

Employer, Insurer, and Industry Perspectives on Patient-Centered Comparative Effectiveness Research

Final Report

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Preface

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit, nongovernmental organization authorized under the Affordable Care Act of 2010 and funded by Congress to help close the gaps in research evidence needed to improve key health outcomes. To do this, PCORI identifies critical research questions, funds patient-centered comparative clinical effectiveness research, and strives to disseminate the results in ways that stakeholders, including patients, providers, health insurance purchasers, payers, and industry, will find useful. In recognition of the broad range of communities that have a stake in the effectiveness of the American health care system, PCORI actively seeks to engage these communities to help guide their activities and the research they fund.

To better understand the views and experiences of the payer, purchaser, and industry stakeholder communities, PCORI commissioned RAND and the National Pharmaceutical Council (NPC) to perform an independent study. The goal of this study was to help PCORI identify the current levels of research activity, the extent to which comparative effectiveness research (CER) is understood and used, and opportunities for potential engagement of these stakeholder communities in PCORI work. This report describes the study findings and provides insights into the research interests and needs of purchasers, payers, and industry representatives to further PCORI's work with these groups. The study was conducted under contract to, and with the input from, PCORI, but the conclusions drawn are those of RAND and NPC investigators alone and do not necessarily represent the views of PCORI's Board of Governors, Executive Director, or staff.

This report should be of particular interest to the PCORI Board of Governors, Executive Director, and staff and to federal policymakers, such as Congress and the agencies of the Department of Health and Human Services. It should also be of interest to funders of CER, the stakeholder communities included in this report, health system leaders, and clinicians and health service researchers, who have been invited to help set the research agenda and may benefit from future collaborations with PCORI.

RAND Health, a division within the RAND Corporation, is one of the largest private health research groups in the world. More than 300 projects are currently under way, addressing a wide range of health care policy issues. A profile of RAND Health, abstracts of its publications, and additional information can be found at www.rand.org/health.

NPC is a health policy research organization dedicated to the advancement of good evidence and science and to fostering an environment in the United States that supports medical innovation. Founded in 1953, NPC focuses on research development,

information dissemination, and education on the critical issues of evidence, innovation, and the value of medicines for patients. For more information, visit www.npcnow.org.

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Summary

In recognition of the broad range of communities with stakes in the American health care system, the Patient-Centered Outcomes Research Institute (PCORI) actively seeks to engage these communities to help guide PCORI activities and the research they fund. To better understand how three of these stakeholder communities—purchasers, payers, and industry—view, understand and use comparative effectiveness research (CER), PCORI commissioned the RAND Corporation and the National Pharmaceutical Council (NPC) to perform an independent study.

To do this, we conducted ten telephone and Web-enabled focus groups with four purchaser, three payer, and three industry subcommunity representatives. *Purchaser* subcommunities included small employers (<50 employees), medium-sized employers (50–499 employees), large employers, (500+ employees), and business coalitions. *Payer* subcommunities included private, public, and integrated payers.¹ *Industry* subcommunities included device and diagnostics manufacturers, biopharmaceutical companies, and durable medical equipment manufacturers. The study involved a total of 75 participants, with 28 in purchaser discussions, 22 in payer discussions, and 25 in the industry discussions.

Focus group questions centered on getting stakeholder views on health-related decisions, information needs, and CER; stakeholder involvement in research; PCORI’s mission, research, and initiatives; and the value of CER. A number of key findings emerged:

- Purchasers, payers, and industry communities make a variety of health decisions and seek information from multiple sources.
- Familiarity with CER is high among payers and industry but not among purchasers.
- Involving purchaser, payer, and industry stakeholder communities in CER beyond an advisory capacity will be difficult unless CER is aligned with business interests.
- Purchaser, payer, and industry stakeholder communities support PCORI’s mission, but there were concerns that its scope is too broad, which could limit its effectiveness. Concerns were also expressed about a perceived lack of focus on translation of research findings.
- Purchaser, payer, and industry stakeholders agree that PCORI’s funding portfolio prioritizes high-prevalence, high-cost medical conditions. The conditions that are

¹ Integrated payers are organizations that serve the dual role of both the provider of health care services and the payer of services.

priorities for each group differ, which is to be expected, given the large number of ways to rank more than a dozen conditions.

- The idea of priority populations resonated with some payers but did so less with employers and industry stakeholders.²
- Purchaser, payer, and industry stakeholders viewed the two example “real world” studies as useful but had different opinions about how the results could be translated into actionable information for their stakeholder communities. They also suggested ways in which the study design could be changed to better address their information needs.
- Familiarity with the National Patient-Centered Clinical Research Network (PCORnet) is low for most groups, but stakeholders saw value once PCORnet was described. They also had opinions about potential research topics relevant to their interest areas that could be explored using PCORnet.
- All purchaser, payer, and industry stakeholder communities saw value in CER for patients, providers, and other stakeholders.
- Cost information is important in the decisions of both purchasers and payers.

The findings in this report should help funders and researchers strengthen engagement opportunities with difficult-to-engage but vital stakeholder groups. An important implication of these findings is the emphasis these stakeholders placed on the need for relevant, usable, and properly translated information. They told us about the information they need, how to share it, and how to make it usable. Discussions with these stakeholder communities also revealed some gaps in their familiarity with CER and PCORI work. To maximize the opportunities for CER to have an impact, these gaps need to be closed.

² To better address disparities in the U.S. health system, PCORI has identified *priority populations*, or groups of individuals who shoulder a disproportionate burden of disease. PCORI’s priority populations include racial and ethnic minorities; low-income individuals; older adults; residents of urban areas; residents of rural areas; women; individuals with multiple comorbid conditions; children; individuals with low health literacy; individuals with disabilities; individuals with rare diseases; veterans; and the lesbian, gay, bisexual, and transgender community.

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We extend our sincerest gratitude to the 75 individuals who participated in focus groups by discussing their views and experiences regarding health care decisionmaking, the use of research, and their perspectives on the use and usefulness of patient-centered outcomes research and comparative effectiveness research. We are indebted to these individuals for sharing their insights candidly and for the valuable input they provided.

The RAND quality assurance process employs peer reviewers, including at least one who is external to RAND. This report benefited from two rigorous technical reviews that improved the quality and clarity of the material presented.

Additionally, we thank our RAND colleagues, M. Susan Ridgely, Cheryl Damberg, and Justin W. Timbie for their contributions to the focus group protocol. Special recognition is warranted for staff who dedicated themselves to the challenging task of participant recruitment. These included Patricia McGarry and Clara Aranibar, without whom the coordination and scheduling of focus group discussions would not have been possible. Finally we'd like to thank Paul Steinberg for his editorial contributions to the report.

Abbreviations

AHRQ	Agency for Healthcare Research and Quality
ASCVD	atherosclerotic cardiovascular disease
CAD	coronary artery disease
CER	comparative effectiveness research
CMS	Centers for Medicare and Medicaid Services
COMPASS	Comprehensive Post-Acute Stroke Services
CT	computed tomography
DME	durable medical equipment
EHR	electronic health record
FDA	Food and Drug Administration
FDAMA	Food and Drug Administration Modernization Act of 1997
LGBT	lesbian, gay, bisexual, transgender
MCC	multiple chronic conditions
NIH	National Institutes of Health
NPC	National Pharmaceutical Council
PCT	pragmatic randomized clinical trial
PCOR	patient-centered outcomes research
PCORI	Patient-Centered Outcomes Research Institute
PCORnet	National Patient-Centered Clinical Research Network
PPRN	patient-powered research network
TPN	total parenteral nutrition

1. Introduction

Authorized by Section 6301 of the Patient Protection and Affordable Care Act (ACA), the purpose of the Patient Centered Outcomes Research Institute (PCORI) is to “assist patients, clinicians, purchasers, and policymakers in making informed health decisions by advancing the quality and relevance of evidence” (ACA, 2010, Sec 6301). A key mechanism through which PCORI has sought to achieve this purpose and advance the field of health research is involving patients, clinicians, and other stakeholders in research. PCORI defines *engagement in research* as the “meaningful involvement of patients, caregivers, clinicians, and other health care stakeholders throughout the research process” who work in close partnership with researchers (PCORI, 2015g).

In PCORI-funded research, stakeholders may serve on research projects as partners, staff, consultants, or advisors and may participate in every stage of research, including nominating topics, developing research questions, helping with research designs, conducting research, assessing results, translating findings to peers, and implementing findings in their own health-related decisions. Beyond asking important questions, PCORI-funded research is conducted with the involvement of stakeholder communities and must be translated into results that are useful to these communities (PCORI, 2015i).

PCORI commissioned the study of purchaser, payer, and industry views about patient-centered comparative effectiveness research (CER) described here to understand how these stakeholders may use CER evidence and be involved in producing CER. This report begins by providing background information on PCORI, patient-centeredness, CER, and stakeholder involvement in research (Section 2). After a brief description of study methods (Section 3), Section 4 presents a synthesis of the views of the stakeholders we spoke with. The body of the report concludes with a brief discussion of the implications for PCORI’s future work (Section 5).

Appendix A provides detailed information about study methods, including the selection of focus group participants, focus group discussion guides, and data analysis approaches. Appendix B summarizes the results of the pre-discussion group survey and describes the three stakeholder communities included in the study. Appendix C provides a detailed description of our findings, with illustrative quotations, organized by topic: stakeholder knowledge and use of research in health-related decisionmaking; familiarity with and views of CER, PCORI, and the National Patient-Centered Clinical Research Network (PCORnet); and the value of CER in the stakeholders’ current or future work. Appendix D provides abstracts of three example PCORI studies that were explored in each of the focus group discussions.

This research has some limitations. First, a focus group approach is not conducive to producing generalizable findings. Although we developed a recruitment strategy designed to achieve broad participation from a variety of stakeholder types, the results are subject to

selection bias. The views presented here may therefore not generalize to the larger purchaser, payer, and industry communities. However, a focus group methodology offers an important benefit: It enables researchers to explore the meaning of individual views through discussion and probing. This is an opportunity that is not often realized in survey methodologies. Second, our results may be influenced by the professional roles and ranks of individuals who were selected to represent institutions, particularly large institutions. There is unknown heterogeneity in views across roles within an institution, a factor we could not account for in this small focus group project. Third, groupthink is a known limitation of any group-based data collection. Discussion moderators were mindful of this potential limitation and frequently encouraged less-active participants to share their views during the focus groups.

2. Background

The U.S. clinical research enterprise is both expensive and inefficient (English, Lebovits, and Griffin, 2010). Less than one-half of health care in the United States is based on “the best scientific knowledge” (English, Lebovits, and Griffin, 2010), and many current treatments are drawn from studies that are 10 to 15 years old or are not based on clinical research at all (Institute of Medicine, 2001). Finally, patients and their caregivers are “often excluded from important discussions and left feeling in the dark about . . . how to navigate the overwhelming array of diagnostic and treatment options” that have been identified in clinical research (Bastian, Glasziou, and Chalmers, 2010).

An alternative vision imagines a health care research and delivery system that puts health care stakeholders—especially patients and their caregivers—at the center (Epstein and Street, 2011; Shaller, 2007) to develop a system of care “that is respectful of and responsive to individual patient preferences, needs, and values” (Institute of Medicine, 2001). This vision is in part what led to the establishment of PCORI in 2010 (Selby, Beal, and Frank, 2012). PCORI’s mission is

[to help] people make informed healthcare decisions, and [improve] healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community. (PCORI, 2014e)

This mission is aimed at changing the way research evidence is produced and used in practice.

Patient and stakeholder involvement in research is at the heart of every PCORI activity. Table 2.1 lists nine stakeholder “communities” PCORI has identified. PCORI seeks to meaningfully involve patients, caregivers, clinicians, purchasers, payers, industry, and other health care stakeholders in the entire research process, from topic generation to dissemination and implementation of results (PCORI, 2014a; PCORI, 2014b; PCORI, 2014d, PCORI, 2015g; PCORI, 2015h; PCORI, 2015i). It engages stakeholders by requiring all research applicants to identify their stakeholder partners in their applications (PCORI, 2014b; PCORI, 2015g) and requires research applicants to develop detailed engagement plans and involve stakeholders throughout the research cycle (PCORI, 2015h). In addition, PCORI engages stakeholders at national conferences, public meetings of its Board of Governors, regional events, multistakeholder and stakeholder-specific meetings, and webinars (PCORI, 2015i).

To describe the perspectives of stakeholder communities on CER, PCORI has funded independent studies of clinician, patient, caregiver, and researcher views (Forsythe, 2015). Stakeholders in health care have been described as having one or both of two characteristics:

Table 2.1. PCORI's Stakeholder Communities

Stakeholder	Definition
Patients	Persons with current or past experience of illness or injury, family members or other unpaid caregivers of patients, or members of advocacy organizations that represent patients or caregivers
Clinicians	Providers of health care in a clinical setting, including physicians, nurses, physician assistants, rehabilitative professionals, pharmacists, mental health care providers, complementary and alternative health care providers, and professional societies serving clinicians
Researchers	Those who conduct clinical research, including investigators or funders of research and organizations or associations representing the research community
Purchasers	Those who purchase health benefits for employees and their dependents, including individual businesses as well as local, state, regional, and national business groups, coalitions that represent businesses, and health coalitions
Payers	Those who function as financial intermediaries in the health system, including private insurers and public insurers, and organizations representing insurers, such as America's Health Insurance Plans
Industry	Companies that design, invest in, or manufacture diagnostics, devices, pharmaceuticals, electronic records systems, and mobile apps, and organizations representing the life sciences industry, such as the Advanced Medical Technologies Association
Hospitals and health systems	Organizations where care is delivered, including public and private hospitals and health systems, urgent care centers, retail health clinics, and community health centers, and organizations representing these facilities
Policymakers	Those who help craft public policy at any level of government, including federal, state, and local government officials; federal, state, and local units of government; and organizations that represent policy makers
Training institutions	Those that deliver health professional education, including public and private universities and colleges, individuals affiliated with the delivery or administration of health professional education, and trade or professional associations representing these institutions, organizations, and individuals

SOURCE: PCORI, 2014b.

They are health decisionmakers, are affected by health decisions, or both (Concannon et al., 2012). Thus, members of a stakeholder community may be actors in one health care decision and affected parties in some other stakeholder's decision. For instance, employers select health insurance plans; insurers decide what should be offered in health plans; and industry representatives determine the clinical interventions for which to pursue health plan coverage. These actors are affected by each other's decisions; they are also stakeholders in the information that is used to support their own and each other's decisions.

This report focuses on the views of purchasers, payers, and industry stakeholders. Because probabilistic sampling from these communities is not realistic, a focus group approach rather than a survey approach was used in this study. Focus group discussions explored health-related decisions stakeholders make in their work; their familiarity with and opinions about patient-centered CER; the PCORI research portfolio, including special initiatives, such as "real-world"

studies and PCORnet; and factors that may encourage or discourage stakeholder engagement in research.³

³ *Real-world* studies are large, pragmatic studies that compare outcomes between two or more approaches to addressing high-priority clinical issues in real-world settings. This differs from traditional clinical research, which conducts studies in highly controlled settings. PCORnet is a national network for conducting CER that establishes a resource of standardized health data to facilitate data sharing and use for network users.

3. Methods

Prior to each focus group discussion, participants were directed to a five-question online survey about their familiarity with CER and their current or anticipated use of CER. Following the survey, we conducted ten telephone and Web-enabled focus groups with four purchaser, three payer, and three industry subcommunity representatives (N=75).⁴ Purchaser subcommunities included small employers (<50 employees), medium-sized employers (50–499 employees), large employers, (500+ employees), and business coalitions. Payer subcommunities included private, public, and integrated payers.⁵ Industry subcommunities included device and diagnostics manufacturers, biopharmaceutical companies, and durable medical equipment (DME) manufacturers.

Each focus group was moderated by two experienced researchers. Discussions followed a protocol that included both core and community-specific sets of questions and probes. Focus group discussions were audio recorded and subsequently transcribed and anonymized for thematic analysis using MaxQDA, a qualitative data management software package that facilitates the analysis of large amounts of textual data. Appendix A details our approach to data collection and analysis.

⁴ The term *subcommunity*, as used here, refers to subgroups of individuals or organizations within the three larger stakeholder communities.

⁵ Integrated payers are organizations that serve the dual role of both the provider of health care services and the payer of services.

4. Key Themes

This section identifies key themes that emerged from the ten focus group discussions. Because of limitations related to focus group research, our findings should be interpreted as *themes* about the shared and conflicting views held within and across the three stakeholder communities we studied. This presentation of shared and conflicting views should not be interpreted as an established consensus or disagreement among all members of these communities. Key findings are drawn from the extensive presentation of research results in Appendices B and C.

Purchasers, Payers, and Industry Communities Make a Variety of Health Decisions and Seek Information from Multiple Sources

No effort to involve stakeholders in research can be successful if the research agenda is poorly designed to meet their decisionmaking needs. The prospect of meaningful stakeholder involvement and better-informed decisionmaking starts with addressing relevant questions. Thus, our research team began focus group discussions by asking participants to describe health-related decisions they make and the information and sources they use to support the decisions.

Employers reported being concerned about purchasing a health insurance benefit that advances their employees' health and well-being at a cost both the employee and the employer can afford. Additionally, all employer groups mentioned decisions regarding wellness incentives, although small and medium-sized employers were more vocal about wellness programs than large employers and business coalitions were.

Payers reported being concerned about the details of designing health insurance and other health benefits: which interventions to cover and for whom; how to establish copays; which providers to include in networks, etc. Payers sharing risk with provider organizations—especially integrated organizations—are also concerned about the details of operating a health care delivery system and providing care recommendations to providers. Public payers are concerned about establishing programs for vulnerable populations, eligibility thresholds, and vendor and provider payment policy; private payers described making decisions about which software to use for their claim system, conducting technology assessments, and selecting vendors for plan programs.

Industry stakeholders were focused on establishing product coverage and reimbursement, understanding when treatments were more effective among particular patient populations, understanding the real-world effectiveness of treatments, establishing information about the appropriate use of their products, and informing the selection and development of new products or technologies. For participants in the device and diagnostics focus group, there was additional

emphasis on providing information to clinicians about the appropriate selection of diagnostic tests, procedures, and devices.

Stakeholders reported using a variety of information resources to support their health-related decisions. Purchaser and payer communities consider both clinical and cost information to be important ingredients in their decisionmaking about health benefits, especially about health plans. Private payers put these concepts together to say they seek information about the “value” of interventions. Similarly, industry stakeholders expressed the need for clinical effectiveness results to be translated into payment policy because “if the payer doesn’t pay for it, it isn’t going to get done.” For industry stakeholders, the demand for both clinical outcomes and cost information from payers translated into a corresponding need to develop information about the value of their products.

