Extended Real-World Data:
The Life Science Industry's Number One Asset

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The life science industry (pharmaceutical, medical device, biotech, digital therapeutics companies, and other innovators) has invested significantly in data—specifically, extended real-world data (RWD)/real-world evidence (RWE). More importantly, the industry has realized that focusing on population health management (PHM) and outcomes improvement is its guiding principle and top goal, and data is one part of how it will achieve that goal.

Patient-centric data beyond commoditized claims data assets is becoming instrumental in the drug development pipeline. It's informing the process from discovery, new indications, clinical development, trial design, and measuring outcomes (e.g., side effects) to identifying who is using an approved drug and why and determining value-effectiveness for drug reimbursement. As data has become a staple decision driver at most life science companies, organizations are increasingly aware of the need to bridge the divide between these two data imperatives:

- Leveraging the fully digital experiences (e.g., patient-facing applications) and the existing clinical, provider setting.
- Working with large healthcare systems and their providers and patients, with a thorough understanding of clinical operations, to drive meaningful change.

This report explains the importance of extended RWD and RWE for the life science industry and how partnering with the right healthcare transformation company provides access to the patient-centric data needed to improve the drug development process.

Leveraging Data to Overcome the Inefficiencies of Drug Development

 The drug development process takes from 8 to 15 years, costs up to $11 billion, and relies on an expensive and often inefficient clinical trials process, as well as costly, sparse data (often only claims data) that doesn't provide a full picture of patient health. For example, claims data will show that a patient fills a prescription but gives no insight into outcomes, side effects, etc.

Life science companies can improve this process and save costs by partnering with a healthcare transformation company to access and leverage extended RWD and RWE to better understand the populations using their drugs and their outcomes. As the entire system (regulators, payers, manufacturers, and providers) aligns around outcomes, the following knowledge areas will enable a fair, outcomes-driven healthcare system:

- Real-world patient care (e.g., care variability between and within health systems).
- Measuring outcomes in real time across a diversity of provider types, shapes, sizes, and geographies.
- Understanding the clinical processes that drive those outcomes.

Healthcare today has a crucial opportunity, as, for the first time, key industry players are aligning on the same key goals. Regulatory, cost, and reimbursement pressures are driving the urgency to deliver the right treatment to the right patient, as measured by real-world outcomes and monitoring. This means that manufacturers, payers, and providers all benefit from solving similar challenges:

- How do I identify patients with lower risk and highest benefit from treatment "X"?
- How do I manage my patient population, at a PHM level, to drive overall balance between clinical and financial outcomes?
- How do I predict, identify, minimize, monitor, and measure drug safety issues?
- How do I ensure high standards of treatment adherence, patient education, support, and follow-up to maximize outcome potential?

While RWD (mostly claims and some EHR data) has shown more useful potential for life sciences in the past few years, its impact has often been limited to specific settings, disease areas, geographies, payers, etc. The coming years will likely see a substantial impact across most therapeutics, both for their development, launch, and post-launch activities.

The FDA Leads the Way: RWD for Regulatory Approvals

The 21st Century Cures Act was signed into law in 2016, boosting the value of RWD and RWE for the FDA and life science industry. The Cures Act aims to accelerate medical product development and innovation and places more focus on RWE- and RWD-driven decision making. According to Congress, RWE is data from sources other than clinical trials (e.g., randomized trials and observational studies) on the use and the potential benefits or risks of a

drug. Congress describes RWD as data on patient health status and/or delivery of care that's routinely collected from EHRs, claims and billing, patient-generated data, and more. Both RWE and RWD are growing in volume and depth with the increasing use of computers, mobile devices, and wearables and gaining utility as advanced analytics capabilities (e.g., AI and machine learning) enable more personalized and actionable insights.

