

2015
COMPARATIVE
EFFECTIVENESS
RESEARCH

AND THE
ENVIRONMENT
FOR **HEALTH CARE**
DECISION-MAKING

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Introduction

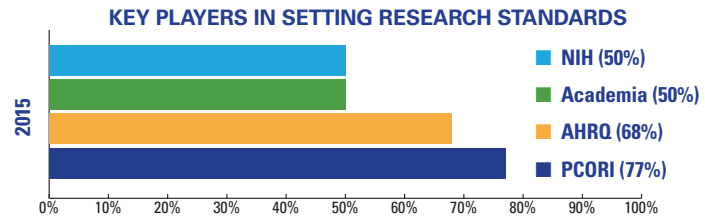
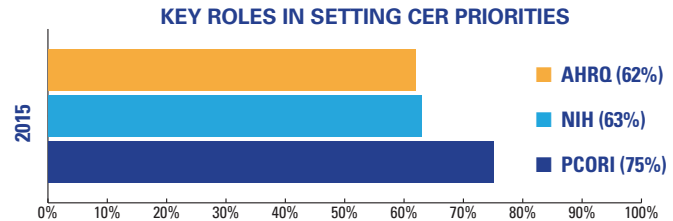
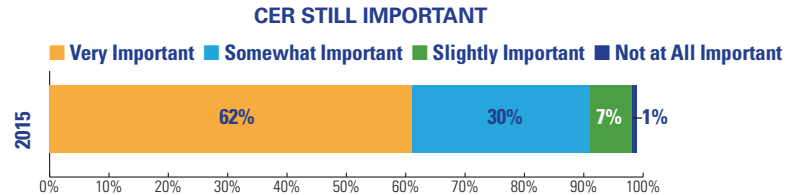
In the five years since the National Pharmaceutical Council (NPC) has been conducting this annual stakeholder survey, much has changed in the health care environment. We've seen the full implementation of the Affordable Care Act and its many provisions, including the creation of the Patient-Centered Outcomes Research Institute (PCORI), the development of health exchanges and a move toward value-based payments, most notably in Medicare programs. In all of this, what impact will comparative effectiveness research (CER) have on health care decision-making?

To understand its potential impact, it's important to first understand what CER is and its intention. CER is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions. By generating better information about different treatment alternatives, CER can provide patients, providers, payers and other stakeholders with the information to improve decision-making about treatments, coverage options and other issues affecting health care quality and outcomes.

Through this survey of stakeholders—insurers/health plans, government, employers, researchers/thought leaders, business coalitions and trade associations with ample awareness and knowledge of CER—NPC hopes to gain insight into whether CER has had an impact on health care and its use by stakeholders today. What we found is that many stakeholders have not seen much impact to date, but they believe in the importance of CER's role in health care decision-making and remain optimistic about the effects it will have over the next three to five years.

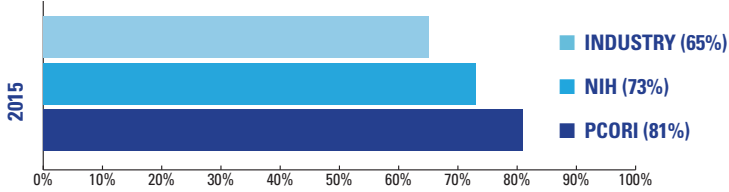
Executive Summary and 2015 Key Findings

The 2015 edition of the National Pharmaceutical Council’s comparative effectiveness research survey found that health care stakeholders, by and large, consider CER to be important today, but expect it to take on greater importance in the near future. The Patient-Centered Outcomes Research Institute is expected to take the lead in areas such as establishing research standards and priorities, as well as funding and monitoring research. Academia and the Agency for Healthcare Research and Quality are expected to lead on conducting research and translating and disseminating research, respectively.

