# MEASURING PATIENT OUTCOMES FOR USE IN ECONOMIC EVALUATIONS - I

Dr. Muhannad R. M. Salih
B.Sc, M.Pharm, Ph.D
Pharmacy Department
Al-Rasheed University College
muhanad_rmk@yahoo.com

# 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 the service, good or bad, has on the patient or customer needs to be investigated.

# INTRODUCTION

Cost is not an outcome measure.

In order to assess the benefit of healthcare, correct identification and measurement of patient outcomes is essential in health economics.

An understanding of these outcomes is a prerequisite for economic evaluation.

# INTRODUCTION

This lecture defines and describes the main categories of outcome measure used:

- Effectiveness
- Quality of life
- Utility
- Expressing benefits as monetary values
- Appraises the importance of using the appropriate outcome measure

# 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*

# EFFECTIVENESS

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*

# EFFECTIVENESS

These measures are relatively simple to use and are often reported in clinical trials of interventions.

Therefore, they are the most common type of outcome measure and the most frequently used in economic evaluation.

They are sometimes called **intermediate outcome measures** because there is the implication that changes in them will extrapolate to an effect on the **patient's ultimate health status**.

# EFFECTIVENESS

For example, a study of diet-based 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.

We know that this assumption is true because there is epidemiological evidence to support the link between cholesterol levels and coronary heart disease.

# EXAMPLE OF EFFECTIVENESS

Table below reports the results from a trial that compared daily recombinant human deoxyribonuclease (rhDNase), alternate-day rhDNase and hypertonic saline for treating children with cystic fibrosis. The **primary outcome measure** was forced expiratory volume in one second (**FEV-1**, a measure of lung function). The results show that rhDNase was more effective at improving lung function than hypertonic saline, but there was no difference between the two different dosage schedules for rhDNase.

**Table 4.1** Clinical effectiveness of treatments for children with cystic fibrosis (adapted from Grieve *et al.*, 2003)

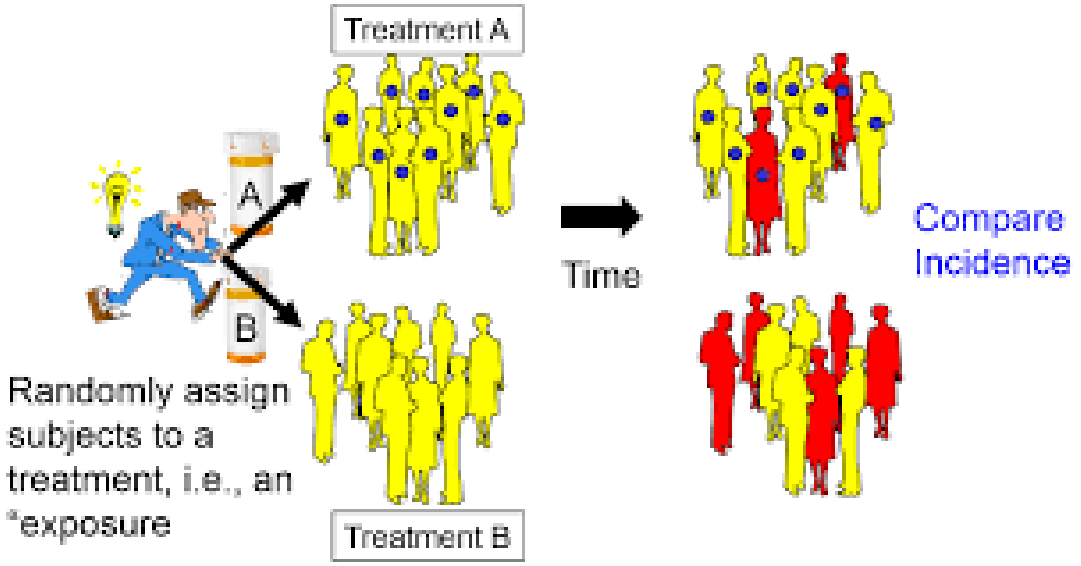
| *Treatment* | *Daily rhDNase* | *Alternate-day rhDNase* | *Hypertonic saline* |
|---|---|---|---|
| Percentage improvement in $FEV_1$ (SD) | 14 (27)* | 12 (19)* | 0 (27) |

*$p < 0.05$ compared with hypertonic saline.

