

CASE STUDY

Successfully delivering an ultra-rare disease clinical trial in a competitive landscape



As an inherited, ultra-rare disease, limb-girdle muscular dystrophy type 2I (LGMD2I) is a common form of limb-girdle muscular dystrophy (LGMD), which causes weakness and loss of muscle mass, among other symptoms.

As part of delivering a sponsor's Phase III study of an LGMD2I treatment, Fortrea was tasked with recruiting 93 people living with LGMD2I in a highly competitive clinical trial environment. Learn how the team demonstrated flexibility, addressed numerous challenges and served as a true partner to the sponsor.

Recognizing the challenges

Supporting this trial required addressing the inherent challenges with rare disease studies along with recognizing the unique complexities presented by this specific trial.

- **A competing trial:** Another sponsor was simultaneously recruiting for LGMD2I patients, increasing the demand for a limited pool of qualified patients and experienced sites in an already difficult-to-recruit space
- **An unfamiliar area for the sponsor:** While the sponsor was skilled in other therapeutic areas, they were new to the neuromuscular space and relied on an experienced partner for strategic guidance
- **Unexpected protocol delays:** Feedback from the U.S. FDA and the Clinical Trials Information System (CTIS), along with multiple study design modifications, posed significant risks to the trial's timeline

KEY HIGHLIGHTS

Delivered timely recruitment: Recruited 93 patients across 8 countries and 21 sites in a highly competitive landscape that included another LGMD2I trial.

Developed a productive partnership with early engagement: Fortrea established strong connections with the sponsor and sites to deliver tailored training and rapidly solve issues.

Achieved timely results: Interim data analysis helped the sponsor meet its investment milestones, while the final data allowed the sponsor to file earlier than planned.



Establishing early engagement

At the start of the engagement, Fortrea's Diseases, Advanced Therapies and Pediatrics Team (RAPT) prioritized understanding the sponsor's overall culture and evaluated which areas of clinical operations needed the most support. They then carefully selected a team to deliver the right fit for the sponsor and connected the sponsor with Fortrea's Patient Recruitment and Engagement team to share their expertise.

While establishing a foundational relationship with the sponsor, Fortrea also took proactive steps to address site-related challenges. The sponsor was delayed in site selection, so Fortrea worked to find appropriate sites, creating a balance of key opinion leaders (KOLs) and investigators. As sites were enlisted, the team worked to nurture site relationships and ensured the sites had appropriate training and felt heard throughout the study. Fortrea also partnered with the sponsor and advocacy groups to develop patient materials and evaluate patient-centric practices for the trial.

Promoting positivity while facing adversity

Anticipating inevitable obstacles, Fortrea helped set the tone for a productive working environment, emphasizing a shared commitment among the sponsor, sites and Fortrea team. This spirit of partnership was demonstrated through several impactful initiatives, such as:

- **Providing tailored education** to help the sponsor and naïve sites better understand the therapeutic area and ensure uniformity across CRAs and vendors
- **Delivering timely communications** with a calm, positive tone to promote problem-solving and teamwork rather than assigning blame
- **Implementing course corrections** and mitigating issues as soon as they arose
- **Meeting investment milestones** for the sponsor by performing rapid analysis of interim data
- **Celebrating achievements** by creating a "kudos corner" for team members to recognize each other

Delivering results

Despite the challenges, Fortrea recruited **93 qualified participants across 8 countries and 21 sites**, exceeding the sponsor's expectations. Interim review of the data helped empower decision-making and met the sponsor's investment milestones. Finally, with the completion of the study, the sponsor could file earlier than planned, bringing the hope of a potential treatment to patients in need.

Reflecting on the value of a strong partnership

As a result of the flexibility and dedication demonstrated by the team, the sponsor evaluated Fortrea with a “10” in the Net Promoter Score (NPS) and provided a glowing testimonial of the teams’ joint efforts:



“This has been a herculean effort by every single person on this team to get to this milestone; thinking back on the last 18 months, every step of the way, there were challenges, delays and obstacles. This team pulled together and DELIVERED with our participant community at the forefront of each decision. We are beyond proud to have Fortrea as our partner in this project. Thank you for all your hard work and dedication to LGMD.”

Learn more about our innovative approaches to advance [rare disease studies](#).



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