Promise and Perils of Guidelines in Quality Cancer Care

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Vignette

A 48-year-old woman with a history of hypertension presents to the medical oncologist with newly diagnosed invasive breast cancer. She had an abnormal screening mammogram, leading to biopsy and ultimately to right-sided lumpectomy. Pathology from her recent surgery reveals a 2.1-cm invasive ductal carcinoma, grade 2, endocrine receptor positive and human epidermal growth factor receptor (HER2) negative. There is no lymphovascular invasion, and two sentinel lymph nodes are negative.

After discussion of prognosis and adjuvant therapy options, the medical oncologist sends a multigene prognostic assay that reveals an intermediate risk for recurrence and uncertain benefit from adjuvant chemotherapy. The oncologist explains the rationale for endocrine therapy with tamoxifen and the evidence for possible benefit from chemotherapy and recommends four cycles of adjuvant chemotherapy. The patient declines chemotherapy but tells the oncologist that she has read about a European study showing that bisphosphonates might help prevent recurrence. The oncologist then acknowledges that the Austrian Breast Cancer Study Group (ABCSCG) 12 trial of adjuvant zoledronic acid added to tamoxifen and ovarian suppression demonstrated a benefit comparable to chemotherapy in terms of lowering the risk of disease recurrence among comparable patients in a large randomized trial.¹ He explains that this is not currently considered standard therapy, is not recommended in current breast cancer guidelines, and that a similar trial failed to show a benefit from adjuvant bisphosphonates.² However, he also acknowledges that the benefits seen in the ABCSCG trial do appear to persist over time and that this could be viewed as an evidence-based option if she declines chemotherapy.³

After discussion, the patient expresses an understanding of the rationale for the ABCSCG trial and the uncertainties surrounding the precise risk and benefit of this regimen, and states that she is highly interested in pursuing this therapy. The oncologist presents her case at a multidisciplinary tumor board and is reminded by colleagues that providing therapy that is not specified in the breast cancer guidelines may lead to lack of insurance coverage, higher medicolegal liability, and direct financial penalties due to recently established guideline-based quality-improvement initiatives in the practice.

In an era of guideline propagation, this case helps raise the question, Does quality cancer care now require that we always follow guidelines exactly, or do some circumstances still require a more individualized approach? Guidelines seek to provide evidence-based expert consensus on quality care for patients in

specific clinical scenarios. Ideally, such standardization can improve outcomes and promote efficient use of health care resources. However, as guidelines for cancer care proliferate and become viewed not just as recommendations for consideration in the right clinical context, but as benchmarks of quality with implications for reimbursement and access, we must ask what, if anything, is gained and lost in the real world of complex clinical decisions.

The case above illustrates some of both the advantages and challenges posed by guidelines. The oncologist is considering nonstandard care, and as such, the risks and benefits of the proposed care are incompletely understood. Adherence to guidelines, enforced by reimbursement, practice norms, or otherwise, will lead to the patient being offered standard treatment with quantifiable risks and benefits that she can then freely decline or accept. However, the patient has strong personal preferences that have led her to decline chemotherapy and consider a nonstandard approach.

The physician has obligations to try to help the patient and to avoid causing harm, termed beneficence and nonmaleficence, respectively, in bioethics parlance. The physician also has obligations to respect the patient's informed choices (autonomy) and a professional obligation to promote efficient and appropriate use of societal resources (justice). Rigorous adherence to guidelines in this case may or may not truly promote beneficence and nonmaleficence, depending on one's view of the likely risks, benefits, and toxicity from the nonstandard approach described versus standard, more intensive alternatives. The patient's prognosis is good regardless of her decisions, and the ultimate benefits she will gain or harms she will suffer from any of the possible treatment options are truly unknown. In the face of this uncertainty, some might question whether there are sufficient grounds for restricting the patient's autonomy through adherence to guidelines. In contrast, others might argue that guidelines support a higher standard of evidence for clinical practice that will, on average, benefit more patients and encourage appropriate care and use of resources. Whether restricting a patient's access to a less evidence-based path is in fact a meaningful loss of autonomy is certainly subject to debate and may be highly dependent on the clinical context. We consider here some of the pros and cons of guidelines in cancer care, not to question the ultimate value in further refinement and promotion of guidelines, but to shed light on issues that need to be considered as we strive for care that is both high quality and highly individualized.