In 2018, the FDA published the Framework for FDA's Real-World Evidence Program, which further details the usefulness of RWD in trials for new therapies. The emphasis on RWD is leading to a new approach that includes pragmatic trials (e.g., trials where the control arm is based on RWD from the standard of care) and synthetic cohorts (generating historical controls from historically accumulated trial controls, and/or simulating them on current RWD cohorts). Because pragmatic trials are poised to slash costs and reduce timelines drastically, life science companies that adopt them early will differentiate themselves in the market.

Key RWD/RWE challenges are emerging around access to health systems and patients as different organizations compete to enroll patients in traditional studies, as well as innovative studies that leverage data-driven approaches. It's not sufficient for life science companies to leverage data; they must also create clear value for providers and patients, ensuring that innovations in clinical development help health systems achieve certain goals:

- Develop population health and clinical operations strategies that align clinical trials with standards of care.
- Choose trials that are truly of value to the patient and to the health system that manages them.

Leveraging Broader and Deeper Real-World Data to Improve Outcomes and Avoid Waste in the Clinical Development Process

For much of the past decades, inefficiencies in clinical drug development amounted not only to money the pharmaceutical industry spent (from research to launch) but also in poorer overall outcomes for patients, who experienced a rigid, synthetic clinical trial environment (i.e., being put on a placebo arm for the sake of the trial design rather than the sake of the patient or excluded from promising trials due to the complexity of trial designs). A real-world approach marries clinical development with the realities of healthcare:

- Comorbidities.
- Complexity.
- The need to measure real-world outcomes (versus synthetic outcomes within a sanitized trial setting).

3

Critical Value Across the Life Science Pipeline

By leveraging extended RWD/RWE, life science companies gain critical value across their pipeline (Figure 1), allowing them to:

- Save money.
- Accelerate clinical trials time and time to market.
- Create a more insightful digital marketing strategy.
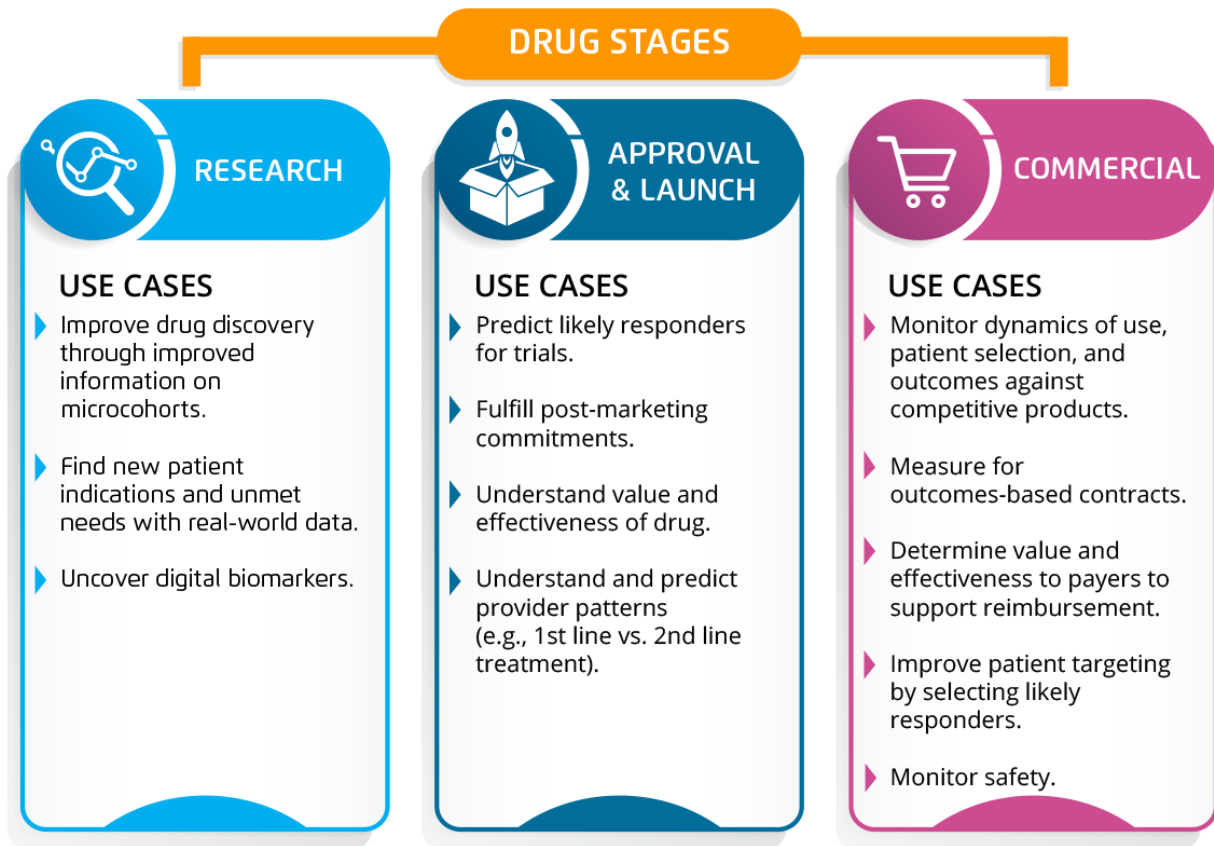- Improve matching of patients with drugs.

