CASE STUDY

Driving a biotech's early phase development in a competitive rare disease clinical trial landscape

A KEY QUESTION

How can a proactive approach and early engagement help accelerate recruitment in a rare disease study?

KEYWORDS Rare Disease, Early Phase Clinical Trials, Site Strategy, Patient Recruitment, Biotech

A biotech sponsor engaged Fortrea to support its Phase Ib, open-label, dose-escalation study evaluating an immunomodulating agent in patients with relapsed or refractory active chronic graft-versus-host disease (cGVHD) following allogeneic hematopoietic stem cell transplantation.

Patients with cGVHD face a multifaceted burden disease, including physical, functional and psychosocial deficits, which negatively influence quality of life. For patients with high-risk disease who fail corticosteroids, five-year survival is approximately 30–40%.

Developing therapies for cGVHD presents inherent complexities, but this rare disease study faced additional challenges. Learn how Fortrea applied a proactive strategy to address recruitment challenges despite the rarity of the indication and competing studies.

Key highlights

Operating within a biotech-dedicated delivery culture, Fortrea applied a site-centric approach and enabled early engagement with scientific leadership. Through this approach, the study:

- Exceeded recruitment expectations
- Reduced the enrollment timeline by 50%
- Developed effective collaboration between the sponsor and the participating sites
- Increased the sponsor's visibility in the EU in a competitive recruitment landscape
- Established a solid foundation for Phase II development

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Understanding the challenges

Limited patient population: The eligible population was extremely limited, with only a small subset of patients progressing after prior treatments. In one EU country alone, only 160–265 patients per year were eligible.

Highly competitive landscape: Multiple trials were targeting the same sites and investigators.

Reduced footprint introduced a new risk: An initial dual-country strategy had to be reduced to a single country in the EU after low U.S. site engagement, partly due to the approval of another cGVHD treatment.

Limited sponsor visibility: The biotech sponsor had limited visibility in the EU.

Creating a proactive strategy

Recognizing that these factors threatened to delay or under-enroll the study, Fortrea took a proactive approach. Rather than expanding geographically, the team deliberately chose to apply its efforts at the site level. The approach centered on three principles designed for rare disease execution:

Focusing on a small number of high-potential sites: Fortrea targeted hematopoietic stem cell transplantation (HSCT) centers with direct access to cGVHD patients.

Demonstrating credibility and leadership: Fortrea strengthened the study's clinical legitimacy and sparked interest in the study by appointing an experienced investigator and worldwide KOL in cGVHD as the coordinating principal investigator, with support from a Fortrea study physician, an EU board-certified onco-hematologist.

Enhancing visibility before recruitment: Fortrea conducted in-person co-visits with the sponsor at every site before recruitment to increase the sponsor's visibility, present the study's scientific value and address any concerns about the protocol.

Taking action to enhance transparency

Fortrea's strategy came to life through highly coordinated, hands-on engagement:

Providing consistent communication: Fortrea openly shared enrollment progress, including a centralized waiting list, to provide transparency and maintain recruitment momentum.

Proactively preparing for Phase II and strengthening commitment: Fortrea held an investigator meeting aligned with a major European hematology congress to encourage participation and long-term study commitment. This also helped strengthen the program's scientific positioning and supported readiness for Phase II development.

Reviewing the results

Fortrea's targeted, site-centric approach and early engagement with scientific leadership enhanced study visibility and investigator engagement, helping stabilize a high-risk study into a high-performing one.

By supporting Phase Ib recruitment and strengthening investigator relationships, Fortrea helped position the biotech sponsor to enter Phase II with momentum and a well-established execution model, informed by experience in a competitive rare disease landscape.

Results at a glance

- **Recruitment exceeded expectations,** despite the rarity of the indication and competing studies
- **Enrollment timelines were reduced by 50% relative to initial projections,** from 27 months to approximately 13.5 months
- **Strong Sponsor-site-Fortrea relationships were established,** driving long-term collaboration and promoting more predictability as development evolves

Discover how **Fortrea** supports Onco Hematology programs in competitive and complex study landscapes.

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