PhRMA FOUNDATION

AMCP Academy of Managed Care Pharmacy®

COMPARATIVE EFFECTIVENESS AND PATIENT-CENTERED OUTCOMES RESEARCH:

Enhancing Uptake and Use by Patients, Clinicians and Payers

CONFERENCE REPORT
March 2017

Executive Summary

The purpose of comparative effectiveness research (CER) is to facilitate decision-making and improve health outcomes by developing and disseminating to patients, clinicians, and other decision-makers, evidence about which interventions are most effective under specific circumstances. Today, we have significant resources and expertise in the conduct of CER and patient-centered outcomes research (PCOR). This increased capability, combined with the increasing demand for evidence to support value-based decision making, has caused a rapid accumulation of CER/PCOR evidence in the literature. However, there is a need to better integrate CER/PCOR evidence into decision-making. In January 2017, the Pharmaceutical Research and Manufacturers Association (PhRMA) Foundation, together with the Academy of Managed Care Pharmacy (AMCP) held an invitational conference in Washington, DC. titled "Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians, and Payers." The conference was attended by 70 experts and opinion leaders representing clinicians, patients, government, academia, and payers. The specific goals of the conference were to (1) provide an overview of the existing landscape on strategies to enhance uptake and use of CER/PCOR by patients, clinicians, and payers; (2) identify and discuss the needs and gaps in the uptake and use of CER/PCOR evidence by patients, clinicians, and payers; (3) identify the best methods or approaches to enhance the uptake and use of CER/PCOR evidence by patients, clinicians, and payers; (4) provide an opportunity for networking among attendees; and (5) develop a consensus document or other enduring material that provides benefit beyond the conference by providing a framework for recommendations and tools for training current and future users of CER/PCOR evidence. The conference proved to be highly valuable to those in in attendance – it provided a venue for leaders and key stakeholders in the field to learn from each other's collective experiences and share new ideas. The conference organizers plan to submit a supplement to be published in the Journal of Managed Care Pharmacy which are intended to be enduring materials capturing the experience and discussion shared at the conference, and to inform interested parties by providing frameworks for recommendations and tools for training current and future users of CER/PCOR evidence.

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I. Introduction

Spending on health care in the United States (US) exceeds that of all other countries both regarding total dollars and as a percent of GDP, yet data are mixed on the value received for money spent.[1] For example, in 2007 the US ranked twenty-third in life expectancy among Organization for Economic Cooperation and Development (OECD) countries.[2] The discrepancy between health care spending and health outcomes has also been apparent in regional variations in medical practice within the US.[3,4] These gaps suggests not only a lack of consensus regarding the effectiveness of treatment interventions but also opportunities to reduce cost while improving quality. Moreover, these data highlight that patients, caregivers, clinicians, and policy-makers lack either access to or the ability to use objective, scientifically-derived evidence comparing the relative merits of the treatment options they can select.

Comparative effectiveness research (CER) has offered a potential solution. CER, which gained attention over the last 10-15 years, 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 in real-world settings."[5] The purpose of CER is to facilitate decision-making and improve health outcomes by developing and disseminating to patients, clinicians, and other decision-makers, evidence about which interventions are most effective under specific circumstances.

Early efforts to support CER focused primarily on the development of research methods and on the training of researchers. For example, as early as 2006, the Agency for Healthcare Research and Quality (AHRQ) held conferences about methods in CER and published related resources.[6-8] With the advent of "patient-centered outcomes research" (PCOR) and the Patient-Centered Outcomes Research Institute (PCORI) in 2010, such efforts expanded.[9] Importantly, funding to train CER researchers was made available by AHRQ, PCORI, the National Institutes of Health (NIH), the Pharmaceutical Research and Manufacturers Association (PhRMA) Foundation, and others in the form of individual and institutional training awards, conference grants, center grants, and contracts.[9]

Today, we have significant resources and expertise in the conduct of CER and PCOR. As a result, CER/PCOR evidence is rapidly accumulating in the literature.[10] However, there remains a need to better integrate CER/PCOR evidence into decision-making.[11-15] A major limitation may be that potential users of this evidence need to be educated on the strengths and weaknesses of CER and its place within the hierarchy of evidence levels. In fact, a recent survey identified user application of CER and its role in

decision-making among the top educational needs.[11] Other surveys reported that the majority of health care professionals were not adequately prepared to use CER/PCOR.[13] Others have noted that insufficient attention is paid to communication about CER/PCOR evidence to end-users.[12,14] Together, studies point to the need for better: education of users of CER/PCOR, understanding of the barriers to using CER/PCOR evidence, design of effective strategies and tools to ensure uptake and use of CER/PCOR by clinicians, patients, payers/policy-makers. Lastly there is a need to teach patients, payers, and policy makers how to use CER results to achieve value in purchasing products and services.

On January 26-27, 2017 the PhRMA Foundation, together with the Academy of Managed Care Pharmacy (AMCP) held an invitational conference in Washington, DC. Titled "Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians and Payers", the conference was attended by 70 experts and opinion leaders representing clinicians, patients, and payers. This conference report provides a summary of the conference overview, goals and proceedings, themes and recommendation surrounding uptake of CER/PCOR, and includes the next steps planned.

II. Conference Development

Drs. Glen T. Schumock and A. Simon Pickard, awardees of the one of the most recently funded PhRMA Foundation's Center for Excellence in Comparative Effectiveness Research and Education chaired the conference planning committee. This highly collaborative conference planning committee joined them (**Box 1**) to operationalize and execute the conference activities.

Box 1 – Conference planning committee

NAME	ORGANIZATION
Glen Schumock	University of Illinois at Chicago
Simon Pickard	University of Illinois at Chicago
Ernest Law	University of Illinois at Chicago
Beth Devine	University of Washington
Eleanor Perfetto	University of Maryland
Elisabeth Oehrlein	University of Maryland
Eileen Cannon	PhRMA Foundation
Joe Vandigo	PhRMA
Jean Paul Gagnon	PhRMA Foundation
Soumi Saha	Academy of Managed Care Pharmacy

The conference planning committee served several functions. First, the committee identified potential invitees. Invitees were known contributors (e.g. academicians) or representatives of key users (e.g. patient

advocacy groups, professional organizations, managed care organizations, and industry) to CER/PCOR. Second, the committee deliberated over specific conference aims (**Box 2**). As the theme was "*enhancing uptake of CER/PCOR,*" the committee agreed that barriers to uptake needed to be identified, followed by strategies to overcome such barriers. Third, the committee considered the conference format. It was recognized that issues surrounding knowledge translation are complex; therefore, a smaller conference format was thought to be advantageous for facilitating discussions, through networking and/or formal breakout sessions. Finally, the committee acknowledged that, in anticipation of fruitful discussion among attendees, there should be a concerted effort to create enduring materials that summarize the issues and resolutions formulated during the meeting. Therefore, the committee agreed to continue collaborations even after conference conclusion to ensure such documents would be developed and disseminated.

Box 2 – Conference aims

Aim #1: Provide an overview of the existing landscape on strategies to enhance uptake and use of CER/PCOR by patients, clinicians, and payers.

Aim #2: Identify and discuss the needs and gaps in the uptake and use of CER/PCOR evidence by patients, clinicians, and payers

Aim #3: Identify the best methods or approaches to enhance the uptake and use of CER/PCOR evidence by patients, clinicians, and payer.

Aim #4: Provide the opportunity for networking among attendees

Aim #5: Develop a consensus document or other enduring material that provides benefit beyond the conference by providing a framework for recommendations and tools for training current and future users of CER/PCOR evidence

A. About the Participants

The conference engaged the "users" of CER/PCOR, defined for the purposes of recruitment as patients, clinicians, and payers. The aim of including representation across these key stakeholder groups was to foster better understanding of challenges faced by each unique group and to encourage greater adoption of successful strategies across user groups. The 70 invited participants represented academic institutions, professional associations, healthcare provider groups, insurance companies and other payer organizations, patient advocacy groups, government agencies, research groups, pharmaceutical and biotech

manufacturers, and others in the CER/PCOR field. A complete list of participants is found in Appendix A.

B. Pre-Conference Survey

The planning committee developed a pre-conference survey to inform and appropriately frame breakout sessions. All conference invitees were given the opportunity to share their perceptions of barriers and strategies to enhance the uptake of PCOR/CER from the standpoint of knowledge users (patients, clinicians, and payers). Developing the survey instrument involved three main stages: 1) a focused literature search; 2) an iterative approach to revising the questionnaire; 3) pre-testing.

