

Comparative Effectiveness Research and Decisionmaking: A Conversation With Two Health Care System Administrators

COMPARATIVE EFFECTIVENESS RESEARCH provides evidence to support the decisionmaking of clinicians with their patients who face multiple options for the testing or treatment of health conditions. But what role does it play among health policymakers? Michael Fordis, M.D., of the John M. Eisenberg Center for Clinical Decisions and Communications Science, David Hickam, M.D., of the Scientific Resource Center, and Stephanie Chang of AHRQ sat down with two system administrators — Dick Justman, M.D., National Medical Director of UnitedHealthcare, a national health service-delivery company, and Alan B. Rosenberg, M.D., Vice President of Medical Policy, Technology Assessment and Credentialing Programs for WellPoint, Inc., to discuss the ways in which the results of comparative effectiveness research are used in their work.

MICHAEL FORDIS: Dick, why don't we start with your telling us about some of the types of decisions you make at UnitedHealthcare?

DICK JUSTMAN: I support medical policy development within United Health Group and that involves the development of medical policies and the identification of which clinical practice guidelines we would like to promote. I also chair our National Pharmacy and Therapeutics Committee. This committee makes recommendations, based upon a review of clinical evidence, of the comparative value of different medications used to treat a variety of conditions. So, for diabetes, as an

example, we would be making decisions with regard to the comparative clinical value of various kinds of glycemic agents, including insulin analogues and noninsulin glycemic agents.

MICHAEL FORDIS: Thank you very much. And Alan, do you want to give us just a brief rundown on decisions that you make at WellPoint?

ALAN ROSENBERG: I chair and participate in a variety of committees for WellPoint that make medical policy determinations regarding new technologies, new procedures, pharmaceutical agents, and biologic agents in the context of our benefit determinations. And it is in that context that we use comparative effectiveness information in trying to

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determine whether these interventions are or are not medically necessary and are or are not still investigational.

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MICHAEL FORDIS: Dick, do any specific examples come to mind?

DICK JUSTMAN: There are lots of them. One is computerized tomographic (CT) colonography. While optical colonoscopy is the gold standard in colorectal cancer screening, its use among consumers is very low because they perceive optical colonoscopy to be an invasive and uncomfortable test. It is also a test that frequently requires either sedation or anesthesia. CT colonography is not as good a test, but consumers may perceive it to be less unpleasant than optical colonoscopy, and may, therefore, be more willing to undergo CT colonography. In order to improve the rate of screening for colorectal cancer, it would be nice for consumers to have evidence-based information about both tests. Some consumers might choose the better test; others might choose the test they believe is less invasive. In any event, hopefully such consumer-centric information would improve rates of colorectal cancer screening.

Treatment of localized prostate cancer is another example. Consider somebody who is otherwise well, who's only crime, so to speak, was being 50-years-old and having a prostate-specific antigen (PSA) test as part of his preventive examination, finding it elevated, eventually having a four-quadrant biopsy, and finding that he has localized prostate cancer that is relatively mature based upon Gleason scoring. What is he supposed to do? There's more than one correct answer. Should he have a radical prostatectomy, with its attendant morbidities like urinary incontinence and erectile dysfunction? Radiation therapy? Periodic reevaluations without treatment unless he becomes symptomatic or his PSA level rises? It would be nice if the pros and cons of any of the approaches could be explained in a rational way, so that people can make rational decisions, again recognizing that not everybody is going to make the same decision. The way things work right now, at least in my experience, is the kind of doctor you go to is very likely going to guide the consumer's decisionmaking. If [the

man] sees a urologist, he will be guided, probably, toward a radical prostatectomy. If he goes to a radiation oncologist, he will likely be directed toward either seed brachytherapy or some form of external-beam radiation. And if he goes to his family doctor or general internist, he may be directed to any number of places. It would be nice if there were information available so that people would be able to make rational decisions in an objective way based upon their individual values and their individual needs.



MICHAEL FORDIS: What about drug treatments? Does comparative effectiveness research help there as well?