Some but not many of the information sources reported by one stakeholder subcommunity were reported by the others (see Appendix C). While all employers use some form of internal or external benefits counselor, small businesses use employee surveys to support their decisions, while large employers do not. Small employers go online to search for additional information, while employers with 50 or more employees suggest that online resources lack credibility and are therefore risky to use for decisionmaking. Large employers consult clinical research literature, on-site clinicians, and professional society practice guidelines to get information on the benefits and harms of interventions, drugs, and other health products.

Health care decisions made by private payers, including but not limited to health insurance coverage, are often also grounded in the medical literature, but private payers additionally mentioned expert reviews. Public and integrated payers make use of these same resources but additionally cited administrative records as a central information source (integrated payers) and outside groups to collect information for them (public payers).

Industry participants focused on evidence they develop as part of product approval, through postmarketing safety surveillance registries (biopharmaceutical and device and diagnostic) and collaborative partnerships with other stakeholders, such as academic researchers, clinical societies, and payers (biopharmaceuticals and device and diagnostic). Participants reported that evidence development is less likely to be funded or generated by DME organizations.

Familiarity with CER Is High Among Payers and Industry but Not Among Purchasers

PCORI seeks to fill information gaps and become a trusted source of information by offering patient-centered CER as a resource to stakeholders and decisionmakers.⁶ As with any research,

⁶ *Patient-centered CER* is research that considers patients’ needs and preferences and focuses on outcomes that are most important to patients, while also comparing the effectiveness of two or more treatments or approaches to health care, examining their risks and benefits. This definition represents the specific manner in which PCORI carries out CER.

CER will be relevant only if it addresses the decisions and questions stakeholders have. Our focus group discussions continued with an exploration of these questions: Are stakeholders familiar with CER? What decisions can CER support? Who will benefit from CER?

While all payer and nearly all industry participants were already familiar with CER, only about one-half of purchasers were. All large employers but only a minority of small and medium-sized employers were familiar with CER before the discussions began.

Participants in all groups agreed that CER could support a number of health care–related decisions. Participants described a wide range of health care–related decisions that could benefit from CER. Purchasers and payers described using patient outcomes, access, and affordability data to make coverage decisions and care recommendations for specific populations. Industry representatives described decisions about the use of products in the real world, identifying subpopulations for whom a product is more (or less) effective, and improving decisions about investing in emerging products or technologies. Device and diagnostics firm representatives said they currently are seeking to add patient-centered outcomes to existing postmarketing registries; however, these registries do not allow comparisons with other treatment alternatives. DME stakeholders suggested this kind of information might help move their products out of a “commoditized” market—which is largely sustained by Centers for Medicare & Medicaid Services (CMS) competitive bidding rules—and help them establish value-based, rather than cost-based, purchasing arrangements with payer organizations and CMS. CER was viewed as a potential way to differentiate one product or service from others.

Involving Purchaser, Payer, and Industry Stakeholder Communities in CER Beyond an Advisory Capacity Will Be Difficult Unless CER Is Aligned with Business Interests

“Involvement” means that stakeholders may serve on research projects as partners, staff, consultants, or advisors and be involved in any stage of research, from nominating topics to developing questions, designing the research, conducting the research, assessing the results, translating findings to peers, and implementing findings in their own health-related decisions. As envisioned by PCORI, research must ask the right questions, must be conducted with the involvement of stakeholder communities to be fully transparent, and must be translated into stakeholder-specific information to be usable. Our focus group discussions therefore continued with an exploration of stakeholder views on engagement in research: Are they willing to be involved, in what activities, and in what ways?

Nearly all participants described a willingness to play an advisory role in research, but fewer had experience or willingness to lead or fund research. Only some subcommunities and participants were willing to lead, partner to lead, or fund research projects. Some but not all participants in all three payer groups, the biopharmaceutical group, the device and diagnostics group, and one member of the large employer group described having previous experience

leading CER studies. Some private payer organizations reported having an existing research arm, serving as a data contributor for independent research studies, or currently participating in a CER collaborative. Similarly, integrated payers reported participation in research studies, with several participants reporting that their organizations have been involved in non-PCORI-funded CER.

All participants cited past experience with CER or expressed a willingness to be involved in CER if the research topic design is aligned with business interests. Participants considered alignment of business interests with study aims and design to be critical to decisions to participate in research; they noted, however, that it is a challenge to find topics and research questions on which there is cross-organizational and cross-community interest. Research leaders should clearly articulate the expected benefits of involvement; the research team should be organized and efficient about asking for contributions of time and resources from stakeholders; and results should be translated into information that is usable by the community.

Most of the stakeholder communities expressed more interest in the activities leading up to and following research than in the activities during the conduct of research. Topic nomination, question development, research design, and implementation in practice settings were the activities that fit best with stakeholder interests across the board. Representatives from five of the ten discussion groups—large employers, biopharmaceutical firms, device and diagnostics firms, integrated provider-payer organizations, and private payers—expressed a willingness to conduct CER studies.

Sharing data is one way that purchasers can participate in research. However, participating purchasers had different opinions about sharing their employees' deidentified personal health data. Small and medium-sized employers described a willingness to share deidentified employee data, while most large employers expressed reservations about sharing data or facilitating employee participation in research. Large employers did not wish to be seen by their employees as endorsing a specific trial or study.

Some participants from biopharmaceutical and device and diagnostics firms expressed reservations about participating in the dissemination of research. Label restrictions and other regulations have a dampening effect on participation in translating and disseminating the results of studies. Dissemination is prohibited unless the information is limited to indications included in the product label or permitted under Section 114 of the Food and Drug Administration Modernization Act (FDAMA) of 1997 (FDAMA 114). The act was designed to allow companies to disseminate health care economic information about their products, but requirements regarding the type of audience, standards of evidence, and product use have posed several barriers to industry's use of the act (Perfetto, 2015).

PCORI Mission

We explored participant reactions to PCORI's mission statement:

PCORI's mission is to help people make informed health care decisions and improve health care delivery and outcomes by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader health care community. (PCORI, 10)

After reading the mission aloud during the focus group discussion, the moderator asked participants to identify words or phrases that they considered important and to make note of any important topics or issues that were not captured in the mission. In every discussion, the statement was read aloud a second time by request of one of the participants. Although most participants liked the mission statement, some participants expressed concerns because of its breadth and its perceived lack of emphasis on research translation and implementation.

Purchaser, payer, and industry stakeholder communities identified aspects of PCORI's mission that stand out. Following a prompt from the focus group facilitator, every participant in all ten focus groups picked out words and themes in PCORI's mission that stood out to him or her. Among those most frequently mentioned were "high integrity," "improved delivery," "informed decisionmaking" "evidence-based information," "improvements in health care delivery," "improvement in health outcomes," and "to help people." Participants also routinely called out the mission's emphasis on patient-centeredness and a multistakeholder approach, and all groups agreed that patient-centered CER would be of benefit to patients and clinicians.

There was concern from some stakeholders about the mission's scope. Participants from the business coalition and large employer groups expressed reservations that the mission is too lofty, has too many outcomes, is too broad in scope, and may be unattainable. While participants in the biopharmaceutical and DME groups commended the use of information for decisionmaking, participants in the public payer, small business, and large employer groups criticized a perceived lack of emphasis on research translation, dissemination, and implementation. To these participants, establishing "usable" information is a challenge not fully addressed; they expressed concerns that translation of evidence from clinical jargon into language that takes their environments and decisional problems into account is a potential roadblock the mission does not address head on. These were among the same groups that repeatedly returned to the lack of cost information in PCORI-funded research, saying that findings without this information are not useful to support coverage- or payment-related decisions.

Purchaser, Payer, and Industry Stakeholders Agree that PCORI's Funding Portfolio Prioritizes High-Prevalence, High-Cost Medical Conditions, but Each Community Has Different Priorities

Holding high-cost and high-prevalence conditions as top priorities was a clear area of agreement, and all groups felt the list of priorities matched priorities for their communities, although the employer stakeholder groups noted the importance of adding wellness and

prevention interventions for people with or without specific clinical conditions and diseases. However, participants both within and across focus groups had differing opinions about the specific ranking of priorities.

For medium-sized and large purchasers, long-term outcomes of diabetes and musculoskeletal issues rose to the top of the list. Conditions that contribute to presenteeism and absenteeism were also prioritized,⁷ including conditions of aging that can affect the productivity of workers who serve as caregivers at home.

Private and integrated payers were concerned about cancer and cardiovascular conditions, and public payers were concerned about mental and behavioral health conditions, particularly in combination with any of the other high-cost conditions. Private payers expressed interest in research on autism, a condition not appearing as its own separate condition on the list. Public payers emphasized that such services as care coordination and patient education should be top priorities, and integrated payers recommended studies on the social determinants of health. One public payer suggested that PCORI allocations by condition resemble the approximate budget allocations to the National Institutes of Health's (NIH's) institutes and centers (i.e., as a share of total NIH funding) and suggested that these allocations may be the result of established researchers applying for and winning PCORI funds in large numbers.

Aside from their agreement on multiple chronic comorbidities, industry groups diverged from each other on other priorities. For example, one biopharmaceutical representative stressed the importance of supplementing clinical trial findings with information about how treatments perform in the real world rather than in clinical trials. Another participant, representing the device and diagnostics industry, was interested in research on personalized approaches for care delivery. Within the DME group, members suggested research that would segment patient populations based on sites or transitions of care (e.g., community-based care versus inpatient care versus outpatient care versus multiple sites of care). Industry representatives affirmed that PCORI funding allocations by clinical population seemed about right.

The Idea of Priority Populations Resonated with Some Payers but Did so Less with Employers and Industry Stakeholders

Representatives from purchaser, payer, and industry organizations agreed that PCORI's priority populations reflect public health priorities.⁸ Payer representatives were supportive of a focus on priority populations. Their interests centered on racial and ethnic minority groups;

⁷ *Presenteeism* is the practice of coming to work despite being ill, often resulting in reduced productivity.

⁸ To better address disparities in the U.S. health system, PCORI has identified "priority populations," or groups of individuals who shoulder a disproportionate burden of disease. PCORI's priority populations include racial and ethnic minorities; low-income individuals; older adults; residents of urban areas; residents of rural areas; women; individuals with multiple comorbid conditions; children; individuals with low health literacy; individuals with disabilities; individuals with rare diseases; veterans; and the lesbian, gay, bisexual, and transgender community.

people with disabilities; and for the large and integrated payer groups, lesbian, gay, bisexual and transgender (LGBT) populations. However, the utility of information grouped by priority populations had little relevance for employer and industry stakeholders.

Purchasers agreed with each other that PCORI's priority populations were of interest insofar as they reflect the compositions of their workforces. Larger employers considered information about pre- and postretirement age helpful, but they agreed with business coalition and medium-sized employers that they would not organize information by these priority groupings. Offering health and insurance benefit design options, they suggested, is about meeting the needs of all employees and not about targeting priority populations. The one exception was a priority for populations with multiple chronic comorbidities, which can be costly in an insurance benefit. Small employers expressed positive views about PCORI's priority populations, perhaps because such employers are able to consider individual rather than collective employee needs in selecting benefits.

Industry representatives had similar views. While priority populations include people typically underrepresented in clinical studies, industry representatives said they include all patients who may be eligible for care. Industry-funded studies do not typically prioritize one population over another unless the population is specific to the condition studied or the decision the research seeks to inform (e.g., for Medicare coverage).

Purchaser, Payer, and Industry Stakeholders View Real-World Studies as Useful but Have Different Opinions About Design Elements in the Two Example Studies

PCORI's "real-world" studies seek to provide information that can be adopted by providers. They address critical clinical choices faced by patients, caregivers, clinicians, and systems, are often conducted in routine clinical settings, and are often large and less complex than traditional trials.⁹ In the context of the focus group discussions, stakeholders were presented with information about two such studies: "Strategies for Active Surveillance of Patients with Small Pulmonary Nodules" (a study of the optimal frequency of screening for patients at risk of lung cancer) and "Early Supported Discharge for Improving Functional Outcomes after a Stroke" (a study that compares care approaches for stroke patients following discharge from the hospital). Appendix D describes both in greater detail.

Stakeholders agreed that real-world studies address important topics and questions, with some caveats. In all groups, most participants liked the large study sample sizes,

⁹ *Complexity* refers to the level of detail in a study protocol. Real-world studies are less complex insofar as they allow participants to enroll with fewer restrictions and evaluate interventions as they are practiced in usual care settings. Such studies are less intrusive to routine clinical practice and are considered less complex than a typical efficacy trial.

randomized study designs, and the notion of studying interventions among a variety of patients as they are treated in usual care settings.

Participants also commented on the potential usefulness of real-world studies to other stakeholder groups. All payer focus groups noted that real-world studies could theoretically help support their payment or treatment guideline decisions but cautioned that translation into payer-relevant findings, policies, or actions is important. As one participant from the integrated payer group summarized, there is “a difference between having evidence that it might work, and then actually being able to make it work.” Finally, industry participants also had positive impressions of the real-world studies; considered them to be complementary to, rather than duplicative of, studies other stakeholders are funding; and stated that they were “very consistent with the mission of PCORI.”

However, while purchasers could articulate the value of these studies to patients as they work with their health care providers, some employers expressed reservations about the value these studies could bring directly to employers. The value to employers was indirect, mediated through the potential for improved health and productivity of employees. Thus, some employers did not see a direct connection between real-world studies and information needed to support their decisions.

Stakeholders had different opinions about the specific design elements of the two example studies. Purchasers recommended that the outcomes of the stroke study be expanded to include return-to-work measures. Payers suggested that studies comparing an intervention with “usual care” need input from the insurance community about what usual care entails for a particular population. Industry representatives sought additional information on the study design features. For example, device and diagnostics groups sought to justify the need for the sample size for the pulmonary nodules, and the DME representatives wanted to know more about which DME supplies and services were being captured in the stroke care study.

The large enrollment in real-world studies has implications for the length of time it will take to produce findings, so there is interest in the feasibility of disseminating early or interim results from these studies. Purchasers viewed dissemination of early results favorably, even while recognizing that the results could change. Payers were more cautious about early dissemination, expressing concern that the reputation of funders and researchers could be harmed from releasing results that are subject to revision. Similarly, some industry representatives cautioned that publishing interim findings should be considered on an individual study basis. If interim results are clinically meaningful, unlikely to change over the longer term, and actionable, they may be valuable. Less support was voiced for sharing interim results that are not conclusive or actionable.

Familiarity with PCORnet Is Low in Most Groups, but Stakeholders Saw Value in the Initiative once It Was Described

Familiarity with PCORnet was mixed in the payer and industry groups and not very high among purchasers but was well received when presented. A minority of participants in our focus groups had prior knowledge of PCORnet. More participants in the private payer, biopharmaceutical, and device and diagnostics groups reported familiarity with the initiative than did participants in other groups. Once we presented information about the initiative and its signature trial, ADAPTABLE,¹⁰ the notion of establishing a distributed network of this nature was well received. Participants noted that PCORnet uses new types of data that otherwise may not be easily available, has the potential to facilitate the conduct of large-scale studies with limited resources, can facilitate comparative analyses of health care delivery systems and their patient-level outcomes, and can create “an objective neutral playing field for investigation” of important health care questions. Both payers and industry groups wanted to know more about how PCORnet differs from other research data networks. All communities expressed the desire for more information about PCORnet.

As with prior comments about PCORI funding allocations, **there were a variety of opinions about the topics and research questions that PCORnet could help address.** Purchasers talked about provider quality measurement, benefit design, provider network composition, formulary development, and smoking cessation. Payers suggested appropriate use criteria for knee and hip replacement and comparison of interventions across intervention types (such as drug versus device versus surgery). Other mentions included treatment recommendations, patient safety, generic versus nongeneric products, hypertension, diabetes, multiple sclerosis, and rare diseases. Industry stakeholders recommended including administrative claims data and device information to complement the electronic health record (EHR) infrastructure. While industry groups offered a long list of advantages related to PCORnet—network scale, diversity of health systems, access to efficient research infrastructure, ability to collect patient-generated information, capacity for longitudinal studies, assessment of health care utilization, and an opportunity to identify links between patient symptoms and use of DME—they also had a number of questions about data quality, interoperability, governance, and access.

All Three Communities Saw Value in CER for Patients, Providers, and Other Stakeholders

PCORI was established to develop information that helps patients make informed decisions about their health care, and PCORI develops patient-centered CER to address that need. In this

¹⁰ ADAPTABLE—“Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-term Effectiveness”—is a PCORI-funded study that aims to identify the optimal dose of aspirin to improve health outcomes for individuals with cardiovascular disease. Appendix D provides more information about the ADAPTABLE trial.

context, it is critical to understand how stakeholder groups view the value of CER, both in general terms and in the manner in which PCORI has carried it out.

There was agreement across stakeholder communities that CER is valuable to patients and clinicians; CER has the potential to be valuable to health systems, payers, purchasers, and industry. Participants stated that CER information would be especially beneficial to patients and providers and they described a number of ideas about how CER might offer benefits in their own communities. The perceived potential value of CER was considered especially high when design, implementation, and translation can be carefully tuned to the needs of each stakeholder group.

Purchasers viewed CER as being informative to benefits consultants and thus indirectly to employers who hire these consultants. Large employers and business coalition representatives also suggested CER would help consumers make better health care decisions, providing indirect benefit to employers. Participants in the small business group suggested that CER offers limited direct benefit to their community because it is too population, condition, or drug specific, but it may inform their benefits advisors and, thereby, provide indirect benefits to their community. Private payers suggested that CER has a potential to inform the design of prescription drug benefits. Public payers stated that it could directly inform health care payment policy and lead to changes for both public and private insurers. Finally, integrated payers suggested that CER would be useful for developing care guidelines. On the industry side, producing new CER evidence presents a hurdle for biopharmaceutical organizations insofar that it requires adding research capability where it may not have existed before. One stakeholder suggested this evidence could help them target investments in products that are truly innovative from a value standpoint. Similarly, the biopharmaceutical and DME participants noted that better understanding of patient perspectives and satisfaction could inform the quality of health care provided. One participant in the DME group stated that CER is “a method to maintain a market-driven product line” and to illustrate the importance of the role DME plays in patient care. DME stakeholders suggested this kind of information might help move their products out of a “commoditized” market—which is sustained by CMS competitive bidding rules—and help them establish value-based purchasing arrangements with payer organizations.

Cost Information Is Important to the Decisions of Purchasers and Payers

While our focus group protocol did not call on participants to speak about the use of cost information in health-related decisions or about its inclusion in research, participants in all stakeholder communities raised the issue independently. For these participants, the anticipated absence of cost information in PCORI-funded CER will be a major roadblock to its usefulness.¹¹

¹¹ Although PCORI does not fund cost-effectiveness studies, it can fund studies that measure costs.

Most participants in the large employer, business coalition, public payer, private payer, and integrated payer focus groups said the value of CER is limited if it is not linked to cost information. Purchasers described cost information as important for the health care decisions they make: The cost of services is an important factor in determining the true value of health care, especially as it “relates to the copay and the coverage”—things that patients, purchasers, and payers all are concerned about. Payers also discussed cost-effectiveness while describing the health care decisions they make, noting that “cost-effectiveness is an important issue. It’s part of the business decisionmaking.” Some payers, especially representatives of integrated payers, noted that, to encourage payer engagement in research, “cost has to be one of the key outcomes.”

