The standard definition of clinical practice guidelines explicitly states their aim as “to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances.”1 Over the past several years, the goal of allowing patients direct involvement in managing their health care has been pushed to the forefront, with the emphasis on shared decision-making2 and patient-centered care.3 The old paternalistic model of unassailable physician-directed recommendations is no longer a tenable approach.4 Patients are expected to understand the reasons for and consequences of the procedures they undergo and the care they receive to be able to decide between alternatives when available. Therefore, clinical practice guidelines, especially versions specifically designed for patient understanding, serve a valuable function in providing the information required for this informed decision-making.5

The implications of using guidelines recommendations as a vehicle for shared decision-making between caregiver and patient are both subtle and profound. If the guideline recommendations reflect only the judgments of a set of expert professionals, do they reflect the sum of the factors that should be considered in advancing those recommendations? Although the goal of any experts developing guidelines is to use the best available evidence in deriving recommendations, physicians recognize that the results of even high-powered clinical trials must be subjected to expert evaluation to ensure proper interpretation and that the results are congruent with other values and practical considerations.6 In the Ottawa Practice Guidelines Development Cycle, evidence-based recommendations derived by a panel of experts are sent to a broad range of practitioners for comments about acceptability, with resultant changes in the final guidelines.7 This ensures that the guidelines have received input from the broadest range of professional perspectives.

Another inclusiveness dimension of guideline development must be addressed, however. Halpern8 pointed out that guidelines devised solely by professionals reflect their views as to what is appropriate. In essence, clinicians use their assessment of what outcomes are important for guiding recommendations. However, as mentioned before, we now recognize that using physician values as the driving force in the patient-physician relationship is not optimal for achieving patient-centered care.9 Similarly, nursing professionals must also respect patient autonomy.10

Thus, another important and necessary guidelines attribute is clinical flexibility, and a key manifestation of this is the physician-patient team’s ability to consider patient preferences while using the recommendations.11 Viewed from the patient’s perspective, however, the implications of being asked to decide on guideline recommendations that are completely professionally derived may be troublesome: they are being presented with a restricted list of options, a list the professionals deem appropriate.8 Are they really being given the full panoply of options for their condition? Are there alternatives that they might choose if they knew about them?

An interesting example of this is the study by Slevin et al.12 evaluating the willingness of patients to undergo intensive chemotherapy compared with the
willingness of control subjects that included medical oncologists and radiation oncologists. Patients were willing to receive therapy for a 12-month prolongation of life and a 10% chance of symptomatic relief versus a 24- to 60-month increment and a 75% chance needed by controls. Thus, a guideline development panel composed only of physicians might not consider a third-line chemotherapy regimen as justified given the small positive clinical benefit. Patients, however, might believe that the guidelines developers had denied them a choice that they saw as valuable or beneficial.

How, then, does patient input affect a guideline? In certain guideline areas, professionals accept that, given the uncertain status of the evidence for benefit of a specific intervention, the patient should be the one to decide whether the intervention should be used or not. In these instances, respect for patients’ preferences and autonomy must be a paramount concern. To make this decision, the patient must not only be apprised of the balance of risks and benefits of the therapy, but the perception of risk must also be adequately explained. Helping the patient gain an accurate comprehension of risk is exceedingly difficult. Thus research has shown that women may either underestimate or overestimate the risk of developing breast cancer and that the factors leading to these misconceptions are complex and multifaceted, including communication and psychosocial issues.

Clinicians must also recognize that allowing the patient to decide is not a dead-end in the guideline process. Patient follow-up must be detailed for whichever path is chosen. An excellent example of this is the NCCN Breast Cancer Risk Reduction guideline. After the qualitative and quantitative risk assessment has been performed, and the patient has been given risk-reduction counseling, the algorithm branches into two pathways, one for women who do not want risk reduction therapy (i.e., tamoxifen) and another for those who do want to receive it. The algorithm then guides the user along a set of follow-up procedures based on whether tamoxifen is to be incorporated into the management scheme. In this instance, the “logic” of the guideline, the sequence of clinical decision-making, is built around patient preferences.

The NCCN guideline did not include a patient representative, so the developers are reassured that the recommendations are echoed in the American Society of Clinical Oncologists (ASCO) Technology Assessment, which states that, “In all circumstances, tamoxifen use should be discussed as part of the informed decision-making process with careful consideration of risks and benefits.” The ASCO Working group did include two patient representatives.

