute Lymphoblastic Leukemia (R Chakravarthy) (**Attachment 5**)
c. **PROP 2509-185** Identifying the Clinical Impact of Germline Variants in Pediatric AML Requiring Hematopoietic Stem Cell Transplantation (S Zarnegar-Lumley/ J Pollard) (**Attachment 6**)

Proposed studies; not accepted for consideration at this time

d. **PROP 2509-05** Optimal Donor Characteristics for Pediatric Allogeneic Hematopoietic Cell Transplantation Using Post-Transplant Cyclophosphamide (L Alkhouli). ***Dropped due to overlap with current study/publication.***
e. **PROP 2509-06** Effectiveness of Donor Lymphocyte Infusion in Children: A CIBMTR Analysis of Malignant and Non-Malignant Diseases (L Alkhouli). ***Dropped due to need of supplemental data.***
f. **PROP 2509-145** Comparing Progression-Free Survival and Overall Survival of Allogeneic Stem Cell Transplantation versus 2CDA and ARA-C in Relapsed/Refractory Langerhans Cell Histiocytosis (M Pamukcuoglu). ***Dropped due to small sample size.***
g. **PROP 2509-233** Outcomes of children and adolescents undergoing CAR-T, autologous, or allogeneic hematopoietic stem cell transplantation for first relapse or refractory non-Hodgkin lymphoma (J Belsky/ R Hayashi/ S Alexander). ***Dropped due to small sample size.***

6. Other business

MINUTES
CIBMTR WORKING COMMITTEE FOR PEDIATRIC CANCER WORKING COMMITTEE
Honolulu, HI
Saturday, February 15, 2025, 1:00 – 3:00 PM

Co-Chair:	**Kirk R. Schultz, MD; The University of British Columbia, Vancouver, BC, Canada; Telephone: 604-875-2322; Email: kschultz@mail.ubc.ca**
Co-Chair:	**Akshay Sharma, MBBS; St. Jude Children's Research Hospital, Memphis, TN; Telephone: 901-595-2238; E-mail: Akshay.sharma@stjude.org**
Co-Chair:	**Parinda Mehta, MD; Cincinnati Children's Hospital, Cincinnati, OH; Telephone: 513-636-5917; E-mail: Parinda.mehta@cchmc.org**
Co-Chair:	**Christine Phillips, MD; Cincinnati Children's Hospital, Cincinnati, OH; Telephone: 513- 803-3216; E-mail: christine.phillips@cchmc.org**
Scientific Director:	**Larisa Broglie, MD, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-4108; Email: lbroglie@mcw.edu**
Statistical Director:	**Zhongyuan Chen, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Email: zhchen@mcw.edu**
Statistical Director:	**Kwang Woo Ahn, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414- 456-7387; Email: kwooahn@mcw.edu**
Statistician:	**Sarthak Kumar, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-805-0163; E-mail: sarkumar@mcw.edu**

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   b. **AC17-01** CD-19 chimeric antigen receptor T-cells with or without hematopoietic cell transplantation for treatment of refractory acute lymphocytic leukemia (M Perales/ J Park/ S Nikiforow). ***Submitted.***

c. **PC22-01** Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). ***Oral Presentation, Tandem 2025***

4. Studies in progress (Attachment 3)

a. **PC19-02** Does mixed peripheral blood T Cell Chimerism predict relapse? (S Prockop/ J Boelens/ K Peggs). **Protocol Development**

b. **PC19-03** The impact of pre-transplant extramedullary disease on the outcome of Allogeneic Hematopoietic Cell Transplantation for Acute Myeloid Leukemia in children (H Rangarajan/ P Satwani /D Chellapandian). **Manuscript Preparation.**

c. **CT20-02** Resource utilization with chimeric antigen receptor T cells (M Battiwalla/ H Rangarajan/ C Scheckel). **Protocol Development.**

d. **PC20-02** Germline genetics of pediatric Myelodysplastic Syndromes (J Poynter/ L Spector). **Data File Preparation.**

e. **PC22-01** Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). **Manuscript Preparation**

f. **PC22-02** Evaluating predictors of access and outcomes with hematopoietic cell transplantation in pediatric and adolescent patients with relapsed/refractory classical Hodgkin lymphoma after treatment on an initial cooperative group clinical trial (S Castellino/ J Kahn). **Protocol Development.**

g. **PC23-01** Post-transplant cyclophosphamide vs. TCR αβ/CD19+ deplete approaches for haploidentical transplant in pediatric patients with acute leukemias and myelodysplastic syndrome: A CIBMTR/EBMT collaborative study (A Li/ H Rangarajan/ P Satwani). **Data File Preparation.**

h. **PC23-02** Comparison of bone marrow and peripheral blood stem cells as graft source in children undergoing allogeneic hematopoietic stem cell transplantation for hematological malignancies with unmanipulated haploidentical grafts utilizing post-transplant cyclophosphamide as GvHD prophylaxis (A Srinivasan/ J Krueger). **Protocol Development**

i. **PC24-01** Transplantation and cellular therapy for children and young adults with down's syndrome and acute leukemia (L Appell/ S Rotz). **Protocol development.**

5. Future/proposed studies

a. **PROP 2410-40** Comparison of different TBI doses in relation to MRD status in pediatric acute lymphoblastic leukemia (T Takahashi/ A Keating) (Attachment 4)

- *Presented by Dr. Takato Takahashi.*
- ***Hypothesis:*** *TBI at 12Gy has comparable disease-free survival to higher TBI doses.*
- ***Objectives:*** *Compare disease-free survival, overall survival, TRM, relapse, GVHD, and late effects between high dose TBI (>12Gy) to standard myeloablative TBI (12Gy).*

b. **PROP 2410-85** Is There an Optimal CD34+ Cell Dose In Pediatric Allogeneic Hematopoietic Cell Transplantation Performed for Malignant Diseases? (E Fraint/ T Knight) (Attachment 5)

- *Presented by Dr. Ellen Fraint.*
- ***Hypothesis:*** *Higher CD34+ and TNC doses improve survival, reduce relapse rate, and accelerate engraftment.*
- ***Objectives****: Determine optimal CD34+ and TNC doses for pediatric patients undergoing allotransplant.*

c. **PROP 2410-94** Effect of disease burden and pre-transplant therapy in pediatric patients with myelodysplastic syndrome in the current era (J Rossoff/ S Chaudhury) (Attachment 6)

- *Presented by Dr. Sonali Chaudhury.*
- ***Hypothesis:*** *Lower blast percentage pre-transplant improves disease-free survival for patients receiving HCT for MDS*
- ***Objectives****: Determine the effect of pre-transplant bone marrow blast percentage on outcomes.*

d. **PROP 2410-176** Comparison of Risk Factors Associated with Early and Late Disease Relapse Among Patients in Complete Remission at One Month after Tisagenlecleucel (Kymriah) therapy in Pediatric, Adolescent and Young Adult (AYA) Patients Treated for Relapsed or Refractory (r/r) B Cell Acute Lymphoblastic Leukemia (B Cell ALL) (L Davis/ P Satwani) (Attachment 7)

- *Presented by Dr. Laurie Davis.*
- ***Hypothesis:*** *Patients with high disease burden at D28 will experience higher rates of early and late relapse after Kymriah infusion.*
- ***Objectives:*** *Identify risk factors influencing relapse in pediatric and AYA patients treated for relapsed/refractory B-cell ALL who achieved CR by D28 post-CART.*

e. **PROP 2410-182** Impact of Planned Post-Transplant Granulocyte Colony Stimulating Factor (G-CSF) on Transplant-Related Outcomes in Pediatric Patients with Malignant Disease Undergoing Haploidentical Hematopoietic Cell Transplant (HCT) with Post-Transplant Cyclophosphamide (ptCy) for Graft vs. Host Disease (GVHD) Prophylaxis (L Davis/ P Satwani) (Attachment 8)

- *Presented by Dr. Laurie Davis.*
- ***Hypothesis****: Planned GCSF administration will result in lower overall survival and disease-free survival, with greater relapse and non-relapse mortality in patients receiving haploidentical HCT with PTCy.*
- ***Objectives:*** *Analyze hematopoietic recovery, overall survival, GVHD, and major transplant complications, comparing those who received GCSF to those who did not.*

f. **PROP 2410-200** Hematopoietic Stem Cell Transplant Outcomes for Infant B-cell Acute Lymphoblastic Leukemia (N Lalefar/ H Rangarajan) (Attachment 9)

- *Presented by Dr. Nahal Rose Lalefar.*
- ***Hypothesis:*** *Improved outcomes for infant ALL following HCT in the contemporary era.*
- ***Objectives:*** *Compare leukemia-free survival (LFS) and overall survival (OS) at one year and three years post-transplant, following trends across time periods 2003-2022 and , analyze treatment-related mortality, and explore late effects.*

g. **PROP 2410-204** Transplantation Outcomes for Children with Hypodiploid Acute Lymphoblastic Leukemia in the Modern Era (A Bidgoli/ U Kapoor) (Attachment 10)

- *Presented by Dr. Urvi Kapoor.*
- ***Hypothesis:*** *Transplantation for pediatric hypodiploid ALL, when performed in the setting of disease control, offers outcomes comparable to other ALL transplants.*
- ***Objectives:*** *Evaluate leukemia-free survival, relapse, non-relapse mortality, and overall survival.*

Proposed studies; not accepted for consideration at this time

h. **PROP 2408-14** Comparing the Progression-free Survival and Overall Survival of Autologous Stem Cell Transplantation and Allogeneic Stem Cell Transplantation in Refractory Langerhans Cell Histiocytosis (M Pamukcuoglu). ***Dropped due to small sample size.***
i. **PROP 2410-73** The impact of prior allogeneic HSCT on outcomes following subsequent CD**19**.CAR-T cell infusion for pediatric patients with relapsed/refractory B-cell ALL (S Naik/ M Pulsipher). ***Dropped due to overlap with current study/publication.***
j. **PROP 2410-136** Comparison of alternative donor options in pediatric AML with varying residual disease status (T Takahashi/ A Keating). ***Dropped due to overlap with published study***
k. **PROP 2410-207** The Impact of Hematopoietic Cell Transplantation in Complete Remission with Incomplete Count Recovery in Pediatric AML (E Krieger/ K Magee). ***Dropped due to supplemental data needed.***
l. **PROP 2410-211** Impact of KYMRIAH potency on incidence of relapse and cytokine release syndrome (U Kapoor/ P Satwani). ***Dropped due to supplemental data needed.***

6. Other business

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	4700	1525	1743
Source of data			
CRF	2769 (59)	730 (48)	1039 (60)
TED	1931 (41)	795 (52)	704 (40)
Number of centers	163	124	202
Disease at transplant			
AML	1417 (30)	524 (34)	531 (30)
ALL	2037 (43)	614 (40)	768 (44)
Other leukemia	30 (1)	5 (<1)	10 (1)
CML	276 (6)	96 (6)	133 (8)
MDS	580 (12)	166 (11)	213 (12)
Other acute leukemia	118 (3)	50 (3)	26 (1)
NHL	182 (4)	49 (3)	41 (2)
Hodgkin Lymphoma	46 (1)	9 (1)	15 (1)
MPN	14 (<1)	12 (1)	6 (<1)
AML Disease status at transplant			
CR1	627 (44)	258 (49)	218 (41)
CR2	455 (32)	154 (29)	136 (26)
CR3+	34 (2)	11 (2)	16 (3)
Advanced or active disease	279 (20)	96 (18)	136 (26)
Missing	22 (2)	5 (1)	25 (5)
ALL Disease status at transplant			
CR1	615 (30)	174 (28)	193 (25)
CR2	873 (43)	292 (48)	314 (41)
CR3+	338 (17)	98 (16)	125 (16)
Advanced or active disease	175 (9)	43 (7)	74 (10)
Missing	36 (2)	7 (1)	62 (8)
MDS Disease status at transplant			
Early	186 (32)	41 (25)	37 (17)
Advanced	185 (32)	75 (45)	60 (28)
Missing	209 (36)	50 (30)	116 (54)
NHL Disease status at transplant			
CR1	33 (18)	12 (24)	14 (34)
CR2	50 (27)	23 (47)	10 (24)
CR3+	19 (10)	2 (4)	1 (2)
PR	14 (8)	2 (4)	1 (2)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Advanced	62 (34)	10 (20)	8 (20)
Missing	4 (2)	0	7 (17)
Recipient age at transplant			
0-9 years	2258 (48)	724 (47)	835 (48)
10-17 years	2442 (52)	801 (53)	908 (52)
Median (Range)	10 (0-18)	11 (0-18)	10 (0-18)
Recipient race			
White	3707 (86)	1200 (85)	1218 (82)
Black or African American	340 (8)	97 (7)	140 (9)
Asian	151 (3)	58 (4)	80 (5)
Native Hawaiian or other Pacific Islander	12 (<1)	2 (<1)	12 (1)
American Indian or Alaska Native	34 (1)	15 (1)	10 (1)
Other	17 (<1)	10 (1)	8 (1)
More than one race	73 (2)	31 (2)	22 (1)
Unknown	366 (N/A)	112 (N/A)	253 (N/A)
Recipient ethnicity			
Hispanic or Latino	881 (25)	250 (21)	302 (25)
Non Hispanic or non-Latino	2524 (71)	875 (74)	582 (48)
Non-resident of the U.S.	145 (4)	53 (4)	340 (28)
Unknown	1150 (N/A)	347 (N/A)	519 (N/A)
Recipient sex			
Male	2771 (59)	918 (60)	1022 (59)
Female	1929 (41)	607 (40)	721 (41)
Karnofsky score			
10-80	727 (15)	269 (18)	306 (18)
90-100	3791 (81)	1197 (78)	1331 (76)
Missing	182 (4)	59 (4)	106 (6)
HLA-A B DRB1 groups - low resolution			
<=3/6	3 (<1)	4 (<1)	1 (<1)
4/6	65 (1)	9 (1)	6 (<1)
5/6	1041 (22)	280 (20)	349 (22)
6/6	3540 (76)	1126 (79)	1235 (78)
Unknown	51 (N/A)	106 (N/A)	152 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	216 (5)	8 (1)	26 (3)
6/8	383 (8)	34 (3)	54 (5)
7/8	1237 (27)	246 (25)	308 (31)
8/8	2759 (60)	712 (71)	611 (61)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Unknown	105 (N/A)	525 (N/A)	744 (N/A)
HLA-DPB1 Match			
Double allele mismatch	1283 (30)	163 (26)	159 (27)
Single allele mismatch	2244 (53)	327 (52)	322 (54)
Full allele matched	693 (16)	134 (21)	116 (19)
Unknown	480 (N/A)	901 (N/A)	1146 (N/A)
High resolution release score			
No	875 (19)	1516 (99)	1581 (91)
Yes	3825 (81)	9 (1)	162 (9)
KIR typing available			
No	3548 (75)	1523 (>99)	1727 (99)
Yes	1152 (25)	2 (<1)	16 (1)
Graft type			
Marrow	3728 (79)	1215 (80)	1321 (76)
PBSC	967 (21)	298 (20)	419 (24)
BM+PBSC	3 (<1)	3 (<1)	1 (<1)
PBSC+UCB	0	5 (<1)	1 (<1)
Others	2 (<1)	4 (<1)	1 (<1)
Conditioning regimen			
Myeloablative	4355 (93)	1437 (94)	1616 (93)
RIC/Nonmyeloablative	320 (7)	84 (6)	102 (6)
TBD	25 (1)	4 (<1)	25 (1)
Donor age at donation			
To Be Determined/NA	15 (<1)	36 (2)	17 (1)
0-9 years	2 (<1)	2 (<1)	0
10-17 years	1 (<1)	0	2 (<1)
18-29 years	2103 (45)	742 (49)	695 (40)
30-39 years	1458 (31)	475 (31)	585 (34)
40-49 years	918 (20)	218 (14)	346 (20)
50+ years	203 (4)	52 (3)	98 (6)
Median (Range)	31 (3-61)	30 (4-61)	32 (17-61)
Donor/Recipient CMV serostatus			
+/+	1063 (23)	448 (29)	380 (22)
+/-	762 (16)	196 (13)	296 (17)
-/+	1301 (28)	387 (25)	447 (26)
-/-	1493 (32)	424 (28)	533 (31)
CB - recipient +	0	5 (<1)	1 (<1)
CB - recipient -	0	3 (<1)	0
CB - recipient CMV unknown	0	1 (<1)	0
Missing	81 (2)	61 (4)	86 (5)
GvHD Prophylaxis			
No GvHD Prophylaxis	13 (<1)	5 (<1)	8 (<1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
TDEPLETION alone	46 (1)	9 (1)	25 (1)
TDEPLETION +- other	286 (6)	86 (6)	138 (8)
CD34 select alone	29 (1)	12 (1)	9 (1)
CD34 select +- other	58 (1)	21 (1)	28 (2)
Cyclophosphamide alone	9 (<1)	2 (<1)	3 (<1)
Cyclophosphamide +- others	75 (2)	64 (4)	44 (3)
FK506 + MMF +- others	256 (5)	84 (6)	61 (3)
FK506 + MTX +- others(not MMF)	1404 (30)	538 (35)	305 (17)
FK506 +- others(not MMF,MTX)	98 (2)	13 (1)	18 (1)
FK506 alone	56 (1)	16 (1)	12 (1)
CSA + MMF +- others(not FK506)	237 (5)	65 (4)	65 (4)
CSA + MTX +- others(not MMF,FK506)	1643 (35)	462 (30)	779 (45)
CSA +- others(not FK506,MMF,MTX)	202 (4)	61 (4)	96 (6)
CSA alone	148 (3)	48 (3)	89 (5)
Other GVHD Prophylaxis	107 (2)	27 (2)	34 (2)
Missing	33 (1)	12 (1)	29 (2)
Donor/Recipient sex match			
Male-Male	1780 (38)	570 (37)	610 (35)
Male-Female	1075 (23)	334 (22)	372 (21)
Female-Male	983 (21)	337 (22)	405 (23)
Female-Female	848 (18)	264 (17)	342 (20)
CB - recipient M	0	3 (<1)	1 (<1)
CB - recipient F	0	6 (<1)	0
Missing	14 (<1)	11 (1)	13 (1)
Year of transplant			
1986-1990	73 (2)	9 (1)	30 (2)
1991-1995	437 (9)	107 (7)	203 (12)
1996-2000	579 (12)	212 (15)	331 (19)
2001-2005	704 (15)	153 (11)	325 (19)
2006-2010	855 (18)	154 (11)	188 (11)
2011-2015	1005 (22)	215 (15)	243 (14)
2016-2020	675 (15)	339 (23)	237 (14)
2021-2025	382 (7)	336 (18)	186 (10)
Follow-up among survivors, Months			
N Eval	2470	849	878
Median (Range)	71 (0-353)	37 (0-295)	57 (0-385)

