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POSTER ABSTRACTS

613.ACUTE MYELOID LEUKEMIAS: CLINICAL AND EPIDEMIOLOGICAL

Clinical Features and Treatment in Patients Diagnosed with Blastic Plasmacytoid Dendritic Cell Neoplasm: Interim Analysis from the Pethema Epidemiologic Registry (EPI-BLAS study)

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Background

BPDCN (Blastic Plasmacytoid Dendritic Cell Neoplasm) is a rare and aggressive hematological neoplasm, and due to its rarity there are scarce registry studies analyzing the disease in real-world. The proposed study seeks to fill this knowledge gap and gain insights into various aspects of the disease, including demographics, presentation features, biological data, treatment patterns, and individual patient outcomes. Ultimately, the study aims to shed light on how to best manage BPDCN and identify patients who could benefit from curative treatments, including front-line agents followed by allogeneic stem cell transplant (allo-HSCT) and novel therapies, like IL3R-diphteric toxin compound (SL-140; Tagraxofusp) which showed remarkable activity in front-line therapy.

Methods

The EPI-BLAS is a retrospective, multicenter, non-interventional chart review study of patients diagnosed with BPDCN. Approximately 150 patients treated at PETHEMA participating sites in Spain will be included in final analyses. The review will focus on medical records of patients diagnosed with BPDCN, and the data will be entered into an electronic case report form (eCRF) for all patients who meet the inclusion and exclusion criteria. For this analysis, we included a first cohort of 68 adult patients (≥18 years old) who were diagnosed BPDCN between January 2010 and January 2022.

Results

The median age at diagnosis was 66 years (range 18-87 years). The majority of the patients (74%) was de novo, while 26% of them had a prior history of neoplastic disease [43% of them had prior solid neoplasm (thyroid cancer, prostate adenocarcinoma, melanoma and breast ductal carcinoma) and 57% suffered from hematological neoplasms as large granular lymphocytic leukemia, myelodysplastic syndromes, non-Hodgkin T-cell lymphoma and chronic myelomonocytic leukemia]. Previous anti-cancer therapy was received by 36 patients. Table 1 shows main baseline characteristics of BPDCN patients.

Regarding first-line treatment, 34 patients (50%) received AML-like regimens [19 underwent anthracycline and cytarabine (3+7 regimen), 4 FLAGIDA, 4 received FLUGA (fludarabine plus low-dose cytarabine), 1 cytarabine alone, and 1 azacitidine alone]; 9 (13%) acute lymphoblastic leukemia-like regimens; 8 patients (12%) were treated with lymphoma-like regimens; 6 (9%) received medication within clinical trial; 2 (3%) compassionate use of Tagraxofusp, and 9 (13%) supportive care only.

Among 59 treated patients, front-line treatment resulted in 27 (47%) complete remission, 1 (2%) morphologic leukemia-free state (MLFS), 6 (10%) partial remission, 14 (24%) showed resistance, 7 (12%) died during induction (causes of death were cerebral bleeding [3] and multiorgan failure [4]), and 4 (7%) had not available response. Of among 42 treated patients with available post-remission treatment, 9 (21%) received an allogeneic hematopoietic stem cell transplant in first CR. With a median follow-up of 9.6 months, the median overall survival of the cohort was 8.58 months. Median OS was 6 months in patients not transplanted and not reached among those transplanted in first CR (p=0.000695) (Figure 1).

Conclusions

Our preliminary analysis confirms the dismal prognosis of this rare entity (median OS of 8.6 months). We found large heterogeneity of therapeutic approaches, low complete remission rates and scarce rates of allogeneic transplant in the front-line. Improvement of outcomes through well-defined treatment protocols and innovative agents are needed for this high unmet need disease.

