Are all treatment alternatives for a given condition equally effective?

There are multiple treatment alternatives for most medical conditions. If you have high blood pressure, there are six major drug classes from which you could choose: diuretics, alpha-blockers, beta-blockers, ACE inhibitors, calcium channel blockers and angiotensin receptor blockers. Included within each of these medication classes are numerous individual medications, and more than 100 medications are approved for treatment of high blood pressure. You and your doctor are then left to decide which antihypertensive drug is best for you.

**How do I know which treatment is most effective?**

As stated in Chapter 2, it is uncommon for one treatment to be much better than the alternatives. In fact, it is more common for leading medications to be fairly equal. Unfortunately, there is no regulation requiring new medications to be compared to existing treatment alternatives before they are approved. The FDA prefers new medications to be compared to placebos in order to document efficacy and safety with certainty (see Chapter 27).

As a result, there are few good clinical trials comparing the major alternative drugs for a given disease. Instead, marketing of medications focuses on ease of dosing, clinically unimportant biochemical markers, and surrogates that may reflect important benefits to patients but have not been validated. Pharmaceutical companies appear to be less than enthusiastic about participating in comparative trials. In any direct comparison of two medications, there might be one winner and one loser. Being inferior to a competitor’s medication could be a major financial setback, translating into enormous losses of future profits. Not participating in a comparison and not knowing how one medication compares to another may be preferable from a business viewpoint.

**How reliable are comparative trials?**

Comparative trials are indeed being conducted. In particular, medications that are late entries into the market are sometimes compared to earlier established ones. Emphasis is often placed on some supposed unique feature of the newer medication. The major problem with industry-sponsored comparative trials is that many of them may not be fair comparisons. The sponsor of a comparative trial (usually a pharmaceutical company) has the advantage of being able to
select the comparator medication, its dose, measures of efficacy and the timing of these measurements. The sponsor may take advantage of this opportunity by tailoring the trial in favor of its own product. A recent article reported that 90 percent of 33 trials comparing two treatments for schizophrenia favored the sponsor’s drug. To illustrate this point further, there were nine trials comparing two leading medications. The manufacturer of one of these drugs sponsored five trials, and all five reported better results for the sponsor’s drug. Four trials were sponsored by the other manufacturer and three of them favored that manufacturer’s drug. What should consumers believe?

Comparative trials are complex, and their findings may be difficult to interpret due to the selection of comparator and its dose, outcome measures, and when these are assessed. Many of these methodological issues go beyond what physicians, in general, appreciate.
Which medication is best for me?

The answer depends on your reason for taking the medication. If the purpose is to alleviate a symptom, you may be the best judge. You would have to try several alternatives until you find the one most suitable for you (see Chapter 25). For prophylactic medications (preventing complications of a disease), the situation is very different. You should consult with your doctor and discuss your treatment options and, among those, select a well-documented one most likely to benefit you.

Key messages

✔ There are multiple treatment alternatives for most diseases.
✔ More often than not, there is no clearly preferred choice.
✔ Although clinically needed, there is a lack of good, reliable comparative trials.
✔ The existing comparative trials can be difficult to interpret.
✔ The sponsor of comparative trials can influence results so that its product emerges as “the winner.”