

## Oncology Practice Guidelines: Do They Work?

One of the major premises supporting the development of clinical practice guidelines is that they promote increased consistency in health care practices.<sup>1</sup> A decrease in random variation in this care, we reason, will, in turn, lead to better patient outcomes, increased patient confidence and satisfaction, and more efficient use of resources.<sup>2</sup>

Is this premise correct? In a review of 59 non-cancer guidelines, Grimshaw and Russell<sup>3</sup> reported that 55 led to a change in medical practice, and 9 of 11 showed an improvement in patient outcomes. In a review of oncology-related guideline studies, Smith and Hillner<sup>4</sup> noted some evidence of the effect of guidelines and critical care pathways on decreasing costs, but pointed out the need for more data about other outcomes.

In attempting to answer the question of whether oncology guidelines “work,” we can assess a multiplicity of end-points:

- Have oncologists accepted the basic premise that guidelines are useful?
- Are oncologists aware that guidelines exist, and do they access them?
- Do the practice patterns of oncologists conform to standard guidelines recommendations?
- Does the implementation of guidelines change oncology practice behavior?
- Does the use of guidelines lead to better patient outcomes?

### Oncologist Acceptance of the Concept

In line with other specialties,<sup>5</sup> modern oncologists seem to acknowledge the utility of clinical practice guidelines. In a survey of Canadian oncologists,<sup>6</sup> 86% agreed with a statement that guidelines were a useful educational tool, and 83% believed they were a good source of advice, whereas only 4% of those surveyed disagreed with the statements. Additionally, only a minority of oncologists agreed with negative connotations of guidelines as “cookbook” medicine (26%) or a challenge to physician authority (16%).

A survey of Australian surgical oncologists revealed similar responses,<sup>7</sup> with over 78% agreeing that guidelines were useful sources of information about new research and a convenient source of advice. Given the goal of guidelines creators to assist in clinical decision-making,<sup>8</sup> 63.6% of these surgeons thought they gave confidence to decision-making and 55.9% believed they assisted in difficult cases. On the negative side, 44.6% believed guidelines were “cookbook” medicine, and a third believed they were dismissive of clinical experience and represented an elite group. These surgeons also voiced a concern that they would increase malpractice cases. These studies indicate that the balance has tilted towards acceptance, although work still must be done to deal with some persistent reservations.

### Oncologist Awareness of Guidelines and Access to Them

When guidelines are developed by major medical societies or groups, their content appears to be familiar to the appropriate specialists.<sup>9</sup> To assess its guidelines program, the American Society of Clinical Oncology (ASCO) surveyed its

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## Oncology Practice Guidelines: Do They Work?

membership to ascertain awareness and stated use of the program's guidelines.<sup>10</sup> This study indicated that five ASCO guidelines were widely known, with readership ranging from 76% for the growth factor guidelines to 55% for the tamoxifen/raloxifene statement. Of these readers, 76% to 83% agreed with the guidelines overall, with only 1% to 7% disagreeing.

An interesting issue in awareness is what type of guidelines attracts the attention of oncologists. A National Comprehensive Cancer Network (NCCN) analysis of hits on its guideline Web site indicated that a broad range of treatment algorithms were consulted by 50% to 80% of users (A. Brody; 2004, personal communication). These tumor sites included common tumors (breast 82%, colon 72%) and less common sites (esophagus 64%, bladder cancer 60%). Conversely, supportive care and screening guidelines were much less frequently perused. An average of 17 guidelines were accessed after a user logged onto the site. This suggests that after clinicians become acquainted with guidelines, they incorporate them into their list of resources.

### Conforming Practice Patterns to Standard Guidelines Recommendations

Based on the dissemination of guidelines developed by a diverse set professional sources such as the National Institutions of Health (NIH) Consensus process, NCCN, ASCO, and the Canadian Medical Society, one method of measuring the impact of guidelines is to survey broad swathes of the medical community to see if current practices correspond to widely accepted guideline recommendations. Unfortunately, studies in this area have used a variety of data sources and vastly different patient bases located in different geographic locations. Therefore, judging the true prevalence of a particular practice pattern is difficult, as is judging the impact of a guideline compared with the impact of other information sources.

