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J A Wennberg
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In his scholarly and exciting report on the history of the Agency for Health Care Policy and Research (AHCPR); Brad Gray rightly cites the practice variations phenomenon as the intellectual shock that first brought the crisis in the scientific and ethical bases of medicine to the attention of Congress. In my testimonies I tried to drive home the point that variations arise because of two fundamental defects in health care markets: (1) weaknesses in the clinical science that occur because medical ideas and theories are not well tested; and (2) weaknesses in the ethical basis for clinical decision making that allow the physician’s preferences for outcomes and treatments to dominate the choice of treatment when the right of choice properly belongs to the patient.

The variations that exist between care of residents of New Haven and Boston, two of the nation’s most medically sophisticated cities, were particularly useful in raising the “which rate is right” question in the halls of Congress. Many were surprised to find out that New Havenites with angina were more than twice as likely to be treated with surgery than were Bostonians, who more often received alternative treatments such as angioplasty or drugs.- Bostonians with threatened stroke had twice the risk for surgical management, while residents of New Haven were more often prescribed aspirin.

I also made the point that the magnitude of the problem exposed by practice variations required a new initiative in federal science policy. The nation’s extraordinary investments in biomedical research do not provide for the evaluation of medical theories. The role of biomedical science is to generate ideas and technologies; it is the role of the evaluative sciences to provide the necessary clinical information linking treatments to outcomes. But the evaluative sciences are neglected; their mission, their intellectual content, and their niche within academia and
federal science policy are underdeveloped. I called for an adjustment in funding priorities to jump-start the evaluative sciences, but I did not want to start the inevitable budget war that would occur if the growth of the evaluative sciences were to be fueled by general tax revenues and therefore at the possible expense of the National Institutes of Health (NIH). This is why the Proxmire bill called for a “tax” on health care dollars, setting an ambitious level of funding-equal to 0.5 percent of the Medicare trust fund. What emerged was legislation that was less by an order of magnitude in its budgetary authorization. As Gray explains, subsequent history shows how sensitive NIH is to even a modest demand on its resources when they go to a “new kid on the block.”

The commitment of AHCPR to deal with the weakness in the scientific basis of medicine is embodied in its Medical Treatment Effectiveness Program (MEDTEP). The centerpiece is the so-called Patient Outcomes Research Teams (PORTs), an idea that first emerged in the 1986 Durenberger bill. Experience shows that there are three main tasks facing the work of the PORTs. Here I use my experience as principal investigator of the prostate disease PORT to illustrate these tasks.

### Diagnosing And Resolving Variations

Outcomes research is a classic illustration of how science often begins with the observation of unexplained variation. We asked urologists in Maine why their rates of surgery varied as much as fourfold among different communities in that state. We found that their practice styles were guided by two conflicting theories. First, some urologists believed that prostate disease in most men would progress to the point at which the kidney or bladder was obstructed. If treatment is postponed until obstruction occurs, the patients are older and sicker, and their mortality rates are higher (true enough). If each one receives surgery early on, on average, men will live longer (an untested hypothesis). A second group of urologists believed that the progression to kidney or bladder obstruction was relatively infrequent. Their principal reason for doing surgery in men without kidney or bladder obstruction was to relieve symptoms and improve the quality of life.

We were able to show that the preventive theory was incorrect—that the main reason for doing surgery was to reduce symptoms and improve quality of life; this it does well about 80 percent of the time. But-and here is the rub-some patients, even those who were severely symptomatic, said they were not bothered all that much by their symptoms. Patients also differed in their attitudes toward treatment risks, particularly surgery-induced impotence and operative mortality. It became
apparent that there was no information in the patient’s medical history, clinical examination, laboratory findings, or level of symptoms that could serve as the basis for prescribing surgery. Patients' attitudes toward the risks and benefits of watchful waiting versus surgery were the critical factors. In a nutshell, reducing unwanted variations depends on learning what patients want, and this can only be ascertained by asking patients.

PORTS are also responsible for the broad dissemination of their results. Many share the opinion that this task is best accomplished by practice guidelines targeted to physicians. Yet our diagnosis of the reasons for variation points in another, complementary, direction. The patient must also be informed. To help inform patients that they indeed have a choice and that their choice should depend on their own preferences, we developed a standardized way of conveying the results of our research to patients in the clinical setting. We learned that patients truly want to participate in the choice of treatment and that when they do, they make decisions that more closely fit their own preferences than when decision making is delegated to physicians. To the astonishment of many in our research group, only one of five severely symptomatic men actually chose surgery when informed that they had a choice.

