

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	
Acceleron Pharma	ACE-083 Locally acting follistatin therapeutic in the TGF-beta superfamily	FSHD1 and FSHD2						https://clinicaltrials.gov/ct2/show/NCT02927080 https://clinicaltrials.gov/ct2/show/NCT03943290
*aTyr Pharma	Resolaris First in class intravenous protein therapy	FSHD						https://www.globenewswire.com/news-release/2017/09/27/1133698/0/en/aTyr-Pharma-to-Present-Final-Data-for-Resolaris-Phase-1b-2-Trial-in-
Fulcrum Therapeutics	Losmapimod Selective p38a/b mitogen-activated protein kinases inhibitor	FSHD1						https://clinicaltrials.gov/ct2/show/NCT04003974 https://clinicaltrials.gov/ct2/show/NCT04004000
Genea Biocells	GBC0905 Small molecule suppressor of DUX4 function	FSHD						https://www.biospace.com/article/releases/genea-biocells-announces-fda-orphan-drug-designation-for-gbc0905-for-the-treatment-of-facioscapulohumeral-muscular-dystrophy
Ultragenyx Pharmaceutical	UX018 Small molecule	FSHD						http://ir.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
Evotec AG and Facio Therapies	Undisclosed	FSHD						https://www.facio-therapies.com/news/facio-achieves-first-ever-in-vivo-proof-of-principle-in-fshd/

*aTyr Pharma have not stated that this has been discontinued, however, there is very little information available from the past two years.

This information is accurate as of 01/07/19