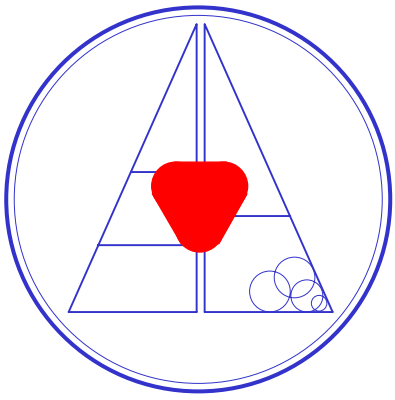
Handbook for

Health Care Research



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PREFACE

Learning to do research is like learning to ride a bicycle, reading a book is not much help. You need to learn by doing, with someone holding you up the first few times. Yet, the student of health sciences research must be familiar with basic concepts that can be studied by reading. The trick is to select the right topics and present them in a way that is both relevant and interesting.

This book is the result of over 20 years of experience doing research in the field of respiratory care. I have tried to select topics and statistical procedures that are common in medical research in general, and to allied health care in particular. It is by no means an exhaustive treatise on any particular aspect of medical research. Rather, it is more of a practical guide to supplement specialized statistics textbooks. Yet it can function as a stand-alone text for a short course in research in a 2 or 4 year respiratory care or other allied health program. In fact, this book grew out of the notes I have used for the last 6 years to teach research at Cuyahoga Community College.

At one level, the book is geared for the student or health care professional who wants to become involved with research. Basic concepts are presented along with real world examples. Naturally, because I am a respiratory therapist, the examples have to do with respiratory care. However, the concepts are applicable to any area of medical research. I have tried to keep the theory and mathematics at the most basic level. I assume that the reader will have basic computer skills and will have access to software that will handle the math. For that reason, unlike many books on the topic, there are no probability tables for calculating things like the critical values of the *t* statistic. Computers have made hand calculations all but obsolete. What the student really needs to know is which procedure to use, when, and why.

For the experienced researcher, the book is organized so that basic research procedures and definitions can be quickly looked up. This is important because when you are in the middle of a project you don't want to be interrupted to pour through pages and pages of theory when all you want is to be reminded of which test to use or how to format the data for computer entry.

Not every health care professional will be directly involved with research. However, everyone will be involved with the results of research. And most will be involved at some time with some sort of continuous quality improvement project, which will inevitably require some familiarity with research techniques. Therefore, this book, if nothing else, should serve as an excellent tool to help you become an "educated consumer" of research. After all, how can you appreciate the information in professional journals if you don't even know what a *p* value is? Researchers who publish in journals are trying to sell you their ideas. If you don't understand the procedures they use to generate the ideas and the language they use to sell them, you could end up buying a "lemon".

There are several features in this book that I think are unique. For example, the descriptions of statistical tests are standardized in a practical format. For each procedure, a hypothetical (or sometimes real-world) study problem is introduced, the hypothesis is stated, the data are given in the format that they are entered into the computer, then a detailed report from an actual statistical program is given.

Another unique feature is the chapter on writing the stand-alone abstract. The new researcher's first experience with publishing research will usually be in the form of an abstract, rather than a full text article. For this reason, I have placed particular emphasis on how to write an abstract that will pass peer review. There is a model abstract that has actually been published in Respiratory Care journal along with several abstracts that were submitted but rejected. I review each abstract in detail, just as I did when I reviewed them for the journal, and explain the mistakes made. These detailed examples are intended to

give the reader a sense of having a mentor looking over their shoulder giving help and encouragement. Just like riding a bike. In fact, the text throughout is worded in almost a conversational style. This really helps to illustrate the relevance of each new concept that might otherwise seem dull and intangible.

Also included in the Appendices is a model manuscript that was actually published in Respiratory Care. Not only that, but the comments of the peer reviewers is included along with the authors' responses. One of the biggest obstacles for new researchers is that they have a hard time accepting critical comments about a manuscript they have submitted for publication. Many, maybe even most, are so discouraged that they do not make the suggested revisions and their work goes to waste. My hope is that reading the reviewer's comments and the responses, you will get the idea that (1) every researcher, no matter how experienced, will be criticized and (2) the criticism only leads to a better product if you follow through. I always tell my students that the very first thing they have to learn is to "put your ego on the shelf".

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DEDICATION

Allied health professionals are rarely given formal training in research methodology. And even when they are, it is never more than a cursory overview. The real learning happens in apprenticeship. One must have a good mentor who can pass on the benefit of his knowledge and experience. I have been blessed with three of the best mentors a person could have.

The first is Marvin Lough, MBA, RRT, FAARC. Marv gave me my first job in the profession and helped me create a dedicated research position. He taught me that it is not what a person holds in memory that counts, but rather what he knows how to find. He has exemplified to me, in every way, what it means to be a professional, a leader, and a gentleman.

The second is Frank P. Primiano Jr., PhD. Frank has the most disciplined, logical and penetrating mind that I have ever encountered. He taught me the basic skills of a scientist. He taught me that brilliance lies in paying attention to the details and the supreme importance of defining and understanding the words you use. But most importantly, he taught me "If you explain something so that even a *fool* can understand it…then only a fool *will* understand it."

The third is Terry Volsko, BS, RRT, FAARC. She would say that *I* am *her* mentor, but the truth is that she has taught me as much as I have taught her. I have never met anyone with a greater hunger for knowledge or a stronger will to succeed. She has been a brilliant and tireless student, an insightful critic, and a compassionate friend. My other mentors showed me how to succeed; Terry showed me why.

SECTION I INTRODUCTION

Chapter 1. Why Study Research?

The chances that you, the reader, will become a famous researcher may be slim. For example, nearly 100,000 people are practicing respiratory therapy in the United States. Of those, only about 20,000 are members of the American Association for Respiratory Care. Out of all those people, less than 600 were involved with presenting research at the 47th International Respiratory Congress in 2001. Yet, every one of those 100,000 people needs to know how to read and understand scientific articles in medical journals. The same holds true for all health care workers. Even if you never conduct a study, you must be familiar with the basic concepts of research in order to practice as a professional whose understanding grows from continuing education.

The main purpose of this handbook is to help you become an educated consumer of medial research. If you want to actually perform research, the best thing you can do is find a mentor; someone who has experience conducting scientific studies and publishing the results. A mentor can help you turn the ideas in this handbook into practical realities.

THE IMPORTANCE OF RESEARCH IN HEALTH CARE

Health care professionals must acquire the knowledge and skills needed to assess the usefulness of new equipment, the effectiveness of present and proposed treatment modalities, the quality of services provided, and the adequacy of teaching materials available. *The most important of these skills is the ability to read and critically evaluate the published reports presented by other investigators.* Without this skill, no meaningful evaluation of current practices can be made and no research can be planned. The word *research* is typically used in a generic sense to mean a systematic method of inquiry.

The pursuit of scientific knowledge in any field must ultimately rest on the testing and retesting of new ideas and their practical application. Growing numbers of clinicians, educators, and administrators are conducting their own investigations and critically examining research done by others in their particular field of interest.

The experimental approach may be broken down into five phases (Table 1-1). Health care workers are usually involved with the application of research results in the clinical setting. Within the research continuum, however, an infinite number of opportunities exist to become involved in seeking the answers to questions relating to the practice of health care.

Table 1-1 The Five Phases of Research

1. *Basic Research.* Seeks new knowledge and furthers research in an area of knowledge rather than attempting to solve an immediate problem.
2. *Applied Research.* Seeks to identify relationships among facts to solve an immediate practical problem.
3. *Clinical lnvestigations.* Seek to evaluate systematically the application of research findings in the clinical setting, usually in a relatively small patient population.
4. *Clinical Trials.* Seek to determine the effectiveness and safety of clinical treatments in samples of patients drawn from larger populations.
5. *Demonstration and Education Research.* Seeks to examine the efficacy of treatments designed to promote health or prevent disease in defined populations.

The following discussion outlines several areas of health care where we may apply the principles of scientific analysis to provide a more sound basis for patient care. These include health care education, professional accountability, and administration of services.

Health Care Education

Colleges are responsible for graduating practitioners who are knowledgeable and current in the practice of their profession. Educators must stay up-to-date with new ideas and technology in medicine that affect the diagnosis and treatment of disease.

