Principles of Pharmacoeconomics

Measuring Patient Outcomes for Use in Economic Evaluations

Introduction

- An economic evaluation looks at all the implications of deciding to choose one way of providing care over another, not just the costs. **This means that any effect to the service, good or bad, has on the patient or customer needs to be investigated** .
- **Benefits, outcomes and consequences refer to the effect on the patient, not the effect on people providing the service. Cost is not an outcome measure.**
- This lecture defines and describes the main categories of outcome measure used: **effectiveness**, **quality of life**, **utility**.

1-Effectiveness

- Effectiveness is the **outcome of an intervention or service measured in natural units.** These can be general outcome measures, such as:
- -Cases successfully diagnosed.
- -Cases successfully treated.
- -Life years saved.

It is also possible to use clinical indicators, such as:

- -Number of asthma attacks avoided
- -Pain-free days
- -Change in infection rate
- -Percentage reduction in blood pressure
- -Effect on nausea and vomiting frequency.

- example, a study of **lipid-lowering therapy in the prevention of coronary heart disease** could use the drop in plasma cholesterol level to assess the effectiveness of this intervention. It is assumed that by **reducing a person's cholesterol level, the risk of developing coronary heart disease is reduced**.

Effectiveness versus efficacy

- **Efficacy** is **the consequence (benefit) of a treatment under ideal and controlled conditions** (ideal trial study). However, real life does not behave like an ideal trial study. In practice, **different types of patients** from those in the trial may receive the intervention.

- **Effectiveness is the therapeutic consequence of a treatment in real-world conditions**. The *effectiveness of a treatment or service is often **lower** than its efficacy*, and so using information from an ideal clinical trial may ***overestimate*** the impact of the intervention.

Limitations of effectiveness measures

A problem with effectiveness measures is that:

- **A-There may be more than one outcome reported for a particular treatment or service**. For example, an analgesic may be more effective than another at relieving pain, but cause more nausea.

- B-Many effectiveness measures **are disease specific,** such as lung function, and so cannot be used to compare outcomes for different disease states. For example, it would not be possible to use lung function to assess the outcome of an operation for a ruptured Achilles tendon.

Mortality used as an effectiveness measure

- Mortality has been used to measure the effectiveness of treatments in patients. Examples of this are the studies looking at the use of **aspirin after myocardial infarction**, **lipid-lowering agents in coronary heart disease**, and the **treatment of hypertension in patients with diabetes**. Mortality is a useful outcome measure because it is **objective** and easy to measure.

- However, there are **problems** associated with it.
- **People may die from causes other than that of interest to the study**, which can mask mortality linked to the intervention of interest and thus confound the results.
- **Most illnesses affect quality of life rather than mortality,** and so quality of life improvements due to interventions will not be detected or included in the economic evaluation.

- Third, **mortality requires a study with many patients followed up over a long period of time**.
- People of **different ages and sex have different risks of mortality**, so it is important that patient groups have similar age and sex profiles if they are to be compared.
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2-Quality of life

- Most modern medicine improves quality rather than quantity of life .
- **Definitions**: it may be helpful to distinguish between the terms quality of life (**QoL**) and health-related quality of life (**HRQoL**). The first term, QoL, is a broad concept with many aspects that measures people's overall perception of their life. QoL includes both health-related and non-health-related aspects of their lives (e.g., economical, political, cultural). HRQoL is the part of a person's overall QoL that "**represents the functional effect of an illness and its consequent therapy upon a patient, as perceived by the patient.**"

- A broad definition of health proposed by the World Health Organization more than 50 years ago is: "**Health is a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity.**".

Measuring QoL

- Measuring QoL is methodologically complex. There are many functional, social, psychological, cognitive and subjective factors that affect QoL.
- QoL measures can be divided into **generic and disease specific.**
- **A-Disease-specific measures** have been developed for patients with **chronic diseases**; such measures can only be used **to assess patients or treatments within those disease states**.

- Examples of specific areas investigated with disease-specific questionnaires include **nausea and vomiting for cancer treatment**, and **range of movement for arthritis treatment.**

B-Generic measures

- are more useful when looking at groups of patients who may have different illnesses, and can be used to compare outcomes in different patient groups. This looks at:
- **Physical functioning**
- **Physical role**
- **Body pain**
- **General health**
- **Vitality**
- **Social functioning**
- **Emotional role**
- **Mental health**.

3-Utility

- (**Utility is a measure of the relative preference for various options**). Utility is the value attached by an individual to a specific level of health or a specific health outcome. Different individuals may attach different values to the same health state. For example, some people may be prepared to tolerate a lot of nausea to allow them to be pain free. Others may prefer to tolerate more pain and reduce the level of nausea. **The important concept here is that utility measurement allows patients to value their health status based on their own preferences**.

- The specific methods used to derive utility are complex and are still under development. They are **preference-based**, which means that they allow individuals to indicate the direction and strength of their preference for a particular health state.
- Attaching values to health states can be carried out using **standard gamble** **or** **time trade-off methods**, **or a** **rating scale.** The last is rarely used.