Current Issues and Trends in Comparative Effectiveness Research

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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
2014

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<th>Abbreviation</th>
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<tr>
<td>AHRQ</td>
<td>Agency for Healthcare Research and Quality</td>
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<td>CER</td>
<td>Comparative Effectiveness Research</td>
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<td>CMTP</td>
<td>Center for Medical Technology Policy</td>
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<td>DIA</td>
<td>Drug Information Association</td>
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<td>GDP</td>
<td>Gross Domestic Product</td>
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<td>FDA</td>
<td>Food and Drug Administration</td>
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<td>IT</td>
<td>Information Technology</td>
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<td>IOM</td>
<td>Institute of Medicine</td>
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<td>MRI</td>
<td>Magnetic Resonance Imaging</td>
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<td>PCOR</td>
<td>Patient-Centered Outcomes Research</td>
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<td>PCORI</td>
<td>Patient-Centered Outcomes Research Institute</td>
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<td>QALY</td>
<td>Quality-Adjusted Life Year</td>
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<td>RCT</td>
<td>Randomized Clinical Trial / Randomized Controlled Trial</td>
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Abstract

Comparative Effectiveness Research (CER) has received national attention in recent years and since the passage of the American Recovery and Reinvestment Act of 2009 as a way to improve health outcomes and reduce healthcare spending. This paper explores current trends and issues in CER, and illustrates the use and future potential of CER through a recent case study.

According to the Institute of Medicine, CER is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care (Sox, 2010). Simply put, the purpose of CER is to help patients and their healthcare providers, especially physicians, make better-informed healthcare decisions. CER embraces the concept of patient-centeredness by ensuring the right treatment for the right patient at the right time (Biskupiak et al., 2012). It has potential to not only improve individual patient outcomes, but also population health through the use of evidence to recommend care guidelines and standards. By making healthcare more efficient and effective, it is also thought that CER can reduce healthcare spending. It is anticipated that healthcare payers, providers, biopharmaceutical companies, medical device companies and patients all have great benefits to reap from CER.

The Patient-Centered Outcomes Research Institute (PCORI), which was established as an independent non-profit corporation in 2010 by the Patient Protection and Affordable Care Act, is a driving force behind recent interest and support of CER, and has funded high-profile studies. This paper reviews the benefits of CER as a methodology to compare treatment options by synthesizing existing evidence or generating new evidence to inform decision-making. It illustrates these benefits though a currently ongoing, PCORI funded, uterine fibroids case study. It also provides a brief overview of some of the many ways to perform CER, emphasizing consideration of the research question at hand to select the most effective method for assessing
comparative effectiveness. Finally, it briefly reviews benefits and criticisms of CER and identifies areas for further discussion. An important conclusion is that the future of CER is about making research more patient-friendly, useful and scientifically credible. To do so, continued collaboration is needed between all healthcare stakeholders – most importantly – patients. In this way, CER can play an important role to achieve national goals of reduced healthcare spending and improved health outcomes.

**Introduction**

In the recent past and with the passing of the American Recovery and Reinvestment Act of 2009, Comparative Effectiveness Research (CER) has taken center stage as one of the nation’s top priorities to improve health outcomes and reduce healthcare spending. According to the Institute of Medicine (IOM), CER is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat and monitor a clinical condition or to improve the delivery of care (Sox, 2010). Simply put, the purpose of CER is to help patients and their healthcare providers, especially physicians, make better-informed healthcare decisions by synthesizing the latest evidence from a wide range of data sources.

To aid the decision-making process, the benefits of different treatment options are compared using various methods. Benefits are typically summarized as an increase in quality-adjusted life years (QALYs), taking into account both morbidity and mortality to give patients and their physicians a 360 degree view of how effective various treatments are at improving health outcomes. Most importantly, CER embraces the concept of patient-centered decision-making by providing information that helps the individual patient and their physician evaluate clinical characteristics as well as personal preferences to decide on the best treatment option among alternatives. This idea of patient-centered care is at the heart of CER, ensuring the right
treatment for the right patient at the right time (Biskupiak et al., 2012). CER has potential to not only improve individual patient outcomes, but also population health through the use of evidence to recommend care guidelines and standards. By making healthcare more efficient and effective, it is also thought that CER can reduce healthcare spending, which is growing rapidly.

