Evidence-Based Decision Making: Global Evidence, Local Decisions

The challenge now is to further develop and translate the worldwide evidence base for local application.

by Carolyn M. Clancy and Kelly Cronin

ABSTRACT: Variations in health care services have been well documented worldwide. The result is that increased health care spending is not uniformly associated with improved health. Interest in increasing the value obtained from health care investments has stimulated efforts to develop the best science and apply it to health care delivery. Advances in communications and information technology have made such developments of the scientific basis for health care a truly global enterprise, but its application must remain local. Consumers’ use of evidence-based information to choose providers, make treatment decisions, and play a more active role represents the ultimate local application of scientific information.

Substantial variations in health care services have been well documented in the United States and abroad. The net result is that increased health care spending is not uniformly associated with improvements in patients’ health status. Indeed, this lack of consistency led the New York Times to editorialize about the need to “turn idiosyncratic medical practice into a more reliable science.” Intense interest in increasing the value obtained from investments in health care has stimulated a broad array of efforts to develop and apply the best possible science to inform health care delivery.

Advances in communications and information technology (IT) have made development of the scientific basis for health care a truly global enterprise, but its application is and should remain specific to the local policy context, to enable appropriate use of evidence at the point of care. This paper provides an overview of the rationale, current applications, and future opportunities for improving evidence-based clinical decision making—that is, the application of science to improve health care and increase its value to the public.

Evolution of the use of evidence. For centuries, medical practice has been...
based mostly on clinical experience and judgment. Several recent developments have increased the promise and imperative of evidence-based decision making: tremendous growth in biomedical science and innovation; development of the evaluative clinical sciences; advances in communication and IT; and growing recognition that evidence-based decision making provides a framework for addressing health care policy challenges.

First, medical studies have undergone explosive growth: More than 11,000 publicly funded trials are under way in the United States alone. This has increased our ability to link decisions to evidence. Rapid growth of medical studies has also sharpened the need for tools that clinicians, patients, and policymakers can use to sort through the confusing and sometimes conflicting array of evidence. Second, the growth and maturation of methods and expertise for conducting and using systematic reviews have increased the reliability of evidence for use in health care decisions. Consistent and transparent methods have been developed for weighing evidence and synthesizing the results of multiple studies. This has resulted in a broad array of reliable and objective sources that can be consulted to augment expert opinion. Third, the emerging health information technology (HIT) revolution makes it possible to push evidence to the point of care and to identify where (and why) practice and evidence diverge. Fourth, reliable evidence can address the dual imperatives of controlling costs and improving quality.

In recent decades much attention has been focused on research and specific applications that promote the use of evidence in clinical decision making to reduce unwanted or inappropriate practice variations (variation that is not the result of patient preference or clinical severity). The existence of practice variations and unabated increases in health spending have sharpened policy interest in identifying strategies that can demonstrate a clearer relationship between health care inputs and outputs. Increasing the relevance of scientific evidence to clinical and policy decisions relies on both a transparent approach to evaluating the quality of scientific studies and a broad debate about the interpretation of scientific findings and their optimal application.

Research findings derived from a single study are rarely definitive, while replication of results in multiple studies offers assurance that the findings are reliable. Systematic reviews, based on quantitative techniques to evaluate and synthesize a body of research in a particular area, represent a core component of efforts to incorporate science into clinical decisions, yet there is a recognized need to expedite this process to keep up with the continuously growing literature and the need to transfer knowledge to the consumer and clinician at the point of care.

