

Drug Development Pipeline for Myotonic Dystrophy Type 1 (DM1) and Myotonic Dystrophy Type 2 (DM2)

Company	Drug name and approach	Condition	Location of clinical trial/developer	Stage of development					Source of information
				Discovery	Pre-clinical	Phase I	Phase II	Phase III	
Lupin Pharmaceuticals	Mexiletine (NaMuscla) Repurposed antimyotonia small molecule	DM (≥ aged 6 years)	Not listed						Clinical trials
Tor Vergata University of Rome	Metformin Repurposed type 2 diabetes medication that modifies RNA splicing, autophagy, insulin sensitivity or glycogen synthesis	DM1	Italy						Clinical trials
AMO Pharma	Tideglusib Glycogen synthase kinase 3 beta inhibiting small molecule	Congenital DM1	North America and UK						Clinical trials
Osaka University Hospital	Erythromycin (MYD-0124) Repurposed oral antibiotic to reduce RNA toxicity	DM1	Japan						Clinical trials
Expansion Therapeutics	ERX-963 RNA inhibiting small molecule	DM1	USA						Clinical trials
MyoGem	MYODM (caffeine and theobromine) Food supplement	DM1	Spain	No study phase listed on Clinicaltrials.gov					Clinical trials
The Scripps Research Institute, University of Florida and Iowa State University	Cugamycin Small molecule	DM1	USA						Publication
Harmony Biosciences	Pitolisant Repurposed antiepileptic small molecule	DM1	USA						Company pipeline
University of Florida and Osaka University	Erythromycin and pafuramidine Repurposed oral antibiotic enhanced by small molecule	DM1	USA/Japan						Publication
Pompeu Fabra University	Mirtazapine Repurposed antidepressant small molecule	DM1	Italy						Publication
University of Valencia	Chloroquine Repurposed antimalarial small molecule	DM1	Spain						Publication
Arthex Biotech	ARTHEX-01 MicroRNA small molecule	DM1	Spain						Company pipeline
Audentes Therapeutics	AT466 AAV-antisense gene therapy	DM1	USA						Company pipeline
NeuBase Therapeutics	NT0200 Modular antisense peptide nucleic acid	DM1	USA						Company pipeline
Enzerna Biosciences	ENZ-003 Artificial site-specific RNA endonucleases gene therapy	DM	USA						Company pipeline
Osaka University	JM642 Small molecule	DM1	Japan						Publication
Genethon	CRISPR-Cas9 Gene editing	DM1	France						Publication
Nexien BioPharma	Cannabidiol and tetrahydrocannabinol	DM	USA						Company filing
Locana Biosciences	RNA-targeted gene therapy	DM1	USA						Conference presentation
Avidity Biosciences	Antibody-oligonucleotide conjugate	DM1	USA						Company pipeline
Dyne Therapeutics	Antibody-oligonucleotide conjugate	DM1	USA						Company pipeline
MDUK Oxford Neuromuscular Centre	Peptide-conjugated oligonucleotide	DM1	UK						Funding award
University of Washington	RNAi gene therapy	DM1	USA						Conference presentation
Massachusetts General Hospital and Harvard Medical School	Antisense oligonucleotide	DM1	USA						Conference presentation
IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore	Resveratrol Natural compound	DM1	Italy						Publication
The First Affiliated Hospital of Chongqing Medical University	Rapamycin Repurposed antibiotic compound	DM1	China						Publication
Vertex Pharmaceuticals and CRISPR Therapeutics	CRISPR-Cas9 Gene editing	DM1	USA						Press release
University of Cardiff	CRISPR-Cas9 Gene editing	DM1	UK						Funding award
Houston Methodist Research Institute	LshCas13a Gene editing	DM1	USA						Publication
Dewpoint Therapeutics and Pfizer	Biomolecular condensates	DM1	USA						Press release
Ramon Llull University	Small molecule	DM1	USA						Publication
LoQus23 Therapeutics	Small molecule	DM	UK						Other
The Scripps Research Institute and Expansion Therapeutics	Small molecule	DM1	USA						Publication
University of Illinois at Urbana-Champaign	Small molecule	DM1	USA						Funding award
Faze Medicines	Small molecule	DM1	USA						Press release
AskBio	Gene therapy	DM	USA						Press release
Amicus Therapeutics	Gene therapy	DM	USA						Press release
Ionis Pharmaceuticals and Biogen	Antisense oligonucleotide	DM1	USA						Company pipeline
Triplet Therapeutics	Antisense oligonucleotides and small interfering RNAs	DM	USA						Company pipeline
Syros Pharmaceuticals	Small molecule	DM1	USA						Press release
Design Therapeutics	Small molecule	DM	USA						Press release
Expansion Therapeutics	Undisclosed	DM2	USA						Company pipeline
Fulcrum Therapeutics	Undisclosed	DM	USA						Company pipeline
Vertex Pharmaceuticals and Affinia Therapeutics	AAV gene therapy	DM1	USA						Press release
NeuBase Therapeutics	Undisclosed	DM2	USA						Company pipeline

Locations of clinical trials are taken from publicly available clinical trial registry entries
International non-proprietary names or developmental codes are provided in bold

This information is accurate as of 01/02/21

We appreciate all those developing new treatment options for DM patients and would be very thankful for any researchers who may have been missed, to update us on your research. Please contact Ben Porter at ben.porter@newcastle.ac.uk