

Turning rapid study start-up into enrollment momentum

Coordinated planning across clinical operations, IP supply, and labs helped a large global rare cardiovascular outcomes trial reach critical start-up and enrollment milestones ahead of plan.

Development challenge

A mid-sized biotech focused on complex therapeutics for rare diseases was undertaking one of the largest global Phase III cardiovascular outcomes trials in transthyretin amyloidosis with cardiomyopathy (ATTR-CM), a rare, progressive disease in which abnormal protein deposits build up in the heart and impair its function. The study required start-up and enrollment across approximately 280 sites in 37 countries and 1,750 patients, with a footprint nearly twice the size of prior programs. Identifying and recruiting patients for rare diseases such as ATTR-CM can be challenging given the small eligible patient population, compounded in this case by the increasing availability of commercially available therapies.

These recruitment challenges, combined with the scale of the global site network, created significant pressure across regulatory start-up, site activation, and enrollment. Because the trial was event-driven, it meant that the study would continue until a predefined number of cardiovascular events occurred, therefore any delays in activating sites or recruiting patients could slow event accrual and extend development timelines.

Accelerating start-up through coordinated planning

To accelerate start-up and build enrollment momentum, Thermo Fisher Scientific coordinated regulatory, clinical supply, laboratories, and clinical operations through an integrated delivery model, with shared priorities and early visibility across feasibility, site selection, regulatory submissions, supply planning, and site activation.

Critical-path countries and sites were identified early, allowing regulatory submissions, contracting, and site-readiness activities to move forward in parallel. Established clinical trial agreement (CTA) templates, vendor relationships, prior program experience, and streamlined sponsor reviews helped reduce cycle times.

In the U.S., the first global site was part of Trialmed, Thermo Fisher's clinical research site network. Standard contract language and use of a central institutional review board (IRB) helped enable activation approximately three months after final protocol.

From faster site activation to enrollment ahead of plan

Building on that early start-up progress, the team prioritized high-enrolling sites and countries to accelerate recruitment and rapidly scaled the study to more than 125 active sites within the first nine months. Detailed pre-activation CRA calls helped prepare sites before formal site initiation visits, reducing redundancies and rework.

Experienced resources across multiple programs supported the pace and volume of activity, while coordinated governance enabled faster decisions and early risk identification. This combination of strategic site activation and disciplined execution helped the study outperform enrollment forecasts despite a limited patient population and increasingly competitive treatment environment.

Results

- **Final protocol to FSA:** 41 days faster than the industry benchmark
- **FSA:** 3 days ahead of plan
- First global site activated approximately 3 months after final protocol
- **First subject screened:** 6 days ahead of plan
- 50% enrollment achieved 8 months ahead of plan
- Monthly accrual rates consistently 3–5x above plan

Development outcome

Accelerated start-up translated into enrollment performance well ahead of plan. The first site was activated three days early and 41 days faster than the industry benchmark, while the first subject was screened six days ahead of plan. **The study reached 50% enrollment eight months early, with monthly accrual rates consistently 3–5x above plan.**

For the customer, this meant more sites recruiting sooner and greater patient exposure time, supporting earlier event accrual and the potential to shorten the overall study timeline. For patients, it meant earlier access to clinical trial participation in a rare, progressive disease with limited treatment options.

At a glance

Program focus	Global Phase III cardiovascular outcomes trial in ATTR amyloidosis with cardiomyopathy across 37 countries, approximately 280 sites, and 1,750 patients
Approach	Coordinated regulatory, clinical supply, laboratories, and clinical operations, with early identification of critical-path countries and sites and prioritization of high-enrolling locations
Operational parameters addressed	Feasibility, site selection, regulatory submissions, contracting, supply planning, site readiness, site activation, patient enrollment, and cross-functional coordination
Key technical outcome	First global site activated approximately 3 months after final protocol; the study scaled to more than 125 active sites within the first 9 months, creating a strong foundation for enrollment
Development impact	Final Protocol to FSA 41 days faster than industry benchmark; FSA 3 days ahead of plan; First Subject Screened 6 days ahead of plan; 50% enrollment 8 months early; monthly accrual rates consistently 3–5x above plan



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