

Clinical Trials for Duchenne Muscular Dystrophy

Gene Therapy



Sarepta - SRP-9001, NCT#03769116*

Study End Date: October 10, 2022

Trial Phase II

Criteria

Ages: 4-7; Steroids: Yes; No impaired ECHO; No prior gene therapy/exon skipping agents



Pfizer - PF-06939926, NCT# 03362502*

Study End Date: August 26, 2025

Trial Phase I

Criteria

Ages: 4 -12; Ambulation: Ambulatory; Steroids: Yes; No prior gene therapy or exon skipping agents



Solid Bioscience - Ignite, NCT# 03368742

Study End Date: March 2024 (Clinical Hold)

Trial Phase I/II

Criteria

Ages: 4-17; Steroids: Yes; Anti-AAV9 antibodies; No prior investigational drugs/drugs affecting dystrophin or utrophin



Audentes - NCT #04240314

Study End Date: January 2025

Trial Phase I/II

Criteria

Ages: 6 months - 13 years; Pre-ambulant and Ambulant; Exon 2 duplication; No cardiomyopathy; No AAV-binding antibodies; No autoimmune disease



Disclaimer: The form aims to offer a comprehensive look into the current available clinical trials for DMD. Please consult a health specialist before making a decision on treatment.

*Not yet recruiting

NCT# can be used to search up more information on clinicaltrials.gov

Clinical Trials for Duchenne Muscular Dystrophy *Exon Skipping*



Sarepta - SRP-4043, SRP-4045 (Essence), NCT#02500381

Type: Exon skipping; Exon 45/Exon 53 Amenable, Out of frame deletion mutations

Study End Date: May 2023

Trial Phase III

Criteria

Age: 7-13; Ambulation: Ambulatory only; Steroids: Yes



NS Pharma - Viltolarsen (RACER53), NCT #04060199

Study End Date: December 2024

Trial Phase III

Criteria

Ages: 4-7 years; Ambulation Status: Ambulatory; Amenable to Exon 53 skipping; No prior gene therapy; No previous exon skipping



**For more information, visit
clinicaltrials.gov**

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Clinical Trials for Duchenne Muscular Dystrophy

Small Molecule, Anti inflammatory, etc.



Italfarmaco - Givinostat, NCT#02851797

Type: Anti-inflammatory

Study End Date: March 2022

Trial Phase III

Criteria

Ages: 6+ years; Ambulation Status: Ambulatory; Steroids: Yes; No prior gene therapy or exon skipping agents; No exposure to Idebenone



Fibrogen - Pamrevlumab (FG-3019), NCT #02606136*

Study End Date: April 2021

Trial Phase II

Criteria

Ages: 12+; Steroids: Yes; Ambulatory: No; Pending Spine Surgery; Heart Disease; No other investigational drugs; Requires 16 hrs ventilation



PTC Therapeutic - Ataluren, NCT #03179631

Study End Date: October 2023

Trial Phase III

Criteria

Ages: 5+ years; Steroids: Yes; Nonsense Mutation; No elevated Levels of Creatinine or Cystatin C



Santhera - Idebenone (SIDEROS-E), NCT #03603288

Study End Date: January 2024

Trial Phase III

Criteria

Ages: 11+ years; No prior use of any other investigational drug



Astellas - ASP 0367 (MA-0211), NCT #04184882*

Study End Date: September 2021

Trial Phase I

Criteria

Ages: 8-16; Steroids: Yes; No CoQ10; No hypersensitivity to ASP0367; No other investigational drugs; No previous exon skipping



Catabasis - CAT-1004 (Edasalonexent), NCT #03703882*

Type: Small molecule

Trial Phase III

Criteria

Ages: 4-7; Ambulatory: Yes; Steroids: No; No previous investigational drugs; No growth hormones



Cumberland Pharma - Ifetroban, NCT #03340675

Study End Date: July 2023

Trial Phase II

Criteria

Ages: 7+; Steroids: Yes; Stable cardiac function; No other clinical trials within 30 days of study entry



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