One public payer representative suggested that this roadblock extended to patients’ use of CER. The individual stated that “PCORI’s limit of looking at cost is not patient-centered” because patients’ care preferences are anchored on both care outcomes and costs. The person added that “we sort of make it sound warm and fuzzy, but still we aren’t addressing the hardest piece that patients care about most . . . often, not most, but they care about significantly.” This sentiment was echoed immediately by four other participants in the same group.

Purchasers and payers raised the issue more frequently than industry participants did. However, industry groups echoed the general point about tailoring CER information—including costs—to the needs of payers. These participants described producing economic information as part of CER-type studies they fund and conduct for payers. Biopharmaceutical participants described presenting cost information in their self-funded effectiveness research because payers ask for this information. Device and diagnostics firm participants said they are not currently being asked by payers to provide economic information on their products, but they anticipate being asked for this information in the future.

5. Implications

The findings in this report should help PCORI, other funders, and researchers strengthen engagement opportunities with difficult-to-reach but vital stakeholder groups. Purchasers, payers, and industry stakeholders make decisions that affect patients and clinicians every day. For the research community to develop relevant, high-quality CER that can inform the decisions of these stakeholders, researchers should examine the decisions these stakeholders make in their work, the information sources they currently use, and how CER can help to inform those decisions. The focus groups we conducted explored these issues in detail, and our findings suggest several important implications:

- Stakeholders described an array of information resources they depend on to support decisionmaking (Appendix C). PCORI can use this information to design dissemination and communication strategies that will reach stakeholders through the information sources they most value.
- Because of the importance of benefits counselors in supporting purchaser decisions, future efforts to understand the information needs of the purchaser community might include benefits counselors in the recruitment strategy.
- Variation in familiarity with CER across these communities is a concern that should be addressed. In particular, significantly more work is needed to reach employers. A consistent message and persistent, bidirectional engagement are needed to explain what CER is, how it can support decisionmaking, and how its findings can be implemented.
- The research community should consider how proposed research topics overlap with the business interests of these stakeholder communities. This information can be used to explain why they should be interested in being involved in the design and conduct of research and in the implementation of findings. For research topics that overlap with the business interests of more than one of these stakeholder communities, careful consideration should be given on how to structure engagement opportunities that can make the research more useful to all stakeholders.
- The concerns we heard about the scope of PCORI's mission focused on two areas: its breadth and a perceived lack of focus on the translation of findings. While these views may reflect a desire to see the mission altered, PCORI is not in a position to alter its mission beyond the authorizing legislation to which it closely adheres. However, the comments we heard about its mission suggest an opportunity to develop more-detailed communications and ensure that stakeholders understand PCORI's origin in the authorizing legislation.
- PCORI's special initiatives—real-world studies and PCORnet—hold promise for meeting the information needs of these three communities, but they are not widely understood. Focused work is needed to ensure the understanding and involvement of stakeholder communities in PCORI's special initiatives.
- The importance of producing useful research findings was a theme that came up frequently and presents an opportunity for PCORI and other research funders. Research

projects will be more useful if they are conducted on topics important to stakeholders, if stakeholders are involved in research design and conduct of research, and if findings are translated into information that meets the specific needs of individual stakeholder communities.

- Payers and purchasers were not asked to address whether costs, cost effectiveness, insurance coverage, and benefits design should be part of patient-centered CER. However, they repeatedly introduced these subjects into the discussions without prompting. They were in wide agreement: Research that does not improve their ability to answer coverage and benefits design questions will be difficult to use. While PCORI does not support cost-effectiveness analysis, many PCORI-funded projects do address some types of the costs of care. PCORI can do a better job of communicating about the types of cost information that may be included in its research. In addition, understanding the importance of cost information to purchaser and payer communities can help PCORI find areas of overlap with their business interests; it can help PCORI draw these communities into projects, design translation materials, and point these communities to outside information that can help them use PCORI-funded CER.
- Agreement among stakeholder communities on funding allocation priorities and the selection of priority populations would be stronger if PCORI can communicate about its ranking and prioritization criteria more effectively. There was agreement in our groups that funding should be directed at answering questions that affect more patients or address high-cost conditions and treatments, and there was reasonably strong agreement that the lists of conditions and populations identified as being of high priority to PCORI contained the right groups of patients. Other important factors include the feasibility of studies and return on investment. Articulating a consistent rationale for priorities could help build support for the priorities.

Appendix A. Approach

This appendix provides more detail on the approach we used in the study reported here, including the rationale, discussion protocol, stakeholder identification and recruitment procedures, data collection, coding and analysis, and reporting procedures.

Why We Used Focus Groups

Representatives of purchaser, payer, and industry stakeholder communities are difficult to survey because there are no national lists or inventories of members for these communities. Further, if there were such lists, it is not clear—without extensive knowledge of the communities—which individual(s) in an organization would be most responsible for health-related decisions. For instance, large employers across a range of industries have a variety of human resources structures for purchasing health insurance. Knowledge about industries, employers, and their administrative structures is critical to finding key informants. To ensure that we solicited input from the appropriate people, we invited key informants to provide their input in a group setting to learn about their perspectives and to allow them to engage with, and learn from, each other.

For these reasons, we chose a focus group strategy for data collection. A typical sampling approach for a focus group strategy seeks to identify key informants with prespecified vantage points on a topic. The discussion protocol is designed to explore views until no substantive new material is provided by participants (referred to as “theoretical saturation”) (Kitzinger, 1995; Liamputtong, 2011; Hopkins, 2007). In collaboration with PCORI staff, ten groups representing three stakeholder communities were invited to participate (see Table A.1).

To increase our sample size and the chances of reaching theoretical saturation, we conducted three or four focus groups with different types of stakeholders within each stakeholder community. We also selected these subcommunities to maximize the diversity of possible views on health-related decisions stakeholders make, information resources they use, CER, and PCORI’s work. To illustrate, employer decisions about which health-related benefits to provide are likely to vary by size of the organization. For this reason, we recruited individuals representing small (<50 employees), medium-sized (50–499 employees), and large (500+ employees) employers to join focus groups composed of their peers.

Segmentation of participants into relatively homogeneous groups facilitates an analysis of common and different themes that emerge across groups (Kitzinger 1995). Moreover, homogeneity of participants may help ensure more active participation and free expression of opinions in situations where too much heterogeneity (e.g., differences in social statuses or power) might suppress the willingness to share views (Freeman, 2006). For example, midlevel

Table A.1. Stakeholder Communities and Subcommunities

Community	Subcommunity	Definition
Purchasers	Small business	Independently owned businesses with fewer than 50 employees (part time or full time)
	Medium-sized employers	Independently owned businesses with 50–499 employees (part time or full time)
	Large employers	Businesses with 500 or more employees (part time or full time)
	Business coalitions	Organizations that support or advocate for business, including local, state, regional, and national business coalitions or chambers of commerce
Payers	Private health insurers	Insurance organizations that market private health insurance plans to employers, individuals, or groups.
	Public health insurers	Organizations that provide public coverage through state or federal programs
	Integrated payers and service delivery organizations	Organizations that either (1) serve the dual role of both provider and payer or (2) have developed an integrated care or service delivery model
Industry	Biopharmaceutical organizations	Organizations that either (1) develop and market pharmaceuticals or (2) represent or provide consulting services to pharmaceutical companies
	Device and diagnostics organizations	Organizations that either (1) develop and market health and medical device and diagnostics products or (2) represent or provide consulting services to device and diagnostics companies
	DME organizations	Organizations that either (1) develop and market durable medical equipment or (2) represent or provide consulting services to DME companies

managers from large payers, purchasers, and industry partners may be unwilling or unable to join group meetings, particularly in person, with individuals from senior management or the executive offices of an organization or with others whom they consider to be outside their peer group (Freeman, 2006).

Discussion Protocol

Following selection of stakeholder communities and subcommunities, we met with representatives from PCORI to determine priority topics and key questions that would be explored during the discussions. In collaboration with PCORI, we developed a discussion protocol to provide a consistent structure to each focus group discussion. The discussion guide included open-ended questions asked during all focus groups and addressed such topics as health-related decisions made by the participants, information needs, familiarity with and use of CER, and PCORI’s mission and goals, research portfolio, and special initiatives. The discussion guide also included probes and follow-up questions to ensure that themes were fully explored. See Box A.1 for the primary questions in the discussion protocol.

Box A.1. Focus Group Topics and Questions

Health-related decisions, information needs, and CER

1. What kinds of health care decisions does your organization typically make in the course of a year?
2. What kinds of information does your organization use to make these decisions?
3. Prior to this discussion, were you familiar with comparative effectiveness research?
4. What kinds of decisions can CER support?
5. Who can benefit from CER?

Stakeholder involvement in research

6. Some different ways that stakeholders become involved in research is to suggest research topics to study, being partners in research, or helping to share research evidence. Would you view it as possible or feasible for [STAKEHOLDER GROUP] to be involved in research in some way?
7. What would help to ensure [STAKEHOLDER GROUP] involvement in research?

PCORI mission, research, and initiatives

8. Following a presentation of PCORI's mission: Is there a particular word or phrase in this mission that stands out to you?
9. Following a presentation of PCORI funding allocations by disease or condition: Are these the same topics that [STAKEHOLDER GROUP] are most interested in?
10. Following a presentation of PCORI funding allocations by priority populations: Are these the same populations that [STAKEHOLDER GROUP] are most interested in?
11. Following a presentation of PCORI real world studies: What do you think of PCORI's real world studies?
12. How would you change these studies to make them more useful to [STAKEHOLDER GROUP]?
13. Following a presentation of PCORnet and its signature trial, ADAPTABLE: Prior to this discussion, had you heard of PCORnet?

Value of CER

14. Having had this discussion, how would you describe the value of CER, considering its advantages and disadvantages?

The discussion guide was supplemented with community-specific probes to address topics of concern for individual stakeholder communities or subcommunities. For instance, follow-up questions about the impact of CER on technological innovation were added to industry discussions. Moreover, more-detailed follow-up questions were added to the discussion protocol on PCORI special initiatives after previous focus groups showed lower-than-expected familiarity with these initiatives. The focus group discussion guide was thus a living document that evolved as themes and early findings emerged.

Stakeholder Identification and Recruitment

Each focus group consisted of to six to ten participants, a size that allows participants to share their experiences and perspectives, meaningfully interact with each other, and complete the full discussion within two hours.

A structured outreach and recruitment plan was developed. We used our existing network of organizational partners and contacts, PCORI suggestions, online public registries, and nominations from already recruited participants or those who could not participate. A mix of individuals representing different characteristics (e.g., organization size or type) was recruited from each region of the United States for the focus group discussions. For example, regional and national businesses working in the service, technology, construction, and other industries were

recruited for purchaser focus groups, while regional and national health plan representatives were recruited for payer focus groups. Representatives of large, medium-sized, and small organizations working in small and large molecules, diagnostics, devices, and imaging were recruited for industry focus groups.

The recruitment plan also included email templates, telephone and voicemail scripts, and orientation materials. Approximately one month prior to the discussion, an introductory email was sent to all stakeholders on the contact list, after which we reached out by telephone and email at prespecified intervals until recruitment targets were met. After a participant agreed to participate, he or she received a confirmation email and an Outlook calendar invitation, as well as a brief orientation packet that provided high-level information about the study's purpose, topics of discussion, and logistical information about signing on to the Web component and receiving honoraria. The orientation packet also included a link to a brief prediscussion survey designed to gauge the participants' overall familiarity with CER, as described in the Data Collection section below.

The stakeholder identification and recruitment process resulted in outreach to 631 individuals across all stakeholder communities—between 24 and 125 in each stakeholder subcommunity, with an average of 63 individuals contacted for each group. A total of 75 participants participated, with 28 in purchaser discussions, 22 in payer discussions, and 25 in the industry discussions. Table A.2 presents a detailed picture of our recruitment and participation results across all ten groups.

The acceptance of invitations was much higher than anticipated among small employers (45 invitations were made; eight were accepted) and much lower than expected among integrated payers (125 invitations were made; eight were accepted). Small business owners were relatively easy to reach by email and telephone. Integrated payers responded to email and phone outreach at much lower rates. Once reached, there was far less willingness among integrated payers to participate.

Data Collection

Prior to each focus group discussion, all participants who agreed to participate in the study were asked to complete a five-question survey (Box A.2) that explored participants' familiarity with CER, how much they or their organizations have previously used CER, their expectations for the use of CER in the future, and their perception of the value of CER. The survey consisted of both closed- and open-ended questions and was administered online. A link to the online survey was sent to focus group participants in the confirmation email, and reminders were sent one week and, again, one day prior to the discussion group. This information was used to help gauge the participants' familiarity with the discussion topics and help moderators prepare follow-up questions.

Table A.2. Results of the Focus Group Recruitment Process

	Purchasers				Payers			Industry			Total
	Large Employers	Medium-sized Businesses	Small Businesses	Business Coalitions	Public Insurers	Private Insurers	Integrated Payer	Bio-Pharmaceutical	Device & Diagnostics	Durable Medical Equipment	
Total contacted	60	95	45	35	75	92	125	24	26	54	631
Agreed to participate	7	8	8	10	8	9	8	10	9	10	87
Completed pre-focus group survey	6	4	4	9	7	6	7	9	7	7	65
Participated in focus group discussions	7	6	6	9	8	7	7	9	7	9	75

Box A.2. Pre-Focus Group Survey Questions

1. How important is patient-centered CER to your industry or sector?
 - a. Very important
 - b. Somewhat important
 - c. Not important
2. How have you or your organization used patient-centered CER? (Choose all that apply.)
 - a. Sponsoring the research
 - b. Using CER in our work
 - c. Not involved in any CER
3. In the next 3 years, how do you or your organization expect to use CER in the conduct of your work? (Choose all that apply.)
 - a. Sponsoring the research
 - b. Using CER in our work
 - c. Not involved in any CER
4. In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?
 - a. Sponsoring the research
 - b. Using CER in our work
 - c. Not involved in any CER
5. How would you describe the value of patient-centered CER, considering its advantages and disadvantages?
[Open-text response]

From June through October 2015, we conducted ten Web- and telephone-enabled focus groups. All focus groups lasted two hours and included between six and ten participants.

Participants were encouraged to speak from their own experience and the perspective of their subcommunity.

Discussions were led by an experienced discussion moderator and supported by a comoderator. Moderators and comoderators were trained on the discussion guide and protocol. A research assistant was trained to take detailed notes using a form that mirrored the major topics of the discussion guide. The note-taker and moderators used a tracking tool to note participants' participation in the discussion and to signal the need to call on participants who were not participating fully in the discussion on each topic. Each discussion was audio recorded and professionally transcribed; all recordings were destroyed after transcripts were checked for completeness. Focus group transcripts were deidentified prior to coding and analysis.

Coding and Analysis

Focus group transcripts were coded thematically. We created a hierarchically organized codebook, starting with the focus group discussion questions as a framework. The codebook, which consisted of a list of codes accompanied by a description and simple examples, and all focus group transcripts were entered into MaxQDA, a qualitative data-management software (MaxQDA, 2015). The use of MaxQDA facilitated data coding, helped ensure coding consistency across focus group transcripts, and made it easier to update the coding scheme and extract comparable information from all focus group transcripts. Analysis of the transcripts started after the first focus group discussion took place. The codebook was refined following the completion of each subsequent focus group discussion by adding new codes and merging existing codes.

We used both deductive (i.e., based on the focus group core discussion guide questions) and inductive (i.e., data-driven) approaches to thematic data coding. After initial deductive coding of several focus group transcripts following the discussion guide structure, we began identifying and inductively coding unanticipated emerging themes that spanned discussion topics. All transcripts were coded by the same researcher with extensive experience in qualitative data analysis, supported by consultation with focus group moderators where necessary. Once all transcripts were coded, the coder reviewed the coding scheme and coding output to ensure quality. Coding results were used to produce subcommunity-specific reports (Appendix C). Finally, the research team used a purposeful approach to the constant comparative method of qualitative analysis to identify differences and similarities within and between stakeholder communities and their views of patient-centered CER and PCORI's mission and research (Boeije, 2002).

Reporting

Findings from focus group interviews were reported iteratively to PCORI throughout the data collection and analysis period. An initial summary produced from notes taken during each discussion was provided to PCORI ten business days after the discussion was held. This report included a summary of the prediscussion survey results, moderator's impressions, and a

summary of the responses to major topics addressed in the discussion protocol. A formal report was produced from transcription coding and analysis and was provided to PCORI 45 business days after each discussion was held. This report followed the same basic structure of the initial report but was developed in greater detail from rigorous qualitative data coding. Appendix C synthesizes and summarizes these detailed reports; key findings derived from the results appear in the main report. Additionally, we presented midterm study findings to the PCORI Engagement Dissemination and Implementation Committee and at the PCORI annual research meeting.

Appendix B. Pre–Focus Group Survey Results

Prior to each focus group discussion, all participants were asked to complete a five-question online survey (Box A.2) that explored the participants’ familiarity with CER, how much they or their organizations have previously used CER, their expectations for the use of CER in the future, and their perception of the value of CER. The survey consisted of both closed- and open-ended questions and was administered online. This information was used to help gauge participants’ familiarity with discussion topics and help moderators prepare follow-up questions. In this appendix, we present results from this survey.

Description of Survey Participants and Their Experience with CER

Completion rates for the prediscussion survey were high, with 87 percent (n=65) of all participants completing the online survey. Of those who completed the survey, all but two individuals (both from the purchaser community focus groups) answered all four multiple-choice questions and the one open-ended question, and the remaining two individuals answered only the text-based open-ended question. Participants included individuals serving in a variety of positions within a range of organization types and sizes.

Purchasers

Purchaser focus group participants were more likely to come from the Midwest (34 percent) and Northeast (34 percent) than from the South (21 percent) or West (10 percent). The following participant roles and organization types were represented:

- *The business coalition focus group* included presidents, chief executive officers, managers, and executive directors of business coalitions and chambers of commerce. Participants from local, state, regional, and national organizations participated in the focus group.
- *The large employer focus group* included senior vice presidents, benefits managers, and program leaders from the retail, construction, technology, grocery, and utility industries.
- *The medium-sized employer focus group* consisted of human resources executives and represented organizations from the hospitality, knowledge services, legal, and services industries.
- *The small business focus group* included owners and leaders of businesses representing the retail, health, construction, and skilled trade industries.

Table B.1 summarizes their responses.

