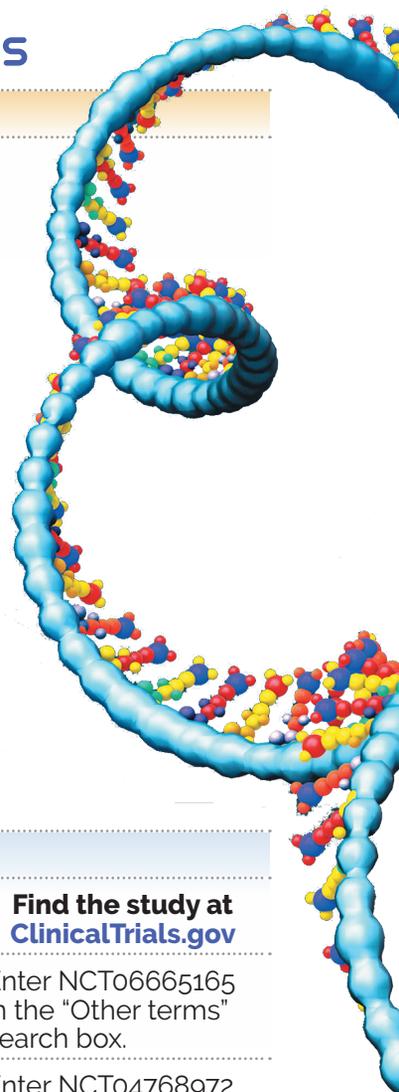


A Guide to RNA-Targeted Therapies



APPROVED RNA THERAPIES

Disease treated	Drug	Distributor	Therapy type	Approved in
ALS	tofersen (Qalsody)	Ionis Pharmaceuticals/ Biogen	ASO targeting SOD1 mRNA	2023
DMD	casimersen (Amondys 45)	Sarepta Therapeutics	ASO targeting exon 45 skipping	2021
DMD	eteplirsen (Exondys 51)	Sarepta Therapeutics	ASO targeting exon 51 skipping	2016
DMD	golodirsen (Vyondys 53)	Sarepta Therapeutics	ASO targeting exon 53 skipping	2019
DMD	viltolarsen (Viltepso)	NS Pharma	ASO targeting exon 53 skipping	2020
SMA	nusinersen (Spinraza)	Biogen	ASO to increase production of survival motor neuron protein	2016
SMA	risdiplam (Evrysdi)	Genentech	Small-molecule drug	2020

NOTABLE RNA THERAPIES IN CLINICAL TRIALS

Disease treated	Investigational drug	Sponsor	Therapy type	Trial phase	Find the study at ClinicalTrials.gov
ALS	AMX0114	Amylyx Pharmaceuticals	ASO targeting calpain-2 mRNA	Phase 1	Enter NCT06665165 in the "Other terms" search box.
ALS	ION363	Ionis Pharmaceuticals	ASO targeting FUS mRNA	Phase 3	Enter NCT04768972 in the "Other terms" search box.
FSHD	AOC 1020 (del-brax)	Avidity Biosciences, Inc.	siRNA targeting DUX4 mRNA	Phase 3	Enter NCT07038200 in the "Other terms" search box.
FSHD	ARO-DUX4	Arrowhead Pharmaceuticals	siRNA to reduce levels of DUX4 RNA	Phase 1/2	Enter NCT06131983 in the "Other terms" search box.
DMD	AOC 1044 (del-zota)	Avidity Biosciences, Inc.	Antibody-oligo conjugate (AOC) for exon 44 skipping	Phase 2	Enter NCT06244082 in the "Other terms" search box.
DMD	NS-089/NCNP-02 (brogidirsen)	NS Pharma	ASO targeting exon 44 skipping	Phase 2	Enter NCT05996003 in the "Other terms" search box.
DM1	AOC-1001 (del-desiran)	Avidity Biosciences, Inc.	siRNA to reduce levels of DMPK RNA	Phase 3	Enter NCT07008469 in the "Other terms" search box.
DM1	PGN-EDODM1	PepGen	ASO to block CUG repeats in DMPK RNA	Phase 2	Enter NCT06667453 in the "Other terms" search box.