- **Research at home and abroad.** The conduct of research—as well as the development of strategies to assess and synthesize its outputs in the form of systematic reviews and meta-analyses—is an enterprise that transcends national borders. A growing proportion of clinical research sponsored by the drug industry is multinational and has relevance to regulators and clinicians across borders. In the 1990s
alone, the scope of global clinical trials grew from twenty-eight to seventy-nine countries.\(^5\) The application of science in policy and clinical decision making, however, depends on local or national professional norms, consumers' expectations, and the information infrastructure and tools at the point of care. John Eisenberg noted that Americans are intensely interested in technological innovation and place a high value on individual choice, while issues of equity and efficiency (the greatest good for the greatest number) are more dominant in Western European and other developed nations.\(^6\)

Applications that support the consumer's role in decision making will be a strong focus in the United States. Clinicians must tailor scientific information derived from population-based studies to individual patients' needs and preferences, and policymakers must identify which approaches are most likely to succeed for their programs. Policy interventions, such as the use of formularies, coverage, cost sharing, and financial and other incentives, have an important albeit indirect impact on clinical decisions.\(^7\)

### Examples Of Evidence-Based Decision Making

Exhibit 1 presents selected examples of health care decisions categorized by type of decision, decisionmaker, and the importance of scientific evidence to the decision process. Proponents of evidence-based decision making have always rec-

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<th>EXHIBIT 1</th>
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<td>Care options(^b)</td>
<td>Individuals; patient and disease groups</td>
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**SOURCE:** Authors' analysis.

**NOTES:** Level I: rigorous evidence required (absolute requirement); level II: evidence is predominant input when available, supplemented by expert judgment; level III: available evidence is one but not the only input to decisions; level IV: available evidence limited, other considerations important. FDA is U.S. Food and Drug Administration. PBM is pharmacy benefit manager.

\(^a\) Pilot programs.

\(^b\) Emerging focus.
ognized that evidence is never the sole determinant of clinical or policy decisions. Decisions for individual patients must integrate evidence with information on clinical circumstances and patients’ preferences, while policy decisions need to consider values and resources along with evidence.8 The importance of scientific evidence in various decisions is also a function of available evidence, the number of competing considerations, and the culture and context of the decisionmakers.

In general, clinical interventions have received far more research investment than organizational interventions. In some instances, studies of specific organizational and financial strategies have received major attention only recently. This categorization of levels of evidentiary input is illustrative and descriptive of the U.S. health care system.

- **Drug approval.** Some countries attempt to identify rigorous evidence prior to approving pharmaceuticals for a national formulary, an approach that maximizes efficiency but limits individual choice.9 In the United States, Food and Drug Administration (FDA) approval for marketing a new drug product is based on a well-defined pathway. Approval cannot occur absent clear evidence of effectiveness.

- **Practice guidelines.** Clinical practice guidelines have evolved during the past twenty years from recommendations based largely on expert judgment to recommendations grounded primarily in evidence. Expert consensus comes into play in guideline development only when evidence is lacking.10

- **Shared decision making.** John Wennberg’s work in multiple clinical arenas in which there are two or more alternative treatments has resulted in growing interest in shared decision making between clinicians and patients. At its best, this process involves presentation of accurate information about alternative strategies, including no treatment, in an unbiased fashion so that individual preferences and values can be integrated with science. For example, if patients’ preferences were to be considered routinely, rates of elective hysterectomies across regions might be similar. Instead, women in New Haven are 50 percent more likely to undergo a hysterectomy than women in Boston.11

- **Quality improvement.** Interest in assessing and improving health care quality is another example of the application of evidence-based methods to decision making—whether for purposes of internal improvement, public reporting, or paying for performance. Metrics used by the National Committee for Quality Assurance (NCQA), the Joint Commission on Accreditation of Healthcare Organizations (JCAHO), and other accrediting bodies rely on available evidence, but they supplement that evidence with expert opinion, particularly in important clinical areas for which the evidence base is underdeveloped (such as mental health).