Despite the generally high rates of these types of responses among the large employer and business coalition groups, some individuals were less familiar with CER or supportive of its value. As one skeptical participant from the large employer focus group stated, “Comparative

Table B.1. Purchaser Focus Group Results: Prediscussion Survey (N=23)

Survey Question	Number of Responses		
	Very Important	Somewhat Important	Not Important
How important is patient-centered CER to your industry or sector?	10 (43%)	9 (39%)	2 (9%)
	Sponsoring the Research	Using CER in Our Work	Not Involved in Any CER
How have you or your organization used patient-centered CER?	0 (0%)	7 (30%)	16 (70%)
In the next 3 years, how do you or your organization expect to use CER?	0 (0%)	14 (61%)	9 (39%)
In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?	2 (9%)	15 (65%)	7 (30%)

NOTE: 23 out of 28 purchaser participants completed the survey. Participants could select more than one response to the questions “How have you or your organization used patient-centered CER?” “In the next 3 years, how do you or your organization expect to use CER?” and “In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?”

effectiveness research could be a powerful tool if done right and communicated properly. To date I have seen neither.”

Fewer small and medium-sized employers than large employers and business coalitions reported that they considered CER important for their sector or industry (four total participants from both groups) or foresaw participation in CER work in the near future. However, most were supportive of CER or identified potential uses for it in their work. As one participant stated, “any work that is done that can clarify, simplify, and assist employees who are also patients make informed health care decisions is a benefit to both the employee and the employer.” This sentiment was generally reflected across most survey responses for these groups, although two participants from the medium-sized employer focus group were not familiar with CER and therefore felt that they could not identify advantages or disadvantages with this type of research.

Payers

We were interested in how individuals from payer organizations make decisions about health care and the ways in which CER could contribute to that work. Participants were more likely to come from the West (36 percent) and Northeast (36 percent) than from the South (23 percent) or Midwest (5 percent). The following participant roles and organization types were represented:

- *The public payer focus group* included medical and program directors, research managers, and deputy commissioners from state Medicaid agencies, state-run health exchanges, CMS, and private health plans that contract with state Medicaid programs.
- *The private payer group* included senior vice presidents, research directors, chief medical officers, and program directors from regional and national health plans.

- *The integrated payer focus* group included chief executive officer, chief quality officers, vice presidents, program directors, and scientific directors from local, state, and regional payer organizations.

Table B.2 summarizes their responses. Purchaser familiarity with CER varied by organization size, with the business coalition and large employer focus groups reporting that CER is “very” or “somewhat important” for their industry or sector, and many participants providing detailed descriptions of the value of CER). Most survey participants from these two focus groups reported that CER brings value to individual patients, employer decisionmaking capacity, or the health system at large. As one business coalition survey participant stated, CER is “critical to informing precision medicine, important to underlie a cultural shift to patient centeredness throughout the care system.”

All 20 of the focus group participants who completed the prediscussion survey reported that CER is “very” or “somewhat important” to their industry or sector. The importance of CER to payers was further reflected with the high rate of participants (19 out of 20) who reported that they expect their organizations to use CER in the next three years. Although almost all the participants in each focus group indicated that “using CER in our work” is the primary way that they currently use or expect to engage with CER, about one-third of the participants in each focus group indicated that they would also be interested in “sponsoring the research.” This high level of interest in CER was further reflected in the participants’ descriptions of the value of CER and how they felt it could be used in their industry or by other stakeholder communities. For example, all the integrated payer prediscussion survey participants reported that CER is valuable for their organization’s decisionmaking, with two participants suggesting that CER could “improve the quality of care.” One participant reported that CER is “critical to decision

Table B.2. Payer Focus Group Results: Prediscussion Survey (N=20)

Survey Question	Number of Responses		
	Very Important	Somewhat Important	Not Important
How important is patient-centered CER to your industry or sector?	14 (70%)	6 (30%)	0 (0%)
	Sponsoring the Research	Using CER in Our Work	Not Involved in Any CER
How have you or your organization used patient-centered CER?	8 (40%)	16 (80%)	3 (15%)
In the next 3 years, how do you or your organization expect to use CER?	8 (40%)	19 (95%)	1 (5%)
In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?	10 (50%)	19 (95%)	0 (0%)

NOTE: 20 out of 22 payer participants completed the survey. Participants could select more than one response to the questions “How have you or your organization used patient-centered CER?” “In the next 3 years, how do you or your organization expect to use CER?” and “In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?”

making as we move towards a population health model of care delivery [and is] central to making financial investments for the organization.” Such views were echoed among the other two stakeholder subcommunities, with one participant from the public payer focus group stating that they “find CER valuable in helping inform policy decisions around plan benefit design as well as [benefit] approval and coordination processes.”

The views from the private payer participants were similarly supportive of CER and referred to it as “highly valuable” or “very valuable,” though one participant expressed a more tempered view. This participant stated, “With the proliferation of patient choices and rising costs, it is important to find out what matters to patients and answer the questions PCORI has formulated so they can make the best possible choices. The downside is a lot of new methodology and need for validation of these studies.” One of the public payer participants also suggested that the value of CER is “mixed,” though “well done CER targeting meaningful health outcomes can be very effective for policy development.”

Industry

Participants were more likely to come from the Northeast (54 percent) than from the West (19 percent), South (15 percent) or Midwest (12 percent). The following participant roles and organization types were represented:

- *The biopharmaceutical focus group* included vice presidents, managing and program directors, and department heads of small, medium-sized, and large biopharmaceutical companies; one life-sciences investment firm; and one biopharmaceutical consulting organization.
- *The device and diagnostics focus group* included vice presidents and program directors of device or diagnostic industry associations, device manufacturers, and one imaging organization.
- *The durable medical equipment employer focus group* included vice presidents and executive directors of state and national DME associations, home care associations, DME manufacturers, and one regional medical equipment supplier and home health provider.

Table B.3 summarizes their responses. Participants across the three industry focus groups viewed patient-centered CER as important to their industries or sectors, with 22 participants reporting that CER was “very” or “somewhat” important in the prediscussion survey (Table B.3). For example, one particularly enthusiastic participant referred to CER as “the new standard. Adopt or risk inadequacy.” A more-typical response from the biopharmaceutical and DME participants was reflected in one comment from a biopharmaceutical participant, who stated, “Patient-centered CER offers the potential to produce study results that are more likely to lead to the use of treatments that are acceptable to patients with respect to their benefit-risk profiles, compared to non-patient-centered-CER.” In general, the survey results largely reflect that participants from all the industry groups tended to frame the benefits of CER as primarily accruing to patients rather than industry.

Table B.3. Industry Focus Groups: Prediscussion Survey Results (N=23)

Survey Question	Number of Responses		
	Very Important	Somewhat Important	Not Important
How important is patient-centered CER to your industry or sector?	11 (48%)	11 (48%)	1 (4%)
	Sponsoring the Research	Using CER in Our Work	Not involved in Any CER
How have you or your organization used patient-centered CER?	7 (30%)	9 (39%)	11 (48%)
In the next 3 years, how do you or your organization expect to use CER?	13 (57%)	16 (70%)	2 (9%)
In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?	13 (57%)	19 (83%)	2 (9%)

NOTE: 23 out of 25 industry participants completed the survey. Participants could select more than one response to the questions “How have you or your organization used patient-centered CER?” “In the next 3 years, how do you or your organization expect to use CER?” and “In your opinion, how would other organizations like yours wish to use patient-centered CER, if at all?”

A few participants from the device and diagnostics industry were less concerned about the potential health outcomes of CER and more concerned about how CER could inform their stakeholders about its direct application to their business operations. One participant reported that CER is “increasingly important to reimbursement for diagnostics,” with another stating that CER is “increasingly valued [and] required by FDA [the Food and Drug Administration], payers, and clinicians.” In sum, participants from this subcommunity were interested in CER’s application to their ability to get health and medical products reimbursed by payers increasingly seeking CER to make payment decisions.

Appendix C. Focus Group Results by Topic

This appendix presents detailed results from our focus group discussions on the following topics:

- *health-related decisions, information needs, and CER*: the types of decisions that stakeholders make in the course of their work; the types and trusted sources of evidence they need and use to support health-related decisions, and their current familiarity with CER, the types of decisions that CER could support in the future; and their views on who benefits from CER
- *stakeholder engagement in CER*: how much they currently engage in health care research, the feasibility of their participation in future research efforts, and facilitators or barriers to such participation
- *PCORI's mission*: stakeholders' impressions of the language in PCORI's mission statement
- *PCORI research and funding portfolio*: their views on PCORI's current research portfolio and the extent to which these align with the topics and populations they are interested in.
- *real-world studies*: their views on large, pragmatic studies, including comments on two PCORI-funded examples of this research
- *PCORnet*: their familiarity with PCORnet and how it could be used to support their information needs
- *value of CER*: a summative exploration of the value of CER, given its advantages and disadvantages.

For each topic, we provide a brief introduction to the types of information that we sought to collect and then summarize the findings by stakeholder community.

Health-Related Decisions, Information Needs, and CER

Overview

A warm-up question for purchaser and payer focus groups asked participants to describe health-related decisions they make in their work and the type of evidence they use to support these decisions. Participants were then asked to describe the value of CER in supporting these decisions and to identify those who benefit most from CER. Industry focus groups began with a question on familiarity with CER and proceeded to discuss decisions CER could inform and who benefits from CER.

Employers reported supporting the health and well-being of their employees by offering health insurance and other benefits at an affordable cost. The most important health-related decision an employer makes is to select an overall benefit package. This decision includes the selection of wellness benefits and a health plan benefit. Information employers use to support

these decisions focuses on two dimensions: what their employees need and the design and coverage options that are available. For the first dimension, employers are able to examine their own employees' health for the previous year and the first half of the calendar year through employee needs assessments and surveys. For the second dimension, employers rely on a variety of information sources: benefits consultants, public and private online resources, medical professionals and societies, and information on what competitors are doing.

Payer organizations reported focusing on health plan coverage decisions; benefit design; and, in some cases, the challenges of managing a large system of care. In addition, integrated provider payers discussed decisions related to running a delivery system. Public payers discussed decisions related to coverage for vulnerable populations, working with a densely populated yet limited network of providers, and making eligibility determinations. Payer groups described a long list of information sources, and there was little overlap in this list from group to group.

Industry groups are most interested in information that can be used to inform their customers (e.g., payers, providers) or that may help inform trial design; to target patient populations for new product development; and to make decisions that would help to bring their products to market, including identifying product needs and meeting regulatory research requirements.

Although there is some overlap between communities in the types of research evidence used to support these decisions, industry groups reported conducting their own CER, comparative efficacy studies, and effectiveness studies, while other participants seldom mentioned CER as a primary source of information in their decisionmaking processes. Despite this, all stakeholder communities and subcommunities were quick to identify different information needs and decision points that CER could support, as well as challenges and facilitators to its implementation in their decisions.

Types of Decisions Made in the Course of Work

Purchasers

Participants in all four purchaser focus groups reported that their primary health-related decisions are associated with health plan and benefit design, including the review of employee health data, selection of benefit and health insurance plans, and the level of employer and employee contributions to the benefit package. Additionally, small, medium-sized, and large employers also reported making decisions about wellness programs, including their type, design, and incentive structure. Table C.1 summarizes these decision types.

The evidence used to support these decisions varied somewhat from discussion to discussion, although the small, medium-sized, and large employers all reported making ample use of benefit consultants to help them navigate different plan offerings and fee structures.

Table C.1. Types of Decisions Purchasers Make

Business Coalitions	Small Businesses	Medium-Sized Employers	Large Employers
<ul style="list-style-type: none"> • Selecting health insurance benefits • Selecting dental insurance benefits • Formulary design • Designing benefit participation incentives 	<ul style="list-style-type: none"> • Selecting health insurance benefits • Selecting dental insurance benefits • Designing or selecting wellness programs 	<ul style="list-style-type: none"> • Selecting health insurance benefits • Selecting dental insurance benefits • Designing or selecting wellness programs • Designing wellness incentive programs (e.g., free flu shots) 	<ul style="list-style-type: none"> • Selecting health insurance benefits • Selecting dental insurance benefits • Designing or selecting wellness programs • Designing wellness incentive programs (e.g., higher reimbursement for effective treatments) • Formulary design

NOTE: Areas of overlap among the purchaser focus groups are indicated in **bold**.

One participant in the small business group indicated that Internet searches are a prime strategy for obtaining information to support health care decisionmaking: “I’ll type into Google—if it’s a question of who is eligible for Medicaid, if I want to find out what the threshold is and how that changed—I just type it into Google and usually there’ll be a government website that just explains it.” This participant described using specific online resources, including the ACA, state Medicaid, and state or federal health insurance exchange websites. Online searches were not mentioned in the other groups. We probed this in the medium-sized employer discussion and learned that they go to their benefit counselors for this information; fear of Web-based scams kept Internet searches from being a regular tool for this group.

Employee surveys are a source of information for small and medium-sized employers, and large employers mentioned medical professionals and societies. Table C.2 summarizes the information sources participants identified.

Payers

All three payer subcommunity focus groups reported that coverage decisions (including plan coverage, formulary composition, and drug tier placement) and delivery system design (e.g., the selection of providers or creation of new programs targeted to treat certain conditions) are a central part of their work (Table C.3).

Table C.2. Types or Sources of Information Purchasers Use to Support Health Decisions

Small Businesses	Medium-Sized Employers	Large Employers
<ul style="list-style-type: none"> • Benefits counselor • Employee survey or consensus • Health insurance exchange website or call-in line • ACA Website • Internet searches 	<ul style="list-style-type: none"> • Benefits counselor • Participation in benefits surveys • Employee health needs assessment • Competitive analysis of peer organization benefit design 	<ul style="list-style-type: none"> • Benefits counselor • Professional societies • Pharmacy benefit managers • On-site medical professionals

NOTE: Areas of overlap among the purchaser focus groups are indicated in **bold**. Because the discussion protocol continued to evolve over the course of the data-collection process, we did not ask members of the business coalition focus group (which was the first to participate in the study) about the types or sources of information they use to support health decisions.

Table C.3. Types of Decisions Payers Make

Public Payers	Private Payers	Integrated Payers
<ul style="list-style-type: none"> • Health benefit coverage design • Formulary design • Coverage and reimbursement • Delivery method for covered services • Selection of care quality metrics • Participant eligibility thresholds • Enrollment requirements and thresholds 	<ul style="list-style-type: none"> • Health benefit coverage design • Formulary design • Selecting EHR software Selecting vendors for programs • Metrics to assess technology assessments 	<ul style="list-style-type: none"> • Health benefit coverage design • Formulary design • Selecting EHR software Delivery system process design • Quality improvement initiatives, programs, and incentives • Deployment of facilities or resources

NOTE: Areas of overlap among the payer focus groups are indicated in **bold**.

To support these kinds of decisions, participants reported making use of a diverse array of sources, including medical literature, independent research, and review of claims or registry data. One participant from the public payer focus group summarized:

[Payers] need information that shows actual health outcomes and demonstrates whether a procedure or a treatment, a device, or a drug is effective. And sometimes that means effective compared to something else and sometimes that's compared to some control or some sham or placebo to understand its base efficacy. That's the starting point. And then, ultimately, of course, we'd like to know [its] effectiveness in the real world.

Participants in all three payer groups described information on the cost of care as relevant to decisions about coverage and plan design. Public and private payers and integrated providers described value—how much is paid for a specific benefit—as the real concern because this piece of information that payers need to make decisions is often missing. One participant described this in terms of medication costs:

I guess we're looking at . . . the financial piece that's always been missing from some data, but that's not the only thing we look for. [We also look at] the value of the products. And I think that's . . . where we all need a lot of work: 'What is the value of medications, and what should they . . . cost?'

There were differences among the payer subcommunities in some other areas. For instance, participants in the private and integrated payer focus groups described their decisionmaking about how best to streamline services in their networks and delivery systems, respectively. For example, participants in the private payer group reported that research about the effectiveness of genetic testing, lab tests, medical necessity, and care appropriateness supports coverage decisions. Participants also reported making several system-related decisions that affect care delivery, such as selecting software for claim reporting and patient tracking to identify and target outreach to patients due for checkups or prescription refills, types and use of technology assessments to shift to or improve electronic medical record-keeping during clinic visits, and selecting vendors for plan-related programs. Integrated payers were similarly concerned about decisions about the care process, including those related to “workflow and efficiency.” One participant summarized:

One decision is deploying resources around the clinical processes. There's a whole series of decisions around setting goals for improvement and then measuring whether we had achieved those goals. There are decisions around where we deploy facilities, location of facilities. There are decisions around technologies—by technologies, I don't just mean new devices or pharmaceuticals, but very often it's around organizational structures or professional roles and locations. So there's a whole array of questions about how you operate a delivery system.

Public payers, however, have a different set of priorities and decisions because of the populations they serve and the highly regulated nature of their provider pools. For example, public payers described decisions related to providing health insurance coverage to vulnerable population groups (such as children, pregnant women, or the elderly) and establishing requirements that providers must meet to be eligible for payment. Also included are decisions on eligibility and enrollment thresholds, provider reimbursement and payment policies, and development of care quality metrics.

Almost none of the information sources one payer subcommunity reported were reported in any of the other payer subcommunities. The decisions of private payers are often grounded in the medical literature or expert reviews. Integrated payers also make use of these resources but cited administrative records more frequently as a central information source, and public payers use

medical literature as well but are more likely to work with outside groups (such as research vendors or universities) to collect information to support their decisionmaking processes. Because the sources of trusted information varied widely among these groups (Table C.4), it is possible that we did not completely exhaust this topic or that they really use different information sources. More work may be needed with payers to identify trusted sources of information.

Industry

We asked industry-specific questions on the types of evidence they use or create, particularly in product marketing and development. In addition, unlike purchasers and payers, industry groups are typically considered stakeholders rather than decisionmakers. As Table C.5 shows, the decisions the focus group participants discussed referred primarily to clinical research trials and the decisions relating to the development of evidence used to support regulatory approval and product reimbursement and to inform new product investment decisions.

As a result, the evidence these organizations develop typically focuses on payer decisions and, to a lesser extent, provider decisions for the device and diagnostics members. Industry focus group participants reported engaging in independent or collaborative research ventures, including demonstration research projects with hospitals or academic centers, multispecialty groups, and payers, and identified several different types of clinical studies that they use to support market reimbursement. Other research sources, such as studies or reports from traditional clinical research outlets, the federal government, or specialist societies, were other options that representatives from the device and diagnostics and biopharmaceuticals focus groups mentioned.

Table C.4. Types or Sources of Research Evidence Payers Use to Support Health Decisions

Public Payers	Private Payers	Integrated Payers
<ul style="list-style-type: none"> • Technical Expert Panels • Medical literature review • Research contractors • Participation in a med group • Conduct grant-funded projects • Hayes Directory • Professional societies • Local universities 	<ul style="list-style-type: none"> • Technical Expert Panels • Agency for Health Care Research and Quality (AHRQ) • PubMed • National Institute for Health and Care Excellence • Blue Cross and Blue Shield Specialty Pharmacy Network • Institute for Clinical and Economic Review • Meta-analyses 	<ul style="list-style-type: none"> • Federal government sources (AHRQ, Centers for Disease Control and Prevention) • EHRs • Claim Data • Prospective nonrandomized controlled trials • Public health data • Patient registry data • Market source data

NOTE: Areas of overlap among the payer focus groups are indicated in **bold**.

