

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

| Company | Drug name and approach | Condition | Location of clinical trial/developer | Stage of development | | | | | Source of information |
|---|---|---------------------|--------------------------------------|---|--------------|---------|----------|-----------|---|
| | | | | Discovery | Pre-clinical | Phase I | Phase II | Phase III | |
| Lupin Pharmaceuticals | Mexiletine (NaMuscla) Repurposed antimyotonia small molecule | DM (≥ aged 6 years) | Not listed | | | | | | Clinical trials |
| Tor Vergata University of Rome | Metformin Repurposed type 2 diabetes compound | DM1 | Italy | | | | | | Clinical trials |
| AMO Pharma | Tideglusib Small molecule | Congenital DM1 | North America and UK | | | | | | Clinical trials |
| Osaka University Hospital | Erythromycin (MYD-0124) Repurposed antibiotic compound | DM1 | Japan | | | | | | Clinical trials |
| Expansion Therapeutics | ERX-963 Small molecule | DM1 | USA | | | | | | Clinical trials |
| MyoGem | MYODM (caffeine and theobromine) Food supplement | DM1 | Spain | No study phase listed on Clinicaltrials.gov | | | | | Clinical trials |
| The Scripps Research Institute, University of Florida and Iowa State University | Cugamycin Small molecule | DM1 | USA | | | | | | Publication |
| Harmony Biosciences | Pitolisant Repurposed anticonvulsant small molecule | DM1 | USA | | | | | | Company pipeline |
| University of Florida and Osaka University | Erythromycin and pafuramidine Repurposed oral antibiotic enhanced by small molecule | DM1 | USA/Japan | | | | | | Publication |
| Pompeu Fabra University | Mirtazapine Repurposed antidepressant small molecule | DM1 | Italy | | | | | | Publication |
| University of Valencia | Chloroquine Repurposed antimalarial small molecule | DM1 | Spain | | | | | | Publication |
| Arthex Biotech | ARTHEX-01 MicroRNA small molecule | DM1 | Spain | | | | | | Company pipeline |
| Audentes Therapeutics | AT466 AAV-antisense gene therapy | DM1 | USA | | | | | | Company pipeline |
| NeuBase Therapeutics | NT0200 Modular antisense peptide nucleic acid | DM1 | USA | | | | | | Company pipeline |
| Enzerna Biosciences | ENZ-003 Artificial site-specific RNA endonucleases gene therapy | DM | USA | | | | | | Company pipeline |
| Osaka University | JM642 Small molecule | DM1 | Japan | | | | | | Publication |
| Genethon | CRISPR-Cas9 Gene editing | DM1 | France | | | | | | Publication |
| PepGen | PGN-EDDM1 Peptide conjugated oligonucleotide | DM1 | USA | | | | | | Conference presentation |
| Avidity Biosciences | AOC 1001 Antibody-oligonucleotide conjugate | DM1 | USA | | | | | | Company pipeline |
| Nexien BioPharma | Cannabidiol and tetrahydrocannabinol | DM | USA | | | | | | Company filing |
| Locana Biosciences | RNA-targeted gene therapy | DM1 | USA | | | | | | Conference presentation |
| Dyne Therapeutics | Antibody-oligonucleotide conjugate | DM1 | USA | | | | | | Company pipeline |
| University of Washington | RNAi gene therapy | DM1 | USA | | | | | | Conference presentation |
| Massachusetts General Hospital and Harvard Medical School | Antisense oligonucleotide | DM1 | USA | | | | | | Conference presentation |
| University of Alabama and University of Florida | Ascochlorin Antibiotic compound | DM1 | USA | | | | | | Publication |
| IRCCS Fondazione Don Carlo Gnocchi and Università Cattolica del Sacro Cuore | Resveratrol Natural compound | DM1 | Italy | | | | | | Publication |
| The First Affiliated Hospital of Chongqing Medical University | Rapamycin Repurposed antibiotic compound | DM1 | China | | | | | | Publication |
| Vertex Pharmaceuticals and CRISPR Therapeutics | CRISPR-Cas9 Gene editing | DM1 | USA | | | | | | Press release |
| University of Cardiff | CRISPR-Cas9 Gene editing | DM1 | UK | | | | | | Funding award |
| Houston Methodist Research Institute | LshCas13a Gene editing | DM1 | USA | | | | | | Publication |
| Dewpoint Therapeutics and Pfizer | Biomolecular condensates | DM1 | USA | | | | | | Press release |
| Ramon Llull University | Small molecule | DM1 | USA | | | | | | Publication |
| LoQus23 Therapeutics | Small molecule | DM | UK | | | | | | Other |
| The Scripps Research Institute and Expansion Therapeutics | Small molecule | DM1 | USA | | | | | | Publication |
| University of Illinois at Urbana-Champaign | Small molecule | DM1 | USA | | | | | | Funding award |
| Faze Medicines | Small molecule | DM1 | USA | | | | | | Company pipeline |
| AskBio | Gene therapy | DM | USA | | | | | | Press release |
| Amicus Therapeutics | Gene therapy | DM | USA | | | | | | Company pipeline |
| Ionis Pharmaceuticals and Biogen | Antisense oligonucleotide | DM1 | USA | | | | | | Company pipeline |
| Triplet Therapeutics | Antisense oligonucleotides and small interfering RNAs | DM | USA | | | | | | Company pipeline |
| Syros Pharmaceuticals | Small molecule | DM1 | USA | | | | | | Company pipeline |
| Design Therapeutics | Small molecule | DM | USA | | | | | | Company pipeline |
| Expansion Therapeutics | Undisclosed | DM2 | USA | | | | | | Company pipeline |
| Fulcrum Therapeutics | Undisclosed | DM | USA | | | | | | Company pipeline |
| Vertex Pharmaceuticals and Affinia Therapeutics | AAV gene therapy | DM1 | USA | | | | | | Press release |

Locations of clinical trials are taken from publically available clinical trial registry entries

This information is accurate as of 08/03/21

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