

Myotonic Dystrophy Drug Development Pipeline - May 2024

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	2023 Industry Update	2023 Webinar
Lupin Neurosciences	Mexiletine	Small Molecule	DM1 / DM2					Sodium Channels	Lupin News		2021 Webinar
Avidity Biosciences	AOC 1001 <i>del-desiran</i>	Antibody Conjugated Oligonucleotide	DM1					DMPK	Avidity News	2023 Industry Update	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	2023 Industry Update	2024 Webinar
ARTHEX Biotech	ATX-01	Antisense microRNA Oligonucleotide	DM1 / DM2					MBNL and DMPK via miR-23b	ARTHEX News	2023 Industry Update	2023 Webinar
Vertex	VX-670	Peptide Conjugated Oligonucleotide	DM1					DMPK	Vertex News		
PepGen	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1					DMPK	PepGen News	2023 Industry Update	2023 Webinar
Arrowhead Pharmaceuticals	ARO-DM1	Investigational RNA interference (RNAi) Therapeutic	DM1					r(CUG) of DMPK	Arrowhead News		
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	2023 Industry Update	
Regenta Therapeutics	PMS1	Small Molecule	DM1					r(CUG) of DMPK	Regenta News	2023 Industry Update	
Sanofi	--	miRNA Technology in Adeno-Associated Virus	--					DMPK	Sanofi News	2023 Industry Update	
Astellas Gene Therapies	AT466	Adeno-Associated Viral Antisense	DM1					DMPK	Astellas News		
GrittGene Therapeutics	--	--	DM2					--	GrittGene Publications	2022 Industry Update	
Enzerna Biosciences	--	Artificial Site Specific RNA Endonucleases (ASREs)	DM1					r(CUG) of DMPK	Enzerna News		
Design Therapeutics	--	Gene Targeting Chimera Small Molecule	DM1					r(CUG) of DMPK	Design News		
Dewpoint Therapeutics	--	Biomolecular Condensates	DM1					DMPK	Dewpoint News		
Kate Therapeutics	--	--	DM1					--	Kate Therapeutics News		
Prime Medicine	--	--	--					--	Prime Medicine News		
Denali Therapeutics	--	--	DM1					DMPK	Denali News		
EditForce, Inc.	EF-210	Gene Transfer based Delivery of Pentatricopeptide Repeat Protein	DM1					r(CUG) of DMPK	EditForce News		
Scriptr Global, Inc.	--	--	DM1					--	--		
Healx Ltd.	--	--	DM1					--	Healx News		

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. To review the Myotonic Dystrophy Research Map visit: www.myotonic.org/myotonic-dystrophy-research-map.

