INCORPORATING PATIENT INSIGHTS INTO A RARE DISEASE CLINICAL PROTOCOL

CASE STUDY

Innovative Patient-Centric Approach Builds Patient Awareness and Strengthens Protocol

A biopharmaceutical specializing in rare and orphan disease asked Covance to run their global Phase III clinical trial in patients with Fabry disease – a rare, inherited disorder that affects multiple areas in the body and can cause life-threatening complications.

Engaging with patients and designing a patient-centric protocol in any rare disease clinical trial is a major challenge. To learn more about Fabry patients' experience with the disease and their potential concerns with joining a study, Covance designed a new strategy combining patient data from a globally deployed survey, as well as leveraging proprietary clinical data.

Key Takeaways

- Uncovered new insights on the patient experience with Fabry disease
- Gathered feedback from more than 300 Fabry patients to inform clinical design and create study awareness
- Incorporated a process to capture genetic results for downstream analysis and understand genotype/phenotype effects

Focusing on the Voice of the Patient

Implementing a patient-centric approach, Covance helped the sponsor create and deploy a user-friendly, web-based survey to more than 300 Fabry patients around the world. The survey design was also developed with input from many advocacy groups, such as the Fabry International Network, National Fabry Disease Foundation and Fabry Support & Information Group. These connections helped Covance and the sponsor gain insights on how to capture patient perspectives on clinical trial participation and better understand how patients are affected by the disease.

The invitation to participate in the survey was distributed via a network of global Fabry disease advocacy groups as well as treating physicians and investigators to cover multiple patient touch points. With these collaborations in place, Covance helped the sponsor directly launch the global online survey in 15 different languages.

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Shaping the Clinical Protocol with Real-World Patient Data

While evaluating the initial survey results, the team uncovered an entirely new perspective on Fabry patients' experience with the disease, with compelling findings that the sponsor plans to publish once the final analysis is complete. The survey data also revealed information about patients' willingness to suspend their current treatments and their concerns about potential side effects – critical factors that weigh into the decision to join a trial. These powerful data were leveraged to provide direct input on how to shape the clinical trial protocol, engage patients and structure ongoing communications.

At a deeper level in the clinical protocol, the team also considered the comorbidities of Fabry, as the disease can lead to severe cardiac, renal and central nervous system complications. They consulted with a company geneticist and developed a process that will accurately capture genetic results in the clinical database during the study. This foresight will optimize the sponsor's ability to analyze for genotype/phenotype effects at the end of the study, an important factor in treating Fabry disease.

Mining Proprietary Data to Improve Patient Recruitment

The second half of the patient-centric approach used advanced analytics to help the team enhance patient recruitment efforts. Analyzing de-identified health information from LabCorp's database of 70 million patients and results from 4,000 clinical assays, they filtered down on specific lab tests to identify the location of Fabry patient clusters based on their testing location. As a result, the team found 250 potential referring physicians for this rare disease.

The team also reviewed historical trial performance data and determined which sites and investigators would be most likely to keep trial timelines on track by mining data from Xcellerate® Trial Design, which gathers central laboratory metrics from more than 40% of the world's clinical trials. Based on this trove of proprietary information, more than 150 global investigators sites for Fabry were identified.

Creating Crucial Differentiators for Rare Disease Studies

Today's patients – especially those with rare diseases – have become empowered consumers and taken a more active role in their health care. Sponsors need to adjust to this new paradigm by incorporating the patient's voice in drug development, working with advocacy groups and fully leveraging critical input to engage patients and create informed clinical protocols.

By combining patient insights with data-driven approaches to pinpoint pools of potential patients, investigators and sites, Covance hopes this model will become a new standard for elevating the voice of the patient and ultimately improving recruitment and retention in these critical rare disease studies.

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