

		Clinical Trials			EMA/MHRA Approval	To Patients
		Phase I (safety)	Phase 2 (efficacy)	Phase 3 (proof)		
SMN-dependent	antisense oligonucleotide (targeting <i>SMN2</i>)					
	Ionis/Biogen Spinraza/nusinersen					
	<i>SMN1</i> gene replacement					
	Novartis Gene Therapies/Novartis Zolgensma/onasemnogene abeparvovec (intravenous)				SMA Type 1 Only *	
	Novartis Gene Therapies/Novartis Zolgensma/onasemnogene abeparvovec (intrathecal)			Phase 3 Recruiting		
	small molecule (targeting <i>SMN2</i>)					
PTC/Roche Evrysdi/risdiplam						
Combination	combined treatments					
	Roche GYM329/RO7204239	PTC/Roche risdiplam		Phase 2/3 Recruiting		
	Scholar Rock SRK-015/apitegromab	nusinersen or risdiplam		Phase 3 Recruiting		
	Biohaven Pharmaceuticals taldefgrobep alfa	nusinersen or risdiplam or onasemnogene abe.		Phase 3 Recruiting		

* Subject to country eligibility criteria