# 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 VERSUS EFFICACY

**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.

Using information from an ideal clinical trial may overestimate the impact of the intervention.

Many researchers are attempting to overcome this problem by

designing clinical trials that reflect practice more closely

# LIMITATIONS OF EFFECTIVENESS MEASURES

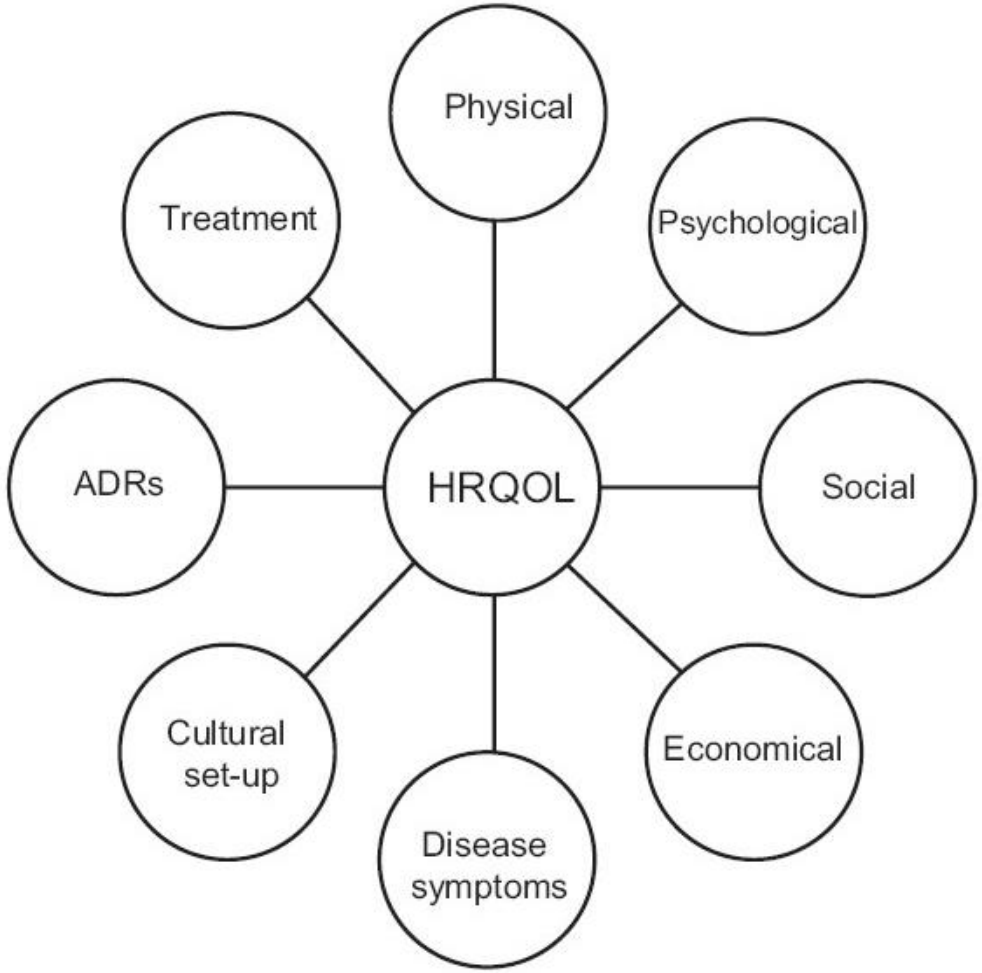
There may be more than one outcome reported for a particular treatment or service.

For example, an analgesic may be more effective than another at relieving pain, but cause **more nausea**.

# LIMITATIONS OF EFFECTIVENESS MEASURES

Does one choose the analgesic with more power, or the one with a lower incidence of nausea?