DRUG STAGES

RESEARCH

USE CASES
- Improve drug discovery through improved information on microcohorts.
- Find new patient indications and unmet needs with real-world data.
- Uncover digital biomarkers.

APPROVAL & LAUNCH

USE CASES
- Predict likely responders for trials.
- Fulfill post-marketing commitments.
- Understand value and effectiveness of drug.
- Understand and predict provider patterns (e.g., 1st line vs. 2nd line treatment).

COMMERCIAL

USE CASES
- Monitor dynamics of use, patient selection, and outcomes against competitive products.
- Measure for outcomes-based contracts.
- Determine value and effectiveness to payers to support reimbursement.
- Improve patient targeting by selecting likely responders.
- Monitor safety.

Figure 1: Data uses across the life science pipeline

Offerings have started to grow around certain therapeutic areas (e.g., oncology EHRs), and healthcare analytics vendors are now expanding offerings to meet the demand for integrated data from many sources (e.g., labs, consumer behavior, and more [Figure 2]) that capture the breadth of patient health.

Figure 2: Life science companies are interested in a variety of data

As Clinical Trials Evolve, Extended RWD/RWE Becomes Paramount

The life science industry historically placed its largest bets on heavily sanitized clinical trials, as regulatory approval equated to reimbursement. As the field evolved, developers learned that the synthetic trial setting didn't reflect drug performance in the real world. When commercialized and available to larger populations, drugs may produce different safety profiles and effectiveness than in the select trial population.

The limited populations and controlled nature of clinical trials have sparked the extended RWD/RWE movement to understand patient behavior and real-world performance via data outside the clinical trial setting. And as regulators and payers adapt, the pressures have mounted to develop therapies that are safe and effective in the real-world setting.

Much of the initial evidence, however, was confined to large, but shallow, claims-derived datasets. These datasets present many challenges in the clinical trials setting:
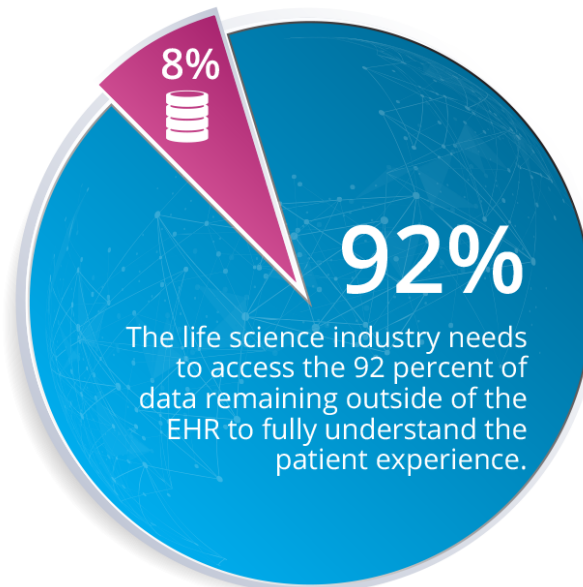
- Conversion of claims into specific visits and a clear history of the patient (coding challenges).
- Understanding completeness/incompleteness of the datasets (data quality challenges).
- Categorization of providers and locations of service (provenance tracing).
- Selecting the most useful measures of utilization and expenditures (outcomes metrics).

