KEY TAKEAWAYS

How to use EHR-based real world data to demonstrate value

Key Takeaways from the 2019 Cross-Industry Value Summit Workshop

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About the 2019 Cross-Industry Value Summit

Value-based care.

Fee-for-**value**.

Value-based contracts

In 2019, “value” may be the most overused yet least agreed-upon term in our health care lexicon. On the surface, most stakeholders align on an aspirational definition of value: the right treatment for the right patient in the right setting at the right time. But legacy mistrust, siloed perspectives, and competing incentives often impede the advancement of common definitions or shared frameworks for truly assessing value in U.S. health care.

In an effort to break down industry barriers and encourage honest dialogue, Advisory Board hosted its first **Cross-Industry Value Summit** on September 23–24, 2019. The Summit convened 40 medical and pharmacy executives from payer, provider, and life sciences organizations – as well as thought leaders who influence how we think about value. Through panels, networking sessions, and expert-led workshops, participants discussed how they define, measure, and drive medical value. They also identified ways to build trust and transparency around both evidence generation and broad models for cross-sector collaboration.

About this workshop

Payers, providers, and patients want more than the FDA standards of safety and efficacy to **justify the value of drugs and devices**.

As new sources of data proliferate they expect actionable evidence. They want to know the value of treatments for specific subsets of patients in order to support formulary and purchasing decisions, clinical guideline development, and decisions at the point of care. With the bar for medical evidence rising, EHR-based real-world evidence (RWE) is widely purported as a promising tool for delivering on those demands.

Despite this demand for evidence that supports decision making in real-world contexts, those same evaluators—and the FDA—struggle to accept RWE as a reliable tool to demonstrate medical value. Most health care leaders still view randomized controlled trials (RCTs) as the pinnacle of rigorous clinical inquiry, regardless of their well-documented and inherent limitations. And until more stakeholders understand and acknowledge the relative merits of RWE, its impact will remain unrealized.

To better understand the **challenges and opportunities around EHR-based RWE**, we convened a cross-industry cohort with leaders from provider, manufacturer, payer, and evaluator organizations for a candid discussion.

The perspectives of participants were diverse: some were intimately involved in evidence generation activities at their organizations, and had direct experience with RWE. Others had experience evaluating RWE as part of third party technology assessments. Some were clinicians, acutely familiar with the richness of information captured in EHRs, while others were seeking to understand the relative merits of EHRs versus other sources of real-world data. The conversation was dynamic, centering on current barriers to using EHR-based RWE, and what medical leaders can do today to overcome them.

Read on to see what we learned

Key **takeaways**

1. Randomized controlled trails (RCTs) are the gold standard for medical evidence, but they’re far from perfect, and EHRs can help.

2. To date, use of EHR-based RWE has been limited to demonstrating safety and efficacy, not value.

3. EHRs can better demonstrate value by answering three questions: what is the impact, compared to what, and for which patients?

4. The primary challenge limiting progress on RWE is not operational or technical deficiencies, it’s that stakeholders don’t trust the methodology.

5. To drive acceptance, medical leaders must evangelize RWE internally and externally, rather than wait for the FDA to do so.

RCTs are the gold standard for medical evidence, but they're far from perfect, and EHRs can help.

Randomized controlled trials (RCTs) continue to command a vast majority of life science firms' time, effort, and resources, but there is a lot wrong with them. **Almost all key decision makers—from payers to providers to HCPs—consider RCTs to be the only robust and legitimate source of evidence, but the evidence is usually only meaningful for regulators.** Most trials finish late and over budget, and still only include a subset of populations affected by the disease. Of course, the root causes of these challenges are deeply complex, multi-faceted, and multi-stakeholder. Summit participants discussed several factors underpinning the inefficiencies of RCTs, including most notably, misaligned stakeholder incentives and a poor patient experience.

EHRs are widely touted as a promising tool to improve clinical studies—and for good reason. Despite the limitations that come with unstructured data sources, **EHRs contain a wealth of clinically rich information compared to claims or pharmacy data.** EHR data is also viewed as more trustworthy than data generated from unvalidated sources like apps and wearables, because it is entered by a trained, clinical professional. Moreover, recent FDA guidance to expand the use of RWE in regulatory decision making specifically cites EHRs as an integral part of that effort. Not surprisingly, investments in EHR-enabled RWE are growing, from public funds and universities as well as private investors.

