

A Research Agenda for Outcomes Research

Stephen C. Crane, PhD, MPH

ABSTRACT

This paper reports on the results of a Congressionally mandated conference that was held last April to develop a research agenda for outcomes research for the Agency for Health Care Policy and Research (AHCPR) and for Congress. The conference was conducted by the Foundation for Health Services Research (FHSR) and the Alpha Center, with funding from AHCPR. The meeting involved about 200 individuals from around the country representing both users and producers of outcomes and health services research information.

The purpose of this report is to contribute some of the observations and recommendations from this conference to the development of an agenda for outcomes research in nursing.

The paper begins with three important distinguishing characteristics of outcomes research: (1) a focus on **conditions**, and the **alternative treatments** for addressing conditions, rather than on more narrowly defined individual treatments; (2) a concern not just with clinical or physiological outcomes, but with measures of **health-related quality of life**, including physical and emotional functioning, general perceptions of health and well-being, and satisfaction with the process of care; and (3) a recognition of the fact

that the effectiveness and outcome of any treatment process is a function as much of **non-clinical** factors, such as the socio-demographic characteristics of the patient, the organization of the delivery of care, the financial incentives facing both patients and providers, as it is of clinical factors, thus necessitating a **multi-disciplinary approach** to the conduct of outcomes research.

The paper then presents the recommendations of the April conference in seven major areas:

1. The scope of outcomes research efforts;
2. Issues of measurement;
3. Issues of research design and methods;
4. Issues of data;
5. Factors determining the effectiveness of treatments and the outcomes of care;
6. The potential sites for outcomes research; and finally
7. Issues relating to the dissemination and implementation of the findings from outcomes research.

The paper also identifies five major obstacles in the conduct of outcomes research that should be addressed by an agenda for outcomes research in nursing:

1. The **time delay between intervention and outcome**, which makes attribution of cause and effect difficult to ascertain;

2. The need to take account of **non-medical patient characteristics**, such as health insurance coverage, income, and educational level, that are important determinants of the effectiveness and outcomes of health care;

3. The **potential for multiple providers and multiple therapies** in any given treatment episode, which makes disaggregation of the impact and effect of any single intervention very difficult;

4. The problem of a **lack of good baseline population measures** of health status and health-related quality of life, which makes analysis of treatment effects and outcomes for individual patients difficult to assess;

5. The **autonomy of the patient in the treatment process**, which can further confound and confuse the already difficult task of assessing treatment effectiveness and outcomes.

The paper concludes with the recommendation to study patients as individuals and people, and not simply as organs, diseases, conditions, or disabilities.

Introduction

The assignment that I was given for this session was to talk about the effect of the interventions of different disciplines, or health professions, on the outcomes of care, or what have we learned about the conduct of outcomes research in other areas that will contribute to the formulation of a better focused and more targeted agenda for outcomes research in nursing. To this end, I would like to report briefly on the results of a Congressionally-mandated conference, which was held last April, that developed a research agenda for outcomes research for the Agency for Health Care Policy and Research (AHCPR) and the Congress. This conference was conducted by the Foundation for

Health Services Research (FHSR) and the Alpha Center, with funding from AHCPR. The meeting involved about 200 individuals from around the country representing both users (e.g., clinicians, public and private policymakers, payers, etc.) and producers of outcomes and health services research information.¹ The meeting provided an excellent summary of what is known about the conduct of outcomes research as of the present moment, and therefore should be of use to this meeting as it develops an agenda for outcomes research in nursing.

I will highlight just a few of the major observations and recommendations of conference participants in seven major areas:

1. The scope of outcomes research efforts;
2. Issues of measurement;
3. Issues of research design and methods;
4. Issues of data;
5. Factors determining the effectiveness of treatments and the outcomes of care;
6. The potential sites for outcomes research; and finally
7. Issues relating to the dissemination and implementation of the findings from outcomes research.

In the interest of time, I will be relatively brief in my listing of points in each area, and will leave for later discussion any of the points I raise. Before beginning, however, I would like to say a few words about the overall intent and focus of outcomes research.

Intent and focus of outcomes research

A good summary definition of outcomes research is provided in a recent IOM report on conflict of interest in PORTs (Patient Outcomes Research Teams). Let me read to you what it says:

Outcomes research examines the treatment of clinical conditions rather than individual procedures or treatments. It is the systematic assessment of clinical practice, encompassing both outcomes that are relevant to patients — mortality, morbidity, complications, symptom reduction, and functional status improvement — as well as physiologic or biologic indicators; it involves all reasonably held theories and alternative clinical practices.²

This is a useful definition because it emphasizes two points that were given substantial emphasis in the April conference:

1. First, the focus of outcomes research is on **conditions**, and the **alternative treatments** for addressing conditions, rather than on more narrowly defined individual treatments. In effect, outcomes research asks the question “What works to achieve the best results in terms of amelioration of a condition,” rather than the more focused question of “How well does a particular treatment work?”