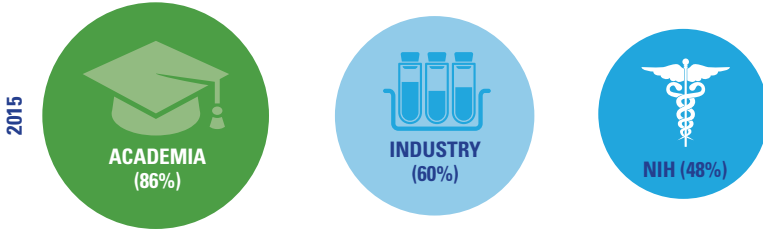




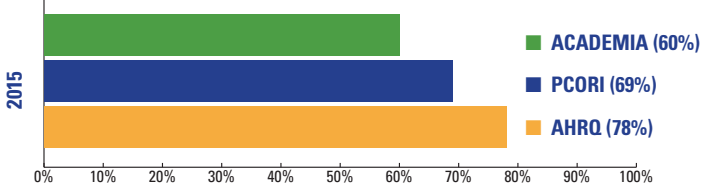
KEY ROLES IN FUNDING, MONITORING RESEARCH



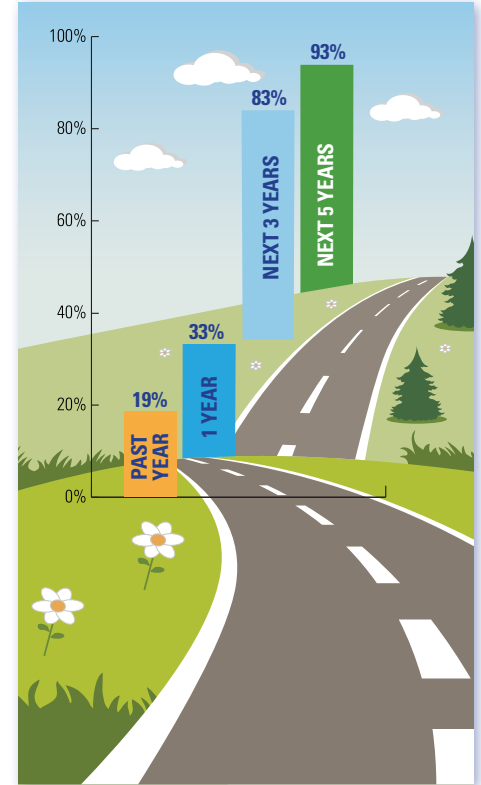
KEY GROUPS IN CONDUCTING CER



KEY PLAYERS IN DISSEMINATING CER



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 biopharmaceutical industry. N=122 for Stakeholders Surveyed. N=115 for Importance of CER. N=114 for Impact of CER in the Past Year and 1 Year; N=115 for the Next 3 Years and Next 5 Years. N=117 for remaining figures.



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

Background

Comparative effectiveness research is not a novel concept, but it has gained broader attention in recent years thanks to significant federal funding under the American Recovery and Reinvestment Act of 2009 and passage of the Affordable Care Act, which established the Patient-Centered Outcomes Research Institute.

PCORI was created to oversee and sponsor CER in the United States and provide health care decision-makers with current, relevant and reliable data that can enable them to make better, more informed choices about treatment and coverage options. During 2014 and the survey timeframe, PCORI was particularly active, issuing to date nearly three quarters of a billion dollars in grants for more than 365 different research studies and developing a clinical data infrastructure to enable research to be conducted faster and more efficiently.¹ This highly visible work has undoubtedly had an effect on the NPC survey results in strengthening the perception of PCORI's role in comparative effectiveness research.

At the same time, there are a number of other public and private sector organizations involved in this work—from individual

biopharmaceutical companies and private health plans to government institutions like the National Institutes of Health (NIH) and the Agency for Healthcare Research and Quality (AHRQ). Combined, NIH and AHRQ had nearly \$675 million in funding for CER in 2014.^{2,3} Academic institutions also have a long-standing engagement in this research, and many of these institutions have received federal or PCORI grants to conduct CER.

Because there are such diverse institutions engaged and interested in CER, there are many different perspectives on how this research should be conducted and used. Optimistically, CER would provide the data necessary to deliver the right treatment to the right patient at the right time, reducing problems and costs associated with over-treatment and under-treatment, improving the quality of care, and making health systems more cost-efficient and effective.

