# Making Sense of Comparative Effectiveness

## Policy Analysis Overview 2009

We in health policy often don't understand how complex our systems are and what manner of challenges—some of which we created—present themselves in clinical care.

It was Dr. Christine Sinsky who enlightened me. She said that just five years ago, a patient with a 143/82 blood pressure, 1.4 creatinin, 128 fasting blood sugar and 189 cholesterol was advised to exercise and lose weight. Today, that same person has hypertension, stage 3 kidney disease, diabetes, hyperlipidemia and is a candidate for four medications and ongoing monitoring.

Citing data from the *New England Journal of Medicine*, she added that a Medicare patient with diabetes, hypertension and depression with a complaint of headaches is subject to 56 different quality measures.

The complexity of those—and other—clinical situations is something she manages with smart system integration; no doubt, you've seen her publications on those methods. I can't help but wonder if CER—not as it is imagined and promised, but as it will be realized—will help or hurt clinicians like her and patients like hers. As a policy-maker of longstanding, I have to admit that we generally had the best of intentions, but always created negative unintended effects.

Will we do that again? Have good intentions but negative effects? Only time will tell. Since all of us will soon be walking the territory, I'm offering the beginnings of a map in this blog; a map intended to navigate the territory better. I look forward to the ways others might add to our collective understanding of the landscape. The map is not the territory, as they say, but I believe that the better we explore it now, the better the chance will be that those who traverse it can avoid Donner Pass scenarios.

I currently see seven components in the CER territory and each of those has a set of interrelated policy issues. I'm interested if you see the territory differently and if you have components and policy issues that illuminate this endeavor further. As you do, remember Dr. Sinsky.

1. **Players.** ***Who is engaged?***
   - Public sector; e.g., AHRQ, CMS/QIOs, FDA, NIH, VA/DoD, State Governments
   - Private sector, including for-profit groups; e.g., Leapfrog, NBCH, NBGH, NCQA, PBMs, Health plans, insurers, URAC, JACHO, medical centers, producers of products and services (biopharmaceuticals, devices, games), CROs
   - Public-Private Joint Groups; e.g., NQF, OHSU Center for Evidence-Based Policy
   - Foundations; e.g., RWJ, CHCF
   - Academics, professional societies and study groups; e.g., Institute for Clinical and Economic Review (ICER), American College of Physicians (ACP),Drug Effectiveness Review Project (DERP), NY Academy of Medicine Evidence Based Medicine Resource Center, IOM, Rand
   - Consumers; e.g., Patient advocates, prescribers, professional societies
   - Global Influencers; e.g., NICE

2. **Targets.** ***What do they propose to study?***
   - Prevention; e.g., primary, secondary, tertiary
   - Diagnosis; e.g., pt/caregiver report, lab assessment, imaging
   - Condition-related; e.g., all hypertension control methods, singly or in combination
   - Treatments: e.g., high cost-low volume, high volume-low cost
   - Chronic care and maintenance
   - Palliative or supportive care; e.g., anti-nausea drugs used with chemotherapy
   - Health care delivery system options; e.g., office care vs. telemedicine, physician vs. physician extender

3. **Evidence Types.** ***What types of evidence will be used to conduct studies?***
   - Economic/claims analyses
   - EHR records/data mining
   - Clinical guidelines
   - Expert opinion
   - Registries
   - Practical clinical trials
   - Predictive modeling

4. **Evidence Hierarchy. Will traditional evidence hierarchies guide preferences for data sources?**
   - Meta-analyses of individual patient data
   - Large multi-center RCT
   - Meta-analyses of group data
   - Small, single-site RTCs
   - Prospective cohort studies
   - Case control, retrospective cohort or cross-sectional studies
   - Poorly controlled studies
   - Uncontrolled studies
   - Inductive, observational studies

5. **Uses.** ***How will the results be used?***
   - Decision support by clinicians and patients
   - Educational for clinicians and patients
   - Coverage and payment determinations
   - Marketplace entry clearance through product licensure, accreditation or contracting
   - Pay-for-performance
   - Evidence-based clinical guideline updates
   - Quality report cards
   - Consumer reports

6. **Results Communications.** ***How will results be communicated?***
    - Federal agency publications
    - Professional society peer-review journals
    - Coverage bulletins
    - Web-sites
    - Lay press

7. Results ***implementation*** and ***outcome expectations***. ***How will results be implemented and when will outcomes be expected from the studies?***
    - Implementation; e.g., next insurance contract year, first day of next quarter
    - Outcome expectations; e.g., price reductions, lower costs of care per episode or course of illness, cost trend decline, patient satisfaction, declines in mortality, improved quality of life

