



November 2020

# COMPARATIVE EFFECTIVENESS RESEARCH

## Patient-Centered Outcomes Research Institute and HHS Continue Activities and Plan New Efforts

Accessible Version

## Why GAO Did This Study

The 2010 Patient Protection and Affordable Care Act (PPACA) authorized establishment of PCORI to conduct CER and improve its quality and relevance. PPACA also established new requirements for HHS to, among other things, disseminate findings from federally funded CER and coordinate federal programs to build data capacity for this research. To fund CER activities, PPACA established the Trust Fund, which provided a total of about \$3.6 billion to PCORI and HHS for CER activities during fiscal years 2010 through 2019. The Further Consolidated Appropriations Act, 2020, added new CER requirements and extended funding at similar levels through fiscal year 2029.

PPACA and the Appropriations Act 2020 included provisions that GAO review PCORI and HHS's CER activities. This report describes (1) the CER activities PCORI and HHS carried out to meet legislative requirements, (2) how PCORI and HHS allocated funding to those CER activities, and (3) PCORI and HHS efforts to evaluate the effectiveness of their CER dissemination and implementation activities, such as changes in medical practice.

GAO reviewed legislative requirements and PCORI and HHS documentation and data for fiscal years 2010-2019. GAO also interviewed PCORI and HHS officials and obtained information from nine selected stakeholder groups that were familiar with PCORI's or HHS's CER activities. These groups included payer, provider, and patient organizations. GAO incorporated technical comments from PCORI and HHS as appropriate.

View [GAO-21-61](#). For more information, contact John Dicken at (202) 512-7114 or [dickenj@gao.gov](mailto:dickenj@gao.gov).

## COMPARATIVE EFFECTIVENESS RESEARCH

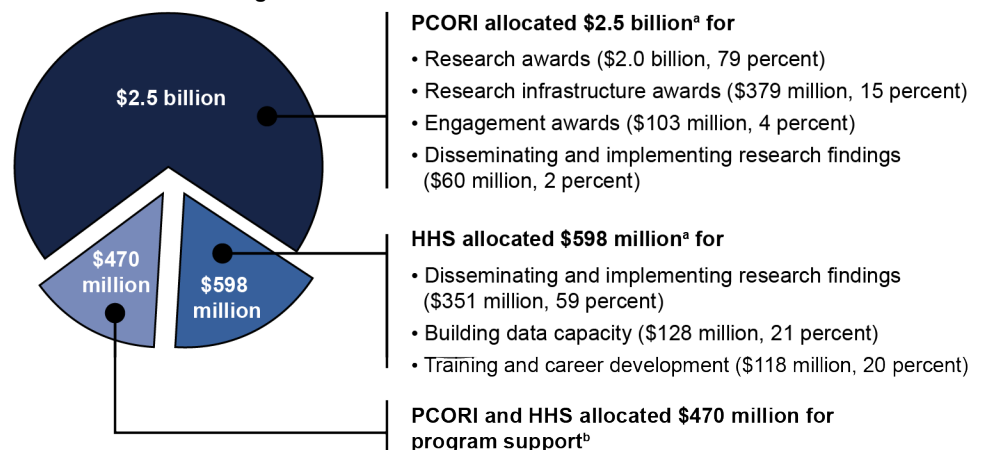
### Patient-Centered Outcomes Research Institute and HHS Continue Activities and Plan New Efforts

## What GAO Found

GAO found that the Patient-Centered Outcomes Research Institute (PCORI)—a federally funded, nonprofit corporation—and the Department of Health and Human Services (HHS) have continued to perform comparative clinical effectiveness research (CER) activities required by law since our prior report issued in 2015. CER evaluates and compares health outcomes, risks, and benefits of medical treatments, services, or items. The requirements direct PCORI and HHS to, among other things, fund CER and disseminate and facilitate the implementation of CER findings.

GAO's analysis of PCORI and HHS documents show that they allocated a total of about \$3.6 billion for CER activities and program support during fiscal years 2010 through 2019 from the Patient Centered Outcomes Research Trust Fund (Trust Fund). Specifically, PCORI allocated about \$2 billion for research awards and another \$542 million for other awards, to be paid over multiple years. HHS allocated about \$598 million for activities such as the dissemination and implementation of CER findings. PCORI and HHS also allocated about \$470 million for program support.

**PCORI and HHS Allocations for Comparative Clinical Effectiveness Research (CER) Activities, Fiscal Years 2010 through 2019**



Source: GAO analysis of Patient-Centered Outcomes Research Institute (PCORI) and Department of Health and Human Services (HHS) data. | [GAO-21-61](#)

<sup>a</sup>Totals may not add up due to rounding.

<sup>b</sup>PCORI and HHS allocated \$457 million and \$13 million for program support, respectively.

PCORI assessed the effectiveness of its activities using performance measures and targets. Since fiscal year 2017, when early CER projects were completed, PCORI officials reported that the institute met its performance targets, such as an increased number of research citations of its CER findings in news and online sources. HHS described accomplishments or assessed the effectiveness of its dissemination and implementation activities. PCORI and HHS officials told GAO they are planning comprehensive evaluations of their CER dissemination and implementation activities as part of their strategic plans for the next 10 years.

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## Abbreviations

AHRQ	Agency for Healthcare Research and Quality
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Appropriations Act 2020	Further Consolidated Appropriations Act, 2020
ASPE	Assistant Secretary for Planning and Evaluation
CDS	clinical decision support
CER	comparative clinical effectiveness research
COVID-19	Coronavirus Disease 2019
HHS	Department of Health and Human Services
NIH	National Institutes of Health
PCORI	Patient-Centered Outcomes Research Institute
PPACA	Patient Protection and Affordable Care Act
Trust Fund	Patient-Centered Outcomes Research Trust Fund
VTE	venous thromboembolism

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November 18, 2020

Congressional Committees

Comparative clinical effectiveness research (CER) evaluates and compares the health outcomes and the clinical effectiveness, risks, or benefits of two or more medical treatments, services, or items.<sup>1</sup> The goal of CER is to provide information to clinicians, patients, payers, and other stakeholders that can be used to make informed decisions and improve the quality of care, according to the National Academies of Medicine.<sup>2</sup> Therefore, the dissemination of research findings to these stakeholders is an important aspect of applying CER in health care delivery.

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 carry out CER and improve its quality and relevance.<sup>3</sup> More specifically, PCORI is required to prioritize, fund, and publicly release CER, among other things. Since then, PCORI-funded research has produced several findings. For example, one PCORI-funded study found that people with type 2 diabetes who are not treated with insulin and who self-monitor their blood glucose levels, compared to those who did not monitor, saw no

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<sup>1</sup>While clinical research is often conducted to evaluate the effectiveness of a specific treatment for a disease or condition, research is less often conducted to compare among available treatment options. For example, pharmaceutical companies conduct clinical trials to evaluate the safety and effectiveness of a new drug to treat a specific disease or condition. However, prior to federal funding for CER, there was little funding available for research to determine whether one drug is safer or more effective than another.

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

<sup>3</sup>Pub. L. No. 111-148, §§ 6301(a), 10602, 124 Stat. 119, 727, 1005 (codified at 42 U.S.C. § 1320e). PCORI's patient-centered outcomes research builds upon the definition for CER previously established by the Federal Coordination Council for Comparative Effectiveness Research. See Department of Health and Human Services (HHS), *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 work PCORI and HHS conduct as CER.

added benefit from monitoring—this finding enabled patients to save both time and money.<sup>4</sup>

In order to ensure that CER findings reach those who can benefit from them, PPACA established requirements for HHS to broadly disseminate findings from federally funded CER, including findings published by PCORI, as well as to incorporate these findings into health information technology.<sup>5</sup> HHS was also tasked to train researchers on CER methodological approaches and coordinate relevant federal health programs to build data capacity for this research.

To fund PCORI's and HHS's CER activities, PPACA established the Patient-Centered Outcomes Research Trust Fund (Trust Fund), which provided a total of about \$3.6 billion for their CER activities during fiscal years 2010 through 2019. In December 2019, the Further Consolidated Appropriations Act, 2020 (Appropriations Act 2020) made appropriations to the Trust Fund for PCORI and HHS to continue their CER activities from fiscal year 2020 through fiscal year 2029.<sup>6</sup>

PPACA also included provisions for us to report on PCORI's and HHS's activities every 5 years since the law was enacted in 2010 and to review their overall effectiveness including the extent to which federally funded

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<sup>4</sup>This study found no significant differences after 1 year in hemoglobin A1C, a measure of blood sugar control, or in health-related quality of life between patients who did and did not test their blood sugar daily. Researchers also found that, if all eligible patients stopped testing their blood sugar daily, savings of \$2.3 billion per year, or \$11.6 billion over 5 years. See, L. A. Young, J. B. Buse, M. A. Weaver, et al., "Glucose Self-monitoring in Non-Insulin-Treated Patients With Type 2 Diabetes in Primary Care Settings," *JAMA Internal Medicine*, vol. 177, no. 7 (2017) p. 920.