A targeted literature search identified published studies relating to common barriers and strategies to the implementation of evidence-based practice. PubMed (January 2000-November 2016) search strings included: "*evidence-based practice*", "*comparative effectiveness research*", "*barriers*", "*strategies*", and "*implementation*". Based on this search, an initial pool of potential survey items that addressed a specific barrier or strategy to CER/PCOR was formed. This initial survey item pool was reviewed by the planning committee and revised to remove any redundant, unclear, and/or irrelevant items. The questionnaire instrument was independently pre-tested with three graduate students to identify any questions that were difficult to understand or answer, and then further refined.

The questionnaire was organized into three sections. The first section asked the respondent to describe their primary work setting and to indicate which of the three key stakeholder perspectives (patient, clinician, or payer) they would adopt while answering the rest of the questionnaire. In the second section, taking this perspective, the respondent rated 10 barriers ("…the extent that a barrier is an issue") and 6 strategies ("…effectiveness of the strategy") on a Likert scale, from "1" (indicating that a barrier was an issue "none of the time" or a strategy was "not effective") to "4" (indicating that a barrier was "always an issue" or a strategy "extremely effective"). The final section provided an opportunity for respondents to provide free-text suggestions for additional barriers and strategies. For analysis, summary scores were calculated using the Likert scale ratings for barriers and strategies and used to rank barriers and strategies by least to most frequently encountered and effective, respectively. Rankings were reported for the overall sample and stratified by stakeholder perspective.

An email was sent to conference registrants that included a link to the web-based survey questionnaire. A total of 46 attendees responded to the survey (73% of the 64 registrants to whom an email was sent). Of these, 23 (50%) respondents adopted the clinician perspective, 12 (26%) and 11 (24%) respondents chose payer and patient viewpoints, respectively. The most commonly identified work settings were academia

(54%), industry (11%), payer organization (8%), and patient advocacy and government (both 7%). Other work settings (13%) included policy research, technology companies, professional organizations, and consultancies. No respondents identified clinical practice as their primary work setting.

Table 1 summarizes the rankings of barriers and strategies for the overall sample and by stakeholder perspectives. Complete results of the pre-conference are reported in **Appendix B**. While rankings for strategies were relatively consistent among all stakeholder perspectives, there were several barriers that were viewed discordantly:

- "*Access [to] CER related-studies*" was ranked as the second least frequently encountered barrier by both clinicians and payers, but patients ranked this as third most encountered.
- "*Uncertainty around regulations around unpublished data for public use*" was considered the most frequently encountered barrier for payers, but 9th and 7th, respectively, for patients and clinicians.
- "*Lack of CER evidence applicable to relevant patient subpopulations*" was identified as the 4th most frequently encountered barrier by both patients and clinicians, but 8th for payers.
- "Lack of tools to incorporate CER into decision-making" was frequently encountered for clinicians (3rd) and payers (2nd) but less so by patients (7th).
- "*Lack of high-quality CER studies to support decision-making*" was considered the most frequently encountered barrier for clinicians but 6th and 7th for patients and payers, respectively.

There are several limitations to this survey. Eligibility to participate in the survey was dependent on being identified as a potential conference attendee by the selection committee; therefore, a degree of selection bias is inherent. Further, some respondents may not best identify with role of a patient, clinician, or payer and therefore may have felt disingenuous adopting one of these stakeholder perspectives. For example, no respondent identified clinical practice setting as their primary work setting, yet half of respondents selected the clinical perspective. Therefore, one should avoid overextrapolation of these results to the broader stakeholder populations.

Table 1 - Summary rankings for barriers and strategies by conference attendees, overall and by key stakeholder perspective adopted by respondent*

BARRIER	OVERALL	Patient	Clinician	Payer
There is a lack of high-quality CER studies to support decision-making	**1**	6	1	7
There is a lack of tools to incorporate CER into decision making (e.g. patient decision-aids)	**2**	7	3	2
There is insufficient education about how to interpret and apply results of CER studies	**3**	2	5	3
There is not enough CER studies to support decision-making	**4**	1	2	4
There is a lack of CER evidence applicable to relevant patient subpopulations	**5**	4	4	8
There is uncertainty around regulations around unpublished data for public use	**6**	9	7	1
There is a lack of trust or acceptance of CER methods and results	**7**	8	6	5
CER as a concept is poorly understood	**8**	5	8	6
It is difficult to access CER related-studies (e.g. journal publications)	**9**	3	9	9
CER evidence is not applicable, lacks relevance	**10**	10	10	10
STRATEGY				
Direct incorporation of CER-based recommendations into practice guidelines	**1**	1	1	2
More high quality and peer-reviewed summaries of CER that provide direct recommendations for decision-making	**2**	3	2	1
Creation of a registry/repository of CER evidence that is indexed and easily accessible	**3**	2	3	3
More outreach with face-to-face academic detailing sessions	**4**	5	4	5
Provision of direct-to-patient CER-based education materials that patients can use to help change practitioner behavior (e.g. educational material such as pamphlets, posters or audiovisual information in waiting rooms, patient decision aids)	**5**	4	5	6
More interactive workshops and conferences that explain the purpose, scope, and application of CER to stakeholders	**6**	6	6	4

*Ranking determined by combining Likert scale responses into a single summary score, then ordered from largest to smallest value; for example "*There is a lack of high-quality CER studies to support decision-making*" and "*Direct incorporation of CER-based recommendations into practice guidelines*" are ranked #1 barrier and strategy, respectively) - the higher the rank, the more an item was considered a frequently encountered barrier or effective strategy

C. Conference Overview

The full agenda featured presentations by experts in the field and included time for breakout discussions and networking (**Appendix C**). The program started on the afternoon of January 26 with an introduction and welcome by Eileen Cannon, President of the PhRMA Foundation, and Glen Schumock from the University of Illinois at Chicago. The opening session featured an overview of the history of CER education programs, and the motivation behind the PhRMA Foundation's Centers of Excellence in CER Education, presented by Michael Murray of the Regenstrief Institutes and Purdue University. Following that were three presentations from PhRMA Foundation-funded CER Centers. First, Lou Garrison from the University of Washington gave a summary of the activities and accomplishments of the centers at Johns Hopkins University, Harvard, the University of Utah, and the University of Washington. Next, the two most recently funded centers presented. Simon Pickard presented on behalf of the University of Illinois at Chicago, and Eleanor Perfetto presented for the University of Maryland.

The next session provided an opportunity for attendees to hear the latest on funding, major initiatives, and direction of the three major sponsors of CER/PCOR research – PCORI, AHRQ, and NIH. These presentations were delivered by William Lawrence, Sharon Arnold, and Josephine Briggs, for PCORI, AHRQ, and NIH, respectively. The information presented generated a robust question and answer exchange. Next on the agenda was an equally interactive stakeholder panel, moderated by Scott Smith of the Department of Health and Human Services. The purpose of the panel was to discuss the needs and gaps in the uptake and use of CER/CPCOR from the user's perspective. Eleanor Perfetto represented the National Health Council and gave the patient perspective. Caleb Alexander of Johns Hopkins University presented thoughts from the clinician perspective. Soumi Saha of AMCP spoke from the payer's perspective, Murray Ross from Kaiser Permanente presented from the health system perspective, and Julie Locklear from EMD Serono gave the pharmaceutical industry perspective. A set of follow-up questions from the moderator and audience helped explore various aspects of the different views.

The evening of the first day of the conference was highlighted by a networking session, dinner, and keynote address. The networking session gave attendees ample opportunity to connect and share ideas. This session was complimented by other networking opportunities that occurred during breaks and before and after sessions. The keynote speaker was Kavita Patel. Dr. Patel is a nonresident fellow at the Brookings Institute and a practicing primary care internist at Johns Hopkins Medicine. She also served in the Obama Administration as Director of Policy for the Office of Intergovernmental Affairs and Public Engagement in the White House. Dr. Patel had a deep understanding of health issues and policy

discussions occurring in the Capitol and shared her insight on CER/PCOR moving forward in the transition between administrations and beyond. The presentation was well-received and prompted much discussion around the dinner tables.

