



Our Pipeline

Information is current as of 6/25/2024, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical
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RNA Targeted Therapies PPMO¹

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

Gene Therapy

SRP-9003 (bidridistrogene xeboparovec)	LGMD2E/R4 β -sarcoglycan	
SRP-9004 (patidistrogene bexoparovec)	LGMD2D/R3 α -sarcoglycan	
SRP-6004	LGMD2B/R2 Dysferlin	
Other LGMD Targets ³	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

³Other LGMD targets in development: SRP-9005 (LGMD2C/R5 γ -sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and SRP-9010 (LGMD2A/R1)

Duchenne - Duchenne muscular dystrophy

LGMD - Limb-girdle muscular dystrophy