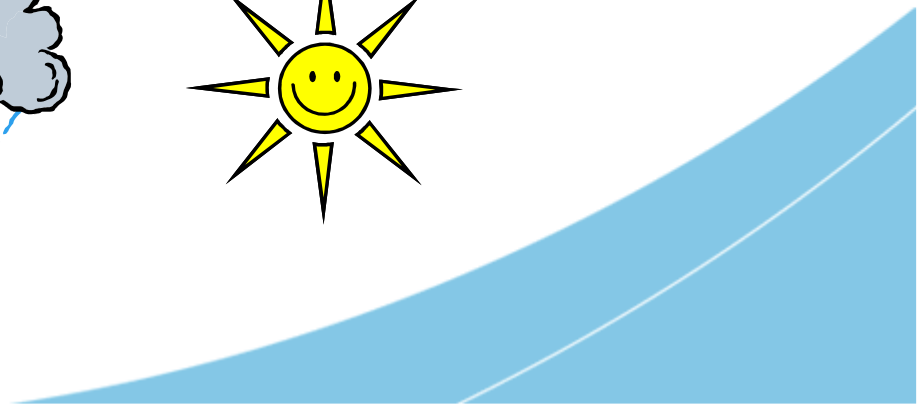
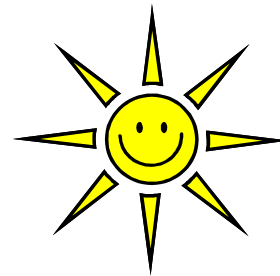


# Presentation 3

## Valuing health outcomes



# Types of Economic Evaluation

Type of analysis	Value of resources	Value of health gain



Warning – ‘cost-effectiveness’ often used as general term for any analysis with non-monetary measure of health gain

## NICE reference case – choice of analysis

- “For the reference case, the perspective on outcomes should be all direct health effects, whether for patients or, when relevant, other people (principally carers).”
- “...(C)ost-effectiveness (specifically **cost–utility**) analysis is the preferred form of economic evaluation. This seeks to establish whether differences in costs between options can be justified in terms of changes in health effects. Health effects should be expressed in terms of **QALYs**.”
- “Given its widespread use, the QALY is considered to be the most appropriate generic measure of health benefit that reflects both mortality and HRQL effects. It is recognised that alternative measures exist (for example, the healthy-year equivalent), but few economic evaluations have used these methods and their strengths and weaknesses are not fully established.”

