Participants in the 2012 Dialogue Series /

Riaz Ali, MPP (Moderator)
Avalere Health, LLC

Tanisha Carino, PhD (Moderator)
Avalere Health, LLC

Sung Hee Choe (Moderator)
Avalere Health, LLC

Reginald Williams (Moderator)
Avalere Health, LLC

Philip Alberti, PhD
Association of American Medical Colleges

Jeff Allen, PhD
Friends of Cancer Research

Scott Allocco
BioMarker Strategies

Roy Beveridge, MD
McKesson

Marc Boutin, JD
National Health Council

Randy Burkholder
Pharmaceutical Research and Manufacturers of America

Gail Cawkwell, MD, PhD
Pfizer

Mary Davis, MD
Dean Health Plan, Inc.

Andrea Douglas
Pharmaceutical Research and Manufacturers of America

Jacqueline French, MD
NYU Comprehensive Epilepsy Center

Todd Gillenwater
California Healthcare Institute

Scott Gottlieb, MD
American Enterprise Institute

Tony Hebden, PhD
Bristol-Myers Squibb

Brad Hirsch, MD, MBA
Duke University

David Hirsch, MD, PhD
Longitude Capital

Sharon Isonaka, MD, MS
Amgen

Zeba Khan, PhD, MS
Celgene

Jonathan Leff, MBA
Previously Warburg Pincus

John Martin, MPH
Premier, Inc.

Bill McGivney, PhD
McGivney Global Advisors

Peter Neumann, ScD
Tufts Center for the Evaluation of Value and Risk in Health

Angela Ostrom, JD
Epilepsy Foundation of America

Than Powell, MBA
GlaxoSmithKline

Amy Comstock Rick, JD
Parkinson’s Action Network

Amy Rudolph, PhD
Novartis

Alan Rosenberg, MD
WellPoint

Murray Ross, PhD
Kaiser Permanente

Matthew Rousculp, PhD, MPH
GlaxoSmithKline

Hemal Shah, PharmD
Optimer Pharmaceuticals, Inc.

Alan Sokolow, MD
AccelusHealth Partners

Katy Spangler
University of Michigan

Sean Tunis, MD
Center for Medical Technology Policy

Harlan Weisman, MD
And-One Consulting, LLC
Advances in pharmaceutical treatments have transformed care for many diseases. At the same time, rising costs are leading to calls for greater value in healthcare and the generation of evidence to inform the decisions of purchasers, clinicians, and patients. This has brought to light the tension between pursuing value and supporting continued investments in new products that address the unmet needs of patients. Resolving this tension represents a central challenge for policymakers. While it is not a new issue, mitigating this tension is important to consider in light of several key forces reshaping our healthcare system:

- Growing margin pressures for government and insurers to offer affordable coverage;
- Increasing consolidation of healthcare payment and delivery and efforts to eliminate inefficiencies;
- Expanding government and private-sector investment in clinical research and data infrastructure intended to support research and decision-making; and
- Developing recognition of the need to engage patients in all aspects of research – from generation to translation.

Increasingly, payers and policymakers are calling for new approaches to support value-based decision-making. However, the issue of how to drive value in ways that align with innovation in life sciences (and, equally important, with the value perspectives of patients and providers) has received little attention. Despite the growing pressure to demonstrate value in healthcare, relevant stakeholders frequently cannot reach consensus on what constitutes “value” and how to support it. These challenges can be particularly acute in relation to biomedical innovation, for which understanding of clinical and economic value evolves over time and varies among and between different stakeholders. This tension is likely to intensify in coming years as a growing number of targeted therapies emerge from the field of personalized medicine. The role of the government in driving value in healthcare also remains in flux, as illustrated by language in the Patient Protection and Affordable Care Act (ACA) prohibiting the Centers for Medicare & Medicaid Services (CMS) from applying cost-effectiveness thresholds to Medicare decision-making. In recent years, the demand for evidence has grown, particularly in the post-market environment. At the federal level, policymakers have been more and more interested in how to generate evidence on the relative risks and benefits of alternative clinical or healthcare interventions, known as comparative effectiveness research (CER) to guide the decisions of patients and clinicians. The creation of the Patient-Centered Outcomes Research Institute (PCORI) by the ACA reflects this interest and will spur continued focus on CER in the years ahead. Finally, emerging provider-level incentives such as performance bonuses and those embedded into accountable care organizations (ACOs) will further catalyze demand for evidence that demonstrates added clinical value of novel treatments. For the life sciences industry, these developments create new imperatives to ensure that policy proposals for value-based care are aligned with the dynamics of and incentives for innovation and that research investments adequately capture new definitions of value and anticipate how evolving provider and health plan performance metrics may influence product uptake and use.

Over the past decade, the pharmaceutical industry has sought to recalibrate development and commercialization approaches and processes for new products. These efforts have included greater and earlier engagement with public and private payers; investments in CER to better produce credible, relevant, and timely research; and reinforcing its role as a trustworthy and full partner to patients, clinicians, payers, and government in the pursuit of quality improvement and value. At the same time, the sector has resisted calls for greater centralization of authority to define product value, arguing that experience in other countries illustrates the potential negative impact that centralized value thresholds can have on product developers and patients alike.

In the growing debate over value-based healthcare, it is critically important to understand the interplay of payer policy, patient engagement, incentives for innovation, and the processes that drive scientific and clinical progress. Yet the interaction of these dynamics is not well understood, and key questions about how to align demands for value with the process of, and incentives for, continued pharmaceutical innovation remain unaddressed. Given this tension, Avalere convened a dynamic group of senior healthcare leaders in a Dialogue series over two days in July and October of 2012 to:

1. Advance a common understanding of the dynamics facing pharmaceutical innovation in light of an advancing value-based healthcare system, including the role of government, the private sector, patients, and clinicians;

2. Establish a mutually agreed upon ideal future state for how the pursuit of investments in new medicines can continue in light of the cost pressures facing payers, employers, and patients; and

3. Identify a core set of solutions—policies and research—that have broad buy-in across stakeholders and will support greater collaboration and advocacy in future years.

Leaders representing patients, product developers, providers, payers, and the research community identified key domains in which stakeholders can work together to attain a vision for a healthcare system that rewards innovation and delivers effective, efficient, and equitable care to patients. Attendees worked together to prioritize several solutions within each domain, based on whether the solutions embodied eight key principles that attendees viewed as important and the estimated timing and magnitude of impact on innovation.