This paper explores current trends and issues in CER, providing readers with an appreciation for the promise and future potential of CER for individual patients, communities and the nation. It uses the terms CER and clinical CER interchangeably, but it is important to note that ‘clinical’ CER focuses on the benefit-risk evaluation of alternate treatment options, excluding cost comparisons. The paper concludes by providing a case example of CER that is currently ongoing and suggesting areas for further discussion.

**Evaluating Comparative Effectiveness**

There are many ways to perform CER, each with its own strengths and weaknesses. Methods for evaluating comparative effectiveness include performing systematic reviews of existing research; leveraging existing datasets to answer current research questions; and designing new, prospective research studies for the collection of primary data (Congress of the United States Congressional Budget Office, 2007). The “Advancement of CER: Mindsets & Methodologies” section of this paper complements this section by addressing advancements in ways of thinking and new tools, standards and methodologies to help researchers perform CER.

Systematic reviews represent a cost-effective method because they involve meta-analyses of all available studies; however, the results from existing studies are sometimes not generalizable to a larger, specific population of interest. One prominent example of an organization that performs systematic reviews is the Technology Evaluation Center of the Blue Cross Blue Shield Association, which produces 20 to 25 effectiveness assessments of drugs,
devices and other technologies each year based on reviews of available literature from clinical trials or other studies that have already been conducted (Congress of the United States Congressional Budget Office, 2007). Another example is the Agency for Healthcare Research and Quality (AHRQ), which sponsors the development of systematic reviews of the comparative effectiveness of treatment options for common, costly medical conditions, such as chronic urinary retention, to assist public and private-sector organizations in their efforts to improve the quality of health care in the United States (Brasure et al., 2014).

Like systematic literature reviews, the use of existing datasets for CER is also a cost-effective method for evaluation; however, since these datasets were not collected with the current research question in mind, they are often missing critical information necessary to draw conclusions about comparative effectiveness, such as patient-reported outcomes and long-term follow-up. In addition, existing datasets may not contain sufficient information on subpopulations of interest for CER, such as minorities. The uterine fibroids case example highlighted in this paper is a good example of a CER study that leverages existing data from electronic health records.

Prospective research studies, including clinical trials, observational studies such as patient registries, and newer approaches such as practical clinical trials, can collect data in the real-world and offer greater generalizability to study results, but at a higher cost. One example of a prospective research study is the AHEAD registry of early Alzheimer’s disease among 50 to 75 year olds (AHEAD Registry, 2014). Since each method to perform CER has its own strengths and weaknesses, it is important to consider the research question at hand to select an effective method for assessing comparative effectiveness.
Beneficiaries of CER

It is anticipated that healthcare payers, providers, biopharmaceutical companies, medical device companies and patients all have great benefits to reap from CER. Payers believe that such research can help them reduce unnecessary healthcare expenditures by providing patients with the right treatment option at the right time. As an example, CER can identify the most effective blood pressure medication for a subset of patients, thereby helping these patients effectively manage their condition and reduce the likelihood of costly disease progression. Providers can leverage the most current research to improve the quality of care delivered to patients, thereby improving patient health outcomes. Biopharmaceutical companies can employ CER to gain a competitive advantage, showing that their drug performs better in the real-world than a competitive product. Last, but certainly not least, patients stand to benefit from better quality healthcare that takes into account their personal preferences and clinical condition to recommend the best treatment option among alternatives. To help bring the benefits of CER to all healthcare stakeholders and close the evidence gap between clinical trials and studies of effectiveness, regulatory agencies are also showing their support through recent industry guidance, such as the U.S. Food and Drug Administration’s (FDA) draft guidance on Postmarketing Studies and Clinical Trials (FDA, 2011).

