Introduction

Real-world evidence (RWE) has become an increasingly important component of drug development. Serving as a complement to conventional randomized clinical trials (RCTs), RWE collects supporting outcomes and safety data beyond RCTs that provide a more comprehensive view of a product’s real-life therapeutic and economic value to patients, payers, providers and sponsors. With the right data and analytics support, RWE informs a common understanding of a drug product’s efficacy and safety profile that is used by healthcare stakeholders to drive decisions.

This white paper explains RWE, the usage and benefits, and how it can be leveraged to better evaluate drug safety and effectiveness, as well as the advanced data analytics platforms necessary to integrate the vast amounts of electronic data now available from many disparate sources to gain a clear understanding of real-world outcomes.
What Is the Value of RWE?

Randomized controlled trials, the gold standard for proving safety and efficacy before launch, do not present a full picture of the effectiveness of a new treatment in a real-life environment. Increasingly, stakeholders are looking for information about the holistic patient journey and outcomes. By providing both real-life clinical practice and actual health outcomes of drug products, RWE can generate broader scientific evidence and commercial insights. Integrating RCTs and RWE-based approaches could achieve a reliable proof of concept and a good benefit-risk profile for new pharmaceuticals, drive reimbursement and ultimately impact provider actions and patient care.

Further, RWE studies can help inform questions about the likelihood of patient adherence, physicians prescribing the drug, insurers giving it favorable formulary placement, and of regulatory acceptance. Studying the use of a drug in a real-world setting can also provide insights into burden of disease, costs, optimal co-therapies, and medical best practices, which are not available from clinical trials. Payers use RWE to support decisions about drug reimbursement and use. Sponsors and other organizations also use the data, coupled with scientific, clinical and commercial expertise, to prove and support value throughout the product lifecycle.

RWD (real-world data) augmented with big data and advanced analytics technologies help deepen the understanding of diseases and treatments, identify risk issues earlier, better direct the design of new products, and generate evidence for product approval. The complementary data also help gain and defend market access, pricing and reimbursement.

Another analytics application utilizing RWD is comparative analysis, assessing a product’s performance compared to alternative treatment regimens and competitor products in the complex environment of comorbidities in varied and largely non-treatment naïve populations. Statistical meta analysis methods, dynamically driving visual displays, such as forest plots, risk-benefit graphs and time-to-event (Kaplan-Meier) plots support an exploratory approach to comparative outcomes analytics.

An example of how the data, brought together from multiple real-world sources, is being utilized to improve patient treatment is Sanofi’s recent collaboration with Duke University and Massachusetts General Hospital to create new tools to help predict how patients with type 2 diabetes adhere to their medications. The collaboration could transform chronic disease population management by analyzing how predictive analytics through big data might forecast medication adherence and result in more personalized patient engagement programs and better outcomes.

Growing Interest in RWE

The breadth and depth of RWE data is growing at an exponential rate and we are only beginning to realize its potential value. Physician utilization patterns, the patient treatment journey, and drug comparative effectiveness are just a small sampling of the use cases for which organizations across the clinical and commercial continuum want to leverage RWE today.
There is a general consensus that RWE offers $300 to $450 billion in top-down opportunity for U.S. healthcare alone. At the same time, many life science companies are seeing ad hoc value from selected RWE case studies, often demonstrating as much as $100 million of bottom-up impact. A company that captures $1 billion in RWE value ultimately creates improvements in the healthcare system valued in the tens of billions of dollars.

Many converging factors are changing pharma’s role in the evolving healthcare ecosystem, requiring new business models and practices. Companies are facing soaring R&D costs with decreased development productivity and more stringent regulations. Patients shift from the physician to a wide array of influencers requires new types of customer engagement. Healthcare reforms increasingly incentivize integrated, outcomes driven care delivery models. At the same time, the healthcare ecosystem is evolving toward a patient-centric care delivery model.

**Regulatory Position on RWE**

Organizations such as such as the National Institutes of Health (NIH) Collaboratory, National Patient-Centered Clinical Research Network (PCORNet) and the FDA’s Sentinel initiative are working to use RWE data to improve clinical trial efficiency and drug safety monitoring. In 2013, the European Medicines Agency (EMA) issued guidelines for RWE studies, requiring risk-benefit data in addition to post-authorization safety studies. The U.S. and other countries have been following the EMA guidelines.

