Breaking the Cycle:
The Uncertain Future of Comparative Effectiveness Research

By
Andrew Iannuzzi

A Master’s Paper submitted to the faculty of the University of North Carolina at Chapel Hill in partial fulfillment of the requirements for the degree of Master of Public Health in the Public Health Leadership Program.

Chapel Hill
2011

Sue Tolleson-Rinehart, PhD, Advisor and First Reader

____________________
Date

John Paul, PhD, Second Reader

____________________
Date
Abstract

The Affordable Care Act founded the Patient-Centered Outcomes Research Institute with the purpose of performing comparative effectiveness research (CER) on treatments for the most common and widespread conditions. Advocates believe the founding of this institute marks a “turning point” for CER (Dentzer 2010), but others disagree, and the road to this point was paved with political speedbumps and stakeholder concerns.

This paper explores the political difficulties faced by CER throughout its history and attempts to elucidate the variables that came together for CER prior to the passage of the Affordable Care Act. The paper uses a search of the gray literature generated by policy stakeholders during the debate about CER, and in-depth interviews with knowledgeable key stakeholders engaged in CER policy.

At present, federal CER activity will be housed in the Patient-Centered Outcomes Research Institute (PCORI). My analysis of the gray literature and synthesis of stakeholder perspectives suggest that CER’s future is cautiously optimistic. On the positive side, proponents of PCORI succeeded in placing it outside of the government agency hierarchy and the appropriations process. On the negative side, CER cannot realize its promise unless PCORI’s work is allowed to be connected to cost and coverage.
Acknowledgements

I would like to thank my advisor and first reader, Dr. Sue Tolleson-Rinehart, PhD, whose insights into health policy and encouragement throughout the research process have been invaluable to my work. I would also like to thank all of the interview respondents who graciously donated their time to my project. These include Mr. Bart Barefoot, Dr. William Roper, Dr. Debra Barksdale, Dr. Peter Hussey, Dr. Gail Wilensky, and Dr. Eugene Rich. Finally, I would like to thank my wife, Jazmin Brown-Iannuzzi, for all of the editing, encouragement and sympathy.
Preface

This paper is meant to be an analysis of the debate surrounding comparative effectiveness research (CER), written from the perspective of a student in medicine and public health. I chose the topic of CER because I believe in the power of scientific evidence broadly and in the push for more evidence-based medicine more specifically. I was originally confused as to why CER had not played a more prominent role in American health care, and I was further flummoxed by the manner in which industry leaders and organizations representing physician interests were not only reluctant but also able to successfully lobby against the funding of federal agencies performing CER. During the course of my research, I came to a better understanding of the opposition’s concern about how CER might be performed and used.
Table of Contents

Abstract ......................................................................................................................................................... ii
Acknowledgements ........................................................................................................................................ iii
Preface ........................................................................................................................................................ iv
List of Tables .................................................................................................................................................. 6
History Behind the Current Debate About CER ......................................................................................... 1
The Role of Stakeholders in the Debate About CER .................................................................................. 3
Methods: Elite Interviews and Gray Literature Review ............................................................................. 4
The Compromises that Made PCORI and its Uncertain Future .................................................................... 7
References .................................................................................................................................................... 15
Appendix 1: Gray Literature Search ........................................................................................................ 18
  Introduction ................................................................................................................................................ 18
  Methods .................................................................................................................................................... 19
  Limitations ............................................................................................................................................... 20
  Results .................................................................................................................................................... 21
    Results of the Gray Literature Search .................................................................................................. 21
    Results of Inductive Coding ................................................................................................................ 22
    Promoting CER but with Careful Guidance ........................................................................................ 24
    The Government’s Role in Promoting CER ......................................................................................... 26
  Table 1: Gray Literature Search Results ................................................................................................ 30
  Table 2: Summary of Themes from Gray Literature Review .................................................................. 31
  Table 3: Coding Examples ..................................................................................................................... 32
  Table 4: Main Themes and Quotations from Gray Literature Review .................................................. 33
  References ................................................................................................................................................. 35
Appendix 2: Interview Respondents and Positions .................................................................................. 37
Appendix 3: Interview Questions ............................................................................................................... 38
List of Tables

Table 1: Gray Literature Results
Table 2: Summary of Themes from Gray Literature Review
Table 3: Coding Examples
Table 4: Main Themes and Quotations from Gray Literature Review
History Behind the Current Debate About CER

It was first cost and then second an understanding of a lack of evidence and that that lack of evidence or a lack of implementation of evidence was driving variations in practice. And that was not improving health outcomes.

- Bart Barefoot, Director of Public Policy, GlaxoSmithKline

Federally supported research into the effectiveness of different treatments began in the late 1980’s with sponsorship from the National Center for Health Services Research (NCHSR), the first predecessor of today’s Agency for Healthcare Quality and Research (AHRQ). This research, however, was limited in scope until stakeholders seized upon the idea that it might limit the rising costs of the American health care system. To that end, one of the most important influences on CER’s rise might be John Wennberg. His research on practice variations suggested the inappropriate use of common surgical procedures and provided the opportunity to link the CER agenda to issues for which “an important constituency already existed on Capitol Hill” (Gray, Gusmano & Collins 2003, 286). Specifically, federal support for CER grew from the concern that geographic variations in medical care were inefficient, unwarranted, and contributing to increasing costs of care (Epstein 1990; Wood 1990; Mitchell & Durenberger 1990, Gray, Gusmano & Collins 2003, Marwick 1990). Proponents of CER suggested that it would inform doctors as to which type of care was most effective for a given disease or condition, thus combatting such inefficiencies (Gornick, Lubitz & Riley, 1991, Mitchell & Durenberger 1990, Wood 1990).

CER’s rise to the national agenda was reflected in the actions of legislators in 1989, who introduced four separate bills suggesting increased funding for effectiveness research (Mitchell & Durenberger 1990). The legislation that eventually emerged, in the Omnibus Budget Reconciliation Act of 1989 (OBRA), transformed the NCHSR into the Agency for Health Care Policy and Research (AHCPR), AHRQ’s immediately proximate predecessor. OBRA gave AHCPR (rather than the weaker center) status within the Department of Health and Human
Services and gave it a larger budget authorization, with $50 million set aside in the first year specifically for effectiveness and guidelines research (Mitchell & Durenberger 1990). OBRA also reflected the expectation that the AHCPR would use its outcomes research to change medical practice (Gray, Gusmano & Collins 2003). In general, this marked the beginning of a successful period for the AHCPR in general and CER specifically in which funding continued to increase from 1991 to 1995 (Gray, Gusmano & Collins 2003).

In 1995, however, the AHCPR nearly lost its funding, when a group of orthopedic surgeons, disturbed by AHCPR’s back pain guideline, almost succeeded in convincing a number of Republicans to “zero out” the agency’s budget. The orthopedists were upset by the guideline’s recommendation against back surgery in most cases of simple back pain, and their goal was to “eliminate funding for the AHCPR and [curtail] the powers of the Food and Drug Administration” (Deyo 1997, 1176). A similar effort was undertaken by other organizations in response to the AHCPR’s research contesting the diagnosis of multiple chemical sensitivity in general and various immunological diagnostic tests specifically.

The efforts of these and similar groups resulted in AHCPR’s inability to continue to develop explicit treatment guidelines (as opposed to “evidence reports,” which the Agency still sponsors). Some three years later, in 1999, the agency changed its name, dropping “policy,” once again in response to Republican congressional distaste for government intervention into treatment recommendations. In the years following AHRQ’s “near death experience,” CER remained on the national agenda to one degree or another. Various proposals included provisions for funding CER, and the Medicare Prescription Drug, Improvement and Modernization Act of 2003 authorized $50 million for AHRQ\(^1\) to perform CER, returning funding to its previous level (108\(^{th}\) Congress). This research, however, was described by some as “limited” in nature (Wilensky 2009, 724), and as the overall costs of health care in the U.S. grew, so did interest in supporting CER (Wilensky 2006).

