ELSEVIER

Blood 142 (2023) 4988–4990

The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

732.ALLOGENEIC TRANSPLANTATION: DISEASE RESPONSE AND COMPARATIVE TREATMENT STUDIES

Patient Characteristics and Outcomes of Allogeneic Hematopoietic Stem Cell Transplantation for Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)

Muzaffar H. Qazilbash, MD[1], *Hina N. Khan, MD*[1,2], *Denái R. Milton, MS*[3], *Oren Pasvolsky, MD*[4,5,1], *Mark R. Tanner, PhD*[1], *Uday R. Popat, MD*[1], *Partow Kebriaei, MD*[1], *Chitra Hosing, MD*[1], *Issa F. Khouri, MD*[1], *Katayoun Rezvani, MD PhD*[1], *Yago Nieto, MD PhD*[1], *Betul Oran, MD MS*[1], *Samer A. Srour, MD*[1], *Neeraj Y. Saini, MD*[1], *Amanda L. Olson, MD*[1], *Sairah Ahmed, MD*[1,6], *Gheath Alatrash, PhDDO*[1], *Gabriela Rondon, MD*[1], *Marina Y. Konopleva, MD PhD*[7], *Richard E. Champlin, MD*[1], *Elizabeth J. Shpall, MD*[1], *Qaiser Bashir, MD*[1], *Naveen Pemmaraju, MD*[7]

[1] Department of Stem Cell Transplantation and Cellular Therapy, The University of Texas MD Anderson Cancer Center, Houston, TX
[2] Department of Hematology and Oncology, The University of Texas Health Science Center McGovern Medical School, Houston, TX
[3] Department of Biostatistics, The University of Texas MD Anderson Cancer Center, Houston, TX
[4] Faculty of Medicine, Tel Aviv University, Tel Aviv, Israel
[5] Institute of Hematology, Davidoff Cancer Center, Rabin Medical Center, Petah-Tikva, Israel
[6] Department of Lymphoma and Myeloma, The University of Texas MD Anderson Cancer Center, Houston, TX
[7] Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX

Background: Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare, aggressive hematologic malignancy arising from plasmacytoid dendritic cells. It commonly presents as skin lesions with or without bone marrow, lymph node, central nervous system (CNS), or systemic involvement. Limited retrospective data have shown durable remissions after allogeneic (allo) hematopoietic cell transplantation (HCT).
Methods: In this retrospective single-center analysis, we evaluated outcomes of 31 BPDCN patients treated with allo-HCT between September 2000 and June 2023 at our institution.
Results: Median age of our cohort was 52 years [range 16-76 y]. Patient characteristics are summarized in the attached **Table**. Only 6 patients (19%) had their disease confined to skin and 4 (13%) had CNS/CSF involvement at diagnosis. Four patients (13%) had prior hematologic malignancies (HM), and 9 patients (29%) had a cytogenetic abnormality at baseline. Thirteen (42%) patients, who were evaluated by a next-generation sequencing Leukemia Mutation Panel, had *TET2* mutation. Median time from diagnosis to HCT was 6.2 (3.2-42.4) months. Eight (26%) patients received tagraxofusp, matched unrelated donor was the most common donor type (39%), and fludarabine + busulfan-based conditioning was used in 17 (55%) patients. Post-transplant cyclophosphamide (PTCy) was used for GVHD prophylaxis in 23 (74%) patients. Median follow up was 23.9 (0.9-119.9) months. One-hundred day and 1-year non-relapse mortality was 9.7% and 23.1%, respectively. Grade 2 acute GVHD was seen in 9 patients (29%), and no grade 3-4 GVHD has been seen so far. Limited or extensive chronic GVHD was seen in 6 (21%) of 28 evaluable patients. Only 6 of the 31 (19%) allo-HCT patients progressed after HCT. Two-year PFS and OS was 56% and 67%, respectively. Patients receiving allo-HCT in first remission had significantly better median PFS (not reached vs. 11.3 months; p=0.034) and OS (not reached vs. 12.6 months; p=0.045).
Conclusions: These results demonstrate the safety and efficacy of allo-HCT in BPDCN. Patients undergoing allo-HCT in first remission had significantly better outcomes. Prospective studies are needed to better define the role of allo-HCT in BPDCN.