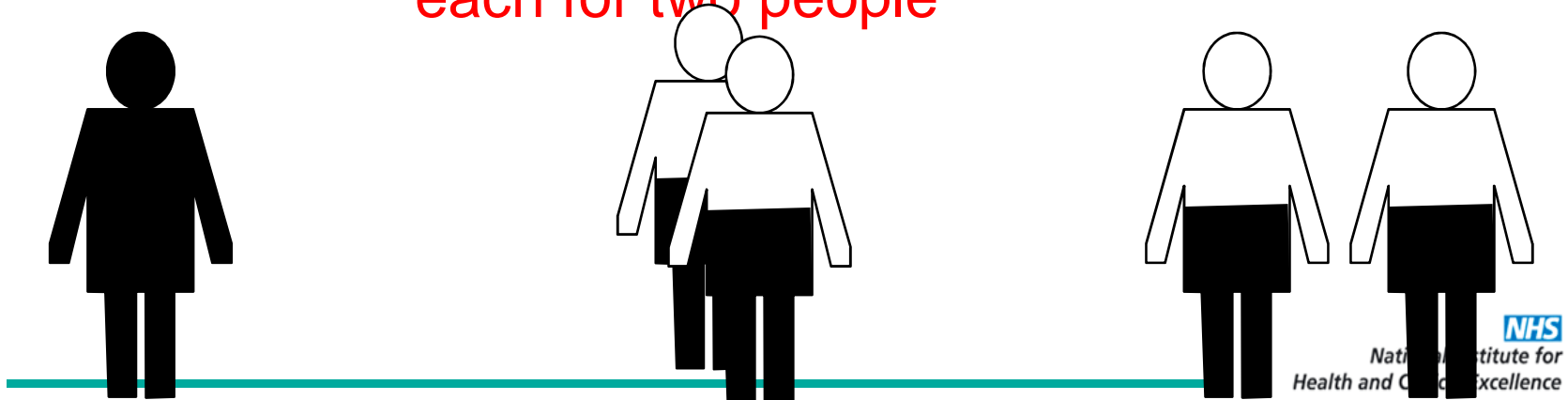
# Quality Adjusted Life Years

- **What is a QALY?**
  - A QALY combines both quantity and health-related quality of life (QoL) into a single measure of health gain
  - The amount of time spent in a health state is weighted by the QoL score attached to that health state
  - QoL scores should reflect peoples' preferences over health
  - QoL is usually scored with 'perfect health'=1 and death=0
- **Why use QALYs?**
  - Can weigh up net effect of treatment for patients
    - Survival vs. QoL (e.g. for cancer chemotherapy)
    - Long-term QoL for chronic & recurrent conditions (e.g. arthritis)
    - Benefits vs. harms (e.g. COX II inhibitors)
  - Allows broader comparisons between patient groups

# “A QALY is a QALY is a QALY”

Usual value judgements used to calculate QALYs:

- 1 QALY = one year of ‘perfectly healthy’  
life for one person
- = two years of life with QoL of 0.5  
for one person
- = one year of life with QoL of 0.5  
each for two people



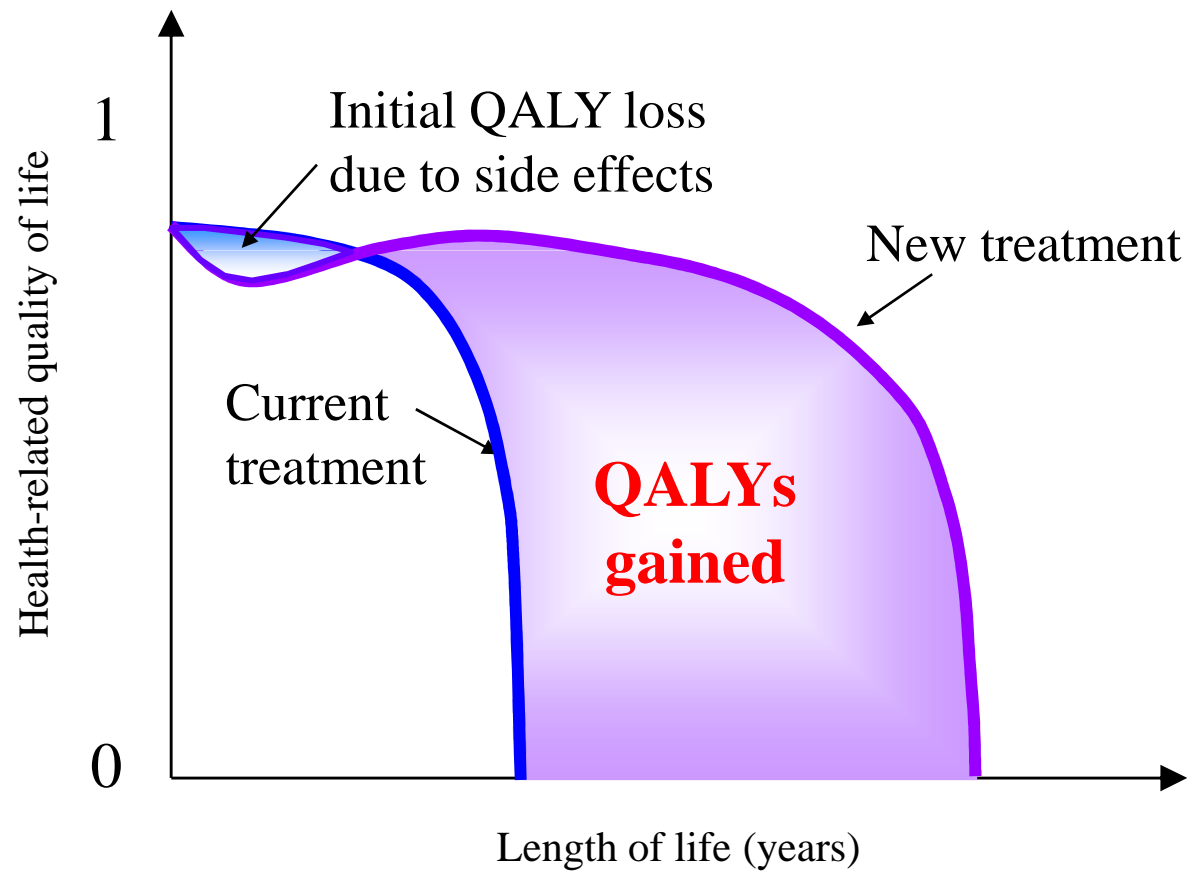
# NICE reference case and equity weighting of QALYs