The second day focused on strategies to improve uptake and use of CER/PCOR. The morning session was kicked-off by a comprehensive presentation on the evidence around dissemination and uptake of CER/PCOR. Elaine Morrato, from the University of Colorado, and Nilay Shah, Mayo Clinic, shared their understanding of major frameworks for dissemination and implementation, but also the findings of recent studies of the uptake of CER in practice. Next were presentations delivered by Ernest Law, of the University of Illinois at Chicago, and Jennifer Graff from the National Pharmaceutical Council (NPC). Both presented results of surveys on the main issues around uptake and use of CER. Dr. Law presented the results of the pre-conference survey completed by those registered for the conference (discussed above). Dr. Graff presented the results of a survey of NPC members conducted by her organization.

The morning session continued with breakout group discussions. There were three breakout groups organized by user type - clinicians, patients, and payers. Each group was instructed to take into consideration the pre-conference survey results, and other presentations and discussion at the conference, and then identify the top barriers to CER/PCOR uptake and use relevant to their group. The breakout groups also discussed effective strategies to improve the use of CER/PCOR. The group discussions were lively. Following the breakout sessions, the entire audience reconvened and heard summaries from representatives of each group.

During lunch on the second day, Barry Blumenfeld of RTI International presented the concept of a learning network for improving dissemination of PCOR-based clinical decision support. His presentation was followed by the final session, which focused on the future of CER/PCOR education and use of CER/PCOR evidence in practice. The first presentation was by Diana Brixner of the University of Utah and president-elect of AMCP. Diana provided unique insights from the perspective of AMCP on this topic. The next presentation was by Bill Galanter, of the University of Illinois at Chicago. Dr. Galanter focused on clinical decision support systems as a means to implement CER evidence and provided his experience and insights from that perspective. Finally, Lou Garrison of the University of Washington, and current president of the International Society of Pharmacoeconomics and Outcomes Research (ISPOR) presented his thoughts and those of ISPOR. The conference concluded with a summary and wrap-up of by Glen Schumock of the University of Illinois at Chicago.

III. Outcomes and Recommendations

The collective discussion and recommendations from the conference focused on the needs and gaps in the uptake and use of CER/PCOR evidence by patients, clinicians, and payers (**Table 2**); and the methods or approaches to enhance the uptake and use of CER/PCOR evidence by patients, clinicians, and payers (**Table 3**). The outcomes of the three groups – clinicians, patients, and payers – are summarized below.

Table 2. Selected barriers and strategies to increasing CER/PCOR uptake by stakeholder perspective

BARRIERS	STRATEGIES
PATIENTS	
Need for more understanding of patient needs and preferences by other stakeholders	Researchers and policy makers should leverage patients' desire to be involved in decision-making on system- and group-levels
Need for CER/PCOR findings that are more easily understood by patients	Publically available summaries for CER/PCOR results, presented in lay terms and contextualized to specific patient population
Lack of research that is readily accessible on platforms frequently used by patients – *"how do we know what information sources to trust?"*	Development of tools that can be used to reconcile fragmented or conflicting information in CER/PCOR
Patients are "*more than one disease at a time*" – need for evidence that adequately addresses specific patient subpopulations	Implementation strategies should include consideration of culture; connect local sites that have success with other sites to increase dialogue and "coaching"
CLINICIANS	
Lack of time for clinicians to effectively seek out and apply CER/PCOR	Advancement of clinical decision-support systems that increases use of CER/PCOR in routine care
Lack of high-quality evidence to address clinically relevant questions in specific patient subpopulations and heterogeneity of clinicians treating these conditions	Introduction of quality-of-care metrics that reflect best practice and are linked to reimbursement
Research does not help to increase clinician self-efficacy	Implementation strategies should include consideration of culture; connect local sites that have success with other sites to increase dialogue and "coaching"
Practice setting culture can be a barrier to implementation of best evidence	
PAYERS	
Lack of tools to incorporate CER into decision-making, resulting in inconsistent evaluation of the evidence	Use of study registries to help identify studies relevant to decision-making
Concern that some decision will be perceived as discrimination, depending on the CER results used	Provide more training opportunities for decision-makers, e.g. formulary committee members experienced with CER/PCOR
Difficulty accessing high-quality CER/PCOR that reflect relevant subpopulations in decision-making in a timely fashion	Outcomes organizations coordinate CER dissemination for greater knowledge translation and outreach to members

A. Patients

The patient group suggested that greater understanding is needed of what matters most to patients as it relates to their treatment, and to align CER/PROCR research with those priorities. Researchers need to determine what are patient priorities and provide value focused results patients will understand and use in their decision-making. Further, CER/PCOR needs to be translated in a way that can be accessed by patients and disseminated through platforms that patients use. The language used to discuss CER/PCOR evidence needs to be understandable to patients. The group suggested that resources be created that summarizes CER/PCOR evidence in lay terms, be made publically available, and reside where patients currently go to obtain information. Importantly, it should be emphasized to stakeholders that it is the responsibility of all, but especially researchers, to educate and facilitate uptake of evidence appropriate to users, and not just users (e.g. patients) educating themselves. Lastly, this group suggested we should find ways to help patients reconcile fragmented information about and adoption of CER/PCOR across the different providers with whom they interact.

B. Clinicians

The clinician group reported that lack of time on the part of clinicians to effectively seek out and apply CER/PCOR was a significant barrier. They also identified other barriers common to implementation science (i.e., not unique to CER/PCOR) that are important, such as like knowledge, attitudes, beliefs, and self-efficacy. The perceived low quality of CER/PCOR studies was identified as a major barrier to its use, as was the lack of evidence in many clinical areas/indications. Strategies discussed included incorporating CER/PCOR into clinical decision support systems and other tools that make it part of routine activities in care.

C. Payers

The key barriers to uptake and use of CER/PCOR from the payer perspective are the timeliness of the availability of results of CER/PCOR studies (e.g., not available when decisions are being made), and the robustness of the data (not directly transferable to the payer's population, or too many gaps in the research evidence available). Lack of resources for CER/PCOR and lack of education on how to use CER/PCOR data are also barriers. Last, the clinical nuances that are important in decision-making are not always incorporated into CER/PCOR evidence. Strategies proposed included better organization and coordination of CER/PCOR evidence (perhaps incorporating it into existing registries), education programs (especially for regulators), the availability of high-quality summaries for CER/PCOR data that can be presented to decision-makers, and the need for a CER/PCOR–trained person on formulary committees.

IV. Conference Evaluation

There appeared to be consensus that the conference was successful. In fact, the planning committee received numerous positive comments about both the content and the opportunity for networking and interaction. To capture formal feedback, the planning committee created and distributed an online post-conference survey at the end of February 2017.

The post-conference questionnaire contained several items intended to capture the degree to which respondents: 1) agreed each of the conference goals was achieved; 2) felt overall satisfied with the conference; 3) would use the topics presented and discussed regularly in their work. There was also a section for attendees to provide free-text comments about what was particularly good about the conference and what could be improved.

Post-conference survey results

Email invitations were sent all 71 participants on the final conference registrant list who attended the conference in its entirety. The survey has a response rate of 45% (n=33). Survey results indicated that attendees "strongly" to "somewhat" agreed that conference goals were reached, with more than 90% indicating strongly or somewhat agree across all conference aims (**Figure 1**). Notably, all 33 respondents reported that the presentations were of high quality and the conference was excellent (**Figure 2**). Most respondents (87%) indicated they were likely to use conference materials and discussions, respectively, in their daily work (**Figure 2**).

