

Myotonic Dystrophy Drug Development Pipeline - January 2023

COMPANY	PROGRAM	MODALITY	DM PATIENT	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET/ MECHANISM	PRESS RELEASES & MORE	2022 MDF ANNUAL CONFERENCE UPDATES	WEBINARS WITH MDF
AMO Pharma Ltd	Tideglisib	Small Molecule	CDM					Glycogen synthase kinase 3 beta (GSK3B)	AMO Pharma News	MDF Conference Update	Meet the DM Drug Developers
Harmony Biosciences	Pitolisant	Small Molecule	DM1					histamine-3 receptor (H3R)	Harmony News	MDF Conference Update	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	MDF Conference Update	Meet-the-DM-Drug-Developer
Dyne Therapeutics	Dyne 101	Antibody Oligonucleotide Conjugate	DM1					DMPK	Dyne News	MDF Conference Update	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	MDF Conference Update	Meet-the-DM-Drug-Developer
ARTHEX	ATX01	Antisense microRNA oligonucleotide	DM1 / DM2					MBNL and DMPK via miR-23b	ARTHEX News	MDF Conference Update	Meet-the-DM-Drug-Developer
Astellas Gene Therapy	AT446	Adeno-Associated Viral Antisense	DM1					DMPK	Astellas News		
Juvena Therapeutics	JUV-161	Stem Cell-Secreted Proteins	--					--	Juvena News	MDF Conference Update	
Entrada - Vertex	ENTR-701	Endosomal Escape Vehicle	DM1					r(CUG) of DMPK	Entrada News	MDF Conference 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		
Locana Bio	--	Gene Therapy	DM1					r(CUG) RNA	Locana 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 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.