Not for publication or presentation

A G E N D A
CIBMTR WORKING COMMITTEE FOR NON-MALIGNANT DISEASES WORKING COMMITTEE
Salt Lake City, UT
Friday, February 6, 2026, 1:00 – 3:00 PM (MT)

Co-Chair:	**Ashish Gupta, MD, MPH; University of Minnesota, Minneapolis, MN; Telephone: 612-626-2691; Email: gupta461@umn.edu**
Co-Chair:	**Carmem Bonfim, MD, PhD; Hospital de Clínicas - Federal University of Parana, Curitiba, Brazil; Telephone: 55- 41-999388444; E-mail: carmembonfim@gmail.com**
Co-Chair:	**Kasiani Myers, MD; Cincinnati Children's Hospital Medical Center, Cincinnati, OH Telephone: 513-636-4943; E-mail: kasiani.myers@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:	**Soyoung Kim, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-8271; E-mail: skim@mcw.edu**
Statistician:	**Yongzi Yu, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-3694; E-mail: yoyu@mcw.edu**
Page Scholar:	**Jane Koo, MD, MA, BA; Cincinnati Children's Hospital Medical Center, Cincinnati, OH; Telephone: 408-824-4881; E-mail: jane.koo@cchmc.org**

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1. **Introduction**

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

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

3. **Presentations, Published or Submitted papers**

   a. **NM16-03b**: Gale RP, Hinterberger W, Young NS, Gennery AR, Dvorak CC, Hebert KM, Heim M, Broglie L, Eapen M. What causes aplastic anaemia? **Leukemia. 2023 Jun 1; 37(6):1191-1193. doi:10.1038/s41375-023-01892-2. Epub 2023 Apr 27. PMC10353698.**
   b. **NM19-01:** Nakamura R, Patel BA, Kim S, Wong FL, Armenian SH, Groarke EM, Kessler DA, Hebert KM, Heim M, Eapen M, Young NS. **Conditional survival and standardized mortality ratios of patients with severe aplastic anemia surviving at least one year after hematopoietic cell transplantation or immunosuppressive therapy. Haematologica. 2023 Dec 1; 108(12):3298-3307. doi:10.3324/haematol.2023.282781. Epub 2023 Jun 1. PMC10690917.**
   c. **NM20-01** Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa). ***Oral Presentation, ASH 2024.***

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

a. **NM15-01** Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria (A Saad/H Abdel-Azim/J Bloomer). **Manuscript Preparation.**
b. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with **HLH** (A Horne/KS Baker/K Beutel). **Protocol Development.**
c. **NM18-01** Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease (A Prakash/D Wall/K Paulson). **Protocol Development.**
d. **AC18-02** Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis (G Georges/K Sullivan). **Manuscript Preparation.**
e. **NM20-01** Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa). **Manuscript Preparation.**
f. **NM22-01** Outcomes after second or greater allogeneic stem cell transplants in patients with severe aplastic anemia: A contemporary analysis (H Rangarajan/P Satwani). **Protocol**
g **Development.**
NM23-01 Impact of conditioning intensity and donor type on outcomes in patients with severe aplastic anemia undergoing upfront or salvage hematopoietic stem cell transplant (A Rayes/ S Otoukesh/ R Nakamura/ M Pulsipher). **Protocol Development.**
h. **NM24-01** The outcomes of PTCY based GVHD prophylaxis for allogeneic stem cell transplantation in patients with severe aplastic anemia patients who lack a HLA-matched sibling donor (N Khaire/ L Gowda/ A Mirza/ R Kumar/ B Ball). **Protocol Development.**
i. **NM24-02** Impact of somatic mutations in aplastic anemia after allogeneic stem cell transplantation (B Ball/ R Nakamura). **Protocol Development.**
j. **NM25-01** Outcomes after Allogeneic Hematopoietic Stem Cell Transplant in Diamond-Blackfan Anemia: A contemporary CIBMTR analysis (J Koo/ N Gloude/ N Hossain/ P Munshi/ H Rangarajan/ N Shah). **Protocol Pending.**

5. Future/proposed studies

a. **PROP 2504-03** Outcomes with Sickle Cell Trait Donors Compared to Non-Sickle Cell Trait Donors (A Gupta/ S Modi) (**Attachment 4**)
b. **PROP 2509-44; 2509-201** Evolution of Hematopoietic Cell Transplantation Outcomes in Inherited Platelet Disorders: A CIBMTR Cohort Study. (D Citla-Sridhar/ J Dalal/ B Alanazi/ T Truong) (**Attachment 5**)
c. **PROP 2509-130** Outcomes of autologous transplantation in Crohn's disease (E Ayala) (**Attachment 6**)
d. **PROP 2509-219** Outcomes of Older sibling donor Vs. Younger alternate donor allogeneic HCT in adults with severe Aplastic Anemia (S Manjappa) (**Attachment 7**)
e. **PROP 2509-224** Outcomes of Allogeneic Hematopoietic Cell Transplantation for Acquired Pure Red Cell Aplasia (S Park) (**Attachment 8**)

Proposed studies; not accepted for consideration at this time

f. **PROP 2509-68** Optimal donor option for patients with non-malignant pediatric disorders (C Martinez). ***Dropped due to low scientific impact and need to focus the population.***
g. **PROP 2509-181** Efficacy and toxicity of treosulfan based reduced intensity conditioning regimens in inherited bone marrow failure syndromes (C Roy/ R Stubbins). ***Dropped due to overlap with current study (BMT-CTN 1904).***
h. **PROP 2509-197** Myeloid Malignancy Risk in Sickle Cell Disease After Curative Therapy (M-M Sainvil/ S Hong). ***Dropped due to overlap with current study (LE17-01, LE16-01)/publication.***

i. **PROP 2509-204** Post-transplant cyclophosphamide (PTCy) GVHD prophylaxis in HLA-matched, -mismatched and haploidentical donor transplant in adults with severe aplastic anemia (P Attaphol). ***Dropped due to overlap with current study/publication (NM24-01).***

6. Other business

MINUTES
CIBMTR WORKING COMMITTEE FOR NON-MALIGNANT DISEASES WORKING COMMITTEE
Honolulu, HI
Friday, February 14, 2025, 1:00 – 3:00 PM HST

Co-Chair:	**Ashish Gupta, MD, MPH; University of Minnesota, Minneapolis, MN; Telephone: 612-626-2691; Email: gupta461@umn.edu**
Co-Chair:	**Carmem Bonfim, MD, PhD; Hospital de Clínicas - Federal University of Parana, Curitiba, Brazil; Telephone: 55- 41-999388444; E-mail: carmembonfim@gmail.com**
Co-Chair:	**Kasiani Myers, MD; Cincinnati Children's Hospital Medical Center, Cincinnati, OH Telephone: 513-636-4943; E-mail: kasiani.myers@cchmc.org**
Page Scholar:	**Brian Ball, MD; City of Hope, Duarte, CA; E-mail: brball@coh.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:	**Soyoung Kim, PhD; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-955-8271; E-mail: skim@mcw.edu**
Statistician:	**Yongzi Yu, MS; CIBMTR® (Center for International Blood and Marrow Transplant Research), Milwaukee, WI; Telephone: 414-805-0700; E-mail: yoyu@mcw.edu**

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3. **Presentations, Published or Submitted papers**

   NM20-01 Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa). **Presented at ASH 2024.**

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

   a. **NM15-01** Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria (A Saad/H Abdel-Azim/J Bloomer). **Manuscript Preparation.**
   b. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with **HLH** (A Horne/KS Baker/K Beutel). **Protocol Development.**
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f. **NM22-01** Outcomes after second or greater allogeneic stem cell transplants in patients with severe aplastic anemia: A contemporary analysis (H Rangarajan/P Satwani). **Protocol**

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NM23-01 Impact of conditioning intensity and donor type on outcomes in patients with severe aplastic anemia undergoing upfront or salvage hematopoietic stem cell transplant (A Rayes/ S Otoukesh/ R Nakamura/ M Pulsipher). **Protocol Development.**

h. **NM24-01** The outcomes of PTCY based GVHD prophylaxis for allogeneic stem cell transplantation in patients with severe aplastic anemia patients who lack a HLA-matched sibling donor (N Khaire/ L Gowda/ A Mirza/ R Kumar/ B Ball). **Protocol Development.**

i. **NM24-02** Impact of somatic mutations in aplastic anemia after allogeneic stem cell transplantation (B Ball/ R Nakamura). **Protocol Development.**

5. Proposed studies

a. **PROP 2408-01; 2409-26; 2410-217** Outcomes after Allogeneic Hematopoietic Stem Cell Transplant in Diamond-Blackfan Anemia: A contemporary CIBMTR analysis (J Koo/ N Gloude/ N Hossain/ P Munshi/ H Rangarajan/ N C Shah) (Attachment 4)

- *Presented by Dr. Nicholas Gloude*
- ***Hypothesis:*** *Reduced-intensity conditioning (RIC) regimens provide comparable overall survival to myeloablative conditioning (MAC) regimens for patients with Diamond Blackfan Anemia*
- ***Objectives:*** *Estimate two-year overall survival, evaluate the impact of donor type, and determine the incidence of HSCT-related complications.*

b. **PROP 2409-17; 2410-218; 2410-251** Outcomes of Autologous Hematopoietic Stem Cell Transplantation in Multiple Sclerosis (E Ayala/ M Iqbal/ S Mirza/ T Nishihori/ S Hosahalli Vasanna/ J Dalal) (Attachment 5)

- *Presented by Dr. Smitha Vasanna.*
- ***Hypothesis:*** *The intensity of conditioning regimen (myeloablative vs. non-myeloablative) has no impact on post auto transplant outcomes for MS*
- ***Objectives:*** *Compare progression-free survival, assess health-related quality of life, and evaluate secondary outcomes such as overall survival and second malignancies.*

c. **PROP 2410-11** Impact of mixed chimerism post stem cell transplantation on the long term outcome of patients with Fanconi anemia (M Ayas) (Attachment 6)