This phenomenon is readily seen when looking at the most commonly investigated type of cancer, early stage breast cancer. In a study drawn from the New Mexico tumor registry from 1991 through 1997, only 47% of patients with stage II disease received cytotoxic therapy.<sup>11</sup> Similarly, a survey of surgical oncology practices in the Philadelphia area revealed that only 34.8% of eligible patients in the period of 1995 through 1999 received postoperative chemotherapy.<sup>12</sup>

These studies stand in stark contrast to a chart review of patients in Massachusetts and Minnesota. That study showed that cytotoxic chemotherapy was given to 97% and 94% of eligible patients, respectively, during 1993 to 1995.<sup>13</sup> A Canadian study showed a 96% adherence to chemotherapy for node-negative patients.<sup>14</sup> This marked discrepancy may be partially attributable to such factors as a true difference in the practice patterns of treating physicians or different patient preferences based on geographic setting. However, differences in patient selection and characteristics such as age, the selection of which physicians to survey, and the completeness and assiduousness of data also very likely account for these discordant findings.

Although most studies concentrate on breast cancer, some investigators have chosen to evaluate compliance with guidelines for less common malignancies. An evaluation of practice patterns for lip cancer showed that

## Oncology Practice Guidelines: Do They Work?

preoperative workup and treatment of patients over 60 did not adhere to regionally developed guidelines.<sup>15</sup> In a record audit of patients treated for soft-tissue sarcomas, conformity to guidelines for radiation, chemotherapy, and follow-up evaluation was in the 80% to 95% range, but surgical intervention conformed only 52% of times.<sup>16</sup> Only 7% of patients were evaluated by a formal multidisciplinary committee, which correlated with adherence to the guidelines.

A groundbreaking study looking at the adherence of practice patterns to supportive care guidelines was reported from Australia. In 1995, the Australian National Health and Medical Research Council released a comprehensive, evidence-based guideline regarding appropriate supportive care for patients undergoing treatment for breast cancer.<sup>17</sup> A telephone survey of 544 women was performed 6 to 12 months after treatment. The survey asked about psychosocial support as detailed in the guidelines.<sup>18</sup> For many parameters, the level of care was good to excellent. For example, the diagnosis was delivered in an open manner for 90% of women, 81% felt they had been given adequate information, and 73% felt their involvement in decision-making was at an appropriate level.

Some areas showed deficiencies, however. Only 56% of women reported being told that a 2-week delay in decision-making would not be harmful, and a mere 13% were encouraged to participate in a clinical trial. Because quality of life and patient satisfaction are two of the primary outcomes in cancer treatment,<sup>19</sup> more studies and efforts to facilitate adherence to guideline standards in this vital dimension should be a priority for researchers and policy makers.<sup>20</sup>

The National Initiative in Quality of Cancer Care (NIQCC), an important initiative in developing methodology for care evaluation, is currently being analyzed by a team of ASCO-supported investigators.<sup>21</sup> Researchers in this study have developed a large set of quality markers for breast and colon cancer, many based on ASCO guidelines, and have gathered as comprehensive a set of medical records as possible to see how the delivery of care conforms to generally accepted standards. With its precisely defined specifications, this initiative undoubtedly will provide invaluable information about how to assess patterns of care.

### Guidelines Impact on Changing Practice

The most completely documented assessment of the impact of introducing oncology guidelines was performed in the Rhône-Alpes region in France.<sup>22</sup> A set of guidelines for the management of breast and colon cancer were developed in a network of 11 public and 15 private hospitals. The guidelines were developed by modifying a set of national guidelines for regional use under the guidance of a local respected oncology leader. The guidelines were then distributed to all physicians in the area, but no other measures were used to promote their acceptance or use. The recommendations covered diagnostic and staging workup, primary and adjuvant treatment, follow-up evaluation, and management of metastatic disease.

The assessment consisted of two comparisons. The first compared adherence to the guideline recommendations at introduction and 2 years later. In addition, adherence at these two time periods was assessed in several hospitals in another region of France that did not have a formal guidelines program. This

## Oncology Practice Guidelines: Do They Work?

enabled the investigators to evaluate the impact of the guidelines as opposed to other temporal events that might have contributed to a change in behavior.

An overall assessment of adherence was performed by constructing a global score for each patient based whether the care they received conformed to the guideline for all pertinent areas of care. At the start of the program, care was totally in line with the standards for only 12% of breast cancer patients and 14% of colon cancer patients. Two years later, these numbers had improved to 36% and 46%, respectively. In the non-guidelines control hospitals, the baseline figures were 6% for breast and 32% for colon, with essentially no change in the follow-up evaluation (7% for breast and 36% for colon). The conclusion was that guidelines per se led to observable changes in clinical management. Further analyses of the adherence measures revealed that the most impacted areas of care were in diagnostic testing, adjuvant therapy, and follow-up testing.

Given the low global scoring, the team took an additional step and analyzed whether non-conformance to a guideline might conform to other evidence in the literature, for example, use of adjuvant epirubicin when the guideline called for doxorubicin. When conformity was graded as positive if the intervention conformed to the guideline or to evidence in the literature, the compliance figures improved substantially. However, the study continued to show improvement over time in the guideline program institutions and that they performed better than the facilities with no guidelines.

