Methods And Measurement In Patient Outcomes Research: Universal Issues

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ABSTRACT

The increased availability of funding for effectiveness research provides a unique opportunity to evaluate existing interventions as well as innovative approaches to patient care. Studies of clinical problems that occur frequently, that are costly to society, and for which there is no consensus regarding the most effective treatment are particularly attractive to funding agencies. For many conditions, there is little agreement about the best course of action and as a result, there is considerable variation among providers in how they manage the condition. Determining which of several alternative approaches is most effective is fundamental to efforts to improve the quality of health care.

A wide variety of research designs and methods can be used in outcomes research and the choice of a specific method will depend on the state of knowledge about a particular problem and the resources available to the investigator. Initially, literature syntheses and analyses of existing databases may be most appropriate. However, the literature is often unable to provide clear answers and existing databases often lack clinical detail and information on outcomes. However, data useful for outcomes studies might be obtained from patients' medical records, through questionnaires, or from cohort studies. While these methods can provide valuable insights, the most convincing scientific evidence for or against a specific intervention will require experimental designs such as randomized controlled trials.

Nurses involved in outcomes research will be faced with several challenges. First, data relevant for studies of the outcomes of nursing interventions will rarely be obtainable from existing databases which are typically designed to capture procedure and cost information. Second, education outcomes researchers outside of nursing about the potential contribution of nurse interventions will be necessary to build multidisciplinary research teams that have the greatest potential to produce useful findings. Finally, the introduction of innovative nurse interventions, even if shown effective, may be difficult in a cost-conscious and conservative health care system. Nevertheless, the health care system is undergoing fundamental change and the nursing perspective is likely to become increasingly appreciated as outcome measures that have depended only on physician observation and measurement are supplanted by patient-centered measures such as functional status, quality of life, and satisfaction with care.

I recently overheard my 5-year old daughter singing a children's song that went like this:

> Miss Suzy had a baby, his name was Tiny Tim
> She put him in the bathtub to see if he could swim
> He drank up all the water, he ate up all the soap

He tried to eat the bathtub, but it wouldn't go down his throat.

Miss Suzy called the doctor
Miss Suzy called the nurse
Miss Suzy called the lady with the alligator purse

"Mumps" said the doctor
"Measles" said the nurse
"Nothing said the lady with the alligator purse

Miss Suzy punched the doctor
Miss Suzy kicked the nurse
Miss Suzy *paid* the lady with the alligator purse.

It is rare to hear a children's song that relates so closely to one's professional interests. After singing it over and over to myself it hit me: Of course! This song was an allegory for the current crisis in American health care! Tiny Tim obviously represents the insatiable consumer of health services whose easy access to medical care had to gluttonous consumption and soaring costs. Miss Suzy clearly represents the party who is footing the bill for health care, probably Tiny's employer or the federal government. The doctor, nurse and lady with the alligator purse undoubtedly exemplify our pluralistic health care system wherein people with a particular problem can receive care from a wide variety of experts. The lack of agreement among the three experts consulted by Miss Suzy illustrates that the diagnosis and treatment on receives may depend more on which expert is consulted than on the nature of the underlying problem. Miss Suzy, as the payer, rejects the diagnoses of the two traditional health care providers and then rewards the lady with the alligator purse. (In reality, payers <u>are</u> becoming increasingly concerned about the costs, appropriateness and effectiveness of care and <u>are</u> starting to refuse to pay for care that has been shown to be ineffective or inappropriate.) But <u>who</u> exactly is this enigmatic lady with the mysterious alligator purse? Maybe she represents the ideal low cost health professional of the future who payers would love to see supplant the present day high cost providers. With diagnoses like "nothing wrong", she is probably cheap—but is she effective? Is she cost-effective?

I believe that this children's rhyme, or at least my interpretation of it, illustrates why AHCPR's medical effectiveness program is so important. Payers are reaching their limits in terms of the amount of health services they are willing and able to pay for and they need information regarding which treatments are effective and which are not. This is in large part why Congress has funded outcomes research.

In the next few minutes I would like to discuss some of the broader methodological issues that confront those of us interested in outcomes research. I will first comment on the types of clinical problems that funding agencies consider priority areas for investigation and will give particular emphasis to the issue of practice variation. I will then describe the range of research designs that my be employed to address these types of problems. Finally, I will list several challenges that those pursuing research on outcomes of nursing practice may encounter. Since I have been working with the Low Back Pain Patient Outcome Research Team for the past 2 years I will illustrate my points with examples involving low back pain.

<u>Clinical Problems Most Worth Studying</u>

What clinical problems are most worth studying? Clearly problems that occur frequently , that are most costly to society, and for which there is no consensus about diagnosis and treatment are in greatest need of outcomes research. Let me illustrate with low back pain which, in the United States, is exceedingly common, costly, and controversial. It is common in that it will

significantly affect almost 80 percent of us before we retire. It is the second most common symptom presented to physicians in general and is the leading reason for visits to orthopedic and neurosurgeons. In the hospital, DRG 243 "Medical Back Problems" was the second most prevalent DRG exceeded only by normal deliveries in 1987. While there are also numerous visits to physical and occupational therapists for back problems and possibly to nurses, the number or content of such visits is not captured in any national database.

