

Company	Compound	Phase	Indication	Locations	Clinical Trial Study Title
Anti-inflammatory					
Children's National Research Institute https://childrensnational.org/clinical-trials	Canakinumab Injection [Ilaris]	Phase 1 & 2	Duchenne Muscular Dystrophy 2 Years and older Ambulatory	USA (DC)	Single Escalating Dose Pilot Trial of Canakinumab (ILARIS®) in Duchenne Muscular Dystrophy
Taiho Pharmaceutical Co., Ltd. https://www.taiho.co.jp/en/science/pipeline/	TAS-205	Phase 3	Duchenne Muscular Dystrophy 5 Years and older Ambulatory	JAP	A Phase 3 Study of TAS-205 in Patients With Duchenne Muscular Dystrophy (REACH-DMD)
ReveraGen BioPharma, Inc. https://www.reveragen.com/clinical-trials/	Vamorolone	Phase 2	Duchenne Muscular Dystrophy 2 Years to 17 Years	CAN	A Study to Assess Vamorolone in Boys Ages 2 to <4 Years and 7 to <18 Years With Duchenne Muscular Dystrophy (DMD)
Children's Hospital of Chicago Children's Hospital Medical Center, Cincinnati Nationwide Children's Hospital https://www.nationwidechildrens.org/research/clinical-research/gene-therapy-clinical-research	Prednisolone	Phase 4	Duchenne Muscular Dystrophy 1 Month to 30 Months	USA (OH)	Once Weekly Infant Corticosteroid Trial for DMD
ReveraGen BioPharma, Inc. / Santhera Pharmaceuticals https://www.santhera.com/patients-overview#our-research	Vamorolone	Phase 2	Becker Muscular Dystrophy 18 Years to 64 Years Ambulatory	USA (PA)	A Study to Assess Vamorolone in Becker Muscular Dystrophy (BMD)
University of Florida / U.S. Army Medical Research and Development Command https://raredisease.powellcenter.med.ufl.edu/clinical-trial/clinical-trials/duchenne/more-from-our-collaborators/	Prednisone	Phase 2	Duchenne Muscular Dystrophy 5 Years to 8 Years Ambulatory	USA (FL)	Twice Weekly Steroids and Exercise as Therapy for DMD
Cardiac Function					
Medical University of Gdansk https://medmd.gumed.edu.pl/	Metoprolol Succinate	Phase 3	Duchenne Muscular Dystrophy 8 Years to 17 Years	POL	The Efficacy and Safety of Metoprolol as add-on Treatment to Standard of Care in Preventing Cardiomyopathy in Patients With DMD
Peking Union Medical College Hospital https://www.pumch.cn/en/detail/14928.html	Bisoprolol Fumarate	Phase 2 & 3	Duchenne Muscular Dystrophy 7 Years and older	CHN	Bisoprolol in DMD Early Cardiomyopathy