- **Coverage decisions.** Clinical evidence can be and is used to inform coverage decisions made by purchasers and employers. For example, Medicare relies on systematic reviews supported by the Agency for Healthcare Research and Quality (AHRQ) as well as input from the Medicare Coverage Advisory Committee (MCAC) to make coverage decisions. The lack of relevant evidence, however, poses
a challenge regarding which party bears the “burden of proof” if scientific knowledge is inconclusive or lacking.12

**Improving Evidence-Based Practice**

Several systematic reviews have demonstrated that a supportive practice environment and incentives for change are prerequisites for improving evidence-based clinical care. Interest in evidence-based management—informing decisions about organizational or financial strategies to improve health care (for example, utilization management, use of hospitalists, disease management programs, or pay-for-performance programs)—has greatly increased in recent years. Today that interest is not matched by a robust scientific base. Multiple initiatives are in progress—including demonstrations required by the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003—and are likely to expand existing knowledge in the near future.

Patients also are increasingly aware of the ways in which evidence is a prerequisite for both good health care decisions and policies. The Women’s Health Initiative was largely catalyzed by women who realized how little was known about gender-specific treatment effects and the natural history of disease. Moreover, the vigorous debate over the appropriate frequency for screening mammography among patient advocacy groups, policymakers, and clinicians highlighted the inadequacy of the evidence available to make an informed recommendation for women ages forty to fifty. In general, increased public awareness of the importance of evidence to inform clinical decisions has led to increased consumer demand for this information.13

**Can It Make A Difference?**

Despite the notable progress to date, evidence-based decision making has been largely overshadowed by the persistence of poor-quality care in the United States. Elizabeth McGlynn’s landmark report on U.S. health care quality, AHRQ’s *National Healthcare Quality Report*, and a recent cross-national report on quality indicators raise important questions about the gap between the promise of evidence-based health care and its current level of adoption.14 All stakeholders in the health care system presumably find the current seventeen-year delay from evidence to practice unacceptable.15 This translation chasm is even more intolerable, given the increased array of choices resulting from large public and private investments in biomedical science. Although many factors, including local professional norms and patients’ values and preferences, contribute to deviations from evidence-based care, a fundamental question remains: Why does the gap persist?

- **Limited resources.** Investments in biomedical science have resulted in a wide variety of diagnostic and therapeutic options for clinicians and patients. The extant infrastructure for conducting systematic reviews—including AHRQ’s Evidence-based Practice Centers (EPCs), the worldwide Cochrane Collaboration, and inde-
dependent private-sector organizations—has led to much progress in developing methods and conceptual enhancements for systematic reviews. Nevertheless, the field is not advancing as rapidly as it could because of limited resources.

**Knowledge chasm.** Moreover, by definition, systematic reviews rely on available studies. Since the link between decisionmakers’ needs and establishment of clinical research priorities is somewhat circuitous, the net result is that decisionmakers have few resources for learning quickly which patients are likely to benefit from new options and which patients will experience marginal benefits or outright harm. Payers and consumers confront the same knowledge chasm and lack good information for coverage decisions, cost sharing, and treatment choices.

**Need for a systems approach.** We now know that knowledge about best practice is necessary but not sufficient to effect change in practice and policy. Impatient purchasers are testing innovations to identify incentives and programs that reward evidence-based (“best”) practice, but they have a limited knowledge base on which to derive or evaluate new approaches. Although the Institute of Medicine’s reports on medical errors and quality have reinforced the importance of a systems approach to improvement, major support for research to inform such an approach has only recently become available.

**Poor accessibility.** Finally, evidence is infrequently available in a form that can be acted upon at the time decisions must be made. From clinical encounters to policy decisions, there are few clear pathways between the evidence that is available through peer-reviewed literature reviews and the point of decision making. Clinicians searching for information all too often find that existing knowledge is not accessible in real time and may not necessarily map to the issue at hand. Also, although consumers are increasingly active in seeking information about health and specific conditions, most of this activity is peripheral to care delivery. Personalized health information that facilitates involvement in decision making, including provider and treatment selection as well as self-management, looms on the horizon. Development and adoption of personal health records could support individual choice based on current information.