Figure 1 – Degree to which attendees felt conference goals were met

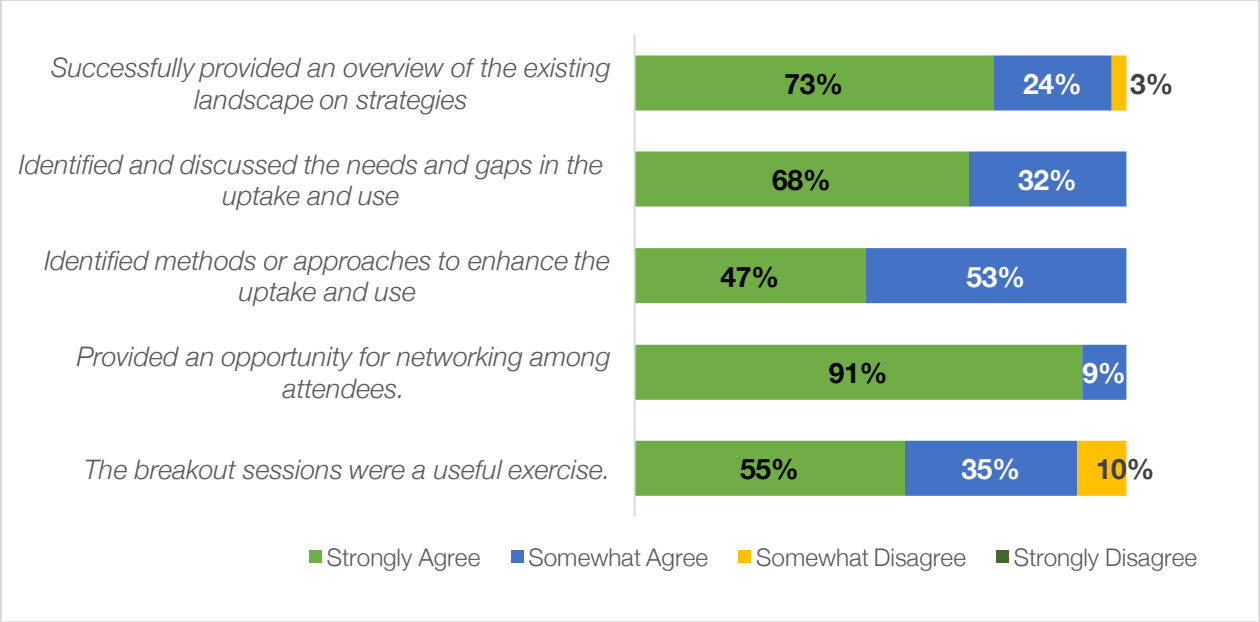
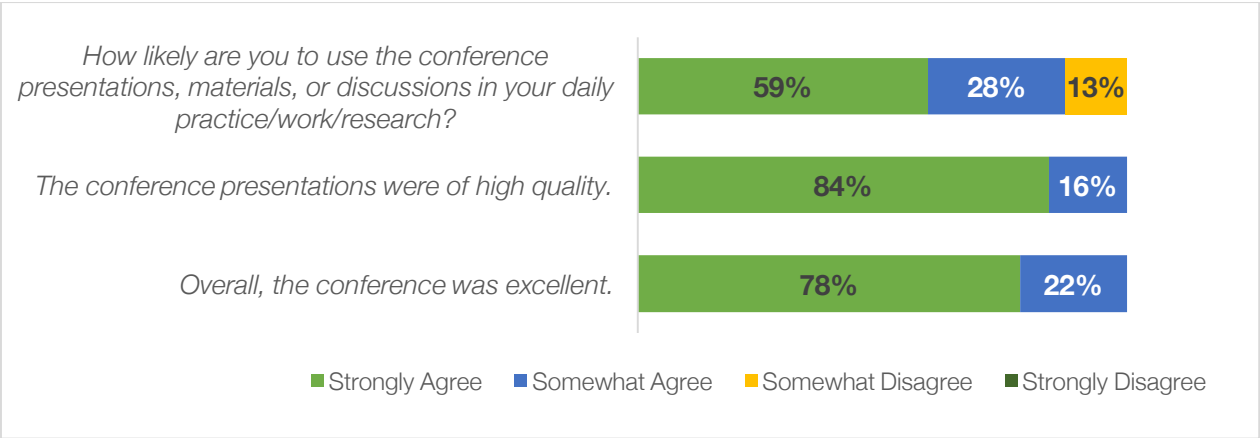


Figure 2 – Overall opinions of attendees on overall presentation and conference quality, and likelihood of incorporating conference information in daily practice/work/research



In the open-response survey items, attendees were positive about the small conference size, diverse range of stakeholders in attendance, well-selected presentations, and the thoughtful discussion. When asked to share how the conference could be improved, some attendees reported that the breakout sessions could have been longer and more structured.

Table 4 – Selected comments describing the conference

OVERALL COMMENTS
• *Having all stakeholders working toward the same goal, aligning perspectives, and open discussion that can be translated into practice changes. Very meaningful discussions, and engaged participants!*
• *The small size was great, I felt that I could approach any of the speakers with questions and get to know each of the attendees. I also really enjoyed many of the presentations and thoughtful discussions.*
AREAS FOR IMPROVEMENT
• *Potentially providing a 'what to expect' for first time attendees. I wasn't sure whether this would be round-tables, presentations, etc.*
• *More time in round table discussion. Maybe a round robin with various topics at the tables.*
• *More time, and more focus for the breakout.*

IV. Summary and Next Steps

In summary, the conference was largely successful in accomplishing the goals established at the outset, as evidenced by the executed agenda and the formal post-conference feedback. An overview of the landscape and current strategies for enhancing CER/PCOR uptake (aim #1), including experiences with educational efforts, was provided to attendees. Barriers to and strategies for implementing CER/PCOR were identified through several approaches: the pre-conference survey, conference presentations, and during breakout sessions (aims #2 and #3). Attendees were also given ample time to network during frequent breaks in the agenda and during the evening dinner (aim #4). Lastly, the conference organizers will submit a supplement to be published in the *Journal of Managed Care Pharmacy*. The supplement will feature several manuscripts that reflect the different aspects of CER/PCOR uptake addressed by the conference, such as the interplay between health policy and research support, knowledge dissemination strategies, barriers and strategies in CER/PCOR, clinical decision-making systems, and state of play for

education and tools for incorporating CER/PCOR. Development of the supplement is currently ongoing, and authors will include several members of the planning committee, as well as conference presenters and attendees. Along with this summary, these articles are intended to be enduring materials capturing the experience and discussion shared at the conference, and to inform interested parties by providing frameworks for recommendations and tools for training current and future users of CER/PCOR evidence (aim #5).

In conclusion, the conference proved to be highly valuable to those in in attendance – it provided a venue for leaders and key stakeholders in the field to learn from each other's collective experiences and share new ideas. It is clear much progress has been and will be made in increasing the uptake and implementation of CER/PCOR; however, other themes were highlighted and warrant further deliberation: with continuing investment, more research is being conducted and more data is available to researchers than ever before, how can (and should) this data be utilized? How can researchers take the additional step beyond effectiveness and demonstrate value of interventions, programs, and services to patients, clinicians, and payers? How can research better align with the preferences and goals of each stakeholder? While this conference did not specifically address these questions, the issues underlying are consequential and deserve attention – in similar conferences, in research, and in clinical and policy decision-making.

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13. Tang DH, Warholak TL, Hines LE, et al. Evaluation of Pharmacy and Therapeutic (P&T) Committee member knowledge, attitudes and ability regarding the use of comparative effectiveness research (CER) in health care decision-making. Res Social Adm Pharm. 2014;10(5):768-780.
14. Avorn J, Fischer M. 'Bench to behavior': translating comparative effectiveness research into improved clinical practice. Health Aff (Millwood). 2010;29(10):1891-1900.
15. Morrato EH, Concannon TW, Meissner P, Shah ND, Turner BJ. Dissemination and implementation of comparative effectiveness evidence: key informant interviews with Clinical and Translational Science Award institutions. J Comp Eff Res. 2013;2(2):185-194.

APPENDICES

Appendix A – List of Participants

FIRST NAME	LAST NAME	AFFILIATION
Caleb	Alexander	Johns Hopkins Bloomberg School of Public Health
Chinenye	Anyanwu	Patient-Centered Outcomes Research Institute
Sharon	Arnold	Agency for Healthcare Research and Quality
Alan	Balch	Patient Advocate Foundation
Tericke	Blanchard	BioMarin
Barry	Blumenfeld	RTI International
Josephine	Briggs	NIH/NCCIH
Diana	Brixner	University of Utah
Elizabeth	Brusig	Optima Health Plan
Wendy	Camelo Castillo	University of Maryland Baltimore
Jon	Campbell	University of Colorado
Eileen	Cannon	PhRMA Foundation
Eric	Cannon	Selecthealth
Tim	Carey	UNC Chapel Hill
Jim	Carey	Merck
Gregory	Cooper	University Hospitals Cleveland Medical Center
Dan	Danielson	Premera Blue Cross
Jessica	Daw	UPMC Health Plan
Beth	Devine	University of Washington
Beth	DiGiulian	Booz Allen Hamilton
Lynn	Disney	University of Maryland
Karissa	Dunkley	Curadite
Guy	Eakin	Arthritis Foundation
Kelly	Fernandez	Healthcare Leadership Council
Jean Paul	Gagnon	Consultant
William	Galanter	University of Illinois at Chicago
Lou	Garrison	University of Washington
Jennifer	Graff	National Pharmaceutical Council
Rachel	Harrington	University of Illinois at Chicago
Carolyn	Jones	Biogen
Cille	Kennedy	DHHA/ASPE/ Office of Health Policy
Larry	Kessler	University of Washington
Ernest	Law	University of Illinois at Chicago
William	Lawrence	PCORI
Lisa	Lentz	NCCN
Julie	Locklear	EMD Serono
Dan	Malone	University of Arizona
Carrie	McAdam-Marx	University of Utah
Jody	McNannay	Curadite
Robert	McQueen	University of Colorado
Laura	Miller	NACDS
Donna	Moncuso	NCCN (National Comprehensive Cancer Network)
Elaine	Morrato	Colorado School of Public Health
Michael	Murray	Regenstrief Institute and Purdue University

