Global Biopharmaceutical Leverages Medidata Study Design Optimization Service for Late Phase Transplant Rejection Study

The Challenge

A global mid-market biopharmaceutical company—focused on developing and delivering therapies for patients with severe and life-threatening diseases—was conducting a large confirmatory study of a rare disease where cells break down earlier than normal. The therapy looked to prevent delayed graft function (DGF) in recipients of transplants who are at increased risk of DGF. In order to streamline development of the treatment to help its patients recover quickly from transplant surgery, it was vital that the sponsor optimized its study design for better trial execution.

The Solution

The sponsor chose Medidata Study Design Optimization (SDO) Service to evaluate the operational soundness of its study design. The service combines Medidata expertise with a study design application that accesses the information in Medidata's proprietary PICAS® database. The sponsor was interested in benchmarking its design against similar industry studies, ensuring the design was less burdensome on the patient while making sure that the appropriate activities were planned to meet the study endpoints. Through a data-driven process supported by the Medidata Clinical Cloud®, Medidata identified a key opportunity for study optimization, reducing the number of follow-up physical exams. The complexity benchmark was analyzed along with primary drivers of complexity such as activity quantity. The study had relatively high complexity and activity quantity when compared with industry studies in the same indication group. The unique activity quantities as well as the total activity quantities were near the industry's high (75th percentile) benchmark. Each activity is benchmarked to identify activities that are candidates for review.

Through this process and after review discussions with the study team, a key opportunity was found to decrease the number of the sponsor's "Follow Visits with Physical Exam and Vitals" activity from 14 to as low as seven occurrences.

Medidata Study Design Optimization Service optimizes your study design for dramatically better operational execution. The service combines Medidata's design tools and expertise to:

- **Improve operational costs.** Optimizing study design now dramatically reduces costs later. Structured design with complete line of sight optimizes procedure frequency, minimizes costly protocol amendments and boosts resource efficiency.
- **Speed study execution.** Reducing subject burden helps enroll more patients sooner.
- **Slash complexity and compliance risk.** Optimized studies are simpler for investigators and patients to follow—reducing risk. Clear line-of-sight linkage from objectives to procedures lowers complexity and reduces investigator and subject deviation without compromising study objectives.

Business Impact

The reduction in procedure frequency from 14 to seven significantly reduced the burden on the patients, potentially enhancing enrollment, reducing the potential of patient dropout and easing patient compliance issues. This was done without affecting the scientific excellence of the trial. Operationally the sponsor achieved a direct site cost reduction of approximately $150 per subject or $60K per visit for a total of $420K for the study. Additional savings included the overhead cost of performing the activity to be paid to the sites, the collection and monitoring of the data, as well as the data cleansing and analysis efforts.

The study design also contained over 20 activities in the schedule of activities that were ambiguously linked to a study objective or endpoint. These activities required more explicit explanation of purpose and use, representing over $700 in direct site costs per subject, and nearly 20 percent of the study's overall complexity. Clarifying these activities early in the design process removes unnecessary cycles of review and revision time spent downstream in the process. In a worst case scenario, unaddressed issues with poorly linked activities may cause amendments or trigger more rigorous Institutional Review Board (IRB) processes.

The inclusion criteria defined a particular activity to be performed during screening which was omitted from the study schedule. Since the investigators had to read the inclusion criteria, assumptions were made that they would collect the data and know how to evaluate the subject. Operating on assumptions would have led to data with questionable quality and accuracy. This activity should have been made explicit and included in the schedule of activities. The recommendation to add a specific questionnaire to the study schedule for handling the evaluation avoided such a quality gap.

About Medidata

Medidata Solutions is the leading global provider of cloud-based solutions for clinical research in life sciences, transforming clinical development through its advanced applications and intelligent data analytics. The Medidata Clinical Cloud® brings new levels of productivity and quality to the clinical testing of promising medical treatments, from study design and planning through execution, management and reporting. We are committed to advancing the competitive and scientific goals of global customers, which include over 90% of the top 25 global pharmaceutical companies; innovative biotech, diagnostic and device firms; leading academic medical centers; and contract research organizations.

info@mdsol.com | mdsol.com
+1 866 515 6044