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EVIDATION REPORT

5 industry insights to enhance real-world evidence strategies:

Unlocking the full potential of data for healthcare insights

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Introduction

Real-world evidence (RWE) plays a vital role in understanding the value proposition of new products or programs in healthcare. However, current RWE strategies based on point-in-time real-world data (RWD) — such as electronic health records (EHRs), claims, and patient registries — provide a limited view of an individual's daily experiences in managing their health or diseases/conditions.

Consequently, there is a growing demand among healthcare stakeholders for enhanced RWE that provides insights into the quantity and severity of disease-related symptoms, as well as mental and physical well-being and quality of life. To address this, organizations are exploring the incorporation of continuous data from day-to-day life, such as electronic person-reported outcomes (ePROs) and digital health data throughout the entire product development and commercialization lifecycle. In this report, we present five insights to enhance your RWE strategy — compiled from recent conferences, articles, and discussions we've had across the industry.

1. Better development and commercialization decisions are enabled with a true understanding of people's lived experiences

What we're hearing

Life science and healthcare organizations struggle to know the optimal timing for preventive and therapeutic interventions — when people would be most willing to engage with their product or program. Discussions in this area center around how to achieve a full end-to-end view of a person's experience with their health, with common questions including:

- Can we identify predictors of a disease diagnosis, and what data and biomarkers are available prior to diagnosis to inform this?
- At what point in the disease do people seek care?
- What influences treatment decisions?
- What do patients care about in terms of treatment outcomes, and do our assessments accurately capture that?
- Do outcomes (such as quality of life or mortality) improve after starting or switching the treatment? If not, was it because of the treatment itself or because the right assessments weren't being used?

In addition, companies are realizing that many of the traditionally used assessments inadequately capture the value of novel treatments and are seeking ways to generate more objective data. PROs and clinical observation tend to be highly subjective, burdensome, capture endpoints people do not always care about, and are not sensitive enough to document subtle, yet important, changes in health/disease status. This is especially true for rare diseases or neurodegenerative conditions, for which day-to-day variations in function and quality of life are common. When combined with small sample sizes, companies developing new, highly anticipated treatments find it challenging to demonstrate the clinical and cost-effectiveness to regulators and payers.

Many organizations, however, struggle with the resource-intensive, mostly manual process of consolidating and analyzing the data (e.g., from wearables and PROs) from different sources to provide these insights.

> Data is too siloed — it's like finger lakes. We need to find ways to interconnect between these lakes.
>
> ***Quote from Pharma USA***

The importance of understanding people's lived experiences

Using RWE across the entire development and commercialization life cycle contributes to more efficient research, enhanced pharmacovigilance, and an understanding of real-world use. Exciting developments in this area were presented at ISPOR. Sessions described a Duchenne muscular dystrophy (DMD) project using digital measures from wearables to derive **new, objective biomarkers** to predict disease progression and potentially track the response to therapy. Another project involves a **natural history model to create more accurate quality of life** assessments that will help fill the gap in understanding how new treatments benefit people with DMD.

In addition, companies can use information about a person's lived experience to identify supportive services such as mental health interventions that could help improve overall outcomes, not just those specifically related to the disease of interest.

KEY CONSIDERATIONS IN THIS AREA

- Which measurements can be conducted digitally?
- Are the measurements validated? If not, should they be, and what is the best method and timing to do so?
- Which complementary data sources are needed to describe the "whole" person and the full value proposition?
- To effectively use the vast amounts of available data, what is required to efficiently integrate, contextualize, and analyze unstructured data?
- How will data from multiple sources be linked to generate the "whole" person view?

2. RWD gathered directly from individuals can reveal their true day-to-day experiences with their health

What we're hearing

Along this vein, conversations at conferences increasingly center around "people" rather than "patients," and the term "everywhere care" emerged at Pharma USA. This shift in word choices reflects the healthcare and life science industry's growing recognition of the multifactorial influences on health, that a disease or condition does not exist in a vacuum, and that "healthy" means something different for everyone. For example, for people with a health condition, healthy is a feeling that changes often: It could mean not feeling pain at the moment or being able to move more.