**Table C.5. Types or Sources of Research Evidence
Used by Industry to Support Health Decisions**

DME	Device and Diagnostics	Biopharmaceuticals
<ul style="list-style-type: none"> • Clinical Trials • Demonstration Projects with Hospitals • Self-Run CER 	<ul style="list-style-type: none"> • Prospective Randomized Controlled Trials • Postmarketing surveillance registries • Clinical Registries • Laboratory-Developed Tests • Federal Government Sources (AHRQ) • Advisory Panels • Physician Specialty Societies • Investigator Sponsored Research • Key Opinion Leaders (KOL) 	<ul style="list-style-type: none"> • Clinical Trials • EHRs • Claims Data • Research partnerships with health care providers • Patient registry data • HCV-TARGET Registry • Federal government sources (FDA)

NOTE: Areas of overlap among the industry focus groups are indicated in **bold**.

Familiarity with and Understanding of CER

We asked participants in all ten focus groups whether they had previously heard the terms *patient centered* and *comparative effectiveness research*. After taking an informal poll, we offered a definition of *patient-centered CER* and then asked whether participants could describe who may benefit from this kind of CER and how.

Purchasers

Roughly half of all participants (15 of the 28) in the four purchaser focus groups reported being familiar with CER. Of those who responded that they were familiar with CER or patient-centered outcomes research (PCOR), two participants from the small business focus group reported having heard about CER through the news media (newspapers and radio), while two participants from the medium-sized employer focus group reported hearing about it in their current or previous work in the health policy or research field. All seven participants in the large employer focus group reported familiarity with the term, although they did not discuss how they had become familiar with the term. Similarly, four participants in the business coalition group reported familiarity with CER.

Payers

All 22 of the participants who participated in the three payer focus groups reported having previous familiarity with CER or PCOR, although participants in both the private and integrated payer focus groups reported that CER has been going on for a long time but was previously

called by another name. One participant from the integrated payer focus group stated that this type of research used to be called “clinical effectiveness,” whereas another suggested that it used to be called “equivalency trials.” The participants did not explain where or when they had first become familiar with the terms CER or PCOR, though private and integrated payer groups suggested that they had first heard the terms several years ago.

Industry

Nearly all the 25 industry focus group participants were familiar or “somewhat familiar” with CER. Familiarity ranged from somewhat superficial in the DME group to more sophisticated in the biopharmaceutical group. Similarly, participants’ organizations also vary in their use of patient-centered CER: Three participants in the DME, three in the device and diagnostic, and eight in the biopharmaceutical groups reported using some form of CER in their work. For example, participants in the device and diagnostics group reported using clinical registries for postmarketing surveillance. While these studies are trying to make these registries “a little more real world than they might have been before” and include more patient-reported outcomes, they do not involve head-to-head comparisons with other products.

Decisions CER Can Support, and Who Benefits from CER

Focus group participants were asked about decisions that could be supported with CER findings and about stakeholders who benefit from CER.

Purchasers

Participants reported a few different ways to incorporate CER into their existing decisionmaking processes. Participants in all purchaser focus groups, with the exception of those participating in the small business group, suggested that CER could be used to further support health benefit decisions, including coverage decisions, program offerings, participation incentives, and formulary design. The business coalition and large employer focus group participants reported that CER could help contribute to improved health and health insurance literacy among their workforce, which could not only enable their employees to access health care services more efficiently, but also lead to improved health outcomes. Similarly, the large employer focus group participants had a rich discussion about the potential for CER to fill much-needed information gaps about new or specialized treatment options whose benefits are often ill-defined. Participants from this group identified in-vitro fertilization, three-dimensional mammography, and surgical centers of excellence as candidate treatment options that could benefit from a robust CER study. In contrast, small business participants argued that CER information is of limited direct use to employers. Participants in this focus group also reported that small businesses will have little direct use for CER because most of the research literature is too technical or population, condition, or drug specific to be of immediate use. However, the results could be of interest to benefit consultants who advise them on which health care plans to

choose and to insurance companies that decide “what treatments they will fund and which treatment they won’t fund.”

Participants across the groups agreed that patients and clinicians benefit from CER. As one small business participant explained, “the patient benefits because it’s a little clearer as to which treatments are going to be more effective or provide better treatment options. And, certainly, practitioners are going to benefit as well because it’s going to give them more information about what’s effective.” The medium-sized employer group identified two additional benefits to CER, including positive workforce productivity as a result of a healthier workforce engaged in its own health care, as well as the reduction of health care disparities associated with the inability to obtain culturally appropriate care.

The business coalition and large employer focus groups also identified challenges associated with using CER that largely revolved around the absence of cost information from PCORI’s funded CER work. As summarized by one participant from the business coalition business group, “something could be highly effective, but it might only be effective for a few people or . . . the cost might be prohibitive.” The importance of cost to businesses was further illustrated by a participant from the large employer group, who stated that, “in the end, employers want to provide a sustainable health care benefit for our employees, and the only way to do that is to keep costs under control.”

Payers

All the payer focus groups reported that CER could be used to support the decisions they make. These included decisions about benefit design, drug-specific coverage options, establishment of treatment guidelines for in-network providers, and dissemination of best practices in clinical care.

Public payers reported that CER could be used to help ground benefit decisions in data that suggest favorable health, access, or affordability outcomes for patients. Specifically, participants said that understanding how different benefit levels affect individuals could help support the design of value-based insurance products. CER was also mentioned as a potential source of information for the selection of care quality metrics (e.g., measures of vendor performance in patient experiences of care or health outcomes); developing payment policy that includes “more value-based efforts”; and using evidence-based information about the effectiveness of drugs, devices, or procedures to develop robust coverage policies. The need for this evidence was made clear by one participant who stated “we have standard [benefit] designs, . . . but we don’t really know if they’re working or not for getting people the right care at the right time.” Another participant expressed a similar opinion: “What we need right now in the CHIP [Children’s Health Insurance Program] world is research around what the optimal benefit limit is.”

Private payers suggested CER could support decisionmaking for stakeholders at every level of the health care system, particularly as it relates to the creation of useful and reliable information about health outcomes. One participant summed this up by saying,

The data that we got [in the past] was almost always sponsored from pharmaceuticals. . . . There was never a study that said their drug was any worse. . . . Up until extremely recently, there was no comparative data at all. So when you wanted to look at drug A or drug B or treatment A or treatment B, . . . you not only did not have any data, but it was really hard to talk to any other clinical physicians, prescribers, about the data.

Integrated payer and delivery system participants suggested that CER could be used in creating care recommendations (e.g., “which drugs to use for given conditions for different classes of patients”), informing service delivery model selection (e.g., “whether to deploy care management nurses into primary care”), or “outcomes-based cost comparison to determine preferred network options.”

Despite the general benefits of CER for the decisionmaking processes of payer organizations, all payer focus group participants agreed that excluding cost information or comparisons from CER is not consistent with the actual factors that affect stakeholders of all types. One participant’s opinion was echoed by four others:

PCORI’s limit of looking at cost is not patient-centered. . . . Preferences are often anchored not just on outcomes, but also on what they cost . . . We still aren’t addressing the hardest piece that patients care about most.

Other stakeholders in the health care system also find it important to understand the cost ramifications of different treatment options. One participant in the integrated payer group said that “it can be quite difficult to get payers, clinical, and operational leaders to understand why we can’t talk about cost [because it is] . . . really important to them.” Participants suggested that, for PCORI’s CER to be relevant to health care stakeholders generally and to payers specifically, cost information will need to be incorporated into the research findings and translated into actionable information that reflects the information needs of each stakeholder group.

Industry

Across each of the three industry focus groups, participants felt that CER could be used in multiple ways to inform the use of existing products. First, most participants acknowledged that CER could support health care policy and reimbursement decisions. For example, DME industry representatives felt that CER could aid the development of coverage and reimbursement policies at CMS and among state departments of health and demonstrate the value of DME services to the health system. Similarly, members of the device and diagnostics group shared that CER could determine the value of their diagnostic tests by answering “the question [about] how would the patient [would] have been treated with the test versus in the absence of the diagnostic tests, and what decision or treatment decision did it change.” The biopharmaceutical focus group participants reported conducting “economic and clinical effectiveness and safety evaluations [of drugs] using real-world evidence.”

Second, industry members reported that CER could support decisions to guide best practices for product use, determine appropriate care pathways, and identify subpopulations of patients for

whom one product or technology is more (or less) effective. As one biopharmaceutical representative put it, CER can help identify populations of patients “where a product works particularly well or doesn’t work” through subgroup analyses.

Despite these common themes associated with CER use, each industry subcommunity reported somewhat different decisions that CER could support. For example, in the biopharmaceutical field, CER could help to understand how “different classes of drugs . . . are utilized in the real world,” “determine why drugs aren’t being used optimally,” and focus on behavioral modifications that can improve real-world use and, potentially, health outcomes. In the device and diagnostics subcommunity, information about providers is needed because they decide which device or diagnostic test is used. Finally, at least five participants in the DME focus group mentioned the CMS Durable Medical Equipment, Prosthetics, Orthotics, and Supplies Competitive Bidding Program. Representatives of the DME subcommunity saw CER as a way to differentiate products on the basis of value and not on price alone, allowing DME products to move away from “commoditized markets.”

In general, CER studies are not typically used in product development or preapproval studies. However, CER becomes a priority for companies as they consider where to invest in new products, licensing, or company partnerships. For example, a biopharmaceutical representative reported that CER techniques could be used to determine new therapeutic areas to explore. A device and diagnostics participant echoed that statement: “I’m not sure I would say that we’re doing formal CER studies to evaluate potential new diagnostic products. But I would say we’re certainly applying a CER-style framework to thinking about the potential value of those new products.”

Moreover, CER “raises the bar for innovator pharmaceutical companies to have to make significant improvements in the next product that they try to put on the market.” Products that are not able to demonstrate the incremental value in the real world “are going to be short lived” and may affect the decision to invest in products based on the comparative effectiveness of existing products. Another DME participant agreed that CER could help their organizations develop and “maintain a market-driven product line.” Finally, one participant highlighted the importance of understanding how products in development compare with other products, to inform decisions to invest.

Participants across the three industry focus groups described patient-centered CER as increasingly important to their industry or sector. As one device and diagnostics participant summarized: “So, in our field of laboratory-developed tests, I would say the payers have lagged behind, say, the pharmaceutical industry, where they’ve been asking for this kind of information for a couple of decades.”

Stakeholder Involvement in Research

Current stakeholder engagement in research activities—especially as it relates to CER—varies by organization size, the company’s client or product focus, and available resources. Only a few representatives of large organizations or organizations that specialize in health research or product development reported leading or partnering in research activities; most participants indicated they do not take an “active role” in research of any kind. However, most participants indicated that participation in studies is feasible within certain time, preparation, and resource constraints, with the types and levels of involvement varying by subcommunity. For example, while some industry focus groups indicated that they could participate in research as investigators of the work, the purchaser focus groups were more interested in serving in an advisory role to help guide research questions or progress. Stakeholder engagement in research across all groups is largely dependent on specific facilitators and barriers that either meet the stakeholder community’s needs or address time and resource constraints that otherwise limit participation.

Types of Potential Research Involvement

Purchasers

Purchasers in this study suggested they most likely could participate as research advisors or consultants. Both small businesses and medium-sized employers suggested that they could share “anonymized data” with researchers. Participants in the business coalition focus group were alone in suggesting that employers could make employees available for studies or encourage participation in health-based research. Similarly, only the medium-sized employer focus group identified the dissemination of research findings to employees as a way of supporting research priorities. A participant in the large employer focus group suggested that employers could engage in a dialogue with researchers about a study’s design to ensure that the study’s future findings are both relevant and useful to the employer community. However, experiences within the large employer focus group were mixed, with some participants reporting that their lack of research expertise precluded them from participating as active partners in research, whereas another reported currently leading a research study. One participant summarized:

Depending on where the employer is today and how active they are currently trying to manage and make decisions themselves, that will determine if they’re really engaged and want to participate on a higher level. . . . I think there will be different appetites to participate.

Payers

Many participants felt that the experience of the payer community could lead to studies that provide information useful to stakeholders outside the traditional research community. Participating payers indicated a willingness to contribute to selecting research topics and designing studies. Public payers reported that they might also be interested in funding research,

serving as partners or members of the research team, and using or disseminating research findings. Private payers suggested that the insights and resources of payer organizations (e.g., claims data) could help a researcher to define “usual care” that could be used in CER or related research.

Many of these ideas were informed by experience engaging in research. Public payers reported funding research projects, suggesting research topics, being part of research teams led by academic researchers, and using and disseminating research findings. Some private payers said their organizations had an existing research arm, contributed data to independent research studies, or were currently participating in a CER collaborative. Similarly, integrated payers reported participation in research studies, with several participants reporting that their organizations have been involved in non-PCORI-funded CER. One participant reported: “we’ve been doing [CER] for a very, very long time. We just called it different things over time. Payer focus groups thus generally considered it feasible and likely that they would participate in future research activities.

Industry

There was broad agreement across all three industry focus groups that they could participate in identifying research topics and questions and could bring expertise to study design. For example, a participant from the device and diagnostics focus group reported that “it’s really about making sure that studies are done well, that they’re designed well and so forth up front.” Members of the biopharmaceutical and device and diagnostics focus groups shared that they will likely engage in “more research collaborations with payers, with employers, and also with organized providers” in the future.

These ideas were also informed by experience engaging in research. Participants of the biopharmaceutical and device and diagnostics focus groups had engaged with AHRQ on proposed funding areas and suggested research questions that deserve further exploration. Another biopharmaceutical representative reported experience funding research and working closely with different groups to review research proposals and improve study design. Others in both the biopharmaceutical and device and diagnostics groups participated in collaborative, multistakeholder research with clinical specialty organizations and other stakeholders and joined advisory boards and panels. At least three biopharmaceutical group participants and two device and diagnostics participants had reported they had prior stakeholder experience in PCORI-related activities.

Barriers and Facilitators to Potential Research Involvement

Purchasers

Misalignment of research and business interests was described as a significant barrier to participating in research activities. In this context, stakeholder involvement in research is a function of the availability of resources for efforts that are not directly related to their core

business interests. Several of the barriers to health care purchaser participation in CER are related to concerns about time commitment and alignment of research with business interests. All the focus group participants reported that they do not have time to engage in demanding research activities that offer no direct benefit to daily business operations. Both the business coalition and large employer focus groups also reported that studies often fail to translate research evidence into usable information that purchasers and patients can understand, further making research activities inaccessible. Small and medium-sized employers also focused on the importance of the timing of a study because certain times of year are particularly busy for some industries (such as the summer and holidays for small businesses and the end of the calendar year for medium-sized employers) and prohibit even the consideration of “extracurricular” activities.

Specific aspects of research were also cited as barriers to participation, with business coalitions and large businesses noting that employers’ general lack of research expertise makes them less likely to pursue or agree to these activities, while medium-sized and large employers expressed some concerns about the ethics and logistics of sharing sensitive employee information (such as protected health information) with researchers.

Despite these barriers, participating purchasers reported being willing to engage in CER, especially if it was conducted on a topic that is relevant to their business interest at least in some way. As one participant from the business coalition focus group stated, “[Employers] need to understand the value proposition and how it’s going to make it easy to provide benefits effectively to our employees.” Thus, a key facilitator to engaging purchasers in CER is the selection of a particular topic that will result in actionable recommendations that can improve employee health or reduce employer costs.

Several logistical issues were also identified as facilitators to purchaser participation in research. Both the small and medium-sized employer focus groups reported that advance notice of a study’s time line, clear guidance about the specific contribution of the purchaser to a study, and an organized and efficient planning and implementation process are critically important for ensuring their contribution. These logistics are important for employers with limited time to engage in activities outside the usual course of business, with one medium-sized participant indicating that he would not be able to spend more than “one to three hours a month toward something like this.” These groups also reported that financial incentives can make participation more attractive for small and medium-sized employers.

Payers

Participants in the payer focus groups were generally amenable to participating in research outside their current activities and cited several factors that would enable their participation. Public and integrated payers reported that they were more likely to engage in research activities if the project goals aligned with their internal needs or if the topic was relevant to their current work. Related to this, participants in both of these focus groups stressed that the results of the study needed to be actionable, have “near-term applicability,” and demonstrate an appreciable

impact on client services, patient care, or service delivery efficiency. The integrated payer focus group further emphasized that research organizations need to clearly articulate the benefit of participation to payer organizations, not only in terms of the goals and outcomes but also in terms of research outputs or deliverables. Specifically, participants requested research groups “not just [provide] the overall aggregate results of the study, but give [the participating payer organization] their own stuff back. Give them [results] compared to [a] benchmark.”

Public payer participants reported that they are more likely to be involved in CER activities if “the host entity is organized and helps keep me on track,” noting that payers are most concerned about providing services to their client bases and may not have the bandwidth to engage in research activities if the payer’s role is unclear, the time line is undefined or slips, or the onus of participation lies with the payer organization.

Barriers to research participation were generally few across the payer focus groups. The bureaucratic structure of some large public payer organizations could limit engagement “because the overall machinery moves kind of slow.” Additionally, the public payer focus group cited the need to overcome potential conflicts of interest before being willing to engage in outside research work. In contrast, integrated payers stated that the academic nature of most research was often a barrier to their participation because the research goals may be inapplicable to payer needs or may fail to provide information that payers find relevant to their decisionmaking.

Although none of the groups suggested there were specific times of the year or modes of research participation that were better or worse for their organization or industry, the private payer focus group recommended collocating research engagements with existing conferences. As reported by one participant, “piggybacking on other meetings . . . eliminates your time-of-the-year [barrier] because those are meetings that people have on their calendar year after year.” They also suggested that in-person meetings were preferable during the early phases of engagement, when people are getting to know each other, whereas virtual meetings (such as conference calls, video conferences, webinars, etc.) were appropriate for later study stages.

Industry

Broader involvement of industry stakeholders is likely if the research topic is relevant to the company’s portfolio; if resources are available; and if regulatory constraints can be mitigated. For example, one participant in the device and diagnostics group reported that, if the study topic is relevant to a company’s therapies, “if there is a lot of innovation possible,” and if company resources (e.g., funding and time) are available, they would like to be involved.

The DME group identified several participation barriers. For example, one barrier was the existing limited research opportunities in the field of DME. Few DME organizations have the financial resources to sponsor a CER study. As one participant explained, “I’m probably speaking for 95 percent of all the DME suppliers in this country. . . . As much as we would love to be more a part of it [CER] and be able to provide the evidence and being involved in research, . . . right now we probably would not find any money.” Other DME participants reported that

DME-sponsored studies would be different (e.g., less objective) than those sponsored by an independent third party and that DME organizations “traditionally haven’t been included in any type of research.”