Pessimistically, CER would be used to define what treatment works best for the “average” patient, which could lead to a scenario in which access is limited for other treatments and therapies that fall outside the determined effectiveness range but may still be the best available treatments for particular individuals. This latter situation is being addressed by PCORI and other organizations that are requiring the studies they fund to take individual treatment effects into account as part of the study design.

As health care decision-makers grapple with how to use this research to inform patient care, NPC’s stakeholder survey takes on an increasingly greater importance. The impact of CER on the health care system will ultimately be determined by whether it is perceived as actionable information and utilized by stakeholders.

The NPC survey shows the state of play and the five-year 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. As questions continue to be raised about how CER will be integrated into health care decision-making and

whether the concept of comparative effectiveness and the movement toward pay-for-value health care will reconfigure the landscape of health care research, this survey sheds needed light on the importance stakeholders are placing on this research and the institutions that are performing, supporting and disseminating it.

“As health care decision-makers grapple with how to use this research to inform patient care, NPC’s stakeholder survey takes on an increasingly greater importance.”

¹ Patient-Centered Outcomes Research Institute. Research and Results: What We’ve Funded. Available at <http://www.pcori.org/research-results>.

² Department of Health and Human Services: Fiscal Year 2016. Agency for Healthcare Research and Quality. Justification of Estimates for Appropriations Committees. Available at <http://www.ahrq.gov/cpi/about/mission/budget/2016/cj2016.pdf>.

³ National Institutes of Health Research Portfolio Online Reporting Tools. Funding: Estimates of Funding for Various Research, Condition, and Disease Categories. Available at http://report.nih.gov/categorical_spending.aspx.

About the Research

This year's survey, conducted with Social & Scientific Systems, Inc., asked health care stakeholders to identify which organizations they perceived as playing significant roles in key areas of comparative effectiveness research, the current state of CER and its impact on decision-making, and to rate the status of several issues affecting the health care environment.

We sent surveys to 382 individual and organizational stakeholders starting on September 12, 2014, collecting 122 completed responses through January 17, 2015. To further encourage responses, we offered an incentive—a donation of \$50 to one of four listed charities for a completed questionnaire.

These stakeholders included researchers/thought leaders; government; insurers/health plans; employers; business coalitions; and associations (**Figure 1**). Within these stakeholder groups, the goal was to obtain a high-level perspective from individuals who are likely to be involved on a regular basis with CER and related issues and thus, were likely to have given serious thought to them. The individuals surveyed were not always the same each year because some individuals may have left or changed roles within their organizations. This shifting composition of respondents may be a factor impacting the survey trends over the years. Those who

responded that they were not at all familiar with CER were asked to send back the unanswered questionnaire.

FIGURE 1. Stakeholders Surveyed



Key Findings

Importance and Impact of CER

Since the initial survey five years ago, roughly the same percentage of stakeholders—over 90 percent—have continued to state that CER is “very” and “somewhat important.” This year, 62 percent found CER to be “very important,” with 30 percent responding “somewhat important.”

