



# Our Pipeline

Information is current as of 9/27/2023, 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

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>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