(PB2800) EFFICACY AND SAFETY OF LIPOSOMAL MITOXANTRONE COMBINED WITH VENETOCLAX, AZACITIDINE, AND CHIDAMIDE (VACM) IN ACUTE MONOCYTIC LEUKEMIA

Topic: 04. Acute myeloid leukemia - Clinical

Dairong Xie*[1], Qiuling Wu[2]

[1]Union Hospital, Tongji Medical College, Huazhong University of Science and Technology, Wuhan, China., Institute of Hematology, Wuhan, China; [2]Union Hospital, Tongji Medical College, Huazhong University of Science and Technology, Wuhan, China., Institute of Hematology,, Wuhan, China;

Background

Acute monocytic leukemia (AML-M5) is a distinct and clinically challenging subtype of acute myeloid leukemia (AML). It is characterized by inherent resistance to standard azacitidine plus venetoclax (Aza+Ven) therapy, manifesting as lower response rates and a higher propensity for relapse. Liposomal mitoxantrone offers advantages through enhanced drug delivery, extended circulation time for better tumor accumulation, and reduced systemic toxicity, making it suitable for combination therapy in AML-M5.

Aims

This study aimed to evaluate the efficacy and safety of Liposomal Mitoxantrone combined with Venetoclax, Azacitidine, and Chidamide (VACM) regimen in treating acute monocytic leukemia, with primary focus on complete remission rate (CR), MRD negativity, and treatment response.

Methods

This prospective, multicenter clinical study was conducted across two centers including Union Hospital, Tongji Medical College, Huazhong University of Science and Technology and another participating center. The study included AML-M5 patients treated with VACM regimen between 2025-2026. The treatment protocol consisted of Liposomal Mitoxantrone (20-24 mg/m², ivgtt, d1), azacitidine (75 mg/m², i.v. or i.h., d1-7), venetoclax (100 mg d1, 200 mg d2, 400 mg d3-7, p.o.), and chidamide (30 mg d3, d7, p.o.). Response assessment included morphological evaluation and MRD detection by flow cytometry. Primary endpoints were CR rate and MRD negativity, with secondary endpoints including overall response rate (ORR) and treatment safety.

Results

Thirteen AML-M5 patients were analyzed (10 males, 3 females; median age 50.5 years, range 15–66). With a median follow-up of 12 months (IQR 6–18), the CR rate was 92.31% (12/13), and the ORR was 92.31% (12/13). Notably, 84.62% (11/13) of evaluable patients achieved MRD negativity. Among CR patients, the median time to MRD negativity was 14 days (range 5–39). The safety profile was manageable, characterized by predictable and reversible hematological toxicity. The primary adverse event was Grade 3/4 neutropenia, with a median duration of bone marrow suppression of 18 days (range 14–21). Crucially, no treatment-related cardiotoxicity or unexpected Grade 3/4 non-hematological toxicities were observed, confirming the safety advantage of the liposomal formulation.

Summary/Conclusion

The VACM regimen demonstrates promising efficacy in AML-M5, achieving high CR and MRD negativity rates. These preliminary findings suggest that VACM may represent a viable therapeutic alternative for AML-M5 patients with poor response to standard Aza+Ven regimens, warranting validation in larger prospective studies.