- *Presented by Dr. Mouhab Ayas.*
- ***Hypothesis:*** *Mixed chimerism post-transplant for Fanconi Anemia has no harmful effect on outcomes compared to full donor chimerism.*
- ***Objectives:*** *Determine the impact of mixed chimerism on GVHD, graft failure, incidence of MDS/AML, and solid malignancies.*

d. **PROP 2410-246** The Impact of Donor-derived Clonal Hematopoietic Mutations in children and patients less than 18 years of age following allogeneic Hematopoietic Stem Cell Transplantation for non-hematological and non-malignant hematological Conditions (M Kulasekaran/ G Hildebrandt) (Attachment 7)

- *Presented by Dr. Monika Kulasekaran.*
- ***Hypothesis:*** *Comprehensive molecular profiling and risk assessment of donors are necessary.*
- ***Objectives:*** *Evaluate the prevalence of donor clonal hematopoiesis mutations and correlate their impact on long-term outcomes, include secondary malignancies.*

Proposed studies; not accepted for consideration at this time

e. **PROP 2410-27** Effect of age, donor source and preparative regimen on outcome of hematopoietic cell transplantation in patients with Glanzmann Thrombasthenia (D Citla-Sridhar/ J Dalal). ***Dropped due to small sample size.***

f. **PROP 2410-56** Effect of mixed host-donor chimerism on graft failure/rejection after hematopoietic cell transplantation for non-malignant hematological disorders (A Lipsitt/ A Sharma). ***Dropped due to heterogeneous population and overlap with current study.***

g. **PROP 2410-160** Post-transplant cyclophosphamide vs. TCR αβ/CD19 deplete Haploidentical Transplant in Inborn Errors of Immunity: A CIBMTR Analysis (H Rangarajan/ M Albert). ***Dropped due to supplemental data needed.***

h. **PROP 2410-169** Evaluating hematopoietic cell transplant outcomes in patients with HbSC sickle cell disease: A CIBMTR Study (S Hosahalli Vasanna/ J Dalal). ***Dropped due to small sample size.***

i. **PROP 2410-192** Evaluating Outcomes of Autologous Hematopoietic Cell Transplantation in patients with Severe Systemic Sclerosis (Scleroderma) in the contemporary era (M Iqbal/ E Ayala). ***Dropped due to overlap with current study.***

j. **PROP 2410-205** Real-world gene therapy experience for sickle cell disease and comparing it with allogeneic stem cell transplant for toxicities (K Chetlapalli/ L Gowda). ***Dropped due to small sample size.***

k. **PROP 2410-212** Allogeneic transplant outcomes in Vexas Syndrome (S R Cingam/ J M Lewis-Gonzalez). ***Dropped due to small sample size.***

l. **PROP 2410-233** Evaluating outcomes of Hematopoietic Cell Transplantation in VEXAS syndrome (H Murthy/ Y Moreno Vanegas). ***Dropped due to small sample size.***

m. **PROP 2410-264** Transplant-Related Outcomes in Patients Undergoing Allogeneic Stem Cell Transplant vs. Gene Therapy for Sickle Cell Disease and Thalassemia (M Gallogly/ L Metheny). ***Dropped due to small sample size.***

6. Other business

Allogeneic Transplants for Immune Deficiencies reported to the CIBMTR from 2000-2025

Characteristic	TED N	CRF N	Total
No. of patients	4985	3284	8269
No. of centers	180	79	185
Disease, no. (%)			
Immune Deficiencies (ID)	146 (3)	33 (1)	179 (2)
SCID ADA deficiency:	80 (2)	133 (4)	213 (3)
SCID absence of T and B cells:	166 (3)	204 (6)	370 (4)
SCID absence of T, normal B cell SCID:	159 (3)	249 (8)	408 (5)
Omenn syndrome:	90 (2)	105 (3)	195 (2)
Reticular dysgenesis:	9 (0)	11 (0)	20 (0)
MHC Class II Deficiency (Bare lymphocyte syndrome)	114 (2)	42 (1)	156 (2)
SCID, NOS:	163 (3)	179 (5)	342 (4)
SCID other, specify:	246 (5)	374 (11)	620 (7)
Wiskott Aldrich syndrome:	362 (7)	343 (10)	705 (9)
DiGeorge anomaly:	9 (0)	8 (0)	17 (0)
Chronic granulomatous disease:	457 (9)	382 (12)	839 (10)
Chediak-Higashi syndrome:	66 (1)	31 (1)	97 (1)
Common variable immunodef:	89 (2)	35 (1)	124 (1)
SAP deficiency (XIAP-1)	131 (3)	73 (2)	204 (2)
Leukocyte adhesion deficiencies:	69 (1)	53 (2)	122 (1)
Kostmann agranulocytosis:	127 (3)	57 (2)	184 (2)
Neutropenia with combined immune deficiency (MKL1 deficiency, Actin deficiency)	1 (0)	0 (0)	1 (0)
Cartilage hair hypoplasia:	55 (1)	29 (1)	84 (1)
Immune deficiency plus neutropenia	1 (0)	0 (0)	1 (0)
CD40 ligand deficiency:	96 (2)	29 (1)	125 (2)
Griscelli syndrome type 2:	19 (0)	12 (0)	31 (0)
Combined immunodef dis (CID), NOS:	5 (0)	7 (0)	12 (0)
CID other, specify:	1 (0)	16 (0)	17 (0)
Other immunodeficiencies, specify:	865 (17)	225 (7)	1090 (13)
Histiocytic disorder	34 (1)	8 (0)	42 (1)
FELH Familial erythrohemophagocytic lymphohis:	899 (18)	446 (14)	1345 (16)
Langerhans Cell Histiocytosis:	67 (1)	34 (1)	101 (1)
Hemophagocytosis:	120 (2)	83 (3)	203 (2)

Characteristic	TED N	CRF N	Total
Malignant histiocytosis:	16 (0)	2 (0)	18 (0)
Other histiocytic disord:	60 (1)	56 (2)	116 (1)
SCID, T- B+ NK-, JAK3 mutation (2411)	5 (0)	0 (0)	5 (0)
SCID, T- B+ NK-, IL2RG mutations, X-linked SCID (2412)	29 (1)	4 (0)	33 (0)
SCID, T- B- NK+, RAG 1/2 deficiency (2413)	30 (1)	3 (0)	33 (0)
SCID, T- B- NK+, DCLRE1C (Artemis) deficiency (2414)	10 (0)	1 (0)	11 (0)
SCID, T- B- NK-, NOS (2415)	3 (0)	1 (0)	4 (0)
DOCK8 Deficiency (2416)	23 (0)	0 (0)	23 (0)
ZAP-70 deficiency (2417)	6 (0)	0 (0)	6 (0)
NEMO Deficiency Syndrome (2418)	3 (0)	0 (0)	3 (0)
Activated PI3 Kinase Delta Deficiency Syndrome (APDS1 or PIK3CD) (2419)	5 (0)	0 (0)	5 (0)
XIAP-2 deficiency (2420)	4 (0)	0 (0)	4 (0)
Autoimmune Lymphoproliferative Syndrome (ALPS) (2422)	3 (0)	1 (0)	4 (0)
CTLA4 deficiency (2423)	7 (0)	1 (0)	8 (0)
IPEX, Immune Dysregulation Polyendocrinopathy, enteropathy X-linked (FOXP3 deficiency) (2424)	5 (0)	0 (0)	5 (0)
LRBA Deficiency (2425)	6 (0)	1 (0)	7 (0)
STAT3 Gain of Function (2426)	3 (0)	0 (0)	3 (0)
GATA2 deficiency (2427)	17 (0)	0 (0)	17 (0)
STAT1 Gain of Function (2428)	8 (0)	2 (0)	10 (0)
Familial Hemophagocytic Lymphohistiocytosis, Perforin deficiency (FHL2) (2511)	24 (0)	1 (0)	25 (0)
Familial Hemophagocytic Lymphohistiocytosis, UNC13D (FHL3) (2512)	26 (1)	5 (0)	31 (0)
Familial Hemophagocytic Lymphohistiocytosis, STX11 (FHL4) (2513)	4 (0)	0 (0)	4 (0)
Familial Hemophagocytic Lymphohistiocytosis, STXBP2 (FHL5) (2514)	16 (0)	2 (0)	18 (0)
Familial Hemophagocytic Lymphohistiocytosis, no mutation identified (2515)	9 (0)	3 (0)	12 (0)
Familial Hemophagocytic Lymphohistiocytosis, other mutations (2516)	17 (0)	0 (0)	17 (0)

Allogeneic Transplants for Inborn Errors of Metabolism reported to the CIBMTR from 2000-2025

Characteristic	TED N	CRF N	Total
No. of patients	1240	1184	2424
No. of centers	90	57	102
Disease, no. (%)			
Inherited disorders of metabolism, NOS	23 (2)	3 (0)	26 (1)
Osteopetrosis	266 (21)	145 (12)	411 (17)
Lesch-Nyhan(HGPTR defic)	0 (0)	2 (0)	2 (0)
Neuronal ceroid lipofuscinosis	3 (0)	5 (0)	8 (0)
Other inherited metabolism disorders, specify	85 (7)	44 (4)	129 (5)
Mucopolysaccharidosis, NOS	18 (1)	7 (1)	25 (1)
IH Hurler syndrome	256 (21)	379 (32)	635 (26)
IS Scheie syndrome	1 (0)	1 (0)	2 (0)
II Hunter syndrome	27 (2)	26 (2)	53 (2)
III Sanfillippo	7 (1)	26 (2)	33 (1)
IV Morquio	6 (0)	1 (0)	7 (0)
VI Maroteaux-Lamy	23 (2)	26 (2)	49 (2)
VII B-glucuronidase deficiency	3 (0)	1 (0)	4 (0)
V Mucopolysaccharidosis	8 (1)	3 (0)	11 (0)
Other mucopolysaccharidosis	1 (0)	3 (0)	4 (0)
Mucolipidoses, NOS	1 (0)	3 (0)	4 (0)
Gaucher disease	10 (1)	4 (0)	14 (1)
Metachromatic leukodystrophy(MLD)	107 (9)	88 (7)	195 (8)
Adrenoleukodystrophy(ALD)	274 (22)	272 (23)	546 (23)
Globoid leukodystrophy/Krabbe disease	54 (4)	92 (8)	146 (6)
Neiman-Pick disease	11 (1)	12 (1)	23 (1)
I-cell disease	9 (1)	15 (1)	24 (1)
Wolman disease	8 (1)	6 (1)	14 (1)
Glucose storage disease	1 (0)	0 (0)	1 (0)
Hereditary diffuse leukoencephalopathy with spheroids (HDLS)	1 (0)	0 (0)	1 (0)
Other mucolipidoses	0 (0)	1 (0)	1 (0)
Asparty1 glucosaminuria	3 (0)	1 (0)	4 (0)
Fucosidosis	3 (0)	6 (1)	9 (0)
Mannosidosis	31 (3)	12 (1)	43 (2)

