

DISCOVERY

PRE-CLINICAL

CLINICAL

COMMERCIAL

RNA-TARGETED THERAPIES PMO

EXONDYS 51 (eteplirsen)*

Duchenne

VYONDYS 53 (golodirsen)*

Duchenne

Casimersen (SRP-4045)

Duchenne

Exon 52

Duchenne

Other Exon Targets**

Duchenne

RNA-TARGETED THERAPIES PPMO

SRP-5051

Duchenne

SRP-5053

Duchenne

SRP-5045

Duchenne

SRP-5052

Duchenne

SRP-5044

Duchenne

SRP-5050

Duchenne

GENE THERAPY

SRP-9001 Micro-Dystrophin

Duchenne

GALGT2 *Nationwide Children's*

Duchenne

Micro-dystrophin *Genethon*

Duchenne

SRP-9003 (LGMD2E β -sarcoglycan)

LGMD

SRP-9004 (LGMD2D α -sarcoglycan)

LGMD

SRP-9005 (LGMD2C γ -sarcoglycan)

LGMD

SRP-6004 (LGMD2B Dysferlin)

LGMD

SRP-9006 (LGMD2L Anoctamin 5)

LGMD

Calpain 3 (LGMD2A) *Nationwide Children's*

LGMD

Neurotrophin 3 (CMT 1A) *Nationwide Children's*

CMT

LYS-SAF 302 (MPS TYPE IIIA) *Lysogene*

MPS

Cardiomyopathy *University of Florida*

CM

Pompe Disease *Lacerta*

Pompe

CNS *Lacerta*

CNS

Multiple Sclerosis *University of Florida*

MS

Rett Syndrome 1 *University of Massachusetts*

Rett

Rett Syndrome 2 *StrideBio*

Rett

Dravet Syndrome *StrideBio*

Dravet

Angelman Syndrome *StrideBio*

Angelman

Niemann-Pick Type C *StrideBio*

Niemann-Pick

Four Muscle/CNS Targets *StrideBio*

Muscle/CNS

Muscle/CV Targets *Dyno Therapeutics*

Muscle/CV

Emery-Dreifuss Muscular Dystrophy Type 1 *Columbia University*

EDMD

Duchenne *Institute of Myology****

Duchenne

GENE EDITING

CRISPR/CAS9 *Duke University*

Duchenne

Duchenne *Harvard University*

Duchenne

*Candidate received accelerated approval in the U.S., confirmatory studies required

**Other exon targets in development: 43, 44, 50, and 55

***Gene therapy and PPMO

Duchenne - Duchenne muscular dystrophy
 EDMD - Emery Dreifuss Muscular Dystrophy
 LGMD - Limb-girdle muscular dystrophy
 MPS - Mucopolysaccharidosis IIIA
 CMT - Charcot-Marie-Tooth disease
 CNS - Central nervous system
 CM - Cardiomyopathy
 MS - Multiple Sclerosis