

**Research Training in Clinical Effectiveness:
Replacing "In My Experience..."
with Rigorous Clinical Investigation**

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Several-fold variations from one geographic area to another continue to be reported in the frequency with which people are subjected to the surgical removal of uterus, prostate, and gall bladder,¹ and to coronary bypass surgery,² to mention just a few expensive and risk-laden procedures. Sometimes doctors recommend them because there is inadequate evidence not to proceed. Perhaps even more frequently a physician justifies a course of action not on scientific grounds, but rather with a statement that begins, "In my experience..."

Gaps in our knowledge, however, preclude laying out conclusive indications for many of today's most prevalent practices.^{3,4} While it is highly likely that variations in the rates of procedures generally reflect too much or too many in some areas rather than too little or too few in others, this is surely not invariably the case. Although the need to address such gaps in knowledge is increasingly recognized,⁵ relatively

few academic physicians are trained to carry out relevant research.

In this era of extraordinary strides in scientific medicine, our inability to evaluate scientifically the effectiveness of clinical procedures seems paradoxical. More and more, physicians trained in biological science are in leadership positions in academic medicine and in clinical practice. Such training, however, does not generally equip them to evaluate clinical effectiveness scientifically. For example, doctors trained in cardiac surgery and in cell biology may be well prepared both to care for patients in need of heart surgery and to study the structure and function of myosin. But such a background alone has not prepared them to compare critically coronary bypass surgery with medical measures for the management of the patient with coronary artery disease. Similarly, an oncologist may be sophisticated in both administering mitomycin and understanding its mode of action, but unprepared to weigh the benefits of a brief remission of widespread cancer against the diminished quality of life caused by the drug.

The need to avoid unwarranted risks to patients has always been a powerful incentive for systematic evaluation of clinical practices. More recently, society's concern for conserving health care resources has presented a second reason by focusing more attention on the issue of unnecessary costs. Now, a third reason exists; as medical capabilities and needs increase and

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resources become more constrained, the medical profession is increasingly confronted with questions of how to choose among worthy interventions when we cannot do them all. Methodology for making such choices is urgently needed, as are physicians and other health professionals who are trained to develop and use that methodology.

In recent years, several programs have been directed at addressing the need for physicians trained in the quantitative analytic sciences, and thereby equipped with appropriate skills for their clinical research, as well as their clinical care. The Clinical Scholars Program,⁶ which was begun by the Carnegie Corporation and the Commonwealth Fund in 1969, and which has been funded by the Robert Wood Johnson Foundation since 1973, now supports six programs nationwide. The University of Michigan School of Public Health recently reported a program in Clinical Research Design and Statistical Analyses⁷ leading to a Master of Science degree. In addition, fellowship programs in general internal medicine and primary care have been funded by the National Institutes of Health and by the Health Resources Services Administration. Such programs have usually had one of two types of curricula: either one that is largely free-standing and taught specifically to fellows independent of any degree granting program; or courses taught by the faculty at an affiliated school of public health.

At Harvard Medical School, postdoctoral fellowship training in the quantitative sciences for general internists formally began in 1979 with one of the grants made to several medical schools by the Henry J. Kaiser Family Foundation. The program's fellows, who were principally from three major Harvard-affiliated teaching hospitals (Beth Israel, Brigham and Women's, and Massachusetts General Hospitals) and occasionally from other Harvard-affiliated institutions (Cambridge Hospital, New England Deaconess Hospital, and the Harvard Community Health Plan), received additional support from the Boston Foundation, the Japan Shipbuilding Foundation, and from the hospitals and their medical services.

The program featured a not-for-credit curriculum in research methods that served as the general template for the more comprehensive curriculum of the current Clinical Effectiveness Program. After the Kaiser Family Foundation ceased funding

fellowship training programs, the general internal medicine component has been subsequently supported by two sequential grants from the Health Resources Services Administration and by an institutional National Research Services Award from the National Institutes of Health. Although a few fellows from the medical subspecialties participated in this program prior to 1986, most were from general internal medicine. Of more than 40 enrollees in this program prior to 1986, all but two are currently in academic positions.

