Jett FOUNDATION

Empowering families.
Fighting Duchenne.

CLINICAL TRIAL CHEAT SHEET

Our goal at Jett Foundation is to empower patients and families with the knowledge needed to be their own best advocates, spread awareness about Duchenne, and help accelerate research and development. **We believe for Duchenne patients, education equals survival.** Please use this sheet only as an outline when learning about potential clinical trial options for you, your child, or your patient.

Parent Project Muscular Dystrophy's Duchenne Deletion Tool

CureDuchenne's Chart for Duchenne Individuals Potentially Amenable to Exon Skipping

Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Avidity Biosciences	Study of AOC 1044 in Healthy Adult Volunteers and Participants With Duchenne Muscular Dystrophy (DMD) Mutations Amenable to Exon 44 Skipping (EXPLORE44)	AOC 1044	Exon 44 Skipping	1/2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	7-55 yrs	Ambulatory and Non-Ambulatory	US	NCT05670730
Capricor Inc.	Open-label Extension of the HOPE-2 Trial (HOPE-2-OLE)*	CAP-1002	Cardiosphere-Derived Cells (CDCs)	2	IV Infusion	Mutation Agnostic - All eligible	Active, not Recruiting	≥10 yrs	Ambulatory and Non-Ambulatory	US	NCT04428476
Capricor Inc.	A Study of CAP-1002 in Ambulatory and Non-Ambulatory Patients With Duchenne Muscular Dystrophy (HOPE-3)	CAP-1002	Cardiosphere-Derived Cells (CDCs)	3	IV Infusion	Mutation Agnostic - All eligible	Recruiting	≥10 yrs	Ambulatory and Non-Ambulatory	US	NCT05126758
Children's National Research Institute	Single Escalating Dose Pilot Trial of Canakinumab (ILARIS®) in Duchenne Muscular Dystrophy	ILARIS	anti-interleukin antibody injection	1/2	Subcutaneous Injection	Mutation Agnostic - All eligible	Active, not Recruiting	≥ 2 yrs and < 6 yrs	Ambulatory	US	NCT03936894
Cumberland Pharmaceuticals	Oral Ifetroban in Subjects with Duchenne Muscular Dystrophy (DMD)	Ifetroban	Selective Thromboxane Receptor Antagonist	2	Oral	Mutation Agnostic - All eligible	Recruiting	≥7 yrs	Ambulatory and Non-Ambulatory	US	NCT03340675

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Dyne Therapeutics	Safety, Tolerability, Pharmodynamic, Efficacy, and Pharmacokinetic Study of DYNE-251 in Participants with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping (DELIVER)	DYNE-251	Exon Skipping	1/2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	4-16 yrs old	Ambulatory and Non-Ambulatory	US/AU/EU/CA	NCT05524883
Edgewise	A Study of EDG-5506 in Children with Duchenne Muscular Dystrophy (LYNX)	EDG-5506	Oral Small Molecule Muscle Stabilizer	2	Oral	Mutation Agnostic - All eligible	Recruiting	4-9 yrs old	Ambulatory	US	NCT05540860
Edgewise	A Study of EDG-5506 in Adult Males with Becker Muscular Dystrophy (ARCH)	EDG-5506	Oral Small Molecule Muscle Stabilizer	1b	Oral	Mutation Agnostic - All eligible	Active, not Recruiting	18-55 yrs old	Ambulatory	US	NCT05160415
Edgewise	A Phase 2 study of EDG-5506 in Adolescents and Adult Males living with Becker Muscular Dystrophy (CANYON)	EDG-5506	Oral Small Molecule Muscle Stabilizer	2	Oral	Mutation Agnostic - All eligible	Active, not Recruiting	12-50 yrs old	Ambulatory	US/EX-US	NCT05291091
Edgewise	A Phase 2, pivotal cohort to study EDG-5506 in Adult Males living with Becker Muscular Dystrophy (GRAND CANYON)	EDG-5506	Oral Small Molecule Muscle Stabilizer	2	Oral	Mutation Agnostic - All eligible	Recruiting	18-50 yrs old	Ambulatory	US/EX-US	NCT05291091
ENCell	Phase 1 Clinical Trial to Determine the Safety and Dose of EN001 in Patients with Duchenne Muscular Dystrophy (DMD)	EN001	Stem Cells	1	IV Infusion	Mutation Agnostic - All eligible	Completed	2-18 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT05338099
FibroGen	Phase 3 Trial of Pamrevlumab or Placebo in Combination With Systemic Corticosteroids in Participants With Ambulatory DMD (LELANTOS-2)	Pamrevlumab	mAb to CTGF	3	IV Infusion	Medical history includes diagnosis of DMD and confirmed Duchenne mutation, including status of exon 44 using a validated genetic test.	Active, not recruiting	6 - 11 yrs	Ambulatory	US/EX-US	NCT04632940

