

FDA/EMA approved Oligonucleotide-based drugs

Trade name	Drug name	Developing company	FDA approval	EMA approval	Disease indication	Modality	Administration/target organ
Vitravene*	Fomivirsen	Ionis	Aug 1998	Jul 1999	CMV retinitis	ASO	Intravitreal (eye)
Macugen*	Pegaptanib	Pfizer/Eyetech	Dec 2004	Jan 2006	Age-related macular degeneration	Aptamer	Intravitreal (eye)
Kynamro*	Mipomersen	Ionis	Jan 2013	N/A	Homozygous familial hypercholesteremia	ASO	Subcutaneous (liver)
Exondys 51	Eteplirsen	Sarepta	Sep 2016	N/A	Duchenne muscular dystrophy	ASO (PMO)	Intravenous (muscle)
Spinraza	Nusinersen	Ionis	Dec 2016	May 2017	Spinal muscular atrophy	ASO	Intrathecal (CNS)
Heplisav-B	Hepatitis B vaccine	Dynavax	Nov 2017	Feb 2021	Hepatitis B	CpG ODN	Intramuscular
Onpattro	Patisiran	Alnylam	Aug 2018	Aug 2018	Polyneuropathy of hereditary transthyretin amyloidosis	siRNA	Intravenous (liver)
Tegsedi	Inotersen	Ionis	Oct 2018	Jul 2018	Polyneuropathy of hereditary transthyretin amyloidosis	ASO	Subcutaneous (liver)
Waylivra	Volanesorsen	IONIS/Akcea	N/A	May 2019	Familial chylomicronemia syndrome	ASO	Subcutaneous (liver)
Givlaari	Givosiran	Alnylam	Nov 2019	Mar 2020	Acute hepatic porphyria	siRNA	Subcutaneous (liver)
Vyondys-53	Golodirsen	Sarepta	Dec 2019	N/A	Duchenne muscular dystrophy	ASO (PMO)	Intravenous (muscle)
Viltepso	Viltolarsen	Nippon Shinyaku Pharma	Aug 2020	Jun 2020	Duchenne muscular dystrophy	ASO (PMO)	Intravenous (muscle)
Oxlumo	Lumasiran	Alnylam	Nov 2020	Nov 2020	Primary hyperoxaluria type 1	siRNA	Subcutaneous (liver)
Amondys 45	Casimersen	Sarepta	Feb 2021	N/A	Duchenne muscular dystrophy	PMO-ASO	Intravenous (muscle)
Leqvio	Inclisiran	Alnylam/Novartis	Dec 2021	Dec 2020	Heterozygous familial Hypercholesteremia, primary hyperlipidemia	siRNA	Subcutaneous (liver)
Amvuttra	Vutrisiran	Alnylam	Jun 2022	Sep 2022	Polyneuropathy of hereditary transthyretin amyloidosis	siRNA	Subcutaneous (liver)
Qalsody	Tofersen	IONIS/Biogen	Apr 2023	Dec 2022	Amyotrophic lateral sclerosis	ASO	Intrathecal (CNS)
Izervay	Avacin-captad pegol	Iveric Bio/Astellas	Sep 2023	Aug 2023	Geographic atrophy of age-related macular degeneration	Aptamer	Intravitreal (eye)
Cyfundus	Anthrax vaccine adsorbed	Emergent	Sep 2023	N/A	Postexposure prophylaxis for anthrax	CpG ODN	Intramuscular
Rivfloza	Nedosiran	Dicerna/Novo Nordisk	Oct 2023	N/A	Primary hyperoxaluria	siRNA	Subcutaneous (liver)
Casgevvy	Exagamglogene autotemcel	CRISPR Therapeutics/Vertex	Dec 2023	Feb 2024	Sickle cell anemia	CRISPR-Cas9	N/A
Wainua	Eplontersen	IONIS/AstraZeneca	Dec 2023	Mar 2025	TTR-mediated polyneuropathy/TTR-mediated cardiomyopathy	ASO	Subcutaneous (liver)
Tryngolza	Olezarsen	IONIS	Dec 2024	Sep 2025	Familial chylomicronemia syndrome (FCS)	ASO	auto-injector
Rytelo	Imetelstat	Geron Netherlands	Jun 2024	Mar 2025	Transfusion-dependent anaemia of MDS patients	ASO	Intravenous (systemic)
QFITLIA	Fitusiran	Sanofi (Alnylam)	Mar 2025	N/A	Hemophilia A and B	siRNA	subcutaneous (liver)
Dawnzera	Donidalorsen	IONIS	Aug 2025	Jan 2026	Hereditary angioedema (HAE)	ASO	subcutaneous (liver)
REDEMPLO	Plozasiran	Arrowhead	Nov 2025	N/A	Familial Chylomicronemia Syndrome (FCS)	siRNA	subcutaneous (liver)