<sup>5</sup>In addition to CER conducted by PCORI, CER is also conducted by federal agencies, including the Department of Veterans Affairs, HHS's Agency for Healthcare Research and Quality (AHRQ), and its National Institutes of Health (NIH). For example, NIH funds CER as part of the "Pragmatic and Implementation Studies for the Management of Pain to Reduce Opioid Prescribing" project and the "Helping to End Addiction Long-term" initiative. These research efforts provide support for pragmatic clinical trials focused on the non-pharmacologic management of pain.

<sup>6</sup>Pub. L. No. 116-94, div. N, title I, § 104(a), 133 Stat. 2534, 3097 (2020). The Appropriations Act 2020 also added new requirements for PCORI and stipulated other changes.

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CER findings have been disseminated and implemented, among other things.<sup>7</sup>

In this report, we

1. review the CER activities that PCORI and HHS have carried out to meet legislative requirements,
2. describe how PCORI and HHS allocated funding to CER activities, and
3. describe PCORI and HHS efforts to evaluate the effectiveness of their CER dissemination and implementation activities.

To describe how PCORI and HHS have carried out CER activities required by law, we reviewed relevant laws and documents from PCORI and HHS about the CER activities they carried out to implement these requirements since 2015, when we issued our last reports on this topic. In addition, we interviewed PCORI officials about the institution's activities to establish research priorities, fund research, and disseminate research findings, among other things. We also interviewed HHS officials—specifically officials from the Agency for Healthcare Research and Quality (AHRQ) and the Assistant Secretary for Planning and Evaluation (ASPE) responsible for carrying out HHS's requirements—about their efforts to implement legislative requirements related to CER dissemination,

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<sup>7</sup>See Pub. L. No. 111-148, § 6301(g), 124 Stat. 119, 736 (codified, as amended, at 42 U.S.C. § 1320e(g)). The Appropriations Act 2020 made certain amendments to GAO's reporting requirements, including the provision providing for review of overall effectiveness of CER activities conducted by PCORI and HHS, such as requiring analysis of performance metrics.

GAO completed two reviews on this topic in 2015. See GAO, *Comparative Effectiveness: Initial Assessment of the Patient-Centered Outcomes Research Institute*, [GAO-15-301](#) (Washington, D.C.: Mar. 9, 2015); *Comparative Effectiveness Research: HHS Needs to Strengthen Dissemination and Data-Capacity-Building Efforts*, [GAO-15-280](#) (Washington, D.C.: Mar. 3, 2015). PPACA also included a provision that we review PCORI's and HHS's use of the Trust Fund for CER activities by 2018. See GAO, *Comparative Effectiveness Research: Activities Funded by the Patient-Centered Outcomes Research Trust Fund*, [GAO-18-311](#) (Washington, D.C.: Mar. 23, 2018). In addition, PPACA requires the Comptroller General to appoint members to PCORI's Board of Governors that represent a broad range of perspectives—including patients, payers, and providers that collectively have scientific expertise in clinical health sciences research—to carry out the duties of the institute, and to conduct annual reviews of PCORI's financial audits.

training, and building data capacity.<sup>8</sup> We compared information gathered from relevant documents and interviews, including prior GAO reports, with the appropriate legislative requirements.<sup>9</sup>

We also selected and interviewed officials from large organizations that broadly represent stakeholder groups identified in legislative requirements, and that were involved in PCORI, AHRQ, or ASPE CER activities, to understand their perspectives about these efforts and information disseminated by PCORI and AHRQ, among other things.<sup>10</sup> Finally, we also reviewed documents and interviewed PCORI, AHRQ, and ASPE officials about their efforts to coordinate with each other and with relevant stakeholders on CER-related activities, including dissemination and implementation activities.

To describe how PCORI and HHS allocated funding to CER activities, we reviewed PCORI data on CER award commitments and program support expenditures, which included administrative support services, and HHS data on obligated funds for CER activities.<sup>11</sup> We reviewed data from fiscal year 2010, the first year funding for CER activities was made available, through fiscal year 2019, the most recent year for which data on funding allocated for CER activities were available for the entire fiscal year during

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<sup>8</sup>PPACA requires HHS's AHRQ to carry out specified functions with respect to the dissemination of CER, and the Secretary of HHS has delegated responsibility for carrying out the functions related to the coordination of federal programs to build data capacity for CER to HHS's ASPE. Pub. L. No. 111-148, § 6301(b), 124 Stat. 119, 738-740 (codified at 42 U.S.C. § 299b-37).

<sup>9</sup>See, for example, [GAO-15-280](#) and [GAO-15-301](#).

<sup>10</sup>Targeted stakeholder groups are identified in legislation, such as for PCORI's advisory panels and Board of Governors and as the audience for HHS's dissemination activities. We interviewed nine organizations representing various stakeholders, including physicians and health care providers (American Association of Cardiovascular and Pulmonary Rehabilitation, American Board of Family Medicine, American Medical Association), patients (National Health Council, Partnership to Improve Patient Care), payers (America's Health Insurance Plans), health care policy makers, including professional associations (National Academy of Medicine), and vendors of health information technology (Cerner, ECRI Institute). Findings based on information we obtained from these organizations cannot be generalized to all relevant stakeholders.

<sup>11</sup>According to PCORI, commitments represent the total amount of funding PCORI intends to award or has awarded to contractors over several fiscal years. For example, funds committed to a CER study in 2019 could be expended over the next 5 years. HHS obligations in a given year represent a definite commitment of funds that create a legal liability for the payment of goods and services ordered or received, although funds may be expended in subsequent years.



the time of our analysis.<sup>12</sup> We also reviewed relevant documents and previous GAO reports describing PCORI's and HHS's CER activities. We conducted interviews with PCORI officials and officials from HHS's AHRQ and ASPE responsible for planning and carrying out CER activities. To assess the reliability of PCORI commitment and expenditure data and HHS obligation data, we collected information from PCORI, AHRQ, and ASPE officials regarding the reliability of the data, including the accuracy of data entry and the systems that contain the data. We also reviewed PCORI's annual audited financial statements and GAO's review of these audits.<sup>13</sup> On the basis of these steps, we determined the data were sufficiently reliable for the purposes of our reporting objectives.

To describe PCORI and HHS efforts to evaluate the effectiveness of their CER dissemination and implementation activities, we reviewed documents from PCORI and AHRQ—the HHS agency responsible for CER dissemination and implementation—on their dissemination and implementation activities, including any data related to their performance measures and other evaluations of these activities since PPACA was enacted in 2010. We also interviewed PCORI and AHRQ officials to learn about their efforts to evaluate the effectiveness of their dissemination and implementation activities. To assess the reliability of PCORI's CER data related to its dissemination and implementation activities, we reviewed related data to the extent they were available and collected information from, and interviewed PCORI officials regarding, the accuracy of data entry and the systems that contain the data. We determined the data were sufficiently reliable for the purposes of our reporting objectives.

We conducted this performance audit from December 2019 to November 2020 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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<sup>12</sup>HHS received funds transferred from the Trust Fund from fiscal years 2011 through 2019 in contrast to PCORI, which began receiving funding in fiscal year 2010, as provided under PPACA.

<sup>13</sup>See, for example, GAO, *Patient-Centered Outcomes Research Institute: Review of the Audit of the Financial Statements for FY 2019*, [GAO-20-469R](#) (Washington, D.C.: Mar. 26, 2020).

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## Background

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### PCORI CER Activities

PCORI is required to perform key activities related to CER:

- identifying research priorities, and development of a research agenda;
- establishing advisory panels;
- funding research;
- establishing a peer review process;
- disseminating research findings; and
- performing certain oversight functions.

