Legal Backgrounder

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# "COMPARATIVE EFFECTIVENESS": GOVERNMENT'S WAY TO CONVERT PATIENTS INTO COST CENTERS?

by

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Like other cost-based practices, comparative effectiveness (aka "healthcare technology assessment")—the effort to manage medical technologies by evaluating their relative value—is designed to reduce costs and restrict patient care. But, properly designed in a way to provide and pay for the best care for patients, it can also be patient-centric. Just as we need new and better tools for drug development, we need them for effective measurement as well.

Today, health technology assessment is a short-term, short-sighted, politically-driven policy that results in one-size-fits-all medicine. While it may provide transitory savings in the short-term, current strategies result in a lower quality of care that result in higher healthcare costs over time.

***Comparing Cost Effectiveness.*** The United Kingdom's National Institute for Health and Clinical Excellence (NICE) has issued cost effectiveness decisions for many new medicines. More often than not, after a one- to three-year period of review after a drug has received market approval, the institute has recommended against using the drugs because they are not cost effective compared to existing treatments. That is why policy folks in the UK, instead of using "NICE" prefer "NASTY", for "Not available, so treat yourself."

The drugs the institute has rejected or rationed include Gleevec, a drug that targets a specific pathway that causes stomach cancer. In 2001, Gleevec became frontline therapy for stomach cancer in the United States. To the outrage of most cancer specialists, the institute took three years to decide that it would use Gleevec as a last resort, in a limited dose. It took Gleevec away from patients who have some tumor sites that are responding to treatment and some tumor sites that are not.

The institute has also recommended against paying for Herceptin to treat metastatic breast cancer, as well as other drugs used to treat osteoporosis, Alzheimer's and multiple sclerosis based on comparative effectiveness reviews. Similarly, independent review agencies in Australia, New Zealand and Canada have done the same.

The institute did not regard any new drugs as clinically less effective then older drugs. Rather, officials judged them to be, relative to their cost, not worth paying for given the additional benefit the drugs provided. The benchmark used in each case was something called a "quality of life year" or a year of life free of disease or infirmity.

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Sir Michael Rawlins, chairman of NICE, told the British House of Commons that comparative effectiveness, a means of health technology assessment, is not based on empirical research: "There is no empirical research anywhere in the world, it is really based on the collective judgment of the health economists we have approached across the country," he said. "It is elusive." *See* http://www.publications.parliament.uk/pa/cm200708/cmselect/cmhealth/uc27-i/uc2702.htm.

The problem is that health technology assessment, as it is currently designed, places into conflict the short-term budgeting dilemmas of governments elected for relatively short periods of time with the ever-lengthening life spans of their electorates.

***$50,000 Per Year of Life.*** In every analysis described, health systems assumed an additional quality of life year was worth about $50,000, the average price of a fully loaded Land Rover. Because most new medicines are targeted therapies that are tested first in critically ill patients, it will be almost impossible to demonstrate significant improvement in well-being or life expectancy for any new medication. The United States Medicare Payment Advisory Commission (MedPAC – the independent federal body established to advise Congress on issues affecting Medicare) never mentions a number, but invokes efficiency enough times to make the point that comparative effectiveness is tool for controlling costs, not improving the lives of people. Indeed, it notes "increasing the capacity to examine the comparative effectiveness of health care services," will lead to "[increased] federal administrative spending relative to current law." That would mean more price controls and government interference in medicine.

It happens that MedPAC and America's Health Insurance Plans favor large randomized clinical trials to compare older drugs sponsored in part by government agencies and private companies. Randomized trials tend to ignore differences in clinical outcomes due to side effects or genetic variations. So whether you analyze them together or individually, researchers will most always find no difference in the effect of medicines, a result that is biased in favor of older, cheaper drugs.

Proponents point to two troubling examples. For instance, The Department of Veterans Affairs, along with the National Institute of Mental Health, conducted a study comparing older and cheaper drugs for schizophrenia to newer ones. Patients with chronic schizophrenia were assigned to treatment with perphenazine, olanzapine, risperidone, quetiapine or ziprasidone for up to eighteen months.

The first thing the National Institute of Mental Health and the Department of Veterans Affairs did was exclude people who froze up and went numb from the older drugs, a common reaction that led to the development of second-generation medicines in the first place. Then the study only compared how well each patient did on each drug. Even then, the researchers assigned their own value to the reasons patients switched, substituting their preferences for those of patients and patient groups. Even though most patients wound up staying on newer drugs for longer periods of time—because of side effects that the study devalued—the researchers claimed that the older drugs were cost-effective.

***A Judgment Controversy.*** Another example of the kind of research proponents of comparative effectiveness swoon over is the Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack Trial. That was a five-year, 42,000 patient trial that studied the comparative effectiveness of first-generation blood pressure drugs (diuretics) against second-generation medicines in reducing heart attacks. Patients were allowed to switch to a combination of drugs only if they failed on the older medicine first.

The authors of the study concluded that diuretics were cheaper and as effective in reducing death from all forms of heart failure, if not heart attacks and were therefore cost effective. But like the trial conclusion in the study that compared drugs to treat schizophrenia, this judgment was not without controversy. As Michael Weber, one of the members of the steering committee overseeing the trial noted, the entire cost benefit of the older drugs was based on the fact that African-American patients suffering forty percent more deaths from stroke because they were assigned to precisely the wrong treatment for high blood pressure compared to the diuretics.

