PCORI, Comparative Effectiveness and the ACA: Improving Patient Outcomes or Cookbook Medicine?

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In my preliminary article of this series, I explained that the Affordable Care Act (ACA) can be split into two rough sections: improving the quality of health care services and reducing the cost of care.

With that as a framework, this series provides brief explanations of various components of the ACA, alternating between quality and financial initiatives. This installment takes a closer look at how the ACA envisions improved outcomes for patients individually and nationally through research-driven standardization of medical care.

PCORI and the ACA

Although it has not received a lot of attention from the public as of yet, the ACA created the Patient-Centered Outcomes Research Institute (PCORI) that could significantly impact how physicians treat chronic disease and how patients participate in their own medical decision-making. The organizing theme is that not all treatments are effective, and not all patients are appropriate for all treatments.

Two tenets of the ACA are represented by PCORI. For one, health care outcomes can be improved by standardizing care toward treatments that have been proven to be effective. In addition, health outcomes will improve when patients are provided adequate information such that they are able to fully participate in making decisions about their care.

From the outset, it is important to note that the government by no means invented quality improvement or research-based practice patterns. The necessity of rigor in research design and implementation has been a fundamental principle in the development of medical interventions, particularly in the last 60 years.[1]

At the same time, it has been well-documented that physicians do not necessarily adopt practices consistent with reputable research in their own field. Research highlighted by the Institute of Medicine (IOM) in the pivotal publication *To Err is Human*, revealed an average 17-year lag between the publication and incorporation into patient care of evidence-based practice adjustments.[2] Throughout medicine, there are examples suggesting resistance and inconsistency in adopting new practice patterns, including:

- Post-treatment of heart attack patients continues to include a procedure invalidated by the 2006 OAT Trial that resulted in guidelines from both the American Heart Association and the American College of Cardiology advising against the treatment.[3]
- Hemodialysis remains the primary, tertiary mode of treatment of end stage renal disease (ERSD), despite significant studies demonstrating the efficacy, reduced cost, and improved patient experience offered by peritoneal dialysis.[4]
- Prescription rates for children over the age of two with otitis media have not changed, even though jointly issued treatment recommendations in 2004 by the American Academy of Pediatrics (AAP) and the American Academy of Family Practice (AAFP) advocated a monitoring period before antibiotic intervention.[5]

The clarion call for standardization of medical treatments, particularly in the arena of chronic diseases, came from the IOM in 2001. The report "Crossing the Quality Chasm" noted the need for a "far more effective infrastructure...to apply evidence to health care delivery" with "greater emphasis [upon] systematic approaches to analyzing and synthesizing medical evidence for clinicians and patients." The report continued, "The dissemination of guidelines alone has not been a very effective method of improving clinical practice."

What is needed, the report argued, is a "public- and private-sector partnership [focused upon]: ongoing analysis

and synthesis of the medical evidence, delineation of specific practice guidelines, and enhanced dissemination efforts to communicate evidence and guidelines to the general public and professional communities."[1]

A systematic approach to the "ongoing analysis and synthesis of the medical evidence" envisioned by the IOM requires an entity devoted to the arena of comparative effectiveness, which can be defined as: a type of health care research that compares the results of one approach for managing a disease to the results of other approaches. Comparative effectiveness usually compares two or more types of treatment, such as different drugs, for the same disease. Comparative effectiveness also can compare types of surgery or other kinds of medical procedures and tests. [6]

Given the core focus on patients in PCORI, it is ironic that the public may have the most vehement reaction to its research, just as patients have to other comparative effectiveness initiatives in the past. Many have viewed comparative-effective results as "rationing" health care.

Not only is comparative effectiveness not a new concept, PCORI is not the first initiative centered on effectiveness research. Evidence-based treatment has been a core component of health care delivered under pay for performance programs since the California Pay for Performance Pilot Study (2001-2006).[7]

The Patient Safety and Quality Improvement Act of 2005 (PSQIA) created a model for the standardization as well as sharing of error reporting and risk reducing practices. There are multiple examples of government-sponsored standardization of medical care, but the primary one is the Agency for Healthcare Research and Quality (AHRQ).