While each individual stakeholder group stands to benefit from CER, innovative collaborations between multiple healthcare stakeholders are required to truly bring the benefits of CER to life. One prominent example of collaboration is between Utah-based health system Intermountain Healthcare, pharmaceutical company Forest Labs, and health IT consultancy Deloitte. These stakeholders have joined forces to employ a data sharing tool to enable CER based on Intermountain clinical insights. In doing so, they have helped to demonstrate measureable improvements in health outcomes. Of particular note, they have decreased sepsis
deaths from 20 percent in 2004 to 9 percent in 2009 by using real-world evidence of the
effectiveness of different approaches to drive changes in clinical practice (Hobbs, 2014).

**Drivers of CER**

Several drivers are facilitating the move toward CER. First are continuing concerns over
healthcare quality as documented on a national scale by the seminal reports of the IOM (IOM,
2000; IOM, 2001). Other drivers include the trend toward personalized medicine and the arrival
of “Big Data” and advanced information technology (IT) to process it. Big Data is defined as
high-volume, high-velocity and high-variety information assets that demand cost-effective,
innovative forms of information processing for enhanced insight and decision making (Gartner,
2014). Big Data, including the explosion of available healthcare data in an electronic format –
claims data, electronic health records, clinical trial data – together with advances in health IT
merit further exploration as they are having a profound impact on the availability and
accessibility of information for enhanced CER decision-making.

Perhaps one of the major driving forces behind CER is healthcare costs. Experts suggest
that less than half of all medical care is based on adequate evidence about its effectiveness,
stemming instead from anecdotal evidence, conjecture and the experienced judgment of
individual physicians (Congress of the United States Congressional Budget Office, 2007).
Limited, narrowly defined evidence can lead to inefficient and ineffective healthcare,
contributing to rising costs without improving health outcomes.

Healthcare spending has grown at a rapid rate over recent decades, and continues today.
For example, it rose from 8 percent of the U.S. economy in 1975 to 16 percent in 2007, and is
projected to reach nearly 20 percent of gross domestic product (GDP) by 2016. Nearly half of
healthcare is privately financed while the other half is publically financed through programs like
Medicare and Medicaid, which together accounted for nearly 4 percent of GDP in 2007 (Congress of the United States Congressional Budget Office, 2007). In 2012, publically financed health expenditures by federal, state and local governments constituted 44 percent of national health spending and $1.2 trillion, and, if current trends continue, by 2023 such expenditures are projected to account for 48 percent of national health spending and to reach a total of $2.5 trillion (Centers for Medicare and Medicaid Services, 2014). Further fueling this rise in spending, the United States must absorb another 32 million uninsured people into the healthcare system over the next decade as a result of the Patient Protection and Affordable Care Act (Sox, 2010).

Healthcare spending varies widely from one region to another and so do health outcomes. In 2009, average healthcare spending ranged from $5,031 per capita in Utah to $10,349 in the District of Columbia (Henry J. Kaiser Family Foundation, 2009). One would think that higher spending leads to better health outcomes; however, regional differences suggest that this is not the case. Utah spends the least on healthcare but ranks number six in overall health in the United States (United Health Foundation, 2013). This suggests that opportunities may exist to cut spending without negatively impacting health.

The federal government, as the largest single financer of healthcare, has a vested interest in reducing healthcare spending and improving health outcomes. President Obama has stated that ever-increasing healthcare costs are a principle threat to the nation’s fiscal solvency and that research can help to solve the problem (Sox, 2010). As such, he spearheaded the American Recovery and Reinvestment Act of 2009, which allocated $1.1 billion for CER, and the Patient Protection and Affordable Care Act of 2010, which created the Patient-Centered Outcomes Research Institute (PCORI) to establish a portfolio of CER projects. Importantly, PCORI’s research projects are focused on a range of treatments – including medications, surgical
procedures, and other health interventions – as well as improving methods for CER and building a large data infrastructure to support CER. With drug costs representing less than 15 percent of healthcare spending (Congress of the United States Congressional Budget Office, 2007), effective surgical procedures and other interventions could have a much larger impact on bringing down healthcare costs.