\(^1\) Of the authorized amount, only $15 million was appropriated for CER in fiscal year 2004 (Wilensky 2006)
The volume of the debate about CER increased significantly when President Obama signed the American Recovery and Reinvestment Act, which set aside $1.1 billion for CER research. In the following weeks and months, various groups spoke out against increased funding for CER. Drug and device developers worried that CER and subsequent changes in reimbursement and coverage would threaten future biomedical innovation (Iglehart 2009). Medical professional groups worried that CER would lead to guidelines that would serve as an imposition between doctor and patient (Gerber, Patashnik et al 2010, Turner 2009). Patient interest groups worried that CER would not take into account heterogeneous response to medications and thus would lead to less individualized care (Jacobson 2007). And conservative pundits saw CER as a stepping stone to the rationing of health care (Avorn 2009). These concerns were marketed and eventually crossed traditional ideological lines to be reflected in less historically conservative groups (Jacobson 2007, Iglehart 2010).

Nevertheless, the Affordable Care Act increased funding for CER, and a new institution – the Patient Centered-Centered Outcomes Research Institute (PCORII) – was established to perform and coordinate CER research. This paper aims to reach a better understanding of how this occurred and how it will affect health care in the future.

The Role of Stakeholders in the Debate About CER

The current approach to research operates within the context of previous public policy theory and practice. At the most basic level, this research is an example of Kingdon’s (1984) theory of multiple-streams of problems, policies and politics flowing independently through political institutions and periods. In this theory, none of the streams necessarily presents a new problem, policy or political situation, but occasionally they merge to provide windows of opportunity in which governments can enact a specific policy to solve a particular problem. I use
Kingdon’s theory to describe the problem of the rising cost of health care, a solution emerging from policy supporting more CER, and the politics of strong support for health care reform and a Democratic majority – historically pro-CER – in both Houses and the Oval Office in one given moment. None of these elements were novel, but their contiguity in this moment presented a window of opportunity for the government to enact policy for CER.

The presentation of this window of opportunity, however, did not mean that legislature enacting a greater role for CER was inevitable. The cards were stacked in favor of Democratic legislators, and CER was a policy they had historically preferred, and yet the shaping and enactment of any public policy is not limited to government officials. In fact, “over the past 10 years scholars have acknowledged a shift in the nature of policy and policy-making, which points to the involvement of a much larger array of actors in the policy process” (Buse, Mays & Walt 2005 as cited in Walt, Shiffman, Schneider et al. 2008, 309). These actors, whether individuals or organizations, are often referred to as stakeholders, and in health policy debates, these stakeholders range from medical professional groups to the pharmaceutical industry and are uniquely placed to influence the policy process because of “their knowledge, technology, access to political processes and stake in life and death issues” (Walt, Shiffman, Schneider et al. 2008, 308).

Methods: Elite Interviews and Gray Literature Review

Given their unique position, policy stakeholders are the focus of both of the methods I have used in this project. Specifically, I undertook an analysis of “gray literature” about CER, defined as “that which is produced on all levels of government, academics, business and industry in print and electronic formats, but which is not controlled by commercial publishers” (New Frontiers 1999). I also interviewed elite stakeholders who are representative of key policy domains.
My gray literature search consists of analysis of reports on CER archived in the New York Academy of Medicine’s Gray Literature Report (http://nyam.waldo.kohalibrary.com/) and through PolicyFile.com. The initial searches returned 74 results, and of those twenty-five met initial inclusion criteria and fourteen were included in the final analysis. Analyzing these reports allowed me to develop, test and refine hypotheses and to establish both who should be interviewed and what questions were most salient to the debate about CER. I also analyzed these data using Aberbach’s and Rockman’s (2002) coding framework. More detailed information on the methods and results of this search are included in Appendix 1: Gray Literature Search.

The purpose of my stakeholder interviews was to “acquire information and context that only that person [could] provide” (Hochschild 2005, 1). I chose the interview respondents to reflect a diversity of viewpoints representative of the various stakeholders in the debate about CER. Background research into this debate mentioned numerous and varied important stakeholders. In light of this diversity of opinions, the structure of the PCORI Board of Governors served as a guidepost for which stakeholders were most commonly thought to be important to this discussion. The twenty-one member governing board for PCORI includes individuals meant to represent consumers, hospitals, industry, nurses, payers, physicians, researchers, surgeons, and two government agencies: the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH).

To reflect these stakeholder views, ten individuals were contacted for interview. Two never replied, one refused to be interviewed, and seven agreed to be interviewed. Unfortunately, I could not resolve a scheduling conflict with one of those seven, but the other six were interviewed between May 17th and June 22nd. All interviews took place over the phone; interviews lasted between 17 and 52 minutes depending on how much time respondents were able to give and what they wished to convey. This information is also presented in Appendix 2:
Interview Respondents and Positions.

The interviews consisted of numerous open-ended questions that elicited the respondents’ opinions on the history of CER, the recent debate about CER and various aspects of the new Patient-Centered Outcomes Research Institute, including how future CER results would be used. I used open-ended questions for three reasons. First, open-ended questions permit responses that are sufficiently focused and broad as to allow for the emergence of previously unknown themes (Brugha & Varvasovszky 2000b). Second, open-ended questions permit respondents to organize their answers within their own frameworks, increasing the validity of their responses (Aberbach & Rockman 2002). Finally, previous research suggests that elite stakeholders are more receptive to open rather than closed-ended questions (Aberbach & Rockman 2002). A full list of the interview questions is included in Appendix 3: Interview Questions.

The UNC IRB judged the interviews to be exempt from further review, and I obtained verbal consent from each participant to record, transcribe, and include their responses in my analysis. One respondent declined to have the interview recorded, but allowed me to take notes, quote her and attribute information both to her name and title. The rest of the respondents agreed to have the interview recorded with full permission to quote and attribute quotations.

Later, I alone transcribed interview recordings into written documents. Each respondent was given the opportunity to review the notes or transcripts of his or her interview and to correct for transcription errors or disallow use of any or all content. In the process of transcription, I opted to use “denaturalized transcripts” in which the “uhhs,” “ums” and repeat words were eliminated in the final transcript (Oliver, Serovich, & Mason 2005). I did this for two reasons: first, I was more interested in the informational content of the interviews than in the speech patterns of the respondents; and second, the interview respondents agreed to be identified by name and title, and I found it more professional to correct those idiosyncrasies of speech that
are less common in written works. Finally, I analyzed the data by identifying the portion of each interview that pertained to the following topics: the history and historical roots of CER, the debate about CER prior to the Affordable Care Act and the use of CER results. Then, I compiled and analyzed the data across all interviews for each topic.

Before offering my conclusions from the research, I would like to clarify the limitations of this kind of study. First, because the study is based on the responses of a small number of individuals, if those individuals are not representative of the population being studied, the results may be biased. Furthermore, this means that the data from which to draw conclusions and with which I develop complex relationships is somewhat limited. Finally, this qualitative research cannot definitively define relationships of cause and effect. To make up for these shortcomings, I attempted to contact a representative sample of stakeholders with a broad array of expertise, and I allowed respondents to develop their own responses to open-ended questions, so as not to limit the type or amount of data collected. I also triangulated my results by using a second method of research, reviewing gray literature reports. This allowed me to test whether and the degree to which results from each method converged on the same answer.

The Compromises that Made PCORI and its Uncertain Future

Support for CER and specifically the establishment of PCORI was very broad…Now that’s not to say that everyone who supported this outcome, PCORI, agreed with everything that ended up being in the final language that was enacted into law. It was undoubtedly a compromise measure as most are as they go through the legislative process.