Source: Advisory Board research and analysis.

Key challenges in generating evidence through clinical trials

Study design	Patient recruitment	Study conduct	Analysis/ reporting	Study acceptance
• Creating diverse patient cohorts • Developing patient-centric endpoints	★ Identifying patients that meet inclusion and exclusion criteria • Generating patient awareness	• Integrating with physician workflows ★ Minimizing patient drop-off	• Addressing missing or poor quality data • Accounting for real-world variation in clinical practice	• Tailoring to evaluator-specific questions • Extrapolating to specific populations

DATA SPOTLIGHT

70% of clinical trials miss their deadlines, with patient enrollment as most cited cause

85% of patients want doctors to tell them about trials relevant to their disease

Source: Advisory Board research and analysis; Digital Research Network leaders and developers; Tufts Center for the Study of Drug Development (Jan/Feb 2014), 15(1).

Key challenges in generating evidence through clinical trials (cont.)

★ Provider incentives can hurt RCT recruitment.

Providers' commonly cited motivations for participating in clinical research—institutional reputation, downstream revenue, access to new therapies, and physician engagement—can create incentives and behaviors around patient enrollment that undermine manufacturer goals.

For example, because provider sites compete with one another for selection by manufacturers or CROs, there's an incentive to over-estimate the number of eligible patients they could recruit. And because of pressure to meet enrollment targets that may be artificially high, physicians might only be inclined to drive patient awareness for studies they are directly involved in.

★ Poor patient experience exacerbates drop off.

Even when trial enrollment targets are hit initially, patient drop off is a big problem. In fact, drop off for Phase 3 clinical trials is often as high as 30%. At the summit, stakeholders shared how poor experiences like stressful appointments or insufficient responses to patients' concerns can push trial participants away. But stakeholders often don't have the time or insight to meaningfully improve patient experience, especially given pressure to optimize trial operations and recruitment.

However, new tools like TrialScout, sometimes called "the Yelp of clinical trials", are emerging to help patients compare trials by hearing directly from other participants. These tools could pressure manufacturers, CROs, and providers to re-prioritize patient experience. They could also serve as a key source of real-world data by disentangling drivers of poor patient experience that stem from trial operations vs the treatment itself.

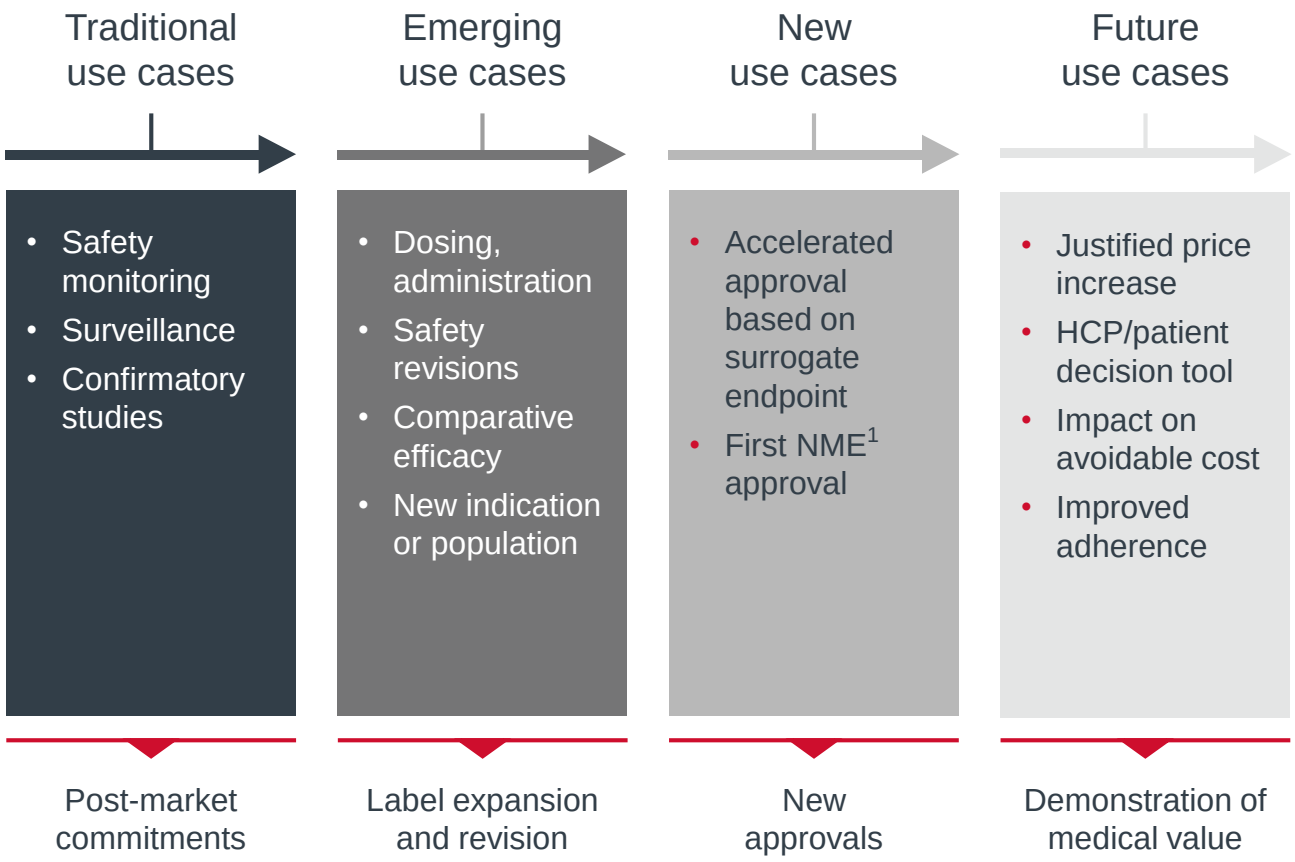
Source: Advisory Board research and analysis; Digital Research Network thought leadership pieces (accessed at https://www.optum.com/campaign/ls-ms/optum-drn/thought-leadership.html); The Prevention and Treatment of Missing Data in Clinical Trials, N Engl J Med 2012; 367:1355-1360; https://www.trialscout.com.

