Impact of body mass index (BMI) on response and tolerance to azacitidine (AZA) in myeloid neoplasms (MN)

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Introduction

Myeloid neoplasms (MN) include clonal disorders such as acute myeloid leukemia (AML), chronic myelomonocytic leukemia (CMML), and myelodysplastic syndromes (MDS). Azacitidine (AZA) remains a key therapy for higher-risk MDS, CMML, and selected AML patients unfit for intensive chemotherapy. While its efficacy is established, outcomes may vary according to patient-related factors. Body mass index (BMI) has been linked to both MN incidence and variable treatment responses, yet its impact on AZA efficacy and tolerance remains unclear. Understanding this relationship may improve prognostic evaluation and guide personalized management.

Objective

To evaluate whether BMI influences the efficacy, survival, and tolerance of AZA) in patients with MN (AML, MDS, CMML) treated in a single-centre haematology-oncology cohort.

Methods

Retrospective study of 168 adults treated with AZA (2008-2024). Patients categorised by BMI (kg/m²) : <18.5 (UW), 18.5-24.9 (NO), 25-29.9 (OW) and ≥30 (OB). Primary endpoints were overall response rate (ORR), overall survival (OS), progression-free survival (PFS), transfusion dependence and ≥ grade 3 haematologic toxicities. Comparisons were performed using χ^2 tests, Kaplan-Meier curves with log-rank test, and multivariable Cox regression models.

Results

AML Results : Among 100 AML patients (54 men, median age 66 y; 6% UW, 47% NO, 36% OW, 10% OB), most had de novo disease (87%) and unfavourable ELN risk (58%). The overall response rate was 37.8% (NO 43.1%, OW 43.4%, OB 10%), with complete responses only in NO (35%) and OW (38%) only. Median time to best response was 2.1 months (shortest in NO 1.7 m). Progression-free survival was 3.9 months and **significantly longer across BMI groups** (p = 0.0071), while overall survival (8.6 months) showed no BMI association (p = 0.25). Grade ≥3 toxicities occurred in 30% (mainly neutropenia 20.2%, thrombocytopenia 11.5%, anaemia 4.8%) and dose reductions in 35.6%, both without BMI impact.

MDS/CMML Results : The MDS/CMML cohort (n = 68; median age 72 y; 4% UW, 41% NO, 37% OW, 18% OB) showed an overall response rate of 62.2%, independent of BMI, with a median time to best response of 3.5 months. Median overall survival was 15.4 months (p = 0.21), but patients with BMI ≥ 27.5 kg/m² had longer OS (21.8 vs 12.8 months, p = 0.012; NS in multivariate). Progression-free survival was 12.6 months (p = 0.25). Grade ≥3 toxicities occurred in 34%, with thrombocytopenia significantly more frequent in UW and OB (p = 0.0238), while neutropenia and anaemia showed no BMI association.

