



# 2014 Comparative Effectiveness Research

---

AND THE **Environment**  
FOR **Health Care Decision-Making**

**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

Now in its fourth year, the National Pharmaceutical Council's annual survey of health care stakeholders is continuing to shed light on the current environment for comparative effectiveness research (CER) and health care decision-making.

CER has the potential to better inform patients, their care providers, payers and other stakeholders as they make critical decisions about treatments, coverage options and other health care challenges. As our survey results showed this year, that potential is still on the horizon.

The stakeholders we surveyed—insurers/health plans, government, employers, researchers/thought leaders, business coalitions and associations who were all knowledgeable about CER—indicated some shifts this year in their attitudes toward the CER landscape. In particular, there were some changes in which organizations were perceived as influential in areas such

as funding, monitoring and conducting CER, as well as in other aspects of the health care decision-making environment.

We also noted some consistencies in perceptions from previous years. First, stakeholders are continuing to view the Patient-Centered Outcomes Research Institute as a leader in the CER effort. Second, the Agency for Healthcare Research and Quality and the National Institutes of Health remain key players in this space. Perhaps most important, many health care stakeholders are not seeing an immediate impact from CER, although they do expect CER to have a larger impact on decision-making during the next five years.

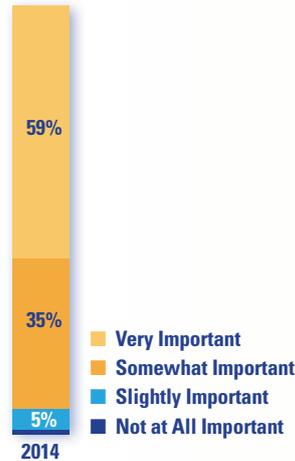
# Executive Summary

This 2014 edition of the National Pharmaceutical Council's comparative effectiveness research (CER) survey found that health care stakeholders expect CER to have a broader impact on decision-making in the near future. Stakeholders also look to certain organizations to play key roles in the CER effort.

