

Disease	Study Description	Brief Criteria if recruiting
All NM Diseases	Recruitment Database and Specimen Biobank	Anyone with a neuromuscular condition
All NM Diseases	Exercise tolerance in Pompe disease and other disorders	Adults with a neuromuscular condition
ALS	Cytokinetics drug trial of CK-2127107	Recruiting closed
ALS	Natural History study of ALS	Recruiting in clinic
ALS	Biogen drug trial for people with C9orf72 ALS mutations	Recruiting adults with C9orf72 ALS
CMT	Natural History study of CMT	Recruiting in clinic
DMD	Sarepta drug trial	Not recruiting
DMD	Bristol-Myers Squibb/Roche drug trial of BMS-986089	Recruiting boys 6-11 with DMD
DMD	PTC drug trial of ataluren	Recruiting ambulatory DMD age 5+ with nonsense mutations
Dysferlinopathy	Natural History study of Dysferlinopathy	Not recruiting
LEMS	Expanded Access Protocol for Firdapse	Enrollment by invitation only
LEMS	Compassionate use of 3,4-Diaminopyridine	Not recruiting
Congenital Myasthenic Syndrome	Compassionate use of 3,4-Diaminopyridine	Not recruiting
Myasthenia Gravis	Momenta drug trial of M281	Recruiting soon
Myasthenia Gravis	argenX drug trial of ARGX-113	Recruiting soon
Myotonic Dystrophy	EEG and questionnaire studies	Recruiting
Myotonic Dystrophy	Natural History study of adult DM1	Recruiting adults with DM1
Myotonic Dystrophy	Expansion drug trial of ERX-963	Recruiting adults with DM1
Myotonic Dystrophy	AMO pharmaceuticals trial of Tideglusib	Coming soon
Pompe	Natural History study of Pompe and lysosomal storage disorders	Stanford Neuromuscular patients with Pompe
Pompe	Genzyme drug trial of NeoGAA (GZ402666) and Alglucosidase Alfa	Not recruiting
SMA	IONIS drug trial extension of Nusinersen	Not recruiting
SMA	Natural History study of SMA	Recruiting in clinic
SMA	AveXis trial of AAV gene therapy (aka STRONG)	Recruiting children with SMA2 under 60 months of age
SMA	AveXis trial of AAV gene therapy (aka STRIVE)	Not recruiting
SMA	AveXis trial of AAV gene therapy (aka SPRINT)	Recruiting Infants with SMA up to age 6 weeks
SMA	Roche drug trial of VP39055/56 in SMA (aka FIREFISH)	Not recruiting
SMA	Roche drug trial of VP39055/56 in SMA (aka SUNFISH)	Not recruiting
SMA	Roche drug trial of VP39055/56 in SMA (JEWELFISH)	Recruiting SMA ages 6 months to 60 years on Spinraza
SMA	Scholar Rock trial of SRK-015 in SMA (TOPAZ)	Recruiting Later-onset SMA
Tay Sachs	Retrotope compassionate use	Not recruiting