Perspective

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Comparative effectiveness research in the USA: when will there be an impact on healthcare decision-making?

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Five annual surveys of healthcare decision-makers potentially affected by comparative effectiveness research (CER) indicate sustained recognition of its importance and potential impact. Initial expectations of immediate CER impact have over time turned to stakeholder assessments of little short-term impact. In successive surveys, they project a continuous horizon of 3–5 years for CER to have a moderate or substantial improvement in decision making. The prominence of Patient-Centered Outcomes Research Institute in CER is highlighted by stakeholders, but greater emphasis on translating and disseminating research findings is needed, a role in which Agency for Healthcare Research and Quality is expected to contribute. Stakeholders, including patients, must be engaged throughout to ensure that findings provide the flexibility for decision makers to apply them to their patient populations.

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Comparative effectiveness research (CER) in the USA has been around in some form for decades, beginning as early as 1972 with the Office of Technology Assessment, which advised Congress on health and other technology issues [1]. The purpose of CER has always been to inform decision-making, but there has been an increasing focus on serving a broader sphere of stakeholders, and increased momentum from a growing emphasis on improving population health and moving to value-based care. This new focus and momentum stems from the passage of the Affordable Care Act, which established the Patient-Centered Outcomes Research Institute (PCORI) and provides the current foundation of federal support for CER [2]. PCORI funding for CER through FY2014 totals nearly US$1 billion and is expected to be 'as much as US$1.5 billion' [3] for 2014–2016. By the end of FY2014, PCORI had made US$671 million available in awards [4]. In addition to PCORI, there are a number of public and private sector entities that play a role in funding or conducting CER – including the Agency for Healthcare Research and Quality (AHRQ), the NIH, academic institutions and pharmaceutical research firms.

Purchasers of healthcare – public and private payers, employers, and benefits managers/consultants – will be increasingly dependent on the results of CER to aid how they allocate resources to structure products and benefits. In order to understand the adoption and diffusion of CER and its impact on healthcare decision-making, the National Pharmaceutical Council (NPC) has sponsored five annual surveys of healthcare decision-makers from various arenas affected by CER. The purpose of this article is to present findings from these surveys and explore stakeholder perspectives in the context of this environment. For the purpose of this discussion, the Institute of Medicine definition of CER is used: 'CER is the generation

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Table 1. Overview of survey participants by stakeholder group.

CER survey respondents by categories	Survey year; number and percent of participants				
Stakeholder group	2011	2012	2013	2014	2015
Government	11	11	13	11	14
	10%	9%	11%	10%	10%
Researchers/thought leaders (Individuals)	32	34	32	40	45
	29%	29%	28%	35%	39%
Insurers and health plans	19	22	21	17	10
	17%	19%	18%	15%	7%
Employers	6	14	15	7	12
	5%	12%	13%	6%	10%
Business coalitions/HR specialists	34	30	26	27	25
	30%	26%	23%	24%	22%
Trade groups	10	6	7	11	16
	9%	5%	6%	10%	12%
Total	112	117	114	113	122
	100%	100%	100%	100%	100%

and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care' [5].

Methods

The NPC Assessment of 'Comparative Effectiveness Research and the Environment for Health Care Decision-Making' was conducted to obtain the perspectives of elite decision-makers in the new environment that focuses on CER and the application of its findings for healthcare decisions. As such, the sample was developed to be broadly representative of 'informed' opinions [6]. Six groups of stakeholders were targeted for inclusion in this survey; with one exception, these were organizational rather than individual stakeholders. Organizations included federal government agencies; health insurers, health management com-

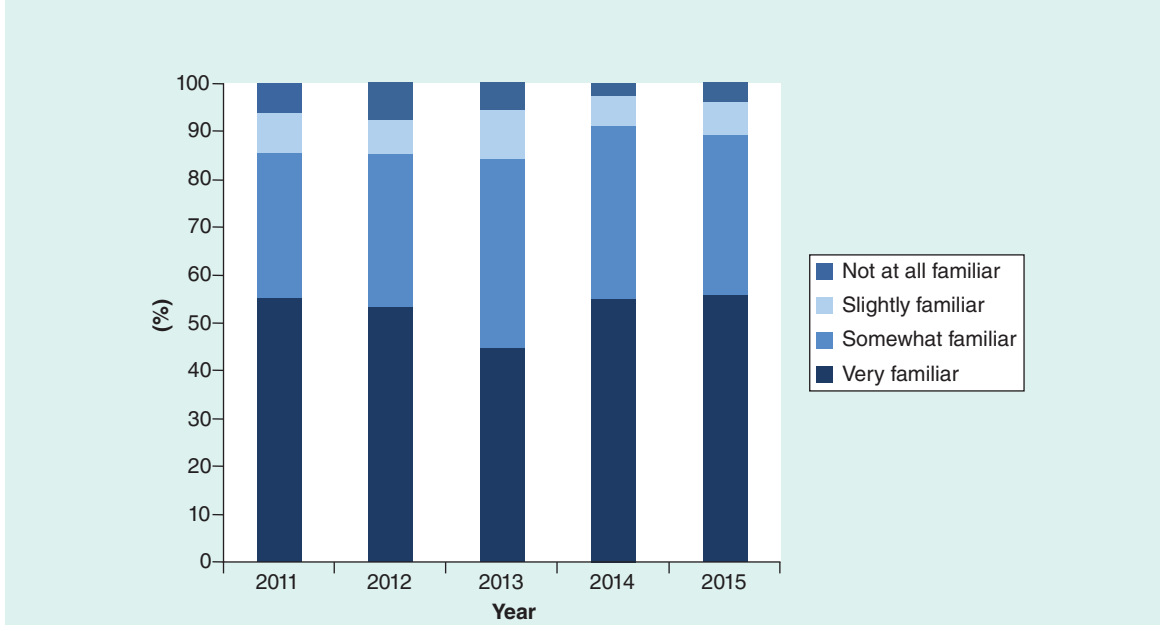

Figure 1. Respondent familiarity with comparative effectiveness research.