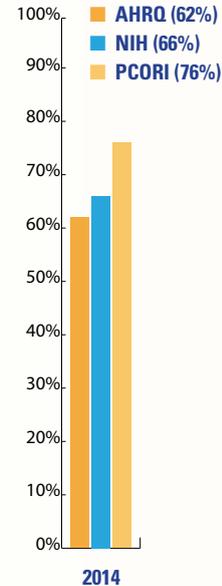
## 2014 KEY FINDINGS



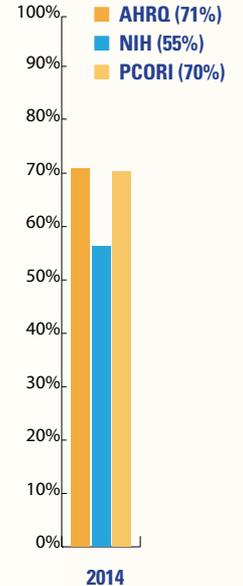
STAKEHOLDERS SURVEYED



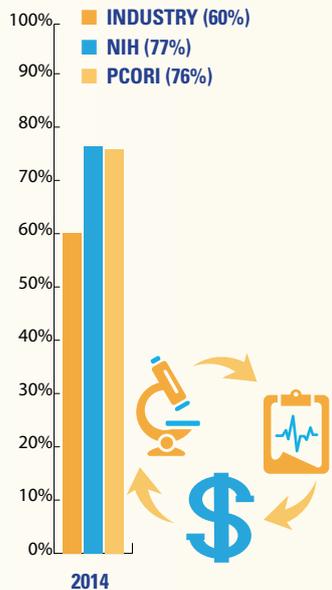
CER STILL IMPORTANT



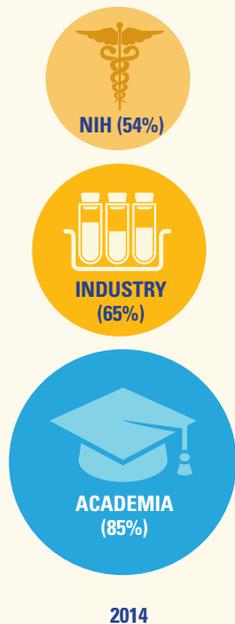
KEY ROLES IN SETTING CER PRIORITIES



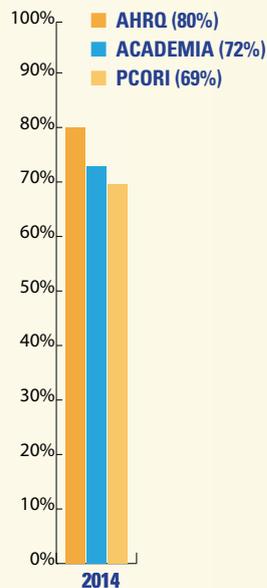
KEY PLAYERS IN SETTING CER STANDARDS



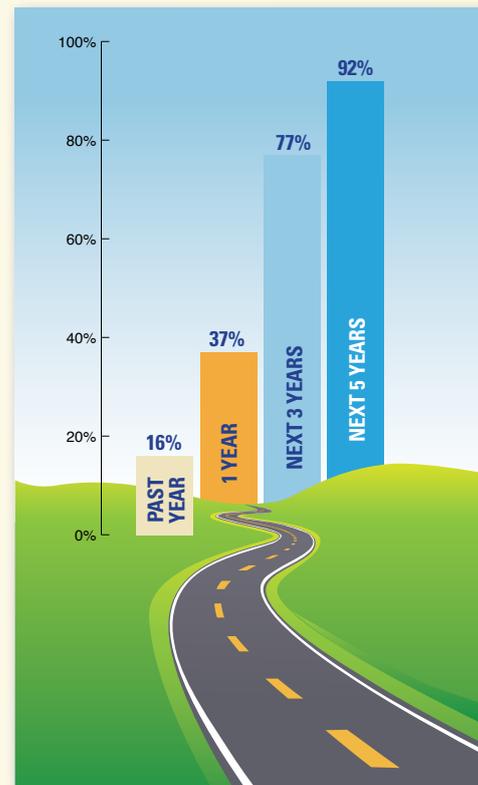
**2014**  
**KEY ROLES IN FUNDING,  
 MONITORING RESEARCH**



**2014**  
**KEY GROUPS IN  
 CONDUCTING CER**



**2014**  
**KEY PLAYERS IN  
 DISSEMINATING CER**



**IMPACT OF CER ON DECISION-MAKING  
 IN THE PAST, PRESENT AND FUTURE**

N=110 for all figures. For key roles, stakeholders were asked to choose among the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health (NIH), the Food and Drug Administration (FDA), the Patient-Centered Outcomes Research Institute (PCORI), academia, private health plans and the pharmaceutical industry.

## Background

Although comparative effectiveness research (CER) has been in use for decades, the Affordable Care Act has enhanced its prominence in broader health care discussions by establishing the Patient-Centered Outcomes Research Institute (PCORI) to oversee and sponsor CER to assist key health care stakeholders in making better and more informed choices about treatment options.

Since its creation, PCORI has continued to build out its infrastructure, host public meetings and provide hundreds of millions of dollars in funding for research projects. Despite its increased public profile, PCORI is not the only organization involved in CER. There are a number of public and private sector organizations engaged in CER efforts, such as the Agency for Healthcare Research and Quality, the National Institutes of Health, academia and the pharmaceutical industry, to name a few.

Although these and other stakeholders share similar goals of conducting research to answer questions that can improve health care delivery and patient outcomes, they often view CER and its potential impact through different lenses. There is an optimistic view, particularly among payers, that bringing an expanded use

of evidence into health care decision-making will lead to more cost-efficient health system operations and greater effectiveness in getting the right treatment to the right patient, limiting the need for additional hospitalizations and avoidable health care services. Along these same lines, leaders of health care delivery systems see CER as a potentially valuable tool in bolstering clinical effectiveness.

Yet there are concerns about the possible impact of CER on patient access to health care treatment options. Patients are not all the same and can react in different ways to various pharmaceutical therapies and treatments. There are worries that comparative effectiveness studies will ultimately lead to insurance coverage for comparably few, or even single, treatments that are determined

through CER to be the most effective for the “average” patient. This leads to hypothetical questions and concerns as to how many patients could be harmed if the most suitable treatments and protocols for their particular conditions fall outside of the range of therapies deemed comparatively most effective. These questions will take on a greater urgency as health care payers and providers gravitate toward delivery and payment systems, such as accountable care organizations and packaged reimbursements, which provide a pre-determined dollar amount to treat a specific condition. This evolution will heighten the importance of effective treatments.