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Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	1587	519	648
Source of data			
CRF	1135 (72)	332 (64)	342 (53)
TED	452 (28)	187 (36)	306 (47)
Number of centers	89	77	121
Disease at transplant			
AML	627 (40)	192 (37)	239 (37)
ALL	666 (42)	245 (47)	284 (44)
Other leukemia	10 (1)	2 (<1)	4 (1)
CML	20 (1)	5 (1)	9 (1)
MDS	163 (10)	46 (9)	70 (11)
Other acute leukemia	45 (3)	15 (3)	23 (4)
NHL	49 (3)	14 (3)	14 (2)
Hodgkin Lymphoma	5 (<1)	0	4 (1)
MPN	2 (<1)	0	1 (<1)
AML Disease status at transplant			
CR1	299 (48)	105 (55)	109 (46)
CR2	217 (35)	54 (28)	70 (29)
CR3+	13 (2)	0	7 (3)
Advanced or active disease	97 (15)	33 (17)	48 (20)
Missing	1 (<1)	0	5 (2)
ALL Disease status at transplant			
CR1	226 (34)	81 (33)	107 (38)
CR2	317 (48)	112 (46)	111 (39)
CR3+	99 (15)	38 (16)	48 (17)
Advanced or active disease	23 (3)	13 (5)	18 (6)
Missing	1 (<1)	1 (<1)	0
MDS Disease status at transplant			
Early	61 (37)	15 (33)	36 (51)
Advanced	57 (35)	22 (48)	18 (26)
Missing	45 (28)	9 (20)	16 (23)
NHL Disease status at transplant			
CR1	10 (20)	2 (14)	3 (21)
CR2	21 (43)	9 (64)	8 (57)
CR3+	5 (10)	1 (7)	0
PR	3 (6)	0	1 (7)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Advanced	10 (20)	2 (14)	2 (14)
Recipient age at transplant			
0-9 years	1027 (65)	358 (69)	411 (63)
10-17 years	560 (35)	161 (31)	237 (37)
Median (Range)	7 (0-18)	7 (0-18)	7 (0-18)
Recipient race			
White	1103 (74)	364 (75)	404 (72)
Black or African American	221 (15)	73 (15)	76 (14)
Asian	71 (5)	22 (5)	43 (8)
Native Hawaiian or other Pacific Islander	5 (<1)	3 (1)	11 (2)
American Indian or Alaska Native	20 (1)	5 (1)	6 (1)
Other	0	0	1 (<1)
More than one race	64 (4)	19 (4)	21 (4)
Unknown	103 (N/A)	33 (N/A)	86 (N/A)
Recipient ethnicity			
Hispanic or Latino	481 (31)	143 (28)	128 (20)
Non Hispanic or non-Latino	1058 (68)	352 (70)	332 (53)
Non-resident of the U.S.	14 (1)	10 (2)	166 (27)
Unknown	34 (N/A)	14 (N/A)	22 (N/A)
Recipient sex			
Male	925 (58)	285 (55)	367 (57)
Female	662 (42)	234 (45)	281 (43)
Karnofsky score			
10-80	255 (16)	87 (17)	103 (16)
90-100	1280 (81)	403 (78)	492 (76)
Missing	52 (3)	29 (6)	53 (8)
HLA-A B DRB1 groups - low resolution			
<=3/6	13 (1)	5 (1)	3 (<1)
4/6	469 (30)	141 (29)	166 (27)
5/6	794 (51)	245 (51)	321 (52)
6/6	280 (18)	92 (19)	130 (21)
Unknown	31 (N/A)	36 (N/A)	28 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	586 (42)	137 (37)	190 (40)
6/8	410 (29)	122 (33)	131 (28)
7/8	259 (18)	73 (20)	97 (21)
8/8	146 (10)	41 (11)	54 (11)
Unknown	186 (N/A)	146 (N/A)	176 (N/A)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
HLA-DPB1 Match			
Double allele mismatch	275 (39)	53 (35)	64 (36)
Single allele mismatch	368 (52)	87 (57)	91 (51)
Full allele matched	70 (10)	12 (8)	25 (14)
Unknown	874 (N/A)	367 (N/A)	468 (N/A)
High resolution release score			
No	1037 (65)	488 (94)	636 (98)
Yes	550 (35)	31 (6)	12 (2)
KIR typing available			
No	1147 (72)	514 (99)	638 (98)
Yes	440 (28)	5 (1)	10 (2)
Graft type			
UCB	1567 (99)	510 (98)	638 (98)
PBSC+UCB	8 (1)	5 (1)	7 (1)
Others	12 (1)	4 (1)	3 (<1)
Number of cord units			
1	1478 (93)	0	603 (93)
2	109 (7)	0	45 (7)
Unknown	0 (N/A)	519 (N/A)	0 (N/A)
Conditioning regimen			
Myeloablative	1504 (95)	489 (94)	592 (91)
RIC/Nonmyeloablative	82 (5)	30 (6)	54 (8)
TBD	1 (<1)	0	2 (<1)
Donor age at donation			
To Be Determined/NA	1284 (81)	256 (49)	555 (86)
0-9 years	287 (18)	231 (45)	88 (14)
10-17 years	9 (1)	28 (5)	3 (<1)
18-29 years	4 (<1)	0	0
30-39 years	3 (<1)	3 (1)	1 (<1)
40-49 years	0	1 (<1)	0
50+ years	0	0	1 (<1)
Median (Range)	4 (0-37)	4 (0-42)	4 (0-52)
Donor/Recipient CMV serostatus			
CB - recipient +	962 (61)	331 (64)	392 (60)
CB - recipient -	601 (38)	177 (34)	228 (35)
CB - recipient CMV unknown	24 (2)	11 (2)	28 (4)
GvHD Prophylaxis			
No GvHD Prophylaxis	5 (<1)	3 (1)	3 (<1)
TDEPLETION alone	1 (<1)	0	0
TDEPLETION +- other	6 (<1)	4 (1)	3 (<1)
CD34 select alone	0	1 (<1)	0
CD34 select +- other	6 (<1)	1 (<1)	3 (<1)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Cyclophosphamide +- others	5 (<1)	0	5 (1)
FK506 + MMF +- others	341 (21)	155 (30)	107 (17)
FK506 + MTX +- others(not MMF)	106 (7)	28 (5)	40 (6)
FK506 +- others(not MMF,MTX)	32 (2)	16 (3)	14 (2)
FK506 alone	9 (1)	7 (1)	5 (1)
CSA + MMF +- others(not FK506)	835 (53)	228 (44)	292 (45)
CSA + MTX +- others(not MMF,FK506)	49 (3)	11 (2)	23 (4)
CSA +- others(not FK506,MMF,MTX)	160 (10)	54 (10)	119 (18)
CSA alone	22 (1)	6 (1)	24 (4)
Other GVHD Prophylaxis	8 (1)	4 (1)	7 (1)
Missing	2 (<1)	1 (<1)	3 (<1)
Donor/Recipient sex match			
CB - recipient M	925 (58)	285 (55)	366 (56)
CB - recipient F	662 (42)	234 (45)	281 (43)
CB - recipient sex unknown	0	0	1 (<1)
Year of transplant			
1996-2000	0	0	2 (<1)
2001-2005	46 (3)	39 (8)	14 (2)
2006-2010	561 (36)	124 (25)	208 (33)
2011-2015	549 (35)	128 (25)	227 (36)
2016-2020	287 (18)	131 (26)	103 (16)
2021-2025	144 (8)	97 (16)	94 (12)
Follow-up among survivors, Months			
N Eval	929	322	358
Median (Range)	65 (0-196)	44 (0-213)	48 (0-186)

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

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	1456	226	99
Source of data			
CRF	287 (20)	45 (20)	16 (16)
TED	1169 (80)	181 (80)	83 (84)
Number of centers	54	43	38
Disease at transplant			
AML	501 (34)	71 (31)	38 (38)
ALL	665 (46)	114 (50)	48 (48)
Other leukemia	2 (<1)	0	0
CML	42 (3)	1 (<1)	2 (2)
MDS	117 (8)	23 (10)	9 (9)
Other acute leukemia	56 (4)	4 (2)	1 (1)
NHL	61 (4)	11 (5)	1 (1)
Hodgkin Lymphoma	9 (1)	2 (1)	0
MPN	3 (<1)	0	0
AML Disease status at transplant			
CR1	327 (65)	51 (72)	22 (58)
CR2	118 (24)	16 (23)	10 (26)
CR3+	6 (1)	1 (1)	1 (3)
Advanced or active disease	48 (10)	1 (1)	5 (13)
Missing	2 (<1)	2 (3)	0
ALL Disease status at transplant			
CR1	237 (36)	43 (38)	20 (42)
CR2	337 (51)	54 (47)	20 (42)
CR3+	78 (12)	14 (12)	6 (13)
Advanced or active disease	13 (2)	3 (3)	2 (4)
MDS Disease status at transplant			
Early	27 (23)	5 (22)	2 (22)
Advanced	70 (60)	10 (43)	4 (44)
Missing	20 (17)	8 (35)	3 (33)
NHL Disease status at transplant			
CR1	18 (30)	3 (27)	0
CR2	24 (39)	2 (18)	1 (100)
CR3+	2 (3)	0	0
Advanced	16 (26)	6 (55)	0
Missing	1 (2)	0	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Recipient age at transplant			
0-9 years	621 (43)	107 (47)	43 (43)
10-17 years	835 (57)	119 (53)	56 (57)
Median (Range)	12 (0-18)	11 (1-18)	11 (1-18)
Recipient race			
White	977 (75)	149 (75)	67 (80)
Black or African American	167 (13)	29 (15)	3 (4)
Asian	75 (6)	13 (7)	7 (8)
Native Hawaiian or other Pacific Islander	6 (<1)	3 (2)	1 (1)
American Indian or Alaska Native	18 (1)	3 (2)	1 (1)
More than one race	57 (4)	2 (1)	5 (6)
Unknown	156 (N/A)	27 (N/A)	15 (N/A)
Recipient ethnicity			
Hispanic or Latino	518 (36)	88 (40)	29 (31)
Non Hispanic or non-Latino	879 (62)	126 (58)	59 (63)
Non-resident of the U.S.	24 (2)	4 (2)	5 (5)
Unknown	35 (N/A)	8 (N/A)	6 (N/A)
Recipient sex			
Male	837 (57)	113 (50)	64 (65)
Female	619 (43)	113 (50)	35 (35)
Karnofsky score			
10-80	267 (18)	47 (21)	20 (20)
90-100	1151 (79)	174 (77)	73 (74)
Missing	38 (3)	5 (2)	6 (6)
HLA-A B DRB1 groups - low resolution			
<=3/6	452 (32)	70 (33)	32 (38)
4/6	143 (10)	22 (10)	12 (14)
5/6	39 (3)	9 (4)	6 (7)
6/6	767 (55)	114 (53)	35 (41)
Unknown	55 (N/A)	11 (N/A)	14 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	572 (42)	91 (43)	44 (52)
6/8	24 (2)	6 (3)	0
7/8	27 (2)	3 (1)	5 (6)
8/8	754 (55)	112 (53)	35 (42)
Unknown	79 (N/A)	14 (N/A)	15 (N/A)
HLA-DPB1 Match			
Double allele mismatch	1 (<1)	1 (1)	1 (2)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Single allele mismatch	464 (46)	56 (67)	31 (70)
Full allele matched	534 (53)	27 (32)	12 (27)
Unknown	457 (N/A)	142 (N/A)	55 (N/A)
High resolution release score			
No	832 (57)	220 (97)	99 (100)
Yes	624 (43)	6 (3)	0
Graft type			
Marrow	1037 (71)	124 (55)	64 (65)
PBSC	387 (27)	91 (40)	34 (34)
UCB	1 (<1)	9 (4)	0
BM+PBSC	3 (<1)	0	1 (1)
BM+UCB	3 (<1)	2 (1)	0
Others	25 (2)	0	0
Conditioning regimen			
Myeloablative	1358 (93)	214 (95)	92 (93)
RIC/Nonmyeloablative	94 (6)	10 (4)	5 (5)
TBD	4 (<1)	2 (1)	2 (2)
Donor age at donation			
To Be Determined/NA	3 (<1)	2 (1)	0
0-9 years	376 (26)	54 (24)	21 (21)
10-17 years	387 (27)	62 (27)	25 (25)
18-29 years	287 (20)	44 (19)	26 (26)
30-39 years	229 (16)	43 (19)	21 (21)
40-49 years	146 (10)	14 (6)	4 (4)
50+ years	28 (2)	7 (3)	2 (2)
Median (Range)	17 (0-61)	18 (0-61)	19 (1-53)
Donor/Recipient CMV serostatus			
+/+	564 (39)	96 (42)	41 (41)
+/-	165 (11)	18 (8)	13 (13)
-/+	395 (27)	49 (22)	23 (23)
-/-	313 (21)	48 (21)	20 (20)
CB - recipient +	4 (<1)	7 (3)	0
CB - recipient -	0	4 (2)	0
Missing	15 (1)	4 (2)	2 (2)
GvHD Prophylaxis			
No GvHD Prophylaxis	34 (2)	3 (1)	0
TDEPLETION alone	88 (6)	30 (13)	11 (11)
TDEPLETION +- other	43 (3)	18 (8)	8 (8)
CD34 select alone	12 (1)	0	1 (1)
CD34 select +- other	16 (1)	8 (4)	2 (2)
Cyclophosphamide alone	3 (<1)	1 (<1)	0
Cyclophosphamide +- others	454 (31)	43 (19)	27 (27)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
FK506 + MMF +- others	101 (7)	14 (6)	5 (5)
FK506 + MTX +- others(not MMF)	429 (29)	55 (24)	24 (24)
FK506 +- others(not MMF,MTX)	3 (<1)	1 (<1)	0
FK506 alone	9 (1)	2 (1)	1 (1)
CSA + MMF +- others(not FK506)	36 (2)	8 (4)	2 (2)
CSA + MTX +- others(not MMF,FK506)	193 (13)	30 (13)	16 (16)
CSA +- others(not FK506,MMF,MTX)	1 (<1)	2 (1)	0
CSA alone	28 (2)	7 (3)	1 (1)
Other GVHD Prophylaxis	4 (<1)	1 (<1)	1 (1)
Missing	2 (<1)	3 (1)	0
Donor/Recipient sex match			
Male-Male	487 (33)	53 (23)	31 (31)
Male-Female	291 (20)	53 (23)	16 (16)
Female-Male	347 (24)	54 (24)	33 (33)
Female-Female	327 (22)	55 (24)	19 (19)
CB - recipient M	3 (<1)	6 (3)	0
CB - recipient F	1 (<1)	5 (2)	0
Year of transplant			
2006-2010	36 (3)	3 (1)	0
2011-2015	272 (20)	31 (15)	16 (17)
2016-2020	560 (41)	92 (45)	33 (36)
2021-2025	588 (37)	100 (38)	50 (47)
Follow-up among survivors, Months			
N Eval	1104	180	64
Median (Range)	25 (0-147)	24 (0-103)	23 (0-97)

