UC DAVIS HEALTH

UC Davis Neuromuscular Research Lab Clinical Studies

Diagnosis	Study Title	Study Description	Study Population	Status	PI	NCT Number
DMD	**Capricor OLE**	This Phase 2, multi-center, open-label extension trial will provide CAP-1002 to subjects that were enrolled in the HOPE-2 trial and completed 12 months of follow-up. The trial will explore the safety and efficacy of eight intravenous administrations of CAP-1002, each separated by three months.	Male subjects 10 year and older who have previously enrolled in the HOPE-2 trials and completed the trial follow up through Month 12	Enrollment Full	Craig McDonald, M.D.	NCT04428476
DMD	**Capricor HOPE 3**	HOPE-3 is a multi-center, randomized, double-blind, placebo-controlled clinical trial evaluating the safety and efficacy of a cell therapy called CAP-1002 in study participants with Duchenne muscular dystrophy (DMD) and impaired skeletal muscle function. Non-ambulatory and ambulatory boys and young men who meet eligibility criteria will be randomly assigned to receive either CAP-1002 or placebo every 3 months for a total of 4 doses during a 12-month period. All participants will be eligible to receive CAP-1002 for an additional 12 months as part of an open label extended safety assessment period.	Male subjects at least 10 years of age at time of consent who are willing and able to provide informed consent to participate in the trial and diagnosed with DMD as confirmed by the Investigator	Open	Craig McDonald, M.D.	NCT05126758
DMD	**DoD Biomarker**	The purpose of this study is to identify proteins in blood that can be used to measure the severity and progression of DMD. This may help to determine whether early treatments can benefit	Males including infants, toddlers, and young children	Open	Craig McDonald, M.D.	N/A

		young children with DMD. Enrolled patients will have blood drawn once a year until the age of 9.				
DMD, BMD, FSHD, CMT, SMA, typically developing volunteers	**MDA Walk for Me**	This research study utilizes the Walk4Me iPhone application to use machine learning techniques and motion sensors to measure how children and adults with and without different neuromuscular diseases walk and run at different speeds. We are looking for participants with Duchenne/Becker muscular dystrophy, Facioscapulohumeral Muscular Dystrophy, Charcot-Marie-Tooth Disease and Spinal Muscular Atrophy as well as typically developing individuals 2 years and older. These computers can use common mobile devices to recognize how well someone is walking in their community.	Participants 2 years and older with or without Duchenne/Becker Muscular Dystrophy, Facioscapulohumeral Muscular Dystrophy, Charcot-Marie-Tooth Disease and Spinal Muscular Atrophy	Open	Erik Henricson, Ph.D.	N/A
DMD	**Italfarmaco Ext**	This is an open label, long-term safety, tolerability, and efficacy study of GIVINOSTAT in all DMD (Duchenne's muscular dystrophy) patients who have been previously treated in one of the GIVINOSTAT studies.	Patients who previously participated in Italfarmaco Original	Enrollment Full	Craig McDonald, M.D.	NCT03373968
DMD	**PTC-016**	The objective of this study is to assess the safety and tolerability of 10, 10, 20 milligrams per kilogram (mg/kg) ataluren in participants with nmDBMD who had prior exposure to ataluren in a PTC sponsored clinical trial or treatment plan, and siblings of those participants (provided those participants have completed the placebo-controlled portion of the trial).	Patients who have previous exposure to Ataluren	Enrollment Full	Craig McDonald, M.D.	NCT01247207
DMD	**Sarepta 103 (ENDEAVOR)**	This is an open-label gene transfer therapy study evaluating the safety of and expression from SRP-9001		Open (Cohort 5)	Craig McDonald, M.D.	NCT04626674

