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.

Sponsor	Study Name	Drug Name	Drug Type	Phase	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Astellas	A Study to Assess the Safety, Tolerability and Preliminary Efficacy of ASP0367 (MA-0211) in Pediatric Male Participants With Duchenne Muscular Dystrophy (DMD)	ASP0367 (MA-0211)	Oral Small Molecule - Mitochondrial Modulator	1b	Recruiting	8-16 yrs old male	Ambulatory and Non-Ambulatory	US	NCT04184882
Capricor Inc.	Open-label Extension of the HOPE-2 Trial (HOPE-2-OLE)	CAP-1002	Cardiosphere-Derived Cells (CDCs)	2	Enrolling by Invitation	≥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	Not yet recruiting	≥10 yrs	Ambulatory and Non-Ambulatory	US	NCT05126758
Cumberland Pharma	A Randomized, Double-blind, Placebo-controlled Study of Oral Ifetroban for Duchenne Muscular Dystrophy (DMD) Cardiomyopathy	Ifetroban	Oral Small Molecule - Thromboxane Receptor Antagonist	2	Recruiting	≥7 yrs old	Ambulatory and Non-Ambulatory	US	NCT03340675
Edgewise	A Study of EDG-5506 in Adult Males with Becker Muscular Dystrophy (ARCH)	EDG-5506	Oral Small Molecule Muscle Stabalizer	1b	Recruiting	18-55 yrs old	Ambulatory	US	NCT05160415
FibroGen	Trial of Pamrevlumab (FG-3019), in Non-Ambulatory Subjects with DMD	Pamrevlumab	mAb to CTGF	3	Recruiting	≥12 yrs old	Non-Ambulatory	US/EX-US	NCT04371666
FibroGen	Trial of Pamrevlumab (FG-3019) in Ambulatory Subjects With (DMD)	Pamrevlumab	mAb to CTGF	3	Recruiting	6 - 11 years	Ambulatory	US/EX-US	NCT04632940

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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
Italfarmaco	Clinical Study to Evaluate the Efficacy and Safety of Givinostat in Ambulant Patients with DMD	Givinostat	HDAC Inhibitor	3	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	Recruiting by Invitation	≥7.5 yrs old	Patients previously treated in Givinostat study	US/EX-US	NCT03373968
NS Pharma	Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys with DMD	Viltolarsen	Exon Skipping 53	3	Recruiting	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	Viltolarsen	Exon Skipping 53	2	Recruiting	≥4 yrs old	Ambulatory and Non-Ambulatory	US/EX-US	NCT04956289
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 Phase 3 Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	PF-06939926	Gene Therapy	3	Active, not recruiting	4-7 yrs old	Ambulatory	EX-US	NCT04281485
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	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
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

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
ReveraGen Biopharma and Santhera Pharmaceuticals	A Study to Assess the Efficacy and Safety of Vamorolone in Boys with DMD	Vamorolone	Anti-Infammatory	2b	Completed	4-7 yrs old	Ambulatory	US/EX-US	NCT03439670
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 Muscylar Dystrophy (DMD) Patients (MIS51ON)	Eteplirsen	Exon Skipping 51	3	Recruiting	4-13 yrs old	Ambulatory	US/EX-US	NCT03992430
Sarepta	A Phase 2 Study for Dose Determination of SRP-5051, Then Dose Expansion 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 (only recruiting 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	Recruiting	4-7 yrs old	Ambulatory	US	NCT05096221
Sarepta	An Extension Study to Evaluate Casimersen and Golodirsen in Patient with DMD	Casimersen & Golodirsen	Gene Therapy	3	Enrolling by Invitation	7-23 yrs old	Ambulatory and Non-Ambulatory	US	NCT03532542
Sarepta	A Gene Delivery Study to Evaluate the Safety of and Expression From SRP-90001in DMD (ENDEAVOR)	SRP-9001	Gene Therapy	1	Enrolling by Invitation	≥3 yrs old	Ambulatory and Non-Ambulatory	US	NCT04626674
Sarepta	A 48-Weel, Open-Label, Study to Evaluate the Efficacy and Safety of Casimersen, Eteplirsen and Golodirsen in Subjects with DMD Carrying Eligible DMD Duplications	Casimersen, Eteplirsen & Golodirsen	Gene Therapy	1	Active, not Recruiting	≥ 6 months old	Ambulatory and Non-Ambulatory	US	NCT04179409

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Sponsor	Study Name	Drug Name	Drug Type	Phase	Status	Age	Ambulation Status	Location	ClinicalTrials.gov Identifier
Solid Biosciences, LLC	Microdystrophin Gene Transfer Study in Adolescents and Children With DMD (IGNITE DMD)	SGT-001	Gene Therapy	1/2	Recruiting	4-17 yrs old	Ambulatory and Non-Ambulatory	US	NCT03368742
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	5-12 yrs old	Ambulatory and Non-Ambulatory	EX-US (Canada/UK)	NCT04906460

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Scan here to be directed to our website for the latest in Clinical Trial information.

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