

SVILUPPO DI POTENZIALI TERAPIE PER LA DISTROFIA MIOTONICA DFI TIPO 1 E TIPO 2 (1)

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, autoophagy, insulin sensitivity or elvcoeen svnthesis	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 anticonvulsant 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 antimalarial small molecule	DM1							Publication
Arthex Biotech	ARTHEX-01 MicroRNA small molecule	DM1							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

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 12/10/20

SVILUPPO DI POTENZIALI TERAPIE PER LA DISTROFIA MIOTONICA DFI TIPO 1 E TIPO 2 (2)

Company	Drug name and approach	Condition	Clinical trial location	Stage of development					Source of information
				Discovery	Pre-clinical	Phase I	Phase II	Phase III	
Audentes Therapeutics	<i>AT466</i> AAV-antisense gene therapy	DM1							Company pipeline
NeuBase Therapeutics	<i>NT0200</i> Modular antisense peptide nucleic acid	DM1							Company pipeline
Enzerna Biosciences	<i>ENZ-003</i> Artificial site-specific RNA endonucleases gene therapy	DM							Company pipeline
Osaka University	<i>JM642</i> Small molecule	DM1							Publication
Genethon	<i>CRISPR-Cas9</i> 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

SVILUPPO DI POTENZIALI TERAPIE PER LA DISTROFIA MIOTONICA DFI TIPO 1 E TIPO 2 (3)

Company	Drug name and approach	Condition	Clinical trial location	Stage of development					Source of information
				Discovery	Pre-clinical	Phase I	Phase II	Phase III	
IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore	<i>Resveratrol</i> Natural compound	DM1							Publication
The First Affiliated Hospital of Chongqing Medical University	<i>Rapamycin</i> Repurposed antibiotic compound	DM1							Publication
Vertex Pharmaceuticals and CRISPR Therapeutics	<i>CRISPR-Cas9</i> Gene editing	DM1							Company press release
University of Cardiff	<i>CRISPR-Cas9</i> 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