A few deeper, EHR-derived datasets emerged, especially for some diseases (e.g., cancer) from specialized companies or from specific regions, payers, etc. Most of these datasets, however, are not broad and deep enough and can often only be partially linked together.

Looking Beyond the EHR for Broad and Deep Data

Driving outcomes improvement requires integration of data across sources, but with much of RWD to date focused on claims and with limited EHR data, the life science industry lacks the breadth and depth to leverage RWD in drug development. As Figure 3 explains, and the experience of healthcare transformation companies confirms, only 8 percent of the needed data resides in the EHR. Having large claims data and some EHR data is only scratching the surface of true outcomes measurement and transformation work for both population health and personalized approaches.

Only 8 percent of data required for driving outcomes, population health, and precision medicine strategy resides in today's EHRs.

8%

92%

The life science industry needs to access the 92 percent of data remaining outside of the EHR to fully understand the patient experience.

Source: Alberta Innovates Health Solutions Secondary Data Use Project, March 2016

Figure 3: Only 8 percent of required data resides in the EHR

To fully understand the patient experience, life science needs to access the remaining 92 percent of data that resides in other systems. Specialty EHRs (e.g., oncology and cardiology) fill in some commercialized data gaps but miss a lot of the data that drives population health (e.g., cost, patient satisfaction, lab results, etc.). Extended RWD/RWE requires broader sources to truly understand patients; life science companies can access this knowledge by partnering with

an established healthcare transformation company. Health Catalyst, for example, has more than 200 data sources integrated within its systems. As data sources expand into patient-reported information (e.g., from patient-facing apps), integrated vendor analytics will become more critical to round out extended RWD (Figure 4).

Figure 4: Extended RWD

To produce extended RWD, a healthcare transformation company must have five key capabilities:

1. Ability to measure real world outcomes.
2. Direct and maintain trusted relationship with providers across the continuum of care, versus one specialty area (e.g., oncology).
3. Ability to bridge the gap between actionable insight and real-world action, (e.g., interventions at population health level and patient-level).
4. Access to the breadth and depth of knowledge across data, analytics, and clinical operations to tackle problems and identify actionable solutions.
5. Interdisciplinary teams to achieve measurable success with data science (e.g., subject matter experts [SMEs], data engineers, and analysts), ready-to-use technological tools and platforms and, crucially, the clinical operations experience and insight that allows true healthcare transformation and meaningful change by operating at the level of governance, culture, and process improvement.

From Insights to Real-World Action

Having RWD and RWE is only the start to driving outcomes; life science companies need the ability to act on the data by turning it into provider-level actions that benefit the patient (e.g., running a clinical study, a patient education/engagement program, an adherence program, a safety program, etc.). Partnering with the right healthcare transformation organization helps life sciences achieve real-world action by connecting companies with multiple health systems and patients, as well as the data that determines whether hypotheses are operational and lead to improvement. For example, a life science company can use shallow data to predict drug response, find patterns, and develop insights, but only when it deploys the drug in the real-world hospital setting can it understand the actual real-world impact.

