ACCESS Clinical Trial Eliminates Disparity in Identifying a Donor for All Patients in Need of Stem Cell Transplantation

Authors and Affiliations: Janelle A. Olson[1], Sarah Smith[1], Stephanie Bo-Subait[1], Brent Logan[2], Erin Leckrone[1], Juan Wu[1], Heather E. Stefanski[1], Jeffery J. Auletta[1], Stephen R. Spellman[1], Craig Malmberg[1], Thomas Silverman[1], Deborah Mattila[1], Michelle Kuxhausen[1], Caitrin Bupp[1], Rachel Cusatis[3], Larisa Broglie[3], Bronwen E. Shaw[3], Antonio Martin Jimenez Jimenez[4], Monzr M. Al Malki[5], Steven Michael Devine[1]

[1]CIBMTR® (Center for International Blood and Marrow Transplant Research), NMDP℠, Minneapolis, MN; [2]Division of Biostatistics; Medical College of Wisconsin, Milwaukee, WI; [3]CIBMTR® (Center for International Blood and Marrow Transplant Research), Medical College of Wisconsin, Milwaukee, WI; [4]Sylvester Comprehensive Cancer Center, Miller School of Medicine, Miami, FL; [5]City of Hope National Medical Center, Duarte, CA

CIBMTR (Center for International Blood and Marrow Transplant Research) is a research collaboration between the Medical College of Wisconsin and NMDP.

PURPOSE

- **Hematopoietic stem cell transplantation (HSCT)** is often the **only cure for patients with blood cancer.**

Only **30%** Have a match in their family

nmdp FIND CURES. SAVE LIVES. Unrelated donor registries fill that gap

- Many patients, especially those from **diverse ethnic backgrounds, struggle to find a perfect match** due to genetic differences and fewer matched donors in registries.

Every patient deserves a donor

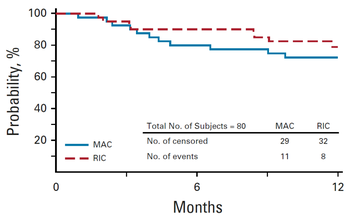
ODDS OF FINDING A MATCH BASED ON ETHNIC BACKGROUND

Black or African American	Asian or Pacific Islander	Hispanic or Latino	American Indian and Alaskan Native	White
29%	47%	48%	60%	79%

There is a lack of equality of access to a potentially life-saving transplant.

BACKGROUND

- A prospective, Phase II multi-center trial (15-MMUD) assessed the effectiveness of **mismatched unrelated donor (MMUD) HSCT using cyclophosphamide (PTCy), a post-transplant chemotherapy drug, to prevent graft versus host disease (GVHD)**, a severe complication in which donor cells attack the patient.



- Achieved its primary endpoint with **76%** 1-year **Overall Survival (OS)**, showing that this MMUD transplant approach is safe and effective.
- **48%** of patients were racially/ethnically diverse

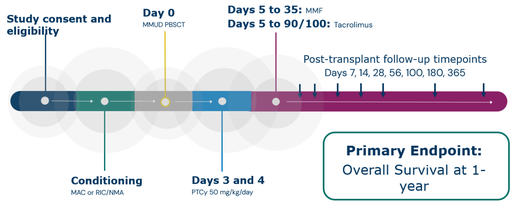
Shaw et al. J Clin Oncol 2021 Jun 20;39(18):1971-1982

- The 15-MMUD study used stem cells from donor bone marrow (BM). The ACCESS study investigates the use of peripheral blood stem cells (PBSC) because PBSCs are most commonly used in HSCT.
- **We sought to determine whether OS in adults receiving PBSC from MMUD would be comparable or superior to 15-MMUD results.**

METHODS

- **Study design** – A prospective, Phase II multi-center trial (ACCESS; NCT04904588) to assess the impact of PTCy-based GVHD prevention on OS following MMUD transplantation.
- **Study Population/duration** –

Participants with blood cancer requiring a stem cell transplant, with good physical performance scores and kidney function. Donors were partially matched (4/8-7/8) and under 36 years old with stem cells from PBSC. Participants were excluded if they had a fully matched (8/8) donor available, high HLA antibodies against the donor, a previous stem cell transplant, or were in another GVHD prevention trial.

