A Message from Dr. John W. Day

We would like to take a minute to thank you for joining our Stanford Neuromuscular Recruitment Database, and update you on the progress of our Neuromuscular Division over the last year. Our Database was created in 2013 to aid recruitment to research studies, drug trials, and outreach events. We have enrolled more than 600 people, initiated more than 30 studies and trials, and organized several conferences and outreach groups. If you know of anyone who is not registered in our Recruitment Database that might like to participate in driving neuromuscular research forward, including friends or family members who do not have a neuromuscular condition, please have them contact our team using the information provided below.

We are poised to have an even more exciting and productive year in 2016, as our team grows and we launch more studies and trials. A list of projects is found on the back of this page. We encourage you to stay in touch with our team through the following:

- **Recruitment Database and study questions** - Contact Katharine Hagerman at (650) 723-9574 or email khagerma@stanford.edu.

- **Social Media** - Join our “Stanford Neuromuscular Disorders” group on Facebook or stay tuned as we post videos to our new “Stanford Neuromuscular Program” YouTube Channel.

Again, thank you for joining the Recruitment Database, and we wish you a safe and happy 2016.

Sincerely,

John W. Day, MD, PhD

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**Stanford events in 2016**
- DM family meeting - January 30th, 2016
- SMA conference - July 2016
- DMD family meeting - September 2016
- CMT conference - September 2016
- MG and FSHD support groups meet quarterly
- DMD young adult group meets quarterly
Research studies, programs, and trials organized by condition:

**All Neuromuscular Conditions**
- Recruitment Database: Enrolling people with neuromuscular conditions into a database for recruitment to upcoming studies (recruiting)
- Biobank: Enrolling people with neuromuscular conditions who are interested in donating biological samples (recruiting)

**ALS**
- Genetics of ALS: Collecting DNA from adults to identify genes influencing ALS (recruiting soon)
- DPS: Determining whether diaphragm pacing systems improve survival or function in adults with ALS (recruiting)
- Cytokinetics: Drug trial testing safety and efficacy of Tirasemtiv in adults with ALS (recruiting soon)

**Charcot-Marie Tooth (CMT)**
- Chart review of pulmonary functions (recruiting)
- Observational study of symptoms, progression, and genetics of CMT (recruiting in clinic)

**Congenital Myopathies**
- Valerion: Study of natural symptom progression in patients with Myotubular Myopathy (recruiting)
- Nemaline and Selenoprotein (SEPN1) myopathies: Recruiting to our database for upcoming studies

**Duchenne Muscular Dystrophy (DMD)**
- Sarepta: Drug trial testing safety/efficacy of drug in boys aged 4-6 and 7-16 with DMD (recruiting)
- BMS: Drug trial testing safety/efficacy of drug in boys aged 5-11 with DMD (recruiting soon)

**Facioscapulohumeral muscular dystrophy**
- ATYR: Drug trial testing safety of ATYR1940-C-0300 in children and adults with FSHD (recruiting soon)

**Limb Girdle Muscular Dystrophies (LGMD)**
- COS: Observational study of adults with LGMD2B, measuring symptom progression (closed)

**Myotonic Dystrophy (DM)**
- DMCRN: Observational study of symptoms and biomarkers over 1 year in adults with DM1 (recruiting)
- Isis/Ionis CS2: Drug trial testing safety/efficacy/dose of DMPKRx in adults with DM1 (recruiting)
- CHRI: Observational study of children ages 8-17 with DM1 and healthy controls, measuring biomarkers and neuropsychological symptoms (recruiting)
- Sleep Study: Observational study of sleep and neuropsychology of adults with DM1 (recruiting)
- Metabolism: Observational study of metabolism in adults with DM (recruiting)

**Neuromuscular Junction Conditions**
- Lambert-Eaton Myasthenic Syndrome (LEMS) trial: Drug trial testing safety/efficacy of Amifampridine Phosphate in adults with LEMS (closed)
- Myasthenia Gravis (MG): Alexion Drug trial testing safety/efficacy of Eculizumab in adults with refractory generalized MG (closed)

**Pompe Disease (PD)**
- Pompe Registry: Observational study tracking outcomes of people with PD (recruiting)
- IPANEMA: Natural history study of PD (recruiting in clinic)
- BioMarin drug trial on PD (recruiting)

**Spinal Muscular Atrophy (SMA)**
- Isis/Ionis CS3B: Drug trial testing safety/efficacy of SMNRx in babies ≤7 months with SMA type 1 (recruiting)
- Isis/Ionis CS4: Drug trial testing safety/efficacy of SMNRx in children ages 2-12 with SMA type 2 (closed)
- PNCRN: Observational study of patients with SMA, collecting samples, medical history, and tracking progression over 3 years (recruiting in clinic)
- Cytokinetics: Drug trial testing efficacy of CK-2127107 in children and adults with SMA2, 3, or 4 (recruiting)

***For all conditions with studies listed as “closed” and other neuromuscular conditions not listed, we are still enrolling participants in our Recruitment Database for studies and trials that are currently in the planning phase.***