Elizabeth	Nardi	National Comprehensive Cancer Network
Lauren	Neves	PhRMA
Elisabeth	Oehrlein	University of Maryland, Baltimore
Eduardo	Ortiz	American Society of Clinical Oncology
Kavita	Patel	Brookings Institution and Johns Hopkins Medicine
Eleanor	Perfetto	National Health Council
Simon	Pickard	UIC
Matthew	Pickering	Pharmacy Quality Alliance
Murray	Ross	Kaiser Permanente
Soumi	Saha	Academy of Managed Care Pharmacy
Elizabeth	Sampsel	Dymaxium, Inc.
Nancy	Santanello	Consultant
Glen	Schumock	University of Illinois at Chicago
Jodi	Segal	Johns Hopkins University
Nilay	Shah	Mayo Clinic
Mark	Shelby	CVS
Scott	Smith	HHS-ASPE-Office of Health Policy
Jason	Spangler	Amgen
Til	Sturmer	UNC
Prasun	Subedi	Pfizer
Iris	Tam	Otonomy
Pam	Traxel	ACS CAN
Thomas	Trikalinos	Brown University
Sara	Van Geertruyden	Partnership to Improve Patient Care
Joe	Vandigo	PhRMA
Lee	Vermeulen	University of Kentucky
Meera	Viswanathan	RTI International
Kat	Wolf Khachatourian	Qualchoice Health Plan Services

Appendix B – Pre-conference survey results

The full results of the pre-conference survey are shown in this appendix. Barriers and strategies are displayed in each figure for the overall sample, then subsamples of each key stakeholder perspective (i.e. patient, clinician, and payer). Additional barriers and strategies identified by respondents via free-text questionnaire items are listed after each section.

Figure B1 – Extent to which the following issue is a barrier to the uptake of CER/PCOR from the overall sample (n=46)

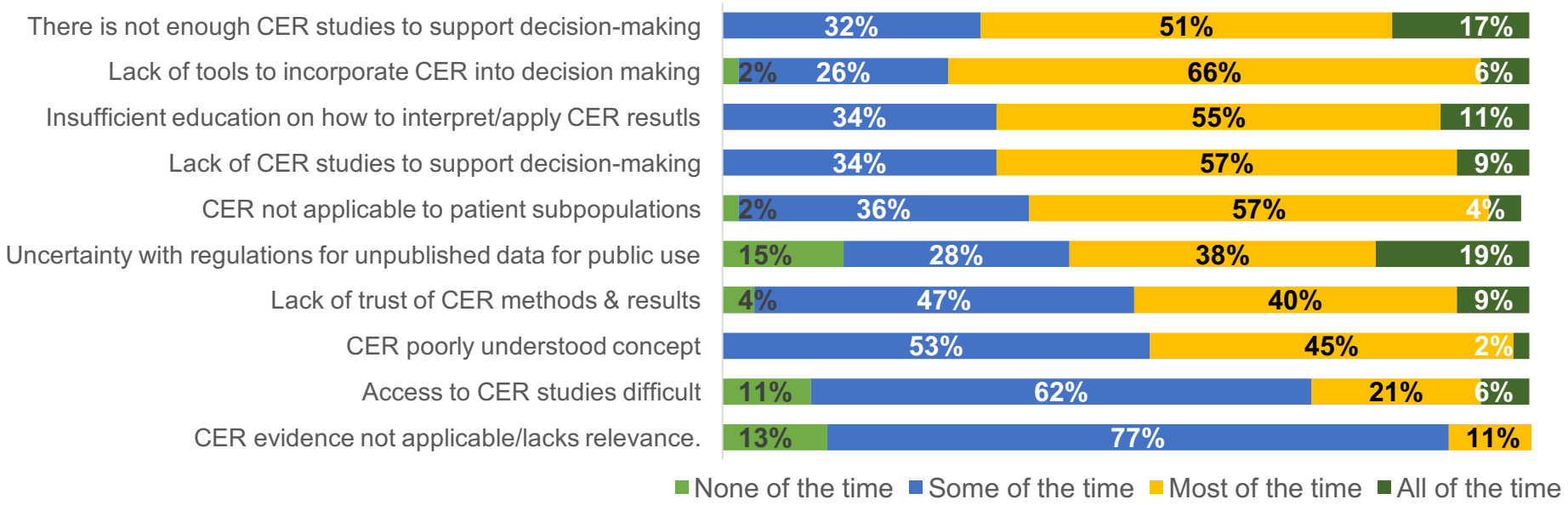


Figure B2 – Effectiveness of strategies to enhancing the uptake of CER/PCOR from the overall sample (n=46)

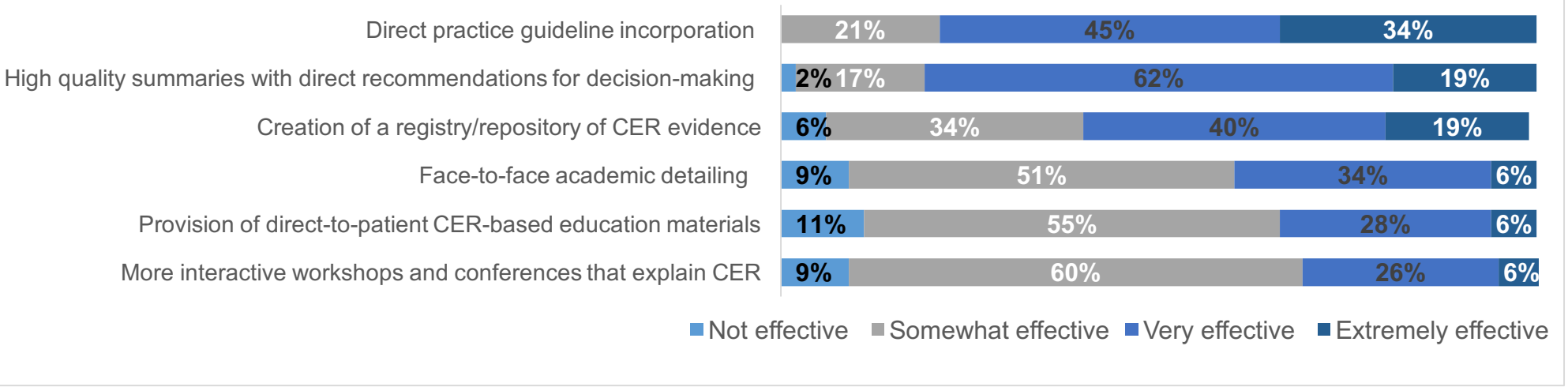


Figure B3 – Extent to which the following issue is a barrier to the uptake of CER/PCOR from the patient perspective (n=11)