Critical Evaluation of Published Reports. Before a particular piece of equipment or treatment modality is accepted for introduction to the student, the instructor must first discern whether the claims for its use and potential benefits rest on a solid scientific foundation. Keeping abreast of new product developments requires that instructors read and critically evaluate reports and tests of function and reliability. A critical reading of scientific journals will provide the basis for their decisions concerning classroom demonstrations, guides, and the planning process. Educators may wish to conduct their own investigations as well.

The results of published reports should never be accepted uncritically. The use of intermittent mandatory ventilation (IMV), for example, was claimed to decrease the time required to wean a patient from mechanical ventilation. Yet recent studies have shown that the average length of time a patient spends on the ventilator and in the hospital actually *increased* by the use of IMV.

How much credence should we give to each of these studys' results? Is one or the other limited by its design? Does a non-uniformity of patient populations exist? Were the types of IMV systems used the same in each study? What criteria were used for judging a patient's readiness for removal from mechanical ventilation? Health care educators must ask these types of questions of all studies before passing the results on to their students; they must do more than simply take a study's conclusions at face value.

Continuing Education. In order that health care practitioners keep informed of recent developments in cardiopulmonary medicine, hospital department managers must establish and maintain continuing education programs. These inservice programs serve to explore and provide a forum for new trends, ideas, and developments that occur in the field as research is completed in varying areas of special interest. Allied health professionals are taking an increasing role in patient education as well as in clinical practice. As they are kept current on data relating to, for example, the relationship of cigarette smoking to heart disease or cancer, they can increase a patient's awareness of the appropriateness of particular treatment modalities.

The results of research on health care practices serve to reeducate practitioners and update department procedure manuals. Thus, guidelines are provided for the improvement of clinical competence. This occurs as state of the art data on equipment, care modalities, physical diagnosis, and monitoring procedures are made available and their validity tested.

Professional Accountability

Health care professionals are accountable not only to their patients, departments, and hospital administrators, but to government agencies, third-party reimbursers, and the public at large. Our nation's entire health care system is under increasing pressure to justify the cost of services it provides. Government agencies and third-party reimbursers are asking us to show that the services we provide are both necessary and beneficial.

With our country's present state of economic austerity, allocation of funds to health care agencies, such as the Federal Drug Administration (FDA) and Centers for Disease Control (CDC), has been reduced. The functioning of these agencies, as well as Medicare, Medicaid, and Blue Cross/Blue Shield, affects health care both directly and indirectly. Investment in health care for the elderly and poor by the government is under close scrutiny to make sure that funds are going to pay for justifiable services. Understandably, with an increased federal role in paying the bills, there is increased pressure to assure the quality and quantity of care and that it is cost-efficient.

The high cost of health care must be supported by scientific justification. Regulations governing medical services and reimbursement are based on the current state of knowledge. Relevant questions about a service regard its necessity for the treatment of an established medical problem and whether it is of demonstrable benefit to a patient. The task of medical officials is to assure that the appropriate regulatory body has this information at its disposal. The task of health care researchers to make certain that the information is based on scientific data.

Administration of Health Care Services

Health care department managers and hospital administrators alike look toward the results of carefully completed studies to help solve problems relating to areas of concern such as cost containment, productivity assessment, departmental organization, and employee stress management. Managers are responsible for staffing their departments with qualified personnel, providing services that are delivered in a professional and timely manner, and making certain that infection control, safety, and preventative maintenance programs are ongoing and productive. How can managers best evaluate these services and programs? Which method of providing infection control, for instance, should a manager decide on? Knowing that equipment can be a major source of nosocomial infection, a method is needed of assessing the resultant change in infection rate that a program of disinfection or sterilization will hopefully affect. The cost-effectiveness of different methods must also be taken into consideration. The same type of

questions may be asked of patient and employee safety programs, and of other organization, delivery, and evaluation of patient care.

Evaluation of the quality of departmental programs and services is a difficult challenge. Empirical observation must not be the basis for acceptance or rejection. The costs of trial and error remedies are too prohibitive for this type of decision-making.

Continuous Quality Improvement The Joint Commission on Accreditation of Health Care Organizations (JCAHO) defines quality assurance as "a manner of demonstrating consistent endeavor to deliver optimal patient care with available resources and consistent with achievable goals. The correction of deficiencies is inherent to the process." This correction process is accomplished through the careful and rigid manipulation of variables and the measurement of any effects; in other words, using the scientific method. Only in this way can the physician, patient, patient's family, hospital, and government administrator be assured the quality of cost-effective services.

Evaluating New Equipment and Methods

Validating Manufacturer's Claims. To meet the changing needs of health care, medical equipment manufacturers introduce to the market new diagnostic and support instruments. Because of the relatively short product life cycle in the market of technical equipment, new products are introduced frequently. But *new* does not necessarily mean *better*. At times, the development of new technology outpaces the need for that technology. When this happens, product marketers have not done their job in accurately assessing demand. Medical professionals must then take the lead in assuring that they are not left in the position of trying to invent ways to use new equipment. Rather, new equipment should satisfy a well-established need. Although manufacturers often engage in extensive testing and market research, the final burden of proof as to a product's ultimate function and benefit falls to the end user, us.

For example, the introduction of synchronized intermittent mandatory ventilation (SIMV) on the Bourns Bear I ventilator came about as a result of the clinical observation that some patients breathing through standard IMV systems sometimes had mandatory machine breaths delivered during exhalation or on top of their spontaneous tidal breath. This *stacking* of breaths was believed to be harmful, or at least inefficient. SIMV ensures that a mandatory machine breath is not delivered until the ventilator senses a patient's respiratory effort, thus being ready to receive a large ventilator tidal volume. Synchronizing spontaneous breathing with mandatory ventilation, it was thought, would solve the problem of stacking and encourage more efficient breathing.

In principle, SIMV makes sense. But does it make a difference in any measurable sort of way? Does it make a difference in terms of alveolar ventilation, peak airway pressure, arterial PO_2, arterial PCO_2, or patient comfort? Are the potential benefits worth the added expense of this new ventilator feature?

These types of critical questions must be asked and systematically addressed when any new piece of equipment is made available to the field. Regarding SIMV, clinical research has indicated that breath stacking is indeed not clinically significant and that hemodynamic and arterial blood gas measurements do not improve when patients are switched from IMV to synchronized IMV.

Rather than accept on faith that a new technology will do exactly what its manufacturer claims, we should validate claims and conduct comparison tests with existing equipment. We should ask questions such as: What is the chance of nosocomial infection with this equipment? Does this equipment work equally well on a patient with chronic obstructive pulmonary disease (COPD) as it does on one with a flail chest? How accurate are the pressure manometers, spirometers, and gas analyzers provided?

Empirical observations often indicate a need for a new piece of equipment or procedure. But to insure safe and effective application, its final implementation must rest on sound scientific judgment.

QUESTIONS

Definitions

Explain the meaning of the following terms:

- Basic research
- Applied research
- JCAHO
- Quality assurance

True or False

1. The most important reason for studying research methodology is to gain the ability to read and critically evaluate studies published in medical journals.
2. The best thing you can do if you want to really learn how to do research is to find a mentor.

Multiple Choice

Which of the following are areas where we may apply the principles of scientific analysis to improve patient care:

a. Education.

b. Continuous quality improvement.

c. Evaluation of new equipment.

d. All of the above.

Chapter 2. Ethics and Research

In the health care industry today we are confronted with a multitude of laws, regulatory constraints, and standards that govern the conduct of the industry itself and the individuals who work in it. Conducting health care in this environment requires constant attention to a multitude of details. Conducting health care research demands additional attention to a special set of regulatory and ethical considerations.

Research involving human subjects, which we will refer to as *clinical* research, invokes legal, ethical, and sociologic concerns related to the safety and protection of the subject's basic human rights. Research involving animals requires responsible attention to several important concerns as well. Regardless of the type of study subjects, those engaged in medical research must be reminded that the importance of their work should never overshadow but, rather, complement society's health care goals. Complex procedures must strictly adhere to legal guidelines so that subjects are not exploited. Innovative and controversial research must be ethically conducted and honestly reported

The current and future prospects for productive and informative research in health care are as high now as they have ever been. In pursuing these prospects, the health care researcher must not only be concerned with the proper methodologies and logistics of running the actual study, but with legal and ethical issues that are no less important. Structuring research that is within the bounds of ethically and scientifically rigorous standards is an important and complex task, with a multitude of subtleties. The research investigator must achieve scientific rigor, while at the same time maintaining the highest ethical standards.