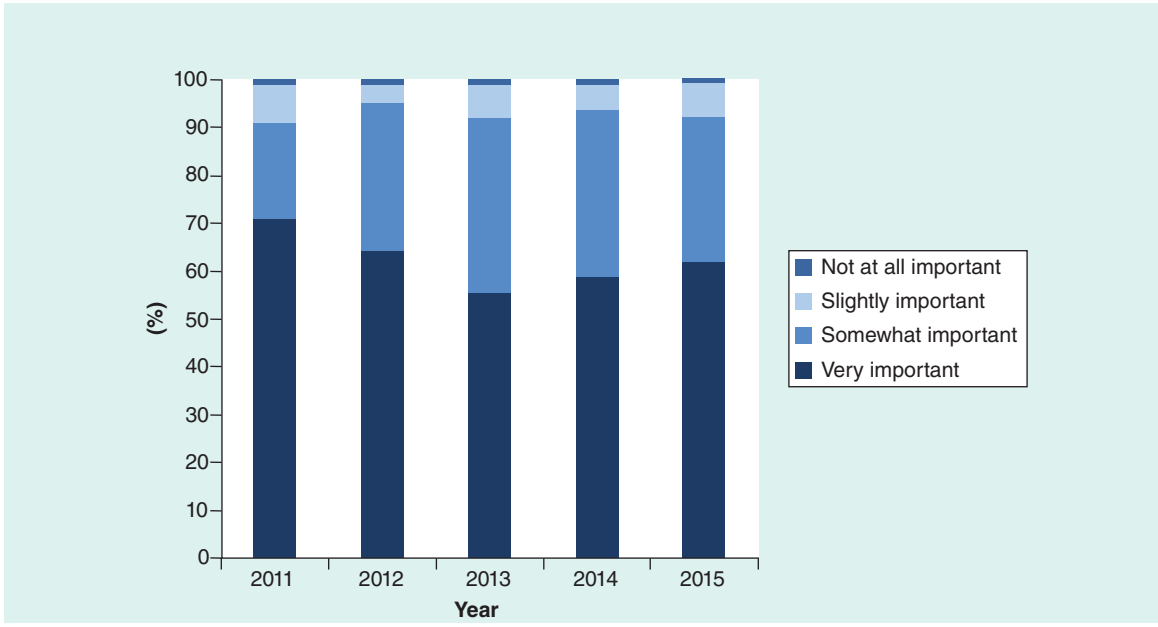

Figure 2. Perceived importance of comparative effectiveness research.

panies, and health systems; employers; business coalitions/human resources (HR) specialists; and trade or advocacy groups. The individual stakeholders were researchers/thought leaders. Because the focus was on the views of those who had thought seriously about the issues, respondents who indicated in the question-