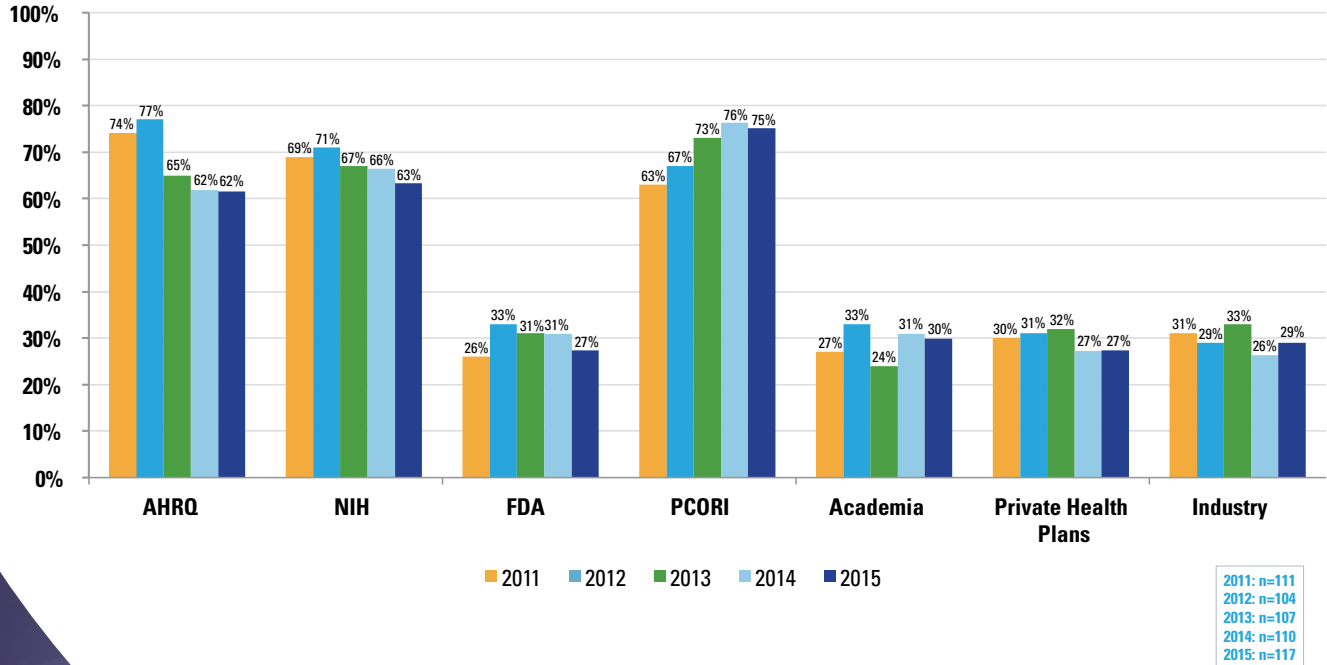
When asked whether CER has had an impact on health care decision-making in the past 12 months, 82 percent of respondents said that it had “no effect” or led to a “slight improvement,” while 19 percent said it led to a “moderate” or “substantial improvement.” Regarding the impact over the next 12 months, 33 percent of respondents anticipated CER will result in a “moderate” or “substantial improvement.” Respondents felt more confident about the impact of CER on health care decision-making over the next three and five years, with 83 percent and 93 percent anticipating “moderate” or “substantial improvement” over the next three and five years, respectively.

Role of Organizations in Developing, Funding and Disseminating CER

The increasing activity of the Patient-Centered Outcomes Research Institute not only in issuing research grants, but also in maintaining open and robust dialogues with stakeholders has likely had an impact on these survey trends. PCORI is widely regarded as one of the leading players in most aspects of comparative effectiveness research, with its numbers trending upward over the five-year span of the NPC survey.

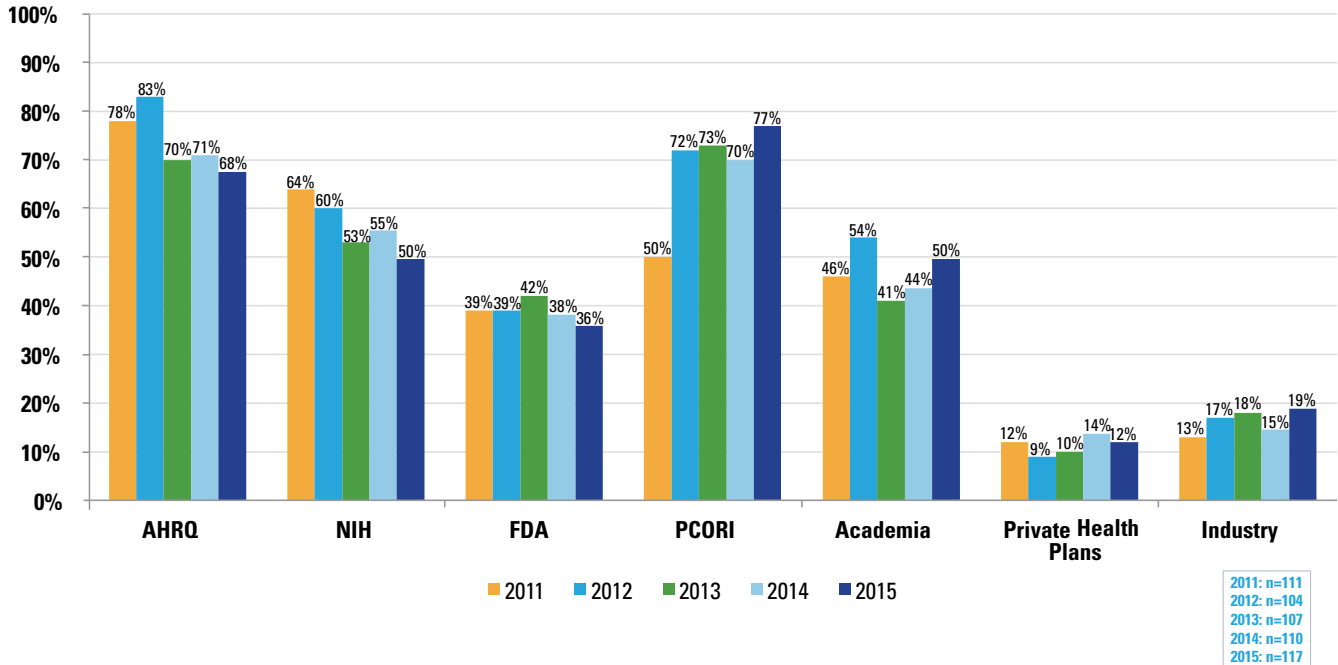
Setting Research Priorities. Stakeholders continued to view the same three organizations as leaders in setting the agenda for the types of research questions that will be answered by CER. These include PCORI (75 percent), the National Institutes of Health (63 percent) and the Agency for Healthcare Research and Quality (62 percent). **(Figure 2)**