The Appropriations Act 2020 requires PCORI to include intellectual and developmental disabilities and maternal mortality in its research priorities and to take into account the potential burdens and economic impacts of medical treatments in its research, such as patients' out-of-pocket medical costs.<sup>14</sup>

PCORI's research funding process takes several years—roughly 2 years to over 6 years—to reach completion and includes several steps:

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<sup>14</sup>Specifically, the Appropriations Act 2020 requires that PCORI's research priorities include intellectual and developmental disabilities and maternal mortality, reflect a balance between long-term priorities and short-term priorities, and be responsive to changes in medical evidence and in health care treatments. It also stipulated that PCORI consider the full range of outcomes to include the potential burdens and economic impacts of the utilization of medical treatments, items, and services on different stakeholders, such as medical out-of-pocket costs, including health plan benefit and formulary design; non-medical costs to the patient and family, including caregiving; effects on future costs of care; workplace productivity and absenteeism; and healthcare utilization. The Appropriations Act 2020 also changes responsibility for the appointment of PCORI's Methodology Committee from the Comptroller General to PCORI. This committee is responsible for developing methodology standards that provide specific criteria for internal validity, generalizability, feasibility and other aspects of research design, and to provide guidance on research methods that are most likely to address a specific research question.

announce funding, select applications, negotiate contracts, conduct research, undergo peer review, and disseminate findings. As a result, as we have reported, many of the studies started in PCORI's early years are still underway or may have only recently been completed. Therefore results from these studies may not yet be available or have only recently become available to be disseminated or implemented.<sup>15</sup>

Further, PCORI has supported the development of PCORnet as a distributed research network initiative that enables electronic health-related data from multiple sources to be available for research. According to PCORI officials, PCORnet was developed to improve the nation's capacity to conduct patient-centered clinical research efficiently with existing data. This research is done through a network model that helps build data capacity for research by enabling participating networks to translate certain fields of existing electronic health-related data from multiple sources into a common data model, so that the translated data can be used to conduct research.

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## HHS CER Activities

PPACA requires HHS to perform several activities related to CER, which it has implemented through AHRQ and ASPE. Specifically,

- AHRQ is required to disseminate and support the incorporation of CER funded by PCORI and other federal entities, as well as to foster capacity for conducting CER by supporting training in the methods used to conduct such research.
- ASPE, in turn, is required to coordinate relevant federal health programs to build data capacity for CER in order to develop and maintain a comprehensive, interoperable data network that collects, links, and analyzes CER data.

In our 2015 report, we made five recommendations to HHS to direct AHRQ and ASPE to address various CER-related issues related to their activities, as appropriate.<sup>16</sup> For example, we recommended that HHS direct (1) AHRQ to take several actions related to its dissemination efforts, including expanding dissemination efforts to federal and private health plans and vendors of health information technology on clinical decision support, and (2) ASPE to include clearly defined objectives,

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<sup>15</sup>See [GAO-18-311](#).

<sup>16</sup>See [GAO-15-280](#).

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milestones, and time frames, or other indicators of performance, in its strategic road map used to identify its CER-funded projects. HHS has subsequently implemented all five recommendations.

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### Trust Fund for PCORI and HHS CER Activities

PPACA established the Trust Fund through which PCORI and HHS receive funds for CER activities. The law provided funds for fiscal years 2010 through 2019, including \$1.26 billion in appropriations from the general fund of the Treasury, and other varying amounts transferred from the Medicare trust funds and fees collected by the Department of the Treasury from private insurance and self-insured health plans. PPACA required that 80 percent of the amounts in the Trust Fund be made available to PCORI in fiscal years 2011 through 2019, and the Department of Treasury was required to transfer the remaining 20 percent to the Secretary of HHS. The Trust Fund provided a total of about \$3.6 billion to PCORI and HHS for CER activities during fiscal years 2010 through 2019.

In December 2019, the Appropriations Act 2020 extended funding for PCORI and HHS through the Trust Fund through fiscal year 2029 to allow them to continue their CER activities. For fiscal years 2020 through 2029, the act increased appropriated amounts to \$3.3 billion from the general fund of the Treasury, and extended funding from the collections of insurance fees.<sup>17</sup> The Congressional Budget Office estimated that during fiscal years 2020 through 2029, the Trust Fund will receive about 95 percent of the funds it had received during the previous 10 years.<sup>18</sup>

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### PCORI and HHS Continued to Perform CER Activities Required by Law and Plan to Address New Requirements

We found that PCORI and HHS have continued to perform CER activities required by law since our prior report issued in 2015. For example,

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<sup>17</sup>The Appropriations Act 2020 did not provide for appropriations from the Medicare trust funds or affect the division of funds. The law continues to direct 80 percent of funds to PCORI.

<sup>18</sup>Senate Republican Policy Committee, *House Message on H.R. 1865 – The Further Consolidated Appropriations Act, 2020* (Washington, D.C.: Dec. 19, 2019).

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PCORI performed work on several required activities, including to identify research priorities and to fund and disseminate research. Within HHS, AHRQ and ASPE performed work on their required CER activities, including disseminating, training, and building data capacity for research.

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### PCORI's CER Activities

Based on our review of documents and interviews with PCORI officials, we found that PCORI continued to perform the required activities we previously described in our 2015 report. For example, the institute established advisory panels and a peer review process. In other cases, PCORI continued to perform required CER activities on an ongoing basis including identifying research priorities and funding research. PCORI has also expanded its dissemination activities as a significant amount of research had just been completed starting in fiscal year 2017 (see table 1).<sup>19</sup>

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<sup>19</sup>See [GAO-15-301](#).

**Table 1: PCORI Activities to Address Key Legislative Requirements Related to CER, as of September 2020**

Description of requirements	Description of PCORI activities to address requirements
<p><b>Identify research priorities:</b> Identify national research priorities and establish and update a research project agenda to address the priorities identified by PCORI.</p>	<p>PCORI identified five broad research priorities in 2012 that have remained consistent—</p> <ul style="list-style-type: none"> <li>• assessing prevention, diagnosis, and treatment options;</li> <li>• improving healthcare systems;</li> <li>• communicating and disseminating research;</li> <li>• addressing disparities; and</li> <li>• accelerating patient-centered outcomes research and methodological research.</li> </ul> <p>Within this framework, PCORI developed a research agenda with specific research areas to address each priority. PCORI has engaged with multiple stakeholders to modify its research agenda over the years to reflect changing national issues, such as the prevalence of comorbidities and emergency responses to Coronavirus Disease 2019.</p>
<p><b>Establish advisory panels:</b> Appoint advisory panels composed 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, among other requirements. Establish a methodology committee to develop and improve the science of CER.</p>	<p>PCORI established advisory panels for rare diseases and clinical trials, and established other advisory panels, such as for healthcare delivery and disparities research, to involve stakeholders in the identification of research priorities and other efforts. PCORI solicits potential advisory panel members each year, and its Board of Governors appoints members and confirms that the composition of its panels include stakeholders outlined in the legislation, such as clinicians and patients. In addition, PCORI’s Methodology Committee, which includes experts in the fields of research and methodology, defines and recommends methodological standards for research to the Board.</p>
<p><b>Fund research:</b> Carry out research using systematic reviews and assessments of existing and future research; primary research, such as randomized clinical trials; and other methodologies, as recommended by PCORI’s Methodology Committee and adopted by PCORI, among other requirements.</p>	<p>From fiscal year 2012 through 2019 PCORI funded 471 CER projects on topics such as cancer, cardiovascular diseases, and mental or behavioral health. It continues to use a multi-step merit review process to score and select applications for funding research that take into account PCORI’s priorities. This research includes primary research, observational studies, randomized clinical trials, and other methodologies. In addition, PCORI established a process to regularly update evidence from its completed research projects and posts updates to its website.</p>
<p><b>Establish peer review process:</b> Provide a peer review process to review primary research to assess its integrity and its adherence to PCORI’s methodological standards.</p>	<p>PCORI established a peer review process in 2015 that it uses to assess its research’s adherence to methodological standards and the integrity of the research it funds. PCORI also publicly posts any potential conflicts of interest that its peer reviewers may have on its website.</p>
<p><b>Disseminate 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.</p>	<p>PCORI ensures that its research findings are publicly available within 90 days and takes other steps to broadly disseminate its research findings to multiple stakeholders. For example, PCORI requires researchers to make their study results public, provides for the free availability of journal articles, and posts findings on its website. The institute also translates the findings into versions that are specifically tailored to be useful to patients and providers in making health care decisions.</p>

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Letter

Description of requirements	Description of PCORI activities to address requirements
<b>Perform oversight functions:</b> Conduct oversight of activities and finances.	PCORI conducted activities to ensure transparency, such as publicly posting Board meeting announcements 7 days in advance, releasing annual reports, and disclosing Board members' potential conflicts of interest. The institute also hired an outside financial auditor and took other steps to ensure oversight and transparency in its work.

