

DISCOVERY	PRE-CLINICAL	CLINICAL	COMMERCIAL
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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