Allogeneic Transplants for non-malignant disorders reported to the CIBMTR from 2000-2025

Characteristic	TED N	CRF N	Total
No. of patients	10791	8984	19775
No. of centers	234	211	280
Disease, no. (%)			
PNH Proxysmal nocturnal hemoglobinuria:	274 (3)	206 (2)	480 (2)
NHL diffuse, large B-cell:	0 (0)	1 (0)	1 (0)
Severe aplastic anemia	555 (5)	27 (0)	582 (3)
SAA idiopathic:	5478 (51)	4926 (55)	10404 (53)
SAA secondary to hepatitis:	296 (3)	224 (2)	520 (3)
SAA secondary to toxin-other:	73 (1)	92 (1)	165 (1)
Amegakaryocytosis(not congenital):	19 (0)	19 (0)	38 (0)
Schwachmann-Diamond:	42 (0)	36 (0)	78 (0)
Acquired Pure Red Cell Aplasia:	73 (1)	56 (1)	129 (1)
Telomere Biology Disorders including Dyskeratosis congenita (DKC1, TERT, TERC, and other mutations)	37 (0)	43 (0)	80 (0)
Other acquired cytopenic syndrome, specify:	178 (2)	164 (2)	342 (2)
Inherited abnormal of erythrocyte differ.	8 (0)	10 (0)	18 (0)
Fanconi anemia:	728 (7)	771 (9)	1499 (8)
Diamond-Blackfan anemia (pure red cell aplasia):	192 (2)	148 (2)	340 (2)
Acquired AA secondary to chemotherapy (313)	5 (0)	2 (0)	7 (0)
Acquired AA secondary to immunotherapy or immune effector cell therapy (314)	3 (0)	7 (0)	10 (0)
Other constitutional anemia (not THALs):	114 (1)	64 (1)	178 (1)
Thalassemia, NOS:	1206 (11)	487 (5)	1693 (9)
Type B+ Thalassemia major	6 (0)	1 (0)	7 (0)
Type B0 Thalassemia major	0 (0)	1 (0)	1 (0)
Sickle Thalassemia major:	44 (0)	70 (1)	114 (1)
Sickle cell anemia:	1006 (9)	926 (10)	1932 (10)
Beta thalassemia major:	399 (4)	660 (7)	1059 (5)
Other hemoglobinopathy, specify:	55 (1)	43 (0)	98 (0)

Autologous Transplants for autoimmune diseases reported to the CIBMTR from 2000-2025

Characteristic	TED N	CRF N	Total
No. of patients	2058	125	2183
No. of centers	62	17	64
Disease, no. (%)			
Autoimmune disease unclassified	24 (1)	0 (0)	24 (1)
Myasthenia gravis	24 (1)	1 (1)	25 (1)
Multiple sclerosis	1415 (69)	59 (47)	1474 (68)
Rheumatoid arthritis	8 (0)	3 (2)	11 (1)
Psoriatic arthritis or psoriasis	0 (0)	1 (1)	1 (0)
Systemic lupus erythematosis (SLE)	51 (2)	9 (7)	60 (3)
Polymyositis-dermatomyositis	6 (0)	0 (0)	6 (0)
System Scleroderma	334 (16)	39 (31)	373 (17)
Other vasculitis	1 (0)	0 (0)	1 (0)
Antiphospholipid syndrome	9 (0)	0 (0)	9 (0)
Other autoimmune disease, specify	29 (1)	0 (0)	29 (1)
Other arthritis, spec	2 (0)	0 (0)	2 (0)
Other Connective tissue dis	10 (0)	0 (0)	10 (0)
Churg-Strauss	1 (0)	0 (0)	1 (0)
Behcets Syndrome	4 (0)	0 (0)	4 (0)
JIA systemic	2 (0)	0 (0)	2 (0)
JIA Other, specify	2 (0)	0 (0)	2 (0)
Other neuro disorder, spec	62 (3)	7 (6)	69 (3)
ITP- Idiopathic thrombocytopenic purpura	2 (0)	2 (2)	4 (0)
Hemolytic anemia	1 (0)	0 (0)	1 (0)
Evan syndrome	1 (0)	0 (0)	1 (0)
Crohns disease	68 (3)	3 (2)	71 (3)
Other bowel disorder, spec	1 (0)	1 (1)	2 (0)
Diabetes mellitustype I	1 (0)	0 (0)	1 (0)

CIBMTR®
A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

TO: Non-Malignant Diseases Working Committee Members

FROM: Larisa Broglie, MD, MS; Scientific Director for the Non-Malignant Diseases Working Committee

RE: 2025-2026 Studies in Progress Summary

NM15-01: Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria (A Saad/H Abdel-Azim/J Bloomer) The aim of the study is to describe the population of children or adults with Erythropoietic Porphyria who have undergone HCT and examine the outcomes post-transplant. Analysis has been completed. **Manuscript has been prepared by our EBMT colleagues and being prepared for submission.**

Status: **Manuscript Preparation**

NM17-01: Late effects after hematopoietic stem cell transplantation in patients with HLH (N Bhatt/KS Baker/R Marsh/J Talano) The purpose of this study is to investigate the long-term outcomes and late effects of patients with hemophagocytic lymphohistiocytosis (HLH) who are survivors after hematopoietic cell transplantation (HCT). The main hypothesis is that HLH survivors will be at risk for significant long term medical and neuropsychological late effects, which is dependent upon pre-transplant disease related factors and post-transplant chimerism. **Protocol development is underway**.

Status: **Protocol Development**

NM18-01: Impact of choice of serotherapy in pediatric stem cell transplantation for Sickle Cell Disease (A Prakash/D Wall/K Paulson) The purpose of this study is to compare outcomes following allogeneic HCT for pediatric patients with non-malignant disease based on the specific serotherapy used. Based on recent feedback and discussion with the PIs, the population has been updated to focus this analysis on patients receiving HCT for Sickle Cell Disease and will compare between patients given alemtuzumab and patients given ATG (rabbit or horse). The primary outcome has been refined and will focus on graft failure, with secondary outcomes including overall survival, acute and chronic GVHD, and graft-failure free survival. **Protocol development is underway and demographics tables are being finalized.**

Status: **Protocol Development**

AC18-02: Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis (G Georges/K Sullivan) The objective of this study is to explore patient characteristics and post-transplant outcomes of patients undergoing autologous transplant for systemic sclerosis. Supplemental data collected and analysis complete. Study presented in 2021, and **Manuscript is being prepared for submission.**

Status: **Manuscript Preparation**

NM20-01: Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa) This study aims to assess the impact of prognostic factors and describe the outcomes of patients undergoing transplant for Fanconi anemia, including overall survival, non-relapse mortality, and acute and chronic GVHD. Additionally, the study's goal is to obtain information on late effects including the rate of solid tumors and the association with radiation and GVHD. The study has completed analysis and the results presented at the American Society of Hematology Conference. **Manuscript Preparation is underway.**

Status: **Manuscript Preparation**

NM25-01 Outcomes after Allogeneic Hematopoietic Stem Cell Transplant in Diamond-Blackfan Anemia: A contemporary CIBMTR analysis (J Koo/ N Gloude/ N Hossain/ P Munshi/ H Rangarajan/ N Shah). The purpose of this study is to evaluate survival outcomes of patients with Diamond-Blackfan Anemia (DBA) receiving allogeneic HSCT and assess the impact of donor type and conditioning intensity on transplant outcomes. We will be working with the DBA Registry to try to incorporate pre-HCT disease information as well. **Protocol development and planning for collaboration with DBAR is underway.**

Status: **Protocol Pending**

Aplastic Anemia Studies:
A master database of all patients receiving allogeneic HCT for aplastic anemia has been generated to help guide the studies noted below. Significant work has gone into protocol development and data cleaning and categorization. **We are refining the populations and outcomes for each study and will be circulating the Protocols to the Working Committee for feedback soon**.

NM22-01: Outcomes after second or greater allogeneic stem cell transplants in patients with severe aplastic anemia: A contemporary analysis (H Rangarajan/P Satwani) This study aims to evaluate outcomes of a contemporary cohort of patients with aplastic anemia who received second allogeneic transplantation for graft failure.

NM23-01: Impact of conditioning intensity and donor type on outcomes in patients with severe aplastic anemia undergoing upfront or salvage hematopoietic stem cell transplant (R Ahmad/O Salman). The study has 2 aims: 1) to assess rates of graft failure in patients receiving upfront alternative donor transplant, and 2) to assess outcomes using increased intensity regimens (Flu/Cy/ATG/TBI) for mismatched (related and unrelated) alternative donor transplantation.

NM24-01: The outcomes of PTCY based GVHD prophylaxis for allogeneic stem cell transplantation in patients with severe aplastic anemia patients who lack a HLA-matched sibling donor. (N Khaire/ L Gowda/ A Mirza/ R Kumar/ B Ball). The study has 2 aims: 1）: Assessing outcomes of PTCY in SAA across all donor types (Haploidentical, MUD, MMUD) 2): Comparing outcomes of haplo-PTCY transplants for SAA with MUD transplants for SAA.

NM24-02: Impact of somatic mutations in aplastic anemia after allogeneic stem cell transplantation (B Ball/ R Nakamura). The study has 2 aims: 1) To evaluate if the presence of somatic mutation detected in AA recipients is associated with overall survival after alloHCT 2) To evaluate if the presence of high-risk somatic mutations (DNMT3A, ASXL1, TP53, RUNX1, CSMD1) detected in AA recipients is associated with overall survival after alloHCT.