This also underscores the significance of the National Pharmaceutical Council’s stakeholder survey. Much of the ultimate importance of CER is tied to the way in which it is perceived and accepted by stakeholders. It is noteworthy that stakeholders have been invited to play an active role in the CER process, including establishing research priorities, reviewing study designs and participating in peer-review activities. It is to the credit of the PCORI leadership that it has actively reached out to clinicians, providers, payers and patient organizations to maintain strong lines of communication and to encourage their input. The NPC survey shows the state of play and the evolution of stakeholder perceptions, assessing how key players in the health care ecosystem are viewing CER and judging its potential to change health care delivery. Today, as PCORI continues to expand its volume of research, questions are mounting.

Will CER be integrated into day-to-day health care decision-making, and what impact will this have on the patient population and their broad array of genetic and health differences? Will the prevalence of new comparative effectiveness data shift the landscape of health care research, bringing payers into a more visible role alongside academia and industry? And how will CER become integrated into new pay-for-value health care delivery systems?

---

“Will CER be integrated into day-to-day health care decision-making, and what impact will this have on the patient population and their broad array of genetic and health differences?”

---

No one can know with absolute certainty how extensive CER’s role will be in the health care environment two, five or even ten years from now. We do know, though, that stakeholder perceptions and acceptance will be critical factors in how this future is shaped. To that end, the NPC stakeholder survey provides important guidance into how those perceptions are changing as CER becomes more prevalent.

# 91%

of stakeholders are “very” or “somewhat familiar” with CER.

# 92%

expect CER to have a “moderate” to “substantial” impact on health care decision-making in the next five years.

## 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. 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 asked to send back the uncompleted questionnaire.

We sent surveys to 435 individual and organizational stakeholders on September 13, 2013, collecting responses through January 31, 2014. These stakeholders included researchers/thought leaders; government; insurers/health plans; employers; business coalitions; and associations. In all, 110 complete responses were received with a distribution between various stakeholders similar to the three previous NPC surveys. **(Figure 1)**

**FIGURE 1. Stakeholders Surveyed**



To encourage responses, we offered an incentive—a donation of \$50 to one of four listed charities was promised to respondents for a completed questionnaire.

# Key Findings

## Awareness, Importance and Impact of CER

Given that four years have passed since the Affordable Care Act (ACA) was signed into law, it's not surprising to find that a higher percentage of stakeholders are now very familiar with CER. Today, 91 percent of respondents said they are "very" or "somewhat familiar" with CER, which is 6 percentage points more than in 2011. Although awareness of CER has increased, the importance of CER has decreased somewhat in the eyes of these stakeholders. Fifty-nine percent of respondents found CER to be "very important," with another 35 percent ranking it "somewhat important."

There could be several reasons for this decrease in importance since our first survey. At that time, implementation of the ACA and CER was broadly reported on in Washington, DC-based media, amid ongoing health policy conversations. Expectations also could have been higher because stakeholders might not have had a full understanding of CER and the length of time it takes to engage patients, develop questions, conduct the research and incorporate it into decision-making.

Those reasons could explain why stakeholders expect CER to have a greater impact in the future, rather than in the near-term. When asked whether CER had an effect on health care decision-making over the past 12 months, 84 percent said that it had "no effect" or led to a "slight improvement," while 13 percent said it led to a "moderate

improvement." Thirty-one percent of respondents said CER will have a "moderate improvement" on health care decision-making over the next 12 months, which is unchanged from last year.

Respondents felt 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 56 percent and 50 percent, respectively, and a "substantial improvement" indicated by 21 percent and 42 percent, respectively.

## Role of Organizations in Developing, Funding and Disseminating CER

When it comes to taking a leading role in developing CER priorities, standards and funding research, one organization is consistently among the top three cited by stakeholders: the Patient-Centered Outcomes Research Institute (PCORI). During the last year alone, PCORI has hosted numerous patient-oriented meetings, posted calls for research, and awarded hundreds of millions of dollars in funding for research projects. This increased profile and ability to provide substantial funding for CER likely accounts for some of PCORI's perception among stakeholders.