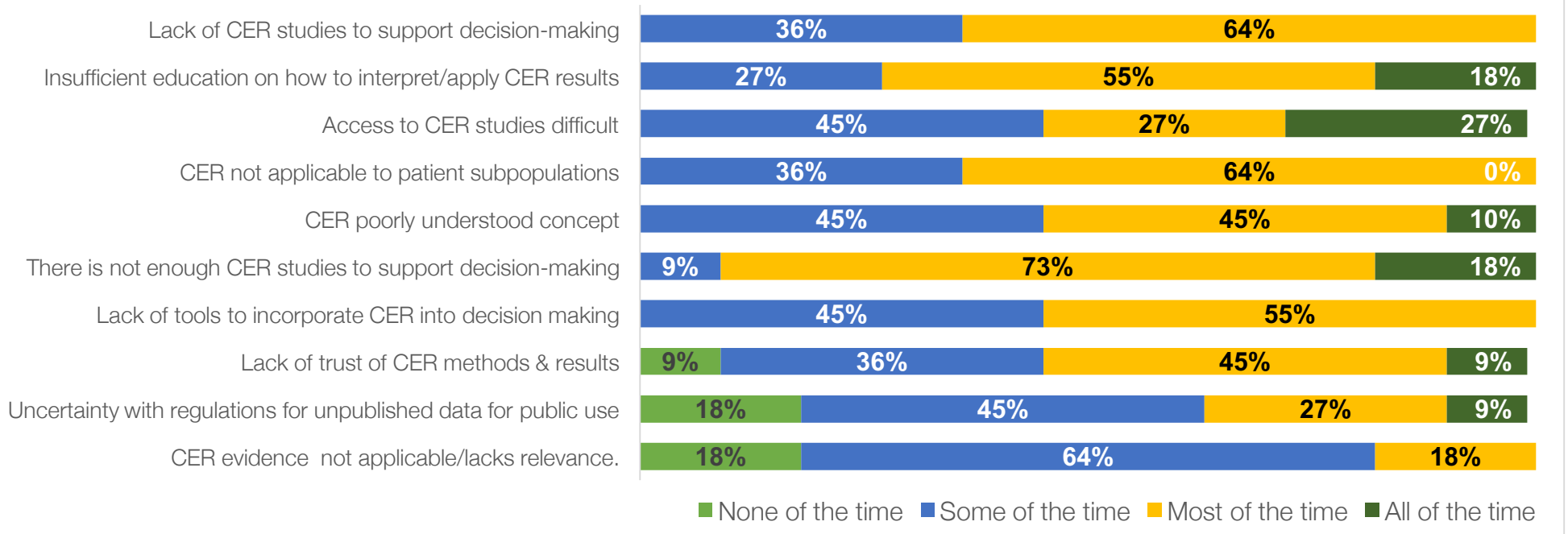
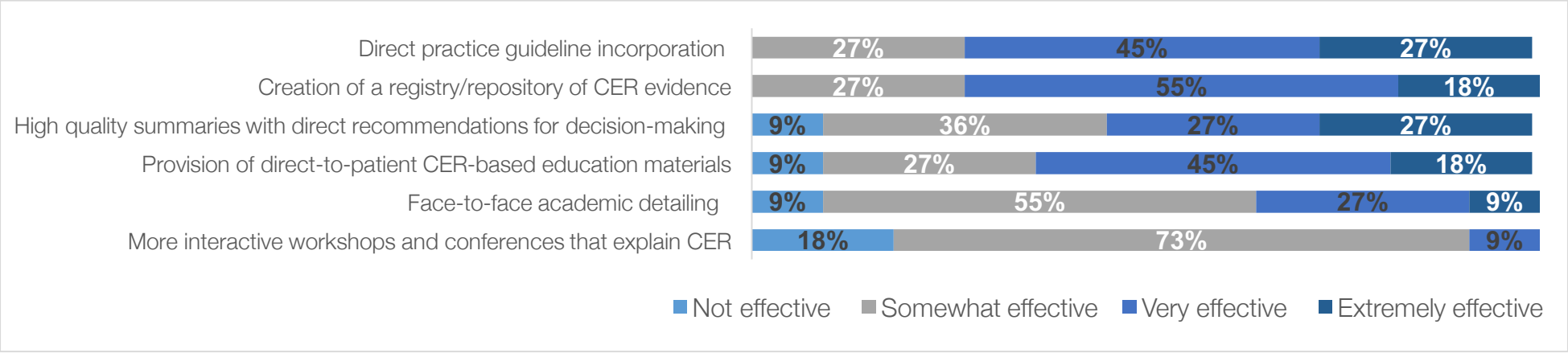


Figure B4 – Effectiveness of strategies to enhancing the uptake of CER/PCOR from the patient perspective (n=11)



Additional barriers: PATIENTS

1. Competing CER assessments leads to uncertainty
2. CER research doesn't generally cover sub populations
3. CER is not readily and easily available to prescribers
4. Funding
5. Collaboration amount groups
6. Lack of patient engagement
7. Not addressing the right question
8. Not capturing the outcomes patients think are important
9. Unclear the extent to which cost information is incorporated
10. Information on comparative effectiveness difficult to find
11. Recommendations for a population may not apply to an individual
12. Health literacy
13. Outreach to hard-to-reach populations
14. Understanding heterogeneity within patient populations
15. It is difficult to know which sources of information to trust, e.g., NIH web sources vs. Industry web promotion
16. Even patients with health care knowledge may be uninformed about CER and PCOR.
17. Patients do not understand the value of CER data in the healthcare decisions their clinician or payer make
18. Payers make top-down decisions based on CER, but the information is not communicated appropriately to the patients
19. Clinicians lack time/inclination to share/explain/make relevant CER data
20. I am in a setting where access to publications is not a problem, but I know from anecdotal evidence that it is a big struggle for others.
21. Peer reviewed manuscripts are intimidating to read, peer reviewed lay person summaries would help
22. Identifying trusted sources of CER information
23. Understanding weight of evidence/level of uncertainty associated with CER outcomes

Figure B5 – Extent to which the following issue is a barrier to the uptake of CER/PCOR from the clinician perspective (n=23)

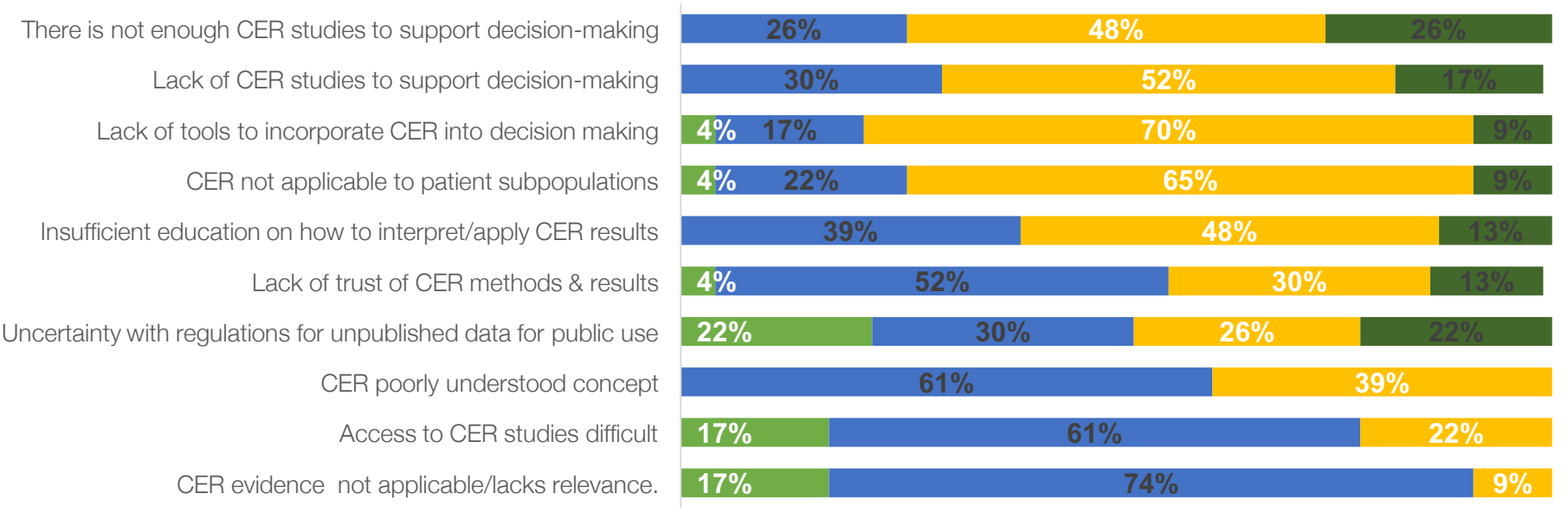
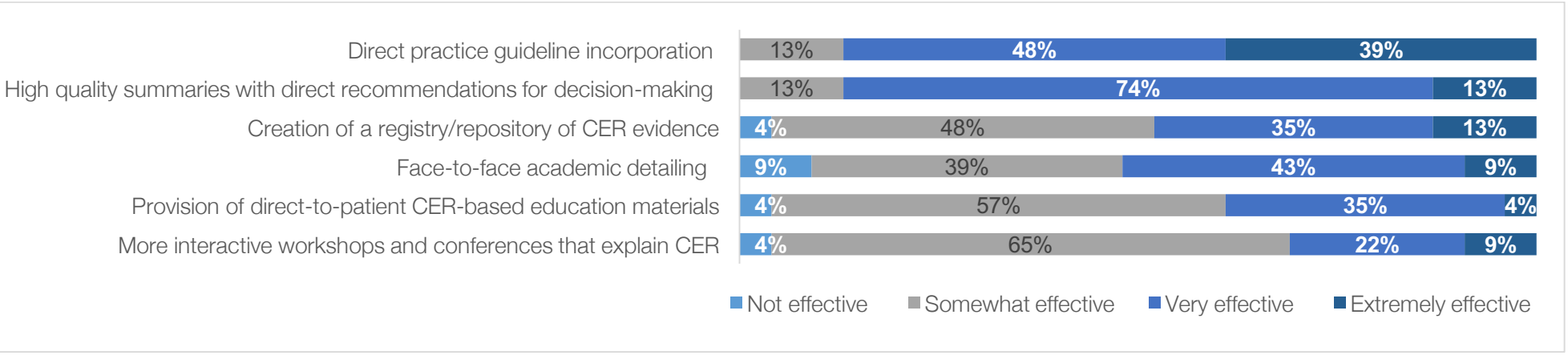