Role of Guidelines

Medical guidelines were initially defined as “a set of systemically developed statements to assist practitioner and patient decisions about appropriate health care for one specific clinical condition.”^{4,5} The National Comprehensive Cancer Network (NCCN) has more than 50 comprehensive oncology guidelines whose topics span the entire care continuum, from methods of prevention to methods of detection, treatment, and adverse effect management. ASCO has created clinical practice guidelines for specific cancer settings and provisional clinical opinions in areas of emerging evidence with practice-changing implications. Such guidelines are currently used to encourage high-quality, evidence-based management that will increase the use of proven interventions while curtailing use of those that lack evidence or are known to be ineffective.

Guidelines may also have an increasing role in guiding reimbursement and containing health care costs through denial of coverage for services outside of the guidelines. Currently regimens approved in NCCN guidelines are listed in the NCCN Drugs and Biologics Compendium and are considered by the Centers for Medicaid & Medicare Services as a basis to support coverage decisions for cancer therapy. Adherence to guidelines is increasingly seen as one potential mechanism to constrain the growth in spending for cancer care while maintaining or improving quality of care.⁶ Supporting such arguments, a study of lung cancer care sponsored by Aetna demonstrated a roughly 35% reduction in the cost of care for patients treated according to guidelines compared with those treated outside of guidelines, with no difference in overall survival.⁷

Adherence to guidelines may also reduce medico-legal risk and curb malpractice costs, although this has not been studied in the context of oncology.⁸ There are now numerous initiatives between payers and physician practices, as well as initiatives within practices, to provide direct feedback on adherence with guidelines, and financial incentives to clinicians for providing guideline-directed therapy.⁹⁻¹²

Outside of oncology, there is some evidence that adoption of and adherence to guidelines can improve patient outcomes.¹³⁻¹⁷ There is also evidence that guidelines can help reduce length of hospital stay; the number of hospitalizations; and/or cost of care for patients with stroke, cardiovascular disease, and pulmonary disease.^{16,18,19} Evidence within oncology has been limited to observational studies, such as that by Olivetto et al,²⁰ which demonstrated an improvement in breast cancer outcomes as providence-wide guidelines for tamoxifen and chemotherapy were instituted in British Columbia. However, randomized trials or even natural experiments with concurrent control groups have been lacking.

Guidelines have several additional potential benefits. By providing a routine algorithm for common clinical scenarios, they may facilitate use of midlevel oncology providers and therefore help address the looming oncology workforce shortage. They can facilitate auditing of care delivery and subsequent quality improvement at the level of the individual provider, practice, or institution. Similarly, they allow a more reliable mechanism for payers to predict health care costs. Finally, by

defining the current standard of care, guidelines can help highlight priorities for cancer research and define the appropriate control arm for late-phase randomized trials.

There is also some evidence that guideline adherence can reduce health disparities. Olomu et al found that using a guideline-based discharge tool improved evidence-based cardiac care for both white and nonwhite Medicare patients, although nonwhite patients still received less discharge counseling than their white counterparts.²¹ Several studies have documented a lack of uniform application of guidelines among patients on the basis of ethnic and socioeconomic background, suggesting that evaluation of guideline adherence may help identify and address some disparities.^{22,23}

Limitations of Guidelines

Guidelines have several theoretical downsides. One potential concern is that guidelines will lead to less individualized care by not accounting for the unique circumstances that any individual patient, including symptoms, prognosis, unique features of disease presentation, personal preferences, social supports, and other relevant social or medical details that might determine optimal care.