The Clinical Effectiveness Program was created in 1986, with support from the W.K. Kellogg Foundation and the Klingenstein Fund. It was designed to train physicians, initially from subspecialties of internal medicine (not general internists), and from other medical specialties as well, in the quantitative analytic sciences that are necessary to evaluate the effectiveness of clinical practices. We applied the Kellogg Foundation funds to physicians other than general internists because of our belief that the skills of effectiveness research are broadly applicable across all clinical fields.

The program extends over two years and begins on July 1 of Year One with an intensive summer seminar which includes four courses: epidemiology (5 credits), biostatistics (5 credits), health services research (2.5 credits), and decision sciences (2.5 credits). These courses are tailored specifically for fellows in the Program. The research program begins in September of Year One and continues through June of Year Two. It is customarily carried out under the direction of both a clinical mentor in the fellow's specialty and an expert in the quantitative analytic sciences from the Clinical Effectiveness Program's core faculty or from the Harvard School of Public Health. During the 22 months of their research most fellows take supplemental course work in epidemiology, biostatistics, and/or health policy and management with the option of obtaining a master's level degree at the Harvard School of Public Health.

Selection of Fellows

Fellows have represented a wide range of clinical fields (Table 1). All fellows from the General Internal Medicine Program at Harvard who are supported by the National Research Services Award and the Health Resources Services

Table 1. Clinical Specialties of Fellows in the First 4 Cohorts of the Clinical Effectiveness Program.

Specialty	1987	1988	1989	1990
General Internal Medicine	7	6	4	12
Cardiology	3	2	7	7
Geriatrics	2	3	1	2
Nursing	1	1	—	—
Infectious Diseases	—	2	3	1
Oncology	—	1	2	—
Rheumatology	—	1	—	1
Hematology	—	1	—	—
Emergency Medicine	—	1	—	—
Gastroenterology	—	—	—	1
Nephrology	—	—	2	—
Pediatrics	—	—	2	2
Obstetrics and Gynecology	—	—	1	1
Orthopedic Surgery	—	—	1	1
Total	13	18	23	28

Administration grants are enrolled in the Clinical Effectiveness Program. In addition, general internal medicine fellows supported through the Veterans Administration or by hospital funds may apply with the sponsorship of their divisions. Applicants from medical subspecialties and from other departments must be sponsored by the chief of their clinical subspecialty division or department. A prerequisite for all fellows is commitment to an academic career in which their research would utilize the skills offered in the Program.

General internal medicine fellows begin the curriculum during the summer of their first fellowship year. Fellows from subspecialties of internal medicine generally complete a clinical year in their subspecialty and then enroll in the curriculum at the beginning of their subsequent two-year research block. Fellows from other areas of medicine usually enroll after completion of their first year of clinical subspecialty fellowship training.

Funding

Fellows in general internal medicine typically receive either full federal stipend and tuition support, if they are funded by the National Research Services Award, or partial federal stipend and tuition support supplemented by hospital support, if they are funded through the Health Resources Services Administration award. Other fellows generally receive full stipend support from their clinical division or department. Their

tuition support may be covered by their division's National Institutes of Health training grant, by the sponsoring division or department, or in part or in full by the core grant to the Clinical Effectiveness Program.

In academic year 1989-90, the sponsoring divisions and departments, including two federal grants for general internal medicine fellowships, contributed in excess of \$750,000 for the 23 first-year fellows and well over \$500,000 for the 18 second-year fellows. The Clinical Effectiveness Program's core budget for this year from the Kellogg Foundation was about \$225,000, which was used for faculty and staff salaries, fellows' stipends, and tuition support.

Core Curriculum

The Program's didactic curriculum emphasizes the numerical sciences. During the first summer, the participants receive intensive exposure to special courses in biostatistics, epidemiology, health services research, and decision sciences. Each of these courses is taught in seminar format by members of the Program faculty at the Harvard School of Public Health and the Harvard Medical School. By engaging the participants in a series of projects and workshops, they provide a practical basis for developing skills important for clinical research. The summer curriculum lasts eight weeks and is open only to fellows in the Clinical Effectiveness Program. Fellows spend five hours in the classroom for five days each week. By

their own estimation, they spend an average of 25 to 30 hours per week on out-of-class assignments.