Why representative data are important

Through RWD captured in a person's everyday life, companies can understand meaningful aspects of health outside of the healthcare and "lab" environments, which are often not representative of a person's experience with their health. This information:

- Facilitates the development of outcome measures through an understanding of the daily impact of treatment
- Captures the nuances of disease and treatment across a broad spectrum of individual experiences
- Contributes to health equity by providing data that accurately reflects the characteristics of the target population

KEY CONSIDERATIONS IN THIS AREA

- Which strategies will most effectively reach a broad range of people?
- Which data collection methods fit into daily life and reflect the outcomes of interest?
- What is the best way to build trust and respect into the research?
- How can the experience be designed around people's lives to reduce points of friction?
- Which features and functions would make the research an enjoyable and valuable experience so people want to participate?

3. With appropriate planning, healthcare stakeholders' demand for RWE can be met (and exceeded)

What we're hearing

Treatment success goes beyond the research itself. All healthcare stakeholders need to be on board with RWD, how they are collected, how they capture the outcomes of interest, and their place in the treatment pathway. Organizations and physicians both have been hesitant to adopt digital measures for RWD because they do not know how to integrate and interpret the data. Therefore, the industry needs to identify strategies to incorporate person-generated RWD (e.g., data from wearables, apps, ePROs) not only into research but also into clinical practice. Without this translation into treatment decisions in real life, the data lose their value for payers, healthcare providers, and individuals.

The perceived value of person-generated RWD (e.g., data from wearables, apps, ePROs) by different stakeholders

STAKEHOLDER	VALUE OF RWD
Individuals	• Ability to monitor their own health • Opportunity to be seen both in and out of the clinic walls • More convenient methods of contributing data for their healthcare • Opportunity to have their desired outcomes measured (quality of life, being able to complete activities of daily living, being able to walk down stairs or with grandchildren, having one-time administration with a long-term effect, treatment with minimal disruptions to daily life)
Healthcare providers	• Ability to provide better care for patients, which would also contribute to patient satisfaction • Economic outcomes, such as increasing diagnostic efficiency or accuracy and decreased staff time • Additional ways to monitor health and treatment between visits
Biopharma	• Ability to identify gaps in the treatment paradigm pre-launch • Ability to truly show the value of novel treatments that isn't adequately captured by traditional assessments (validated surrogate endpoints) • Opportunity to identify early exploratory outcomes • Ability to quantify the product's performance in the market post-launch • Tools for precision targeting for recruitment

STAKEHOLDER	VALUE OF RWD
Governments	• Ability to understand factors driving health care access • Opportunity to monitor public health surveillance trends (i.e. influenza, COVID, upper respiratory tract infections)
Regulatory agencies	• Ability to truly understand the value of novel treatments that isn't adequately captured by traditional assessments (validated surrogate endpoints) • Opportunity to review early exploratory outcomes • Ability to better conduct pharmacovigilance/post-marketing surveillance
Payers	• Ability to better understand the burden of disease and full economic impact of the disease and the specific interventions on both the patient and the health plan • Opportunity to understand the real-world use of medical products

KEY CONSIDERATIONS IN THIS AREA

- Which data points are of interest to each stakeholder?
- In what format should the information be presented and delivered? (This will depend on whether the intent is for communication with a healthcare provider, a peer-reviewed publication to further knowledge, part of a regulatory package to document safety and efficacy, or a payer dossier evidence package for formulary placement review.)
- What gaps in understanding is each group trying to fill, within which resource constraints (e.g., budget) and priorities (e.g., generation of evidence)?

4. Aligning RWD early in the product pipeline helps overcome regulatory and approval hurdles

What we're hearing

Novel approaches to data collection are important; however, unforeseen data issues could occur due to less experience with the methods by all stakeholders. Therefore, new assessments typically require validation against the gold standard, in-clinic evaluation (i.e., MMSE for cognitive decline), regardless of whether the digital measure is more objective and comprehensive.

RWD sources include both retrospective (i.e., claims and EHRs) and prospective (i.e., PROs, digital measures) data. Using a mixture of both can meet regulators' and payers' expectations, while balancing ease of access and lower costs with retrospective data sources with the more credible data from prospective sources that regulators are looking for.

Why early planning is important

Organizations need to create their RWD strategy early to ensure it will provide the data regulators expect. Failing to meet regulatory and payer requirements can mean delayed approvals and the need for additional data collection and/or analyses.