Table 1: Four Domains and Consensus Solutions to Drive toward an Ideal State

<table>
<thead>
<tr>
<th>GOAL</th>
<th>DATA INFRASTRUCTURE</th>
<th>FRAMEWORKS FOR EVALUATING EVIDENCE</th>
<th>EVIDENCE COMMUNICATION</th>
<th>COVERAGE AND PAYMENT</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Allows for access to the full array of data sources and generates answers to questions about treatments on a real-time basis</td>
<td>Reflect stakeholder consensus on what constitutes high-quality evidence and provide greater transparency and predictability for researchers</td>
<td>Ensures that all actors judging the value of new technologies receive information based on high-quality and timely evidence</td>
<td>Facilitates incentives that are responsive to the evolving evidence base for treatments and standards of care</td>
</tr>
<tr>
<td>SOLUTIONS</td>
<td>Invest in standardized data, Create a reciprocal data access network, Develop unique patient identifiers</td>
<td>Enable new platforms for multi-stakeholder evidence need discussions, Create tools for evaluating evidence frameworks, Integrate patient perspectives in the research process</td>
<td>Develop and disseminate best practices in evidence communication, Establish guidelines for incorporating new information, Involve patients in translation and dissemination, Advance methods for communicating uncertainty about benefits and risks, Develop a safe harbor for multi-stakeholder scientific</td>
<td>Enhance pathways for innovation in novel provider payment models, Ensure transparency of incentives in clinical pathways</td>
</tr>
</tbody>
</table>

The Appendix includes a full listing of attendees of both of these events.
During the Dialogue Series, several important themes emerged:

- **Partnership and collaboration** are critical across all domains and necessary to ensure all participant needs are addressed when implementing solutions.

- Regarding **data infrastructure**, solutions that align evidence and innovation must be built on a foundation of robust data networks for continuous learning. Yet considerable challenges exist to make this vision a reality, which will require a long-term investment.

- It is important to improve **frameworks for evaluating evidence** by developing better tools and procedures for transparent, predictable, and appropriate evaluation of evidence by healthcare decision-makers. Yet given the plurality of perspectives and decision-makers and the dynamic nature of innovation, evaluation frameworks must be flexible, accommodate uncertainty, and reflect an understanding that value evolves over time and varies among stakeholders.

- **Communication** is a cross-cutting domain that will shape how innovation is valued. Timely communication of relevant, accurate information to patients and providers represents an essential element of aligning evidence demands with innovation. Solutions must be developed to ensure communication is appropriately balanced with the most up-to-date and relevant content that meets the needs of a diverse group of stakeholders, including patients, payers, and policymakers.

- Regarding **coverage and payment**, actors have faced challenges in creating centralized coverage policies to nimbly accommodate and manage the diffusion of new technologies. With the shift of financial risk away from traditional insurers to other types of entities, such as ACOs, new payment models must be transparent and flexible enough to maintain clinicians’ and patients’ ability to choose treatment options for individual needs.

This paper describes the current landscape for pharmaceutical innovation and memorializes the output of our Dialogue series. While this paper does not seek to present comprehensive solutions to the complex challenges in this area, this document provides a foundation for consensus-based solutions that can be applied to support innovation in the healthcare environment. We hope that the ideas and discussion described within this paper provide guidance for individual stakeholder organizations and policymakers on how to align their efforts to ensure future investments in new technologies will be recognized, valued, and rewarded in the U.S. healthcare system.
# Table of Contents

Executive Summary: A Collaborative Approach to Aligning Value and Innovation 03

Background 07
A Multi-Sector Perspective on the Changing Innovation Paradigm in the U.S. 07
Real-World Evidence: A Paradigm Shift in Post-Market Evidence Demands 08
Government’s Evolving Role in Post-Market Evidence Demands 10

Dialogue I: Establishing a Shared Vision for Creating Value and Sustaining Innovation 12

Dialogue II: Clearing a Path toward the Ideal State 14
Establishing Consensus on Current Challenges and Potential Solutions 14
   Data Infrastructure 14
   Frameworks for Evaluating Evidence 17
   Evidence Communication 19
   Coverage and Payment 22
Investing in the Future: Steps toward the Ideal State 25

Appendix 26
Background / 

A Multi-Sector Perspective on the Changing Innovation Paradigm in the U.S./

Over the past decade, the research and development process in the pharmaceutical industry has undergone significant changes. This altered landscape is the result of a variety of factors, including increasingly complex scientific advances and a more challenging reimbursement environment. To provide patients access to new products that satisfy their unmet medical needs, securing U.S. Food and Drug Administration (FDA) approval remains an essential step in the drug innovation lifecycle. The number of new molecular entities and biological license applications approved by FDA is one of the first statistics examined in evaluations of the robustness of pharmaceutical research and development.\(^7,8\)

Changes in the post-market payer policy and research environment are creating new incentives for innovation and changing the mix of products coming through the approval process. For example, payers are increasingly turning to decision-support tools to encourage cost-efficient, evidence-based care that promotes high quality and patient safety, and providers are increasingly being held accountable for considering value in their decision-making. The push toward a value-based healthcare system has also resulted in the centrality and prominence of clinical guidelines and the underlying evidence base that supports them.

While some have called for greater integration of the requirements for FDA approval and reimbursement,\(^9\) pre- and post-approval requirements remain largely separate. As a result of growing post-market evidence demands, product developers are increasingly considering the pathway to market access within the drug development process.

As captured below in Figure 1, these changes emphasize the life cycle of innovation beyond initial FDA approval and require demonstration of value to payers after launch.

**Figure 1: Pharmaceutical Innovation Paradigm Continues Beyond FDA Approval**

In response to these changes, pharmaceutical companies are taking steps to promote better coordination between research and development, and commercial functions.\(^10\) Their efforts have included piloting new distribution, contracting, and pricing models. Central to their actions is a focus on using real-world evidence and CER to support the value proposition of their products and serve as the basis of quality improvement programs promoting adherence to these therapies.

---


A Paradigm Shift in Post-Market Evidence Demands

Growing demands for evidence of clinical difference, real-world comparative effectiveness, and economic outcomes undergird many of the changes taking place in the U.S. health system related to value-based decision-making. Figure 2 displays a range of stakeholders creating and using evidence, particularly CER. Beyond product developers, certain stakeholder groups, such as academic centers and private technology assessment organizations, are traditionally the generators of research while others, such as payers, are usually the users. However, as we describe in this section, these lines are no longer distinct.

Figure 2: CER Stakeholder Map

Today, the post-market environment can be best understood by examining the new sources of data and the new users of this data.

New Generators and Data Sources

For several years, the private sector has been accelerating the development of new data resources and supporting health information technology infrastructure. These new sources include research data sets and repositories, aggregates of existing data sources, and tools to query and analyze existing data sets. Health plans and other stakeholders with an interest in cutting healthcare costs and improving quality have invested in data, with an initial objective of informing coverage policies and aiding in attempts to reduce spending—as seen with Medco’s observational CER study analyzing the antiplatelet agents Plavix® (clopidogrel) and Effient® (prasugrel). However, the data investments made by these organizations have also presented the opportunity for leveraging their data assets to support secondary business lines or objectives. WellPoint’s HealthCore subsidiary, for instance, conducts observational studies based on the insurer’s claims data to drive safety, health outcomes, and CER.