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Italfarmaco	Clinical Study to Evaluate the Efficacy and Safety of Givinostat in Ambulant Patients with DMD	Givinostat	HDAC Inhibitor	3	Oral	Mutation Agnostic - All eligible	Active, not recruiting	6-17 yrs old	Ambulatory	US/EX-US	NCT02851797
Italfarmaco	Givinostat in Duchenne Muscular Dystrophy Long Term Safety and Tolerabillity Study	Givinostat	HDAC Inhibitor	2/3	Oral	Mutation Agnostic - All eligible	Completed	≥7 yrs old	Patients previously treated in Givinostat study	US/EX-US	NCT03373968
Nationwide Children's Hospital	Gene Transfer Clinical Trial to Deliver rAAVrh74.MCK. GALGT2 for Duchenne Muscular Dystrophy	Biological: rAAVrh74.MCK. GALGT2	Gene Therapy	1/2a	IV Infusion	Confirmed mutations in the DMD gene using a clinical accepted technique that completely defines the mutation 1,2	Active, not recruiting	≥4 yrs old	Ambulatory	US	NCT03333590
Nationwide Children's Hospital	Phase 1/2a Systemic Gene Delivery Clinical Trial of scAAV9.U7.AACA for Exon 2 Duplication-Associated Duchenne Muscular Dystrophy	Biological: scAAV9.U7.ACCA	Gene Therapy	1/2a	IV Infusion	Confirmed duplication of exon 2 in the DMD gene using a clinically accepted technique that completely defines the mutation	Active, not recruiting	6 Months - 13 yrs old	Pre-Ambulatory and Ambulatory	US	NCT04240314
Nationwide Children's Hospital	Once Weekly Infant Corticosteroid Trial for DMD	Prednisolone	Corticosteroid	4	Oral	Mutation Agnostic - All eligible	Recruiting	1 month - 30 months	Pre-Ambulatory and Ambulatory	US	NCT05412394
NS Pharma	NS-089/NCNP-02-201 in Boys with Duchenne Muscular Dystrophy (DMD)	NS-089/NCNP-02(Brogidirsen)	Exon Skipping 44	2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	4-14 yrs old	Ambulatory	US/Japan	NCT05996003

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
NS Pharma	A Phase 1/2 of NS-050/ NCNP-03 in Boys with DMD (Meteor50)	NS-050/NCNP-03	Exon Skipping 50	1/2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Not yet recruiting	4-14 yrs old	Ambulatory	US/Japan	NCT06053814
NS Pharma	Study to Assess the Safety and Efficacy of Viltolarsen in Ambulant Boys with DMD (RACER 53-X)	Viltolarsen	Exon Skipping 53	3	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Active, not recruiting	Child, Adult, Older Adult	Ambulatory	US/EX-US	NCT04768062
NS Pharma	Long-term Use of Viltolarsen in Boys With Duchenne Muscular Dystrophy in Clinical Practice (VILT-502)	Viltolarsen	Exon Skipping 53	4	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Active, not recruiting	Child, Adult, Older Adult	Ambulatory and Non-Ambulatory	US/EX-US	NCT04687020
NS Pharma	Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys with DMD (RACER53)	Viltolarsen	Exon Skipping 53	3	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Completed	4-7 yrs old	Ambulatory	US/EX-US	NCT04060199
NS Pharma	Study to Assess the Safety, Tolerability, and Efficacy of Viltolarsen in Ambulant Boys and Non-Ambulant Boys with DMD (Galactic53)	Viltolarsen	Exon Skipping 53	2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Completed	≥8 yrs old	Ambulatory and Non-Ambulatory	US/EX-US	NCT04956289
Percheron Therapeutics	A Multicentre, Randomised, Double-blind, Placebo-controlled and Open Label Extension Study to Assess the Efficacy, Safety, and Pharmacokinetic Profile of of ATL1102 in Non-ambulatory Participants With Duchenne Muscular Dystrophy	ATL1102	Anti-Infammatory	2b	Subcutaneous Injection	Mutation Agnostic - All eligible	Recruiting	10-17 years of age	Non-Ambulatory	EX-US	NCT05938023