Originally created in 1989 as the Agency for Health Care Policy and Research (AHCPR),[8] AHRQ is one of 119 agencies[10] within the U.S. Department of Health and Human Services (HHS) that work cooperatively to improve health care in America, at least in part through the collection of data that can be used to enhance treatment nationally. AHRQ has three key focus areas: reducing risk of harm by promoting delivery of the best possible care, improving outcomes by encouraging the use of evidence to make informed decisions, and transforming research into practice to facilitate wider access to effective care services and reducing unnecessary costs.[11]

Specific examples of research conducted by AHRQ that have directly altered the delivery of care (through Medicaid, Medicare, and the private sector) are detailed on the agency's website.[12]

The role of PCORI

Working cooperatively with AHRQ, IOM and other federal agencies, PCORI was created to focus on comparative effectiveness from the patient perspective. The Institute is primarily a funding organization that supports research that compares treatment options from that patient perspective. Patient-centered comparative research, as defined by PCORI, focuses on four patient questions:

1. "Given my personal characteristics, conditions and preferences, what should I expect will happen to me?"
2. "What are my options, and what are the potential benefits and harms of those options?"
3. "What can I do to improve the outcomes that are most important to me?"
4. "How can clinicians—and the care delivery systems they work in—help me make the best decisions about my health and health care?"[13]

Patients are purposefully incorporated throughout all functions of PCORI. They are asked to help select which research projects will receive funding, design how the research will be conducted, and choose platforms for communicating the research findings that will be available to various patient groups.[14] PCORI's strong emphasis on patient input and education, as evidenced by its mission statement,[15] sets it apart from federal agencies like AHRQ. Its findings, however, are to be incorporated and utilized by all entities involved in comparative effectiveness research.[16]

PCORI is an independent, non-profit research organization.[17] It is run by a 21-member Board of Governors who represent a wide range of stakeholders in health care.[18] Grants awarded by PCORI are funded through a 10-year, multi-billion dollar fund to support comparative effectiveness research named the Patient-Centered Outcomes Research Trust Fund (PCORTF).[19] In addition to Congressional appropriation, the PCORTF is funded through fees collected on health insurance plans also created by the ACA[19,20,21]

PCORI is not involved in deciding what treatments will be reimbursed through Medicare, Medicaid or other government programs, and it is prohibited from funding research that is based on cost effectiveness.[14] The ACA prohibits the Secretary of HHS from using PCORI research findings to make coverage determinations that place any value judgment on the value of life, particularly in the elderly, disabled or terminally ill.[19]

How will the public react to PCORI?

Given the core focus on patients in PCORI, it is ironic that the public may have the most vehement reaction to its research, just as patients have to other comparative effectiveness initiatives in the past. Many have viewed comparative-effective results as "rationing" health care, such as the debacles over the mammogram and PSA frequency of screening revisions that were suggested by the government in recent years.[23]

Some non-medically trained commentators believe comparative effectiveness is nothing more than a plan to deny individualized care,[24] while others have blamed specific high-profile deaths to "evidence-based medicine, which is nothing more than a health care system that values profit over patients."[25]

The idea that physicians may be protected from liability by complying with practice guidelines has been derided as "fundamentally unfair," because if "medical societies are allowed to participate in writing guidelines [that] they know will exempt their members from liability, conflicts of interest and bias will escalate."[26] Another vocal opponent suggests practice patterns are an avenue for lazy physicians to make more money without having to exercise professional judgment.[27]

The belief that a partnership between engaged, educated patients and trained clinicians will create safer care, higher outcomes and lower costs is a core principle of health care reform generally and the Affordable Care Act in particular. This is evident in the National Strategy for Quality Improvement in Health Care reported to Congress by HHS as mandated in the ACA.[28]

Therefore, it is imperative that the medical community meet any widespread public resistance to standardized care based on comparative-effectiveness research with accurate, continuing dialogue. It would appear that PCORI is an attempt to bring the public into the tent of health reform.

