

Table 1. *Pivotal phase 3 trials and selected investigational strategies in myeloid neoplasms.*

Disease/ Setting	Trial (Phase)	Population	Intervention vs Control	Primary Endpoint	Key Result	Key Safety Signal	Ref
LR-MDS, TD, RS+	MEDALIST (III)	ESA-refractory	Luspatercept vs placebo	TI≥8 weeks	38% vs 13%	Fatigue, GI AEs	[18]
LR-MDS, ESA-naïve	COMMANDS (III)	TD	Luspatercept vs epoetin alfa	TI + Hb↑	Superior TI & durability	Favorable safety	[19]
LR-MDS, post-ESA	IMerge (III)	TD, non-del(5q)	Imetelstat vs placebo	TI ≥8 weeks	40% vs 15%	Neutropenia, thrombocytopenia	[20]
HR-MDS	AZA-001 (III)	Untreated HR	Azacitidine vs CCR	OS	OS benefit	HMA-related cytopenias	[24]
HR-MDS/CMML	PANTHER (III)	Untreated HR	Pevonedistat + AZA vs AZA	EFS	NS	Manageable toxicity	[28]
ND AML (unfit)	VIALE-A (III)	Newly diagnosed	AZA + Ven vs AZA	OS	14.7 vs 9.6mo	Myelosuppression	[36]
ND AML IDH1-mut	AGILE (III)	Unfit	AZA+ ivosidenib vs AZA	OS	24.0 vs 7.9 mo	Differentiation syndrome	[38]
R/R AML FLT3-mut	ADMIRAL (III)	Relapsed	Gilteritinib vs chemo	OS	OS 9.3 vs 5.6 mo	Cytopenias, QT	[40]
ND AML FLT3-ITD	QuANTUM-First (III)	Intensive	Quizartinib + “7+3” vs “7+3”	OS	31.9 vs 15.1 mo	QT prolongation	[41]
R/R AML	FF-10101 (I)	Post-FLT3i	Covalent FLT3 inhibitor	Safety	Early activity	Investigational	[42]
R/R KMT2A/NPM1	AUGMENT-101 (II)	Pretreated	Revumenib	CR/CRh	MRD-neg responses	Differentiation syndrome	[43,44]

Abbreviations: AML, acute myeloid leukemia; AZA, azacitidine; CCR, conventional care regimens; CR/CRh, complete remission/complete remission with partial hematologic recovery; EFS, event-free survival; HR, higher risk; LR, lower risk; OS, overall survival; RS+, ring sideroblast-positive; TD, transfusion-dependent; TI, transfusion independence; VEN, venetoclax.