

SELECTED SUMMARY

How does a nation decide what healthcare to pay for?

SRIDHAR SRIKANTIAH

Medico Friend Circle. Address for correspondence: 302, Shantam Residency, 3 Harinagar Society, Vadodara 390 023 INDIA email: sridharmfc@yahoo.com

Susan Gilbert. The nesting-egg problem: why comparative effectiveness research is trickier than it looks. *Hastings Center Report*, 2009 Nov-Dec; 39(6): 11-4.

The US healthcare debate is an old one and has long defied easy solutions. But it continues to entertain, and to stimulate. President Obama's ongoing attempts to persuade the nation to give up a degree of perceived freedom of choice in healthcare, so that basic healthcare could become available to all, has injected more energy and at least a temporary sense of urgency into the debate. A series of essays in the November-December 2009 issue of the *Hastings Center Report* highlights some of the current struggles of that society to provide itself an acceptable system of determining who has access to what kind of healthcare. It is easy to dismiss some of these struggles as self-imposed, but they are instructive for the rest of the world, particularly for more chaotic democracies such as ours, and especially in the context of rapidly increasing options in diagnostic and therapeutic technologies.

The current US government has begun to move towards containing healthcare costs and thus spreading access more evenly. One of the several initiatives in this direction has been to fund comparative effectiveness research (CER). CER, in simple terms, is research directed at comparing newer technologies to known ones for relative effectiveness. The premise for funding this is straightforward: in the US president's words, "There is going to be some disagreement, but if there's broad agreement that in this situation, the blue pill works better than the red pill, and it turns out the blue pills are half as expensive as the red pills, then we want to make sure that doctors and patients have that information available to them." However, notice that this statement is carefully calibrated: he does not say, "... then we want to make sure we have a policy for doctors to use the blue pills instead of the red." As Susan Gilbert points out, there are very good reasons why he cannot say this, and why CER alone may not allow him to say it.

For one, CER has certain inherent limitations. Gilbert calls one of them the nesting-egg problem, where the research opens up more questions than it answered. She quotes two recent studies that compared vertebroplasty, an expensive form of treating osteoporotic fractures of the spine in the elderly, involving the use of injections of acrylic cement into the damaged bone, to a placebo injection. Both studies found that both treatments provided a certain degree of pain relief but were no different

one from the other. While one concluded that vertebroplasty could not be recommended, the other called for more studies, considering the possibility that the anaesthetic used in both injections might have had something to do with the extended pain relief. Presumably, such conclusions indefinitely prolong the wait for definitive answers.

The other inherent limitation is that most CER studies are unlikely to provide more than a relative idea of effectiveness in populations of patients on the assumption of randomisation. The research usually ends up concluding, say, that treatment A is 10% more likely than treatment B to provide relief or produce a cure. The study is usually not designed to say which subgroup constitutes the 10% who benefited, or whether at all such a subgroup exists. This evidence alone is therefore not sufficient to dictate what a physician should do in an individual case, and there is justified concern that such research should not lead to "cookbook" practice, where the physician is forced to abandon the more commonsensical approach of tailoring treatments to the contexts of individual patients. Apparently, last year's economic stimulus bill, of which the CER funding was a part, specifically prohibits the use of reports or recommendations of such research "as mandates or clinical guidelines for payment, coverage, or treatment". Sensible, but then, how does the government justify why it funded the research in the first place, if there is no way to ensure its use? Apparently, the federal structure allows individual states to then determine how to use the findings of these studies. For some years, now, a couple of states have apparently been using evidence from such studies to decide what treatments to offer under state-funded insurance schemes to good effect, achieving modest cost savings and the maintenance of quality, such as by choosing less harmful treatments – also based on comparative studies. For instance, when studies found Merck's Vioxx (rofecoxib, an anti-inflammatory medicine commonly used for arthritis) to be linked to heart disease, Washington state immediately took it off the Medicaid list, well before it was taken off the shelves in the market.