Source: GAO analysis of legislative requirements and Patient-Centered Outcomes Research Institute (PCORI) activities related to comparative clinical effectiveness research (CER). | GAO-21-61

All nine of the stakeholders we interviewed commented on PCORI's activities to meet legislative requirements, such as those related to conducting research, and were generally complimentary of PCORI's efforts. Seven of the nine stakeholders noted that PCORI's work to include patients' participation in the research process has had a significant positive impact on the way research is conducted, on research outcomes, or on the dissemination of research to better meet patients' needs.<sup>20</sup> Looking ahead, given that PCORI will receive funding through fiscal year 2029, seven of the stakeholders suggested that PCORI expand its research, with two suggesting more research on primary care issues that have a significant impact on patients and providers. Six stakeholders also stated that they hoped PCORI would continue to expand PCORnet research, and one stakeholder noted that PCORnet was already being leveraged for research related to the Coronavirus Disease 2019 (COVID-19).<sup>21</sup>

PCORI has begun meeting with stakeholders as part of its plans to implement provisions of the Appropriations Act 2020, which extended PCORI's funding through fiscal year 2029 and included new requirements

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<sup>20</sup>As an example of how patient perspectives have been incorporated into CER, PCORI funded a study to determine whether a program that includes parents as active participants in pediatric unit rounds at seven hospitals would reduce medical errors, in comparison to parents who did not participate in the program. In fact, the program reduced harmful medical errors—preventable adverse events—by 38 percent, and the research team plans to expand the program to more hospitals. See Alisa Khan, Nancy D. Spector, Jennifer D. Baird, Michele Ashland, et al., "Patient Safety After Implementation of a Coproduced Family Centered Communication Programme: Multicenter Before and After Intervention Study." *BMJ*, no. 363 (2018).

<sup>21</sup>PCORnet is a PCORI-funded network that supports the translation of existing clinical, patient, and health plan networks' data into a common data model resulting in the availability of a nationally representative sample of individuals that can be used in randomized clinical trials, large observational studies, and other research to make it faster, easier, and less costly to conduct clinical research. PCORI's fact sheet shows that, as of 2019, PCORnet's clinical research network includes protected health data on roughly 60 million patients from more than 100 health systems nationwide who have had a medical encounter in the past 5 years. According to a PCORnet press release on May 15, 2020, its leadership has created a COVID-19-specific common data model that will allow researchers to use information gathered from patients across PCORnet's network to better define and understand who is getting infected and how the virus affects them.



for the institute.<sup>22</sup> For example, as of September 2020, our review of documentation shows that PCORI had begun to engage stakeholders about incorporating developmental disabilities and maternal health and mortality into PCORI's research priorities and incorporating cost considerations into its research. PCORI has also begun outlining its process to select and appoint its Methodology Committee members in order to place new members during the next appointment cycle, now that the organization has direct responsibility for these appointments. In addition, our review of documentation shows that PCORI has begun planning to expand its research awards and other CER activities after funding was extended for 10 more years. These planned efforts include increasing the level of funding for existing projects to enable researchers to incorporate treatment or prevention efforts related to COVID-19 or to incorporate changes to the research underway that was complicated by disruptions caused by the virus.

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## HHS's CER Activities

According to HHS documentation and interviews, AHRQ and ASPE have continued to perform the required ongoing activities we previously described in our 2015 report that relate to dissemination, training, and building data capacity (see table 2).<sup>23</sup>

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<sup>22</sup>As previously noted, the Appropriations Act 2020 requires that PCORI's research priorities include intellectual and developmental disabilities and maternal mortality and that PCORI consider outcomes that include potential burdens and economic impacts, among other changes.

<sup>23</sup>See [GAO-15-280](#). For example, ASPE officials told us that they continued to use the strategic framework they developed that focuses on five areas—standards, services, policies, federal data, and governance structures—necessary to build data capacity. In addition, ASPE funded projects to advance one or more of the five core functionalities necessary for enhancing and improving data infrastructure for CER: (1) collection of patient-generated data; (2) standardized collection of clinical data; (3) linking clinical and other data for research; (4) use of clinical data for research; and (5) enhancement of publicly funded data systems for research.

**Table 2: HHS Activities to Address Legislative Requirements Related to CER, as of September 2020**

Brief description of requirements	Description of HHS activities to address requirements
<p><b>Disseminate research:</b> Agency for Healthcare Research and Quality (AHRQ), in consultation with the National Institutes of Health, to broadly disseminate research findings produced by the Patient-Centered Outcomes Research Institute and other government-funded CER.</p>	<p>AHRQ officials told us that they disseminate CER in consultation with the National Institutes of Health. Officials said that they contribute to the dissemination of research findings in various ways, such as through the development of systematic reviews.<sup>a</sup> They also disseminate these findings through several mechanisms, including social media, electronic newsletters, and websites (such as AHRQ’s Library of Patient-Centered Outcomes Research and AHRQ’s Effective Health Care Program). AHRQ developed the Patient-Centered Clinical Decision Support (CDS) Learning Network—an information technology mechanism that provides clinicians, staff, patients, or other individuals with knowledge and individual-specific information to enhance health care decision-making—with the goal of disseminating research findings.<sup>b</sup></p> <p>AHRQ’s dissemination activities also comprise efforts to implement CER findings. AHRQ officials told us that the agency has contributed to the dissemination and implementation of CER findings through several large-scale activities, such as TAKEheart: AHRQ’s Initiative to Increase Use of Cardiac Rehabilitation (which is designed to help hospitals and health systems implement strategies from research on cardiac rehabilitation for eligible patients) and Screening, Brief Intervention for Alcohol Misuse, and Medication-Assisted Therapy for Alcohol Use Disorder (which supports primary care practices efforts to address patients’ unhealthy alcohol use).</p>
<p><b>Disseminate research:</b> AHRQ to create tools that organize and disseminate research findings for physicians, health care providers, patients, vendors of health information technology focused on CDS, appropriate professional associations, and federal and private health plans. Disseminated information should include a description of considerations for specific subpopulations, the research methodology, and the limitations of the research, among other things.</p>	<p>AHRQ has created informational tools to disseminate CER. For example, various stakeholders—including providers, patients, and policymakers—can access systematic reviews and tools, such as clinician summaries and consumer reports, on AHRQ’s Effective Health Care Program website. AHRQ officials told us that the website has a procedural manual that outlines, for example, how systematic reviews should be conducted and expectations for including pertinent information about the specific subpopulation or targeted audience, research methodology, and limitations, among other information.</p> <p>For users of health information technology focused on CDS AHRQ launched the CDS Connect project to advance research findings into clinical practice. According to AHRQ officials, health information technology vendors, clinicians, payers, and other stakeholders collaborate to develop tools that help make CDS more shareable, standards-based, and publicly available for implementation. These stakeholders contribute to a repository of artifacts, such as computerized alerts, that represent medical knowledge synthesized and disseminated from various evidence-based sources, such as peer-reviewed articles and clinical guidelines.</p>
<p><b>Disseminate research:</b> AHRQ to develop a publicly available database that collects and contains government-funded evidence and research from public, private, not-for-profit, and academic sources.</p>	<p>To address the publicly available database requirement, AHRQ launched the Library of Patient-Centered Outcomes Research website in February 2016. It offers researchers, clinicians, policymakers, patients and their families, and others access to several existing publicly available databases, such as the National Library of Medicine’s ClinicalTrials.gov and PubMed and the Patient-Centered Outcomes Research Institute’s Research and Results Database, that collect information on CER studies and findings.</p>

Letter

Brief description of requirements	Description of HHS activities to address requirements
<b>Establish CER training:</b> AHRQ, in consultation with the National Institutes of Health, to establish a training grant program to build capacity for CER. Such a grant program shall provide for the training of researchers in the methods used to conduct CER, including systematic reviews of existing research and primary research such as clinical trials.	AHRQ, in consultation with the National Institutes of Health, developed 11 training projects designed to build capacity for conducting CER that were implemented between fiscal years 2011 and 2019. AHRQ's training program evaluation project is currently ongoing and will assess eight of the 11 training projects. Preliminary results are expected in the fall of 2020, and the evaluation will be completed by September 2021.
<b>Build data capacity:</b> The Secretary of HHS—who, by delegation, charged the Assistant Secretary for Planning and Evaluation (ASPE)—to provide for the coordination of relevant federal health programs to build data capacity for CER, including the development and use of clinical registries and health outcomes research data networks, in order to develop and maintain a comprehensive, interoperable data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources, including electronic health records.	Since 2011, ASPE has funded and supported 52 projects to build data capacity for CER on topics such as opioids, emergency preparedness and response, and interoperability of electronic health records. Fifteen HHS agencies and offices currently participate in the ASPE-managed data capacity program. In 2017, ASPE contracted with RTI International, a research organization, to conduct a comprehensive evaluation of its data-capacity building activities. In 2019, it contracted with NORC, a University of Chicago research institute, to conduct an assessment of ASPE's efforts to address several gaps RTI International had identified. ASPE officials told us that they have taken steps to address recommendations RTI International and NORC highlighted, including enhancing awareness of ASPE's data-capacity building activities and products and developing performance measures associated with those efforts.

