

Table 1. Agents under active investigation in patients with myelodysplastic syndromes (MDS)

Mechanism	Agent	NCT identifier	Phase	Relevant study population	Status
Epigenetic regulators					
Hypomethylating agents	Guadecitabine	#NCT02935361	II	Int-1 or HR-MDS + HMAf	Recruiting
		#NCT02131597	II	HR-MDS	Active, not recruiting
		#NCT02907359	III	MDS + HMAf	
Histone deacetylase inhibitors	Vorinostat, mocetinostat, panobinostat, etc.	No active studies in this population			
Mutant IDH1/2 inhibitors	Enasidenib	#NCT03383575	II	mIDH2 MDS +/- HMAf	Recruiting
		#NCT03744390	II		
	Ivosidenib	#NCT02074839	I	mIDH1 r/r MDS	
		#NCT03471260	Ib/II	IDH1-mutant MDS	
		#NCT03503409	II	mIDH1: HR-MDS, treatment-naïve MDS, ESA-resistant LR-MDS	
LSD1 inhibitors	Tranylcypromine	#NCT02273102	I	R/r MDS	Active, not recruiting
		#NCT02717884	II	Int-/HR-MDS + HMAf	Recruiting
	GSK2879552	No active studies			
Signal transduction regulators					
TGF-beta signaling modulators	Galunisertib	No active studies in this population			
	Sotatercept				
	Luspatercept	#NCT02631070	II	Very low, low, or int-risk MDS refractory to ESA	Active, not recruiting
#NCT03682536		III	Very low, low, or int-risk MDS in ESA-naïve	Recruiting	
TLR inhibitors	Tomaralimab (OPN-305)	No active studies in this population			
Multi-kinase inhibitors	Rigosertib	#NCT01926587	I/II	Int-1, Int-2 (Int-2) or HR-MDS	Active, not recruiting
		#NCT01904682	II	LR or Int-1 risk-MDS	
		#NCT01928537	III	MDS+excess blasts + HMAf	
		#NCT01241500	III	Very high-risk MDS + HMAf	Recruiting
#NCT02562443	III				
FLT-3 inhibitors	Midostaurin	#NCT00819546	I	R/r MDS and AML	Active, not recruiting
	Gilteritinib	No active studies in this population			
	Sorafenib	#NCT02728050	II	HR-MDS	Recruiting
Immunotherapy					
PD-1 inhibitors	Nivolumab	#NCT02530463	II	MDS +/- HMAf	Recruiting
		#NCT02464657	II		
		#NCT03417154	II		
	Durvalumab	#NCT02775903	II	Treatment naïve, HR-MDS	Active, not recruiting
		#NCT02281084	II	MDS + HMAf	
	Pembrolizumab	#NCT02936752	I	MDS +/- HMAf	Recruiting
		#NCT03094637	II	Int-1 or HR-MDS +/- HMAf	
	Atezolizumab	#NCT02935361, see guadecitabine			
CTLA-4 inhibitors	Ipilimumab	#NCT02530463, see nivolumab			
		#NCT02890329	I	MDS +/- HMAf	Recruiting

Anti-CD47 antibody	Hu5F9-G4	#NCT03248479	I	R/r and treatment-naïve MDS	Recruiting
Bispecific T cell engaging antibodies	MCLA-117	No active studies in this population			
	AMG330				
	AMV564	#NCT03516591	I	Int-2 or HR-MDS with HMAf or standard AML CTX	Active, not recruiting
Cell death regulators					
Bcl-2 inhibitors	Venetoclax	#NCT02966782	I	HR-MDS + HMAf	Active, not recruiting
		#NCT04017546	I	MDS with ≥10% blasts	Recruiting
		#NCT02942290	I	Treatment-naïve HR-MDS	
		#NCT03113643	I	HR-MDS	
		#NCT04160052	I/II	HR-MDS +/- HMAf	
		#NCT03404193	II	HR-MDS + HMAf	
		#NCT02115295	II	MDS with ≥10% blasts	
NEDD 8 activating enzyme	Pevonedistat	#NCT03772925	I	HR-MDS + HMAf	Recruiting
		#NCT03813147	I	HR-MDS	
		#NCT03459859	I	MDS +/- HMAf	
		#NCT03814005	I	HR-MDS + HMAf	
		#NCT03238248	II	MDS+ HMAf	
		#NCT02610777	II	HR-MDS	Active, not recruiting
		#NCT03268954	III	HR-MDS	Recruiting
		Other agents			
RNA splicing modulators	H3B-8800	#NCT02841540	I	HR-MDS + HMAf, LR-MDS refractory to ESA	Active, not recruiting
Cytotoxic agents	CPX-351	#NCT02019069	I	HR-MDS + HMAf	Recruiting
		#NCT03896269	I		
		#NCT03957876	II	MDS + HMAf	
		#NCT03672539	II	HR-MDS + HMAf	

CTX = chemotherapy, ESA = erythropoiesis-stimulating agents, HMAf = hypomethylating agent failure, HR = high risk Int = intermediate, LR = low risk, MDS = myelodysplastic syndromes, mIDH = mutant IDH, R/r = relapsed/refractory, TLR = toll-like receptor.