Biostatistics: Concentrating on pertinent examples that are chosen from the clinical literature, the biostatistics course covers the basic quantitative techniques that are useful for clinical research. In addition, a series of "overview" lectures are given to describe more advanced techniques that are not usually covered in introductory courses of biostatistics. After completing this course, the participants can use basic quantitative techniques to analyze their own data and to interact actively with biostatisticians in the analysis stage of research projects. To build experience with clinical data sets, the course ends with a series of workshops using micro-computers and appropriate software packages to analyze data from actual clinical research projects.

Epidemiology: Emphasizing clinical applications, the course in epidemiology covers the basic epidemiologic principles for planning and carrying out clinical research projects. Among the topics covered are options for the design and analysis of studies, questionnaire design, screening, measurement, bias, and confounding. By evaluating a series of published articles that use different study designs, the fellows consolidate their understanding of the basic epidemiologic principles of clinical research. In addition, participants develop a research plan for addressing a clinical problem of their choice, and describe the principal goal of the study, the basic study design, the proposed data-gathering instruments and analytic methods, and the anticipated problems and limitations. The formal presentation of the proposal to the class allows each participant to receive useful comments and criticisms. This course is designed to help the participants not only to improve their skills to evaluate the medical literature but, more importantly, to design and plan their own clinical investigations.

Decision Sciences: The course in Decision Sciences is designed to introduce the student to the methods and the growing range of applications of decision analysis, cost-effectiveness analysis, and benefit-cost analysis in health care technology assessment and medical decision-making. Using a standard computer software package, participants obtain practical experience in preparing the structure and content of a clinical decision analysis. Upon

completion of this course, the participant should be able to implement these skills to address research questions of interest.

Health Services Research: Covering the major issues in health care policy and methods for health services research, this course is designed to provide the tools to perform investigations in the area of health services research. Among the topics that are covered are a description of the hospital payment system, health maintenance organizations and other prepaid variants, issues in caring for the uninsured, survey methods, and health status measurements. Participants are required to prepare a grant application that addresses a research question of interest. Upon completion of this course, participants should have obtained an understanding of the major issues in health policy, have gained an appreciation for the methodologic issues and tools for doing research on these problems, and have acquired an appreciation of how one might develop a research project in this area.

In addition to these didactic courses, a weekly luncheon meeting is held during which distinguished investigators from the medical community discuss their experiences. These informal meetings are designed to give the participants a useful perspective on various aspects of clinical research.

Thus, the Clinical Effectiveness Program combines the free-standing curricular approach of some fellowship programs with the quantitative analytic orientation of formal course work at a school of public health. The courses, however, are tailored for the clinical setting, as distinct from the population orientation of traditional coursework in schools of public health. Because the summer courses are taught in a seminar format and exclusively to Clinical Effectiveness Fellows, they can proceed at a very rapid pace (although not so rapid, perhaps, as the pace of the house officers' schedule the fellows have all recently left). The summer curriculum allows fellows to gain core skills before they begin their research projects in the fall.

Subsequent Coursework

After completing the core curriculum, most participants enroll in additional courses at the School of Public Health, so as to fulfill the requirements for a Master of Science degree in Biostatistics, Epidemiology, or Health Policy

and Management (Table 2). Fellows are encouraged to register in a degree program, for the required advanced coursework provides important background for their research. Credits towards a degree can also be earned for the supervised research (see below), which is the cornerstone of the program. Of the 29 physicians in the summer programs of 1987 and 1988, 19 (66%) have received a Master's degree, and two others are continuing to take courses part-time.

Types of Clinical Investigation

The fellows have embarked on a wide range of investigation, both in clinical topics and methodology. For example, some have studied ethical issues related to "Do Not Resuscitate" orders, as well as clinical outcomes in elderly patients who have been resuscitated. Fellows have devised new methodology, such as a hand diagram to evaluate patients with suspected carpal tunnel syndrome. Some have evaluated the utility of new technologies, such as ambulatory monitoring for patients with cardiac ischemia to determine whether complications of noncardiac vascular surgery can be predicted. Others have analyzed the cost-effectiveness of various approaches to patients with pulmonary symptoms and known or suspected AIDS. One has identified prognostic guides in oncology patients with fever and neutropenia. Their studies have ranged from very common problems, such as evaluating the prognostic factors in patients with community-acquired pneumonia, to unusual ones, such as an epidemic of asbestos-related diseases in workers at a cigarette filter manufacturing company. The remarkable diversity of both clinical topics and research methodologies demonstrates the varied interests of the fellows who have enrolled in the Clinical Effectiveness Program, as well as the capacity of the Program to help them develop many kinds of methodologic skills.