Biopharmaceutical participants reported limitations in the types of research they can conduct and what studies they can disseminate. For example, participants reported they would have regulatory challenges were they to conduct a study of a population or for an indication not included in the FDA-approved package insert. Also, participants in the biopharmaceutical group described Section 114 of the Food and Drug Administration Act of 1997 (FDAMA 114) as a barrier to dissemination of some research results. FDAMA was “designed to allow companies to more readily disseminate health care economic information . . . to those who need it for formulary decision making” (Perfetto, 2015). However, specific requirements in the act regarding the type of audience, standards of evidence, and use of the product have posed several barriers to its use by industry.

PCORI Mission

We explored participant reactions to PCORI’s mission. We read the following statement:

PCORI’s mission is to help people make informed health care decisions and improve health care delivery and outcomes by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader health care community. (PCORI, 2014e)

After reading the mission aloud during the focus group discussion, the moderator asked participants to identify words or phrases that they considered important and to make note of any important topics or issues that were not captured in the mission. In every discussion, the statement was read aloud a second time at the request of one of the participants. Although most participants liked the mission statement, some participants expressed concerns because of its breadth and its perceived lack of emphasis on research translation and implementation.

Purchasers

Overall, purchaser participants expressed a positive view of PCORI’s mission statement. Participants from the small business focus group made special note of the patient focus of the mission, while the medium-sized employer group stated that several words in the mission stood out, including *improved delivery*, *evidence*, *outcomes* and *to help* people. The business coalition and large employer focus groups also reported that they liked the mission statement, although the former suggested that the mission was unfocused because of the high number of desired outcomes, and the latter referred to the mission as “a little daunting and unattainable” because of the large scope.

When asked whether any important concepts were missing from the mission statement, one participant from the small business focus group said,

I think the fact that this research is being done and talking to all of these . . . different participants in health care is really good, but what happens to the research? Who's looking at it? What decisions are actually being made? What changes are occurring?

A participant from the large employer focus group echoed this concept by arguing that it is important to articulate the need to incorporate CER findings into the “delivery system and the provider base” if PCORI seeks to support a high-quality health system that leads to improved health outcomes.

Payers

The PCORI mission statement appealed to all the payer focus group participants. All appreciated PCORI's focus on multiple stakeholder communities, especially the emphasis on patients and patient engagement. Other words in the mission appealed to different groups, with private and integrated payer groups expressing their approval of the focus on “informed decisionmaking” and private payers emphasizing “evidence-based information,” “improvements in health care delivery,” and “improvements in health outcomes.”

At the same time, the public and private payer groups expressed some hesitation about how the mission might be implemented. Public payer participants interpreted this implementation issue a little differently, with one participant stating, “I like [that PCORI's mission] is guided by everyone, but to what end . . . in what ways? . . . We, as payers, want to be helped, so do patients, so do clinicians, but we want to be helped in different ways.”

Public payers also reiterated the importance of cost information in translating research results into actionable information, and stated that

it's really hard to have a mission that doesn't involve some aspects of efficiency and effectiveness and cost around there. Because we all know that one of the big challenges of health care is the cost of health care. I understand why it's not out there. I understand the political ramifications of that. . . . But it's also the elephant in the room that nobody wants to talk about.

Industry

Within each of three industry focus groups, participants responded positively to PCORI's mission. Participants reported that PCORI's mission extends beyond clinical research to include patient preferences. In the biopharmaceutical and device and diagnostics focus groups, participants also reported that the mission focuses on the entire care delivery system rather than on any particular treatment modality. Other participants highlighted the niche that PCORI fills by asking questions that other stakeholders are unlikely to investigate. As one participant summarized:

[T]he mission [of PCORI] isn't research. The mission is . . . providing information to stakeholders with the goal of improving care and that that information be based on high-quality research. So I view PCORI's mission statement as intentionally establishing itself as being a source of information to

try and improve outcomes for patients. And that involves research, for sure, but it's definitely beyond that.

However, a few cautions were expressed. For example, one participant in the device and diagnostics group felt that it might be difficult for health services researchers who “do traditional research” to conduct studies that can help PCORI accomplish its mission. Another participant in the device and diagnostics group raised concerns that the mission could be construed as having a goal related to cost-containment, that the “perception may be out there” that we have to constrain spending.

PCORI Research and Funding Portfolio

To discuss the information priorities of stakeholder communities in greater detail, we asked participants to react to two bar charts (Figures C.1 and C.2) summarizing PCORI’s funding portfolio. Participants were then shown an additional figure (Figure C.3) describing the percentage of studies addressing PCORI priority populations and participants were again asked

Figure C.1. PCORI-Funded Projects by Disease or Condition (n=254)

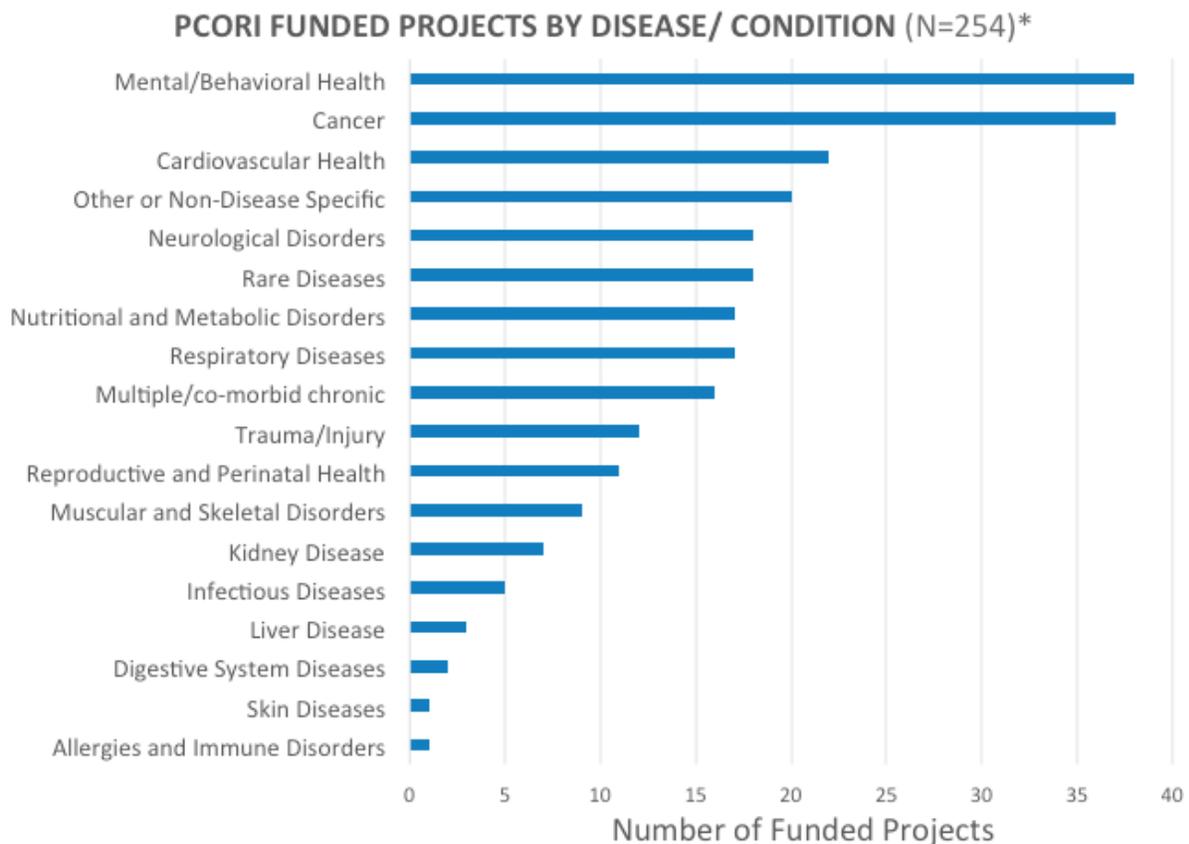
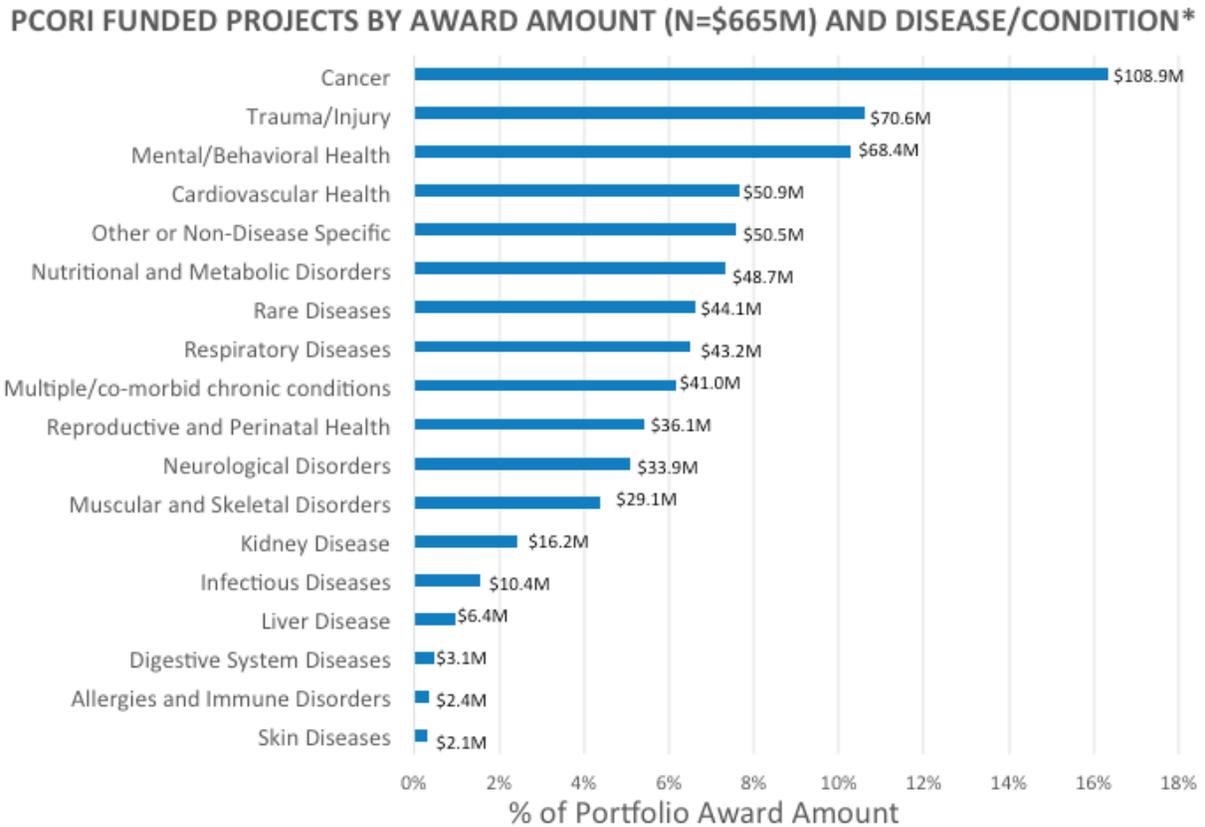


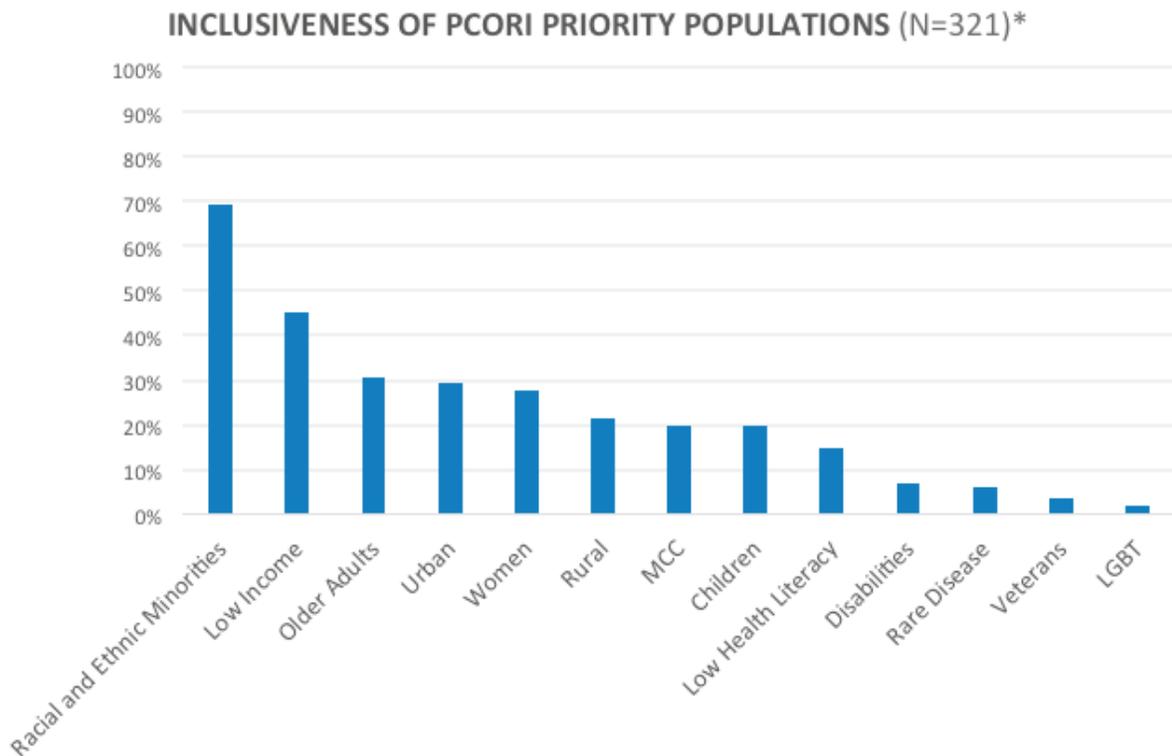
Figure C.2. PCORI-Funded Projects by Disease or Condition and Award Amount (total \$665 million)



to compare PCORI’s research priorities with their own. These graphs show PCORI’s research portfolio by the total funding allocated to specific diseases and conditions, by the number of funded projects associated with each disease or condition, and by the percentage of projects addressing a specific priority population.

Participants were asked to compare PCORI’s allocations with their own priorities and to identify diseases, conditions, and populations that may be missing from the portfolio. Holding high-cost and high-prevalence conditions as top priorities was a clear area of agreement, and all groups felt the list of priorities matched priorities for their community, although the employer stakeholder groups reported the importance of adding wellness and prevention interventions for people with or without specific clinical conditions and diseases. However, participants both within and across focus groups did not fully agree with each other on the specific ranking of priorities.

Figure C.3. Inclusiveness of PCORI Priority Populations (n=321)



NOTE: R/E Minorities: racial and ethnic minorities; MCC: multiple chronic conditions; LGBT: lesbian, gay, bisexual, and transgender.

Finally, two illustrative real-world studies were presented, and participants were asked to react to these examples. Real-world studies were described the following way:

Real-world studies seek to provide information that can be adopted by providers. They address critical clinical choices faced by patients, caregivers, clinicians, and systems, are often conducted in routine clinical settings, and are often large and less complex than traditional trials.

Real-world studies were well received; several groups agreed that the real-world study model is an important research development that can benefit stakeholders of all types. Most participants in all groups liked the large study samples and favored the notion of studying interventions among usual patients in usual care settings. Payers and purchasers agreed on the need to ensure community-specific translation of real-world study results, without which the findings would be unusable in many contexts.

Participants in some groups shared opinions that did not arise in other groups, such as a concern in the payer community that “usual care” is often poorly defined in such studies and a concern among employers that return to work after hospitalization is an outcome of interest they wished to see in one of the examples. Industry representatives sought additional information on the study designs and wanted to know more about how data were being captured in both studies.

Purchasers viewed dissemination of early results from these trials favorably, even while recognizing that study results could change. Payers were more cautious about early dissemination, expressing concern that the reputation of funders and researchers could be harmed from releasing results that are subject to revision.

PCORI's Current Funding Portfolio

Purchasers

All purchaser focus groups indicated that their priority topics are directly related to the cost and prevalence of a particular disease or condition in their employee population, which may or may not necessarily reflect the priorities in PCORI's research portfolio. As participants from the business coalition focus group summed up, the priorities for businesses are to find "which is the best drug to treat a certain kind of condition? What gets people back at work sooner?" and "how can health care transformation lead to . . . lower cost?"

The business coalition focus group identified nutritional and metabolic disorders (specifically, diabetes), musculoskeletal conditions, asthma, allergies, cardiovascular conditions, and mental health as the most prevalent and highest cost conditions coalition members encounter. This group also acknowledged that inflammatory diseases and obesity are high-cost drivers because of the prices of specialty drugs and the limited effectiveness of treatment programs and aging-related conditions (e.g., Alzheimer's, dementia) affect overall productivity models because employees often serve in caregiving roles that take them away from the workplace for hours or days at a time.

Similarly, participants in the large employer focus group agreed that PCORI's current funding portfolio reflects topics that are important to the employer community but felt that the order of the topics does not necessarily reflect their own priorities. In general, large employer participants reported that mental and behavioral health problems were not their number one priority, whereas musculoskeletal disorders (such as lower back pain), reproductive and perinatal health, and diabetes would have been higher on their list of priorities. The medium-sized employer group also identified diabetes as a priority condition but suggested that focusing on restoring patients' functional abilities and tracking longer-term outcomes after a traumatic event are also health-related issues that they frequently encounter in the course of their work. Only the small business focus group suggested that the top three funding priorities in PCORI's research portfolio reflected their needs or concerns, although this group suggested that research into evidence-based wellness programs is missing from the list.

Payers

There were notable differences among payer subcommunities in their senses of the alignment between their own health care spending and PCORI's research portfolio. The private and integrated payer focus groups indicated that PCORI's funding allocation is largely consistent

with their own spending. Several participants in the private payer focus group commented that cancer is one of their top priorities, with one participant summarizing that, “the big spending categories for a large, multistate, multiproduct plan are cardiovascular disease, cancer, orthopedics, and then the other categories down the line. . . . So I think that [PCORI’s] distribution of funding is fairly consistent.” The integrated payer focus group largely echoed this sentiment, although one participant felt that research into comorbid conditions should be much closer to the top of the priority list because of the high cost and high prevalence of complications resulting from comorbidity.

Public payers, however, responded that their areas of highest spending—musculoskeletal issues, pediatric conditions, comorbid conditions, and palliative care—were not the top conditions in PCORI’s portfolio. However, despite these differences, public payers acknowledged that PCORI’s research portfolio appears to reflect topic or condition priorities similar to NIH, and may reflect that established research communities apply for and get PCORI funding proportional roughly in proportion to their existing share of NIH funding awards.

