Implications of Comparative Effectiveness Research for Academic Medicine

by Eugene Rich, MD, FACP

June 5, 2012

Over the past nine years, policy makers from across the political spectrum have called for greater efforts to bring the power of science to bear on fixing the problems of the US health care system. From the 2003 Medicare Modernization Act to the 2010 Affordable Care Act (ACA), legislation has expanded public investments in comparative effectiveness research (CER), now also termed Patient-Centered Outcomes Research (PCOR). In 2011, I published a paper with colleagues from the Association of American Medical Colleges (AAMC) building on the insights I had gained through my academic and policy work on CER. We noted that CER offers a great opportunity, albeit with some considerable challenges, for academic medicine to play a central role in health care reform. We emphasized that scientists conducting CER will learn their methodological rigor in the training programs of academic medical centers. And the clinicians interpreting the medical literature and applying the insights from CER to the unique problems of individual patients will need to learn this evidence-based, patient-centered care from the educators, mentors and role models at America's health science schools and teaching hospitals. We also noted that to meet the demand for much greater information for decision makers, many new CER research teams will be needed, and that these teams will need to establish effective partnerships far outside the walls of the traditional academic setting. We cautioned that "…achieving this will require investment in research infrastructure, adaptations of institutional culture, development of new disciplines and research methods, establishment of new collaborations, training of new faculty, and the expansion and refocusing of educational capacity." But we also saw this as a great new opportunity for academic health centers. "By successfully responding to this challenge academic medicine can further strengthen its longstanding commitment to the scientific practice of medicine and the use of evidence in patient-centered, personalized care."[1]

Note: For this month's column, colleagues at Creighton posed some questions on this topic that Dr. Rich responds to.

Questions were prepared by:
Richard O'Brien, MD, CHPE
John Stone, MD, PhD, CHPE

Question 1: You mention the need for new intellectual "expertise" and "skills in interdisciplinary and cross-institutional research." What are some of these needs and why are they important?

Dr. Rich's Answer: Various authorities have noted that not all CER questions can be addressed by traditional randomized controlled trials. Indeed it is anticipated that a variety of research methodologies will be required.[2] Therefore a broad range of methodological skills are needed to answer different CER questions, including conduct of pragmatic studies in practice-based research networks and observational studies employing analyses from administrative data linked with information from electronic health records or clinical registries. Various authorities have judged these areas of expertise to be in short supply in the US.[3,4,5] The ACA funded CER entity, the Patient Centered Outcomes Research Institute (PCORI), has also emphasized that these studies must answer questions relevant to specific patients. Therefore patient-centered outcomes measurement will be another methodological emphasis of CER. Thus, CER will demand a mix of research skills and expertise, from clinical epidemiology and health economics to sociology, psychology, outcome measurement, and implementation science. Furthermore CER must involve studies in real world settings, determining how well interventions work in routine practice for typical patients. Therefore CER research teams must involve both clinicians and patients served in those settings.

Question 2: You discuss the need to develop partnerships outside the ". . . traditional academic setting . . ." and to elicit the ". . . insights of patients and clinicians . . ." and respond to ". . . expressed needs." Any specific suggestions about how to do that?

Dr. Rich's Answer: As mentioned above, one approach to engaging patients and clinicians outside the traditional academic setting is development of a practice-based research network;[6] additional approaches include organized and sustained research collaborations between researchers and large practice organizations (e.g. the AAMC's Healthcare Innovation Zones) or community-based participatory research efforts that establish academic collaborations with communities representative of typical patients and providers. The question of how best to elicit the insights of patients and clinicians in CER research design is a topic of very active research by PCOR Institute, which just announced a series of awards to study this issue. Work on this is also ongoing through projects funded under the $1.1 billion investment in CER provided by the Recovery Act.[7]

The Center for Medical Technology Policy has pioneered in this area and offers numerous relevant publications.[8]

Question 3: Should CER ignore costs? Why?

Dr. Rich's Answer: The proper role of cost analysis in CER has proven one of the most controversial issues in this developing area of health care science. Some have observed that comparative effectiveness information, absent any data on cost, is comparable to providing clinicians and patients an uninformative "menu without prices;"[9] certainly many patients struggle to understand the likely personal financial consequences of their health care decisions. Nonetheless others have feared that comparative cost information will lead to policymakers employing "cost effectiveness analysis" to ration care, thereby depriving individuals of life saving but costly interventions. Indeed PCORI is forbidden by law from funding work that calculates outcomes in "quality adjusted life years," a routinely used metric in comparative cost analysis. Nonetheless PCORI is to answer such patient-centered questions as: "What are my options and what are the potential benefits and harms of those options?" and "What can I do to improve the outcomes that are most important to me?" It seems likely that at least for some research questions, cost will be an important outcome from the patients' perspective.

[1] Rich EC, Bonham AC, Kirch DG. The implications of comparative effectiveness research for academic medicine. Acad Med. 2011 Jun;86(6):684-8.
[2] Ballou J, Rich EC, Kehn M. Matching Study Designs to Research Questions in Disability-Related Comparative Effectiveness Research. Research Brief. 2011 July [cited 2012 May 31]. http://www.mathematica-mpr.com/publications/pdfs/health/comparative_designs_rschbrief.pdf.
[3] Patient-Centered Outcomes Research Institute [Internet]; [cited 2012 May 31]. Patient-Centered Outcomes Research. http://www.pcori.org/what-we-do/pcor/.
[4] Olsen L, Grossman C, McGinnis JM. Learning What Works: Infrastructure Required for Comparative Effectiveness Research: Workshop Summary. 2011 [cited 2012 May 31]. http://www.nap.edu/catalog.php?record_id=12214.
[5] Federal Coordinating Council for Comparative Effectiveness Research. Report to the President and Congress. Washington, DC: Department of Health and Human Services; 2009 Jun [cited 2012 May 31]. http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf.
[6] Agency for Healthcare Research and Quality [Internet]; [cited 2012 May 31]. Practice-Based Research Networks (PBRNs). http://pbrn.ahrq.gov/portal/server.pt/community/practice_based_research_networks_%28pbrn%29__home_page/851.
[7] Federal Coordinating Council for Comparative Effectiveness Research. Report to the President and Congress. Washington, DC: Department of Health and Human Services; 2009 Jun [cited 2012 May 31]. http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf.
[8] Center for Medical Technology Policy [Internet]; [cited 2012 May 31]. http://www.cmtpnet.org/.
9 Garber AM. A menu without prices. Ann Intern Med. 2008;148:964–966.