However, the data investments made by these organizations have also presented the opportunity for leveraging their data assets to support secondary business lines or objectives. WellPoint’s HealthCore subsidiary, for instance, conducts observational studies based on the insurer’s claims data to drive safety, health outcomes, and CER. Premier Healthcare Alliance utilizes member-provided data to manage hospital costs and improve quality through benchmarking and collaborative engagements based on measurement and CER.
activities. The American College of Cardiology (ACC) maintains a National Cardiovascular Data Registry to provide the basis for the organization’s work in quality improvement and performance measure development. Finally, although the ACC CathPCI Registry and Kaiser Permanente’s HealthConnect electronic medical record were not originally devised with the intent of supporting or conducting CER, their capability to do so is promising for future evidence development.\(^\text{14}\)

PCORI is also contributing to ongoing efforts to create a national data infrastructure to support research. In the process, their members have been debating issues such as governance, data standards and interoperability, architecture and data exchange, privacy and ethical issues, methodological best practices, and incorporation of patient-reported outcomes into healthcare data.\(^\text{15}\) Given the abundance of existing initiatives, PCORI and other stakeholders will need to continue to leverage knowledge from experts in the field.

At the same time, payers and others have continued to support and invest in public platforms and third-party organizations, such as the Cochrane Collaboration, ECRI Institute, Blue Cross Blue Shield (BCBS) Association’s Technology Evaluation Center (TEC), and Hayes, Inc., to help review evidence and inform their assessment of new technologies. The Institute for Clinical Effectiveness and Research’s New England Comparative Effectiveness Public Advisory Council (CEPAC) is an example of a platform that synthesizes evidence reviews performed by the federal Agency for Healthcare Research and Quality (AHRQ). CEPAC then incorporates information on comparative value based on cost-effectiveness analyses to produce actionable information to aid regional policymakers in the medical policy decision-making process.

Increasingly, industry has been responding to this evolving landscape in two ways. First, companies such as GlaxoSmithKline, Pfizer, and Novartis have recently made public statements regarding programs for the conduct and support of CER as an integral part of their drug development and commercialization processes.\(^\text{16, 17}\) Second, some organizations are seeking ways to collaborate with payers and other stakeholders to aid in the development of real-world comparative data. In early 2012, Delaware became the first state to participate in the Real-World Evidence Collaborative with AstraZeneca and WellPoint’s HealthCore.\(^\text{18}\)

**New Users and New Uses**

A range of users and uses of evidence, and in particular CER, has emerged in recent years. This type of effectiveness data informs a range of uses from advisory support functions, such as consensus-building accomplished via research funded by the National Institutes of Health (NIH) and the U.S. Preventive Services Task Force, to binding coverage decisions such as those put forth by payers (Figure 3). Below we describe several of the major categories of users and their motivations.

![Figure 3. Translating Evidence into Action](http://ir.wellpoint.com/phoenix.zhtml?c=130104&p=irol-newsArticle&ID=1664597&highlight)
Clinicians and patients are important end-users of research. Many clinicians will embrace new research because it contains valuable information that will help them in their practices. New tools, such as clinical pathways, have emerged to provide clinicians with evidence-based decision support, although they have also given rise to concerns of hampering providers’ ability to tailor care for individual patients. Patients are also more actively looking to use research to inform their decisions. Through its mission, PCORI is leading the charge in driving the generation and dissemination of patient-centered research that can give patients a deeper understanding of the prevention, treatment, and care options available to them. Organizations focused on quality improvement, such as those that develop provider performance measures (e.g., National Committee for Quality Assurance, American Medical Association – Physician Consortium for Performance Improvement); member organizations that use the measures in practice for benchmarking and quality improvement (e.g., Premier, Truven); and those that endorse measures (e.g., National Quality Forum), are open to the possibilities that CER findings hold for informing and refining quality measurement. These measurement programs are critical components of the evolving payment schemes implemented by Medicare, Medicaid, and private payers. Finally, public and private payers are finding new uses of information obtained through CER beyond traditional uses of research to inform benefit decisions. While Medicare use of CER has been limited, and application of cost-effectiveness analysis (CEA) prohibited under the ACA, health plans are exploring approaches to balancing financial pressures while increasing value by incentivizing consumers to choose high-value products and services in order to manage costs without compromising on quality. On a more targeted level, payers and pharmaceutical companies have been experimenting with outcomes-based contracts, as well as programs geared toward increasing adherence to medications or defined clinical pathways.

**Government’s Evolving Role in Post-Market Evidence Demands**

Given this broader post-market landscape, it is important to focus on the key role of government. Globally, many nations have formally relied on centralized bodies to conduct assessments examining the short- and long-term consequences of the application of a healthcare technology. These global health technology assessment (HTA) bodies have the responsibility of evaluating the clinical and (often) the cost-effectiveness of health technologies, including drugs, and making recommendations to government entities charged with healthcare delivery. In contrast, the U.S. system relies on a plurality of decision-makers in the public and private sectors applying various approaches and incentives to foster value.

As a result, a variety of public and private organizations conduct assessments of the safety, efficacy, real-world effectiveness, cost, cost-effectiveness, and, occasionally, the social, legal, ethical, and political impacts of the product. This includes assessments performed by AHRQ for CMS, as well as evaluations performed by private payers such as BCBS TEC and private organizations like Hayes Inc., Cochrane, and ECRI. As a result of the health reform law, PCORI will play a growing role in the generation and assessment of CER. Understanding this particular dynamic in the U.S. marketplace is a critical component to understanding the government’s role in shaping value.

However, assessing technology, in the traditional sense, is not the only role the federal government plays within the context of driving value and shaping innovation; hence, it cannot be the sole domain of inquiry when seeking to build a policy platform. In recent years, the government has sought a multi-

---

21 EBM Insights, Avalere Health: “Gerispan Health and Merck Team Up to Improve Medication Adherence” (June 25, 2012); “Large Payers Developing Clinical Pathways to Incentivize Adherence to Standards of Care” (April 9, 2012); “CVS Caremark Publishes State-By-State Medication Adherence Report” (April 2, 2012); “Atraa Announces New Value-Based Program to Improve Medication Adherence” (November 21, 2011); “Health Affairs Highlights CERMA’s Alternative Quality Contract” (January 24, 2011).
The multiple roles that government plays, however, has created tension between policies that demand evidence to demonstrate the value of innovation and those that seek to support and provide patient access to valuable innovation. Inherent in calls for a healthcare system that allows for “continuous learning” is an acknowledgement that these competing pressures must be reconciled. In response to this tension, governments worldwide, including in the United States, have adopted “access with evidence development” schemes. Based on its experience with CED over the last few years, CMS has acknowledged that there is room for improvement in the CED process, highlighting the challenges of developing policies that reconcile the aims to provide patients with access to new technologies and to generate additional clinical information on those technologies.
Dialogue I: Establishing a Shared Vision for a Health System that Rewards Innovation

To help frame the Dialogue, Avalere surveyed the available literature, proposed a framework for considering the interplay of evidence and innovation, and identified the range of actors and new demands for evidence on these innovative therapies. Leaders representing patients, product developers, providers, payers, and the research community met in Washington, DC, for the first Dialogue on July 25, 2012.