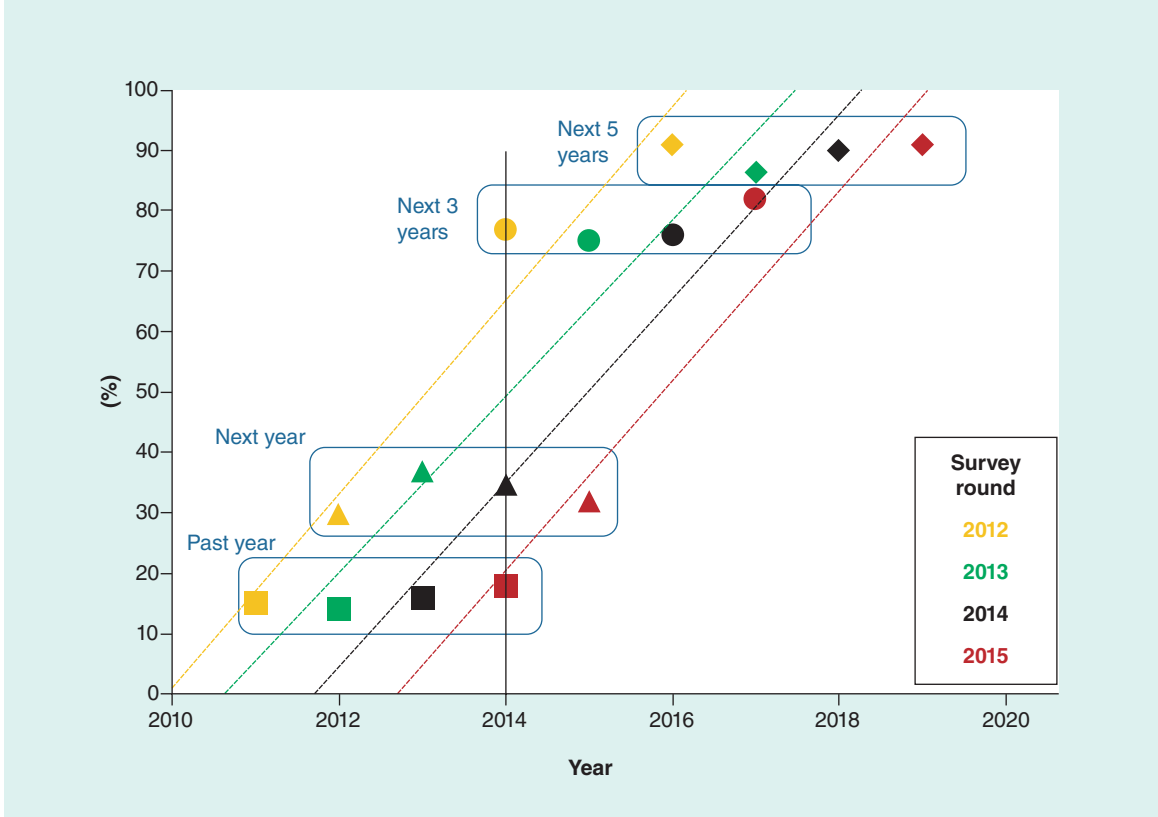

Figure 3. Comparative effectiveness research on the horizon.
Expectations of comparative effectiveness research's impact across four rounds of the stakeholder survey – percent of respondents answering 'moderate/substantial improvement' in decision-making from comparative effectiveness research in past year, next year, in 3 years and in 5 years.

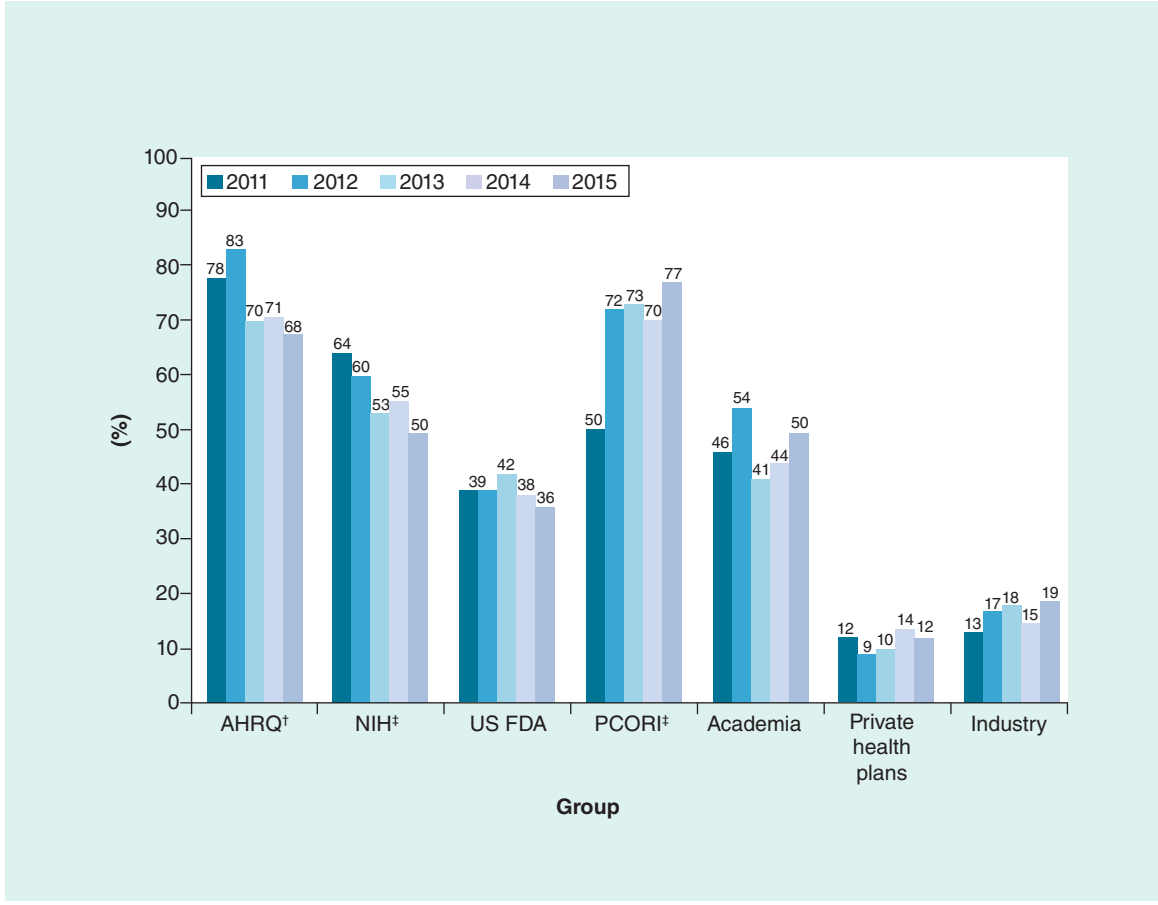

Figure 4A. Expected roles in comparative effectiveness research – establishing research standards: PCORI, AHRQ have leadership roles. Groups expected to play a significant role in the next 5 years.
†Change between 2012 and 2015 statistically significant ($p < 0.05$).
‡Change between 2011 and 2015 statistically significant ($p < 0.05$).
AHRQ: Agency for Healthcare Research and Quality; PCORI: Patient-Centered Outcomes Research Institute.

naire that they were 'not at all familiar' with CER were screened out at the initial stage. Due to the small sample size of each group of stakeholders, meaningful comparisons between groups of participants cannot be made.