To date, use of EHR-based RWE has been limited to demonstrating safety and efficacy, not value.

Industry experience with RWE so far has focused on regulatory requirements for surveillance and safety. Although life science companies have recently used EHR data to support label expansion and new drug applications (NDAs), it's still with a limited scope. Approvals typically focus on rare diseases or populations with high unmet medical need, and tend to rely on synthetic control arms to prove safety and efficacy. For example, Roche achieved accelerated FDA approval for Alecensa in 2015 for patients with ALK-positive NSCLC using oncology EHR data from Flatiron.

Source: Advisory Board research and analysis; Genentech press release, December 2015 (accessed at https://www.gene.com/media/press-releases/14615/2015-12-11/fda-grants-genentechs-alecensa-alectinib); "Synthetic control arms can save time and money in clinical trials," *STAT News,* February 2019; "Roche pays $1.9 billion for Flatiron's army of electronic health record curators," *Nature Biotechnology*, 2018.

Industry experience with RWE to date centers on regulatory requirements



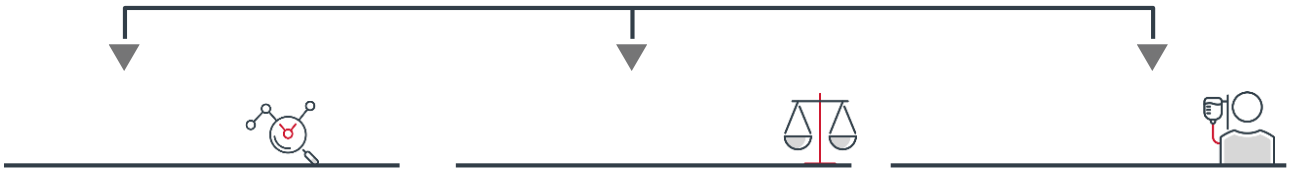
Source: Advisory Board research and analysis.

EHRs can better demonstrate value by answering three questions: what is the impact, compared to what, and for which patients?

Beyond safety and efficacy, EHR-based RWE can support more meaningful demonstrations of value that resonate with a broader set of evaluators by answering precise questions around treatment impact, comparators, and appropriate patient cohorts.

Framework for demonstrating value using EHRs

EHRs can help answer three value questions



	Impact	Comparison	Cohort
EVALUATOR QUESTION	What is the impact and value of the treatment?	How does the treatment compare to alternatives?	For which patients is the treatment most effective?
HOW EHRs HELP	• Wider array of clinically-rich data • Patient-centered value indicators	• Outcomes analysis across therapies, regimens, care sites • Detailed cost-effectiveness info when combined with cost/claims data	• Specific appropriate use criteria • Precision patient recruitment

Source: Advisory Board research and analysis.