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Pfizer	A Phase 3 Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	Fordadistrogene Movaparvovec	Gene Therapy	3	IV Infusion	All except any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting any exon between exon 9 and exon 13, inclusive; OR A deletion that affects both exon 29 and exon 30;OR A deletion that affects any exons between 56-71, inclusive.	Active, not recruiting	4-7 yrs old	Ambulatory	US/EX-US	NCT04281485
Pfizer	A Study to Evaluate the Safety and tolerability of PF-06939926 Gene Therapy in DMD	Fordadistrogene Movaparvovec	Gene Therapy	1b	IV Infusion	All except any any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting any exon between exon 9 and exon 13, inclusive; OR A deletion that affects both exon 29 and exon 30.	Active, not recruiting	≥4 yrs old	Ambulatory and Non-Ambulatory	US	NCT03362502
Pfizer	A Study of Fordadistrogene Movaparvovec in Early Stage Duchenne Muscular Dystrophy	Fordadistrogene Movaparvovec	Gene Therapy	2	IV Infusion	All except any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting any exon between exon 9 and exon 13, inclusive; OR A deletion that affects both exon 29 and exon 30;OR A deletion that affects any exons between 56-71, inclusive.	Active, not recruiting	2-3 yrs old	Ambulatory	US/EX-US	NCT05429372

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Pfizer	A Study to Understand the Long-term Safety and Effects of an Experimental Gene Therapy for Duchenne Muscular Dystrophy	Fordadistrogene Movaparvovec	Gene Therapy	3	IV Infusion	All except any mutation (exon deletion, exon duplication, insertion, or point mutation) affecting any exon between exon 9 and exon 13, inclusive; OR A deletion that affects both exon 29 and exon 30;OR A deletion that affects any exons between 56-71, inclusive.	Recruiting	≥0 years old	Ambulatory and Non-Ambulatory	US/EX-US	NCT05689164
PTC Therapeutics	A Study to Assess Dystrophin Levels in Participants with Nonsense Mutation DMD (nmDMD)	Ataluren	Nonsense Mutations Inhibitor	2	Oral	Documentation of the presence of a nonsense point mutation in the dystrophin gene as determined by gene sequencing.	Completed	2-7 yrs old	N/A	US	NCT03648827
PTC Therapeutics	A Study to Evaluate the Safety and Pharmacokinetics of Ataluren in Participants From ≥6 Months to <2 Years of Age With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD)	Ataluren	Nonsense Mutations Inhibitor	2	Oral	Documentation of the presence of a nonsense point mutation in the dystrophin gene as determined by gene sequencing.	Completed	6 months - 2 years old	Ambulatory	US	NCT04336826
PTC Therapeutics	Long-Term Outcomes of Ataluren in Duchenne Muscular Dystrophy	Ataluren	Nonsense Mutations Inhibitor	3	Oral	Documentation of the presence of a nonsense point mutation in the dystrophin gene as determined by gene sequencing.	Completed	7-16 yrs old	Ambulatory	US/EX-US	NCT03179631