True to form, the centre of the healthcare debate in the US often rests on whether at all the government should involve itself in this matter. The state needs to fund such research, presumably because it needs to direct policy based on evidence, which is often not available, since the free market of privately-funded research has no incentives for generating sufficient evidence. Also, the case for state-funded research

gains impetus each time there is suspicion of bias in industry-funded drug trials, a phenomenon that is increasingly evident. Gilbert quotes Marcia Angell, a former editor-in-chief of the *New England Journal of Medicine*, to make the point that industry-sponsored trials “consistently favor sponsors’ drugs – largely because negative results are not published, positive results are repeatedly published in slightly different forms, and a positive spin is put on negative results.” The opposition to independent, government-sponsored, assessment of technologies has come, not surprisingly, from two groups – professional physicians’ associations and the health industry – which unfortunately can no longer be assumed to represent mutually different interests today. Previous attempts by the federal government and the Congress to generate independent evidence have been scuttled because such groups became incensed at “governmental intrusion.” The example provided, relating to one such federally-appointed agency being virtually disbanded because it dared to issue a guideline that stated, on the basis of solid evidence, that much of the spinal surgery being performed in the country was unnecessary, is sobering. The body was renamed, and now merely provides advice (on a website) that is binding to no one, not even government-funded health insurance.

Nevertheless, following the new funding for CER from the stimulus bill, a fresh start has been made. A committee of experts constituted by the Institute of Medicine leads the effort to determine how best to use the funds. A shortlist of 100 priority topics for research has been drawn up from a larger list that was canvassed from all interest groups, based on a range of concerns: conditions whose treatment is costly and varies widely across the country, conditions that are common but whose current treatments lack evidence of effectiveness, conditions that affect vulnerable groups such as the elderly and African Americans, and health problems of specific groups such as women and people with disabilities. Some of the top priorities include low back pain, attention deficit hyperactivity disorder, dental cavities and emotional disorders – illustrative of the nature of the prioritisation, and a refreshing departure from the esoteric choice of research topics when business is as usual. With assured funding for at least two years*, and a large and capable base of researchers, evidence should soon start becoming available. Considering the burnt fingers of yore, how this evidence will be used is something no one yet wants to bet on, and it will be interesting to see how the society adapts to new realities.

The US context is one of a democratic society where a lot of medical care is provided by private, for-profit providers,

paid for by a mix of private and state-funded insurance and regulated by a “free” market in which the health industry wields enormous influence. It is a society that instinctively abhors regulation by the government, and loves to litigate. Other prosperous democracies, such as in western Europe, have evolved significantly different models of healthcare, apparently because there is greater acceptance of the role of government in regulating and providing for healthcare. While we in India can compare the relative merits and learn lessons from these and from other models in socialist states, it is particularly instructive to note the methods adopted to steer what are ultimately moral, ethical and political decisions.

One of the most important reasons to engage in CER is to understand healthcare costs and keep them manageable, even while improving effectiveness of healthcare. The current initiative for CER in the US began with a candid assessment of the fact that healthcare costs were irrational, in the sense that differences in healthcare costs across regions and countries did not co-vary with health outcomes, and that these high costs were a challenge for both, the government and the private sector (1). The initiative then survived a major change in the federal government, procured a funding of \$1.1 billion from the current dispensation, and has made a solid start in identifying priorities for research. From the perspective of India, where policy making is led by hazy evidence at best, this willingness of a government to seriously engage in generating and examining evidence about the effectiveness and real costs of healthcare should be seen as remarkable. The jury is still out on whether this initiative will lead to more accessible healthcare for all, much less improve health, but the US is at least taking a rational approach to determine how to move forward. The approach is probably more relevant for us in India than the specific results of research. Such an approach, which looks closely at our own situation to find a package of services that will be beneficial to most and at a cost that we can afford, may not solve all the problems of healthcare access, but is surely a necessary step in that direction. Engaging the public in examining such evidence, owning it, and determining for itself what is desirable, may actually make the task easier.

** The Institute of Medicine has made a strong case for making this an ongoing funding priority of the government.*

Reference

1. Congressional Budget Office. Research on comparative effectiveness of medical treatments: issues and options for an expanded federal role. Washington, DC: Congress of the United States. Congressional Budget Paper; 2007 Dec.