Creating value from next-generation real-world evidence

Leading pharma companies are applying advanced analytics to real-world evidence generation to deliver impact at scale. How can leaders keep innovating, and what should others do to catch up?

by David Champagne, Alex Davidson, Lucy Pérez, and David Saunders
As the healthcare industry focuses increasingly on outcomes, pharma companies are looking to sources beyond randomized clinical trials (RCTs) to measure and demonstrate the value they bring. Real-world evidence (RWE) has been in use for decades, but recent advances in digital and advanced analytics allow it to be employed in new ways. It can help us understand how patient characteristics and behaviors affect health outcomes—thereby helping to predict the progression of a disease, a patient’s responses to a therapy, or the risk of adverse events, for instance—while also increasing the efficiency of R&D investments and accelerating time to market. For any company considering deploying advanced RWE analytics, success will depend on building the right framework and capabilities.

From table stakes to high stakes
Cost and competitive pressures, scientific advances, digital-savvy stakeholders, progressive regulatory shifts, and the increasing breadth and interoperability of data and technologies are among many trends driving participants in the healthcare ecosystem to intensify their focus on value and patient outcomes. Payers are gradually shifting to outcomes-based contracts; providers are angling to gain privileged status with them; and patients are taking more ownership of their own outcomes. In this changing environment, insights from real-world evidence are becoming more important in getting the right treatment to the right patient at the right time, measuring outcomes, and demonstrating the value of interventions. Given the significant disruption to RCTs and the need to rapidly understand burden by patient phenotype, and potential therapies for COVID-19, RWE is more in the spotlight than ever.

Pharma companies have been using real-world evidence for decades to inform their decision making, respond to requests from external stakeholders, and improve their therapies’ market positioning. More recently, growing regulatory acceptance, rising demand from payers and physicians, and increasing familiarity with digital and analytics have enabled some companies to derive much broader benefits from RWE. Examples include Pfizer’s use of electronic medical record (EMR) data in obtaining approval for Ibrance to treat male breast cancer; Roche’s use of a synthetic trial

Real-world evidence helps us understand how patient characteristics and behaviors affect health outcomes.

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arm to secure reimbursement for its lung cancer drug Alecensa; and AstraZeneca’s use of real-world data to demonstrate the real-world effectiveness of its diabetes therapy Farxiga compared to competitors.

In the past few years, the introduction of advanced RWE analytics has made real-world data an even more powerful resource for pharma companies. Unlike traditional RWE analytics—which uses descriptive analyses to characterize patients, and established matching techniques to compare groups of patients with similar characteristics—advanced RWE analytics uses predictive models, machine learning, probabilistic causal models, and unsupervised algorithms to extract deeper insights from rich data sets (see sidebar, “Comparing traditional and advanced RWE analytics”). It enables pharma companies to draw on thousands of patient characteristics to gain a better understanding of what drives outcomes, to uncover insights into drug performance and differentiation at sub-population level, to run accurate scenarios with predictive models, and to generate hypotheses at scale across multiple therapies, comparisons, and endpoints (Exhibit 1).

Exhibit 1

Advanced real-world-evidence analytics can play an important role across the pharma value chain.

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Leading organizations are already capturing value from advanced RWE through a range of applications including predicting outcomes in type 2 diabetes,5 predicting findings of an ongoing phase IV cardiovascular trial,6 and modeling the progression of non-Hodgkin’s lymphoma to predict therapy escalation.7

We estimate that an average top-20 pharma company that adopted advanced RWE analytics across its whole value chain for in-market and pipeline products could unlock more than $300 million a year over the next three to five years. A typical cost base offers scope to save $100 million in development spending through the optimization of RCT design, the use of RWE studies rather than RCTs in some cases, and the implementation of synthetic trial arms. Cost savings apart, the introduction of advanced RWE analytics could help companies identify new targets for molecules, accelerate time to market, improve formulary position and payer negotiations, and generate stronger evidence of differentiation and benefit/risk balance for in-market products. Our analysis suggests that applying these actions to key assets could generate top-line value of $200 million or more.


