Health outcomes research: its influence on clinical decision making and the development of new imaging technologies.

V A Bucci

AJNR Am J Neuroradiol 1991, 12 (3) 397-399
http://www.ajnr.org/content/12/3/397.citation

Special Article

Health Outcomes Research: Its Influence on Clinical Decision Making and the Development of New Imaging Technologies

Vincent A. Bucci[1]

The decade of the 1980s brought forth a significant number of profound changes in the manner in which health care goods and services are provided to the U.S. population. In 1983, Congress established the Prospective Payment System (PPS), which radically altered the manner in which hospitals were reimbursed for treating Medicare patients. By the end of the decade, Congress established a new fee schedule, to be implemented in 1992, under which all physicians' services for Medicare patients were to be paid. This was known as the Relative Value Scale (RVS). In between, the federal government and, in particular, the Health Care Financing Administration (HCFA) implemented additional policy changes that, while not as dramatic as either the PPS or the RVS, significantly affected the delivery of health care to the American people.

The decade of the 1990s is beginning with a new policy initiative, known as "outcomes research," which may prove to be every inch as important, if not more so, than the earlier initiatives of the 80s. Also referred to as "patient outcomes research," "appropriateness studies," or "effectiveness initiative," the general thrust of outcomes research is to provide both the physician and the patient with improved clinical information about the expected or potential outcomes of various medical or surgical interventions in the treatment of illness.

Some have referred to health outcomes research as the third great health care "era" of the post–World War II period, the first two being the "Era of Expansion" (1965–1983) and the "Era of Cost Containment" (1983–1990). Others have commented that outcomes research is an effort to provide the body known as our health care system with a "brain" and a "central nervous system." However one wishes to conceptualize this outcomes phenomenon, one thing is eminently clear: the process of medical decision making is about to change.

It is difficult to understand the genesis of health outcomes without understanding the current process by which most new technologies and, to some extent, medical and surgical procedures enter the marketplace for use by the medical community. For new devices and drugs, a manufacturer must establish to the Food and Drug Administration (FDA) that the new product is both safe and effective (effective being defined as providing "clinically significant results"). As part of its mandate, the FDA does not possess the authority to require proof of efficacy relative to other products as part of its approval criteria, that prerogative being denied to the FDA by Congress on numerous occasions. The approval process is both time-consuming and costly. While $200 million has often been cited as the cost of bringing a new drug to market, few people realize that the cost may be just as high for new medical devices. What is not open to speculation is the time lag for bringing a new device to the marketplace: data show

Received January 16, 1991; accepted January 16, 1991.

[1] Vice President, Clinical and Regulatory Affairs, Infusaid, Inc., Pfizer Hospital Products Group, 1400 Providence Highway, Norwood, MA 02062. Address reprint requests to V. A. Bucci.

AJNR **12:397–399, May/June 1991** 0195–6108/91/1203–0397

that the clinical trial–FDA approval process time lag for a new device is in excess of 47 months, and this does not include preclinical time frames. For diagnostic technologies, such as MR imaging, the clinical trial–FDA approval process time alone exceeded 26 months for the first several devices approved. For complex devices, it is not unusual for the engineering and preclinical period to exceed 5 years.

While the approval of a new device or drug by the FDA permits that product to be sold in the marketplace, it does not ensure that it will be considered a "covered" service or item by third-party payers. It is the process of technology assessment, the determination of whether we should or should not pay for a new technology or service, that follows the FDA approval process. As performed by the HCFA for Medicare, this process consumes about 2½ years. Its purpose is to consider the safety, effectiveness, appropriateness, and, rarely, the cost-effectiveness of the new product or procedure. If the decision is favorable, Medicare will pay for the new product or procedure.

The final leg of the stream of regulation is that of payment, the decision as to how much third-party payers will actually pay for a new product or procedure. This process also is lengthy, often requiring more than a year. Collectively, the FDA-assessment-payment process consumes in excess of 8 years, excluding all preclinical work. Given the rapidity with which technological change can occur, time lags of this magnitude can threaten profitability, return on investment, and ultimately the innovative process itself.

Despite the lengthy time lag, critics of the existing developmental process have claimed, and correctly so, that the process does not provide either the type or quality of clinical data necessary in today's health care environment. Despite the technological brilliance of new diagnostic imaging equipment, such as MR and CT scanners, or sonographic technology, the data produced by such technological wizardry provides, as David Eddy points out, only "intermediate" data points and not end point or final health outcomes data. The image itself does not make the patient feel better. What the image does provide is information the physician can use to select one or another therapeutic options. What is missing in the equation is prescient information as to what option is best for achieving an optimal end point based on the intermediate data point of the image.

For nonimaging technologies, the failure of the current review process also is evident. In the approval of percutaneous transluminary coronary angioplasty (PTCA), the process ensured that a balloon catheter could be inserted safely into the patient, properly positioned and inflated, and then deflated, leaving a previously stenosed vessel patent. What the process failed to adequately tell us was the ultimate or end-point status of the patient. Did the vessel remain patent? How long did it remain patent? Was coronary by-pass graft (CABG) surgery ultimately required? Did the procedure improve the functional status of the patient? The remarkable thing about PTCA was that, while it was intended to reduce the incidence of CABG, the facts show that CABG surgeries increased steadily by the same percentage the 2 years before as well as the 2 years after PTCA approval. In short, what did PTCA provide the health care system?