Source: GAO analysis of legislative requirements and the Department of Health and Human Services' (HHS) comparative clinical effectiveness research (CER) activities. | GAO-21-61

Note: AHRQ is to establish a process for receiving feedback from entities to which information is disseminated about the value of the disseminated information as well.

<sup>a</sup>Systematic reviews evaluate and synthesize existing research on a clinical issue to compare the effectiveness and harms of different healthcare interventions, and typically include descriptions of the research findings.

<sup>b</sup>CDS programs provide health care providers and other individuals with knowledge and person-specific information, intelligently filtered or presented at appropriate times, to enhance health and health care. CDS encompasses a variety of tools, such as clinical guidelines, to enhance decision-making in the clinical workflow.

AHRQ and ASPE officials told us that they have begun planning a continuation of their CER activities, given that CER funding for the agencies has been extended through fiscal year 2029. For example, AHRQ officials told us they have begun a strategic planning process that will outline AHRQ's overall framework for defining how CER activities will be conducted in the future, as well as the agency's development of evaluation and stakeholder engagement plans. ASPE officials told us that, in addition to developing a strategic plan for the next 10 years and working to engage external stakeholders, they are conducting a literature review on data infrastructure gaps and opportunities for CER related to intellectual and developmental disabilities and maternal health that can inform potential areas for future work.<sup>24</sup> ASPE officials told us that they will evaluate their data capacity-building activities over the next 10 years to assess their progress on the strategic plan.

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## PCORI Allocated Funds Primarily for Research Activities; HHS Allocated Funds Primarily to Disseminate and Implement Research Findings

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### PCORI Has Allocated About \$2.5 Billion to CER Activities through Fiscal Year 2019

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Our review of data and documentation shows that PCORI allocated a total of about \$2.5 billion to its CER activities from fiscal year 2010 through fiscal year 2019, the majority of which (about \$2 billion) was committed for research awards, and the remainder was committed to research infrastructure, dissemination and implementation, and

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<sup>24</sup>In September 2020, ASPE officials told us that they are in the process of awarding two contracts to engage both internal federal partners and external stakeholders.

In addition to intellectual and developmental disabilities and maternal health, ASPE officials told us that ASPE is also conducting a literature scan on the economic impacts of the utilization of medical treatments, items, and services on different stakeholders and decision-makers respectively. These economic impacts include medical out-of-pocket costs, such as health plan benefit and formulary design; non-medical costs to the patient and family, such as caregiving, effects on future costs of care, workplace productivity and absenteeism; and healthcare utilization. ASPE officials said that they will use the results of the scan in its planning efforts.

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engagement awards (see table 3).<sup>25</sup> In addition to committing funding to awards, PCORI spent \$457 million during this period for program support services such as staff salaries, staff benefits, and administrative services.

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<sup>25</sup>As noted earlier, PCORI's award process takes several years to reach completion and includes several steps such as to announce funding, select applications, and negotiate contracts. As a result, we use 2010 as the year this process began, although PCORI committed its first research awards in fiscal year 2012. Similarly, PCORI committed its first engagement awards and dissemination and implementation awards in later years after research had been completed.

**Table 3: PCORI Award Commitments for CER Activities, Fiscal Years 2010 through 2019**

Award type	Dollars in millions							Total
	2010 -2013	2014	2015	2016	2017	2018	2019	
Research awards	274	304	372	327	314	248	148	<b>1,987</b>
Research infrastructure awards	9	95	142	44	65	17	7	<b>379</b>
Engagement awards	0	3	15	23	22	15	25	<b>103</b>
Dissemination and Implementation awards	0	0	0	10	2	19	29	<b>60</b>
<b>Total commitments</b>	<b>283</b>	<b>402</b>	<b>529</b>	<b>404</b>	<b>403</b>	<b>299</b>	<b>209</b>	<b>2,529</b>

Source: GAO analysis of Patient-Centered Outcomes Research Institute (PCORI) information. | GAO-21-61

Notes: According to PCORI, “commitments” represent the total amount of funding PCORI intends to award or has awarded to contractors over several fiscal years. For example, funds committed to a research study in fiscal year 2019 could be expended over the next 5 years. We combined commitment data for fiscal years 2010 through 2013 because of the low level of commitments in those years, as PCORI was in the process of formation and beginning its comparative clinical effectiveness research (CER) activities. Furthermore, PCORI changed the months of its fiscal year during this period. Research awards fund studies in priority areas imposing a substantial burden on patients and the healthcare system, research infrastructure awards are used for building data capacity and training CER researchers, dissemination and implementation awards help researchers to publicize and support the use of their findings, and engagement awards provide support for bringing clinicians, patients, and other stakeholders into the research process.

Details of PCORI's awards related to research, research infrastructure, dissemination and implementation of its research findings, and engagement are as follows:

- **Research awards.** For fiscal years 2010 through 2019 PCORI committed a total of about \$2 billion—or 79 percent of its total commitments—to research awards. PCORI's research awards have focused on funding research studies on conditions that impose substantial health or financial burdens on patients and the healthcare system. Of the 26 health condition portfolios PCORI has committed research funding toward, the mental and behavioral health, cancer, neurological disorders, cardiovascular disease, and multiple chronic conditions research portfolios have received the largest amount of committed funds. (See table 4 for commitment amounts and examples of studies PCORI has funded). As of the end of fiscal year 2019, a total of 309 PCORI-funded research studies were completed, and PCORI officials said that 368 studies were still underway.<sup>26</sup>

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<sup>26</sup>PCORI-funded research studies may include CER studies, research on CER methodology, CER pilot studies, or other research related to patient engagement in the research process.

**Table 4: Health Conditions That Received the Highest PCORI Research Award Commitments, Fiscal Years 2010 through 2019**

Conditions	Commitments (dollars in millions)	Description and examples of studies awarded funding by PCORI
Mental and behavioral health	527	130 studies on depression, substance abuse, bipolar disorder, schizophrenia, anxiety disorders, autism, and related topics. For example, one study compared whether it was more effective for people with serious mental illness to manage their symptoms through peer-led group sessions at a clinic or by using a smartphone app.
Cancer	347	88 studies on prevention or treatments for breast, colorectal, lung, prostate, cervical, blood, and other cancers. For example, one study examined whether more screenings for prostate-specific antigens in the first year following prostate cancer treatment led to better health outcomes among men who had zero, one, two, three, or more screenings.
Neurological disorders	331	77 studies on multiple sclerosis, Alzheimer’s disease, dementia, and other cognitive impairment issues. For example, one study is examining the relative effectiveness of a fatigue management course delivered via teleconference, the internet, and in-person modalities among multiple sclerosis patients.
Cardiovascular disease	306	70 studies on congestive heart failure, hypertension, strokes, and other cardiovascular conditions. For example, one study demonstrated that patients who went to a rehabilitation center after stroke were more likely to recover better than patients who went to a skilled nursing center.
Multiple chronic conditions	240	58 studies on patients with two or more chronic conditions such as diabetes, hypertension, obesity, or depression, and other conditions. For example, one study is comparing the effectiveness of two specialty medical home programs using either telemedicine or in-person clinic visits in helping patients with inflammatory bowel disease and behavioral health conditions manage their symptoms.

Source: GAO analysis of Patient-Centered Outcomes Research Institute (PCORI) information. | GAO-21-61

Notes: According to PCORI, “commitments” represent the total amount of funding PCORI intends to award or has awarded to contractors over several fiscal years. For example, funds committed to a research study in fiscal year 2019 could be expended over the next 5 years. PCORI uses inclusive health condition award categories, with some studies counted in more than one category, so adding the number of awards in each category will exceed the total number of awards and total funds committed.



