

Myotonic Dystrophy Drug Development Pipeline - Apr. 2025

COMPANY	PROGRAM/ LEAD CANDIDATE	MODALITY	DM SUBTYPE	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET/ MECHANISM	PRESS RELEASES & MORE	MDF CONFERENCE INDUSTRY UPDATE	MDF MEET-THE-DM- DRUG-DEVELOPERS
AMO Pharma Ltd	Tideglusib	Small Molecule	CDM / DM1					Glycogen synthase kinase 3 beta (GSK3B)	AMO Pharma News	2024 Industry Update	2023 Webinar
Avidity Biosciences	AOC 1001 del-desiran	Antibody Conjugated Oligonucleotide	DM1					DMPK	Avidity News	2024 Industry Update	2024 Webinar
Lupin Neurosciences	Mexiletine	Small Molecule	DM1 / DM2					Sodium Channels	Lupin News		2024 Webinar
Harmony Biosciences	Pitolisant	Small Molecule	DM1					Histamine-3 receptor (H3R)	Harmony News	2023 Industry Update	2022 Webinar
Dyne Therapeutics	Dyne 101	Antibody Fragment Conjugated Antisense Oligonucleotide	DM1					DMPK	Dyne News	2024 Industry Update	2025 Webinar
ARTHEX Biotech	ATX-01	Antisense microRNA Oligonucleotide	DM1 / DM2					MBNL and DMPK via miR-23b	ARTHEX News	2024 Industry Update	2024 Webinar
Vertex	VX-670	Peptide Conjugated Oligonucleotide	DM1					DMPK	Vertex News		
PepGen	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1					DMPK	PepGen News	2024 Industry Update	2024 Webinar
Sarepta (Arrowhead Pharmaceuticals)	SRP-1003	Investigational RNA interference (RNAi) Therapeutic	DM1					r(CUG) of DMPK	Sarepta News		
Sanofi	--	miRNA Technology in Adeno-Associated Virus	--					DMPK	Sanofi News	2023 Industry Update	
Expansion Therapeutics	DM1 (CUG)	Small Molecule	DM1					r(CUG) of DMPK	Expansion News	2021 Industry Update	2021 Webinar
Juvena Therapeutics	JUV-161	Stem Cell-Secreted Proteins	DM1					--	Juvena News	2024 Industry Update	2024 Webinar
Rgenta Therapeutics	PMS1	Small Molecule	DM1					r(CUG) of DMPK	Rgenta News	2023 Industry Update	
Astellas Gene Therapies	AT466	Adeno-Associated Viral Antisense	DM1					DMPK	Astellas News		
Modalis Therapeutics	MDL-202	AAV mediated CRISPR base DMPK silencing	DM1					DMPK	Modalis News		
EditForce, Inc.	EF-210	Gene Transfer based Delivery of Pentatricopeptide Repeat Protein	DM1					r(CUG) of DMPK	EditForce News		
Kinea Bio, Inc.	KNA-123; KNA-145	--	CDM / DM1					--	Kinea Bio News		
Arrakis Therapeutics	--	RNA-targeted Small Molecule (rSM)	DM1					DMPK	Arrakis News		
Design Therapeutics	--	Gene Targeting Chimera Small Molecule	DM1					r(CUG) of DMPK	Design News		
Novartis (Kate Therapeutics)	--	--	DM1					--	Novartis News News		
GrittGene Therapeutics	--	--	DM2					--	GrittGene Publications	2022 Industry Update	
Scriptr Global, Inc.	--	--	DM1					--	--		

Note: Academic institutions are not included above. In addition to this list, there are several more companies in the Discovery Phase that have not yet made their DM programs public. Click green arrows above for clinical trial information on Phases 1-3. For locations and details of each clinical trial, visit www.clinicaltrials.gov. Designed by MDF, this pipeline is in ongoing development and based on publicly available information. Special thanks to Nate Uhl for his comprehensive scan of the DM research landscape and original vision for the drug development pipeline. For questions, or to request updates to the pipeline, please contact MDF at info@myotonic.org. Download the latest DM Pipeline Chart at: www.myotonic.org/pipeline.