Such examples represent only the tip of the iceberg where RWE is concerned. Emerging methodologies—such as generative adversarial networks, federated transfer learning, time-series modeling, and “few-shot” learning—will pave the way to answering novel questions that can’t even be formulated today. Explainable AI methods that enable human experts to understand machine-learning models are enhancing transparency and understanding and catalyzing wider adoption. The explosion in data from electronic medical records (EMRs), health claims, “omics,” sensors, wearables, social media, commercial customer records, and patient-reported outcomes will prove fertile ground for new insights.

Healthcare companies should also develop a strategy to address disruption from tech giants and payers—signs of which are already emerging. Google’s engagement with one of the largest US healthcare systems to collect and analyze data from millions of patients is a sign of growing interest among major companies from outside the sector.

As leading companies deepen their analytics capabilities, they are also scaling up their ambitions beyond one-off use cases for a single brand or market. They are building data pipelines, reusable analytical assets and models, engagement platforms, and ecosystems across groups of mutually reinforcing use cases (Exhibit 2). This approach

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allows companies to integrate multiple datasets, use them to build analytical models, and then “industrialize” the models for use in a broad range of contexts. Over time, companies can embed the models in user-friendly digital tools for a variety of stakeholders, both internal (R&D, market access, medical science liaisons, and so on) and external (healthcare professionals, payers, patients, and others).

What it takes to deliver
As early adopters invest in building capabilities in advanced RWE analytics, eight dimensions are emerging as particularly important (Exhibit 3). Our research shows that companies need not excel at all eight, but should aim to build a leading position in at least a handful.

Exhibit 3
Leaders in advanced real-world evidence analytics get eight things right.

Strategy and vision
- Clearly articulated aspiration
- Link to value
- Top-down sponsorship
- Focus along value chain

Value orientation
- "Lighthouse" project to demonstrate value and galvanize organization
- Study design and methods
- Dissemination
- Impact tracking

Organization, operating model, and processes
- Organizational setup
- Governance
- Integration of evidence generation across functions, geographies, value-chain levers, and evidence types

Capabilities
- Conventional real-world evidence; epidemiology
- Health-economics and outcomes research
- Advanced analytics
- Understanding of evidence generation within functions and brands
- Use of external partners

Culture
- Belief in evidence generation
- Understanding of what it is and isn’t: perspective on where it can add value
- Perception of risks

Partnerships
- Innovative start-ups
- Academic consortia
- Analytics companies
- Regulators
- Payers
- Providers
- Patient groups

Data
- Long-term partnerships for enrichment, curation, quality control, and governance
- Classic sources (e.g., claims, electronic medical record)
- Novel sources (e.g., -omics, patient-reported outcome)

Tools and environments
- Sandboxes for experiments
- At-scale pipelines
- Repositories of analytical assets
- "Industrialized" evidence-generation engines

*Such as genomics, transcriptomics, and proteomics.
following a different approach. To galvanize their organization from the beginning, they execute a “lighthouse” use case that targets an urgent business question and demonstrates the value of RWE analytics in addressing it. Successful lighthouse projects are sponsored by senior leaders, push the boundaries on the use of innovative data and analytics, involve a broad set of stakeholders from across functions, and build enterprise-wide capabilities and reusable assets. By communicating and celebrating such projects internally and externally, companies create demand for RWE analytics from all areas of the business, enabling them to move beyond the lighthouse and drive use cases systematically across the value chain.

**Organization, operating model, and processes.** To make the most of scarce expertise, best-practice companies set up a global capability group to oversee RWE enterprise strategy, capability building, and governance. They give the group resources to coinvest in pioneering use cases with key brands and centralizing some day-to-day RWE baseload activities. They also incorporate RWE into an integrated evidence-generation process that cuts across functions, asset life-cycle stages, evidence types, methodologies, and global, regional, and country-level needs. In addition, they often create a new role to liaise between RWE and key brands and R&D, charged with identifying opportunities, shaping a portfolio of work, and challenging brands and functions to adopt innovative approaches. The ideal postholder will have business acumen (ideally gained inside the company), brand smarts, medical and analytical knowledge, communication skills, project-management capabilities, an entrepreneurial mindset, and political savvy, plus at least ten years’ industry experience across functions.

