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GOOD MANAGEMENT PRACTICE

## **Real-World Evidence: Bridging the Gap Between Clinical and Commercial Development**

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In the highly competitive drug discovery landscape, real-world evidence (RWE) plays an increasingly vital role in commercialization strategies. In fact, the best clinical trial designs deploy RWE to work in tandem with every stage of the product life cycle to ensure ongoing documentation of product value. This way, sponsors are prepared to provide timely answers to key real-world questions.

The reality is that novelty and efficacy, as demonstrated through interventional studies, are no longer adequate indicators of a product's market success. Today's sponsors must address varied information gaps related to RWE that can keep patients, physicians, payers, and policymakers from embracing and using a new therapy in the market.

Fortunately, rapid advancement of health information technology infrastructures and digital solutions in recent years have opened unprecedented access to real-world data. Advanced analytics capabilities enable aggregation and analysis of patient information from electronic health records, claims and billing platforms, product and disease registries, patient-generated data, and other digital sources to power the observational studies that produce RWE. Notably,

recent regulatory initiatives, such as the 21st Century Cures Act, promote use of these data as a driver for better clinical trial design.

### **The Benefits of RWE**

RWE can provide a diverse group of healthcare stakeholders with a deeper understanding of how new therapies work when applied to clinical practice environments. Studies that generate these data can answer questions such as:

- Does the drug or medical device work under actual practice conditions?
- Is it worth the price?
- How does it affect patients' quality of life?
- Is it safe in real-world medical practice?

Traditionally, the scientific community has not done a very good job of integrating interventional discovery with commercial effort. Use of RWE as part of an overarching strategy that documents and communicates the clinical, economic, and human value of a product helps bridge this gap, bringing together the best of interventional research with the best of observational research.

### **Evidence Strategy and Planning**

Like any worthwhile undertaking, research initiatives aimed at building a solid portfolio of evidence require thoughtful planning. Otherwise, the potential positive impact of new therapies can fall flat.

As a first step, sponsors should identify clinical and commercial development priorities and then use formal assessment processes for engaging patient registries and other nontraditional research initiatives to accelerate product acceptance and adoption. Ultimately, the aim is to collect RWE that establishes key value messages for a product that support the clinical trial outcomes data.

Many sponsors engage an experienced contract research organization (CRO) with a dedicated RWE team to develop this plan. Tactics can then be implemented to complement a traditional clinical trial, whether before, during, or after product approval and launch.

## **Health Economics and Outcomes Research**

Understanding the type of RWE that specific stakeholders require before they will accept a new product is critical to successful commercialization. This is accomplished through a health economics and outcomes research (HEOR) strategy that engages various industry groups early in the product development cycle. The goal is to identify key information gaps related to value expectations.

For instance, physicians, patients, payers, and regulators are all interested in how a product improves a patient's quality of life—as well as whether the product is safe in a real-world setting. Additionally, physicians may be interested in how a product works in the care delivery setting. Payers also will likely be interested in health economic data from burden-of-illness and cost-effectiveness studies.

Although these examples provide a glimpse of needed information, stakeholder demands will vary by region and condition, requiring guidance from local experts. An effective HEOR strategy informs what data are needed for an optimal product evidence portfolio and engages approaches such as:

- Economic and patient-reported outcome (PRO) endpoint design
- PRO instrument design and validation
- Economic/PRO literature reviews
- Economic modeling
- Cost-of-illness studies
- Cost-effectiveness analyses
- Retrospective studies
- Product dossier development/updates

## **Observational Research and Patient Registries**

Observational studies also have become an important means of measuring health outcomes, economic viability, the humanistic value of a product, and its safety post market. Supported by

patient registries, these studies collect data such as physician practice patterns and behavior, quality-of-life indicators, health status, resource utilization, and treatment satisfaction.

Like interventional studies, observational research requires a comprehensive approach to design and implementation that must consider such factors as study design and scope, protocol development, regulatory planning, patient enrollment and retention, and data/technology management.

### **Post-Approval Safety Studies**

Time to market is critical once a product receives approval from the U.S. Food and Drug Administration (FDA). Today, the FDA uses RWE to monitor post-market product safety and make key regulatory decisions.

Whether responding to a formal mandate or combining it with a discretionary research initiative, the business case for documenting product safety throughout the product life cycle is an easy one to make. Sponsors can work with a CRO to consider the scope of safety data needs, which can range from focused safety surveillance to broader measures that include clinical effectiveness, cost-effectiveness, and quality of life.

### **Bridging the Gap**

Interventional studies and observational studies complement each other and ensure RWE is available for product commercialization. Strategic clinical trial design guarantees credible RWE supports not only market approval, but also acceptance by key stakeholders.

Because sponsors often have limited resources for conducting clinical research, many turn to an experienced CRO that can deploy a multi-tiered effort. They understand the importance of defining a product's value, as well as supporting its messaging with RWE.

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