As a first step in identifying potential solutions to advance a healthcare system that could continue to support the pursuit of investments in new medicines, participants collectively defined a vision for sustaining future innovation in a healthcare system that is increasingly aligning around value:

A healthcare system that rewards innovation and allows for access to effective and equitable care for patients that is delivered efficiently and improves outcomes

In order to collectively achieve this vision, Dialogue participants agreed that solutions needed to reflect eight guiding principles:

1. **Patient-centeredness** to ensure that patient perspectives inform the decision-making processes of purchasers, providers, policymakers, and researchers, and that all activities are anchored in the aim to improve outcomes for patients

2. **Trust among stakeholders** that policies and recommendations are made with the collective desire for value and innovation for patients in mind

3. **Transparency** to facilitate a common understanding of processes and expectations among all stakeholders

4. **Meaningful collaboration** to reach alignment on trade-offs that may be necessary to achieve the ideal state for innovation and a value-based healthcare system

5. **Public/private partnership** to leverage the totality of resources and expertise

6. **Accountability** to share responsibility in the drive toward the ideal state

7. **Predictability** to ensure consistency in actions and expectations of those investing resources and making decisions in patient care

8. **Flexibility** to recognize that innovation is dynamic and clinical practice evolves over time

Next, Dialogue participants identified various elements that would be required to achieve this ideal vision for the healthcare system (see Appendix). These individual elements were further grouped into four domains (see Figure 4).
In order to achieve the ideal vision, participants viewed a robust **data infrastructure** as key to achieving a learning healthcare system, in which knowledge about the real-world benefits and risks of new products could inform and accelerate the adoption of best clinical practice.

Next, participants recognized the need for **frameworks for evaluating evidence** that reflect the viewpoints of all stakeholders in how the value of new technology would be determined. The value of these frameworks would be in providing product developers with predictability in the type of evidence that could inform decision making by payers, providers, and patients.

Third, participants identified the crucial area of **evidence communication** in shaping how future innovation would be valued in the healthcare system. As new technologies move through the system, the way in which FDA shapes the promotional practices of the pharmaceutical industry will continue to evolve. Government, professional societies, patient advocacy organizations, and others are also increasingly creating educational tools and training for clinicians and patients. Attendees considered communication a common thread across the other three domains: a data infrastructure that can help answer research questions, frameworks for evaluating evidence that can provide predictability on how evidence will be assessed, and coverage and payment incentives that can accommodate an evolving evidence base all rely on a communication framework that is consistent and transparent.

Fourth, Dialogue attendees acknowledged that the system had to maintain sufficient incentives in the **coverage and payment** of new technologies. Participants agreed that future coverage and payment models should not discourage providers from choosing clinical options that are the most appropriate choice for their individual patients.

Lastly, Dialogue attendees acknowledged that in order to achieve the ideal vision and individual aims articulated in these four domains, a heightened level of collaboration among all stakeholders would be necessary.
Dialogue II: Clearing a Path toward the Ideal State /

Establishing Consensus on Current Challenges and Potential Solutions /

As a next phase of the Dialogue, Avalere worked with attendees to draft solutions across the four domains that could advance the healthcare system toward the ideal state. Dialogue attendees provided feedback through an online survey and follow-up interviews that resulted in an aggregate list of current challenges and potential solutions. At the second Dialogue, held on October 25, 2012, attendees prioritized two to five solutions within each domain, based on whether the solutions embodied the identified key principles, estimated timing, and magnitude of impact.

In this section, we summarize the output of our discussion.

Data Infrastructure

Current Challenges

Advances in health information technology have facilitated the collection of increasing amounts of data. While there is broad support for leveraging electronic data to generate evidence that can help further a learning health system, several challenges limit the healthcare system’s ability to harness this wealth of information. Keys among these challenges are a lack of common terminology, access, and consistently applied methods, as well as privacy concerns.

A considerable amount of data is generated and collected through the delivery of healthcare. A major challenge in the current environment is harmonizing and integrating these data, which currently reside in disparate systems owned by different entities and developed for reasons other than research. Though these data points could ultimately serve as the basis for important, decision-informing research, lack of common data definitions and taxonomy has made it difficult to effectively prioritize and link them.

A second barrier that has limited a robust data infrastructure is access. Data networks often have restrictions in place that limit who can access the information; these restrictions can also sometimes be applied inconsistently without distinct parameters for predictable use. Further, there are no mechanisms in place to improve access and predictability for viable parties. Interest in sharing data to expand access has been growing in recent years;

Bringing the Domain to Life: All-Payer Claims Databases /

Multiple efforts are underway to build a data infrastructure that can support research and policy decision-making. Numerous states have developed all-payer claims databases (APCDs) in order to fill information gaps on healthcare spending within their states. By harmonizing data from all payers in a state, the APCDs give policymakers statewide data on cost, quality, and utilization patterns as well as information on access and barriers to care. Ten states have APCDs in place, while another six states are currently implementing them.

State experiences with developing and implementing APCDs provide important insights into the potential challenges of creating a robust data infrastructure. States implementing APCDs have confronted difficulties around governance, identifying disparate sources with inconsistent data points, and determining how the data will be managed, stored, and accessed. Currently, each state APCD initiative establishes its own rules for data contribution and sharing, which has limited the potential for these databases to inform policy on a national level. While standardization is desirable for understanding healthcare quality and spending across states, there is also recognition that some flexibility is necessary to satisfy local information needs. State policies also vary in relation to who has access to the data – some states restrict access to state government exclusively, while others make data available to qualified users.

Though still early, the Department of Health and Human Services (HHS) is developing a national multi-payer claims database to support CER, which could lay the groundwork for future efforts to build harmonized data systems on a national scale.
however, few incentives and protections are in place to encourage data generators, such as product developers and payers, to combine forces and contribute information to a singular source.

Adapting data to be sufficiently comprehensive to yield meaningful analyses and conclusions has also been a barrier to leveraging electronic data for research. For example, it is challenging to draw broad conclusions from claims data without additional information on benefit design and formulary status, which may be influencing patient and provider behavior. Moreover, research capabilities are necessitating greater granularity of data, such as individual and subpopulation differences of genomic expression. The current data infrastructure insufficiently addresses demands for both population-based information and subpopulation-level data.34

In addition, methods are applied inconsistently in data collection and validation. Data collection is often impeded by health information technology constraints, organizational barriers, and lack of public appreciation of the need to validate collection mechanisms.35 Meanwhile, quality assurance processes intended to focus on accuracy, precision, and validity lack a formal standard to address concerns of variability across multi-site studies, human and technical errors, and other data quality issues,36 though increased interest in the environment has led to the early development of methodology standards.37

Finally, developing a robust data infrastructure will require addressing privacy of information by considering how to build a longitudinal, multi-dimensional view of a patient while ensuring that patient-level information remains appropriately protected.

Potential Solutions

In evaluating potential solutions, participants addressed the following question:

What steps can be taken to create a data infrastructure that allows for access to the full array of data sources and can generate answers to questions about treatments on a real-time basis?