How will the medical community react to PCORI?

The implication of favoring one treatment option over another—and encouraging patients to dictate their care to the former—is likely to make PCORI controversial as its research becomes more evident to the medical community. The pharmaceutical and durable medical equipment industries are also likely to mount criticism as they experience the financial repercussions of comparative effectiveness research sponsored by PCORI.[29]

However, physicians have historically been suspicious of comparative effectiveness—terming it "cookbook medicine" or "the practice of medicine by strict adherence to practice guidelines, which may not be an appropriate substitute for clinical judgement."[30]

These derisions were particularly evident in the 1990s when health care plans used the moniker "comparative effectiveness" to justify dictating care in a manner that physicians perceived as being cost-driven, not patient-oriented.

The problem is not comparative effectiveness research, but how it is used.[31] The medical community has commonly argued that the enforcement of quality metrics tied to reimbursement (such as in pay for performance programs) undermine independent medical-decision making.[32] Physicians become resistant when comparative effectiveness research is used as a formula rather than a resource to support clinical judgment.

It is a fundamental principle in medicine, at all stages of life and in all patients, that care must be appropriate to each patient's needs, and that often requires straying from practice protocols.[31] Physicians' support for evidence-based medicine grows as practice standards become more outcome-oriented and less cost-driven.

As scientists, physicians also want to make practice changes based on credible research. Fortunately, with the evolution of digital health care records that facilitate collecting data from a demographically significant sample size, research is becoming more convincing.[34] For example, evidence-based process standards—such as emergency intervention in MRIs and time outs in the surgical suite—have positively affected patient outcomes, and that is largely acknowledged in the medical community.[31] As Philip Betbeze of HealthLeaders Media wrote, "You won't find too many physicians who will debate that point anymore."[34]

All of these arguments are reduced to mere chatter, though, if comparative effectiveness research doesn't result in physicians altering how they treat their individual patients. Perhaps that was why PCORI was created; if physicians won't change, then their informed patients will demand more effective treatment options.

Either way, to standardize treatment of chronic disease nationwide (as envisioned by the ACA), will require a transformation at the level of each physician/patient encounter. Having physicians change their practice patterns to comply with treatment vetted through a comparative effectiveness process is the ultimate goal, and to achieve that requires that physicians have an understanding of the process, ownership of the standards measured, and accept accountability to those measures.[34] Facilitating that degree of culture change within the profession will require significant physician leadership.

The future of medicine and comparative effectiveness

Medicine is a research-driven profession and independent, professional decision making is based both upon patients' individual needs and physicians' available resources to adapt their treatment to current research. Evidence-based medicine is not the antithesis of medical professionalism; it is consistent with the history and foundation of the profession.

Although the field of evidence-based research has been supported by the government for more than two decades, PCORI is likely to bring this research to a whole new level. If patient involvement will increase public support of standardized practice patterns, then patients as a result will want more ability to dictate their own care. Physicians will have to adapt to that new model without resigning their own independent decision-making.

The balance will become even more challenging as reimbursement determinations are based on evidence-based research, as they most undeniably will. Even though PCORI is prohibited from studying cost comparisons, its research (as well as other studies from federal agencies such as AHRQ) will inevitably be taken into consideration by private payers, government decision-makers and (ultimately) the Independent Payment Advisory Board (IPAB).[35]

In that sense, PCORI's success is integral to success of the ACA in its entirety.[23]

The next decade will require an extraordinary effort on the part of physician executives to provide leadership in accepting changes in practice patterns and the reimbursement determinations that will inevitably follow. At the same time, modeling an appreciation for the role of patient-centered care will be important to facilitate the transition of medicine from a solo professional to a truly team-based model.

It will take physician leadership to ensure that the professionalism of medicine is maintained while physicians transition to a new practice model that incorporates more protocols, patient input and evidence-based criteria.

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