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
PTC Therapeutics	A Study to Assess Dystrophin Levels in Participants with Nonsense Mutation DMD (nmDMD) Who Have Been Treated with Ataluren	Ataluren	Nonsense Mutations Inhibitor	2	Oral	Documentation of the presence of a nonsense point mutation in the dystrophin gene as determined by gene sequencing.	Completed	None	Ambulatory	US	NCT03796637
RegenxBio	AFFINITY DUCHENNE: RGX-202 Gene Therapy in Participants With Duchenne Muscular Dystrophy (DMD)	RGX-202	Gene Therapy	1 & 2	IV Infusion	DMD gene mutation in exons 18 and above, and a clinical picture consistent with typical DMD.	Recruiting	4-11 yrs old	Ambulatory	US	NCT05693142
RegenxBio	Anti-AAV8 Antibody Assessment Study of Boys with DMD	N/A	Observational Screening Study	N/A	N/A	Mutation Agnostic - All eligible	Recruiting	0 to <12 years of age	Ambulatory and Non-Ambulatory	US	NCT05683379
ReveraGen Biopharma and Santhera Pharmaceuticals	A Study to Assess the Efficacy and Safety of Vamorolone in Boys with DMD	Vamorolone	Anti-Infammatory	2b	Oral	Mutation Agnostic - All eligible	Completed	4-7 yrs old	Ambulatory	US/EX-US	NCT03439670
ReveraGen Biopharma and Santhera Pharmaceuticals	A Study to Assess Vamorolone in Boys Ages 2 to <4 Years and 7 to <18 Years With Duchenne Muscular Dystrophy (DMD)	Vamorolone	Anti-Infammatory	2	Oral	Mutation Agnostic - All eligible	Recruiting	2-17 yrs old	Ambulatory and Non-Ambulatory	EX-US only	NCT05185622
ReveraGen Biopharma and Santhera Pharmaceuticals	A Study to Assess Vamorolone in Becker Muscular Dystrophy	Vamorolone	Anti-Infammatory	2	Oral	Mutation Agnostic - All eligible	Recruiting	18-64 yrs old	Ambulatory and Non-Ambulatory	US	NCT05166109
Sarepta	Study of SRP-4045 and SRP-4053 in DMD Patients (ESSENCE)	SPR-4045 & SRP-4043	Exon Skipping 45/53	3	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	6-13 yrs old	Ambulatory	US/EX-US (only recruiting EX-US)	NCT02500381

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Sarepta	A Randomized, Double-blind, Placebo-controlled Study of SRP-9001 for Duchenne Muscular Dystrophy (DMD)	SRP-9001	Gene Therapy	2	IV Infusion	A frameshift mutation contained between exons 18 and 58 (inclusive).	Active, not Recruiting	4-7 yrs old	Ambulatory	US	NCT03769116
Sarepta	A Study to Compare Safety and Efficacy of a High Dose of Eteplirsen in Duchenne Muscylar Dystrophy (DMD) Patients (MIS51ON)	Eteplirsen	Exon Skipping 51	3	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	4-13 yrs old	Ambulatory	US/EX-US	NCT03992430
Sarepta	Two-Part Study for Dose Determination of SRP-5051 (Part A), Then Dose Expansion (Part B) in Patients With Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment (MOMENTUM)	SRP-5051	Exon Skipping 51	2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Recruiting	7-21 yrs old	Ambulatory and Non-Ambulatory	US/EX-US	NCT04004065
Sarepta	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of SRP-9001 in Participants with DMD (EMBARK)	SRP-9001	Gene Therapy	3	IV Infusion	A pathogenic frameshift mutation or premature stop codon contained between exons 18 and 79 (inclusive), with the exception of mutation fully contained within exon 45.	Active, not recruiting	4-7 yrs old	Ambulatory	US/EX-US	NCT05096221
Sarepta	A Gene Transfer Therapy Study to Evaluate the Safety of and Expression From Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) (ENDEAVOR)	SRP-9001	Gene Therapy	1	IV Infusion	Mutation Agnostic - All eligible	Enrolling by Invitation	≥3 yrs old	Ambulatory and Non-Ambulatory	US	NCT04626674

