

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

Company	Drug name and approach	Condition	Clinical trial location	Stage of development					Source of information
				Discovery	Pre-clinical	Phase I	Phase II	Phase III	
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
The Scripps Research Institute, University of Florida and Iowa State University	Cugamycin Small molecule	DM1							Publication
Lupin Pharmaceuticals	Mexiletine Repurposed anti-myotonia small molecule	DM							Company pipeline
Harmony Biosciences	Pitolisant Repurposed anticholinergic small molecule	DM1							Company pipeline
University of Florida and Osaka University	Erythromycin and pajuramide Repurposed oral antibiotic enhanced by small molecule	DM1							Publication
Pompeu Fabra University	Mirtazapine Repurposed antidepressant small molecule	DM1							Publication
University of Valencia	Chloroquine Repurposed antimalarial small molecule	DM1							Publication
Arthex Biotech	ARTHEX-01 MicroRNA small molecule	DM1							Company pipeline
Audentes Therapeutics	A7466 AAV-antisense gene therapy	DM1							Company pipeline
NeuBase Therapeutics	NT0200 Modular antisense peptide nucleic acid	DM1							Company pipeline
Enzerna Biosciences	ENZ-003 Artificial site-specific RNA endonucleases gene therapy	DM							Company pipeline
Osaka University	JM642 Small molecule	DM1							Publication
Genethon	CRISPR-Cas9 Gene editing	DM1							Publication
Nexien BioPharma	Cannabidiol and tetrahydrocannabinol	DM							Company filing
Locana Biosciences	RNA-targeted gene therapy	DM1							Conference presentation
Avidity Biosciences	Antibody-oligonucleotide conjugate	DM1							Company pipeline
Dyne Therapeutics	Antibody-oligonucleotide conjugate	DM1							Company pipeline
MDUK Oxford Neuromuscular Centre	Peptide-conjugated oligonucleotide	DM1							Funding award
University of Washington	RNAi gene therapy	DM1							Conference presentation
Massachusetts General Hospital and Harvard Medical School	Antisense oligonucleotide	DM1							Conference presentation
IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore	Resveratrol Natural compound	DM1							Publication
The First Affiliated Hospital of Chongqing Medical University	Rapamycin Repurposed antibiotic compound	DM1							Publication
Vertex Pharmaceuticals and CRISPR Therapeutics	CRISPR-Cas9 Gene editing	DM1							Company press release
University of Cardiff	CRISPR-Cas9 Gene editing	DM1							Funding award
University of Illinois at Urbana-Champaign	Small molecule	DM1							Funding award
AskBio	Gene therapy	DM							Company press release
Amicus Therapeutics	Gene therapy	DM							Company press release
Ionis Pharmaceuticals and Biogen	Antisense oligonucleotide	DM1							Company pipeline
Triplet Therapeutics	Antisense oligonucleotides and small interfering RNAs	DM							Company pipeline
Syros Pharmaceuticals	Small molecule	DM1							Company press release
Design Therapeutics	Small molecule	DM							Company press release
Expansion Therapeutics	Undisclosed	DM2							Company pipeline
Fulcrum Therapeutics	Undisclosed	DM							Company pipeline
Vertex Pharmaceuticals and Affinia Therapeutics	AAV gene therapy	DM1							Press release
NeuBase Therapeutics	Undisclosed	DM2							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

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

This information is accurate as of 08/09/20