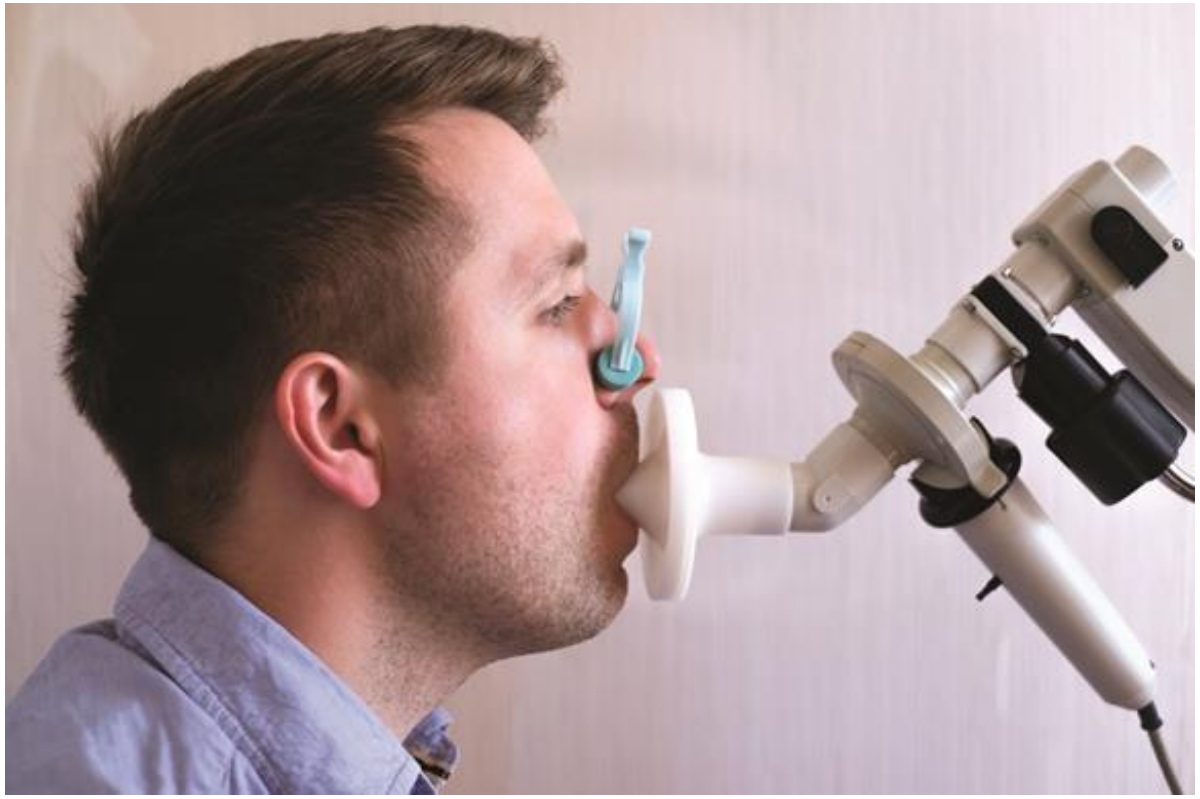
So, effectiveness measures are limited because they only measure one part of an outcome and may not reflect the overall impact of the intervention on the **patient's health-related quality of life** (HRQoL).



# LIMITATIONS OF EFFECTIVENESS MEASURES

For example, in the **cystic fibrosis** study reported above, we know that **FEV1** was improved by the interventions.

However, we do not know whether that improvement was large enough to improve the patient's HRQoL, so it is an intermediate measure of outcome, where we are assuming that improvements in lung function improve overall health status.



# LIMITATIONS OF EFFECTIVENESS MEASURES

Also, we do not know whether the treatments were associated with unwanted side effects.

There may be side effects that are sufficiently bad to affect the patient's health status, or so bad that they refuse to have the treatment at all.

Finally, we do not know whether the improvement in lung function now will continue into the future and extend the life expectancy of the patient.

# LIMITATIONS OF EFFECTIVENESS MEASURES

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, studies looking at the

- *use of aspirin after myocardial infarction*
- *lipid-lowering agents in coronary heart disease*
- *the treatment of hypertension in patients with diabetes*

# MORTALITY USED AS AN EFFECTIVENESS MEASURE

Mortality is a useful outcome measure because it is objective and easy to measure. However, there are problems associated with it.

1. 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.

2. 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.

3. Mortality requires a study with many patients followed up over a long period of time.

4. 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.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

**Table 4.2** Mortality of men aged 45–54 with pre-existing coronary heart disease, and the impact of lipid-lowering drugs on that mortality (adapted from Pharoah and Hollingworth, 1996)

| *Type of mortality* | *Treatment* | | | |
|---|---|---|---|---|
| | *No treatment* | | *With treatment* | |
| | *Coronary heart disease (%)* | *All cause (%)* | *Coronary heart disease (%)* | *All cause (%)* |
| Mortality at 1 year | 2.41 | 5.07 | 1.40 | 4.06 |
| Mortality at 10 years | 7.22 | 13.57 | 4.19 | 10.54 |

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

Table 4.2 reports the results from a trial that examined the mortality of men aged 45-54 with pre-existing coronary heart disease, and assessed the impact of lipid-lowering drugs on that mortality.