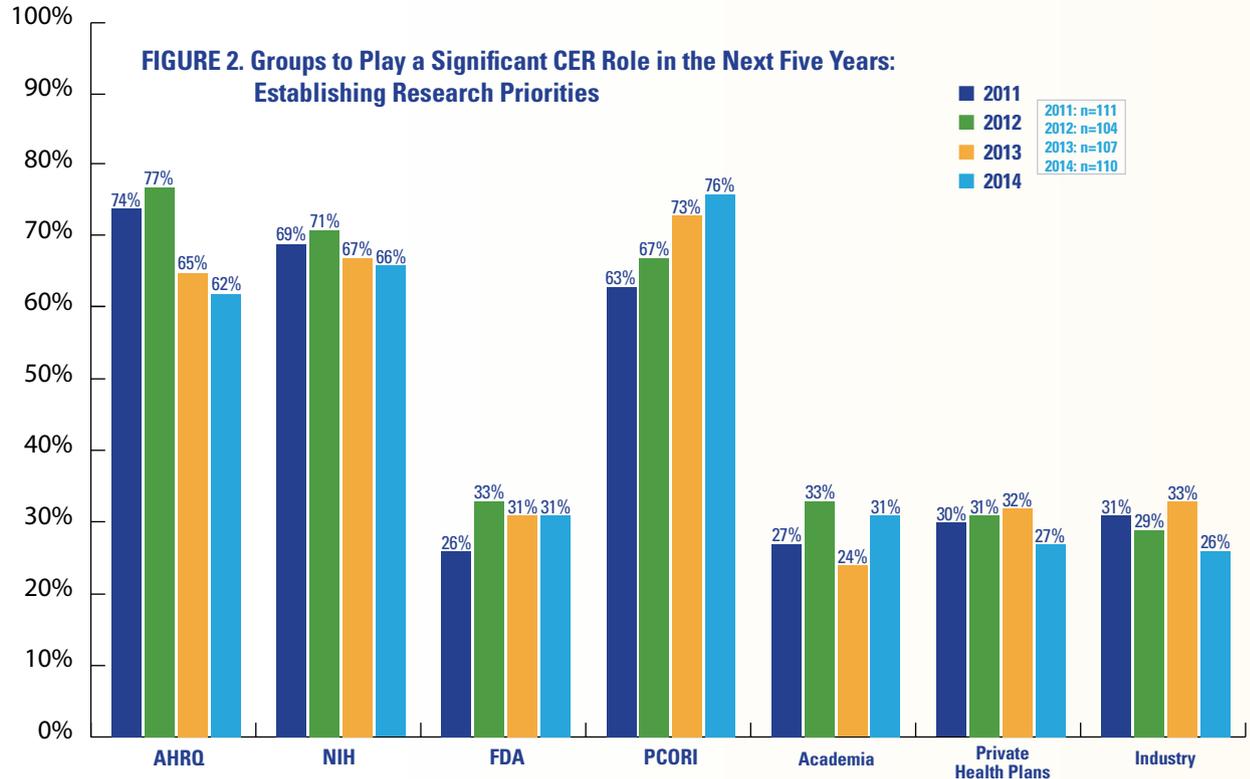
# 47%

see a trend toward more widely agreed-upon research standards.