2. Second, outcomes research is involved not just with clinical or physiological outcomes, but with measures of **health-related quality of life**, including physical and emotional functioning, general perceptions of health and well-being, and satisfaction with the process of care. I will say more about this in a moment.

The April outcomes conference, however, also emphasized a third very important point that is relevant to the proceedings of this conference, which is:

That the effectiveness and outcome of any treatment process is a function as much of **non-clinical** factors, such as the socio-demographic characteristics of the patient, the organization of the delivery of care, the financial incentives facing both patients and providers, as it is of the actual clinical treatment provided.

This is a critical point that bears emphasis as this conference begins to develop an agenda for outcomes research in nursing. This fact makes it abundantly clear that an **inter- and multi-disciplinary effort** involving not only the clinical sciences, but the social, behavioral and managerial sciences as well, is also necessary. It also suggests that a broader definition of outcomes research may be more helpful, such as the following:

Outcomes research is any inquiry that is designed to measure — and ultimately improve — the outcome of medical treatment and, thereby, the health status of individuals and communities.

This definition would encompass efficacy, effectiveness as well as appropriateness research. This definition is implicit in the recommendations of the April conference that I now want to summarize for you.

Let me turn my attention, now, to some of the more detailed recommendations and comments from the conference.

Scope of Outcomes Research

With respect to the scope of outcomes research, the conference produced three major categories of recommendation.

First, the scope of outcomes research, particularly the research currently being conducted in PORTs, should be broadened to include:

1. Chronic conditions and issues of prevention, as well as acute conditions. For instance, hypertension, congestive heart failure, diabetes, and arthritis, as well as early screening for problems such as breast cancer and cervical cancer, and preventive programs such as smoking cessation and weight control, should be evaluated. Nursing should be particularly concerned with these issues.

2. This broadening should also include issues of mental health treatment, and

3. should take account of a wider range of age and cultural groups than is now the case. One group singled out for particular attention by the April conference was **children**, but other groups also mentioned were: blacks, hispanics, the very old, the institutionalized, rural populations; and the economically disadvantaged.

Second, greater emphasis should be given to identifying procedures that are ineffective, or perhaps too costly to use, as well as searching out and reporting on procedures that are deemed to be “cost effective.”

Finally, a recommendation was made that more family-oriented health outcomes should be studied. I think that this recommendation has special relevance for a nursing-oriented agenda for outcomes research.

Issues of measurement

With respect to issues of measurement, there were five major categories of recommendation.

1. First, there is a need to continue to improve measures of both **health status** and **health-related quality of life**. **Health status** measures include indices of clinical, biological and physiological status, such as gross measures of morbidity and mortality, and more specific measures such as blood pressure, blood sugar, hemoglobin, temperature, etc.

Health-related quality of life measures include:

- physical functioning (for instance, Activities of Daily Living [ADLs], Instrumental Activities of Daily Living [IADL], mobility, etc.)
- emotional and psychological functioning and well-being
- social functioning and support
- role functioning
- general health perceptions
- pain
- vitality (energy/fatigue)
- cognitive functioning

2. Second, and as a corollary to the first point, there is a need to continue investments in the development of measures of consumer preferences for different outcomes and treatments. a) For instance, we need to know more about the natural history and course of events for particular diseases and conditions over time. b) We also need to know more about the health status and health-related quality of life levels

in broad population groups to improve the basis for analysis of the outcomes and effectiveness of individual treatments.

3. Third, we need to improve our ability to define episodes of care during which effectiveness and outcomes are to be measured.

This is a difficult but most fundamental task, because there is often considerable uncertainty over when an “illness” or “condition” starts or ends, or over what specific period of an illness or condition, measurements of the effectiveness and outcomes of care should be made.

4. Fourth, we need to develop more sophisticated measures of the factors affecting outcomes and effectiveness in three particular areas: (1) those that relate to the socio-demographic and economic characteristics of patients and providers; (2) those that relate to the organizational characteristics of different delivery systems; and (3) those that relate to the role and influence of co-morbidity factors, such as the present of other medical problems, the mental health status of a patient, history of substance abuse, etc.

- Wherever possible, measures should be used that reflect the “full scale of effect” of a variable influencing effectiveness of treatments and outcomes of health care. Simple dichotomous measures, such as yes/no, 0/1, etc., often do not provide sufficient precision to give a good indication of the actual effect of different variables.

- Also, researchers need to be alert to the possibility of “threshold effects,” rather than simply presuming more simple models of continuous, linear effects.

5. Fifth, there is a need to separate **generic** from **disease-specific** measures of health status and health-related quality of life to improve measurement of each, and to promote the appropriate use of each measure in outcomes studies.