This table shows that using these drugs reduces mortality in this patient group. If 100 men were treated for 10 years, three deaths would be prevented.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

The limitations of the use of mortality can be seen in this example.

- First, it takes up to 10 years' follow-up in a very large group of patients to detect a change in mortality.
- Second, people may die from another cause, which can confound the results.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

It is likely that treating patients with these drugs reduced the progression of many of them to coronary heart disease.

Therefore, the health status of many patients was probably improved by the intervention, such that they could carry on working or their normal daily activities.

This important effect of the treatment is not picked up by measuring mortality only.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

Everybody dies eventually, so it is actually premature mortality we are trying to reduce, such that individuals are able to live out an acceptable lifespan.

Therefore, if a premature death is prevented — for example by lipid-lowering drugs - we have saved years of life for an individual.

Therefore, mortality can be converted into

- life-years saved or life-years gained

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

**A theoretical worked example of using effectiveness measures.**

Patients with chronic renal failure who are on hemodialysis suffer from profound anemia, which is often extremely debilitating.

This is due to a reduction in their production of erythropoietin and loss of blood during hemodialysis.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

Historically, these patients have been managed by the use of blood transfusions.

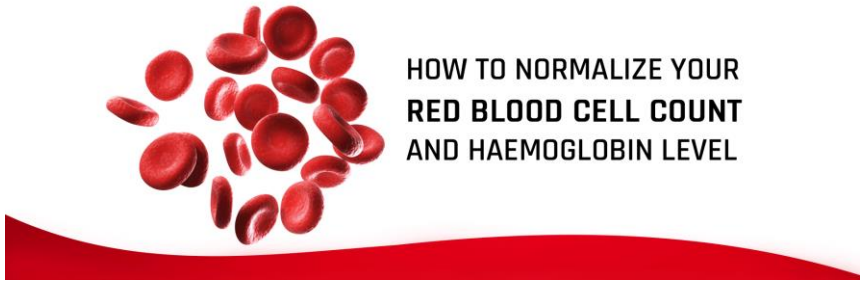
Now, synthetic erythropoietin is available. It is considered to be highly effective, but is very expensive.

So, the alternatives are either to give erythropoietin or to give blood transfusions when the patient's haemoglobin level is below 8g/dl.

# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

Effectiveness data for the two alternatives available from the literature suggest that each intervention can maintain haemoglobin levels above 8g/dl

- erythropoietin for 91 % of the year
- blood transfusions for 76% of the year



# EXAMPLE OF MORTALITY USED AS AN EFFECTIVENESS MEASURE

1. **What is the implicit assumption being made by the use of this outcome measure?**

The assumption is that this is a desirable outcome because the reversal of anemia will increase the patient's energy levels and hence their quality of life.

2. **What is the difference in effectiveness of the two alternatives, for 1000 patients?**

Erythropoietin keeps the Hb level >8g/dl for 15% more of the year than do blood transfusions = 54.75 days per patient per year = 54 750 days per 1000 patients per year.

# QUALITY OF LIFE

Most modern medicine improves quality rather than quantity of life.

The limitations associated with effectiveness measures have led researchers to develop ways of measuring the whole impact of a disease or treatment on a patient.

Definitions: it may be helpful to distinguish between the terms

quality of life (QoL) and health-related quality of life (HRQoL).

# QUALITY OF LIFE

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."

# QUALITY OF LIFE

In health-related research and articles, the term QoL is often used interchangeably with the term HRQoL, and both might be used to indicate the narrower definition pertaining to a person's health.

Importance: The use of HRQoL measures has been increasing since the mid-1980s. Traditionally, health has been considered from a biomedical point of view.

From this viewpoint emphasis is placed on activities associated with repairing injury and reducing the impact or length of illness.

# QUALITY OF LIFE

Although this approach is essential, it does not encompass all of the aspects that are important to health.

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."

# QUALITY OF LIFE

Many health care providers and health services researchers have adopted this expanded view of health and now include measures of the overall impact of diseases and their treatments.

