



March 2015

# COMPARATIVE EFFECTIVENESS

## Initial Assessment of the Patient-Centered Outcomes Research Institute

Accessible Version

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#### Why GAO Did This Study

In 2010, PPACA authorized the establishment of PCORI as a federally funded, nonprofit corporation to improve the quality and relevance of CER. PCORI, which began operation in 2010, is required to identify research priorities, establish a research project agenda, fund research consistent with its research agenda, and disseminate research results, among other things. To fund PCORI, PPACA established the Patient-Centered Outcomes Research Trust Fund, through which the institute is expected to receive an estimated \$3.5 billion from fiscal years 2010 through 2019.

PPACA mandated that GAO review PCORI's activities by 2015 and 2018. This report examines (1) the extent to which PCORI established priorities and processes for funding and disseminating comparative clinical effectiveness research consistent with its legislative requirements; (2) the status of PCORI's efforts to fund comparative clinical effectiveness research; and (3) PCORI's plans, if any, to evaluate the effectiveness of its work. GAO reviewed relevant legislative requirements and PCORI documentation, including funding data, and interviewed PCORI officials. GAO also interviewed relevant stakeholders, including health policy experts and PCORI contractors. PCORI provided technical comments, which GAO incorporated as appropriate.

#### What GAO Found

The Patient-Centered Outcomes Research Institute (PCORI) has established priorities and processes for funding comparative clinical effectiveness research (CER)—which is research that evaluates and compares health outcomes and the clinical effectiveness, risks, and benefits of two or more medical treatments, services, or items such as health care interventions—and is developing dissemination plans, consistent with the legislative requirements of the Patient Protection and Affordable Care Act (PPACA). In 2012, PCORI established five broad research priorities: (1) assessment of prevention, diagnosis, and treatment options; (2) improving health care systems; (3) researching communication and dissemination strategies; (4) comparing interventions to reduce health disparities; and (5) accelerating patient-centered outcomes research and methodological research. PCORI also developed a research agenda to identify how each priority would be addressed. PCORI has established a multi-step research funding process designed to assess and select contract applications for funding. Funded contracts are monitored by PCORI staff. Per legislative requirement, PCORI is developing a peer review assessment process to review final reports submitted by contract awardees and is in the process of developing a plan for the dissemination of funded research potentially beginning in 2015, in coordination with the Agency for Healthcare Research and Quality.

PCORI has started awarding contracts for research and plans to award additional contracts through 2019. As of October 2014, PCORI has awarded 360 contracts to fund research projects, committing a total of \$670.8 million to them. PCORI expects to commit about \$2.6 billion to research contracts, out of \$3.5 billion in total estimated spending. Approximately \$106 million in commitments to date are for PCORnet, a data research network aimed at improving the capacity for and speed of conducting CER. PCORI officials stated that they expect to spend a total of \$271 million on PCORnet through fiscal year 2019. PCORI officials stated that limited amounts of data will be available through PCORnet for researchers to use after September 2015 with the amount of available data increasing over time.

PCORI has established an evaluation plan and is developing efforts to measure outcomes. PCORI has developed initial plans for evaluating the institute's efforts against its three strategic goals, which are to increase information, speed implementation, and influence research. To do so, PCORI has developed primary outcome measures for assessing PCORI's progress related to these strategic goals. In its strategic plan, PCORI notes that these are meant to be long-term measures because research typically requires several years to complete and additional years for the results to be disseminated and implemented. Therefore, since 2013, PCORI has been using early and intermediate process and output measures—such as the number of people accessing or referencing PCORI information—as a way to monitor its progress toward its strategic goals. PCORI anticipates having some early results related to its primary outcome measures starting in 2017 after the first CER studies are completed and their findings released, although full evaluation of the results of these outcome measures will not be possible until around 2020.

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## Abbreviations

AHRQ	Agency for Healthcare Research and Quality
CDRN	clinical data research network
CER	comparative clinical effectiveness research
EHR	electronic health record
HHS	Department of Health and Human Services
IOM	Institute of Medicine
LOI	letter of intent
MMA	Medicare Prescription Drug, Improvement, and Modernization Act of 2003
NIH	National Institutes of Health
PCOR	patient-centered outcomes research

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PCORI	Patient-Centered Outcomes Research Institute
PCORTF	Patient-Centered Outcomes Research Trust Fund
PPACA	Patient Protection and Affordable Care Act
PPRN	patient-powered research network
Recovery Act	American Recovery and Reinvestment Act of 2009
RePORTER	Research Portfolio Online Reporting Tools Expenditures and Results

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U.S. GOVERNMENT ACCOUNTABILITY OFFICE

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# Letter

March 9, 2015

Congressional Committees

In 2010, the Patient Protection and Affordable Care Act (PPACA) authorized the establishment of the Patient-Centered Outcomes Research Institute (PCORI) as a federally funded, nonprofit corporation to improve the quality and relevance of comparative clinical effectiveness research (CER).<sup>1</sup> CER, research that evaluates and compares health outcomes and the clinical effectiveness, risks, and benefits of two or more medical treatments, services, or items such as health care interventions, is important because more than half of all medical treatments may be delivered without clear evidence of effectiveness, according to the Institute of Medicine (IOM). The results of CER can be used by patients and clinicians to make informed health care decisions about which treatment or intervention may be most effective or beneficial for a patient.<sup>2</sup>

PCORI, which began operation in 2010, is required to identify research priorities, establish a research project agenda, fund research consistent with its research agenda, and disseminate research results, among other things. PCORI seeks to make its research patient centered by incorporating patients and other stakeholders in the research process, and PCORI refers to the type of CER it conducts as patient-centered outcomes research (PCOR).<sup>3</sup> To fund PCORI, PPACA established the Patient-Centered Outcomes Research Trust Fund (PCORTF), through which the institute is expected to receive an estimated \$3.5 billion from

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<sup>1</sup>PPACA authorized PCORI by enacting a new section 1181 of the Social Security Act, Pub. L. No. 111-148, §§ 6301(a), 10602, 124 Stat. 119, 727-738, 1005 (codified at 42 U.S.C. § 1320e). In addition to CER conducted by PCORI, CER is also conducted by federal agencies including the Department of Veterans Affairs, the U.S. Department of Health and Human Services' (HHS) Agency for Healthcare Research and Quality, and HHS's National Institutes of Health.

<sup>2</sup>Institute of Medicine, *Initial National Priorities for Comparative Effectiveness Research*, (Washington, D.C.: June 2009).

<sup>3</sup>PCORI's definition of PCOR builds upon the definition for CER previously established by the Federal Coordination Council for Comparative Effectiveness Research. See Department of Health and Human Services, *Federal Coordinating Council for Comparative Effectiveness Research: Report to the President and the Congress* (Washington, D.C.: June 30, 2009). For the purposes of this report, we will refer to the research PCORI conducts as CER.

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fiscal years 2010 through 2019.<sup>4</sup> Under current law, PCORI may not make any expenditures from the PCORTF after September 30, 2019, at which point any remaining funds are to be transferred to the general fund of the United States Treasury.<sup>5</sup>

PPACA mandated that GAO review PCORI's activities in 2015.<sup>6</sup> This report examines (1) the extent to which PCORI established priorities and processes for funding and disseminating comparative clinical effectiveness research consistent with its legislative requirements; (2) the status of PCORI's efforts to fund comparative clinical effectiveness research; and (3) PCORI's plans, if any, to evaluate the effectiveness of its work.

To determine the extent to which PCORI established priorities and processes consistent with its legislative requirements, we reviewed relevant legislative requirements and collected written documentation related to PCORI's research priorities and procedures for funding research, as well as its plans to disseminate research results. We interviewed PCORI staff to learn how PCORI established its research priorities, what plans PCORI has for updating these priorities, what

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<sup>4</sup>PCORTF's appropriation for a given fiscal year depends on the specific sum appropriated by PPACA, amounts transferred from the Medicare trust funds, and, starting in fiscal year 2013, net revenues from fees on health insurance and self-insured plans. PPACA designated PCORTF funds for PCORI's use, except for a 20 percent transfer during fiscal years 2011 through 2019 to the Secretary of HHS to carry out CER-related activities enacted by PPACA pursuant to section 937 of the Public Health Service Act. See 26 U.S.C. § 9511(b), (d).

<sup>5</sup>26 U.S.C. § 9511(f).

