Authors/Editors: Andrea Hofelich and Kimberly Westrich (National Pharmaceutical Council), Kevin Walker (Penn Quarter Partners), and Claudia Schur and Annelise Adams (Social & Scientific Systems, Inc.)
Introduction

Passage of the Affordable Care Act (ACA) in 2010—and the U.S. Supreme Court’s decision to uphold the law this year—ensured that comparative effectiveness research (CER) will play an important role in health care decision-making in the United States. Recognizing the potential impact of the law and CER on health care decision-makers, the National Pharmaceutical Council decided to take the pulse of key stakeholders and gauge how, or if, perceptions have shifted from year to year. NPC’s first survey in 2011—targeting insurers/health plans, government, employers, research/academia, business coalitions, and trade associations who were all knowledgeable about CER—set the perception baseline, while the subsequent two surveys displayed shifts in perceptions about funding, monitoring and conducting CER, as well as other aspects of the health care decision-making environment.

As we look at the results from the 2013 survey, it’s fair to say that the potential for CER to reshape the health care landscape is real, but the actual impact of the activity is still on the horizon. Many health care stakeholders are not seeing a significant impact from CER in the immediate term, but they do expect CER to have a larger impact on decision-making over the course of the next five years.
CER has been in use for decades around the world, but it has only entered the public lexicon in the United States more recently, following the approval of $1.1 billion for CER in the 2009 economic stimulus bill and the creation of the Patient-Centered Outcomes Research Institute (PCORI) as part of ACA in 2010.

PCORI was created with the goal of overseeing and sponsoring CER to assist health care stakeholders, especially patients, providers and payers, make better and more informed choices among available treatment and technology options. The mission and work of PCORI has gained a progressively higher degree of attention since it came into being. Part of this attention stems from the significant amount of funds dedicated to its research. Beyond the dollars, though, PCORI captures stakeholders’ attention because of its potential to explore and address clinical decision-making issues that warrant a more intense focus.

Payers have expressed the hope that better evidence-based decision-making will reduce unnecessary expenditures and, in so doing, increase health system cost efficiency. Leaders of health care delivery systems hope that CER will strengthen clinical effectiveness and ultimately improve quality.

However, optimism about CER is not a uniform point of view. Some patient advocates have expressed concern that comparative effectiveness about various protocols and medications will lead to access restrictions or denials of insurance coverage. There are worries that CER ultimately will lead to reimbursements for single, or comparably few, treatments that are judged the best and most cost-effective for the “average” patient.

A unique and important key to acceptance of CER is the fact that stakeholders have been invited to play an active role in the process, including establishing research priorities, reviewing study designs and participating in peer review activities. Again, efforts have been made to enmesh CER and the work of
PCORI within the existing continuum of health care clinicians, providers, payers and end users.

Now that PCORI’s work is well underway, and ACA is growing closer to full implementation (and, with it, increased health care utilization by more than 30 million previously uninsured Americans1), there is increased anticipation of CER’s role in addressing the essential goals of health care quality and slowing health cost increases.

This leads to an increasingly louder chorus of questions. Will the full impact of CER be realized once actionable research is produced by PCORI? And if so, how will that research be integrated into the day-to-day world of health care decision-making? Will academia and industry continue to exercise their primacy in conducting health research, or will that shift as payer organizations intensify their demands for comparative effectiveness data? And how will CER interact with new developments on the health care landscape, including the implementation of state insurance exchanges and the use of essential health benefits regulations and practices?

Without a working crystal ball, it is impossible to predict exactly what role CER will play over the next few years in altering the health delivery and payment landscape, but through our annual survey, we can assess how key players in the system view CER and its potential to improve the state and future of American health care. These perceptions set the groundwork for the ultimate impact of CER and PCORI on the health care decision-making environment.