Figure B6 – Effectiveness of strategies to enhancing the uptake of CER/PCOR from the clinician perspective (n=23)



Additional barriers: CLINICIANS

1. Conflation of marginal and conditional effects
2. Lack of explicit quantitative bias analyses
3. lack of explicit quantitative generalizability analysis
4. Lack of communication between research and clinical practice
5. Clinicians may reject evidence if not conducted as a randomized trial
6. sophistication needed to evaluate the quality of studies using existing data
7. conflicting guidelines depending on methodology used
8. precision medicine and field moving in this direction
9. Insufficient to change practice; need replication
10. Is based on averages for groups; still has limited usefulness to individual patient management
11. Inconsistent integration into technology CDS
12. Credibility of findings
13. Inconsistent buy-in across sectors (clinical, payer, other)
14. Lack of head-to-head studies conducted by industry that allow for appropriate CER
15. Lack of adequate funding by NIH or other funding entities for CER
16. Lack of well conducted CER in the literature that can be incorporated into evidence-based guidelines and measure development to help guide decision-making
17. Lack of general understanding of CER methodologies
18. Lack of support for use of CER evidence in respective work settings by employing entity
19. General lack of trust in non-RCT evidence among clinicians
20. Difficulty incorporating population-/group-level evidence into bedside decisions
21. Tendency to trust guidelines, which are slow to utilize newest CER
22. Even good CER studies may not answer the question to which I seek the answer.
23. Time, time, time - as clinicians, so much to do.
24. If the CER studies are not yet part of practice guidelines, hard to know how much to trust.
25. literature support
26. understanding
27. population using this data in practice
28. Funding
29. Artificial separation between costs and effectiveness considerations (part of the AHRQ mandate, but also often a function of anxiety about funders)
30. Discomfort with modeling and decision analysis
31. Delivery systems over-focus on regulatory issues, not interventions that may have the most clinical benefit
32. Pharmaceutical companies are sometimes non-transparent regarding access and pricing.
33. Few examples of past experience with some of the possible strategies to overcome the barriers... simply not enough focus paid to CER.
34. Many clinicians who are implementers of CER do not understand the vagaries, biases, and limitations of CER when they have access to the results.
35. Some health systems ignore CER results and simply favor the lowest cost alternative (drug, care, etc.)
36. Difficulty delivering findings at the point of care in EHRs and clinical systems
37. Lack of technical mechanisms for incorporating CER into clinical decision support
38. Lack of standards for representing relevant clinical findings
39. More and more retrospective studies, producing inherent skepticism of results
40. Some important questions are not answered as too little funding on trials
41. Growth of studies is near exponential, as growth of statistical methodology. Many clinicians cannot keep up.
42. Lack of an agreed upon systems perspective of the health condition that is being studied
43. Lack of agreed upon data model - with accepted terms, definitions and relationships so that information can be analyzed more effectively
44. Lack of agreed upon standards for data collection when setting up clinical trials
45. Lack of relevant rigorous trials
46. Challenges of overcoming patient education especially about pharmaceuticals
47. Time
48. Trust, generalizability, methodology

Figure B7 – Extent to which the following issue is a barrier to the uptake of CER/PCOR from the payer perspective (n=12)

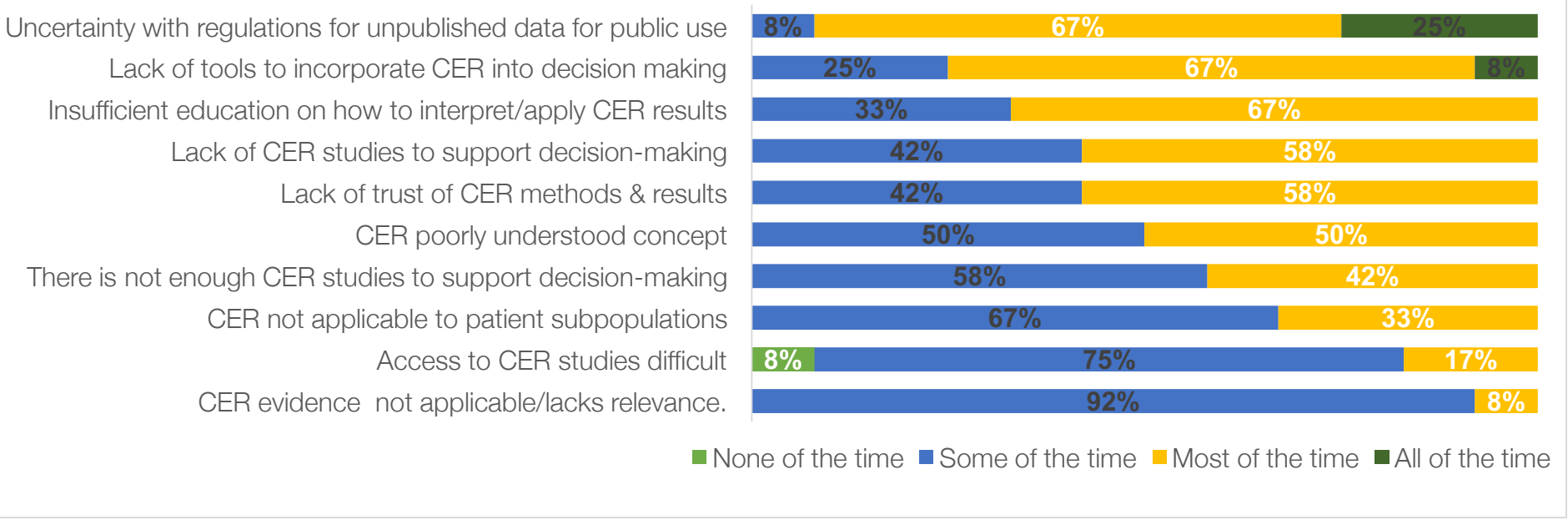
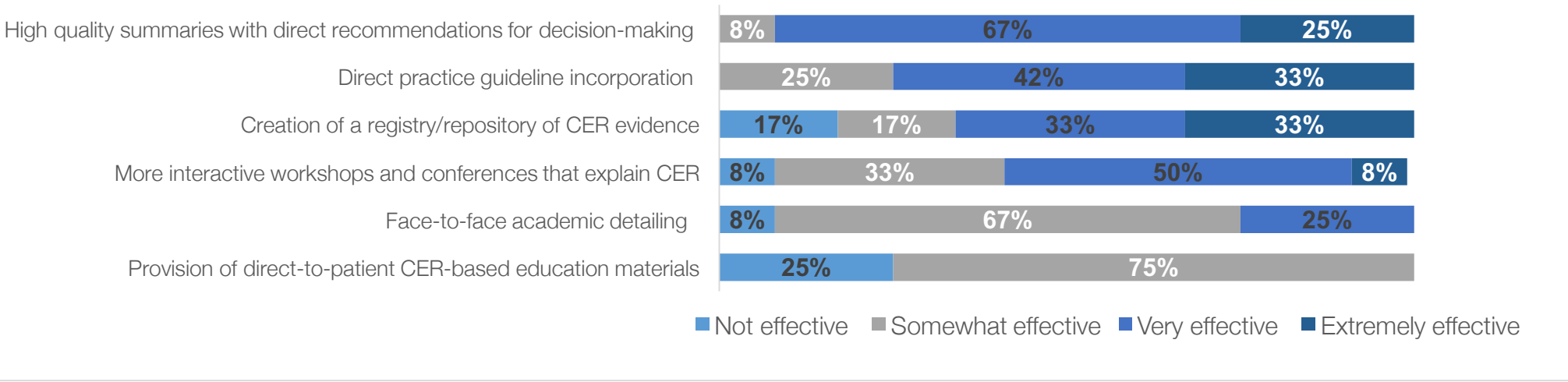


