How Real-World Data and Real-World Evidence are Transforming the Healthcare Industry

White Paper
Introduction

The use of real-world data (RWD) and real-world evidence (RWE) within healthcare systems is growing significantly as stakeholders face pressure to satisfy the needs of a changing industry. According to the US Food and Drug Administration (FDA), RWD is defined as “data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.” Such sources can include electronic medical records (EMRs), genomic studies, and even social media and wearable devices. RWE is then derived from the analysis of RWD, “providing clinical evidence about the usage and potential benefits or risks of a medical product.”[1]

Data is driving health care transformation, with prediction and prevention a central force. Health data is allowing doctors to build better patient profiles and predictive models to more effectively anticipate, diagnose, and treat disease. Technologies such as at-home testing services and telemedicine are empowering patients to be more engaged with and proactive about their own health. Meanwhile, the industry is grappling with the tension between encouraging data sharing to maximize the benefits of collaboration and maintaining patient privacy and trust. All of these developments are altering the role of physicians and their relationships with patients.

There has also been a shift to patient-centric care within the healthcare industry. This calls for a more nontraditional approach to collecting data in order to better serve patients and their needs as well as demonstrate the value of treatments. As such, stakeholders are investing heavily in resources to build their internal RWD capabilities.

A major benefit of RWD and RWE for providers and patients alike is the possibility of reducing the duration of time between identifying a health problem and developing a solution. There is a push for decentralized clinical trials, which would help move the prospective collection of data from outside the boundaries of traditional clinical research facilities. This approach would not only be more cost-effective but also give providers and patients access to important information much sooner than traditional postmarket studies.[2]

RWE in the Pharmaceutical Sector

The pharmaceutical sector is especially in need of advanced ways of retrieving data and serving patients. There has been a steady increase in the development of pharmaceuticals. For instance, over 200 new drugs are expected to come to market by 2020.[3] Federal funding for Medicare and Medicaid is providing consumers the ability to afford and purchase prescription drugs. Moreover, there is high demand as the US population continues to age.

There is also increasing pressure to prove the safety, efficacy, and effectiveness of drugs. RWE can play a crucial role in this demonstration, as it provides deep insight into the actual usage and performance of a drug in the real world across a variety of settings and circumstances that cannot be controlled for in a trial setting.

Compliance and Regulatory Landscape

The FDA recently launched its RWE Framework, as mandated by the 21st Century Cures Act passed in 2016, to help support new indications for already approved drugs or biologics and post approval studies. The program includes stakeholder engagement efforts, demonstration projects, leadership activities, and development of guidance documents to help developers interested in using RWD to support the FDA’s regulatory decisions. The FDA’s RWE Program will evaluate the potential use of RWE to support changes to labeling about drug product effectiveness, including adding or modifying an indication; adding a new population; or adding comparative effectiveness or safety information.[3]

When it comes to observational studies and RWD, the FDA has some concerns. The agency still sees randomized controlled trials as stronger evidence of drug effectiveness. However, despite its caution, the FDA says it will “consider reporting requirements for [observational studies] used to support effectiveness determinations.”[4]

Also of note, the Sentinel Initiative enhances the FDA’s ability to proactively monitor the safety of medical products after they have reached the market. Sentinel is being used to broaden access to RWD and RWE gathered from EHRs and other sources. When successful, this can reduce the need for post-market studies and lessen the amount of time to bring new devices and therapies to market.[4] The FDA has been actively developing data standards for
regulatory use and continues to expand its work in this area to help ensure the efficient review of RWD. This includes identifying the relevant standards and methodologies to maximize the utility of RWD. Regarding electronic source data for RWE, the agency noted several key regulatory compliance issues, including informed consent, validation of electronic systems, audit trails for electronic records, and agency inspections.