**Role Of An Information Infrastructure**

A fundamental reason for the translation gap is the lack of an information infrastructure to facilitate the conducting and rapid dissemination of relevant research. Most hospitals lack an information infrastructure to use evidence at the point of care or measure performance easily. For example, in a region with one of the top academic medical centers (AMCs), less than 40 percent of ideal candidates received a beta-blocker following a heart attack to reduce subsequent mortality. Even among the top AMCs, which often have access to the best technology and most prolific researchers, there are major variations in end-of-life care attributable to poor care coordination and redundant testing.

The lack of an information infrastructure also delays policy decisions that affect...
clinical decisions and protect public health. The FDA is largely dependent on a spontaneous adverse event reporting system to ascertain risks associated with medical products, but the agency could be much more effective and rapidly assess serious public health threats if it had access to providers’ information infrastructure. Following the initial serious adverse event reports related to cerivastatin (Baycol), access to laboratory results and prescription data on statins could have allowed active investigation of the deadly drug effect and management of this risk to prevent further harm. More recently, the withdrawal of Vioxx (rofecoxib) from the market highlights the need for an infrastructure that allows for the active monitoring of serious adverse events among the millions of patients exposed and the expeditious determination of the magnitude of the risk.

**Needs And Opportunities**

- **Closing the gaps.** The pressing need to close gaps in developing and translating the evidence base will require a commitment to creating more reliable and useful evidence, developing and adopting tools that make it easier to use evidence in the wide array of decisions confronting clinicians and policymakers, and designing incentives that reward individual clinicians and systems for evidence-based care.

  Gaps in the evidence base cannot be addressed without frequent consultation with decisionmakers. For example, as the Department of Health and Human Services (HHS) prepares for implementation of the new outpatient drug benefit in 2006, there is a critical need to build an evidence base to inform both regional formulary development and appropriate prescribing for Medicare beneficiaries.

- **Implementing MMA.** MMA Section 1013 requires the HHS secretary to set priorities and target areas where evidence is needed to improve the effectiveness of services delivered, informed by the needs of Medicare, Medicaid, and the State Children’s Health Insurance Program (SCHIP). This process should identify the therapeutic areas most in need of comparative effectiveness research and direct funding of independent research, so that sponsors of prescription drug plans, clinicians, and beneficiaries understand which drugs with similar effects are likely to give the most benefit. Such evidence has the potential to greatly reduce out-of-pocket and government spending for new drug benefits. Section 1013 of MMA thus sets the stage for explicitly incorporating decisionmakers’ needs in setting priorities for the effectiveness of health care interventions.

  MMA also requires sponsors of prescription drug plans to develop formularies based on evidence of safety, efficacy, and economic impact; however, the systematic and appropriate use of this type of evidence will depend on the quality of the information and the expertise of the interpreters. Prescription benefit managers and plans will most likely have data that are generated and synthesized by drug companies, and selections for at least one drug per therapeutic category will be influenced by negotiated discounts with manufacturers.

  In addition, MMA Section 107 requires the regulation of standards to enable
electronic prescribing, including standards necessary to facilitate use of evidence at the point of care. Pilot tests will be necessary to ensure that the standards, software, and knowledge databases will enable the use of accurate, current, and global evidence prior to each prescribing decision.

**Promise of HIT.** The recently announced “decade of health information technology” offers unparalleled opportunities to develop and implement strategies that bring evidence-based information to the point of decision making on an unprecedented scale. Previous studies, conducted largely at a small number of institutions with sophisticated clinical information systems, have produced substantial evidence that electronic pathways—from evidence-based reminders to clinical decision support systems—can improve quality and efficiency and reduce medical errors.

Cooperation between the public and private sectors is required to learn from and build on these results and implement incentives for the adoption of interoperable electronic medical records (EMRs). Because patients often receive care, even within a single episode, from multiple clinicians in different settings, secure, confidential exchange of health care information from interoperable EMRs across health care settings is critical to providing evidence-based care.

