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Six Trends Influencing the Future of Real-World Evidence | PPD Inc

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The evidence is in: From market access to patient centricity, drug developers that conquer these key areas will be best poised for product delivery and commercial success. Here's how to do so.

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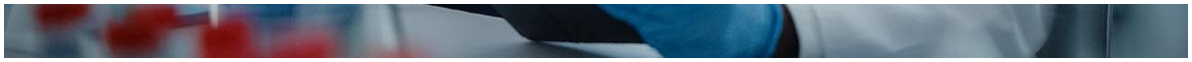

The use of real-world evidence (RWE) in pharmaceutical approval, market access and post-marketing is continuing to evolve — particularly as the U.S. Food and Drug Administration (FDA) and global regulatory agencies begin to expand the role of real-world evidence to guide regulatory decisions and monitor product safety.

Given the growing importance of real-world evidence in demonstrating value for market access and commercialization, it's imperative that drug developers quickly identify and overcome related challenges. They must navigate an explosion of data sources, untangle which data is most relevant, and pinpoint and analyze the insights from real-world evidence that will drive decision-making.

To successfully unlock and leverage evidence, drug developers will need to understand and master six key trends influencing the Real-world evidence landscape.

These trends are a culmination of insights from:

- Industry thought leaders
- Analysis of regular conversations with innovative biotech and biopharmaceutical companies
- Observations from ISPOR, the International Society for Pharmacoeconomics and Outcomes
- Participation in independent multi-stakeholder working groups, such as the Get Real Institute and the Duke Margolis Real-World Evidence Collaborative

- More than 30 years of experience from the experts at Evidera, providers of evidence-based solutions within the PPD clinical research business of Thermo Fisher Scientific

When drug developers expand competencies in these areas — or alongside a strategic partner that guides real-world data research, study design, implementation, analysis and insights — they'll be enabled to move their products forward to success.

1. Real-World Data (RWD)

RWD is data that are routinely collected about patients and their health from multiple sources outside of randomized clinical trials. Types of RWD include:

- In the clinic: Electronic health records (EHRs), claims and billing, and product and disease registries
- Outside the clinic: Patient-generated data collected in a home setting, through mobile devices, such as wearables, and on patient-mediated platforms

Examples of RWD in use include:

- By developers: Medical product developers are taking innovative approaches in resourcing and generating RWD to inform design of clinical trials and observational studies in order to generate new treatment approaches.
- By regulatory agencies: RWD are used in regulatory settings in evaluating product effectiveness and safety for approval decisions and long-term monitoring once the product is on the market.
- By payers and health technology assessment (HTA) agencies:

Evidence of value generated from RWD is used in decisions affecting a product's coverage once approved.

So many rich sources of RWD present a new challenge: How can drug developers best identify and sort through the data to glean relevant and actionable insights for their products and, ultimately, patients?

Varied, elevated expertise in trial design and execution is a strong start. To conduct research in a timely, effective manner, biotechnology, pharmaceutical and medical device companies must have methodical approaches tailored to their research questions and aligned to their strategic objectives, targeted stakeholders and expected impact.

Without proper expertise and rigor around RWD and its blooming possibilities, clients risk an inefficient approach to answer their research questions — and an inferior ability to deliver impactful insights.

2. Advanced Analytics

Increasingly, there is an abundance of data that can generate novel insights for all drug development stakeholders, including to support regulatory decisions. Advanced analytics involves cutting-edge methods and technologies that enable innovative and efficient evidence solutions, such as machine learning, artificial intelligence (AI) and natural language processing (NLP).

Two key drivers of advanced analytics include:

- Predictive analytics, a high-tech area that, with trained staff, fills data gaps, connects scientific principles, and drives strategic and tactical innovation.

- Artificial intelligence (AI), which allows researchers to identify relevant data for specific study needs by analyzing large amounts of data quickly. This can save valuable time in a variety of ways throughout the research process, including patient recruitment and identification of adverse events by extracting clinical notes from EHR data.

  Advanced analytics focus as much on identifying appropriate, clinically rich data as they do on obtaining actionable, data-driven insights. With evidence-generating experts on hand, biopharmaceutical and biotechnology organizations can lean on advanced analytics to more quickly identify patterns and trends in the data — connecting science and strategy to drive medical advancements forward.

  One aspect of data analytics that shouldn't be overlooked is the assessment of data quality. There's no doubt data can be messy, especially in disease areas where data are incomplete or ill-defined. In terms of data analysis, rare diseases can be especially challenging. These obstacles make it even more important to tap into experienced data experts that have demonstrated prowess in assessing hundreds of disparate data sources to generate evidence and unlock value.

3. Patient Centricity

Whether it's with wearables, patient-friendly trials or digital access to providers, patient centricity puts the patient at the center of health care. And each way you turn, stakeholders, agencies and communities at large are turning up the volume on patient centricity:

- Increasingly, regulatory and HTA agencies are asking for patient-involved clinical trial protocol designs, patient experience and preference data.
- Patient advocacy groups are becoming an essential component of the health care development and delivery ecosystem.
- There's a greater focus on cultural and social determinants of health to drive progress on health equity and reduce disparities.
- Clinical trial organizers are ensuring study enrollment incorporates patient diversity and underrepresented groups, which ultimately improves the applicability of research to a broader audience. Recently, this was seen during the COVID-19 pandemic when researchers leveraged digitization for real-world evidence collection.

