

Drug	Type	Clinical trial phase	Condition (Phase)	Company	Mechanism of Action	NCT#	Aliases
Mipomersen	ASO	Marketed; Discontinued	Hyperlipoproteinaemia type Iia (Marketed)/ Atherosclerosis (discontinued)	Genzyme (Sanofi)	ApoB-100 protein synthesis inhibition	NCT01475825	ISIS-301012;Kynamro;Mipomersen sodium
Volanesorsen	ASO	Marketed; Phase III	Hyperlipoproteinaemia type I (Marketed); Hypertriglyceridaemia; Lipodystrophy (III)	Ionis Pharmaceuticals (Akcea Therapeutics)	ApoC-III protein reduction to regulate plasma triglycerides	NCT02300233	IONIS-304801;IONIS-APOCIII;IONIS-APOCIIIRx;ISIS-304801;ISIS-APOCIII;ISIS-APOCIII-Rx;Volanesorsen;Volanesorsen sodium;WAYLIVRA
Inotersen	ASO	Marketed; Phase II	Amyloid polyneuropathy (Marketed); Amyloidosis,Cardiomyopathies (II)	Ionis Pharmaceuticals (Akcea Therapeutics)	Inhibitor of the hepatic production of transthyretin (TTR) protein	NCT03702829	GSK 2998728; Inotersen; Inotersen sodium; IONIS-TTRRx; ISIS 420915 salt; ISIS-420915; ISIS-GSK1Rx; ISIS-TTRRx; TEGSEDI
Eplontersen	ASO	Phase III	Amyloidosis; Transthyretin-related hereditary amyloidosis; Transthyretin-Mediated Amyloid Cardiomyopathy	Ionis Pharmaceuticals (Akcea Therapeutics)	Reduce the production of transthyretin (TTR) protein	NCT04136171	AKCEA-TTR-LRx;ION-682884;ION-TTR-LRx;IONIS-TTR-LRx
Pelacarsen	ASO	Phase III	Hyperlipoproteinaemia	Ionis-Novartis	Reduce apolipoprotein(a) to reduce Lp(a)	NCT04023552	AKCEA-APO(a)-LRx;IONIS-681257;IONIS-APO(a)-LRx;IONIS-APO-LRx;ISIS 681257;ISIS-APO(a)-LRx;ISIS-APOALRx;Pelacarsen sodium;TQJ 230
Olezarsen (IONIS-APOCIII-LRx)	ASO	Phase III	Hyperlipoproteinaemia type I (III); Hypertriglyceridaemia (III)	Ionis Pharmaceuticals (Akcea Therapeutics)	Inhibit the production of apolipoprotein C-III (ApoC-III) to regulate serum triglycerides	NCT04568434 NCT05079919	AKCEA-APOCIII-LRx;;IONIS-APOCIII-LRx;IONISAPOCIII-LRx, sodium salt;ISIS 678354;ISIS-APOCIII-LRx;Olezarsen sodium
IONIS TMPRSS6 LRx	ASO	Phase II; No development reported	Beta-thalassaemia (II); Thalassaemia (NDR)	Ionis Pharmaceuticals	Reduce the production of transmembrane protease serine 6 (TMPRSS6) to increase red blood cell production	NCT04059406 NCT03165864	IONIS TMPRSS6-Lrx;IONIS-TMPRSS6-LRx;ISIS 702843
Fesomersen	ASO	Phase II	Thrombosis	Ionis-Bayer	Reduce the production of clotting Factor XI	NCT04534114	BAY-2976217;Factor XI LICA;ION-957943;IONIS-FXI-LRX

