porexel.

Using real-world evidence to help your product succeed

Real-world Data



>>> We'll help you unlock the potential of real-world data

In today's healthcare paradigm, real-world data (RWD) holds tremendous promise for biopharmaceutical companies as a key aspect of drug development initiatives. Drugs developed using RWD are more likely to launch, and innovative studies that harness RWD are helping to demonstrate value to patients and payers alike, reducing development costs and boosting trial recruitment.

Let us show you how our experience of leveraging RWD/real-world evidence (RWE) can help your product launch succeed.

With Heart

>>> First things first: how are we defining real-world data and real-world evidence?

Technology advances at a breakneck speed, and there is an enormous variety of data types. So it's not surprising that terms such as RWD and RWE are open to interpretation within the industry. A 2018 report, The Innovation Imperative: The Future of Drug Development from the Economist Intelligence Unit (EIU)¹, commissioned by Parexel, offers useful definitions of the two:

Real-world data

RWD is data collected from various sources outside a typical clinical research setting, such as a randomized clinical trial (RCT). Essentially, these data are a by-product of the patient's experience during

1. The Innovation Imperative: The Future of Drug Development, Part I: Research Methods and Findings, The Economist Intelligence Unit, 2018.

their journey through the disease. Data can include electronic health records, pharmacy or payer claims data, product and disease registries, observational studies and patient-reported outcomes, as well as data gathered through personal devices and health applications, such as social media and wearables.

Real-world evidence

RWE is generated according to an analytical plan and interpreted accordingly, essentially transforming the data into meaningful evidence. This data can be used to generate insights about a product's effectiveness.



>>> Innovative approaches to using RWD

It's important to note that the EIU report, mentioned on the previous page, evaluated Phase II and III studies that incorporated RWD to improve drug-development efficiency, resulting in greater launch success. Using RWD in drug development is not a new phenomenon. However, traditional applications, such as informing the feasibility of a study design, or a health economic model, are different to the innovative approaches we are beginning to see today.

In what we at call "emerging use cases," RWD are increasingly used as a clinical data set to support a therapy's safety or efficacy assessment. Indeed, RWE can help in developing hypotheses for testing in randomized controlled trials (RCTs), in identifying potential biomarkers and assessing trial feasibility.

>>> How do RWD and RWE support regulatory and market access decisions?

Today, legislators and policy makers are actively engaging in dialogue about the value of RWD and RWE. The U.S. Food and Drug Administration (FDA) is developing specific guidelines on the use of RWE to support regulatory decision-making.

This is a critical step forward in our ability to apply an understanding of real-world patient care and outcomes to the drug approval process.

The European Medicines Agency (EMA) has convened similar initiatives, and in the Asia-Pacific region, interest in leveraging RWD in clinical research is growing rapidly. Today, regulators and payers are increasingly looking to RWE to support regulatory or market-access decisions.

Drugs developed using RWD in Phase II and III have a

21%

greater likelihood of launch across all therapeutic areas¹

1. The Innovation Imperative: The Future of Drug Development, Part I: Research Methods and Findings, The Economist Intelligence Unit, 2018.

>>> What are the barriers to using RWD/RWE?

Despite the reported benefits of harnessing RWD, the adoption rate is surprisingly low in the clinical trial setting. In the thousands of studies examined in the EIU analysis, RWD trials made up less than 1%. This underutilization can be explained by:

- The growing volume and variety of data types, complex data models, lack of standards and consistency in managing and interpreting data, and difficulty linking disparate sources
- The lack of a data-savvy workforce and the steep learning curve required to overcome this
- General uncertainty among study teams
 for example, about whether budget will support such trial designs, and data quality
- Ambiguity around payer and regulatory acceptance of data for decision-making
- Variations across countries and regions that hinder a global approach, particularly relative to data-privacy standards and data availability



>>> How is the impact of RWD on drug development evolving?

At Parexel, we are working to overcome the barriers to using RWD by fostering innovation in clinical trial processes and exploring new ways of demonstrating their value in drug development. In our experience, when we use RWD to help us understand what's important to clinicians and patients in order to inform the study design, studies are more attractive to patients and clinicians alike.

The EIU report cited the role of RWD in improving enrollment rates – by an average of six months. That can have an enormous impact on reducing study costs

On the payer side, the use of RWD enables early payer engagement – another finding of the EIU report. We have found that using RWD helps teams define the criteria for value-based reimbursement vs. volume-based schemes, moving to outcomes that matter. Additionally, it helps prepare payer bodies for the entrance of the new therapy.

In developing treatments for rare diseases, RWE helps sponsors to better understand payer perspectives, anticipate what questions they will ask and develop answers earlier in the product development cycle. These critical insights ultimately facilitate reimbursement decisions.

Clearly, given these observations, the onus is on the industry to challenge the way we think about study design and to advance our capabilities in this area. From a holistic perspective, we recommend overcoming barriers by encouraging collaboration and connecting people, removing data silos, working more seamlessly across the company and engaging in multi-stakeholder initiatives.

>>> How we can help you

Using RWD will unquestionably make a significant impact on drug development in the near future. As this exciting field continues to evolve, Parexel can provide long-standing experience and wellestablished best practices that are focused on supporting this methodology.

Collectively, our experience in studies generating RWE has allowed us to establish a deep understanding of stakeholders' evidence needs in the context of market landscape, current standard of care, patient profiles and evidence gaps.

We offer:

- The industry's first dedicated late-phase research unit, with over 17 years' experience in designing, implementing and analyzing real-world studies across the globe
- A multidisciplinary approach to building an RWE strategy that meets the needs of patient, payer, internal and regulatory stakeholders



- Data search, storage, integration and analytics supported by an extensive and proprietary technology platform and approach
- Combining large volumes of RWD from multiple primary and secondary sources, integrated from many different databases
- Supporting decision-making with data visualization capability
- > Deep experience in assessing evidence gaps and in evidence-generation planning
- Broad expertise in strategic, operational, clinical development, regulatory and market access consulting, and in successfully conducting real-world studies of all types

Your Journey. Our Mission.®

>>> We're always available for a conversation

To learn more about our Adaptive and Flexible Trial designs, please contact:

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