FIGURE 2. Groups to Play a Significant CER Role in the Next Five Years: Establishing Research Priorities



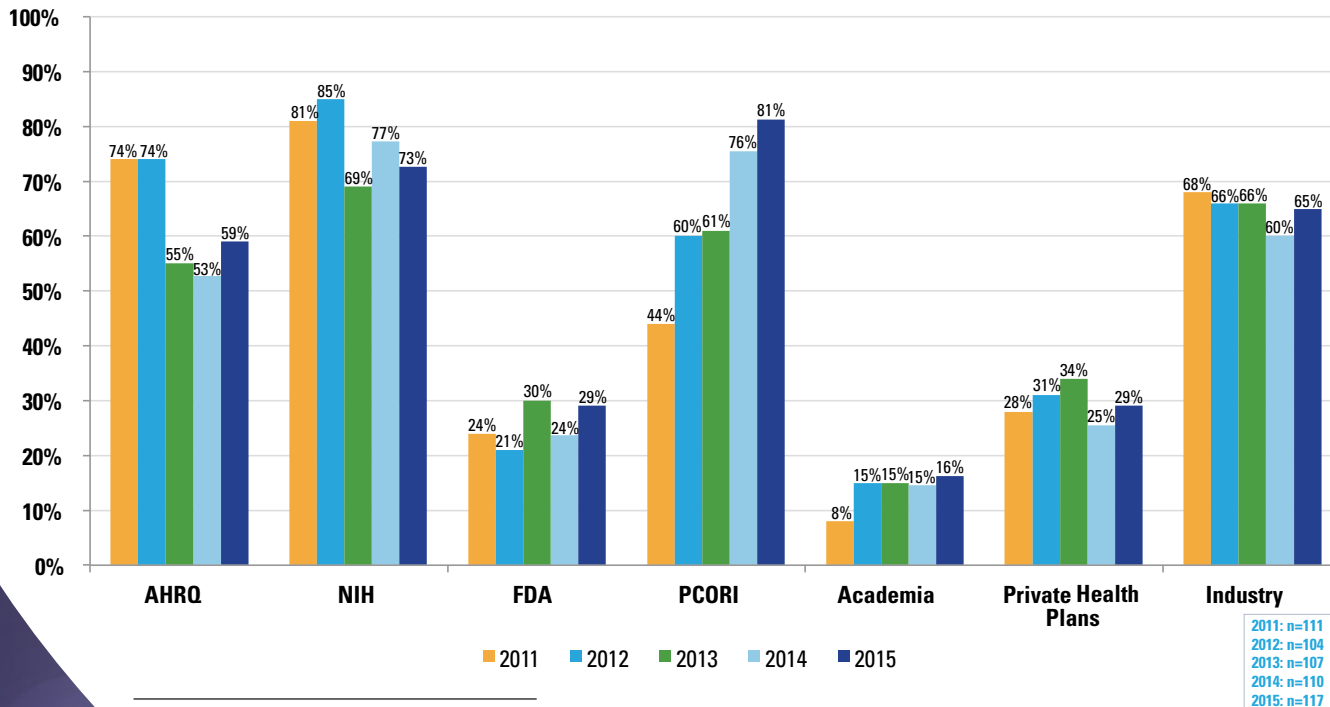
Establishing Research Standards. PCORI is perceived to have a leadership role in this area (77 percent), perhaps because it requires grant recipients to follow the research standards set out in its methodology report. PCORI was followed by AHRQ (68 percent), and NIH and academia were viewed as having equal roles (50 percent). (Figure 3)