- **Research infrastructure awards.** For fiscal years 2010 through 2019 PCORI committed \$379 million—or 15 percent of its total commitments—to awards for enhancing research infrastructure. PCORI committed \$349 million to awards for building data capacity through the development of PCORnet, which coordinates data from multiple partner networks to conduct research using a common data model. As of August 2020, PCORnet included nine clinical research networks, two health plan research networks, and its Coordinating Center.<sup>27</sup> PCORI committed the remaining \$30 million to workforce training awards for clinicians and researchers to train them to conduct patient-centered outcomes research.
- **Engagement awards.** For fiscal years 2014 through 2019 PCORI committed \$103 million—or 4 percent of its total award commitments—to engagement awards, which are intended to bring more patients, caregivers, clinicians, and other healthcare stakeholders into the research process. For example, these awards include the Eugene Washington Engagement Awards intended to bring patients, caregivers, clinicians and other healthcare stakeholders into the research process. One of these awards allowed the University of South Carolina to implement a diabetes-focused Virtual Patient Engagement Studio to emphasize engagement with geographically dispersed and hard-to-reach patients in the state.
- **Dissemination and implementation awards.** For fiscal years 2016 through 2019 PCORI committed \$60 million—or 2 percent of its total award commitments—to dissemination and implementation awards. These awards help researchers and other stakeholders publicize their findings and support the use of findings by patients and providers. For example, PCORI awarded about \$1.5 million to Johns Hopkins University to conduct a multi-year study that examines the effects of nurse and patient education in reducing missed doses of blood clot prevention

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<sup>27</sup>Clinical Research Networks are networks that originate in healthcare systems, such as hospitals, health plans, or practice-based networks, and securely collect health information during the routine course of patient care. Health Plan Research Networks are composed of U.S. health plans that link health insurance claims data with data from the clinical research networks' electronic health records and other sources of data. The PCORnet Coordinating Center is composed of two organizations—the Duke Clinical Research Institute and the Harvard Pilgrim Health Care Institute—working to lead the network's data and engagement activities, to support the overall network infrastructure, and to connect the networks with research partners.

medications. Two awards from this study focused on disseminating and implementing its findings. The first award focused on implementing the study's findings by expanding patient education to the entire inpatient population at the large teaching hospital where the study took place and to another community hospital.<sup>28</sup> The second award is focusing on implementing the program at 10 trauma centers, for use by an estimated 3,500 nurses and 32,000 patients in 60 hospitals, which PCORI officials said would lay the groundwork for broad uptake by trauma centers in the U.S.<sup>29</sup>

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## HHS Has Allocated About \$598 million to CER Activities through Fiscal Year 2019

Our analysis of data and documentation shows that HHS allocated a total of about \$598 million to CER activities from fiscal years 2011 through 2019, the majority of which (\$351 million) was obligated for AHRQ's dissemination and implementation awards.<sup>30</sup> The remainder was obligated for AHRQ's training and career development awards and ASPE's building data capacity awards (see table 5). In addition to funding its CER activities, AHRQ and ASPE obligated about \$13 million for program support (such as administrative and personnel expenses) from fiscal year 2011 through fiscal year 2019.

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<sup>28</sup>PCORI awarded about \$500,000 to the dissemination project, "Preventing Venous Thromboembolism (VTE): Engaging Patients to Reduce Preventable Harm from Missed/Refused Doses of VTE Prophylaxis," to provide nurses at target hospitals VTE prevention training as part of their annual professional education and to provide patients access to a VTE prevention video through the hospital television system upon admission.

<sup>29</sup>PCORI awarded about \$1.4 million to the implementation project, "Implementing Best-Practice, Patient-Centered Venous Thromboembolism (VTE) Prevention in Trauma Centers," to provide nurse and patient education on VTE prophylaxis administration at hospital trauma centers and to examine its impact on missed doses.

<sup>30</sup>HHS received funds transferred from the Trust Fund from fiscal years 2011 through 2019 in contrast to PCORI, which began receiving funding in fiscal year 2010, as provided under PPACA.

**Table 5: HHS Obligations for CER Activities, Fiscal Years 2011 through 2019**

Agency and award type	Dollars in millions							Total
	2011 - 2013	2014	2015	2016	2017	2018	2019	
<b>Agency for Healthcare Research and Quality:</b> Dissemination and implementation	36.0	28.0	81.2	72.3	56.8	29.8	46.8	<b>350.9</b>
<b>Agency for Healthcare Research and Quality:</b> Training and career development	13.8	19.9	22.2	22.8	21.1	14.1	4.4	<b>118.3</b>
<b>Assistant Secretary for Planning and Evaluation:</b> Building data capacity	9.8	11.3	20.7	17.3	25.4	16.2	27.5	<b>128.3</b>
<b>Total obligations</b>	<b>59.6</b>	<b>59.2</b>	<b>124.1</b>	<b>112.4</b>	<b>103.3</b>	<b>60.1</b>	<b>78.7</b>	<b>597.5</b>

Source: GAO analysis of Department of Health and Human Services (HHS) information. | GAO-21-61

Notes: HHS obligations in a given year represent a definite commitment of funds that create a legal liability for the payment of goods and services ordered or received, although funds may be expended in subsequent years. Fiscal years 2011 through 2013 were combined because obligations were lower in these years. Dissemination and implementation awards provide funding for developing and publishing systematic reviews of comparative clinical effectiveness research (CER) findings, developing projects focused on implementing CER findings, and developing projects making CER findings more accessible and understandable. Training and career development awards support training in methods used for conducting CER. Building data capacity awards provide funding to projects intended to build data capacity and infrastructure for conducting CER.

Details of AHRQ's and ASPE's awards related to dissemination and implementation, training and career development, and building data capacity are as follows:

- **Dissemination and implementation awards.** For fiscal years 2011 through 2019 AHRQ obligated a total of \$350.9 million—or 59 percent of HHS's obligations—to 39 projects related to CER dissemination and implementation initiatives. AHRQ's CER dissemination and implementation activities include developing and publishing systematic reviews of research findings, translating and communicating research through the development of trainings and tools, and funding projects focused on the implementation of these findings. For example, the AHRQ Evidence-Based Practice Centers conducted and published a systematic review comparing the efficacy of respirators, surgical masks, and cloth masks in preventing COVID-19.<sup>31</sup> The research protocol and related surveillance report for this project were subsequently published through AHRQ's Effective Health Care Program website. According to AHRQ officials, 53 systematic reviews of research findings have been published by AHRQ's Evidence-Based Practice Centers from fiscal year 2017 through 2019, and three of these reviews have included PCORI-funded CER findings.
- **Training and career development awards.** For fiscal years 2011 through 2019 AHRQ obligated about \$118 million—or 20 percent of total obligations—for 11 projects related to training and career development activities. AHRQ's training activities are intended to support training in the methods used to conduct CER. For example, one grant funded a 5-year effort to support the development of patient-centered outcomes research capacity among institutions that were located in geographic areas that currently lack capacity or that primarily serve minority populations.
- **Building data capacity awards.** For fiscal years 2011 through 2019 ASPE obligated a total of \$128.3 million—or 21 percent of total obligations—for 52 projects designed to build data capacity for conducting CER. These projects are managed by ASPE and are primarily carried out by other federal agencies through

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<sup>31</sup>AHRQ's Evidence-Based Practice Center Program awards 5-year contracts to institutions in the United States and Canada to serve as evidence-based practice centers. These centers review relevant scientific literature on various clinical and health services topics to produce evidence reports. Evidence-based practice centers also conduct research and methodology of evidence synthesis.

interagency agreements. For example, the National Institute of Drug Abuse received funding through an interagency agreement with ASPE in fiscal years 2018 and 2019 to conduct two projects that aim to enhance data collection and capacity to conduct patient-centered outcomes research on opioid use disorder. One of these projects, initiated in fiscal year 2019, aims to establish a new practice-based research network and an electronic patient registry to gather standardized data on patients' characteristics, treatments, and outcomes for patients treated with buprenorphine and naltrexone for opioid use disorder in office-based practices. ASPE officials told us that two data capacity building projects have been leveraged to help address COVID-19, such as a project to develop a mobile device application that allows clinical trial researchers to obtain informed consent when face-to-face contact with patients is not possible due to COVID-19.<sup>32</sup>

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## PCORI and HHS Have Taken Steps to Evaluate the Effectiveness of Their Dissemination and Implementation Activities

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### PCORI Has Developed a Framework and Performance Measures to Evaluate the Effectiveness of Its CER Dissemination and Implementation Activities