Back pain is also costly. A 1984 study estimated the annual cost of back pain in the United States to be almost 16 billion dollars, most of this in direct health care costs. Among men, earnings and productivity losses due to back problems exceeded those for respiratory conditions and ischemic heart disease, totalling $5 billion in 1984.

Finally, back pain is controversial. There is tremendous uncertainty about almost all aspects of back pain, including its etiology, pathophysiology, assessment, and treatment. For only 10-15 percent of people seeing a physician for their back pain can a specific cause of the pain be determined. There is disagreement about whether back pain results from disorders of the muscles, joints, or discs. Diagnostic x-rays are of little clinical value for most patients but are still commonly used by some physicians. Probably the most controversial area, however, has been therapeutic procedures. A whole array of therapies have been employed for back pain over the centuries but there is little solid evidence that any of them is significantly better for most patients than reassurance and allowing nature to take its course. And finally, it is worth noting that the profession branded as an "unscientific cult" by the American Medical Association, chiropractic, is largely devoted to the management of back pain.

In view of this lack of consensus about the diagnosis and treatment of back pain, it should not be surprising that there is enormous variation in how back pain is managed. The rate of back surgery in the United States is double that in most Western countries and five times that in England. Overall back surgery rates are twice as high in the western United States than in the northeast and the rate of spinal fusions is ten times as high. Hospitalizations for medical back problems is four times higher in Boston than in the demographically similar community of New Haven. Patients receiving care from health maintenance organizations are much less likely to be hospitalized for back problems than patients receiving care in the fee-for-service sector. Although data are limited, it is clear that there is also tremendous variation in the outpatient management of low back pain.

The underlying hypothesis that underpins much of the effectiveness research, is that these practice variations are more a function of provider practice style than of patient need. This raises a whole host of questions: Is the amount of observed practice variation in fact more than one would expect by chance? Is it the higher rate or the lower rate the represents the better care? Is the procedure more effective (and more cost-effective) than available alternatives? What outcomes are most important to measure? And finally, once answered to these questions have been found, how can this information be translated into improved clinical practice? It is the lack of the research necessary to adequately answer these questions that makes outcomes research so important.

Research Design Issues

There is no single set of research designs or methods that are uniquely appropriate for outcome studies. The design and methods used will depend on the state of knowledge about a particular problem and the resources available to the investigator and can range from analysis

of existing information to observational studies to experimental trials. As with any type of research, a logical initial step is to determine what might be learned from a synthesis of the scientific literature. Literature syntheses should go beyond merely summarizing the results of previous studies by providing an objective critical evaluation of the quality of the research methods employed in each study. This approach makes it possible to assign greater weight to studies that were more scientifically rigorous. In some cases, it is even possible to combine data from several similar small studies and reanalyze the data as if they came from a single large study. Unfortunately, the literature often fails to provide clear answers. For example, while we found a large number of studies documenting the effectiveness of surgery for spinal stenosis, all of them suffered from serious methodological deficiencies.

The analysis of data from existing databases can also provide a useful starting point. In fact, much of the earliest outcomes research has relied on analyses of administrative databases such as the Medicare data. Jack Wennberg's use of available hospital discharge data documented dramatic geographic variation in the rates of common surgical procedures and is largely responsible for stimulating the current interest in and funding of outcomes research. Analyses of existing data can also provide useful estimates of the costs and complications associated with specific procedures. In our back pain project, we have made extensive use of the Medicare data as well as data from the National Hospital Discharge Survey and hospital discharge data for the state of Washington.

While existing databases can be very useful, they generally lack clinical detail and key outcome data such as functional status, disability, pain, and patient satisfaction. Hence, at some point it will be necessary to design studies that involve the collection of new data. This might be done through such mechanisms as chart reviews, questionnaires, or even cohort studies. For example, we were intrigued by the fact that according to hospital discharge data, over half a million Americans were hospitalized with diagnoses such as herniated discs, lumbago, and back strain and did not have surgery. We wondered why these patients were being hospitalized. The hospital discharge data indicated that about half of these patients had had an imaging study, typically a myelogram, but provided no information about the reasons for the admission and almost no information on the treatments these patients received. In order to obtain this type of information, we abstracted data from the inpatient medical records of a sample of back pain patients discharged from Washington state hospitals.

We have been involved in other activities designed to gain an initial understanding of the reasons for non-surgical hospitalizations for back pain and the potential to shift some of the hospitalizations to the outpatient setting. These include a national survey of physicians concerning their policies for hospitalizing patients with non-surgical back problems and interviews with patients recently hospitalized for non-surgical back pain to determine their perceptions of the rationale for and the benefits of having been hospitalized.

