Bench to Trench: How Evidence and Guidelines Shape Health Care Policy and Practice

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Abstract
Clinical practice guidelines and performance measures are becoming increasingly pervasive. They epitomize the evidence-based movement, which recognizes that mere "clinical judgment" is often inadequate to synthesize all the important data to determine the best management for a particular patient. This movement has at its core the fundamental expectation that medical decisions be based on sound data rather than anecdote. Unfortunately, this concept rarely manifests in daily practices where significant variations in care still exist. Guidelines were designed to improve patient care, reduce unnecessary variation, and reduce attributed costs. Therefore, it is not surprising that associated recommendations are now being incorporated into health care legislation as part of the Patient Protection and Affordable Care Act. In this environment, there is growing urgency for otolaryngologists to participate in rigorous comparative effectiveness research that will direct our Academy's guideline developers and policy makers to make recommendations that optimize care for all our patients.

Keywords
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Regardless of personal reservations, guidelines and performance measures are the future. This is perhaps best exemplified by the incorporation of US Preventive Service Task Force (USPSTF) A and B level recommendations as coverage requirement into the Patient Protection and Affordable Care Act.¹ Their inclusion testifies to the success of guidelines at improving patient care,²⁻⁴ decreasing variation, and reducing economic costs²,⁴ and is a harbinger for their role in future health care policy decisions.

Otolaryngology played an early and instrumental role in the evidence-based medicine movement and in motivating guideline development. However, it was not a pioneering, shining day for the specialty; instead, our care typified unexplainable discrepant variation in medical care. In their 1973 study published in Science, Wennberg and Gittlesohn⁵ exposed dramatic small area variations in tonsillectomy rates among adjacent hospital service areas. They found the probability of having a tonsillectomy by age 20 years in Vermont ranged from 66% in 1 area to between 16% and 22% in the 5 surrounding health service areas, among patients with similar demographics.

Traditional Medical Decision Making Is Flawed
When different physicians recommend different management strategies for similar patients, it is impossible to claim each is providing appropriate care. Wennberg and Gittlesohn’s study⁵ shook the assumption that through the rigors of medical education, journal reading, individual experience, and exposure to colleagues, physicians are making appropriate, informed decisions.⁶ It emphasized that mere "clinical judgment" is often inadequate to synthesize all the important patient information, relevant research, and previous experience with similar patients sufficiently to determine the best management for a particular patient.⁷ It also stimulated the emergence of evidence-based medicine and its enduring principles, first described by Eddy⁸ in American Cancer Society screening recommendations, that (1) there must be good evidence that each test or procedure recommended is medically effective at reducing morbidity or mortality, (2) medical benefits should outweigh the risks, (3) the cost of each test or procedure must be reasonable compared with the expected benefit, and (4) the recommended actions must be practical and feasible.

The principle that medical decisions be based on sound data rather than anecdote is a compelling and appropriate

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expectation that all our patients deserve. However, it is rare to consistently embrace this approach in our daily patient practices. In ours and arguably all medical specialties, decisions regarding patient care are often based on inadequate evidence, errors in reasoning, gross oversimplification, and wide variations in belief. Moreover, significant variation in care still exists, and medical decisions are often parochial with physicians at particular centers and their trainees convincing themselves of the superiority of their algorithms without supporting data.

This straw-man approach needs to change. Clinical judgment will always remain a cornerstone of patient care, but medical decisions must be anchored by rigorous scientific evidence rather than opinion or expert consensus. In essence, we need to embrace and be comfortable with what we do not know. The oft-cited “because that is how it has always been done” is no longer adequate or defensible. A more formal methodology is needed to replace the currently flawed and unsustainable medical decision-making paradigm.

**Evolution of Medical Decision Making and Guidelines**

The historical process of managing the complexity of medical decisions was to convene expert panels and develop consensus statements on the most “appropriate” management. Evidence-based guidelines evolved from this approach and are meant to guide physicians at the point-of-care by summarizing evidence to inform decisions for “typical” patients. Despite their noble motivation, guidelines, like other external influences on practice patterns, are met with skepticism that autonomy will be eroded and practice will become “cookbook” or protocol driven. Skepticism is fueled by many factors, including a pervasive belief in practice exceptionalism and population paradoxes.

To be clear, evidence-based guidelines are neither perfect nor devoid of expert influence and conflict of interest, as each requires “experts” to assess and judge strengths and weaknesses of available evidence. Recently, it has been proposed that even evidence-based guidelines are inadequate as they focus only on whether there is evidence for efficacy without quantifying the magnitude of effectiveness, harms, or economic costs. The implicit premises of evidence-based guidelines are that (1) if there is evidence of effectiveness it should be done, (2) all effective treatments are equally important, and (3) their use will lower costs. Clearly, these assumptions lack foundation. This approach is predicated on the lofty assumption that there are sufficient well-designed studies to support each domain (ie, effectiveness, harms, costs, trade-offs). Evidence in otolaryngology is rarely adequate to fulfill this requirement.