To date, the organizational, financial, legal, technical, and operational challenges of creating and sustaining health information exchange have been highly variable and technically idiosyncratic and have yet to produce sustainable business models that could be used in other communities. To accelerate the growth of regional health information exchange, a more uniform and cost-effective approach is needed. HHS is supporting grants and contracts to facilitate health information exchange and advance the adoption of EMRs.

The proliferation of these regional collaborations will reflect local health care priorities and provide a trusted resource for physicians and patients to achieve quality and safety goals in the community. As they evolve, these entities, or regional health information organizations (RHIOs), could develop new data sources to accelerate the conduct of health research. If done successfully, these databases could, over time, reduce the reliance on administrative data sets. While legal and operational policies are needed to oversee the use of these databases, RHIOs—with guidance from the state or federal government—could work together to determine appropriate requirements for confidentiality, informed consent, approved uses, and access fees.

New methods, including appropriate study designs and analytic techniques, to make effective use of these new, rich data sources for public health surveillance and clinical and health services research will be needed. Using real-time detailed clinical data from large cohorts will bring about a whole new concept of a representative “sample.” Enrollment in prospective studies, including randomized controlled trials, could be expedited, as could data collection and management. These types of developments can greatly reduce the time necessary to fill gaps in the evidence base and reduce the uncertainty in the decision-making process.
The HIT Strategic Framework for Action prioritizes efforts to support market institutions to ensure that reliable and interoperable EMRs will be adopted successfully with features to customize the use of (global) evidence. Clinical decision support must be implemented in a way that balances transparency about the source and strength of evidence with legitimate private-sector interests in deploying workable electronic solutions for clinicians and consumers.

- **HIT linkages to points of care.** Bringing evidence to the point of care requires more than investments in HIT and networks for health care information exchange. To realize the full potential of HIT, advances in the field must be linked with the redesign of care processes, development of new “vehicles” for delivering the right information at the right time to the right patient, and development of an infrastructure to ensure that the evidence underlying clinical decision support—reminders, alerts, quality measures, and other applications—remains current.
  
  For example, knowledge databases that house literature and evidence-based information should be established and maintained in computer-interpretable formats to facilitate effective care management in practices that adopt EMRs. In the near future, development of clinical practice guidelines is likely to include concurrent development of quality measures, and developers will need to anticipate the need for computer-interpretable guidelines. Such guidelines will permit delivery of up-to-date, evidence-based information that is relevant to the specific patient and decision being made. This infrastructure is likely to evolve from current efforts led by AHRQ, clinical specialty organizations, and payers and may well include patient advocacy organizations.

- **Quality assessment and improvement.** A well-designed health information infrastructure will reduce the current burden of quality assessment and enable timely feedback on performance. While increased availability of information per se won’t “fix” quality challenges, it is impossible to envision improvements on the scale required without it.
  
  The imperative to improve is sufficiently urgent that classic research methods may be less relevant than approaches based on learning from short-cycle studies. An important component of these efforts will be demonstrations of payment reforms that reward improvements. Building on current efforts led by quality improvement organizations in four states, MMA Section 649 includes a demonstration focused on improving chronic illness care in physician practices through paying for the use of HIT and improved performance. Section 721, a new disease management initiative, similarly focuses on improving care for beneficiaries with chronic illnesses. This initiative is likely to involve some innovative applications of HIT, including strategies to provide messages directly to beneficiaries. AHRQ’s new investments in this area will evaluate the use of selected applications of HIT to improve health care safety and quality.

- **Use of global evidence.** As clinical decision support is deployed through adoption of interoperable EMRs, policies will be needed to ensure transparency, so
that the sources and levels of evidence are fully disclosed as clinicians and other
decisionmakers use computer-interpretable guidelines. These guidelines also will
need governance and oversight. The opportunity for each provider organization to
develop and implement its own guidelines based on its own clinical experience and
expert knowledge could actually increase variations in care.