We are also conducting a prospective cohort study of the outcomes of surgical and non-surgical care for patients with herniated discs and spinal stenosis. While this study will provide valuable information about the outcomes of care associated with these two treatments, even prospective cohort studies are unable to conclusively determine the relative effectiveness of different interventions since patients and physicians selecting one particular course of treatment may differ in prognostically important ways from those receiving alternative treatments. Hence, the ultimate test of effectiveness is a well-designed

and well executed randomized and controlled trial. Randomized trials are not common in the back pain literature and most suffer from serious defects. However, given the lack of clearly superior back pain treatments for most patients and the large number of back pain patients available for study, randomized trials should be quite feasible. In fact, we are about to pilot test two primary care interventions for low back pain: one comparing spinal manipulation therapy with physical therapy and the other evaluating the benefits of an educational intervention with a specially trained nurse. In the latter study, we will determine if a 15 minute session with a nurse immediately following a standard physician visit plus 3 follow-up phone calls to the patient will improve patient outcomes. This 15 minute nurse intervention will be designed to elicit and address questions of greatest concern to the patients and to convey a positive and caring attitude. We will compare outcomes such as patient satisfaction, functional status, and disability for this group with those for patients receiving standard care alone.

Hence, there is a broad range of designs and methods that can be usefully employed in evaluating the effectiveness and cost-effectiveness of health care interventions.

Challenges for Nursing Outcomes Research

I would like to conclude with some observations about challenges that may face nurses as they become more involved with outcomes research. Some of these challenges apply to outcomes researchers in general while others apply more directly to nurse researchers. I offer these observations from the perspective of someone who primarily functions in the largely medical environment and will therefore risk appearing naive concerning the world of nursing.

Challenge 1: Obtaining Data Relevant to Study the Effectiveness of Nursing Practice

Nurse researchers will be less able to find useful existing data than those of us who have studied outcomes for surgical procedures. In fact, there may be few if any existing databases that collect the type of information that is needed for studies of the effectiveness of nursing practice. Even medical records and nursing notes may lack the kind of information that will be required for these types of studies. As a result, most nursing effectiveness studies will require the collection of data that has not normally been collected pertaining to both the process and outcomes of care. Furthermore, it is my belief that much of the potential for nursing practice to improve patient outcomes will involve that evaluation of new models of care. To the extent that this is true, researchers will not be able to rely on observational studies of existing procedures and will therefore need to develop and evaluate new interventions. This will require familiarity with experimental and quasi-experimental research designs.

Challenge 2: Becoming Involved in Multidisciplinary Research Teams

Much of the current outcomes research has centered around crude outcomes associated with surgical procedures and therefore has not included as much nurse involvement as might be desirable in other types of outcome studies. As outcomes research matures, I believe there will be an increasing emphasis on such areas as patient satisfaction, patient understanding of their problem, patient activation, and patient function—all areas in which nurses already make significant contributions. However, while nurses could pursue outcomes research in isolation from other disciplines, becoming integrated with a team of outcomes researchers from a broad range of disciplines may ultimately prove more productive and rewarding. One of the most promising and exciting aspects of our back pain project has been our ability to bring

experts in research methodology together with clinicians who understand the clinical issues and are open to evaluating whether what they are doing in fact benefits patients. We have benefitted from having a doctoral candidate in nursing working on our project, and as I mentioned earlier, are planning to evaluate the impact of a nurse delivered intervention on outcomes. Some of the individuals currently conducting outcomes research may not be aware of the contributions nurses may be able to make. Hence, an alternative to independently embarking on an outcomes research project is to become familiar with existing outcomes research activities and to then suggest ways in which that research may benefit from the inclusion of a nursing dimension.

Challenge 3: Introducing Innovations in a Cost-Constrained and Conservative System

It has been my impression that the potential for nurses to use their knowledge and skills to benefit patients has been severely limited by the way the health care system is currently structured and the way in which services are reimbursed. I believe that increased use of the skills nurses have in the areas of patient education, motivation, and behavior change have great potential for improving patient outcomes. However, in this era of cost-containment, realizing this potential for improving patient outcomes. However, in this era of cost-containment, realizing this potential will require not only proving through research that specific interventions are effective, but also that they are cost-effective. Even this may not be enough since it often seems that it is easier to continue performing expensive procedures which have never been proven effective than to obtain reimbursement for relatively inexpensive interventions, even if there is good evidence they are effective. Changing standard medical practice, and I have chosen my words deliberately, is a difficult and slow process.

While I would counsel patience, I believe there is reason for optimism. The health care system is undergoing major changes and the limitations of the biomedical model are becoming increasingly apparent. Outcome measures that depend only on physician observation and measurement are being supplanted by patient-centered measures such as self-assessed function and health status, quality of life, and satisfaction with care. Articles documenting the potential for the quality of the patient-provider interaction to affect outcomes of care are beginning to appear with increasing frequency. It is my impression, that because these changes are very much in concert with the values of the nursing profession, the outcomes research to come out of nursing will become increasingly relevant and will have the potential for making a major contribution to improvements in the effectiveness of health care in this country.