

## The Five Options: State-of-the-Art Choices in Medical Therapy

*For any severe illness, I've always dreamed of the ideal therapy: curative in a single dose, this concoction could be mixed with a spoonful of chocolate syrup, and—of course—not cause any side effects. But the harsh reality of many effective medical therapies today is far from that dream.*

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While the topic of health care economics is grabbing all the headlines, an important change has taken place in the patient-doctor relationship, at least in my practice. Regardless of who pays for what, cultural and technical factors at the beginning of this century also profoundly affect a patient's decisions. Developments in patient sovereignty, media exposure, new expectations, information technology, new drugs, medical technology, medical ethics, standards of scientific evidence, and choices of alternative practices now intersect to affect the process of making treatment decisions.

The result of all these developments is that patients with serious illness are faced with a more confusing array of choices than ever before. To simplify, I sort the choices into five basic categories of options. (Cancer therapy is the paradigm here, but the principles could be applied to any other serious illness.) My experience has shown two things: 1) Even when attempting a cure is futile, interested patients still want to thoroughly explore possibilities; and 2) presenting the possibilities as five options provides a framework for discussion with the patient.

### Option 1: "Standard" Medical Care

The first option—"standard" medical care—is elusive and evolving. Therapy plans in this category can be culled from published medical and scientific research or from consensus statements of professional groups; these therapy plans usually have established efficacy or may be in widespread use before efficacy is conclusive. The therapy might include medication, surgery, or radiation therapy—either alone or in combination.

Nutritional, psychosocial, and physical effects of the illness are all considered in standard care. Standard therapy has a proven track record, predictable rates of success and failure, and known side effects. In considering whether to use standard therapy, the physician and patient may discuss evolution of the therapy, equivalent approaches, and failed therapies.

Patient expectations rise after a particular type of

therapy has received media or marketing exposure. To put these expectations in a realistic context, patients must learn that the use of some new drugs and technologies becomes widespread through effective marketing or through extensive media exposure instead of through rigorous science. Patients should know also that American medicine can be as subject to fads as the rest of the culture.

### Option 2: Clinical Trial Therapy

The most convincing scientific way to prove efficacy of a given therapy plan—and ultimately to improve clinical outcomes—is to compare therapy plans (eg, standard vs experimental therapy) directly. When available, therefore, the second option for patients is clinical trial therapy. Patients who choose this option are assigned to treatment groups in a statistically random way. These "Phase III" trials usually use well-tested therapies in new combinations. Formerly the exclusive province of academic medical centers, Phase III clinical trial therapy can now be offered through the private sector.

For many patients, statistical randomization is a difficult concept to accept. It means that neither patients nor their doctors will choose the therapy and that a mathematical model (or computer program) instead will assign the therapy. This procedure is both ethically acceptable and scientifically imperative and is an important way to provide unbiased evidence to advance medicine.

Clinical trials must be approved by local ethics boards or human subject protection committees because patients on a new therapy may have wonderful outcomes, terrible side effects, or both. Patient participation is voluntary, and protections—including the right to withdraw from the program—are integral to the required informed consent.

### Option 3: Experimental Therapy

The third option is "Phase I" or "Phase II" experimental therapy, available for drugs whose efficacy has not been proved. These trials, too, are ethical trials offered on a voluntary basis, but eligibility for these forms of therapy is usually extremely restricted: Most are offered only to patients who have relapsed or for whom other therapy has failed. In these trials, the investigator hopes for treatment effectiveness but emphasizes to patients that side effects may be the only results.

More so than in other trials, Phase I clinical trials



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are positioned at the frontier of the unknown. These trials are used to establish a human track record for a given therapy; therefore, Phase I therapy may be used for the first time in humans. The trial may also maximize doses with the explicit goal of monitoring unforeseen toxic effects. Phase I clinical trials lay the foundation for Phase II studies, which establish whether the given therapeutic agent has any effectiveness in human disease.

**Option 4: Nonmedical (Alternative) Therapy**

The fourth category of therapy options is nonmedical therapy. This category may include naturopathy, homeopathy, or other alternative therapy. Respect for a patient's autonomy is at the core of this option. Patients may use various criteria when evaluating this option in comparison with evidence-based medical treatment options, because relevant data for conventional analysis may not exist.

Many patients use nonmedical therapy to "complement" medical therapy. Because an alternative therapy (eg, use of antioxidant substances) may inhibit standard therapy,<sup>1</sup> open discussion between doctor and patient is important to achieve therapeutic goals.

**Option 5: Palliative Therapy**

The fifth therapy option is to choose active palliation and to withhold therapy that has a curative intent. Given that patients have a sovereign right to make determinations about their own care, this option can be valid. Moreover, even when this option is not the main recommendation, mentioning the option facilitates discussion of the natural history of untreated disease and can lead to frank, important discussions of advanced directives for the end of life or palliative and hospice care. This discussion may be associated with even more ethical and legal considerations for younger patients.

In the "old days," doctors would choose therapy without much patient input; that practice was the norm. Some academic institutions even trained their charges to offer clinical trial therapy only, because assigning patients to clinical trials was standard practice at those institutions. Some patients still prefer that mode of making treatment decisions.

**Treatment Options Empower Our Patients**

Nowadays, however, clinicians must understand their patients' values, cultural mores, and therapeutic goals. When faced with serious illness, a patient

may feel that her or his own choices vanish. Providing information about options doesn't obviate the physician's role in making a recommendation but does provide an opportunity to empower patients to assert their right to choose.

Sharing the options with patients and entering into conversations about values—whatever the ultimate choice—usually leads to a more thoroughly informed consent to treatment. In turn, this result can lead to a better therapeutic alliance and partnership between doctor and patient. Even with all the raw medical information available on the Internet and in other media, patients who come to an office visit equipped with voluminous printouts still want a physician to "walk them through" the available options.

No patient or doctor can ignore the monetary implications of these choices. These implications are part of the real-world equation as are some doctors' research motives, institutional motives, or health plan restrictions.

In the future, perhaps therapy will emerge to suit each patient's individual needs. In the meantime, the tasks for both doctor and patient have expanded: Both must consider five categories of possible choices while we all wait for that magical spoonful of chocolate syrup. ❖

Reference

1. Labriola D, Livingston R. Possible interactions between dietary antioxidants and chemotherapy. *Oncology (Huntingt)*1999 Jul;13(7):1003-8; discussion 1008, 1011-2.

**How do I present these five treatment options? After the diagnosis of cancer is confirmed, I hold a family conference which may last at least 90 minutes. At these conferences, one can expect shock, questions, and tears—then a discussion about feelings, particularly those of fear, blame, and guilt. When I begin teaching about the disease itself, I introduce the idea of treatment options.**  
**I find it useful to distribute an Internet printout about the disease. The National Cancer Institute provides an objective source via its Web site (<http://rex.nci.nih.gov>). With this printout in hand, I begin talking about the "five ways, or options, we can use to treat this disease." Then I deliver my favorite phrase: "I'm a doctor, not an insurance man, so let's talk about these options first and talk about coverage later."**  
**Later, if a patient relapses, I reiterate to that patient the availability of the five options. If possible, I also give the patient a relevant printout from the National Cancer Institute's clinical trials Web site (<http://cnetdb.nci.nih.gov/trialsrch.shtml>). Regardless of the patient's choice or treatment outcome, a key value of our clinical program is to continue monitoring the patient. To that end, we help families navigate referrals for experimental or alternative care and for hospice care when necessary.**