DICK JUSTMAN: I think you're going to find that when you get into treatments — again I'm speaking from the perspective of a payer — when you talk about medical treatments that are administered as a medical benefit, you're going to find that the scenarios are pretty much as I just described and I think as Alan alluded to also. When you talk about drug treatments that are administered as part of a pharmacy benefit, life becomes a little bit more murky. And it becomes more murky because in our standard benefit design, all of the drugs are covered. The difference is going to be what tier they are covered on. In other words, are they going to pay a high copayment? Are [patients] going to pay a lower copayment? Are they part of a health-savings account where, until the deductible is satisfied, they're fully responsible for the cost of treatment?

In the world, for example, of glyce-mic agents for type 2 diabetes, the problem is not that there are not enough treatments. The problem, in my opinion, is there are too many medications, some of which actually have a very, very dubious incremental benefit over other medications. Most people with type 2

diabetes do not, for whatever reason, go to endocrinologists; they are treated by primary care physicians. So from the standpoint of a physician who ultimately is going to be guiding these decisions, how does one make, in a particular situation, a rational decision with regard to what is first-line therapy [and] what is second-line therapy? What are the indications for monotherapy? What are the indications for combination therapy? With regard to the issue of insulin analogues, what is the role of insulin analogues for individual patients? What are the issues that need to be addressed? And from

the standpoint of patients with diabetes, there are at least four different areas that need to be addressed. One is the area of adherence. In other words, the treatment that you have to take seven times a day and [requires] you to test [your blood sugar] seven times a day is less likely to occur than a treatment that needs to be done three or four times a day. The second question is one of safety. Safety from the standpoint of glycemic agents has to do with hypoglycemia, both symptomatic and asymptomatic. The other issue that needs to be addressed is effectiveness, [which] is kind of a murky issue because of the question of definition. Do you define effectiveness by [the level of] glycosylated hemoglobin? Do you define it on the basis of fasting blood sugar? Do you define it on the basis of postprandial blood sugar measurement? As your excellent clinicians summary guide on premixed insulin analogues pointed out, there's no single answer that's going to be able to address all of those things. And, the final [factor] that we need to look at is the issue of cost, as experienced by the consumer, because that is going to affect adherence and duration of treatment. So, those are things, that from the standpoint of usefulness to us, need to be addressed wherever we talk about drugs that are administered as a pharmacy benefit.

DAVID HICKAM: If you are making a decision about several different drugs that are all going to be covered in the benefit plan, but may be covered in different tiers, are they in different tiers because the plan has evaluated the evidence and made a judgment about comparative effectiveness?

ALAN ROSENBERG: We do that using whatever clinical information is available — whether it's a comparative effectiveness report, drug-specific evaluations, or just individual trials of the drugs using a placebo control — where we have to undertake a best-estimate evaluation based on the nondirectly comparable evidence. And the determination is made by a pharmacy and therapeutics committee comprised of physicians from across the country in our networks. We also have input externally from subspecialists that help inform those determinations. After we make that decision, we follow a set of rules that an inferior drug will never be placed on a higher tier, requiring lower co-pays than a drug that's superior. So we do place those drugs that are more effective in a lower cost tier than those that are less clinically effective. But if they're comparable based on other factors, we may tier them based on cost — based on our cost. The tiering decision is made after the clinical evaluation is complete by a value assessment committee that's composed of WellPoint leadership staff. In summary, we apply the comparative effectiveness research when available to the question at hand. We do prefer head-to-head comparisons; however, frequently we find too little head-to-head evidence about drugs within a class. Also, there is often little information about the variability of specific subsets of patients, such as [in] comorbidities. I do wish there were far more of those studies being done by the academic research community.

