WHITE PAPER

Making Real-World Evidence Real

How life sciences companies can generate new insights about their therapies in action

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Contents

Introduction

Imagine: Your pharmaceutical or medical device has been tested in carefully designed clinical trials and shown to meet required standards of efficacy and safety. It has gained regulatory approval and has gone to market. Now you want to know, does it measure up to its promise when used *in the real world*? Does it materially improve patient outcomes? Does it outperform alternative therapies? Does it achieve these outcomes at an appropriate value? Do you know which patients are most likely to benefit from it? Can you prove it, with statistical rigor?

As the health care system moves toward a patient-centric, value-based approach, questions such as these are getting heightened scrutiny from all angles – from regulators, payers, providers and patients. And they should. Real-world evidence (RWE) can provide new insight into the benefits, risks and cost effectiveness of medicines and medical devices in actual use – evidence that can enable life sciences companies to develop better therapies, prove the value and differentiate the brand in the market.

RWE lets investigators see how medicines perform without their training wheels on – how doctors use them in the frantic setting of primary-care offices, and how patients use them when they are not screened for age, weight, education levels and willingness to comply with instructions.

Several factors have aligned to make RWE imperative and practical. The steep rise in the cost of drugs, pitted against finite health-care budgets, keeps payers tossing and turning nightly. New medicines – some for life-threatening diseases – cost tens of thousands of dollars a month. Many of these medicines prolong lives, but in many cases, the data from conventional trials are ambiguous, and it is hard to determine if the medicines are providing real value relative to their stratospheric costs."

You could knock heads with insurance companies trying to oppose their coverage limits for such treatments, or you could partner with them to determine exactly which patients will benefit and make a compelling case. Prove the value, and you're in. The most successful life sciences companies will be the ones that can convince their customers – patients, health care professionals, government authorities and health plans – that new treatments are the most effective and provide true value compared with alternatives. Using medical treatments more effectively could save hundreds of billions of dollars a year by some estimates. Providers and payers will take notice.

The Gap Between Real-World *Data* and Real-World *Evidence*

In spite of the growing quest for real-world evidence, life sciences struggle to put vision into action. It's not for want of the real-world data to base it on. De-identified patient data from electronic medical records, national payer databases, insurance claims, disease registries, observational studies and other sources is waiting to be tapped. And there are novel data sources, such as social media. For instance, PatientsLikeMe, the online community of thousands of patients, has cooperated with life sciences companies and providers to help understand patients with various conditions and how therapies worked for them.

The opportunity is at hand to gain insights from tens of thousands of patients, as opposed to the limited population of a clinical trial. The life sciences industry gets it. Said one respondent in a SAS-sponsored study, "As we go demonstrating overall value of the product … real-world cost assessments, total cost of care … it has to revolve around real data, not just modeling data."[1]

However, transforming real-world *data* to real-world *evidence* has its challenges:

- **The sheer volume of data has been problematic**. In the past, it could take weeks or months to find, access, process and eventually analyze the vast tide of relevant data sources. Inefficiency in data management processes caused delays in discovering what you might need to know quickly, such as a looming safety concern.
- **There aren't enough experts to deal with the complexity**. Many companies struggle to generate even simple analyses from large data sources with their current analytical tools (Excel spreadsheets are commonplace). Real-world data sources have gotten so large and complex that many statistical software products just can't handle them, and there aren't enough data specialists to wrangle them.
- **Outsourcing is no panacea**. Since health economics and outcomes research (HEOR) departments tend to be lean, a lot of work is outsourced to external vendors, particularly for large, long-term projects. Internal users express frustration about their vendors using different tools or delivering lengthy results that are difficult to understand and use.

  Said one respondent in a SAS-sponsored study: "There is a massive report. … We've got reference tables in PDF, Excel sheets with different levels of information. There must be 20 spreadsheets, and I have no idea what to do with it. This is of no use to me. The report is so massive that I don't know where to start with it – 327 pages. I've got this thing that costs €250,000, and we have reams of Excel spreadsheets and reports and … we don't know where to start with extracting it."

The full potential of real-world evidence is often lost in fragmented tools, lack of data integration, shortage of analytic skill and an organizational structure that creates pockets of understanding rather than enterprise-level perspective.

[1] *SAS HEOR Market and Environmental Assessment: Qualitative Interviews*, Andrew Schafer and Rebecca McAvoy, ISR Reports, January 2015.

- **Target audiences have vastly different needs**. First you have your super users – the data scientists, statisticians or other data- savvy users who are proficient in programming and analytical skills. Then there are business users who may have a good deal of medical knowledge but won't know how to create data management routines, models or custom reports. And then there are executive users who want little interaction with the underlying system but must be able to answer questions on the fly through compelling visuals displayed on their laptops or smartphones.

The right system capabilities, the right user interface and the right output will be quite different for each type of user. That is, assuming you want to democratize the data and give decision makers hands-on access to real-world evidence (and you do). However, that's not what you're likely to see today. Here's a more typical scenario:

> A health economics team is meeting with a commercial brand team and the clinical R&D team to develop a strategy for a certain compound. A dozen or more stakeholders are gathered in a conference room, discussing a PowerPoint presentation.
>
> It becomes clear that a different analysis or new data set is required. What now? Part of the discussion is tabled until data specialists can curate the data, create the required analytics, and come back with new answers. The delay could be hours, days or weeks – and it could be followed by more cycles of the same.

