Rethinking Comparative Effectiveness Research

Opponents to the new CER legislation are raising red flags, warning about a one-size-fits-all treatment approach and the rationing of healthcare. But Dr. Donald Berwick, president and CEO of the Institute for Healthcare Improvement, says to look at which interventions are effective — but not what they cost — is irrational and simply bad policy.

An Interview With Dr. Donald Berwick, President and CEO, Institute for Healthcare Improvement

Comparative effectiveness research on a national basis is now a reality. In March, the United States Department of Health and Human Services, one of three government agencies that received CER funds under the American Recovery and Reinvestment Act of 2009 — the other two were the National Institutes of Health and the Agency for Healthcare Research and Quality — named the 15 members of the new Federal Coordinating Council for Comparative Effectiveness Research. The Council is already holding public listening sessions on how to use CER to reduce ineffective and costly medical treatments.

As many predicted, having a federal agency evaluate the comparative effectiveness of prescription drugs, biologic and other targeted therapies, and medical treatments is raising hackles in the healthcare industry. Opponents have initiated a massive campaign to eliminate or water down many of the legislative provisions.

Katherine T. Adams, senior editor of Biotechnology Healthcare, recently spoke with Dr. Donald Berwick, president and CEO of the Institute for Healthcare Improvement, about the value of CER — and whether it will help rein in the runaway healthcare costs in this country. Their interview begins on the following page.

Q: Are we on the right track with a federal CER agency?

A: The United States is not the only country struggling with healthcare costs. The National Institute for Health and Clinical Excellence (NICE) in the United Kingdom and also, to some extent, the Institut National de La Sante in France have developed very good and very disciplined, scientifically grounded, policy-connected models for the evaluation of medical treatments from which we ought to learn.

The mythology about these systems is very toxic. Indeed, those organizations are functioning very well and are well respected by clinicians, and they are making their populations healthier and better off. Nor are their policies resulting in injury to patients in any way like what is being speculated here in the United States. These organizations have created benchmarks of best practices that we could learn from and adapt in this country.

Q: NICE is a bogeyman here in the United States.

A: I know that, and it's a misunderstanding of the deepest sort. NICE is extremely effective and a conscientious, valuable, and — importantly — knowledge-building system. The fact that it's a bogeyman in this country is a political fact, not a technical one.

Q: How would CER work best in the United States?

A: If you take what people call CER, there are three different levels of analysis. The first level is a simple evaluation of effect: Does this drug work at all? We have the scientific enterprise to do that, but it's not as developed, invested in, or as independent as it really should be. The nation's investment in the continuing evaluation of new medications — including the biologics — and technologies is essential. The people who make biologics have a strong interest in showing that they *are* effective and not in finding out *if* they are effective.

The second level is comparative effectiveness, which means that when a drug, device, or treatment is offered, it is not offered against a zero status quo. Having a CER agency asking how much more do you get with B than with A, instead of with B compared with nothing, is an important enterprise. That is at the heart of what CER really ought to be — a well-informed comparative assessment, not an exercise that pretends nothing else exists.

The third level is an analysis of cost-effectiveness. If a new drug or procedure is effective, and has some advantage over existing alternatives, then does the incremental benefit justify the likely additional cost?

Q: So you are saying that the federal CER agency should get involved in cost determinations?

A: You can say, "Well, we shouldn't even look." But that would be irrational. The social budget is limited — we have a limited resource pool. It makes terribly good sense to at least know the price of an added benefit, and at some point we might say nationally, regionally, or locally that we wish we could afford it, but we can't. We have to be realistic about the knowledge base. The degree to which that is linked directly to policy and decision is a matter of choice. You could make it advisory, or you could make it mandatory, or you could make it a policy rule. But to remain ignorant of the cost implications of a drug that is marginally better than what is already out there is simply bad policy.

Q: Critics of CER have said that it will lead to the rationing of healthcare.

A: We can make a sensible social decision and say, "Well, at this point, to have access to a particular additional benefit [new drug or medical intervention] is so expensive that our taxpayers have better use for those funds." We make those decisions all the time. The decision is not whether or not we will ration care — the decision is whether we will ration with our eyes open. And right now, we are doing it blindly.

Q: A national CER decision, as envisioned under the American Recovery and Reinvestment Act, will ask all the stakeholders to accept which medical treatment is most effective and affordable for the appropriate patient population. But each stakeholder has varied and inherent interests. How do we get consensus? And how does that consensus reach the patient?

A: Since we have no real healthcare system, it's hard to deploy knowledge. We do it through awareness, communication, exhortation, and professional norms, and also through individual organizations and their activities. It's a complex and somewhat unreliable system — getting from knowledge to action.

I think we should, for now, appeal to professional values and mission to get a consensus among all the stakeholders in healthcare. I do think that clinicians and hospitals want to do well by their patients, and a much more accessible and

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visible knowledge base that is made routinely available to all concerned at the point of decision will help. Whether you have to add edge to that with requirements, I don't know. I would like us to avoid getting too much into a check-off or compliance-oriented approach. I think we should appeal to professionalism as much as possible.

Q: So do you think that any mandatory compliance with CER directives could be dangerous?

A: Yes, if you overdo the tightness of the connection between the knowledge of effectiveness and the rules of compliance. Then you get into the "proletarianization" of medicine — physicians, payers, and patients being told what to do instead of being able to use their own judgment. There's a balance here between advisory declarations with enough knowledge that they really have some force and requirements. I think we should take it slowly. That doesn't mean we shouldn't develop the knowledge — we should have it, and that's where the investment should go.

There's one other kind of requirement I wouldn't hesitate to make. When we're uncertain about a biologic or a new technology such that it seems promising, but we don't know [enough about it] yet, then on the payer side — Medicare, Medicaid, private payers — the rational thing to do is to say, "All right, for now we certainly don't want to stand in the way of the use of this innovation but there is a requirement to generate information so that we understand its performance better. So the deal is, you can use it if — and only if — your use contributes to a common base of knowledge about this technology or biologic." And I think we can do that.

Q: CER sounds like evidence-based medicine.

A: It *is* evidence-based medicine. Anyone who says differently does not understand evidence-based medicine. It means you are practicing according to knowledge. And it should apply across the board — procedures, clinical strategies, and biotechnology.

Q: What should managed care payers and employers be thinking about in the context of a national CER policy?

A: First, don't dismiss the foreign experience. CER is not toxic or a bogeyman — it's informed and helpful. There are many other countries that are dealing with it appropriately and conscientiously.

Second, let's always assume that daylight is better than darkness. So a general policy framework, whether you're a payer, employer, or clinician, should be that it's better to have the knowledge than not to have it. If you're on the side of an argument in which the other side wins and you are left ignorant, don't buy it. We want knowledge on all three levels — effectiveness, comparative effectiveness, and cost effectiveness — which is valuable in guiding both individual choice and public policy. It's not a formula for comfort — it's a formula for constructive discomfort.

I think what will happen is that when we do turn the lights on, we will have an opportunity to face some of the difficult decisions that need to be made. We can then bear the responsibility for making those choices.

I would say, "Please, employers and payers, ask for knowledge and transparency and invest in it. It doesn't come free; there has to be some payment, but it won't break the bank." Gathering valuable knowledge and transparency need not be inordinately expensive processes.