

TREATMENT PATTERNS AND MOLECULAR TESTING PATTERNS IN A U.S.-BASED COHORT OF NEWLY DIAGNOSED AND RELAPSED/REFRACTORY AML PATIENTS: A REAL-WORLD ANALYSIS

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Key Takeaway

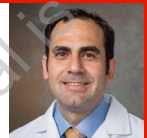
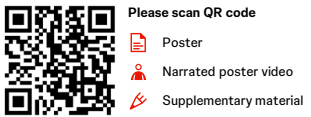
Treatment options remain limited for older and more frail patients with AML; appropriate molecular testing prior to treatment initiation is critical as targeted treatments for these populations become available

Conclusions

While molecular testing rates are generally high within 12 months of an AML diagnosis, approximately 1 in 3 treated in the 1L setting did not have clear evidence of molecular testing prior to treatment initiation.

Despite rapid uptake of newly available AML treatment options, the proportion of patients who do not receive any treatment remained persistently high over 2016-2022.

Our analysis suggests treatment options remain limited for older and more frail patients with AML.



Introduction

- The AML treatment landscape is expanding rapidly, with twelve new therapies approved in the United States since 2017.¹
- The advent of driver-mutation-targeted and low-intensity therapeutic agents has expanded the range of available treatment options, particularly for patients with unfavorable risk profiles and those unfit for traditional chemotherapy.¹
- Clinical guidelines increasingly recommend appropriate molecular testing for treatment and disease management of AML patients.²
- We characterize evolving molecular testing and treatment patterns in a U.S.-based cohort of newly diagnosed (ND) and relapsed/refractory (RR) AML patients from 2016-2022

Methodology

Data Source and Study Population

- Data source:** Optum Clinformatics® Data Mart
- Population:** Adult patients (≥18 years) with newly diagnosed (ND) AML from January 2016 to August 2022. A subpopulation of refractory/relapsed (RR) patients was identified from the ND cohort.
- ND population inclusion criteria:** continuous health plan enrollment from ≥1 year before through 30 days after the index date or death, whichever was earlier; ≥2 confirmatory diagnoses within 60 days of index date.
- RR subpopulation inclusion criteria:** RR AML diagnosis code after ND index date, continuous health plan enrollment between the ND index date and ≥30 days after RR index date (i.e., date of first RR AML diagnosis) or death, whichever was earlier.
- Exclusion criteria:** ≥2 diagnoses for non-AML, non-MDS hematologic malignancy within ≤30 days of the index date, non-MDS antineoplastic treatment, or clinical trial enrollment within 1 year before index date, receipt of first-line (1L) HSCT after the ND index date (for RR subgroup only).

Outcome Measures

- Baseline characteristics:** Patient demographic and clinical characteristics were assessed at baseline, with clinical conditions assessed using claims data up to one year before the index date.
- Identification of treatment regimens and molecular testing:** Molecular testing was identified using CPT codes from published CMS billing and coding guidelines. Treatment regimens were identified using a combination of ICD, CPT, HCPCS, DRG, GPI, and NDC codes, and classified based on a time-based hierarchical classification algorithm.
- Overall Survival (OS):** time to all-cause mortality using the Kaplan-Meier estimator.

Statistical Analysis

- Demographic and clinical characteristics were reported descriptively. Treatment pattern distributions were described by index year; timing of molecular testing was described in relation to key clinical timepoints. All analyses were stratified by line of therapy and treatment status

Results

Study Cohort and Patient Characteristics

- 5,135 patients with ND AML and 987 patients with RR AML were identified over the study period.
- For the ND cohort, mean age at the initial AML diagnosis was 74 years (Table 1).
- Patients with ND AML receiving transplant in 1L were younger and had fewer comorbidities than those receiving non-transplant therapy (61.1 vs 73.8 years; 2.0 vs. 2.8 CCI score; Table 1).

TABLE 1: Baseline patient characteristics

	ND AML cohort (n=5,135; 5,005 excluding RCT-enrolled patients)			RR AML cohort (n=987; 964 excluding RCT-enrolled patients)		
	No tx in 1L (n=1,860)	Non-HSCT tx in 1L (n=3,038)	HSCT in 1L (n=107)	No tx in 2L; no prior HSCT (n=260)	Non-HSCT tx in 2L; no prior HSCT (n=515)	HSCT in any LOT (n=189)
Mean age at diagnosis (SD)	77.8 (10.1)	71.4 (12.5)	61.1 (11.8)	75.2 (9.9)	71.4 (11.9)	59.6 (12.4)
Female, %	855 (46%)	1315 (43%)	48 (45%)	113 (43%)	226 (44%)	80 (42%)
Race/Ethnicity						
Asian	52 (3%)	85 (3%)	3 (3%)	15 (6%)	17 (3%)	8 (4%)
Black	181 (10%)	297 (10%)	3 (3%)	29 (11%)	61 (12%)	9 (5%)
Hispanic	161 (9%)	284 (9%)	12 (11%)	30 (12%)	48 (9%)	18 (10%)
White	1337 (72%)	2215 (73%)	84 (79%)	175 (67%)	369 (72%)	146 (77%)
Missing	129 (7%)	157 (5%)	5 (5%)	11 (4%)	20 (4%)	8 (4%)
Payer Type						
Medicare	1710 (92%)	2397 (79%)	48 (45%)	236 (91%)	414 (80%)	75 (40%)
Commercial	150 (8%)	639 (21%)	59 (55%)	24 (9%)	101 (20%)	114 (60%)
Unknown	0 (0%)	2 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
CCI score	3.6 (2.5)	2.6 (2.4)	2.0 (1.7)	4.6 (2.7)	4.1 (2.5)	3.6 (2.4)
Poor fitness for high-induction chemotherapy	1614 (87%)	2096 (69%)	38 (36%)	232 (89%)	398 (77%)	77 (41%)
MDS diagnosis, N (%)	454 (24%)	744 (24%)	16 (15%)	112 (43%)	184 (36%)	50 (26%)
Duration of follow-up	3.8 (8.9)	7.9 (9.9)	4.4 (2.4)	3.0 (6.5)	8.1 (9.0)	5.5 (7.6)