<sup>6</sup>PPACA also mandated GAO to review HHS's CER-related activities in 2015 and 2018. GAO completed the first of these reviews in a separate report. (See GAO, *Comparative Effectiveness Research: HHS Needs to Strengthen Dissemination and Data-Capacity-Building Efforts* GAO-15-280, Washington, D.C.: March 3, 2015). In addition, GAO has completed annual reviews mandated by PPACA of PCORI's financial statement audits and also issued two previous reports on CER in response to Congressional requests. See GAO, *HHS Research Awards: Use of Recovery Act and Patient Protection and Affordable Care Act Funds for Comparative Effectiveness Research*, GAO-11-712R (Washington, D.C.: June 14, 2011); *Comparative Effectiveness: Agency for Healthcare Research and Quality's Process for Awarding Recovery Act Funds and Disseminating Results*, GAO-12-332 (Washington, D.C.: February 29, 2012); *Patient-Centered Outcomes Research Institute: Review of the Audit of Financial Statements for 2013 and 2012*, GAO-14-415R (Washington, D.C.: March 27, 2014); and *Patient-Centered Outcomes Research Institute: Review of the Audit of the Financial Statements for 2012 and 2011*, GAO-13-390R (Washington, D.C.: March 29, 2013).

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procedures PCORI employs for reviewing and selecting applications to fund, how PCORI monitors its contractors, and how PCORI plans to disseminate research results. We compared information gathered from documentation and interviews to PCORI's legislative requirements. In addition, we determined the extent to which PCORI coordinated the development of its research priorities and proposed or funded CER projects with the National Institutes of Health (NIH) to ensure that its projects were not unnecessarily duplicative of projects proposed or funded by NIH. We also interviewed staff from both PCORI and the Agency for Healthcare Research and Quality (AHRQ) to learn about each organization's efforts to coordinate dissemination activities and plans for disseminating PCORI-funded research in the future. Finally, we interviewed relevant stakeholders: two public policy organizations, a health insurance trade association selected because it has provided input to PCORI on its work, and two health policy experts selected based on their knowledge of comparative clinical effectiveness research.

To determine the status of PCORI's research projects, we collected and analyzed funding data for all projects PCORI has funded as of October 2014, which was the most current data available. We relied on data provided by PCORI; while we did not audit this reported data, we assessed its reliability by reviewing existing information, conducting quality control checks, and interviewing PCORI officials knowledgeable about the data, and determined that they were sufficiently reliable for our purposes. We also obtained written documents that outline PCORI's plans to fund future research, describe funded projects currently in progress, and describe how PCORI funding is distributed across different research areas. We interviewed PCORI officials to determine the status of funded projects and how PCORI makes decisions to fund different areas of research. Finally, we interviewed six PCORI contractors that are conducting work related to a data research network being established by PCORI.

To describe PCORI's plans to evaluate its work, we collected relevant documentation from PCORI on its evaluation plans, such as strategic planning documents and documents about its evaluation framework that contain evaluation metrics developed to date. We interviewed PCORI officials to learn about the institute's plans for evaluating the effectiveness of its work.

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We conducted this performance audit from April 2014 to March 2015 in accordance with generally accepted government auditing standards. Those standards require that we plan and perform the audit to obtain sufficient, appropriate evidence to provide a reasonable basis for our findings and conclusions based on our audit objectives. We believe that the evidence obtained provides a reasonable basis for our findings and conclusions based on our audit objectives.

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## Background

Since the enactment of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA), AHRQ, an agency within the Department of Health and Human Services (HHS), has been one of several federal agencies responsible for supporting and disseminating the results of CER.<sup>7</sup> The dissemination of CER refers to developing and distributing information derived from CER for target audiences, such as clinicians, consumers, or policymakers, in order to inform health care delivery or practice. This process involves translating research findings into terminology and materials that are appropriate for the target audience. Specifically, AHRQ has supported CER activities by awarding grants and contracts to research centers and academic organizations to carry out this work, which includes reviewing and synthesizing scientific evidence through research reviews; generating new scientific evidence and analytical tools in original research reports; compiling research findings; and communicating those findings to a variety of audiences. Under the American Recovery and Reinvestment Act of 2009 (Recovery Act), AHRQ received funding of \$474 million to support and disseminate the results of CER—\$300 million that was appropriated to AHRQ and \$174 million that was appropriated to the HHS Office of the Secretary and allocated to AHRQ. The Recovery Act also required the Secretary of HHS to enter into a contract with the IOM to produce a report that included recommendations on research questions that should receive national priority for study with CER funds made available under the act.<sup>8</sup> The

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<sup>7</sup>This mission was assigned to AHRQ by the MMA. Pub. L. No. 108-173, § 1013, 117 Stat. 2066, 2438-41 (2003)(codified at 42 U.S.C. § 299b-7).

<sup>8</sup>Pub. L. No. 111-5, 123 Stat. 115 at 177 (2009).

result of this work included a list of 100 research questions prioritized for CER.<sup>9</sup>

In 2010, PPACA authorized the establishment of PCORI as a nonprofit corporation aimed at advancing the quality and relevance of evidence through CER to help patients, clinicians, purchasers, and policy-makers in making informed health care decisions. The law requires PCORI to perform a number of duties related to CER. (See table 1.)

**Table 1: Patient-Centered Outcomes Research Institute (PCORI) Key Legislative Requirements Related to Comparative Clinical Effectiveness Research**

<b>Research priorities</b>	Identify national research priorities, taking into account factors that include disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence, practice variations, and health disparities, among other things.
	Establish and update a research project agenda to address the priorities identified by PCORI.
<b>Advisory panels</b>	Appoint permanent or ad hoc expert advisory panels, as determined appropriate, to assist in identifying research priorities and establishing the research project agenda or for any other purposes.
	Appoint expert advisory panels for clinical trials and rare diseases to assist in the design of the research study, among other things.
	Each expert advisory panel shall include representatives of practicing and research clinicians, patients, and experts in scientific and health services research, health services delivery, and evidence-based medicine who have experience in the relevant topic.
	Provide support and resources to help patient and consumer representatives participate effectively on PCORI's Board of Governors and expert advisory panels appointed by PCORI.
<b>Funding research</b>	Carry out research using systematic reviews and assessments of existing and future research; primary research, such as randomized clinical trials and observational studies; and other methodologies, as recommended by PCORI's methodology committee and adopted by PCORI.
	Enter into contracts for the management of funding and conduct of research.
	Research should take into account potential differences in the effectiveness of health care for various subpopulations (including women and racial and ethnic minorities).
	Research should be designed to take into account different characteristics of treatment modalities that may affect research outcomes.
<b>Peer review</b>	Provide a peer review process to review primary research to assess its integrity and its adherence to PCORI's methodological standards.
<b>Disseminating research</b>	Make research findings available to clinicians, patients, and the general public not later than 90 days after the conduct or receipt of research findings. <sup>a</sup>

Source: Patient Protection and Affordable Care Act of 2010. | GAO-15-301

<sup>a</sup>In addition to PCORI's dissemination responsibilities, the Patient Protection and Affordable Care Act requires the Agency for Healthcare Research and Quality, in consultation with the National Institutes

<sup>9</sup>Institute of Medicine (IOM), *Initial National Priorities for Comparative Effectiveness Research*. (Washington, D.C.: 2009.)

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of Health, to broadly disseminate findings from federally funded CER, including findings published by PCORI.

PCORI is governed by a 21-member Board of Governors<sup>10</sup> and employs 153 staff, as of September 2014, including directors for its different program areas as well as staff for engagement, communications, contract management, finance, human resources, and information technology. PCORI's 17-member methodology committee defines methodological standards for its research.<sup>11</sup>

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## PCORI Has Established Priorities and Processes for Funding Comparative Clinical Effectiveness Research and Is Developing Dissemination Plans, Consistent with Its Legislative Requirements

PCORI has developed five broad research priorities and developed a research agenda to identify how each priority will be addressed. The institute has established a multi-step merit review process to score and identify applications for funding and a process for monitoring contractors. PCORI is also developing a peer review process for primary research and a dissemination plan for completed research.

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<sup>10</sup>PCORI's Board of Governors is to include the Directors of AHRQ and NIH (or their designees), and 19 members representing statutorily specified groups appointed by the Comptroller General of the United States. See 42 U.S.C. § 1320e(f). For more information, see [www.pcori.org](http://www.pcori.org).

<sup>11</sup>PCORI's methodology committee makes recommendations to the Board of Governors regarding methodological standards for research. Like PCORI's Board of Governors, the Methodology Committee is to include the Directors of AHRQ and NIH (or their designees), and up to 15 members who are experts in their scientific field and are appointed by the Comptroller General of the United States. The current version of PCORI's methodological standards was adopted by PCORI's Board of Governors in November 2013. See <http://www.pcori.org/research-we-support/research-methodology-standards/>.

## PCORI Has Established Research Priorities and a Research Agenda

In 2012, PCORI established five broad research priorities: (1) assessment of prevention, diagnosis, and treatment options; (2) improving health care systems; (3) communication and dissemination research; (4) addressing disparities; and (5) accelerating patient-centered outcomes research and methodological research. PCORI also developed a research agenda to identify how each priority would be addressed. The research agenda contains a set of more specific research areas within each priority. PCORI expects its research agenda to be updated and refined over time based on more specific analyses of gaps in research. (See table 2.)

