

# 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	
Tor Vergata University of Rome	<b>Metformin</b> Repurposed type 2 diabetes medication that modifies RNA splicing, autophagy, insulin sensitivity or glycogen synthesis	DM1	Italy					<a href="#">Clinical trials</a>
AMO Pharma	<b>Tideglusib</b> Glycogen synthase kinase 3 beta inhibiting small molecule	Congenital DM1	North America and UK					<a href="#">Clinical trials</a>
Osaka University Hospital	<b>Erythromycin (MYD-0124)</b> Repurposed oral antibiotic to reduce RNA toxicity	DM1	Japan					<a href="#">Clinical trials</a>
Expansion Therapeutics	<b>ERX-963</b> RNA inhibiting small molecule	DM1	USA					<a href="#">Clinical trials</a>
The Scripps Research Institute, University of Florida and Iowa State University	<b>Cugamycin</b> Small molecule	DM1						<a href="#">Publication</a>
Lupin Pharmaceuticals	<b>Mexiletine</b> Repurposed anti-myotonia small molecule	DM						<a href="#">Company pipeline</a>
Harmony Biosciences	<b>Pitolisant</b> Repurposed anticholinergic small molecule	DM1						<a href="#">Company pipeline</a>
University of Florida and Osaka University	<b>Erythromycin and pajuramide</b> Repurposed oral antibiotic enhanced by small molecule	DM1						<a href="#">Publication</a>
Pompeu Fabra University	<b>Mirtazapine</b> Repurposed antidepressant small molecule	DM1						<a href="#">Publication</a>
University of Valencia	<b>Chloroquine</b> Repurposed antimalarial small molecule	DM1						<a href="#">Publication</a>
Arthex Biotech	<b>ARTHEX-01</b> MicroRNA small molecule	DM1						<a href="#">Company pipeline</a>
Audentes Therapeutics	<b>A7466</b> AAV-antisense gene therapy	DM1						<a href="#">Company pipeline</a>
NeuBase Therapeutics	<b>NT0200</b> Modular antisense peptide nucleic acid	DM1						<a href="#">Company pipeline</a>
Enzerna Biosciences	<b>ENZ-003</b> Artificial site-specific RNA endonucleases gene therapy	DM						<a href="#">Company pipeline</a>
Osaka University	<b>JM642</b> Small molecule	DM1						<a href="#">Publication</a>
Genethon	<b>CRISPR-Cas9</b> Gene editing	DM1						<a href="#">Publication</a>
Nexien BioPharma	Cannabidiol and tetrahydrocannabinol	DM						<a href="#">Company filing</a>
Locana Biosciences	RNA-targeted gene therapy	DM1						<a href="#">Conference presentation</a>
Avidity Biosciences	Antibody-oligonucleotide conjugate	DM1						<a href="#">Company pipeline</a>
Dyne Therapeutics	Antibody-oligonucleotide conjugate	DM1						<a href="#">Company pipeline</a>
MDUK Oxford Neuromuscular Centre	Peptide-conjugated oligonucleotide	DM1						<a href="#">Funding award</a>
University of Washington	RNAi gene therapy	DM1						<a href="#">Conference presentation</a>
Massachusetts General Hospital and Harvard Medical School	Antisense oligonucleotide	DM1						<a href="#">Conference presentation</a>
IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore	<b>Resveratrol</b> Natural compound	DM1						<a href="#">Publication</a>
The First Affiliated Hospital of Chongqing Medical University	<b>Rapamycin</b> Repurposed antibiotic compound	DM1						<a href="#">Publication</a>
Vertex Pharmaceuticals and CRISPR Therapeutics	<b>CRISPR-Cas9</b> Gene editing	DM1						<a href="#">Company press release</a>
University of Cardiff	<b>CRISPR-Cas9</b> Gene editing	DM1						<a href="#">Funding award</a>
University of Illinois at Urbana-Champaign	Small molecule	DM1						<a href="#">Funding award</a>
AskBio	Gene therapy	DM						<a href="#">Company press release</a>
Amicus Therapeutics	Gene therapy	DM						<a href="#">Company press release</a>
Ionis Pharmaceuticals and Biogen	Antisense oligonucleotide	DM1						<a href="#">Company pipeline</a>
Triplet Therapeutics	Antisense oligonucleotides and small interfering RNAs	DM						<a href="#">Company pipeline</a>
Syros Pharmaceuticals	Small molecule	DM1						<a href="#">Company press release</a>
Design Therapeutics	Small molecule	DM						<a href="#">Company press release</a>
Expansion Therapeutics	Undisclosed	DM2						<a href="#">Company pipeline</a>
Fulcrum Therapeutics	Undisclosed	DM						<a href="#">Company pipeline</a>
Vertex Pharmaceuticals and Affinia Therapeutics	AAV gene therapy	DM1						<a href="#">Press release</a>
NeuBase Therapeutics	Undisclosed	DM2						<a href="#">Company pipeline</a>

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](mailto:ben.porter@newcastle.ac.uk)

This information is accurate as of 08/09/20