6. Sixth, we need to develop more precise measurements of the differences between and changes in health-related quality of life measures with respect to:

- the socio-demographic characteristics of patients (for instance, age, income, etc.);
- the geographic location of patients;
- the ethnic and cultural characteristics of patients; and, most importantly
- the severity of illness.

7. Finally, there is a need to develop better information on how measures of health status and health-related quality of life vary over time. For instance:

- What are the optimal points to measure health status and health-related quality of life?
- What are the trajectories of health and illness, and how are “blips” in illness (or health) to be captured and interpreted meaningfully?
- How can life cycle effects be taken into account? Specifically, how do people’s values and preferences over the course of different stages of their life and illness systematically affect or bias their response to health status questions?

Issues of research design and methods

With respect to issues of research design and methods, conference participants identified four areas for further work:

1. First, there is a need to find ways to increase the generalizability, or applicability, of the results of outcomes and effectiveness research. For instance, is there a way to redesign the research currently being done by PORTs to address additional population groups beyond the elderly? How can we get the most mileage from outcomes research to be undertaken in the future?

2. Second, more studies of the health and functional status of populations should be conducted to complement the “micro-level” clinical studies of individual patients. These studies would include both populations enrolled and not enrolled in clinical test protocols. Such studies would improve the understanding of how health status and health-related quality of life measures vary across the population at large.

Some participants went so far as to suggest the creation of a national profile of the general health and functional status of the population, analogous to the general vital statistics profiles currently available.

3. Third, there is a need to develop ways of handling the significant problem of the time lag that occurs between the intervention or provision of care, and the ultimate end result of interest. This is one of the most troubling methodological problems in outcomes research today, and one that has yet to be resolved successfully in many instances.

4. Finally, we need to improve our capabilities for synthesizing existing information through meta-analyses and other techniques for information and data summarization and presentation. Achieving this objective will help to stretch our limited research dollars, and make most effective use of the work already undertaken and completed.

Issues related to data

The fourth major area dealt with issues related to data.

1. Conference participants indicated that there was a substantial need to **incorporate outcomes measures into existing and new health services research data sets** to enable greater analysis of outcomes and effectiveness in the future, as well as

2. to increase opportunities for **data sharing** among existing research projects and research investigators. In addition to these two broad recommendations, four more specific recommendations were also made:

1. First, there is a need to identify **standard data elements and forms** to promote consistency of data collection across studies. The lack of consistent definitions and measures is perhaps the single most important factor limiting the combination and use of existing outcomes studies.

2. Second, we need to develop and use **a common patient identifier** not only to allow health records from different sources or time periods to be linked together more easily, but also to link data from education, welfare, employment, and other social service systems to provide a more complete profile of a

patient's situation and characteristics. This task is less difficult in an integrated system of care, like a Kaiser or Harvard Community Health Plan, but unfortunately only a small fraction of the population get their care from such systems.

3. Third, we need to develop and implement a common scheme for **labelling health care events**, such as the reason for a provider visit, to provide for greater continuity across research studies, and to promote greater comparability among research findings.

4. Finally, we need to create appropriate **longitudinal data sets over adequate periods of time** to assess the long term as well as short term effects of interventions. On this point, conference participants agreed on the need to educate public and private funders about the importance of longitudinal data and the need to invest in its collection and dissemination, even given its cost.

Factors determining the effectiveness of treatments and the outcomes of care

The fifth major area of recommendations dealt with the factors that contribute to or determine the effectiveness of alternative treatments and the outcomes of care.

1. Conference participants emphasized the importance of analyzing the influence of socioeconomic and demographic characteristics of patients and providers on effectiveness of treatments and outcomes care. For instance among many possible factors, researchers should consider the impact of income and health insurance status on the timeliness of seek-

ing care. The impact of cultural factors, such as religion and tradition on compliance with treatment protocols and drug regimens, should also be assessed.

2. Other equally important factors determining outcomes that need to be considered include: the different ways in which health services are organized and delivered, the personal attributes and characteristics of care givers, the financial incentives facing both providers and patients, and the availability of information about alternative treatments and their consequences, among others.

In general, there is a need to recognize and appreciate the truly complex nature of the health care system, including such issues as:

- How the interactions among the different components of the delivery system affect the effectiveness of treatments and the outcomes of care;
- The barriers that exist within the delivery system that impede the efficient and effective use of the system;
- The nature of internal power structures and the impact of external environmental factors, such as the economy and public and private regulation, on the function and operation of health delivery systems.

3. Third, it is necessary to critically assess the contribution of all types of health care personnel to the treatment process, no matter whether the principal focus of study is on nursing, medicine, or some other health profession.

