Clinical Development Challenge

Use Of RWA To Understand Natural History

Background

A biotechnology company, focused on precision medicine received an FDA request to provide a natural history of the rare, oncology indication for which they were developing a new therapy. With limited information available about the disease, and a small patient population, the client needed to leverage real-world data to generate evidence that could satisfy the regulatory request. However, as a small organization without an in-house real-world evidence team, they did not have adequate resources, connections, or the expertise available to conduct this natural history study. With extensive experience in this domain, Cytel proposed to function as the virtual real-world evidence (RWE) department for the client. A two-step process was established to deliver more information that would inform regulatory decision-making and help the client get its therapy approved more quickly and cost-effectively.

Solution

The client is conducting phase 2 trials for a therapy focused on a rare oncology disease that affects approximately 8,000 to 10,000 individuals a year in the United States. Given the seriousness of the condition and its poor survival prognosis, the biotech company planned on conducting a study using real-world data (RWD). The objective of the study was to increase understanding of the natural history of patients with the disease. Cytel is helping the client to clearly define the scientific question, identify the right statistical solution, and deliver the analysis based on the acquired real-world data. An innovative solution was offered to the sponsor to find a comparator arm to their single-arm Phase 2 trial. This solution would facilitate timely approval to move forward with Phase 3.

Cytel

Cytel Clinical Services Case Study

Cytel Solution

Step 1: Data Feasibility

Data Asset Identification

The selection of the right data sets to inform on a real-world data research question is essential to ensure accurate analysis and the ultimate success of the study. Cytel has researched and cataloged the leading data resources available in the market and established vendor partnerships. This streamlines the process of identifying and selecting the best data sets. Cytel helped the sponsor through the process of data source selection with a constant focus on the quality of data and detail needed for the sponsor's research question

Data Aggregation & Engineering

Once the data sources were identified, a relational database was created to combine the data obtained from these multiple vendor sources with the client-identified data. This capability helps bring data together that might not have been accessible together before. For example, genomic data is usually separated from electronic medical records and claims data. Bringing the data together opens up the opportunity for interesting research insights. The datasets were cleaned and standardized by Cytel's data management and programming teams to make them ready for analysis

Step 2: Data Analysis

The objective of the data analysis was to evaluate overall survival (OS) and progression-free survival for a specific subgroup of patients. Existing RWD is being used to obtain information for patients who received 2nd line or later chemotherapy for the disease. After Cytel helped to identify the data, Cytel also supported the client with the following statistical analysis:

1. Survival Analysis & Modeling

2. Propensity Scoring for the Control Arm

Alternative approaches considered included pragmatic trials and other advanced analytical methods.

Access & Transformation Of Real-World Data

Navigating the field of real-world data can be complex. Cytel simplifies the process by ensuring that we ask the right research questions with the right data for the right analysis. The output from this project will allow the client to present an appropriate response to the FDA's question and smooth their path to accelerated approval.

About Cytel

As a pioneer in evidence generation, with deep expertise in advanced analytical solutions, Cytel is uniquely equipped to unlock the value from increasingly complex data. Life sciences companies count on Cytel to deliver exceptional insight, minimize trial risk, and accelerate the development of promising new medicines that improve human life. Cytel provides data-focused clinical research services and software solutions for the design and analysis of clinical trials, including industry standards East®, StatXact®, and LogXact®. With operations across North America, Europe, and India, Cytel employs 900 professionals, with strong talent in biostatistics, programming, data science, and data management. For more information about Cytel, visit http://www.cytel.com.