Cumberland Pharmaceuticals Vanderbilt University Medical Center https://www.cumberlandpharma.com/	Ifetroban	Phase 2	Duchenne Muscular Dystrophy 7 Years and older	USA (AR)	Oral Ifetroban in Subjects With Duchenne Muscular Dystrophy
Cell Therapy					
Capricor Inc. https://capricor.com/product-pipeline/	CAP-1002	Phase 3	Duchenne Muscular Dystrophy 10 Years and older	USA (AR, CA, CO, GA, IA, MA, MI, OH, UT, WI)	A Study of CAP-1002 in Ambulatory and Non-Ambulatory Patients With Duchenne Muscular Dystrophy
CHU de Quebec-Universite Laval https://www.chudequebec.ca/accueil.aspx	Myoblast transplantation	Phase 1 & 2	Duchenne Muscular Dystrophy 16 Years and older	CAN	Transplantation of Myoblasts to Duchenne Muscular Dystrophy (DMD) Patients
Exon Skipping					
Wave Life Sciences Ltd. https://wavelifesciences.com/pipeline/	WVE-N531	Phase 1 & 2	Duchenne Muscular Dystrophy 5 Years to 18 Years Amenable to skipping exon 53	CAN, GBR	Open-label Study of WVE-N531 in Patients With Duchenne Muscular Dystrophy
Dyne Therapeutics https://www.dyne-tx.com/pipeline/	DYNE-251	Phase 1 & 2	Duchenne Muscular Dystrophy 4 Years to 16 Years Amenable to skipping exon 51	USA (CO, GA, MA)	Safety, Tolerability, Pharmacodynamic, Efficacy, and Pharmacokinetic Study of DYNE-251 in Participants With Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping
Sarepta Therapeutics, Inc. https://clinicaltrials.sarepta.com/MIS51ON	Eteplirsen	Phase 3	Duchenne Muscular Dystrophy 4 Years to 13 Years Ambulatory Amenable to skipping exon 51	USA (GA) COL, CZE, FRA, GRE, IND, IRE, KOR, MEX, NZ, NOR, POL, SPA, TWN, TUR, GBR	A Study to Compare Safety and Efficacy of a High Dose of Eteplirsen in Participants With Duchenne Muscular Dystrophy (DMD) (MIS51ON)
NS Pharma, Inc. Nippon Shinyaku Co., Ltd. https://www.nspharma.com/pipeline	Viltolarsen	Phase 3	Duchenne Muscular Dystrophy 4 Years to 7 Years Ambulatory Amenable to skipping exon 53	USA (CA, IL) AUS, CAN, CHL, GRC, GBR, HKG, ITA, KOR, MEX, NED, NOR, NZL, RUS, SPA, TWN, TUR, UKR, +	Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys With DMD (RACER53)
Sarepta Therapeutics, Inc. https://www.sarepta.com/clinical-trials	SRP-5051	Phase 2	Duchenne Muscular Dystrophy 7 Years to 21 Years Amenable to skipping exon 51	USA (CT, FL, GA, IA, KS, MA, PA, TX, WA) BEL, GER, ITA, NED, SPA, GBR	Two-Part Study for Dose Determination of SRP-5051 (Vesleteplirsen) (Part A), Then Dose Expansion (Part B) in Participants With Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment

Avidity Biosciences, Inc	AOC 1044	Phase 1 & 2	Duchenne Muscular Dystrophy 7 Years to 45 Years Amenable to skipping exon 44	USA (TX)	Study of AOC 1044 in Healthy Adult Volunteers and Participants With Duchenne Muscular Dystrophy (DMD) Mutations Amenable to Exon 44 Skipping
Gene Therapy					
Pfizer	fordadistrogene movaparovec	Phase 3	Duchenne Muscular Dystrophy 0 Years and older	USA (FL, UT) AUS	A Study to Understand the Long-term Safety and Effects of an Experimental Gene Therapy for Duchenne Muscular Dystrophy
Pfizer	PF-06939926	Phase 2	Duchenne Muscular Dystrophy 2 Years to 3 Years	USA (FL, UT) AUS	Study of Fordadistrogene Movaparovec in Early Stage Duchenne Muscular Dystrophy
Pfizer	PF-06939926	Phase 3	Duchenne Muscular Dystrophy 4 Years to 7 Years Ambulatory	USA (AR, CA, IA, KS, VA, WA) AUS, BEL, CAN, FRA, ITA, JAP, KOR, SPA, CHE, TWN, GBR	Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy
RegenxBio	RGX-202	Phase 1 & 2	Duchenne Muscular Dystrophy 4 Years to 11 Years Ambulatory	USA (AR)	AFFINITY DUCHENNE: RGX-202 Gene Therapy in Participants With Duchenne Muscular Dystrophy (DMD)
Muscle Stabilizer					
Edgewise Therapeutics, Inc.	EDG-5506	Phase 2	Duchenne Muscular Dystrophy 4 Years to 9 Years Ambulatory	USA (GA)	A Study of EDG-5506 in Children With Duchenne Muscular Dystrophy
Edgewise Therapeutics, Inc. Medpace, Inc.	EDG-5506	Phase 2	Becker Muscular Dystrophy 12 Years to 50 Years Ambulatory	USA (AR, CA, CO, IA, KS, MD, MA, MO, OH, VA) NED	Phase 2 Study of EDG-5506 in Becker Muscular Dystrophy (CANYON)
Stop Codon Readthrough					
PTC Therapeutics	Ataluren (PF-069399261)	Phase 2	Duchenne Muscular Dystrophy 6 Months to 2 Years with a nonsense mutation	USA (GA)	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)