



Currently Recruiting Research and Clinical Trials at UCLA for Boys with Duchenne or Becker Muscular Dystrophy

To register with CDMD: Contact Amy Martin, CDMD Community Liaison: (310)-983-3402 or amymartin@ucla.edu

FOR-DMD: [Finding the Optimum Regimen for Duchenne Muscular Dystrophy](#)

Intervention: Comparing dosages of Prednisone and Delfazacort corticosteroids

Participants: Males 4-7 years with DMD who have not previously received corticosteroids

Contact: Dr. Perry Shieh or Katrina Nguyen (310) 825-3264 or KatrinaNguyen@mednet.ucla.edu

Sarepta PROMOVI: [Confirmatory Study of Eteplirsen in DMD Patients](#)

Intervention: Measuring the efficacy of weekly Eteplirsen IV infusion

Participants: Males 7-16 years with DMD. Treated group must have genetic confirmation of DMD amenable to exon 51 skipping. Control group will have confirmation of DMD amenable to skipping of an exon other than 51. Six Minute Walk Test distance of 300 meters or greater

Contact: Dr. Perry Shieh or Juan Valderramos (310) 825-3264 or JValderramos@mednet.ucla.edu

Sarepta ESSENCE: [Study of SRP -4045 and SRP-4053 in DMD Patients](#)

Intervention: Measuring the efficacy SRP-4045 and SRP-4053 compared to placebo

Participants: Males 7-16 years with DMD amenable to exon 45 or 53 skipping. Six Minute Walk Test distance of 300-450 meters

Contact: Dr. Perry Shieh or Juan Valderramos (310) 825-3264 or JValderramos@mednet.ucla.edu

Sarepta 4658-203: [Safety Study of Eteplirsen to Treat Early Stage Duchenne Muscular Dystrophy](#)

Intervention: Measuring the safety and tolerability of Eteplirsen in early stage DMD

Participants: Males 4-6 years with DMD amenable to exon 51 skipping

Contact: Dr. Perry Shieh or Juan Valderramos (310) 825-3264 or JValderramos@mednet.ucla.edu

Sarepta 4658-204: [Safety Study of Eteplirsen to Treat Advanced Stage Duchenne Muscular Dystrophy](#)

Intervention: Measuring the safety and tolerability of Eteplirsen in advanced stage DMD

Participants: Males 7-21 years with DMD amenable to exon 51 skipping. Non-ambulatory or Six Minute Walk Test distance less than 300 meters

Contact: Dr. Perry Shieh or Juan Valderramos (310) 825-3264 or JValderramos@mednet.ucla.edu

Marathon MP-104: [A Pharmacokinetic Study of Oral Deflazacort in Children and Adolescent Subjects With Duchenne Muscular Dystrophy](#)

Intervention: Measuring the dosing pharmacokinetics of oral Deflazacort

Participants: Males 4-16 years with DMD who has not previously received Deflazacort

Contact: Dr. Perry Shieh or Clara Sam (310) 825-3264 or chsam@mednet.ucla.edu

Marathon MP-104 Extension: [An Open-Label, Long-Term Extension Study to Evaluate the Safety and Tolerability of Deflazacort](#)

Intervention: Measuring the safety and tolerability of oral Deflazacort

Participants: Males 4-16 years with DMD who participated in the MP-104-CL-005 PK study

Contact: Dr. Perry Shieh or Clara Sam (310) 825-3264 or chsam@mednet.ucla.edu

Pfizer: [A Phase 2 Study to Evaluate the Safety, Efficacy, Pharmacokinetics and Pharmacodynamics of PF-06252616 in Duchenne Muscular Dystrophy](#)

Intervention: Measuring the safety and efficacy of PF-06252616

Participants: Ambulatory males 6-9 years with DMD who are able to perform the 4 stair climb in more than 0.33 stairs/second but less than 1.6 stairs/second

Contact: Dr. Perry Shieh or Juan Valderramos (310) 825-3264 or JValderramos@mednet.ucla.edu

Ohio State University (UCLA Local Site): [Therapeutic Potential for Aldosterone Inhibition in Duchenne Muscular Dystrophy \(Early Treatment of Cardiomyopathy\)](#)

Intervention: Measuring the effects of Eplerenone compared to spironolactone on heart function

Participants: Males 10 years or older, non-ambulatory with DMD

Contact: Dr. Nancy Halnon (310) 267-7667 or nhalnon@mednet.ucla.edu

UCLA: [Brain Imaging Signatures in Duchenne Muscular Dystrophy and Their Correlates with Cognitive Function](#)

Intervention: None, single MRI brain scan and cognitive and behavioral assessments completed. Monetary compensation provided.

Participants: Males 9-15 years with DMD, no implants or other metals contraindicated for scanning

Contact: Dr. Shantanu Joshi (310) 267-5156 or sjoshi@mednet.ucla.edu

UCLA: [Genetic Modifiers of Duchenne and Becker Muscular Dystrophy](#)

Intervention: Blood/saliva sample collection to identify genes and gene variants that may modify the disease process

Participants: Males with DMD/BMD

Contact: Dr. Stanley Nelson (310) 991-2635 or snelson@ucla.edu

UCLA NIH/CIRM: [Molecular Genetics of Degenerative Diseases Study](#)

Intervention: Skin punch biopsy or blood draw to create permanent cell lines for laboratory studies of cells

Participants: Males with DMD/BMD

Contact: Dr. Stanley Nelson (310) 991-2635 or snelson@ucla.edu

Nationwide Children's Hospital (UCLA Local Site): [Sudden Cardiac Death in Duchenne Muscular Dystrophy: Incidence and Prevention Patient Registry](#)

Intervention: Registry to determine the rate at which children suffer from sudden cardiac death and whether therapy with internal cardiac defibrillators is beneficial

Participants: 10 years old and have DMD and a weak heart (ejection fraction less than or equal to 35%) or have a heart device (pacemaker, ICD)

Contact: Dr. Nancy Halnon (310) 267-7667 or nhalnon@mednet.ucla.edu

Parent Project Muscular Dystrophy: [DuchenneConnect Registry](#)

Intervention: None, patient registry, **please create account at duchenneconnect.org**

Participants: Patients with DMD or BMD

Contact: DuchenneConnect Coordinator (201) 937-1408 or coordinator@duchenneconnect.org