

Myotonic Dystrophy Drug Development Pipeline - Sept. 2025

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	2024 Update	2023 Webinar
Avidity Biosciences	AOC 1001 del-desiran	Antibody Conjugated Oligonucleotide	DM1					DMPK	Avidity News	2025 Update	2024 Webinar
Lupin Neurosciences	Mexiletine	Small Molecule	DM1 / DM2					Sodium Channels	Lupin News	2025 Update	2024 Webinar
ARTHEX Biotech	ATX-01	Antisense microRNA Oligonucleotide	DM1					MBNL via miR-23b	ARTHEX News	2025 Update	2024 Webinar
Dyne Therapeutics	Dyne 101	Antibody Fragment Conjugated Antisense Oligonucleotide	DM1					DMPK	Dyne News	2025 Update	2025 Webinar
Harmony Biosciences	Pitolisant	Small Molecule	DM1					Histamine-3 receptor (H3R)	Harmony News	2023 Update	2022 Webinar
Juvena Therapeutics	JUV-161	Stem Cell-Secreted Proteins	DM1					--	Juvena News	2025 Update	2024 Webinar
PepGen (University of Oxford Spinout)	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1					DMPK	PepGen News	2025 Update	2024 Webinar
Sanofi	--	miRNA Technology in Adeno-Associated Virus	--					DMPK	Sanofi News	2025 Update	
Sarepta	SRP-1003	Investigational RNA interference (RNAi) Therapeutic	DM1					r(CUG) of DMPK	Sarepta News		
Vertex	VX-670	Peptide Conjugated Oligonucleotide	DM1					DMPK	Vertex News	2025 Update	
ARTHEX Biotech	ATX-01	Antisense microRNA Oligonucleotide	DM2					MBNL via miR-23b	ARTHEX News	2025 Update	2024 Webinar
Arrakis Therapeutics	--	RNA-targeted Small Molecule (rSM)	DM1					DMPK	Arrakis News	2025 Update	
Astellas Gene Therapies	AT466	Adeno-Associated Viral Antisense	DM1					DMPK	Astellas News		
Design Therapeutics	--	Gene Targeting Chimera Small Molecule	DM1					r(CUG) of DMPK	Design News		
EditForce Inc.	EF-210	Gene Transfer based Delivery of Pentatricopeptide Repeat Protein	DM1					r(CUG) of DMPK	EditForce News		
GrittGene Therapeutics	--	--	DM2					--	GrittGene Publications	2022 Update	
Kinea Bio, Inc.	KNA-123; KNA-145	--	CDM / DM1					--	Kinea Bio News		
LoQus23	--	--	DM1					MSH3	LoQus23 News		
Modalis Therapeutics	MDL-202	AAV mediated CRISPR base DMPK silencing	DM1					DMPK	Modalis News	2025 Update	
Novartis	--	--	DM1					--	Novartis News		
Regenta Therapeutics	PMS1	Small Molecule	DM1					r(CUG) of DMPK	Regenta News	2023 Update	
ScriptBioscience	--	--	DM1					MSH3 CasPlusKO	ScriptBiosciences News		
Scriptr Global, Inc.	--	--	DM1					--	--		

Note: Programs listed alphabetically by phase. 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. Download the latest DM Pipeline Chart at: www.myotonic.org/pipeline.



Archive of Past DM Drug Development Efforts - Aug 2025

Company / Organization	Company Status	DM Program	Notes
Anagenesis Biotechnologies	Closed	Unknown	Company closed in 2022; regenerative medicine focus included DM in early-stage research.
Arrowhead Pharmaceuticals	Active	Sold / Outlicensed	DM program acquired by Sarepta in 2025.
AskBio (Asklepios)	Active	Unknown	Gene therapy research for DM ended; company acquired by Bayer in 2020. Company operates as subsidiary of Bayer.
Audentes	Acquired	Active	Acquired by Astellas in 2020; DM programs carried forward into Astellas pipeline.
Biogen	Active	Unknown	Ionis partnership for antisense therapy ended after failed Phase I/II trial in 2015; published study.
BioMarin Pharmaceutical Inc.	Active	Unknown	Acquired Prosensa in 2014 with myotonic dystrophy program; no activity.
Celgene	Acquired	Unknown	Acquired by Bristol-Myers Squibb in 2019; historical DM relevance from investment in RNA-targeting companies.
CRISPR Therapeutics	Active	Active	Investigational in vivo gene-editing therapy for DM1 in collaboration with Vertex.
Cydan Development Inc.	Closed	Unknown	Company closed in 2021; rare disease accelerator; incubated companies with neuromuscular programs.
Denali Therapeutics	Active	Unknown	Ended DM program; initially pursued neuromuscular CNS-targeting therapeutics.
Dewpoint Therapeutics	Active	Unknown	Ended Pfizer-collaboration on DM program targeting toxic RNA condensates.
DTx Pharma	Acquired	Active	Acquired by Novartis in 2023; DM program ongoing within Novartis Gene Therapy.
Entrada	Active	Sold / Outlicensed	Outlicensed ENTR-670 and ENTR-701 program for DM1 to Vertex in 2023.
Enzerna Biosciences	Active	Unknown	Company inactive in practice; early RNA-targeting platform had DM relevance.
Expansion Therapeutics	Closed	Unknown	Company closed in 2025; small molecule DM1 program ended.
Faze Medicines	Closed	Unknown	Company closed in 2022; DM1 program targeted RNA repeat expansions.
Fortress Biotech & Oncogenity	Active	Unknown	Licensed Columbia University platform with potential DM applications in 2020; no disclosed program progress.
Fulcrum Therapeutics	Active	Unknown	No active DM program; past program on repeat expansion diseases suspended in 2024.

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Genethon	Active	Unknown	Rare disease gene therapy developer; early-stage DM1 research not advanced to pipeline.
Healx Ltd.	Active	Unknown	Ended DM program; focus shifted to other rare diseases.
Idera Pharmaceuticals / Aceragen	Active	Unknown	No active DM program; previously partnered with Scriptr Global for gene therapy research.
Ionis Pharmaceuticals (formerly ISIS Pharmaceuticals)	Active	Unknown	Biogen partnership for antisense therapy ended after failed Phase I/II trial in 2015; published study.
Kate Therapeutics	Acquired	Sold / Outlicensed	Acquired by Novartis in 2025; gene therapy focus in neuromuscular space included DM.
Locana Bio	Closed	Unknown	Company closed in 2023; Cas13d platform targeted DM1 RNA.
Myogem Health Company	Active	Unknown	Nutraceuticals marketed for DM symptom relief; no therapeutic development.
Neubase	Closed	Unknown	Company closed in 2024; proprietary RNA-targeting platform with DM1 program.
Nexien Biopharma	Active	Unknown	Program stopped; cannabinoid-based DM1 program.
Nymirum (Base4 Biotechnology)	Active	Unknown	Early-stage RNA-targeting biotech; no active DM program.
Pfizer	Active	Unknown	Ended DM program in 2021; past collaborations with Dewpoint and Triplet Therapeutics.
Prime Medicine	Active	Unknown	Ended DM program.
Roche	Active	Unknown	Early DM program exploration.
Sangamo	Active	Unknown	Ended gene regulation collaborations relevant to DM. Previous partnerships with Biogen and Novartis.
Syros Pharmaceuticals	Closed	Unknown	Closed in 2025.
Triplet Therapeutics	Closed	Unknown	Company closed in 2022; antisense therapy for repeat expansion diseases included DM. Former partnership with Pfizer.
Wave Life Sciences	Active	Unknown	Past DM1 interest; no current active program.