















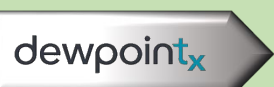

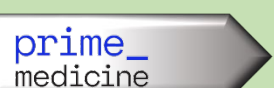


Myotonic Dystrophy Drug Development Pipeline - January 2024

COMPANY	PROGRAM/ LEAD CANDIDATE	MODALITY	DM SUBTYPE	PRE- CLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET/ MECHANISM	PRESS RELEASES & MORE	MDF ANNUAL CONFERENCE INDUSTRY UPDATE	MDF MEET-THE-DM- DRUG-DEVELOPERS
AMO Pharma Ltd	Tideglusib	Small Molecule	CDM					Glycogen synthase kinase 3 beta (GSK3β)	AMO Pharma News	2023 Industry Update	2023 Webinar
Harmony Biosciences	Pitolisant	Small Molecule	DM1					Histamine-3 receptor (H3R)	Harmony News	2023 Industry Update	2022 Webinar
Lupin Neurosciences	Mexiletine	Small Molecule	DM1 / DM2					Sodium Channels	Lupin News		2021 Webinar
Avidity Biosciences	AOC 1001	Antibody Conjugated Oligonucleotide	DM1					DMPK	Avidity News	2023 Industry Update	2023 Webinar
Dyne Therapeutics	Dyne 101	Antibody Fragment Conjugated Antisense Oligonucleotide	DM1					DMPK	Dyne News	2023 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	2023 Industry Update	2023 Webinar
ARTHEX Biotech	ATX01	Antisense microRNA Oligonucleotide	DM1 / DM2					MBNL and DMPK via miR-23b	ARTHEX News	2023 Industry Update	2023 Webinar
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	
Rgenta Therapeutics	PMS1	Small Molecule	DM1					r(CUG) of DMPK	Rgenta 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 Thx	--	Gene Targeting Chimera Small Molecule	DM1					r(CUG) of DMPK	Design News		
Dewpoint Therapeutics	--	Biomolecular Condensates	DM1					DMPK	DewpointTx News		
Kate Therapeutics	--	--	DM1					--	Kate Therapeutics Pipeline		
Prime Medicine	--	--	--					--	Prime Medicine Pipeline		

Note: Academic institutions are not included above. Additionally, several more companies are in the Discovery Phase but have not yet made their DM programs public. Click green arrow for clinical trial information on Phases 1-3. For locations and details of each clinical trial, visit www.clinicaltrials.gov. This pipeline was designed by MDF and is in ongoing development based on publicly available information. Special thanks to Nate Uhl for his comprehensive scan of the DM research landscape and original vision of 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.