- Bart Barefoot, Director of Public Policy, GlaxoSmithKline

A broad range of stakeholders agreed on the need for federal support for comparative effectiveness research (CER), but no single individual, group or coalition led the charge (Rich 2009, Bernstein 2009, Orszag 2007, Russo 2009, Pearson 2009). In fact, each of six interview
respondents indicated a different set of stakeholders as the most influential. When asked, their responses included the President and his staff, health economists, clinician groups and even, interestingly, some individuals in favor of limited government. Each of these groups had a slightly different hope for CER beyond the unifying goal of cost containment. Dr. Gail Wilensky, senior fellow at Project Hope, counted herself among the latter group, who envisioned CER as the route to “better information and better incentives as the way to keep some of the direct control by government at bay.” On the other hand, Dr. Debra Barksdale, a member of the PCORI board of governors and Associate Professor at the UNC School of Nursing, believed CER would diminish the level of health disparities by ensuring that all groups of people received the best possible care. The diversity of these goals, as well as differences of opinion regarding the ultimate use of CER, led to numerous compromises during the creation of PCORI which create more questions regarding its future than many proponents would have expected.

In the debate leading up to the Affordable Care Act, proponents of CER originally advertised it as having the potential to reduce the country’s rising health care costs. In fact, drawing on The National Priorities Partnership’s estimation of the cost of medical overuse, proponents were able to put a number to their estimated savings. They claimed that 30 percent of health care spending – $600 to 700 billion – is unnecessary and wasteful (National Priorities Partnership). CER, they argued, could elucidate which treatments were most effective and which were less so.

This claim was not new. In fact, it was originally based on research indicating that medical practice varied between geographically diverse areas (Chassin, Kosecoff et al 1987; Wennberg & Gittelsohn 1982), and more recent research indicated that those geographical differences remained (Song, Skinner et al 2010).² They argued that “expanded research on comparative effectiveness, if linked to changes in incentives for providers and patients, offers a

² They were validated by research that came out two months after the passage of the Affordable Care Act that indicated regional variations had not changed demonstrably in the intervening decades (Song, Skinner et al 2010).
promising mechanism for reducing health care costs to a significant degree over the long term while maintaining or improving the health of Americans” (Orszag 2007a, 20).

Opponents of CER, however, saw any link between federally supported research and potential changes in coverage and reimbursement as problematic. As the Director of Public Policy at GlaxoSmithKline Mr. Bart Barefoot explained “the opposition was primarily one rooted in ideology, of persons and groups who have a concern about the role of the federal government in our daily lives and in particular in the payment for and delivery of health care.” Dr. Gail Wilensky added that the opposition’s fears were enhanced by the events of the time. In her words:

The context in which [CER] came up made it a lightning rod for controversy because it was in a period where not only under health care reform but the whole larger debate about the appropriate role of government was going on… where you had government now involved in the propping up of banks, the automobile industry and various other sectors of the economy that had not previously had direct involvement. [CER] is regarded as one more intrusive or potentially intrusive intervention and therefore might have sparked more controversy than had it been in a different era or just a different time period.

These concerns were also reflected in the gray literature review, in which two authors argued for greater provision of incentives for the private market to perform CER. They posited that “a better way to generate comparative-effectiveness information would be to undo the series of government missteps that suppresses the market's ability to create and use this important research” (Cannon 2009, 12).

In response to these concerns, many proponents changed their message regarding CER, moderating the claim that CER would save money. According to Dr. Eugene Rich, Senior Fellow at Mathematica and Director of its Center on Health Care Effectiveness:

---

3 The two works espousing the view that the private market should be incentivized to carry out CER are (1) Cannon MF. A Better Way to Generate and Use Comparative-Effectiveness Research; Cato Institute. 2009:1-24 and (2) Gottlieb S. Promoting and Using Comparative Research: What Are the Promises and Pitfalls of a New Federal Effort? American Enterprise Institute for Public Policy Research. 2009. I discuss them in greater detail in Appendix 1: Gray Literature Search.
Later on in the debate, as that conversation became so instantly tied to rationing and stoked fears that effective but expensive innovations would be made unavailable by CER, I think the conversation turned toward increasing value, which is sort of harder to complain about.

This change in message is reflected in the legislation for PCORI in two ways. First, PCORI is prohibited from using cost-effectiveness analyses (CEA) in its research. CEA, unlike CER, measures the value per dollar spent of a given treatment using units such as quality-adjusted life years. In truth, incorporating CEA into the work of PCORI was probably not politically feasible. When asked how things might have played out if CEA were included in the legislation, Dr. Gail Wilensky said simply that “it would be even more controversial,” and Dr. Eugene Rich responded tongue-in-cheek. “You couldn’t get 60 senators to vote in favor of rationing.” Echoing these sentiments, Mr. Bart Barefoot felt the separation of CEA and CER was the right choice. Other stakeholders, however, took this bow to political pressure less calmly. One respondent described the prohibition on CEA as “a crazy notion” and claimed that its prohibition “shows the degree to which this whole scientific and technical debate has gotten perverted by politics.”

Regardless of this prohibition, most stakeholders agreed that CEA using PCORI’s research findings would be performed by someone. Mr. Bart Barefoot:

The fact of the matter is that cost is a key factor in health care decision making…The patients have cost data. They can and, we anticipate, will marry the clinical effectiveness data that they get from PCORI with their specific cost data to essentially do a cost-effectiveness analysis.”

Dr. Debra Barksdale and Dr. Gail Wilensky felt similarly, suggesting that although it had no place in PCORI’s agenda, someone would most likely perform the back-end analyses that married PCORI’s work to cost. The degree to which this occurs may have important consequences for how well CER lives up to its promise to lower the rate of health care spending growth.
The second compromise in the legislation for PCORI was its prohibition on the group to make coverage or reimbursement suggestions. This decision, too, can be seen as a response to conservative fears over rationing, though it was also spurred by the dilemma faced by AHCPR in the mid 1990’s. Much like with the separation of CEA and CER, though, many stakeholders see the use of PCORI research to make coverage and reimbursement decisions as inevitable. In this research, five of six interview respondents and eight of fourteen gray literature reports agreed that such changes were likely. Dr. William Roper, for one, welcomed these changes, saying:

That’s what’s up for grabs. People are diving under the table and saying, “we will never ration health care,” and I just laugh when they say that. Of course we will ration care. I hope we ration it even more than we already are, but we have for decades rationed care. I think it would be much better if we rationed it in public, driven by evidence, and [CER] is the best tool for rationing that we have.

Furthermore, many stakeholders hold to the view that tying CER to changes in incentives, reimbursement or coverage is the best way to ensure that health care costs go down (Orszag 2007), which was the initial driving force behind CER proposals.

The final compromise in the CER legislation deals with its ongoing engagement with the stakeholder community. PCORI’s twenty-one member Board of Governors\(^5\) includes representatives from each stakeholder group. It replaces the Federal Coordinating Council for Comparative Effectiveness Research, an intra-governmental entity established under the American Recovery and Reinvestment Act, which got caught up in antigovernment rhetoric (Rich & Docteur 2010). According to Mr. Bart Barefoot:

The structure really is the only way that Congress could have gone to actually establish this organization. To have all the key stakeholders around the table to be able to express their voice, to provide their expertise, to listen, to share concerns, to bring their various perspectives, I think it was an absolute necessity to have a broad-based, broadly representative board for the creation of PCORI.

---

\(^4\) For more on the fate of AHCPR see above Introduction: History Behind the Current Debate About CER. 

\(^5\) Duties of the Board of Governors include are described as follows “carrying out research projects that provide quality, relevant evidence on how diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed” (GAO)
Though meant to capture broad stakeholder support, Dr. Eugene Rich suggested that it might not be achieving its aim. “One certainly has continued to see a lot of political positioning around comparative effectiveness research.” In a similar manner, PCORI’s activities are designed to be inclusive so as to maintain broad support. According to Dr. Debra Barksdale, those activities have thus far included numerous public meetings in cities ranging from Washington D.C. to Los Angeles, with live webcast and support for public call-in or write-in questions. Furthermore, all decisions are open to public comment for 45 days by statute. These efforts, she hopes, will “capture stakeholder support” and legitimize PCORI’s results.