Participants identified three solutions:

**Invest in Standardized Data**

Attendees proposed the establishment of standardized taxonomy and a data dictionary to eliminate current confusions around the different types, sources, and end usability of data. They viewed this as a necessary step in establishing clarity around the meaning and measurement of various endpoints. As a first step, participants recommended focusing on priority clinical areas where interoperability is most lacking. These may be high-burden therapeutic areas that serve the public health need or systems- and delivery-related areas. In addition, participants recommended that this work be embedded into existing efforts by collaboratives and organizations, such as the National Cancer Institute’s joint efforts with CMS on the Surveillance, Epidemiology, and End Results program or the FDA/Center for Drug Evaluation and Research Data Standards initiative.38, 39

Most recently, the American College of Cardiology Foundation and the American Heart Association developed an updated set of key elements and definitions to support data collection and promote the interoperability and applicability of electronic health records.40 These types of tools would enable the research community to pool data from individual data infrastructures, creating a more robust resource for research. Attendees also recognized the need to create commercial incentives for researchers and data developers to adhere to these standards.

**Create a Reciprocal Data Access Network**

Dialogue participants recommended the creation of a voluntary data sharing system sponsored by a consortium of stakeholders, including product developers, payers, and providers, who would contribute non-clinical, observational data in exchange for access. The data access network would allow for new capabilities to understand health information across the care continuum and to answer research questions that fall across both clinical and cost endpoints. But, more fundamentally, this access network would be a tool that facilitates trust between stakehold-

---

38 Hershman, Dawn L.; Wright, Jason D. “Comparative Effectiveness Research in Oncology Methodology: Observational Data.” J Clin Oncol. 30:4215-4222.
ers through the mutually assured protection of data and accepted guidelines for making the data available. Attendees felt that the reciprocal nature of this collaboration would encourage public and private payers, pharmaceutical and device companies, and researchers to make certain types of data available through such a system. Participants noted HHS’ HealthData.gov, for one, has begun to liberate data, providing access to innovators.41

**Develop Unique Patient Identifiers**

A specific challenge raised by attendees was the difficulty researchers have in understanding patient experience with a drug longitudinally. Attendees noted that patients may initiate drug therapy in one setting of care but then transition into and out of other settings of care (e.g., from community center to inpatient hospitalization to a post-acute care facility, etc.). In addition, much of the existing patient-level data is based on administrative and claims information or clinical information from registries, which do not capture information on the patient experience, including patient satisfaction and preference, quality of life, or functional status. To date, there has been limited effort to capture and centralize patient information across settings of care. Thus, participants voiced the need to create and incentivize the adoption of a unique patient identifier as a potential solution that would enable data linkages across disparate data sources. The concept of unique patient identifiers dates back to the Health Insurance Portability and Accountability Act, though it has been met with congressional resistance due to privacy concerns.42,43 Likewise, participants raised challenges related to privacy and implementation that would need to be addressed in order for this recommendation to move forward.

These three solutions were arrayed by timing and magnitude of impact (see Figure 5). Attendees noted that solutions related to data infrastructure, in particular, would take a long time to implement and that, although there could be some impact in the short term, the greatest impact would be realized over the long term.

**Figure 5: Data Infrastructure Solutions: Timing and Magnitude of Impact**

[Diagram showing data infrastructure solutions with timing and magnitude of impact: 1-2 years, high impact; 2-5 years, low impact; 5+ years, high impact.]

42 Under Section 1173(b), the Department of Health and Human Services (HHS) is required to adopt a standard for a unique health identifier “for each individual, employer, health plan, and health care provider for use in the health care system.” However, Congress has held off on enacting this until privacy concerns have been sufficiently addressed. Source: Health Insurance Portability and Accountability Act (HIPAA) of 1996, Pub. L. No. 104-191, §1173(b), 110 Stat. 1936 (codified as amended at 42 U.S.C. 5 1320d-2(b)).
Frameworks for Evaluating Evidence

Current Challenges

A key issue for innovators is having assurance that they are developing the right evidence to inform the clinical decisions and policies of a range of actors, including payers, clinicians, and patients. In some areas, such as Medicare’s coverage of off label uses of Medicare Part B oncology drugs, policies may provide some level of predictability in what evidence is necessary to support patient access to medically appropriate care.47,48 In other cases, the research needed to support clinical decisions or payer policies (e.g., formulary placement), the requirement for what type of evidence may be most compelling is less clear. The lack of transparency for the research demands creates a challenging environment for product developers. Efforts to overcome this challenge have been met with difficulties, including regulatory restrictions, while dialogues to better understand decision-maker needs have led to outputs that were not entirely reflective of the broad stakeholder perspective.49

Further, existing frameworks for evidence evaluation are based on traditional hierarchies of evidence, which reflects a desire to maximize internal validity and is the standard for most regulatory and clinical decisions. However, this hierarchy is not as well suited when considering the evidence needs of post-regulatory decision-makers who may desire greater generalizability. Given the limitation of RCTs to generate real-world evidence on safety and effectiveness, alternative research methods continue to gain prominence in the clinical development process.50 For example, there is increasing effort to fund observational studies to address questions about real-world comparative effectiveness. Even as investments in research based on novel study designs continue to grow, there are no recognized standards for how decision-makers should incorporate findings from these non-traditional studies into the full breadth of evidence that supports the appropriate use of a product.51

Bringing the Domain to Life: WellPoint Outcomes-Based Formulary /

In 2008, WellPoint created the WellPoint Outcomes-Based Formulary in an effort to communicate its evidentiary and analytical standards in the evaluation of drug products to product developers.44 Guidelines provided by WellPoint include requirements for new products, new indications, and new formulations as well as the re-evaluation of existing products. The purpose of creating and sharing these guidelines was to provide product developers with a clear sense of the information that WellPoint considers useful and to promote investments in research that would produce more information of use to WellPoint, physicians, and its members.45 Since publishing the guidelines, WellPoint has observed an improvement in the quality of formulary submissions from drug companies.46

While this development signals payers’ recognition of the need for more explicit clarity on evidence standards for decision-makers, it is noteworthy that more private payers have taken a similar direction. However, other actors have begun to examine this need closely. Most recently, there has been an effort by the PCORI to promulgate methods standards; it will be important to assess whether these are meaningfully adopted by researchers and consistently evaluated by decision-makers.

49The Implantable Cardioverter Defibrillators (ICD) registry, established by a variety of stakeholders in response to concerns at CMS regarding a surge in positron emission tomography coverage, was housed by the American College of Cardiology; as a result, “the priorities for what registry data would be collected and how it would be used therefore came to reflect the priorities of the governing organizations, and not fully reflective of the priorities of CMS, payers, FDA, and others with specific interests in potential uses of the ICD registry infrastructure.” Source: Mohr, Penny; Tunis, Sean; Samsa, Gail; Mahaffey, Kevin K; Minneci, Paul; Raymond, Claire; McClish, Darrell; Greenfield, Ted. “Evaluation of ICD registries and the evidence they provide: a collaborative process.” JAMA 2002; 287: 2649-54.
Potential Solutions

In evaluating potential solutions, participants sought to address the following question:

How can we reach consensus on what constitutes high-quality evidence and create greater transparency and predictability for researchers?