**Table 2: Patient-Centered Outcomes Research Institute Research Priorities and Related Research Agenda Topics**

Research priority	Related research agenda topics (examples) <sup>a</sup>
Assessment of prevention, diagnosis, and treatment options	Studies that compare effectiveness of strategies for prevention, treatment, screening, diagnosis, or surveillance that have not been adequately studied against alternative options, as well as studies that address gaps in research.
Improving health care systems	Research that compares alternative system-level approaches to supporting and improving patient access to care, receipt of appropriate care, personalized decision making, and self care. Research that compares coordination of care across services or settings, including for patients with chronic conditions.
Communication and dissemination research	Research that compares different communication, dissemination, health literacy, and implementation strategies to improve decision making and patient use of comparative clinical effectiveness research, among other things.
Addressing disparities	Research that compares interventions to reduce or eliminate disparities in health outcomes.
Accelerating patient-centered outcomes research and methodological research	Research that identifies optimal methods for engaging and empowering patients. Research that aims to improve the validity or efficiency of analytic methods for comparative clinical effectiveness research.

Source: Patient-Centered Outcomes Research Institute. | GAO-15-301

<sup>a</sup>For all research priorities, there is special interest in rare diseases and studies in which results may differ among patient groups based on patient characteristics.

PCORI issued its research priorities and agenda in May 2012, following a process that began in July 2011. In the fall of 2011, PCORI formed two workgroups within its Board of Governors—the National Priorities for Research Workgroup and the Research Agenda Workgroup. Along with PCORI staff and members of the Methodology Committee, these workgroups examined the processes and products of other recent priority- and agenda-setting efforts, including those from AHRQ and IOM. They also engaged with and received input from stakeholder groups through a number of public presentations and other modes of communication, such as press releases, focus groups, and feedback through social media.

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PCORI posted on its website its draft research priorities and agenda for public comment from January 23, 2012 to March 15, 2012. In response, PCORI received and analyzed a total of 474 formal comments, and made changes to the draft priorities and agenda based on these comments. A final version of PCORI's research priorities and agenda were adopted by the Board of Governors and made publicly available in May 2012.

PCORI established its research priorities and agenda consistent with PPACA requirements. Specifically, PPACA directed PCORI to establish priorities for research that take into account factors that include disease incidence, prevalence, and burden; gaps in evidence with regard to clinical outcomes; patient needs, outcomes, and preferences; and practice variations and health disparities in terms of delivery and outcomes of care, among other things. The act also directed PCORI to develop a research agenda for addressing these priorities. The act did not specify the content or form of the priorities or agenda. According to documentation on the process PCORI used to develop its research priorities and agenda, the workgroups reviewed and considered these requirements. In the process of developing the research agenda, the workgroups identified where, for example, certain agenda items addressed criteria such as gaps in knowledge, variation in care, and inclusiveness of different populations.

According to PCORI officials, PCORI intended its research priorities to be broad in scope so that they could encompass a broad range of topics in need of study, including different disease areas, conditions, or health care system issues, thus making PCORI's research agenda more flexible than if it identified specific topics. PCORI noted in its research priorities and agenda document that PCORI did not want to exclude any disease from being studied. Some stakeholders we interviewed expressed concern that PCORI's research priorities are too broad and lack specificity. While PCORI officials acknowledged that many of the institute's initial funding announcements were broad in that they did not identify specific topics, they noted that they are in the process of increasing the proportion of funding that goes to specific research topics. For example, PCORI will soon begin funding awards for large pragmatic clinical studies to evaluate patient-centered outcomes.<sup>12</sup> The funding announcement for this effort

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<sup>12</sup>Pragmatic studies are designed to evaluate the effectiveness of interventions in real-life routine practice conditions, the results of which can be generalized and applied in routine practice settings.

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indicates a number of specific research topics of interest to PCORI. PCORI officials noted that applications which are responsive to these specific research topics will be given priority for funding. The specific research topics identified in this funding announcement were developed using, among other things, stakeholder recommendations, IOM's CER Priorities, and input from PCORI's advisory panels.<sup>13</sup>

To identify more specific research questions and topics for use in funding announcements, PCORI utilizes advisory panels, as authorized by PPACA. PPACA directed PCORI to establish advisory panels for rare diseases and clinical trials, which PCORI established in November 2013, and also authorized PCORI to establish other advisory panels as needed. In addition to establishing the two advisory panels required by PPACA, PCORI has also established five additional advisory panels: (1) Assessment of Prevention, Diagnosis, and Treatment Options; (2) Improving Healthcare Systems; (3) Addressing Disparities; (4) Patient Engagement; and (5) Communication and Dissemination Research. Four of these advisory panels align with PCORI's research priorities. To appoint members to its advisory panels, PCORI solicits applications via its website. Advisory panel applicants are reviewed based on common criteria established by PCORI and against the needs of the specific panel for which a position is being filled. The PCORI Board of Governors makes the final selection of advisory panel members.<sup>14</sup>

PCORI's advisory panels assist in the prioritization of research topics. PCORI receives suggested research topics through a number of sources, including stakeholders, social media, and workshops, as well as from AHRQ, NIH, and professional and advocacy groups. As part of the prioritization process, PCORI's advisory panels evaluate and rank suggested research topics using the following criteria: patient-centeredness, potential condition impact, assessment of current options,

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<sup>13</sup>Examples of specific research topics in this funding announcement include treatment options for patients with autism spectrum disorder; biologic agents in the management of patients with Crohn's disease; multi-component interventions to reduce initiation of tobacco use and promote cessation of tobacco use among high-risk populations with known disparities; and particle beam therapy for patients with lung, breast, and prostate cancer.

<sup>14</sup>Members of the advisory panels are required to complete a conflict of interest statement and disclosure form that defines "conflict of interest" and provides guidance to applicants on what types of conflicts of interest should be disclosed (e.g., stock investments, employment in the health care field, etc.).

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likelihood of implementation in practice, and durability of information. Topics identified by advisory panels as being of higher priority proceed for further evaluation while the lower-priority topics enter a pool of topics that may be reconsidered at a later date. Recommendations from PCORI's advisory panels are taken into consideration by PCORI's staff and Board of Governors and are used to refine and prioritize specific research topics and inform the development of PCORI funding announcements. PCORI may also use recommendations from advisory panels to commission reviews of previous and current research on recommended high-priority topics.

According to PCORI, its processes for identifying research include mechanisms for avoiding unnecessary duplication and coordinating research efforts with NIH and AHRQ. For example, when establishing its broad research priorities, officials from both NIH and AHRQ participated in PCORI's workgroups to develop these priorities and the related research agenda. As PCORI has developed more specific research priorities for funding announcements, PCORI staff has consulted with relevant NIH staff on specific topics in an effort to obtain expertise and an understanding of what research is being funded on a particular topic. For example, PCORI officials reported and NIH confirmed that when developing a specific research topic related to cardiovascular disease, PCORI coordinated with staff at NIH's National Heart, Lung, and Blood Institute to ensure that PCORI's planned research in this area had not already been sufficiently addressed by prior research. NIH submitted to PCORI a list of potential topics it considered important but was not actively funding, according to PCORI officials. PCORI officials also stated that when a new topic is suggested to PCORI for inclusion in a funding announcement, technical briefs are prepared to document the existing work that has been completed on that topic. In addition, PCORI staff stated that they conduct searches in ClinicalTrials.gov, NIH's Research Portfolio Online Reporting Tools Expenditure and Results (RePORTER), and other research databases to determine if any similar research is in progress or has already been funded.<sup>15</sup> PCORI staff stated that any proposed research topic that is unnecessarily duplicative is eliminated.

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<sup>15</sup>RePORTER is an electronic database that provides the public with information on the expenditures and results of NIH-supported health research. ClinicalTrials.gov, which is maintained by NIH, is the world's largest registry and results database of publicly and privately supported clinical trials conducted around the world.

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## PCORI Has Established Processes for Funding Research and Monitoring Contractors

PPACA directs PCORI to enter into contracts to carry out its research priorities.<sup>16</sup> PCORI has established a multi-step research funding process, which includes merit review, that is designed to select high quality research that has the best potential to improve patient outcomes, according to PCORI officials. PCORI officials stated that their merit review process is modeled on the peer review processes established by AHRQ and NIH,<sup>17</sup> which are described in law.<sup>18</sup> Unlike AHRQ and NIH, however, PCORI's merit review process utilizes patients and stakeholders in the review and scoring of applications. Key steps in PCORI's research funding process include the development and posting of funding announcements, review and scoring of applications, and final approval by the Board of Governors. (See figure 1.)

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<sup>16</sup>Such research can include systematic reviews and assessments of existing and future research; primary research, such as randomized clinical trials and observational studies; and any other methodologies recommended by PCORI's methodology committee.

<sup>17</sup>PCORI refers to this process as "merit review." NIH and AHRQ use a similar process for determining the scientific merit of submitted applications, which those agencies refer to as peer review.

<sup>18</sup>See 42 U.S.C. § 299c-1; 42 C.F.R. pt. 67, subpt. B and 42 U.S.C. §§ 282(b)(9); 289a(a); 289a-1(a)(2).