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Weber particularly criticized the use of the combination of an ACE inhibitor and a beta-blocker in African-American patients as "absolutely inappropriate." Weber questioned how those responsible for monitoring safety in the trial could have let African-American patients be exposed to certain danger and even death. The Department of Veterans Affairs now has a program where it pays doctors to prescribe blood pressure medications according to guidelines that emerged from the trial.

As currently organized, comparative effectiveness will be used to increase government control over the practice of medicine and expand price controls. It will turn patients into cost centers, not the center of efforts to prevent disease and extend life. Using a combination of cutting-edge information technology and genetic tests, doctors can do a better job than any cost-benefit agency to ensure that their patients get the right treatment at the right time. Many new drugs, including top selling cancer drugs such as Tarceva and Avastin, have associated genetic tests either on the market or in clinical development that will allow doctors to provide individuals with personalized medicine. New drugs for asthma, depression, HIV and blood pressure will have similar tests.

Personalized medicine gives doctors and patients control over healthcare decisions while comparative effectiveness, as it is now defined, will increase government control over the choices doctors and patients make in the future. The battle over the value of medicine and who decides what is valuable will determine who controls healthcare in America over the next decade.

In his movie *SiCKO*, the simplistic and calculated portrayal of how other nations provide healthcare, Michael Moore depicted the British National Health Service and the Canadian health system as particular exemplars of excellence. He backed it up with a lot of statistics, but statistics, as the saying goes, are like a bathing suit. What they show you is interesting, but what they conceal is essential.

And what *SiCKO* concealed was that systems such as those in the United Kingdom and Canada are cost-based rather than patient-centric models. Facts, no matter how inconvenient to one's argument, must not be ignored.

Citizens of countries with government-run health care systems experience long wait times, a lack of access to certain treatments and, in many instances, substandard medical care. For example:

•The five-year survival rate for early diagnosed breast cancer patients in England is just 78 percent, compared to 98 percent in the U.S.

•A typical Canadian seeking surgical or other therapeutic treatment had to wait 18.3 weeks in 2007, an all-time high, according to The Fraser Institute.

•The average wait time for bypass surgery in New York is 17 days compared to 72 days in the Netherlands and 59 days in Sweden.

•More than half of the Canadian adults (56 percent) sought routine or ongoing care in 2005—of these, one in six said they have trouble getting routine care.

•Eighty-five percent of doctors in Canada agree private insurance for health services already covered under Medicare would result in shorter wait times.

•Approximately 875,000 Canadians are on waiting lists for medical treatment.

"Congress has an important role to play in health care reform" said United States Representative John Shadegg, who has introduced health care legislation in support of free-market competition. "We can help patient in this country, not by setting up a massive new government bureaucracy, but by empowering individuals to make the best choices for themselves and their families."

If we are going to look to other healthcare models for solutions, we must uncover and study their

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problems. Healthcare is too important to allow reform by sound bite. "Drugs from Canada" is as much a false promise as "free" healthcare.

Last autumn, the Center for Medicine in the Public Interest interviewed people on the streets of New York City and asked them if they'd prefer "government" healthcare or "universal" healthcare. They overwhelmingly chose "universal" healthcare. But when we asked them to explain the difference between the two, they generally just shrugged their shoulders. *See* http://www.youtube.com/watch?v=CnvQt587TPY.

And when we asked them how much more in taxes they would be willing to pay to support universal healthcare, they shook their heads and said, "No, we want it to be free, like in Europe and Canada." Such are the fallacies that political rhetoric hath wrought.

Equally as prevalent is the notion of "free" or "low cost" drugs "like in Canada and Europe." And here too we need to be honest and examine the other side of the coin – that of cost-savings for the payer (often in the guise of healthcare technology assessment programs such as Britain's National Institute for Health and Clinical Excellence) versus care denied for the patient. *What is overlooked is that price controls equals choice controls*.

Our national conversation about healthcare has to go beyond vague concepts of reform and convenient political rhetoric. We must all be part of the solution and suspicious about false choices.

***New Model Needed.*** We need to develop proposals that modernize the information used in the evaluation of the value of treatments. Just as the key scientific insights guiding the FDA Critical Path program are genetic variations and biomedical informatics that predict and inform individual responses to treatment, we must establish a science-based process that incorporates the knowledge and tools of personalized medicine in reimbursement decisions: true evidence-based, patient-centric medicine.

For instance, the FDA, in cooperation with many interested parties, has developed a Critical Path opportunities list that provides 76 concrete examples of how new scientific discoveries in fields such as genomics and proteomics, imaging, and bioinformatics could be applied during medical product development to improve the accuracy of the tests used to predict the safety and efficacy of investigational medical products.

We need a Critical Path for Comparative Effectiveness to begin the process of developing a similar list of ways new discoveries and tools (such as electronic patient records) can be used to improve the predictive and prospective nature of comparative effectiveness.

It's a complicated proposition—but such a goal is as simple as it is essential—cost must never be allowed to trump care, and short-term savings must not be allowed to trump long-term outcomes. Just as we need new and better tools for drug development, so too do we need them for comparative effectiveness measurements.

A health technology assessment model for the 21st Century should reflect and measure individual response to treatment based on the combination of genetic, clinical, and demographic factors that indicate what keep people healthy, improve their health, and prevent disease. A rapidly aging society demands a new healthcare paradigm capable of providing for its needs in the 21st Century. Equality of care must be matched with quality of care.

In an era of personalized medicine, one-size-fits-all treatments and reimbursement strategies are dangerously outdated. We are early in this debate, but at least we can all agree that this is not, and must not be exclusively, a debate about saving money. It must be about patient care.

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