Another breast cancer study in the Netherlands evaluated the effect of audit, group level feedback, and education in improving compliance with guidelines.<sup>23</sup> The periods studied were 1988 to 1992 and 1996 to 1998. Significant improvements in adherence were seen for reporting over 9 axillary nodes, reporting estrogen-receptor and progesterone-receptor status, and increasing the dose intensity of adjuvant chemotherapy above 85%. The study based a quality program on the findings of an initial audit, showing the efficacy of sequential targeted interventions.

A Canadian study evaluated the impact of the promulgation of guidelines for small cell lung carcinoma.<sup>24</sup> The parameter assessed was the increase in concurrent chemotherapy or radiation therapy over a 5-year period. In 1990, the rate of concurrent therapy was only 17%, compared with 64% in 1995. Interestingly, the study highlights that the use of concurrent therapy involved a disruption of normal practice patterns in that patients received chemotherapy at the radiation centers rather than in the private oncologist's office, a factor that ordinarily would work against guideline acceptance.

An interesting example of disagreement in impact, possibly based on the use of different data sources, relates to the ASCO growth factor guidelines. A survey of American oncologists' use of these agents for primary prophylaxis was based on agreement with their use in varied clinical scenarios. In comparing answers with those on surveys performed in 1994 and in 1997, 3 years after introduction of the guidelines, researchers noted a decrease of inappropriate use in three of four scenarios.<sup>25</sup> A French hospital study that implemented the ASCO guidelines used chart reviews to assess change in usage patterns. Although overall guideline compliance improved compared with control institutions, usage for primary prophylaxis was very low in both periods; 12% and 6%.<sup>26</sup>

## Oncology Practice Guidelines: Do They Work?

These studies—diverse in methodologies, practice settings, size, scope, and geography—begin to lend some credence to the impression that introducing guidelines can, in fact, influence physician behavior and bring practice patterns more in line with the recommendations of well-grounded guidelines. This impact is admittedly spotty, and complex factors are obviously at play in determining whether a specific intervention is adopted or continues to be rejected. As in all areas of oncology, further well-controlled studies are vitally needed to begin to discriminate what these factors are and how they can be accommodated.

We should also note that the vast number of studies have been performed in Europe and Canada. This is an exciting area of health services research and, hopefully, as young oncologist investigators in the United States plan their careers, this will become an area of vibrant interest.

### Guidelines Impact on Patient Outcomes

In the final reckoning, the justification for introducing clinical practice guidelines will be the finding that their use leads to improved outcomes for patients. This factor defines the validity of a guideline and links the process of care to outcomes. A prime example of the validation process is shown by the “Ottawa Ankle Rules,” a guideline developed to detail the physical findings that indicate the need for radiographic examination in patients with injured ankles. These guidelines have been evaluated in many clinical settings and consistently show that they are very sensitive in identifying patients with fractures and are reasonably specific,<sup>27</sup> leading to patients undergoing radiographs when needed and often avoiding them when they are not called for.

Unfortunately, these studies have not been performed on any large-scale systematic basis for oncology patients. A small Florida study showed no difference in survival or quality of life between patients whose treatment complied with the NCCN guidelines versus those whose management did not (although decreased cost was seen for the conforming group). However, the study was limited in sample size, making conclusions difficult.<sup>28</sup> An example of this type of validation study was a randomized trial of a decision support system based on the AHCPR Guidelines for Cancer Pain Management, which showed that treatment of patients using the algorithm led to a significant reduction in pain intensity.<sup>29</sup>

Designing these new large-scale studies will not be easy. Researchers will need to pay scrupulous attention to how compliance is measured, what outcomes are most meaningful to assess, and how study populations are selected to eliminate patient and disease factors that would bias the outcomes independent of guideline adherence. Although breast cancer guidelines will undoubtedly be the most frequently examined, diseases in which adherence to guidelines may have a marked influence on outcomes such as lymphomas and leukemia also should be chosen for study, probably requiring larger groups of clinicians to provide enough patients.

### Do Clinical Practice Guidelines Work?

This brief look at the evidence makes it fairly clear that much more work is needed before we can definitively answer this question. But the early signs are favorable: oncologists appear to recognize the potential usefulness of guidelines

## Oncology Practice Guidelines: Do They Work?

and, to a large degree, read them when they become available. When guidelines are introduced into formal programs, some evidence suggests that care conforms to the recommendations over time. That this translates into benefit for cancer patients remains largely unproven, but so far the signs are hopeful. We all look forward eagerly to the studies that show if our hypotheses are correct.

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## Oncology Practice Guidelines: Do They Work?

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