



Comparing the Effectiveness of Health Care

Fulfilling the Mission of the Patient-Centered Outcomes Research Institute

By Neera Tanden, Zeke Emanuel, Topher Spiro, Emily Oshima Lee, and Thomas Huelskoetter January 24, 2014

In our current health care system, physicians, insurers, and patients must often choose between several treatments without knowing which works better or whether the higher-priced treatment provides added value. Research that evaluates the effectiveness of two or more prevention, diagnosis, or treatment options—known as comparative effectiveness research, or CER—can address this evidence gap. Funding for CER was an important feature of the Affordable Care Act because this research has the potential to lower health care costs over the long term while maintaining or improving the quality of care, according to the independent Congressional Budget Office, or CBO.¹

The Affordable Care Act created a new independent nonprofit, the Patient-Centered Outcomes Research Institute, or PCORI, to fund and disseminate CER. Our analysis finds that four years into its 10-year existence, the institute has dedicated less than 40 percent of its research funding to CER. Moreover, PCORI has not initiated a single CER study of medical devices, launched only a few CER studies of drugs, and produced only a handful of analyses that synthesize existing CER studies. Few studies focus on the priority areas identified by the Institute of Medicine, or IOM.

If PCORI is to truly fulfill its mission, the Center for American Progress urges the institute to rapidly scale up its investment in CER to at least 80 percent of its research funding by fiscal year 2016. This investment should focus on studies that:

- Address important gaps in evidence on treatments for common and high-cost conditions
- Can produce actionable results in one to three years
- Synthesize existing CER studies

Time is running out for PCORI to make this necessary course correction. New CER studies can take several years, and the institute's funding is more than doubling this year before expiring in 2019.² PCORI did announce a plan recently to launch a new CER funding initiative in the first quarter of 2014.³ This plan is highly encouraging, and the institute should build on it to move further in the right direction.

This rebalancing of PCORI's research priorities will not be easy. CER can threaten the financial interests of powerful stakeholders that profit enormously from marketing new, high-priced products or medical procedures that are marginally or no better than existing alternatives.⁴ But that is precisely why PCORI was designed as an independent nonprofit—so it would be immune to undue political and stakeholder influence. To be truly transformative, the institute must finance a bold research agenda and not play it safe.

The importance of comparative effectiveness research

Research on clinical efficacy evaluates whether a single medical intervention works in clinical trials or laboratory studies.⁵ Private industry and the National Institutes of Health, or NIH, typically conduct these studies to ensure the safety and efficacy of a new drug, medical device, or surgical intervention. This research, however, does not typically compare the effectiveness of medical interventions, and new drugs and devices are not required to be more effective than existing alternatives to gain Food and Drug Administration, or FDA, approval.⁶

By contrast, research on comparative effectiveness evaluates health outcomes, clinical effectiveness, risks, and benefits of two or more medical interventions—prevention, diagnosis, or treatment options—including drugs, medical devices, medical procedures, and delivery methods. For example, Avastin and Lucentis are two drugs used to prevent blindness in older people. Six randomized clinical trials have found that Avastin is just as effective and safe as Lucentis.⁷ But Lucentis is about 40 times more expensive than Avastin, costing Medicare billions of dollars for no added value.⁸

Physicians often practice medicine without knowing the comparative effectiveness of medical interventions. For instance, one of the leading medical textbooks writes that it is not known whether drug treatment works better than removing certain heart cells to treat a common type of heart arrhythmia.⁹ While comparative effectiveness research is conducted in other countries, it is less prevalent in the United States.¹⁰

The purpose of PCORI was to fill this research gap. Section 6301 of the Affordable Care Act, which established the institute, states that its purpose is to advance research and evidence synthesis “with respect to the relative health outcomes, clinical effectiveness, and appropriateness” of two or more medical interventions and expressly provides “funding of comparative clinical effectiveness research.”¹¹

This focus makes sense, since other funding is available for clinical efficacy research through the NIH. In other words, comparative effectiveness research is PCORI’s added value and *raison d’être*. Those of us who were involved in drafting the Affordable Care Act believe this interpretation reflects the statute and intent of policymakers.