Outcome

Thus far, the fellows have rated the program extremely favorably. For example, after completing the summer curriculum, each year's enrollees are asked whether they would sign up for the summer curriculum if they had to make the decision again. To date, all have answered affirmatively, except one who was unsure. The individual summer courses have also received

Table 2. Typical Courses Taken by Clinical Effectiveness Fellows After the Summer Program.

Concentrators in Epidemiology:

Elements of Epidemiologic Research
 Design and Interpretation of Cohort and Case Control Studies
 Fundamentals for the Analysis of Case Control and Cohort Studies
 The Analysis of Rates and Proportions
 or
 Discrete Multivariate Analysis
 Various other courses in Epidemiology to fulfill department requirement. Some examples include:

- Clinical Epidemiology
- Practice of Epidemiology
- Epidemiology of Cardiovascular and Respiratory Disease
- Pharmacoepidemiology

Concentrators in Biostatistics:

The Analysis of Rates and Proportions
 Regression and Analysis of Variance in Experimental Research
 Computing Principles and Methods I
 Various other courses in Biostatistics to fulfill department requirement. Some examples include:

- Principles of Clinical Trials
- Methods of Data Analysis
- Seminar on Clinical Decision Analysis
- Discrete Multivariate Analysis
- Applied Survival Analysis

Concentrators in Health Policy and Management Biostatistics:

- The Analysis of Rates and Proportions
 or
- Regression and Analysis of Variance in Experimental Research

Decision Sciences:

- Decision Analysis for Health and Medical Practices
- Cost-Effectiveness and Cost-Benefit Analysis for Health Program Evaluation

Economics:

- Economic Analysis for Public Health

Management:

- Managing Health Delivery Organizations

very high ratings in quality of teaching and usefulness. For example, on a 1 to 9 scale, with 9 the highest, the Biostatistics and Epidemiology courses have consistently received ratings in the 8.3 to 8.8 range. The fellows also have given high ratings to the overall program in its having prepared them for research (Table 3). The rapid growth from 13 fellows in year 1, to 18 in year 2, 23 in year 3, and 28 in year 4 is further testimony both to the favorable reviews of enrolled fellows and to the feeling

Table 3. Evaluation of the Summer Program.

On a scale of 1 to 10, please rate the program's importance for preparing you for your clinical research:

	1987	1988
Minimum response	5	8
Maximum response	10	10
Mean response	7.9	8.7

of their clinical chiefs that the program represents time and money well spent. Even more impressive than their numbers has been the extraordinary quality of the fellows: they include three former or future chief residents in medicine and two in obstetrics-gynecology at Brigham and Women's Hospital, and former or future chief residents in medicine from Beth Israel Hospital, Massachusetts General Hospital, Columbia-Presbyterian, Memorial Sloan Kettering, University of Minnesota, University of Pittsburgh, University of Vermont, University of Washington, and University of California at San Francisco.

Although our graduates could also have an important impact in governmental or other administrative roles as well as academic careers, the early career choices have been overwhelmingly academic. Of the 29 physicians who enrolled in 1987 and 1988, 23 are in academic positions: 12 at Harvard Medical School and the others at major institutions across the country. Of the other six, five are still in fellowship programs, and one works at the Massachusetts Department of Public Health.

The fellows' early research productivity is also impressive. For example, two have won prizes for their research presentations at national scientific meetings of major academic societies, and six others have already been first authors of articles in the *New England Journal of Medicine*, the *Annals of Internal Medicine*, the *Journal of the American Medical Association*, and *Circulation*.

Our Program in the Context of Some Other Current Programs

The major national program with goals similar to ours is the Robert Wood Johnson Clinical Scholars Program.^{6,8} Although it is not our purpose to analyze their effectiveness in detail, several comparisons are interesting.

