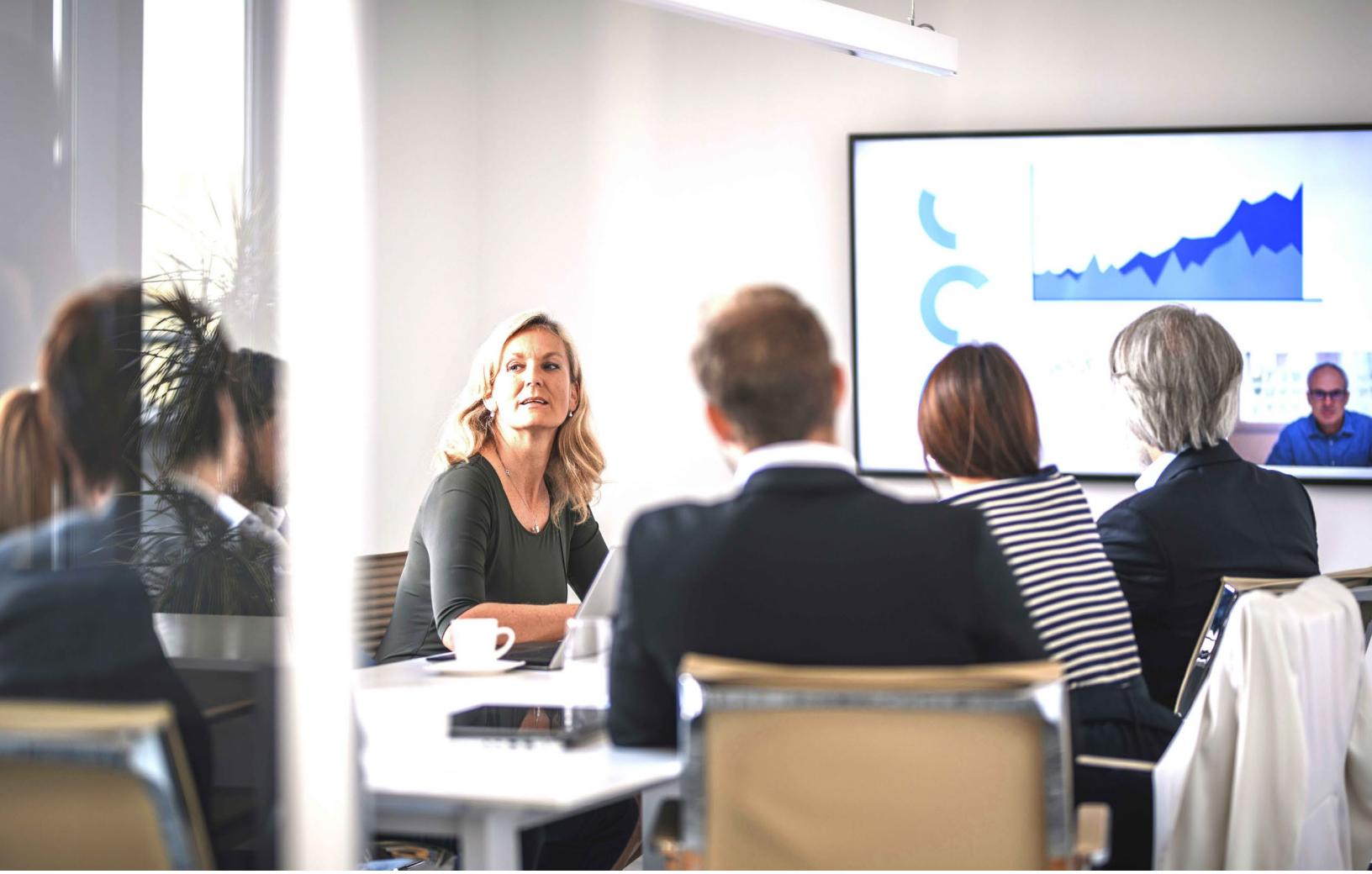
AmerisourceBergen
Xcenda

Addressing an evidentiary gap with real-world prospective studies

Xcenda uncovers beneficial long-term patient outcomes to support a new drug

Case study



The client situation

Upon receiving FDA approval of a new drug and launching the product into the market, pharmaceutical companies are often left with information gaps that need to be addressed in an effort to better understand the impact of their product in a real-world setting. That's where a pharmaceutical company who received accelerated FDA approval for a revolutionary new orphan drug recently found itself. With very limited efficacy data, they recognized a consequence of the expedited drug approval process was the absence of evidence showing the drug's longer-term outcomes for patients with the given disease state, resulting in downstream effects on physician prescribing behaviors, patient requests for the medication, and insurance carriers providing favorable coverage of the drug. They needed help, and needed it fast.