**Figure 1: Patient-Centered Outcomes Research Institute (PCORI) Research Funding Process**

Source: GAO analysis of PCORI information. | GAO-15-301

<sup>a</sup>Step 1 only applies to funding announcements that address specific research topics.

Submitted applications are assessed by reviewers recruited by PCORI and selected based on expertise or knowledge in a particular subject area. Reviewers may be patients, other stakeholders, or scientific reviewers. Prior to reviewing applications, reviewers undergo web-based training.<sup>19</sup> Reviewers score applications using five standard criteria. (See table 3.)

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<sup>19</sup>Reviewers undergo an online two-hour merit review training course that provides information on PCORI funding announcements, the merit review process, and how to review applications. During training, PCORI also provides reviewers with written reference materials, including definitions of frequently used scientific terms, the merit review criteria, mock applications, and sample reviewer critiques. PCORI staff stated that reviewers undergo training each time they review applications, regardless of whether they have reviewed applications previously for PCORI, to ensure that any changes in the merit review process are conveyed to the reviewers.

**Table 3: Patient-Centered Outcomes Research Institute (PCORI) Merit Review Criteria for Submitted Applications for Funding**

<b>Merit review criteria</b>	<b>Description</b>
<b>1. Impact of the condition on the health of individuals and populations</b>	<ul style="list-style-type: none"> <li>• Is the condition or disease associated with a significant burden?</li> <li>• Is there an emphasis on patients with one or more chronic conditions?</li> </ul>
<b>2. Potential for the study to improve health care and outcomes</b>	<ul style="list-style-type: none"> <li>• Does the question address a critical gap in current knowledge?</li> <li>• Has the question been identified as important by patient, caregiver, or clinician groups?</li> <li>• Do wide variations in practice patterns suggest clinical uncertainty?</li> <li>• Is the research novel or innovative in ways likely to improve care?</li> <li>• Is there potential for a sizeable benefit relative to current practice?</li> <li>• Could positive findings be disseminated quickly and affect changes?</li> </ul>
<b>3. Technical merit</b>	<ul style="list-style-type: none"> <li>• A clear research plan with rigorous methods and key milestones</li> <li>• A team with necessary expertise; appropriate organizational structure</li> <li>• An environment sufficient to support the conduct of the work</li> <li>• A diverse study population as appropriate for research</li> <li>• A focus on a defined population for whom effectiveness information is particularly needed</li> </ul>
<b>4. Patient-centeredness</b>	<ul style="list-style-type: none"> <li>• Is the focus on outcomes of specific interest to patients and caregivers?</li> <li>• Does the application address PCORI's definition of patient-centered outcomes research?</li> <li>• How credible are claims that patients will exert meaningful influence on the design and conduct of the research?</li> </ul>
<b>5. Patient and stakeholder engagement</b>	<ul style="list-style-type: none"> <li>• Does the application describe how patients and stakeholders will be identified?</li> <li>• Does the application describe how patients and stakeholders will be engaged?</li> <li>• Are the roles of patients and key stakeholders significant?</li> <li>• Are patient and stakeholder dissemination roles meaningful / effective?</li> <li>• Are patient and stakeholder implementation plans meaningful / effective?</li> <li>• If engagement is not applicable, does the application justify why not?</li> </ul>

Source: Patient Centered Outcomes Research Institute. | GAO-15-301

Applications are scored by reviewers through an online review, and a subset is scored by the full panel during an in-person meeting. For the online review, four reviewers are assigned to evaluate each application—two scientists and two stakeholders (one of them a patient). Scientific reviewers focus on all 5 criteria, while the patient and stakeholder reviewers focus on the potential of the study to improve health care and outcomes, the extent to which the research is patient-centered, and the extent to which the proposed research includes patient and stakeholder engagement. Reviewers assign an overall score to the application and

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provide written comments on each application's specific strengths and weaknesses. Scientific reviewers also check to determine if the application adheres to PCORI's methodological standards.<sup>20</sup> PCORI staff is also involved, working with reviewers to ensure that each reviewer understands how the applications should be assessed. Reviewers have one month to electronically submit both their initial scores and detailed written critiques to PCORI.

Following the online review, applications advance to in-person merit review meetings where they are reviewed again in panels specific to the funding announcement. To determine which applications will advance, PCORI staff considers reviewers' average overall scores for each application, whether applications have scores that differ significantly among reviewers (and could thus benefit from further discussion), and whether applications received a good score for technical merit criteria and are therefore a strong candidate for funding. Reviewers discuss the applications' merits and weaknesses and, as a panel, provide a final overall application score. After applications are scored at the in-person meeting, PCORI staff determine the applications that will be submitted for review to a PCORI selection committee, composed of PCORI Board of Governors members and up to one member from PCORI's methodology committee. The selection committee proposes a list of applications to fund. According to PCORI staff, while the proposed applications generally consist of the top-scoring applications, some lower-scoring applications may be included to achieve program balance or fund research in critical areas. The full Board of Governors votes to approve the recommended applications. While the Board has the authority to make changes, PCORI staff stated that it has not made such changes to date. Once the recommended applications are approved, awards are announced to the public via PCORI's website, PCORI develops final contract requirements, and research contracts are executed. PCORI officials stated that each cycle of the research funding process takes 9 to 12 months to complete, from the time a funding announcement is posted to the time recommended applications are approved by the Board, of which the merit review process takes about 4 to 6 months. There are currently up to four

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<sup>20</sup>Applicants are required to identify, among other things, how their research will adhere to PCORI's methodology standards. PCORI's methodology standards are outlined in PCORI's Methodology Committee Report. These standards, which were developed by PCORI's methodology committee, outline requirements for conducting patient-centered outcomes research, which PCORI defines as including CER.

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funding cycles each year and PCORI staff noted that these cycles overlap.

PCORI officials reported taking steps during the merit review and application selection process to ensure PCORI's funded research is not duplicative of other research within the federal government or private sector. For example, PCORI officials reported seeking input from NIH and AHRQ during the final selection of awards through each agency's involvement in the selection committee and membership on PCORI's Board of Governors. PCORI officials also noted that in some instances NIH and AHRQ staff assist PCORI in reviewing letters of intent submitted in response to PCORI funding announcements. Finally, before funding an application, PCORI program staff check databases that include ClinicalTrials.gov and NIH's RePORTER to identify any ongoing studies on a particular topic that may be duplicative.

Once funded, PCORI contractors are monitored by PCORI staff. According to PCORI staff, following an initial kickoff call with the contractor, PCORI receives a progress report from each contractor every 6 months, and there are additional interactions via phone and through regularly scheduled meetings. Progress reports include updates on key project milestones, a progress statement for public use, a financial status update, and additional documents the contractor deems relevant to the project's progress during the reporting period. PCORI officials stated that if concerns arise regarding a contractor's performance, a site visit to the contractor could be conducted. All contractors are required to submit a final report covering their research at the conclusion of the project.

The law also requires PCORI to develop a peer review process to assess the integrity of primary research funded by PCORI and its adherence to PCORI's methodological standards. PCORI officials stated that they are currently developing a peer review assessment process, as required by law, to review final reports submitted by contractors at the conclusion of a project. A draft of this peer review process was posted on PCORI's website for public comment in September 2014.<sup>21</sup> The draft process requires contractors to submit a draft final report to PCORI within three

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<sup>21</sup>According to PCORI, the updated draft, with an accompanying analysis of the comments and PCORI's responses to them, will be submitted to PCORI's Board of Governors to consider for approval in February 2015, and could be implemented for completed research projects as early as the spring of 2015.

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months of the project's completion for review by peer reviewers, who will include researchers from outside of PCORI. These reviewers will consider whether the research presented in each final report has scientific integrity and adheres to PCORI's methodological standards. Following receipt of the draft final report, the peer reviewers may identify revisions, which the contractor is required to respond to within 45 days. After revisions are made and PCORI formally accepts the final report, its draft process states that the institute will create and post on its website a lay abstract intended for the general public, a medical abstract intended for researchers and clinicians, a stand-alone table that presents key findings, and ancillary information such as the identity of the contractor. The contractor also will be required to ensure that the study results are submitted to ClinicalTrials.gov and to include with that submission links to the abstracts posted on the PCORI website.

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**PCORI Is Developing Dissemination Plans and Coordinating Dissemination Activities with AHRQ**

PCORI is currently developing a plan for the dissemination of the research it funds, as required by PPACA, and expects to begin disseminating research results as early as 2015. Specifically, PCORI has entered into a contract for the development of a dissemination and implementation plan. A draft framework for this plan was provided by the contractor to PCORI for comment in July 2014 and PCORI anticipates that the contractor will submit the revised framework to PCORI in February 2015. The draft framework identifies five key elements as core components of a dissemination and implementation plan. (See table 4.)