DICK JUSTMAN: With regard to drugs, aside from tiering, we also have clinical programs. For example, there might be notification criteria. In other words, to make sure that when somebody orders

Proscar® that they are, in fact, ordering it to treat symptomatic benign prostatic hyperplasia and not male pattern baldness. We also have quantity limits. In other words, the number of units of a medication that would be able to be obtained with a single co-pay, we need to make sure that those are rational. For example, when the Centers for Disease Control and Prevention (CDC) released guidelines on the use of antivirals to treat H1N1 flu, we went back to make sure that the quantity limits allow overrides that would be consistent with those recommendations so that we would not have consumers in a conundrum where their pharmacy benefit is at odds with what the CDC is telling them they should do.

STEPHANIE CHANG: Are the issues different with the coverage of technologies?

ALAN ROSENBERG: The biggest difference is the benefit plan structure. There isn't tiering in the same way. It's either medically necessary or not.

DICK JUSTMAN: Generally, for example, our benefit structure would be that it's covered if it prevents, diagnoses, or treats a disease, unless we consider it to be investigational, unproven, cosmetic, or custodial or unless it's already on a list of excluded services. For example, you'll never find in our benefit document that appendectomy is or is not covered or that inguinal herniorrhaphy is or is not covered, whether it's [performed as an open surgery] or laparoscopically. What you would have to know is that an inguinal herniorrhaphy is not unproven. It's not investigational. It's not cosmetic, and it's not custodial. Because no benefit exclusions apply, it would be a covered health service. And medical policies will usually help with that kind of determination.

MICHAEL FORDIS: How do you make decisions when there is little evidence, or lower quality evidence?

ALAN ROSENBERG: In medicine, that's not an unusual occurrence. There are many medical treatments or proce-

dures that have been done historically for many years. We love, as Dick mentioned, comparative effectiveness studies, but in the absence of them, you often have to look at whether there's any evidence that A is effective and then that A is as effective, more effective, or less effective than B. As an example, let us consider radiation therapy versus surgery for prostate cancer. The Effective Health Care Program study found very low levels of evidence on whether one is better or worse for the population as a whole or specific subpopulations. But we obviously don't go back and say no to all of these therapies; so, then we try to compare specific types of radiation therapy or specific types of surgery. Unfortunately, once again, we know there are also very low levels of evidence regarding that form of radiation therapy, whether IMRT [intensity-modulated radiation therapy], proton-beam radiation therapy, 3D conformal radiation therapy, or brachytherapy works better. The same can be said for different types of surgical techniques. Recognizing the limitations in the clinical evidence, our organization, through its Medical Policy and Technology Assessment Committee processes, evaluates the evidence, evaluates the history of what has been done, and evaluates how these [techniques] have evolved. Definitions around medical necessity and investigational procedure guide these determinations. WellPoint also obtains input from experts at academic medical centers and from specialty societies. It's a complex set of issues, and it needs to be done on hundreds of clinical subjects. ◀

Dick Justman and Alan Rosenberg both serve as members of the AHRQ Effective Health Care Program Stakeholder Group. Additional excerpts from this interview will be included in future issues.

Newly Developed Methods Improve Comparative Effectiveness Research Quality

THE SCIENCE OF COMPARATIVE EVIDENCE GENERATION and systematic review is rapidly evolving, which means that many of the Methods Guides created by the Effective Health Care Program are considered to be “living documents” that are in a constant state of revision. These articles are designed to provide an update on two of the many projects being undertaken to develop methods that ensure quality reviews and reports.

New Chapters Added to Systematic Review Methods Guide

THE ORIGINAL METHODS GUIDE for Comparative Effectiveness Reviews was created through a collaborative effort between the Agency for Healthcare Research and Quality (AHRQ), the Scientific Resource Center, and the Evidence-based Practice Centers (EPCs) and posted on the AHRQ Web site in late 2007. The Effective Health Care Program intended that the guide would serve as a resource for the EPCs, as well as for other investigators interested in conducting comparative effectiveness reviews.