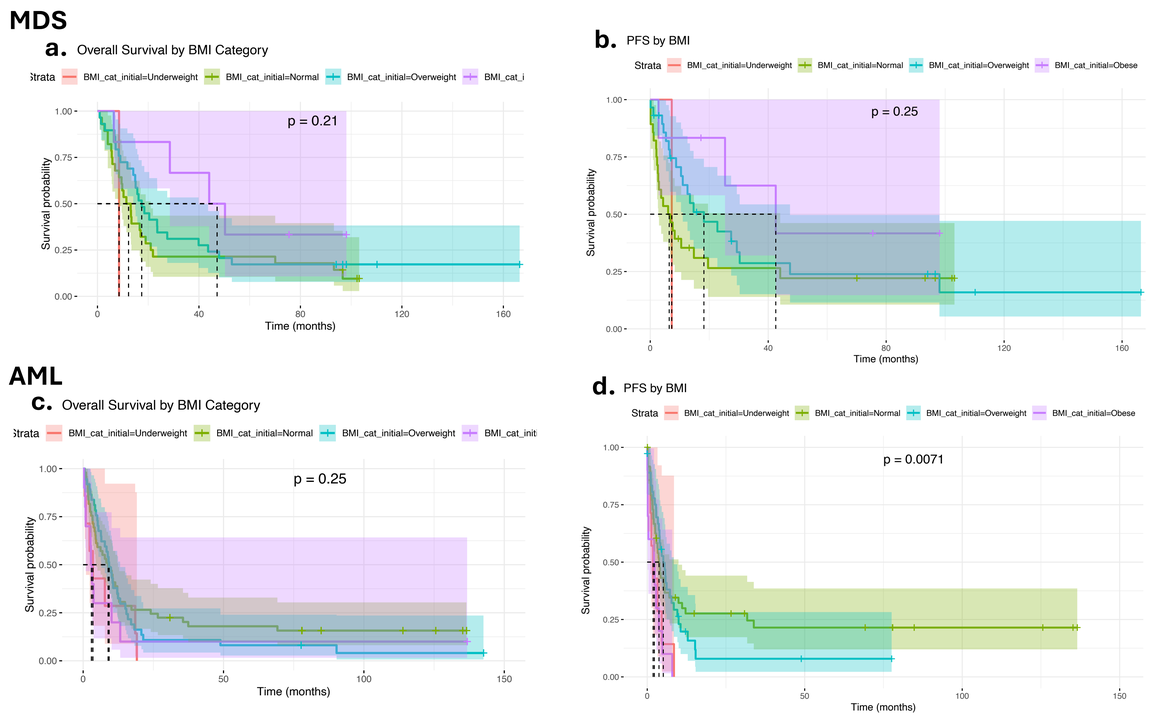

Figure 1. *Overall survival (OS) and progression-free survival (PFS) according to BMI categories in patients with MDS, CMML and AML treated with AZA.* (a) OS in MDS/CMML. (b) PFS in MDS/CMML. (c) OS in AML. (d) PFS in AML.
Kaplan-Meier survival curves are shown for underweight (UW), normal weight (NO), overweight (OW) and obese (OB) patients.

a.

	Best response							
	CR	PR	marrow CR	SD	PD	Total	p-value†	ORR (CR+PR+marrow CR)
BMI category at diagnosis							0.4	
Underweight	0 (0%)	1 (100%)	0 (0%)	0 (0%)	0 (0%)	1 (100%)		1 (100%)
Normal	3 (11%)	12 (43%)	1 (3.6%)	4 (14%)	8 (29%)	28 (100%)		16 (57.1%)
Overweight	8 (28%)	9 (31%)	0 (0%)	8 (28%)	4 (14%)	29 (100%)		17 (58.6%)
Obese	1 (17%)	4 (67%)	0 (0%)	0 (0%)	1 (17%)	6 (100%)		5 (83.3%)
Unknown	3 (30%)	4 (40%)	0 (0%)	3 (30%)	0 (0%)	10 (100%)		
Total	15 (20%)	30 (41%)	1 (1.4%)	15 (20%)	13 (18%)	74 (100%)		

† Fisher's exact test

b.

	Best response							
	CR	PR	MLFS	No response	Not evaluated	Total	p-value†	ORR (CR+PR+MLFS)
BMI category at diagnosis								
Underweight	1 (14%)	0 (0%)	0 (0%)	4 (57%)	2 (29%)	7 (100%)		1 (14.3%)
Normal	17 (35%)	3 (6.1%)	1 (2.0%)	14 (29%)	14 (29%)	49 (100%)		21 (42.9%)
Overweight	14 (38%)	2 (5.4%)	0 (0%)	10 (27%)	11 (30%)	37 (100%)		16 (43.2%)
Obese	0 (0%)	1 (10%)	0 (0%)	2 (20%)	7 (70%)	10 (100%)		1 (10%)
Unknown	0 (0%)	0 (0%)	0 (0%)	1 (100%)	0 (0%)	1 (100%)		
Total	32 (31%)	6 (5.8%)	1 (1.0%)	31 (30%)	34 (33%)	104 (100%)		

† Fisher's exact test

Figure 2. Best response and overall response rate (ORR) to AZA by BMI category in MDS/CMML (a) and AML (b) cohorts. Complete response (PR,) partial response (PR), and marrow leukemia-free state (MLFS) are reported according to baseline BMI.

Conclusion

BMI showed no independent impact on response, survival or tolerance to AZA. Although longer survival was seen in high-BMI MDS/CMML patients and better AML response in NO/OW groups, these associations disappeared after multivariable adjustment. BMI alone should not guide AZA dosing. Standard dosing remains appropriate, and prospective studies including body-composition metrics are warranted.