A complete discussion of all the ethical and legal implications of clinical research is beyond the scope of this text. The goal of this chapter is, first of all, to familiarize researchers with the institutional approval process they will need to navigate to begin research involving human subjects. Second, this chapter is designed to heighten the investigator's awareness with respect to several legal and ethical concerns they will undoubtedly encounter as they design and conduct their research endeavors in our modern environment. Finally, we will touch briefly on current ethical and legislative guidelines for conducting research involving animals.

INSTITUTIONAL REVIEW AND HUMAN SUBJECTS' RIGHTS

When human beings are used in scientific research, great care must be taken to insure that their rights are protected. To guarantee that protection, review boards have been established to ensure that proposed studies do not violate patient rights within a particular institution.

Functions of the Institutional Review Board

The health care researcher cannot and should not begin an investigation involving human subjects without formal approval from the hospital's Institutional Review Board (IRB). Also known as the Institutional Review Committee, Human Subjects Review Committee, Human Investigation Committee, or Research Surveillance Committee, IRB refers to any committee, board, or other group formally designated by an institution to review biomedical research involving human subjects. This committee meets at certain specified intervals to review, recommend, and approve study proposals.

The main functions of the IRB are to protect the rights, well-being, and privacy of individuals, as well as protect the interests of the hospital or center in which the research is conducted. Specific IRB procedures will vary from institution to institution. In each case, health care workers must review those guidelines applicable in their own institution.

Although IRB guidelines may vary somewhat from one institution to the next, IRBs are typically established, operated, and function in conformance with regulations set forth by the US Department of Health and Human Services (DHHS), regulations established to protect the rights of human subjects that apply to all institutions receiving federal funds. The DHHS issued regulations in 1981 that must be followed for biomedical and behavioral human research to receive such funds.

Consideration of risks, potential benefits, and informed consent typically occupies the majority of the IRB's time. Before an IRB can approve a research protocol, the following conditions must be met.

1. The risks to the (research) subject are so outweighed by the sum of the benefits to the subject and the importance of the knowledge to be gained as to warrant a decision to allow the subject to accept these risks.
2. Legally effective informed consent will be obtained by adequate and appropriate methods.
3. The rights and welfare of any such subjects will be adequately protected.

Review of research involving human subjects must always occur before the initiation of research and may be required at specified intervals during the lifetime of the research activity. If an application for external funding is being considered, the researcher should thoroughly review the study proposal before submission to the funding agency. The IRB frequently may ask the investigator to modify the original research plan to comply with Food and Drug Administration (FDA) and DHHS regulations as well ethical norms. However, the IRB is not a police force. There is a presumption of trust that the approved research protocol will indeed be followed consistently. Nevertheless, investigators have been known to deviate from the agreements reached with an IRB.

Composition of the Institutional Review Board

To provide input representing a wide variety of concerns, the IRB committee is typically composed of members with diverse backgrounds. An IRB characteristically includes representatives of administration, staff, and legal areas of both the institution and the community. This diversity encourages that proposed research be reviewed for acceptability, not only in terms of scientific standards, but in terms of community acceptance, relevant law, professional standards, and institutional regulations as well.

As well as a diverse background, committee members exhibit a high standard of personal and professional excellence. IRB members should exhibit sufficient maturity, experience, and competence to assure that the Board will be able to discharge its responsibilities and that its determinations will be accorded respect by investigators and the community served by the institution. The quality of an IRB decision is thus a direct reflection of the degree of maturity, experience, and competence of its members

Approval of the Institutional Review Board

The investigator must formally apply for IRB approval before beginning a study. A thorough IRB application typically includes the components listed in Table 2-1. First, a formal research protocol must be established. This description of the study's intended purpose and procedures is then followed by human subjects information, which should describe sources of potential subjects and the anticipated

number required. Also included should be a description of the consent procedures, and a description of potential risks and benefits as they relate to both the subjects and to society.

An integral part of the study protocol, and a necessary component for IRB review, is the patient or subject consent form, discussed in greater detail later in this chapter. To prepare this form properly, a number of issues (Table 2-1) must be thoroughly addressed. The content of each of these areas of concern must then be prepared with the consent form for the information of the potential study subject.

TABLE 2-1. Typical components of an IRB proposal.

1. A complete description of the study's intended purpose and procedures to be followed.
2. A description of potential risks the subject may incur from participation in the study.
3. A description of potential benefits, either direct or indirect, the subject may incur from participation in the study.
4. A description of how data will be handled such that the subject's identity remains anonymous.
5. A statement that the subject may withdraw from the study at any time without a prejudicial effect on his or her continuing clinical care.
6. The name and number of the investigator, should any questions arise regarding the subject's participation in the study.
7. Copy of the complete Informed Consent form.
8. A list of available alternate procedures and therapies.
9. A statement of the subject's rights, if any, to treatment or compensation in the event of a research-related injury.

INFORMED CONSENT

Informed consent is the voluntary permission given by a person allowing himself to be included in a research study after being informed of the study's purpose, method of treatment, risks and benefits.

A key principle of ethical conduct in research is that participation in studies must be voluntary. In turn, voluntary consent is predicated on communicating all the information the potential subject needs to be self-determining. The consent form represents the culmination of much effort devoted to protect the rights of research subjects through the process of fully informing them before their involvement in clinical research.

Background

The Nuremburg Trials after World War II revealed the atrocities committed by Nazi physicians. As a result of these revelations, voluntary informed consent became a central focus of biomedical ethics. The doctrine of informed consent is designed to uphold the ethical principle of *respect for persons.* As such, this doctrine is now grounded in a body of medicolegal decisions that cite a failure to obtain adequate informed consent as either *battery* or *negligence.*

Having received critical commentary for the past 35 years, the protection of human subject's rights has received formal legislative attention within the past 20 years. In legitimizing this emphasis, the World

Medical Association adopted the Declaration of Helsinki in 1964. This declaration recommended that informed consent be obtained "if at all possible, consistent with patient psychology" for "clinical research combined with patient care." Before this, potential volunteers were protected only by the assumed responsibility of the individual investigator to explain fully the nature of the research. But abuses of this responsibility led to the development and implementation of the informed consent requirement.

Role Today

Today, informed consent is a crucial feature of virtually all clinical trials. No research involving human subjects should be initiated without the informed and voluntary consent of those subjects. Competent patients must be offered the opportunity to accept or reject a medical intervention proposed as part of their participation in a research study. Likewise, incompetent patients must be offered the same opportunity through the mediation of a legal guardian or surrogate.

For consent to be *informed,* the potential subject must be given information regarding all the possible pros and cons of the proposed medical intervention. We always move toward maximizing the patients' best interests while enhancing their participation in decision-making. As a vehicle, the consent form clearly summarizes the IRB application. The consent form must contain all the elements (Table 2-1) necessary so that a patient's rights will be protected should he or she elect to participate in the research study.

Revocation of Consent

A subject may withdraw from a research activity at any time during the course of the study, within the limits of the research. Any request for withdrawal should be honored promptly. As spelled out in the consent form, revocation of consent and participation in the research study should never result in a subject being penalized or made to forfeit benefits to which he or she is otherwise entitled**.** However, the subject's commitment to participate in a research study is seen by some to represent a moral obligation. In this context, research can be viewed as a joint venture between investigator and subject. The subject has made a promise to participate, to bear the inconvenience of testing in return for the benefit he or she hopes to derive.

Nevertheless, should a subject wish to withdraw from participation in research, the investigator must fully inform the subject of the potential dangers. For example, an asthmatic subject who abruptly withdraws from a study examining the efficacy of an investigational bronchodilator should be informed of what improvement or lack thereof he or she had shown with the use of that bronchodilator. Should the subject still choose to withdraw from the study, a smooth transition to an alternative bronchodilator must be provided. If a subject suffering from pneumonia wishes to withdraw from a study of the effect of chest physiotherapy on spirometric and plethysmographic values, that person should be informed of his or her progress since the administration of the investigational treatment regimen. Alternatives to the current mode of therapy must be described so the pros and cons of withdrawal from the study can be properly evaluated. In all instances, the implications of withdrawal from therapy must be made clear to the subject, and arrangements made for a smooth, uneventful transition to standard clinical care.

