MEDPACE

NEUROMUSCULAR DISEASE DRUG DEVELOPMENT

Neuromuscular diseases are particularly challenging when it comes to study design and endpoint selection. Alterations in nerve, muscle, and neuromuscular junction can be due to altered neuromuscular development, degeneration, or metabolic disorders. Causes may be genetic or acquired, but almost all are rare. As a full-service and therapeutically-focused CRO, Medpace specializes in the design and conduct of global trials in neurology and psychiatry, including rare neuromuscular disorders.

Medpace combines our team of neurologists with our cross-functional team of rare disease experts to support Sponsors developing therapies for rare neurological disorders. We bring a global footprint, strategic medical, regulatory and operational leadership as well as fully integrated central labs and core imaging services to enhance and expedite development.

RARE NEUROMUSCULAR INDICATION EXPERIENCE:

- Charcot Marie Tooth Disease
- Familial Amyloidosis Polyneuropathy
- Mucopolysaccharidosis
- Multiple Sclerosis
- Muscular Dystrophies
  - Duchenne
  - Becker
  - Limb Girdle
  - Facioscapulohumeral
- Myasthenia Gravis
- Pompe disease
- Spinal Muscular Atrophy
- Amyotrophic Lateral Sclerosis

EXPERTS

- In-house neurologists, pediatric neurology experts, regulatory experts, imaging experts, and clinical operations professionals that are skilled in neuromuscular disease studies
- Integrated CNS, cardiac and skeletal muscle imaging expertise
- Extensive expertise in gene modulation and editing, gene transfer technology, and other cellular and gene therapies

EXPERIENCE

- Broad experience in Phase I-III neuroscience trials across a wide range of indications
- Strong relationships with key investigative sites
- Experience in complex drug delivery, such as intrathecal/intraparenchymal dose administration and CSF collection
- Established relationships with key patient advocacy groups
- Experience with remote assessments, including video, accelerometry and pulmonary

EXECUTION

- Full-service integrated model to deliver efficient and streamlined execution
- Integrated imaging and ECG core lab with an end-to-end suite of global imaging services
- Global central labs with sequencing capabilities along with safety and biomarker analysis to support neuromuscular disease studies
- Internal patient concierge services, knowledgeable in the special needs of neuromuscular patients and their families

RECRUITMENT AND SITE RELATIONSHIPS

Due to the unique challenges associated with neuromuscular studies, partnering with a CRO with experience recruiting and enrolling patients and relationships with key investigative sites is crucial. Our multi-dimensional recruitment model enables us to implement innovative, comprehensive, and customized recruitment and retention strategies to effectively target members of specific patient populations.

Medpace has also established a productive network of key clinical sites and large academic institutions who specialize in rare neurologic disease disorders ensuring study timelines and key milestones are achieved. Our physicians and professional staff have in-depth knowledge of screening tools and rater scales, and will provide oversight for rater services including subjective assessments, inter-rater reliability, and scale validation.

RELATIONSHIPS WITH PATIENT ADVOCACY GROUPS

Medpace recognizes the importance of collaborating with advocacy groups. Although advocacy groups vary greatly in size, scope, and purpose, they regularly offer insight into who is treating patients, what is important to patients, and lessons learned from past clinical trials. Advocacy group collaboration raises awareness for your clinical trial and can be an invaluable partnership for the life of your drug.

Medpace is well versed in working with sponsors and advocacy groups of different sizes on a global scale in order to ensure your trial is designed and operated with the patients in mind. Our team can help you forge new relationships with advocacy groups or support existing relationships to drive enrollment and keep your trial on schedule.

CENTRAL LABS

Medpace Central Labs offer comprehensive global lab services during all stages of the development process. Focused on both the scientific and service aspects with wholly-owned laboratories in the US, Europe, China and Singapore, our central lab has the reach to support global studies, assist with regulatory requirements, and deliver custom solutions for any need.

CORE IMAGING

High-quality image acquisition and interpretation is crucial for the success of trials that rely on neuroimaging for patient selection or as a primary endpoint. Medpace provides centralized neuroimaging expertise, led by radiologists, neurologists and neuroscientists with decades of clinical and trial experience. Medpace Imaging Core Lab provides a suite of imaging services to enhance and expedite biopharmaceutical and imaging contrast agent development, including a broad spectrum of imaging biomarkers for rare neuromuscular disease trials.

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a scientifically-driven, global, full-service clinical contract research organization (CRO) providing Phase I-IV clinical development services to the biotechnology, pharmaceutical and medical device industries. Medpace's mission is to accelerate the global development of safe and effective medical therapeutics through its high-science and disciplined operating approach that leverages local regulatory and deep therapeutic expertise across all major areas including oncology, cardiology, metabolic disease, endocrinology, central nervous system and anti-viral and anti-infective.

MAKING THE COMPLEX SEAMLESS®