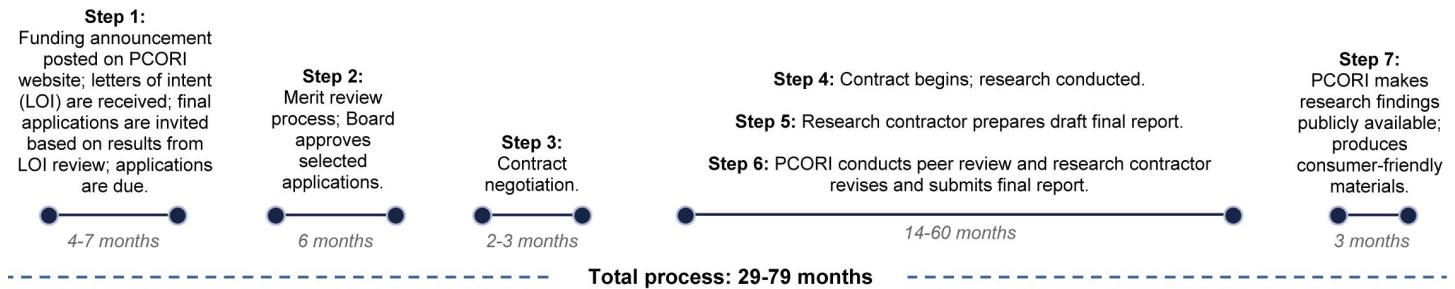
**Table 4: Patient-Centered Outcomes Research Institute Draft Dissemination Framework Components**

<b>Core component</b>	<b>Description</b>
<b>Evidence and context assessment</b>	Evaluate the quality of the individual study, determining the extent to which study results are supported by other existing evidence. Consider the research and environmental contexts within which dissemination and implementation will occur.
<b>Audience identification</b>	Identify the audiences and partners with the potential to adopt evidence or influence adoption. Determine prior to dissemination the audience(s) that will be affected by the evidence. Identify needs, expectations, and values of audience(s).
<b>Reaching the audience (dissemination)</b>	Define goals for adoption. Use the needs, expectations, and values of audience(s) to identify why evidence should be adopted. Develop dissemination approaches tailored to target audience(s). Communicate the essential elements. Utilize partners who can lend credibility and provide access to target audiences and serve as partners in implementation.
<b>Putting evidence into practice (implementation)</b>	Adapt evidence to suit the needs and characteristics of target audiences. Work with end users to define a strategy for use and speed adoption of evidence. Test the evidence of intervention to increase likelihood of success. Use multi-faceted strategies to facilitate successful adoption of evidence.
<b>Evaluation</b>	Using mixed methods and multiple sources of data (qualitative and quantitative), determine whether goals for adoption have been met and, if so, how that occurred.

Source: Patient Centered Outcomes Research Institute. | GAO-15-301

According to PCORI, its dissemination efforts will result in research findings being publicly available within 90 days of receiving final reports from researchers, as required by law. PCORI has determined that this 90-day period will follow the completion of PCORI's peer review process for completed research. Upon accepting the final report on the research conducted by the contractor, PCORI's 90-day period will begin, during which time PCORI will develop abstracts and other materials to post publicly on its website. At the conclusion of this 90-day period, PCORI anticipates posting abstracts, a results table that contains key findings, and ancillary information, such as investigators and conflict of interest information, on its website. The contractor will also be required to ensure that the results tables are submitted to ClinicalTrials.gov, which will link to the abstract posted on PCORI's website. According to PCORI, these dissemination efforts will take place at the end of the 29 to 79 month period for funding, conducting, and disseminating research, depending on the length of the contract. (See figure 2.)

**Figure 2: Patient-Centered Outcomes Research Institute (PCORI) Timeline for Funding, Conducting, and Disseminating Research**



Source: GAO analysis of PCORI information. | GAO-15-301

PPACA gave both PCORI and AHRQ responsibilities for disseminating CER results produced by PCORI. PPACA directs PCORI to make research findings available to clinicians, patients, and the general public within 90 days of the conduct or receipt of research findings. The act also directs AHRQ to disseminate PCORI's research findings as well as other government-funded research relevant to CER. PCORI officials stated that to coordinate AHRQ's and PCORI's dissemination responsibilities, PCORI has formed a workgroup specifically to address engagement, dissemination, and implementation activities, on which both PCORI and AHRQ officials serve. This workgroup currently meets monthly and discusses plans for disseminating CER results, among other things. A PCORI official stated that PCORI and AHRQ are working together to determine which entity will be best suited for disseminating different types of CER results.

## PCORI Has Started Awarding Contracts for Research, and Plans to Award Additional Contracts through 2019

As of October 2014, PCORI has awarded 360 contracts to fund research projects across 12 funding areas. PCORI made a total of \$670.8 million in commitments to fund these contracts.<sup>22</sup> Most of PCORI's projects are funded for periods of between 2 and 3 years, with some larger studies funded for up to 5 years. In total, PCORI expects to make approximately \$2.6 billion in commitments for contracts starting as late as 2019, with about \$1.9 billion in commitments occurring between fiscal years 2015 and 2019.

While commitments occur when PCORI makes awards to contractors, expenses occur when PCORI pays contractors and spends money for PCORI's operations. Expenses include not only money spent on research contracts, but also on research support activities—such as merit review and contractor monitoring—and administrative expenses. According to PCORI, from inception through fiscal year 2014, it has incurred a total of \$235 million in expenses.<sup>23</sup> Of this, approximately \$132 million was to fund research contracts, with the remaining amounts for research support activities and administrative expenses. Through fiscal year 2015, PCORI anticipates expending a total of \$597 million. Overall, PCORI expects to receive an estimated total of \$3.5 billion through fiscal year 2019 from the PCORTF to fund its work.<sup>24</sup> PCORI anticipates spending approximately \$2.6 billion of its \$3.5 billion on contracts for research and research infrastructure, with the remaining amounts spent on research support activities and administrative expenses.

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<sup>22</sup>According to PCORI, "commitments" represents the amount of funding PCORI intends to award or has awarded to contractors.

<sup>23</sup>In fiscal year 2014, PCORI reported that it was experiencing a "delay in spending," that is, contractors were submitting invoices to PCORI and collecting money at a slower rate than initially expected. PCORI officials noted that they have undertaken additional efforts to analyze and better plan for such delays, which can result because of the relatively slow pace of spending that occurs during the beginning of research projects. PCORI has adjusted its spending expectations based on the patterns observed to date, and PCORI officials told us that they will monitor actual expenses as one indicator that funded work is progressing at the appropriate pace. Officials anticipate that PCORI's independent audit of fiscal year 2014 will be issued in early 2015.

<sup>24</sup>PCORI officials stated that if the PCORTF expires in fiscal year 2019 before all funded research is complete they anticipate a plan will be developed to ensure the oversight of the completion of any outstanding research.

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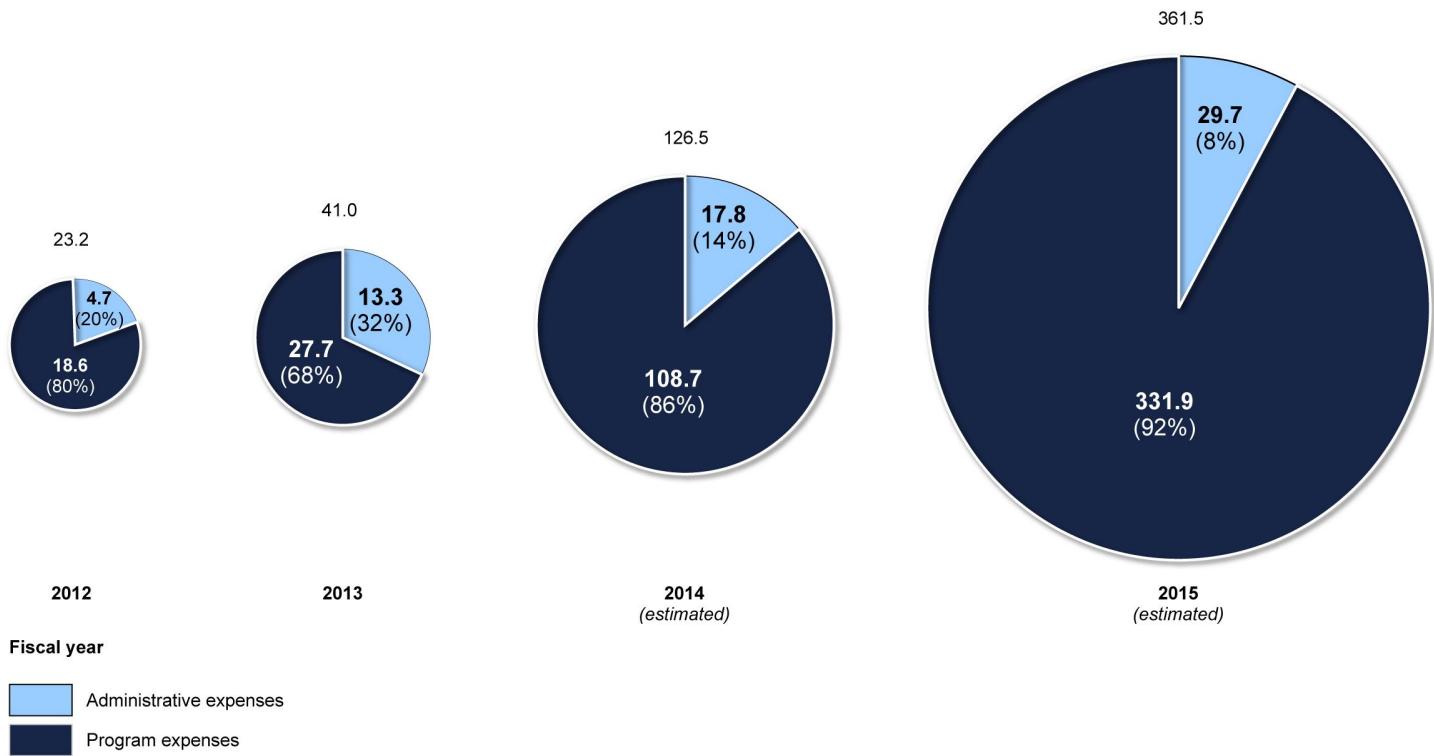
PCORI's total administrative expenses for fiscal years 2012 and 2013 accounted for 20 percent and 32.5 percent of PCORI's total operating budget, respectively.<sup>25</sup> Examples of administrative expenses include salaries for PCORI staff, health benefits, rent for PCORI's headquarters office, and information technology costs. In its fiscal year 2013 financial audit report, issued by a private, independent auditor, PCORI noted that administrative costs for fiscal year 2013 remained high, but were not outside of expected levels given that fiscal year 2013 was a period of investment in systems and infrastructure that would not be sustained at current levels in future years. According to PCORI's financial projections, it expects that total administrative expenses will constitute 14 percent and 8.2 percent of its total expenses in fiscal years 2014 and 2015, respectively. (See figure 3.)