Field	Response
Proposal Number	2504-03-GUPTA
Proposal Title	Outcomes with Sickle Cell Trait Donors Compared to Non-Sickle Cell Trait Donors
Principal Investigator #1: - First and last name, degree(s)	Ashish Gupta, MD
Principal Investigator #1: - Email address	gupta461@umn.edu
Principal Investigator #1: - Institution name	University of MInnesota
Principal Investigator #1: - Academic rank	Attending Physician
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):	Shivani Modi, MD
Principal Investigator #2 (If applicable): - Email address:)	sym297@jefferson.edu
Principal Investigator #2 (If applicable): - Institution name:	Jefferson Einstein Healthcare Network
Principal Investigator #2 (If applicable): - Academic rank:	Resident Physician
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
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:	Shivani Modi
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	Yes, I am a junior investigator and would like assistance identifying a senior mentor for my project
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Graft vs. Host Disease
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No

Field	Response
RESEARCH QUESTION:	Primary Objective: To compare overall survival (OS) and disease-free survival (DFS) in recipients of SCT donors vs. non-SCT donors. Secondary Objectives: To evaluate engraftment kinetics, incidence of GVHD, and transplant-related mortality (TRM) in SCT vs. non-SCT donor recipients. To assess the effect of donor SCT status on relapse rates and non-relapse mortality (NRM). To explore associations between donor SCT status and post-transplant complications (e.g., thromboembolic events, endothelial dysfunction).
RESEARCH HYPOTHESIS:	Sickle cell trait (SCT) is a common hemoglobin variant, particularly among individuals of African descent. While SCT is generally considered benign, recent studies suggest potential implications in the context of hematopoietic stem cell donation. The impact of SCT donors on transplant outcomes, such as engraftment, graft-versus-host disease (GVHD), and overall survival, remains underexplored. This study aims to assess transplant-related outcomes in recipients of SCT donors compared to recipients of non-SCT donors using CIBMTR registry data.
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Data Collection and Variables Primary Outcomes: OS, DFS. Secondary Outcomes: Engraftment (neutrophil and platelet recovery), incidence of acute and chronic GVHD, TRM, NRM, post-transplant complications. Covariates: Age, sex, disease type, transplant conditioning regimen, HLA matching, donor type, GVHD prophylaxis.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Data Collection and Variables Primary Outcomes: OS, DFS. Secondary Outcomes: Engraftment (neutrophil and platelet recovery), incidence of acute and chronic GVHD, TRM, NRM, post-transplant complications. Covariates: Age, sex, disease type, transplant conditioning regimen, HLA matching, donor type, GVHD prophylaxis.

Field	Response
SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	Completion of this study will provide critical insights into the safety and effectiveness of using donors with sickle cell trait (SCT) for allogeneic hematopoietic stem cell transplantation (allo-HSCT). As the demand for suitable donors continues to grow particularly among populations disproportionately affected by sickle cell disease and related conditions understanding whether SCT status impacts transplant outcomes is essential for guiding donor selection. The findings could directly influence clinical decision-making by informing transplant teams about any potential risks or benefits associated with SCT donors, ultimately expanding the donor pool without compromising patient safety. Scientifically, this research will address a gap in the literature regarding donor hemoglobinopathy traits and transplant outcomes, helping to refine existing guidelines and potentially improve access to curative therapies for historically underserved populations.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion Criteria: o Adult and pediatric patients who received allogeneic HSCT. o Donors with confirmed SCT status (SCT-positive and SCT-negative). o Availability of key outcome variables (OS, DFS, GVHD, engraftment, relapse). Exclusion Criteria: o Insufficient donor SCT status data. o Missing follow-up data on transplant outcomes.
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	We are looking into adult population only in this study. If study is positive, we might further dive into pediatric population.

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion-variables to be considered in the multivariate analyses. Outline any supplementary data required.	For this study, we will request a comprehensive dataset from the CIBMTR that includes key patient, disease, donor, and infusion-related variables. Patient-level variables of interest include age at transplant, sex, race/ethnicity, performance status (such as Karnofsky or Lansky scores), pre-existing comorbidities (including HCT-CI scores), transplant number (first vs. subsequent), and pre-transplant organ function assessments. Donor-specific data will include age, sex, race/ethnicity, relationship to the recipient (e.g., matched sibling or unrelated), HLA match status, CMV serostatus, and most importantly, donor hemoglobinopathy status to differentiate between sickle cell trait (HbAS) and non-trait (HbAA) donors. Disease-related variables will include primary diagnosis, disease status at the time of transplant (e.g., remission status or active disease), and prior treatments such as chemotherapy or transfusions. For transplant and infusion-related data, we will collect information on the conditioning regimen intensity (myeloablative vs. reduced-intensity), graft source (bone marrow vs. peripheral blood), GVHD prophylaxis regimen, total nucleated cell count (TNC), CD34+ stem cell dose, and the date of cell infusion. Outcome measures will include time to neutrophil and platelet engraftment, incidence and severity of acute and chronic GVHD, graft failure, relapse (for malignant conditions), transplant-related mortality, overall and disease-free survival, cause of death, and healthcare utilization metrics such as hospital length of stay and ICU admission if available. This robust dataset will allow for comprehensive comparative analysis between SCT and non-SCT donor outcomes following allo-HSCT.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)

REFERENCES:	1) Naik RP, Haywood C Jr. Sickle cell trait diagnosis: clinical and social implications. Hematology Am Soc Hematol Educ Program. 2015;2015(1):160-7. doi: 10.1182/asheducation-2015.1.160. PMID: 26637716; PMCID: PMC4697437. 2) Piel FB, Steinberg MH, Rees DC. Sickle Cell Disease. N Engl J Med. 2017 Apr 20;376(16):1561-1573. doi: 10.1056/NEJMra1510865. PMID: 28423290. 3) D'Souza A, Fretham C, Lee SJ, Arora M, Brunner J, Chhabra S, Devine S, Eapen M, Hamadani M, Hari P, Pasquini MC, Perez W, Phelan RA, Riches ML, Rizzo JD, Saber W, Shaw BE, Spellman SR, Steinert P, Weisdorf DJ, Horowitz MM. Current Use of and Trends in Hematopoietic Cell Transplantation in the United States. Biol Blood Marrow Transplant. 2020 Aug;26(8):e177-e182. doi: 10.1016/j.bbmt.2020.04.013. Epub 2020 May 11. Erratum in: Transplant Cell Ther. 2022 Oct;28(10):715-716. doi: 10.1016/j.jtct.2021.02.006. PMID: 32438042; PMCID: PMC7404814. 4) Walters MC, Patience M, Leisenring W, Eckman JR, Buchanan GR, Rogers ZR, Olivieri NE, Vichinsky E, Davies SC, Mentzer WC, Powars D, Scott JP, Bernaudin F, Ohene-Frempong K, Darbyshire PJ, Wayne A, Roberts IA, Dinndorf P, Brandalise S, Sanders JE, Matthews DC, Appelbaum FR, Storb R, Sullivan KM. Barriers to bone marrow transplantation for sickle cell anemia. Biol Blood Marrow Transplant. 1996 May;2(2):100-4. PMID: 9118298. 5) Copelan EA, Chojecki A, Lazarus HM, Avalos BR. Allogeneic hematopoietic cell transplantation; the current renaissance. Blood Rev. 2019 Mar;34:34-44. doi: 10.1016/j.blre.2018.11.001. Epub 2018 Nov 8. PMID: 30467067. 6) Sorror ML, Storb RF, Sandmaier BM, Maziarz RT, Pulsipher MA, Maris MB, Bhatia S, Ostronoff F, Deeg HJ, Syrjala KL, Estey E, Maloney DG, Appelbaum FR, Martin PJ, Storer BE. Comorbidity-age index: a clinical measure of biologic age before allogeneic hematopoietic cell transplantation. J Clin Oncol. 2014 Oct 10;32(29):3249-56. doi: 10.1200/JCO.2013.53.8157. Epub 2014 Aug 25. PMID: 25154831; PMCID: PMC4178523. 7) Arora M, Nagaraj S, Wagner JE, Barker JN, Brunstein CG, Burns LJ, Defor TE, McMillan ML, Miller JS, Weisdorf DJ. Chronic graft-versus-host disease (cGVHD) following unrelated donor hematopoietic stem cell transplantation (HSCT): higher response rate in recipients of unrelated donor (URD) umbilical cord blood (UCB). Biol Blood Marrow Transplant. 2007 Oct;13(10):1145-52. doi:

Field	Response
	10.1016/j.bbmt.2007.06.004. Epub 2007 Jul 27. PMID: 17889350.
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

proposal 2504-03: Outcomes with Sickle Cell Trait Donors Compared to Non-Sickle Cell Trait Donors

Characteristic	Total
No. of patients	1488
Recipient Age, no. (%)	
Median (range)	12.5 (1.0-65.4)
<13	787 (53)
13+	701 (47)
TED or CRF track, no. (%)	
CRF (RES)	1488 (100)
Primary disease, no. (%)	
Hemoglobinopathies	1488 (100)
Disease, no. (%)	
Sickle cell anemia	1488 (100)
Donor type, no. (%)	
HLA identical sibling	763 (51)
Twin	2 (0)
Haploidentical donor	292 (20)
Other related	90 (6)
Well-matched unrelated (8/8)	141 (9)
Partially-matched unrelated (7/8)	47 (3)
Mismatched unrelated (<= 6/8)	2 (0)
Multi-donor	20 (1)
Unrelated (matching cannot be determined)	11 (1)
Cord blood	120 (8)
Graft source, no. (%)	
Bone Marrow	1058 (71)
Peripheral Blood	308 (21)
Cord Blood	116 (8)
Not reported	6 (0)
Donor sickle cell status, no. (%)	
Yes	547 (37)
No	385 (26)
Not reported	556 (37)
Conditioning regimen, no. (%)	
MAC	
TBI/Cy/Flu	2 (0)
TBI/Cy/Flu/TT	200 (13)
TBI/Flu	1 (0)