Research funding priorities and allocation

Nine independent and governmental organizations have identified key research areas for CER.¹² For example, the IOM identified 100 initial, specific priority topics in 2009 and ranked them by quartile.¹³ (see Table 1)

Section 6301 of the Affordable Care Act also requires PCORI to identify research priorities, taking into account factors such as disease burdens, the potential for improved health, and the effect on national health spending.¹⁴ The institute released its National Priorities for Research and Research Agenda in May 2012.¹⁵ Rather than identifying specific topics for research, however, PCORI identified five broad, cross-cutting areas and funding allocations:

1. Assessment of prevention, diagnosis, and treatment options (40 percent)
2. Improving health care systems (20 percent)
3. Communication and dissemination research (10 percent)
4. Addressing disparities (10 percent)
5. Accelerating patient-centered outcomes research and methodological research (20 percent)

As a result of this prioritization, PCORI is set to spend no more than 40 percent of its research funding on actual CER of prevention, diagnosis, and treatment options. But even within this one priority category, the institute has not allocated all of its funding to CER.

TABLE 1
Institute of Medicine's top CER priorities

Top quartile	Number of corresponding PCORI studies
Compare the effectiveness of treatment strategies for atrial fibrillation	0
Compare the effectiveness of different treatments for hearing loss in children and adults, especially individuals with diverse cultural, language, medical, and developmental backgrounds	0
Compare the effectiveness of primary falls prevention methods versus clinical treatments in preventing falls in older adults	0
Compare the effectiveness of upper endoscopy utilization and frequency for patients with gastroesophageal reflux disease (acid reflux)	0
Comparative effectiveness of dissemination and translation techniques to facilitate the use of CER by patients, clinicians, payers, and others	11
Compare the effectiveness of comprehensive care coordination programs versus usual care for severe chronic disease in adults and children, especially in populations with known health disparities	6
Compare the effectiveness of different strategies of introducing biologics for inflammatory diseases	1
Compare the effectiveness of various screening, prophylaxis, and treatment for eliminating methicillin-resistant <i>Staphylococcus aureus</i> , or MRSA, in communities, institutions and hospitals	0
Compare the effectiveness of various strategies for reducing hospital-acquired infections in children and adults	0
Compare the effectiveness of management strategies versus radiotherapy for localized prostate cancer on survival, recurrence, side effects, quality of life, and costs	1
Establish a prospective registry to compare the effectiveness of low-back-pain-treatment strategies	0
Compare the effectiveness of alternative detection and management strategies for dementia in community-dwelling patients and their caregivers	2
Compare the effectiveness of pharmacologic and nonpharmacologic treatments in managing behavioral disorders in Alzheimer's disease and other dementia patients in home and institutional settings	0
Compare the effectiveness of school-based interventions in preventing and treating obesity in children and adolescents	0
Compare the effectiveness of various strategies to prevent obesity, diabetes, hypertension, and heart disease in at-risk populations	1
Compare the effectiveness of management strategies for ductal carcinoma in situ, or DCIS	1
Compare the effectiveness of imaging technologies in diagnosing, monitoring, and staging cancer patients	3
Compare the effectiveness of genetic and biomarker testing versus usual care in preventing and treating cancers and other conditions for which promising biomarkers exist	0
Compare the effectiveness of various delivery models for preventing dental caries in children	0
Compare the effectiveness of various primary care treatment strategies for ADHD in children	1
Compare the effectiveness of wraparound home and community-based services and residential treatment in managing serious emotional disorders in children and adults	0
Compare the effectiveness of interventions to reduce health disparities in cardiovascular disease, diabetes, cancer, musculoskeletal diseases, and birth outcomes	4
Compare the effectiveness of literacy-sensitive disease-management programs and usual care in reducing disparities in children and adults with low literacy and chronic disease	0
Compare the effectiveness of clinical interventions to reduce incidences of infant mortality, preterm births, and low birth rates, especially among African American women	1
Compare the effectiveness of innovative strategies for preventing unintended pregnancies	2
Total number of corresponding PCORI studies	34
Total number of PCORI studies funded to date	284
Percent of PCORI studies addressing the top quartile of Institute of Medicine priority areas	11.97%