FIGURE 3. Groups to Play a Significant CER Role in the Next Five Years: Establishing Research Standards



Funding and Monitoring Research. Awarding more than \$700 million in grants has unquestionably had an impact on stakeholders' perception of PCORI's role in financing research, and this growing impact is commensurate with PCORI's acceleration in grant activity.⁴ PCORI is recognized as taking the leading role in funding and monitoring research (81 percent), followed by NIH (73 percent), which has committed over \$600 million in grants, and industry (65 percent), which makes large investments in research. (Figure 4)

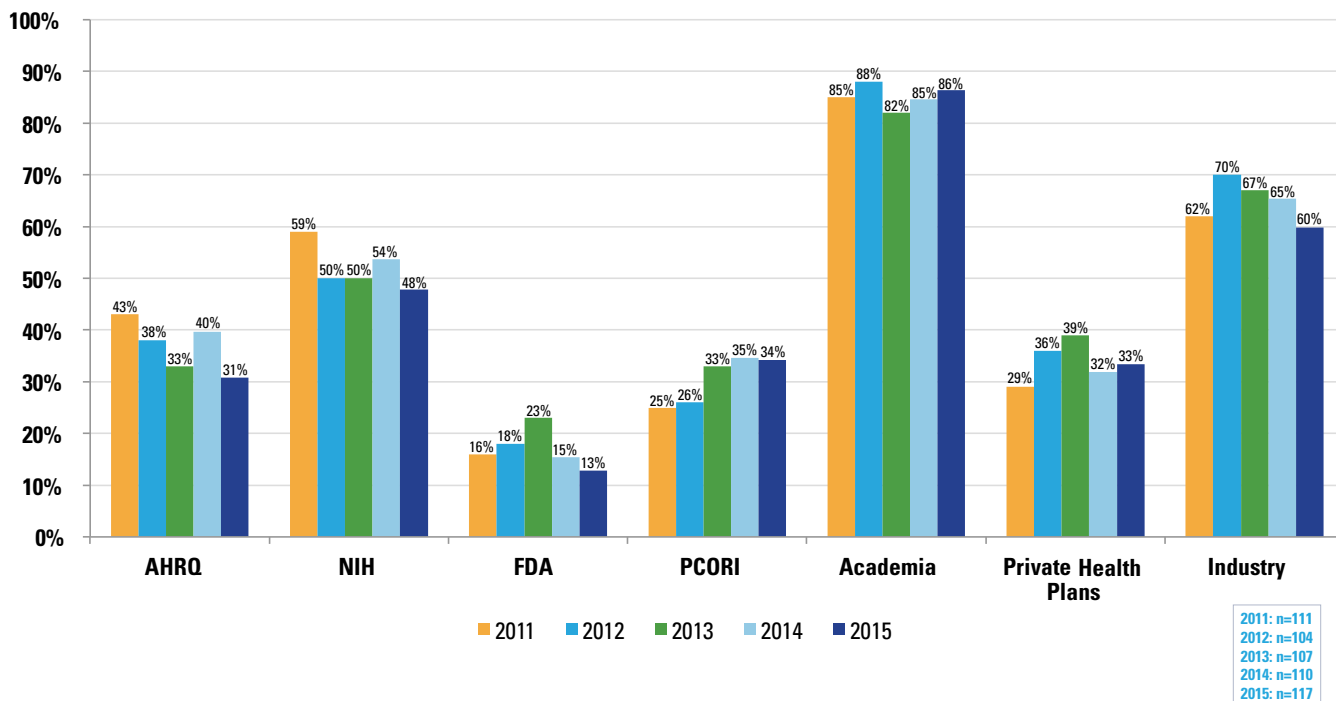
FIGURE 4. Groups to Play a Significant CER Role in the Next Five Years: Funding and Monitoring Research



