Payers are more concerned with how drugs work in the real world than in controlled trial environments. Unfortunately, real-world data is expensive to collect. Fortunately, there are databases filled with real-world evidence that developers can use to support their value case in their submissions.

By Canter Martin, Senior Director, Market Access Consulting & Maria Malmenas, Director Market Access Consulting
When it comes to approving a new drug, regulators are looking for evidence of efficacy (the drug’s desired effect) and safety (the drug’s undesirable effects, and how serious they may be). Payers, however, may have additional interests. They are less concerned with how the drug works in a controlled trial environment than with how it will work in the real world, in real settings, with real patients living real lives. And, most assuredly, they are looking at the price the developer is proposing to charge for the drug. Often they will want to know whether it is cost-effective. That, too, largely depends upon how it performs—and the costs that attach to it—in the real world.

The real-world evidence payers are seeking can be collected by drug developers through observational studies, but these are expensive and take significant time to complete. The drug developer has to recruit patients, work with clinics and treatment centers, and then collect data through surveys or other means. All this time, the new drug, in most cases, has only limited (if any) market access.

Fortunately, there are other, faster, less expensive ways to gather the real-world evidence that payers are increasingly demanding to determine the value of a new drug. At the same time, drug developers can use this data to better understand treatment pathways as well as to gain a more holistic appreciation of their drug’s costs and its impact on medical resource utilization.

WHERE THE WILD DATA LIVES

There are a number of sources for real-world data (RWD) that can provide insight on patient health-care journeys. Each source of data has its own strengths; each has its own limitations. Therefore, it’s often necessary to conduct analyses on data from different sources to develop a comprehensive view of the new drug’s position on the treatment pathway.

ADMINISTRATIVE BILLING CLAIMS. Insurance providers in the U.S. collect data on claims submitted by pharmacies, doctors, hospitals, and just about any service provider. This data is then aggregated and warehoused at the patient level. These are large, historical, longitudinal data sets that can provide insight into the patient journey. But they provide very limited information on patient vitals and clinical results, such as blood pressure, weight, or blood tests.

HOSPITAL EVENT DATA. RWD from hospitalizations is also available for retrospective analysis. From admission to discharge, this data offers detailed histories of what happened to the patient during his or her hospital stay. This includes the drugs they received, surgical procedures, costs and charges, the length of stay, and so on. But they don’t explain why, for instance, a particular treatment was administered.

ELECTRONIC HEALTH RECORDS (EHRs). Typically used for the delivery of care and records management, EHR data is aggregated within databases and provides clinical detail—diagnosis, treatment, lab results, prescribed medications—on the patient at every touch point within the health-care system. While EHRs don’t give drug developers
the detailed cost and resource utilization information usually found in claims databases, they provide valuable RWD to support outcomes research, including comparative treatment effectiveness.

**TRANSCRIPTION DATA.** These are the doctors’ notes from examining patients and assessing their health. This is a new and upcoming source of RWD. Transcription data is typically rich in clinical detail, especially with regard to severity and disease progression.

**REGISTRIES.** These are databases of observational studies. They serve a predetermined scientific, clinical, or policy purpose, and focus on how treatments, tests, and services affect patient health outcomes. Registries usually contain individual patient-level data on a specific disease, condition, or the use of a drug or other treatment. However, they do not always contain comparative data.

**HOW TO USE RWD**

The way in which a drug developer can use RWD—and therefore the types of sources that should be tapped—depends on the research question, the objective of the study, and the therapeutic area.

Drug developers must first define what the study is designed to accomplish. Is it meant to influence the payer decision? Is its purpose to inform internal decisions? Perhaps the goal is to educate.

After gaining clarity as to the purpose of the study, the developer can assess properly what data sets—claims, hospital, EHRs, transcription, and/or registries—will best support the study’s objectives. For an oncology drug, for instance, disease-progression data might be a key factor, and therefore transcription data and registries might be most useful. A study on diabetes, on the other hand, might be more focused on the number of hypoglycemic events, co-morbidities, or other health-related costs, bringing transcription data, rich in clinical detail, to the fore.

In one instance, a PAREXEL client wanted to know why patients switched from one treatment to another for a particular indication. We found a lack of relevant data in Europe; in the U.S., administrative billing claims data told us what treatment patients switched to, and when, but not why—and the why was critical to know. Were patients switching due to adverse events or a lack of efficacy or an adherence problem, or something else entirely? If our client knew the why, it could use that information to inform its own evidence-generation and market-access strategies.

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PAREXEL was able to find the answer (adverse events) in a database of transcriptions of patient consultations conducted by general practitioners. That data is now incorporated into the economic model.

RWD can also guide internal decisions. Database studies—collections of RWD—can be used in the early stages of drug development to conduct a landscape analysis, surveying therapeutic areas for unmet need and burden of illness. It can be used to enhance clinical-trial recruiting by better identifying target-patient parameters, or it can be used post-launch to collect evidence on market uptake, patient outcomes, and adverse events.

For example, PAREXEL was able to demonstrate that adherence to a client’s diabetes therapy was superior to that of a competing compound, due to a number of factors, most importantly, the number of doses per day. This was consistent with the trial findings, and the RWD confirmed that those findings held up in the real world, outside the trial environment. This not only provided evidence attesting to the benefits of the client’s therapy, but by giving the company a better understanding of the patient journey, it also better positioned it to develop further therapies in that therapeutic area.

The greatest challenge in using RWD is that it’s not always there where and when and in what form it’s needed. Registries are rule-bound; transcription data is not readily accessible; EHRs have yet to achieve universal adoption; and administrative claims data does not reliably illuminate disease severity or progression. Therefore, it’s important to be creative with the data that is available.

For instance, in a case when the severity of illness isn’t contained in the available data (as it won’t be in claims data), there could be a prescription, or treatment, that could be used as a proxy. Working with RWD is as much art as science.

Another strategy is to collaborate with patient registries to survey patients and collect RWD not typically available in their data sets. Creativity is not an empty word when it comes to collecting RWD. Some developers have gone into hospitals to pull the charts of patients who have had a particular surgical procedure. With 100 charts, it is possible to populate a small database and run a statistical analysis. This, of course, is an expensive process but, fortunately, easier access to RWD sources is increasing.

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Governments, especially in the Nordic countries and the UK, are making their RWD sources more broadly available, and commercial entities in the U.S., aware of the growing demand for RWD, are looking to occupy that market space. Indeed, PAREXEL has developed alliances that enable access to data that is often not available, including from restricted-access health-care databases in the U.S. We also collaborate with a registry in Sweden, and have access to a large transcription database.
Payers live and work in a constantly changing economic environment. They must balance patient needs and therapeutic advances with the need to manage costs locally and nationally. Increasingly, RWD is driving their commercial decisions. Drug developers need to monitor developments in the payers’ world to understand how they are making their decisions. PAREXEL strives to use its deep understanding of RWD and the payer world to advise our clients on their commercial strategies and source the best, most applicable and useful RWD to support our clients’ evidence-generation efforts.

Going forward, RWD will become ever more essential to drug developers. In the U.S., for example, the question of how many readmissions to hospitals are associated with a given therapy will become more critical to providers as under the Affordable Care Act, outcomes are tracked, with rewards for performance and penalties for substandard outcomes. RWD is the ideal approach to answering that question, and for drug developers it is a cost-effective way to build a commercial argument from the preclinical stage all the way through post-launch.

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CORPORATE HEADQUARTERS
195 West Street
Waltham, MA 02451
USA
+1 781 487 9900

Offices across Europe, Asia and the Americas

www.PAREXEL.com