Electronic consent has recently been introduced as a possible solution to the informed consent issue. When it comes to data that is abstracted from patient platforms or social media sites, patients want the option to consent to having their information used for other purposes. Electronic consent is a methodological tool that would equip healthcare institutions with opt-in consent management solutions. Engaging with third parties that are in charge of collecting consent and making sure that data is anonymized is another strategy to easily obtain a consent, as well as gain access to observational data. Doing so allows organizations to benefit from rich data while minimizing the impact on patients’ privacy.\[^{5}\]

**Related M&A Activity**

Examples of notable transactions relating to RWD and RWE over the past few years include:

- **2019:** *Evidera’s acquisition of Medimix.* Medimix is a global technology company that provides RWE insights and information to the pharmaceutical, diagnostic, and medical device industries (note: Berkery Noyes represented Medimix in this deal).

- **2019:** *TriNetX's $40 million Series D funding round* led by Merck Global Health Innovation Fund. TriNetX is a global health research network that offers clinical research and enables discoveries through the generation of RWE.

- **2018:** *Roche’s acquisition of Flatiron Health for $1.9 billion.* Flatiron Health offers oncology-specific electronic health record (EHR) software, as well as the curation and development of RWE for cancer research.

- **2018:** *Evidation Health’s $30 million Series C funding round* co-led by SV Health Investors and B Capital Group. Evidation Health has launched a new platform built to digest large-scale sensor and behavioral data from real-world settings.

- **2016:** *Quintiles merger with IMS Holdings Inc. (now IQVIA) for $8.8 billion.* One of the main rationales for the merger was to take advantage of the growing use of RWE in clinical drug development and other areas of the pharmaceutical market.

**Challenges and Opportunities**

Despite growth in the adoption of RWD and RWE within the healthcare industry, there are still barriers that are preventing the expansion of its use. The most significant barrier is the consensus that randomized controlled trials (RCT) remain the gold standard for demonstrating the efficacy and safety of medical treatments and products. Data from RCT alone is simply no longer sufficient for informed healthcare decision making in certain situations, and RWD is a great complement due to its ability to collect data from real-life settings.\[^{6}\]

The lack of standardization of RWE analytics can be problematic, potentially leading to poor-quality analyses, limited transparency into methods, and biased results. RCT methodologies and practices are better developed and understood, thus making it easier for RCT to remain a more acceptable option than RWE. A possible solution to improving data quality is more uniform access to existing real-world databases for medical research.

Privacy concerns related to allowing access to these large datasets and the potential results of RWE analytics are also being taken into consideration. When dealing with large datasets, especially of patients’ personal information, companies are seeking appropriate governance and analytics capabilities in order to stay within the acceptable tolerance levels for compliance and reputation risk management.

**Conclusion**

The healthcare industry is rapidly evolving, and RWD and RWE are showing the potential to improve patient care. RWE is being used by payors, providers, and pharmaceutical companies to help make decisions about cost effectiveness and usefulness, especially when other data sources are lacking. RWE is bolstering the knowledge obtained from RCTs, leading to data sets that are more inclusive. Furthermore, there are plenty of instances where clinical trials are insufficient and RWE can be utilized to overcome some of those barriers. All of these factors are
contributing to a high level of interest from both strategic and financial acquirers.

About Berkery Noyes

Founded in 1980, Berkery Noyes is an independent investment bank that provides M&A advisory and financial consulting services to middle market companies in the information and technology industries. The firm offers skilled transaction management to publicly traded and privately held businesses and private equity groups in both sell-side and buy-side transactions. Berkery Noyes has managed over 500 transactions, ranging from several million to more than four billion dollars in value.

About Tom O’Connor

Tom O’Connor is a Managing Director in the Healthcare Group at Berkery Noyes. He has over 20 years of investment banking experience, recently representing notable companies such as Medimix in its acquisition by Evidera and Verisys in its growth investment from Spectrum Equity. Tom holds a BS in accounting from State University of New York at Binghamton and MBA from Fordham University.

Footnotes