Accruals for Pediatric Cancer Working Committee

Table 1: Characteristics of patients aged <= 18 years who received allogeneic HCT between 2008 - 2025, and reported to the CIBMTR

Characteristic	TED	CRF	Total
No. of patients	16325	4830	21155
No. of centers	306	171	308
Primary disease, no. (%)			
AML or ANLL	5595 (34)	1857 (38)	7452 (35)
ALL	7313 (45)	1914 (40)	9227 (44)
Other Leukemia	48 (0)	8 (0)	56 (0)
CML	422 (3)	103 (2)	525 (2)
MDS	1619 (10)	559 (12)	2178 (10)
Acute Leukemia	499 (3)	139 (3)	638 (3)
NHL	561 (3)	158 (3)	719 (3)
HD	126 (1)	74 (2)	200 (1)
Plasma cell disorder	4 (0)	3 (0)	7 (0)
Solid Tumor	138 (1)	15 (0)	153 (1)
Donor type, no. (%)			
HLA-identical sibling	4665 (29)	630 (13)	5295 (25)
Twin	25 (0)	16 (0)	41 (0)
Other related	4238 (26)	865 (18)	5103 (24)
8/8 matched URD	3402 (21)	783 (16)	4185 (20)
7/8 mismatched URD	1076 (7)	378 (8)	1454 (7)
<= 6/8 mismatched URD;	50 (0)	40 (1)	90 (0)
Multi-donor	57 (0)	27 (1)	84 (0)
Unrelated (matching under review)	1183 (7)	90 (2)	1273 (6)
Cord blood	1627 (10)	2001 (41)	3628 (17)
Not reported	2 (0)	0 (0)	2 (0)
Graft Type, no. (%)			
Bone marrow	8978 (55)	1775 (37)	10753 (51)
Peripheral blood	5666 (35)	1041 (22)	6707 (32)
Cord blood	1615 (10)	1989 (41)	3604 (17)
Not reported	66 (0)	25 (1)	91 (0)
Conditioning intensity, no. (%)			
MAC	13660 (84)	4048 (84)	17708 (84)
RIC	926 (6)	326 (7)	1252 (6)
NMA	574 (4)	213 (4)	787 (4)
Need Review	1165 (7)	243 (5)	1408 (7)
GVHD prophylaxis, no. (%)			

Characteristic	TED	CRF	Total
None	235 (1)	116 (2)	351 (2)
Ex-vivo T-cell depletion	1080 (7)	192 (4)	1272 (6)
CD34 selection	423 (3)	130 (3)	553 (3)
PTCy + other(s)	2888 (18)	503 (10)	3391 (16)
PTCy alone	60 (0)	2 (0)	62 (0)
TAC + MMF +- other(s) (except PTCy)	1113 (7)	567 (12)	1680 (8)
TAC + MTX +- other(s) (except MMF, PTCy)	3127 (19)	787 (16)	3914 (19)
TAC + other(s) (except MMF, MTX, PTCy)	124 (1)	82 (2)	206 (1)
TAC alone	226 (1)	58 (1)	284 (1)
CSA + MMF +- other(s) (except PTCy,TAC)	1468 (9)	1117 (23)	2585 (12)
CSA + MTX +- other(s) (except PTCy,TAC,MMF)	4311 (26)	750 (16)	5061 (24)
CSA + other(s) (except PTCy,TAC,MMF,MTX)	217 (1)	325 (7)	542 (3)
CSA alone	730 (4)	149 (3)	879 (4)
Other(s)	243 (1)	50 (1)	293 (1)
Not Reported	80 (0)	2 (0)	82 (0)
Transplant Years, no. (%)			
2008-2011	3218 (20)	1741 (36)	4959 (23)
2012-2016	4012 (25)	1751 (36)	5763 (27)
2017-2021	4908 (30)	948 (20)	5856 (28)
2022-2025	4187 (26)	390 (8)	4577 (22)

Source: HCT Essentials November 2025

Table 2: Characteristics of patients aged <= 18 years who received autologous HCT between 2008 - 2025, and reported to the CIBMTR

Characteristic	TED	CRF	Total
No. of patients	14338	1564	15902
No. of centers	241	104	244
Primary disease, no. (%)			
AML or ANLL	78 (1)	11 (1)	89 (1)
ALL	8 (0)	0 (0)	8 (0)
Other Leukemia	1 (0)	0 (0)	1 (0)
MDS	1 (0)	1 (0)	2 (0)
Acute Leukemia	1 (0)	0 (0)	1 (0)
NHL	322 (2)	49 (3)	371 (2)
HD	1080 (8)	117 (7)	1197 (8)
Plasma cell disorder	9 (0)	3 (0)	12 (0)
Solid Tumor	12838 (90)	1383 (88)	14221 (89)
Sarcoma (osteosarcoma, rhabdomyosarcoma, Ewings, PNET and other sarcoma)	370 (3)	30 (2)	400 (3)
Wilm's Tumor	203 (1)	15 (1)	218 (1)
Testicular	83 (1)	6 (0)	89 (1)
Other gonadal tumors	63 (0)	9 (1)	72 (0)
Extragonadal germ cell tumors	352 (2)	33 (2)	385 (2)
Neuroblastoma	6314 (44)	569 (36)	6883 (43)
Other solid tumor	861 (6)	97 (6)	958 (6)
Medulloblastoma	2332 (16)	346 (22)	2678 (17)
Retinoblastoma	164 (1)	10 (1)	174 (1)
Other CNS tumor	2096 (15)	268 (17)	2364 (15)
Graft Type, no. (%)			
Bone marrow	300 (2)	25 (2)	325 (2)
Peripheral blood	14031 (98)	1533 (98)	15564 (98)
Cord blood	5 (0)	6 (0)	11 (0)
Not reported	2 (0)	0 (0)	2 (0)
Conditioning intensity, no. (%)			
MAC	7 (0)	275 (18)	282 (2)
RIC	2 (0)	99 (6)	101 (1)
NMA	0 (0)	7 (0)	7 (0)
Need Review	14329 (100)	1183 (76)	15512 (98)
Transplant Years, no. (%)			
2008-2011	2721 (19)	545 (35)	3266 (21)
2012-2016	3768 (26)	529 (34)	4297 (27)
2017-2021	4771 (33)	323 (21)	5094 (32)

Characteristic	TED	CRF	Total
2022-2025	3078 (21)	167 (11)	3245 (20)

Source: HCT Essentials Nov 2025

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

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	4700	1525	1743
Source of data			
CRF	2769 (59)	730 (48)	1039 (60)
TED	1931 (41)	795 (52)	704 (40)
Number of centers	163	124	202
Disease at transplant			
AML	1417 (30)	524 (34)	531 (30)
ALL	2037 (43)	614 (40)	768 (44)
Other leukemia	30 (1)	5 (<1)	10 (1)
CML	276 (6)	96 (6)	133 (8)
MDS	580 (12)	166 (11)	213 (12)
Other acute leukemia	118 (3)	50 (3)	26 (1)
NHL	182 (4)	49 (3)	41 (2)
Hodgkin Lymphoma	46 (1)	9 (1)	15 (1)
MPN	14 (<1)	12 (1)	6 (<1)
AML Disease status at transplant			
CR1	627 (44)	258 (49)	218 (41)
CR2	455 (32)	154 (29)	136 (26)
CR3+	34 (2)	11 (2)	16 (3)
Advanced or active disease	279 (20)	96 (18)	136 (26)
Missing	22 (2)	5 (1)	25 (5)
ALL Disease status at transplant			
CR1	615 (30)	174 (28)	193 (25)
CR2	873 (43)	292 (48)	314 (41)
CR3+	338 (17)	98 (16)	125 (16)
Advanced or active disease	175 (9)	43 (7)	74 (10)
Missing	36 (2)	7 (1)	62 (8)
MDS Disease status at transplant			
Early	186 (32)	41 (25)	37 (17)
Advanced	185 (32)	75 (45)	60 (28)
Missing	209 (36)	50 (30)	116 (54)
NHL Disease status at transplant			
CR1	33 (18)	12 (24)	14 (34)
CR2	50 (27)	23 (47)	10 (24)
CR3+	19 (10)	2 (4)	1 (2)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
PR	14 (8)	2 (4)	1 (2)
Advanced	62 (34)	10 (20)	8 (20)
Missing	4 (2)	0	7 (17)
Recipient age at transplant			
0-9 years	2258 (48)	724 (47)	835 (48)
10-17 years	2442 (52)	801 (53)	908 (52)
Median (Range)	10 (0-18)	11 (0-18)	10 (0-18)
Recipient race			
White	3707 (86)	1200 (85)	1218 (82)
Black or African American	340 (8)	97 (7)	140 (9)
Asian	151 (3)	58 (4)	80 (5)
Native Hawaiian or other	12 (<1)	2 (<1)	12 (1)
Pacific Islander			
American Indian or Alaska	34 (1)	15 (1)	10 (1)
Native			
Other	17 (<1)	10 (1)	8 (1)
More than one race	73 (2)	31 (2)	22 (1)
Unknown	366 (N/A)	112 (N/A)	253 (N/A)
Recipient ethnicity			
Hispanic or Latino	881 (25)	250 (21)	302 (25)
Non Hispanic or non-Latino	2524 (71)	875 (74)	582 (48)
Non-resident of the U.S.	145 (4)	53 (4)	340 (28)
Unknown	1150 (N/A)	347 (N/A)	519 (N/A)
Recipient sex			
Male	2771 (59)	918 (60)	1022 (59)
Female	1929 (41)	607 (40)	721 (41)
Karnofsky score			
10-80	727 (15)	269 (18)	306 (18)
90-100	3791 (81)	1197 (78)	1331 (76)
Missing	182 (4)	59 (4)	106 (6)
HLA-A B DRB1 groups - low resolution			
<=3/6	3 (<1)	4 (<1)	1 (<1)
4/6	65 (1)	9 (1)	6 (<1)
5/6	1041 (22)	280 (20)	349 (22)
6/6	3540 (76)	1126 (79)	1235 (78)
Unknown	51 (N/A)	106 (N/A)	152 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	216 (5)	8 (1)	26 (3)
6/8	383 (8)	34 (3)	54 (5)
7/8	1237 (27)	246 (25)	308 (31)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
8/8	2759 (60)	712 (71)	611 (61)
Unknown	105 (N/A)	525 (N/A)	744 (N/A)
HLA-DPB1 Match			
Double allele mismatch	1283 (30)	163 (26)	159 (27)
Single allele mismatch	2244 (53)	327 (52)	322 (54)
Full allele matched	693 (16)	134 (21)	116 (19)
Unknown	480 (N/A)	901 (N/A)	1146 (N/A)
High resolution release score			
No	875 (19)	1516 (99)	1581 (91)
Yes	3825 (81)	9 (1)	162 (9)
KIR typing available			
No	3548 (75)	1523 (>99)	1727 (99)
Yes	1152 (25)	2 (<1)	16 (1)
Graft type			
Marrow	3728 (79)	1215 (80)	1321 (76)
PBSC	967 (21)	298 (20)	419 (24)
BM+PBSC	3 (<1)	3 (<1)	1 (<1)
PBSC+UCB	0	5 (<1)	1 (<1)
Others	2 (<1)	4 (<1)	1 (<1)
Conditioning regimen			
Myeloablative	4355 (93)	1437 (94)	1616 (93)
RIC/Nonmyeloablative	320 (7)	84 (6)	102 (6)
TBD	25 (1)	4 (<1)	25 (1)
Donor age at donation			
To Be Determined/NA	15 (<1)	36 (2)	17 (1)
0-9 years	2 (<1)	2 (<1)	0
10-17 years	1 (<1)	0	2 (<1)
18-29 years	2103 (45)	742 (49)	695 (40)
30-39 years	1458 (31)	475 (31)	585 (34)
40-49 years	918 (20)	218 (14)	346 (20)
50+ years	203 (4)	52 (3)	98 (6)
Median (Range)	31 (3-61)	30 (4-61)	32 (17-61)
Donor/Recipient CMV serostatus			
+/+	1063 (23)	448 (29)	380 (22)
+/-	762 (16)	196 (13)	296 (17)
-/+	1301 (28)	387 (25)	447 (26)
-/-	1493 (32)	424 (28)	533 (31)
CB - recipient +	0	5 (<1)	1 (<1)
CB - recipient -	0	3 (<1)	0
CB - recipient CMV unknown	0	1 (<1)	0
Missing	81 (2)	61 (4)	86 (5)
GvHD Prophylaxis			

