INTRODUCTION

Decision trees are developed to support physicians who must make treatment decisions. Risk estimates are used to find the optimal treatment pathway for a group of patients. Unfortunately, decision trees often are developed in the absence of empirical evidence concerning risk. In particular, long-term risk is almost always unknown. Instead, physician panels are convened, or physician groups are surveyed to give estimates of risk.

However, these outcomes databases discussed in this text can be used to investigate risk, and the relationship of treatment to outcomes. This relationship can be translated into percentages of risk, and that risk used to develop decision trees. Risk versus benefit can be used to find optimal treatment. However, patient benefit is subjective. Pain, especially, is very subjective. Is a patient better off to have surgery to relieve pain, or to just take pain medication continuously? There are attempts to define patient benefit as a function of the patient’s utility. To save costs, should treatment be denied if it fails to increase a patient’s utility? Who should decide a patient’s utility? Often, the patient has little input into the definition of utility that is often used to deny treatment.

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BACKGROUND

Use of Decision Trees

Recently and with increased importance, decision tree models are used in cost effectiveness, or comparative effectiveness studies. These studies examine different treatments or medications and determine which ones are the most cost effective. These studies take patient quality of life into consideration and often use estimated probabilities of effectiveness. (Berner, Kriston, & Harms, 2006; M. M. Brown, et al., 2009; Leszczynska-Gorzelak, Laskowska, & Oleszczuk, 2001; Norris, et al., 2007) These studies are based upon the perceived patient’s utility given the disease burden. The QALY, or quality of life-adjusted years is an estimate of the number of years of life gained given the proposed intervention. Each year of perfect health is assigned a value of 1.0. A patient in a wheelchair is given a correspondingly lower value; this value is not clearly defined and is rarely based upon patient input.

Perfect health is very hard to define and less than perfect health is often defined as a value judgment. A person in relatively good health may assign a very low value to a wheelchair-bound life whereas the wheelchair patient may consider their quality of life to be quite good. Attempts to measure perfect health focus on TTO, or time trade-off. Individuals, usually in good health, are asked to choose between a state of ill health for a period of time, or to be in perfect health for a shorter period of time. Such a trade-off is problematic because it is generally hypothetical. It is far more typical, particularly for a chronic disease, for a patient’s health to deteriorate gradually, allowing the patient to adjust to the change in health status.

The second method is the standard gamble (SG). Some studies have compared the two techniques without addressing the hypothetical nature of the responses. (McNamee, et al., 2004; Puhan, et al., 2007) Respondents are asked to choose between remaining in a state of poor health, or to choose a medical intervention that has a risk of death, assuming that the intervention also has a likelihood of restoring them to perfect health. The final method is the visual analog scale (VAS). Respondents are asked to rate a state of health on a scale from 0 to 100 with 100 representing perfect health. Again, these two remain hypothetical and do not necessarily capture the true choices facing patients. For this reason, there are some serious concerns as to the validity and reliability of the entire use of this methodology to define the concept of “perfect health”. (Arnesen, Trommald, Arnesen, & Trommald, 2004; Bleichrodt, et al., 2003; Dolan, Stalmeier, Dolan, & Stalmeier, 2003) Another thing that has to be considered is whether it is the individual’s needs that should be paramount, or society’s concerns. If society should be paramount, then individuals can be denied care if it is considered too costly. (Burstrom, et al., 2006)

Moreover, when such a choice becomes real, the patient often does not have a choice. Such is the case of the removal of the drug, Vioxx, from the market. It was a drug that improved the quality of life for many with arthritis while increasing the risk of heart disease. A study of hand transplantation shows just how far apart patients and physicians can be in terms of the perception of quality of life. (Edgell, et al., 2001) In this study, patients were overwhelming in their support of hand transplants; transplant surgeons were overwhelming in their rejection of hand transplants. The surgeons believed that prosthesis provided sufficient utility without the risk from medications required after transplant.

These definitions of QALY can cause some long-term problems when treatment is denied because the cost of each QALY is considered to be too high. Britain, in particular, has been particularly aggressive in denying such treatment. The organization, National Institute for Health and Clinical Excellence (NICE), has set a standard restricting the cost per QALY to an upper limit (approximately 20,000 pounds), and