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<sup>25</sup>Prior to 2013, PCORI reported its cash flow on a calendar year basis (January to December). In 2013, PCORI's Board of Governors voted to change the financial reporting period to a fiscal year that begins on October 1 and ends on September 30 each year. As a result, fiscal year 2013 started on January 1, 2013 and ended on September 30, 2013, resulting in a 9-month fiscal year for 2013.

**Figure 3: Patient-Centered Outcomes Research Institute (PCORI) Administrative Expenses as Portion of Total Operating Expenses**

Dollars in millions

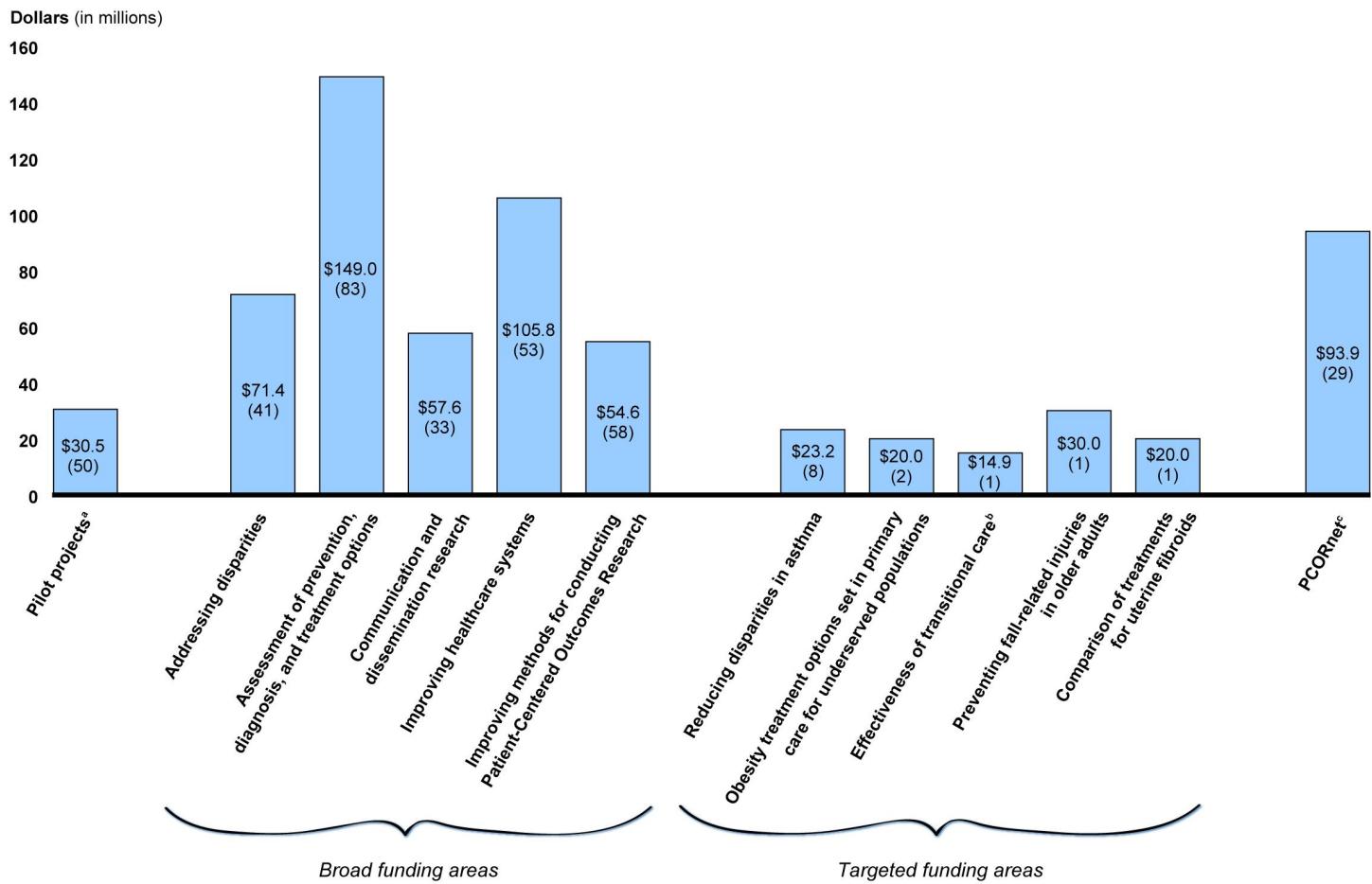


Source: GAO analysis of PCORI data. | GAO-15-301

Note: The sum of expenses in a given fiscal year may not equal the total amount for that year due to rounding. Prior to 2013, PCORI reported its cash flow on a calendar year basis (January to December). In 2013, PCORI's Board of Governors voted to change the financial reporting period to a fiscal year that begins on October 1 and ends on September 30 each year. As a result, fiscal year 2013 started on January 1, 2013 and ended on September 30, 2013, resulting in a 9-month fiscal year for 2013.

Each of the projects PCORI has committed to funding has been awarded within one of twelve funding areas. (See figure 4.) Five of these funding areas closely correspond to PCORI's priority areas. Some of the other areas—such as reducing disparities in asthma—cover specific topics within a priority area.

**Figure 4: Patient-Centered Outcomes Research Institute (PCORI) Funding Commitments (and Contracts Awarded) by Funding Area, as of October 2014**



Source: GAO analysis of PCORI data | GAO-15-301

<sup>a</sup>PCORI's pilot projects, awarded in 2012, were funded with the stated aim of helping PCORI establish its national priorities and support the collection of preliminary data for advancing patient-centered outcomes research, and developing methodologies for conducting such research.

<sup>b</sup>PCORI's "Effectiveness of Transitional Care" award was made to study the effectiveness of transitional care services, which encompass a broad range of interventions designed to promote safe and effective movement of patients across different levels of care, such as from a hospital to a rehabilitation facility or the patient's home.

<sup>c</sup>PCORnet is a data research network being developed by PCORI. PCORI issued two separate funding announcements for PCORnet.

According to PCORI officials, approximately \$106 million in commitments to date are for the PCORnet data research network, the aim of which is to improve the capacity for and speed of conducting CER. PCORI officials stated that they expect to spend a total of \$271 million on PCORnet.

through 2019. PCORnet is a distributed data research network, which means that no central repository of data exists. Instead, multiple organizations, each with their own data, agree to allow users to query their data and combine it with data from the other organizations on a project-by-project basis.<sup>26</sup> PCORnet consists of 29 separate health data networks called clinical data research networks (CDRN) and patient-powered research networks (PPRN), some of which existed prior to PCORnet and some of which were created with PCORnet funding.<sup>27</sup>

PCORnet is still undergoing development and testing. CDRNs and PPRNs are currently working to map their data to the PCORnet common data model. The common data model standardizes the definition, content, and format of data aggregated by CDRNs and PPRNs, which is necessary to allow researchers to use data from multiple CDRNs and PPRNs.<sup>28</sup> An initial test query was conducted using PCORnet in September 2014.<sup>29</sup> PCORnet is expected to be used to conduct an initial clinical research trial starting in 2015. Officials stated that limited amounts of data will be available through PCORnet for queries by researchers after September 2015, with the amount of available data increasing over time.