ETHICAL ISSUES

Basic Principles

Professional ethics in health care ethics is a subset of the category of medical ethics, which in turn is a division of the much broader philosophy of ethics. Although the law sets a minimum level of expected behavior, ethics generally requires more than the minimum, and often aims toward the ideal. Every clinical researcher, regardless of the study, has relevant ethical responsibilities to which he or she may be held accountable. The following discussion will address ethical decisions in the field of clinical research as they concern health care investigations.

Three fundamental ethical principles relevant to clinical research are *respect for persons, justice,* and *beneficence.* Respect for persons is interpreted to mean that those conducting clinical research will endeavor to treat potential subjects as autonomous, self-determining individuals. Furthermore, those subjects not capable of making considered judgments (incompetent), those either immature or incapacitated, are entitled to the protection they deserve. The principle of justice requires that all persons be treated fairly and equally. Finally, beneficence can best be understood as a commitment to do no harm and to maximize the potential benefits while minimizing potential harms. Incumbent in this definition is the understanding that no person will be asked to accept risks of injury in the interest of producing societal benefits.

Research studies that violate these standards have been documented and serve as a basis for the contemporary balance between human experimentation and legal regulation of medical research. In 1932, male prisoners with syphilis were recruited without consent and misinformed as to their treatment. When penicillin became available for the treatment of syphilis, these men were not informed. In another study, patients with various chronic debilitating diseases were injected with live cancer cells. Consent was said to have been negotiated, but was never documented due to the investigator's contention that informing the patients of the procedure would frighten them unnecessarily. These and other abuses have combined to tighten both legal regulations and ethical guidelines for clinical research.

Ethical concepts differ substantially from legal concepts. Ethical concepts have evolved into the various professional standards and principles that guide the practice of medicine. Professional standards do not carry the weight of law; only statutes and common law have any legal authority in this country. However, many statutes and many court decisions have been, and will continue to be, extensively based on the moral and ethical convictions of the health care professions. Health care ethics may be considered a subset of the larger fields of medical ethics. No longer is the physician the absolute ruler and his or her ancillary helpers mere followers who cannot be expected to exercise any moral judgment of their own. Furthermore, medical care is no longer delivered solely by physicians and nurses. The contemporary health care industry employs a variety of professional health care practitioners, each with a high and noble ethical code of conduct no less meaningful than the Hippocratic oath. For example, the field of respiratory care operates under an ethical code, represented by the American Association for Respiratory Care Code of Ethics (Table 2-2). The issues of health care ethics are becoming more numerous and complex with nearly every major medical advance that is implemented. A partial list of the pressing issues of the current time would include death with dignity, euthanasia, discontinuation of life support systems, organ transplantation, genetic engineering, behavior modification, use of animal experimentation, and a further subset of issues that come under the general heading of human experimentation for health care research. In addition to the basic ethical principles of respect for

persons, justice, and beneficence, what other issues can the health care researcher expect to confront? There is several discussed below that deserve consideration.

Table 2-2. Statement of Ethics and Professional Conduct

In the conduct of professional activities the Respiratory Therapist shall be bound by the following ethical and professional principles. Respiratory Therapists shall:

Demonstrate behavior that reflects integrity, supports objectivity, and fosters trust in the profession and its professionals. Actively maintain and continually improve their professional competence, and represent it accurately.

Perform only those procedures or functions in which they are individually competent and which are within the scope of accepted and responsible practice.

Respect and protect the legal and personal rights of patients they care for, including the right to informed consent and refusal of treatment.

Divulge no confidential information regarding any patient or family unless disclosure is required for responsible performance of duty, or required by law.

Provide care without discrimination on any basis, with respect for the rights and dignity of all individuals.

Promote disease prevention and wellness.

Refuse to participate in illegal or unethical acts, and refuse to conceal illegal, unethical or incompetent acts of others.

Follow sound scientific procedures and ethical principles in research.

Comply with state or federal laws which govern and relate to their practice.

Avoid any form of conduct that creates a conflict of interest, and shall follow the principles of ethical business behavior.

Promote health care delivery through improvement of the access, efficacy, and cost of patient care.

Refrain from indiscriminate and unnecessary use of resources.

Objective Patient Care

Under the auspices of a physician, the health care practitioner contractually undertakes to give a patient the best possible treatment. Indeed, at the core of modem medical ethics is the Hippocratic promise to do one's best for every patient and to do no harm. Does the very act of enrolling a patient in a randomized clinical trial violate this obligation? Consider the patient with chronic obstructive pulmonary disease who agrees to participate in a study of the effects of a new bronchodilator. Does this subject fully understand the implications of falling into the placebo group? Does a subject suffering from cystic fibrosis fully understand that randomization to the control group may mean that the frequency of chest physiotherapy will not be increased during the study period regardless of a relative deterioration in his measured work of breathing. Some critics believe that if a clinician or investigator has reason to believe that the experimental treatment is better than the control treatment, he or she must recommend the experimental option. For example, suppose a new aerosolized drug seems to be highly effective and superior to the standard treatment of patients with acute respiratory distress syndrome. A controlled clinical trial is undertaken, with 50 patients randomized to receive conventional therapy of mechanical ventilation with positive end-expiratory pressure, increased FiO_2 and supportive fluid therapy. Another 50 patients are randomized to the treatment group, and receive the new drug in addition to conventional therapy. Now suppose that 15 patients in the experimental group die, as opposed to 30 patients in the control group. Is the clinical investigator guilty of unethical behavior? Is he or she guilty of a crime, a sin of omission?

Unfortunately, there are no clear-cut answers. As is made clear in the Nuremberg codes, the degree of risk to be taken should never exceed that determined by the humanitarian importance of the problem to be solved by the experiment. In other words, there should always be a favorable balance between harm and benefit. The Declaration of Helsinki further reinforces this principle in stating that "Biomedical Research involving human subjects cannot legitimately be carried out unless the importance of the objective is in proportion to the inherent risk to the subject." The fundamental ethical principle is that of beneficence. Furthermore, justice and respect for persons are served when a study's potential harms and benefits are clearly and properly presented to the subject for his or her informed consent

Reporting Research Results

Scientific investigations are based to a very high degree on trust. We trust that each investigator will conduct his or her research in accordance with the protocol approved by the appropriate IRB. And we trust that all research findings will be reported accurately and without intentional bias. Abandoning trust would lead to overwhelming suspicion and make scientific investigation impossible. Without trust in the honesty and integrity of published findings, how would progress in science and medicine be possible?

Fortunately, fraud in science is rare, due to the skepticism of the scientific community. No experiment is accepted until it has been independently repeated. Research results, no matter how sensational, are quickly forgotten if they cannot be obtained from other investigators duplicating the study methodology.

QUESTIONS

Definitions

Explain the meaning of the following terms:

- IRB
- Informed consent

True or False

1. The IRB is intended to protect the rights of patients involved in research studies.
2. The IRB is composed of the people who designed the research study.

Multiple Choice

1. Typical components of an IRB proposal include:
    a. Description of study purpose
    b. Potential risks and benefits
    c. Informed consent form
    d. Description of investigator's previous experience
    e. All of the above.
    f. Only a, b, and c.
2. Three fundamental ethical principles relevant to clinical research are:
    a. Respect for persons.
    b. Cost containment.
    c. Justice.
    d. Beneficence.
    e. a, b, d
    f. a, c, d
3. At the core of modern medical ethics is the Hippocratic Oath, which obligates caregivers to:
    a. Treat everyone fairly.
    b. To do no harm.
    c. To give only treatment proven by scientific methods.
    d. To obtain informed consent before entering a person in a study.

