Why is benefit-to-harm balance essential to treatment decisions?

The 1962 amendments to the U.S. Federal Food, Drug and Cosmetic Act require that for a new drug to be approved for marketing, there needs to be substantial evidence of both safety and efficacy when the drug is prescribed for its intended indication(s). In other words, a drug has to have beneficial effects that outweigh any potential harm; it has to have what is known as a favorable, or positive, benefit-to-harm balance. This is also true of other types of interventions such as medical devices and diagnostic procedures.

What are the goals of treatment?
In general, there are three main goals of treating a patient:
- to make the patient feel better
- to reduce the risk of future disease complications
- to improve survival

There are those who include a fourth goal, “economic benefit,” both to the patient and to society, e.g., returning to work, supporting family, paying
taxes, reducing future demands on the healthcare system. Our view is that economic benefit represents a natural consequence of reaching one or more of the three main goals.

Although a particular treatment might be effective, it may not necessarily achieve all three goals. A painkiller or a drug for nausea might instantly improve a patient’s well-being, but it would not be expected to bring any long-term benefit. In contrast, a drug to treat hypertension may reduce the long-term risks of cardiovascular complications and premature death without any tangible benefit to the patient, since most people with high blood pressure are asymptomatic. Some interventions may achieve all three goals. Effective antibiotic treatment of acute bacterial meningitis relieves symptoms, reduces the risk of neurologic complications, and decreases short-term mortality.

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The goal of the treatment is for you to take one pill three times a day.

How is the benefit of a treatment documented?
Controlled clinical trials designed to determine whether a therapy prolongs life or reduces the risks of major non-fatal complications typically require thousands of study subjects treated for years. Diseases with very high complication rates or high mortality such as subarachnoidal hemorrhage or