4. Fourth, researchers must take greater account of the role of consumer preferences in the treatment process and, as already stated, must develop better measures of these preferences. The nature of

the interaction between patients and providers may also be an important determinant of the effectiveness and outcomes of health care.

5. Finally, research needs to be undertaken to identify the differences in effectiveness that may exist between primary and specialty-type care in the treatment of conditions at early, mid and late stages of disease or illness development.

Issues related to the site of care

With respect to the potential sites for outcomes research, conference participants made several recommendations.

1. Most importantly, participants recommended that more research be done outside academic medical care settings. For many reasons, academic medical centers have been the focus of outcomes research, but these centers are not typical of the care provided to most people. A broader array of sites needs to be used. For instance:

a) The concept of the “firm” needs to be further developed. As used in a research sense, firms represent clusters of patients, within a larger group practice, who have been assigned randomly to a panel of health care providers. Patients are then monitored both within and across firms to identify variations in practice patterns and differences in effectiveness and outcomes of health care. This type of research offers a rich source of comparative data and information.

b) “Practice networks” link otherwise independent providers together again for the purpose of comparative analysis of patterns of treatment and effectiveness of care provided. One major advantage in

using networks, or other consortia of health care providers, is that sample sizes for analyses are often increased, which improves the power of the analyses conducted.

c) Finally, managed care settings represent ideal research laboratories, given that they are “closed health care systems.” Because most all sources of care in these systems either are known or can be tracked, more thorough analysis can be made of the impact of both clinical and non-clinical factors on treatment effectiveness and care outcomes.

Issues related to dissemination and implementation of the findings from outcomes research

Issues of the dissemination and implementation of the findings of outcomes research are usually not considered to be a part of mainstream research activities, but these are vitally important and crucial parts of the whole outcomes effort. Dissemination is also an area where we know relatively little, but where investments in research can return relatively large dividends. For instance:

1. We need to explore the theory and practice of dissemination of information as it relates to different audiences, including: various types of health care providers; patients; payers; policymakers; and health services managers. With respect to these audiences, we need to ask the question: “What techniques work for which groups and how well?” Dissemination techniques that need to be considered include:

- guidelines and practice standards;
- research reports published in professional journals;
- news articles presented in the lay media; and

- formal and informal professional communications.

2. Second, we need to examine different models for how consumer and provider behavior can most effectively and efficiently be changed. Some of the methods that need to be considered include:

- feedback on performance;
- education;
- peer pressure;
- administrative regulation;
- financial and non-financial incentives;
- financial and non-financial penalties.

Most importantly, we need to undertake basic research into the methods of persuasion that can most effectively influence the use of research results in both clinical and policy settings.

3. Third, we need to develop mechanisms for resolving conflicts that may arise between different guidelines or practice standards that may be promulgated for the same condition. It is inevitable that such conflicts will emerge, and we need to have some way, in advance, for resolving these disagreements.

4. Fourth, we also need to create a strategy for monitoring trends in practice patterns and for identifying changes in these patterns over time that may be the result of outcomes research findings.

5. Finally, we need to determine how feedback of information and information management systems can positively (and negatively) affect patient outcomes.

Special difficulties and obstacles in conducting outcomes research

Before concluding, I would like to highlight five potential problems in the conduct of outcomes research that have already been mentioned, but which are deserving of emphasis based on the April conference recommendations. These are:

1. The **time delay between intervention and outcome**, which makes attribution of cause and effect (as in the case of treatment for multiple chronic conditions) difficult to ascertain with any degree of certainty;

This problem is particularly important in studies of the effectiveness of health promotion activities for conditions that may not reveal themselves as problems for years.

2. The effect of **non-medical patient characteristics**, such as health insurance coverage, income, and educational level, that influence the outcomes of health treatments;

Although these characteristics are difficult to measure, they often confound the results of studies when they are not adequately controlled.

3. The **potential for multiple providers and multiple therapies** in any given treatment episode, which can make disaggregation of the impact and effect of any single intervention very difficult;

4. The problem of a **lack of good baseline population measures** of health status and health-related quality of life, which makes analysis of treatment effects and outcomes for individual patients difficult to assess;

5. And finally, the **autonomy of the patient in the treatment process**, which can further confound and confuse the already difficult task of assessing treatment effectiveness and outcomes.

Conclusion

It is perhaps appropriate to conclude with a recommendation that had importance for many of the April conference participants, and I know will have great relevance for the field of nursing as well. That recommendation is:

To study patients as individuals and people, and not simply as organs, diseases, conditions, or disabilities.

One of the significant contributions of the growing field of functional health status within outcomes research is to move us towards this goal, and it remains an important objective for you to meet as you develop an outcomes research agenda for nursing.

Thank you.

² Molla Donaldson and Alexander Capron, eds. Patient Outcomes Research Teams: Managing Conflict of Interest (Washington: National Academy Press, 1991), p. 9.