In total, these legislative compromises may reflect the only manner in which proponents could achieve the realization of a new entity to perform CER. However, they also indicate that PCORI’s future has many questions. On the positive side, proponents of PCORI succeeded in placing it outside of the government agency hierarchy and the appropriations process. This placement buffers PCORI from the political vicissitudes that influenced the fate of AHCPR. Yet, among the stakeholders interviewed, many questioned PCORI’s funding. Its budget for 2010-2012 is $120 million reaching an annual amount of nearly $500 million by 2015. Dr. Gail Wilensky criticized the funding as “not close” to what was needed, especially in comparison to the roughly $30 billion per year the National Institute of Health receives for biomedical science research. Peter Hussey, a Policy Researcher at RAND, agreed that PCORI was underfunded, and he went a step further, suggesting that PCORI may not last.

We’ll see how the program plays out, but it’s under so much scrutiny. The funding level is probably too low. It’s probably too prescriptive already. It’s probably one of the first

---

6 For more on the fate of AHCPR see above Introduction: History Behind the Current Debate About CER.
7 The Institute will be funded through the Patient-Centered Outcomes Research Trust Fund (PCORTF), which will consist of the following funding streams: for 2010-2012, PCORI funding will amount to $210 million from general revenues; for 2013, PCORI funding will be general revenues of $150 million plus an annual $1 fee per Medicare beneficiary transferred from the Medicare Trust Fund plus a $1 fee per individual assessed on private health plans; for 2014-2019, PCORI funding will be general revenues of $150 million plus a $2 fee per Medicare beneficiary plus a $2 fee per privately insured individual. By 2015, total annual funding for the Institute will reach nearly $500 million (Patient-Centered Outcomes Research Institute 2010).
targets – especially if they come out with anything slightly controversial – that’ll get slashed.

This possibility is worrisome for proponents of CER and supporters of PCORI. Though, even if it did not get cut, many stakeholders fear that PCORI will not make a discernible difference to the way health care is delivered. Dr. William Roper worried that without specific connections between PCORI’s research and changes in coverage, reimbursement or incentives little would change. He cited the outcries to changes in mammography guidelines as indicative of the public’s refusal to be guided by expert opinion. Dr. Peter Hussey, on the other hand, suggested that even if PCORI’s research does affect coverage and reimbursement decisions, it probably would not have significant effects on spending, access, or quality of care delivered.

However, not all of stakeholder opinion is pessimistic, and the establishment of PCORI may indicate a shift in public and political acceptance of CER. Dr. William Roper, for one, sees PCORI as a measure of CER’s growing legitimacy in the stakeholder arena. “The notion of using information that’s available to measure results and try to change and improve in the health care system was a pretty fragile notion then [in the late 1980’s], but a much more robust notion today.” Similarly, Mr. Bart Barefoot sees the funding of PCORI as merely “seed money” along the route to a more “comprehensive CER enterprise that will take hold and over time become …what we call a learning health care system.”

Mr. Bart Barefoot is joined by numerous stakeholders in the belief that PCORI represents just a step in a larger process (Conway & Clancy 2009), but where this path leads and how PCORI will answer the numerous questions surrounding its goals and activities remain to be seen. How it defines CER, what is included in its research portfolio, what methods it adopts, and how research findings are disseminated will all affect whether PCORI is a short-

---

8 In reference to the public’s reaction to changes in mammography guidelines, Roper commented that “they don’t care what experts say, and they’re not going to be guided by experts”
lived entity hampered by controversy or a positive step toward a more comprehensive and evidenced-based health care system.
References


Appendix 1: Gray Literature Search

Introduction

I performed a gray literature search for CER and a content analysis on the results in order to better ascertain how CER was addressed by stakeholder organizations during the lead up to the Affordable Care Act. I hoped to determine which themes and messages were most commonly cited in the discussion of CER. This goal was heavily influenced by policy literature suggesting that the voice of elite stakeholders, as opposed to the voice of the public, would shape the debate about CER. In describing this concept, Elmer Eric Schattschneider explained that those groups with high levels of support and resources held the greatest public influence. In his words, "the flaw in the pluralist heaven is that the heavenly chorus sings with a strong upper-class accent." (Schattschneider 1960, 35). This general concept is no less true now than when it was written. The elite have the most influence. This is especially true in debates over health care policies in which the stakeholders, ranging from medical professional groups to the pharmaceutical industry, have greater “knowledge, technology, access to political processes and stake in life and death issues” than does the public or other influences (Walt, Shiffman, Schneider et al 2008, 308).

Because various stakeholders do not always agree, each attempts to shape the debate about health policy by controlling the messages and framework within which policy debates occur. Stakeholder organizations do so because “one’s frame in thought can have a marked impact on one’s overall opinion.” Thus, they attempt to garner the public and policy makers’ support “by encouraging them to think about those policies along particular lines,” and they

---

9 Searching gray literature for references to CER was originally meant to serve two purposes: to understand the frameworks within which stakeholder groups talked about CER and also to show which organizations were most actively discussing CER prior to the passage of the Affordable Care Act. Unfortunately, the search returned a more limited set of results than would be necessary to properly map which organizations were most actively addressing the debate about CER. Therefore, the latter goal was not achieved.
accomplish this “by highlighting certain features of the policy, such as its likely effects or its relationship to important values” (Chong & Druckman 2007, 106). The goal of this literature review, then, is to better understand which messages, themes and frames the elite stakeholders employed to shape the debate about CER.

Methods

To develop a search strategy, I consulted with the University of North Carolina’s Health Sciences Library staff, who suggested that I use the following databases: the New York Academy of Medicine, PolicyFile, and the National Library of Medicine Gateway. Into each of these databases, I entered the following input as a keyword “comparative effectiveness research”. Results were limited to those published or released after 2006, but prior to the passage of the Affordable Care Act on March 23rd 2010 so that results reflected the most current ideas and themes surrounding CER. Books and journal or periodical articles were excluded from review, and only results published in English were accepted.

The first step in reviewing these reports was a title assessment. Those titles that indicated a report that clearly did not address the policy of CER (as opposed to actual CER research) or that clearly did not focus on the American health care system were excluded. When titles alone did not conclusively answer these questions, I kept the report for further review. The abstracts or summaries of the remaining reports were then reviewed with the same criteria. At this point, those reports that were not available were also excluded.

_____________________

10 An example of an article not included based on the title alone would be “Is employer-based health insurance a barrier to entrepreneurship?”.
Limitations

Systematically reviewing gray literature for authors’ and organizations’ opinions on CER has several limitations. First and foremost, the literature is not easily available. Few engines cater specifically to gray literature searches of medical and health services topics, and those that do are often incomplete. More general internet search engines (e.g. Google, Yahoo, etc.) could have been incorporated to supplement the original search strategies, but time constraints limited my ability to sift through the results of such searches. Second, the overlap of some authors and organizations in this specific search (Peter Orszag wrote two of the reports and two were published in a larger pamphlet published by the Engelberg Center for Health Care Reform at Brookings jointly with The Hamilton Project) may have biased the results of the review. Finally, the use of reports to generate more broad conclusions regarding stakeholder sentiment toward CER misses those opinions promoted by stakeholders who are not seeking to publish their ideas but rather are working through different channels (e.g. lobbying, advocating publicly).