While PCORI officials, stakeholders, and PCORnet contractors noted that PCORnet has the potential to significantly improve the ability to conduct CER, they also noted challenges that PCORI will face with regard to the establishment and future operation of PCORnet. For example, they

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<sup>26</sup>Distributed data research networks use a common data model to organize data into a standard structure. This standard structure is expected to allow users to query and combine data from multiple separate research networks that participate in PCORnet. PCORnet's common data model mirrors the same approach used by other large research networks, including the Health Maintenance Organization Research Network and the Food and Drug Administration's Sentinel Initiative, formerly Mini-Sentinel.

<sup>27</sup>CDRNs are established in health care systems, such as hospitals or health plans, and collect health information from electronic health records gathered during the course of patient care. PPRNs are networks of patient organizations focused on particular health conditions that are interested in sharing health information and engaging in research.

<sup>28</sup>PCORnet's common data model is developed and maintained by a task force within PCORnet's coordinating center, which is funded by PCORI to provide technical and logistical support to CDRNs and PPRNs. PCORnet's common data model is based on common data models used by other distributed data research networks. PCORI expects that this common data model will be expanded over time.

<sup>29</sup>PCORI expects to conduct additional test queries in early 2015.

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expect that the process of mapping data to the common data model will be slow and resource intensive because of the lack of standardization among existing data maintained by CDRNs and PPRNs, such as data from electronic health records (EHR). PCORI officials recognize the challenges caused by the lack of standardization. They expect that both the common data model and a future requirement by PCORI for CDRNs to hire additional staff with expertise related to this work will help to address this challenge. PCORnet contractors also noted uncertainty regarding future costs and the sustainability of the network, particularly if the PCORTF is not reauthorized beyond fiscal year 2019. One stakeholder we interviewed noted that some core funding would always be required to maintain the central operation of the network. PCORI officials acknowledge that some future core funding could be needed, but they expect it to be lower than current funding levels. They also expect that future research projects funded by PCORI, NIH, or others such as the pharmaceutical and device industry will pay for the use of PCORnet's data. PCORI officials also noted that each CDRN and PPRN will be required to provide a sustainability plan for continuing operation once PCORI funds are no longer available. Another concern noted by CDRN officials is that their data does not cover all care received by patients due to the fragmented nature of the U.S. health care system, although such "complete" data would be preferred by PCORI.<sup>30</sup> An official from one PPRN noted that the completeness of CDRN and PPRN data could be improved by having PPRNs and CDRNs share data with one another, which is not current practice. PCORI officials said that they are collaborating with other relevant organizations, including state Medicaid offices and private health insurers, to identify how CDRNs could link their EHR data to claims data, which would improve data completeness.<sup>31</sup> In addition, PCORI officials stated they are requiring CDRNs to identify and implement links with other institutions that have additional patient data.

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<sup>30</sup>PCORI defines complete data as including start and stop dates—typically obtained from insurance enrollment data—as well as demographic, EHR, pharmacy dispensing, and claims data. Claims data are not required if the EHR data is reasonably complete. Interviewees noted that due to the fragmented nature of the U.S. health care system and frequent changes in health insurance status, as well as the lack of a national identifier to accurately identify patients across health care settings, it is not possible to have complete data for the majority of individuals.

<sup>31</sup>PCORI is collaborating with the Centers for Medicare & Medicaid Services, the Food and Drug Administration's Sentinel Initiative, and an association representing private health plans in order to identify optimal ways for CDRNs to link their EHR data to private health insurance claims data and, therefore, obtain more complete data on patients.

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## PCORI Has Established an Evaluation Plan and Anticipates Developing Efforts to Measure Outcomes

PCORI's evaluation group—a body composed of members from its Board of Governors, methodology committee, advisory panel on patient engagement, external experts, and PCORI staff—has developed initial plans for evaluating PCORI's efforts against its three strategic goals, which are to increase information, speed implementation, and influence research. To do so, PCORI identified primary outcome measures for each of its strategic goals. In its strategic plan, PCORI notes that these are meant to be long-term measures because research typically requires several years to complete and additional years for the results to be disseminated and implemented. Therefore, since 2013, PCORI has been using early and intermediate process and output measures as a way to monitor its progress toward its strategic goals. PCORI anticipates having some early results related to its primary outcome measures starting in 2017 after the first CER studies are completed and their findings released, although full evaluation of the results of these outcome measures will not be possible until around 2020, after a large number of CER studies have been completed and a few years have elapsed, allowing time for study results to be taken up. (See table 5.)

**Table 5: Measures of Progress toward Patient-Centered Outcomes Research Institute (PCORI) Strategic Goals**

<b>Strategic goal</b>	<b>Intermediate output measures (beginning 2014)</b>	<b>Primary outcome measures (beginning 2017)</b>	<b>Measure of success</b>
<b>Substantially increase the quantity, quality, and timeliness of useful, trustworthy information to support health decisions</b>	<ul style="list-style-type: none"> <li>• Rates and effectiveness of stakeholder participation in PCORI's work</li> <li>• Stakeholder views of the appropriateness of PCORI's research agenda and portfolio</li> <li>• Number and types of studies and topics PCORI has funded</li> <li>• Expected usability of the information that should result from the studies PCORI has funded</li> <li>• Number of studies completed and published</li> </ul>	Numbers (and proportions) of PCORI-funded studies that have "usable" results	Produce a substantial body of usable answers to critical, patient-centered, comparative clinical effectiveness research (CER) questions
<b>Speed the implementation and use of patient-centered outcomes research evidence</b>	<ul style="list-style-type: none"> <li>• Numbers of people accessing or referencing PCORI's information and the purposes for which they are accessing it</li> <li>• Uptake of study findings, such as incorporation in patient materials, clinical practice guidelines, or protocols</li> <li>• Factors affecting speed and extent of uptake of study findings</li> </ul>	Numbers (and proportions) of study results implemented within 5 years	Demonstrate that a substantial proportion of usable findings have been implemented within 5 years of publication
<b>Influence clinical and health care research funded by others to be more patient-centered</b>	<ul style="list-style-type: none"> <li>• Number of partnerships formed</li> <li>• Amount of additional resources brought to PCORI's initiatives through partnerships and collaborations</li> <li>• Amount of evaluation research PCORI has published</li> <li>• Degree to which PCORI's Methodology Standards have become the gold standard</li> <li>• Extent of curriculum PCORI has developed or supported development of</li> <li>• Number of people whose training PCORI has supported</li> <li>• Amount and types of research underway in PCORnet</li> </ul>	Amount (and proportion) of total funding for patient-centered CER that comes from funders other than PCORI	Demonstrate that, over time, PCORI funding is complemented by increases in funding for patient-centered CER from other sources, thus increasing the body of patient-centered CER

Source: Patient-Centered Outcomes Research Institute. | GAO-15-301

Officials stated that to collect information for monitoring PCORI's progress and for evaluating PCORI's impact on the dissemination and uptake of CER study results, PCORI will employ a variety of data collection

methods, including surveys, focus groups, interviews, case studies, and document reviews, to collect data on how potential consumers of CER perceive PCORI's work and CER in general. For example, PCORI is conducting a number of surveys to collect both baseline and ongoing data to gauge changes in perceptions of CER over time. (See table 6.)

**Table 6: Examples of Patient-Centered Outcomes Research Institute (PCORI) Surveys**

<b>Survey</b>	<b>Description</b>	<b>Dates for Data Collection<sup>a</sup></b>
InCrowd <sup>b</sup>	Collects information on patient, caregiver, and primary care clinician attitudes and knowledge regarding comparative clinical effectiveness research (CER) and engagement in such research.	December 2012 – January 2013
Clinician survey	Nationally representative surveys to collect information on attitudes toward CER and engagement in research from primary care clinicians.	August 2014 – December 2014
Patient survey	Nationally representative surveys to collect information on attitudes toward CER and engagement in research from patients with chronic and rare diseases.	September 2014 – October 2014
Researcher survey	Collects information from researchers about CER research practices and perceptions of PCORI programs.	October 2014 – November 2014
Caregiver survey	Nationally representative survey to collect information on attitudes toward CER and engagement in research from caregivers for persons with chronic and rare diseases.	January 2015
Health Information National Trends Survey	Collects nationally representative data routinely about the American public's use of cancer-related information. PCORI will include a set of questions to inquire about public engagement in research.	September 2013 – November 2013 July 2014 – November 2014
Stakeholder data collection via focus groups and interviews regarding PCORI's progress	Will collect opinions of stakeholder communities about PCORI's progress and solicit input on future directions for PCORI.	January 2015 – December 2015 (expected)

Source: Patient-Centered Outcomes Research Institute. | GAO-15-301

<sup>a</sup>According to PCORI officials, future surveys are planned for intervals of every two years.

