CARDIOVASCULAR CASE STUDY

Exceeding challenging milestones through teamwork and innovation.

A small biopharmaceutical company, developing a novel therapeutic platform, engaged Fortrea to set up and conduct a global, Phase III, pivotal clinical trial on a promising treatment for a rare genetic cardiovascular disease. The Sponsor had pressing corporate milestones, the first of these involving entering the first patient (FPI) into the study within six months of award announcement. A major impediment was the fact that a study in this rare disease involving a rival molecule had already engaged premier sites.

Understanding the challenge

- Convincing the best sites of the mechanistic advantages of the molecule over the competition
- Identifying patients with this rare disease
- Finding sites that could be initiated rapidly
- Educating the Sponsor and Fortrea personnel regarding the unusual clinical manifestations of the disease and potential challenges
- Training noncardiology sites to perform cardiovascular examinations, imaging studies and study-specific exercise assessment
- Organizing multispecialty cooperation at sites to accomplish study goals
- Working with only a protocol synopsis

Study start-up exceeds expectations

Study kickoff occurred soon after award and the Sponsor team indicated that they did not work in a standard fashion and would not expect their partner to do so either. We would need to be flexible in our operational approach in working with the self-admitted operational quirks of a small company. This required thinking "outside of the box" to solve issues unique to this protocol. From the start, both Fortrea and the Sponsor agreed that teamwork, collegiality and innovative thinking were key to getting the job done.

Leveraging our expertise and flexibility in cardiovascular clinical trial performance and rapid site start-up, we proactively began identifying and contacting appropriate sites and developing trial infrastructure well before a protocol was available or as the contract was being finalized. The Fortrea physician, who was a published specialist and had academic contacts in this rare disease area, educated the Fortrea and Sponsor teams about disease manifestations, available treatment and associated challenges. Our goals were efficient site and patient recruitment, site setup and preparation of investigators and sites to fulfill study requirements. Identifying prospective subjects would eventually require genetically screening up to 2,000 patients to find approximately 200 subjects with the target genotype and phenotype, but it initially involved finding a single patient available at the right time.

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The first milestone was meeting a daunting deadline to enroll the first patient within six months of study award. This required recognizing the challenges to rapid site start-up in various global regions, understanding the disease epidemiology and patient demographics and contacting leading investigators, mainly academic cardiologists, and convincing them of the importance and medical scientific interest of the molecule and the trial. The Fortrea global site start-up team, in conjunction with the Sponsor and the Fortrea physician, analyzed various start-up scenarios and decided on two approaches to best effect FPI by the target timeline.

The US and the UK were deemed the countries most likely to have the facilities, patients and potential for rapid regulatory turnaround and expeditious start-up. Early site initiation visits, training and physician-to-physician discussions were pursued and unique approaches were followed in both countries. In the UK, this included contracting with a Phase I unit operating at a private hospital that did not require National Health Service (NHS) scientific review prior to Ethics Committee submission.

The site had never performed a clinical cardiology study before in sick patients. A hybrid approach was taken whereby the study site worked in partnership with personnel from an expert academic site. The Phase I site was trained by Fortrea in protocol requirements, including finding the proper venue (required a 30-meter corridor) and operationalizing a six-minute walk test, the primary metric in the trial. The site also was trained in a unique form of fat biopsy. Study personnel from the partner academic site traveled to oversee operations for the FPI. Advanced cardiac imaging and other ancillary medical examination facilities were set up at a second cooperating academic site. At the same time, we worked with multiple UK referral centers in this rare disease in order to identify a patient who could be enrolled as soon as the site was operationally ready.

Exceptional teamwork, flexibility, innovation and a relentless effort to make things work resulted in meeting the deadline for enrolling the first patient two weeks ahead of time, thereby exceeding expectations. In addition, a backup site was concomitantly established in the US, additional patients were identified and facilities and personnel were readied. Always seeking to improve efficiency, we achieved this goal while continuing to recruit sites and patients for the rest of the trial.

Factors for success in this trial included clinical and operational experience, as well as organizational knowledge, effective communication, flexibility and resourcefulness. When you need custom solutions delivered efficiently, trust in a partner with proven results.

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