

Collaborative delivery advances a rare metabolic disease program for an APAC biotech

A KEY QUESTION



How can global experience and local insights help build trust and transparency in a global rare disease study?

KEYWORDS

Metabolic Disease, Global Clinical Trials, Rare Disease, Regulatory Support, Pediatric Research, Regulatory Submission, Asia Pacific Biotech



A leading Asia Pacific (APAC) pharmaceutical innovator selected Fortrea to run its complex Phase II clinical program targeting an ultra-rare metabolic disease affecting newborns, children and adults.

The trial spanned five countries, included two dose levels and required navigation across diverse regulatory environments with evolving expectations. This case study shares how Fortrea addressed challenges by applying established scientific approaches, sharing regulatory know-how and seamlessly coordinating across stakeholders.

Key challenges and solutions

Addressing regulatory hesitancy around pediatric enrollment

Early in the trial, regulatory agencies expressed hesitation in approving pediatric enrollment, citing concerns around ethical and scientific justification. This constraint significantly limited enrollment rates and put pressure on timelines.

Fortrea solution

Fortrea facilitated a coordinated, evidence-driven approach by:

- Aligning with the sponsor's scientific rationale
- Working closely with investigators and patient advocacy groups
- Incorporating regulatory and operational experience from Fortrea

Result

Fortrea's unified strategy helped address agency concerns, ultimately enabling pediatric inclusion and improving enrollment momentum.

Navigating a challenging multi-country regulatory landscape

The study required submissions to different regulatory agencies, each with distinct requirements. The sponsor had limited experience outside of its own country and the program needed to adapt to evolving regulatory requirements and system migrations.

Fortrea solution

As a full-service CRO, Fortrea acted as the central point of coordination for regulatory and operational activities by:

- Educating the sponsor on the expectations and timelines for each global- and country-specific submission
- Assembling regulatory packages
- Managing 16 protocol versions and multiple protocol amendments
- Supporting ongoing IND maintenance
- Overseeing migration to new submission platforms

Result

Working under evolving expectations and demanding timelines, Fortrea helped the biotech navigate regulatory system transitions and support informed interactions with global regulatory agencies.

Managing operational complexity across stakeholders

The rare disease program involved multiple stakeholders across geographies, creating coordination challenges that could lead to misalignment, delays and inefficiencies.

Fortrea solution

Fortrea served as an extension of the biotech team, maintaining alignment across the global program by:

- Matching the sponsor's urgency and demonstrating determination to complete the study as quickly as possible
- Enabling consistent and transparent communication
- Proactively sharing updates and adjusting to evolving needs

Result

Through cross-functional coordination and streamlined communication and execution, Fortrea's centralized approach minimized friction and kept the study on track.

Maintaining alignment and developing trust in a global program

Advancing clinical research in a global rare disease study required careful planning and coordination in areas with limited regulatory and clinical precedence.

Fortrea solution

Fortrea supported the sponsor with an in-country delivery director, who managed the global scope of the study by:

- Providing overarching project monitoring to promote internal visibility
- Applying local leadership informed by an understanding of the sponsor's organizational structure and operating context
- Empowering faster, more effective decision-making

Result

Drawing on global clinical experience and local market knowledge, Fortrea's in-country delivery director built and maintained long-term trust throughout the program.

Embracing urgency with a collaborative, biotech-dedicated delivery culture

Fortrea supported the progression of the highly complex rare disease program through coordinated scientific, regulatory and operational engagement. The collaboration enabled the study to overcome regulatory barriers, maintain global momentum through evolving requirements and support delivery while maintaining quality to help advance the promising product and address unmet medical needs.

Learn how our dedicated team at Fortrea provides global clinical delivery through local insight.

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