Now imagine empowering these teams to interact with the data on the fly, without tasking statistical programmers or data scientists to go away and come back later with results. Imagine if the participants in that meeting could create cohorts of patients from a few different data sources and view the results on demand – or create various tabulations and basic statistics to describe different scenarios. With a self-service RWE platform, cross-functional teams could cycle through many different hypotheses, perform many different analyses and have the results available on demand – not in days or weeks.

Leading data management capabilities ease the time-consuming burden of gathering and preparing real-world data for analysis. The organization spends less time preparing the data and more time learning from it.

Prerequisites of a Real-World Evidence Information System

Technology was formerly a stumbling block to the ideal of real-world evidence. But not anymore. The era of big data has unquestionably arrived in medicine. The volume, variety and velocity of medical data are increasing; organizations are collecting more data from a wider variety of sources at greater speed every day. Modern computing platforms are keeping pace with the requirements of digesting all that data to deliver real-world evidence more efficiently and at lower cost.

The ideal technology platform provides the following capabilities:

Data management. The system gathers relevant real-world data from a strong library of sources and vendors. This could include internal or third-party data sources from point-of-care systems, electronic medical records, insurance claims, patient-reported outcomes, trusted third-party data providers, etc.

The goal is not to directly integrate these data sources, but to have an automated process to prepare the data from various sources and map it to a common data model so it can be compared side by side. Data should be updated regularly, with a record of what has changed.

Analytics and visualization. Once the various data sources are available in the platform, they should be easily accessible via a user-friendly interface. An analytics library of prepackaged, fit-for-purpose analytical methodologies should span from simple descriptive statistics to predictive analytics to machine learning methods.

Users can explore, visualize and report on real-world data sources to generate insights to support decisions on brand strategy, treatment regimens, pricing, reimbursement, formulary access and so on. The analytics shopping list might also include comparative effectiveness, health care utilization and cost metrics, sample size estimation routines for large pragmatic trials, and propensity score matching routines for cohorts.

Your organization's analysts may have built many of these analytical applications with their own algorithms, but are those solutions well-managed and scalable across the enterprise? This is precisely what a strong real-world data platform must be able to deliver.

User-appropriate interfaces. The right information platform democratizes access to data and satisfies many different types of user personas. Those cross-functional teams in the conference room should not be left to wait while the data gurus go off to do something relatively simple, such as create a cohort of patients or update basic visualizations. Ideally, almost anyone with a business question – even someone with little or no analytical background – can use features such as on-the-fly forecasting, auto-charting, "what does it mean?" and drag-and-drop capabilities.

Massive, scalable computing power. Navigate and explore massive data sources with little or no lag time from a point-and-click interface. With high-performance computing, calculations on millions of rows of data can happen in seconds, rather than minutes, hours or days. To make blazing speeds possible:

- *Grid computing* provides a high-tech twist to the adage, "many hands make light work," enabling multiple applications and users to share a network of commodity hardware for fast performance.
- *In-database analytics* moves relevant data management, analytics and reporting tasks to where the data resides, to speed time to insight, reduce data movement and promote better data governance.
- *In-memory analytics* enables the system to quickly solve complex problems without having to go back and forth to disk for repeated data access.

Cloud-readiness. A real-world evidence system can be deployed on a dedicated high-performance analytics appliance, on the existing on-site IT infrastructure or in the cloud – whichever best serves the organization's big data requirements. With the cloud, one or more key elements of the data and analytics platform – data sources, data models, application processing, computing power, analytic models, and sharing or storage of results – reside on shared infrastructure managed by a cloud service provider. Your teams can focus on RWE initiatives instead of on managing on-site technologies.

The latest visualization techniques go far beyond traditional query and reporting, providing unprecedented ability to visually explore data sets of any size quickly and efficiently and create meaningful reports to share via mobile devices.

"With the new infrastructure, a user can query vast amounts of data and generate results rapidly, without affecting the performance of other users. Queries that used to take days now take minutes."

–John Carew,
Assistant Vice President,
Advanced Analytics
Carolinas HealthCare
System

Closing Thoughts

The business of life sciences R&D is fundamentally shifting – from a primary focus on regulatory approval to a broader view on widespread adoption by providers and payers… from showing the value in controlled, isolated clinical settings to proving it in large target populations in real-world conditions.

Real-world evidence helps identify which patients will get the most benefit and value from a therapy, based on genetic, social and lifestyle attributes that might not be captured in clinical trials. It provides a clear picture of a product's safety, effectiveness, economics and value in actual use. It provides a deeper understanding of epidemiology trends and disease management. And ultimately, it provides insights that lead to better products that advance the "triple aim" – better care, better health and better costs.

Even though it requires enormous volumes of data, this kind of analysis is not only possible, it's possible on the fly. The technology building blocks are all available today:

- The means to bring diverse data together via a common data model that relieves the burden of loading, integrating and managing data from multiple sources.
- Fit-for-purpose analytical applications, such as a cohort builder, that enable business users to access at least some level of analytics without having to rely on programmers or data scientists.
- Scalable, high-performance computing power that delivers answers from big data in seconds or minutes, rather than hours or days.
- The option to move that computing and analytical power to the cloud, so life sciences organizations can focus on generating insights, not on the care and feeding of information technology.

Everybody stands to win when analysis goes out of the wind tunnel and into the clouds.

For More Information

SAS® solutions for life sciences

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