Disclosures Qazilbash: *Amgen:* Research Funding; *Angiocrine:* Research Funding; *Janssen:* Research Funding; *Bioline:* Other: Advisory board; *NexImmune:* Research Funding. **Kebriaei:** *Jazz:* Consultancy, Honoraria; *Pfizer:* Consultancy, Honoraria. **Khouri:** *Pfizer:* Research Funding. **Rezvani:** *Affimed:* Other: License agreement; *Takeda:* Patents & Royalties. **Nieto:** *Secura Bio:* Research Funding; *Astra Zeneca:* Research Funding; *Affimed:* Research Funding. **Srour:** *Orca Bio:* Research Funding. **Saini:** *GSK:* Research Funding; *Panbela Theraputics:* Research Funding. **Konopleva:** *AbbVie, Forty Seven, Precision Biosciences, Gilead Sciences, Genentech, Janssen, Sanofi, MEI Pharma, Daiichi Sankyo Pharmaceutical, AstraZeneca Co., Menarini.:* Consultancy; *Reata Pharmaceuticals.:* Current holder of *stock options* in a privately-held company, Patents & Royalties;

Abbvie, Allogene Therapeutics, Cellectis, Forty Seven, Gilead Sciences, Genentech, Sanofi, MEI Pharma, Rafael Pharmaceuticals, Daiichi Sankyo Pharmaceutical, AstraZeneca Co., Menarini, Precision BioSciences.: Research Funding. **Champlin:** *Cell Source:* Research Funding; *Kadmon:* Consultancy; *Arog:* Consultancy; *Takeda Corporation:* Patents & Royalties; *Johnson & Johnson/Janssen:* Consultancy; *Omeros:* Consultancy; *Actinium Pharmaceuticals:* Consultancy; *Orca Bio:* Consultancy. **Shpall:** *Syena:* Other: License agreement; *Takeda:* Other: License agreement; *Axio:* Membership on an entity's Board of Directors or advisory committees; *Fibrobiologics:* Membership on an entity's Board of Directors or advisory committees; *Celaid Therapeutics:* Membership on an entity's Board of Directors or advisory committees; *Navan:* Membership on an entity's Board of Directors or advisory committees; *Adaptimmune:* Membership on an entity's Board of Directors or advisory committees; *NY Blood Center:* Membership on an entity's Board of Directors or advisory committees; *Affimed:* Other: License agreement. **Bashir:** *Pfizer:* Research Funding; *GSK:* Research Funding; *Acrotech:* Research Funding; *Stemline:* Research Funding. **Pemmaraju:** *PharmaEssentia:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Incyte:* Consultancy, Membership on an entity's Board of Directors or advisory committees; *EUSA Pharma:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *ImmunoGen:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Patient Power:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Menarini Group:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Karger Publishers:* Other: Licenses; *ASCO Cancer.Net Editorial Board:* Other: Leadership; *Novartis Pharmaceuticals:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *United States Department of Defense (DOD):* Research Funding; *Dan's House of Hope:* Membership on an entity's Board of Directors or advisory committees; *ClearView Healthcare Partners:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Medscape:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *CTI BioPharma:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Physician Education Resource (PER):* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *OncLive:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *ASH Committee on Communications:* Other: Leadership; *Curio Science:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Stemline:* Consultancy, Membership on an entity's Board of Directors or advisory committees; *Protagonist Therapeutics, Inc.:* Consultancy, Membership on an entity's Board of Directors or advisory committees; *National Institute of Health/National Cancer Institute (NIH/NCI):* Research Funding; *PeerView Institute for Medical Education:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Neopharm:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Pacylex:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Aplastic Anemia & MDS International Foundation:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *CancerNet:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *CareDx:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Celgene:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Dava Oncology:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Harborside Press:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Imedex:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Intellisphere:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Magdalen Medical Publishing:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Cimeio Therapeutics AG:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *BMS:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Astellas:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Blueprint:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Aptitude Health:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *AbbVie:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *HemOnc Times/Oncology Times:* Other: Uncompensated; *Bristol Myers Squibb Pharmaceuticals:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau.