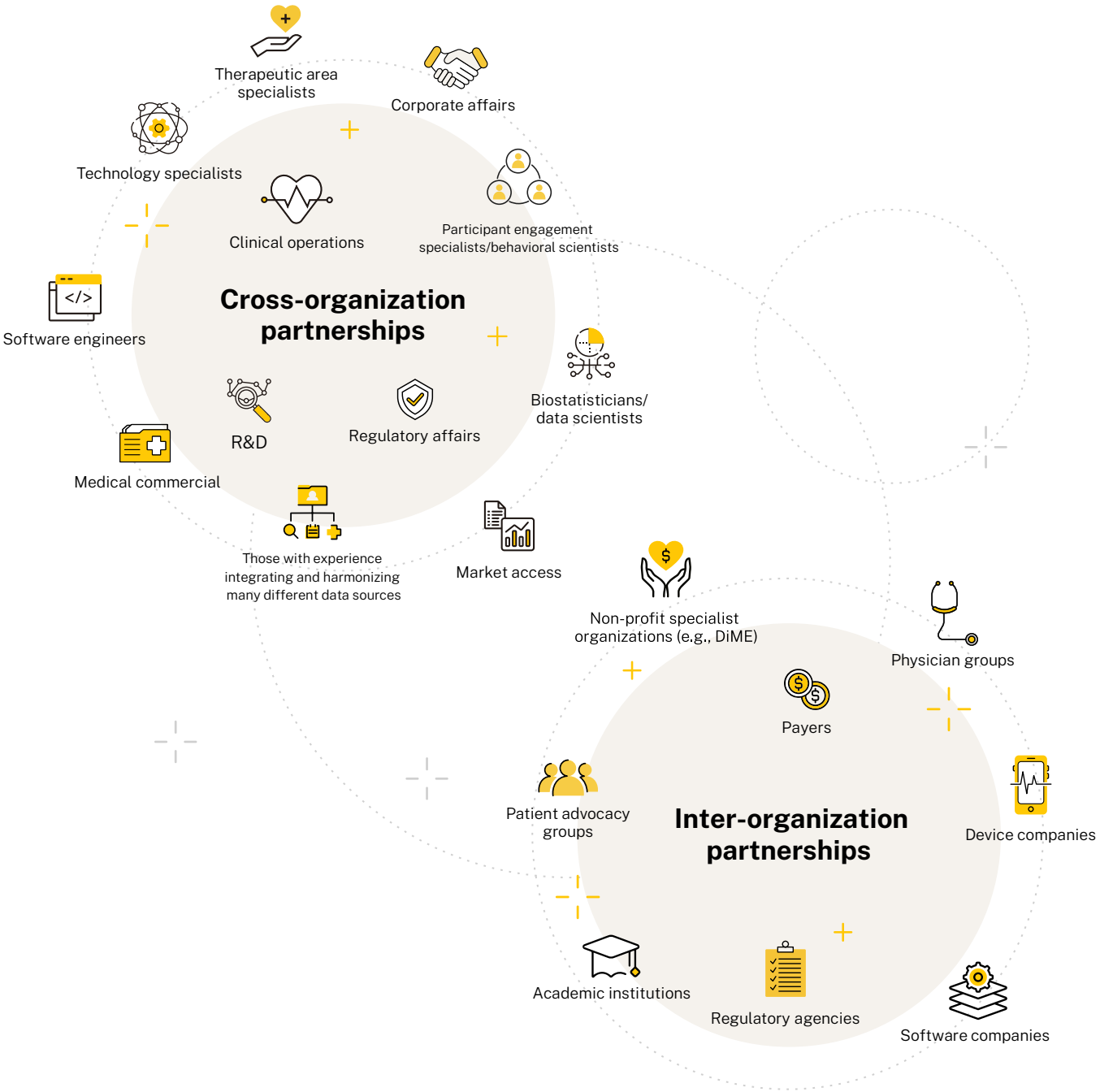
KEY CONSIDERATIONS IN THIS AREA

- Define the right endpoints for the research (e.g., greater walking capacity, reducing the effect of migraines on physical function, better sleep), which will inform the required measures
  - Determine the health concepts that are affected (e.g., walking a specific distance, sleeping for a specific amount of time)
  - Identify outcomes to collect from PROs, wearables, sensors, or other digital measures (e.g., step count, sleep duration)
  - Communicate with regulators to determine if the digital measures need to be validated (e.g., surrogate for a 6-minute walk test) or can remain unvalidated (e.g., subjective satisfaction survey)
  - Discuss with payers whether they feel the measures will adequately measure the outcomes of value
- Review available programs for incorporation of RWE, and plan discussions with regulatory agencies and payers early
  - For example, the FDA's new **Advancing Real-World Evidence Program**, announced in October 2022, "provides sponsors … the opportunity to meet with agency staff — before protocol development or study initiation — to discuss the use of RWE in medical product development"

5. Strategic partnerships drive faster RWE implementation

What we're hearing

Organizations benefit from both internal and external partnerships that provide unique insights to help find gaps in existing strategies, determine how to make data more meaningful, and identify how to use RWD to support commercial messaging.