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
No GvHD Prophylaxis	13 (<1)	5 (<1)	8 (<1)
TDEPLETION alone	46 (1)	9 (1)	25 (1)
TDEPLETION +- other	286 (6)	86 (6)	138 (8)
CD34 select alone	29 (1)	12 (1)	9 (1)
CD34 select +- other	58 (1)	21 (1)	28 (2)
Cyclophosphamide alone	9 (<1)	2 (<1)	3 (<1)
Cyclophosphamide +- others	75 (2)	64 (4)	44 (3)
FK506 + MMF +- others	256 (5)	84 (6)	61 (3)
FK506 + MTX +- others(not MMF)	1404 (30)	538 (35)	305 (17)
FK506 +- others(not MMF,MTX)	98 (2)	13 (1)	18 (1)
FK506 alone	56 (1)	16 (1)	12 (1)
CSA + MMF +- others(not FK506)	237 (5)	65 (4)	65 (4)
CSA + MTX +- others(not MMF,FK506)	1643 (35)	462 (30)	779 (45)
CSA +- others(not FK506,MMF,MTX)	202 (4)	61 (4)	96 (6)
CSA alone	148 (3)	48 (3)	89 (5)
Other GVHD Prophylaxis	107 (2)	27 (2)	34 (2)
Missing	33 (1)	12 (1)	29 (2)
Donor/Recipient sex match			
Male-Male	1780 (38)	570 (37)	610 (35)
Male-Female	1075 (23)	334 (22)	372 (21)
Female-Male	983 (21)	337 (22)	405 (23)
Female-Female	848 (18)	264 (17)	342 (20)
CB - recipient M	0	3 (<1)	1 (<1)
CB - recipient F	0	6 (<1)	0
Missing	14 (<1)	11 (1)	13 (1)
Year of transplant			
1986-1990	73 (2)	9 (1)	30 (2)
1991-1995	437 (9)	107 (7)	203 (12)
1996-2000	579 (12)	212 (15)	331 (19)
2001-2005	704 (15)	153 (11)	325 (19)
2006-2010	855 (18)	154 (11)	188 (11)
2011-2015	1005 (22)	215 (15)	243 (14)
2016-2020	675 (15)	339 (23)	237 (14)
2021-2025	382 (7)	336 (18)	186 (10)
Follow-up among survivors, Months			
N Eval	2470	849	878

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Median (Range)	71 (0-353)	37 (0-295)	57 (0-385)

Table 4: Unrelated cord 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	1587	519	648
Source of data			
CRF	1135 (72)	332 (64)	342 (53)
TED	452 (28)	187 (36)	306 (47)
Number of centers	89	77	121
Disease at transplant			
AML	627 (40)	192 (37)	239 (37)
ALL	666 (42)	245 (47)	284 (44)
Other leukemia	10 (1)	2 (<1)	4 (1)
CML	20 (1)	5 (1)	9 (1)
MDS	163 (10)	46 (9)	70 (11)
Other acute leukemia	45 (3)	15 (3)	23 (4)
NHL	49 (3)	14 (3)	14 (2)
Hodgkin Lymphoma	5 (<1)	0	4 (1)
MPN	2 (<1)	0	1 (<1)
AML Disease status at transplant			
CR1	299 (48)	105 (55)	109 (46)
CR2	217 (35)	54 (28)	70 (29)
CR3+	13 (2)	0	7 (3)
Advanced or active disease	97 (15)	33 (17)	48 (20)
Missing	1 (<1)	0	5 (2)
ALL Disease status at transplant			
CR1	226 (34)	81 (33)	107 (38)
CR2	317 (48)	112 (46)	111 (39)
CR3+	99 (15)	38 (16)	48 (17)
Advanced or active disease	23 (3)	13 (5)	18 (6)
Missing	1 (<1)	1 (<1)	0
MDS Disease status at transplant			
Early	61 (37)	15 (33)	36 (51)
Advanced	57 (35)	22 (48)	18 (26)
Missing	45 (28)	9 (20)	16 (23)
NHL Disease status at transplant			
CR1	10 (20)	2 (14)	3 (21)
CR2	21 (43)	9 (64)	8 (57)
CR3+	5 (10)	1 (7)	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
PR	3 (6)	0	1 (7)
Advanced	10 (20)	2 (14)	2 (14)
Recipient age at transplant			
0-9 years	1027 (65)	358 (69)	411 (63)
10-17 years	560 (35)	161 (31)	237 (37)
Median (Range)	7 (0-18)	7 (0-18)	7 (0-18)
Recipient race			
White	1103 (74)	364 (75)	404 (72)
Black or African American	221 (15)	73 (15)	76 (14)
Asian	71 (5)	22 (5)	43 (8)
Native Hawaiian or other Pacific Islander	5 (<1)	3 (1)	11 (2)
American Indian or Alaska Native	20 (1)	5 (1)	6 (1)
Other	0	0	1 (<1)
More than one race	64 (4)	19 (4)	21 (4)
Unknown	103 (N/A)	33 (N/A)	86 (N/A)
Recipient ethnicity			
Hispanic or Latino	481 (31)	143 (28)	128 (20)
Non Hispanic or non-Latino	1058 (68)	352 (70)	332 (53)
Non-resident of the U.S.	14 (1)	10 (2)	166 (27)
Unknown	34 (N/A)	14 (N/A)	22 (N/A)
Recipient sex			
Male	925 (58)	285 (55)	367 (57)
Female	662 (42)	234 (45)	281 (43)
Karnofsky score			
10-80	255 (16)	87 (17)	103 (16)
90-100	1280 (81)	403 (78)	492 (76)
Missing	52 (3)	29 (6)	53 (8)
HLA-A B DRB1 groups - low resolution			
<=3/6	13 (1)	5 (1)	3 (<1)
4/6	469 (30)	141 (29)	166 (27)
5/6	794 (51)	245 (51)	321 (52)
6/6	280 (18)	92 (19)	130 (21)
Unknown	31 (N/A)	36 (N/A)	28 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	586 (42)	137 (37)	190 (40)
6/8	410 (29)	122 (33)	131 (28)
7/8	259 (18)	73 (20)	97 (21)
8/8	146 (10)	41 (11)	54 (11)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Unknown	186 (N/A)	146 (N/A)	176 (N/A)
HLA-DPB1 Match			
Double allele mismatch	275 (39)	53 (35)	64 (36)
Single allele mismatch	368 (52)	87 (57)	91 (51)
Full allele matched	70 (10)	12 (8)	25 (14)
Unknown	874 (N/A)	367 (N/A)	468 (N/A)
High resolution release score			
No	1037 (65)	488 (94)	636 (98)
Yes	550 (35)	31 (6)	12 (2)
KIR typing available			
No	1147 (72)	514 (99)	638 (98)
Yes	440 (28)	5 (1)	10 (2)
Graft type			
UCB	1567 (99)	510 (98)	638 (98)
PBSC+UCB	8 (1)	5 (1)	7 (1)
Others	12 (1)	4 (1)	3 (<1)
Number of cord units			
1	1478 (93)	0	603 (93)
2	109 (7)	0	45 (7)
Unknown	0 (N/A)	519 (N/A)	0 (N/A)
Conditioning regimen			
Myeloablative	1504 (95)	489 (94)	592 (91)
RIC/Nonmyeloablative	82 (5)	30 (6)	54 (8)
TBD	1 (<1)	0	2 (<1)
Donor age at donation			
To Be Determined/NA	1284 (81)	256 (49)	555 (86)
0-9 years	287 (18)	231 (45)	88 (14)
10-17 years	9 (1)	28 (5)	3 (<1)
18-29 years	4 (<1)	0	0
30-39 years	3 (<1)	3 (1)	1 (<1)
40-49 years	0	1 (<1)	0
50+ years	0	0	1 (<1)
Median (Range)	4 (0-37)	4 (0-42)	4 (0-52)
Donor/Recipient CMV serostatus			
CB - recipient +	962 (61)	331 (64)	392 (60)
CB - recipient -	601 (38)	177 (34)	228 (35)
CB - recipient CMV unknown	24 (2)	11 (2)	28 (4)
GvHD Prophylaxis			
No GvHD Prophylaxis	5 (<1)	3 (1)	3 (<1)
TDEPLETION alone	1 (<1)	0	0
TDEPLETION +- other	6 (<1)	4 (1)	3 (<1)
CD34 select alone	0	1 (<1)	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
CD34 select +- other	6 (<1)	1 (<1)	3 (<1)
Cyclophosphamide +- others	5 (<1)	0	5 (1)
FK506 + MMF +- others	341 (21)	155 (30)	107 (17)
FK506 + MTX +- others(not MMF)	106 (7)	28 (5)	40 (6)
FK506 +- others(not MMF,MTX)	32 (2)	16 (3)	14 (2)
FK506 alone	9 (1)	7 (1)	5 (1)
CSA + MMF +- others(not FK506)	835 (53)	228 (44)	292 (45)
CSA + MTX +- others(not MMF,FK506)	49 (3)	11 (2)	23 (4)
CSA +- others(not FK506,MMF,MTX)	160 (10)	54 (10)	119 (18)
CSA alone	22 (1)	6 (1)	24 (4)
Other GVHD Prophylaxis	8 (1)	4 (1)	7 (1)
Missing	2 (<1)	1 (<1)	3 (<1)
Donor/Recipient sex match			
CB - recipient M	925 (58)	285 (55)	366 (56)
CB - recipient F	662 (42)	234 (45)	281 (43)
CB - recipient sex unknown	0	0	1 (<1)
Year of transplant			
1996-2000	0	0	2 (<1)
2001-2005	46 (3)	39 (8)	14 (2)
2006-2010	561 (36)	124 (25)	208 (33)
2011-2015	549 (35)	128 (25)	227 (36)
2016-2020	287 (18)	131 (26)	103 (16)
2021-2025	144 (8)	97 (16)	94 (12)
Follow-up among survivors, Months			
N Eval	929	322	358
Median (Range)	65 (0-196)	44 (0-213)	48 (0-186)

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

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Number of patients	1456	226	99
Source of data			
CRF	287 (20)	45 (20)	16 (16)
TED	1169 (80)	181 (80)	83 (84)
Number of centers	54	43	38
Disease at transplant			
AML	501 (34)	71 (31)	38 (38)
ALL	665 (46)	114 (50)	48 (48)
Other leukemia	2 (<1)	0	0
CML	42 (3)	1 (<1)	2 (2)
MDS	117 (8)	23 (10)	9 (9)
Other acute leukemia	56 (4)	4 (2)	1 (1)
NHL	61 (4)	11 (5)	1 (1)
Hodgkin Lymphoma	9 (1)	2 (1)	0
MPN	3 (<1)	0	0
AML Disease status at transplant			
CR1	327 (65)	51 (72)	22 (58)
CR2	118 (24)	16 (23)	10 (26)
CR3+	6 (1)	1 (1)	1 (3)
Advanced or active disease	48 (10)	1 (1)	5 (13)
Missing	2 (<1)	2 (3)	0
ALL Disease status at transplant			
CR1	237 (36)	43 (38)	20 (42)
CR2	337 (51)	54 (47)	20 (42)
CR3+	78 (12)	14 (12)	6 (13)
Advanced or active disease	13 (2)	3 (3)	2 (4)
MDS Disease status at transplant			
Early	27 (23)	5 (22)	2 (22)
Advanced	70 (60)	10 (43)	4 (44)
Missing	20 (17)	8 (35)	3 (33)
NHL Disease status at transplant			
CR1	18 (30)	3 (27)	0
CR2	24 (39)	2 (18)	1 (100)
CR3+	2 (3)	0	0
Advanced	16 (26)	6 (55)	0
Missing	1 (2)	0	0

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Recipient age at transplant			
0-9 years	621 (43)	107 (47)	43 (43)
10-17 years	835 (57)	119 (53)	56 (57)
Median (Range)	12 (0-18)	11 (1-18)	11 (1-18)
Recipient race			
White	977 (75)	149 (75)	67 (80)
Black or African American	167 (13)	29 (15)	3 (4)
Asian	75 (6)	13 (7)	7 (8)
Native Hawaiian or other Pacific Islander	6 (<1)	3 (2)	1 (1)
American Indian or Alaska Native	18 (1)	3 (2)	1 (1)
More than one race	57 (4)	2 (1)	5 (6)
Unknown	156 (N/A)	27 (N/A)	15 (N/A)
Recipient ethnicity			
Hispanic or Latino	518 (36)	88 (40)	29 (31)
Non Hispanic or non-Latino	879 (62)	126 (58)	59 (63)
Non-resident of the U.S.	24 (2)	4 (2)	5 (5)
Unknown	35 (N/A)	8 (N/A)	6 (N/A)
Recipient sex			
Male	837 (57)	113 (50)	64 (65)
Female	619 (43)	113 (50)	35 (35)
Karnofsky score			
10-80	267 (18)	47 (21)	20 (20)
90-100	1151 (79)	174 (77)	73 (74)
Missing	38 (3)	5 (2)	6 (6)
HLA-A B DRB1 groups - low resolution			
<=3/6	452 (32)	70 (33)	32 (38)
4/6	143 (10)	22 (10)	12 (14)
5/6	39 (3)	9 (4)	6 (7)
6/6	767 (55)	114 (53)	35 (41)
Unknown	55 (N/A)	11 (N/A)	14 (N/A)
High-resolution HLA matches available out of 8			
<=5/8	572 (42)	91 (43)	44 (52)
6/8	24 (2)	6 (3)	0
7/8	27 (2)	3 (1)	5 (6)
8/8	754 (55)	112 (53)	35 (42)
Unknown	79 (N/A)	14 (N/A)	15 (N/A)
HLA-DPB1 Match			
Double allele mismatch	1 (<1)	1 (1)	1 (2)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
Single allele mismatch	464 (46)	56 (67)	31 (70)
Full allele matched	534 (53)	27 (32)	12 (27)
Unknown	457 (N/A)	142 (N/A)	55 (N/A)
High resolution release score			
No	832 (57)	220 (97)	99 (100)
Yes	624 (43)	6 (3)	0
Graft type			
Marrow	1037 (71)	124 (55)	64 (65)
PBSC	387 (27)	91 (40)	34 (34)
UCB	1 (<1)	9 (4)	0
BM+PBSC	3 (<1)	0	1 (1)
BM+UCB	3 (<1)	2 (1)	0
Others	25 (2)	0	0
Conditioning regimen			
Myeloablative	1358 (93)	214 (95)	92 (93)
RIC/Nonmyeloablative	94 (6)	10 (4)	5 (5)
TBD	4 (<1)	2 (1)	2 (2)
Donor age at donation			
To Be Determined/NA	3 (<1)	2 (1)	0
0-9 years	376 (26)	54 (24)	21 (21)
10-17 years	387 (27)	62 (27)	25 (25)
18-29 years	287 (20)	44 (19)	26 (26)
30-39 years	229 (16)	43 (19)	21 (21)
40-49 years	146 (10)	14 (6)	4 (4)
50+ years	28 (2)	7 (3)	2 (2)
Median (Range)	17 (0-61)	18 (0-61)	19 (1-53)
Donor/Recipient CMV serostatus			
+/+	564 (39)	96 (42)	41 (41)
+/-	165 (11)	18 (8)	13 (13)
-/+	395 (27)	49 (22)	23 (23)
-/-	313 (21)	48 (21)	20 (20)
CB - recipient +	4 (<1)	7 (3)	0
CB - recipient -	0	4 (2)	0
Missing	15 (1)	4 (2)	2 (2)
GvHD Prophylaxis			
No GvHD Prophylaxis	34 (2)	3 (1)	0
TDEPLETION alone	88 (6)	30 (13)	11 (11)
TDEPLETION +- other	43 (3)	18 (8)	8 (8)
CD34 select alone	12 (1)	0	1 (1)
CD34 select +- other	16 (1)	8 (4)	2 (2)
Cyclophosphamide alone	3 (<1)	1 (<1)	0
Cyclophosphamide +- others	454 (31)	43 (19)	27 (27)