EHRs contain clinically rich and often patient-reported information, like pain levels, side effects, or feelings of uncertainty that may be excluded from other data sources. This means EHRs can provide HCPs and patients with a more detailed, patient-centered view of treatment impact. Similarly, EHRs can enable payers and providers to make more precise comparisons of cost and clinical efficacy because they capture nuance that is lost in most claims codes, like which specific device or drug was used, or where care took place.

This kind of rich, nuanced information means EHRs can also help indicate which patients will benefit most from a given treatment. Cohort-specific EHR analyses help payers make informed utilization management decisions by understanding appropriate use. These analyses can also support development of clinical guidelines and treatment pathways. In addition to demonstrating value for marketed products, EHRs can help manufacturers and providers better identify patients who meet eligibility criteria for pre-launch trials, thus improving study efficiency and helping create more diverse, representative patient cohorts.

Source: Advisory Board research and analysis.

The primary challenge limiting progress is not operational or technical, it's that stakeholders don't trust the methodology.

Of course, there's a long way to go before RWE reaches the level of widespread acceptance afforded to RCTs. First, a host of operational issues, including interoperability, privacy, validation, and data integrity limit the appetite to invest in RWE. But aside from operational issues, much of the challenge for RWE centers on a chicken and egg problem: on the one hand, many evaluators think RWD is too messy, unrigorous, and therefore risky, to be seriously considered in decision making; on the other, investing in the capabilities to make RWD less messy requires confidence that evaluators will actually accept RWE.

DATA SPOTLIGHT

12 out of 13

Summit participants said their personal understanding of real-world evidence vastly exceeds that of their organization, and/or their leadership team

Many payers, providers, and other evaluators have a fundamental misunderstanding about RWE. They think it is only retrospective and observational. And they're not alone, even some senior leaders at life science organizations resist embracing RWE, pushing instead for "hypothesis-driven" research. At the Summit discussion, 12 out of 13 medical leader participants indicated their own understanding of RWE vastly exceeded that of their organization.

Since RCTs are in some ways falsely held as the gold-standard, many healthcare leaders fail to understand or acknowledge the shortcomings implicit in RCTs that limit how well they represent real-world efficacy. So the real challenge may not be about the investment required to build better RWE, but the effort required to educate key decision-makers about *why* RWE is equivalent, or in some cases, better than RCTs at demonstrating value.

Source: Advisory Board research and analysis.

To drive acceptance, medical leaders must evangelize RWE internally and externally, rather than wait for the FDA to do so.

The potential for EHR-based RWE to transform how we define, demonstrate, and create medical value for patients is significant, but it starts with evangelizing RWD and RWE across the industry. Despite what the FDA's guidance says about embracing RWE or where the current Commissioner's opinions fall, payers, providers, third party evaluators, and even departmental decision makers at the FDA will have their own conceptions (and misconceptions) about what kind of evidence is credible and useful.

Medical leaders must therefore act as educators and ambassadors for RWE, both inside their organizations and externally. Given their medical expertise and understanding of evidence generation, they are well positioned to make the case to senior leaders for investment in the data and capabilities to improve RWE. But medical leaders must also find opportunities to participate in the public dialogue on medical evidence in order to improve external decision makers' literacy around RWE.

To shift industry perception of RWE as a credible source of medical evidence, medical leaders should:

- Acknowledge decision makers' concerns about RWE, but put concerns in the context of shortcomings and biases that come with any data source or study design (especially RCTs)
- Eliminate concerns about manufacturer bias by ensuring transparency around study results, even if it means publishing data that paints a negative or neutral picture about a product
- Design studies that not only inform, but directly answer actionable questions that will inflect treatment and coverage decisions

Source: Advisory Board research and analysis.

What's **next?**

Check out advisory.com for additional research:

- How medical leaders can improve value using big data and AI right now
- Redefining HTAs: How thought leaders are rethinking value
- 4 'elephants in the room' hindering health care's drive toward value
- For more information about Advisory Board's 2019 Value Summit, visit advisory.com/valuesummit2019

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2. The evolution of value definitions and frameworks
3. The future of medical strategy and operations

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