At a recent public workshop on RWE, participants addressed the question of whether data gathered from healthcare systems can be used to supplement or support regulatory decisions, such as the approval of new indications or label expansions. While the FDA currently uses RWE mainly for postmarket safety surveillance, one attendee suggested the next step might be to use RWE to improve our understanding of what works and what doesn’t work in healthcare delivery and medical technology, and that RWE may offer new pathways to address regulatory requirements. Another said a large study comparing multiple doses of a drug over time could be conducted within the healthcare system to determine how long patients should take a drug, using electronic health records (EHRs) to track patient outcomes over time.

**RWE Fueling Interest in Drug Repurposing**

At the same workshop, Janet Woodcock, director of the Center for Drug Evaluation and Research (CDER), said that if RWE can be validated and studied in a randomized way, it could be used to support new indications or expanded labeling for existing therapies. She sees promise in using data on off-label drug use to inform labeling changes after a product is on the market, and suggested that in the future, maybe the second and third indication, you could perform a randomized controlled trial utilizing the tools of the healthcare system.

RWE is expanding opportunities and interest in drug repurposing, generating new value for an existing drug by targeting diseases other than those for which it was originally intended. Failed drugs can also be considered for repurposing. As a late life cycle management strategy, sponsors may look at additional target populations that may benefit from label extension. Drug repurposing offers a new source of revenue and has a number of R&D advantages, including a reduction of R&D timelines by up to three to five years, reduced development cost, and improved probability of success.

Repurposing can save time and money in developing new treatments, and the side effects of the original drug are already known. Two high-profile examples of drug repurposing are sildenafil and minoxidil. Both began as medications for cardiovascular issues. Eventually sildenafil was rebranded as Viagra and minoxidil spawned Rogaine. Another example in progress is ketamine, an anesthetic agent now being tested for tinnitus, major depression, and chronic pain.

**Integrating Vast Data Sources of RWE**

From real-world data, advanced analytics can extract meaningful patterns of information to help clinicians make appropriate treatment decisions. These analytics platforms can also be used to quickly access, analyze and deliver insights needed for other stakeholders as well. The technology must integrate and enable access to massive amounts of data from myriad sources, such as: the current standard of care, gaps in the care model, pragmatic or practical clinical trials, insurance payment claims, pharmacy prescriptions and bills, patient and product registries, electronic health records (EHRs), electronic medical records (EMRs), data from social media, laboratory test results, radiographic images, biobanks (specimens, tissues, etc.), molecular genomic data, vital statistics data, and patient-generated data. Patient interaction through social networking sites is a newer source of useful data on patients’ treatment experience and side effects.

To benefit from all the lab and real-life data available on a product, pharmaceutical companies need to adopt an integrated evidence development model that brings together RTCs and RWE-based approaches. Collecting clinical effectiveness data could help establish protocols based on evidence generated from use of the drug in the market. This would reduce cycle time by about five years and required R&D investment per product by about 60 percent.

**PerkinElmer’s Solution**

Accelerating the time from data to impactful insights is the primary focus of PerkinElmer’s RWE offering. Life science companies are spending more and more on RWE but struggling with data volumes, data integration, long-running analyses and visualization. Combining PerkinElmer’s expertise in the area of RWE with in-memory data bases such as Teradata®, Hadoop distributed file systems, or enterprise data lakes, combined with TIBCO Spotfire® technology, users are empowered to quickly discover insights through meaningful visualizations, interactively exploring outcomes data without requiring structural or semantic changes to that data. In addition, life science companies can dynamically aggregate, filter and access micro-level data details to produce near-real-time analytic visualizations that highlight where the data value can be found. PerkinElmer’s RWE solution leveraging TIBCO Spotfire® accelerates time to insight with pre-built analysis modules that allow for cohort building with propensity score matching, comparative effectiveness, safety signal detection methods, machine learning and more... all out of the box! The future roadmap of this solution includes aggressive steps towards helping life science companies quantify the patient journey.
Conclusion

Pharmaceutical companies are expanding their R&D and commercial business intelligence capabilities to include RWE analytics. Compared to RCTs, RWE more closely demonstrates how a product will perform in a broader, more representative population over a longer time, and provides information on comparators and outcomes that are not part of the clinical trial process. RWE complements traditional sources and holds the promise of more significantly bringing patient benefits, and serving as a foundation for new pricing strategies more closely linked to therapeutic value for patients, and health outcomes benefits to health systems and risk bearers.

PerkinElmer’s RWE solution will accelerate time to value in your RWE and observational data programs. Click here to request a demo today!

References