Variable	Samples Available for Recipient and Donor N (%)	Samples Available for Recipient Only N (%)	Samples Available for Donor Only N (%)
FK506 + MMF +- others	101 (7)	14 (6)	5 (5)
FK506 + MTX +- others(not MMF)	429 (29)	55 (24)	24 (24)
FK506 +- others(not MMF,MTX)	3 (<1)	1 (<1)	0
FK506 alone	9 (1)	2 (1)	1 (1)
CSA + MMF +- others(not FK506)	36 (2)	8 (4)	2 (2)
CSA + MTX +- others(not MMF,FK506)	193 (13)	30 (13)	16 (16)
CSA +- others(not FK506,MMF,MTX)	1 (<1)	2 (1)	0
CSA alone	28 (2)	7 (3)	1 (1)
Other GVHD Prophylaxis	4 (<1)	1 (<1)	1 (1)
Missing	2 (<1)	3 (1)	0
Donor/Recipient sex match			
Male-Male	487 (33)	53 (23)	31 (31)
Male-Female	291 (20)	53 (23)	16 (16)
Female-Male	347 (24)	54 (24)	33 (33)
Female-Female	327 (22)	55 (24)	19 (19)
CB - recipient M	3 (<1)	6 (3)	0
CB - recipient F	1 (<1)	5 (2)	0
Year of transplant			
2006-2010	36 (3)	3 (1)	0
2011-2015	272 (20)	31 (15)	16 (17)
2016-2020	560 (41)	92 (45)	33 (36)
2021-2025	588 (37)	100 (38)	50 (47)
Follow-up among survivors, Months			
N Eval	1104	180	64
Median (Range)	25 (0-147)	24 (0-103)	23 (0-97)

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A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

TO: Pediatric Cancer Working Committee Members

FROM: Larisa Broglie, MD MS; Scientific Director for the Pediatric Cancer Working Committee

RE: 2025-2026 Studies in Progress Summary

PC19-02 Does mixed peripheral blood T Cell Chimerism predict relapse? (A Lake / S Prockop / J Boelens / K Peggs). This study aims to study the effect of mixed T-cell chimerism on relapse, hypothesizing that it is not associated with post-transplant relapse. There has been an extensive review of the available chimerism data and discussion with our statistical team to select an appropriate analysis plant. Ultimately, the objectives of this study are 1) to determine the incidence of short term mixed chimerism and determine if is associated with relapse and 2) to determine the incidence of persistent mixed chimerism and determine if it is associated with relapse. The demographics tables will be prepared with a contemporary cohort and protocol shared with the Working Committee soon.
Status: Demographic table preparation

CT20-02 Resource utilization with chimeric antigen receptor T cells (M Battiwalla/ H Rangarajan/ C Scheckel). The objective of this study is to:

1. Determine "real world" costs and HCRU incurred during CAR-T therapy for in pediatric ALL patients.
2. Identify patient, disease, and cellular therapy related factors associated with increased HCRU and costs
3. Compare the HCRU and costs incurred by Kymriah treated pediatric (≤21 years) patients with that of pediatric patients who underwent allo HCT between September 2017- June 30 2021.
4. Identify impact of increased HCRU and costs on CART on 1 year LFS, 1 year OS.

Status: Protocol Development - received PHIS data and will begin matching process.

PC20-02 Germline genetics of pediatric Myelodysplastic Syndromes (J Poynter/ L Spector). The objective of this study is to identify genetic susceptibility variants for pediatric patients with MDS in an unselected cohort of pediatric patients. Genotyping will be conducted using the Illumina Global Screening array and controls will include > 2000 DNA samples that have been genotyped for other childhood cancer studies. To improve power, we will focus on regions of the genome expressed in myeloid cells as determined by ATAC-seq in primary MDS cell cultures. The study is currently in sample typing, with demographic and outcome dataset shared with PIs.
Status: Analysis

PC22-01 Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). primary objective of this study is to determine the impact of development of grade I and II acute graft versus host disease (aGVHD) on relapse and leukemia-free survival in children undergoing hematopoietic cell transplant (HCT) for ALL and AML, with the hypothesis that mild to moderate aGVHD is associated with improved Leukemia-free survival in children with

favourable risk disease by pediatric DRI classification. The study found that, when disease risk was taken into consideration with the pediatric DRI, the presence of GVHD did not impact relapse.
Status: The study was presented at the Tandem Meetings 2025 and Manuscript in preparation

PC22-02 Evaluating predictors of access and outcomes with hematopoietic cell transplantation in pediatric and adolescent patients with relapsed/refractory classical Hodgkin lymphoma after treatment on an initial cooperative group clinical trial (S Castellino/ J Kahn). The objective of this study is primary to use a novel data linkage between the Children's Oncology Group (COG) and the CIBMTR to: 1) evaluate the receipt of HCT in a contemporary cohort of children and adolescents with r/r HL; to determine patient- and disease-related factors associated with receipt of HCT including age at initial diagnosis, race/ethnicity, insurance type, and location of care during COG therapy; 2) to evaluate post-transplant survival outcomes (PFS, TRM, OS) in the above transplanted cohort.
Status: The protocol is in development and awaiting DUA agreement between COG and CIBMTR.

PC23-01 Post-transplant cyclophosphamide vs. TCR αβ/CD19+ deplete approaches for haploidentical transplant in pediatric patients with acute leukemias and myelodysplastic syndrome: A CIBMTR/EBMT collaborative study (A Li/ H Rangarajan/ P Satwani). The primary objective of the study is to compare the 2-year leukemia free survival (LFS) between patients who received TCR αβ/CD19+ depletion versus PTCY for GVHD prophylaxis for haploidentical transplant. The initial plan was to collaborate with EBMT, however, we will not combine our data with EBMT but instead have 2 parallel studies and potentially a joint commentary. The datafile has been prepared and analysis is being completed.
Status: Analysis

PC23-02 Comparison of bone marrow and peripheral blood stem cells as graft source in children undergoing allogeneic hematopoietic stem cell transplantation for hematological malignancies with unmanipulated haploidentical grafts utilizing post-transplant cyclophosphamide as GvHD prophylaxis (A Srinivasan/ J Krueger). The hypothesis of this study is that 1-yr chronic GvHD free relapse free survival (CRFS) is similar between recipients of peripheral blood stem cell and bone marrow haploidentical grafts utilizing PT-Cy GvHD prophylaxis. The analysis has been completed and results showing that PBSC-PTCy is associated with more chronic GVHD than when BM-PTCy is used, contributing to a lower CFRS.
Status: abstract to EBMT 2025 and Manuscript is in preparation.

PC24-01 Transplantation and cellular therapy for children and young adults with down's syndrome and acute leukemia (L Appell/ S Rotz). This study is a collaboration between CIBMTR and EBMT. The primary objective will be to evaluate overall survival in children with Down's Syndrome with AML who receive allo-transplant and ALL with CART or HCT. We have met with EBMT and finalized a protocol. The demographics tables are being prepared. We are awaiting EBMT datafile in order to combine the AML cohorts together for analysis.
Status: *Protocol Development.*

PC25-01 Impact of Planned Post-Transplant Granulocyte Colony Stimulating Factor (G-CSF) on Transplant-Related Outcomes in Pediatric Patients with Malignant Disease Undergoing Haploidentical Hematopoietic Cell Transplant (HCT) with Post-Transplant Cyclophosphamide (ptCy) for Graft vs. Host Disease (GVHD) Prophylaxis (L Davis/ P Satwani). The hypothesis of this study is that pediatric patients undergoing haploidentical HCT (HaploHCT) with ptCy who receive planned GCSF will have lower overall survival (OS), disease free survival (DFS) and greater relapse and non-relapse mortality (NRM) compared to those patients who did not receive planned G-CSF. The protocol is being finalized and demographics tables developed.

Status: *Protocol Development.*

Field	Response
Proposal Number	2411-01-HANS
Proposal Title	The Role of Maintenance Tyrosine Kinase Inhibitors Following Allogenic Hematopoietic Stem Cell Transplant for Pediatric Patients with Chronic Myelogenous Leukemia
Key Words	Chronic Myelogenous Leukemia, Pediatrics, Tyrosine Kinase Inhibitors, Stem Cell Transplant
Principal Investigator #1: - First and last name, degree(s)	Rhea Hans, M.D.
Principal Investigator #1: - Email address	hansr1@mskcc.org
Principal Investigator #1: - Institution name	Memorial Sloan Kettering Cancer Center
Principal Investigator #1: - Academic rank	Transplant and Cellular Therapy Fellow
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):	Andrew C. Harris, M.D.
Principal Investigator #2 (If applicable): - Email address:)	harrisa7@mskcc.org
Principal Investigator #2 (If applicable): - Institution name:	Memorial Sloan Kettering Cancer Center
Principal Investigator #2 (If applicable): - Academic rank:	Associate Attending
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:	Rhea Hans
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:	Plasma Cell Disorders
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No

Field	Response
RESEARCH QUESTION:	Does the administration of tyrosine kinase inhibitor (TKI) maintenance therapy following hematopoietic stem cell transplant (HSCT) improve leukemia-free survival for children with chronic myelogenous leukemia (CML)?
RESEARCH HYPOTHESIS:	Recent studies have shown a lack of benefit from the use of TKI maintenance therapy following HSCT in adults with CML. We hypothesize that TKI maintenance therapy improves leukemia-free survival in the pediatric population.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Aims: 1) To compare leukemia-free survival (LFS) between children who receive TKIs as maintenance therapy post-HSCT and those who do not. Secondary Aims: 1) To compare rates of overall survival (OS), acute and chronic graft vs host disease (GVHD), treatment-related mortality (TRM), graft failure and relapse between children transplanted for CML who receive TKIs as maintenance therapy post-HSCT and those who do not.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Post-transplant TKI maintenance is a widely accepted standard of care for children receiving HSCT for

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.	Chronic myeloid leukemia (CML) is a clonal disorder of hematopoietic stem cells, classically marked by a reciprocal translocation that fuses the Abelson oncogene (ABL) on chromosome 9q34 with the breakpoint cluster region (BCR) on chromosome 22q11.2. This translocation, designated as t(9;22)(q34;q11.2), leads to the formation of a shortened chromosome 22 known as the Philadelphia (Ph) chromosome, which produces the BCR-ABL1 fusion oncoprotein. This oncoprotein exhibits continuous tyrosine kinase activity, promoting hematopoietic transformation and myeloproliferation [1]. The first TKI to be introduced was Imatinib in 2001. With the introduction of TKIs the treatment landscape for CML changed with significant improvement in clinical outcomes and offered an alternative therapeutic option to allogeneic HSCT for patients. Since the introduction of TKI therapy, long term survival for patients with CML has increased from <20% to 80-90% [2-4]. Although TKIs have an impactful effect on outcomes for CML, there is currently not enough evidence to determine the efficacy as maintenance therapy after allogenic transplant. There have been several retrospective and prospective trials, including both adult and children, for Ph+ ALL that have demonstrated a survival benefit of TKI maintenance therapy [5-7]. There is limited data on post TKI therapy in CML. A recent large retrospective adult study limited to CML patients found no difference in leukemia free survival and overall survival regardless of TKI maintenance therapy use [4]. In this study, patients were excluded if they had early molecular relapse, before day +100. When intended as post-transplant maintenance, TKI therapy may be started prior to day +100 for many patients and may prove to be beneficial in preventing early molecular relapse. Despite a lack of supporting evidence, current recommendations are to administer TKI maintenance therapy post-HSCT for children with CML [8]. Performing a large retrospective study in pediatrics would allow for direct comparison of those who received planned post-transplant TKI maintenance therapy to those who did not, which in turn will provide physicians with evidence-based information for decision making regarding TKI maintenance therapy in pediatric patients with CML.

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Patients aged 0 17.99 years with CML receiving a first allogenic HSCT, with or without planned post-transplant maintenance TKI from 2008 - 2018. Those with previous TKI intolerance will be excluded. Patients that did not receive TKI therapy before allo-HSCT and patients receiving therapies other than TKIs are maintenance therapy will be excluded.
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 Information: Age at Transplant Gender LPS/KPS Score at Transplant Disease Information: Disease indication for transplant Disease status at transplant Number of TKIs prior to transplant (1 vs >1) TKIs used pre-transplant (e.g. imatinib, dasatinib, etc.) Transplant Information: Year of transplant Conditioning regimen intensity (as defined by CIBMTR) Conditioning regimen used Donor Type (Related/Unrelated) Donor-Recipient HLA match Graft Source (BM/PBSC/Cord) GVHD prophylaxis Planned TKI maintenance therapy (y/n) Patient received maintenance TKI therapy (y/n) Use of serotherapy (e.g. ATG, alemtuzumab) Graft T-cell depletion (y/n) Choice of TKI maintenance therapy Dose of TKI maintenance therapy Duration of TKI maintenance therapy Outcomes/Toxicities: Time to neutrophil engraftment Time to platelet engraftment Incidence of primary graft failure Incidence of secondary graft failure Incidence/severity of acute GVHD Incidence/severity of chronic GVHD Leukemia free survival Relapse incidence, timing Non-relapse mortality Overall survival Post-transplant BCR:ABL1 PCR
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A description of the hypothesis speci	N/A
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	N/A

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	No biologic samples are requested.
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

Field	Response
REFERENCES:	1. Pophali, P.A. and M.M. Patnaik, The Role of New Tyrosine Kinase Inhibitors in Chronic Myeloid Leukemia. Cancer J, 2016. 22(1): p. 40-50. 2. Bj rkholm, M., et al., Success story of targeted therapy in chronic myeloid leukemia: a population-based study of patients diagnosed in Sweden from 1973 to 2008. J Clin Oncol, 2011. 29(18): p. 2514-20. 3. Kantarjian, H., et al., Improved survival in chronic myeloid leukemia since the introduction of imatinib therapy: a single-institution historical experience. Blood, 2012. 119(9): p. 1981-7. 4. DeFilipp, Z., et al., Maintenance Tyrosine Kinase Inhibitors Following Allogeneic Hematopoietic Stem Cell Transplantation for Chronic Myelogenous Leukemia: A Center for International Blood and Marrow Transplant Research Study. Biol Blood Marrow Transplant, 2020. 26(3): p. 472-479. 5. Pfeifer, H., et al., Randomized comparison of prophylactic and minimal residual disease-triggered imatinib after allogeneic stem cell transplantation for BCR-ABL1-positive acute lymphoblastic leukemia. Leukemia, 2013. 27(6): p. 1254-62. 6. Brissot, E., et al., Tyrosine kinase inhibitors improve long-term outcome of allogeneic hematopoietic stem cell transplantation for adult patients with Philadelphia chromosome positive acute lymphoblastic leukemia. Haematologica, 2015. 100(3): p. 392-9. 7. Chen, H., et al., Administration of imatinib after allogeneic hematopoietic stem cell transplantation may improve disease-free survival for patients with Philadelphia chromosome-positive acute lymphobla stic leukemia. J Hematol Oncol, 2012. 5: p. 29. 8. Sembill, S., et al., Management of children and adolescents with chronic myeloid leukemia in blast phase: International pediatric CML expert panel recommendations. Leukemia, 2023. 37(3): p. 505-517.
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: Patients with CML and age <18 years who received Allo HCT in 2008-2023