  Bringing the patient's voice forward is not always an easy task — studies must be designed from soup to nuts to be patient-friendly and should leverage appropriate clinical outcomes assessment (COA) measures for endpoint evaluation to understand the patient's experience. To effectively capture the patient's experience, consider working with an experienced partner, such as one that has the largest patient- centered research group in the industry.

4. Market Access Proficiency

A key aspect of market access is generating, analyzing and communicating data to stakeholders that conveys the value story of a new medical intervention in order to achieve reimbursement for use. Market access challenges can inhibit patient access to essential medications – making early planning critical to drug

developers' real-world evidence plans.

Keys to commercial success for drug products include:

- Incorporating market access plans early in the drug development process
- Proactively accessing evidence-generation capabilities to avoid delays from missing evidence gaps
- Engaging with regulatory agencies and payers early to receive input on specific product development plans and strategies. With feedback from these entities, researchers can plan clinical trials more effectively to gather evidence needed for both approval and access.

Market access challenges occur frequently within the cell and gene therapy space, where a single therapy is administered once but could have a lifelong, lasting impact. The question here regarding market access is: How does this compare to a standard-of-care treatment that a patient might have to take twice a day?

Because of such challenges, cell and gene therapies — often used to treat rare disorders — can be expensive, which creates additional barriers for the patient. This is compounded by the fact that payers are increasingly interested in paying for drugs only if it can be demonstrated that the drug is actually working, which requires knowledge of long-term safety and durability, even for single-use drugs. If the drug is not as effective in the real world as demonstrated in clinical trials, payers are willing to pay less. To help determine costs, payers must also determine how one modality might be better than another.

In the end, regulatory approval is only the first step. Success also

means developing a treatment that payers will pay for, providers will prescribe, and patients will request. Early consideration of these downstream stakeholders — when developing an evidence generation strategy — will provide the best benefit to developers.

Clearly, many factors play a role in determining market access decisions, from early modeling of long-term projections of cost-effectiveness and efficacy, to long-term follow-up data on safety and effectiveness once certain treatments are on market. To carry out these assessments, pharmaceutical companies should look for partners that have:

- Staff trained in regulatory details
- Experience in innovative technology for data collection
- Analytical capabilities to deal with volumes of data and identify insights that can be actioned in decision making throughout the development process

5. Dynamic Evidence Packages

With evolving regulatory and HTA evidence requirements, dynamic evidence packages are a critical method for drug developers to efficiently present evidence of product value to respective stakeholders in formats that meet their specific needs.

Developers generate evidence for multiple stakeholders, but the specific evidence needed — and how it is communicated — is different for each audience in each region of the world. Cross-practice collaboration within companies ensures alignment on well-organized, planned and delivered evidence packages. The increasing use of integrated scientific advice that engages both regulators and payers is one important tool in achieving this

alignment.

The increased interest in how evidence is presented — whether interactive, in dashboards or across customizable displays for global- and country-specific needs — corroborates the need for sponsors to access rich data that will underpin the dynamic formatting. Transforming communication of product value in compelling and dynamic ways that provide the right evidence to the right stakeholder will go a long way in ensuring the message is clearly received. As technology evolves, so does the desire for new and easy-to-comprehend content, such as mini-global value dossiers, to continue to move reviews and approvals along faster.

Even post-marketing, evidence communication continues to be important. For treatments that require long-term follow-up studies and other post-approval requirements, regulators and payers will need continued documentation of product value evidence. This becomes important again in outcomes-based contracting, which seems to be the direction of reimbursement for cell and gene therapies. In these cases, payers reimburse at different levels for treatments as they prove they are safe and effective over time — hence the feedback loop of value back to payers remaining a priority.

6. Collaborative Initiatives

With multi-stakeholder engagement, a biotechnology or biopharmaceutical company can get insight from many experts at once — and that's a good thing. Working in collaborative ways with different stakeholders, from regulators to payers to patients, can enable better outcomes and more efficient processes.

Regulators and HTA agencies are working together through integrated scientific advice efforts to help companies align their evidence needs earlier in development. But they are not the only stakeholders working together. Here are other examples of collaboration among stakeholders in the health care arena:

- **Patient-reported outcome (PRO) instrument development:** There are now several multi-sponsor efforts in the development of single, standardized PRO instruments — such as the EXACT measure for chronic obstructive pulmonary disease (COPD) — to help synchronize efforts and shorten development times for companies requiring consistent questionnaire content in therapeutic areas.
- **Patient-centric focus:** We also see more joint efforts to engage multiple stakeholders to ensure patient-centricity in drug development, including the engagement of patient advocacy groups in study design, recruitment efforts, and patient burden assessment.
- **Real-world evidence advancement:** Collaborative initiatives, such as the Get Real Institute, are advancing important measures like real-world evidence in health care decision-making. These efforts bring together the best and brightest from a variety of perspectives to offer resources that provide value to all stakeholders, and ultimately move new treatments forward, faster for patient benefit.

Real-World Collaborators

Advances in technology and access to information have led to increased demand for evidence of benefits and risks in a real-

world setting by all relevant stakeholders, including regulatory and HTA agencies, health care providers and patients.

Evidera's scientists, researchers and industry experts remain ahead of the curve, implementing trends and partnering with clients to facilitate collaboration, enable more seamless access to data and answer your most challenging research questions.

Our multidisciplinary team offers guidance in the design and execution of studies to fulfill evidence-based requirements. We offer diverse scientific methods and high-quality project and data management expertise. Our methods in advanced analytics, market access and patient centricity, and our ability to partner with you across the evidence spectrum, provide the most collaborative offering on the market.

See how Evidera can help you move your products forward, faster and further.