Networks Of Evaluation

I must mention a third task in the development of the PORTS, which I believe provides a good deal of food for public policy thought. The past five years have seen an explosion of the medical imagination; new ideas about how to treat benign prostatic hyperplasia (BPH) are emerging from all quarters. Here are some examples: (1) Balloon dilation: Reasoning by analogy, a Boston urologist supposes that the balloon angioplasty technology being applied to unclog coronary arteries might be adopted to treat BPH. He invents balloon dilation, a strategy in which the offending prostate tissue causing urination problems is stretched and pushed out of the way by inflating a balloon that is first inserted as part of a catheter. (2) Prazosin: A drug approved by the Food and Drug Administration (FDA) for treatment of hypertension is adopted for use in BPH as physicians note that patients who are taking this drug for their blood pressure seem to gain some relief from their urinary symptoms. (3) Proscar: A major drug company undertakes an elegant research project to come up with a drug that can reduce prostate size by blocking the hormone that contributes to growth of the prostate. (4) Hyperthermia: NIH's investment in biomedical technology produced a machine capable of heating organs selectively. Although it was designed to treat cancer, curious researchers try it out on the prostate, where it appears to
cause some shrinkage. (5) Stents: Another physician invents the stent, a device inserted into the urethra to shore up the offending prostate tissue (rather like a mine shaft is shored up with timbers to prevent cave-ins). (6) Transurethral incision of the prostate (TUIP): Some surgeons invent a new way of doing surgery in which incisions are made into the prostate tissue, but the gland is not extensively removed as in conventional surgery. (7) Laser surgery: In yet another application in the fast-growing field of lasers in medicine, prostates are now being chipped away using a laser in some medical centers.

These new ideas posed a new problem for the BPH PORT. Unlike the situation in the first phase of our work, where a good deal of information on the traditional treatments and outcomes, however flawed, was available in the literature or claims data, this was not the case for many of these new treatments. All that is known about many of them is that they seem to increase urine flow. We knew from our previous work that it is not enough to know whether a treatment improves urine flow. The true test is how well a treatment reduces symptoms and improves the quality of life—and at what costs, in terms of both complications and dollars.

But how can this information be obtained? Apparently, FDA regulation is not the way. At least two of these innovations—Prazosin and TUIP—occur by modifying existing practice, an area outside the FDA’s jurisdiction. FDA procedures governing the evaluation of devices such as stents, balloons, and lasers concentrate on obtaining “proof of safety” and only rarely require efficacy studies. PORTs themselves have no regulatory authority. They depend on the notion that professionalism requires the commitment to learn what works.

We found such a commitment among the leadership of the American Urological Association (AUA). Working together, our group (most often Michael Barry) and the AUA leaders (most often Abe Cockett and Logan Holtgrewe, both of whom have served as AUA president) developed a cooperative strategy designed to assure that all significant new ideas in the treatment of BPH become subjected to the balanced evaluations required to build the scientific basis for clinical decision making. The idea is not for just a single trial, but rather for a network of evaluation. Collaborating centers would undertake a sequence of studies to examine all new ideas and technologies that the PORT believes have sufficient merit to warrant further evaluation. Because these “effectiveness trials” are conducted on treatments that are approved by the FDA, they can be undertaken at a lower cost than trials of treatments that are “experimental.” The collaborative network can be organized at a cost of less than $5 million a year—a bargain compared to most clinical trials. Considering that BPH treatments cost Medicare more than $4 billion a
year, it is also money well spent.

I have come to view the collaboration between PORTs and the leadership of the specialty societies as key to the long-term viability of any nonregulatory approach to learning what works. The AUA/PORT collaboration suggests that the professional leadership model works. But to work in a systematic way, the establishment of networks of evaluation must be part of AHCPR’s MEDTEP. This means that the agency must be given responsibility for their funding and scientific oversight.

AHCPR And Health Care Reform

In the health care debate, one hears very little about the building of a national infrastructure for promoting innovation and the quality of care. The focus is on finance. The message from the medical care epidemiologists and outcomes researchers is clear: Health care is not only about economics. The basic assumption we all make as patients is that health care has value, that the act of utilization is not a sufficient end in itself. For most of what is done in medicine, we do not know what the value is. Once the outcomes are known, we do not know what patients want.

We need a governmental focus on a reform in science policy that brings the evaluative sciences on a par with the biomedical sciences. MEDTEP offers such a focus, but to fulfill this role there must be greater clarity concerning the scope and scale of the mission. There is a certain irony that it should be the British National Health Service (NHS) that understands the scope and scale required to establish the evaluative sciences and develop the infrastructure required to learn what works and what patients want. A program is under way in Britain to spend up to 1.5 percent of the NHS budget on outcomes research and the building of networks among providers to manage quality. As a percentage of budget allocated to the task, this amount far exceeds the 0.5 percent tax on the Medicare budget proposed in the first Proxmire bill. I am convinced that the achievements of AHCPR so far warrant reconsideration of Senator Proxmire’s notion of the scale this program requires to repair the flaws in the scientific and ethical bases of clinical medicine.

NOTE

1. The FDA’s jurisdiction for “proof of efficacy” is restricted to drugs and a few devices. It has no authority to cause the evaluation of surgery or approved drugs used in unadvertised and novel ways such as Proscar. Its authority over new drugs is exercised in the effort to determine whether in some “important attribute” the drug does better than a placebo. In the case of BPH, the metric has usually been urine flow. We found in our studies that urine flow and symptom state are poorly correlated.