Source: CAP analysis based on Patient-Centered Outcomes Research Institute, "PCORI Funding Awards," available at <http://pfaawards.pcori.org/> (last accessed January 2014); Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research" (2009).

According to our analysis of PCORI's 284 research awards in all categories to date, only 37 percent of its research funding is allocated to studies that compare two or more medical interventions—including studies that compare an intervention to usual care or doing nothing.¹⁶ (see Figure 1) Our results are broadly consistent with an analysis by the California Healthcare Institute.¹⁷

PCORI does plan to invest more in CER in the near future. The institute recently made a preannouncement of a new funding initiative for large-scale CER studies in real-life settings.¹⁸ The new investment is potentially significant: up to \$15 million per project for 12 to 18 projects per year. However, PCORI's total estimated budget is set to more than double from \$320 million in FY 2013 to at least \$650 million annually for FY 2014 through FY 2019.¹⁹ Even if the institute finances the maximum of 18 annual awards at the highest level of \$15 million while maintaining the current CER allocation for remaining funding, the new investment would increase CER funding to roughly 70 percent of its research funding.

Moreover, we find that 12 percent of PCORI-funded studies address one of the top 25 priority topics identified by the IOM. Of those PCORI studies that do address a top 25 priority topic, we find that the majority only address a single topic: research on dissemination and translation techniques to facilitate the use of CER. Arguably, this topic is not even CER, as it does not compare the effectiveness of medical interventions. In fact, there is no PCORI study on nearly half of the top 25 priority topics. There are no PCORI studies, for instance, comparing the effectiveness of school-based interventions to prevent and treat childhood and adolescent obesity, although childhood obesity is one of the top 25 priority topics and widely acknowledged as a major public health concern.²⁰

PCORI's plan for a new CER funding initiative identified seven priority topics.²¹ However, only one of these addresses a top 25 IOM priority topic, and only two of the remaining six address a top 100 IOM priority topic.

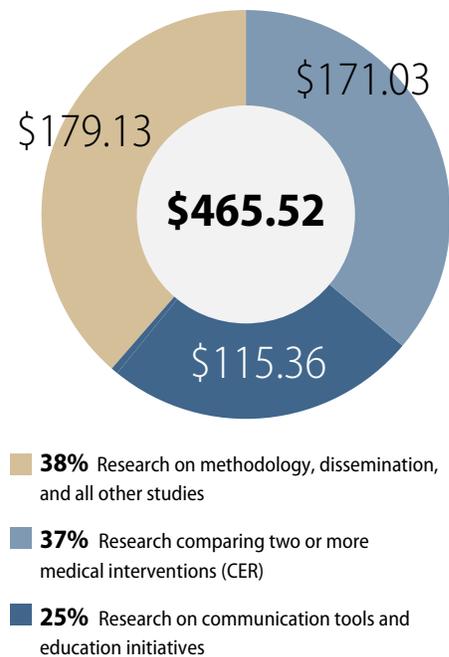
Recommendations to fulfill PCORI's mission

To ensure that PCORI fulfills its mission, the Center for American Progress recommends that the institute do the following:

1. Immediately make an investment plan to boost CER funding to at least 80 percent of its total research funding by FY 2016.

FIGURE 1
PCORI's research funding allocation

Total funding for Cycles I, II, III, and August 2013 awards (as of December 2013) in millions.



Source: CAP analysis based on Patient-Centered Outcomes Research Institute, "PCORI Funding Awards," available at <http://pfaawards.pcori.org/> (last accessed January 2014).