⁴ Patient-Centered Outcomes Research Institute. Research and Results: What We've Funded. Available at <http://www.pcori.org/research-results>.

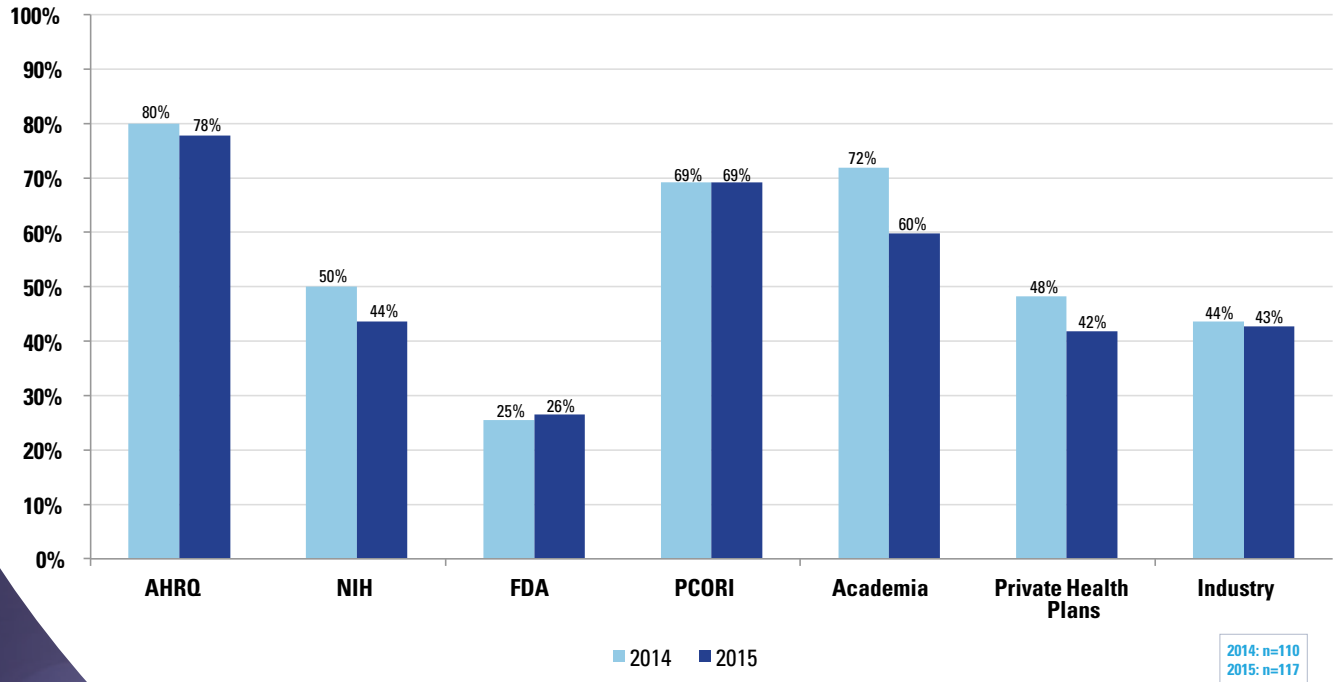
Conducting Research. Although PCORI is viewed as a leader in most surveyed areas, academia (86 percent) is clearly playing the most significant role in conducting research. It's not surprising, given that academic institutions have been awarded most of PCORI's grants. Industry (60 percent) also is expected to play a key role in conducting research, along with NIH (48 percent). **(Figure 5)**