The use of the global evidence and the consensus concerning that evidence by
decisionmakers as a baseline is imperative. Customizing the application of evi-
dence should be left to individual organizations and clinicians. It will be impor-
tant to ensure that the flexibility and integrity of HIT tools are maintained and
that they effectively deliver evidence to the point of care.

Lessons from the U.K. experience. The National Institute for Clinical Excel-
lence (NICE) in the United Kingdom makes recommendations to the National
Health Service (NHS) on treatments to be reimbursed based on evidence of effec-
tiveness, safety, and costs. The intent is to implement these recommendations as
guidelines in the deployment of the NHS “data spine,” a somewhat centralized
health information infrastructure in the United Kingdom, to improve compliance
among clinicians. Considering that a large proportion of care in the United States is
reimbursed by a diverse set of private payers who have failed to demonstrate a will-
ingness to finance a NICE-like organization, RxIntelligence, coupled with the fact
that the U.S. government has historically withdrawn its support from programs
with similar intent (such as the Office of Technology Assessment), it is not likely
that the United States will have a NICE equivalent in the foreseeable future.29 None-
theless, lessons can be learned from the U.K. experience that are applicable to pri-

cipe purchasers and payers and the U.S. clinical decision support infrastructure.

Clinical decision support. Accurate and reliable information on benefits and
formulary status will likely inform electronic prescribing and assist clinicians in se-
lecting cost-effective drugs that will minimize out-of-pocket costs. The same could
hold true for elective surgeries or other services that are not covered by a health
plan. Without computer-interpretable guidelines that are tailored to covered bene-
fits, the potential exists for clinicians to choose options that patients cannot afford.
The Medicare program stands to benefit from a clinical decision support infra-
structure that will minimize inappropriate use and overuse of drugs, particularly
for newly approved new molecular entities with monopolistic pricing structures
and little information on incremental costs and effectiveness. Evidence on the full
array of treatment options should facilitate the best choice for each patient based
on all relevant medical history and ability to pay.

If the promise of evidence-based decision making is realized,
Medicare spending could be reduced by 30 percent during a period of large ex-
pansion of government-funded health benefits and a growing uninsured popu-
lation.30 Capitalizing on HIT to enable the use of evidence at the point of care is
not about limiting options or replacing clinical judgment; rather, it is about assist-

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ing clinicians in choosing the options that are most likely to improve health outcomes while minimizing out-of-pocket costs.

The opportunity to build the infrastructure to enable efficient expansion, synthesis, and deployment of the evidence base has finally arrived. Likewise, the public and private sectors must act together to ensure that the infrastructure, policies, and procedures are established over the next decade so that clinicians, policymakers, and consumers will make decisions to improve the quality of care.

Finally, it is important to be aware of the current limitations of available evidence without letting that detract from the very real potential contained in systematic, evidence-based approaches to practice and policy. Evidence alone will never resolve the numerous complex decisions involved in taking care of individuals or making health care decisions for diverse populations.

For many policy issues, there is too little evidence to be of much help. The challenge now is to ensure that clinicians and policymakers can easily find out what we do know, support research to answer what we do not know, and promote change in the health care system that will continue to narrow the gap between what we know and what we do.

The views expressed in this paper are those of the authors and do not necessarily reflect the official position of the U.S. Department of Health and Human Services.

NOTES
11. J.E. Wennberg, “Unwarranted Variations in Healthcare Delivery: Implications for Academic Medical Cen-


18. Ibid.


24. Grants funded by HHS (HRSA) through the e-health initiative can be found at Foundation for E-Health Initiative, “Communities: CCBH Funded Communities,” ccbh.ehealthinitiative.org/communities/funded.mspx (22 October 2004). AHRQ grants are pending at this writing.

25. The HIT Strategic Framework for Action (see Note 22) was published by the Office of the National Coordinator for Health Information Technology in July 2004.


