

ClinicalTrials.gov Search Results 01/14/2019

| | Title | Status | Study Results | Conditions | Interventions | Locations |
|---|--|-------------------------|----------------------|------------------------------------|--|---|
| 1 | Validating Cardiac MRI Biomarkers and Genotype-Phenotype Correlations for DMD | Recruiting | No Results Available | •Muscular Dystrophy, Duchenne | <ul style="list-style-type: none"> •Other: Cardiac MRI with contrast •Other: Cardiac MRI without contrast •Other: Blood Test •Other: Heart Rate •Other: Pulmonary Function Test •Other: Genetic Testing •Other: Repeat MRI scan | <ul style="list-style-type: none"> •University of California, Los Angeles, Los Angeles, California, United States •Children's Hospital of Orange County, Orange, California, United States |
| 2 | A Study to Assess Dystrophin Levels in Participants With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD) Who Have Been Treated With Ataluren | Not yet recruiting | No Results Available | •Duchenne Muscular Dystrophy | •Other: Dystrophin levels | •University of California, Los Angeles (UCLA), Los Angeles, California, United States |
| 3 | The Study of Skeletal Muscle Blood Flow in Becker Muscular Dystrophy | Recruiting | No Results Available | •Muscular Dystrophy | <ul style="list-style-type: none"> •Drug: Tadalafil 20 MG •Other: beetroot juice extract | •Cedars-Sinai Medical Center, Los Angeles, California, United States |
| 4 | An Extension Study to Evaluate Casimersen or Golodirsen in Patients With Duchenne Muscular Dystrophy | Enrolling by invitation | No Results Available | •Duchenne Muscular Dystrophy | <ul style="list-style-type: none"> •Drug: Casimersen •Drug: Golodirsen | <ul style="list-style-type: none"> •University of California Los Angeles, Los Angeles, California, United States •Ann and Robert H Lurie Childrens Hospital of Chicago, Chicago, Illinois, United States •Johns Hopkins University School of Medicine, Baltimore, Maryland, United States •Fondazione Policlinico Universitario A Gemelli, Milano, Italy •Great Ormond Street Hospital (GOSH), London, United Kingdom •Royal Victoria Infirmary, Newcastle Upon Tyne, United Kingdom |
| 5 | A Phase III Double-blind Study With Idebenone in Patients With Duchenne Muscular Dystrophy (DMD) Taking Glucocorticoid Steroids | Recruiting | No Results Available | •Duchenne Muscular Dystrophy (DMD) | <ul style="list-style-type: none"> •Drug: Idebenone 150 mg film-coated tablets •Drug: placebo | <ul style="list-style-type: none"> •University of Alabama, Birmingham, Alabama, United States •Phoenix Children's Hospital, Phoenix, Arizona, United States •Banner University of Arizona Medical Center, Tucson, Arizona, United States •Arkansas Children's Hospital, Little Rock, Arkansas, United States •Childrens Hospital of Los Angeles, Los Angeles, California, United States •David Geffen School of Medicine at UCLA, Los Angeles, California, United States •UC Davis Department of Physical Medicine and Rehabilitation, Sacramento, California, United States •Loma Linda University Healthcare, San Bernardino, California, United States •Shriners Hospitals for Children-Tampa, Tampa, Florida, United States •Rare Disease Research, Atlanta, Georgia, United States •and 55 more |