Chapter 3. Outcomes Research

As in other areas of medicine, outcomes research is starting to make its mark in defining optimal health care practices. With the need for cost containment, outcomes research becomes a double-edged sword used both to cut nonessential practices and to protect those that maintain quality of care. The profession of health care has a long history of research and a commitment to basing practice on science. However, much of the published research is still focused on devices and procedures rather than the broader issues of patient outcomes and economic effects. We need to evolve our paradigms to accommodate the larger vision of disease management, which encompasses the arenas of outcomes research and evidence-based medical practice.

In this chapter, I will give a brief history of the outcomes research movement to provide some sense of context. Then, I will try to demystify the language of outcomes research and review some of its themes and methods. Finally, I will present specific examples of outcomes research found in the pages of Respiratory Care journal. Hopefully, these examples will illustrate some of the methods of outcomes research and stimulate future studies.

A BRIEF HISTORY

Florence Nightengale may have been the first outcomes researcher in medicine. She had a flair for collecting, analyzing and presenting data. She even invented the polar-area chart, where the statistic being represented is proportional to the area of a wedge in a circular diagram. Yet, she had as much trouble finding appropriate data as we do today. And like modern times, there was much opposition to the reforms proposed by Nightengale. Nevertheless, her most effective weapon was the presentation of solid, relevant data. For example, she showed "...that 'those who fell before Sebastopol by disease were above seven times the number who fell by the enemy.' The opposition could not respond to her statistics and publication of the statistics led to public outcry."

The modern outcomes movement in the United States had its beginnings in the early 1980s. The increasing focus on cost containment led to interest in identifying and eliminating unnecessary procedures. Perhaps more intriguing was the recognition that there were substantial variations in medical practice, apparently based on geography or race. Indeed, some researchers claimed that "geography was destiny" because medical practices as commonplace as hysterectomy and hernia repair were performed much more frequently in some areas than in others, with no differences in the underlying rates of disease.

Given that there are variations in practice and differences in outcomes, we may logically assume that some practices produce better outcomes than others. So the stage was set to improve efficiency and quality if only the right data were available. But where to look? The Office of Technology Assessment estimated that only 10% to 20% of interventions by physicians have been clearly shown in randomized clinical trials to be of value. This is not surprising, given that clinical trials can cost millions of dollars and last years. Some suggested that data collected for administrative or billing purposes (e.g., Medicare and Medicaid tapes collected by the Health Care Financing Administration) might contain valuable outcome data such as mortality, length of hospital stay, resource use, and costs. On the one hand, such data can be quickly analyzed, without requiring patient consent or interfering with medical care. On the other hand, critics argued that this type of research is limited by the quality and completeness of the data.

New data must be collected in a systematic fashion with a specific focus on outcomes. In 1989 Congress created the Agency for Health Care Policy and Research (AHCPR). It consisted of 11 major components including the Center for Outcomes and Effectiveness Research, the Center for Cost and Financing Studies, and the Center for Quality Measurement & Improvement. The initial focus of the AHCPR was to create Patient Outcomes Research Teams (PORTs; 5-year studies of specifically identified diseases with highly focused methods), the Pharmaceutical Outcomes Research Program, and the Minority Health Research Centers. In time, the AHCPR changed its name and its focus. Today, at the Agency for Healthcare Research and Quality, the focus is on Translating Practice Into Research, creating Excellence Centers for Eliminating Disparities (based on race and ethnicity) and supporting the Centers for Education and Research on Therapeutics. According to the AHRQ, the purpose of outcomes research is to answer four basic questions:

- What works?
- What doesn't?
- When in the course of an illness (does it work or not)?
- At what cost.

These questions suggest the scope and focus of modern outcomes research.

UNDERSTANDING THE JARGON

Like any new discipline, the field of outcomes research suffers from a lack of consistent definitions and a unifying conceptual framework. Many seemingly unrelated terms are encountered in the literature such as efficacy, effectiveness, quality of life, patient centered care, evidence based medicine, etc. All these terms signify a paradigm shift in which the emphasis is on populations rather than individuals; on practice guidelines rather than anecdotal justifications for treatment; and on capitation rather than fee-for-service payments. I have found it helpful to view this new paradigm in terms of the general concept of "disease management" within which the specific activities of outcomes research and evidence-based medicine interact in a process of continuous quality improvement.

Disease management (also called outcomes management) can be defined as the systematic, population based approach to identify patients at risk, intervene with specific programs, and measure outcomes. The basic premise of disease management is that an optimal strategy exists for reduced cost and better outcomes. Disease management emphasizes identifying populations of interest, creating comprehensive interventions, explicitly defining and measuring outcomes, and providing a strategy for continuous quality improvement.

Continuous quality improvement (CQI) is a cycle of activities focused on identifying problems or opportunities, creating and implementing plans, and using outcomes analysis to redefine problems and opportunities. CQI was started decades ago by pioneers such as Shewert, Deming, and Juran and is currently embraced by the Joint Commission on Accreditation of Healthcare Organizations. The "plan, do, check, act" cycle endorsed by JCAHO can be viewed as simply creating plans and implementing them. The plan leads to implementation through the creation of specific goals. Implementation leads to more plans through the analysis of results (Figure 3-1).

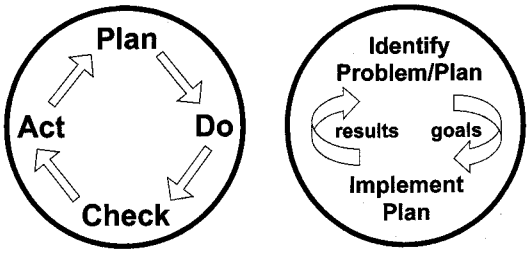


Figure 3-1. Continuous quality improvement expressed in the traditional format of a cycle of "plan, do, check, act" and an equivalent cycle showing the interaction of plans and implementations through goals and measured results.

Outcomes research can be defined as the scientific study of the results 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.

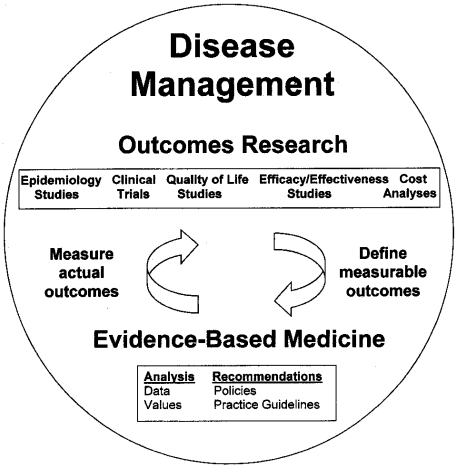
Evidence-based medicine is an approach to practice and teaching that integrates pathophysiological rationale, caregiver experience, and patient preferences with valid and current clinical research evidence. To implement evidence-based medicine, the practitioner must be able to define the patient problem, search and critically appraise data from the literature and then decide whether, and how, to use this information in practice.

If we view disease management as a universe of activities, then outcomes research (e.g., epidemiological studies, clinical trials, quality of life surveys, efficacy and effectiveness studies, and cost analyses) and evidence-based medicine (e.g., creation and use of practice guidelines and care paths) can be seen as subset activities linked by the general structure of continuous quality improvement (Figure 3-2).

OUTCOMES RESEARCH: FOCUS AND METHODS

Outcomes research can be distinguished from traditional clinical research more by its focus than on the methods it employs. This difference in focus is highlighted in Table 3-1. Appropriate outcomes can be roughly grouped into three categories; clinical, economic, and humanistic (Table 3-2).

Figure 3-2. Disease management expressed as a continuous quality improvement cycle showing the interaction of plans (created by outcomes research) and implementations (evidence-based medicine tools) through goals (desired outcomes) and measured results (actual outcomes).



Outcomes research uses a variety of techniques (Table 3-3). *Qualitative research* often produces large amounts of textual data in the form of transcripts and observational field notes. Rather than trying to identify a statistically representative set of observations, qualitative research uses analytical categories to describe and explain social phenomena. Qualitative research generates hypotheses (although not necessarily hypothesis tests), and attempts to identify the relevance of findings to specific groups of people.

Table 3-1. Differences between traditional clinical research and outcomes research.