**Patient-Centered Outcomes Research**

Since its inception, PCORI has awarded more than $316 million for 192 patient-centered outcomes research (PCOR) studies, including CER studies focused on cancer detection, treatment, and surveillance; mental health; cardiovascular diseases; endocrine disorders, such as diabetes mellitus; self-care; and pain management (Selby, 2014). Many of these studies compare two or more treatment options, synthesizing existing evidence, or generating new evidence to inform decision-making.

The PCORI board of governors has adopted three strategic goals: to increase the quantity, quality, and timeliness of usable, trustworthy comparative research information; to accelerate the implementation and use of research evidence; and to exert influence on research funded by others to make it more patient-centered and useful (Selby, 2014). To ensure its research priorities are patient-centered, PCORI assembles multi-stakeholder advisory panels, including patients, caregivers, clinicians, payers, researchers, policy makers and industry representatives, to help identify and refine research priorities and questions – with patients, in particular, playing a large role in assessing the value of healthcare options available to them.

PCORI is the driving force behind recent interest and support of CER, and has funded high-profile CER studies such as the uterine fibroids example highlighted in this paper. Over the next three years, PCORI will commit another $1.5 billion to research projects, with an aim to
make research more useful and likely to be included in healthcare decision-making (Selby, 2014). Medicare and every private health insurance company pay a tax on each of their insured lives to ensure ongoing funding to support PCORI and the nation’s ongoing CER program.

**The Advancement of CER: Mindsets & Methodologies**

Research priorities between investigators and patients are not always perfectly aligned. Although both stakeholders have common broad goals, such as curing disease and improving quality of life, their areas of focus for research may vary. For example, investigators may want to answer the question “how much more effective is this dose than that dose?” whereas patients may care more about answering the question “which dose has fewer side effects?” Traditionally, investigators have prioritized their research agendas without direct consideration of patients’ research priorities in mind. The PCORI model turns tradition on its head, emphasizing patient-centered research questions and priorities, as well as key elements of study design. In this way, complex, narrowly designed protocols are less important than employing the right approach for the right question, leveraging a combination of interventional and observational approaches.

Randomized clinical trials (RCTs), also known as randomized controlled trials, are considered the gold standard of evidence development; however, they can also be relatively costly and time-consuming to conduct. Furthermore, evidence from RCTs may not be broadly applicable or generalizable in the real-world (Gillings & Douglass, 1985). For example, a clinical trial for a particular disease may exclude the elderly or patients with concomitant health problems. To illustrate this point, J. Michael Sprafka, MPH, PhD, Executive Director of General Medicine at Amgen, compared the population studied in RCTs to a handful of blue M&Ms, whereas the patient population in clinical practice represents all colors of M&Ms – blue, red, yellow, brown, etc. (Sprafka, 2014). These broader populations – and the way they respond in a
real-world setting – are of extreme importance in determining comparative effectiveness. This represents an important distinction between RCTs and CER. RCTs are aimed at demonstrating efficacy rather than effectiveness – the distinction being that efficacy reflects optimal conditions, whereas effectiveness requires a demonstration in real-world medical settings (Congress of the United States Congressional Budget Office, 2007).

With an understanding that RCTs have their limitations, researchers are turning to observational studies of comparative effectiveness to enhance them. Observational studies can complement the findings of RCTs and expand them to a broader population. For example, observational studies of pharmaceutical drugs are used for several reasons: to ensure drugs perform according to the expectations set in clinical trials, to demonstrate product stewardship in the real-world, and to deliver on post-marketing safety commitments (Sprafka, 2014). The prominence and growth of observational studies of drugs registered on ClinicalTrials.gov has also been noteworthy, increasing from 26,472 in 2013 to 34,061 in 2014 and counting (Sprafka, 2014).