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| 6 | Study of SRP-4045 and SRP-4053 in DMD Patients | Recruiting | No Results Available | •Duchenne Muscular Dystrophy | <ul style="list-style-type: none"> •Drug: SRP-4045 •Drug: SRP-4053 •Drug: Placebo | <ul style="list-style-type: none"> •Neuromuscular Research Center, Phoenix, Arizona, United States •Children's Hospital Los Angeles, Los Angeles, California, United States •David Geffen School of Medicine, UCLA, Los Angeles, California, United States •UC Davis Medical Center, Sacramento, California, United States •Rady Children's Hospital San Diego/ UCSD, San Diego, California, United States •Stanford University School of Medicine/Medical Center, Stanford, California, United States •Connecticut Children's Medical Center, Hartford, Connecticut, United States •University of Florida, Gainesville, Florida, United States •NW Florida Clinical Research Group, LLC, Gulf Breeze, Florida, United States •Miami, Florida, United States •and 45 more |
| 7 | A Study to Assess Dystrophin Levels in Participants With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD) | Not yet recruiting | No Results Available | •Duchenne Muscular Dystrophy | •Drug: Ataluren | <ul style="list-style-type: none"> •Phoenix Childrens Hospital, Phoenix, Arizona, United States •University of California, Los Angeles (UCLA), Los Angeles, California, United States •University of California (UC) Davis Medical Center, Sacramento, California, United States •Rush University Medical Center, Chicago, Illinois, United States •University of Kansas Medical Center, Kansas City, Kansas, United States •University of Minnesota, Minneapolis, Minnesota, United States •Columbia University College of Physicians & Surgeons, New York, New York, United States •Texas Children's Hospital, Houston, Texas, United States •University of Texas Health Science Center at San Antonio, San Antonio, Texas, United States •Children's Hospital of the King's Daughters, Norfolk, Virginia, United States |

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| 8 | A Study to Evaluate the Safety and Tolerability of PF-06939926 Gene Therapy in Duchenne Muscular Dystrophy | Enrolling by invitation | No Results Available | <ul style="list-style-type: none"> •Duchenne Muscular Dystrophy | <ul style="list-style-type: none"> •Genetic: PF-06939926 | <ul style="list-style-type: none"> •Ronald Reagan UCLA Medical Center Drug Information Center, Los Angeles, California, United States •UCLA (David Geffen School of Medicine), Los Angeles, California, United States •UCLA Mattel Children's Hospital, Los Angeles, California, United States •UCLA Medical Center, Los Angeles, California, United States •Duke Neurology, Durham, North Carolina, United States •Duke University Medical Center, Lenox Baker Children's Hospital, Durham, North Carolina, United States •Biospecimen Repository & Processing Core - BPRC, Durham, North Carolina, United States •Duke Children's Hospital & Health Center, Durham, North Carolina, United States •Duke Early Phase Clinical Research Unit, Durham, North Carolina, United States •Duke University Investigational Drug Services, Durham, North Carolina, United States •and 5 more |
| 9 | Long-Term Outcomes of Ataluren in Duchenne Muscular Dystrophy | Recruiting | No Results Available | <ul style="list-style-type: none"> •Muscular Dystrophy, Duchenne •Muscular Dystrophies •Muscular Disorders, Atrophic •Muscular Diseases •Musculoskeletal Disease •Neuromuscular Diseases •Nervous System Diseases •Genetic Diseases, X-Linked •Genetic Diseases, Inborn | <ul style="list-style-type: none"> •Drug: Ataluren •Drug: PLACEBO | <ul style="list-style-type: none"> •Phoenix Childrens Hospital, Phoenix, Arizona, United States •Children's Hospital of Los Angeles, Los Angeles, California, United States •University of California, San Francisco (UCSF) - Benioff Children's Hospital - Oakland, Oakland, California, United States •Stanford University Medical Center, Palo Alto, California, United States •University of California (UC) Davis Medical Center, Sacramento, California, United States •Loma Linda University Children's Hospital, San Bernardino, California, United States •Yale New Haven Hospital, New Haven, Connecticut, United States •Child Neurology Center of Northwest Florida, Gulf Breeze, Florida, United States •Indiana University Health - Riley Child Neurology, Indianapolis, Indiana, United States •University of Kansas Medical Center, Kansas City, Kansas, United States •and 62 more |