The Clinical Scholars Program enrolls about

25 new fellows per year in 6 programs at 7 universities and their affiliated Veterans Administration Hospitals. In most of these programs, all fellows take a required core curriculum to learn quantitative methods. In 4 programs, these curricula are less than about 50 hours. The other 2 programs have curricula of about 130 hours over 4 months and 250 hours over 12 months, respectively, compared to our 180 hour, 8-week summer curriculum. (Shuster AL, Personal Communication) In the programs with more extensive core curricula, other physicians may participate in these sessions. In 5 of the 6 programs, Clinical Scholars can potentially earn a Master's level degree, but recently the Foundation has discouraged pursuit of degrees.

From 1971-1989, there were 524 graduates of the Clinical Scholars Program, including 319 (61%) who are currently in academic medicine and another 26 (5%) who are in government. A majority of graduates (288, 55%) were from internal medicine, and another 157 (30%) were from pediatrics and psychiatry. Graduates have played a major role in studying appropriateness, effectiveness, and related issues that serve as the focus for the new federal Agency for Health Care Policy and Research.

At the University of Michigan the program in Clinical Research Design and Statistical Analyses⁷ also has a required core curriculum. The course work, which is identical for all enrollees, is taught in 18 sessions, each of which lasts 4 days (Thursday through Sunday) and is held every fourth or fifth week. The students include physicians (at both the faculty and fellow level) as well as other medically-oriented persons with and without doctoral level training.

In summary, several different programs have a variety of specific ways to teach many of the same skills for clinical research. A substantial proportion of the graduates of these programs are already in academic positions in which they are having a major impact on this previously neglected and very important field. However, our experience suggests that the nation's needs are not being met fully by existing programs, and that additional similar efforts should be considered by other institutions.

Continuing Challenges

Despite our extremely favorable experience,

several challenges remain. Some subspecialties have been less enthusiastic than others in recognizing the relevance of training in clinical effectiveness. Some fellows are unable to complete their projects in two years, and it is important to be flexible in providing them with continued protection and guidance. Each fellow requires a substantial faculty commitment, for both research mentorship and general guidance. For the general medicine fellows, a detailed curriculum on teaching skills and methods supplements the Clinical Effectiveness Program's curriculum; this additional preparation is vital to the career of academic generalists but may also be pertinent for many subspecialists, who currently do not participate in it. In addition, the Clinical Effectiveness Program is at risk for "falling between the cracks" of the traditional funding priorities of foundations, which usually start pilot programs but do not plan to maintain them in perpetuity, and the National Institutes of Health, with its single subspecialty orientation. It will be important to find stable funding if the Clinical Effectiveness Program and others like it are to thrive in the future.

Concluding Remarks

In a recent survey, over 75% of MDs in full-time, salaried positions in medical schools reported that they spend some portion of their time in research. The median research effort was about 25%.⁹ Nevertheless, the decline in National Institutes of Health funding for physician-researchers has led to increasing concern about the diminishing supply of well-trained clinical investigators.¹⁰⁻¹³ Although this concern has, to date, focused primarily on basic biomedical researchers, there is also a perhaps less widely recognized, but no less acute need for people equipped to carry out patient-oriented research.¹⁴

To address this need, physicians must be trained in clinical epidemiology, medical decision-making, technology assessment, medical outcomes, medical ethics, health services research, and a full range of topics that could be grouped under the broad umbrella of clinical effectiveness. Just as several initiatives have been designed to increase the pool of biomedical bench investigators,¹⁵⁻¹⁸ we believe that programs like our Clinical Effectiveness Program are urgently required to help equip researchers to carry out

important medical investigation. In our opinion, such training, which has been largely restricted to schools of public health and divisions of general internal medicine in relatively few institutions, should be replicated more broadly. Our model is one that appears to be both demonstrating its usefulness and responding to a demand that is widely perceived on the part of young physicians interested in academic careers in clinical departments.

Acknowledgments

The authors are indebted to Eugene Braunwald, MD and Harvey Fineberg, MD, PhD for assistance and guidance in establishing this collaborative program, and to Ms. Annie Lea Shuster of the Robert Wood Foundation and the Directors of the various Clinical Scholars Programs for providing helpful information.

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