Participants identified three solutions:

Enable New Platforms for Multi-Stakeholder Discussions about Making Decisions Informed by Evidence

Developing multi-stakeholder forums to discuss evidence needs could help improve transparency and predictability in areas where evidence needs are unclear (e.g., what outcomes would different decision-makers value in studies of a new treatment for an unmet medical need). Participants recommended inclusion of industry, payers, regulators, clinicians, and patients to discuss how evidence needs in specific therapeutic areas may differ and this variation’s impact on different types of decisions—including regulatory approval, formulary placement, treatment recommendations in guidelines, and individual patient care choices. The discussion would encompass how each decision-maker uses evidence to inform their decisions, the type of study designs that is most appropriate, and the endpoints that would be most meaningful in addressing each user’s unique research questions. One example cited by participants was the Green Park Collaborative, which brings together diverse stakeholders with the aim of developing guidance for the life sciences industry on the design of clinical studies to meet the needs of CER/HTA bodies and coverage entities. Given that notions of value are continuously evolving, attendees envisioned evidence needs discussions to be ongoing and that engaged stakeholders would need to make a concerted effort to build trust among one another. Finally, health plan stakeholders specifically pointed to the need to ensure appropriate regulatory protections to allow multiple payers to engage in these discussions.

Create Tools for Evaluating Evidence

As new evidence is created, it is often unclear, and at times, controversial, how it should be evaluated in the context of the broader body of evidence. Methods, tools, and best practices for evaluating study results could help address this. Participants noted the need for the development of a framework for evaluating the usability of evidence. For example, the National Health Council has called for the creation of usability criteria, which could help decision-makers determine the significance of research findings within the context of other evidence and current medical practice. The creation of frameworks to support transparent, consistent evaluations of evidence may improve predictability in the evaluation of data from non-traditional studies, such as observational studies.

Integrate Patient Perspectives in the Research Process

Patients, as the end users of healthcare products and services, are rarely engaged to identify outcomes that they value. Given the rapidly growing focus of the healthcare environment on patient engagement and patient-centered care, attendees recommend increasing patients’ involvement in CER. To enable various institutions to move forward with this recommendation, attendees recommended the use of qualitative methods to inform the choice of key questions and primary and secondary endpoints, including the use of patient-reported outcomes and functional status measures. In close collaboration with a skilled research methods workforce, patient communities should also be engaged in the evaluation of research to ensure that information is translatable to the average consumer (see the Evidence Communication section for more information on involving patients in translation and dissemination).

These three solutions were arrayed by timing and magnitude of impact (see Figure 6).

---

Evidence Communication

Current Challenges

Both the public and private sectors are investing billions of dollars to support evidence development. This evidence is the cornerstone of a learning health system that can facilitate continuous innovation in healthcare. Effectively translating and disseminating findings from these research investments will be critical to encouraging this learning environment. However, several challenges must first be addressed, including: a lack of consistency and transparency in the standards that govern dissemination of research findings, the limited ability or willingness of product developers to communicate research results, and the difficulty in translating information in a manner that can be useful to patients.

As investments in research grow and more evidence becomes available, stakeholders have begun to call for guidelines to ensure that the communication of information is directed by sound standards. Lack of these standards has given rise to questions

Bringing the Domain to Life: Government-Funded Academic Detailing

In 2010, AHRQ initiated an academic detailing project, in which trained clinician consultants meet with physicians, pharmacists, nurses, and other healthcare decision-makers to provide information on medications and other therapeutic options. Separately, AHRQ is funding the National Resource Center for Academic Detailing to support organizations developing new detailing programs or improve existing ones. States, too, are implementing academic detailing programs. Massachusetts, New York, Pennsylvania, South Carolina, and the District of Columbia all have academic detailing programs in place.

Academic detailing programs are expected to reach a wide audience of physicians. AHRQ’s academic detailing project, for example, is expected to reach 1,300 primary care clinician sites (including the offices of internists, family practitioners, nurse practitioners, and physician assistants) and 200 health system sites (including hospitals, integrated health systems, and health plans) across the United States. Each participating clinician and healthcare system will be visited six times over a three-year period for a total of approximately 9,000 visits. During these visits, the project’s consultants will present findings of a specific comparative effectiveness review from the Effective Health Care Program. It is unclear what, if any, communication guidelines are provided to the project’s academic detailers. This lack of transparency has raised questions on whether the information communicated is balanced and appropriately contextualized. Further, in this program and others like it, it is unclear whether and how the publication of new CER studies is being integrated into the information presented.
about whether information currently disseminated by various parties is balanced and appropriately contextualized. Guidelines for the dissemination of research findings provided to federally funded CER organizations illustrate this ambiguity. PCORI, per ACA, is required to include in any disseminated materials a description of considerations for specific subpopulations, the research methodology, and the limitations of the research. In contrast, the communication standards that govern AHRQ’s presentation of research findings as part of its academic detailing project are difficult to find.

Product developers must continually generate evidence to support the value of its product to purchasers, patients, and clinicians in today’s environment. While the demands placed on product developers continue to grow, the policies that dictate product developers’ ability to communicate findings from these investments in research have not evolved to accommodate this new reality. Product developers are limited in their ability to communicate late-breaking and clinically relevant new medical information (both safety and efficacy information) that may help determine optimal care. This inability to disseminate new findings in a time-sensitive manner raises the risk that clinicians will make treatment recommendations without the full scope of relevant information.

Demonstrating value will also require making evidence more useful to patients. Research findings need to be translated in a way that puts these findings in the context of existing evidence in order to allow patients to use the full range of information to weigh the benefit and risk trade-offs of their treatment options. Achieving this, however, has proven to be difficult.

Potential Solutions

In evaluating potential solutions, participants addressed the following question:

How can we ensure that the information needs of all actors in judging the value of new technologies is efficiently satisfied and based on high-quality evidence?

Participants identified five solutions:

Develop and Disseminate Best Practices in Evidence Communication

Currently, standards guiding communication of evidence to users such as patients, healthcare professionals, and payers are highly variable. While product manufacturers are subject to stringent FDA guidelines on research communication, independent researchers or other organizations like payers are not subject to similar standards. While some see a need for standards or guidelines that could promote consistency and transparency of communication across stakeholders, there is also recognition that such standards would be difficult to define and enforce. Arriving at uniform standards that could address the varying evidence needs of patients, providers, purchasers, and policymakers would be challenging, made more complicated by legal restrictions that limit the communication of research findings by product developers. As a starting point, attendees proposed the establishment of best practices or goals in evidence communication through a multi-stakeholder collaboration. Such a collaboration could help create momentum among the various stakeholder groups toward common expectations and goals for how information should be communicated as well as the credentials and training required. These goals would be based on principles of timeliness, consistency, and transparency and would serve as the foundation for advancing towards standards that guide evidence communication. Participants recognized that, in addition to the goals themselves, there is a need to link incentives to the adoption of these goals. Members of the pharmaceutical industry have recently proposed a similar concept: “good communication principles, which would guide the provision of transparent, truthful, and scientifically sound comparative effectiveness research information.”

