



Our Pipeline

Information is current as of 1/1/2023, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical	Commercial
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RNA Targeted Therapies PMO¹

EXONDYS 51 (eteplirsen) ²	Duchenne		
VYONDYS 53 (golodirsen) ²	Duchenne		
AMONDYS 45 (casimersen) ²	Duchenne		

RNA Targeted Therapies PPMO³

SRP-5051 (vesleteplirsen)	Duchenne		
Other Exon Targets ⁴	Duchenne		

Gene Therapy

SRP-9001 ⁶ (delandistrogene moxeparvovec)	Duchenne		
GALGT2 - Nationwide Children's	Duchenne		
GNT 0004 - Genethon	Duchenne		
SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 β -sarcoglycan		
SRP-9004 (patidistrogene bexoparvovec)	LGMD2D/R3 α -sarcoglycan		
SRP-6004	LGMD2B/R2 Dysferlin		
Other LGMD Targets ⁶	LGMD		
Other Targets	Multiple		

Gene Editing

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

¹Phosphorodiamidate morpholino oligomers

²Candidate received accelerated approval in the U.S., confirmatory studies are ongoing

³Peptide phosphorodiamidate morpholino oligomers

⁴Other exon targets in development: 44, 45, 50, 52, and 53

⁵Roche has the exclusive rights to launch and commercialize SRP-9001 outside the United States

⁶Other 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