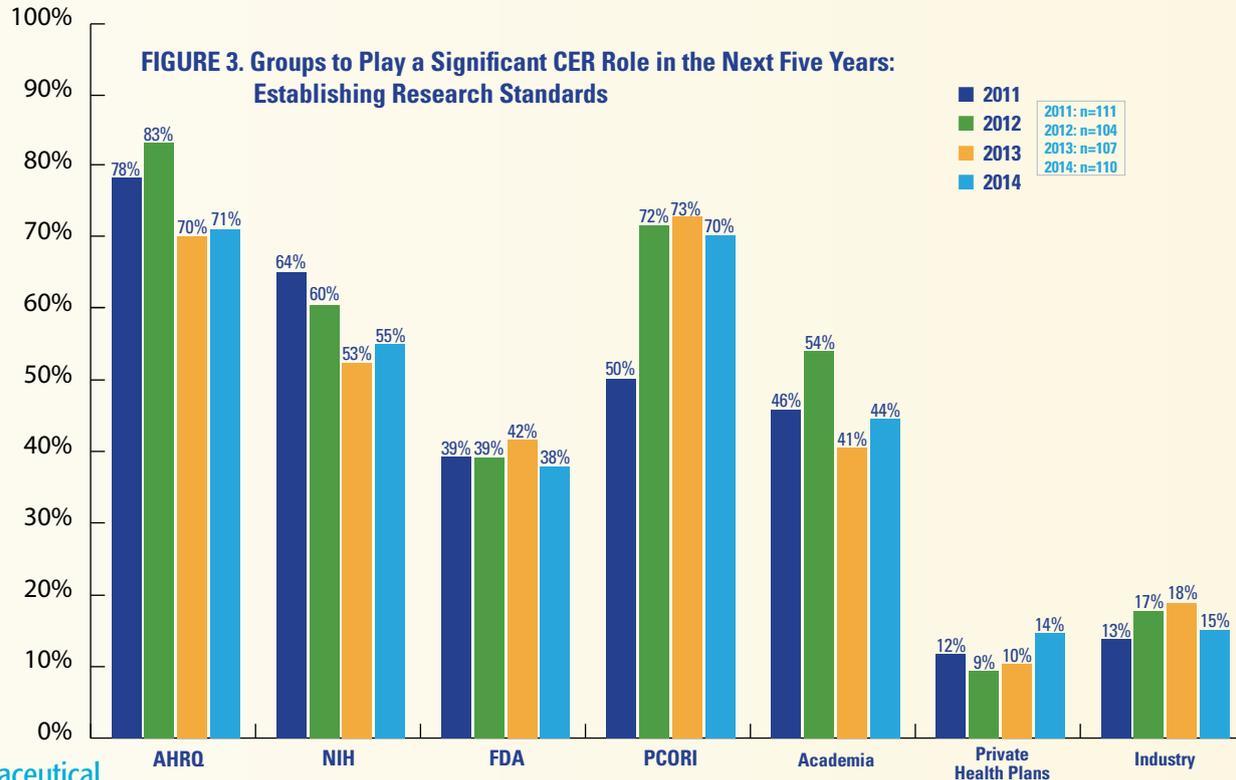
# 62%

say the value of treatments tends to be narrowly focused on only clinical effectiveness.

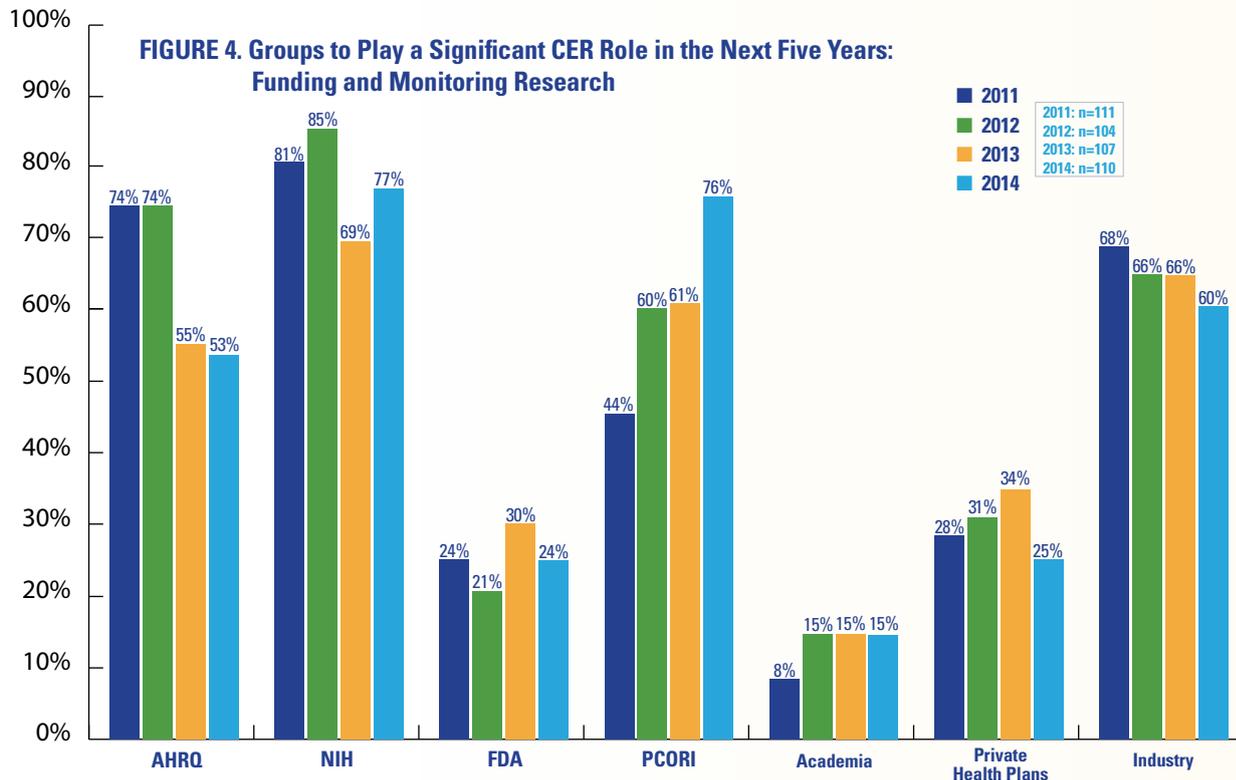
**Setting CER Priorities.** Which groups are setting the agenda for the types of research questions that are being answered? Overwhelmingly, PCORI is seen as leading this charge (76 percent), followed by the National Institutes of Health (NIH) (66 percent) and the Agency for Healthcare Research and Quality (AHRQ) (62 percent). Since this survey's inception, these organizations have been consistently cited among the top three in this area. **(Figure 2)**



