



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.

Sponsor	Study Name	Drug Name	Drug Type	Phase	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
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
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
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	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 Adult Males with Becker Muscular Dystrophy (CANYON)	EDG-5506	Oral Small Molecule Muscle Stabilizer	2	Recruiting	12-50 yrs old	Ambulatory	US	NCT05291091

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ENCell	Phase 1 Clinical Trial to Determine the Safety and Dose of EN001 in Patients with Duchenne Muscular Dystrophy (DMD)	EN001	Stem Cells	1	Recruiting	2-18 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT05338099
FibroGen	Trial of Pamrevlumab (FG-3019) in Ambulatory Subjects With (DMD)	Pamrevlumab	mAb to CTGF	3	Active, not recruiting	6 - 11 years	Ambulatory	US/EX-US	NCT04632940
FibroGen	Trial of Pamrevlumab (FG-3019), in Non-Ambulatory Subjects With Duchenne Muscular Dystrophy	Pamrevlumab	mAb to CTGF	2	Active, not recruiting	≥12 yrs old	Non-Ambulatory	US	NCT02606136
FibroGen	Trial of Pamrevlumab (FG-3019), in Non-Ambulatory Subjects with DMD	Pamrevlumab	mAb to CTGF	3	Active, not recruiting	≥12 yrs old	Non-Ambulatory	US/EX-US	NCT04371666
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
Italfarmaco	Givinostat in Duchenne Muscular Dystrophy Long Term Safety and Tolerability Study	Givinostat	HDAC Inhibitor	2/3	Enrolling by Invitation	≥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	NCT03333590
NS Pharma	Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys with DMD (RACER53)	Viltolarsen	Exon Skipping 53	3	Recruiting	4-7 yrs old	Ambulatory	US/EX-US	NCT04060199

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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	Active, not recruiting	≥8 yrs old	Ambulatory and Non-Ambulatory	US/EX-US	NCT04956289
Pfizer	A Phase 3 Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	PF-06939926	Gene Therapy	3	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	PF-06939926	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	PF-06939926	Gene Therapy	2	Recruiting	2-3 yrs old	Ambulatory	EX-US	NCT05429372
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
PTC Therapeutics	An open-label study evaluating the safety and pharmacokinetics of Ataluren	Ataluren	Nonsense Mutations Inhibitor	2	Recruiting	6 months - 2 years old	Ambulatory	US	NCT04336826
PTC Therapeutics	Long-Term Outcomes of Ataluren in Duchenne Muscular Dystrophy	Ataluren	Nonsense Mutations Inhibitor	3	Active, not recruiting	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
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

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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
Sarepta	Study of SRP-4045 and SRP-4053 in DMD Patients (ESSENCE)	SRP-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 Delivery Study to Evaluate the Safety of and Expression From SRP-9001 in DMD (ENDEAVOR)	SRP-9001	Gene Therapy	1	Enrolling by Invitation	≥3 yrs old	Ambulatory and Non-Ambulatory	US	NCT04626674

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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	Active, not recruiting	3 months-7 yrs old	Ambulatory	US	NCT03375164
Solid Biosciences, LLC	Microdystrophin Gene Transfer Study in Adolescents and Children With DMD (IGNITE DMD)	SGT-001	Gene Therapy	1/2	Active, not recruiting	4-17 yrs old	Ambulatory and Non-Ambulatory	US	NCT03368742
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	Recruiting	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
Wave	An Open-label Phase 1b/2a Study of WWE-N531 in Patients with Duchenne Muscular Dystrophy	WWE-N531	Exon Skipping 53	1/2	Recruiting	5-18 yrs old	Ambulatory and Non-Ambulatory	EX-US	NCT04906460

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

Every effort has been made to ensure the accuracy, completeness and reliability of the information provided, Jett Foundation assumes no responsibility therefore and the user of the information agrees that the information is subject to change without notice.