Nevertheless, the systematic review does offer some insight into the question, “How were stakeholders addressing and framing the debate about CER prior to the passage of the Affordable Care Act?” Generally, results indicate a more positive than negative outlook toward the potential of CER to improve care and help contain the growing costs of health care in the United States. In the following sections, I will discuss in greater depth the results of the search and coding of the literature. In greater detail, I will discuss the positive and negative aspects of CER according to the gray literature as well as two of the more contentious questions of the debate: what should the government’s role be in promoting CER and how will results affect reimbursement and coverage decisions.
Results

Results of the Gray Literature Search

The first search was performed using the New York Academy of Medicine Gateway (http://nyam.waldo.kohalibrary.com/) on 4-7-2011. I entered “comparative effectiveness research” into the engine as a single keyword. This search yielded 62 total returns. Of those 62 returns, 43 were excluded based on their titles or their indicated date of publication, leaving 19 results for abstract review. Of these 19 reports, 3 did not pertain to CER, 2 were books, and 1 was unavailable which left 13 reports for review.

The second search used the search engine on PolicyFile (www.policyfile.com) and took place on 4-7-2011. I entered “comparative effectiveness research” into the search engine as a single keyword, and limited results to those between 1-1-2006 and 3-23-2010. This strategy yielded 12 results. Of these results, all were available and pertained to CER policy. Review of abstracts indicated that 6 results were duplicates of results found in previous searches, 1 was merely a pointer to another article revealed in the search, and 1 was a summary of a previously returned report, leaving only four results from the PolicyFile search for further review.

My third search was of the data in the National Library of Medicine Gateway. I performed it on 3-17-2011, and my strategy, although ultimately unsuccessful, was meant to identify gray literature. Using “comparative effectiveness research” as a keyword, this database returned 18893 results, including 16986 journal citations and abstracts, 269 books, AVs and serials, 1446 biomedical books, 190 toxicology citations, 1 developmental and reproductive toxicology report and 1 meeting abstract. Of these many results, the meeting abstract was the only potential report for inclusion, but review of the abstract revealed that the meeting took place prior to 2006.

In total, the three searches yielded 17 reports for full review. I read the results to assure that they were pertinent to CER policymaking; and this step enabled me to eliminate three additional reports from the final analysis. One discussed CER’s use in other countries, another
was limited to CER in the state of Massachusetts, and the last discussed only the role of cost or cost effectiveness in comparative effectiveness research. The final analysis included fourteen reports from a variety of sources. The majority of the reports were written by non-governmental organizations with a history of interest in public health policy (e.g. Robert Wood Johnson Foundation, The Henry J. Kaiser Family Foundation); two results were published by the Congressional Budget Office. Results of the searches are presented in Table 1.

After assessing each result for appropriateness to the topic, I reread each report in order to develop a coding strategy. I decided to use a thematic content analysis strategy because it allowed me to go beyond merely counting words or extracting objective content from texts but rather to examine the meanings, themes and patterns present (Zhang & Wildemuth 2009; Macnamara 2006). Thus, I derived coding themes directly and inductively from the raw data (Zhang & Wildemuth 2009; Hsieh & Shannon 2005; Chong & Druckman 2007). I analyzed and coded results for the presence or absence of the themes listed below and for the main theme of the report.

Results of Inductive Coding

For the first category, presence or absence of particular themes, the document must clearly espouse that view to be counted as present, not just mention it as the argument of the opposition. The themes that emerged from the data included the following:

(1) That CER will lower the costs of the American health care system or will increase the “efficiency” of the health care system

(2) That CER will improve upon the problem of geographic variation and/or unnecessary treatments

(3) That CER will increase doctor’s knowledge or will improve patient care

(4) That CER will change insurance companies’ coverage or reimbursement of certain drugs
a. That changes in coverage and reimbursement may limit drug availability for some populations, harming some who would otherwise benefit from those drugs

(5) That CER will hurt future drug and device development

The results of coding for the presence or absence of specific themes are presented in Table 2, in which a “1” indicates the presence of that theme and a blank indicates the absence of that theme (see Table 3 for examples of text indicating a given theme). The summated results and percentage of reports endorsing each theme are presented in the bottom row of the table.

The first three themes treat CER positively, and the last two themes frame CER in a negative light. The fourth theme – that CER would change reimbursement or coverage of certain drugs or devices – can be either a positive or negative aspect of CER, so it was further qualified by whether or not the authors suggested such a change would hurt a subgroup of individuals for whom the limited drug was in fact beneficial. This sub-categorization was meant to help distinguish those reports in which the author was making the argument that limiting drugs would be necessary to cost-containment, and thus a positive, as opposed to those authors who suggested limited availability to be a detriment to the health of patients. Finally, the results of coding for the main theme or idea of each report are presented in Table 4 and are accompanied by one or more quotations that support my conclusions as to the main idea of that report. Ideally, coding would also be performed by a second social science researcher whose results would be used to judge the validity, reliability and reproducibility of the coding scheme. Unfortunately, given limited time and resources, the use of a second reader was not possible by the time of this paper’s production.
Promoting CER but with Careful Guidance

The first three, pro-CER themes were endorsed most frequently, with the single most common being that CER would be generally beneficial to patient care by helping providers and patients to choose among treatment options (86% of reports). The next most frequent theme was that CER would improve upon the inefficiencies or lower the costs of the current health care system (79% of reports). Finally, the third pro-CER concept, that CER would reduce geographical variation, was endorsed by eight of fourteen (57%) of reports.

Among the fourteen reports, half endorsed all three of the pro-CER themes. Furthermore, of those reports in which the first three, pro-CER themes were present, only one also endorsed either of the two clearly con-CER themes. This homogeneity suggests that CER became a highly polarized issue among stakeholder organizations. More importantly, the fact that the pro-CER themes were this common would suggest that a large proportion of the organizations producing reports believed increasing the role of CER in the health care system would be beneficial.

The most common main idea of the seemingly pro-CER reports was that CER had a lot of potential, but that realizing this potential would require careful guidance and implementation of CER studies and results. This concept is summed up best by McClellan and Benner in their report for the Engelberg Center for Health Care Reform at Brookings and The Hamilton Project. “For CER to make a substantial, positive contribution to reforming health care, the critical implementation issues of prioritizing CER spending to “high-value” studies, creating an efficient research infrastructure, and creating an environment that promotes the effective use of evidence from CER must be addressed” (2009, 14).

Similar sentiments were expressed by each of the other pro-CER reports and by some of the reports that were not as clearly in favor of the promotion of CER. For example, the reports
published by the Institute of Health Economics (IHE)\textsuperscript{11} and by the New England Healthcare Institute (NEHI)\textsuperscript{12} were more moderate in tone and seemed to be conglomerates of information produced by other sources. To wit, the report by the IHE relied heavily upon other reports published by the Congressional Budget Office, the Agency for Healthcare Research and Quality and by authors in the field of health policy. Similarly, the report by the NEHI derived from interviews and roundtable discussions with expert stakeholders, and it focused specifically on how CER would affect biomedical innovation. The author’s stance toward CER in these papers was less apparent than was the stance taken by other authors; however, they nonetheless drive home the message that any advancement of CER must be accompanied by careful oversight. In fact, of the fourteen reports, five have this or a similar message as the main theme of their report.

Finally, negativity toward CER was rare. Only the report published by the National Bureau of Economic Research\textsuperscript{13} was truly negative toward CER as a concept. In this report, the authors performed economic analyses by modeling demand shifts and treatment effects in public and private insurance markets based on changes in coverage due to CER. Their analyses suggested that in our current market system CER might adversely affect both patient health and spending on health care. Again, though, the authors point out that with care and consideration, CER may have a role to play in future health care endeavors. In their words, “simplistic thinking about the impact of traditionally perceived CER may have adverse effects. However, this does not mean that CER may not have a useful role to play and that good forms of CER should not take place” (Basu & Philipson 2010, 28). Therefore, the reports seem to indicate a broad support for CER so long as it is accompanied by careful oversight.

The Government's Role in Promoting CER

The role the government should play in promoting CER was one of the more contentious questions in the recent debate. This question may seem to be late happening after the fact, considering that the government passed the American Recovery and Reinvestment Act which put $1.1 billion into supporting future CER. However, with the looming health care reform offering the possibilities of more money for CER and maybe even a new CER entity, this debate continued to hold center stage for many organizations. In the results of this systematic review, seven reports discussed the role the government should play in CER. Five endorsed government promotion of CER to one degree or another\textsuperscript{14}, and two just as firmly insisted that CER should be performed by private organizations\textsuperscript{15}.