Stakeholder organizations were identified through a variety of sources, including an internal list of CER stakeholders developed by NPC and membership lists from the National Business Group on Health, the National Business Coalition on Health, and America's Health Insurance Plans. Researchers and thought leaders were identified from two main sources: a literature review and the AHRQ clinical research centers (CERT, DEcIDE and EPC Network). The literature search used the term 'comparative effectiveness' on common search engines, such as PubMed, Medline, and EBSCO's Academic Search Complete, along with the health journals Health Affairs, JAMA, and Journal of Managed Care. Results were limited to articles written in English and published in the past 5 years. The time requirement was shortened to 2 years to include only a reasonable number of results from each source. Authors with more than one article in a search were included in the sample.

Appropriate federal government agencies – primarily within the Department of Health and Human Services – were selected based on their involvement in health-related matters. In addition, Congressional staff working on relevant Senate and House committees were identified by NPC and added to the sample.

Within each organization in the sample, an appropriate position type was identified as a point of contact. Government respondents were typically the directors of relevant offices. The chief medical or pharmacy officer was selected for health organizations. For employers, it was the head of human resources or director of benefits. For human resource specialists, it was the director of benefits, and for local business groups on health, the executive director. The person most likely to be involved with CER at each trade group was identified

as well. Whenever possible, stakeholders in each year's sample were included in the next year's sample. Within the selected organizations, respondent substitutions were allowed if the originally targeted respondent suggested a more appropriate informant. A decision was made to cast the net as broadly as possible to obtain a diverse set of respondents. In addition, because the sample consisted of very high-level, busy professionals not inclined to respond to surveys, including a large sample was intended to assist in obtaining a reasonable number of responses.

Five rounds of the survey have been conducted. Telephone calls were made to encourage respondents to complete the questionnaire either by web or hard copy; multiple mailings, emails, and phone calls were made to each sample listing. In addition, to further encourage responses, we offered an incentive – a donation of US$50 to one of four listed charities was given for a completed questionnaire. Each year the field periods began in mid-September and lasted through mid-January.

The distribution of survey participants, by stakeholder group and survey year, is shown in Table 1. Because the list of organizations and researchers invited to and agreeing to participate in this elite stakeholders' survey each year changed somewhat, there are limitations to the inferences that can be drawn from the survey findings. Nonetheless, across the 5 years, the results paint a broad picture of the views of healthcare decision-makers with respect to CER.

Findings

Knowledge & expected impact

Elite decision-makers responding to the survey indicated substantial familiarity with CER across all the survey periods. Eighty-five percent in 2011 and 90% in the 2015 survey indicated they were 'very familiar' or 'somewhat familiar' with CER (Figure 1). The proportion of respondents regarding CER as 'very important' or 'somewhat important' was also high, 91% in 2011 and 92% in 2015; however, while 71% of respondents rated CER as 'very important,' the percentage was smaller in each of the succeeding surveys. In 2015, 62% rated CER as 'very important' (Figure 2).

In each round of the survey, respondents were asked how much impact they feel CER has had or will have in the future. Few respondents considered CER to have had a substantial impact in the past 12 months.