**Capabilities.** When companies first introduce advanced RWE analytics, they often get excited about novel modeling approaches and hire a bunch of data scientists. But to deliver at scale, they must also build standardized, reusable data substrates from multiple different sources and design factory-style platforms for handling automated evidence generation—tasks that require them to hire data engineers and machine-learning specialists as well. To inject medical, clinical, epidemiological, and business rigor into every process, they also need “translators” who understand how RWE operates and delivers value. They act as intermediaries between colleagues working in business, scientific, and methodological areas and those working in data engineering and data science, helping to convert business requirements into executable directives for the technical team and interpret the team’s outputs into material that business stakeholders can engage with. They also help to ensure integrity, quality, and transparency in a field that has yet to earn the trust that external stakeholders routinely place in registries and RCTs.

**Culture.** Adopting advanced RWE analytics at scale requires two significant mindset shifts. First, RWE and other post-approval evidence must be treated not as a back-up to evidence generated from RCTs but as a key element in strategic discussions and development programs from the outset. Second, the move from conventional to advanced analytics requires a willingness to go beyond established epidemiological methodologies, adopt novel approaches, and accept a degree of risk in trying new things. Both shifts call for sponsorship from top executives and leadership from managers at all levels with the mandate and conviction to drive change.

**Partnerships.** Developing the scarce skillsets for delivering innovative RWE analytics can take years. Building long-term partnerships with a small number of analytics companies, designers, academic consortia, and innovative start-ups can offer a faster alternative route to access capabilities. In the most effective partnerships, external

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specialists don’t simply do the work and hand over deliverables but are offered incentives to build capabilities inside the pharma company’s organization. To help shape thinking and advance the field, pharma companies also need to engage stakeholders including regulators, payers, health technology assessment (HTA) bodies, healthcare providers, and patients.

Data. Building a network of relationships with carefully chosen data providers helps pharma companies secure privileged access to data and develop proprietary enriched data sets to answer business-critical questions about key assets. To do this, companies scan multiple market landscapes to identify emerging data generators and aggregators, develop a clear process for acquiring and accessing data, and adapt their enterprise governance to support collaboration. Some companies are starting to link RWE datasets with their own data while reanalyzing their RCT data in parallel to build a comprehensive yet granular view of the effectiveness and safety of their therapies.

Tools and environments. At a minimum, companies that aspire to scale up their RWE analytics need a “sandbox” for conducting basic experiments with use cases and delivery models. More advanced companies use scaled-up cloud platforms to build automated pipelines, repositories of analytical assets, and visualizations for use by multiple stakeholder groups. A few companies go further still by building platforms that generate evidence across indications, therapies, and use cases and allow hundreds of analyses to be run across multiple patient outcomes and thousands of sub-populations. These evidence-generation engines deploy advanced and traditional RWE analytics side by side to derive insights into disease biology, unmet needs, real-world therapy usage, safety and effectiveness, drivers of healthcare professionals’ choices, and other factors to inform decision making (Exhibit 4).

Exhibit 4
Creating value at scale requires an evidence-generation engine.

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<th>Plans</th>
<th>Engine</th>
<th>Insights</th>
<th>Stakeholders</th>
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<td>Biological-pathway insights</td>
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<td>Granular view on response and unmet need at subpopulation level</td>
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1Randomized clinical trial. 2Electronic medical record. 3Patient-reported outcome. 4Such as genomics, transcriptomics, and proteomics. 5Health-technology assessment.
The evidence generated in this way is also used in head-to-head comparisons of effectiveness and safety, cost-effectiveness analyses to support submissions to payers and regulators, and other forms of communication with external stakeholders. Last but by no means least, evidence from the engines can be used to improve patient outcomes. Predictive models of patient-level outcomes can be used in clinical decision support, for example, while segmentation can be used to get the right drug to the right patient at the right time.

In combination with tech-enabled planning to integrate evidence generation across functions and brands, these engines will transform RWE from a source of insights for medical affairs to a pillar of corporate strategy and a key part of the value chain. Companies that lack this vision could struggle to compete with others that are able to base their decisions on richer insights generated at a fraction of the usual time and cost.

By taking full advantage of real-world evidence and advanced analytics, pharma companies can accelerate their shift from product-focused to patient-focused organizations. A few leaders have drawn up a blueprint for execution. Now it is time for the rest of the industry to set its sights on the next horizon of evidence-generation capabilities.

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