Many reasons were given for why the government should take a larger role in the promotion of CER. In his report detailing options for expanding the role of CER, Peter Orszag, director of the Congressional Budget Office, argued that the private market was not incentivized to produce CER given that it was costly to produce and easily disseminated. In his words, a private entity would have only “a limited incentive to produce information that could benefit many entities” (2007, 3) and thus would “probably not produce as much research on comparative

\textsuperscript{14} The following articles endorsed the government’s support or funding of a new CER entity:


\textsuperscript{15} Those who supported allowing the private market to perform and distribute CER findings included:

effectiveness as society would value” (2007, 6). In *The Facts about Comparative Effectiveness Research*, Jeffrey Bernstein explained that the private market performers of CER are funded by drug and device manufacturers, which leads them to produce biased results. He posited that “CER [should] be funded by neutral parties who do not have an economic interest in the result,” which necessitated that “the federal government should expand its funding and support for comparative effectiveness research” (2009, 2). Finally, Michael Russo applauded individual efforts to produce CER, but saw them as lacking in the coordination that a federal entity could provide. “The country’s ailing health care system lacks a coordinated, national effort to support comparative effectiveness research aimed at discovering which treatments work best” (2009, 12-13). Taken together, the reports offered ethical and logistical arguments for why the government should support CER.

In opposition, stakeholders who were against government support of a new CER entity believed that market forces could provide the same information more efficiently. In his article *A Better Way to Generate and Use Comparative-Effectiveness Research*, Michael Cannon postulated that a federal CER agency would lead to rationing, and that the government produces ineffective agencies. In his words, “a better way to generate comparative-effectiveness information would be to undo the series of government missteps that suppresses the market’s ability to create and use this important research” (2009, 12). Similarly, Scott Gottlieb argues that “like many other seductively simple ideas, enthusiasm for comparative effectiveness research (CER) outpaces its practical promise and obscures the downside of having governments take on these sorts of studies and the clinical considerations that go into them” (2009, 1). More specifically, he argues that the government’s track record with CER, seen through the ALLHAT and CATIE trials, indicates a propensity to make coverage decisions without rigorous evidence. Instead, Gottlieb argues that efforts be made to “leverage CER being

---

16 According to Scott Gottlieb, these are two large trials in which the researchers came to the conclusion, prematurely, that newer, more expensive drugs were no better than existing medicines. In each case, the initial conclusion was reviewed and changed.
done by private groups, as well as to create more incentives for companies to undertake this kind of scientific work” (2009, 5) before we end up “squarely on a path that more closely resembles the process used in Britain—with all its shortcomings on access, innovation, and health outcomes” (2009, 7). Taken together, the reports offer arguments for greater efficiency and against misuse of the information as reasons to keep the government from having an active role in promoting CER.

In conclusion, the view that the government should support ongoing and future efforts to produce CER was more popular than was the view that the private market should furnish such information. Furthermore, many reports left this question unremarked, instead treating federal support of CER as a given (Balancing Act 2009; Explaining Health Reform 2009). The most important question among these reports, then, was where any new CER entity should be placed. Bills proposed by Congressional leaders alternatively placed such an entity within the purview of AHRQ or outside of the existing structure, to function as a non-profit institute governed by a multi-stakeholder board. However, this issue was not widely discussed in the reports.

**CER and Coverage Decisions: What Would Changes Mean?**

Many stakeholders believe that CER will be used to change coverage and reimbursement for certain drugs, devices and procedures. However, whether this is a positive or a negative is widely debated, and in this review the issue remained contentious. Eight of fourteen reports believed that CER results would be used to change coverage or reimbursement of certain treatments. Furthermore, half of those eight believed that such changes would harm patients by limiting their access to effective drugs, while half did not endorse this belief.

Those reports endorsing the belief that CER might lead to harming patients suggested that results would not adequately take into account the heterogeneity in patients’ response to treatments. They posited that “limits on access to new medical products that are based on
assumptions about the cost to a population will also deny access to individuals who can nonetheless benefit from the medical product” (Gottlieb 2009, 6). These opponents often strengthened their rhetorical argument by likening it to the “rationing” of health care (Cannon 2009), a stance promoted by Republican leaders and conservative pundits (Iglehart 2010). Framing the argument around the concept of rationing allowed these authors to play on extant popular sentiment and increase public concern over the use of CER results.

Proponents of allowing CER to direct changes in coverage and reimbursement, on the other hand, suggested that CER would actually ensure that patients were receiving the best medical treatment available. They proposed that many treatments currently have no evidentiary basis (Docteur & Berenson 2010; Orszag 2007a), and that changes in reimbursement or coverage would only reflect greater certainty that one treatment performed better than another. Furthermore, they suggested that “expanded research on comparative effectiveness, if linked to changes in incentives for providers and patients, offers a promising mechanism for reducing health care costs to a significant degree over the long term while maintaining or improving the health of Americans” (Orszag 2007a, 20). These reports, however, are split down the middle, leading to no simple conclusion as to how CER results will be used or whether they will produce the harms feared or benefits hoped for by the various stakeholders.
### Table 1: Gray Literature Search Results

<table>
<thead>
<tr>
<th>Search Engine</th>
<th>Date of Search</th>
<th>Search Input</th>
<th>Returned Articles</th>
<th>Met Title Requirements</th>
<th>Met Abstract Requirements</th>
<th>Included in Final Analysis</th>
</tr>
</thead>
<tbody>
<tr>
<td>New York Academy of Medicine Gateway</td>
<td>4-7-2011</td>
<td>&quot;Comparative Effectiveness Research&quot;</td>
<td>62</td>
<td>43</td>
<td>19</td>
<td>10</td>
</tr>
<tr>
<td>Policy File</td>
<td>4-7-2011</td>
<td>&quot;Comparative Effectiveness Research&quot;</td>
<td>12</td>
<td>12</td>
<td>6</td>
<td>4</td>
</tr>
<tr>
<td>National Library of Medicine Gateway</td>
<td>3-17-2011</td>
<td>&quot;Comparative Effectiveness Research&quot;</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>TOTAL: 14</td>
</tr>
</tbody>
</table>

*Note that reports were limited to those which (1) were published between 1/1/2006 and 3/23/2010, (2) were considered “gray literature” (i.e. not journal articles, periodical articles or books, (3) were focused on the policy of CER.

For Tables 2 and 4, organizations are abbreviated as follows:

