VENETOCLAX-BASED THERAPY IS ASSOCIATED WITH IMPROVED SURVIVAL IN ACUTE MYELOID LEUKEMIA: A LARGE REAL-WORLD STUDY

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INTRODUCTION

Venetoclax combined with a hypomethylating agent (HMA) is a standard option for patients with acute myeloid leukemia (AML) who are ineligible for intensive chemotherapy. Although randomized trials established its efficacy, comparative effectiveness in routine clinical practice remains incompletely defined.

AIM

To compare overall survival, early mortality, and healthcare utilization among adults with AML treated with venetoclax plus HMA versus HMA alone in a large real-world population.

METHOD

- Retrospective, propensity score–matched cohort study using the TriNetX federated electronic health record network, comprising 110 healthcare organizations.
- Adults with HLH were included if HLH was diagnosed within one month before or after lymphoma diagnosis.
- Initial unmatched cohorts included 1,262 patients with B-cell lymphoma–associated HLH and 528 with T/NK-cell lymphoma–associated HLH. Cohorts were matched 1:1 for demographics and baseline comorbidities.
- Outcomes were assessed from one month through five years after HLH diagnosis.

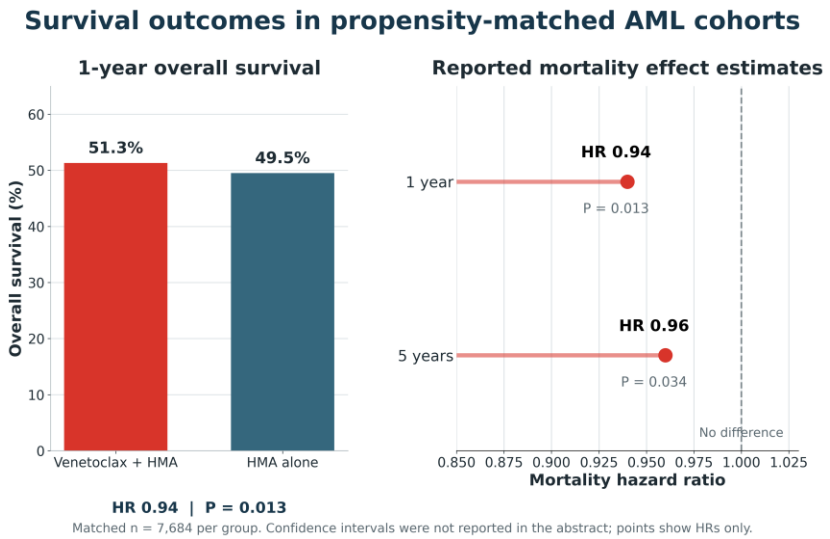
RESULTS

At 1 year, overall survival was 51.3% with venetoclax plus HMA versus 49.5% with HMA alone (HR for mortality, 0.94; P = 0.013).

The mortality association remained modest but significant at 5 years (HR, 0.96; P = 0.034).

Thirty-day mortality was similar between groups.

Hospitalization, infections, and overall healthcare utilization did not differ significantly across the assessed timepoints.



CONCLUSIONS

In this large propensity-matched real-world cohort, venetoclax-based therapy was associated with modestly improved survival compared with HMA alone.

The findings support its effectiveness in routine AML care, while the small effect size and observational design underscore the need to define which patient subgroups derive the greatest benefit

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