(PF393) UPDATED RESULTS FROM THE PHASE II STUDY OF DUAL EPIGENETIC TARGETING WITH CHIDAMIDE AND AZACITIDINE IN PATIENTS WITH HIGH-RISK ACUTE MYELOID LEUKEMIA AFTER ALLO-HSCT

Topic: 02. Acute lymphoblastic leukemia - Clinical

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Background

Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is potentially the only curative option for high-risk acute myeloid leukemia (AML) patients. However, disease relapse remains the principal cause of treatment failure of these patients, and outcomes of salvage treatments are poor. This research seeks to evaluate the efficacy and safety of a dual epigenetic targeting maintenance therapy with chidamide and azacitidine (AZA) in patients with high-risk AML post-allo-HSCT.

Aims

To evaluate the efficacy and safety of a dual epigenetic targeting maintenance therapy with chidamide and azacitidine (AZA) in patients with high-risk AML post-allo-HSCT.

Methods

This multicenter, open-label, phase 2 prospective clinical trial (ChiCTR2300067593) recruited and followed up 52 patients diagnosed with high-risk AML post-allo-HSCT from 3 hospitals in China from November 2021 to October 2024. Chidamide (5 mg) was administered orally once daily for 5 days, combined with AZA (75 mg/m2) subcutaneously daily for 5 days, respectively. Treatment started as early as 3 months after transplantation. All patients were in complete remission before each maintenance cycle. A total of 6 cycles was recommended.

Results

The median age of the patients was 39 years (range, 17-60). As of the cutoff date for the final survival analysis on January 8, 2026, the median follow-up time was 38.8 (12.6-56.5) months, the 3-year CIR was 7.7 (2.6-9.0) %, the 3-year OS and RFS rates were 91.4 (87.2-95.6)% and 90.4 (86.3-94.5) %, respectively. Four patients relapsed: one declined intensive treatment and died; two relapsed post-second transplantation and died; one remains alive in remission following second transplantation. The most common AE was hematological toxicity, with an incidence of 86.5% (45/52). However, most patients experienced only mild bone marrow suppression, which required either outpatient administration of hematopoietic growth factors or simple observation without medication. Only 11 (21.2%) patients received platelet transfusions, and 1 (1.9%) patient received red blood cell transfusions. Among the 6 infected patients, 4 had pulmonary infections, including 1 patient with EB viremia reactivation. Additionally, 1 patient had an intestinal infection following unclean eating, and 1 had cytomegaloviremia. All AEs were tolerable with active treatment and did not affect subsequent therapy. No grade>3 AEs were noted. Overall, 15 (28.8%) patients developed cGVHD and only 1 of them developed aGVHD. One patient died of lung GVHD, and non-relapse mortality (NRM) was 1.9 (1/52) %.

Summary/Conclusion

The results of our study suggested that dual epigenetic targeting maintenance treatment with CHI-AZA has an acceptable toxicity profile and might potentially be effective to prevent relapse after allo HSCT.