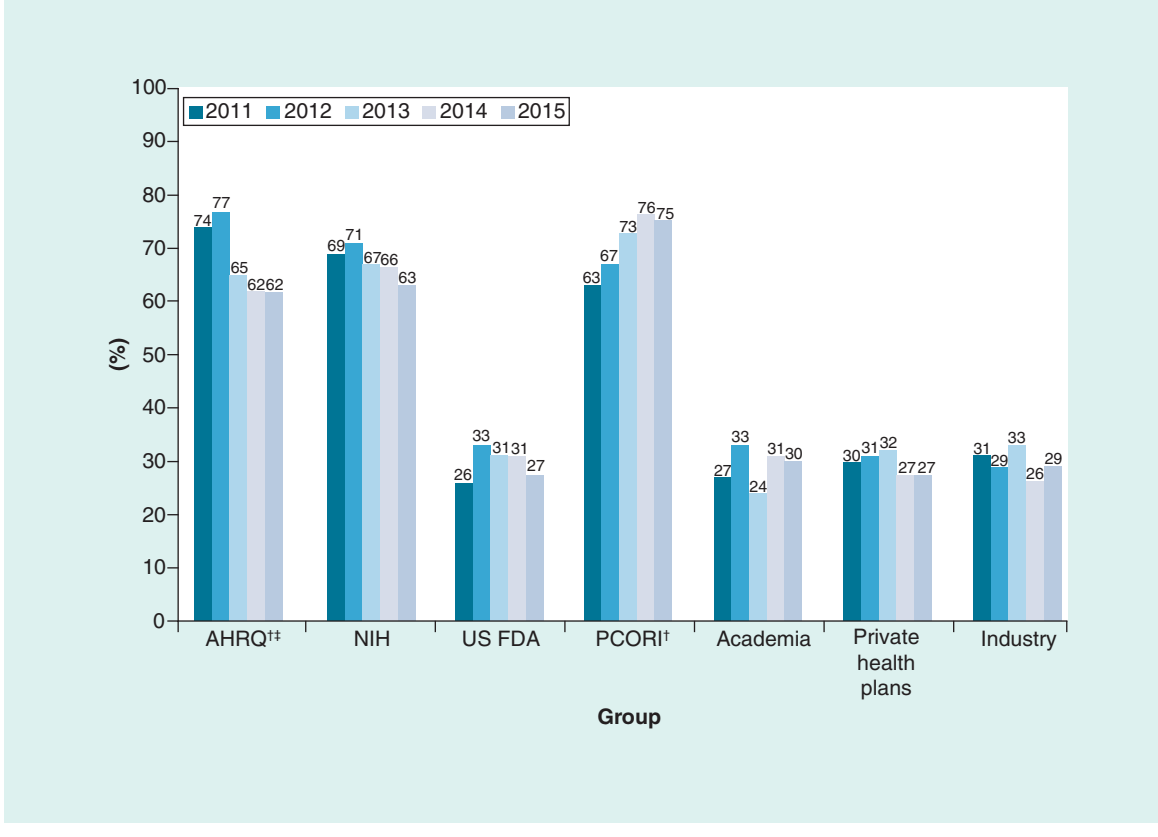

Figure 4B. Establishing research priorities: PCORI, NIH, AHRQ are key players.
†Change between 2011 and 2015 statistically significant ($p < 0.05$).
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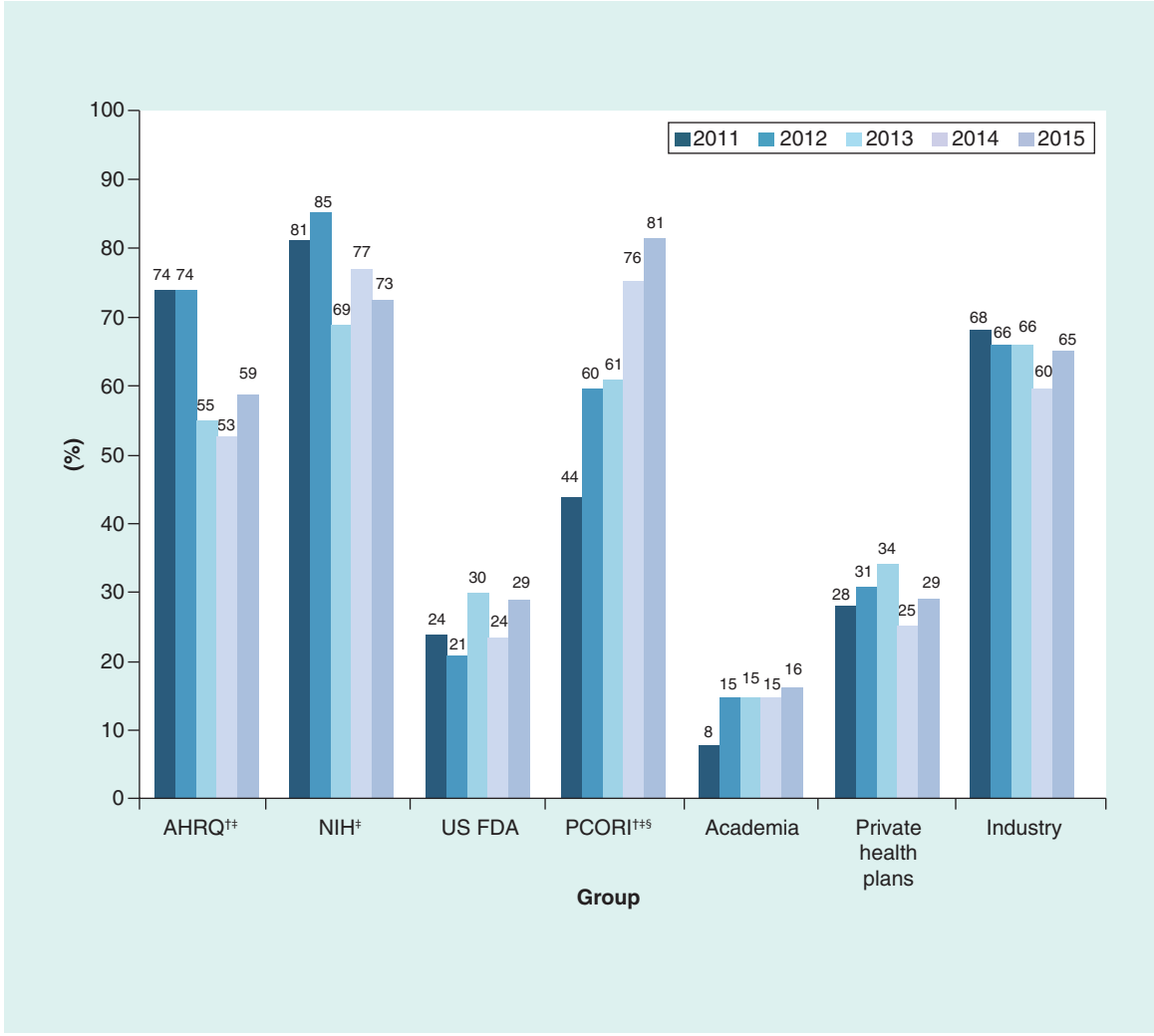

Figure 4C. Funding and monitoring research: PCORI efforts recognized; NIH and industry play key roles.
†Change between 2011 and 2015 statistically significant ($p < 0.05$).
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Most expect that CER will have a positive impact on decision-making in the future, and almost none of the respondents asserted that CER's impact is or will be negative.

