

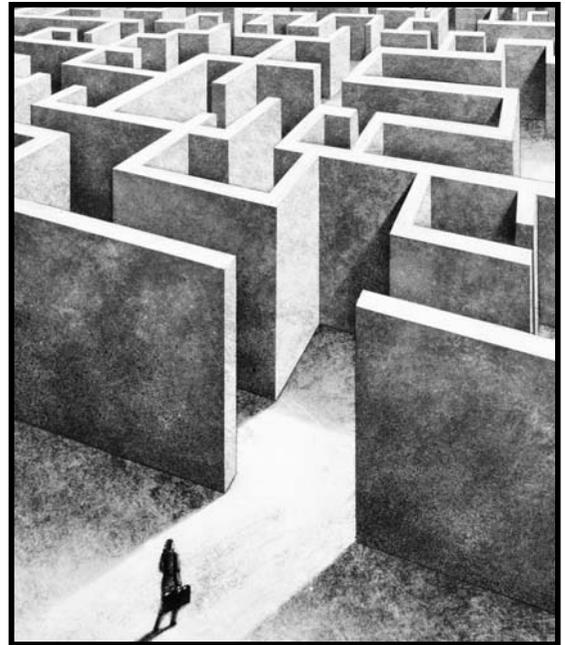
BY GARY S. BECKER

A well-known physician who was suffering from a serious illness once told me not to take too seriously what doctors tell patients they would do if they had the patients' diseases. After his illness was diagnosed, he became convinced that no doctor, no matter how experienced and well intentioned, could really put himself or herself in a patient's shoes.

Treatment of serious diseases usually offers options that trade off various risks, for example, forcing comparisons between the likely quality of life and its expected length. No one is really able to read the thoughts of others well enough to make informed decisions on their behalf. This is why patients – not regulators or even physicians – should have ultimate control over treatment.

I mention regulators because government authorities, in particular the Food and Drug Administration, make it extremely difficult for the very ill to gain access to unproven therapies. This barrier became more daunting when the FDA instituted regulations in 1962 that significantly raised the cost and lengthened the time it takes to bring new drugs to market.

Before that year, pharmaceutical makers had to show only that drugs appeared to be safe for the vast majority of patients likely to take them. Assuring safety required clinical trials along with other evidence and was not an easy obstacle to overcome. But I do not want to argue here the issue of how safe is



safe enough, and I assume that an FDA safety standard, perhaps even a strengthened one, is desirable.

The 1962 regulations, however, went beyond safety to add an efficacy standard. That is, clinical trial evidence would from then on have to strongly support claims that products significantly aid in the treatment of specific diseases or conditions. Indeed, in the final stage of mandated clinical tests, randomized

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trials must show that those treated with a drug are significantly helped compared with a control group.

This efficacy test greatly lengthened the average time between discovery and approval. Although in recent years the FDA has maintained a “fast track” for high priority drugs, bringing a new therapy to market takes an average of 12 to 15 years. The typical drug must be tested on some 6,000 patients, increasing

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total development costs by about 40 percent.

It follows that a return to a safety standard alone would lower costs and raise the number of therapeutic compounds available. In particular, this would include more drugs from small biotech firms that do not have the deep pockets to invest in extended efficacy trials. And the resulting increase in competition would mean lower prices – without the bureaucratic burden of price controls. In turn, cheaper and more diverse drugs would induce insurance companies and public providers to cover many more new drugs, even when their efficacy was uncertain.

Elimination of the efficacy requirement would give patients, rather than the FDA, the ultimate responsibility of deciding which drugs to try. Presumably, the vast majority of patients would continue to rely on the opinions of physicians about which drugs to use. But many people whose lives are at risk want

to believe that they ultimately make the decisions – even when they essentially follow the experts’ advice.

To be sure, some sick individuals would try ineffective treatments that would otherwise have been prevented from reaching market under present FDA regulations. But the quantity of reliable health information now available with only a little initiative is many times greater than when the efficacy standard was introduced four decades ago. WebMD and other Internet sources, along with better and more extensive health reporting by the news media, offer medical consumers superior access to information than even their doctors enjoyed not long ago.

As part of any relaxation of the efficacy standard, the FDA could further facilitate public access to relevant information. For example, it could allow drug labels to list separately claims that are supported by clinical evidence and those that are not. And it could be proactive in reporting what is known about the value of drugs in treating diseases, making data available through the Internet and other consumer-friendly media.

One of the more depressing aspects of serious disease is the sense of impotence – that very sick persons can do little to help themselves. This may explain why placebos sometimes generate positive effects. Indeed, Anup Malani of the University of Virginia found that patients receiving placebos in double-blind trials of ulcer drugs reported significantly less pain than they had before the trials.

Giving people whose lives are threatened by serious diseases greater access to safe, promising (albeit unproven) drugs and other treatments would help their psychological state. More important, it would lower the cost and hasten the development of therapies that could really make a difference. **M**