---

Comparative Effectiveness Research

About the Research

Working with Social & Scientific Systems, Inc., we asked health care stakeholders to gauge the environment for making health care decisions, especially the current state of CER and its impact on medical decision-making. In order to obtain the perspectives of elite decision-makers, we used a sample of individuals and organizations to be broadly representative of “informed” opinions. Because the focus was on the views of those who had thought seriously about the issues, we included an initial screening question that asked about the respondent’s level of familiarity with “the broad area of CER.” Those who responded that they were “not at all familiar” were not invited to complete the survey.

We sent surveys to 288 individuals and organizations, and received responses from 114 participants from insurers/health plans, government, employers, research/academia, business coalitions, and trade associations (Figure 1).

To further encourage responses, we offered an incentive—a donation of $50 to one of four listed charities was promised to respondents for a completed questionnaire. The first invitations went out on September 6, 2012, and responses were received from September 10, 2012 through January 31, 2013.

FIGURE 1. Survey Targets & Respondents

2 The advantages of interviewing “elites” is described in H.V. Kincaid and M. Bright, “Interviewing the Business Elite,” American Journal of Sociology, Vol. 63, No. 3 (Nov, 1957).
Key Findings

Importance of Comparative Effectiveness Research

With increased news coverage in recent years, comparative effectiveness research, sometimes referred to as “patient-centered outcomes research,” is a term that is becoming more familiar to health care stakeholders. In fact, this year 80 percent of survey respondents said they were “very familiar” or “somewhat familiar” with CER, up three percentage points from the prior year. And while respondents are more aware of CER, it does not translate into importance. In fact, CER has decreased in its perceived importance (54 percent said it was “very important” in the 2013 survey, compared to 71 percent in 2011) (Figure 2).

This shift in importance could be due to an anticipated future impact of CER, rather than an immediate one. During the coming year, there is tempered optimism about CER, with 31 percent of respondents expecting that CER will have a “moderate improvement” on health care decision-making over the next 12 months—a seven percentage point increase from 2012.

FIGURE 2. CER Still Important, But Decreasingly So

![Bar chart showing the perception of CER importance from 2011 to 2013](chart.png)

2011: n = 111
2012: n = 107
2013: n = 107
However, it’s during the next few years in which the full impact of CER will be realized. Respondents felt much more confident about the impact of CER on health care decision-making over the next three to five years, with a “moderate improvement” indicated by more than half of the respondents in the next three years, and nearly a third in the next five years. A “substantial improvement” was indicated by 24 percent over the next three years and 55 percent over the next five years (Figure 3).

**Role of PCORI and Other Groups**

By legislative design, the majority of CER activity conducted within the federal government will be initiated and managed by PCORI. In the three years since its establishment, PCORI has intensified its efforts to establish research methods, hire staff, set national research priorities, and increase its public outreach to patients and other health care stakeholders. These are all likely contributing reasons to PCORI’s enhanced perception as a leader in research methods, standards and priority setting.

Not surprisingly, nearly three quarters of respondents viewed PCORI as the leader in setting research priorities and standards—perhaps because the organization released a draft of its methodology report just a few months earlier. However, other organizations remain important in this space, although to a lesser degree than in previous years.
FIGURE 4. Groups to Play a Significant CER Role in the Next Five Years: Establishing Research Priorities

FIGURE 5. Groups to Play a Significant CER Role in the Next Five Years: Establishing Research Standards

<table>
<thead>
<tr>
<th>Year</th>
<th>AHRQ</th>
<th>NIH</th>
<th>FDA</th>
<th>PCORI</th>
<th>Academia</th>
<th>Private Health Plans</th>
<th>Pharmaceutical Products Industry</th>
</tr>
</thead>
<tbody>
<tr>
<td>2011</td>
<td>74%</td>
<td>65%</td>
<td>57%</td>
<td>73%</td>
<td>33%</td>
<td>24%</td>
<td>73%</td>
</tr>
<tr>
<td>2012</td>
<td>77%</td>
<td>60%</td>
<td>67%</td>
<td>67%</td>
<td>33%</td>
<td>30%</td>
<td>63%</td>
</tr>
<tr>
<td>2013</td>
<td>67%</td>
<td>67%</td>
<td>69%</td>
<td>69%</td>
<td>33%</td>
<td>30%</td>
<td>67%</td>
</tr>
</tbody>
</table>

