

# Expediting regulatory submission: Supporting biotech milestones in infant gene therapy trials

## A KEY QUESTION



How can CROs help biotech sponsors overcome regulatory delays and meet critical financial milestones in high-stakes pediatric clinical trials?

## KEYWORDS

Pediatric Clinical Trials, Rare Disease, Gene Therapy, Regulatory Submission, Accelerating Process, Investor Milestone, Biotech



A biotech company selected Fortrea to support its Phase I/II rare disease clinical trial for a gene therapy in infants. To meet its financial commitments, the biotech needed to obtain regulatory approval in its first targeted country. This case study shares how Fortrea shortened timelines, expedited the preparation of country-specific regulatory packets, and helped the biotech achieve a key milestone for its investors.

## Taking ownership of a challenging situation

After facing a series of delays in finalizing its core documents, the biotech was behind schedule, pushing its regulatory submission timeline by three months.

Fortrea typically requires four weeks to prepare a country submission, including review and quality control (QC) processes. However, due to the unexpected delays, the biotech required a faster turnaround time to fulfill its commitments to its investors.

## KEY TAKEAWAYS

Fortrea understands the importance of helping biotechs meet key milestones for their investors. To achieve an aggressive deadline for regulatory approval in this pediatric clinical trial, Fortrea:

- Identified documents that could be prepared in parallel before the final protocol
- Prioritized review of “at risk” informed consent forms
- Secured commitments from teams to expedite the preparation of materials
- Promoted transparent communication and identified risks with weekly review meetings
- Trimmed the regulatory submission timeline from four weeks to 4.5 business days

To meet the revised timeline without compromising quality, Fortrea implemented a tailored, creative problem-solving approach:

- **Evaluating how to create more efficiencies:** Fortrea engaged its core and extended teams to review the four-week submission preparation period. The teams identified documents that could be drafted and collected in advance of the final protocol to trim the timeline
- **Creating new review agreements:** Collaborating with review leads and the site readiness team, Fortrea expedited the review of the master informed consent form (ICF) and the country-specific ICFs identified as “at risk”
- **Setting new targets for preparation:** Fortrea met with its startup team to emphasize the urgency for the regulatory submission and secured commitment to a shorter preparation period
- **Communicating progress and enabling transparency:** Fortrea held internal weekly reviews to discuss progress with its site readiness team members. The biotech and Fortrea also met weekly to share status updates and identify potential risks

## Accelerating processes while maintaining quality

Throughout this intense process, Fortrea maintained quality standards by filing SOP deviations and performing agreed-upon expedited QC processes. As a result of Fortrea's flexibility and strong collaboration with the biotech company, they achieved:

- Approval of the master ICF within seven business hours
- Approval of the country ICF within two business days after the master ICF
- Preparation and submission of the regulatory packet within 4.5 business days

## Anticipating complexity and promoting predictability

At Fortrea, we recognize the importance of meeting financial milestones for biotech investors. While each clinical trial often presents unique challenges, we work to provide more predictability in the process. Our team is equipped to navigate development complexities, anticipate future risks, and flexibly adjust our approach to meet your needs.

Learn how our dedicated team at Fortrea invests in your success and delivers strategic solutions: [fortrea.com/therapeutics/rare-diseases](https://fortrea.com/therapeutics/rare-diseases).