Characteristic	Total
Bu/Cy	335 (23)
Flu/Bu/TT	96 (6)
Flu/Bu	84 (6)
Flu/Mel/TT	104 (7)
Cy/Flu	5 (0)
Mel/other(s)	1 (0)
Other(s)	2 (0)
RIC/NMA	
TBI/Cy/Flu	81 (5)
TBI/Mel	1 (0)
TBI/Flu	3 (0)
TBI/other(s)	97 (7)
Bu/Cy	2 (0)
Flu/Bu	210 (14)
Flu/Mel	199 (13)
Cy/Flu	1 (0)
Treosulfan	2 (0)
Other(s)	2 (0)
TBD	
TBI/Flu	2 (0)
TBI/other(s)	8 (1)
Flu/Bu	3 (0)
Treosulfan	43 (3)
Other(s)	3 (0)
Missing	1 (0)
Country, no. (%)	
USA	964 (65)
Argentina	1 (0)
Australia	7 (0)
Belgium	1 (0)
Brazil	35 (2)
Canada	57 (4)
Denmark	5 (0)
United Kingdom	17 (1)
India	17 (1)
Israel	1 (0)
Netherlands	1 (0)
New Zealand	1 (0)

Characteristic	Total
Saudi Arabia	373 (25)
Sweden	6 (0)
Mexico	1 (0)
Turkey	1 (0)
Year of Transplant, no. (%)	
2008-2014	229 (15)
2015-2023	1259 (85)

Study title: Evolution of Hematopoietic Cell Transplantation Outcomes in Inherited Platelet Function Disorders: A CIBMTR Cohort Study

Investigators:

Divyaswathi Citla-Sridhar, MD, MS (Lead Principal Investigator)

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- Academic Rank: Associate Professor
- Junior Investigator Status (≤5 years from fellowship): Yes

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- Institution: Alberta Children's Hospital, University of Calgary
- Academic Rank: Associate Professor, Pediatric Oncology
- Junior Investigator Status: No

CURRENT ONGOING WORK WITH CIBMTR: NONE

Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months? NO

PROPOSAL

Research question:

What are the safety and efficacy outcomes of hematopoietic cell transplantation (HCT) in patients with Glanzmann Thrombasthenia (GT) and Bernard Soulier Syndrome (BSS) and how have these outcomes evolved over time with advances in donor selection, conditioning regimens, transplant practices and availability of recombinant factor VIIa (rFVIIa).

Research hypothesis:

We hypothesize that outcomes following allogeneic HCT for GT and BSS have improved over time and that younger age at transplant, optimized donor selection, and contemporary conditioning approaches are associated with improved survival and reduced transplant-related morbidity. We hypothesize that HCT outcomes in patients with GT have significantly improved following the FDA approval of rFVIIa in 1999.

Specific objectives:

Primary Objectives

1. To evaluate overall survival (OS) at 100 days, 1 year, 3 years, and 5 years following first allogeneic HCT in patients with GT and BSS.
2. To evaluate event-free survival (EFS) over the same time intervals.

Secondary Objectives

1. To assess transplant-related mortality (TRM) and non-relapse mortality (NRM).
2. To evaluate rate of engraftment, including days to neutrophil recovery, rate of graft failure (primary and secondary), and days to platelet recovery.
3. To assess the incidence and severity of acute and chronic GVHD.
4. To evaluate GVHD-free, disease-free survival.
5. To identify risk factors for adverse outcomes, including age, donor type, graft source, conditioning intensity, and transplant era.

Scientific impact:

GT and BSS are rare inherited platelet function disorders associated with severe, recurrent bleeding and substantial morbidity. While advances in supportive care and bleeding management have improved short-term outcomes for both disorders, allogeneic HCT remains the only established curative therapy for patients with severe disease refractory to conventional treatments. Large registry-based analyses evaluating contemporary outcomes are lacking. There is limited systematic data examining how advances in bleeding management—such as the introduction of recombinant factor VIIa (rFVIIa) in 1999 for GT—as well as improvements in donor selection, conditioning regimens, graft sources, and GVHD prophylaxis have influenced transplant outcomes over time. Completion of these aims will provide robust, up-to-date outcome data on allogeneic HCT in patients with inherited platelet disorders using the CIBMTR registry. This study will evaluate survival, transplant-related mortality, and key post-transplant complications, while identifying clinical and transplant-related factors associated with favorable and adverse outcomes. This

research will fill a critical knowledge gap by moving beyond anecdotal experience to provide evidence derived from a large, international dataset. The findings will directly inform clinical decision-making, improve counseling of patients and families regarding risks and benefits of HCT, and help refine patient selection and transplant strategies for both GT and BSS. Ultimately, this study will play a pivotal role in optimizing care and improving long-term outcomes for patients with these rare inherited platelet disorders.

Scientific justification

Allogeneic HCT has been used for GT and, less frequently, BSS for several decades, primarily in patients with severe, transfusion-refractory bleeding. Published experience consists largely of case reports, small case series, and limited retrospective reviews demonstrating correction of the underlying platelet defect, durable engraftment, and resolution of bleeding symptoms using a range of donor sources, graft types, and conditioning regimens. However, these studies are constrained by very small sample sizes, heterogeneous transplant practices, and inconsistent reporting of transplant-related outcomes, limiting their ability to guide evidence-based clinical decision-making. Data specific to BSS remains particularly sparse.

Importantly, HCT in inherited platelet disorders presents unique and substantial risks that require careful patient selection. Paradoxically, severe bleeding may occur during the peri-transplant period, particularly in the setting of thrombocytopenia, mucosal injury from conditioning regimens, or the presence of anti-platelet antibodies. Life-threatening hemorrhage has been reported during routine procedures such as tunneled central venous catheter placement. In addition, acute and chronic graft-versus-host disease may occur, necessitating prolonged immunosuppression. Full-intensity conditioning regimens are associated with significant long-term toxicities, including infertility, growth impairment, and secondary malignancies, while reduced-intensity conditioning—although less toxic—carries an increased risk of graft rejection. These competing risks underscore the need for a clearer understanding of outcomes to appropriately balance potential cure against procedure-related morbidity.

Preliminary data from the Center for International Blood and Marrow Transplant Research (CIBMTR) indicate that a substantially larger number of patients with inherited platelet disorders have undergone allogeneic HCT than is reflected in the published literature. A preliminary review identified 64 patients with GT or BSS who underwent first allogeneic HCT across 30 international centers, with transplants spanning multiple eras and incorporating diverse donor types, graft sources, and conditioning regimens. Early outcomes appear favorable, with a 100-day overall survival of 95.2%. Despite this, these data remain unpublished and have not been systematically analyzed to evaluate predictors of outcome, transplant-related complications, or temporal trends in practice.

In contrast, analyses using other large national datasets highlight the limitations of non–transplant-focused data sources in capturing curative therapies for rare platelet disorders. In a cohort of 965 patients with GT identified within the EPIC Cosmos database, fewer than 10 individuals were documented as having undergone HCT. Similarly, the ATHN Glanzmann Thrombasthenia Registry

does not currently include patients who have received HCT. Together, these findings demonstrate that transplant experience in this population is largely invisible outside of the CIBMTR.

Given the unique risks associated with HCT in GT and BSS—including peri-transplant bleeding, alloimmunization, graft-versus-host disease, and long-term toxicities of conditioning regimens—there is a compelling need for a rigorous, transplant-focused evaluation using a comprehensive and modern dataset designed to capture these outcomes. The CIBMTR, with its standardized data collection, longitudinal follow-up, and international scope, represents the only feasible platform to comprehensively assess HCT outcomes in these disorders. Systematic analysis and dissemination of these data are essential to move beyond anecdotal experience and provide the rare disease community with evidence needed to inform clinical decision-making, patient counseling, and future therapeutic development.

PARTICIPANT SELECTION CRITERIA:

Patient Eligibility Population:

Age:	All ages
Disease:	Glanzmann Thrombasthenia, Bernard Soulier Syndrome
Disease stage/status at transplant:	Any
Year of Transplant:	1981-present
Transplant Type:	Allogeneic

Exclusion criteria: None

Domain	**Variables Collected**
Demographics	Age at transplant (continuous) Sex (male, female) Race Ethnicity Geographic region of transplant center
Disease-Specific Variables	Diagnosis (GT vs BSS) Underlying genetic mutation (when available) Severity of bleeding diathesis (frequency of severe bleeding episodes) Pre-transplant transfusion dependency History of alloimmunization / platelet refractoriness
Baseline Clinical Status	Performance status (Lansky/Karnofsky) Comorbidities (including infectious disease status, pre-existing organ dysfunction) Hematopoietic Cell Transplantation–Specific Comorbidity Index (HCT-CI)
Donor Characteristics	Donor type: – Matched sibling donor (MSD) – Matched unrelated donor (MUD) – Mismatched unrelated donor (MMUD)

Domain	Variables Collected
	– Haploidentical donor Donor/recipient CMV serostatus HLA matching at A, B, C, DRB1, DQB1
Graft Characteristics	Stem cell source: – Bone marrow (BM) – Peripheral blood stem cells (PBSC) – Cord blood (CB) Cell dose: – Total nucleated cells (TNC) – CD34+ cell count – CD3+ cell count
Conditioning Regimen	Conditioning intensity: – Myeloablative (MAC) – Reduced-intensity (RIC) – Non-myeloablative (NMA) Specific agents and doses used Use of total body irradiation (TBI vs no TBI)
GVHD Prophylaxis	GVHD prophylaxis regimen (e.g., cyclosporine, methotrexate, tacrolimus, post-transplant cyclophosphamide) Use of T-cell depletion (in vivo or ex vivo)
Post-Transplant Outcomes	Time to neutrophil engraftment Time to Platelet Engraftment Graft failure (primary, secondary) Acute GVHD (incidence and grade) Chronic GVHD (incidence and severity)
Laboratory and Chimerism Data	Platelet counts post-transplant Platelet function studies (when available) Chimerism data
Outcome Measures	Overall survival Event-free survival Treatment-related mortality (TRM) Day +100 survival Survival status at 1 year and 5 years Cause of death (TRM vs other)

Sample Requirements: n/a

Non-CIBMTR Data Source:
We have had preliminary discussions with EBMT regarding the feasibility of a combined dataset, although no definitive plans or commitments have been made. We are also considering using a combined dataset with Pediatric Health Information System® (**PHIS**) database.