Several of the NCCN Clinical Practice Guidelines in Oncology Panels such as those for distress, breast cancer treatment, and Hodgkin’s disease, have made extensive use of patient representative input. This input is exemplified by the inclusion in the Surveillance section of the Hodgkin’s pathway of recommendations for counseling regarding reproduction, health habits, psychosocial support, cardiovascular follow-up, breast self-examination, skin cancer reduction, and end of treatment discussion, plus a call for written follow-up instructions for the patient. The insertion of these critical elements in patient care undoubtedly increase the usefulness of these pathways as physician and patients travel down the paths of clinical decision-making.

In these instances in which patient input provides recommendations in areas frequently overlooked by clinicians, this augmentation of the scope of
the guidelines probably generates little controversy and does not cause disharmony on the guidelines panels. The issue becomes more complicated when patient preferences directly conflict with clinical effectiveness evidence. Interestingly, an ASCO working group that outlined the primary outcomes to be used in guideline development—survival, quality of life, toxicity, and cost-effectiveness—generated this list from the perspective of the clinician and therefore did not address patient preferences as an outcome. Eddy pointed out the importance of ensuring that the outcomes used in evaluating treatment effectiveness are those outcomes that are important to patients. One problem is that despite the use of rigorous evidence-based methods, patient values may still be unknown.

Is this a real issue or an academic dialogue of little importance when confronted with the realities of guideline development? Silvestri et al. performed an interesting study that sheds some light on the issue of patient preferences while investigating the preferences of patients with advanced non-small cell lung cancer for receiving cytotoxic chemotherapy. Meta-analyses of the effectiveness of chemotherapy in these patients has shown improved survival of about 3 months compared with best supportive care, leading the investigators to recommend the use of chemotherapy. Given the high level of evidence (meta-analysis), this study led to the recommendation of these agents in clinical practice guidelines.

Silvestri et al. surveyed 81 patients who had received at least one course of platinum-based therapy. Patients were given two scenarios for a patient with incurable lung cancer who had an expected survival of 4 months without therapy. The two scenarios differed only by the description of the toxicity of the proposed regimens. The first described mild, tolerable side effects, and the second suggested severe side effects with a potential need for hospitalization and a 1% chance of death. Patients were then asked how much of an increase in survival over the 4 months they would want to take the chemotherapy. Overall, patients wanted 4.5 months improvement in survival for the low toxicity regimen and 9 months for the severe toxicity agents. Thus, for both scenarios, patient expectations exceeded the actual improvement expected based on the meta-analysis. In a separate question, patients were asked whether they would take a regimen with severe toxicity or chose supportive care if the regimen only improved survival from 4 to 7 months—the 3-month increment shown in the meta-analysis. In this scenario, 78% of patients chose supportive care.

This study highlights the difficulties of assuming that patient and physician goals are always congruent. Does the evidence support the effectiveness of chemotherapy if patient values indicate that the measure of effectiveness is not valid to the patient? These are difficult areas for guidelines developers. Most guideline efforts concentrate on the clinical outcomes. Probably this is appropriate in the majority of cases. Considerations of how clinical outcomes and cost outcomes interact can be very difficult, especially with the expensive technologies that are emerging. Determining appropriate outcomes in the context of patient preference is complicated even further by the heterogeneity of the cancer patient population. Another difficulty is that no systematic method for capturing patient preferences exists, and clinicians might legitimately question whether a guideline participant can speak for all patients from an “expert” vantage point.
Patients and Clinical Practice Guideline Development

Despite these difficulties, however, the idea that patients’ views should be represented on guidelines panels seems clear. A striking example of a sincere effort to achieve this goal is seen in the North of England guideline program.29 In an effort to ensure an open and on-going opportunity for a patient voice in developing asthma guidelines, the program activated four initiatives. First they appointed two patients to the panel. With no formal role designated to these patients, the developers found that they provided considerably less input than the clinicians. Therefore, a second step was to form a group of patients who reviewed the draft of the guideline and offered criticism and observations from their experience. They were especially active in providing guidance on education and counseling.

As a last initiative, the panel enlisted the membership of a leading consumer advocate, the head of a national patient asthma group, who could speak authoritatively about the needs and preferences of the asthma population. Because of the initial small involvement of patients in the development process, a workshop was designed to inform patients about medical jargon and explore ways that patients could achieve greater understanding of the guidelines. This group is frank in stating that although each of the four strategies has some advantages, the optimal plan for achieving patient input will require on-going testing and perhaps new approaches. At the same time, the group concluded that meaningful patient input into the guideline development process is possible.

The need to incorporate patients into the guideline process appears necessary to ensure that guidelines reflect a patient-physician partnership. This can become a reality only with a concerted effort to inject patient preferences regarding outcomes in the development process. The methods for achieving this difficult goal are certainly not well established, but we can hope that recognition of the need will lead to more conscientious efforts to achieve success.

References