The More Things Change...: The Federal Government’s Role In The Evaluative Sciences

NIH and academic medicine can join in partnership to build the evaluative sciences and stabilize the mission of academic medicine.

by John E. Wennberg

ABSTRACT: The unfortunate political history of the Agency for Health Care Policy and Research (AHCPR) illustrates the risks to the agencies attempting to evaluate the common practices of medicine and reform clinical decision making to take account of patients’ preferences. The evaluative sciences have yet to regain the congressional attention they had when Senators George Mitchell and David Durenberger championed their cause. But the fundamental problems remain, and they are getting worse. Sooner or later Congress will need to revisit the debate over where in the federal government the evaluative sciences should find their base, and questions concerning the role of the National Institutes of Health (NIH) will be raised once again, as they were at the time of AHCPR’s founding.

Almost 100 years have passed since Abraham Flexner sparked the revolution that drove out substandard, proprietary medical schools and led to the modern academic medical center (AMC). The prodigious growth of AMCs and the biomedical sciences in the twentieth century is a testament to Flexner’s vision. Yet the academic enterprise has had only limited success in improving the scientific basis for clinical decision making, even within its own walls. Indeed, the patterns of practice among AMCs—as among other institutions—are often idiosyncratic and unscientific, and local medical opinion and local supply of resources are important in determining how medical care is delivered. In short, after nearly 100 years much of medicine remains “empiric.”

Why is this so? One reason is the assumption that medical science progresses in a linear fashion, beginning with biomedical theory and bench research, followed by translational research and, finally, by clinical trials that establish the rules for the “evidenced-based” practice of medicine. Scientific progress sometimes follows this model (for example, the decision rules governing the use of beta-blockers following acute myocardial infarction). But the theories that drive the everyday practice of medicine have eclectic origins. The clinicians themselves invent many such theories, as they seek to sort out and provide remedy for the complex sets of problems patients bring to them. Improving the scientific basis of clinical medicine in such cases requires different strategies and methods than those characteristically employed in biomedical research.

In this broader view of medical innovation,
the evaluative sciences and the biomedical sciences should be understood as complementary strategies for improving the scientific basis of medical practice. But in federal science policy and in academe, the tendency has been to neglect the evaluative sciences.

Brad Gray and his colleagues provide an informative history of a rare exception: the efforts in the 1990s to establish the Agency for Health Care Policy and Research (AHCPR) as the “home base” in the federal government for the evaluative sciences. As they tell it, Congress became convinced that improving the scientific basis of clinical decision making through outcomes research would result in better quality and possibly lower costs. Enthusiasm for this agenda became the justification for creation of a new agency, and Patient Outcomes Research Teams (PORTs) became the major vehicle for its implementation. PORTs were asked to extend the evaluation process to include all relevant treatments for a specific condition. Most concentrated on a single condition such as benign prostatic hyperplasia (where the treatment options included surgery, medical management, or simply watchful waiting), and low-back pain and stable angina (conditions for which major discretionary surgery was one treatment option).

- **Reducing scientific uncertainty.** The typical PORT was interdisciplinary: It included clinicians, epidemiologists, statisticians, cognitive psychologists, decision analysts, medical ethicists, and experts in informatics, among others. PORTs were organized with an emphasis on the long term. They were responsible for continuous monitoring of changes in technologies and medical theories for the given condition and for undertaking iterative assessments, which (according to plan but never implemented) would include clinical trials when necessary.

  The PORTs’ methods and strategies included (1) focus groups with practicing physicians to develop a detailed understanding of why practice styles differed and identify competing clinical hypotheses concerning the efficacy and value of treatment options; (2) focus groups and structured interviews with patients to learn about their concerns and to obtain a list of outcomes that are important to them; (3) construction of standardized measures of patients’ preferences and relevant outcomes (as identified by patients as well as physicians); (4) structured review of the literature to create an “evidence-based” assessment of what was known and not known about outcome probabilities, depending on the patient subgroup and treatment chosen; (5) use of large databases to obtain estimates for probabilities for outcomes not otherwise available; (6) cohort studies among patients recruited from “everyday practice” to learn more about who was being treated and to fill in gaps in the probability estimates for outcomes; (7) testing of theory using decision models that incorporate patient-relevant outcomes and examine the sensitivity of choice of treatments to patients’ values; and (8) organization of clinical trial networks to test promising new technologies to assure that medical science keeps up with innovation. The application of these methods and strategies proved useful in reducing scientific uncertainty concerning correct theory and provided a more accurate list of relevant outcomes and better estimates for outcome probabilities than were available in the literature.