References:

1. Smith AB, et al. Hematopoietic stem cell transplantation as a cure for a pediatric patient with Glanzmann's thrombasthenia. J Pediatr Hematol Oncol. 2018.
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combined proposal 2509-44/2509-201: Evolution of Hematopoietic Cell Transplantation Outcomes in Inherited Platelet Disorders: A CIBMTR Cohort Study.

Characteristic	Total
No. of patients	40
Recipient Age, no. (%)	
Median (range)	9.9 (0.9-53.1)
<18	32 (80)
18+	8 (20)
TED or CRF track, no. (%)	
TED	29 (73)
CRF (RES)	11 (28)
Disease, no. (%)	
Glanzmann thrombasthenia	40 (100)
Donor type, no. (%)	
HLA identical sibling	17 (43)
Other related	2 (5)
Well-matched unrelated (8/8)	11 (28)
Partially-matched unrelated (7/8)	1 (3)
Unrelated (matching cannot be determined)	1 (3)
Cord blood	8 (20)
Conditioning regimen, no. (%)	
MAC	
Bu/Cy	23 (58)
Flu/Bu/TT	3 (8)
Flu/Bu	2 (5)
Flu/Mel/TT	1 (3)
RIC/NMA	
TBI/Cy	1 (3)
TBI/Cy/Flu	1 (3)
Flu/Bu	1 (3)
Flu/Mel	7 (18)
TBD	
Flu/Bu	1 (3)
Graft source, no. (%)	
Bone Marrow	26 (65)
Peripheral Blood	6 (15)
Cord Blood	8 (20)
Country, no. (%)	
USA	29 (73)

Characteristic	Total
Australia	2 (5)
Saudi Arabia	1 (3)
Pakistan	8 (20)
Year of Transplant, no. (%)	
2008-2014	12 (30)
2015-2023	28 (70)

Field	Response
Proposal Number	2509-130-AYALA
Proposal Title	Outcomes of autologous transplantation in Crohn's disease
Key Words	Crohn's disease
Principal Investigator #1: - First and last name, degree(s)	Ernesto Ayala
Principal Investigator #1: - Email address	ayala.ernesto@mayo.edu
Principal Investigator #1: - Institution name	Mayo Clinic Florida
Principal Investigator #1: - Academic rank	Associate Professor of Medicine
Junior investigator status (defined as 㛃、5 years from fellowship)	No
Do you identify as an underrepresented/minority?	Yes
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	Submitted proposal on VEXAS syndrome to leukemia working party
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Non-Malignant Diseases
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	Autologous transplantation (AutoHCT) has been used to reset the immune response on patients with refractory autoimmune disease. The largest amount of data is on Scleroderma and Multiple Sclerosis. In patients with Crohn's disease, autoHCT is able to induce durable remissions free of therapy with improvement in the clinical manifestations of the disease and quality of life.
RESEARCH HYPOTHESIS:	AutoHCT reestablishes tolerance in patients with refractory Crohn's disease.
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Primary outcome is CR rate. Secondary outcomes are OS, PFS, relapse, non-relapse mortality, initiation of Crohn's therapy, infections.
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.	AutoHCT has been used to induce remission in patients with refractory Crohn's disease for the past 15 years. However, the collective volume of data has not been analyzed. Analyzing these results is important to establish the efficacy of this approach and describe associated risks. This would inform Gastroenterology and Cellular Therapies specialists to discuss with prospective patients. It would also be the base to make improvements in autoHCT for Crohn's disease by improving results and decreasing morbidity. It will also help select the best candidates for the treatment.
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion: Any patient with refractory Crohn's disease that has undergone autoHCT and has been reported to CIBMTR. Exclusion: Other inflammatory bowel diseases.
Does this study include pediatric patients?	Yes

Field	Response
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion- variables to be considered in the multivariate analyses. Outline any supplementary data required.	Crohn's specific outcomes (relapse, new therapies, late morbidity) may be missing from CIBMTR forms. As very few centers in the country perform autoHCT for Crohn's disease, a short survey with outcomes may be sent to the identified centers and treating specialists.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A description of the hypothesis speci	NA
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	NA
SAMPLE REQUIREMENTS: If the study requires biologic samples from the CIBMTR Repository, the proposal should also include: 1) A detailed description of the proposed testing methodology and sample requirements; 2) A summary of the investigator's previous e	NA
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	NA
REFERENCES:	Hawkey et al. JAMA 2015; 314: 2524-2534 Gupta A et al. Lancet/gastrohep 2024 9: 499 Brierley C, et al. Journal of Crohn's and Colitis 2018; 1097-1103 Guisado D, et al. Gut 2025; 0: 1-12
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

proposal 2509-130: Outcomes of autologous transplantation in Crohn's disease

Characteristic	Total
No. of patients	45
Recipient Age - no. (%)	
5-18	7 (15.6)
18+	38 (84.4)
Donor type - no. (%)	
Autologous HCT	45 (100)
Graft source - no. (%)	
Peripheral Blood	45 (100)
TED or CRF Track - no. (%)	
TED	43 (95.6)
CRF (RES)	2 (4.4)
Conditioning regimen - no. (%)	
Bu/Cy	3 (6.7)
Cy/Flu	1 (2.2)
Cy alone	30 (66.7)
BEAM	1 (2.2)
Mel/other(s)	1 (2.2)
Other(s)	2 (4.4)
Missing	7 (15.6)
Conditioning intensity - no. (%)	
MAC	4 (8.9)
RIC	1 (2.2)
NMA	32 (71.1)
TBD	1 (2.2)
Not Reported	7 (15.6)
Serotherapy used - no. (%)	
ATG	35 (77.8)
Alem	1 (2.2)
Not reported	9 (20.0)
Current CENTER_STATE of patient - no. (%)	
USA	25 (55.6)
Brazil	6 (13.3)
Canada	7 (15.6)
Spain	6 (13.3)
Switzerland	1 (2.2)
Year of Transplant - no. (%)	

Characteristic	Total
2008-2014	15 (33.3)
2015-2023	30 (66.7)

Field	Response
Proposal Number	2509-219-MANJAPPA
Proposal Title	Outcomes of Older sibling donor Vs. Younger alternate donor allogeneic HCT in adults with severe Aplastic Anemia
Key Words	older sibling, younger alternate donor, severe aplastic anemia, PTCY
Principal Investigator #1: - First and last name, degree(s)	Shivaprasad Manjappa, MD, MPH
Principal Investigator #1: - Email address	smanjapp@fredhutch.org
Principal Investigator #1: - Institution name	Fred Hutchinson Cancer Center
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	Yes, I am a junior investigator and would like assistance identifying a senior mentor for my project
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Non-Malignant Diseases
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	Is an older matched sibling donor still the preferred choice when a younger alternate donor is available for alloHCT in adult patients with severe aplastic anemia, in the current era, especially with the availability of effective GVHD prophylaxis
RESEARCH HYPOTHESIS:	Alternate donor (MUD, MMUD, Haplo) alloHCT with a younger donor (<35 years of age) results in better outcomes compared to matched sibling donor (MSD) alloHCT with an older donor (>=50 years of age)
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	Primary Aim 1. Compare overall survival (OS) between younger alternate donor alloHCT Vs. Older MSD alloHCT Secondary aims 1. Describe post-transplant: Cumulative incidence of non-relapse mortality (NRM), acute GVHD, and Chronic GVHD. 2. Identify factors associated with survival, NRM, and GVHD 3. Subgroup analysis-Compare outcomes of younger alternate donor HCT using PTCY-based GVHD prophylaxis vs. older matched sibling donor HCT

Field	Response
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	While alloHCT from a sibling donor remains the preferred donor choice in sAA, it is unclear if using an older matched sibling donor should still be favored if a younger alternative donor is available. This study will provide evidence on donor choices for patients with multiple donor options, helping to guide treatment decisions.
SCIENTIFIC JUSTIFICATION: Provide a background summary of previous related research and their strengths and weaknesses, justification of your research and why your research is still necessary.	Allogeneic hematopoietic cell transplant (alloHCT) is a potentially curative treatment for patients with severe aplastic anemia (sAA). A matched sibling donor is the preferred choice, both as an upfront option and after failing IST, whereas an alternate donor is considered only in the absence of a matched related donor. This is in part due to historically higher NRM and inferior outcomes reported with alternate donor alloHCTs in patients with sAA. However, advances in transplant management, including improved supportive care, enhanced donor matching, infection prophylaxis and treatment, and especially with the use of PTCY-based GVHD regimens, have resulted in improved outcomes with alternative donors. In fact, a large registry-based study using data from the CIBMTR, which included patients with myeloid malignancies who underwent MUD or MMUD alloHCT, demonstrated that the use of PTCY abrogates the effect of HLA disparity on transplant outcomes.1 Among patients with sAA, an EBMT study demonstrated comparable outcomes between MSD and MUD in pediatric and young adults.2 Similarly, phase 2 trials of haplo HCT have shown outcomes that are comparable to MSD alloHCT.3,4 Furthermore, there is increasing evidence from multiple studies suggesting that an alloHCT from a younger alternate donor is favored over a sibling alloHCT with an older donor among patients with AML/MDS.5-8 This raises a question as to whether it will hold true even with transplants in patients with sAA, especially given the differences in transplant approaches that are unique to transplants in sAA, such as the increased use of ATG and a preference for using bone marrow grafts over peripheral blood sources in sAA compared to myeloid malignancies. Hence, it needs to be evaluated and ascertained in patients with sAA whether the outcomes of sibling alloHCT with an older donor remain superior to those of alternate donor HCT with a younger donor, given that the data favors younger alternate donors in myeloid malignancies.