Treatment Patterns

- The distribution of treatments changed markedly over the study period; 41% of 1L patients and 31% of 2L+ patients were being treated with novel therapies by the end of the study period in 2022
- Rates of non-treatment remained similar across the study period for both ND and RR patients (36% and 28%, respectively).

FIGURE 1: Percentage (%) of patients receiving no active treatment, by age group and over time

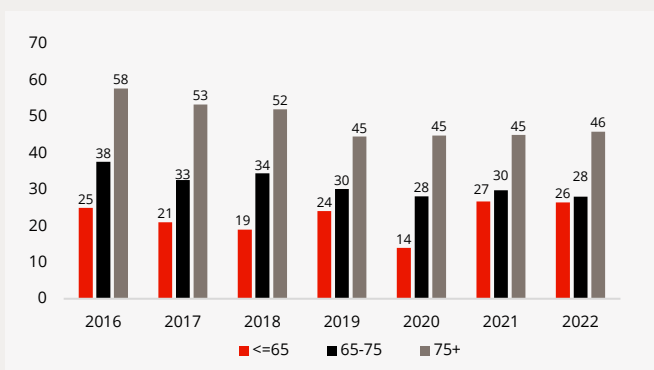
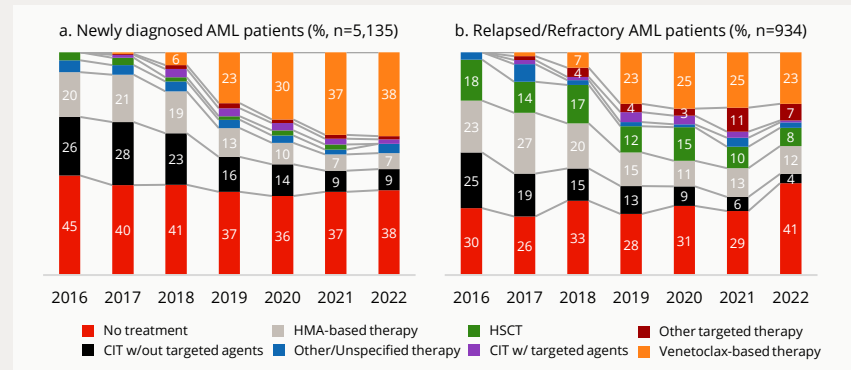
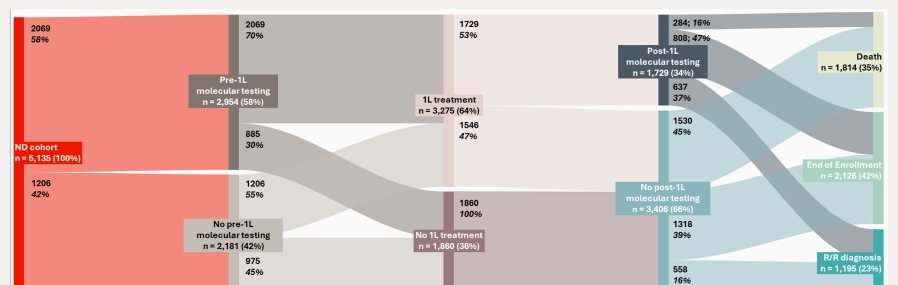


FIGURE 2: Treatment regimen distribution by calendar year

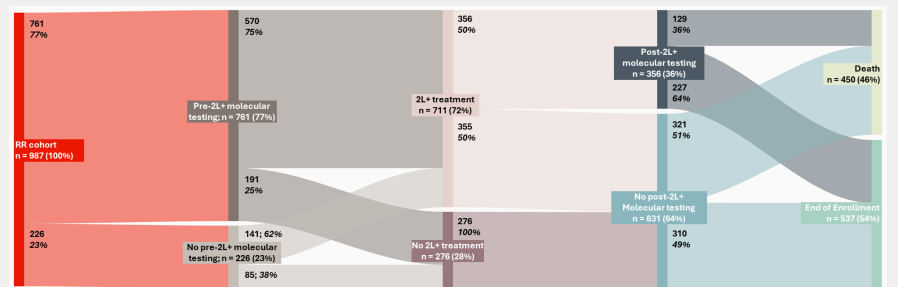


Molecular testing patterns

- Among ND patients receiving some treatment in 1L (n=3,275), 63% had molecular testing performed prior to treatment and 53% had molecular testing performed after 1L treatment.



- Among RR patients (n=987), testing rates after disease recurrence were high (77%), suggesting repeated testing across lines of therapy.



Limitations

- Claims-based identification of molecular testing may not capture procedures billed as part of DRG-based payments, potentially resulting in an underestimate of true molecular testing rates among AML patients receiving inpatient treatment

References

- Forsberg, M., & Konopleva, M. (2024). AML treatment: conventional chemotherapy and emerging novel agents. *Trends in Pharmacological Sciences*.
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