World Journal of Gastrointestinal Endoscopy

Online Submissions: http://www.wjgnet.com/1948-5190office
wjge@wjgnet.com
doi:10.4253/wjge.v4.i6.236

World J Gastrointest Endosc 2012 June 16; 4(6): 236-240
ISSN 1948-5190 (online)

GUIDELINES FOR BASIC SCIENCE

Outcomes research in gastroenterology and endoscopy

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Received: October 7, 2011 Revised: November 16, 2011
Accepted: May 27, 2012
Published online: June 16, 2012

Abstract

Although the field of outcomes research has received increased attention in recent years, there is still considerable uncertainty and confusion about what is "outcomes research". The following editorial is designed to provide an overview on this topic, illustrate specific examples of outcomes research in clinical gastroenterology and endoscopy, and discuss its importance as a whole. In this article, we review the definition and specific goals of outcomes research. We outline the difference between traditional clinical research and outcomes research and discuss the benefits and limitations of outcomes research. We summarize the types of outcomes studies and methods utilized for outcomes assessment, and give specific examples of the impact of outcomes studies in the field of gastroenterology and endoscopy.

Key words: Clinical research; Outcomes; Outcomes research

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Gupta P, Buscagli JM. Outcomes research in gastroenterology and endoscopy. *World J Gastrointest Endosc* 2012; 4(6): 236-240 Available from: URL: http://www.wjgnet.com/1948-5190/full/v4/i6/236.htm DOI: http://dx.doi.org/10.4253/wjge.v4.i6.236

INTRODUCTION

The contemporary outcomes research movement in the United States began about three decades ago when an increasing emphasis on cost reduction led to interest in determining and obviating unnecessary procedures. The movement was induced by the discovery of substantial variation in medical practice based on geography and race, with no observable differences in health outcomes[1]. This movement was further propagated by the evidence of inconsistent use of diagnostics, rising healthcare costs and concerns about adverse effects on quality of care from changes in healthcare reimbursement models[2]. These discoveries lead us to realize that there were deficits in our understanding of the safety, indications, and efficacy of medications and diagnostics, as well as therapeutic procedures. It can be assumed that some interventions produce better outcomes than others given these variations in practice and differences in results.

Outcomes research has been defined as "the scientific study of the result of diverse therapies used for particular diseases, conditions, or illnesses". The specific goals of this type of research are to create treatment guidelines, document treatment effectiveness and to study the effect of reimbursement policies on outcomes[3]. In addition to measuring clinical and physiological endpoints, outcomes studies may assess the effects of an intervention on health-related quality of life, functional status, patient satisfaction, and cost[4].

Although overlap clearly exists, outcomes research is different from traditional clinical research in its focus and methods. Outcomes research tends to be observational rather than experimental, and it is patient-centered as compared to clinical research which is more disease-centered. Outcome measures concentrate more on processes

Table 1 Differences in focus between outcomes research and traditional clinical research

	Outcomes research	Traditional clinical research
Focus	Observational	Experimental
Example	Retrospective analysis assessing the factors associated with mortality in patients with severe acute pancreatitis	Randomized placebo-controlled trial of drug X administered to patients presenting with severe acute pancreatitis
Focus	Patient-centered	Disease-centered
Example	Long-term outcomes in patients with dysplastic Barrett's esophagus treated with radiofrequency ablation	Detection of subsquamous intestinal metaplasia ("buried Barretts") on repeat surveillance esophageal biopsies
Focus	Socioeconomic factors	Physiological factors
Example	Survey study assessing the impact on quality of life in teenaged patients diagnosed with ulcerative colitis (UC)	Retrospective analysis on postoperative complications in patients with UC undergoing total proctocolectomy

and delivery of care rather than on drugs and instruments. It aims to study the impact of diseases on patients rather than the mechanisms of disease, and it measures the effects of socioeconomic factors, not the effect of biochemical and physiological factors (Table 1).

Outcomes studies can help physicians in advising patients about what works, what doesn't, when in the course of illness does it begin working, and what it costs to actually work in the real world of clinical practice. These data can help physicians, payers and patients make rational, insightful choices on medical care-related issues[5]. The outcomes research movement is gaining momentum with the recognition of its importance by physicians, specialty medical societies and managed care organizations. This movement towards assessment and accountability has been termed the "third revolution in medical care"[6].

Outcomes research, however, has its own limitations[7]. Applying outcomes research data is difficult when making complex and individualized patient care decisions. In addition, very few of the commonly used and continuously evolving procedures and devices used in medicine are supported by evidence from randomized controlled trials, given that these trials often cost millions of dollars and frequently last years in duration. Finally, compliance with practice guidelines (put forth as a result of outcomes research) is extremely difficult to assess throughout the medical community as a whole.