An updated version has been in progress since

2008. Four chapters of the revised guide have already been published, with versions of the chapters appearing in the *Journal of Clinical Epidemiology*:

- **Forward: Comparing Medical Interventions: AHRQ and the Effective Health Care Program.** AHRQ Manuscript: Published 18 Nov 2008. Also published as: Slutsky J, Atkins D, Chang S, et al. Comparing medical interventions: AHRQ and the Effective Health Care Program. *J Clin Epidemiol* 2008 Sep 30. [Epub ahead of print]
- **Principles in Developing and Applying Guidance for Comparing Medical Interventions.** AHRQ Manuscript: Published 08 Aug 2009. Also published as: Helfand M, Balshem H. Principles for developing guidance: AHRQ and the Effective Health Care Program. *J Clin Epidemiol* 2009 May 6. [Epub ahead of print]
- **Assessing Harms When Comparing Medical Interventions.** AHRQ Manuscript: Published 18 Nov 2008. Also published as: Chou R, Aronson N, Atkins D, et al. Assessing harms when comparing medical interventions: AHRQ and the Effective Health Care Program. *J Clin Epidemiol* 2008 Sep 25. [Epub ahead of print]

New Research Methodology Symposium

AS PART OF ITS CONTINUING EFFORT to refine the research methodology for the generation of new effectiveness and comparative effectiveness evidence, AHRQ hosted an invitational symposium on Potential Methods of Conducting Comparative Effectiveness Research at the AHRQ Conference Center in Rockville, MD, on June 1–2, 2009. This symposium provided a forum for scholarly deliberation on new and emerging research methods. Real-time access to the symposium was provided via an Internet (WebEx®) broadcast. The symposium was sponsored by the DEcIDE Network, a part of the AHRQ Effective Health Care Program.

Day one started with brief comments on the role of AHRQ in advancing methods for and the future directions of comparative effectiveness research and, discussing the challenges for the comparative effectiveness agenda. These comments were followed by a day-long session on Optimizing Clinical Heterogeneity and Longitudinal Outcomes: The Role of Study Design and Data Collection Methods.

The morning session on day two focused on Optimizing Clinical Heterogeneity and Longitudinal Outcomes: The Role of Statistical Techniques and Analytic Models. The afternoon session covered topics on Comparative Effectiveness Research Methods: Policy and Practice Applications and Implications.

Proceedings from the symposium will be published in 2010 as an open-access journal supplement, and on the AHRQ Web site.

- **Identifying, Selecting and Refining Topics for Comparative Effectiveness Systematic Reviews.** AHRQ Manuscript: Published 27 Apr 2009. Also published as: Whitlock E, Lopez S, Chang S, et al. Identifying, selecting, and refining topics for comparative effectiveness systematic reviews: AHRQ and the Effective Health Care Program. *J Clin Epidemiol* 2009 June 21. [Epub ahead of print]

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- **Grading the Strength of a Body of Evidence When Comparing Medical Interventions.** AHRQ Manuscript: Published 05 Aug 2009. Also published as: Owens D, Lohr K, Atkins D, et al. Grading the strength of a body of evidence when comparing medical interventions: AHRQ and the Effective Health Care Program. *J Clin Epidemiol* 2009 Jul 11. [Epub ahead of print]

Two more chapters will be published shortly:

- **Using Existing Systematic Reviews to Replace de Novo Processes in CERs.** AHRQ Manuscript: Draft posted for public comment on 26 May 2009.
- **Updating Comparative Effectiveness Reviews.** AHRQ Manuscript: Draft

posted for public comment on 22 September 2009.

Each chapter within the Methods Guide is drafted by a workgroup composed of EPC investigators, AHRQ staff, and Scientific Resource Center staff. This guide describes recommended approaches for addressing difficult, frequently encountered methodological issues when preparing a systematic review.

New draft guidance for conducting systematic reviews on medical tests will soon be available. EPC investigators are developing the guidance documents, which are scheduled to be published in draft form in the fall of 2009.