Traditional Clinical Research	Outcomes Research
Disease-centered	Patient and community centered
Drugs and devices	Processes and delivery of care
Experimental	Observational
Methods from "hard sciences" (physics, biochemistry)	Methods from "social sciences" (economics, epidemiology, etc)
Efficacy	Effectiveness
Mechanisms of disease	Consequences of disease on patients
Effects of biochemical and physiologic factors	Effects of socioeconomic factors

Quantitative research uses both experimental and non-experimental designs. The classic experimental design of the randomized controlled trial can be applied to outcomes research if it is set up to evaluate effectiveness (as opposed to efficacy, see definitions below). Non-experimental designs can focus either on data synthesis or observational study designs.

In keeping with the population-based theme of outcomes research, methods are needed to synthesize data from numerous studies, as opposed to interpreting the results of a single study. One such method is called *meta-analysis*. The National Library of Medicine defines meta-analysis as follows:

A quantitative method of combining the results of independent studies (usually drawn from the published literature) and synthesizing summaries and conclusions which may be used to evaluate therapeutic effectiveness, plan new studies, etc, with application chiefly in the areas of research and medicine. The method consists of four steps:

1. A thorough literature review,
2. Calculation of an effect size for each study,
3. Determination of a composite effect size from the weighted combination of individual effect sizes
4. Calculation of a fail-safe number (number of unpublished results) to assess the certainty of the composite size

Table 3-2. Various types of outcome measures used in outcomes reserach**.**

Category	Type	Example
Clinical	Clinical events	Myocardial infarct
	Physiologic measures	Pulmonary function indices
	Mortality	Asthma deaths
Economic	Direct medical costs	Hospital and outpatient visits
	Indirect costs	Work loss, restricted activity days
Humanistic	Symptoms	Dyspnea scores
	Quality of life	SF-36 Questionnaire, St. Georges Respiratory Questionnaire
	Functional status	Activities of daily living
	Patient satisfaction	Cleveland Health Quality Coalition

Decision analysis is used to determine optimal strategies when there are several alternative actions and an uncertain or risk-filled pattern of future events. This technique is a derivative of operations research and game theory. It involves identifying all available choices and the potential outcomes of each. Usually a model is created in the form of a decision tree. The tree is used to represent the strategies available to the clinician and the likelihood that each outcome will occur if a particular strategy is chosen. The relative value of each outcome can also be described.

There are several basic types of economic evaluations that are applied to health issues. *Cost identification* is simply the description of the costs of providing the intervention. It is the first step in all the other types of analyses, but is often the only one reported in a study. *Cost of illness* analysis estimates the total cost of a disease or disability to society (e.g., heart disease costs the United States $128 billion per year). *Cost minimization* is applied when two or more interventions are being compared on the same outcomes and the outcomes seem to yield similar effectiveness. Then the question is simply, which is least expensive. An example would be the question of whether to repair or replace a mechanical ventilator. When the same outcomes are measured but the effectiveness differs, then they are compared on the basis of cost per outcome (e.g., dollars per life saved or dollars per additional year of life) using *cost effectiveness* analysis. If both outcomes and effectiveness differ, then a *cost-benefit analysis* first attempts to express both outcomes and benefits in terms of dollars. Then the interventions are evaluated in terms of the overall economic tradeoffs among them. In this way the cost of, for example, a smoking prevention program can be compared to that of lung reduction surgery and both can be compared to other programs such as highway development or job training. *Cost utility* analysis is similar to cost

effectiveness except that the effectiveness is expressed as a "utility" which is the product of a clinical outcome, such as years of life saved, and a subjective weighing of the quality of life to be had during those years. Utility is often expressed as quality-adjusted life years (QALYs). For example, quality of life is often measured on a linear scale where 0 indicates death (or indifference to death) and 1.0 represents perfect health. Suppose a patient is discharged to a chronic ventilator weaning facility for 6 months and dies on the ventilator. If the patient rates the utility of life on the ventilator as 0.2, the patient has experienced 0.5 x 0.2 = 0.1 QALYs. If the assumptions are correct, this means that 6 months on a ventilator in a weaning facility is approximately equal in value to the patient as 1 month (0.1 year) in perfect health. Economic analyses can seem overly complicated. For a very readable introduction written in the style of a conversation between two doctors, see the article by Eddy.

Table 3-3. Methods used in outcomes research.

- **Qualitative methods** (formal hypothesis testing not necessarily required)
  - Generate hypotheses
  - Describe complex phenomena
  - Identify relevance of findings to specific patient groups/
- **Quantitative Methods**
  - *Experimental*
    - Randomized controlled trials
  - *Non-experimental*
    - Data synthesis
      - Meta-analysis
      - Decision analysis
      - Economic analysis
  - *Observational studies*
    - Cohort
    - Case-control
    - Survey

Quality of life (QOL) measures have been important in research since the 1970s. Uses of QOL data include distinguishing patients or groups, evaluating therapeutic interventions, and predicting patient outcomes. However, there are many QOL instruments and much theory but no unified measurement approach. And there is little agreement on definitions and interpretations. Some authors argue that because QOL is a uniquely personal perspective, patient-specific measures should be used.

Another issue that seems confusing is the difference between efficacy studies and effectiveness studies. An example of the type of question answered by an *efficacy* study is as follows: "Does the intervention

work in a tertiary care setting with carefully selected patients under tightly controlled conditions?" This type of study generally requires a priori hypotheses, randomization of subjects to predefined treatments, homogeneous patient populations at high risk for the outcome, experienced investigators following a specific protocol, a comparative intervention (e.g., a placebo) and intensive follow-up. Conclusions from this type of study prompt relatively high levels of confidence. However, because the design is so restrictive, the results may not be generalizable to a broad range of patients in usual practice settings. Thus, efficacy studies may not be appropriate for cost-effectiveness analyses.

In contrast, *effectiveness* studies are designed to answer questions such as: "Does the intervention work in clinical practice settings with unselected patients, typical care providers and usual procedures." Many effectiveness studies have been conducted as observational (often retrospective) studies where observed groups were not randomly assigned and neither patients nor providers knew they were being studied. The weakness of this study design is that selection bias may be a problem (i.e., the groups may not have the same prevalence of confounding variables) so adjustment for factors such as severity of illness and case mix becomes important. Prospective effectiveness trials have been reported. They differ from typical clinical trials in that they enroll heterogeneous participants, impose few protocol-driven interventions, and report outcome measures relevant to the delivery system.

THE OUTCOME OF OUTCOMES RESEARCH

The Lewin Group has created a report of outcomes and effectiveness research (OER) at the Agency for Health Care Policy and Research that describes the accomplishments and lessons of the past decade. The report describes a conceptual framework for understanding and communicating the impact of OER on health care practice. Four levels of impact are defined:

1. Findings that contribute to but do not alone reflect a direct change in policy or practice, such as new analytic methods or outcome instruments.
2. Research that prompts the creation of a new policy or program, such as an AARC clinical practice guideline.
3. A change in what clinicians or patients do.
4. Actual changes in health outcomes.

A survey was mailed to all principal investigators (PIs) funded by AHCPR's Center for Outcomes and Effectiveness Research between 1989 and 1997. The results suggest that PIs have been most successful in (a) providing detailed descriptions of what actually occurs in health care, (b) developing tools for measuring costs of care and patient reported outcomes, and (c) identifying topics for future research. Few PIs reported findings that provide definitive information about the relative superiority of one treatment strategy over another. Furthermore, there were few examples of findings that have been incorporated into policy (level 2 impacts) or clinical decisions (level 3), or interventions that have measurably improved quality or decreased costs of care (level 4). The report concludes that "*One of the main challenges for the next generation of outcomes studies is to move from description and methods development to problem solving and quality improvement*."

I should point out that not everyone believes that outcomes research is good. Some authors voice both practical and philosophical arguments against the outcomes movement. They claim the outcomes movement exaggerates its usefulness by understating several difficulties. For example, how much time and money will be required to determine the effectiveness of many commonly used (and continuously evolving) medical procedures? How will physicians use outcomes data when making multiple

consecutive decisions in the rush of daily patient care? And how will compliance with practice guidelines be enforced? Some data suggest that clinical practice guidelines have been remarkably unsuccessful in influencing physician behavior. Reasons for this include the fact that some guidelines are not written for practicing physicians, the issue of physician disagreement with or distrust of guidelines written by so-called national experts, and physicians choosing to ignore guidelines because of non-clinical factors such as financial incentives or fear of malpractice litigation. This last issue is echoed by the opinion that many physicians are opposed to the kind of micromanagement and attendant loss of clinical autonomy-envisioned by the participants in the outcomes movement. Some proclaim that uncertainty and subjectivity are at the heart of the clinical encounter and this will always be the case. Also, by criticizing the uncertainty of physicians, the outcomes movement may set the unrealistic goal of creating important certainties for practitioners and thereby misrepresents the terms of the clinical encounter and inadvertently undermines confidence in the physician's ability to act wisely in the face of inevitable uncertainty.