Disclosures Bergua Burgues: *Hospital San Pedro de Alcántara. Servicio de Hematologia. Cáceres. SPAIN:* Current Employment; *Daychii:* Consultancy; *Fundesalud. Grants of Europena funds.Daychii:* Research Funding. **Martinez-Cuadron:** *Otsuka:* Consultancy, Other: Travel, Accommodations; *Astellas:* Consultancy, Speakers Bureau; *Pfizer:* Other: Travel, Accommodations. **Montesinos:** *Janssen:* Speakers Bureau; *Celgene:* Consultancy; *Daiichi Sankyo:* Consultancy, Research Funding; *BMS:* Consultancy, Other, Research Funding; *Pfizer:* Consultancy, Research Funding, Speakers Bureau; *Abbvie:* Consultancy, Research Funding, Speakers Bureau; *Jazz pharma:* Consultancy, Research Funding, Speakers Bureau; *Menarini-Stemline:* Consultancy, Research Funding; *Kura oncology:* Consultancy; *Ryvu:* Consultancy; *Astellas:* Consultancy, Speakers Bureau; *Novartis:* Consultancy, Research Funding; *Takeda:* Consultancy, Research Funding; *GILEAD:* Consultancy; *OTSUKA:* Consultancy; *BEIGENE:* Consultancy; *INCYTE:* Consultancy; *NERVIANO:* Consultancy.

Figure 1. Overall survival (OS) in patients undergoing allogeneic steam cell transplant (allo-SCT) [green] versus non-allo-SCT.

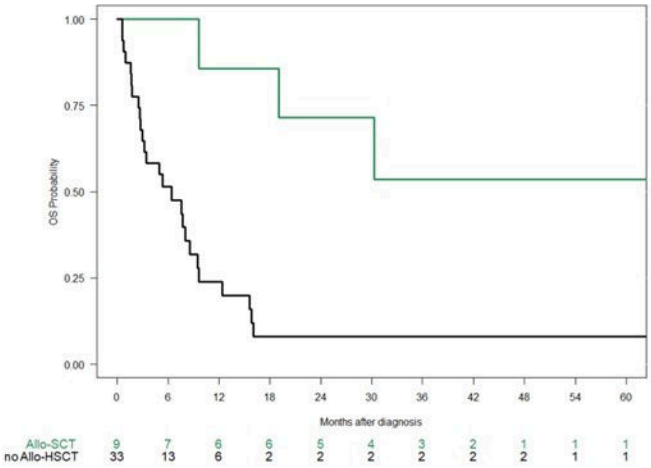

Table 1. Demographic and baseline characteristics of the study population.

	n (%)
Total	**68 (100)**
Median age, years (range)	65 (16-87)
Gender	
Male	55 (81)
Female	13 (19)
ECOG	
≤2	53 (78)
>3	15 (22)
Prior anti-cancer therapy	
Yes	36 (53)
No	32 (47)
Extramedullary involvement (excluding skin) (n = 21)	
Adenopathy	9 (43)
Splenomegaly	8 (38)
Hepatomegaly	3 (14)
Nervous central system	1 (5)
Lab parameters	
Hemoglobine, g /dL (range)	9.6 (5.6-16.8)
Leukocytes, 10^3/µL (range)	8.1 (0.8-125.6)
Platelets, 10^3/µL (range)	102 (7-335)
Peripheral blood blasts, % (range)	18 (0-95)
Bone marrow blasts, % (range)	63 (0-96)
Serum LDH, IU/L (range)	316 (137-2447)
Serum creatinine, mg/dL(range)	1.0 (0.5-5.0)
Serum uric acid, mg/ dL (range)	5.4 (2.72-10.2)
Frontline treatment regimen (n = 58)	
Acute myeloid leukemia regimen (n = 33)	
3+7 (Ara-C + Anthracyclines)	18 (31)
FLUGA[a]	5 (9)
FLAG-IDA[b]	4 (7)
Supportive care	4 (7)
Cytarabine	1 (2)
Azacitidine	1 (2)
Acute lymphoblastic leukemia regimen (n = 9)	
Lymphoma- like regimen (n = 8)	
CHOP[1]	6 (75)
Etoposide + Cytarabine	1 (12)
SMILE[2]	1 (12)
Clinical trial (n = 6)	
Anti-CD123 monoclonal antibody (IMGN-632)	4 (50)
Decitabine plus anti CD123	1 (13)
Decitabine plus anti CD33	1 (13)
Compassionate (n = 2)	
Tagraxofusp	2 (100)
Stem cell transplantation	
Total	**43 (100)**
Yes	10 (23)
No	33 (77)

[1]**CHOP**: cyclophosphamide, doxorubicin, vincristine and prednisolone ; [2]**SMILE**: dexamethasone, methotrexate, ifosfamide, L-asparaginase, and etoposide ; [a]**FLAGIDA**: fludarabine, cytarabine, idarubicin and G-CSF; [b]**FLUGA**: fludarabine, cytarabine and G-CSF

Figure 1

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