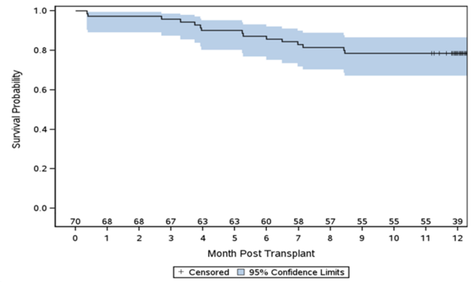


Primary Endpoint: Overall Survival at 1-year

- We report the results of a planned analysis of the first 70 adult participants to complete follow-up. The study was designed to detect a primary endpoint of 1-year OS of 75%.

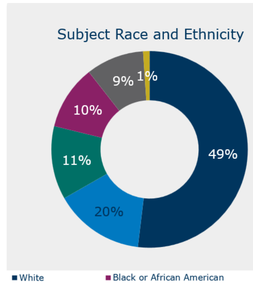
RESULTS

- The primary endpoint of **Overall Survival (OS)** at 1-year post-HSCT was **79%.**
- Key secondary endpoints included a rate of severe acute GVHD of 9% at 6 months and a disease relapse rate of 21% at 1 year. **Rates of GVHD and other complications appear comparable to other transplant settings.**



RESULTS

- Notably, **half of the enrolled subjects were ethnically diverse,** considerably higher than typical clinical trials.



Characteristic	n (%)
No. of subjects	70
No. of centers	13
Age at HCT	
Median (min-max)	**65.0** (24.0-77.0)
Sex	
Male	35 (50.0)
Female	35 (50.0)
Cryopreservation	
Cryopreserved	60 (85.7)
Fresh	10 (14.3)

- The social determinants of health, as indicated by patient-reported outcome (PRO) data, reveal differences in age, financial toxicity, and social vulnerability between ethnically diverse and not ethnically diverse (White, non-Hispanic) ACCESS subjects.

	Not Ethnically Diverse	Ethnically Diverse
Average age at transplant (P value <0.01)	**64**	**55**
Average FACIT-COST (Financial Toxicity) score (P value <0.01)	29.5 (No financial toxicity)	22.0 (Mild financial toxicity)
Average National Overall Social Vulnerability Index (SVI) Score (P value <0.01)	**0.5** (Medium High)	**.08** (High)

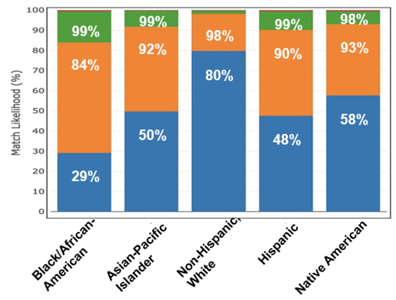
Note: Hypothesis testing: [a]Kruskal-Wallis test, [b]Pearson chi-square test. COST=Comprehensive score for financial toxicity. FT=Financial Toxicity. SVI=Social Vulnerability Index.
[1]https://www.atsdr.cdc.gov/placeandhealth/svi/interactive_map.html

IMPACT

MMUD HSCT can expand access to patients with diverse ethnic backgrounds and **those with greater social vulnerabilities**. Such patients have long faced disparities in access to transplants, worse post-HSCT outcomes, and low clinical trial enrollment.

Accrual was brisk, and site participation was broad, suggesting the **ACCESS Study addresses an unmet patient need.**

MMUD HSCT benefits all patients for whom a perfect match is unavailable.



MMUD HSCT can expand access to transplant for all patients in need and identify a Donor for All.

Chowdhury A et al. Transplant Cell Ther 2023 Nov;29(11):686

FUTURE DIRECTIONS

- Full analysis of ACCESS Study Data (Adult; n=268) is expected in 2025.
- Despite its ability to expand access to life-saving therapy, **future research must be done to evaluate the safety and efficacy of PTCy–based GVHD prophylaxis** and future applications to cure non-malignant diseases after MMUD HSCT. **Further research is underway** to mitigate the risks of PTCy toxicities (OPTIMIZE; NCT06001385).

DISCLOSURE

Monzr Al Malki: Gilead, NexImmune, Hasna Biopharma, Miltenyi Biotec, Incyte, CareDx; Steven Devine: Orca Bio; Muna Qayed: Novartis, Vertex; Jeffery Auletta: AscellaHealth; Heather Stefanski: Novartis; all other authors reported no conflict of interest

CONTACT: ACCESS@nmdp.org
cibmtr.org