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Our analysis of documentation shows that PCORI used an evaluation framework with specific performance measures and targets to assess the effectiveness of the institute's dissemination activities and the implementation of its CER findings among providers and patients. PCORI developed and continued to revise these measures after a significant number of research findings were completed in 2017. PCORI's Board of Governors determines which performance measures to use, evaluates PCORI's effectiveness on these measures using a quarterly performance dashboard, and reviews the measures annually to make adjustments

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<sup>32</sup>ASPE officials said that they are funding COVID-19 related projects based on four priorities: (1) leveraging health data and methods; (2) technology and the pandemic; (3) therapeutics and vaccines; and (4) social and medical risk factors.

based on the evolution of its work as more CER is completed.<sup>33</sup> Examples of these performance measures for dissemination and implementation include the following:

- **Dissemination measures.** PCORI conducts scans of research publications and media to track and evaluate its performance on various dissemination measures, including the number of research articles published each year, mentions in various mediums such as in the news media, and the number of presentations made on CER findings. For example, during PCORI's first 10 years, PCORI-funded researchers published 247 articles in peer-reviewed journals with CER findings.<sup>34</sup> In 2019 alone, PCORI-funded researchers published findings from 110 CER studies, and these studies were mentioned over 500 times in the news media, 30 times in blogs, and 3,500 times on Twitter. PCORI also reported attention scores for published research articles that help to describe the extent of their reach.<sup>35</sup>
- **Implementation measures.** PCORI conducts scans of clinical practice guidelines and other clinician or policy guidance to track

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<sup>33</sup>As noted earlier, PCORI's Board of Governors is responsible for carrying out the duties of the institute. Members include the directors of AHRQ and NIH (or their designees) and others appointed by the Comptroller General. Those appointed by the Comptroller General must include representation from a variety of stakeholder groups, including patients, providers, payers and researchers.

PCORI's performance dashboard includes measures, and in many cases specific performance targets, that the Board uses to evaluate the institute's performance on several key activities, such as on funding research and completing peer review within specific time frames, as well as on the dissemination and implementation of its findings.

<sup>34</sup>PCORI reported that a total of 2,191 articles associated with PCORI-funded research were published, which may include publications related to the main CER findings, research on methodology, or other research. As noted earlier, PCORI provides Dissemination and Implementation Awards to researchers to publicize their findings to those stakeholders most likely to use the results. For example, PCORI reports that 60 percent of its funded researchers reported making at least one presentation—totaling over 5,300 presentations—to researchers, clinicians, policymakers, patients or consumers, community organizations, and caregivers through December 2019. PCORI also translates each of the findings into versions appropriate to clinicians, and to patients or consumers, and posts research findings on its public website.

<sup>35</sup>To measure attention to its findings, PCORI uses Altmetric scores to track mentions from various media sources, including news outlets, blogs, and social media. PCORI documents state that Altmetric compiles a weighted score as a measure of attention based on volume (higher scores for more mentions), sources (higher scores for news mentions than for tweets), and authors (higher scores for authors referencing scholarly articles).

and evaluate its performance on various implementation measures, including the number of times CER findings have been incorporated into clinical practice guidelines, clinical decision support (CDS) programs, systematic reviews, and policy documents. For example, PCORI reported that an increasing number of its research findings have been incorporated into a prominent CDS program each year.<sup>36</sup>

In addition, PCORI and some of the researchers funded by PCORI also summarize the effect of CER findings—that is, the extent to which CER findings have affected, or could potentially affect, changes in medical practice, reduced health disparities, or health care spending. Specifically, PCORI has asked researchers to project how their research findings, if implemented, could affect such changes. For example, 2017 CER findings that compared antibiotics for children with respiratory infections—and showed better outcomes for narrow-spectrum antibiotics—have already been included in clinical practice guidelines, were the most viewed findings on the PCORI website, and received significant attention in news or other media sources.<sup>37</sup> PCORI estimated that, if findings from this study were widely implemented, an additional 2.7 million children may receive narrow- rather than broad-spectrum antibiotics. If so, there would be an estimated 35,750 fewer adverse drug reactions, 4,750 fewer emergency department visits, 300 fewer hospitalizations, and 50,530 fewer outpatient visits. They also projected that the implementation of these findings had the potential to save payers (such as insurance plans) \$118 million, Medicaid \$43 million, and patients \$1.9 million each year.

Since fiscal year 2017, the first year PCORI-funded researchers completed a significant number of studies, PCORI officials reported that the institute had met its dissemination and implementation targets for

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<sup>36</sup>As noted earlier, CDS programs provide health care providers and others with knowledge and person-specific information, intelligently filtered or presented at appropriate times, to enhance health and health care. CDS encompasses a variety of tools, such as clinical guidelines, to enhance decision-making in the clinical workflow. PCORI tracks the number of its CER study results that have been incorporated into the UpToDate CDS program; specifically, citations of its CER findings included on its topic pages. The UpToDate website states that over 7,100 physician authors, editors, and reviewers synthesize available medical information into trusted, evidence-based recommendations.

<sup>37</sup>This study found that the use of narrow spectrum antibiotics had better outcomes than broad spectrum antibiotics, and were associated with lower rates of adverse events for patients. Gerber, J., Ross, R., Bryan, M., et al., "Association of Broad- vs Narrow-Spectrum Antibiotics with Treatment Failure, Adverse Events, and Quality of Life in Children with Acute Respiratory Tract Infections," *JAMA*, vol. 318, no. 23 (2017): p. 2325-2336.

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CER findings that were included in the performance dashboard reviewed by its Board of Governors. Table 6 describes PCORI's performance on dissemination and implementation measures and targets that are included on its performance dashboard.



**Table 6: PCORI Dissemination and Implementation Performance Measures for Comparative Clinical Effectiveness Research Findings, Targets and Status, Fiscal Years 2017 through 2019**

Performance measures	Targets	2017 (status)	2018 (status)	2019 (status)	Overall status
<b>Dissemination:</b> Research results page views on PCORI’s website	Average of over 100 results page views per page per quarter  (controlling for increasing results over time)	Not yet tracked	112 average results page views per quarter	167 average results page views per quarter	Met targeted page views
<b>Dissemination:</b> Research findings’ attention scores based on citations in news and online outlets amongst audiences that may influence public policy	More than 10 percent of findings in the top 10th percentile of attention scores each year (controlling for journal and date of publication)	17.1 percent of findings in the top 10th percentile	12.5 percent of findings in top 10th percentile	13.5 percent of findings in top 10th percentile	Met targeted percentile
<b>Implementation:</b> Research findings incorporated into an online clinical decision support (CDS) program	Increasing number of findings included each year	4 findings incorporated	12 findings incorporated	20 findings incorporated	Met targeted increases
<b>Implementation:</b> Research findings cited in systematic reviews, clinical practice guidelines, or other policy documents	Increasing number of citations each year	6 findings cited	28 findings cited	30 findings cited	Met targeted increases

Source: GAO analysis of Patient-Centered Outcomes Research Institute (PCORI) performance measures. | GAO-21-61

Notes: CDS programs provide health care providers and other individuals with knowledge and person-specific information, intelligently filtered or presented at appropriate times, to enhance health and health care. CDS encompasses a variety of tools, such as clinical guidelines, to enhance decision-making in the clinical workflow. Systematic reviews evaluate and synthesize existing research on a clinical issue to compare the effectiveness and harms of different healthcare interventions, and typically include descriptions of the research findings.

Seven of the nine stakeholders we interviewed were generally aware of PCORI's efforts to disseminate information to health care providers and patient advocacy groups, including those most affected by its research. However, officials from three stakeholder organizations—representing patient, health policy and health technology organizations—thought that patients may have difficulty finding CER results that applied to them or suggested making these results easier for patients to find. PCORI officials said that they were in the process of expanding their dissemination activities, including efforts to make CER findings more accessible to patients, as more research continues to be completed.

PCORI officials told us that they plan to conduct a comprehensive evaluation of the effectiveness of the organization's CER activities that would cover the first 10 years of PCORI's work. According to officials, the assessment will use the evaluation framework the institute has developed to evaluate its dissemination and implementation efforts. For example, officials said that PCORI continues to evaluate its dissemination and implementation efforts as described in its evaluation framework, and plans to assess some mid- to long-term metrics as the results from studies have been available for longer periods of time. Officials said that, as more of PCORI's funded research studies are completed, PCORI will be able to place more emphasis on evaluating the dissemination and implementation of the study results. In addition, officials said that they have mainly focused on dissemination performance measures and targets, over which PCORI may exert some control. These officials noted that implementation measures are still important to assess, even though it may be difficult or impossible to credibly attribute implementation changes to PCORI as PCORI does not directly control implementation which must be undertaken by others, such as patients or health care providers. They also said that PCORI is planning to conduct the comprehensive evaluation as part of its strategic planning process for the next 10 years, during which they anticipate reassessing its evaluation framework and performance measures.