Outcome measurements in outcomes research may be evaluated based on the categories of clinical measures, economic measures or humanistic indices. Clinical measures include data for clinical events (e.g., need for repeat hospitalization following an upper GI bleed), physiological measures (e.g., assessing acid reflux by esophageal pH measurement studies) or mortality. Economic measures include direct and indirect medical costs (e.g., outpatient visits, work loss, *etc.*), and analyses of resource use. Humanistic indices evaluate symptoms, functional status (e.g., health-related quality of life) and patient satisfaction. Appropriateness of medical interventions, conformance to standards of care or shifts in practice patterns may also be evaluated. In short, outcomes research uses a variety of methods and the following paragraphs provide a general summary of the extent of research embraced by this field of interest.

OUTCOMES ASSESSMENT USING LARGE ADMINISTRATIVE DATABASES

Data collected for billing and coding or management purposes might contain valuable objective data such as cost, length of hospital stay, outpatient visits, resource use or mortality. These data can be analyzed promptly and cheaply without requiring patient consent or interfering with the doctor-patient relationship. Medicare, Medicaid and large private databases have been extensively used to investigate a variety of outcomes such as the risk of re-hospitalization for patients using clopidogrel with a proton pump inhibitor[8], or the disparities in demographics among hospitalized patients with pancreatitis-related mortality[9].

The surveillance, epidemiology, and end results (SEER) program of the National Cancer Institute provides considerable information on cancer statistics not available for other digestive conditions. For example, in 2004 approximately 233 000 people were diagnosed with digestive system cancers, representing 18% of all malignancies[10]. A recent analysis of the SEER database revealed that patients with early esophageal cancer managed with endoscopic therapy have equivalent long-term survival compared to those treated with surgical resection[11]. These types of data are generally limited by quality and completeness of the available information. Detailed clinical information is lacking as it is collected for administrative purposes. Nonetheless, when exercised cautiously by seasoned researchers, analysis of such data can provide important evidence-based information to supplement randomized controlled trials, or provide the framework for other clinical studies.

DECISION ANALYSIS

Decision analysis is the methodology of using mathematical computation for the evaluation of clinical decisions. It is used to ascertain best strategies when there are several different courses of action, and an indefinite or hazardous pattern of outcomes. A decision-tree is created after identifying all accessible choices and likely outcomes. The tree is used to symbolize the available strategies and the likelihood of occurrence of each outcome if a particular strategy is selected. Decision analysis is used to identify the crucial factors in the decision-making exercise and can be used to make healthcare policy recommendations and develop clinical management guidelines. For example, decision analysis played an important role in the development of the current American College of Gastroenterology guidelines[12] for the management of dyspepsia[13].

META-ANALYSIS

A meta-analysis combines the results of several clinical studies which address a set of related research hypotheses that meet pre-determined standards of quality. An expertly conducted meta-analysis can improve statistical power if the sample size of individual studies is small. Meta-analyses are becoming increasingly important in the determination of clinical efficacy and harm, to plan future studies and to make clinical recommendations for therapy. It is an important source of outcomes data for the practice of evidence-based medicine. For example, a meta-analysis of the role of endoscopic variceal ligation in the primary prophylaxis of esophageal variceal bleeding[14] was instrumental in formulating the American Association for Study of Liver Diseases guidelines for the prevention and management of gastroesophageal varices and variceal hemorrhage in cirrhosis[15].

COST-EFFECTIVENESS ANALYSES

Cost-effectiveness analysis is a form of economic analysis that compares the relative costs and outcomes of two or more courses of action to determine the most productive use of limited resources. The cost-effectiveness ratio evaluates alternative patient management strategies, programs or services. The most commonly used outcome measure is quality-adjusted life years. This type of analysis is a measure to critically evaluate clinical practices and weigh outcomes against their costs. These data can be used for the distribution of limited funds. Such studies have also been used to compare the cost-effectiveness of practices in gastroenterology with the cost-effectiveness of other medical practices. For example, colonoscopy has been compared with computed tomographic colonography in cost-effectiveness studies[16].

HEALTH SERVICES RESEARCH

The measurement of health status, patient preferences, and quality of care are a part of health services research[17]. Health services research examines how people gain access to health care[18], how much care costs, and what happens to patients as a result of this care. The main goals of health services research are to identify the most effective ways to organize, manage, finance, and deliver high quality care, as well as to reduce medical errors and improve patient safety[19].

The measurement of quality of life is also an important topic of research under health services research. General and specific quality of life measures have been developed for research purposes. The Crohn's disease activity index[20], the Harvey-Bradshaw index and the Inflammatory Bowel Disease Questionnaire are examples of such measures. Health services research also encompasses measurement of healthcare use. For example, does early endoscopy alter healthcare use patterns or satisfaction in patients with dyspepsia [21]?