Together, the life science industry and the right healthcare transformation companies can drive change, monitor, and measure drug performance. This completes the full circle of real-world action (Figure 5), from opportunity to action, with a solution that achieves five key goals:

1. Leverages extended RWD from trusted provider networks.
2. Generates extended and actionable RWE, leveraging consulting services, professional services, data science, analytics, SMEs, clinical operations and PHM expertise.
3. Identifies real-world actions for providers to test with patients (leveraging SMEs, e.g., hospital operational expertise).
4. Deploys and measures performance and refines the real-world action deployment in collaboration with providers.
5. Scales successful real-world actions across provider networks in collaboration with providers.
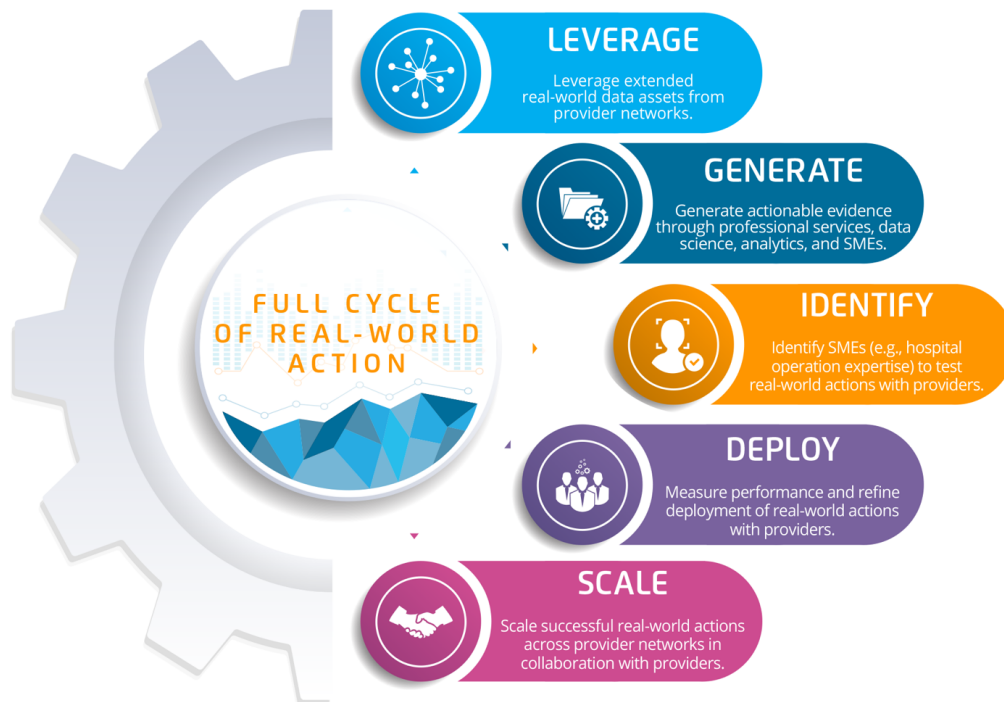

Figure 5: Extended RWD

Two Benefits of Healthcare Transformation Companies Committed to Outcomes improvements

By partnering with the right healthcare transformation company, life science companies gain two key capabilities:

1. **Core Capabilities**—Because an effective healthcare transformation company is focused around outcomes improvement, it brings essential capabilities to the life science drug pipeline. The three core capabilities of best practice, analytics, and adoption that support outcomes improvement can also serve as an effective framework in drug development.

2. **Strategic Consulting and Professional Services**—Strategic consulting and professional services offerings (e.g., SMEs, operational expertise, tech skills around analytics/data engineering, and governance) have unique data to quickly identify solutions, leverage technologies for data ingestion and visualization, and complement it with deep operational expertise to contextualize the human factors and processes that drive success. With a trusted network of providers, life science companies can merge data and professional skills and refine a solution until it can function and scale across providers.

Powered by Expanded Data, the Next Wave of Healthcare Transformation Serves All Key Industry Players

While the life science industry is accustomed to utilizing data for certain insights, its next step is to scale those insights into actions—similar to how regulators and payers have realized the value of extended RWD for key decisions concerning regulatory approvals and reimbursements. By partnering with organizations committed to healthcare transformation and leveraging extended RWD, real world insights, trusted provider networks, PHM approaches, and the definitions and real-time measurements of real-world outcomes, life science companies can achieve meaningful outcomes-driven approaches to the development, regulation, launch, reimbursement, and monitoring of new therapies.