MEDPACE

DUCHENNE MUSCULAR DYSTROPHY (DMD) STUDIES

Experts. Experience. Execution.

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Medpace Strategy for Duchenne Muscular Dystrophy (DMD) Studies

Medpace specialty teams have experience in rare diseases, and pediatric studies, and in particular DMD.

The term dystrophinopathy is commonly used to describe a group of allelic disorders that share an underlying abnormality in the production or structure of dystrophin, a critical protein for striated muscle function. Within the spectrum of dystrophinopathy, DMD represents a typical prototype, and the most common clinical phenotype, affecting about 1 in 4,000 male births. DMD is characterized by progressive proximal muscle weakness that starts in early childhood and continues relentlessly through early teenage years, causing severe disability and progressive cardiomyopathy. While DMD is associated with the absence of any detectable dystrophin, a milder form, Becker muscular dystrophy (BMD), is associated with a reduced amount or abnormal structure of dystrophin. Multiple mutations can lead to dystrophinopathy with deletions accounting for the majority of DMD cases. Currently, there is no effective treatment for DMD and most later-stage investigational treatments are designed to treat specific mutations thereby only addressing a sub-population of patients affected by DMD.

Why choose Medpace for a DMD study?

- Key Clinical Operations experts will be conducting your study
- Pediatric specialists who have conducted studies in DMD a part of your team
- Leveraging relationships with Patient Advocacy groups in DMD to expedite patient recruiting
- Deep regulatory expertise in pediatric studies on a global basis
- Leading partner on 7 DMD studies phases 1-3 in 28 countries

Relationships with Patient Advocacy Groups

Rare pediatric studies can be quite challenging from a recruitment standpoint. However, Medpace understands that patients and families facing the life-threatening condition of DMD are very motivated to participate in research studies. It is expected that Investigators will know their DMD patients very well, and that they will be able to rather quickly determine potentially eligible patients at their center. Medpace is experienced in driving patient referrals from nonparticipating centers to further bolster enrollment. The DMD community is greatly supported by a network of parent advocacy organizations, which have a keen interest in DMD clinical trials. Connecting with these organizations continues to help to facilitate visibility of the studies and generate interest in both Investigators and families.

Medpace has collaborated with many local and global advocacy groups including Jett Foundation, TREAT-NMD, and Parent Project Muscular Dystrophy (PPMD) to facilitate and support patient recruitment for these difficult to enroll studies. Medpace has also utilized data from advocacy groups and global as well as local registries to enhance enrollment strategies. Medpace has supported patients traveling from at least 10 nonparticipating countries to participating centers to ensure patients are permitted equal access to trial participation no matter where they are from.

Deep regulatory expertise for Pediatric Studies

- Medpace has numerous board-certified pediatricians on staff who are trained in clinical pediatrics and have extensive clinical experience treating sick children as well as designing, conducting, and managing pediatric trials
- Experienced in understanding the unique concerns raised by ethics committees, investigators, and parents, and in developing strategies to address specialized needs
- Familiar with many unique DMD endpoints and best practices meet regulators expectations
- Ethics Committee reviews are often prolonged in pediatric trials; Medpace works closely with sites to lower the risk of lengthy negotiations, deficiencies and/or queries

DMD Imaging with Medpace

Medpace Imaging Core Lab (MICL) is led by experts with years of experience. The lab is a wholly-owned entity full-integrated with all Medpace systems, including our proprietary CTMS, ClinTrak®. MICL has global imaging operations, including site management in the USA, France, and China. MICL maximizes the consistency of image acquisition across sites by ensuring quality training and site qualification. MICL offers DMD imaging capabilities such as MR Spectroscopy, T2 Mapping, Muscle Fat Fraction, PDFF – Complex, and MR Elastography.

Experience

Medpace specializes in the management of studies across a variety of therapeutic areas, including musculoskeletal indications, with nearly two decades of experience in conducting trials of orphan indications/ rare disease populations. Current experience includes 3 global Phase III Duchenne Muscular Dystrophy (DMD) trials, each enrolling more than 200 patients across the globe. Medpace's relevant experience also includes the management of multiple international pediatric studies across a variety of indications, including a number of studies for other orphan indications (Facioscapulohumeral Muscular Dystrophy, multiple sclerosis, Charcot Marie Tooth Disease, cystic fibrosis, eosinophilic esophagitis, homozygous familial hypercholesterolemia, and neuroblastoma).

Medpace recognizes the vulnerability and the unique challenges of special patient populations, as well as the ethical considerations unique to studies in children and adolescents. Medpace is well-versed in structuring DMD trials to ensure the best experience possible for patients and their families.

About Medpace

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. Headquartered in Cincinnati, Ohio, Medpace employs approximately 2,500 people across 35 countries.