Traditionally, observational studies have fallen below RCTs on the hierarchy of evidence development because they do not adhere to the strict guidelines found in RCTs. Today, as the potential strengths of observational studies for comparative effectiveness become more widely recognized, particularly in terms of including large, representative patient populations and collecting long-term follow-up data, efforts to strengthen CER guidelines have taken shape. Just as Good Clinical Practice Guidelines are used to ensure the validity and reliability of data from RCTs, there are now tools that researchers can use to improve the credibility of observational studies. One tool is the Good ReseArch for Comparative Effectiveness (GRACE) checklist, a validated tool for screening the quality of observational CER studies to facilitate their use in
decision-making (Dreyer et al., 2014). Another tool is the Agency for Healthcare Research and Quality (AHRQ) User’s Guide, “Developing a Protocol for Observational Comparative Effectiveness Research” (Velentgas et al., 2013). The guide includes best practices for developing study objectives, questions, design, data sources and analysis methods. Complementing these tools, PCORI launched the “PCORI Methodology Report” to outline requirements for conducting scientifically valid patient-centered outcomes research (Hickman et al., 2013). It includes cross-cutting standards, such as those associated with patient-centeredness, encompassing study design and methods, and standards for data registries.

Researchers now have a variety of tools at their fingertips to make observational CER studies more trustworthy, reliable and likely to be used in clinical decision-making. The following section presents a case study to illustrate how these high standards are being applied.

**Case study: PCORI CER Study of Uterine Fibroids**

In the summer of 2014, I completed my master’s practicum within the Scientific Affairs unit of the Real-World Late Phase Research division at Quintiles, the leading contract research organization. This unit is led by world renowned epidemiologist Dr. Nancy Dreyer, MPH, PhD, FISPE, and my practicum took place in her unit’s Health Policy and Government Projects division, which carries out prospective and retrospective study design and analysis, including CER studies. It has driven high-profile projects such as editing the AHRQ user’s guide mentioned earlier, “Developing a Protocol for Observational Comparative Effectiveness Research” (Velentgas et al., 2013).

A good example of CER, and one on which I focused my practicum, is Quintiles’ recently awarded project from PCORI to compare patient-centered outcomes after treatment for uterine fibroids. This retrospective observational study of comparative effectiveness directly
reflects PCORI’s goals for CER by focusing on a pressing, patient-centered public health priority and by engaging patients and other key stakeholders in the research process to identify and refine study priorities and questions. The research is ongoing at the time of writing this paper, and is being led by Quintiles in collaboration with the Center for Medical Technology Policy (CMTP) and the Department of Obstetrics and Gynecology at Duke University. The investigators are taking a comprehensive approach to evidence development, analyzing more than 33,000 patient records from electronic medical records, claims data and data from integrated healthcare delivery systems to evaluate patient demographics, diagnoses, symptoms, treatments and laboratory results. Ultimately, the study will provide patients and their caregivers with information that will help them make better-informed care decisions (Gliklich, 2013). It is important to note that this study is one part of a holistic research strategy to evaluate the comparative effectiveness of treatment options for uterine fibroids. Part two includes a prospective patient registry, which is not discussed further in this paper as details are still being worked out.

**Uterine Fibroids Background**

Uterine fibroids occur in women of childbearing age, and can cause symptoms such as heavy menstrual bleeding and pain. In pregnant women, fibroids can lead to miscarriage, preterm birth and an increased risk of cesarean delivery. More extreme cases may contribute to infertility. Treatment options include surgical procedures, such as hysterectomy and myomectomy, as well as non-surgical procedures and medications, yet little scientific evidence exists about which treatment options are better than others, particularly for addressing a patient’s specific treatment objectives (Gliklich, 2013). Approximately 200,000 hysterectomies, 30,000 myomectomies, and thousands of selective uterine artery embolizations and high-intensity focused ultrasound procedures are performed annually in the United States to remove or destroy
uterine fibroids, with the annual economic burden of these tumors estimated to be between $5.9 billion and $34.4 billion (Bulun, 2013).