With the rapid advances in the field of medicine, there are an ever-increasing number of choices for medical testing,

although evidence regarding the benefits and risks may be scant or lacking. Because it is uncommon for randomly controlled trials to be conducted for medical testing, the systematic effectiveness review of medical testing can be challenging. To address many of the

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difficult and significant variations in the reporting of medical testing, the authors of this chapter of the Methods Guide present a structural

outline for the evaluation process. The series consists of three sections covering the evaluation of clinical tests — from considering the context, conducting the review, and synthesizing the evidence — with extra material on the historical context. There also are special issues related to the evaluation of genetic testing and prognostic tests. ◀

New Advances in Shared Decisionmaking Highlight June International Conference

THE 5TH INTERNATIONAL SHARED DECISION MAKING CONFERENCE was held in Boston, Massachusetts, June 14-17, 2009. Specialists in the science of decisionmaking, policymakers, clinicians, and payers of health care from across the globe gathered to discuss the rapidly growing field of shared decisionmaking. Consistent with this year’s conference theme of “vision to reality,” considerable attention was given to the issue of implementation, with a focus on how to promote shared decisionmaking across clinical contexts.

Plenary sessions were conducted by leaders in the field. Glyn Elwyn, M.B. B.Ch., Ph.D., from Cardiff University in Wales argued that shared decisionmaking and evidence-based medicine were one in the same. Jack Wennberg, M.D., of Dartmouth College reviewed the history of research on practice variation, noting striking geographic differences in surgical rates and other preference-sensitive decisions. Gerd Gigerenzer, D.Phil., from the Max Planck Institute

in Berlin argued that physicians can make good decisions using less information, and formal prognostic modeling may be no better than approaches using decision heuristics (or decisionmaking “short cuts”). This provocative area of research raises interesting questions for decision scientists, where it might be argued that decision heuristics can lead to “good” but perhaps not “informed” decisionmaking.

Susan Dentzer, Editor-in-Chief of Health Affairs, talked about the role of shared decisionmaking in U.S. health-care reform. She noted the current backlash against comparative effectiveness research, some claiming it to be no more than a cost-saving or rationing approach, and wondered if shared decisionmaking efforts might meet similar challenges as health-care reform moves ahead. Richard Thomson, D.M., from the University of Newcastle raised the issue that shared decisionmaking interventions could lead to poor outcomes for some patients. In the case of patients with atrial fibrilla-

tion, Dr. Thomson observed that some informed patients would decide against treatment with warfarin, thereby increasing their risk for a subsequent stroke.

In the applied realm, the number of patient decision aids developed and formally evaluated over the past decade has increased tremendously. The Cochrane review of decision aids for patients facing health treatment or screening decisions was recently updated and published in 2009.¹ Fully 55 randomized controlled trials of patient decision aids have been conducted, and the updated review confirmed the 2003 review findings: decision aids improve care in terms of increased knowledge, decreased conflict about decisions, and greater preference for involvement in decisionmaking.² The more recent evidence supports decision aids as enhancing the accuracy of risk perceptions among patients.

The International Patient Decision Aids Standards (IPDAS) Collaboration

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sponsored a workshop on its progress since the 2007 Freiberg meeting. In 2006, the IPDAS Collaboration published the results of an international online Delphi consensus process to identify quality criteria for developers and adopters of patient decision aids. The IPDAS Collaboration used the opportunity to announce plans to update the background documents that served as evidence summaries for drafting and voting on proposed decision aid quality criteria. The background documents are now more than 4 years old, and there has been a substantial increase in the number of patient decision aids developed while the science of decisionmaking has advanced as well.

The conference also launched the second edition of Shared

Decision-Making in Health Care: Achieving Evidence-Based Patient Choice (New York: Oxford University Press; 2009), which is edited by Dr. Elwyn and his colleague Adrian Edwards, M.B., B.S., Ph.D. The book has grown to 56 chapters grouped within the broad areas of evidence-based patient choice, theory, conceptual developments, decisionmaking in practice, and future directions.