Benefits of collaboration

Companies do not need to develop their RWD strategy from the ground up. Instead, cross-organization partnerships bring together people to enhance and diversity skill sets, while inter-organization partnerships can fill gaps in experience and skills and help interpret the multi-sourced data for clinical relevance.

A session at **ISPOR 2023** highlighted the following benefits of inter-organizational partnerships depending on company size:

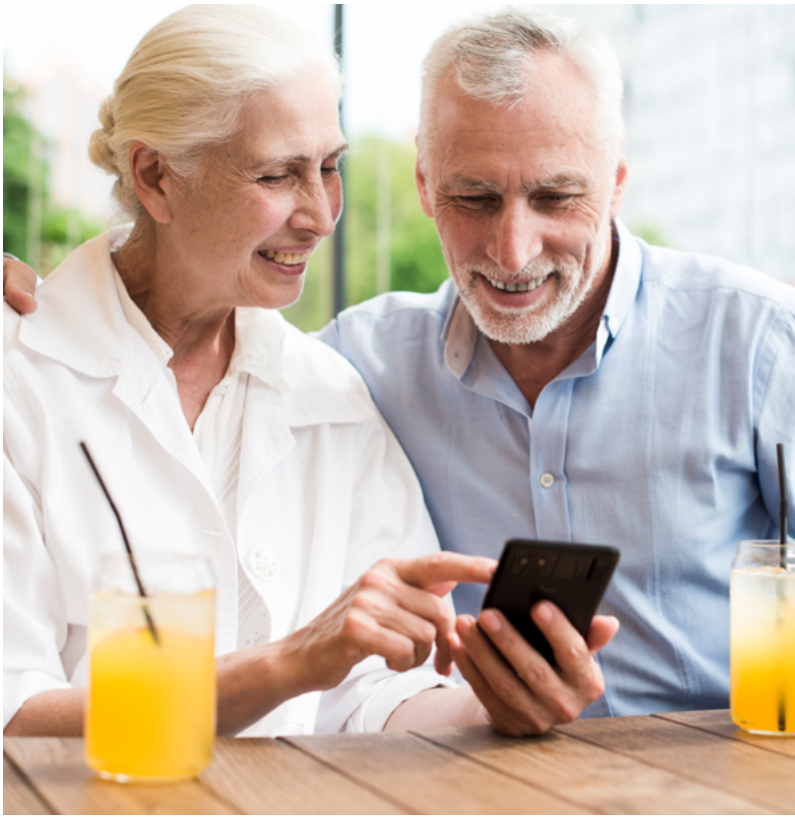
- Small companies can supplement capabilities without constraining the budget
- Mid-size companies can overcome challenges with resource constraints and disease-specific RWE generation
- Large companies can overcome challenges with data transportability and generalizability across programs, platforms, and disease areas

KEY CONSIDERATIONS IN THIS AREA

- What framework should be used to optimize the collaborative relationships?
- How will data sharing models be designed to promote safety and sustainability?
- How will everything be integrated and utilized across functional groups in charge of various aspects of a product life cycle?
- What is each department and/or organization's role, and what value does each bring to the table?
- Which capabilities exist in-house, and which need to be outsourced?
- Which organizations (such as DiME) are developing systematic processes and guidance for the implementation and approval of technology and digital measures in research?

Person-generated RWD is emerging as a critical component of RWE strategies

The RWE landscape is rapidly evolving as RWE becomes entrenched in product development and evaluation. Companies should start now to set the foundation for integrating RWE into development and commercialization. Robust, yet flexible, RWE strategies will benefit from the emerging developments for collecting data that reflect a person's whole experience and accurately capture the value provided by novel treatments.



To learn how Evidation can help generate, combine, and interpret the right data to enhance your organization's RWE strategy, **connect with a member of our commercial team.**