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

Company	Drug name and approach	Condition	Clinical trial location	Stage of development (Discovery / Pre-clinical / Phase I / Phase II / Phase III)	Source of information
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 antimyotonia small molecule	DM			Company pipeline
Harmony Biosciences	***Pitolisant*** Repurposed anticataplectic small molecule	DM1			Company pipeline
University of Florida and Osaka University	***Erythromycin*** *and* ***pafuramidine*** Repurposed oral antibiotic enhanced by small molecule	DM1			Publication
Pompeu Fabra University	***Mirtazapine*** Repurposed antidepressant small molecule	DM1			Publication
University of Valencia	***Chloroquine*** Repurposed antimalerial small molecule	DM1			Publication
Nexien BioPharma	***NX04S1*** Sublingual cannabinoid formulation	DM1			Company pipeline
Nexien BioPharma	***NX04S2*** Sublingual cannabinoid formulation	DM2			Company pipeline
Arthex Biotech	***ARTHEx-01*** MicroRNA small molecule	DM1			Company pipeline
Audentes Therapeutics	***AT466*** 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
Locana Biosciences	RNA-targeted gene therapy	DM1			Funding award
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
University of Illinois at Urbana-Champaign	Small molecule	DM1			Funding award
Genethon	***CRISPR-Cas9*** Gene editing	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
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 publically available clinical trial registry entries

International non-proprietary names or developmental codes are provided in bold

This information is accurate as of 19/05/20

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

The John Walton Muscular Dystrophy Research Centre

The Newcastle upon Tyne Hospitals NHS Foundation Trust

Newcastle University