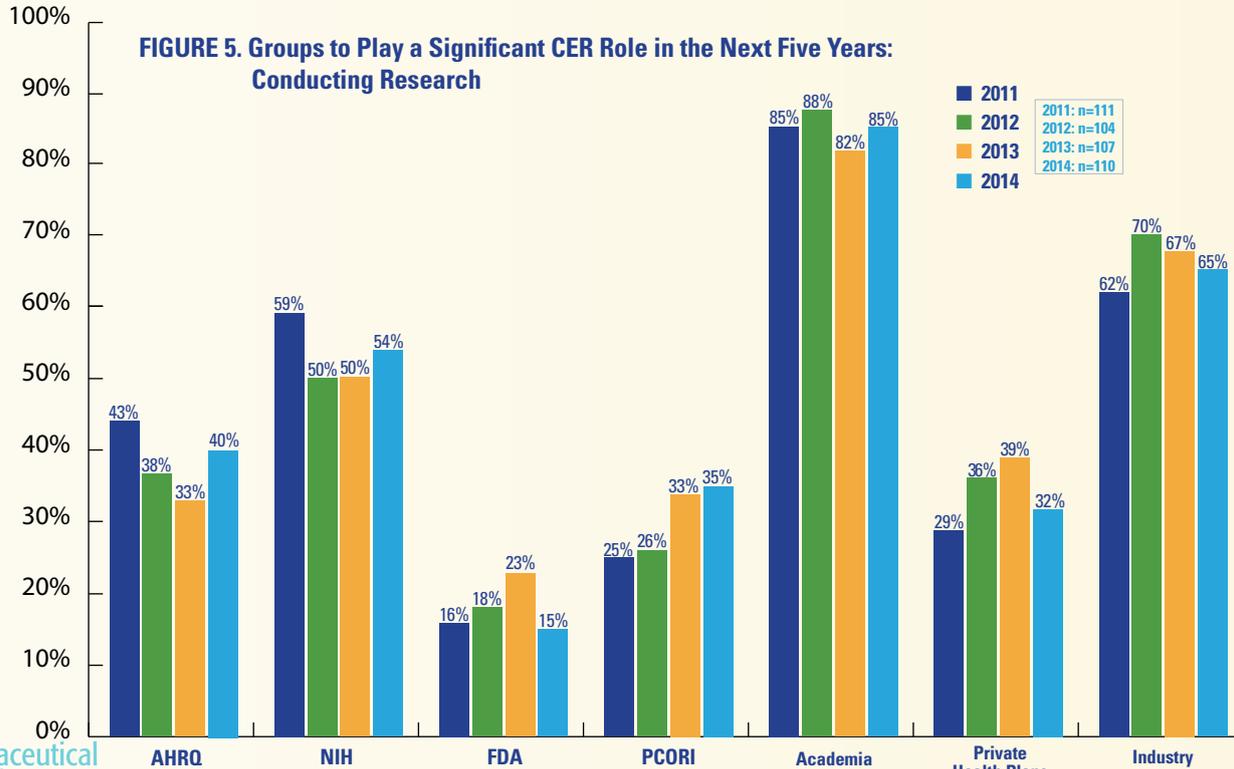
**Establishing Research Standards.** AHRQ (71 percent) and PCORI (70 percent), followed by NIH (55 percent), were viewed as leaders in this category. In November 2013, PCORI released the final version of its methodology report, which set standards for the conduct of CER, and PCORI has been very clear about requiring grant recipients to follow the standards set out in the methodology report. **(Figure 3)**



