



Empowering families.  
Fighting Duchenne.

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.

Sponsor	Study Name	Drug Name	Drug Type	Phase	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	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	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	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	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	Recruiting	≥7 yrs	Ambulatory and Non-Ambulatory	US	NCT03340675

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Dyne Therapeutics	Safety, Tolerability, Pharmacodynamic, 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	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	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	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	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	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	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	Active, not recruiting	6 - 11 yrs	Ambulatory	US/EX-US	NCT04632940
Italfarmaco	Clinical Study to Evaluate the Efficacy and Safety of Givinostat in Ambulant Patients with DMD	Givinostat	HDAC Inhibitor	3	Active, not recruiting	6-17 yrs old	Ambulatory	US/EX-US	NCT02851797

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Italfarmaco	Givinostat in Duchenne Muscular Dystrophy Long Term Safety and Tolerability Study	Givinostat	HDAC Inhibitor	2/3	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	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	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	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	Gene Therapy	1b	Active, not recruiting	≥4 yrs old	Ambulatory and Non-Ambulatory	US	NCT03362502
(Brogidirsen)"	Exon Skipping 44	2	Recruiting	≥4 to <15 yrs old	Ambulatory	US/EX-US	NCT05996003	EX-US	NCT05429372
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	Recruiting	≥4 to <15 yrs old	Ambulatory	US/EX-US	NCT06053814
NS Pharma	Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys with DMD (RACER53)	Viltolarsen	Exon Skipping 53	3	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	Completed	≥8 yrs old	Ambulatory and Non-Ambulatory	US/EX-US	NCT04956289

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NS Pharma	Long-term Use of Viltolarsen in Boys With Duchenne Muscular Dystrophy in Clinical Practice (VILT-502)	Viltolarsen	Exon Skipping 53	4	Active, not recruiting	Child, Adult, Older Adult	Ambulatory and Non-Ambulatory	US/EX-US	NCT04687020
NS Pharma	NS-089/NCNP-02-201 in Boys With Duchenne Muscular Dystrophy (DMD)	"NS-089/NCNP-02	Anti-Inflammatory	2b	Completed	4-7 yrs old	Ambulatory	US/EX-US	NCT03439670
(Brogidirsen)"	Exon Skipping 44	2	Recruiting	≥4 to <15 years	Ambulatory	US/EX-US	NCT05996003	EX-US only	NCT05185622
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	Recruiting	≥4 to <15 years	Ambulatory	US/EX-US	NCT06053814
Pfizer	A Phase 3 Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy		Gene Therapy	3	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		Gene Therapy	1b	Active, not recruiting	≥4 yrs old	Ambulatory and Non-Ambulatory	US	NCT03362502
Pfizer	A Study of Fordadistrogene Movaparovec in Early Stage Duchenne Muscular Dystrophy		Gene Therapy	2	Active, not recruiting	2-3 yrs old	Ambulatory	US/EX-US	NCT05429372
Pfizer	A Study to Understand the Long-term Safety and Effects of an Experimental Gene Therapy for Duchenne Muscular Dystrophy		Gene Therapy	3	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	Completed	2-7 yrs old	N/A	US	NCT03648827

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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	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	Completed	7-16 yrs old	Ambulatory	US/EX-US	NCT03179631
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	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	Recruiting	4-11 yrs old	Ambulatory	US	NCT05693142
RegenxBio	Anti-AAV8 Antibody Assessment Study of Boys with DMD	N/A	Observational Screening Study	NA	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-Inflammatory	2b	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-Inflammatory	2	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-Inflammatory	2	Recruiting	18-64 yrs old	Ambulatory and Non-Ambulatory	US	NCT05166109

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Sarepta	Study of SRP-4045 and SRP-4053 in DMD Patients (ESSENCE)	SPR-4045 & SRP-4043	Exon Skipping 45/53	3	Recruiting	6-13 yrs old	Ambulatory	US/EX-US (only recruiting EX-US)	NCT02500381
Sarepta	A Randomized, Double-blind, Placebo-controlled Study of SRP-9001 for Duchenne Muscular Dystrophy (DMD)	SRP-9001	Gene Therapy	2	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 Muscular Dystrophy (DMD) Patients (MIS51ON)	Eteplirsen	Exon Skipping 51	3	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	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	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	Enrolling by Invitation	≥3 yrs old	Ambulatory and Non-Ambulatory	US	NCT04626674

Sponsor	Study Name	Drug Name	Drug Type	Phase	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	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	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	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	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	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 estrogen receptor modulator (SERM)	3	Completed	78 months-16 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT03354039
University of Florida	Tadalafil as an Adjuvant to Therapy for Duchenne Muscular Dystrophy	Tadalafil	Phosphodiesterase 5 (PDE5) inhibitor	2/3	Recruiting	7 - 13 yrs old	Ambulatory	US	NCT05195775

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Wave	An Open-label Phase 1b/2a Study of WVE-N531 in Patients with Duchenne Muscular Dystrophy	WVE-N531	Exon Skipping 53	1/2	Recruiting	5-18 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT04906460

Updated December 12, 2023



Scan here to be directed to [clinicaltrials.gov](https://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](https://clinicaltrials.gov).