Table. Patient Characteristics.

	Allo-HCT (N=31)
Age at HCT, median (range)	52 (16-76)
Gender, n (%)	
Male	26 (84)
Female	5 (16)
Organs involved, n (%)	
Skin-only	6 (19)
Systemic-only	10 (32)
Skin + Systemic	15 (48)
CNS/CSF	4 (13)
Relapsed before HCT, n (%)	
No	24 (77)
Yes	7 (23)
Induction, n (%)	
Cytotoxic chemo only	17 (55)
Tagraxofusp followed by cytotoxic chemo	5 (16)
Tagraxofusp alone	3 (10)
Other CD123/cytotoxic chemo	6 (19)
Preparative regimen, n (%)	
FM	9 (29)
FB	17 (55)
Other	5 (16)
Donor type, n (%)	
Matched sibling	9 (29)
Haplo-identical	7 (23)
Matched unrelated	12 (39)
Allo-cord	3 (10)
Response to HCT, n (%)	
CR	27 (87)
PR	1 (3)
ED	1 (3)
NE	2 (6)
GVHD prophylaxis, n (%)	
Tacro/MMF/PTCy	17 (55)
Tacro/PTCy	6 (19)
Tacro/MTX	7 (23)
Tacro/MMF	1 (3)

FM = fludarabine, melphalan; FB = fludarabine, busulfan

Figure. Progression-Free Survival (A) and Overall Survival (B) Comparing Patients Transplanted in First Remission vs. Relapsed Disease.

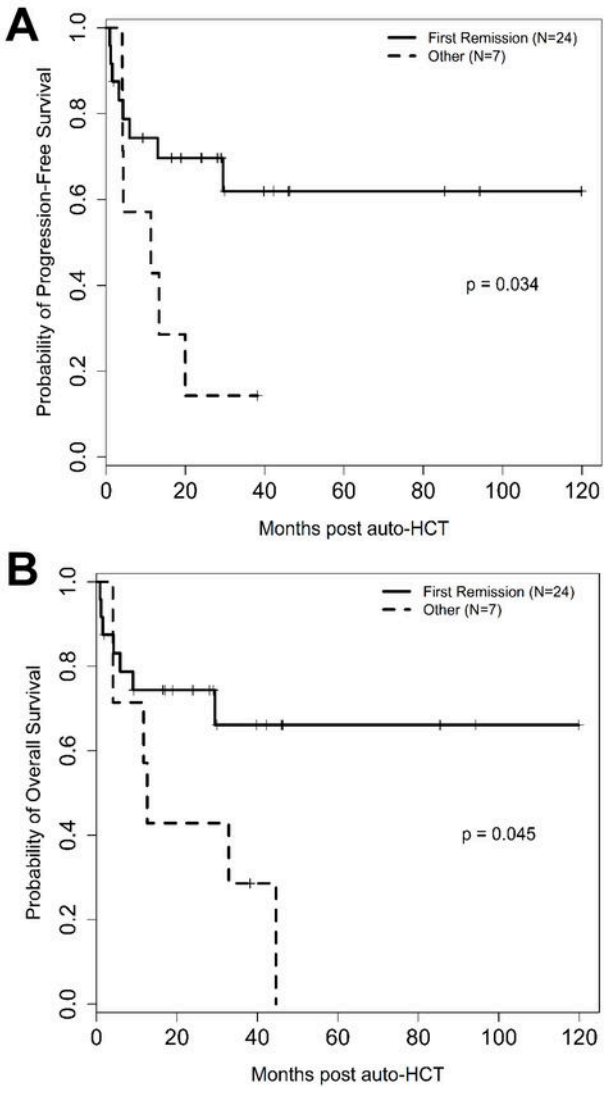

Figure 1

https://doi.org/10.1182/blood-2023-181031