<sup>b</sup>InCrowd is a corporation focused on assisting clients in gathering market feedback from health-care professionals.

PCORI officials stated that these baseline data will be compared against future similar data collection efforts in an attempt to see whether PCORI's work is contributing to improved understanding and increased use of CER. PCORI anticipates having preliminary baseline data by the end of 2014.

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PCORI has identified some limitations and challenges related to their evaluation methods. Specifically, PCORI's evaluation plans rely on survey development, focus groups and interviews, data extraction from PCORI databases, and expert panels. In its plans, PCORI identifies potential response bias and self-report bias as limitations to some of those data collection methods. Further, PCORI officials stated that measuring outcomes such as reducing practice variation and changing health care delivery can be challenging, particularly within a 5- or 10-year timeframe. PCORI staff stated that disseminating research results in an effort to improve health care is a long-term challenge, as past research suggests that it takes more than 17 years for research evidence to affect clinical practice settings.<sup>32</sup> PCORI staff stated that they hope the inclusion of end users in their research process—such as patients and clinicians—will expedite the uptake of PCORI's research findings, but it is too soon to tell if this will be the case.

Finally, officials stated that it will be difficult to know for certain whether any measured changes in health care delivery and practice are attributable to PCORI-funded research or due to other efforts. Therefore, officials stated that they will have to rely on the measures identified in PCORI's strategic plan for a small subset of PCORI's studies, to determine the extent to which these studies may influence reductions in practice variation or other changes in health care delivery.

PCORI is also undertaking efforts to assess the extent to which its funded research addresses CER priority topics identified by the IOM in its 2009 report and AHRQ's Future Research Needs.<sup>33</sup> PCORI issued a request for proposal to evaluate whether the research topics they funded address the CER priority topics identified by IOM and AHRQ. Contractors were selected and began conducting this work in June 2014, and it is anticipated that the work will continue through 2015, with the possibility of it extending into 2016. PCORI plans to use the results from this evaluation to inform additional funding announcements in the future.

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<sup>32</sup>E. A. Balas and S.A. Boren, "Managing Clinical Knowledge for Health Care Improvement," *Yearbook of Medical Informatics* (2000).

<sup>33</sup>AHRQ's Future Research Needs is the agency's list of gaps in evidence identified during AHRQ's development of comparative effectiveness reviews, which are systematic reviews of existing research on the effectiveness and harms of different health care interventions. Future Research Needs topics are posted to AHRQ's website and are intended to be used by researchers and funders of research to help improve the body of available CER.

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PCORI officials stated that preliminary analysis has shown that about half of the research studies PCORI has funded to date directly related to a CER priority topic identified in IOM's 2009 report.

PCORI has conducted a preliminary effort to compare the extent to which mental health research PCORI has funded aligns with mental health topics in the IOM report, as well as some additional analyses to show the extent to which PCORI-funded research aligns with IOM's 100 CER priorities, according to officials. For example, an analysis in September 2013 showed that of PCORI's 55 projects at that time, 6 were closely related, 23 were somewhat related, and 26 were unrelated to the IOM's 100 CER priorities. PCORI officials noted that the IOM's CER priorities were developed in 2009 and that, given the amount of time that has passed, it is likely that some of the topics listed in the IOM report are no longer critical, while other CER topics have increased in importance. PCORI officials stated that, as a result, the IOM's CER priorities alone are not a good indicator of PCORI's progress related to CER.

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## Agency Comments

We provided a draft of this report to PCORI for review and comment. PCORI provided technical comments, which we incorporated as appropriate.

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We are sending copies of this report to the Executive Director of PCORI and other interested parties. In addition, the report will be available at no charge on GAO's website at <http://www.gao.gov>.

If you or your staffs have any questions about this report, please contact me at (202) 512-7114 or at [kingk@gao.gov](mailto:kingk@gao.gov). Contact points for our Office of Congressional Relations and Office of Public Affairs can be found on the last page of this report. Other major contributors to this report are listed in appendix I.



Kathleen M. King  
Director, Health Care

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# Appendix I: GAO Contact and Staff Acknowledgments

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## GAO Contact

Kathleen M. King, (202) 512-7114 or [kingk@gao.gov](mailto:kingk@gao.gov)

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## Staff Acknowledgments

In addition to the contact named above, Will Simerl, Assistant Director; Jennie Apter; LaSherri Bush; Ashley Dixon; Colbie Holderness; Andrea E. Richardson; and Jennifer Whitworth made key contributions to this report.

# Appendix II: Accessible Data

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## Data for Figure 1: Patient-Centered Outcomes Research Institute (PCORI) Research Funding Process

- 1) Develop funding announcement
  - a) PCORI staff prioritize research topics received from the public and other sources
  - b) Advisory panels rank topics
  - c) Board reviews research topics and approves
- 2) Post funding announcement on PCORI's website
  - a) Research topics incorporated into funding announcements
  - b) Funding announcements posted on PCORI's website
- 3) Receive letters of intent (LOI)
  - a) Researchers interested in PCORI funding submit an LOI in response to funding announcement
  - b) PCORI staff evaluate LOIs for responsiveness
  - c) Researchers that submitted responsive LOIs are invited to submit full application
  - d) Merit reviewer recruitment begins
- 4) Receive full application
  - a) PCORI staff reviews full application to ensure it is responsive to the funding announcement
  - b) Applications are referred to merit review panels
- 5) Preliminary review
  - a) Merit reviewers are trained
  - b) Merit reviewers evaluate applications for adherence to PCORI's methodology standards and against
  - c) merit review criteria
  - d) Critiques and score reports are sent to PCORI staff
  - e) PCORI staff identify applications to move forward for discussion at in-person meeting
- 6) In-person meeting
  - a) Merit reviewers discuss and score applications
  - b) Chair of meeting records discussion summary
  - c) PCORI staff produce summary statements for each application discussed
- 7) Set list of recommended applications for program
  - a) PCORI staff consider score reports and portfolio balance when proposing applications to fund
- 8) Selection committee meeting
  - a) PCORI staff present recommended list of applications to fund to selection committee
  - b) Selection committee reviews recommended list of applications and merit review scores, and recommends applications for approval

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- 9) Board of governors' review
- a) PCORI staff presents recommended list of applications and rationale to Board for review and approval
  - b) Board votes to approve list of recommended applications
  - c) PCORI announces funding awards to public via Board meeting and website
- 

**Data table for Figure 2: Patient-Centered Outcomes Research Institute (PCORI) Timeline for Funding, Conducting, and Disseminating Research**

<b>Date</b>	<b>Occurance</b>
2010 March:	Passage of the Patient Protection and Affordable Care Act of 2010
2010 September:	GAO appoints ACOR Board of Govenors
2011 January:	GAO appoints methodology committee
2010 November:	First PCORIa Board meeting
2010 May:	National priorities and research agenda adopted by Board
2011 June:	Pilot projects awarded
2012 November:	Methodology standards adopted
2012 December:	First CERb awards
2013 April:	PCORNet plans announced
2013 December:	First targeted awards (reducing disparities in asthma)
2014 January:	PCORNet Phase I Begins
2014 May:	PCORNet Common Data Model released
2015 February:	First pragmatic clinical study awards to be made Completion of dissemination & implementation framework
2015 October:	PCORNet Phase II Begins

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**Data Table for Figure 3: Patient-Centered Outcomes Research Institute (PCORI) Administrative Expenses as Portion of Total Operating Expenses (dollars in millions)**

Year 2012

<b>Administrative expenses</b>	<b>Program expenses</b>
20	80

Year 2013

<b>Administrative expenses</b>	<b>Program expenses</b>
32	68

Year 2014 (estimated)

<b>Administrative expenses</b>	<b>Program expenses</b>
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## Appendix II: Accessible Data

Administrative expenses	Program expenses
14	86
Year 2015 (estimated)	
Administrative expenses	Program expenses
8	92

**Data Table for Figure 4: Patient-Centered Outcomes Research Institute (PCORI) Funding Commitments (and Contracts Awarded) by Funding Area, as of October 2014 (in millions of dollars)**

Funding Area	Funding amount
Pilot Projects	30.5
<b>Broad funding areas</b>	
Addressing Disparities	71.4
Assessment of Prevention, Diagnosis, and Treatment Options	148.966
Communication and Dissemination Research	57.6
Improving Healthcare Systems	105.8
Improving Methods for Conducting Patient-Centered Outcomes Research	54.565
<b>Targeted funding areas</b>	
Reducing Disparities in Asthma	23.201
Obesity Treatment Options Set in Primary Care for Underserved Populations	19.997
Effectiveness of Transitional Care	14.9
Preventing fall-related injuries in older adults	30
Comparison of treatments for uterine fibroids	20
PCORnet	93.9

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