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
No. of patients	24	255	77	356
No. of centers	18	97	49	113
Patient age (years), median (range)	13.8 (4.5-17.9)	13.2 (1.3-17.9)	13.3 (1.7-17.9)	13.2 (1.3-17.9)
Age Groups, no. (%)				
<2 year	0 (0)	2 (1)	1 (1)	3 (1)
2-10 years	5 (21)	85 (33)	22 (29)	112 (31)
11-<18 years	19 (79)	168 (66)	54 (70)	241 (68)
TED or RES (CRF) track determined for this event, no. (%)				
TED	24 (100)	190 (75)	60 (78)	274 (77)
CRF(RES)	0 (0)	65 (25)	17 (22)	82 (23)
Race, no. (%)				
White	10 (42)	143 (56)	45 (58)	198 (56)
Black or African American	2 (8)	28 (11)	10 (13)	40 (11)
Asian	3 (13)	41 (16)	9 (12)	53 (15)
Native Hawaiian or other Pacific Islander	1 (4)	3 (1)	0 (0)	4 (1)
American Indian or Alaska Native	0 (0)	1 (0)	0 (0)	1 (0)
More than one race	3 (13)	9 (4)	6 (8)	18 (5)
Not reported	5 (21)	30 (12)	7 (9)	42 (12)
Ethnicity, no. (%)				
Hispanic or Latino	4 (17)	37 (15)	13 (17)	54 (15)
Non-Hispanic or Latino	12 (50)	140 (55)	51 (66)	203 (57)
Non-resident of the U.S.	7 (29)	75 (29)	13 (17)	95 (27)
Not reported	1 (4)	3 (1)	0 (0)	4 (1)
Karnofsky score prior to HCT, no. (%)				
90-100%	23 (96)	228 (89)	67 (87)	318 (89)
<90%	1 (4)	22 (9)	10 (13)	33 (9)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Not reported	0 (0)	5 (2)	0 (0)	5 (1)
Graft Type, no. (%)				
BM	15 (63)	174 (68)	49 (64)	238 (67)
PBSC	8 (33)	57 (22)	17 (22)	82 (23)
UCB	1 (4)	24 (9)	11 (14)	36 (10)
Conditioning regimen intensity-Planned, no. (%)				
MAC	23 (96)	241 (95)	70 (91)	334 (94)
RIC	0 (0)	7 (3)	3 (4)	10 (3)
NMA	1 (4)	0 (0)	2 (3)	3 (1)
Not reported	0 (0)	7 (3)	2 (3)	9 (3)
Conditioning regimen-Planned, no. (%)				
TBI/Cy	8 (33)	37 (15)	14 (18)	59 (17)
TBI/Cy/Flu	1 (4)	9 (4)	7 (9)	17 (5)
TBI/Cy/Flu/TT	0 (0)	1 (0)	0 (0)	1 (0)
TBI/Cy/TT	0 (0)	14 (5)	4 (5)	18 (5)
TBI/Cy/VP	3 (13)	1 (0)	2 (3)	6 (2)
TBI/VP	2 (8)	2 (1)	0 (0)	4 (1)
TBI/Mel	0 (0)	2 (1)	0 (0)	2 (1)
TBI/Flu	1 (4)	14 (5)	2 (3)	17 (5)
TBI/other(s)	0 (0)	0 (0)	1 (1)	1 (0)
Bu/Cy/Mel	0 (0)	1 (0)	0 (0)	1 (0)
Bu/Cy	7 (29)	109 (43)	32 (42)	148 (42)
Bu/Mel	0 (0)	6 (2)	2 (3)	8 (2)
Flu/Bu/TT	2 (8)	6 (2)	0 (0)	8 (2)
Flu/Bu	0 (0)	35 (14)	10 (13)	45 (13)
Flu/Mel/TT	0 (0)	7 (3)	0 (0)	7 (2)
Flu/Mel	0 (0)	2 (1)	0 (0)	2 (1)
Cy/Flu	0 (0)	0 (0)	1 (1)	1 (0)
Mel/other(s)	0 (0)	2 (1)	0 (0)	2 (1)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Treosulfan	0 (0)	6 (2)	1 (1)	7 (2)
Other(s)	0 (0)	0 (0)	1 (1)	1 (0)
Not reported	0 (0)	1 (0)	0 (0)	1 (0)
GVHD prophylaxis (Administered), no. (%)				
Ex-vivo T-cell depletion	1 (4)	9 (4)	1 (1)	11 (3)
CD34 selection	1 (4)	1 (0)	1 (1)	3 (1)
PtCy + other(s)	4 (17)	35 (14)	10 (13)	49 (14)
TAC + MMF +- other(s) (except PtCy)	0 (0)	17 (7)	7 (9)	24 (7)
TAC + MTX +- other(s) (except MMF, PtCy)	12 (50)	52 (20)	25 (32)	89 (25)
TAC + other(s) (except MMF, MTX, PtCy)	0 (0)	2 (1)	0 (0)	2 (1)
TAC alone	0 (0)	1 (0)	1 (1)	2 (1)
CSA + MMF +- other(s) (except PtCy,TAC)	1 (4)	17 (7)	9 (12)	27 (8)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	4 (17)	97 (38)	20 (26)	121 (34)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	0 (0)	9 (4)	0 (0)	9 (3)
CSA alone	0 (0)	10 (4)	3 (4)	13 (4)
Other(s)	1 (4)	1 (0)	0 (0)	2 (1)
Not Reported	0 (0)	4 (2)	0 (0)	4 (1)
Donor type, no. (%)				
HLA identical sibling	11 (46)	87 (34)	19 (25)	117 (33)
Twin	0 (0)	1 (0)	1 (1)	2 (1)
Haploidentical donor	5 (21)	37 (15)	7 (9)	49 (14)
Other related	0 (0)	7 (3)	3 (4)	10 (3)
Well-matched unrelated (8/8)	6 (25)	52 (20)	27 (35)	85 (24)
Partially matched unrelated (7/8)	1 (4)	23 (9)	6 (8)	30 (8)
Mismatched unrelated (<= 6/8)	0 (0)	2 (1)	0 (0)	2 (1)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Multi-donor	0 (0)	0 (0)	1 (1)	1 (0)
Unrelated (matching cannot be determined)	0 (0)	22 (9)	2 (3)	24 (7)
Cord blood	1 (4)	24 (9)	11 (14)	36 (10)
Was post-HCT TKI therapy planned?, no. (%)				
No	0 (0)	246 (96)	30 (39)	276 (78)
Yes	24 (100)	0 (0)	47 (61)	78 (22)
Dasatinib	12	0	12	24
Imatinib mesylate (Gleevec, Glivec)	9	0	28	37
Nilotinib	0	0	3	3
Ponatinib	3	0	2	5
Bosutinib	0		1	
Other Therapy or therapy not reported	0 (0)	2	0 (0)	2 (1)
Was TKI therapy given for maintenance (ie reasons other than relapsed, persistent, or progressive disease?, no. (%)				
No	0 (0)	114 (45)	18 (23)	132 (37)
Yes	24 (100)	0 (0)	33 (43)	71 (20)
Bosutinib	0	0 (0)	1	1 (0)
Dasatinib (Sprycel)	19	0 (0)	20	39 (11)
Nilotinib (AMN107, Tasigna)	2	0 (0)	6	8 (2)
Ponatinib	5		3	
Other Therapy or therapy not reported	0 (0)	141 (55)	26 (34)	153 (43)
Transplant Year Grouping, no. (%)				
2008-2010	0 (0)	62 (24)	14 (18)	76 (21)
2011-2013	1 (4)	64 (25)	14 (18)	79 (22)
2014-2016	2 (8)	42 (16)	13 (17)	57 (16)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
2017-2019	9 (38)	43 (17)	13 (17)	65 (18)
2020-2022	12 (50)	32 (13)	15 (19)	59 (17)
2023	0 (0)	12 (5)	8 (10)	20 (6)
Follow-up of survivors, median (range), months	41.9 (14.8-92.9)	68.2 (0.03-193.8)	59.0 (3.3-190.9)	62.2 (0.03-193.8)

*TKI planned, TKI received: Includes patients with planned post-HCT TKI (on F2400) and reported receipt of post-HCT TKI (on F2450)

** TKI not planned, TKI not received: Includes patients without planned post-HCT therapy (on F2400) and did not receive post-HCT therapy (on F2450) and Patients that were planned to receive and received other types of therapy (non-TKI).

***Others: Includes patients with planned post-HCT TKI on F2400 but did not report post-HCT TKI on F2450 and vice versa i.e. patients that were not planned to receive post-HCT TKI on F2400 but reported post-HCT TKI on F2450.

Field	Response
Proposal Number	2508-09-CHAKRAVARTHY
Proposal Title	Outcomes Following Post Transplant Tyrosine Kinase Inhibitor (TKI) Maintenance Therapy in Pediatric Patients with Ph+ Acute Lymphoblastic Leukemia
Key Words	Tyrosine Kinase Inhibitors, pediatrics, Ph+ ALL, transplant
Principal Investigator #1: - First and last name, degree(s)	Rohini Chakravarthy MD, MPH
Principal Investigator #1: - Email address	rohini.chakravarthy@bsd.uchicago.edu
Principal Investigator #1: - Institution name	University of Chicago/Comer Children's Hospital
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 兾、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	Involved in writing a CIBMTR systematic review on female-specific late effects post hematopoietic stem cell transplant. I am the leader of the "sexual function" section.
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Pediatric Cancer
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	- Disease free survival (DFS) in patients who resumed a tyrosine kinase inhibitor (TKI) post-transplant vs those who did not - Overall Surivival (OS) in patients who resumed aTKI post-transplant vs those who did not - Cumulative incidence (CI) of relapse for patients who resumed TKI post-transplant vs those who did not - Cumulative incidence of transplant related mortality (TRM) in patients who resumed TKI post-transplant vs those who did not - CI and severity of acute and chronic graft versus host disease (GVHD) in those who received TKI post-transplant vs those who did not - CI of graft failure in in those who received TKI post-transplant vs in those who received TKI post-transplant vs those who did not
RESEARCH HYPOTHESIS:	We hypothesize that outcomes are comparable in patients who resumed a TKI post hematopoietic stem cell transplant (HSCT) to those who did not, with fewer toxicities.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary objectives: - DFS at 2-years in patients who received TKI post-transplant vs those who did not Secondary objectives: - OS and EFS at 2-years - CI of relapse at 2-years - CI of grade II-IV acute GVHD and severe acute GVHD (III-IV) - CI of limited and extensive chronic GVHD - Severe cGVHD free survival at 2-years - CI of viral reactivation, bacterial, and fungal infection post-HSCT
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Philadelphia chromosome positive (Ph+) acute lymphoblastic leukemia (ALL) comprises a rare subset of ALL in the pediatric population and generally confers a poor prognosis. Tyrosine kinase inhibitors (TKI)s are routinely incorporated into traditional chemotherapy regimens and have revolutionized outcomes. However, given the rarity of this leukemia and an even smaller proportion of these patients that proceed to hematopoietic stem cell transplant (HSCT), there is a paucity of data on the potential benefit of continued TKI use post HSCT. Comparing outcomes in patients post HSCT who received TKIs to those who did not receive TKIs as maintenance therapy will allow clinicians to be able to decide if post-transplant TKIs are beneficial without subjecting patients to unnecessary toxicities in the pediatric population. In addition, by further stratifying the results by pre transplant disease status, the specific type of TKI used, duration of TKI use, and donor choice, we may be able to better understand certain groups of patients who would have maximum benefit of a TKI post HCT or be at risk for more adverse events.

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.	While acute lymphoblastic leukemia (ALL) is the most common malignancy seen in pediatric patients, unlike in adults, Philadelphia chromosome positive (Ph+) ALL comprises a small subset (1). Outcomes have improved significantly since the addition of tyrosine kinase inhibitors (TKI)s to conventional chemotherapy (2,3); however; high-risk, relapsed and refractory disease continues to be challenging, warranting HSCT (4). The benefits of TKIs in the pre- transplant setting has been well established, but the use of TKI's post HSCT remains unclear. Various adult studies have tried to evaluate this with mixed results (5-7). Small single center pediatric studies have suggested possible benefits, but these are very difficult to generalize to a more heterogeneous population (6,8). Furthermore, TKIs are not without risks and have been associated with adverse effects such as infectious complications, cytopenias, hepatotoxicity, cardiotoxicity, and long term endocrine abnormalities such as growth restriction (9-16). Given that post HSCT relapse is the most common cause of treatment failure, it is essential to determine if there is a benefit to TKI use post HSCT while making sure to balance any adverse effects that may lead to increased morbidity and mortality. A CIBMTR based study will allow for a large, pediatric cohort to be analyzed to provide generalizable results.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria: Patients ages 0-30 years with FISH proven Ph+ALL who have received first HSCT between the years of 2010 and 2024 and documented in CIBMTR Exclusion criteria: Patients who received an HCT outside of above time period, outside of above age group, patients who did not have Ph+ ALL, but had documented use of a TKI
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.	Acute Lymphoblastic Leukemia Pre-InfusionRevision: 5.0 Acute Lymphoblastic Leukemia Post-InfusionRevision: 4.0 Fungal Infection Supplemental Data Pre-InfusionRevision: 5.0 Fungal Infection Supplemental Data Post-InfusionRevision: 4.0 Viral Infection Diagnostic and Treatment Post-InfusionRevision: 1.0 Donor Lymphocyte InfusionRevision: 1 Pre-Transplant Essential Data (Pre-TED)Revision: 10.0 Post-HSCT DataRevision: 8.0 Recipient Baseline DataRevision: 6.0
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)

Field	Response
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

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Field	Response
	doi:10.1016/j.idc.2020.02.008 10.Lipsky A, Lamanna N. Managing toxicities of Bruton tyrosine kinase inhibitors. Hematology. 2020;2020(1):336-345. doi:10.1182/hematology.2020000118 11. Tillman BF, Pauff JM, Satyanarayana G, Talbott M, Warner JL. Systematic review of infectious events with the Bruton tyrosine kinase inhibitor ibrutinib in the treatment of hematologic malignancies. Eur J Haematol. Apr 2018;100(4):325-334. doi:10.1111/ejh.13020 12. Jabbour E, Deininger M, Hochhaus A. Management of adverse events associated with tyrosine kinase inhibitors in the treatment of chronic myeloid leukemia. Leukemia. Feb 2011;25(2):201-10. doi:10.1038/leu.2010.215 13. B chade D, Chakiba C, Desjardin M, B couarn Y, Fonck M. [Hepatotoxicity of tyrosine kinase inhibitors: Mechanisms involved and practical implications]. Bull Cancer. Mar 2018;105(3):290-298. Toxicit h patique des inhibiteurs des tyrosines kinases : m canismes en cause et cons quences pratiques. doi:10.1016/j.bulcan.2017.11.015 14. Shah RR, Morganroth J, Shah DR. Hepatotoxicity of tyrosine kinase inhibitors: clinical and regulatory perspectives. Drug Saf. Jul 2013;36(7):491-503. doi:10.1007/s40264-013-0048-4 15. Heinzerling L, Eigentler TK, Fluck M, et al. Tolerability of BRAF/MEK inhibitor combinations: adverse event evaluation and management. ESMO Open. 2019;4(3)doi:10.1136/esmoopen-2019-000491 16. Cai J, Liu H, Chen Y, et al. Effect of the tyrosine kinase inhibitors on the growth in children with Philadelphia chromosome-positive acute lymphoblastic leukemia: a case-control study. Lancet Reg Health West Pac. Sep 2023;38:100818. doi:10.1016/j.lanwpc.2023.100818
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: Patients with Ph+ ALL and age <30 years who received Allo HCT in 2008-2023