Understanding the urgency of the situation, Xcenda's Prospective Studies team was chosen as a partner and designed a prospective study to support clinical value of the treatment in a real-world setting. Data was collected from patients and their caregivers, who are a largely overlooked population. Results from the study provided vital information on long-term efficacy outcomes, participant disease burden and quality of life, and healthcare resource utilization over a 6-month duration, positively impacting the success of the product.

The Xcenda solution

Upon partnering with the client, Xcenda's Prospective Studies team collaborated on the study objectives, needed outcomes, timelines, and budget. With this information, the Prospective Studies' experienced group of research professionals created a customized solution that addressed the client's need for real-world, long-term outcomes data for the given disease state.

Given that the drug was already FDA approved and commercially available with no patient-centric data to leverage, Xcenda recommended a prospective study evaluating the impact of the drug on patient's quality of life (QoL), health outcomes, healthcare resource utilization (HRU), and burden of disease. A series of patient-reported outcomes (PROs) was administered electronically, via mail, or via telephone interview (as per the patient's and caregiver's preference) with the goal of building evidence to support the clinical value of the treatment in a real-world setting. In addition, Xcenda discussed the importance of gathering insights from a largely overlooked but important group—patient caregivers. By including caregivers as a second study cohort, Xcenda identified different sources of resource utilization and burden of disease information that proved relevant and valuable to the client's ultimate outcomes.

The teams agreed to conduct a non-interventional, US-based, prospective study in patients newly initiating the client's drug, while also gathering data from caregivers. The study would assess the clinical symptoms, disease burden, HRU, and QoL as reported from patients and caregivers for a 6-month duration.

Why Xcenda?

The Prospective Studies team at Xcenda excels in challenging research studies with novel designs, recruitment strategies, and data collection methods. Unlike traditional clinical trials, Prospective Studies at Xcenda are often unique in their design and objectives, requiring innovative and creative approaches to successfully execute the study while adhering to good clinical practices, FDA regulations, and international standards. Studies commonly conducted include: observational/non-interventional, interventional, pragmatic, burden of disease, chart reviews, and patient registries. Sources of patient recruitment are tailored specifically for each study to ensure expedited enrollment of the needed population while remaining focused on patient retention and quality of data. The Prospective Studies team has successfully conducted studies with virtual recruitment as well as recruitment from physicians' offices, healthcare systems, patient support programs, advocacy groups, pharmacists, and employers. In addition, data collection sources are designed for each study and often include PROs via validated instruments, surveys developed specifically for the study, patient interviews, provider interviews, medical charts, electronic medical records, and claims data collected in a variety of ways such as electronic data capture (EDC) systems, mobile applications, data transfers, or via direct patient contact.

With extensive clinical trial experience in all phases of research, the Prospective Studies team recognizes the importance of creating partnerships with each client and dedicating a core team for project collaboration, understanding the client's needs and providing quality deliverables within timelines and budget. Clients leveraging the Prospective Studies team gain access to full-service clinical trial expertise with a proven track record of success in all phases of research from study concept through publication.

The service package

Xcenda's dedicated core team of Prospective Studies researchers worked directly with the client from concept through publication. This continuity and consistency provided the client with a personalized approach and allowed for efficiencies between Xcenda and the client resulting in a successful research study.