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| 10 | Clinical Trial to Evaluate the Efficacy, Safety, and Tolerability of RO7239361 in Ambulatory Boys With Duchenne Muscular Dystrophy | Recruiting | No Results Available | •Duchenne Muscular Dystrophy | •Drug: RO7239361 •Drug: Placebo for RO7239361 | <ul style="list-style-type: none"> •Neuromuscular Research Center, Phoenix, Arizona, United States •Arkansas Children's Hospital; Pediatrics, Little Rock, Arkansas, United States •David Geffen School of Medicine at UCLA; Clinical Trials Contract Unit, Los Angeles, California, United States •Stanford University, Palo Alto, California, United States •University of California Davis Medical Center, Sacramento, California, United States •Yale University School of Medicine ; Pulmonary & Critical Care, New Haven, Connecticut, United States •University of Florida, Gainesville, Florida, United States •Nemours Children's Hospital, Orlando, Florida, United States •Rare Disease Research, LLC, Atlanta, Georgia, United States •Rush University Medical Center - PPDS, Chicago, Illinois, United States •and 43 more |
| 11 | Clinical Study to Evaluate the Efficacy and Safety of Givinostat in Ambulant Patients With Duchenne Muscular Dystrophy | Recruiting | No Results Available | •Duchenne Muscular Dystrophy | •Drug: givinostat •Drug: placebo | <ul style="list-style-type: none"> •University of California - Davis Medical Center - Devis Physical Medicin & Rehabilitation, Davis, California, United States •David Geffen School of Medicine - UCLA Neurology, Los Angeles, California, United States •Rady Children's Hospital center - UCSD Department of Neuroscience, San Diego, California, United States •Children's Hospital Colorado, Aurora, Colorado, United States •Connecticut Children's Medical Center - Division Neurology, Hartford, Connecticut, United States •Child Health Research Institute - Department of Pediatrics, Gainesville, Florida, United States •Nemours Children's Hospital, Orlando, Florida, United States •University of Iowa Children's Hospital, Iowa City, Iowa, United States •Wayne State University - University Pediatrics - Children's Hospital of Michigan, Detroit, Michigan, United States •University of Minnesota - Department of Neurology, Minneapolis, Minnesota, United States •and 30 more |

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| 12 | A Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of a Single Dose of SRP-5051 in Patients With Duchenne Muscular Dystrophy (DMD) | Recruiting | No Results Available | <ul style="list-style-type: none"> •Muscular Dystrophy, Duchenne | <ul style="list-style-type: none"> •Drug: SRP-5051 | <ul style="list-style-type: none"> •David Geffen School of Medicine at UCLA, Los Angeles, California, United States •Neuromuscular Research Center, Sacramento, California, United States •University of Florida, Gainesville, Florida, United States •NW FL Clinical Research Group, LLC, Gulf Breeze, Florida, United States •Rare Disease Research, LLC, Atlanta, Georgia, United States •Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, Illinois, United States •University of Kansas Medical Center, Kansas City, Kansas, United States •Washington University in St. Louis, Saint Louis, Missouri, United States •The Research Institute at Nationwide Children's Hospital, Columbus, Ohio, United States •Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pennsylvania, United States •Children's Medical Center Dallas, Dallas, Texas, United States •London Health Sciences Centre, London, Ontario, Canada |
| 13 | A Study of CAP-1002 in Ambulatory and Non-Ambulatory Patients With Duchenne Muscular Dystrophy | Recruiting | No Results Available | <ul style="list-style-type: none"> •Muscular Dystrophies •Muscular Dystrophy, Duchenne •Muscular Disorders, Atrophic •Muscular Diseases •Neuromuscular Diseases •Nervous System Diseases •Genetic Diseases, X-Linked •Genetic Diseases, Inborn | <ul style="list-style-type: none"> •Biological: CAP-1002 •Drug: Placebo | <ul style="list-style-type: none"> •Ronald Reagan UCLA Medical Center, Los Angeles, California, United States •University of California, Davis, Sacramento, California, United States •Children's Hospital Colorado, Aurora, Colorado, United States •Nemours Children's Hospital, Orlando, Florida, United States •Rare Disease Research, Atlanta, Georgia, United States •University of Iowa Stead Family Children's Hospital, Iowa City, Iowa, United States •University of Massachusetts Medical Center, Worcester, Massachusetts, United States •Washington University, Saint Louis, Missouri, United States •Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio, United States •Oregon Health and Science University, Portland, Oregon, United States •and 4 more |