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## HHS Has Taken Steps to Evaluate the Effectiveness of Some of Its CER Dissemination and Implementation Activities and Plans a Comprehensive Evaluation in the Future

AHRQ officials told us that they had decided to evaluate some of AHRQ's individual dissemination and implementation activities rather than conduct

a comprehensive evaluation of all of its activities, because AHRQ had completed a large number of disparate activities since 2011 with different objectives and target audiences. AHRQ officials told us that they began to evaluate the effectiveness of some of its individual CER dissemination and implementation activities started after 2014, which include performance measures unique to each activity.<sup>38</sup> For example, one AHRQ activity—Improving Opioid and Pain Management through Interoperable Clinical Decision Support—aims to develop, implement, disseminate, and evaluate CDS for both patients and clinicians related to chronic pain management. AHRQ developed several dissemination-related performance measures for this activity to track its progress, such as the number of implementation guides published on CDS Connect, the number of artifacts (such as computerized alerts) available on CDS Connect, and the number of white papers published and peer-reviewed papers submitted and accepted.<sup>39</sup> In addition, AHRQ officials said that an evaluation of two key dissemination and implementation activities related to CDS using an independent contractor to identify future needs for disseminating CER findings is underway.<sup>40</sup>

In 2019, AHRQ documented the accomplishments of individual CER dissemination and implementation activities in its internal Summary of Accomplishments FY 2011 – 2019 report. AHRQ’s report provides a high-level description of the accomplishments of about 50 individual

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<sup>38</sup>Within HHS, AHRQ has responsibility for CER dissemination and implementation activities.

AHRQ had undertaken a number of projects to disseminate and implement CER since fiscal year 2011, but many projects in the earlier years did not include performance measures. Officials said that most projects conducted prior to 2014 were focused on engaging stakeholders, understanding the CER landscape, and creating evidence tools and methods.

<sup>39</sup>CDS provides health care providers and other individuals with knowledge and person-specific information, intelligently filtered or presented at appropriate times, to enhance health and health care. CDS encompasses a variety of tools, such as clinical guidelines, to enhance decision-making in the clinical workflow. AHRQ awarded a contract to MITRE Corporation to develop CDS Connect, an online platform that includes a repository of CDS artifacts, an authoring tool to enable the creation of CDS, and prototype tools for sharing and testing CDS. CDS Connect uses the term “artifact” to refer to a variety of CDS types or interventions. These interventions include order sets, documentation templates, dashboards, infobuttons, and other functionalities. Artifacts enable CDS by structuring and encoding clinical knowledge so that it can be integrated with electronic health records.

<sup>40</sup>In September 2019, AHRQ awarded a 3-year contract to NORC at the University of Chicago to conduct a comprehensive evaluation related to CDS.

activities—those currently ongoing and completed—and the evaluations associated with many of them. For example, the EvidenceNOW: Advancing Heart Health in Primary Care activity—a \$112 million project intended to accelerate the use of evidence to improve the delivery of primary care and heart health—includes an evaluation that will examine the impact of interventions on practice improvement and the delivery of heart health care. The evaluation will also provide information about which practice support services and quality improvement strategies are most effective in increasing the implementation of new evidence.

AHRQ officials told us that they continue to receive feedback about the value of AHRQ’s CER dissemination and implementation activities and materials from stakeholders—both formally and informally—as required.<sup>41</sup> For example, officials said that, for some of its activities, AHRQ convenes focus groups and advisory panels to assess the needs of stakeholders and determine how best to disseminate and implement CER findings. Officials told us that they also made presentations about AHRQ’s dissemination and implementation activities at conferences—such as PCORI’s Annual Meeting and the National Institutes of Health/Academy Health’s Annual Dissemination and Implementation Conference—where they received feedback on these activities from multiple stakeholders.

Several stakeholders we interviewed suggested that AHRQ could improve its efforts to engage stakeholders, including providers and patients, and thereby raise awareness and further the implementation of CER findings. For example, one of these stakeholders supported AHRQ’s Evidence-Based Practice Centers’ Methods Workgroup’s recommendations to enhance the agency’s dissemination and implementation efforts with health systems, such as developing tools to assist them with applying CER evidence in decision-making processes and partnering with organizations like the American Hospital Association.<sup>42</sup> As we noted earlier, AHRQ officials told us that they will

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<sup>41</sup>AHRQ is required to establish a process to receive feedback from physicians, health care providers, patients, and vendors of health information technology focused on clinical decision support, appropriate professional associations, and federal and private health plans about the value of the information disseminated and the assistance provided. 42 U.S.C. § 299b-37(c). See also [GAO-15-280](#). We previously described AHRQ’s efforts to receive feedback from stakeholders about the agency’s CER dissemination and implementation activities.

<sup>42</sup>For more information about AHRQ’s Evidence-Based Practice Centers’ Methods Workgroup recommendations, see AHRQ, *A Framework for Conceptualizing Evidence Needs of Health Systems*, 18-EHC0014-EF (Rockville, M.D.: December 2017).

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integrate stakeholder engagement efforts into AHRQ's strategic planning process as they continue to plan future dissemination and implementation activities.

Moreover, AHRQ officials told us they are planning a comprehensive evaluation for dissemination and implementation activities funded in 2021 and later as part of AHRQ's new strategic planning process, which would be consistent with findings in our prior work that evaluations can help agencies assess program effectiveness.<sup>43</sup> As of June 2020, AHRQ officials told us that they are planning to set aside funds for this evaluation. Officials told us that the new strategic planning process, including AHRQ's plans for conducting a comprehensive evaluation, is under development. The officials told us that staff and senior leadership are working to determine the goals for AHRQ's CER dissemination and implementation activities as well as an outline for the evaluation framework.

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## Agency Comments

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

We are sending copies of this report to the appropriate congressional committees, the Executive Director of PCORI, the Secretary of HHS, the Director of AHRQ, the Assistant Secretary for ASPE, and other interested parties. In addition, the report will be available at no charge on the GAO website at <http://www.gao.gov>.

If you or your staff have any questions about this report, please contact John Dicken at (202) 512-7114 or [dickenj@gao.gov](mailto:dickenj@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.

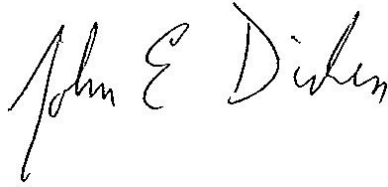
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<sup>43</sup>See GAO, *Program Evaluation: Strategies to Facilitate Agencies' Use of Evaluation in Program Management and Policy Making*, GAO-13-570 (Washington, D.C.: June 26, 2013).

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Letter

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A handwritten signature in black ink that reads "John E. Dicken". The signature is written in a cursive style with a large, prominent "J" and "D".

John Dicken  
Director, Health Care

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# Appendix I: GAO Contact and Staff Acknowledgments

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## GAO Contact

John Dicken, (202) 512-7114 or [dickenj@gao.gov](mailto:dickenj@gao.gov)

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## Staff Acknowledgments

In addition to the contact named above, Michael Zose, Assistant Director; La Sherri Bush, Analyst-in-Charge; Kye Briesath, Justin Cubilo, Laurie Pachter, Vikki Porter, and Jennifer Whitworth made contributions to this report.



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# Appendix II: Accessible Data

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## Data Tables

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### Accessible Data for PCORI and HHS Allocations for Comparative Clinical Effectiveness Research (CER) Activities, Fiscal Years 2010 through 2019

#### PCORI allocated \$2.5 billion for

- Research awards (\$2.0 billion, 79 percent)
- Research infrastructure awards (\$379 million, 15 percent)
- Engagement awards (\$103 million, 4 percent)
- Disseminating and implementing research findings awards (\$60 million, 2 percent)

#### HHS allocated \$0.6 billion for

- Disseminating and implementing research findings (\$351 million, 59 percent)
- Building data capacity (\$128 million, 21 percent)
- Training and career development (\$118 million, 20 percent)

#### PCORI and HHS allocated \$470 million for program support

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