EXAMPLES FROM RESPIRATORY CARE

Outcomes research, along with its methodologies and core curriculum, can be viewed as an important discipline for the field of respiratory care. Specific areas where outcomes research techniques could be employed include:

1. Determining the effectiveness of CQI initiatives.
2. Comparing variations in respiratory care practices in order to identify optimum strategies.
3. Developing and assessing innovations.
4. Evaluating resource utilization in areas employing respiratory care professionals compared to similar settings without them.

Indeed, the profession's scientific journal, Respiratory Care, has published a substantial amount of outcomes research in the last few years. A quick survey of articles in the Original Contribution category of Respiratory Care from 1997 through 2000 showed about 28% of articles could be classified as outcomes research. While, the majority of articles are still focused on devices and procedures, a number of those focused on problem solving and quality improvement may serve as examples. What follows is a brief description of the methodology used in a few of these studies:

Stoller JK, Orens D, Ahmad M. Changing patterns of respiratory care service use in the era of respiratory care protocols: An observational study. Respir Care 1998;43(8):637-642.

This was an observational study that qualifies as outcomes research because it was an evaluation of clinical outcomes in a "real world" setting during variations in respiratory therapy practices. The authors hypothesized that the use of a respiratory care consult service would decrease over-ordering of respiratory care services and decrease the volume of respiratory care services delivered. Data were obtained from departmental management information system (Clinivision, Puritan-Bennett) and from the hospital's cost management software (Transitions Systems). They compared baseline data from 1991, prior to establishment of a respiratory care consult service in 1992 to clinical data from 1996. Results were reported using descriptive statistics (averages, percentages, and trend graphs) of numbers of therapies, numbers of patients treated, and costs of therapies.

Adams AB, Shapiro R, Marini JJ. Changing prevalence of chronically ventilator-assisted individuals in Minnesota: Increases, characteristics, and the use of noninvasive ventilation. Respir Care 1998;43(8)643-649.

This is an example of an epidemiology study. Such studies describe the distribution and size (prevalence and incidence) of disease problems in human populations. The authors developed a study question, "Did cost constraints and changes in care settings and techniques affect the number of ventilator-assisted individuals (VAI), their sites of care, or methods used for ventilatory assistance." They defined VAIs and specified inclusion/exclusion criteria. Data were generated from surveys sent to all sites providing VAI care. Results were reported using descriptive statistics (averages, medians, percentages, and bar graphs) numbers of patients treated and diagnostic categories.

Myers TR, Chatburn RL, Kercsmar CM. A pediatric asthma unit staffed by respiratory therapists demonstrate positive clinical and financial outcomes. Respir Care 1997;43(1):22-29.

Here is an example of a controlled clinical trial designed as an effectiveness study (i.e., a heterogenous patient population treated by usual caregivers in a standard acute care environment). The authors tested the hypothesis that using respiratory therapists in a disease management model using a dedicated asthma unit and a standardized treatment protocol would improve efficiency of care compared to the historic method of random placement of patients with care dictated by individual physician preference. An algorithmic treatment protocol was defined. Patients treated by protocol were compared to historic controls. Data were obtained from patient charts and the hospital information system. Outcomes were stratified by an asthma severity index. Results were reported using inferential statistics to compare hospital length of stay, cost/case and care path variances. Nonparametric tests were used to assure the comparability of the two treatment groups on confounding factors such as age, race, and distribution of disease severity.

Parker, Walker. Effects of a pulmonary rehabilitation program on physiologic measures, quality of life, and resource utilization in an HMO setting. Respir Care 1998;43(3):177-182.

This study provides a good example of how to assess quality of life (QOL) issues. The researchers created a priori hypotheses and described the study population based on diagnosis and physiologic measures. They defined the intervention as rehabilitation classes at a specific frequency and duration along with an exercise program. Their QOL survey was abstracted from other published, validated QOL instruments. They used inferential statistics to compare charges and QOL scores. Results were reported using graphs and mean values.

Smith KJ, Pesce RR. Pulmonary artery catheterization in exacerbations of COPD requiring mechanical ventilation: A cost-effectiveness analysis.

Despite its title, this study is an example of cost-utility analysis, as I have defined previously, because the results are expressed in terms of patient utility (using a QOL score on a scale of 0 = death to 1.0 = perfect health) and quality-adjusted life-years. This article provides an excellent description of a complex topic, showing how a decision tree model is constructed (Figure 3-3), how probabilities of different outcomes are estimated, how costs are attributed and how utility is calculated. In addition, it provides an example of how sensitivity analysis is used to evaluate the effects of varying baseline values (i.e., assumptions) within the model.

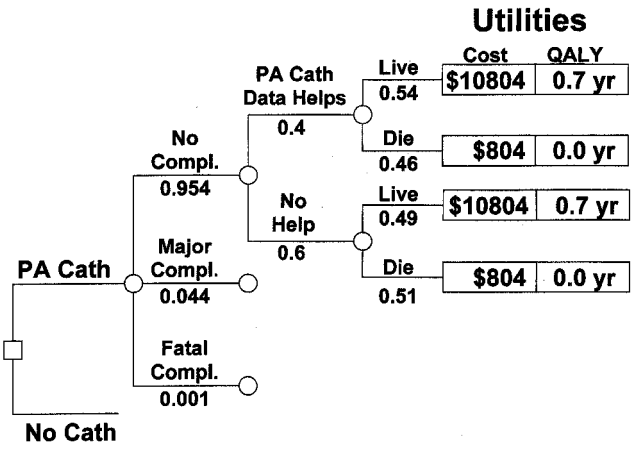


Figure 3-3. A portion of a decision tree used in a cost-study analysis. The model includes baseline values for probabilities, costs, and quality-adjusted life years (QALY). Probabilities are expressed as decimal numbers below the tree branch labels. The square node represents the decision whether to perform pulmonary artery catheterization (PA Cath) or not (no Cath). The circular nodes are the possible outcomes. QALY = life expectancy x quality of life utility value.

BENCHMARKING

Most of the medical procedures we practice each day have never been and never will be supported by formal scientific research. There simply is not enough time or money to do so. However, we can still logically justify what we do. The next best thing to scientific research is *benchmarking*. A benchmark is literally a standard or point of reference in measuring quality. As it relates to industry or health care, benchmarking is the process of comparing your performance with your peers to see who is the most successful. Benchmarking is often defined as a continuous process of measuring products, services, and practices against one's toughest competitors or renowned industry leaders, and then learning from them.

Three types of benchmarking are generally recognized: collaborative, functional, and internal. Collaborative benchmarking enables an organization to learn from the best practices within a voluntary network of health care providers. Collaborative benchmarking is often managed by a professional organization such as the University Hospitals Consortium.

Functional benchmarking compares a work function with the functional leader even when the leader is in a different industry. However, clinical functions, by their technical nature, restrict the search for benchmarking partners to health care organizations.

Internal benchmarking involves the identification of best practices within one's own organization. Internal benchmarking is both an effort to improve performance and a low risk way to share performance data. By publishing your performance data in medical journals, others can learn what a high-performing organization is doing to achieve results.

Benchmarking depends on the disciplined collection and use of objective information. The paradigm is simple, and entails:

- Identifying critical success factors and determining key indicators;
- Collecting information relevant to the key indicators;
- Searching to identify extraordinary performers, as defined by the data;
- Identifying the factors that drive superior performance;
- Adopting or adapting those factors that fit into your processes.

Benchmarking indicators are of three types:

Ratio Indicators: Indicators that establish a relationship between two measures (e.g., worked hours/unit of service). Ratio indicators are generally indicative of productivity or of a volume measurement. They provide a comparative performance point to other departments or hospitals, but do not reveal information about the practices that drive the performance.