**Funding and Monitoring CER.** Here is where PCORI's activity in awarding funds for CER projects has had a noticeable effect on stakeholder perceptions. In 2011, only 44 percent of respondents saw PCORI as having a significant role in funding and monitoring CER. In 2014, that number has jumped to 76 percent. It should be noted, though, that stakeholders see this as a shared responsibility: NIH (77 percent), pharmaceutical companies (60 percent) and AHRQ (53 percent) also have significant funding and monitoring responsibilities. **(Figure 4)**



**Conducting Research.** By far, stakeholders pointed to academia (85 percent) as the group charged with conducting research, with a solid majority of respondents citing the role of pharmaceutical companies (65 percent). This view has been consistent since 2011. Last year, with the backdrop of private health plans partnering to harness big data and the Food and Drug Administration’s (FDA) growing efforts in data mining, stakeholders indicated that they were seeing the potential for health plans and the FDA to take a larger role in conducting research. That trend was reversed in this year’s survey, however, as respondents’ views of FDA and private health plans on conducting research returned to their previous survey levels. **(Figure 5)**



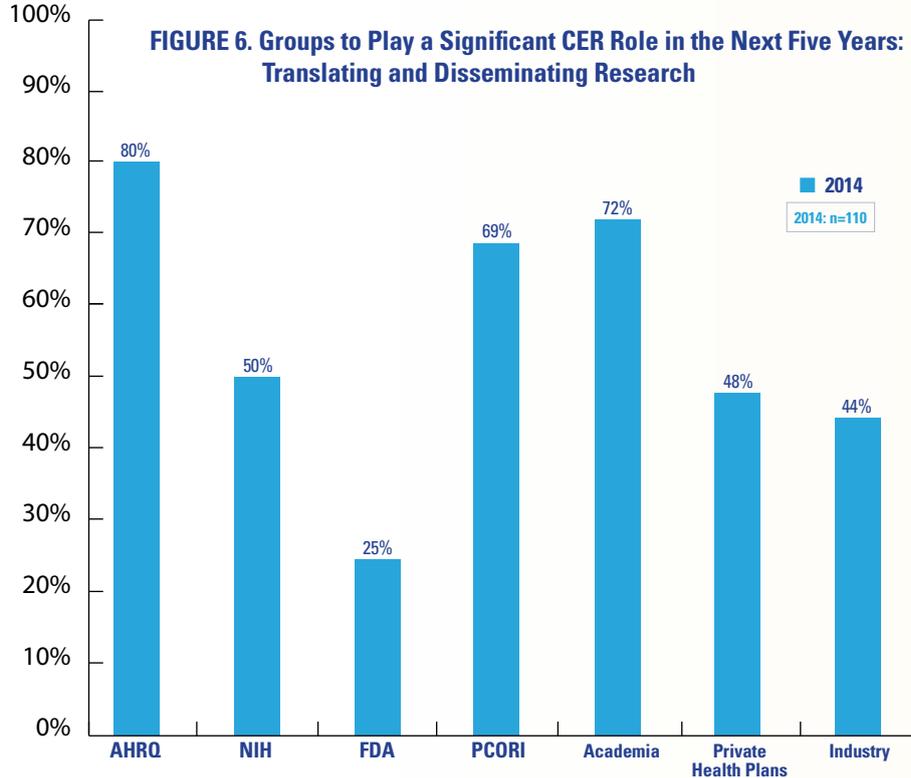
# 67%

say there is not enough evidence available to inform the choices faced by patients and providers.

# 47%

say the use of real-world evidence in decision-making is limited.

