Evaluation of the efficacy and safety of guadecitabine in patients with high-risk myelodysplastic syndrome or low blast count acute myeloid leukemia refractory or relapsing after azacitidine

GFM sponsored multicenter (13 French centers) phase II clinical trial

56 patients with acute myeloid leukemia refractory or relapse after azacitidine (AZA)

Guadecitabine (SGI-110) (60 mg/m2 subcutaneously, on days 1-5 of 28-day treatment cycles)

Primary endpoints

			(n)
Median number of treatments	3 (0-27)	Only 1 cycle	1
		Dose reduction	18
Treatment response	8 (14.3%)	Complete response	2
		Partial response	1
		Hematological improvements	3
		Marrow complete response	2

Secondary endpoints

Median duration of response	11.5 months	IC95%: [9; NA]
Median overall survival (OS)	7.1 months	IC95%: [5.6; 11.8]
1-year survival	33%	IC95%: [22.9; 48.4]
Median OS in responders to guadecitabine	17.9 months	

Multivariate analysis

International prognostic scoring system (IPSS-R) (p=0.03)
Demetylation rate in blood (p=0.03)
Type of AZA failure (Primary vs secondary, p=0.01)

→ Longer survival

Sébert *et al.*, Haematologica, 2019