

Company	Compound	Phase	Indication & Inclusion Criteria	Locations	Clinical Trial
Exon Skipping					
Wave Life Sciences	WVE-N531	Phase I & II	Duchenne Muscular Dystrophy 5 Years to 18 Years Amenable to skipping Exon 53	CAN, GBR	NCT04906460
Dyne Therapeutics	DYNE-251	Phase I & II	Duchenne Muscular Dystrophy 4 Years to 16 Years Amenable to skipping Exon 51	USA (CA, CO, GA, MA, OH, UT, VA) BEL, CAN, ESP, GBR	NCT05524883
Sarepta Therapeutics	SRP-5051 (Vesleteplirsen)	Phase II	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	NCT04004065
Sarepta Therapeutics	AVI-4658 (Eteplirsen)	Phase III	Duchenne Muscular Dystrophy 4 Years to 13 Years Ambulatory Amenable to skipping Exon 51	USA (AL, FL, GA) COL, CZE, FRA, DNK, DEU, GRC, HUN, IND, IRE, KOR, NLD, MEX, NZL, ROU, NOR, POL, SPA, SVN, TWN, TUR, GBR	NCT03992430
NS Pharma Nippon Shinyaku	Viltolarsen	Phase III	Duchenne Muscular Dystrophy 4 Years to 7 Years Ambulatory Amenable to skipping Exon 53	USA (CA, IL) AUS, CAN, CHL, GRC, GBR, HKG, JPN, ITA, KOR, MEX, NED, NOR, NZL, RUS, SPA, TWN, TUR, UKR	NCT04060199
Avidity Biosciences	AOC 1044	Phase I & II	Duchenne Muscular Dystrophy 7 Years to 55 Years Amenable to skipping Exon 44	USA (TX)	NCT05670730
Gene Therapy					
Pfizer	PF-06939926	Phase III	Duchenne Muscular Dystrophy 0 Year and older	USA (NC)	NCT05689164
Pfizer	PF-06939926	Phase II	Duchenne Muscular Dystrophy 2 Years to 3 Years <i>Exclude</i> : Exon 9-13 (any mutations), 29-30 (deletions), 56-71 (deletions)	USA (FL) AUS Upcoming: USA (PA, UT)	NCT05429372
Pfizer	PF-06939926	Phase III	Duchenne Muscular Dystrophy 4 Years to 7 Years Ambulatory <i>Exclude</i> : Exon 9-13 (any mutations), 29-30 (deletions), 56-71 (deletions)	USA (AR, CA, IA, KS, PA, UT, VA, WA) AUS, BEL, CAN, DEU, FRA, ITA, JPN, KOR, SPA, CHE, TWN, GBR	NCT04281485

RegenexBio	RGX-202	Phase I & II	Duchenne Muscular Dystrophy 4 Years to 11 Years Ambulatory Mutation in Exons 18 and above	USA (AR, IL)	NCT05693142
Stop Codon Readthrough					
PTC Therapeutics	Ataluren (PF-069399261)	Phase II	Duchenne Muscular Dystrophy 6 Months to 2 Years With a nonsense mutation	USA (GA)	NCT04336826
Cell Therapy					
Capricor	CAP-1002	Phase III	Duchenne Muscular Dystrophy 10 Years and older	USA (AR, CA, CO, GA, IA, MA, MI, OH, UT, WI)	NCT05126758
CHU de Quebec-Universite Laval	Myoblast transplantation	Phase I & II	Duchenne Muscular Dystrophy 16 Years and older	CAN	NCT02196467
Anti-inflammatory					
Children's National Research Institute	Canakinumab (Ilaris)	Phase I & II	Duchenne Muscular Dystrophy 2 Years and older Ambulatory	USA (DC)	NCT03936894
Taiho Pharmaceutical	TAS-205	Phase III	Duchenne Muscular Dystrophy 5 Years and older Ambulatory	JPN	NCT04587908
ReveraGen BioPharma Santhera Pharmaceuticals	Vamorolone	Phase II	Duchenne Muscular Dystrophy 2 Years to 17 Years	CAN	NCT05185622
ReveraGen BioPharma Santhera Pharmaceuticals	Vamorolone	Phase II	Becker Muscular Dystrophy 18 Years to 64 Years Ambulatory	USA (PA)	NCT05166109
Nationwide Children's Hospital Children's Hospital of Chicago Children's Hospital Medical Center, Cincinnati	Prednisolone	Phase IV	Duchenne Muscular Dystrophy 1 Month to 30 Months	USA (OH)	NCT05412394

University of Florida U.S. Army Medical Research and Development Command	Prednisone	Phase II	Duchenne Muscular Dystrophy 5 Years to 8 Years Ambulatory	USA (FL)	NCT04322357
Cardiac Function					
Medical University of Gdansk	Metoprolol Succinate	Phase III	Duchenne Muscular Dystrophy 8 Years to 17 Years	POL	NCT05066633
Peking Union Medical College Hospital	Bisoprolol Fumarate	Phase II & III	Duchenne Muscular Dystrophy 7 Years and older	CHN	NCT03779646
Cumberland Pharmaceuticals Vanderbilt University Medical Center	Ifetroban	Phase II	Duchenne Muscular Dystrophy 7 Years and older	USA (AR, CA, DC, GA, IL, IN, MD, MO, OH, TN)	NCT03340675
Muscle Stabilizer					
Edgewise Therapeutics	EDG-5506	Phase II	Duchenne Muscular Dystrophy 4 Years to 9 Years Ambulatory	USA (AR, GA, IA, KS, MA, MO, OH) Upcoming: USA (CA, CO, FL, MD, TX)	NCT05540860
Edgewise Therapeutics Medpace	EDG-5506	Phase II	Becker Muscular Dystrophy 12 Years to 50 Years Ambulatory	USA (AR, CA, CO, IA, KS, MD, MA, MO, OH, VA) NED, GBR	NCT05291091