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
No. of patients	102	952	369	1423
No. of centers	65	245	144	278
Patient age, median (range)	21.5 (2.2-29.8)	19.7 (0.4-30.0)	21.3 (1.1-30.0)	20.3 (0.4-30.0)
Age Groups, no. (%)				
<2 year	0 (0)	6 (1)	1 (0)	7 (0)
2-10 years	19 (19)	208 (22)	66 (18)	293 (21)
11 - <18 years	14 (14)	193 (20)	72 (20)	279 (20)
18 - <30 years	69 (68)	545 (57)	230 (62)	844 (59)
TED or RES (CRF) track determined for this event, no. (%)				
TED	101 (99)	748 (79)	318 (86)	1167 (82)
CRF(RES)	1 (1)	204 (21)	51 (14)	256 (18)
Race, no. (%)				
White	66 (65)	450 (47)	239 (65)	755 (53)
Black or African American	3 (3)	55 (6)	18 (5)	76 (5)
Asian	8 (8)	110 (12)	32 (9)	150 (11)
Native Hawaiian or other Pacific Islander	0 (0)	6 (1)	4 (1)	10 (1)
American Indian or Alaska Native	3 (3)	6 (1)	2 (1)	11 (1)
More than one race	0 (0)	13 (1)	8 (2)	21 (1)
Not reported	22 (22)	312 (33)	66 (18)	400 (28)
Ethnicity, no. (%)				
Hispanic or Latino	15 (15)	194 (20)	81 (22)	290 (20)
Non-Hispanic or Latino	53 (52)	386 (41)	188 (51)	627 (44)
Non-resident of the U.S.	32 (31)	362 (38)	93 (25)	487 (34)
Not reported	2 (2)	10 (1)	7 (2)	19 (1)
Karnofsky score prior to HCT, no. (%)				
90-100%	87 (85)	744 (78)	296 (80)	1127 (79)
<90%	14 (14)	192 (20)	66 (18)	272 (19)
Not reported	1 (1)	16 (2)	7 (2)	24 (2)
Graft Type, no. (%)				
BM	19 (19)	348 (37)	136 (37)	503 (35)
PBSC	77 (75)	479 (50)	203 (55)	759 (53)
UCB	6 (6)	125 (13)	30 (8)	161 (11)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Conditioning regimen intensity-Planned, no. (%)				
MAC	94 (92)	851 (89)	326 (88)	1271 (89)
RIC	2 (2)	32 (3)	19 (5)	53 (4)
NMA	4 (4)	30 (3)	15 (4)	49 (3)
Not reported	2 (2)	39 (4)	9 (2)	50 (4)
Conditioning regimen-Planned, no. (%)				
TBI/Cy	46 (45)	390 (41)	144 (39)	580 (41)
TBI/Cy/Flu	10 (10)	107 (11)	32 (9)	149 (10)
TBI/Cy/Flu/TT	0 (0)	1 (0)	4 (1)	5 (0)
TBI/Cy/TT	6 (6)	60 (6)	23 (6)	89 (6)
TBI/Cy/VP	2 (2)	25 (3)	16 (4)	43 (3)
TBI/VP	7 (7)	115 (12)	35 (9)	157 (11)
TBI/Mel	2 (2)	10 (1)	7 (2)	19 (1)
TBI/Flu	17 (17)	76 (8)	58 (16)	151 (11)
TBI/other(s)	2 (2)	6 (1)	4 (1)	12 (1)
Bu/Cy/Mel	0 (0)	2 (0)	1 (0)	3 (0)
Bu/Cy	2 (2)	56 (6)	12 (3)	70 (5)
Bu/Mel	0 (0)	2 (0)	1 (0)	3 (0)
Flu/Bu/TT	2 (2)	13 (1)	6 (2)	21 (1)
Flu/Bu	2 (2)	36 (4)	11 (3)	49 (3)
Flu/Mel/TT	3 (3)	13 (1)	2 (1)	18 (1)
Flu/Mel	0 (0)	10 (1)	5 (1)	15 (1)
Cy/Flu	0 (0)	1 (0)	1 (0)	2 (0)
Cy alone	0 (0)	5 (1)	1 (0)	6 (0)
Mel/other(s)	0 (0)	1 (0)	0 (0)	1 (0)
Treosulfan	1 (1)	6 (1)	1 (0)	8 (1)
TLI	0 (0)	0 (0)	2 (1)	2 (0)
Other(s)	0 (0)	12 (1)	3 (1)	15 (1)
Not reported	0 (0)	5 (1)	0 (0)	5 (0)
GVHD prophylaxis (Administered), no. (%)				
Ex-vivo T-cell depletion	7 (7)	26 (3)	3 (1)	36 (3)
CD34 selection	0 (0)	10 (1)	6 (2)	16 (1)
PtCy + other(s)	35 (34)	122 (13)	95 (26)	252 (18)
PtCy alone	2 (2)	3 (0)	2 (1)	7 (0)
TAC + MMF +- other(s) (except PtCy)	2 (2)	49 (5)	18 (5)	69 (5)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
TAC + MTX +- other(s) (except MMF, PtCy)	29 (28)	187 (20)	111 (30)	327 (23)
TAC + other(s) (except MMF, MTX, PtCy)	1 (1)	24 (3)	8 (2)	33 (2)
TAC alone	3 (3)	18 (2)	10 (3)	31 (2)
CSA + MMF +- other(s) (except PtCy,TAC)	6 (6)	69 (7)	28 (8)	103 (7)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	16 (16)	205 (22)	76 (21)	297 (21)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	0 (0)	13 (1)	4 (1)	17 (1)
CSA alone	1 (1)	146 (15)	4 (1)	151 (11)
Other(s)	0 (0)	49 (5)	1 (0)	50 (4)
Not Reported	0 (0)	31 (3)	3 (1)	34 (2)
Donor type, no. (%)				
HLA identical sibling	37 (36)	280 (29)	114 (31)	431 (30)
Twin	0 (0)	1 (0)	0 (0)	1 (0)
Haploidentical donor	22 (22)	122 (13)	69 (19)	213 (15)
Other related	0 (0)	24 (3)	14 (4)	38 (3)
Well-matched unrelated (8/8)	27 (26)	163 (17)	96 (26)	286 (20)
Partially-matched unrelated (7/8)	6 (6)	59 (6)	26 (7)	91 (6)
Mismatched unrelated (<= 6/8)	0 (0)	6 (1)	0 (0)	6 (0)
Multi-donor	1 (1)	8 (1)	4 (1)	13 (1)
Unrelated (matching cannot be determined)	3 (3)	165 (17)	17 (5)	185 (13)
Cord blood	6 (6)	123 (13)	29 (8)	158 (11)
Not reported	0 (0)	1 (0)	0 (0)	1 (0)
Is additional post-HCT therapy planned?, no. (%)				
No	0 (0)	681 (72)	187 (51)	868 (61)
Yes	102 (100)	66 (7)	180 (49)	348 (24)
Bosutinib	0 (0)	0 (0)	1 (0)	1 (0)
Dasatinib	38 (37)	0 (0)	14 (4)	52 (4)
Imatinib mesylate (Gleevec, Glivec)	28 (27)	0 (0)	109 (30)	137 (10)
Nilotinib	2 (2)	0 (0)	0 (0)	2 (0)
Ponatinib	19 (19)	0 (0)	9 (2)	28 (2)
Not reported	0 (0)	205 (22)	2 (1)	207 (15)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Specify other therapy:, no. (%)				
AZACYTIDINE	0 (0)	1 (0)	0 (0)	1 (0)
BLINATUMOMAB	0 (0)	1 (0)	0 (0)	1 (0)
BOSUTINIB	1 (1)	0 (0)	0 (0)	1 (0)
CHEMOTHERAPY - CYCLOPHOSPHAMIDE	0 (0)	1 (0)	0 (0)	1 (0)
CTLA4IG PRIMED DONOR LYMPHOCYTES	0 (0)	1 (0)	0 (0)	1 (0)
CTX	0 (0)	1 (0)	0 (0)	1 (0)
CYCLO/MESNA, TACROLIMUS CIV/MMF	0 (0)	0 (0)	1 (0)	1 (0)
CYCLOPHOSPHAMIDE	0 (0)	1 (0)	3 (1)	4 (0)
CYTOXAN	0 (0)	3 (0)	0 (0)	3 (0)
DASATANIB	0 (0)	2 (0)	0 (0)	2 (0)
DASATINIB	4 (4)	0 (0)	10 (3)	14 (1)
DASATNIB	0 (0)	1 (0)	0 (0)	1 (0)
DESATINIB	0 (0)	0 (0)	1 (0)	1 (0)
DISATINIB DUE TO SUPERIOR CNS PENETRATION.	0 (0)	0 (0)	1 (0)	1 (0)
IL-2	0 (0)	1 (0)	0 (0)	1 (0)
NILOTINIB	1 (1)	0 (0)	3 (1)	4 (0)
PLANNED TO CONTINUE GLEEVAC POST HSCT	0 (0)	0 (0)	1 (0)	1 (0)
PONATINIB	2 (2)	0 (0)	3 (1)	5 (0)
POSSIBLE TKI	0 (0)	0 (0)	1 (0)	1 (0)
POST CY	0 (0)	1 (0)	0 (0)	1 (0)
PREDNOL+ARA-C	0 (0)	1 (0)	0 (0)	1 (0)
SPRYCEL	0 (0)	4 (0)	0 (0)	4 (0)
TKI	2 (2)	0 (0)	2 (1)	4 (0)
TKI - UNKNOWN SPECIFIC DRUG	0 (0)	0 (0)	1 (0)	1 (0)
TKI THERAPY, NILOTINIB	1 (1)	0 (0)	0 (0)	1 (0)
TKI, DRUG UNSPECIFIED AT THIS TIME	1 (1)	0 (0)	0 (0)	1 (0)
TYROSINE KINASE INHIBITOR	0 (0)	0 (0)	1 (0)	1 (0)
TYROSINE KINASE INHIBITOR (TKI NOT YET SPECIFIED) THERAPY	1 (1)	0 (0)	0 (0)	1 (0)
TYROSINE KINASE INHIBITOR (TKI)	1 (1)	0 (0)	0 (0)	1 (0)
UNSPECIFIED TKI	1 (1)	0 (0)	0 (0)	1 (0)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
VENETOCLAX	1 (1)	0 (0)	0 (0)	1 (0)
Not reported	86 (84)	933 (98)	341 (92)	1360 (96)
Was therapy given for reasons other than relapsed, persistent, or progressive disease?, no. (%)				
No	0 (0)	301 (32)	50 (14)	351 (25)
Yes	102 (100)	43 (5)	231 (63)	376 (26)
Systemic therapy	102 (100)	37 (4)	230 (62)	369 (26)
Bosutinib	2 (2)	0 (0)	3 (1)	5 (0)
Dasatinib (Sprycel)	69 (68)	0 (0)	142 (38)	211 (15)
Nilotinib (AMN107, Tasigna)	8 (8)	0 (0)	11 (3)	19 (1)
Not reported	0 (0)	608 (64)	88 (24)	696 (49)
Specify other systemic therapy:, no. (%)				
ASCIMINIB	0 (0)	1 (0)	0 (0)	1 (0)
BLINATUMOMAB	0 (0)	1 (0)	0 (0)	1 (0)
BLINCYTO	1 (1)	0 (0)	0 (0)	1 (0)
INJ.VCR 2MG TAB DEXA 5MG	0 (0)	1 (0)	0 (0)	1 (0)
INOTUZUMAB	0 (0)	1 (0)	1 (0)	2 (0)
INTHRATECAL CHEMOTHERAPY	0 (0)	0 (0)	1 (0)	1 (0)
IT CHEMO	0 (0)	1 (0)	1 (0)	2 (0)
IT CHEMO, PONATINIB	0 (0)	0 (0)	1 (0)	1 (0)
IT MTX	1 (1)	4 (0)	0 (0)	5 (0)
IT MTX+ARAC+PREDNOL	0 (0)	1 (0)	0 (0)	1 (0)
METHOTREXATE	0 (0)	0 (0)	1 (0)	1 (0)
PEG INTERFERON	0 (0)	1 (0)	0 (0)	1 (0)
POMATINIB	0 (0)	1 (0)	0 (0)	1 (0)
PONATINIB	29 (28)	0 (0)	40 (11)	69 (5)
PONATINIB (ICLUSIG)	1 (1)	0 (0)	0 (0)	1 (0)
PONATINIB, ASCIMINIB	0 (0)	0 (0)	1 (0)	1 (0)
PONATINIB.	0 (0)	0 (0)	1 (0)	1 (0)
PONATNIB	0 (0)	0 (0)	1 (0)	1 (0)
PONTINIB	0 (0)	0 (0)	1 (0)	1 (0)
RUXOLITINIB	0 (0)	0 (0)	1 (0)	1 (0)
SCEMBLIX	0 (0)	0 (0)	1 (0)	1 (0)
TAB VEENAT 400MG OD	0 (0)	1 (0)	0 (0)	1 (0)
TIT	0 (0)	1 (0)	0 (0)	1 (0)
VENETOCLAX	1 (1)	1 (0)	0 (0)	2 (0)

Characteristic	TKI planned, TKI received*	TKI not planned, TKI not received**	Others***	Total
Not reported	69 (68)	937 (98)	318 (86)	1324 (93)
Transplant Year Grouping, no. (%)				
2008-2010	1 (1)	277 (29)	28 (8)	306 (22)
2011-2013	2 (2)	238 (25)	37 (10)	277 (19)
2014-2016	12 (12)	149 (16)	67 (18)	228 (16)
2017-2019	20 (20)	128 (13)	95 (26)	243 (17)
2020-2022	45 (44)	114 (12)	103 (28)	262 (18)
2023	22 (22)	46 (5)	39 (11)	107 (8)
Follow-up of survivors, median (range), months	36.4 (2.9-168.6)	46.6 (0.03-195.3)	49.9 (3.0-203.9)	47.6 (0.03-203.9)

*TKI planned, TKI received: Includes patients with planned post-HCT TKI (on F2400) and reported receipt of post-HCT TKI (on F2450)

** TKI not planned, TKI not received: Includes patients without planned post-HCT therapy (on F2400) and did not receive post-HCT therapy (on F2450) and Patients that were planned to receive and received other types of therapy (non-TKI).

***Others: Includes patients with planned post-HCT TKI on F2400 but did not report post-HCT TKI on F2450 and vice versa i.e. patients that were not planned to receive post-HCT TKI on F2400 but reported post-HCT TKI on F2450.