2011: n = 111  
2012: n = 104  
2013: n = 107
Those include the Agency for Healthcare Research and Quality (AHRQ) (70 percent) and the National Institutes of Health (NIH) (53 percent) (Figures 4 and 5). When it comes to funding and monitoring research, however, there are other players in the mix. NIH is still perceived as playing the leading role in many of these areas, but to a significantly lesser extent than in previous years (69 percent in 2013 vs. 85 percent in 2012), much like AHRQ (55 percent vs. 74 percent in 2012). The pharmaceutical industry followed at 66 percent, with PCORI at 61 percent, which are nearly identical to 2012 responses. What’s interesting to note is that both the U.S. Food and Drug Administration (FDA) and private health plans are beginning to be viewed by some stakeholders as playing a role in funding and monitoring comparative research.

**FIGURE 6. Groups to Play a Significant CER Role in the Next Five Years: Funding and Monitoring Research**

<table>
<thead>
<tr>
<th>Year</th>
<th>AHRQ</th>
<th>NIH</th>
<th>FDA</th>
<th>PCORI</th>
<th>Academia</th>
<th>Private Health Plans</th>
<th>Pharmaceutical Products Industry</th>
</tr>
</thead>
<tbody>
<tr>
<td>2011</td>
<td>74%</td>
<td>55%</td>
<td>24%</td>
<td>60%</td>
<td>15%</td>
<td>31%</td>
<td>66%</td>
</tr>
<tr>
<td>2012</td>
<td>74%</td>
<td>69%</td>
<td>30%</td>
<td>61%</td>
<td>15%</td>
<td>31%</td>
<td>66%</td>
</tr>
<tr>
<td>2013</td>
<td>55%</td>
<td>44%</td>
<td>21%</td>
<td>60%</td>
<td>8%</td>
<td>28%</td>
<td>66%</td>
</tr>
</tbody>
</table>

2011: n = 111  
2012: n = 104  
2013: n = 107
effectiveness research. This makes sense in that the FDA (23 percent) has been vocal in recent months about its interest in more intensive studies (Figure 6).

Mirroring the trend from previous years, the bulk of conducting the research work is still expected to fall overwhelmingly to academia (82 percent) and the pharmaceutical industry (67 percent). There are also expectations that health insurers (39 percent) will be active in conducting research (Figure 7). This finding fits with the current trend of payers partnering with other health care organizations and manufacturers to mine data and assess issues such as patient care, medication adherence and cost efficiencies.

**FIGURE 7. Groups to Play a Significant CER Role in the Next Five Years: Conducting Research**

<table>
<thead>
<tr>
<th>Group</th>
<th>2011</th>
<th>2012</th>
<th>2013</th>
</tr>
</thead>
<tbody>
<tr>
<td>AHRQ</td>
<td>43%</td>
<td>38%</td>
<td>33%</td>
</tr>
<tr>
<td>NIH</td>
<td>50%</td>
<td>50%</td>
<td>50%</td>
</tr>
<tr>
<td>FDA</td>
<td>16%</td>
<td>23%</td>
<td>25%</td>
</tr>
<tr>
<td>PCORI</td>
<td>23%</td>
<td>26%</td>
<td>28%</td>
</tr>
<tr>
<td>Academia</td>
<td>33%</td>
<td>33%</td>
<td>33%</td>
</tr>
<tr>
<td>Private Health Plans</td>
<td>85%</td>
<td>82%</td>
<td>82%</td>
</tr>
<tr>
<td>Pharm-Medical Products Industry</td>
<td>82%</td>
<td>70%</td>
<td>67%</td>
</tr>
</tbody>
</table>
CER and Key Health Care Functions