The International Shared Decision Making Conference was first held in Oxford, UK (2001), then in Swansea, UK (2003), Ottawa, Canada (2005), and Freiberg, Germany (2007). The event in 2009 was held for the first time in the United States. ◀

1. O'Connor AM, Bennett CL, Stacey D, et al. Decision aids for people facing health treatment or screening decisions. *Cochrane Database Syst Rev* 2009;(3):CD001431.
2. O'Connor AM, Stacey D, Entwistle V, et al. Decision aids for people facing health treatment or screening decisions. *Cochrane Database Syst Rev* 2003;(2):CD001431.

BOOK REVIEW:

This Changes Everything

IN CONTEMPLATING THE GROWING TECHNOLOGY that was transforming science, conservation biologist Michael Soule stated, "Since we have no choice but to be swept along by [this] vast technological surge, we might as well learn to surf."¹ Jerry Parker, Ph.D., and Esther Thorson, Ph.D., both from the University of Missouri, would agree. In a collaboration between medicine and journalism (Dr. Parker is the associate dean of research at Missouri's School of Medicine; Dr. Thorson is dean of the School of Journalism), Drs. Parker and Thorsen provide a comprehensive set of recommendations to health-care professionals, public health officials, and health communication experts attempting to realize the full potential of new media technologies in health communication.

The result is an impressive collection of essays by 41 academics, medical professionals, and policymakers that offers a broad and comprehensive set of recommendations for using technology to its fullest.

For those less familiar with standard health communication theory and application, the book begins with a section that serves as an advanced primer, with chapters covering worldwide health status, health-care models, and racial and cultural disparities and a compact summation of current theory and emerging trends. From there, the focus remains firmly on the topic at hand, with practical approaches to everything from enhancing consumer involvement to e-health for persons with chronic conditions. Although each chapter provides a broad enough overview for the uninformed, the chapters move quickly to identifying (and successfully using) technological tools to accomplish the multiple goals of health. For example, Christina Zarcadoolas, Ph.D., an assistant professor at Mount Sinai School of Medicine, partners

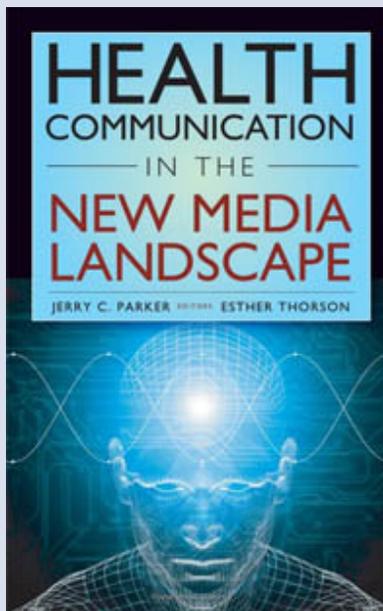
with health-literacy expert and author Andrew Pleasant, Ph.D., to introduce the use of geographic information systems (GIS) mapping for emergency preparedness and to introduce "health literacy load analysis," a structural and functional analysis for clarifying the demands of health-related material on consumers.

Another good example is found in the chapter by Missouri doctoral student Petya Eckler and physicians Gregory M. Worsowicz and Katherine Downey on physician-patient communication. Quickly bringing readers up-to-date on the concept and its theoretical underpinnings, they move quickly to explore the new tools of e-mail, telemedicine, online health information, and cell phone communication. As in many of the chapters in the book, the authors avoid the star-struck wonder of a teenage boy in a Best Buy store; the challenges, barriers, and even cautions are clearly identified, and the trade-offs between technological abilities and effective communication are often discussed.

The final section outlines the future, and includes a superb chapter on evidence-based communication messages and knowledge translation by Boston University's E. Sally Rogers, Sc.D., and Marianne Farkas, Sc.D.

One theme that resounds throughout the book is the acknowledgment that the new media has already affected the ways in which American health consumers think about health and health care. The future has already arrived — at issue is how well those in the field of medicine and health care are able to respond. ◀

¹ Soule M. In: Western D and Pearl MC, eds. *Conservation for the 21st Century*, New York: Oxford University Press; 1989.



Jerry C. Parker and Esther Thorson *Health Communication in the New Media Landscape*. New York: Springer Publishing; 2009