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion criteria: -Patients with sAA who underwent alloHCT from 2014 to 2024 -Matched related allo HCT with donor >= 50 years of age - Matched unrelated, mismatched unrelated, and haploidentical alloHCT with donor <35 years of age Exclusion Criteria: -Cord-blood transplant -2nd Allo HCT -Ex vivo T-cell depletion
Does this study include pediatric patients?	No
If this study does not include pediatric patients, please provide justification:	This study is comparing older sibling donors (>50 years of age) with alternate donors which by the design of the study, is applicable only to the adult population
DATA REQUIREMENTS: After reviewing data on CIBMTR forms, list patient-, disease- and infusion-variables to be considered in the multivariate analyses. Outline any supplementary data required.	Patient-related variables • Patient age at the time of alloHCT • Patient gender • Race: White vs. African American vs. Hispanics vs. others • Recipient performance score (KPS 90-100 versus <90) • Recipient HCT-CI (0-1 vs. 2) Disease-related variables • Prior IST (yes vs. No) • Time from diagnosis to transplant Transplant-related variables • Donor gender • Donor age • Donor/Recipient CMV status: -/+ vs. +/- vs. +/+ vs. -/- • Conditioning regimen • Graft source (peripheral blood vs. marrow) • Donor type (MSD vs. MUD vs. MMUD vs. Haploidentical) • GVHD prophylaxis regimen • Use of ATG (yes vs. no) • Use of Alemtuzumab (yes vs. no)
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)

Field	Response
REFERENCES:	1. Shaffer BC, Gooptu M, DeFor TE, et al. Post-Transplant Cyclophosphamide-Based Graft-Versus-Host Disease Prophylaxis Attenuates Disparity in Outcomes Between Use of Matched or Mismatched Unrelated Donors. J Clin Oncol 2024; 42(28): 3277-86. 2. Dufour C, Veys P, Carraro E, et al. Similar outcome of upfront-unrelated and matched sibling stem cell transplantation in idiopathic paediatric aplastic anaemia. A study on behalf of the UK Paediatric BMT Working Party, Paediatric Diseases Working Party and Severe Aplastic Anaemia Working Party of EBMT. Br J Haematol 2015; 171(4): 585-94. 3. DeZern AE, Eapen M, Wu J, et al. Haploidentical bone marrow transplantation in patients with relapsed or refractory severe aplastic anaemia in the USA (BMT CTN 1502): a multicentre, single-arm, phase 2 trial. Lancet Haematol 2022; 9(9): e660-e9. 4. DeZern AE, Zahurak M, Symons HJ, et al. Alternative donor BMT with posttransplant cyclophosphamide as initial therapy for acquired severe aplastic anemia. Blood 2023; 141(25): 3031-8. 5. Mehta RS, Ramdial J, Kebriaei P, et al. Haploidentical vs HLA-matched sibling donor HCT with PTCy prophylaxis: HLA factors and donor age considerations. Blood Adv 2024; 8(20): 5306-14. 6. Nath K, Zhang MJ, Bye M, et al. Transplant outcomes using older matched sibling donors compared with young alternative donors: a CIBMTR analysis. Blood Adv 2025; 9(14): 3469-78. 7. Pereira MP, Remberger M, Chen C, et al. Choosing Between Older Matched Sibling Donor and Younger Matched Unrelated Donor in Allogeneic Hematopoietic Cell Transplantation: Comparison of Clinical Outcomes in Acute Myeloid Leukemia and Myelodysplastic Syndrome. Transplant Cell Ther 2023; 29(11): 697.e1-.e10. 8. Sanz J, Labopin M, Choi G, et al. Younger unrelated donors may be preferable over HLA match in the PTCy era: a study from the ALWP of the EBMT. Blood 2024; 143(24): 2534-43.
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

proposal 2509-219: Outcomes of Older sibling donor Vs. Younger alternate donor allogeneic HCT in adults with Severe Aplastic Anemia

Characteristic	older matched sibling donor	matched unrelated donor and <35y	haplo/MMUD and donor <35y
No. of patients	163	986	444
Recipient Age, no. (%)			
<18	0 (0)	366 (37)	209 (47)
18-40	7 (4)	345 (35)	152 (34)
40+	156 (96)	275 (28)	83 (19)
Disease, no. (%)			
SAA idiopathic	161 (99)	924 (94)	420 (95)
SAA secondary to hepatitis	0 (0)	52 (5)	20 (5)
SAA secondary to toxin-other	2 (1)	10 (1)	4 (1)
Donor age, no. (%)			
<18	0 (0)	1 (0)	84 (19)
18-35	0 (0)	985 (100)	360 (81)
50+	163 (100)	0 (0)	0 (0)
Donor type, no. (%)			
HLA identical sibling	163 (100)	0 (0)	0 (0)
Haploidentical donor	0 (0)	0 (0)	437 (98)
Well-matched unrelated (8/8)	0 (0)	986 (100)	0 (0)
Mismatched unrelated (<= 6/8)	0 (0)	0 (0)	7 (2)
Conditioning regimen, no. (%)			
MAC			
TBI/Cy/Flu	0 (0)	0 (0)	1 (0)
TBI/Cy/Flu/TT	1 (1)	0 (0)	2 (0)
TBI/Cy/TT	0 (0)	1 (0)	0 (0)
Bu/Cy	1 (1)	32 (3)	16 (4)
Bu/Mel	0 (0)	0 (0)	1 (0)
Flu/Bu/TT	0 (0)	0 (0)	2 (0)
Flu/Bu	0 (0)	1 (0)	2 (0)
Flu/Mel/TT	0 (0)	4 (0)	2 (0)
Cy/Flu	1 (1)	0 (0)	0 (0)
RIC/NMA			
TBI/Cy	8 (5)	106 (11)	4 (1)
TBI/Cy/Flu	24 (15)	483 (49)	356 (80)
TBI/Mel	0 (0)	2 (0)	18 (4)

Characteristic	older matched sibling donor	matched unrelated donor and <35y	haplo/MMUD and donor <35y
TBI/Flu	4 (2)	16 (2)	7 (2)
TBI/other(s)	0 (0)	1 (0)	0 (0)
Bu/Cy	0 (0)	0 (0)	1 (0)
Flu/Bu	0 (0)	16 (2)	3 (1)
Flu/Mel	5 (3)	37 (4)	7 (2)
FCR	1 (1)	3 (0)	1 (0)
Cy/Flu	50 (31)	232 (24)	6 (1)
Cy alone	64 (39)	34 (3)	3 (1)
TLI	0 (0)	0 (0)	4 (1)
Other(s)	1 (1)	2 (0)	0 (0)
TBD			
TBI/Cy	0 (0)	5 (1)	1 (0)
TBI/Cy/Flu	0 (0)	5 (1)	1 (0)
TBI/other(s)	0 (0)	0 (0)	2 (0)
Treosulfan	0 (0)	2 (0)	2 (0)
Other(s)	2 (1)	3 (0)	2 (0)
Not reported			
None	0 (0)	1 (0)	0 (0)
Missing	1 (1)	0 (0)	0 (0)
Serotherapy used, no. (%)			
ATG	121 (74)	730 (74)	319 (72)
Alem	14 (9)	189 (19)	4 (1)
Not reported	28 (17)	67 (7)	121 (27)
GVHD prophylaxis, no. (%)			
Ex-vivo T-cell depletion	2 (1)	20 (2)	11 (2)
CD34 selection	0 (0)	14 (1)	8 (2)
PtCy + other(s)	11 (7)	90 (9)	357 (80)
PtCy alone	0 (0)	2 (0)	6 (1)
TAC + MMF +- other(s) (except PtCy)	8 (5)	81 (8)	29 (7)
TAC + MTX +- other(s) (except MMF, PtCy)	67 (41)	352 (36)	0 (0)
TAC + other(s) (except MMF, MTX, PtCy)	1 (1)	20 (2)	2 (0)
TAC alone	2 (1)	51 (5)	1 (0)
CSA + MMF +- other(s) (except PtCy,TAC)	5 (3)	32 (3)	8 (2)
CSA + MTX +- other(s) (except PtCy,TAC,MMF)	46 (28)	238 (24)	5 (1)
CSA + other(s) (except PtCy,TAC,MMF,MTX)	0 (0)	10 (1)	0 (0)
CSA alone	14 (9)	50 (5)	1 (0)

Characteristic	older matched sibling donor	matched unrelated donor and <35y	haplo/MMUD and donor <35y
Other(s)	3 (2)	8 (1)	2 (0)
Missing	4 (2)	18 (2)	14 (3)
Country, no. (%)			
USA	104 (64)	748 (76)	260 (59)
Argentina	0 (0)	1 (0)	1 (0)
Australia	9 (6)	33 (3)	7 (2)
Belgium	0 (0)	8 (1)	1 (0)
Brazil	10 (6)	87 (9)	71 (16)
Canada	12 (7)	56 (6)	16 (4)
Chile	0 (0)	1 (0)	0 (0)
Denmark	0 (0)	9 (1)	0 (0)
United Kingdom	5 (3)	2 (0)	1 (0)
India	14 (9)	11 (1)	41 (9)
South Korea	2 (1)	6 (1)	4 (1)
New Zealand	2 (1)	11 (1)	6 (1)
Saudi Arabia	3 (2)	3 (0)	18 (4)
Sweden	0 (0)	1 (0)	0 (0)
Switzerland	0 (0)	3 (0)	0 (0)
Taiwan	0 (0)	1 (0)	0 (0)
Mexico	0 (0)	0 (0)	4 (1)
Turkey	0 (0)	1 (0)	2 (0)
Uruguay	1 (1)	0 (0)	0 (0)
Singapore	1 (1)	3 (0)	1 (0)
Pakistan	0 (0)	0 (0)	11 (2)
Czech Republic	0 (0)	1 (0)	0 (0)
Year of Transplant, no. (%)			
2014-2018	87 (53)	427 (43)	100 (23)
2019-2023	76 (47)	559 (57)	344 (77)

Field	Response
Proposal Number	2509-224-PARK
Proposal Title	Outcomes of Allogeneic Hematopoietic Cell Transplantation for Acquired Pure Red Cell Aplasia
Key Words	Acquired Pure Red Cell Aplasia, hematopoietic cell transplantation
Principal Investigator #1: - First and last name, degree(s)	Sunmin Park
Principal Investigator #1: - Email address	supark@coh.org
Principal Investigator #1: - Institution name	City of Hope
Principal Investigator #1: - Academic rank	Assistant Professor
Junior investigator status (defined as 㑊、5 years from fellowship)	Yes
Do you identify as an underrepresented/minority?	No
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:	Sunmin Park
If you are a junior investigator and would like assistance identifying a senior mentor for your project please click below:	Yes, I am a junior investigator and would like assistance identifying a senior mentor for my project
Please list any ongoing CIBMTR projects that you are currently involved in and briefly describe your role.	None
Do any of the PI(s) within this proposal have a CIBMTR WC study in manuscript preparation >6 months?	No
PROPOSED WORKING COMMITTEE:	Non-Malignant Diseases
Please indicate if you have already spoken with a scientific director or working committee chair regarding this study.	No
RESEARCH QUESTION:	What are the outcomes of allogeneic hematopoietic cell transplantation (HCT) in patients with acquired pure red cell aplasia (aPRCA)?
RESEARCH HYPOTHESIS:	Allogeneic HCT can provide durable transfusion independence in refractory aPRCA and specific clinical and biological factors are associated with post-HCT outcomes.

