

Myotonic Dystrophy Drug Development Pipeline - September 2022

COMPANY	PROGRAM	MODALITY	DM PATIENT	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET/ MECHANISM	PRESS RELEASES & MORE	WEBINARS WITH MDF
AMO Pharma Ltd	Tideglisib	Small Molecule	CDM					Glycogen synthase kinase 3 beta (GSK3B)	AMO Pharma News	Meet-the-DM-Drug-Developer
Harmony Biosciences	Pitolisant	Small Molecule	DM1					histamine-3 receptor (H3R)	Harmony News	Meet-the-DM-Drug-Developer
Lupin Neurosciences	Mexiletine	Small Molecule	DM1/DM2					Sodium Channels	Lupin News	Meet-the-DM-Drug-Developer
Avidity Biosciences	AOC 1001	Antibody Oligonucleotide Conjugate	DM1					DMPK	Avidity News	Meet-the-DM-Drug-Developer
Dyne Therapeutics	Dyne 101	Antibody Oligonucleotide Conjugate	DM1					DMPK	Dyne News	Meet-the-DM-Drug-Developer
Expansion Therapeutics	DM1 (CUG)	Small Molecule	DM1					r(CUG) of DMPK	Expansion News	Meet-the-DM-Drug-Developer
PepGen	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1					DMPK	PepGen News	Meet-the-DM-Drug-Developer
ARTHEX	ATX01	Antisense microRNAs	DM1					MBNL	ARTHEX News	Meet-the-DM-Drug-Developer
Triplet Therapeutics	TTX-3360	Antisense Oligonucleotide	DM1 (CNS) DM1 (Muscle)					MSH3	Triplet News	
Neubase	NT-0200	Peptide-Nucleic Acid Antisense Oligonucleotides	DM1					r(CTG)	Neubase News	
Astellas Gene Therapy	AT446	Adeno-Associated Viral Antisense	DM1					DMPK	Astellas News	
Juvena Therapeutics	JUV-161	Stem Cell-Secreted Proteins	--					--	Juvena News	
Entrada	ENTR-701	Endosomal Escape Vehicle	DM1					r(CUG) of DMPK	Entrada News	
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	
Locana Bio	--	Gene Therapy	DM1					r(CUG) RNA	Locana News	
Novartis Gene Therapy	--	Gene therapy	DM1					RNA	Novartis News	
Sanofi-Genzyme	--	miRNA Technology in Adeno-Associated Virus	--					DMPK	Sanofi News	
Faze Medicines	--	Biomolecular Condensates	DM1					--	Faze News	
Sangamo-Biogen	--	Gene Therapy	DM1					--	Sangamo News	
Vertex-Crispr	--	Gene Editing	--					--	Vertex News	
AskBio	--	Adeno Associated Virus (AAV) Gene Therapy	--					--	AskBio News	

Note: Academic institutions are not included above. Additionally, several companies are in the Discovery Phase but have not yet made it public, so they have not been included. Click green arrow for clinical trial information on Phases 1-3. For 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 drug development pipeline inspiration. 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.