



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

616.ACUTE MYELOID LEUKEMIAS: INVESTIGATIONAL THERAPIES, EXCLUDING TRANSPLANTATION AND CELLULAR IMMUNOTHERAPIES**Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN): Multi-Center Collaboration and Global Registry Program**

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Background

BPDCN is a rare, aggressive hematologic malignancy derived from the precursors of plasmacytoid dendritic cells (pDC), typically presenting with primary cutaneous dissemination and involvement of bone marrow (BM) as well as extramedullary sites including central nervous system (CNS). In 2008, it was recognized by WHO as a distinct entity and is now recognized under Myeloid/Histiocytic/Dendritic neoplasms in the 2022 5th edition update of WHO Classification. Since the recognition of this unique entity, recent efforts have been put forward for greater understanding and international collaboration, however usually focused on North America and Europe (Pemmaraju et al and Pagano et al), with this current effort representing the largest known international efforts including more parts of the world, including Asia, Middle East and Africa.

Objective

Due to its historical rarity and heterogeneous presentation of disease, as well as differences in availability of therapies across geo-locations, at present there is no worldwide consensus on the management of BPDCN. The outcomes remain poor, and the optimal therapy of disease remains to be determined. Launched on July 1st, 2022, the aim of our global registry is through a multi-center international collaboration to build a large database of patients with BPDCN in order to generate data-driven diagnostic and treatment recommendations.

Materials and Methods

The registry collects retrospective and prospective data on clinical presentation, diagnostics, treatment regimens and outcomes of patients diagnosed with BPDCN after January 1st, 2010. The data is collected globally through eCRF and Excel form. The Registry is included in ClinicalTrials.gov database (NCT05430971).

Results

Through July 15th, 2023, 15 centers from 11 countries (Armenia, Canada, Cyprus, Egypt, Georgia, India, Italy, Taiwan, Turkey, UK and USA) have joined the registry and another 13 centers from USA, UK, Egypt, Iraq, Canada, Netherlands, Tajikistan, Czech Republic, Guatemala, Brazil, Serbia, Germany are onboarding. 27 retrospective and 2 prospective pts are currently included in the registry. 76 % of pts are male, in keeping with historical expectation BPDCN. Median age at diagnosis was 62 years (4-97). In 90 % of patients the tumor cells were triple positive with CD4+, CD56+, CD123+ immunophenotype. 53.6% of patients presented with cutaneous manifestation. 21% of pts received the initial treatment with AML-based regimens, 66% with ALL-based and 7% with lymphoma-based regimens. 21% of pts underwent alloSCT. 35% of pts were diagnosed after 2018, only 1 patient has received tagraxofusp, a CD123-directed therapy that received FDA approval for previously untreated or relapsed/refractory BPDCN in 2018 (Pemmaraju N et al NEJM 2019). 69 % of patients achieved complete response (CR), from which 90 % were treated with ALL-based regimens. 52% of patients experienced relapse.

Conclusion

Current analysis of a 29-patient worldwide cohort of BPDCN demonstrates a high degree of heterogeneity in treatment regimens based on a multitude of considerations including geographic, socio-economic, drug availability, and varied clinical preferences. The outcome of ALL-based treatment was superior as compared to AML-based, but relapse rate remained high. Further global collaboration is needed to collect additional data and to identify the best diagnostic and therapeutic approaches for this challenging disease.

Disclosures 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. **Alencar:** Genentech: Membership on an entity's Board of Directors or advisory committees; SeaGen: Membership on an entity's Board of Directors or advisory committees; Epizyme: Membership on an entity's Board of Directors or advisory committees; Janssen: Membership on an entity's Board of Directors or advisory committees; Incyte: Membership on an entity's Board of Directors or advisory committees; Beigene: Membership on an entity's Board of Directors or advisory committees; TG Therapeutics: Membership on an entity's Board of Directors or advisory committees; Amgen: Membership on an entity's Board of Directors or advisory committees; Lilly: Membership on an entity's Board of Directors or advisory committees; Dr Reddy: Honoraria. **Owen:** F. Hoffmann-La Roche Ltd, AbbVie, Astrazeneca, Beigene, Merck, Incyte, Seattle Genetics, Novartis: Honoraria. **Kazandjian:** Sanofi: Consultancy, Honoraria; Plexus Communications: Ended employment in the past 24 months, Honoraria; MMRF: Ended employment in the past 24 months, Honoraria; MJH Life Sciences: Current Employment, Honoraria; Karyopharm Therapeutics: Current Employment, Speakers Bureau; Curio Science: Ended employment in the past 24 months, Honoraria; Bristol Myer Squibb: Consultancy, Honoraria; Bridger Consulting Group: Consultancy, Honoraria; Arcellx: Consultancy, Current Employment, Honoraria; Aptitude Health: Consultancy, Honoraria; Aperture Medical Technology, LLC: Consultancy, Honoraria; Alphasights: Consultancy, Honoraria. **Martelli:** Abbvie: Consultancy, Honoraria; Amgen: Consultancy, Honoraria; BMS: Consultancy, Honoraria; Jazz Pharmaceuticals: Consultancy, Honoraria; Pfizer: Consultancy, Honoraria; Laboratoires Delbert: Consultancy, Honoraria. **Voso:** Celgene/BMS: Research Funding, Speakers Bureau; Novartis: Research Funding; Astellas: Speakers Bureau; Jazz: Speakers Bureau; Abbvie: Speakers Bureau; Novartis: Speakers Bureau; Astra Zeneca: Speakers Bureau; Celgene/BMS: Other: Advisory Board; Jazz: Other: Advisory Board; Syros: Other: Advisory Board. **Pemmaraju:** 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; CTI BioPharma: Consultancy, Membership on an entity's Board of Direc-

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