Process Indicators: Indicators that measure a process with a beginning point and an ending point (for example, blood gas measurement turn-around-time). Process indicators lead to investigations of the practice that drives the performance.

Outcome Indicators: Indicators that measure clinical outcomes (for example, patient returns to the emergency department within 24 hours). Outcome indicators lead to an understanding of the practices that provide the best possible clinical outcomes.

Once the key indicators have been identified, useful information (data) about existing processes are collected. Most quality improvement tools depend on accurate data. The methods of data collection (except perhaps financial data) are not much different from the methods of formal research. Keep in mind that when one attempts to compare data from one department to another, the comparison is impossible unless the measures are defined in such a way that you are comparing "apples to apples".

Once data are gathered, they are analyzed using the same procedures as those used in formal research projects. These procedures include both descriptive and inferential statistics and graphical illustrations. In benchmarking jargon, this phase is sometimes called "gap analysis" because you are trying to identify any gaps or differences among benchmarking participants.

Once the gap analysis is complete and the results are known, individuals typically respond in one of three ways: denial, rationalizing, or learning.

Seldom will the results of a benchmarking project proclaim any department "best of class" across the board. More often, the news is less than uplifting, and perhaps, even discouraging. The natural response from a manager is "These data can't be correct." Unfortunately, they probably are. Facing reality is often the most difficult part of benchmarking.

The second response is rationalization. In the attempt to explain away the gaps identified in the data analysis, managers usually try to find errors in the data or methods used to collect the data. If an error can be uncovered, then they think business can continue as usual. The cry is often "We're unique!" and the implication is that just because a methodology worked in Hospital A does not mean that it will work for us because we are different.

Learning is the third response. Learning comes from accepting reality and taking actions to change it. Corrective action begins with accepting that the benchmarking data are probably correct, asking the right questions, and realizing that lessons can be learned.

The overriding objective of benchmarking is to identify and learn about best practices. But unless we implement the best practices, we have engaged in nothing more than an intellectual exercise with little value.

SUMMARY

Outcomes research seeks to understand the end results of particular health care interventions. End results include effects that people experience and care about, such as change in ability to function. In particular,

for individuals with chronic conditions (where cure is not always possible) outcome results include quality of life as well as mortality. By linking the care people get to the outcomes they experience, outcomes research has become the key to developing better ways to monitor and improve the quality of care.

The methods of outcomes research vary significantly from those of traditional clinical research. Health care workers need to be familiar with these methods be educated consumers of (and to participate in) future studies.

QUESTIONS

Definitions

Explain the meaning of the following terms:

- Disease management
- Continuous quality improvement
- Outcomes research
- Evidence-based medicine
- Benchmarking

True or False

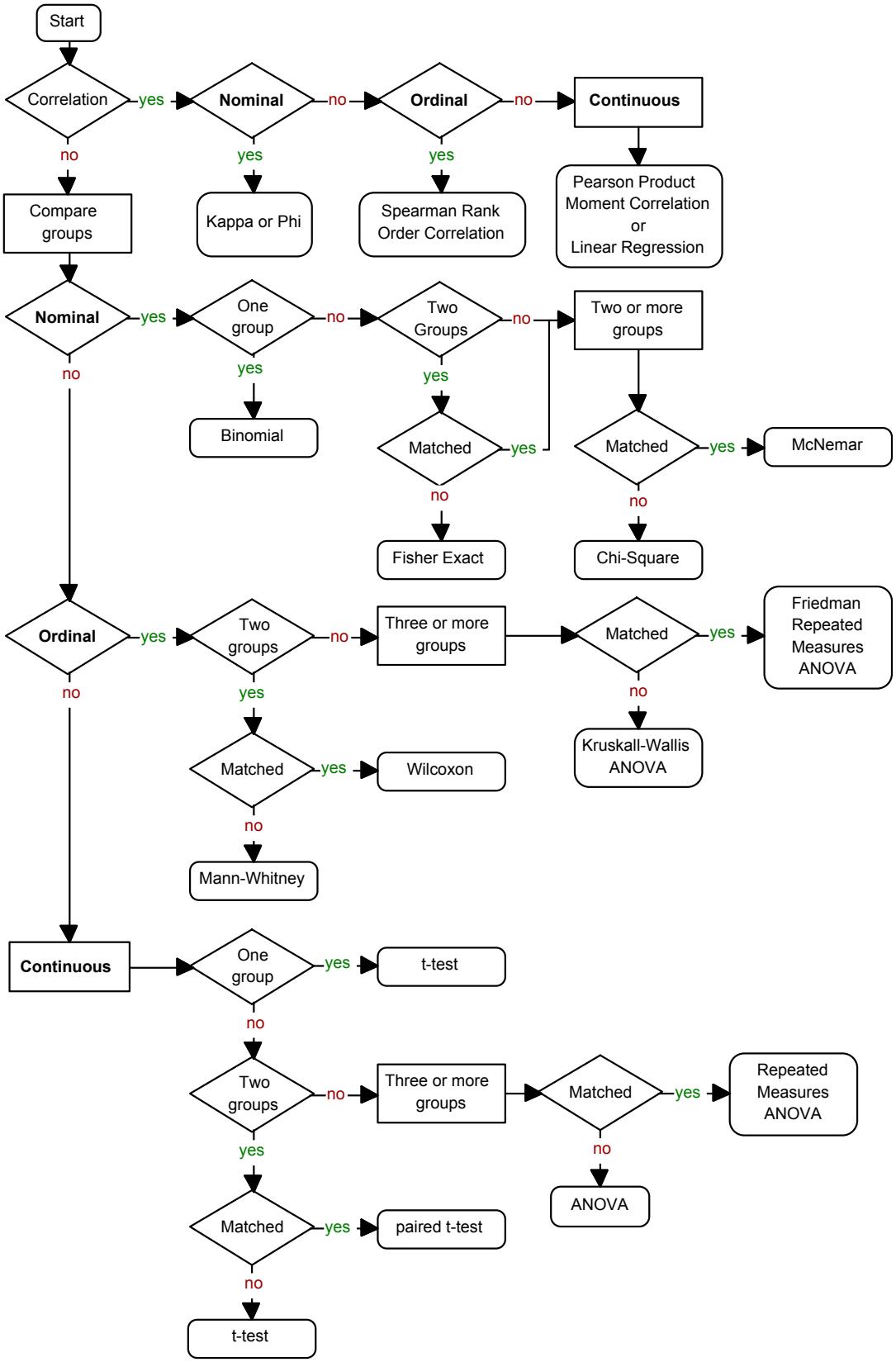
1. Qualitative research uses classical experimental designs whereas quantitative research relies on textual data in the form of observational field notes.
2. Outcomes research is centered on patients and communities while traditional clinical research is disease-centered.
3. Two types of *clinical* measures used in outcomes research are patient symptoms and quality of life.
4. One of the main challenges for outcomes studies is to move from description and methods development to problem solving and quality improvement.
5. *Efficacy* studies attempt to answer the question "Does the intervention work in a tertiary care setting under controlled conditions" while *effectiveness* studies attempt to answer the question " Does the intervention work in clinical practice settings."

Multiple Choice

1. An economic evaluation that is applied when two or more interventions are compared on the same outcomes and the outcomes have similar effectiveness is:
    a. Cost identification.
    b. Cost minimization.
    c. Cost effectiveness.
    d. Cost utility.

2. An economic analysis used when the same outcomes are measured but effectiveness differs is:
    a. Cost identification.
    b. Cost minimization.
    c. Cost effectiveness.
    d. Cost utility.
3. The main value of benchmarking is that;
    a. It is a practical alternative when there is not enough time or money for a scientific study.
    b. It is better than continuous quality improvement.
    c. No patient data are needed.
    d. Many hospitals can collaborate.
4. A benchmarking indicator that establishes a relationship between two measures such as worked hours per unit of service is called a:
    a. Process indicator
    b. Ratio indicator
    c. Outcome indicator
5. Common responses of managers confronted with benchmarking results include all but:
    a. Arguing that the data are incorrect.
    b. Attempting to explain away results by asserting that their situation is unique.
    c. Learning from the experience of others.
    d. Insisting on performing a gap analysis.

Appendix A. Statistics Selector



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