Regarding future impact, respondents in the 2012–2015 surveys were asked about the impact of CER over the next 12 months, the next 3 years, and the next 5 years. In each of these surveys, 30–40% of respondents believed that CER would have a 'moderate/substantial improvement' on decision-making during the next year. However, most respondents, 75 to 91% across the survey years, expect CER to have a 'moderate/substantial improvement' over a 3- to 5-year horizon. Figure 3 displays the horizon for CER impact in each survey by plotting the percentage of responses against the year in which a 'moderate/substantial improvement' is expected. This figure illustrates the continued long-term view held by most respondents for CER's positive effects on healthcare decision-making.

Figure 3 also allows a retrospective (past 12 months) comparison of perceptions of CER's impact with future expectations among different cohorts. This comparison provides a revealing insight into shifting perceptions about when CER's impact will be felt. The vertical bar in Figure 3 represents different cohorts' opinions about the impact of CER on decision making in 2014. Of those looking backwards on 2014 (the 2015 cohort), only 19% believed CER had led to a 'moderate/substantial improvement' in 2014. Of those looking ahead 12 months (the 2014 cohort), 37% predicted a 'moderate/substantial improvement'

in 2014, while 77% of those looking ahead 3 years (the 2012 cohort) predicted a 'moderate/substantial improvement' in 2014.

These results indicate that many respondents expected a greater impact from CER than they had observed in reality. In part, this may help to explain the decline in the proportion of respondents rating CER as 'very important' between 2011 and 2015 – high initial expectations of immediate impact have not been met and respondents are lowering their expectations of the importance of CER. Despite their disappointment at the pace of CER, respondents continued to assign high values to both its importance and future impact.

Key contributors to CER

Survey respondents were asked to identify the agencies or groups they expected to play a significant role over the next 5 years in the following CER areas: 'establishing research standards,' 'establishing priorities for research,' 'funding and monitoring research,' 'conducting research,' and 'translating and disseminating research.' Respondents were asked to select 'all that apply' from the following list of agencies/groups: AHRQ, NIH, US FDA, PCORI, academia, private health plans, and the pharmaceutical–medical products industry.

One notable shift is the rise of PCORI as an entity expected to play a significant role in establishing CER standards, priority setting, and funding in the next 5 years. Growing understanding of PCORI's role among elite healthcare decision-makers is reflected in the rapid increase in the share of respondents who identified PCORI as having a significant role in establishing research standards, as well as funding and monitoring CER (Figure 4A–E). In practice, this is where PCORI has had a major impact, by releasing a methodology report [7] and making nearly half a billion US dollars in grants annually.

Expectations of AHRQ's role in establishing CER standards, setting priorities, and funding and monitoring CER have decreased over time as PCORI's anticipated role has increased in these areas, although AHRQ is still expected to play a key – but supporting – role in establishing standards and setting priorities. However, consistent with AHRQ's responsibility for disseminating patient-centered outcomes research