2. Maximize the number of projects and funding per project under its recent plan for a new CER funding initiative.
3. Prioritize CER that addresses the IOM's top 25 priority topics. While PCORI's research can be limited by the focus and quality of research applications submitted, the institute can work with contract research organizations and others to conduct studies on selected topics. Furthermore, targeted funding announcements and out-reach could contribute to a more focused research agenda.
4. Prioritize funding for analyses in the short term that synthesize existing CER studies. For example, a clear synthesis of the studies comparing Avastin and Lucentis might lead private payers and physicians to favor the more cost-effective drug.
5. Make available a comprehensive list of all research grants on its website. This information is currently only accessible state by state through an interactive map.²²

These changes are critical to fulfilling PCORI's mission and improving the health care system. The institute must make this transition quickly because the private insurers, employers, and enrollees contributing to its budget expect actionable results with good reason. PCORI must urgently scale up investments in CER to maximize its potential to lower health care costs over the long term and improve the quality of care.

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Endnotes

- 1 Congressional Budget Office, "Research on the Comparative Effectiveness of Medical Treatments" (2007).
- 2 Patient-Centered Outcomes Research Institute, "How We're Funded," available at <http://www.pcori.org/about-us/how-were-funded/> (last accessed January 2014).
- 3 Patient-Centered Outcomes Research Institute, "Pre-Announcement: Large Pragmatic Trials to Evaluate Comparative Clinical Effectiveness," available at <http://www.pcori.org/funding-opportunities/funding-announcements/pre-announcement-pragmatic-studies-to-evaluate-comparative-clinical-effectiveness/> (last accessed January 2014).
- 4 Stephen S. Hall, "The Cost of Living," *New York Magazine*, October 20, 2013.
- 5 Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research" (2009).
- 6 U.S. Food and Drug Administration, "Development & Approval Process (Drugs)," available at <http://www.fda.gov/drugs/developmentapprovalprocess/> (last accessed January 2014).
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- 10 Kalipso Chalkidou and others, "Comparative Effectiveness Research and Evidence-Based Health Policy: Experience from Four Countries," *Milbank Quarterly* 87 (2) (2009): 339–367.
- 11 *The Patient Protection and Affordable Care Act*, Public Law 148, 111th Cong., 2nd sess. (March 23, 2010), Section 6301.
- 12 Patient-Centered Outcomes Research Institute, "National Priorities for Research and Research Agenda" (2012).
- 13 Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research."
- 14 *The Patient Protection and Affordable Care Act*, Section 6301.
- 15 Patient-Centered Outcomes Research Institute, "National Priorities for Research and Research Agenda."
- 16 The analysis includes Pilot Projects, Cycles I-III, and August 2013 awards through December 2013.
- 17 California Healthcare Institute, "Federally-Funded CER: Where Are We Now?," available at http://www.chi.org/wp-content/uploads/2013/10/131023_avalere_federally_funded_CER.final_.png.
- 18 Patient-Centered Outcomes Research Institute, "Pre-Announcement: Large Pragmatic Trials to Evaluate Comparative Clinical Effectiveness."
- 19 Patient-Centered Outcomes Research Institute, "How We're Funded."
- 20 Institute of Medicine, "Initial National Priorities for Comparative Effectiveness Research."
- 21 The priority topics are: diagnosis and management of bipolar disorder in children, adolescents, and young adults; management of ductal carcinoma in situ; reduction of cardiovascular disease risk in racial and ethnic minorities; strategies for preventing the progression of episodic acute back pain into chronic back pain; treatment strategies for adult patients with migraine headache; treatment strategies for symptomatic osteoarthritis, including joint replacement; and use of antipsychotics in children, adolescents, and young adults.
- 22 Patient-Centered Outcomes Research Institute, "PCORI Funding Awards," available at <http://pfaawards.pcori.org/> (last accessed January 2014).