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 ehrcoxen synthesis	DM1	Italy	Phase III	Clinical trials
AMO Pharma	***Tideglusib*** Glycogen synthase kinase 3 beta inhibiting small molecule	Congenital DM1	North America and UK	Phase III	Clinical trials
Osaka University Hospital	***Erythromycin (MYD-0124)*** Repurposed oral antibiotic to reduce RNA toxicity	DM1	Japan	Phase II	Clinical trials
Expansion Therapeutics	***ERX-963*** RNA inhibiting small molecule	DM1	USA	Phase II	Clinical trials
The Scripps Research Institute, University of Florida and Iowa State University	***Cugamycin*** Small molecule	DM1		Pre-clinical	Publication
Lupin Pharmaceuticals	***Mexiletine*** Repurposed antimyotonia small molecule	DM		Pre-clinical	Company pipeline
Harmony Biosciences	***Pitolisant*** Repurposed anticataplectic small molecule	DM1		Pre-clinical	Company pipeline
University of Florida and Osaka University	***Erythromycin and pafuramidine*** Repurposed oral antibiotic enhanced by small molecule	DM1		Pre-clinical	Publication
Pompeu Fabra University	***Mirtazapine*** Repurposed antidepressant small molecule	DM1		Pre-clinical	Publication
University of Valencia	***Chloroquine*** Repurposed antimalerial small molecule	DM1		Pre-clinical	Publication
Arthex Biotech	***ARTHEx-01*** MicroRNA small molecule	DM1		Pre-clinical	Company pipeline
Audentes Therapeutics	***AT466*** AAV-antisense gene therapy	DM1		Pre-clinical	Company pipeline
NeuBase Therapeutics	***NT0200*** Modular antisense peptide nucleic acid	DM1		Pre-clinical	Company pipeline
Enzerna Biosciences	***ENZ-003*** Artificial site-specific RNA endonucleases gene therapy	DM		Pre-clinical	Company pipeline
Osaka University	***JM642*** Small molecule	DM1		Pre-clinical	Publication
Genethon	***CRISPR-Cas9*** Gene editing	DM1		Pre-clinical	Publication
Nexien BioPharma	Cannabidiol and tetrahydrocannabinol	DM		Pre-clinical	Company filing
Locana Biosciences	RNA-targeted gene therapy	DM1		Pre-clinical	Conference presentation
Avidity Biosciences	Antibody-oligonucleotide conjugate	DM1		Pre-clinical	Company pipeline
Dyne Therapeutics	Antibody-oligonucleotide conjugate	DM1		Pre-clinical	Company pipeline
MDUK Oxford Neuromuscular Centre	Peptide-conjugated oligonucleotide	DM1		Pre-clinical	Funding award
University of Washington	RNAi gene therapy	DM1		Pre-clinical	Conference presentation
Massachusetts General Hospital and Harvard Medical School	Antisense oligonucleotide	DM1		Pre-clinical	Conference presentation
IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore	***Resveratrol*** Natural compound	DM1		Discovery	Publication
The First Affiliated Hospital of Chongqing Medical University	***Rapamycin*** Repurposed antibiotic compound	DM1		Discovery	Publication
Vertex Pharmaceuticals and CRISPR Therapeutics	***CRISPR-Cas9*** Gene editing	DM1		Discovery	Company press release
University of Cardiff	***CRISPR-Cas9*** Gene editing	DM1		Discovery	Funding award
University of Illinois at Urbana-Champaign	Small molecule	DM1		Discovery	Funding award
AskBio	Gene therapy	DM		Discovery	Company press release
Amicus Therapeutics	Gene therapy	DM		Discovery	Company press release
Ionis Pharmaceuticals and Biogen	Antisense oligonucleotide	DM1		Discovery	Company pipeline
Triplet Therapeutics	Antisense oligonucleotides and small interfering RNAs	DM		Discovery	Company pipeline
Syros Pharmaceuticals	Small molecule	DM1		Discovery	Company press release
Design Therapeutics	Small molecule	DM		Discovery	Company press release
Expansion Therapeutics	Undisclosed	DM2		Discovery	Company pipeline
Fulcrum Therapeutics	Undisclosed	DM		Discovery	Company pipeline
Vertex Pharmaceuticals and Affinia Therapeutics	AAV gene therapy	DM1		Discovery	Press release
NeuBase Therapeutics	Undisclosed	DM2		Discovery	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 12/10/20

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

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