- **Role of patients’ preferences.** Scientific uncertainty, however, was not the only source of unwarranted variations in medical practice. For many conditions that the PORTs studied, rational choice among treatment options involved important trade-offs that should depend on the patient’s own preference. For example, in the treatment of an enlarged prostate, patients who underwent surgery were much more likely to experience improvement in urinary tract symptoms but were at risk for changes in sexual functioning. Patients with expectant management (watchful waiting) avoided changes in sexual functioning, but their urinary tract symptoms were unlikely to improve.

  The traditional way of making clinical decisions—delegating responsibility to the physician—was not geared toward diagnosing patients’ preferences. This required the active
involvement of the patient in the choice of treatments, which meant a change to shared
decision making or "patient-centered choice." PORTs thus became involved in the develop-
ment of patient decision aids that described the advantages and disadvantages of various
treatment options. Clinical trials indicate that patients using decision aids are more knowl-
edgeable about the treatment options and tend to make choices more in keeping with their
preferences than is true for controls. Decision aids also tend to result in less use of surgery,
which suggests that the prevailing surgery rates in many parts of the United States are
higher than what informed patients might want.

The fate of outcomes research. Outcomes research is not ivory tower research. It attracts a lot of attention, sometimes from the
 disgruntled. Gray and colleagues describe how the PORTs and much of the outcomes re-
search agenda fell victim to the lobbying ef-
forts of a small but determined group of ortho-
pedic surgeons: Offended by the implications for their practice patterns of the findings of the
back pain PORT, they persuaded their allies in
Congress that the program should not be sup-
ported. Emerging from its near-death experi-
ence, AHCPR shed its mandate to evaluate the
common practices of medicine, changed its
name, and focused on a safer and more narrowly
defined agenda: the study of medical error.
The unfortunate political history of the
PORT program and AHCPR's failure to sustain
the effectiveness program illustrate the risks
inherent in efforts to evaluate the common
practices of medicine and reform clinical deci-
sion making so that demand for discretionary
treatments depends on patients' preferences. In-
stitutionalization of the evaluative sciences
requires the long view: stable funding; strong peer review to assure good science and free-
dom from conflicts of interest that affect judg-
ments; policies that sustain the careers of lead-
ing scientists over a professional lifetime
(keeping them free of dependency on funding
from the drug and device companies whose
products they evaluate); and deep commit-
ment on the part of the scientific establish-
ment, sufficient to withstand the wrath of practitioners and others with vested interests
who find their favorite theories slain by evi-
dence or demand for their services reduced be-
cause informed patients want less.

Pressing problems ahead. The evaluative sciences have yet to regain the con-
gressional attention they had when Senators
George Mitchell and David Durenberger champi-
oned the cause and mandated that a
new agency be established to forward the
agenda. But the basic problems remain and are
going worse: Variations in medical practice
and in Medicare per capita spending continue
to plague efforts for reform, and the nation has
finally woken up to the fact that low-spend-
ing, low-use states and regions are subsidizing
care provided in high-use places. Because
health care is primarily viewed as being ap-
plied science, the debate over variations in use
and spending and associated transfer payment
continues to point to the need for better clini-
cal science and the role of the informed patient
in determining the demand for discretionary
care.

Sooner or later, Congress will need to re-
visit the debate over where in the federal gov-
ernment the evaluative sciences should find
their base, and questions concerning the role
of the National Institutes of Health (NIH) will
be raised once again, as they were at the time of
AHCPR's founding. The complementary roles
of the biomedical and evaluative sciences in
promoting and governing innovation, and in
building the scientific basis for clinical deci-
sion making, and the stature required to pro-
tect science against rogue critics argue for
NIH as the locus for such activity. The argu-
ment is further strengthened by the success of
NIH's special partnership with academic med-
icine in building biomedical science. The idio-
syncratic, unscientific nature of practice pat-
terns among the nation's AMCs and the
current lack of scientific infrastructure and
funding required to undertake the needed re-
search suggest that a similar partnership is
needed to build the evaluative sciences and
stabilize the mission of academic medicine as
standard bearer for the clinical sciences.