



Our Pipeline

Information is current as of 5/16/2023, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical
--------------	-----------------------	----------

RNA Targeted Therapies PPMO¹

SRP-5051 (vesleteplirsen)	Duchenne	
Other Exon Targets ²	Duchenne	

Gene Therapy

SRP-9001 ³ (delandistrogene moxeparvec)	Duchenne	
GALGT2 - Nationwide Children's	Duchenne	
GNT 0004 - Genethon	Duchenne	
SRP-9003 (bidridistrogene xeboparvec)	LGMD2E/R4 β -sarcoglycan	
SRP-9004 (patidistrogene bexoparvec)	LGMD2D/R3 α -sarcoglycan	
SRP-6004	LGMD2B/R2 Dysferlin	
Other LGMD Targets ⁴	LGMD	
Other Targets	Multiple	

Gene Editing

CRISPR/CAS9 - Duke University	Duchenne	
CRISPR/CAS9 - Harvard University	Duchenne	

¹Peptide phosphorodiamidate morpholino oligomers

²Other exon targets in development: 44, 45, 50, 52, and 53

³Roche has the exclusive rights to launch and commercialize SRP-9001 outside the United States

⁴Other LGMD targets in development: SRP-9005 (LGMD2C/R5 γ -sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and Calpain 3 (LGMD2A/R1)

Duchenne - Duchenne muscular dystrophy

LGMD - Limb-girdle muscular dystrophy