Rare diseases

Deliver transformative therapies to individuals living with rare neuromuscular diseases

Accelerate your clinical trials with our expert, patient-centered approach

United by a common goal of bringing independence and transformative therapies to those living with muscle disease, the experts at the PPD™ clinical research business of Thermo Fisher Scientific are at the forefront of clinical research and development. Drug developers partner with us to safely deliver treatments to rare neuromuscular disease patients, ethically and with speed. Our extensive experience and industry partnership mindset, patient-centric approach, and robust capabilities ensures challenges are overcome and trials succeed.

Expertise in action: Neuromuscular experience

As the industry leader in the implementation and execution of rare disease clinical trials, we have become many drug developers' contract research organization (CRO) of choice. Over the past five years, sponsors have repeatedly turned to us for our expertise in rare neuromuscular clinical trials across all phases.

- Amyotrophic lateral sclerosis (ALS)
- Duchenne muscular dystrophy (DMD)
- Facioscapulohumeral muscular dystrophy
- Limb-girdle muscular dystrophy
- Multifocal motor neuropathy
- Myasthenia gravis
- Myotonic dystrophy

The promise of cell and gene therapies

Cell and gene therapies (CGT) hold a lot of promise for treating rare neuromuscular diseases — though these complex treatments present additional development complications. Our dedicated Cell and Gene Therapy Institute enables drug developers to create and execute successful trial strategies for these innovative treatments, leveraging our extensive medical, regulatory and operational knowledge. We've supported more than 130 CGT trials in the past five years, including neuromuscular indications and rare disease populations.

In the past five years we have conducted:

- **65** neuromuscular studies
- **3,876** patients
- **1,092** sites
- **130+** cell and gene therapy studies
- **325+** pediatric studies
- **585+** rare disease studies

Every patient counts

Finding the right patients is key to any clinical trial's success, but recruiting and retaining patients is a common challenge, especially in rare neuromuscular clinical trials. Sponsors must navigate diagnostic heterogeneity and geographically dispersed patient populations, all while taking steps to reduce patient and caregiver burden.

We stop at nothing to provide patient-centric care and services that make it easier for those affected by muscular dystrophy to handle the intricacies of participating in neuromuscular clinical trials. Cross-border enrollment, flexible and remote study solutions, logistical support, and patient concierge services are just some of the options we offer that lessen the demands on patients — and ultimately improve enrollment and retention.

Rare disease and pediatric center of excellence

With many muscular dystrophies affecting children, it's critical to partner with a CRO that has expertise in pediatric clinical trials. Our Rare Disease and Pediatric Center of Excellence — staffed by 30 experienced pediatricians — has conducted over 325 pediatric studies and over 585 rare disease studies, providing seamless collaboration and expertise.

Site feasibility

The rare nature of many neuromuscular diseases means it's not uncommon for trials to enroll only one patient per site. Our global experience informs our proven feasibility practices and engagement strategies for these hard-to-recruit rare disease studies. When selecting sites, we consider individualized site capabilities and resources to ensure needs of your specific study are met and outcomes can be achieved on your desired timeline.

Our expertise extends beyond just selecting sites. When qualified, experienced sites aren't available, we leverage our SiteCoach training program to equip naïve sites in new regions with the capabilities needed to support rare neuromuscular studies. This not only expands the site network, but also provides the opportunity to find additional patients that may not sit at experienced sites.

Because of our global footprint, sponsors also benefit from our deep understanding of the regulatory landscape in different countries. Our cross-functional regulatory experts enable you to navigate evolving global regulatory environments, accelerate outcomes and increase the probability of approval success.

The expertise to advance your study

Together, we are driven by a sense of urgency to advance treatments for neuromuscular diseases. Accelerate your program by tapping into our demonstrated expertise in effectively and efficiently navigating the complex development journey for rare neuromuscular diseases.

Learn more at **ppd.com**