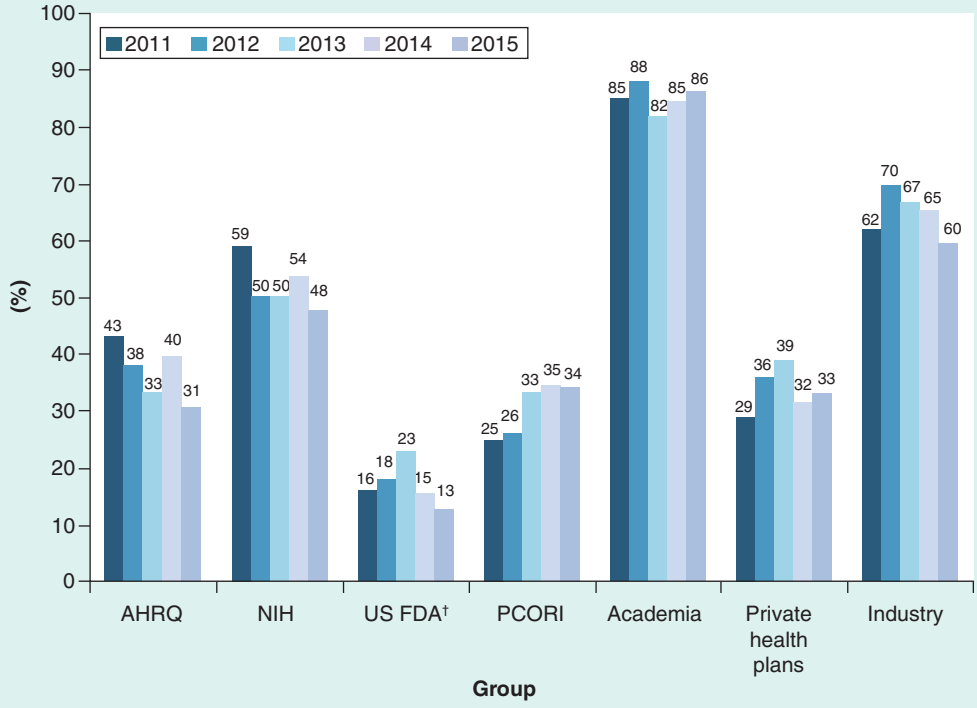

Figure 4D. Conducting comparative effectiveness research: considered the domain of academia and industry.
†Change between 2013 and 2015 statistically significant ($p < .05$).
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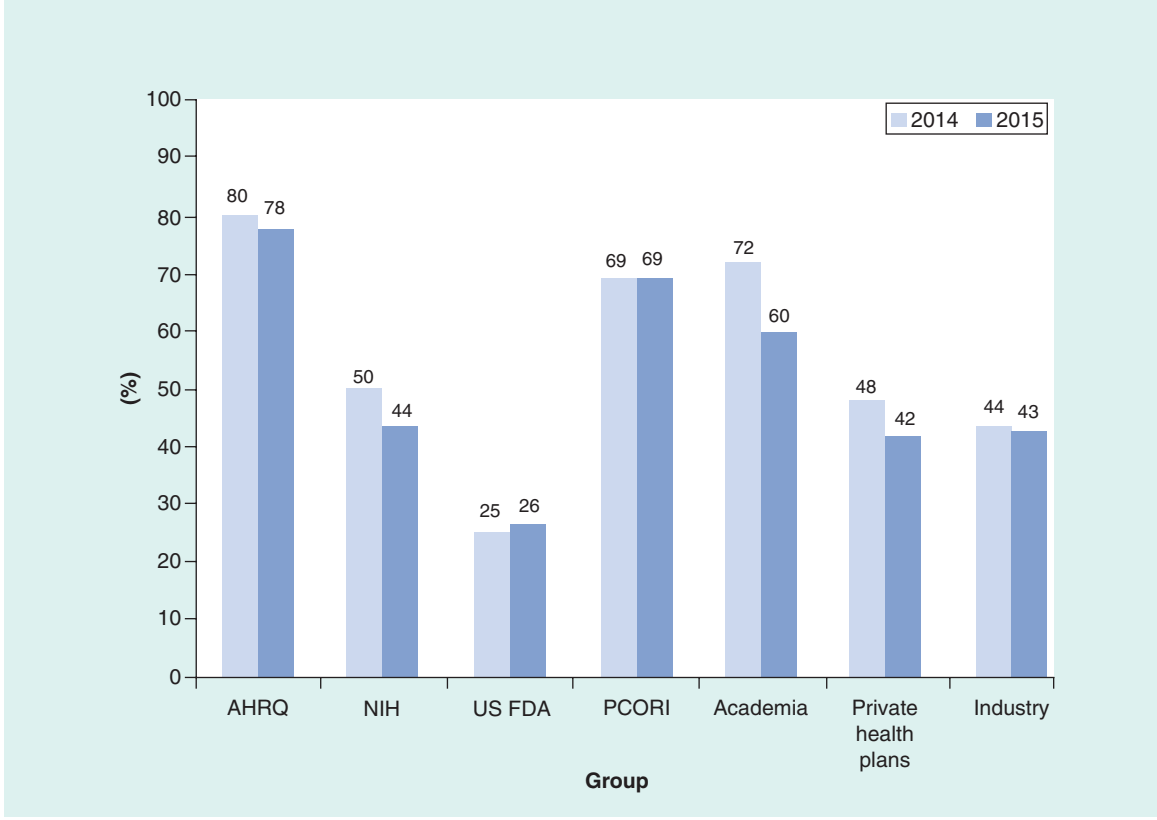

Figure 4E. Translating and disseminating research: role for AHRQ, academia and PCORI.
AHRQ: Agency for Healthcare Research and Quality; PCORI: Patient-Centered Outcomes Research Institute.

under federal law, AHRQ is expected to have primary responsibility for translating and disseminating research.

Similar to AHRQ, NIH's anticipated role in establishing CER standards, funding, and monitoring CER has diminished somewhat over the survey years. Despite the small decline, NIH continues to be viewed as having key roles in funding and monitoring research, as well as establishing research priorities.

The biopharmaceutical–medical products industry has consistently been identified as having a key role in both funding and monitoring CER research, as well as in conducting the research. Perceptions of academia also have remained relatively stable over the years, with anticipated responsibilities for conducting the research, as well as translating and disseminating it.

Neither the US FDA nor private health plans have surfaced as expected key players in any of the areas. The proportion of responses for funding and monitoring research, and conducting research has remained consistently low for US FDA and private health plans. Although the change in results over survey years has oscillated, there have been no secular trends.

Discussion

The survey results presented here indicate continued widespread awareness of the majority of CER issues among stakeholder groups of interest. Perceptions about which organizations play significant roles in creating research standards, creating research priorities, and funding and monitoring research show shifts in the perceptions of PCORI's growing role, consistent with its increasing prominence. For conducting research, and for translating and disseminating research, expectations remained fairly constant over time. However, translating and disseminating research has only been explored in the two most recent years of the survey. Future years of the survey will include 'patient groups' as a response option in this section.

