

# Accelerated submission in rare disease: A Fortrea FSP case study

## A KEY QUESTION



How did Fortrea's FSP model enable a small biotech company to meet accelerated FDA submission timelines for a rare disease therapy?

## KEYWORDS

FSP (Functional Service Provider), Accelerated Submission, Rare Disease, Regulatory Expertise



## Executive summary

A small U.S.-based biotech company that's focused on [rare disease treatment](#) needed to accelerate their Biologics License Application (BLA) submission timelines. With Fortrea's Functional Service Provider (FSP) experience added to their team, they successfully met complex regulatory demands, rapidly scaled global resources and completed their accelerated submission on schedule. The adaptability, high-quality deliverables and keen regulatory understanding delivered by Fortrea enabled the sponsor to meet the FDA's aggressive deadlines. This outcome strengthened their partnership with Fortrea significantly, opening the door for additional collaboration.

## Problem and background

**The sponsor faced a major hurdle:** The FDA had granted accelerated submission approval, shrinking their timeline to just six months. Limited internal submission experience, rolling data availability, shifting statistical analysis plans (SAPs), changing endpoints and repeated ad-hoc requests from regulatory authorities all combined to create an exceptionally complex hurdle. The biotech sponsor also had to manage multiple parallel studies to support their submission, which further amplified study challenges and pressure.

### Sponsor profile

- **Company type:** Small U.S.-based biotech
- **Therapeutic area:** Rare disease
- **Study phases:** Early Phase (I/II), natural history and integrated summary of efficacy (ISE) studies
- **Main goal:** Accelerated BLA submission approval

**Sponsor headwinds:** Following the FDA's grant of accelerated submission approval, the sponsor was confronted with a formidable challenge: significantly compressed timelines of just six months. With limited internal expertise in submission processes and sparse availability of natural disease data, the organization faced a confluence of complexities, including shifting clinical endpoints and frequent ad-hoc inquiries from regulatory authorities. These factors collectively created an exceptionally intricate submission landscape further compounding the situation, the sponsor was concurrently managing

multiple parallel studies to support the submission, thereby intensifying operational demands and strategic pressure.

**Fortrea considerations:** Fortrea encountered considerable challenges due to accelerated timelines, compounded by ongoing changes to statistical analysis plans (SAPs) and endpoints. These changes necessitated repeated reviews, CDISC version upgrades and coordination across multiple concurrent studies, further increasing operational complexity.

## Fortrea's solution

Fortrea stepped in and crafted a comprehensive, agile solution tailored to the sponsor's specific issues. The CRO leveraged their deep industry experience and signature operational flexibility to meet a variety of regulatory milestones.

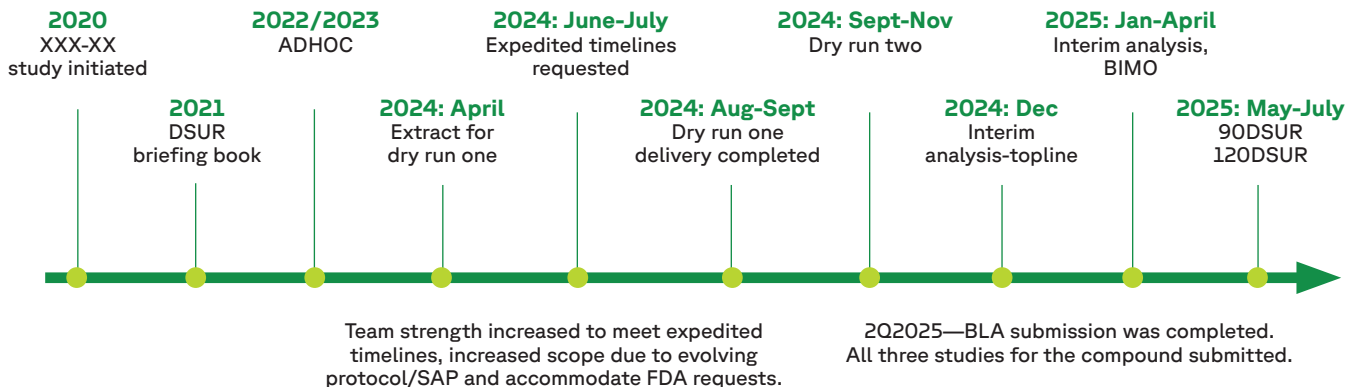
### Alignment and experienced guidance

Fortrea initiated the project with a detailed gap assessment and thorough timeline development. Drawing on their extensive submission experience, senior subject matter experts (SMEs) provided proactive guidance that empowered the sponsor to anticipate and swiftly address FDA queries/feedback and evolving submission needs.

### Flexible, scalable team resourcing

An experienced global team was swiftly assembled, with a combination of full-time equivalent (FTE) and unitized contracting models. Agile workflows, daily stand-up meetings and iterative deliverables ensured continuous, real-time fine-tuning of project requirements. The flexible resourcing model enabled dynamic team scaling, such that resources were rapidly increased or decreased precisely as the project demanded.

## Case study: XXX-XX\* accelerated BLA submission



\*Study participant identity redacted to maintain data privacy.

## Personalized collaboration and communication

Fortrea ensured continuous, open dialogue by providing direct sponsor access to the CRO's SMEs and senior leadership. Clear escalation pathways and weekly leadership calls delivered strategic reassurance, and detailed updates at regular intervals that kept the sponsor fully informed at every juncture.

## Results

Fortrea's adaptable approach and agile execution resulted in the biotech company successfully meeting their aggressive FDA timelines. The ability to quickly scale resources, maintain high-quality outputs and adapt to regulatory requirements was instrumental in the project's success. Specific achievements included:

- On-time completion of two critical dry runs (300+ outputs within four weeks; 400+ outputs in four to six weeks)
- Timely completion of interim analysis with 450+ high-quality outputs
- Submission completed without delays, meeting all FDA deadlines
- Zero major findings or rework on key deliverables
- Simultaneous delivery of sister studies with zero major findings
- eSUB packages for all studies were completed within two to four weeks

## Lessons learned and key takeaways

- **Agile workflows:** Real-time prioritization through daily stand-ups ensured continuous alignment
- **Crystal-clear communication:** Transparent and direct access to SMEs and senior leadership enabled quick decisions
- **Resource scalability:** The ability to rapidly adjust team size and composition enhanced project success and efficiency
- **Proactive risk management:** Identifying and mitigating risks early helped avoid delays and quality issues
- **Comprehensive regulatory insight:** Deep regulatory experience allowed for quick comprehension and integration of FDA feedback

## Sponsor feedback



"We greatly appreciate the professionalism and dedication your team has demonstrated in this huge effort. You consistently met all the agreed-upon timelines, ensuring the project stayed on track and progressed smoothly."

"I wanted to take a moment to sincerely thank you again for the outstanding work on the top-line results. The deliverable was of great quality!"

"Thank you for your outstanding work on the TFL delivery! Having the TFLs available one day ahead was extremely helpful, giving us extra room to identify issues—which you solved very quickly. This high-quality delivery puts us in a great position for CSR writing."



## Conclusion

**Fortrea FSP's** tailored clinical research capabilities enabled this biotech company to successfully achieve their accelerated BLA submission. With a combination of regulatory expertise, agile and scalable resources and exceptional attention to detail, Fortrea consistently delivered high-quality, compliant results.

**To explore how Fortrea can support your clinical or regulatory program's unique challenges, contact our FSP team today.**

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