

Right Care, Right Patient, Right Time: Comparative Effectiveness Research in the U.S.

The authorization for the Patient-Centered Outcomes Research Institute (PCORI) is set to expire sometime this year. A decade after the creation of the institute, conversations around comparative effectiveness research (CER), health care value, patient-centered care, and real-world evidence continue. This Alliance toolkit seeks to ensure policymakers are informed about CER and its potential impact by providing the basics of CER, facts on PCORI, and links to additional resources.

The Basics of Comparative Effectiveness Research

Comparative effectiveness research (CER) evaluates and compares the health outcomes and the clinical effectiveness, risks, and benefits of two or more medical treatments or services including health care interventions and procedures, medical devices, and pharmaceuticals.

Theory. CER constitutes a broad **spectrum of methodologies**, including systematic literature reviews, decision analyses, real-world observational studies, and double-blind randomized control trials.

Over time, CER has evolved to evaluate outcomes that matter to patients. The information gained from CER is just one facet or an extension of the literature and evidence around health treatments and services. CER studies are intended to clarify which treatment or service works best for which patient, and at what time, so that **consumers, clinicians, payers, and policymakers can make more informed choices.**

Some examples of CER studies are:

- [PCORI-funded study](#) found that those with type 2 diabetes and not taking insulin can manage their blood sugar levels with A1C monitoring rather than daily blood sugar level tests (which eliminates a painful finger prick and expensive testing supplies).
- [Mathematica study](#) published in *Pain Medicine* found that adjusting the electronic health record default setting for opioid prescriptions increases the number of prescriptions made at that new default amount. This could have an impact on overprescribing rates.

Impact. CER provides an opportunity for diverse patient voices to be integrated into health care research and works to reduce the amount of time it takes for new clinical evidence to be implemented in practice. It also has the potential to inform broader health care policy conversations on value, costs, social determinants of health, and delivery system reform.

Challenges and Trade-Offs. Although the value of CER is widely accepted, there are trade-offs and challenges about how studies are designed and the ways that results can be applied, including:

- Balancing actionable results with making sure they are also impartial and timely;
- Balancing consumer input with the need to standardize outcomes and results;
- Mediating concerns about whether results could be used to limit access to individualized or expensive treatments; and
- Creating policies and payment models that align and support new evidence derived from CER results.

History of CER Legislation. As rising U.S. health care costs became a central topic of reform discussions in the 2000s, experts and stakeholders pointed to CER as one way to support efforts to lower costs and address overutilization.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 is best known for allowing the addition of a prescription drug benefit in the Medicare program, but also marks one of the first times that federal funding was authorized for CER. [Section 1013](#) authorized “research on outcomes of health care items and services.”

The [American Recovery and Reinvestment Act of 2009](#) authorized \$1.1 billion to expand CER efforts in the U.S. Later in 2010, legislative language that authorized the creation and funding of PCORI for ten years was included in the Patient Protection and Affordable Care Act (PPACA).

Patient-centered outcomes research (PCOR) is interrelated with CER and focuses on ensuring that patients are more involved throughout the research process. PCOR places emphasis on allowing patients to provide input on study designs and questions as well as incorporating patient feedback into policy translation.

Patient-Centered Outcomes Research Institute

Purpose. The Patient-Centered Outcomes Research Institute (PCORI) is one of the main entities that conducts CER in the U.S. It was established as an independent, quasigovernmental 501(c)(1) organization in 2010.

PCORI's [statutory purpose](#) is to “assist patients, clinicians, purchasers, and policy-makers in making **informed health decisions** by advancing the quality and relevance of evidence...”

By [statute](#), Medicare cannot rely solely on PCORI's research for coverage or payment decisions, and neither PCORI nor Medicare can use the metric known as quality-adjusted life-years (QALYs).

Funding. A variety of U.S. entities conduct CER but the authorization of PCORI in 2010 established the first steady stream of CER funding.

PCORI is funded through the Patient-Centered Outcomes Research Trust Fund (**PCOR Trust Fund**).

The PCOR Trust Fund receives income from:

- General Treasury fund appropriations;
- Transfers from the Centers for Medicare and Medicaid trust funds; and
- A fee assessed and collected by the IRS on private insurance and self-insured health plans.

To date, PCORI has awarded more than \$2.5 billion for more than 700 research and research-related projects through award commitments to various organizations and individuals. More information about PCORI's financial information can be found [here](#).



Governance. A [Board of Governors](#) oversees PCORI, along with a Methodology Committee and other focus area committees. **The Board is comprised of 21 members from various stakeholder groups**, including consumers, providers, private payers, and pharmaceutical and device manufacturers. Most of the members are appointed by the Comptroller General who directs the Government Accountability Office.

Reauthorization. PCORI's ten-year authorization is currently set to expire on November 21, 2019. Congress passed a continuing resolution ([H.R. 4378](#)) on September 26 that temporarily extends funding levels for several health programs, including PCORI, beyond September 30, 2019.

Both chambers of Congress are considering reauthorization legislation that would extend PCORI funding for several more years. If a reauthorization bill moves to the floor, it is expected to be part of a “health extenders” package that will likely reauthorize several other health programs simultaneously.

Below is a timeline of key legislative activity to date:

- On June 26, Rep. Don Beyer (D-VA) led a [mark-up](#) of the [Ways and Means bill H.R. 3439](#). This legislation would reauthorize PCORI for seven years and make minor changes to research priorities.
- On July 19, Chairman Frank Pallone, Jr. (D-NJ) announced that the [House Committee on Energy and Commerce advanced H.R. 2328](#), which would reauthorize PCORI for three years.
- Senators Bill Cassidy (R-LA), Shelley Moore Capito (R-WV), Mark Warner (D-VA), and Chris Van Hollen (D-MD) are leading reauthorization efforts in the Senate.

Additional Alliance Resources on CER and PCORI

[CER Briefing.](#)

Learn about how researchers conduct studies and the many ways various stakeholders utilize the results.

[Selected Experts](#)

[Selected Resources](#)

[Legislative Outlook.](#)

Learn about some of the critical questions facing congressional staff as they consider reauthorization legislation.

The Alliance for Health Policy is a nonpartisan, not-for-profit organization dedicated to helping policymakers and the public better understand health policy, the root of the nation's health care issues, and the trade-offs posed by various proposals for change. This resource was made possible by a Patient-Centered Outcomes Research Institute Eugene Washington PCORI Engagement Award, but the Alliance does not lobby or advocate for any particular policy position. www.allhealthpolicy.org