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IONIS AGT LRx	ASO	Phase II	Chronic heart failure; Hypertension	Ionis Pharmaceuticals	Reduce production of liver-derived angiotensinogen to decrease blood pressure	NCT04836182 NCT04714320	IONIS-AGT-LRx;ISIS 757456;ISIS-AGT-LRx
Donidalorsen	ASO	Phase II	COVID 2019 infections; Hereditary angioedema	Ionis Pharmaceuticals	Reduce the level of prekallikrein (PKK)	NCT04307381 NCT04549922	Donidalorsen sodium;IONIS-PKK-LRx;ISIS 721744
Vupanorsen	ASO	Phase II; Phase I/II	Dyslipidaemias; Hyperlipoproteinaemia type I; Hypertriglyceridaemia; Lipodystrophy; Non-alcoholic fatty liver disease; Type 2 diabetes mellitus (II); Hyperlipoproteinaemia type Iia (I/II)	Ionis-Pfizer	Reduce angiopoietin-like 3 protein & reduce Triglyceride and LDL-C	NCT04516291 NCT03360747 NCT04916795	AKCEA ANGPTL3 LRx;IONIS ANGPTL3 LRx;ISIS 703802 salt;ISIS-703802;PF 07285557;Vupanorsen sodium
AZD 8233	ASO	Phase II Phase I	Dyslipidaemias, Hyperlipidaemia (II); Hypercholesterolaemia (I)	Ionis-AstraZeneca	Reduce plasma levels of proprotein convertase subtilisin/kexin type 9 (PCSK9) and LDL-C	NCT05065463 NCT04823611	AZD-8233;ION-449;IONIS-AZ4-2.5-LRx
CDR 132L	ASO	Phase I	Heart failure	Cardior Pharmaceuticals	Inhibit miR-132 to reduce adverse cardiac remodeling	NCT04045405	CDR-132L
MRG-110	ASO	Phase I (No development reported)	Heart failure; Ischaemia; Wounds (NDR)	miRagen-viridia	Reduce miR-92a and improve vascularization	NCT03603431	Anti Mir92a;MRG-110;S95010
SPC 4955	ASO	Discontinued	Hypercholesterolaemia	Roche (Santaris Pharma)	Inhibit ApoB (aploprotein B) and reduce LDL-C	NCT01365663	SPC-4955
SPC 5001	ASO	Discontinued	Hypercholesterolaemia	Roche (Santaris Pharma)	Target Proprotein convertase subtilisin/kexin type 9 (PCSK9) expression Reduce LDL-C	NCT01350960	SPC-5001
IONIS APOARx	ASO	Discontinued	Hyperlipoproteinaemia	Ionis Pharmaceuticals	Reduce level of Lp(a)	NCT02160899	IONIS-APO(a)Rx;IONIS-APOA Rx;ISIS-494372;ISIS-APO(a)RX;ISIS-APOA Rx

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ISIS CRPRx	ASO	Discontinued	Atrial fibrillation; Rheumatoid arthritis	Ionis Pharmaceuticals	Target C-reactive protein (CRP)	NCT01710852	ISIS-329993;ISIS-CRP;ISIS-CRPRx
GTX Drug eluting Coronary Stent System	ASO	Discontinued	Coronary Artery Disease	Sarepta-Cook Group Incorporated	Target c-myc to reduce arteries reclosing	NCT00777842	AVI-5126; GTX 5126; Restin-CP
Inclisiran	siRNA	Registered; Phase III	Hypercholesterolaemia (Registered)/ Atherosclerotic Cardiovascular Disease (III)	Alnylam-Novartis	Inhibit hepatic synthesis of (PCSK9) to lower LDL-C	NCT04929249 NCT03814187	ALN-60212;ALN-PCSSc;KJX-839;Leqvio;PCSK9si
Patisiran	siRNA	Marketed; Phase III	Amyloid polyneuropathy; Amyloidosis (Marketed); ATTR with Cardiomyopathy (III)	Alnylam Pharmaceuticals	Inhibit transthyretin (TTR) synthesis	NCT03997383 NCT01960348	ALN-18328;ALN-TTR02;GENZ-438027;ONPATTRO;Patisiran sodium;patisiran-LNP;SAR-438037
Vutrisiran	siRNA	Pre-registration; Phase III	Transthyretin-related hereditary amyloidosis; ATTR With Cardiomyopathy	Alnylam Pharmaceuticals	Inhibit transthyretin (TTR) synthesis	NCT04153149 NCT03759379	ALN-65492;ALN-TTRsc02
Teprasiran	siRNA	Phase III	Acute kidney injury (patients undergoing cardiac surgery); Delayed graft function	Quark Pharmaceuticals	Inhibit p53-mediated cell death	NCT03510897	AKIi-5;DGF;I-5NP;QPI-1002;QPI002;Teprasiran sodium
Fitusiran	siRNA	Phase III	Haemophilia A; Haemophilia B	Alnylam/Sanofi	Target anti-thrombin III to increase thrombin generation	NCT03974113 NCT03754790	ALN-AT3;ALN-AT3SC;SAR-439774
ARO APOC3	siRNA	Phase III; Phase II	Dyslipidaemias; Hypertriglyceridaemia (II); Hyperlipoproteinaemia type I (III)	Arrowhead Pharmaceuticals	Reduce production of Apolipo-protein C-III (apoC-III) to lower very low density lipoprotein	NCT04720534 NCT04998201 NCT05089084	ARO-APOC3
Zilebesiran	siRNA	Phase II; No development reported	Hypertension (II); Preeclampsia (NDR)	Alnylam Pharmaceuticals	Liver specific silencing of AGT (angiotensinogen)	NCT04936035	AGT siRNA;ALN-AGT;ALN-AGT-01
ARO ANG3	siRNA	Phase II	Dyslipidaemias	Arrowhead Pharmaceuticals	Target angiopoietin like protein 3 (ANGPTL3) to lower LDL	NCT04832971	ARO-ANG3