**Each of those seven components has different, but clearly-interrelated, policy questions, of which the following are but a sample.**

***Player***-related policy questions.

- Are the players all on equal footing, or are some more important than others?
- Must players who control major segments of the health care market meet different standards of behavior regarding target selection or evidence type because their decisions are more consequential than the role that others might play?
- Are the players who pay for larger shares of the health care budget more important than the players who do not?
- How can the interests of the various stakeholders be accommodated when so many of those are—and will always be—conflicting?
- How will studies account for the reality that stakeholders each defined *value* in different ways?
- Who will be allowed to conduct useable CER studies? Will credentialing be required of individuals and/or organizations that do CER studies? That is, can anyone with a laptop and data generate a CER, or must they be somehow certified or licensed to do so?
- Can companies do CER studies of their own products in comparison to other modalities (e.g., statin vs. oatmeal)? If so, can these studies be used to change product labels and promotional approaches?
- Can patients opt out of databases on which CER is conducted? If so, what are the consequences for their care and the validity of the data?
- How will patient outliers be protected? What if patients are harmed by the results? Who is accountable in the chain of those who study, communicate and make decisions based on the results?
- Will there be exceptions or exclusions for rare diseases, and for areas of disease care with considerable unknowns?

***Target***-related policy questions.

- What aspects of health care might be/should be/are being targeted for CER?
- Should there be a balance for all of the possible targets selected?

- Must there always be 'cross-target' comparisons (e.g., between treatments and prevention) or should it be type-type targets (drug-drug)?
- How will the various priorities be set and who will do that? If funders of health care fund the studies, is that evidence of conflict of interest? Likewise, if product or service companies fund the studies, is that evidence of conflict of interest?
- Should 'hidden costs,' (e.g., economic and consequences for caregivers) be accounted for in the selection of priorities or are direct medical costs the only interest?

***Evidence-type*** related policy questions.

- Will some types of evidence be preferred over others?
- Should some types of evidence be disallowed?
- Will cross-type comparisons be allowable?
- Since some evidence involves self-report on the part of patients or clinicians, how will accuracy be assured?

***Evidence hierarchy***-related policy questions.

- Will there be a preferred hierarchy of evidence?
- How much evidence is required? What number of studies and quality of studies is required?
- Must all CER studies account for age, gender, racial, ethnic and health system diversity?
- Since few studies do account for all those distinctions, what provisions will be made for patients who are in those groups if decisions are made as a result?

***Use-related*** policy questions.

- How will the results be used?
- Can results be used to create changes in market entry or payment for products and services?
- If other agencies (e.g., FDA) have approved products and labels, can CER studies be used to create changes in those regulatory rulings?

***Communication-method*** related policy questions.

- How will the results be communicated? By whom? In what timeframe?
- What requirements will there be for updating CER results and for rescinding those published in error, in unclear ways or prematurely?
- What provisions will be made to account for low health literacy levels and diverse cultural and language groups?
- Do communicators have a responsibility to monitor errors in 'downstream' communications and misinterpretation of results?
- Does the communicator of the CER study 'own' the information? For example, if a professional society is selected as the principal communicator of the evidence in their area of clinical expertise, does it 'own' the published information? For areas where more than one professional society serves a field, how would the most appropriate society selection be accomplished?

***Implementation- and outcome-***related policy questions.

- $1.1B is hardly sufficient to implement CER. How will adequate funds be secured? Will CER-related user fees be imposed on products and services entering the market? Can private plans or payers supplement funding? Will provider types pay user fees?
- What is the reasonable timeframe for implementing the CER results? How long does a patient, clinician, payer or health system have to adapt to the change?
- Given contract timeframes, if CER evidence results in recommendations for more costly care (e.g., through increasing volume through improved access, or increasing intensity with higher-cost innovations), what is a reasonable timeframe for adaptation for the health system?
- If new evidence leads to a different conclusion later (as sometimes happens) is the decision-maker (e.g., an AHIP member) held harmless or must they restore payments if the patient has purchased OOP care?
- Will there be oversight of the CER process and if so, who will do that?
- What is the relationship of health care CER to other evidence-based policy-making (e.g., The Council for Excellence in Government)?
- How and when will CER effectiveness overall be judged? What metrics will determine its success or failure: process measures such as the number of studies or changes in health care practices, or outcome measures such as lowering of rate of disease incidence or health cost increases?
- If CER becomes a condition of coverage for new drugs, imaging or therapeutic devices, does this unfavorably impact those innovations most needed now? Can exclusions be granted if data are not adequate for comparisons, for rare diseases, or for areas of unmet need?