<table>
<thead>
<tr>
<th>Families</th>
<th>Families USA</th>
</tr>
</thead>
<tbody>
<tr>
<td>HJKFF</td>
<td>Henry J. Kaiser Family Foundation</td>
</tr>
<tr>
<td>RWJF</td>
<td>Robert Wood Johnson Foundation</td>
</tr>
<tr>
<td>US PIRG EF</td>
<td>United States Public Interest Research Group, Education Fund</td>
</tr>
<tr>
<td>NBER</td>
<td>National Bureau of Economic Research</td>
</tr>
<tr>
<td>Cato</td>
<td>Cato Institute</td>
</tr>
<tr>
<td>AEI</td>
<td>American Enterprise Institute for Public Policy Research</td>
</tr>
<tr>
<td>CBOa</td>
<td>Congressional Budget Organization (Research on… 2007)</td>
</tr>
<tr>
<td>IHE</td>
<td>Institute of Health Economics</td>
</tr>
<tr>
<td>NEHI</td>
<td>New England Healthcare Institute</td>
</tr>
<tr>
<td>US PIRG FS</td>
<td>United States Public Interest Research Group, Federation of State PIRGs</td>
</tr>
<tr>
<td>CBOb</td>
<td>Congressional Budget Office (Letter to… 2007)</td>
</tr>
<tr>
<td>ECHCR &amp; HPa</td>
<td>Engelberg Center for Health Care Reform at Brookings; The Hamilton Project (McClellan &amp; Benner)</td>
</tr>
<tr>
<td>ECHCR &amp; HPb</td>
<td>Engelberg Center for Health Care Reform at Brookings; The Hamilton Project (Pearson)</td>
</tr>
<tr>
<td>Organization Associated with the Report</td>
<td>CER increases health system efficiency / lowers health care costs</td>
</tr>
<tr>
<td>----------------------------------------</td>
<td>---------------------------------------------------------------</td>
</tr>
<tr>
<td>Families</td>
<td></td>
</tr>
<tr>
<td>HJKFF</td>
<td>1</td>
</tr>
<tr>
<td>RWJF</td>
<td>1</td>
</tr>
<tr>
<td>US PIRG EF</td>
<td>1</td>
</tr>
<tr>
<td>NBER</td>
<td></td>
</tr>
<tr>
<td>Cato</td>
<td>1</td>
</tr>
<tr>
<td>AEI</td>
<td></td>
</tr>
<tr>
<td>CBOa</td>
<td>1</td>
</tr>
<tr>
<td>IHE</td>
<td>1</td>
</tr>
<tr>
<td>NEHI</td>
<td>1</td>
</tr>
<tr>
<td>US PIRG FS</td>
<td>1</td>
</tr>
<tr>
<td>CBOb</td>
<td>1</td>
</tr>
<tr>
<td>ECHCR &amp; HPa</td>
<td>1</td>
</tr>
<tr>
<td>ECHCR &amp; HPb</td>
<td>1</td>
</tr>
<tr>
<td><strong>Total reports with that theme</strong></td>
<td><strong>11 (79%)</strong></td>
</tr>
</tbody>
</table>
### Table 3: Coding Examples

<table>
<thead>
<tr>
<th>Theme</th>
<th>Example Text 1</th>
<th>Example Text 2</th>
</tr>
</thead>
<tbody>
<tr>
<td>That CER will lower the costs of the American health care system or will improve “efficiency” of the health care system</td>
<td>&quot;Identifying the most effective and efficient interventions has the potential to reduce unnecessary treatments, which in turn, may help lower costs.&quot; (<a href="#">Explaining</a> 2009, 1)</td>
<td>&quot;Expanded research on comparative effectiveness, if linked to changes in incentives for providers and patients, offers a promising mechanism for reducing health care costs to a significant degree over the long term while maintaining or improving the health of Americans.&quot; (<a href="#">Orszag</a> 2007, 20)</td>
</tr>
<tr>
<td>That CER will improve upon the problem of geographic variation and/or unnecessary treatment</td>
<td>&quot;Across the health care system, there is consensus that better clinical evidence is needed to support decision making at the point of care to reduce variation and promote health care quality. This has generated wide support for clinical effectiveness studies.&quot; (<a href="#">New England Healthcare Institute</a> 2009, 10)</td>
<td>&quot;To improve patient care and reduce the costs of unnecessary and improper treatment, the federal government should expand its funding and support for comparative effectiveness research.&quot; (<a href="#">Bernstein</a> 2009, 2)</td>
</tr>
<tr>
<td>That CER will increase doctor’s knowledge of treatment decisions or will improve patient care</td>
<td>&quot;To improve patient care and reduce the costs of unnecessary and improper treatment, the federal government should expand its funding and support for comparative effectiveness research.&quot; (<a href="#">Bernstein</a> 2009, 2)</td>
<td>&quot;compelling evidence will lead patients and their doctors to make better choices among various medical treatments, leading to better outcomes and in cases where an equally or more effective treatment may cost less lower health care spending&quot; (<a href="#">McClellan &amp; Benner</a> 2009, 8)</td>
</tr>
<tr>
<td>That CER will change coverage or reimbursement of certain drugs</td>
<td>&quot;If we shift to a system that demands incontrovertible proof of superior efficacy through comparative studies prior to covering a drug, device, or procedure, the impact on access and subsequent innovation would be large.&quot; (<a href="#">Gottlieb</a> 2009, 3)</td>
<td>&quot;Conservatives warn that a federal comparative-effectiveness agency would lead to government rationing of medical care—indeed, that’s the whole idea.&quot; (<a href="#">Cannon</a> 2009, 1)</td>
</tr>
<tr>
<td>That changes in coverage and reimbursement may limit drug availability for some populations who would otherwise benefit from those drugs</td>
<td>&quot;Limits on access to new medical products that are based on assumptions about the cost to a population will also deny access to individuals who can nonetheless benefit from the medical product.&quot; (<a href="#">Gottlieb</a> 2009, 6)</td>
<td>&quot;if the results of a CER study of alternative treatments are strictly applied to a broad population—for example, through a decision not to cover a treatment based on the CER results—then outcomes may worsen for particular patients who, for various reasons such as comorbidities, race and ethnicity, genetics, or preferences, may have responded better&quot; (<a href="#">McClellan &amp; Benner</a> 2009, 9)</td>
</tr>
<tr>
<td>That CER will hurt future drug and device development</td>
<td>&quot;CER would lead to slower adoption of effective technologies, hinder the discovery of new benefits from existing products, and halt investment in novel research.&quot; (<a href="#">Gottlieb</a> 2009, 6)</td>
<td>&quot;With CER poised to become a critical tool for improving health care decision making and health outcomes, there is a need to balance its implementation with its potential impact on the all-important force of innovation in health care.&quot; (<a href="#">NEHI</a> 2009, 23)</td>
</tr>
<tr>
<td>Organization</td>
<td>Main Theme</td>
<td>Quotes from the Reports</td>
</tr>
<tr>
<td>--------------</td>
<td>-----------</td>
<td>------------------------</td>
</tr>
<tr>
<td>Families</td>
<td>CER leads to improved knowledge which can lower racial disparities in care</td>
<td>“Comparative effectiveness research will be used to help providers and patients make more informed treatment decisions.” (1)</td>
</tr>
<tr>
<td>HJKFF</td>
<td>CER can be an important mechanism in improving quality and reducing cost if supported and utilized correctly</td>
<td>“The aim of comparative effectiveness research is to improve health outcomes by developing and disseminating evidence-based information to patients, providers, and health care decision-makers about the effectiveness of treatments relative to other options. Identifying the most effective and efficient interventions has the potential to reduce unnecessary treatments, which in turn, may help lower costs.” (1)</td>
</tr>
<tr>
<td>RWJF</td>
<td>CER has the potential to help the American health care system if done correctly</td>
<td>“In sum, the fears associated with CE are very much related to the potential misuse of information developed on comparative effectiveness. They can be averted by recognizing that CE provides useful information and valuable input for making decisions that would otherwise be made in the absence of information, but that good decisions depend on sound decision making as well as on good input.” (9)</td>
</tr>
<tr>
<td>US PIRG EF</td>
<td>CER will lead to better care and reduced cost for the health care system</td>
<td>“Opponents’ claims that CER results in the rationing of health care or a government takeover are belied by the true nature of such research: it is simply fundamental scientific research of medical treatments aimed at determining the most effective ways to treat sickness and injury. It is the basis of all advancements in the field of medical science and has been used throughout history to improve medical treatment.” (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>“To improve patient care and reduce the costs of unnecessary and improper treatment, the federal government should expand its funding and support for comparative effectiveness research.” (2)</td>
</tr>
<tr>
<td>NBER</td>
<td>Given the authors’ understanding of market forces, CER will lead to worse health outcomes at higher cost</td>
<td>“Overall, our main conclusions from the conceptual analysis is that CER has indeterminate effects on spending and patient health and, under natural assumptions on how markets respond to new quality information, may even adversely affect both.” (4)</td>
</tr>
<tr>
<td>Cato</td>
<td>CER is a powerful tool, but reform should encourage the private sector to perform it</td>
<td>“Government provision of comparative-effectiveness information may do little or nothing to increase efficiency compared to a policy of laissez faire” (6)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>“Rather than create yet another ineffective government agency, a better way to generate comparative-effectiveness information would be to undo the series of government missteps that suppresses the market’s ability to create and use this important research.” (12)</td>
</tr>
<tr>
<td>AEI</td>
<td>The federal government has a troubled history when it comes to CER, so we should push for this research to be done privately instead.</td>
<td>“like many other seductively simple ideas, enthusiasm for comparative effectiveness research (CER) outpaces its practical promise and obscures the downside of having governments take on these sorts of studies and the clinical considerations that go into them” (Gottlieb 2009, 1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>“CER would lead to slower adoption of effective technologies, hinder the discovery of new benefits from existing products, and halt investment in novel research.” (Gottlieb 2009, 6)</td>
</tr>
<tr>
<td>Source</td>
<td>Statement</td>
<td></td>
</tr>
<tr>
<td>----------</td>
<td>---------------------------------------------------------------------------</td>
<td></td>
</tr>
<tr>
<td>CBOa</td>
<td>1. CER is a public good that will not be created to the extent the public would value unless it is supported at the federal level. 2. To lower health care costs, changes in Medicare need to accompany CER research.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“the private sector will probably not produce as much research on comparative effectiveness as society would value. The knowledge created by such studies is costly to produce—but once it is produced, it can be disseminated at essentially no additional cost, and limiting that dissemination may be difficult” (Orszag 2007, 6)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“Expanded research on comparative effectiveness, if linked to changes in incentives for providers and patients, offers a promising mechanism for reducing health care costs to a significant degree over the long term while maintaining or improving the health of Americans.” (Orszag 2007, 20)</td>
<td></td>
</tr>
<tr>
<td>IHE</td>
<td>There are many issues being considered in the development and debate about the use of CER in the U.S.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“It is difficult to see how suggestions regarding CE influencing decisions on individual patients are much different to what physicians do routinely during their consultations.” (Institute of Health Economics 2009, 14)</td>
<td></td>
</tr>
<tr>
<td>NEHI</td>
<td>CER has the potential to lower geographical variation and improve quality, but it must be implemented correctly or else it will have unintended negative consequences.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“With CER poised to become a critical tool for improving health care decision making and health outcomes, there is a need to balance its implementation with its potential impact on the all-important force of innovation in health care.” (NEHI 2009, 23)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“Across the health care system, there is consensus that better clinical evidence is needed to support decision making at the point of care to reduce variation and promote health care quality. This has generated wide support for clinical effectiveness studies.” (NEHI 2009, 10)</td>
<td></td>
</tr>
<tr>
<td>US PIRG FS</td>
<td>The U.S. needs a coordinated effort at producing CER to realize its potential for increasing the efficiency of the health care system.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“Adoption of the findings in evidence-based treatment protocols and guidelines can help ensure we are paying for the most effective treatments.” (4)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“But while individual efforts are laudable, the country’s ailing health care system lacks a coordinated, national effort to support comparative effectiveness research aimed at discovering which treatments work best” (Russo 2009, 12-13)</td>
<td></td>
</tr>
<tr>
<td>CBOb</td>
<td>CER could constrain health care costs, but savings would be a long time in developing.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“It would probably be a decade or more before new research on comparative effectiveness had the potential to reduce health care spending in a significant way.” (2)</td>
<td></td>
</tr>
<tr>
<td>ECHCR &amp; HPa</td>
<td>CER can help bend the cost curve but only with careful implementation, guidance and dissemination.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“These diverse views suggest that the ultimate impact of CER for better or worse is very uncertain. The key unresolved questions deal with whether CER as it will actually be implemented—and it will—can reduce costs and improve outcomes” (9)</td>
<td></td>
</tr>
<tr>
<td>ECHCR &amp; HPb</td>
<td>CER has a lot of potential for improving upon geographical diversity and inefficiencies in the system, but its ultimate effects will depend on how it is implemented.</td>
<td></td>
</tr>
<tr>
<td></td>
<td>“for CER to make a substantial, positive contribution to reforming health care, the critical implementation issues of prioritizing CER spending to “high-value” studies, creating an efficient research infrastructure, and creating an environment that promotes the effective use of evidence from CER must be addressed” (14)</td>
<td></td>
</tr>
</tbody>
</table>
References


