

Drug Development Pipeline for Facioscapulohumeral Muscular Dystrophy Type 1 (FSHD1) and Facioscapulohumeral Muscular Dystrophy Type 2 (FSHD2)

| Company | Drug name and approach | Condition | Stage of development | | | | | Source of information |
|--------------------------------------|---|-----------|----------------------|--------------|---------|----------|-----------|---|
| | | | Discovery | Pre-clinical | Phase I | Phase II | Phase III | |
| Fulcrum Therapeutics | Losmapimod Selective p38a/b mitogen-activated protein kinases inhibitor and DUX4 suppressor | FSHD1 | | | | | | https://clinicaltrials.gov/ct2/show/NCT04003974 https://clinicaltrials.gov/ct2/show/NCT04004000 |
| Murdoch Childrens Research Institute | Creatine monohydrate Nutritional supplement aimed at improving muscle strength | FSHD | | | | | | https://clinicaltrials.gov/ct2/show/NCT02948244 |
| University of Rochester | Testosterone enanthate and somatropin Androgen/anabolic steroid with recombinant human growth hormone | FSHD | | | | | | https://clinicaltrials.gov/ct2/show/NCT03123913 |
| Genea Biocells | GBC0905 Small molecule DUX4 suppressor | FSHD | | | | | | https://www.biospace.com/article/releases/genea-biocells-announces-1da-orphan-drug-designation-for-gbc0905-for-the-treatment-of-facioscapulohumeral-muscular-dystrophy |
| Ultragenyx Pharmaceutical | UX018 Small molecule | FSHD | | | | | | http://tr.ultragenyx.com/static-files/b8fc9bb-dd22-4073-9a02-6f06ff25baec |
| Dyne Therapeutics | FORCE™ platform Oligonucleotide delivery system | FSHD | | | | | | https://www.dyne-tx.com/dyne-therapeutics-presents-nonhuman-primate-proof-of-concept-data-on-force-therapeutic-platform-at-international-myotonic-dystrophy |
| Nationwide Children's Hospital | AAV6.U6.mi405 DUX4-targeted RNAi-based gene therapy | FSHD | | | | | | https://doi.org/10.1016/j.nmd.2019.06.078 |
| University of Minnesota | IP300w Spirocyclic EP300/CBP inhibitor and DUX4 suppressor | FSHD | | | | | | http://doi.org/10.1126/sciadv.aaw7781 |
| Avidity Biosciences | Antibody-oligonucleotide conjugate | FSHD | | | | | | https://www.biospace.com/article/releases/avidity-biosciences-to-present-at-the-2019-myotonic-annual-conference |
| miRecule Inc | DUX4-targeted RNA therapeutic | FSHD | | | | | | https://www.fshdsociety.org/2019/11/08/mirecule-inc-is-developing-a-novel-treatment-for-fshd/ |
| King's College London | Vitamin C, vitamin E or Coenzyme Q10 Targeting HIF1A activity | FSHD | | | | | | https://gtr.ukri.org/projects?ref=MR%2FP023215%2F1 |
| King's College London | Biochanin A, daidzein or genistein ERR agonist | FSHD | | | | | | https://gtr.ukri.org/projects?ref=MR%2FS002472%2F1 |
| Facio Therapies | Small molecule casein kinase 1 inhibitors | FSHD | | | | | | https://doi.org/10.1093/hmg/ddy405 https://www.facio-therapies.com/wp-content/uploads/2019/09/Facio-WMS-October-2019.pdf |

This information is accurate as of 22/01/20