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Sarepta	A Gene Transfer Therapy Study to Evaluate the Safety of SRP-9001 in Participants With Duchenne Muscular Dystrophy (DMD)	SRP-9001	Gene Therapy	1/2	IV Infusion	Mutation Agnostic - All eligible	Completed	3 months-7 yrs old	Ambulatory	US	NCT03375164
Sarepta	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Non-Ambulatory and Ambulatory Participants With Duchenne Muscular Dystrophy (DMD) (ENVISION)	SRP-9001	Gene Therapy	3	IV Infusion	A pathogenic frameshift mutation or premature stop codon contained between exons 18 and 79 (inclusive).	Recruiting	≥8 to <18 yrs	Ambulatory and Non-Ambulatory	US	NCT05881408
Solid Biosciences	Microdystrophin Gene Transfer Study in Adolescents and Children With DMD (IGNITE DMD)	SGT-001	Gene Therapy	1/2	IV Infusion	Mutation Agnostic - All eligible	Completed	4-17 yrs old	Ambulatory and Non-Ambulatory	US	NCT03368742
Solid Biosciences	A Study of SGT-003 Gene Therapy in Duchenne Muscular Dystrophy (INSPIRE DUCHENNE)	SGT-003	Gene Therapy	1/2	IV Infusion	All mutations except established clinical diagnosis of DMD that is associated with any deletion mutation in exons 1 to 11 or 42 to 45, inclusive.	Not yet recruiting	4-7 yrs old	Ambulatory	US	NCT06138639
Taiho Pharmaceutical Co., Ltd.	A Phase 3 Study of TAS-205 in Patients with Duchenne Muscular Dystrophy	TAS-205	Selective hematopoietic prostaglandin D synthase (HPGDS) inhibitor	3	Oral	Mutation Agnostic - All eligible	Recruiting	≥5 yrs old	Ambulatory	EX-US	NCT04587908
University Hospital, Basel, Switzerland	Tamoxifen in Duchenne Muscular Dystrophy: A Multicenter, Randomised, Double-blind, Placebo-controlled, Phase 3 Safety and Efficacy 48-Week Trial	Tamoxifen	Selective estrogren receptor modulator (SERM)	3	Oral	Mutation Agnostic - All eligible	Completed	78 months-16 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT03354039

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MORE TOOLS

PPMD's Duchenne Deletion Tool

CureDuchenne's Chart for Duchenne Individuals Potentially Amenable to Exon Skipping

Sponsor	Study Name	Drug Name	Drug Type	Phase	Route of Administration	Exons Eligible	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
University of Florida	Tadalafil as an Adjuvant to Therapy for Duchenne Muscular Dystrophy	Tadalafil	Phosphodiesterase 5 (PDE5) inhibitor	2/3	Oral	Mutation Agnostic - All eligible	Recruiting	7-13 yrs old	Ambulatory	US	NCT05195775
Wave	An Open-label Phase 1b/2a Study of WVE-N531 in Patients with Duchenne Muscular Dystrophy	WVE-N531	Exon Skipping 53	1/2	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	Active, not Recruiting	5-18 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT04906460

Scan here to be directed to clinicaltrials.gov for the latest Clinical Trial information.

Scan here to be directed to Jett Foundation's Clinical Trial Flight Plan.

While we do our best to keep this information current, the information above changes without notice. For the most up-to-date information on the trials, or to look at inclusion/exclusion criteria, please scan the QR code to go directly to clinicaltrials.gov.

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FDA Approved Drugs

This list includes drugs approved by the Federal Drug Administration for the treatment of Duchenne muscular dystrophy.

Company	Drug Name	Drug Type	Route of Administration	Exons Eligible	Age	Ambulation Status
Catalyst Pharmceuticals	aGamree (Vamorolone)	Corticosteroid	Oral	Mutation Agnostic - All eligible	2 years of age and older	Ambulatory and Non-Ambulatory
ITF Therapeutics	Duvyzat (givinostat)	HDAC Inhibitor	Oral	Please see Exon Deletion Tool or Chart for more information*	6 years of age and older	Ambulatory and Non-Ambulatory
NS Pharma	Viltepso (vitolarsen)	Exon Skipping 53	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	All ages	Ambulatory and Non-Ambulatory (Must start drug when Ambulatory)
PTC Therapeutics	Emflaza (deflazacort)	Corticosteroid	Oral	Mutation Agnostic - All eligible	2 years of age and older	Ambulatory and Non-Ambulatory
Sarepta Therapeutics	Exondys 51 (eteplirsen)	Exon Skipping 51	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	All ages	Ambulatory and Non-Ambulatory (Must start drug when Ambulatory)
Sarepta Therapeutics	Elevidys (delandistrogene moxeparvovec-rokl)	Gene Therapy	IV Infusion	All mutations except those with any deletion in exon 8 and/or exon 9 in the DMD gene	4-5 years of age	Ambulatory
Sarepta Therapeutics	Amondys 45 (casimersen)	Exon Skipping 45	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	All ages	Ambulatory and Non-Ambulatory (Must start drug when Ambulatory)
Sarepta Therapeutics	Vyondys 53 (golodirsen)	Exon Skipping 53	IV Infusion	Please see Exon Deletion Tool or Chart for more information*	All ages	Ambulatory and Non-Ambulatory (Must start drug when Ambulatory)

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