Establish Guidelines for Incorporating New Information

Increasingly, tools such as clinical decision-support systems and patient decision aids are being deployed as vehicles for disseminating research findings. As the use of these vehicles grows, Dialogue attendees recommended the creation of standards to ensure that these tools reflect the best and most up-to-date clinical evidence. These standards should
address the process for incorporating new research findings into the body of evidence on an ongoing basis so that studies are viewed in the context of the existing literature and can contribute to advancements in expert thinking. To ensure their adoption, attendees viewed incentives for use of sound, patient-centered decision-support tools as an important component of this solution.

As these guidelines are developed, it will be important to consider variations that exist within provisions of the ACA. Namely, the ACA requires patient decision aids to present “up-to-date clinical evidence” and, where appropriate, “explain why there is a lack of evidence to support one treatment option over another.” Separately, the ACA requires PCORI to include in any dissemination materials a description of considerations for specific subpopulations, the research methodology, and the limitations of the research.

Involving Patients in Translation and Dissemination

Making patients partners in translation and dissemination plans will be essential to ensuring information is useful to patients for making decisions. Attendees recommended that patient and advocacy communities work alongside clinical experts to develop and implement translation and dissemination plans. Specific areas that patients could provide input include: whether research results address a question that patients seek answers to; the believability of research findings, particularly when they appear to contradict standard practice; and factors relating to socioeconomic status, language, culture, and different ways of assimilating information that could shape the development of dissemination materials. The most prominent effort in this area is led by PCORI, which is currently evaluating ways to engage patients to lead and accelerate the dissemination of findings from the research, per its mandate.

Advance Methods for Communicating Uncertainty about Benefits and Risks

Researchers and clinicians must often communicate uncertain evidence about the benefits and risks of treatment options. Uncertainty about statistical risk, uncertainty that emerges with use outside of controlled trial settings, and uncertainty about the strength and quality of available evidence affect the usefulness of evidence in making healthcare decisions. Little is known, however, about how to communicate this uncertainty. One recommendation is to fund additional research to advance methods for how best to communicate benefit, risk, and uncertainty, and create training programs to help guide clinicians in conveying information in a way that would enable patients to factor this information into their decision-making. AHRQ has recently initiated a systematic review of communication and dissemination strategies that includes an examination of various ways of communicating uncertainty to different target audiences.

Develop a Safe Harbor for Multi-Stakeholder Scientific Discussions

Given that determinations of value can be based on factors beyond a product’s label, attendees felt there should be opportunities for product developers, payers, providers, patients, and CER/HTA organizations to have scientific discussions about evidence that may inform these determinations. Further, there was a sense among some attendees that if product developers are expected to continue to invest in research to satisfy the growing demands for evidence in the marketplace, there should be opportunities to communicate, in a time-sensitive manner, the results of methodologically rigorous research that may be clinically relevant.

Participants arrayed the solution by timing and magnitude of impact (see Figure 7):

---

### Coverage and Payment

#### Current Challenges

Some policymakers and thought leaders have called for alternatives to the traditional fee-for-service model and have encouraged the testing and adoption of value-based coverage and payment policies as a solution to rising healthcare costs.\(^{71,72,73}\) Increasingly, public and private payers are experimenting with alternative payment models, such as bundled payments and shared savings programs, which rely on the use of financial incentives to encourage providers to choose the most efficient approach to patient care, while also maintaining quality.

A notable characteristic of models being tested in the private sector as well as through the Center for Medicare & Medicaid Innovation is the requirement for providers, such as integrated delivery systems, hospitals, post-acute care facilities, and large phys-

---

**Figure 7: Evidence Communication Solutions: Timing and Magnitude of Impact**

<table>
<thead>
<tr>
<th>I-2 YEARS</th>
<th>2-5 YEARS</th>
<th>5+ YEARS</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>HIGH IMPACT</strong></td>
<td><strong>LOW IMPACT</strong></td>
<td><strong>BEST PRACTICES IN EVIDENCE COMMUNICATION</strong></td>
</tr>
<tr>
<td>Involving Patients in Translation and Dissemination</td>
<td>Guidelines for Incorporating New Information</td>
<td>Safe Harbor for Multi-stakeholder Scientific Discussions</td>
</tr>
<tr>
<td>Methods for Communicating Uncertainty about Benefits and Risks</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

---

Some stakeholders have noted the need for greater predictability and transparency in CED policy.\(^{67,68}\) Despite calls on CMS to do so, CMS’ 2012 draft CED guidance did not articulate evidentiary standards that guide its selection of appropriate items and services suited for CED.\(^{69}\) It also did not establish a time horizon or clear end point at which data collected through CED would be evaluated.\(^{70}\)

Although the NCD process and CED have not historically been applied to pharmaceuticals, the issues raised by stakeholders around the absence of transparent and consistent criteria used by CMS to apply CED and the lack of clearly defined endpoints for data collection relate to broader questions about how evidence is developed, evaluated, and used in the post-market setting.

---


cian group practices, to bear financial risk for the cost and quality of the patients they serve. By rewarding providers that deliver the most efficient care with higher profits, these new models may present challenges for new technologies. Providers may be discouraged from adopting new technologies that are more expensive than existing alternatives. New payment models may inadvertently incentivize providers to favor low-cost treatments without adequately accounting for comparative marginal improvements in quality of care for individual patients. This poses a problem for payers, policymakers, and product developers, as it remains to be seen the effect of these new models on the evaluation of and access to new medical technologies.

**Potential Solutions**

In evaluating potential solutions, participants addressed the following question:

**How can coverage and payment incentives be refined to be more responsive to an evolving evidence base for treatments and standards of care?**

Two solutions were identified:

**Enhance Pathways for Innovation in Novel Provider Payment Models**

Participants recommended the development of policies to ensure that novel provider-level payment reforms advance value-based decision-making while also preserving incentives for innovation. To this end, participants voiced concern that payment policies may only recognize the costs of services, including drug costs, within an episode of care or a bundled payment. Constructed well, with appropriate quality measures and patient protections, such an approach can represent an opportunity for adoption of newer interventions that improve patient outcomes and provide marginal value compared to existing alternatives. Constructed poorly, however, without such protections, payment reforms like bundling and ACOs can incentivize cost cutting that discourages adoption of newer, potentially beneficial treatment options. For example, participants noted that the Medicare Inpatient Prospective Payment System may hinder the adoption of newer pharmaceuticals that may be more expensive, as the existing payment amounts may be inadequate to reimburse the costs associated with a novel alternative. Previous solutions to address these concerns have included providing additional payments for select new technologies, such as through Medicare’s New Technology Add-On Payment Program and pass-through payments for a drug, device, or biological used in the Outpatient Prospective Payment System.