**Translating and Disseminating CER.** This marked the first year NPC asked stakeholders which groups they believe will play a significant role in translating and disseminating CER. Not surprisingly, AHRQ—the group named by federal law to aid in the dissemination of PCORI-funded research—was cited as first by 80 percent of stakeholders. Academia (72 percent) and PCORI (69 percent) also are expected to play a role, while approximately half of the stakeholders surveyed named NIH, private payers and the pharmaceutical industry. **(Figure 6)**



## The Current Health Care Decision-Making Environment

As in previous years, stakeholders were asked to assess the status of several issues affecting the current health care environment, using a seven-point scale indicating a perceived negative, neutral or positive assessment. Overall, stakeholders have a slightly more positive view on the issue of agreed-upon standards for CER, but remained downbeat on issues such as the transparency of processes for how evidence is interpreted and the integration of purchasing health care services.

**Agreed-Upon Research Standards.** Today, **47%** of respondents believe that we are trending toward having more widely agreed-upon research standards, which would provide more consistency in the conduct of CER. This is almost a mirror image of the responses in 2011, when **50%** of respondents said there were few agreed-upon standards.

**Research Priorities.** Stakeholders were asked whether they felt that research priorities adequately addressed treatment choices faced by patients and providers. Although respondents aren't overwhelmingly positive in this area (**37%**), we saw marked improvement over 2013, when **22%** of respondents thought that research priorities somewhat/adequately reflected treatment choices.

**Transparent and Objective Processes.** According to survey respondents, there is little transparency in the processes used by decision-makers to interpret evidence. This sentiment changed little over the four years.

**Focus of Treatment Assessments.** Notably, **62%** of stakeholders, same as in 2013, are continuing to point out that the value of treatments remains more narrowly focused on only clinical effectiveness, rather than also taking into account quality of life, workplace productivity, adherence to treatment and other outcomes.

**Integrated Purchasing of Health Services.** Last year, stakeholders indicated a subtle shift toward processes of medical decision-making that better integrate the impact of all health care services used by patients, possibly driven by the growing trend in accountable care organizations. This year, opinion shifted back to a slightly more siloed view of health care purchasing decisions.

**Outcomes-Based Contracting.** Similar to 2013, **68%** of respondents felt that there is little to no outcomes-based contracting.

# 47%

felt individual treatment response tends not to be taken into consideration in coverage and treatment decisions.

Three new questions were added to the survey this year, reflecting growing trends and discussions among health care stakeholders. These include:

■ ***The Completeness of the Comparative Effectiveness***

***Evidence Base.*** Stakeholders were asked whether the breadth of evidence is “complete enough to inform the choices faced by patients and providers.” When it comes to finding evidence that answers treatment questions, **67%** of surveyed stakeholders said there is not enough evidence available.

- ***Use of Real-World Evidence.*** Real-world evidence—generally how treatments work outside of a carefully monitored study—is increasingly being collected because it can tell researchers and other stakeholders how treatments work under everyday circumstances. Although more of this type of research is being funded, **47%** of stakeholders note that its use in decision-making is still limited.

- ***Variability in Individual Patient Response.*** A concern among many patients is whether they will have access to the treatment that will work best for them. It’s widely understood that one treatment might work well for some patients, but not for others. However, **47%** of stakeholders felt that individual treatment effects are not often taken into consideration in coverage and treatment decisions.

## Conclusion: CER Still on the Horizon

The annual survey provides a snapshot of the health care and CER landscape, one that is changing with each passing year.

While the impact of CER on health care decision-making has not yet been felt, there is a strong expectation that it will make an impression within the next three to five years. There is, in fact, a great deal of optimism that this research will lead to health care system improvements. There also is a strengthening of perceptions in terms of which entities in the public and private sectors will guide the development of this research as well as its funding and dissemination.

Assuming that the stakeholders' prediction holds true and that the effects of CER will be seen five years from now, if not sooner, there are still questions that must be answered before that time comes. Given all of the evidence that is required in setting clinical recommendations, making coverage determinations and deciding on treatments, how does CER fit into those processes? Who will determine the extent to which CER affects access to therapies and technologies?

And how will the quality of care be affected for those patients who do not fit the "average" profile in terms of effective treatments? Given the current perception that patient differences and real-world evidence are not given a weighty priority in research, will that change as CER comes closer to fruition?

These questions underscore the importance of forthcoming National Pharmaceutical Council annual stakeholder surveys. With each year, as CER continues to progress, it becomes all the more imperative to understand the perceptions and expectations of those who will be overseeing transformative changes in American health care.







1717 Pennsylvania Avenue, NW • Suite 800 • Washington, DC 20006

Phone: 202.827.2100 • Fax: 202.827.0314

[www.npcnow.org](http://www.npcnow.org)