In addition to physical functioning, the overall concept of HRQoL includes other aspects of health, called domains, such as psychological and social functioning, that are important to the patient.

# 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.

# MEASURING QOL

It is often necessary to focus on the impact that a certain disease or condition has on patients.

In this case, condition- or disease-specific measures are often used to collect more narrowly focused patient views on the impact of the disease.

Examples of specific areas investigated with disease-specific questionnaires include

- ***nausea and vomiting for cancer treatment***
- ***range of movement for arthritis treatment***

# DISEASE-SPECIFIC HRQOL MEASURES

| |
|---|
| *Hypertension* |
| • Physical Symptoms Distress Index (PSDI)[16] |
| • The Subjective Symptom Assessment Profile[17] |
| *Benign Prostatic Hyperplasia* |
| • American Urological Association Symptom Index (AUASI)[18] |
| • BPH Impact Index[19] |
| *Asthma and Allergy* |
| • Living with Asthma Questionnaire[20] |
| • Life Activities Questionnaire for Adult Asthma[21] |
| *Diabetes Mellitus* |
| • Diabetes-Specific QoL Instrument (DQOL)[22] |

# EXAMPLES OF ITEMS FROM THE ASTHMA QUALITY OF LIFE QUESTIONNAIRE

| *Activities Domain* |
| --- |
| How limited have you been during the past 2 weeks in these activities as a result of your asthma? |
| Question 1. Strenuous activities (e.g., hurrying, exercising, running upstairs, sports). |
| Question 3. Social activities (e.g., talking, playing with pets or children, visiting friends or relatives). |
| *Response choices are 1) totally limited, 2) extremely limited, 3) very limited, 4) moderate limitation, 5) some limitation, 6) a little limitation, and 7) not limited at all.* |
| *Symptoms Domain* |
| Question 6. How much discomfort or distress have you felt over the past 2 weeks as a result of chest tightness? |
| *Response choices are 1) a very great deal, 2) a great deal, 3) a good deal, 4) a moderate amount, 5) some, 6) very little, and 7) none.* |
| Question 20. In general, how much of the time during the past week did you wake up in the morning with asthma symptoms? |
| *Response choices are 1) all of the time, 2) most of the time, 3) a good bit of the time, 4) some of the time, 5) a little of the time, 6) hardly any of the time, and 7) none of the time.* |

# MEASURING QOL

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.

One of the most widely used is the Short Form (SF)-36 health survey. This looks at:

- *Physical functioning*
- *Physical role*
- *Bodily pain*
- *General health*
- *Vitality*
- *Social functioning*
- *Emotional role*
- *Mental health*

# MEASURING QOL

Typical questions asked include: Does your health limit you in these activities?

| | *Yes Limited a lot* | *Yes Limited a little* | *No Not limited at all* |
|---|---|---|---|
| Walking more than a mile | ○ | ○ | ○ |
| Bathing or dressing yourself | ○ | ○ | ○ |
| Lifting or carrying groceries | ○ | ○ | ○ |

# EXAMPLES OF ITEMS FROM THE SF-36 GENERAL HRQOL INSTRUMENT

| |
|---|
| ***Vitality Domain*** |
| Answer choices are 1) all of the time, 2) most of the time, 3) a good bit of the time, 4) some of the time, 5) a little of the time, and 6) none of the time. |
| Questions: *How much of the time during the past 4 weeks*: |
| Q9a. Did you feel full of pep? |
| Q9e. Did you have a lot of energy? |
| Q9g. Did you feel worn out? |
| Q9i. Did you feel tired? |
| ***Role—Emotional Domain*** |
| Answer choices are 1) yes and 2) no. |
| Questions: *During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of any emotional problems (such as feeling depressed or anxious)?* |
| Q5a. Cut down on the amount of time you spent on work or other activities. |
| Q5b. Accomplished less than you would like. |
| Q5c. Did work or other activities less carefully than usual. |

# MEASURING QOL

Using these types of tool can give a much better indication of the impact of the treatment or service on the patient's QoL than using effectiveness measures.

In the example of the study of lipid-lowering drugs in men aged 45-54 with pre-existing heart disease, a tool such as the SF-36 health survey could assess the effect of reducing the incidence of coronary heart disease on the QoL of the patients who have the treatment.