



# Our Pipeline

Information is current as of 8/23/2023, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical
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## RNA Targeted Therapies PPMO<sup>1</sup>

SRP-5051 (vesleteplirsen)	Duchenne	
Other Exon Targets <sup>2</sup>	Duchenne	

## Gene Therapy

GALGT2 - Nationwide Children's	Duchenne	
GNT 0004 - Genethon	Duchenne	
SRP-9003 (bidridistrogene xeboparvec)	LGMD2E/R4 $\beta$ -sarcoglycan	
SRP-9004 (patidistrogene bexoparvec)	LGMD2D/R3 $\alpha$ -sarcoglycan	
SRP-6004	LGMD2B/R2 Dysferlin	
Other LGMD Targets <sup>3</sup>	LGMD	
Other Targets	Multiple	

## Gene Editing

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

<sup>1</sup>Peptide phosphorodiamidate morpholino oligomers

<sup>2</sup>Other exon targets in development: 44, 45, 50, 52, and 53

<sup>3</sup>Other LGMD targets in development: SRP-9005 (LGMD2C/R5  $\gamma$ -sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and SRP-9010 (LGMD2A/R1)

Duchenne - Duchenne muscular dystrophy

LGMD - Limb-girdle muscular dystrophy