Field	Response
Proposal Number	2509-185-ZARNEGAR-LUMLEY
Proposal Title	Identifying the Clinical Impact of Germline Variants in Pediatric AML Requiring Hematopoietic Stem Cell Transplantation
Key Words	Germline variants, AML, HSCT
Principal Investigator #1: - First and last name, degree(s)	Sara Zarnegar-Lumley, MD, MS
Principal Investigator #1: - Email address	szarnegarlumley@luriechildrens.org
Principal Investigator #1: - Institution name	Ann & Robert H. Lurie Children's Hospital of Chicago
Principal Investigator #1: - Academic rank	Associate Professor of Pediatrics
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):	Jessica Pollard, MD
Principal Investigator #2 (If applicable): - Email address:)	jessica_pollard@dfci.harvard.edu
Principal Investigator #2 (If applicable): - Institution name:	Boston Children's Hospital / Dana Farber Cancer Institute
Principal Investigator #2 (If applicable): - Academic rank:	Associate Professor of Pediatrics
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:	Sara Zarnegar-Lumley
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:	Pediatric Cancer
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:	Larisa Broglie, MD

Field	Response
RESEARCH QUESTION:	Our research team is conducting research to identify germline variants (GV) in genes that increase risk of myeloid malignancy in pediatric patients with newly diagnosed acute myeloid leukemia (AML) who were enrolled on Children's Oncology Group (COG) upfront studies. We acknowledge that GV can increase the risk of AML development, but based on the affected gene, patients may also be more susceptible to short- and long-term treatment toxicity based on chromosomal and DNA fragility, infection susceptibility, organ vulnerability or at increased risk of subsequent malignant neoplasms. GV can affect therapeutic response by altering target expression or drug metabolism. In light of these issues, those with GV may benefit from hematopoietic stem cell transplant (HSCT) regardless of somatic mutational profile to eradicate the aberrant hematopoietic clone. When considering HSCT for patients with GV, one must consider optimal donor choice since family members may be affected by the same GV as the intended recipient, and the potential for increased toxicity with HSCT conditioning with certain GV. In our ongoing research, we have identified germline variants from two large clinically annotated databases from two primary sources of germline genomic data for pediatric patients with newly diagnosed AML enrolled on COG trials. From The Therapeutically Applicable Research to Generate Effective Treatments (TARGET) AML [phs000218.v26.p8.c1] and the Gabriella Miller Kids First (GMKF) [phs002187.v1.p1] cohorts, we have identified over 1000 germline variants that we are putting through our variant curation and annotation pipeline according to American College of Medical Genetics (ACMG) guidelines. Each of the clinically relevant germline variants will be linked to a patient specific ID assigned through COG. In the proposed CIBMTR research project, we aim to link patient specific IDs in the COG database to the same individual patient in the CIBMTR database. We propose in this study to understand the donor choice, conditioning regimens, survival and toxicity outcomes of HSCT in pediatric patients with AML and germline variants in clinically significant genes.

Field	Response
RESEARCH HYPOTHESIS:	We hypothesize that a proportion of pediatric patients treated for “de novo” AML in previous clinical trials had clinically unrecognized/undetected GV that may have conferred lower treatment response and increased treatment toxicity. We further hypothesize that a proportion of these patients may have had a related HSCT donor with a familial germline mutation.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary aim: Using genomic data from a large clinically annotated cohort, identify germline variants in clinically relevant genes in pediatric patients with newly diagnosed AML (GV-pAML). This aim is underway. Secondary aim: Describe the clinical characteristics at the time of HSCT, donor choice, conditioning regimens, transplant- and non-transplant-related morbidity and mortality (including data on infectious toxicity, cardiac toxicity, subsequent malignant neoplasm, graft-versus-host disease when available), event-free and overall survival in this cohort of GV-pAML.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	If our hypothesis is correct, we hope to provide justification for universal screening for germline variants in clinically significant genes for all pediatric patients diagnosed with AML, allowing for optimization of donor selection and treatment regimens, as well as genetic counseling for patients and related family members.

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.	In one of the first studies reporting on the prevalence of mutations in germline predisposition genes in childhood cancer, investigators found that 4.4% of 588 patients treated for leukemia had a pathogenic or likely pathogenic (P/LP) mutation [1]. More recent studies of smaller patient cohorts with expanded gene lists reported the prevalence of GV in pAML to be 11.8% - 19.4% [2-4], suggesting that previous prevalence might have been underestimated. Before considering universal evaluation for GV in pAML, it is first important to determine (1) the prevalence of GV in genes that predispose to pAML (GV-pAML) in a large clinically annotated cohort as well as compare (2) treatment response, survival outcomes and (3) treatment toxicity profiles for patients with GV-pAML versus children with AML that lack such variants (non-GV-pAML). Our proposed analysis will discern if children with GV-pAML have higher risk disease features, treatment toxicity, and/or decreased treatment response than those in the non-GV-pAML cohort and may inform the pediatric oncology community as to the value of universal evaluation for GV for those children enrolled on clinical trials for newly diagnosed AML. The evolution of clinical trial risk stratification for newly diagnosed AML reflects the emerging understanding of the somatic mutational landscape in those affected, while highlighting the lack of awareness of, or testing for, GV that predispose to AML. In historical COG clinical trials, patients were excluded if they had Fanconi anemia, Kostmann Syndrome, Shwachman-Diamond syndrome (SDS), any other known bone marrow failure syndrome (BMFS), treatment-related or secondary AML due to underlying myelodysplasia. Children with Trisomy 21 (constitutional or mosaic) were excluded outright in some trials, or restricted in enrollment to age >4 years [5,6]. The most recent study (NCT04293562; AAML1831) excluded the aforementioned diagnoses though did allow germline patients not expected to be at risk for increased treatment toxicity (e.g. germline RUNX1, GATA2, etc). Due to phenotypic variability and incomplete gene penetrance, pediatric patients may not have a known diagnosis of germline predisposition or iBMFS at the time that AML presents. Thus, it is likely that patients with GV were enrolled on previous clinical trials despite the intent to exclude such patients for most of such trials. For instance, SDS is an iBMFS with a variable phenotype that can be clinically unrecognized [8]. An analysis of the Center for International Blood and Marrow Transplant

	Research myelodysplastic syndrome database identified 4% of patients <40 years old who received an allogeneic HSCT with biallelic germline SBDS ribosome maturation factor (SBDS) mutations and no prior diagnosis of SDS [9]. Clinical outcomes for patients with SDS and myeloid malignancy are exceptionally poor due to therapy-resistance and treatment-related toxicities [10]. This example underscores the importance of our objective to describe how treatment response and toxicity were impacted by the presence of a GV that was undetected at the time of AML diagnosis and clinical trial enrollment. We will use two primary sources of germline genomic data for pediatric patients with newly diagnosed AML enrolled on COG trials. The Therapeutically Applicable Research to Generate Effective Treatments (TARGET) AML includes 200 patients with diagnosis and remission bone marrow [BM] pairs as well as 100 relapse specimens constituting trios. An additional 30 patients with induction failure (IF): >15% BM blasts after two induction cycles had a “trio” of specimens at diagnosis, a sample derived from BM fibroblasts (i.e. tissue source intended to represent germline), and a BM sample at end of induction. The Gabriella Miller Kids First (GMKF) cohort includes pediatric patients with newly diagnosed AML enrolled on AAML103112 from which we anticipate 750 diagnosis/remission pairs, 150 with an additional relapse specimen to constitute a trio. Thus, in our combined cohort, we expect data from 980 pAML patients with germline sequencing available for analysis. Preliminary Data Analyses of WGS data from 365 patients with pAML conducted by members of our extended research team: Harmon L. and Triche Jr, T et al., provide an estimate of P/LP GV in genetic loci implicated in hematologic malignancy (HM), cancer predisposition syndromes, iBMFS, and primary immunodeficiency. This analysis included the TARGET-21 cohort (n=29) for whom WGS was performed on BM-derived fibroblasts and the GMKF cohort (n=336) for whom WGS was performed on BM pairs at diagnosis/relapse and remission. GV were defined as any variant surpassing a variant allele frequency (VAF) of 0.3 at each timepoint. P/LP status of GV was determined using the ACMG guidelines [11]. P/LP variants classified as “established” variants in genes previously well-defined to be associated with heritable HM or iBMFS were detected in 1.9% of cases, lower than reported in the literature. Notably, Harmon L. et al., identified a higher

	frequency of GV in "provocative" variants in genes where emerging evidence suggests a role in leukemia risk (e.g., 5 P/LP variants in CHEK2, a gene involved in DNA damage response and reported as a GV predisposing to AML in adults19). Provocative variants will benefit from review of clinical annotation and adjudication by our research team to determine clinical relevance in pAML. From this multidisciplinary review, we aim to provide a clinically meaningful prevalence to inform practical screening guidelines. Additional preliminary analysis found those with P/LP GVs in established genes had a relatively short EFS (relapse/death). However, with the inclusion of 29 TARGET-21 cases, this data may be enriched for high-risk features. This preliminary data provides a pipeline for analysis and the foundation for the proposed project. In this project, we will increase our sample size to include 980 patients and focus our analysis on clinically relevant genes with established risk for myeloid malignancy. We will include clinical annotation to further analyze outcomes in the context of a patient's assigned risk stratification and treatment allocation. Prior analyses from COGAAML0531 described treatment-related toxicities including early-onset cardiotoxicity and prolonged neutropenia [12,13]. Getz et al., reported a 12% cumulative incidence of cardiotoxicity at 5-year follow-up associated with significant reduction in survival in patients with versus without cardiotoxicity [13]. A subset (n=41) were evaluated for variants in cardiomyopathy-predisposing genes, only detected in a small number (7.3%) suggesting these variants cannot fully explain cardiotoxicity suspectiblity.22 In Gerbing et al.'s analysis, lower telomere count was significantly associated with prolonged neutropenia after later chemotherapy courses [14]. Polymorphisms in telomere maintenance genes associated with telomere biology disorders (TBD) were only detected within the expected VAF of self-described patient race without enrichment in delayed versus expected count recovery groups [14]. Beyond TBD, those with other BMFS can have shorter telomeres than unaffected individuals [15], underscoring the importance of analyzing toxicity in a broader germline context. These studies highlight interindividual variability in treatment toxicity but did not evaluate toxicity in the context of all clinically relevant GV. Our analysis might better identify subsets of GV-pAML at higher risk of toxicity. None of the previous studies have linked patient data from the time of diagnosis with AML

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	through completion of HSCT nor considered the potential role of germline variants in donor choice. Our study will expand our understanding of the clinical impact of germline variants in clinically relevant genes through the continuum from diagnosis through HSCT.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Pediatric patients with AML enrolled on COG AAML0531 and AAML1031 with a patient specific ID that can be linked to a CIBMTR ID. Patients with germline variants in previously identified clinically relevant genes from the TARGET AML and Gabriella Miller Kids First
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.	Will utilize AML Pre-infusion 2010R4, AML Post-infusion 2110R4, HSCT Infusion 2006R6, Infection data forms, subsequent neoplasms. We expect that that patient cohort we identify with germline variants in pediatric AML who proceeded to HSCT will be small and this will be an exploratory analysis with use of descriptive statistics.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
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.	1) Genomic cohorts described in proposal (TARGET and GMKF) as well as COG Clinical Trial Databased 2) Neither databased contains all the data required to answer the study question: how do germline variants affect AML treatment including transplant toxicity and outcomes.

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	syndrome and myelodysplastic syndrome or acute myeloid leukaemia: a multicentre, retrospective, cohort study. Lancet Haematol. Mar 2020;7(3):e238-e246. doi:10.1016/S2352-3026(19)30206-6 11. Richards S, Aziz N, Bale S, et al. Standards and guidelines for the interpretation of sequence variants: a joint consensus recommendation of the American College of Medical Genetics and Genomics and the Association for Molecular Pathology. Genet Med. May 2015;17(5):405-24. doi:10.1038/gim.2015.30 12. Getz KD, Sung L, Ky B, et al. Occurrence of Treatment-Related Cardiotoxicity and Its Impact on Outcomes Among Children Treated in the AAML0531 Clinical Trial: A Report From the Children's Oncology Group. J Clin Oncol. Jan 1 2019;37(1):12-21. doi:10.1200/JCO.18.00313 13. Garcia-Pavia P, Kim Y, Restrepo-Cordoba MA, et al. Genetic Variants Associated With Cancer Therapy-Induced Cardiomyopathy. Circulation. Jul 2 2019;140(1):31-41. doi:10.1161/circulationaha.118.037934 14. Gerbing RB, Alonzo TA, Sung L, et al. Shorter Remission Telomere Length Predicts Delayed Neutrophil Recovery After Acute Myeloid Leukemia Therapy: A Report From the Children's Oncology Group. J Clin Oncol. Nov 1 2016;34(31):3766-3772. doi:10.1200/JCO.2016.66.9622 15. Alter BP, Giri N, Savage SA, Rosenberg PS. Telomere length in inherited bone marrow failure syndromes. Haematologica. Jan 2015;100(1):49-54. doi:10.3324/haematol.2014.114389
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

Table1: Patients with AML and age <30 years who received Allo HCT in 2008-2023

Characteristic	N (%)
No. of patients	9796
No. of centers	367
Patient age, median (range)	18.3 (0.3-30.0)
Age Groups, no. (%)	
<2 year	618 (6)
2-10 years	2148 (22)
11 - <18 years	2027 (21)
18 - <30 years	5003 (51)
TED or RES (CRF) track determined for this event, no. (%)	
TED	7311 (75)
CRF(RES)	2485 (25)
Race, no. (%)	
White	6167 (63)
Black or African American	723 (7)
Asian	846 (9)
Native Hawaiian or other Pacific Islander	58 (1)
American Indian or Alaska Native	67 (1)
More than one race	231 (2)
Not reported	1704 (17)
Ethnicity, no. (%)	
Hispanic or Latino	1483 (15)
Non-Hispanic or Latino	5713 (58)
Non-resident of the U.S.	2450 (25)
Not reported	150 (2)
Karnofsky score prior to HCT, no. (%)	
90-100%	7490 (76)
<90%	2064 (21)
Not reported	242 (2)
Graft Type, no. (%)	
BM	3553 (36)
PBSC	4878 (50)
UCB	1363 (14)
Not reported	2 (0)
Conditioning regimen intensity-Planned, no. (%)	
MAC	8384 (86)
RIC	726 (7)

Characteristic	N (%)
NMA	219 (2)
Not Reported	467 (5)
Conditioning regimen-Planned, no. (%)	
TBI/Cy	972 (10)
TBI/Cy/Flu	644 (7)
TBI/Cy/Flu/TT	42 (0)
TBI/Cy/TT	73 (1)
TBI/Cy/VP	73 (1)
TBI/VP	74 (1)
TBI/Mel	127 (1)
TBI/Flu	628 (6)
TBI/other(s)	53 (1)
Bu/Cy/Mel	241 (2)
Bu/Cy	3203 (33)
Bu/Mel	348 (4)
Flu/Bu/TT	380 (4)
Flu/Bu	2062 (21)
Flu/Mel/TT	195 (2)
Flu/Mel	296 (3)
Cy/Flu	20 (0)
Cy alone	12 (0)
Mel alone	8 (0)
Mel/other(s)	49 (1)
Treosulfan	125 (1)
Carb/other(s)	1 (0)
TLI	2 (0)
Other(s)	137 (1)
Not reported	31 (0)
GVHD prophylaxis (Administered), no. (%)	
Ex-vivo T-cell depletion	316 (3)
CD34 selection	197 (2)
PtCy + other(s)	1612 (16)
PtCy alone	37 (0)
TAC + MMF +- other(s) (except PtCy)	727 (7)
TAC + MTX +- other(s) (except MMF, PtCy)	2560 (26)
TAC + other(s) (except MMF, MTX, PtCy)	243 (2)
TAC alone	139 (1)
CSA + MMF +- other(s) (except PtCy,TAC)	1008 (10)

Characteristic	N (%)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	2268 (23)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	144 (1)
CSA alone	334 (3)
Other(s)	124 (1)
Not Reported	87 (1)
Donor type, no. (%)	
HLA identical sibling	2698 (28)
Twin	15 (0)
Haploidentical donor	1563 (16)
Other related	272 (3)
Well-matched unrelated (8/8)	2467 (25)
Partially-matched unrelated (7/8)	656 (7)
Mismatched unrelated (<= 6/8)	45 (0)
Multi-donor	37 (0)
Unrelated (matching cannot be determined)	697 (7)
Cord blood	1344 (14)
Not reported	2 (0)
Transplant Year Grouping, no. (%)	
2008-2010	1765 (18)
2011-2013	1857 (19)
2014-2016	1793 (18)
2017-2019	1906 (19)
2020-2022	1803 (18)
2023	672 (7)
Follow-up of survivors, median (range), months	59.7 (0.03-199.0)