CLINICAL GUIDELINE DEVELOPMENT

Due to wide-spread cost containment measures, clinical guidelines detailing healthcare recommendations have become abundant, however, these guidelines have been based on varying degree of scientific evidence. The Agency for Healthcare Research and Quality (AHRQ) has defined strict criteria for the development of guidelines. Guidelines should be based on robust scientific evidence rather than on expert opinion. It has not been shown conclusively that guidelines change physician behavior. Reasons for this finding may be because some guidelines may not be designed for community physicians, the practicing physicians may disagree with the expert opinion of the guideline author or they may elect not to follow the guidelines because of fear of litigation.

RANDOMIZED CLINICAL TRIALS

Randomized clinical trials (RCTs) are generally considered the gold standard in clinical research. For example, the National Polyp Study was the landmark randomized clinical trial to evaluate effective surveillance of patients discovered to have one or more colorectal adenomas[22]. Traditional RCTs encompass efficacy studies which generally have a strict code of conduct and require predefined hypotheses, randomization of carefully selected subjects to pre-specified intervention arms, largely similar populations, experienced investigators, a specific protocol, a comparable intervention and intense follow-up. Results from these types of studies are robust. However, because of the restrictive design, the results may not be valid in community practice.

On the other hand, outcomes research focuses on effectiveness studies which are designed to evaluate interventions in community settings with unselected patients, typical care providers and usually-performed procedures. Effectiveness studies are often observational and retrospective, without randomized allocation of patient population. Selection bias may be a problem in such studies and adjustment for severity of illness and case mix is an important aspect to retain validity.

CLINICAL EPIDEMIOLOGY

Clinical epidemiology employs rigorous epidemiological methods to study diagnoses, effective management, and natural progression or prognosis of diseases. Clinical epidemiologic studies such as observational studies help in the development of guidelines in the absence of randomized clinical trials[23,24].

IMPORTANCE OF OUTCOMES RESEARCH TO GASTROENTEROLOGY

Digestive diseases have a heavy medical, social, political and economic burden in the United States. In 2004, the

direct health care costs of digestive diseases were more than $97 billion, up from $40 billion in 1985. The total cost of digestive diseases, including direct and indirect, in the United States in 2004 was estimated to be $141.8 billion. More than 72 million ambulatory care visits with patients with a first-listed diagnosis of a digestive disease were reported in 2004. Digestive diseases were also common diagnoses at hospital discharge with approximately 4.6 million discharges of patients with a first-listed diagnosis of a digestive disease and 13.5 million discharges with a digestive disease as a primary or secondary diagnosis. In 2004, there were > 236 000 deaths in the United States with a digestive disease as an underlying cause, which represented 9.8% of all deaths[25].

It is estimated that > 20 million upper and lower endoscopies are performed yearly in the United States[26]. There is no single national database that can provide accurate, population-based information on the absolute number of gastrointestinal (GI) endoscopies and their indications and diagnostic outcomes. To bridge this important gap in knowledge on the burden of GI disease, a National Endoscopic Database (NED) has been started by the Clinical Outcomes Research Initiative (CORI).

CORI was developed to study outcomes of GI endoscopic procedures in "real life" settings with the primary goal to use the NED to acquire information that will improve the quality of clinical practice in gastroenterology. Physicians participating in the CORI consortium produce GI endoscopy reports using a specialty electronic health record. Data from the reports are sent electronically to a central data repository where they are pooled with data from other consortium participants in the NED. The CORI project began in 1995 under the auspices of the American Society for Gastrointestinal Endoscopy. In 2007, the NED received over 250 000 reports from 70 practice sites in 24 states with approximately 400 participating endoscopists. Practice sites include hospitals, ambulatory care centers, private practices, universities, and Veteran's Affairs hospitals. The NED now contains close to 2 million reports[27]. These data have been analyzed to examine endoscopic practice patterns, to develop research hypotheses, to support quality measure reporting, and as a resource for prospective research on topics such as colon polyp surveillance. Although the participating sites are over-represented by veteran and military facilities, the patterns of endoscopy in NED have been shown to be quite similar to that of a national sample of the Medicare population and may well be applicable to the United States as a whole[28].

CONCLUSION

No longer just the domain of a small group of researchers, outcomes research has altered the culture of clinical practice and health care research by changing how we assess the end results of healthcare services. In doing so, it has provided the foundation for measuring the quality of care. The results of AHRQ outcomes research are becoming part of the "report cards" that purchasers and consumers can use to assess the quality of care in health plans[29]. For public programs such as Medicaid and Medicare, outcomes research provides policymakers with the tools to monitor and improve quality both in traditional settings and under managed care. Outcomes research in this regard can be the key to knowing how we better achieve and deliver quality healthcare.

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