Field	Response
SPECIFIC OBJECTIVES/OUTCOMES TO BE INVESTIGATED (Include Primary, Secondary, etc.):	1. Characterize patient, disease, and transplant features among individuals undergoing HCT for aPRCA. 2. Estimate the overall survival (OS), transplant-related mortality (TRM), engraftment, graft-versus-host-disease (GVHD), and transfusion independence after HCT. 3. Identify clinical risk factors associated with transplant outcomes in aPRCA 4. Explore the presence of clonal T cell expansion and somatic mutations in pre-HCT samples from patients with aPRCA
SCIENTIFIC IMPACT: Briefly state how the completion of the aims will impact participant care/outcomes and how it will advance science or clinical care.	Acquired PRCA is a rare autoimmune, bone marrow failure syndrome managed initially with immunosuppressive therapy (IST). A subset of patients become refractory to IST and remain dependent on lifelong transfusions, which are associated with infectious complications, iron overload and organ dysfunction. In a large Japanese consortium, survival of aPRCA patients was shorter than that of the age- and sex-matched general population, regardless of whether disease was idiopathic, thymoma-associated, or LGL-associated aPRCA.1 Allogeneic HCT represents a potentially curative therapy, but data are scarce. The only registry-level report to date, an EBMT analysis published in 2019, described a modest survival in a small cohort of 33 adults transplanted prior to the widespread use of modern conditioning regimens and GVHD prophylaxis.2 Contemporary registry-level data from the CIBMTR would address a major knowledge gap and provide critical insights into feasibility, risks and long-term outcomes of HCT in acquired PRCA. Furthermore, integration of correlative analyses could generate growing evidence for the role of clonal T-cell expansion and somatic mutations in the pathogenesis of aPRCA, guiding future mechanistic and therapeutic studies. To our knowledge, no prior CIBMTR analysis has systematically examined HCT outcomes in aPRCA, making this study the first comprehensive registry-based evaluation in the contemporary transplant era.

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.	PRCA is defined by normocytic anemia, severe reticulocytopenia, and absence of erythroid precursors in the marrow.3 Acquired PRCA (aPRCA) may occur as a primary autoimmune disorder or secondary to conditions such as thymoma, T-cell large granular lymphocytic leukemia (T-LGL), or autoimmune disease. Primary aPRCA may be mediated by autoantibodies inhibiting erythropoiesis or clonal T cells recognizing erythroid precursors.4,5 Up to 20% of aPRCA cases are associated with T-LGL.5 Studies have demonstrated enrichment for restricted TCR Vb1 and recurrent STAT3 mutations in CD8+ T cells from aPRCA patients.6 8 Somatic STAT3 mutations are frequent in both primary aPRCA and T-LGL associated aPRCA. Other mutations associated with myeloid neoplasms, such as KMT2D, KDM6A, BCOR, DNMT3A, TET2 were found in adults with aPRCA, but their functional contribution in aPRCA remains unclear.8 10 First-line therapy is IST, with cyclosporine A (CsA) as the most effective agent.5,11 While IST induces transfusion independence in many, refractory disease carries a poor prognosis due to infection, iron overload and end-organ dysfunction. Allogeneic HCT has been reported as curative in case reports.12 14 The largest series of HCT in PRCA (EBMT, n=33) reported 5-year OS of 51%, with infections and GVHD as leading causes of death. 2 However, the study predated current transplant strategies, leaving critical uncertainty about the role of HCT in the contemporary era. This study will leverage the CIBMTR database to 1) define outcomes of allogeneic HCT in the largest cohort of aPRCA patients reported to date, 2) identify prognostic and potentially modifiable clinical factors, and 3) explore biologic correlates, including TCR clonality and co-occurring somatic mutations to generate mechanistic hypotheses. Challenges include the rarity of aPRCA, potential diagnostic misclassification (e.g. under SAA/PNH or under T cell neoplasms), and limited data on pre-HCT IST, transfusion burden or ferritin. Lack of a non-HCT comparator arm will limit direct comparisons to salvage IST. Despite these challenges, this will likely represent the largest contemporary analysis of HCT for aPRCA, providing guidance for clinicians regarding referral, feasibility and long-term outcomes following HCT.

Field	Response
PARTICIPANT SELECTION CRITERIA: State inclusion and exclusion criteria.	Inclusion: Adults 18y with aPRCA who underwent HCT between 2001-2023. aPRCA associated with hematologic conditions (e.g. LGL, CLL, CVID) will be included. Exclusion: Congenital PRCA (e.g. Diamond-Blackfan anemia), PRCA secondary to prior HCT (e.g. ABO-incompatibility), and cases miscoded as SAA/PNH. Biologic sample availability is not required for inclusion; patients with available samples in the NMDP Biobank will undergo correlative testing. Outcomes: Primary: overall survival (OS) at 3-year post HCT Secondary: GVHD (acute GVHD grade 2-4, chronic GVHD at 1, 3 and 5 years post-HCT), graft failure, engraftment of neutrophils and platelets, treatment-related mortality (TRM), Event-free Survival (EFS), Relapse, OS (100 days, 6 mo, 1 year), and cause of death Exploratory: transfusion-independence (8 weeks without RBC transfusion)
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.	The proposal is specifically for acquired PRCA, which mainly presents in the adult population.
Types of cellular therapy data this proposal includes:	Hematopoietic Cell Transplantation (HCT)
PATIENT REPORTED OUTCOME (PRO) REQUIREMENTS: If the study requires PRO data collected by CIBMTR, the proposal should include: 1) A detailed description of the PRO domains, timepoints, and proposed analysis of PROs; 2) A description of the hypothesis speci	none
MACHINE LEARNING: Please indicate if the study requires methodology related to machine-learning and clinical predictions.	none

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	For patients with pre-HCT biospecimens (peripheral blood or marrow) in the NMDP Biobank, we will perform TCR gene rearrangement analysis to assess clonal expansion and Hopeseq mutation assay. This platform includes DNA full exon sequencing of up to 523 genes and RNA fusion detection of up to 165 genes, and the methods have been described and published in detail in other diseases characterizing mutational landscapes.15 17 Institutional funds will support these assays.
NON-CIBMTR DATA SOURCE: If applicable, please provide: 1) A description of external data source to which the CIBMTR data will be linked; 2) The rationale for why the linkage is required.	Given the rarity of PRCA, collaboration with EBMT or other international registries may be considered to enhance sample size and statistical power.

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Field	Response
	R. Donor lymphocyte infusions for refractory pure red cell aplasia relapsing after both autologous and nonmyeloablative allogeneic peripheral stem cell transplantation. Bone Marrow Transplant. 2004;33(7):769-771. doi:10.1038/sj.bmt.1704419 14. M ller B, Tichelli A, Passweg J, Nissen C, Wodnar-Filipowicz A, Gratwohl A. Successful treatment of refractory acquired pure red cell aplasia (PRCA) by allogeneic bone marrow transplantation. Bone Marrow Transplant. 1999;23(11):1205-1207. doi:10.1038/sj.bmt.1701785 15. Mei M, Pillai R, Kim S, et al. The mutational landscape in chronic myelomonocytic leukemia and its impact on allogeneic hematopoietic cell transplantation outcomes: a Center for Blood and Marrow Transplantation Research (CIBMTR) analysis. Haematologica. 2022;108(1):150-160. doi:10.3324/haematol.2021.280203 16. Ali H, Aldoss I, Yang D, et al. MIPSS70+ v2.0 predicts long-term survival in myelofibrosis after allogeneic HCT with the Flu/Mel conditioning regimen. Blood Adv. 2019;3(1):83-95. doi:10.1182/bloodadvances.2018026658 17. Aldoss I, Pham A, Li SM, et al. Favorable impact of allogeneic stem cell transplantation in patients with therapy-related myelodysplasia regardless of TP53 mutational status. Haematologica. 2017;102(12):2030-2038. doi:10.3324/haematol.2017.172544
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

proposal 2509-224: Outcomes of Allogeneic Hematopoietic Cell Transplantation for Acquired Pure Red Cell Aplasia

Characteristic	Total
No. of patients	33
agegp, no. (%)	
Median (range)	56.2 (20.8-74.9)
18-34	6 (18)
35-50	7 (21)
50+	20 (61)
Donor type, no. (%)	
HLA-identical sibling	6 (18)
Other related	2 (6)
Well-matched unrelated (8/8)	20 (61)
Partially-matched unrelated (7/8)	4 (12)
Multi-donor	1 (3)
Related donor transplant sample types available, no. (%)	
Samples Available for Recipient and Donor	8 (24)
Not Avaliable	25 (76)
Graft (Product) type, no. (%)	
Bone marrow	18 (55)
Peripheral blood stem cells	15 (45)
Ted or CRF, no. (%)	
TED	14 (42)
CRF (RES)	18 (55)
moved CRF (RES) to TED	1 (3)
conditioning intensity, no. (%)	
Myeloablative	9 (27)
RIC	24 (73)
Country code for CCN, no. (%)	
USA	32 (97)
Canada	1 (3)
Year of Transplant, no. (%)	
2008-2014	8 (24)
2015-2023	25 (76)
Follow-up of survivors, median (range), months	24.9 (5.1-122.6)