Study design, development, and implementation

Xcenda crafted a customized protocol, an informed consent form, and case report forms for the study in collaboration with the client. In addition, Xcenda created study-specific surveys to be administered to the patients and caregivers along with validated PROs. To ensure the validity of and to assess patient burden for completion of these surveys, Xcenda conducted patient interviews leveraging a panel of patients diagnosed with the disease prior to implementing the surveys in the study. In parallel, Xcenda partnered with their sister company, Lash Group, to utilize a patient support program from which patients would be recruited, and implemented processes for remote enrollment with verbal consent. Leveraging the patient support program for recruitment was essential due to the rarity of the disease requiring treatment with the client's drug, resulting in a limited population from which to recruit. The patient support program allowed ease of identification of patients who were newly prescribed the client's drug and resulted in expedited recruitment. Upon finalizing the essential study content, Xcenda submitted all necessary documents to an institutional review board (IRB) for review and approval. They then built a custom intuitive, cloud-based EDC system with auto email capabilities for PRO administration to patients and caregivers.

Study conduct

Upon implementing the study, Xcenda continued to work with the patient support program to identify potentially eligible patients who were interested in participating in the study. Xcenda contacted patients expressing interest via implemented IRB-approved processes and obtained and documented verbal consent. In an effort to remain flexible to the patient's and caregiver's needs, Xcenda administered the PROs via one of three methods: electronically, postal mail, or telephone interview. Xcenda worked with each patient and caregiver over the course of their 6-month study duration to collect the study data and ensure the data quality. In addition, Xcenda contacted patients via an IRB-approved process as needed to increase patient retention and improve the quantity of data for the analysis. All data was centralized within the EDC system, allowing for real-time monitoring and data cleaning, thus expediting the database lock and study analysis.

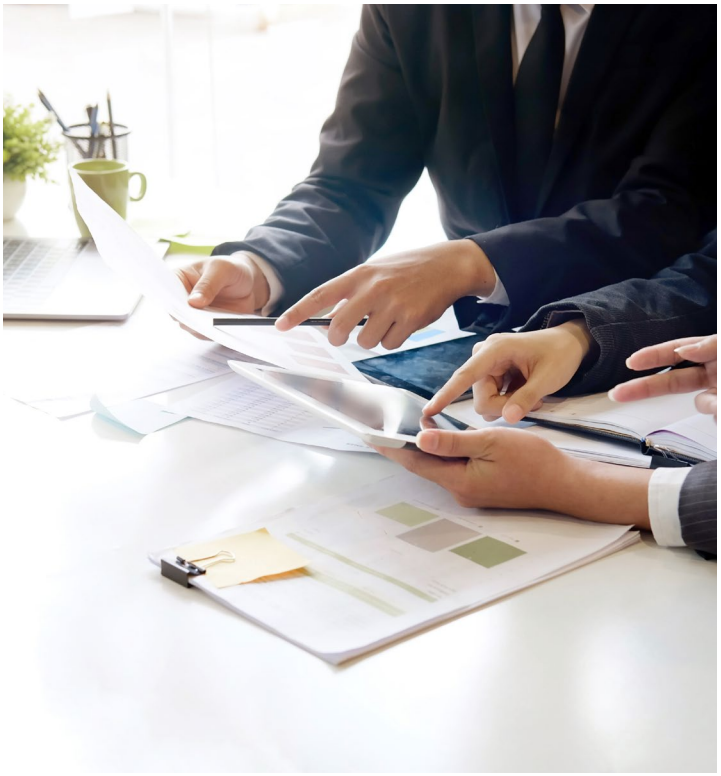
Throughout the duration of the study, Xcenda maintained IRB approval for the study, maintained the electronic trial master file (eTMF), administered patient honoraria, and provided study status reports to the client.

Data analysis

Two weeks following the entry of data for the last patient's survey completion time point, the study database was locked. Analyses were available to the client within 4 weeks following database lock, providing the client with an abundance of favorable data, supporting long-term positive outcomes of their drug. Xcenda provided the Clinical Study Report to the client as per their standard cadence within 4 weeks after the finalization of the study analysis.

The outcome

As a result of this successful study, the client has evidentiary data showing their drug's longer-term outcomes for patients with the given disease state, giving rise to numerous accepted abstracts, posters, and manuscripts. In addition, the data from this study has been presented globally at various congresses, increasing awareness of the disease and the client's FDA-approved drug. Xcenda continues to collaborate with the client on publications even after the close of the study, and leads writing initiatives as needed per the client's request.



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