15. Orszag PR. *Letter to the Chairman of the Subcommittee on Health*.; Congressional Budget Office. 2007a:1-3.


## Appendix 2: Interview Respondents and Positions

<table>
<thead>
<tr>
<th>Respondent</th>
<th>Position</th>
<th>Interview Date</th>
<th>Interview Length</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bart Barefoot</td>
<td>Director of Public Policy, GlaxoSmithKline</td>
<td>5/17/11</td>
<td>52 minutes</td>
</tr>
<tr>
<td>William Roper, MD, MPH</td>
<td>Dean of the School of Medicine, Vice Chancellor for Medical Affairs, and Chief Executive Officer - UNC Health Care System</td>
<td>5/23/11</td>
<td>20 minutes</td>
</tr>
</tbody>
</table>
| Debra Barksdale, PhD, RN  | Associate Professor – UNC School of Nursing  
Commissioner – Patient-Centered Outcomes Research Institute                                                                                                                                          | 5/24/11        | 19 minutes       |
| Peter Hussey, PhD         | Policy Researcher, RAND                                                                                                                                                                                  | 6/3/11         | 18 minutes       |
| Gail Wilensky, PhD        | Senior fellow at Project Hope                                                                                                                                                                            | 6/13/11        | 17 minutes       |
| Eugene Rich, MD           | Senior Fellow and Director of Mathematica’s Center on Health Care Effectiveness                                                                                                                         | 6/22/11        | 32 minutes       |
Appendix 3: Interview Questions

I’ve done my best to learn about the history and debate over CER by reading published articles and reports, but I want and need your own expert perspective on the history of CER and the debate over CER in recent health care reform.

So, in your opinion, when it was first proposed in the late 1980’s, what were the most important influences pushing CER forward?

When this process started, what would you say people thought the outcome would look like?

How would you describe the trajectory of CER since then?

Thank you. And now, I’d also like your view on how the recent CER debate played out.

What was your own organization’s position regarding CER at the beginning of the discussion for health care reform?

In your opinion, who would you say were the most influential stakeholders in the debate?

How did they influence the debate over CER?

How would you describe the coalitions that formed around support and opposition?
What do you think proponents thought would be the single biggest benefit of CER?

And what about the opponents? What was the thing they feared most about CER?

Is there anything else I'm missing about the debate over CER?

The Affordable Care Act created the Patient-Centered Outcomes Research Institute (PCORI). As you know, the PCORI will be responsible for coordinating a major new national push on CER.

How familiar are you with the planning for the actual implementation of the PCORI?

PCORI is supposed to generate research findings. How do you think the results of this research will be used?

Do you happen to know how broadly the proposed structure of the board was discussed in the stakeholder community while the Affordable Care Act was being debated?

Do you think this particular board structure will capture support for PCORI from all its stakeholders, even the ones who might be concerned about the uses of CER research?

Do you think PCORI's funding realistically will be enough to achieve its aims?
As you know, some people have been concerned that PCORI research will be seized on to change coverage and reimbursement. Some people think that’s a good thing, and other people are worried about this. How likely do you think that is, and what do you think its consequences will be?

Thank you so much for your time. We are almost done. I just have a couple more questions. One provision of the health reform legislation’s treatment of CER is that it prohibits the use of cost effectiveness. How do you think that prohibition on cost effectiveness will affect the utility of what CER can produce?

What do you think would change if CER could include cost effectiveness analyses?

Thank you so very much for your time and thoughts! Do you have any additional questions or comments? Would you like a copy of this interview once it is transcribed? Thank you again!