Another major challenge is that guidelines are only as good as the data available and the process used to evaluate and promote relevant data. Guidelines may be incomplete; outdated; biased by the interests of guideline panel participants; or impractical because they are too complex, too vague, or not compatible with available resources. Establishment of guidelines on the basis of insufficient evidence for optimal treatment may lead to use of inappropriate therapy or curtail use of beneficial treatment. In rapidly evolving fields, establishment of guidelines may “cement” a standard of care that is quickly outdated, inhibiting adoption of more effective treatments as they emerge. Oncologists most familiar with emerging data might face real or perceived conflicts with adherence to guidelines versus their own view of the most evidence-based practice.

There are data supporting some of these concerns. Evidence indicates that guidelines can be limited by lack of rigor, focus on a single clinical condition, poor clarity, poor presentation, conflict of interest, and low applicability.²⁴⁻²⁸ There is also evidence that keeping pace with medical advancement is challenging.²⁹ In an evaluation of six oncology compendia, Abernethy et al³⁰ found variable quality, lack of transparency, and inconsistent standards of evidence. Because of the variation in quality, there have been efforts to standardize the methods for composing and implementing guidelines, but more work is clearly needed.²⁶

To Adhere or Not to Adhere?

Guidelines are intended to improve quality and may have additional benefits such as efficiency, but legitimate concerns are raised above. Our first consideration must be the quality of the guidelines. In developing guidelines, expert panels must consider whether there is a sufficiently discrete clinical scenario and a sufficient body of evidence for quality care to even consider initiating development. If we proceed with guideline develop-

ment, the evidence must be reviewed and translated into practice guidelines through a transparent and comprehensive process, informed by clinical expertise in the area under consideration. In the process of developing guidelines for a given area, we must consider whether there are reasonable grounds for alternative interventions or approaches. When there are not, and there is one clear standard for care, perhaps we need something stronger such as a “pathway” (implying less guidance, more direction) to determine quality care. When a pathway can be defined, assessment and reinforcement of adherence through quality metrics and reimbursement policy may be appropriate. In other areas, where guidelines can be recommended but numerous evidence-based alternatives may be considered, guideline-making bodies should state this explicitly, and where possible, detail specific considerations that should influence care (age, molecular features of the tumor, comorbidities).

In any scenario involving restrictions on access, if a health care provider and patient together decide on a non–guideline-driven care plan, there must be efficient mechanisms for appeal if this plan is not covered by the patient’s insurance so that there is at least a chance that evidence-based and/or well-reasoned care plans can proceed. Furthermore, physicians must not be penalized for taking on patients who, because of the complexity of their disease, appropriately require or desire care plans that do not fit neatly within the evidence base supporting the guidelines.

To ensure that the benefits of guidelines are realized, several criteria must be met. The guidelines must be “living documents” that are frequently revised to incorporate new data and input from providers. Outcomes from guideline-driven practice should be reviewed to ensure that the guidelines are having their desired effects. We must ensure that authors and institutions involved in drafting guidelines be disclosed and references that formed the basis for guidelines be documented.

Once guidelines are drafted, they must be carefully implemented. It should be noted that simple distribution of guidelines and published reports does not necessarily change practice.^{31–34} In order to ensure implementation, guidelines must provide specific steps toward implementation, including an educational tool. To improve adoption, the guidelines must be specifically tailored for a given practice, taking into account a given practitioner’s knowledge base and resources.^{35,36} To change practice behavior, providers need specific reminders of

guidelines at the time of consultation, as well as feedback for their care processes and outcomes.

As research continues to improve the development, dissemination, and implementation of guidelines, they have the potential to serve as part of the solution to some of the most critical issues facing oncology, including maintaining or improving quality while controlling costs.^{6,37} However, although we strive to create perfect guidelines, for all of the good reasons noted above, we need to remember that there is still a role for art in the practice of medicine and in developing the appropriate care for an individual patient.

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