Figure B8 – Effectiveness of strategies to enhancing the uptake of CER/PCOR from the payer perspective (n=12)



Additional barriers: PAYERS

1. Ample time to systematically evaluate, validate, and apply CER data
2. Consistent format, dissemination, and applicability of CER data
3. Relevant and reproducible CER data to subpopulations
4. Timely studies to impact decision-making. Studies and results are after decisions have been made.
5. Wrong questions are often asked; Endpoints are not endpoints that matter to payers (or they are not disaggregated from a composite endpoint.
6. Lack of transparency in underlying data, methods used to conduct, or results.
7. Most CER studies are based on registration trials that do not reflect real-world populations
8. Lack of trust in models created by or for pharmaceutical manufacturers, no independent "Seal of Approval" by a review organization
9. CER models do not include comparators that are relevant to real-world clinical practice
10. Not timely
11. Not relevant to the decision maker
12. Not asking the right question
13. including financial aspects of a coverage decision with the clinical comparative effectiveness
14. generating coverage policies that are consistent with comparative effectiveness evidence for particular subgroups
15. Understanding the methods and reliability of data
16. Lack of CER data for relevant comparators
17. Small sample sizes may prevent application to larger population
18. Resources/expertise to interpret CER at smaller health plans
19. Having good comparative effectiveness information on new treatments and interventions.
20. Political environments in public and private sectors can impede use of CER for coverage.
21. Traditional marketing and social medial influence patients and clinicians, thereby undermining evidence-based approaches to care.
22. changing the mindset that the RCT is the best way to evaluate a product
23. resistance to change
24. Understanding CER and how to use it in decision making
25. Resources to overcome barrier 1
26. Timeliness of CER in decision making
27. High Quality Robust Evidence
28. Easily accessible evidence
29. Timeliness of evidence as it relates to when P&T decisions need to be made

Appendix C – Conference Agenda

Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians and Payers

Thursday, January 26 – Friday, January 27, 2017
The Ronald Reagan Building
1300 Pennsylvania Avenue, NW | Washington, D.C.

Thursday, January 26, 2017
Polaris Conference Room, Concourse Level

1:15 PM – 2:15 PM	Registration
2:00 PM – 2:05 PM	Welcome Remarks Eileen Cannon, President, PhRMA Foundation
2:05 PM – 2:15 PM	Opening Remarks Glen Schumock, PharmD, MBA, PhD, University of Illinois at Chicago
2:15 PM – 2:45 PM	A Look Back: The History of CER Education Programs and The Motivation for PhRMA Foundation Centers of Excellence Introduction: Glen Schumock, PharmD, MBA, PhD, University of Illinois at Chicago Speaker: - Michael Murray, PharmD, MPH, Regenstrief Institute, Inc., Purdue University
2:45 PM – 3:30 PM	How Has the Landscape Changed Since the Creation of the PhRMA Foundation Centers of Excellence in CER Education? Introduction: Glen Schumock, PharmD, MBA, PhD, University of Illinois at Chicago Presenters: - Beth Devine, PharmD, MBA, PhD, University of Washington - Simon Pickard, PhD, University of Illinois at Chicago - Eleanor Perfetto, PhD, MS University of Maryland, Baltimore
3:30 PM – 3:45 PM	Break
3:45 PM – 4:45 PM	CER/PCOR Related Overview and Update on Funding Programs Introduction Simon Pickard, PhD, University of Illinois at Chicago Presenters: - Bill Lawrence, MD, MS, Patient-Centered Outcomes Research Institute - Sharon Arnold, PhD, Agency for Healthcare Research and Quality - Josephine Briggs, MD, NIH- National Center for Complementary and Integrative Health

Thursday, January 26, 2017 (Continued) Polaris Conference Room, Concourse Level	
4:45 PM ñ 5:45 PM	Stakeholder Perspectives: Identifying the Needs and Gaps in the Uptake and Use of CER/PCOR Introduction: Eleanor Perfetto, PhD, MS, National Health Council Moderator: Scott Smith, PhD, Health and Human Services Panelists: - Eleanor Perfetto, PhD, MS, National Health Council - Caleb Alexander, MD, MS, Johns Hopkins University - Soumi Saha, PharmD, JD, Academy of Managed Care Pharmacy - Murray Ross, PhD, Kaiser Permanente - Julie C. Locklear, PharmD, MBA, EMD Serono
5:45 PM ñ 6:30 PM	Networking Reception in the Rotunda
6:30 PM ñ 8:15 PM	Dinner and Keynote Address: The Future of CER/PCOR - Navigating Uncertainty Introduction: Eleanor Perfetto, PhD, MS, National Health Council Keynote: Kavita Patel, MD, The Brookings Institution

Friday, January 27 , 2017 Polaris Conference Room, Concourse Level	
7:00 AM ñ 7:30 AM	Registration
7:30 AM ñ 8:00 AM	Continental Breakfast
8:00 AM ñ 9:00 AM	In Action: Dissemination and Uptake of CER/PCOR Introduction Glen Schumock, PharmD, MBA, PhD, University of Illinois at Chicago Speakers : - Elaine Morrato, DrPH, MPH, CPH, University of Colorado - Nilay Shah, PhD, Mayo Clinic
9:00 AM ñ 10:00 AM	Addressing Barriers and Strategies to Enhance the Use of CER/PCOR - Moderator: Simon Pickard, PhD, University of Illinois at Chicago A Look at Pre -Conference Survey Results - Ernest Law, BScPharm, PharmD, University of Illinois at Chicago What We've Learned: Overview of NPC Work on Stakeholder Views and Address ing Barriers to Use - Jennifer Graff, Pharm D, National Pharmaceutical Council Instructions for Small Group Discussions

Friday, January 27 , 2017 (Continued) Polaris Conference Room, Concourse Level	
10:00 AMñ 10:15 AM	Break
10:15 AMñ 11:30 AM	A Deeper Dive: Small Group Discussion s
11:30 AMñ12:15 PM	Observations: Reports from Small Group Discussions and Overall Consensus Moderator: Simon Pickard, PhD, University of Illinois at Chicago
12:15 PM ñ1:15 PM	Lunch and Presentation: A Learning Network - Improving the Dissemination of PCOR - Based Clinical Decision Support (with Lunch) Introduction: Beth Devine, PharmD, MBA, PhD, University of Washington Remarks: Barry Blumenfeld, MD, MS, RTI International, Division of eHealth, Quality, and Analytics (eQUA)
1:15 PM ñ 2:45 PM	What Is the Future of CER and CER Education ? How Will CER Be Integrated Into Practice? Introduction Beth Devine, PharmD, MBA, PhD, University of Washington Presenters : - Bill Galanter, MD, University of Illinois at Chicago - Diana Brixner, RPh, PhD, FAMCP, University of Utah & President-Elect, Academy of Managed Care Pharmacy (AMCP) - Lou Garrison, PhD, University of Washington & President, International Society of Pharmacoeconomics and Outcomes Research (ISPOR)
2:45 PM ñ3:00 PM	Conference Summary and Next Step s? Glen Schumock, University of Illinois at Chicago
3:00 PM	Conference Adjourns

1. Lorenzoni L, Belloni A, Sassi F. Health-care expenditure and health policy in the USA versus other high-spending OECD countries. *Lancet.* 2014;384(9937):83-92.
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5. Federal Coordinating Council F. *Federal Coordinating Council for comparative effectiveness research report to the President and Congress.* Washington, DC2009.
6. Institute of Medicine I. *Knowing what works in health care: A roadmap for the Nation.* Washington, DC: National Academies Press;2007.
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8. Smith S. Preface. *Medical Care.* 2007;45(Supp2):s1-s2.
9. Selby JV, Beal AC, Frank L. The Patient-Centered Outcomes Research Institute (PCORI) national priorities for research and initial research agenda. *JAMA.* 2012;307(15):1583-1584.
10. Hueng DY, Tsai CL, Hsu SW, Ma HI. Publication patterns of comparative effectiveness research in spine neurosurgery. *Neurosurg Focus.* 2012;33(1):E9.
11. Villa L, Warholak TL, Hines LE, et al. Health care decision makers' use of comparative effectiveness research: report from a series of focus groups. *J Manag Care Pharm.* 2013;19(9):745-754.
12. Halley MC, Rendle KA, Frosch DL. A conceptual model of the multiple stages of communication necessary to support patient-centered care. *J Comp Eff Res.* 2013;2(4):421-433.
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