

DISCOVERY

PRE-CLINICAL

CLINICAL

COMMERCIAL

RNA-TARGETED THERAPIES PMO

EXONDYS 51 (eteplirsen)*	Duchenne
VYONDYS 53 (golodirsen)*	Duchenne
AMONDYS 45 (casimersen)*	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 <i>Nationwide Children's</i>	Duchenne
GNT 0004 <i>Genethon</i>	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) <i>Nationwide Children's</i>	LGMD
Neurotrophin 3 (CMT 1A) <i>Nationwide Children's</i>	CMT
LYS-SAF 302 (MPS TYPE IIIA) <i>Lysogene</i>	MPS
Cardiomyopathy <i>University of Florida</i>	CM
CNS <i>Lacerta</i>	CNS
Pompe Disease <i>Lacerta</i>	Pompe
Niemann-Pick Type C <i>StrideBio</i>	Niemann-Pick
Rett Syndrome 2 <i>StrideBio</i>	Rett
Dravet Syndrome <i>StrideBio</i>	Dravet
Angelman Syndrome <i>StrideBio</i>	Angelman
Four Muscle/CNS Targets <i>StrideBio</i>	Muscle/CNS
Emery-Dreifuss Muscular Dystrophy Type 1 <i>Columbia University</i>	EDMD
Multiple Sclerosis <i>University of Florida</i>	MS
Rett Syndrome 1 <i>University of Massachusetts</i>	Rett
Duchenne <i>Institute of Myology***</i>	Duchenne

GENE EDITING

CRISPR/CAS9 <i>Duke University</i>	Duchenne
Duchenne <i>Harvard University</i>	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