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Olpasiran	siRNA	Phase II	Cardiovascular disorders	Arrowhead-Amgen	Reduce the production of Lp(a) by targeting apolipoprotein A (LPA) gene.	NCT04987320 NCT04270760	AMG-890;ARC LPA;ARO LPA;RNAi ARC-LPA
Bamosiran	siRNA	Phase II	Glaucoma; Ocular hypertension	Sylentis	Inhibit beta 2 adrenergic receptors to reduce glaucoma	NCT02250612	SYL-040012
ApoB SNALP	siRNA	Phase I (No development reported)	Hypercholesterolaemia	Arbutus Biopharma	Reduce apoB production to control cholesterol	NCT00927459	PRO-040201;TKM-ApoB
LY 3561774	siRNA	Phase I	Cardiovascular disorders; Dyslipidaemias; Metabolic disorders	Dicerna/Elli lilly	Target angiopoietin-like protein 3 (ANGPTL3)	NCT04644809	DCR-CM1;LY-3561774
LY 3819469	siRNA	Phase I	Cardiovascular disorders; Metabolic disorders	Dicerna/Elli lilly	Target the lipoprotein A (LPA) gene	NCT04914546	DCR-CM2;LY-3819469
SLN 124	siRNA	Preclinical; Phase I	Haemochromatosis; Iron overload; Polycythaemia vera (Pre); Beta-thalassaemia; Myelodysplastic syndromes (I)	Silence therapeutics	Silence TMPRSS6 to control iron level and healthy red blood cell production	NCT04718844	SLN-124
SLN 360	siRNA	Preclinical; Phase I	Cardiovascular disorders (Pre); Dyslipidaemias; Hyperlipidaemia (I)	Silence therapeutics	Target lipoprotein Lp(a)	NCT04606602	SLN-360
AGN 211745	siRNA	Discontinued	Age-related macular degeneration,Choroid Neovascularization	Abbvie (Allergan)	Target vascular endothelial growth factor receptor-1 expression Reduce pathologic angiogenesis	NCT00395057	AGN-211745;AGN-745;Sirna 027
Revusiran	siRNA	Discontinued	Familial amyloid neuropathy; Transthyretin-related hereditary amyloidosis	Alnylam Pharmaceuticals	Targets hepatic transthyretin (TTR) production	NCT02319005	ALN-TTRsc;SAR438714;siTTRsc
ALN PCS	siRNA	Discontinued	Hypercholesterolaemia	Alnylam Pharmaceuticals	Inhibit PCSK9 synthesis to reduce LDL-C	NCT01437059	ALN-PCS;ALN-PCS01;ALN-PCS02

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Revascularization operation	miRNA	Investigational	Peripheral Arterial Disease, Vascular Diseases, Peripheral, Arterial Occlusive Diseases, Atherosclerosis	Baylor University-National Institute on Aging (NIA)	Study of the role of microRNA-210 in regulating Oxidative Stress	NCT04089943	None
Pegaptanib	RNA aptamer	Marketed; Discontinued; No development reported	Age-related macular degeneration (Marketed); Choroidal neovascularisation; Retinal vascular occlusion (Discontinued); Diabetic macular oedema; Diabetic retinopathy (NDR)	Eyetech/ Pfizer	Directed against vascular endothelial growth factor (VEGF)-165 to prevent ocular neovascularization	NCT00324116 NCT01189461 NCT01589718 NCT00327470 NCT01486771	anti-VEGF aptamer;BLO 021;EYE-001;Macugen;NX-1838;Pegaptanib octasodium;Pegaptanib sodium
BT 200	RNA aptamer	Phase II; Phase I; Preclinical	Hemophilia A, Von Willebrand disease (II); Atheroseclerosis, Stroke (I); Arterial thrombosis (PRE)	Band Therapeutics (Guardian Therapeutic)	Inhibit von Willebrand factor binding to platelet glycoprotein GbIb to prevent thrombosis	NCT04677803 NCT04103034	BT-200
AON D21	RNA/ DNA aptamer	Phase I	Paroxysmal nocturnal haemoglobinuria	Aptarion Biotech	Complement C5a receptor antagonists	NCT05018403	AON-D21
Anivamersen-pegnivacogin	RNA aptamer	Discontinued	Acute coronary syndromes; Venous thrombosis	Regado Biosciences, Inc.	Target coagulation factor IXa to prevent coagulation	NCT01848106	Anivamersen;Pegnivacogin;Pegnivacogin/anivamersen;RB 006;RB 006/RB 007;RB 007;REG-1;REG-2;Revolixys
Egaptivon pegol	RNA/ DNA aptamer	Discontinued	Haemolytic uraemic syndrome; Thrombosis; Thrombotic thrombocytopenic purpura; Von Willebrand disease	Archemix Corporation	Inhibits von Willebrand factor binding to platelet receptor glycoprotein Ib to reduce thrombosis	NCT00632242	ARC1779
AZD 8601	mRNA	Phase II	Heart failure	Moderna-AstraZeneca	VEGF-A overexpression	NCT03370887	AZD-8601