



# Our Pipeline

Information is current as of 9/29/2022, updates are made on a quarterly basis

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

EXONDYS 51 (eteplirsen) <sup>2</sup>	Duchenne		
VYONDYS 53 (golodirsen) <sup>2</sup>	Duchenne		
AMONDYS 45 (casimersen) <sup>2</sup>	Duchenne		

## RNA Targeted Therapies PPMO<sup>3</sup>

SRP-5051 (vesleteplirsen)	Duchenne		
Other Exon Targets <sup>4</sup>	Duchenne		

## Gene Therapy

SRP-9001 <sup>5</sup> (delandistrogene moxeparvovec)	Duchenne		
GALGT2 - Nationwide Children's	Duchenne		
GNT 0004 - Genethon	Duchenne		
SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 $\beta$ -sarcoglycan		
SRP-9004 (patidistrogene bexoparvovec)	LGMD2D/R3 $\alpha$ -sarcoglycan		
SRP-6004	LGMD2B/R2 Dysferlin		
Other LGMD Targets <sup>6</sup>	LGMD		
Other Targets	Multiple		

## Gene Editing

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

<sup>1</sup>Phosphorodiamidate morpholino oligomers

<sup>2</sup>Candidate received accelerated approval in the U.S., confirmatory studies are ongoing

<sup>3</sup>Peptide phosphorodiamidate morpholino oligomers

<sup>4</sup>Other exon targets in development: 44, 45, 50, 52, and 53

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

<sup>6</sup>Other LGMD targets in development: SRP-9005 (LGMD2C/R5  $\gamma$ -sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and Calpain 3 (LGMD2A/R1)

Duchenne - Duchenne muscular dystrophy

LGMD - Limb-girdle muscular dystrophy