**Ensure Transparency of Incentives in Clinical Pathways**

Increasingly, clinical pathways have been created with the goal of assisting providers and clinicians in better managing the cost and quality of care for specific patients. Clinical pathways, which can be derived from practice guidelines, have the chance to promote greater consistency and quality in care delivery. However, the evidence that informs these new decision-support tools, which are beginning to serve as the basis for coverage and payment decisions, is not adequately transparent. Further, when provider financial incentives are attached to adherence with pathways, they can discourage appropriate customization of care to the needs of individual patients and fail to recognize the introduction of medically beneficial treatment options. Attendees noted that patients are often unaware that their clinicians may be subject to financial incentives that encourage treatment based on a clinical pathway. Participants also remarked that these pathways are not always developed through transparent procedures and may not be based on the most rigorous clinical evidence, reflect the existence of new treatment approaches, or take into account patient preferences. In order to ensure that clinical pathways adopted by payers and providers are reflective of the best evidence and patient preferences, participants recommended that a multi-stakeholder organization review pathways to ensure that they are grounded in evidence, maintain flexibility to include new technologies in a timely manner, and ensure adherence to patient preferences.

Participants arrayed the solutions by timing and magnitude of impact (see **Figure 8**), noting the importance of solutions that could be achieved in the short term with high impact.

---

26 Ibid.
Figure 8: Coverage and Payment Solutions: Timing and Magnitude of Impact

<table>
<thead>
<tr>
<th>1-2 YEARS</th>
<th>2-5 YEARS</th>
<th>5+ YEARS</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>HIGH IMPACT</strong></td>
<td><strong>Pathways for Innovation in Novel Provider Payment Models</strong></td>
<td></td>
</tr>
<tr>
<td><strong>LOW IMPACT</strong></td>
<td><strong>Transparency of Incentives in Clinical Pathways</strong></td>
<td></td>
</tr>
</tbody>
</table>
Investing in the Future: Steps toward the Ideal State /

To further prioritize the solutions, Dialogue attendees participated in a resource allocation exercise, in which attendees individually and collectively committed hypothetical resources (time, labor, investment, social and/or political capital) to the solutions.

Notably, attendees dedicated the greatest amount of resources to solutions in the domains for frameworks for evaluating evidence and communication of evidence. The experts committed resources primarily to solutions they viewed as potentially having the highest impact on innovation, including: creating platforms for multi-stakeholder discussions about evidence needs and value, working with patients as partners in the development and communication of research, and establishing tools for evaluating evidence frameworks, such as usability criteria. These solutions reflect an emphasis by the Dialogue attendees on collaboration as the primary path forward toward the ideal state. Many of the prioritized solutions, in fact, call for the creation of venues or platforms that would allow stakeholders to come together to discuss definitions of value or to establish consensus-based standards for moving forward in using or communicating evidence.

Surprisingly, solutions related to data infrastructure were somewhat de-emphasized in the resource allocation exercise. There was a sense among attendees that a data infrastructure is so fundamental to realizing the ideal vision for a healthcare system that most assumed a robust infrastructure would already be in place before any of the solutions could be implemented. This supposition raises the question as to who within the healthcare system should assume the responsibility for advancing this area. There appears to be a view of data infrastructure as a public good, creating a potential role for the government or a nationally recognized organization, such as PCORI, to invest in areas that could facilitate the creation of a data infrastructure to support evidence generation.

Also noteworthy in participants’ discussion of coverage and payment policy reforms were the approaches they did not embrace as short-term, high-impact solutions. Participants briefly discussed commonly identified approaches, such as outcomes-based agreements and CED, that are frequently cited as examples for how the coverage and payment system could potentially align and encourage innovation. However, attendees were skeptical of the success of these policies in actually expediting market access for new product developers or creating research that could support future innovation. Instead, participants believed that higher priority should be placed on creating safeguards for innovation and patient access to new technologies in light of new payment and delivery models.

Finally, as the capacity of the research community grows and new analytics form the basis of evidence, Dialogue participants noted the importance of ensuring that these solutions will be flexible to accommodate future methods for evidence generation.

The solutions identified through the Dialogue are consistent with preliminary thinking and early efforts within the healthcare community across the four domain areas. While work has begun in these areas, many of these solutions have not been previously evaluated within the context of pharmaceutical innovation. These solutions present options for balancing the drive to realize value in the healthcare system with the continued pursuit of new and innovative medicines. We hope the ideas and solutions described provide guidance for individual stakeholder organizations and policymakers on how to align their efforts in ensuring future investments in innovative new technologies will be recognized, valued, and rewarded in the U.S. healthcare system.
Appendix A: Establishing the Domains

In an effort to identify policy concepts for achieving the ideal vision of the U.S. healthcare system, Avalere solicited input from Dialogue attendees on environmental drivers needed to move toward this vision. The flow chart below represents Avalere’s synthesis of the feedback and the categorization of these drivers into four broad domains.

DATA INFRASTRUCTURE
- Data availability to support continuous learning and evidence generation
- Research infrastructure and workforce issues to support it
- Agreed-upon methods on how to manage uncertainty (how to broaden) for specific decisions and the determination of what constitutes evidence to encourage innovation
- Regulatory framework focused on product approval and market access to encourage innovation

FRAMEWORKS FOR EVALUATING EVIDENCE
- Regulatory framework for communication about real-world efficacy among all stakeholders
- Tools for translating evidence to decision-makers
- Greater public understanding and awareness of vision and the tradeoffs to achieve this vision
- Predictability of the evidence standards and incentives that reward it

EVIDENCE COMMUNICATION
- Alternative payment systems that embed incentives for innovation

COVERAGE & PAYMENT
Appendix B: Investing in the Future

The figure below depicts the results of the breakout session and resource allocation exercise. The size of the circles represent the magnitude of resource allocation (i.e., solutions that are larger received a greater number of hypothetical investment, reflecting broad stakeholder interest).

Resource Allocation Prioritization Based on Timing and Magnitude of Impact

![Resource Allocation Diagram]

**Data Infrastructure**
- DII: Standardized Data
- DII: Reciprocal Data Access Network
- DII: Unique Patient Identifiers

**Frameworks for Evaluating Evidence**
- EF1: New Platforms for Multi-Stakeholder Evidence Need Discussions
- EF2: Tools for Evaluating Evidence Frameworks
- EF3: Integration of Patient Perspectives in the Research Process

**Evidence Communication**
- CM1: Best Practices in Evidence Communication
- CM2: Guidelines for Incorporating New Information
- CM3: Involving Patients in Translation and Dissemination
- CM4: Methods of Communicating Uncertainty about Benefits and Risks
- CM5: Safe Harbor for Multi-Stakeholder Scientific Discussions

**Coverage & Payment**
- CPI: Pathways for Innovation in Novel Provider Payment Models
- CP2: Transparency of Incentives in Clinical Pathways

Legend: Size indicates magnitude of resource allocation.
Avalere would like to thank its sponsors:

Association of American Medical Colleges
Epilepsy Foundation of America
National Health Council
Pharmaceutical Research and Manufacturers of America