Health Policy in Medicine: Inside the Beltway

More on Patient-Centered Outcomes

by Eugene Rich, MD, FACP

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Note: For this column, Dr. Rich responds to questions posed by colleagues at Creighton on his recent publication in the *Journal of Comparative Effectiveness Research* entitled Primum non nocere: reconciling patient-centered outcomes with evidence-based care.
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Question 1: You state on 108 that: "In addition, advances in study designs relevant to urgent clinical problems (such as improved observational analysis of retrospective data) may help clinicians learn more quickly which interventions confer benefit for seriously ill or dying patients. Thus the emphasis on PCOR may helpfully moderate clinicians' willingness to take drastic action when evidence of benefit is unclear and strengthen the commitment to approach each of these difficult decisions as an opportunity to more systematically learn what works best for whom."

However, in a given patient with an urgent need, reasons to take drastic action don't seem mitigated by new research approaches that would generate results more quickly. To be sure, if an approach is more quickly shown effective or not, then hopefully the total future patient population benefited may increase and the total harmed may decrease. But such aggregated results don't seem to help the instant patient. Can you clarify?

Dr. Rich's Answer: This excellent question presses me to clarify two related and perhaps overly subtle points. I will first address my observation that clinicians might "strengthen the commitment to approach each of these difficult decisions as an opportunity to more systematically learn what works best for whom." With new sources of data for conducting PCOR and new analytic techniques, clinicians might be more motivated to make sure that novel interventions are undertaken and documented in a more systematic way so that these circumstances become data for future analysis and thus part of the information base that can improve future decision-making. Currently there is often no opportunity for clinicians to share these experiences except by anecdote. With the development of large networks of linked data, clinical circumstances which may be unusual or unique in the practice of one clinician may be systematically observed across thousands of physician practices and myriad patient encounters. While each individual case may be an anecdote, in aggregate these instances may provide powerful information regarding what interventions are beneficial in such circumstances.

My related point was that "the emphasis on PCOR may helpfully moderate clinicians' willingness to take drastic action when evidence of benefit is unclear..." My hope is that when physicians are confronted by better evidence that some of their well-reasoned and well-intended interventions have been proven fruitless, they may be less tempted to take drastic action without better evidence. And with better data systems for conducting comparative studies in the future, when they are motivated to undertake novel interventions they will have reason to provide better documentation of the circumstances and rationale, to augment the evidence available for future analysis.

Question 2: Cite some examples in which ". . . wielders of invasive procedures . . ." underestimating ". . . the rate of poor outcomes . . ." have resulted in adverse effects on patients?

Dr. Rich's Answer: Sadly there are all too many examples of invasive procedures that were perceived by their providers to confer more good than harm, only to be subsequently proven otherwise by careful comparative effectiveness research. Perhaps the most extensively discussed recent example is bone marrow transplantation for breast cancer, documented in the book False Hope by Richard Rettig, et al. (Oxford University Press 2007). The books Overtreated (Shannon Brownlee, Bloomsbury Press 2007) and Hope or Hype (Deyo and Patrick, AMACOM 2005) also offer numerous other recent examples from coronary artery stents to spinal fusions.

Question 3: Cite an example of ". . . interventions [that] confer benefit for seriously ill or dying patients."?

Dr. Rich's Answer: Not surprisingly numerous interventions have been evaluated for potential benefit in seriously ill patients. For example for patients with septic shock, recent trials have investigated the benefits of corticosteroids, granulocyte colony-stimulating factor, and various forms of pressors. Similarly alternative approaches to managing respiratory failure have been explored for patients with adult respiratory distress syndrome, including high-frequency oscillatory ventilation, and more dramatically, extra-corporeal membrane oxygenation. Other recent studies in critically ill patients have evaluated intensive glucose control and parenteral nutrition.

Question 4: Is there good evidence for, or reason to believe that, humans have ". . . the urge to 'do something' for fellow humans in distress . . ."?

Dr. Rich's Answer: It was beyond the scope on my commentary in JCER to present this case, and on reflection beyond my available resources to present definitive evidence here. Indeed this might make its own excellent topic for discussion by various faculty of the Creighton Center for Health Policy and Ethics. I will instead offer a few observations. The Hippocratic Oath (http://www1.umn.edu/phrm/oaths/oath1.html) certainly urges physicians to take action on behalf of those in distress ("I will use treatment to help the sick") although it notes these actions must be "according to {the physician's}... ability and judgment." The expression of empathy for others has been noted as a common element of child development, and functional MRI studies have suggested that images of other people in distress activates relevant pain centers in the observer's brain. Various evolutionary biologists and popular science commentators have argued that there may even be an evolutionary advantage to altruistic behavior. Dr. Richard O'Brien, CHPE Emeritus Faculty, has published a thoughtful exploration of these issues; "Choosing medicine: motive, incentive, obligation." Pharos Alpha Omega Alpha Honor Med Soc. 1995 Summer;58(3):38-42.

Question 5: It is stated on p. 108 that: "Accordingly, when unproven interventions are inherently dangerous, the clinician employing evidence in a patient-centered way must ensure that patients are well informed of the risks of harm and the uncertainty of benefit." However, unproven interventions may also have uncertain risks. Thus shouldn't the uncertainty encompass risks as well?

Dr. Rich's Answer: In this case I may have created ambiguity in an effort to make the sentence more interesting, using different words that I intended to invoke the same concept. My reference to "risk" was as a synonym for with chance or "uncertainty"- i.e. risk of harm in the context of chance or possibility of harm. Perhaps a less ambiguous phrasing would be "...ensure that patients are well informed of the uncertainties regarding both harms and benefits."

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