Health Outcomes—The Hope

A definition of the term health outcomes is difficult to come by, primarily because every organization appears to be doing something called outcomes research. While difficult to define, the focus of the research is intended to determine with extraordinary specificity what the ultimate end point is of a particular intervention in the treatment of a disease. Depending on the disease being treated, what constitutes the ultimate end point may vary significantly.

What can be said about health outcomes research, in whatever form it takes, is that it embodies both a substantive and methodological change in our thinking and that, if performed properly, it will provide all participants in the health care sector some measure of benefit.

Substantive Changes

Health outcomes research seeks to provide the health care system with critical information about interventional medicine. Obviously, as with traditional analysis, safety and efficacy are the cornerstones of any such evaluation. But with health outcomes research, there is more.

Cost, Cost-Effectiveness, Cost-Benefits

While cost and cost-effectiveness were important catchwords in the health care system of the 1980s, there still remain few studies that satisfactorily address the costs of competitive therapeutic options. Depending on the particular outcomes project, researchers have used cost, cost-effectiveness, and cost-benefit analysis methodologies.

Quality of Life

Substantively, this is perhaps the most important new area of interest. While quality-of-life analyses have long been viewed as important, outcomes research elevates quality-of-life analyses to the status of a primary issue.

Final Outcome

The crux of health outcomes is to determine the end point or final outcome of a particular intervention. For diagnostic imaging equipment this is difficult, since such tests only provide us with intermediate data. In nondiagnostic procedures, the issues may be similar, such as the intermediate evaluation of PTCA. Final outcomes brings us to look at the patient in terms of safety, efficacy, quality of life (functional status), and cost over a far longer period of time than before.

Methodological Changes

Health outcomes research also has embraced a number of dramatic methodological changes in both how one thinks about certain clinical criteria and how information is developed. What are the changes in methodological thinking?

Meta-analysis

All health outcomes research grants utilize, in their effort to frame clinical issues, large data bases of medical literature. Meta-analysis permits the pooling of varied data sources into a single data base for statistical analysis. While critics of meta-analysis have long argued against the statistical soundness of this approach, it is clear in the outcomes proposals thus far funded that meta-analysis is fundamental to the research.

Cost-Effectiveness

As mentioned earlier, cost, cost-effectiveness, and cost-benefit have been important criteria in health care decision making in the 1980s. However, the full use of such criteria has been impeded by criticism of their methodological soundness. As in the case of meta-analysis, all major outcomes research now embraces the cost issue and has done so without resolving the methodological infirmities.

Quality of Life

While each outcomes grant identifies the quality-of-life aspect of a particular intervention as a goal of the research, it is believed by many that a number of the specific tools of quality-of-life measurement are, methodologically, still in their infancy.

Claims Data Bases

Outcomes research has utilized Medicare and other third-party payer/provider claims data bases as a means of identifying outcomes of particular interventions. This has occurred despite enormous concerns about the quality or accuracy of the data bases themselves or the coding mechanisms upon which they are built.

The Danger of Health Outcomes Research to New Technology

The phenomenon of health outcomes research does indeed promise the health care system a great deal: improved clinical data for the physician regarding the outcomes of various interventions, information for third-party payers on cost and cost-effectiveness, and important quality-of-life information for the patient. It would appear that in health outcomes research, there is something for everybody.

If there is a danger inherent in the health outcomes initiative, it is that, if implemented improperly, it could seriously threaten what has already become a fragile environment for technological innovation. For decades, the innovative segment of the device industry has been characterized by small, venture-financed start-up companies. However, with the cost and long time frames required to develop new technology, data will show that the device industry is increasingly being characterized as oligopolistic in nature. In purely economic terms, oligopolization is a classic economic response to a market possessing excessive barriers to marketplace entry. Health outcomes research with its demands for more data and more complex data, will surely create more barriers to marketplace entry than ever before. If industry is to finance the work of answering outcomes-type questions for new technology, then it is certain that only larger companies will be able to afford the time and costs of such development.

A second danger of health outcomes lies in how the policymakers and third-party payers will use the information generated in outcomes research. Will Medicare and other third-party payers use the results in a purely educational manner in order to reduce unexplained geographic variation in physician practice, or will they use the data to establish practice guidelines inextricably linked to reimbursement policy? Currently, payment policy and the effect on innovation are the two primary concerns of the outcomes research initiative.

For those involved in either the development or use of imaging technologies, outcomes research will present particular difficulties, since the research will have to link the intermediate data developed by imaging technologies to the eventual outcomes of therapeutic interventions. This additional step will create immeasurable complexity to outcomes research in the imaging area. Investigators will have to be particularly mindful of the need to separate the benefits/shortcomings of the imaging technology itself from the risk/benefit of the particular therapeutic intervention that follows.

Conclusion

There is emerging a significant change in the manner in which clinical information is being developed in this country, both in the substance of what we are seeking to produce and the manner in which we produce that information. While it remains to be seen precisely how the results of outcomes research will be used by policymakers, third-party payers, or physicians, it is likely that the point of clinical decision making is about to be gently (?) nudged toward a greater centralization. It is certain that the wide geographic variance in physician practice will diminish rapidly. Finally, it is essential for the health care system as a whole to be mindful of the impact that outcomes research may have on new and developing technologies and, in particular, the innovative environment for new technologies. The cost and time lags for developing this information may be so burdensome that the technological advances achieved in the device industry in the 1980s may soon be a distant memory.

The reader's attention is directed to the commentary on this article, which appears on the following pages.