Currently, guidelines rely heavily on results of randomized controlled trials (RCTs). The American Academy of Otolaryngology—Head and Neck Surgery Foundation (AAO-HNSF) guidelines are no exception, as exemplified by our clinical practice guideline on tonsillectomy, which gleaned information from more than 700 RCTs and systematic reviews. However, not all our guidelines can reasonably be founded so heavily on level I evidence. In fact, it is unreasonable to expect that outcomes-based guidelines on any topic in any specialty could be based solely on clinical trials. There are too many questions, limited funding, and their results are not generalizable and become outdated with technological innovations. Instead, outcomes-based guidelines recognize the evidence hierarchy to be illusory and that, particularly for therapeutic interventions, all available evidence, including observational studies, must be considered in the decision-making process. Assessing outcomes of clinical care is difficult and relies on methods of observational epidemiology to address inherent probability, bias, and confounding factors.

The shift toward outcomes-based medicine requires embracing mathematical modeling such as those used in computer science, economics, and other fields. Powerful data sources and software are increasingly available, and computing power is expanding exponentially. Extensively validated mathematical models based on physiology and patient preferences that help predict patient outcomes are the future of medical decision making. This will require otolaryngologists to engage and align with epidemiologists and biostatisticians as part of their patient care teams. Clinicians who care for patients must participate in defining the outcomes and conduct the necessary studies to direct these modeling efforts. Only through this process will policy makers, epidemiologists, and biostatisticians be able to create appropriately informed models using clinical and economic outcomes and time horizons that are important and relevant to our patients and decision processes.

**Lack of Evidence of Effectiveness Does Not Equal Evidence of Absence of Effectiveness**

Regardless of methodological specificity, it cannot be overemphasized that clinical practice guidelines are only as good as the evidence on which they are based. This places the burden on guideline developers to identify topics with sufficient evidence to synthesize but, perhaps more so, on clinician- and communication-scientists who interact with and study the patient population and disease processes of interest. Insufficient evidence results in guidelines with weak recommendations. Consumers of guidelines (ie, clinicians, purchasers/payors, policy makers, patients) often misconstrue “lack of evidence of effectiveness” to mean “evidence of absence of effectiveness.”

An editorial by Smith and Pelt illustrated this point well when they argued for trust in parachutes without a double-blinded RCT of their effectiveness. Recognizing the distinction between lack of evidence and absence of effectiveness is important albeit subtle. It behooves the otolaryngology community not to rely on consumers to appreciate such subtleties. Instead, we should concentrate our efforts on pursuing rigorous comparative effectiveness research that accurately measures and documents treatment outcomes, risks and benefits, and economic consequences. It is important to realize that the data from which guidelines were derived are the best available.
guideline development for the past 10 years, recognizing early that participation in this process is critical to optimize patient care and reduce the risk of regulatory oversight. Guidelines and performance measures are becoming increasingly pervasive, as demonstrated by the USPSTF. This shows how outcomes research can be cogently translated into actionable bedside recommendations. Instead of seeing guidelines as an affront to autonomy and individualized care, we need to recognize how the clinical studies and trials that we perform (or do not perform) will shape policies of the future. Guidelines are not static tomes but rather, as demonstrated by the USPSTF’s recapitulation of topics, should be considered living and working documents requiring diligent updating as pertinent data become available.

The goal is not to create automatons but rather to help us to synthesize the complexity of data to “guide” patient-centered and preference-based information that can be readily accessed at the site of care. It is expected that the role of guidelines will be expanded as the Centers for Medicare & Medicaid Services moves toward a value-based purchasing approach based on five distinct principles: (1) defining the end goal, not the process for achieving it; (2) aligning incentives of all providers; (3) developing outcome measures and implementing them in a rapid cycle; (4) actively supporting quality improvement; and (5) active engagement of clinical community and patients. We owe it to our patients to elevate the standard of research by embracing novel epidemiologic concepts and sharing data so that we can demonstrate to the greater medical community, patients, payers, and policy makers the tremendous impact we have on our patients’ lives.

Moreover, we need to recognize that significant variability still exists in our specialty and harness it to improve care through comparative effectiveness research that can demonstrate treatment efficacy and effectiveness. Using data, these studies can promote broad-scale implementation of effective practices that can improve health care quality and population health for all patients with communication and head and neck disorders. It is imperative that the success of and barriers to providers incorporating developed Academy guidelines be carefully studied to improve the development process, consumer “buy-in,” and dissemination. While some medical societies begin scaling back on sponsorship of clinical practice guidelines due to cost restraints and legal risk, our specialty’s leadership has embraced the concept, understanding that we must lead efforts or risk oversight. We are moving from an example of unexplainable discrepant care that sparked the evidence-based movement to an example of a specialty that is thoughtful, analytical, and systematic in applying outcomes-based medicine to optimize care for all its patients.

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