A Model Is a Model: A Decision Analysis for Rectal Cancer

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Many years ago, when I was a medical intern, I had a patient who presented with a very early stage breast cancer. The resident wished to get a bone scan on the patient. Given the very low risk of a positive test, I inquired whether this was really necessary. His response was, “Well, what if it is positive?” Although not usually stated that bluntly, many of the decisions we as clinicians make are often based on a similar low level of clinical insight into the possible benefits of a procedure. Clinicians are routinely faced with the question of how much should be done to a cancer patient. The question is faced at all phases of patient management—with components of a physical examination, treatment, or, as discussed by Telford et al. (1) in this issue of the Journal, initial evaluation of the patient with cancer. Unfortunately, there are often minimal data to provide objective guidance in the initial evaluation. More diagnostic studies clearly supply more information. However, sometimes this additional information is not useful, and sometimes it is downright confusing. More information may lead to more tests and greater uncertainty for the clinician regarding proper patient management.

The article by Telford et al. (1) attempts to address this issue for patients with rectal cancer. The title is somewhat misleading, because the article does not address whether staging should be done; instead, it addresses whether endorectal staging, usually by ultrasound, of the primary tumor and regional lymph nodes is clinically useful when added to conventional staging of blood tests, chest x-ray, colonoscopy, and abdominal–pelvic computed tomography scan (all of which are assumed to show no evidence of metastatic disease).

How does one decide which studies to perform? Most clinicians would agree that pre-therapeutic evaluations should be designed to determine the best therapy (in order to improve tumor control or decrease morbidity) or to provide important prognostic data. Therapies to improve tumor control are comparatively easier to establish from randomized clinical trials demonstrating that one therapy is superior to another. However, therapies that reduce morbidity are not so simple to identify, because morbidity is the result of a combination of effects that physicians routinely think about and analyze (e.g., peri-operative infection, operative mortality, or severe diarrhea) and of subjective factors that are more difficult to quantify, such as the impact of living with a colostomy, occasional soilage, fatigue, and issues related to self image. Telford et al. (1) have used Markov modeling, taking many of these factors into consideration, to answer the question of how local tumor staging influences outcome. They have made a valiant attempt to answer the question and should be applauded for tackling this difficult issue. However, it is not entirely clear that they have been successful.

Is it possible to make such a model valid? And are the results believable and useful? Because their analysis supports many of our preconceived biases, we think their results are believable. If preoperative therapy increases survival more than postoperative therapy in selected patients, then full preoperative staging to identify such patients should be the preferred management strategy. Unless there were major toxicity differences between the approaches, the results could not be otherwise.

It is, however, much less convincing that the results are useful. The authors properly make no assumptions that pre- or postoperative chemoradiation therapy produces better survival. Because the utilities within the Markov model do not reflect the opinions of individual patients, are the authors suppressing individual patient preferences by performing modeling experiments that use average utilities related to subjective patient factors? How risk averse is a patient so that he or she would, for example, accept a higher risk of tumor recurrence by having a local excision compared with a more extensive operation? How strongly does the patient wish to avoid a colostomy? And would avoiding a colostomy be traded for increased toxicity? Individual patients may, and in fact do, have strongly divergent opinions on these issues. Consequently, is the modeling by Telford et al. (1) relevant for an individual patient? Or could these results be used as an excuse for not delving into the desires and needs of each patient? We are certain that the authors would state that their results should not be used in isolation. However, in a model that is sensitive to small changes in these utilities, it is hard to know how to use these results in clinical practice.

Indeed, their model is sensitive to individual utilities. For example, altering the utility for having a colostomy would likely have a substantial effect on the outcome. Clinicians must also consider how inconvenient, uncomfortable, and/or expensive the staging study is in determining whether it is worthwhile. It is easier to justify performing a simple and inexpensive test than one that is difficult for the patient and expensive. These issues are not addressed by Telford et al. (1). Physicians need to use the tools that are available in the context of the individual patient to determine how each patient should be treated. If a rectal examination by an experienced observer demonstrates a circumferential tumor that is clinically fixed, the incremental value of ultrasound will be quite low. Decisions should be made in the context of understanding how perturbations of the model, because of individual variations, will affect the results.

At the present time, management of rectal cancer by experienced physicians is becoming more individualized. Local excision, despite the assumptions made by Telford et al., is being used less commonly in the United States than previously. Adjuvant radiation therapy is not given to all patients with T3N0 tumors (especially those located high in the rectum and operated...
on by experienced surgeons), and its benefit is being questioned for selected patients with T2N1 disease. We believe that this individualization of patient management is good, but it raises difficult questions about designing studies that define optimal management in subsets of patients. This difficulty with subsets is magnified when dealing with modeling studies such as the one by Telford et al. (1), in which the uncertainties are multiplied.

Despite the concerns expressed above, Telford et al. (1) should be congratulated for approaching the difficult and important issue of the role of staging procedures and their influence on outcome. Their study and others like it should be read carefully so that the clinician understands how perturbations of the model are likely to affect outcome. Then, the physician, in consultation with the patient, should use clinical judgment to define proper management. We must remember that models are not reality. They are models of reality that depend on the accuracy and completeness of the data used, and that an important component of this information is subjective. Models cannot substitute for clinical judgment that is based on a patient’s wishes and needs, as well as on clinical variations in individual tumors. However, models can provide a framework on which intelligent clinical decision making can be developed.

**Reference**