Fulfilling the Promise: Real-World Data and Real-World Evidence in Action

3 MIN READ

Real-world data (RWD) *and* ***real-world evidence (RWE)*** *have become more prominent in the health care landscape, but questions remain about how to use them effectively in health care decision making.*

By providing insights about outcomes associated with pharmaceuticals and devices in clinical practice, RWD and RWE can complement clinical trial findings, help to fill critical knowledge gaps, and support both product launches by manufacturers and decision making by payers and regulatory bodies.

By their nature, however, RWD are collected from disparate sources in a variety of settings, often lacking the standards and controls available in clinical trials. This can present unique challenges when it comes to isolating treatment effects and ensuring data validity.

Analysis Group has deep expertise in handling, analyzing, and generating RWD and RWE to ensure that the data are high quality and fit for purpose, and tell a coherent story about a product's effectiveness and safety.

Here are three examples of our work in this area.

RWE in Regulatory Submissions – Early Engagement, Bias Minimization

Key question: What can pharmaceutical companies do to improve the chances that the US Food and Drug Administration (FDA) will accept RWE?

The 21st Century Cures Act called for the use of RWE in regulatory decision making. The FDA created a framework for the potential use of RWD to generate RWE to complement clinical trial data as a way to accelerate the pace of approval of new therapies or new indications. Yet the FDA did not provide clear guidelines for designing and executing adequate RWE studies for successful submissions.

Working in collaboration with Pfizer Oncology, Analysis Group researchers set out to address this knowledge gap by undertaking a thorough review of successful oncology product submissions to the FDA between 2015 and 2020 that included RWE. They analyzed what RWE was submitted, whether it was accepted, and why. The study revealed a key theme:

Rigor is critical

RWD are held to the quasi-clinical trial standard. The following guidance can increase the likelihood of RWE's acceptance.

Engage early

Engage the FDA early in the process to verify the strength of the data sources with regulators, and also confirm whether the RWE will be used for contextualization of clinical trial results (e.g., a natural history study) or as an external control arm study for comparison with the clinical trial.

***Click here to learn more about the study**, or contact **Mei Sheng Duh** or **Maral DerSarkissian**.*

Data Integration – Connecting the Dots

Key question: How can RWD from different data sources be integrated to enhance quality and optimize their value for clinical research and market access?

Different types of RWD focus on different aspects of patient care in real-world settings, resulting in fragmentary data with compromised research value. Working with organizations researching blood disease in China – where high-quality evidence to support the placement of a treatment on the National Reimbursement Drug List (NRDL) is key to its success – an Analysis Group team established a data-driven disease model to close the gap.

The model connects different data sources and uses artificial intelligence to obtain a complete picture of the patient journey. It includes a progression algorithm capable of reconstructing lines of therapy, multi-agent treatment regimens, and treatment responses – key elements in understanding real-world treatment patterns and effectiveness.

Click here to learn more about the study, *or contact* ***Eric Wu*** *or* ***Jia Zhong***.

Data Access – Leveraging Patient Support Programs to Generate RWD

Key question: How can biopharmaceutical companies speed up the process of collecting, compiling, and employing patient-reported RWD?

Collecting and synthesizing RWD from multiple third-party sources is a complex and time-consuming process, especially when stakeholders (e.g., regulators, payers, clinicians) are interested in patient experiences through patient-reported outcomes about diseases and associated therapies. Analysis Group has designed an innovative methodology, *L*ongitudinal Surv*e*ys of P*a*tients with Recruitment Through Patient Support *P*rograms (LEAP), which leverages pharmaceutical companies' patient support programs (PSPs) for particular therapies. This has proven to be a breakthrough process for generating early and timely RWD directly from a clinical practice setting.

A faster track to RWD

As part of a post-marketing study of the effectiveness of dupilumab, an FDA-approved treatment for atopic dermatitis, a pharmaceutical company set up a support group for participating patients.

Recruitment

Patients enrolled in the PSP were recruited to "opt in" to Analysis Group's study, which shortened the recruitment timeline and made it possible to establish a true baseline for each patient before they started treatment.

***Click here to learn more about the study**, or contact **Min Yang**.*