Industry

All three industry focus groups viewed the portfolio of PCORI-funded projects favorably. Participants indicated that PCORI-funded projects were aligned with diseases and medical conditions that are “the high prevalence . . . high impact, high cost, and often variably treated” conditions. As one participant in the biopharmaceutical group explained,

[o]bviously, from a public health perspective, cancer and cardiovascular health are huge drivers of overall morbidity and mortality from a population level, [and] we just have a broken health care system to deal with mental health issues. So, personally, for the top three, I’m happy to see them.

PCORI-funded projects were seen to offer unique contributions. One participant in the biopharmaceutical group said, “having additional focused research that is looking at outcomes that really matter to patients, I think it’s a very good initiative overall.”

Other Topics of Interest

Purchasers

When asked to propose additional or alternative research topics for future study, the business coalition and medium-sized employer focus groups suggested studying the development of strategies to improve employee health in general terms rather than through research on specific diseases or conditions. Participants in the business coalition focus group recommended disease prevention and health promotion activities, which they argued could help employers remain competitive by keeping their workforces healthy and productive. This recommendation was echoed in the medium-sized and large employer focus groups, with the former suggesting that research around evidence-based wellness programs and motivations to change unhealthy behaviors (e.g., smoking cessation motivation research) would be most useful to them.

Participants in the large employer group suggested specific project ideas, including “which statin is the best for treating heart disease? . . . Which antihypertensive should I start with as first line therapy? . . . Should I do an MRI for back pain or should we jump right to surgery?” One participant concluded the discussion by stating “I mean, there are so many things that we could put on the list,” reflecting that employers are a rich and valuable source of potential research topics. Table C.6 summarizes purchaser focus group research priorities.

Payers

Private payers reported that they are largely concerned about the treatment of specific conditions and identified autism as an important condition that did not appear on PCORI’s list of priority topics. In addition to condition-specific studies, some purchaser subcommunities expressed an interest in condition-agnostic projects that instead focus on environmental, system-based, or other issues that affect health care delivery and quality. Specifically, the public payer focus group expressed an interest in projects that focus on care coordination, patient engagement tools, and communication of study results to patients. However, integrated payers reported a strong interest in social determinants of health and comorbidities that can contribute to or exacerbate other health issues and significantly affect patient quality of life and health outcomes. Table C.7 summarizes payer focus group research priorities.

Industry

Each of the three industry focus groups emphasized or recommended areas of research focus associated with care delivery in the real world. For example, one biopharmaceutical representative stressed the importance of supplementing clinical trial findings with information about how treatments perform in the real world rather than in clinical trials. Another participant representing the device and diagnostics group was interested in research on personalized

Table C.6. Topic Priorities of Purchaser Focus Groups

Business Coalitions	Small Businesses	Medium-sized Businesses	Large Businesses
<ul style="list-style-type: none"> • Cancer • Nutritional and metabolic disorders (diabetes) • Allergies • Cardiovascular issues, conditions, and treatments • Asthma • Disease prevention program • Health promotion activity 	<ul style="list-style-type: none"> • Cancer • Trauma and injury • Mental and behavioral health 	<ul style="list-style-type: none"> • Nutritional and Metabolic disorders (diabetes) • Allergies • Musculoskeletal issues (e.g., joint replacements) • Immune disorders 	<ul style="list-style-type: none"> • Nutritional and metabolic disorders (diabetes) • Musculoskeletal issues • Reproductive and Prenatal health

NOTE: Although the focus group participants tended to agree on the general topics of special interest to their subcommunity, focus groups were not asked to reach a consensus on the relative priority of these topic areas to one another.

Table C 7. Topic Priorities of Payer Focus Groups

Public Payers	Private Payers	Integrated Payers
<ul style="list-style-type: none"> • Comorbid conditions • Care coordination • Patient engagement tools • Patient education materials • Musculoskeletal issues • Pediatric conditions • Palliative care 	<ul style="list-style-type: none"> • Cancer • Cardiovascular disease • Orthopedics • Reproductive health • Autism 	<ul style="list-style-type: none"> • Comorbid conditions • Cancer • Mental and behavioral health • Social determinants of health • Aging-related conditions (Alzheimer's, dementia) • Patient safety

NOTE: Although the focus group participants tended to agree on the general topics of special interest to their subcommunity, focus groups were not asked to reach a consensus on the relative priority of these topic areas to one another. This table is therefore representative of the top three to five topics but does not reflect the topics' priority within a given subcommunity. Areas of overlap between the payer focus groups are indicated in **bold**.

approaches for care delivery. Within the DME group, members suggested research that would segment patient populations based upon sites or transitions of care (e.g., community-based care versus inpatient care versus outpatient care versus multiple sites of care). Finally, all three groups were interested in research focused on patients with multiple chronic conditions.

Diseases and conditions that correlated with the device and diagnostics and DME focus group company portfolios were of additional interest. For example, one device and diagnostics group participant was interested in funding for “other or non–disease specific” topics because these conditions represent opportunities for diagnostics. Similarly, a DME representative was interested in musculoskeletal disorders, wound care, rehabilitation and “cardiovascular health nutrition, metabolic diseases with tube feeding and TPN [total parenteral nutrition] . . . trauma and injury.” Table C.8 summarizes industry focus group research priorities.

PCORI Priority Populations

Purchasers

Although purchaser focus groups tended to acknowledge that identifying specific populations for research studies is important from a public health perspective, the groups were split about whether or not this information would be useful to them in the course of their work. The business coalition, medium-sized employer, and small business focus groups suggested that a better understanding of the unique needs of various subpopulations is useful. Participants in the business coalition and medium-sized employer groups reported that they were interested in several of the priority populations PCORI identified (racial and ethnic minorities, immigrants, women, LGBT populations, women, older adults, and children) because of their employee or coalition member makeup. The business coalition focus group also identified individuals with

Table C.8. Topic Priorities of Industry Focus Groups

DME	Device and diagnostics	Biopharmaceuticals
<ul style="list-style-type: none"> • Multiple chronic conditions • Cardiovascular issues, conditions, and treatments • Nutritional and metabolic disorders (tube feeding, total parenteral nutrition) • Trauma and injury (prosthetics, power rehabilitation) • Multiple comorbid chronic conditions • Muscular and skeletal disorders (prosthetics, orthotics) • Skin diseases (wound care: chronic wounds, diabetic foot ulcers, leg ulcers, burns) • General rehabilitation • Transitions of care 	<ul style="list-style-type: none"> • Multiple chronic conditions • Cardiovascular issues, conditions, and treatments • Cancer • Nutritional and metabolic disorders • Mental and behavioral health (eating disorders) • Trauma and injury (pain management) • Neurological disorders • Other, not disease specific • Skin diseases 	<ul style="list-style-type: none"> • Multiple chronic conditions • Cardiovascular issues, conditions, and treatments • Cancer • Mental and behavioral health • Nutritional and metabolic disorders (diabetes, obesity) • Real-world effectiveness

NOTE: Although the focus group participants tended to agree on the general topics of special interest to their subcommunity, focus groups were not asked to reach a consensus on the relative priority of these topic areas to one another. This table is therefore representative of the top three to five topics but does not reflect the topics' priority within a given subcommunity. Areas of overlap among the industry focus groups are indicated in **bold**.

low education levels as a particularly important population that is not in PCORI’s priority population group. As one participant explained, “people of lower education respond differently to some programs than those of higher (education),” which can affect the overall success of health or wellness programs that rely on a baseline level of health or health insurance literacy.

Small business focus group participants were also interested in PCORI priority populations, as long as they reflect a company’s workforce composition. However, these populations are largely irrelevant for decisionmaking because these businesses do not target particular groups when making health care purchasing decisions. The large employer group echoed this sentiment, with one participant stating that they “would never parse the data this way” because they “would not be able to do anything with it.”

Payers

Despite generally positive impressions about PCORI’s priority populations, payer subcommunities had different interpretations about which populations are most meaningful. Each subcommunity identified certain population groups as significant to their health-related decisionmaking:

- *public payers*: racial and ethnic minorities, individuals with disabilities, LGBT populations, non-English speakers, and individuals with low or variable incomes
- *private payers*: racial and ethnic minorities, individuals with multiple comorbid conditions, and individuals with behavioral comorbidity (e.g., depression in addition to diabetes)
- *integrated payers*: racial and ethnic minorities, individuals with disabilities, LGBT populations, individuals with multiple comorbid conditions, individuals with low or variable income.

In addition, integrated payers raised the topic of social determinants of health without explicitly being asked about it, stating that this topic is important for understanding factors that affect health outcomes. This group reported interest in populations with low literacy, low income, insecure housing, and insecure food supplies; some of the participants suggested that these populations are more useful to their organizations than some other populations PCORI prioritized. One participant suggested that more studies should prioritize populations with multiple comorbid conditions and disabilities; this would be “more immediately meaningful” to their current research and decisionmaking priorities.

Industry

The priority populations included in PCORI-funded projects also resonated with the industry focus groups. Participants recognized that these populations “align with public health priorities, [populations associated with] barriers to health care access, and include populations not typically enrolled in clinical trials.” As one participant explained,

I think most of the study examples I’ve seen, if not all, relate more to subpopulations’ specific uses, combinations of products with services, and are, in a way, helping to maybe more fine-tune the use of products within the health care system. So, to me this slide fits with that perception that PCORI is filling in the blanks that we can’t always [fill in] . . . filling in the questions that we can’t always answer in our major registration or even postmarketing medical affairs–type trials.

Despite the interest, participants in the industry groups shared that their organizations do not typically focus on these priority populations in industry-funded studies. For example, most research device and diagnostics companies conduct includes all patients and, therefore, does not selectively “try to include any of these populations, unless it is relevant to the disease” being studied. The exception is studies that seek to enroll a higher proportion of patients because of the study purpose (e.g., studies to support Medicare coverage will seek to enroll a higher proportion of Medicare beneficiaries, women, or minorities).

Real-World Studies

Focus group participants were presented with brief summaries of two real real-world studies PCORI currently funds and were asked to comment on their impressions of the value or usefulness of these studies to supporting the needs of their subcommunities (Appendix D). In

contrast to traditional clinical research, which is performed in highly controlled environments, real-world studies often take place in routine clinical settings and use existing data from a wide range of patients to draw comparisons and assess effectiveness. These studies seek to address the critical clinical choices patients, caregivers, clinicians, and systems face by considering treatment options within the context of the usual course of care. The first study, titled “Strategies for Active Surveillance of Patients with Small Pulmonary Nodules” (PCORI, 2015d), was designed to assess how often smokers and former smokers should undergo routine screenings to effectively detect signs of lung cancer growth without needlessly exposing patients to radiation. The second study, titled “Early Supported Discharge for Improving Functional Outcomes after a Stroke” (PCORI, 2015b), was designed to assess whether a comprehensive package of transitional care and in-home support services is more effective than usual care in improving stroke survivors’ functional abilities and preventing hospital readmission.

Purchasers

Participants in all four purchaser focus groups had positive opinions about the two example real-world studies, with one small business participant stating that these “are good studies that will ultimately improve outcomes and potentially reduce costs.” Additionally, participants emphasized that the primary benefits of these studies would be for the patients, rather than health care purchasers. As a small business representative explained, “they’re not relevant in terms of me as president of my company, but they are relevant to me as a patient working with my doctor to find the best solutions for one of these problems.”

Medium-sized and large employers also expressed a desire to see real-world studies that focus on keeping employees healthy and reducing cost, with medium-sized employers reiterating their interest in studies that focus on improving the functional abilities and productivity of employees returning to work after medical leave.

Representatives of business coalitions and large employers commented that interim results are generally good because they generate more information that could be used to support decisions “downstream.” Large employers suggested that they are very accustomed to making decisions that are not supported by strong evidence, with one participant explaining that “all the data that gets shared with us is so unripe and not statistically valid, and we make decisions all day long on it.” However, participants in this focus group acknowledged that protecting funder credibility is important and that the release of premature interim results could damage a funder’s status as a “trusted source” of information. Since large employers place a high value on the sources of their information, they recognized that interim results should be approached with caution.

Payers

All participants in the three payer focus groups felt that the real-world studies could theoretically support their payment or treatment guideline decisions, but they cautioned that

translation into payer-relevant findings, policies, or actions is important. As one participant from the integrated payer group put it, there is “a difference between having evidence that it might work, and then actually being able to make it work.” This sentiment was further explained by a participant from the public payer focus group, who reported that “it’s still really difficult from a payment perspective to figure out how to put [research findings] into a payment system that we know will work like those studies worked.” Similarly, payers also reported that understanding the cost-effectiveness of a particular treatment or procedure is important for understanding where “sensitive points within the process for deployment and implementation [are]” so that salient study findings can be integrated into care processes.

Participants in both the public and private payer focus groups reported that the lung nodule study aligns with some of their recent research priorities, with one participant from the public payer group stating that the study is “really relevant, particularly right now with CT scans recently being shown to be useful from a health outcomes point of view.” Although the private payer focus group participants echoed this view, some suggested that the overall usefulness of the study to private payers is subject to limitations, including the following: (1) The recommendations of the U.S. Preventive Services Task Force drive an organization’s coverage policies to a much greater degree than study results; and (2) there is skepticism that the results would lead to a widespread change in treatment, a decrease in incidence of lung cancer, or an increase in life expectancy.

Views about the stroke study were similar. Participants in all focus groups reported that the findings could help support coverage policy or design disease management programs, but limitations of the study could inhibit its usefulness to payers. Participants from the public payer group suggested that, because of its multiple components, the stroke study’s home-based intervention would require follow-on research to test replicability in other settings with different home-based interventions. Participants in the integrated payer focus group largely shared this view and suggested that the study should “really break out the changes in care compared to usual care into bite-sized pieces and identify those things that seem to be most effective” because doing so “would be more practical [and facilitate] quick implementation” by health plans. Another participant from this focus group also stated that it might be useful to provide “a bit more evidence of patient and caregiver involvement” to understand the impact of the study on functional ability and the ways in which the study results could be translated into a standard of care.

Participants in payer focus groups had different opinions about the release of interim research findings. On the one hand, integrated payers agreed that publishing interim results could be useful because payers cannot “always wait for the [final] results [before making decisions] . . . because it may take a long time to bring in enough volume [of data].” On the other hand, public and private payer subcommunities reported that, because interim results do not always align with the final results or present clear, significant findings, these results can further complicate the decisionmaking process. Instead, all groups suggested that releasing interim results should be

approached with caution and should firmly contextualize the findings within the current state of the study and its limitations.

Similarly, dissemination of interim results should be well controlled. One private payer participant suggested that findings be reported in peer-reviewed journals and professional societies to both frame the study's limitations and promote its visibility among the health care community. This would further support an advantage reported by a participant from the integrated payer focus group, who stated that interim results "have some value from the standpoint of helping people be aware of pending research" so that organizations can better plan their research pursuits and avoid duplication.

Industry

Industry participants had positive impressions of the real-world studies; considered them to be complementary, rather than duplicative, to studies other stakeholders are funding; and stated that they were "very consistent with the mission of PCORI." Key study elements reported in each focus group included the large sample size, the scale, the randomized design, the relevance of study outcomes to medical practice, and the ability to inform screening guidelines. Unlike the biopharmaceutical group, the device and diagnostics focus group and the DME focus group each discussed the study nuances and raised concerns about one of the real-world study designs. For example, participants with expertise in surveillance and imaging in the device and diagnostics group deliberated on whether there was a need for a large sample size in the nodule study; whether patients were needlessly exposed to radiation; whether the study was likely to enroll patients in a timely manner; and whether the study findings would be irrelevant, given that new imaging technologies (e.g., tomosynthesis) are becoming available. In contrast, the DME focus group discussed the data-collection procedures used in the stroke study. Participants reported that services delivered as part of a bundle (e.g., home devices, mobility aids, enteral nutrition services) are typically not captured adequately.

In general, industry focus group participants felt that the decision to share interim results should be based on the study characteristics. There was support for sharing interim findings if study results have a strong effect, are statistically significant, meet predetermined criteria, and are actionable. Less support was voiced for sharing interim results that are not actionable, if longer-term outcomes are needed, or if the study results may change. Participants in the DME and device and diagnostics focus groups explained their reticence for sharing interim finding because "it becomes more difficult to enroll people" and "there is the concern that "people are getting the idea, okay, problem solved. That's not an area we want to fund heavily for other projects."

PCORnet

PCORnet is a key PCORI initiative that was first implemented in March 2014 (PCORI, 2015e). PCORnet is an effort to build a health care data network that serves as a central node for

many different types of health care data, including EHRs, administrative records, and patient-reported health outcomes (PCORI, 2015f). Two different types of network partners are involved in the initiative: patient-powered research networks (PPRNs) and clinical data research networks. PPRNs are “networks operated by groups of patients and their partners who are focused on a particular condition or population,” while clinical data research networks are “networks that originate in health care systems such as hospitals, health plans, or practice-based networks” (PCORI, 2015e; PCORI, 2015f). Both of these networks securely collect health information and have expressed an interest in “sharing health information and participating in research” (PCORI, 2015e). PCORnet seeks to enable researchers to ask and answer questions about health care that would otherwise take months or years to study.

Given the role that PCORnet could play in the decisionmaking processes of stakeholder communities as well as and the potential contributions of these communities to the PCORnet infrastructure, we asked participants a series of questions designed to gauge their familiarity with PCORnet, how participants thought the network could be used, and the types of research questions that PCORnet could help answer. Although participant familiarity with PCORnet varied by community, participants provided several examples of ways that PCORnet could support their current work and explore topics of interest.

Purchasers

Of the 28 participants who participated in the purchaser focus groups, only one person (representative of a large employer) reported hearing of PCORnet prior to the discussion. Additionally, both the large employer and business coalition focus groups did not seem to understand what PCORnet was when the focus group moderator first introduced this concept, then engaged in additional discussion about the initiative’s goals, structure, and intended research products. Nonetheless, participants from the large employer group reported that they “liked the idea of a research-ready and more flexible . . . national network,” as well as the inclusion of patient-reported outcomes.

As each focus group came to better understand PCORnet, it identified decisions that its members make in the course of their work that could benefit from the type of research evidence that PCORnet is designed to create. There was little overlap in the types of decisions participants in different focus groups mentioned (see Table C.9).

Similarly, of the two focus groups asked to identify potential topics of interest that PCORnet could explore, there was no overlap in the proposed topics. The business coalition focus group proposed learning about how “various patients faired under various treatments”; where patients could receive high-quality, evidence-based care at a lower cost; and how patients with certain demographic characteristics that reflect a given population of employees reacted to a certain program or initiative. The medium-sized employer group indicated an interest in using PCORnet to compare “which treatment plan might be most effective for different types of patients”; to

Table C.9. Purchaser Focus Group: Potential PCORnet Study Topics

Business Coalitions	Small Businesses	Medium-sized Businesses	Large Businesses
<ul style="list-style-type: none"> • Quality measures to evaluate provider and health care facility performance to help drive the selection of health plan provider networks 	<ul style="list-style-type: none"> • Employee benefit design • Using incentives to encourage healthy behaviors • Smoking cessation program strategies • Identifying health care conditions and problems specific to a given industry 	<ul style="list-style-type: none"> • Patient treatment plans • Employee benefit design—better coverage and networks • Cost effective benefit design • Identifying the best network for a company’s employee mix 	<ul style="list-style-type: none"> • Quality measures to evaluate provider and health care facility performance to help drive the selection of health plan provider networks • Employee benefit design • Health plan formulary development

NOTE: Areas of overlap among purchaser focus groups are indicated in **bold**.

identify “better benefits, more cost-effective benefits, and things that work better for my workforce”; and to determine “the different networks that would best serve our employees.”