Survey respondents also were asked to assess the status of each of nine issues affecting the health care decision-making environment. These issues are likely to be affected by CER or require some degree of change as CER becomes more widely available. For all but one issue—research priorities—the survey reflects a progressive movement in a positive direction, compared to three years ago. The results indicated:

- Improved availability of new research methods
- Greater availability of agreed-upon and generally accepted research standards for effectiveness research
- Research priorities only somewhat adequately reflect the choices among types of treatment that patients and providers face
- Increased transparency and objectivity in processes for interpreting evidence
- More predictable decisions by payers and purchasers on the adoption of medical treatments
- More consistency and transparency in the exchange of evidence information across payers, providers, industries and agencies
- Increased emphasis on assessment of the value of treatments that include effects on quality of life, workplace productivity and adherence to treatment

FIGURE 8. Purchasing Decisions: Some Movement Towards Integration Observed

Health Care Services Purchasing Decisions

*Statistically significant differences between three years ago and today. The chart represents data consolidated over three years: in 2011, n=105; 2012, n=99; 2013, n=99.
Processes of medical decision-making that better integrate the impact on all health care services used by patients

Increased purchasing of health care services based on value received

The greatest shifts in attitudes across the three years were observed in the two latter issues. When asked about their view of health care services when making purchasing decisions, respondents indicated a slight shift from a siloed view to a more integrated one. In particular, 62 percent of respondents said they had a siloed view of services three years ago, compared with 39 percent today. By contrast, there was a six percentage point increase in respondents that said they had an integrated view of services today (8 percent) compared with three years ago (2 percent) (Figure 8). These changes could be linked to the growth in accountable care organizations, which encourage an integrated view of patient care and health care purchasing, along with clear financial incentives for meeting quality measures. When asked about the extent of outcomes-based contracting, 71 percent of respondents felt there was little to none three years ago, which declined to 46 percent today, meaning that more respondents are seeing this type of contracting. Only a slight percentage point increase in respondents felt that outcomes-based contracting became widespread over the course of three years (Figure 9).

FIGURE 9. Outcomes-Based Contracting: More Common, But Not Prevalent

<table>
<thead>
<tr>
<th>Extent of Outcomes-Based Contracting</th>
</tr>
</thead>
<tbody>
<tr>
<td>Little to None</td>
</tr>
<tr>
<td>3 Years Ago</td>
</tr>
<tr>
<td>2 Years Ago</td>
</tr>
<tr>
<td>1 Year Ago</td>
</tr>
<tr>
<td>Today*</td>
</tr>
</tbody>
</table>

*Statistically significant differences between three years ago and today. The chart represents data consolidated over three years: in 2011, n=102; 2012, n=97; 2013, n=98.
Conclusion: Entering a Critical Period

The 2013 National Pharmaceutical Council stakeholder survey paints a picture of payers, employers, thought leaders and associations increasingly aware of CER and PCORI, more convinced that CER will have a role in health care decision-making within the next five years, and progressively more optimistic that positive changes to our health care system will result.

But now, as health reform implementation enters its later stages and PCORI’s work begins to fully hit its stride, some key questions will emerge. When PCORI produces tangible, actionable research, will stakeholders begin to sense the full effect of CER? Will academia and industry continue to play the dominant roles in research, or will that balance shift when payer organizations become more engaged in CER and rely upon their own data? And what effect will outside factors, such as the implementation of state health insurance exchanges and the use of essential health benefit parameters, have on the health care landscape and the quest for greater cost effectiveness?

These questions underscore the importance of forthcoming NPC annual stakeholder surveys and, with them, a clearer understanding of the perceptions, assumptions and assessments of those who will be guiding the ongoing evolution in American health care.