The majority of respondents continue to feel that CER has had little impact on healthcare decision-making in the past year. However, respondents are more optimistic about the impact of CER in the next 3–5 years. This finding conforms to the literature – a substantial majority of payers in 2012 (82% of survey participants and 7 of 9 roundtable participants) believed that CER would have an increased role in formulary decisions over the next 5–10 years [8].

Given the abundance of published comparative effectiveness research, why are stakeholders so tentative in their assessment of the influence of CER in the near term? The literature points to a variety of barriers, including: inconclusive or ambiguous results, perceived poor research quality, failure of the research to address the needs of the end users, the capacity of providers and payers to interpret and apply CER, and limited use of decision support by patients and clinicians [9–11]. The recent flurry of CER activity can be expected to yield findings that supplement the evidence base behind more than 2300 evidence-based guidelines already in existence, including 314 guidelines that were published or revised in 2014 alone [12]. The capacity of clinicians, payers, purchasers, and – most of all – patients to assimilate this voluminous and ever-changing information is likely to be a major barrier to adoption of CER findings. The lack of major movement in the views of these elite stakeholders may reflect a need for new methods for incorporating CER findings into decision-making.

While dissemination can increase awareness of CER findings, the financial and organizational incentives clinicians face will influence the extent to which they can apply these findings [10]. Atkins and Kupersmith, drawing on the experience of the Veterans Administration, argue that implementation needs to be an explicit component of CER studies from the very beginning with the needs of end users driving research questions. They also see the need for alignment of incentives and the support of leadership in favor of implementation [13]. These observations suggest that until a critical mass of stakeholders – clinicians, health delivery system administrators, payers, and purchasers – across the healthcare landscape come on board, the application of CER findings will be limited. It is in this context, perhaps, that survey respondents' perception of a limited impact from CER can be better understood.

Conclusion & future perspective

Belief in the importance of CER is largely unwavering, but its impact is yet to be realized or acknowledged by stakeholders in the USA. The impact of CER on medical decision-making remains an ever-moving target that is at least 5 years in the future despite the rapid increase in CER activity over the past several years. It seems clear that the current CER enterprise is about more than just producing new research findings. For example, CER work funded by PCORI has included a focus on the methodological underpinnings and proper conduct of CER, the need for building a broader base of researchers, and engaging patients and stakeholders throughout the conduct of research. Considerable funding has been provided for new research that is grounded in these fundamental elements of CER. But the products of that effort and the cultural shift in conducting research are still mostly to be realized. Although there is a substantial amount of research that has appeared from many other sources, there is, as might be expected, little if any coordination of evaluation and interpretation of the results.

Without some uniformity in interpretation, it is perhaps not surprising that few stakeholders see significant impact of CER in the short term. The new efforts that make up the overall CER agenda can benefit from improved plans for translating the eventual findings into information that is readily available, addresses real world questions, and can be applied to medical decisions that are relevant to specific populations and the parameters within which decision makers must operate. The readily acknowledged importance of CER offers fertile ground for coordinated and sustained efforts to translate the results of new CER that satisfies the methodological criteria for high-quality research.

As part of these efforts, both PCORI and AHRQ have outlined a broader framework for translating and disseminating research, engaging stakeholders in prioritization, conduct, and dissemination of the research rather than simply as recipients of the findings. PCORI has built features into its research process aimed at reducing the time it takes for evidence to be incorporated into clinical practice, an average of 17 years according to previous research [14]. Including key stakeholder organizations throughout the research process helps ensure the research questions are directly on target for these stakeholders, which should enhance adoption of the findings. This engagement throughout the research process enhances stakeholders' ownership of the findings and presumably their willingness to assist with dissemination. It is important to note, however, that the process also requires sustained funding. PCORI funding, in particular, is tied to the Accountable Care Act, which to date has survived numerous efforts to reduce its scope. Demonstrating positive effects of CER and PCORI's work would be important to sustaining its support.

Thus, while most want change for the better in the use of CER, change will be gradual. Future surveys should identify some of the signals that change is occurring – increased involvement of patients and other stakeholders, for example. These signals can reflect a sharper focus on translation to practice so that future measurement of CER stakeholder views will show steadily improving assessments of the impact of CER on medical decisions.

Financial & competing interests disclosure

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Executive summary

- Annual surveys of healthcare decision makers found high awareness and familiarity with comparative effectiveness research (CER) and sustained perception of its importance.
- Although most of these stakeholders believe CER will have a positive impact, they report little impact in the recent past and see little in the short-term future.
- In each of five successive surveys, stakeholders indicated that substantial CER impact is at least 3–5 years in the future.
- Stakeholders note the growing significance of Patient-Centered Outcomes Research Institute's role in establishing research standards and funding and monitoring CER, and they expect Agency for Healthcare Research and Quality to have a leading role in translating and disseminating research.
- The recent surge in CER activity will add to the evidence base, but significant barriers to effective use of this evidence will remain without improved efforts to translate and disseminate findings.
- Stakeholders will realize greater impact from CER only if new efforts provide information that is readily available and can be applied within their healthcare systems to real-world decisions in specific patient populations.

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