Despite participants’ initial unfamiliarity with PCORnet and the heterogeneous responses provided about the network’s potential uses, participants seemed to value PCORnet and the power of the network to respond to research queries. One participant from the small business focus group further praised the initiative for the massive data standardization effort that such a collaborative network structure necessitates. As this participant noted, standardizing health data that is currently stored on incompatible record systems “would make it very easy to move health data from one doctor to another . . . if you guys can start breaking down those barriers, I think that’s a really good thing.”

Payers

Familiarity with PCORnet varied significantly among payer subcommunities. Among public payer participants, only one participant reported being familiar with PCORnet. In fact, this participant communicated a detailed understanding of PCORnet, its research potential, and its structure, stating:

Patient-powered research networks are very cool because they’re organized by patients and usually [target] specific types of patient populations . . . to understand both the patient’s needs and the perspectives on the research [as well as] how we can use patient data that they’re collecting in their daily lives to do research in those populations.

Familiarity with PCORnet was uneven among participants in the integrated payer focus group. Only two participants reported that they had heard of the initiative before the discussion. However, one of these two participants reported being a member of one of the PCORnet networks. In contrast to the public and integrated payer focus groups, all the participants in the private payer focus group reported being familiar with PCORnet, although they reported that

they were unsure how this initiative is different from work performed under the U.S. Preventive Services Task Force; the FDA Mini-Sentinel pilot project; and data networks, such as the HMO Research Network.

After a brief introduction to PCORnet, focus group participants seemed excited to discuss the anticipated usefulness of PCORnet and the studies it could support (see Table C.10). All the focus groups suggested that PCORnet could be used to support coverage decisions, with integrated payers noting that PCORnet could be used to help determine “appropriate use criteria for surgical procedures around knee replacement and hip replacement” or the comparison of “different types of drugs, devices, surgical approaches.” Overall, the perceived potential of PCORnet was succinctly summarized by a participant from the private payer research focus group, who explained that the “development of this kind of infrastructure project, PCORnet, is useful to payers . . . because it will facilitate the conduct of research using some of the new sources—electronic health records.”

When asked about specific decisions or topics that PCORnet could help payers explore, payer subcommunities identified different topics. The public payer focus group suggested that PCORnet could help support coverage decisions, provide treatment recommendations, provide information about patient safety issues and outcomes, and offer comparative information about generic and branded products. In contrast, private payers focused on the research of specific conditions, such as hypertension, diabetes, multiple sclerosis, and rare diseases (e.g., hemophilia), although comparisons between multiple sclerosis treatments were also a priority topic for one participant. Participants in the integrated payer focus group acknowledged that there are “lots of applications for this model of study.” For example, the effectiveness of patient engagement approaches would be especially relevant to their current policy decisions.

To ensure that PCORnet is able to provide useful information to the payer community, focus group participants again reported that the translation of research evidence is important to ensure that findings are understood and implemented in payer care processes. The private payer focus

Table C.10. Payer Focus Group: Potential PCORnet Study Topics

Public Insurers	Private Insurers	Integrated Payers
<ul style="list-style-type: none"> • Health insurance coverage and benefit design • Treatment recommendations • Patient safety risks, protocols, and benefits • Comparison of branded and generic drugs 	<ul style="list-style-type: none"> • Health insurance coverage and benefit design • Decisions relating to population-health 	<ul style="list-style-type: none"> • Health insurance coverage and benefit design • Selection of patient activation programs • Appropriate use criteria for surgical procedures • Drug, device, and treatment approach comparison

NOTE: Areas of overlap among the payer focus groups are indicated in **bold**.

group suggested that PCORI should provide education materials that describe what PCORnet does, explain its value to stakeholders, provide instructions about how to use PCORnet, and detail how the results of PCORnet studies are disseminated. Such materials—and associated outreach—could help to engage payers in this type of research and incorporate their research priorities and interests in future PCORnet work.

Industry

There was mixed awareness about PCORnet among the industry groups, with 16 participants having some familiarity with PCORnet, eight with no familiarity, and one nonresponse. Overall, there was less awareness in the DME focus group than in other industry subcommunities. Among biopharmaceutical and device and diagnostics group participants who were familiar with PCORnet, two had participated in a workshop on PCORnet, and one was exploring partnerships with a single clinical data research network.

When described in greater detail, participants responded positively and viewed PCORnet as a useful and efficient resource for conducting research. Participants in each of the industry focus groups identified advantages and opportunities for using PCORnet as a data platform (see Table C.11). For example, participants were interested in and enthusiastic about the patient perspectives included in the PPRNs. In addition, members of the device and diagnostics focus group felt that PCORnet may be more attractive if linkages from administrative claims information were added to the electronic records. Despite the interest, questions and cautions remained. For example, one focus group member suggested “it ([PCORnet]) just looks like it’s not something that the pharmaceutical or the device companies might have access to. It sounds like it’s more of an independent and academic enterprise.” All three groups had questions regarding PCORnet governance and access, and two of the three groups were cautious regarding data quality and interoperability. Finally, others queried the differentiation of PCORnet from other data sources, such as FDA’s Mini-Sentinel project; the Framingham study; and data networks, such as the HMO Research Network.

In summary, there was interest in learning more about PCORnet, and after the focus groups, two participants followed up with the stakeholder research team to learn more about PCORnet. Similarly, the DME focus group members (and to a lesser degree other groups), felt there was a

Table C.11. Industry Focus Group: Potential PCORnet Study Topics

DME	Device and diagnostics	Biopharmaceutical
<ul style="list-style-type: none"> Assessing the impact of comprehensive home care coverage Linking information between DME devices and patient records 	<ul style="list-style-type: none"> Retrospective and prospective analyses Incorporating patient perspectives to accomplish goals that matter to patients 	<ul style="list-style-type: none"> Conducting trials in a more real-world manner, more effectively, and cost-effectively

NOTE: No topics were mentioned in more than one subcommunity.

need to raise awareness of PCORnet with their peers. The two national DME organization participants were interested in disseminating information to their members, and other focus group members suggested disseminating information about PCORnet on research list servers, at trade shows, or in specialty societies. After the focus group, two DME participants followed up with the stakeholder research team to learn more about PCORnet.

Value of CER

Overall, participants' perceptions of the value or advantages of CER did not significantly change between the completion of the screener survey and the conclusion of the focus group. CER was often described as being valuable to stakeholders across the health care continuum, although its particular use or format may vary by the needs of each stakeholder group.

Purchasers

All the purchaser focus groups identified stakeholders who could benefit and therefore derive value from CER. The benefits participants described varied in accordance with the stakeholder type but were largely consistent between groups: *Patients* could receive higher quality care and a larger role in their health care decisions; *the medical community* could make better informed decisions about effective treatment options for different subpopulations; and *employers* could achieve cost savings through their employees' use of high-quality, low-cost care. The overall value of these benefits—as described by participants in small, medium-sized, and large employer groups—is improved health outcomes and decreased costs for purchasers. Small and medium-sized businesses recognized the value of improved employee health on absenteeism and productivity, with one small business participant stating, “[I want] better quality of health care for my employees. That means my employees are going to have fewer sick days, and that’s important to me.”

However, both the large employer and business coalition focus groups reported that the overall value of CER to business interests is limited without corresponding cost information: Employers are purchasers of health insurance who are not only interested in which treatment options are most clinically effective but also want to know which options are most cost-effective. Similarly, the business coalition focus group suggested that CER’s value is largely inaccessible to the average patient or business associate because of the technical nature of most academic research. As one participant summarized, the primary challenge for achieving CER’s potential to benefit stakeholder groups “is being able to translate it for an audience that can quickly digest it and implement it.” Without the translation of research evidence into actionable policies or programs, employers will encounter significant challenges with capitalizing on the value of CER.

Payers

Overall, payers suggested that CER provides value to stakeholders at all levels of the health care system, starting with patients. As a participant from the public payer focus group put it:

[As a] physician or general internist, I would say that, when I see patients and I have clear comparative effectiveness of two treatments, it informs my shared decisionmaking with the patient where they can choose something that is better for them irrespective of the coverage decision that's been made by a payer. So I think that it does enter directly into the clinical situation.

By helping patients navigate decisions about their health care, participants from the private payer focus group believe that CER will ultimately “improve health care delivery and outcomes” and complement clinical trial research in a way that is meaningful to the patient community. In addition to these general benefits, participants from the integrated payer community suggested that access to patient-centered CER may soon become an expectation among the patient community. A participant from the integrated payer group suggested that, as patients assume responsibility for a larger portion of the cost of care, research is likely to be viewed by consumers as essential:

I think it's going to be an expectation, particularly of the millennials who . . . compile their expectations and their thirst for doing their own research right before they go in to see a health care provider . . . it means an empowered patient. So patients are having higher and higher deductibles and [are] responsible for a larger and larger percent of the cost-share, and there are expectations regarding outcomes and meaningful outcomes to them. . . . So [I] think this is an absolutely essential direction to head.

Payer subcommunity participants also suggested that CER provides value to providers and other health care entities because it uses real-world outcome-focused data to inform better health care decisions. Specifically, a participant from the private payer focus group suggested that providers would benefit because they “would feel more confident if they had that type of research and could advise certain things [for their patients].” The focus groups also identified other stakeholders, such as research funders, state and federal government agencies, public assistance programs, and taxpayers, as potential beneficiaries of CER, although the extent of the benefit varies. As one participant from the private payer focus group stated, “CER is great for both the plan and the member because you're not just making decisions based on cost, you're looking at the outcomes produced.” Participants from the public payer focus group were quick to suggest that there is a potential translational gap between the patient-centered information PCORI provides and the population-based information public payers use and need.

Industry

Within the industry focus groups, participants were able to articulate the value that patient-centered CER brings to the health care system. For example, one participant summarized the value of CER as “something that my mother could use, or that I could use, or loved ones or friends could use in trying to make decisions about their own health care.” Other participants reported that PCORI-funded studies stress outcomes “that actually make a difference to patients and clinicians.” The results from CER studies could “show what value each component provides

in the delivery system, in the treatment of the patient's disease, and what value that brings in the patient's care, as well as the outcomes that the patient receives.”

However, the limited use of patient-centered CER for coverage and reimbursement was reported in all three groups. Because it is seen as a positive that patient-centered CER is more expansive (because of the focus on quality, satisfaction, and patient perspectives) than coverage and reimbursement, the information gleaned from it may be less relevant from a coverage and reimbursement perspective. As one biopharmaceutical participant explained,

if you take comparative effectiveness research out of the context of PCORI and put comparative effectiveness research in the context of payers, I think it's a different discussion because I don't see a huge overlap between what payers are interested in getting out of comparative effectiveness research and what PCORI's interested in getting out of comparative effectiveness.

In the device and diagnostics group, another participant echoed this sentiment and reported that their company “is driven by the reimbursement requirements and the registration requirements,” which may result in diminished interest in substantial investments in patient-centered CER.

Focus group participants highlighted a limited number of potential disadvantages associated with patient-centered CER. Concerns were raised regarding the validity of studies, especially if the findings are “manipulated to present one side of the story that is not necessarily what the actual truth is,” if study results are quickly obsolete because of the availability of new products or technology, or if study results are not translated into practice or care delivery processes.

Despite these disadvantages, participants in all three industry focus groups expressed support for broader education on CER among their peers. As one participant in the device and diagnostics group noted, CER “is not yet well socialized among those who make decisions in health care, . . . [and] we still have a fair amount of missionary work to do to take discussions about patient-centered CER beyond PCORI-sponsored advisory discussions.”

Appendix D. PCORI Example Studies

The three studies that follow in this appendix are the study abstracts available online from the PCORI website (PCORI, 2015a; PCORI, 2015b; PCORI, 2015d). They are included here, as downloaded, to provide additional context for the conversation about PCORnet studies in the Key Themes and Implications sections and in Appendix C.

Pragmatic Trial of More Versus Less Intensive Strategies for Active Surveillance of Patients with Small Pulmonary Nodules

Background

Guidelines now recommend that smokers and former smokers undergo lung cancer screening, which can identify small growths. These pulmonary nodules are typically then monitored with serial CT scans that look for changes suggesting the nodules are cancerous. However, the optimal frequency of such scans has not been determined. The proposed research will compare more-intensive versus less-intensive protocols for CT surveillance.

Objectives

Among individuals with small pulmonary nodules that progress beyond the most curable stage of lung cancer, we will compare two protocols for CT surveillance, both of which are supported by existing guidelines from professional societies and are consistent with current standards of care. We consider patient-reported outcomes of emotional distress, anxiety, general health status, and satisfaction with the evaluation process; resource utilization and exposure to diagnostic radiation; adherence to the recommended protocols for surveillance; and adherence to use of low-radiation-dose techniques.

Methods

Using automated methods for identification, notification, and registration into the study, we will enroll eligible patients at each of 26 hospitals within 14 health care systems. We estimate that almost 47,000 patients will be passively enrolled over 20 months and followed for two years. We will perform analyses to determine which protocol works best for specific subgroups of patients.

Patient Outcomes

Lung cancer tumor stage T1a, the most curable stage of cancer; timeliness of lung cancer treatment; survival from lung cancer; emotional distress, anxiety, and general health status during surveillance; overall satisfaction with evaluation; number of tests and procedures

performed during the surveillance period; number of procedure-related complications during the surveillance period; adherence to recommended surveillance, for both patients and providers; and exposure to potentially harmful radiation.

Patient and Stakeholder Engagement

We have assembled a team of researchers, patients, clinicians, and stakeholders from health systems, advocacy groups, purchasers, and professional societies to help design and execute the study, and to help interpret and disseminate the results.

Anticipated Impact

Surveillance imaging and downstream invasive testing can be inconvenient, costly, and potentially harmful. By comparing two existing options for surveillance in the context of routine clinical practice, our trial will have a large and immediate impact on clinical care. By collaborating with stakeholders from health systems, professional societies, and advocacy groups, we will disseminate our findings widely and facilitate implementation in diverse practice settings.

Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-Term Effectiveness (ADAPTABLE)

Every year, 720,000 Americans have a heart attack, and nearly 380,000 die of coronary artery disease (CAD). Many of the patients who survive develop heart failure, stroke, or other cardiovascular complications. These conditions lead to substantial symptomatic, emotional, and functional difficulties for patients with CAD. Although novel therapies for treating CAD are needed, increasing the use of an inexpensive yet effective therapy, such as aspirin, will likely have a much greater effect on death and disability globally. To date, however, the best dose of aspirin (i.e., the one resulting in the lowest risk of ischemic and bleeding events) for the general population with ischemic heart disease has not been determined. Given the substantial burden of CAD and the continued growth of the population affected by it, defining the optimal aspirin dose will save thousands of lives globally.

The main objective of this pragmatic randomized clinical trial (PCT) is to identify the optimal dose of aspirin for secondary prevention in atherosclerotic cardiovascular disease (ASCVD). This trial will constitute the initial randomized comparative-effectiveness trial conducted by PCORnet: The National Patient-Centered Clinical Research Network.

This PCT will incorporate several essential aspects of the new genre of patient-centered comparative effectiveness trials:

1. By using existing data sources to gather baseline characteristics and a combination of existing data and patient-reported outcomes during follow-up, the PCT will answer its critical question at a relatively low cost;

2. An Internet portal will enable the PCT to collect and monitor data and allow for learning by both patients and clinicians, capitalizing on the frequent use of the Internet by the American public and clinicians;
3. The PCT will not have a placebo control but instead will provide all patients with active treatment at different doses, with careful monitoring to balance benefit and risk;
4. Patient-reported outcomes will be collected;
5. The evolving PCORnet infrastructure will be used to streamline administrative aspects of the trial, including centralization of Institutional Review Board functions and contracts, electronic consent, and use of EHR and claims data;
6. Mechanistic studies that include genetic testing and platelet physiology studies may be performed to improve our understanding of the variable responses of patients to specific doses of aspirin if additional funding is garnered for a substudy in a limited number of participants.

This PCT will allow us to identify the optimal dose of aspirin—one that will provide maximal benefit to patients with ASCVD, with low risk of bleeding. This PCT will save lives and reduce the burden of morbidity resulting from ASCVD globally.

Early Supported Discharge for Improving Functional Outcomes After Stroke

We are planning a randomized trial of 50 North Carolina hospitals, in partnership with the North Carolina Stroke Care Collaborative registry, to compare approaches to care for stroke patients. We are asking whether Comprehensive Post-Acute Stroke Services (COMPASS), which combines transitional care and early supported discharge for stroke patients who go home directly from the hospital, improves patients' daily function compared with usual care. We will also consider caregiver strain, hospital readmission rates, and mortality, use of health care, consistency of physician care, use of transitional care services, and death. We will also compare outcomes in some subgroups (race, sex, age, stroke severity, and insured versus uninsured).

Participating hospitals will be assigned randomly to receive COMPASS or usual care. Phase 1 compares COMPASS with usual care. In Phase 2, the usual-care hospitals will also receive COMPASS, while the other hospitals continue the intervention. In addition to COMPASS, which combines Medicare-approved transitional care services from advanced practice providers (4s; nurse practitioners or physician assistants) and early supported discharge services coordinated by the APPs, our intervention includes a community coordinator, who will work with local organizations to improve services for stroke survivors and their caregivers, and a stroke scorecard report, so hospital and primary care providers can see how they are doing in improving care for patients after a stroke. Together with the patient and caregiver, the APPs will develop an individualized care plan for each patient. Trained post-acute-care coordinators will help organize community groups to improve and continue care for recovering stroke patients.

We will assess 90-day and 1-year health outcomes. The primary outcome of our study is function as reported by the patients. Secondary outcomes at 90 days include caregiver stress, all-cause readmissions 30 and 90 days after discharge (assessed via insurance data), cognitive status,

taking medicines as needed, blood pressure control, depression, continuity of care, and use of community resources. One year after stroke, outcomes will include death, recurrent stroke, utilization of transitional care management billing codes, proportion of patients rehospitalized within 7 or 14 days after their first stroke hospitalization, physician follow-up, and use of health care.

Our Patient and Stakeholder Engagement Committee will work with our community coalitions to advise and support the implementation of COMPASS, provide feedback to the researchers, and recommend ways to continue COMPASS in the future. These coalitions will help us tell others about the COMPASS results and (if merited) how to begin similar programs across the United States to improve life for stroke survivors and their caregivers.

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