**Study Rationale**

It is believed that research, guided by patients, will increase the clinical evidence base of treatments for uterine fibroids, thereby equipping patients and their clinicians with evidence to make an informed decision. Significant trade-offs exist for each treatment option, particularly hysterectomy, and without trustworthy evidence of the risks and benefits of surgical as well as non-surgical procedures, a patient cannot fully evaluate her options. This is especially the case for a woman of child-bearing age, who must consider whether or not she wants to have children when evaluating her options.

**Study Objectives**

The primary objective of the study is to answer critical questions such as how long treatment effects (i.e., relief from symptoms) last for treatments other than hysterectomy, as well as for all treatments, including hysterectomy. The research question was identified by stakeholders, including patients, and answering it will help to strengthen the clinical evidence base so that patients and clinicians can ensure the right treatment at the right time.

**Study Design**

This observational study employs a retrospective analysis of electronic medical records, claims data and data from integrated healthcare delivery systems to evaluate the effectiveness of treatment options for uterine fibroids. It draws upon two independent data sources to identify two separate cohorts of uterine fibroids patients. These patients are followed, retrospectively, for a minimum of two years, with their demographic and medical history data used as markers to evaluate symptom relief after five procedures of interest: myomectomy, endometrial ablation,
uterine artery embolization, Magnetic Resonance Imaging (MRI)-guided focused ultrasound ablation and hysterectomy (Observational Study Protocol, 2014).

Uniquely, the study also employs a stakeholder engagement plan to evaluate the ability of important stakeholders to noticeably change the study design and analysis plan. The stakeholder population includes 17 individuals representing patients and consumers, clinicians, insurers and/or payers, federal agencies and researchers (Observational Study Protocol, 2014). To date, stakeholders have already contributed in meaningful ways, suggesting an extension of the patient follow-up period to a minimum of two years, and the evaluation of pregnancy as an outcome of interest.

**Study Analyses and Reporting**

A final study report will be produced at the close of 2015, encompassing all planned analyses. At that point, a dissemination plan will also be executed to inform the public of the findings. Importantly, the study’s stakeholder advisory panel will help to craft the dissemination plan to ensure communication of study findings in a meaningful, patient-friendly way, and to encourage rapid uptake and use of the data in clinical practice.

**Lessons Learned**

This project, and my practicum experience, expanded my knowledge of CER, and how it can be used to make healthcare more patient-focused. The study embraces the concept of “patient centeredness” from inception through to completion, allowing patients and others participating in the study to review and make comments on the protocol, and to craft the dissemination plan to maximize study understanding and uptake. Specifically, this case provides examples of the following characteristics of well-designed CER studies that I got exposure to:
• Developing high-quality protocols, research questions, reports and other study documents to make evidence from CER studies more credible, trustworthy and likely to be used in clinical decision-making;

• How to perform literature reviews, and the wealth of available information and published studies on uterine fibroids;

• Understanding the strengths and weaknesses of a retrospective CER study design;

• Overcoming the challenges involved in normalizing and analyzing claims and EHR data to determine comparative effectiveness;

• The importance of involving multiple stakeholders in CER studies; and

• The goals and objectives of PCORI, and how their funded research could help improve health outcomes and lower spending.

Discussion & Conclusion

This paper has reviewed current issues and trends in CER, and the potential for CER to improve health outcomes and reduce unnecessary healthcare spending. Public financing and support of CER through government agencies like PCORI and AHRQ are laying the foundation for continued use and understanding of CER and improved study designs. The PCORI uterine fibroids study is an exemplary case of CER in action. It highlights many topics and issues that merit further discussion, such as the importance of private/public partnerships to the future of CER; the need for a holistic research strategy to answer CER questions; the growth of patient registries as a valuable source of CER data; and the continued development of standards, guidelines and advocacy groups to enhance the scientific integrity and usefulness of CER.