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



Translating and Disseminating Research. This question was added to the survey in 2014, recognizing the importance of how CER is presented to stakeholders for possible action. AHRQ is assigned by federal law with the task of disseminating PCORI-funded research and is seen as the lead entity in handling these responsibilities (78 percent). AHRQ is followed by PCORI (69 percent) and academia (60 percent). (Figure 6)

FIGURE 6. Groups to Play a Significant CER Role in the Next Five Years: Translating and Disseminating Research



The Current Health Care Decision-Making Environment

While the first part of NPC's annual survey focused on comparative effectiveness research, the second part of the survey examined the status of several issues affecting the current health care environment, using a seven-point scale indicating a perceived negative, neutral or positive assessment.

Agreed-Upon Research Standards. Stakeholders remain optimistic that there is growing movement toward widely agreed-upon research standards, which would provide more consistency in the conduct and evaluation of CER. Today, **49 percent** of respondents acknowledged this trend, a statistically significant difference from 24 percent in 2011.

Research Priorities. Given the growing focus on patient-centered care, stakeholders were asked whether they felt that research priorities adequately addressed the treatment choices faced by patients and providers. This is an area that recovered lost ground and returned to the baseline level, with **41 percent** noting that research priorities somewhat and/or adequately reflected treatment choices in 2015. This is statistically significantly different than 22 percent in 2013 (the low-water mark for research priorities), and similar to the baseline of 37 percent in 2011.

Transparent and Objective Processes. When it comes to transparency in the processes used by decision-makers to interpret evidence, stakeholders have changed little in their views during the past five years. Slightly less than half of survey respondents said that there is no or little transparency in evidence evaluation.

Focus of Treatment Assessments. Many organizations have been making a concerted effort to bring patient values into treatment decisions, but stakeholders indicated that progress is still limited in this area. Fifty-eight percent of respondents said that the value of treatments remains narrowly focused on only clinical effectiveness, rather than taking into account factors that matter to patients, such as quality of life, workplace productivity, adherence to treatments and other outcomes.

Integrated Purchasing of Health Services. With the growth in accountable care organizations and use of quality measures, it's important to understand whether there is a shift toward medical purchasing that better integrates the impact of all health care services. When asked if they noticed a trend toward integration, nearly two-thirds of respondents said that purchasing health services trends toward a siloed view.

Outcomes-Based Contracting. The response to this question has not changed significantly during the last four years: **70 percent** of respondents felt there is little to no outcomes-based contracting.

In 2014, three new questions were added to the survey, so 2015 offered an opportunity to measure the year-over-year change.

Completeness of the Comparative Effectiveness Evidence Base.

When asked whether the breadth of evidence is “complete enough to inform the choices faced by patients and providers,” the response was unchanged. Here, **67 percent**—same as in 2014—said that there is not enough evidence available to answer treatment questions.

Use of Real-World Evidence. Stakeholders were roughly in line with their answer in 2014—**44 percent** said that use of real-world evidence is limited in decision-making. This response is not surprising; many health care stakeholders are still working to improve the methods, data quality and other factors hindering the broader use of real-world evidence in making formulary and treatment decisions.

Variability in Individual Patient Response. When it comes to accounting for variability in patient responses, **43 percent** of respondents felt that individual treatment effects are not taken into consideration, not significantly different than the 47 percent in 2014.

Conclusion

This annual survey provides us with a snapshot of stakeholders' perceptions of the key players in the main aspects of the CER process, from setting priorities to translating and disseminating the research findings. There are a few survey findings that have become clearer with each year: PCORI is the dominant player in this space, CER is important, and the goalposts for when we will see CER's impact are—still—three to five years down the field.

Many of the CER projects that have been funded by the 2009 government stimulus, PCORI, NIH and other entities in recent years will be coming to fruition within the next one to two years. As those findings are disseminated, we'll want to understand whether that CER will be taken up by stakeholders and if and when it will have an impact on health care decision-making.

And, as CER results become more prevalent, we'll also want to monitor how the health care environment continues to change. Will our evidence base become more complete with this new information? How will real-world evidence be considered in decision-making? Will research priorities more adequately address patient needs? The environment is evolving, but as with CER, those changes are likely still down the road.



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