

# Myotonic Dystrophy Drug Development Pipeline - Sept. 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
<a href="#">AMO Pharma Ltd</a>	Tideglusib	Small Molecule	CDM / DM1					Glycogen synthase kinase 3 beta (GSK3B)	<a href="#">AMO Pharma News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2023 Webinar</a>
<a href="#">Avidity Biosciences</a>	AOC 1001 <i>del-desiran</i>	Antibody Conjugated Oligonucleotide	DM1					DMPK	<a href="#">Avidity News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2024 Webinar</a>
<a href="#">Lupin Neurosciences</a>	Mexiletine	Small Molecule	DM1 / DM2					Sodium Channels	<a href="#">Lupin News</a>		<a href="#">2021 Webinar</a>
<a href="#">Harmony Biosciences</a>	Pitolisant	Small Molecule	DM1					Histamine-3 receptor (H3R)	<a href="#">Harmony News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2022 Webinar</a>
<a href="#">Dyne Therapeutics</a>	Dyne 101	Antibody Fragment Conjugated Antisense Oligonucleotide	DM1					DMPK	<a href="#">Dyne News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2024 Webinar</a>
<a href="#">ARTHEX Biotech</a>	ATX-01	Antisense microRNA Oligonucleotide	DM1 / DM2					MBNL and DMPK via miR-23b	<a href="#">ARTHEX News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2023 Webinar</a>
<a href="#">Vertex</a>	VX-670	Peptide Conjugated Oligonucleotide	DM1					DMPK	<a href="#">Vertex News</a>		
<a href="#">PepGen</a>	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1					DMPK	<a href="#">PepGen News</a>	<a href="#">2023 Industry Update</a>	<a href="#">2023 Webinar</a>
<a href="#">Arrowhead Pharmaceuticals</a>	ARO-DM1	Investigational RNA interference (RNAi) Therapeutic	DM1					r(CUG) of DMPK	<a href="#">Arrowhead News</a>		
<a href="#">Expansion Therapeutics</a>	DM1 (CUG)	Small Molecule	DM1					r(CUG) of DMPK	<a href="#">Expansion News</a>	<a href="#">2021 Industry Update</a>	<a href="#">2021 Webinar</a>
<a href="#">Juvena Therapeutics</a>	JUV-161	Stem Cell-Secreted Proteins	DM1					--	<a href="#">Juvena News</a>	<a href="#">2023 Industry Update</a>	
<a href="#">Rgenta Therapeutics</a>	PMS1	Small Molecule	DM1					r(CUG) of DMPK	<a href="#">Rgenta News</a>	<a href="#">2023 Industry Update</a>	
<a href="#">Sanofi</a>	--	miRNA Technology in Adeno-Associated Virus	--					DMPK	<a href="#">Sanofi News</a>	<a href="#">2023 Industry Update</a>	
<a href="#">Astellas Gene Therapies</a>	AT466	Adeno-Associated Viral Antisense	DM1					DMPK	<a href="#">Astellas News</a>		
<a href="#">GrittGene Therapeutics</a>	--	--	DM2					--	<a href="#">GrittGene Publications</a>	<a href="#">2022 Industry Update</a>	
<a href="#">Enzerna Biosciences</a>	--	Artificial Site Specific RNA Endonucleases (ASREs)	DM1					r(CUG) of DMPK	<a href="#">Enzerna News</a>		
<a href="#">Modalis Therapeutics</a>	MDL-202	AAV mediated CRISPR based DMPK silencing	DM1					DMPK	<a href="#">Modalis News</a>		
<a href="#">Design Therapeutics</a>	--	Gene Targeting Chimera Small Molecule	DM1					r(CUG) of DMPK	<a href="#">Design News</a>		
<a href="#">Dewpoint Therapeutics</a>	--	Biomolecular Condensates	DM1					DMPK	<a href="#">Dewpoint News</a>		
<a href="#">Kate Therapeutics</a>	--	--	DM1					--	<a href="#">Kate Therapeutics News</a>		
<a href="#">Prime Medicine</a>	--	--	--					--	<a href="#">Prime Medicine News</a>		
<a href="#">Denali Therapeutics</a>	--	--	DM1					DMPK	<a href="#">Denali News</a>		
<a href="#">EditForce Inc.</a>	EF-210	Gene Transfer based Delivery of Pentatricopeptide Repeat Protein	DM1					r(CUG) of DMPK	<a href="#">EditForce News</a>		
<a href="#">Scriptr Global Inc.</a>	--	--	DM1					--	--		
<a href="#">Healx Ltd.</a>	--	--	DM1					--	<a href="#">Healx News</a>		

**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](http://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](mailto:info@myotonic.org). To review the Myotonic Dystrophy Research Map visit: [www.myotonic.org/myotonic-dystrophy-research-map](http://www.myotonic.org/myotonic-dystrophy-research-map).