The government has played a leading role in supporting the acceleration and adoption of CER by establishing agencies like PCORI to create and fund an ongoing portfolio of CER
The PCORI uterine fibroids study showcases how government and industry are working together to conduct CER studies, mixing the talents of industry (Quintiles), academia (Duke University Medical Center), and non-profits (CMTP) to execute the study. These types of private/public partnerships will be critical to the future of CER, bringing the best talents together to design and execute patient-centered research. Without government funding of expensive CER studies, many of these unique collaborations would never have gotten off the ground, demonstrating the need for continued public financing of CER. This type of funding and focus will encourage continued partnership models between all healthcare stakeholders – patients, physicians, payers, biopharmaceutical and medical device companies, and policy makers – to make research more credible, valued and useful.

The PCORI uterine fibroids study also highlights the need for a holistic research strategy when attempting to answer CER questions. In this example, the research strategy encompasses two main parts – a retrospective observational study, currently in progress, and a prospective patient registry funded in late 2014. The primary purpose of the retrospective study is to more quickly and easily leverage existing data to gain an understanding of the research question at hand, and to inform the design of the more complex (and expensive) patient registry. The researchers understood that the retrospective study design would have its limitations. For example, the study could be open to information bias caused by inconsistencies in coding practices and data collection, or even missing data. By way of example, many physicians may not record important information such as over-the-counter pain medications or race. In addition, the conclusions drawn from the research sample may not be generalizable to the entire U.S. population. Since claims data are collected from people who have healthcare insurance, these people may differ in significant ways from the rest of the U.S. population. To overcome these
challenges and add to the findings of the retrospective study, the researchers also plan a prospective registry of uterine fibroids patients. Together, these two elements represent a holistic research strategy that will inform treatment guidelines and decision-making for uterine fibroids care.

The PCORI uterine fibroids example also showcases the use of patient registries as a valuable data source for CER, a trend that is likely to continue. Registries allow researchers to prospectively collect the right level of information to study the benefit/risk profile of a variety of treatment options. In addition, they allow for the collection of patient-reported outcomes, which are critical to understanding the trade-offs between treatment options from the patient’s perspective.

As investment continues in CER, there will, no doubt, be more guidelines, tools, standards and advocacy groups that emerge to champion CER and make it more scientifically credible and useful. PCORI has been a leading force in shaping opinion and action, both through its funding of research and Methodology Report (Hickman et al., 2013), which offers a helpful tool for those conducting CER. Expect updated versions of the PCORI Methodology Report, AHRQ User’s Guide (Velentgas et al., 2013) and GRACE checklist (Dreyer et al., 2014), together with guidelines around the use of Big Data to conduct CER, in the years to come as lessons are learned and practices are perfected.

It is yet to be seen if CER will help the nation improve health outcomes and reduce healthcare spending. Some critics question the role of government in CER, claiming that these studies will lead to government domination of the doctor–patient relationship, “cookbook medicine,” and rationing (Avorn, 2009). Others debate the role of cost as an outcome measure in CER, an area of considerable controversy in the United States, which has chosen to eliminate
cost from the CER equation as other countries weigh both cost and benefit to determine the best treatment option among alternatives. Still other critics question whether CER findings will actually influence clinical practice. A leading marker of whether CER will produce desired outcomes will be whether the information produced is actually helpful to physicians and patients in real-world decision making (D’Arcy et al., 2012). Even with these critiques in mind, there is little doubt that research to provide the right treatment to the right patient at the right time holds great promise for all healthcare stakeholders involved, especially the patient. The future of CER is about making research more patient-friendly, useful and scientifically credible. To do so, continued collaboration is needed between all healthcare stakeholders. In this way, CER may live up to its promise of helping the nation reduce healthcare spending and improve health outcomes for all patients and communities.
References