		(delandistrogene moxeparvovec) in participants with DMD over 260 weeks.				
DMD	**Sarepta 301 (EMBARK)**	The study will evaluate the safety and efficacy of gene transfer therapy in boys with DMD. It is a randomized, double-blind, placebo-controlled study. The participants who are randomized to the placebo arm will have an opportunity for treatment with gene transfer therapy at the beginning of the second year.		Enrollment Full	Craig McDonald, M.D.	NCT05096221
DMD	**Sarepta Prospective Chart Review**	This is a prospective observational study of patients receiving Eteplirsen treatment in routine clinical practice. The objective of this observational study is to collect long-term data on DMD patients receiving Eteplirsen in routine clinical practice. No study medication will be provided by the Sponsor as part of this observational study. A 5-year follow-up period will gather additional data regarding exposure to Eteplirsen and the clinical course of DMD patients receiving Eteplirsen.	Males currently on Exondys 51, Golodirsen, and Amondys	Open	Craig McDonald,M.D.	
BMD	**Edgewise**	The ARCH study is an open-label, single-center, Phase 1b study of EDG-5506 to assess the safety and pharmacokinetics (PK) of EDG-5506 in adults with Becker muscular dystrophy (BMD). EDG-5506 is an investigational product intended to protect and improve function of dystrophic muscle fibers.	Male participants ages 18 to 55 who have completed the EDG-5506-001 Study	Open	Craig McDonald, M.D.	NCT05160415
SMA	**AveXis Chart Review**	This is a prospective, multi center, multinational, non-interventional observational study. All patients will be managed according to the clinical site's normal clinical practice, i.e., the diagnostic and clinical	The study will enroll at least 500 patients with a diagnosis of SMA. The registry will attempt to enroll all patients treated	Open	Nanette Joyce, D.O.	NCT04174157

		treatment/practice process that a clinician chooses according to their clinical judgement for an SMA patient. Clinical care will not be driven by the protocol. No additional visits or investigations will be performed beyond normal clinical practice. Patients will be followed for 15 years from enrolment or until death, whichever is sooner.	with AVXS-101 in the registry during 5 years of recruitment.			
DMD	**Radicava**	REFINE-ALS is a prospective, observational, longitudinal, multicenter study designed to identify biomarkers to serve as quantifiable biological non-clinical measures of Edaravone effects in ALS. Epigenetic and protein biomarkers will also be investigated.	Participants who have Sporadic or Familial Amyotrophic Lateral Sclerosis (ALS) who are at least 18 years old	Open	Nanette Joyce, D.O.	NCT04259255
CMT	**Pharnext CMT**	The purpose of this study is to determine whether PXT3003 is effective and safe in the treatment of Charcot-Marie-Tooth disease - Type 1A. This double-blind study will assess in parallel groups 1 dose of PXT3003 compared to Placebo in CMT1A patients treated for 15 months.	Subjects diagnosed with CMT, 16 years to 65 years old	Enrollment Full	Lisa Williams, M.D.	NCT04762758
Chronic Kidney Disease (CKD) and those without CKD	**ESTEEM**	The purpose of this study is to conduct a randomized controlled trial to test the impact of combined aerobic and resistance exercise training on skeletal muscle dysfunction in CKD. A nested cross-sectional investigation will compare the skeletal muscle function of patients with kidney disease to controls without kidney disease and to patients with muscular dystrophinopathies without kidney disease.	Patients between the ages of 30 and 75 years old	Open	Baback Roshanravan, M.D.	N/A
Knee Osteoarthritis	**GIDOA-03**	This study is a pivotal study to evaluate the efficacy and safety of a single injection of autologous adipose-derived SVF produced using the GID SVF-2 device	Subjects 35 to 85 years old	Open	Charles DeMesa, D.O.	NCT04440189

		system for treatment of pain with concomitant improvement in function associated with osteoarthritis of the knee joint.				
LGMD	**Sarepta LGMD**	This study will follow participants who are screened and confirmed with a genetic diagnosis of Limb-girdle muscular dystrophy type 2E (LGMD2E/R4), Limb-girdle muscular dystrophy type 2D (LGMD2D/R3), or Limb-girdle muscular dystrophy type 2C (LGMD2C/R5). These enrolled participants will be followed to evaluate mobility and pulmonary function for up to 3 years after enrollment. Additional participant data will be collected from the time the individual began experiencing LGMD symptoms to the present.	The population for this study is ambulatory or non-ambulatory participants ≥ 4 years of age with confirmed genetic diagnosis of LGMD2E/R4, LGMD2D/R3, or LGMD2C/R5.	Open	Craig McDonald, M.D.	NCT04475926
Hyperlipidemia	**Statin**	The purpose of this study is to observe how an individual's metabolism and/or use of a cholesterol-lowering drug (statin) affects the composition of lipids (fats) in their blood. This affects the inflammation in arteries that promotes heart disease. Researchers hope to use tools developed in the laboratory ("artery-on-a-chip") to analyze a simple blood sample and reliably detect the markers of inflammation to predict a person's risk for disease or response to treatment.	Individuals between the ages of 40 and 75 with no history of chronic inflammatory diseases that may have recently been prescribed a statin but have not begun taking the medication.	Open	Anthony Passerini, Ph.D.	N/A