Our Pipeline

Information is current as of 02/2025, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical

GENE THERAPY

SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 β-sarcoglycan
SRP-9004 (patidistrogene bexoparvovec)	LGMD2D/R3 α-sarcoglycan
SRP-6004	LGMD2B/R2 Dysferlin
Other Targets[1]	Multiple

siRNA

SRP-1001 (ARO-DUX4)	Facioscapulohumeral muscular dystrophy, Type 1 (FSHD1)
SRP-1003 (ARO-DM1)	Myotonic dystrophy, Type 1 (DM1)
SRP-1004 (ARO-ATXN2)	Spinocerebellar ataxia type 2 (SCA2)
SRP-1002 (ARO-MMP7)	Idiopathic pulmonary fibrosis (IPF)
Other Targets[2]	Multiple

GENE EDITING

CRISPR/CAS9	Duchenne

[1] Other gene therapies comprise muscular dystrophy, neuro and cardiac indications including SRP-6006 (LGMD2B/R2 dysferlin), SRP-9005 (LGMD2C/R5 γ-sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), SRP-9010 (LGMD2A/R1 calpain-3-related) and Charcot-Marie-Tooth

[2] Other siRNA indications include Huntington's disease, SCA1 and SCA3