- “An additional QALY has the same weight regardless of the other characteristics of the individuals receiving the health benefit”
- Other characteristics include “socio-demographic details, or their pre- or post-treatment level of health”
- “There are several unresolved methodological issues concerning how and in what circumstances to apply additional weights to QALY calculations. Until such issues are resolved, the use of differential QALY weights is not recommended as part of the reference case”

# Other assumptions / issues

- The value of a health state is the same regardless of the length of time in the state and where it lies in a sequence of states → it is assumed that you can add the utilities from constituent health states (additive independence between health states)
- Constant proportional time trade off (CPTTO) assumption for QALYs calculated from health state values generated by the time-trade off method → individuals are willing to sacrifice a constant proportion of his or her remaining life years in order to achieve a given improvement in health, *irrespective of the number of life years that remain.*

# The **Q**uality **A**adjusted **L**ife **Y**ear



# NICE reference case - measuring and valuing benefits

- The HRQL 'weighting' usually comprises two elements: the description of changes in HRQL itself and a valuation of that description of HRQL.
- Information on changes in HRQL as a result of treatment should be reported directly by patients (***and directly by carers when the impact of treatment on the carer's health is also important***).
- The ***valuation*** of changes in HRQL reported by patients should be based on ***public preferences*** elicited using a ***choice-based method*** in a representative sample of the UK population.
- To quantify the effects of technologies on HRQL, the ***EQ-5D*** (a standardised and validated generic instrument) is preferred.

# EQ-5D

- The EQ-5D comprises five dimensions of health: mobility, ability to self-care, ability to undertake usual activities, pain and discomfort, and anxiety and depression.
- Each dimension has three levels – “1” is best, “3” is worst
- Together these five dimensions define a total of 243 health states formed by different combinations of the levels (i.e.  $3^5$ ).
- Designed so that people can describe their own HRQL using a standardised descriptive system. *A set of **preference values** elicited from a large **UK population** study using a **choice-based method of valuation** (the time trade-off method) is available for the EQ-5D classification system.* This set of values can be applied to people’s self-reported descriptions of their HRQL to generate health-related utility values.

# Definition of health states: EQ-5D

## **Mobility**

I have no problems in walking about

I have some problems in walking about

I am confined to bed

## **Self-care**

I have no problems with self care

I have some problems washing or dressing myself

I am unable to wash or dress myself

## **Usual activities** (e.g. work, study, housework, family or leisure activities)

I have no problems with performing my usual activities

I have some problems with performing my usual activities

I am unable to perform my usual activities

## **Pain/discomfort**

I have no pain or discomfort

I have moderate pain or discomfort

I have extreme pain or discomfort

## **Anxiety/depression**

I am not anxious or depressed

I am moderately anxious or depressed

I am extremely anxious or depressed

# Why public preferences?

- Whose values?
- Patient values to score health states?
  - they are in the best position to know their own state
  - Can value hypothetical states
  - Can value their own current state (a measure of experience rather than preference)
- The general public
  - in a publicly funded health care system it is society's resources that are being allocated, and therefore it is the views of the general population that are relevant
  - Washington Panel (Gold et al, 1996) – “veil of ignorance”
- Ultimately a value judgement by the decision making authority.

# Choice-based methods of valuation

- Time trade off (TTO)
  - respondents have to trade quality of life for length of life
- Standard Gamble (SG)
  - respondents trade health improvement for a risk of death
- SG better?
  - Grounded in expected utility theory
  - But concerns about understanding of risks by respondents
- SG & TTO both cognitively challenging?
- Methods generate different values!

# The preference for EQ-5D...

- Most widely used generic preference-based measure
- Acceptable psychometric properties across a wide range of common conditions (including rheumatoid arthritis, hip fracture, intermittent claudication, liver transplantation), but *not* all.
- May be insensitive (compared to other preference-based measures) to important differences in e.g. cases of visual impairment and hearing loss
- Data using the EQ-5D instrument may not always be available (can map EQ-5D utility data from other HRQL measures).
- Current version of the EQ-5D not designed for use in children

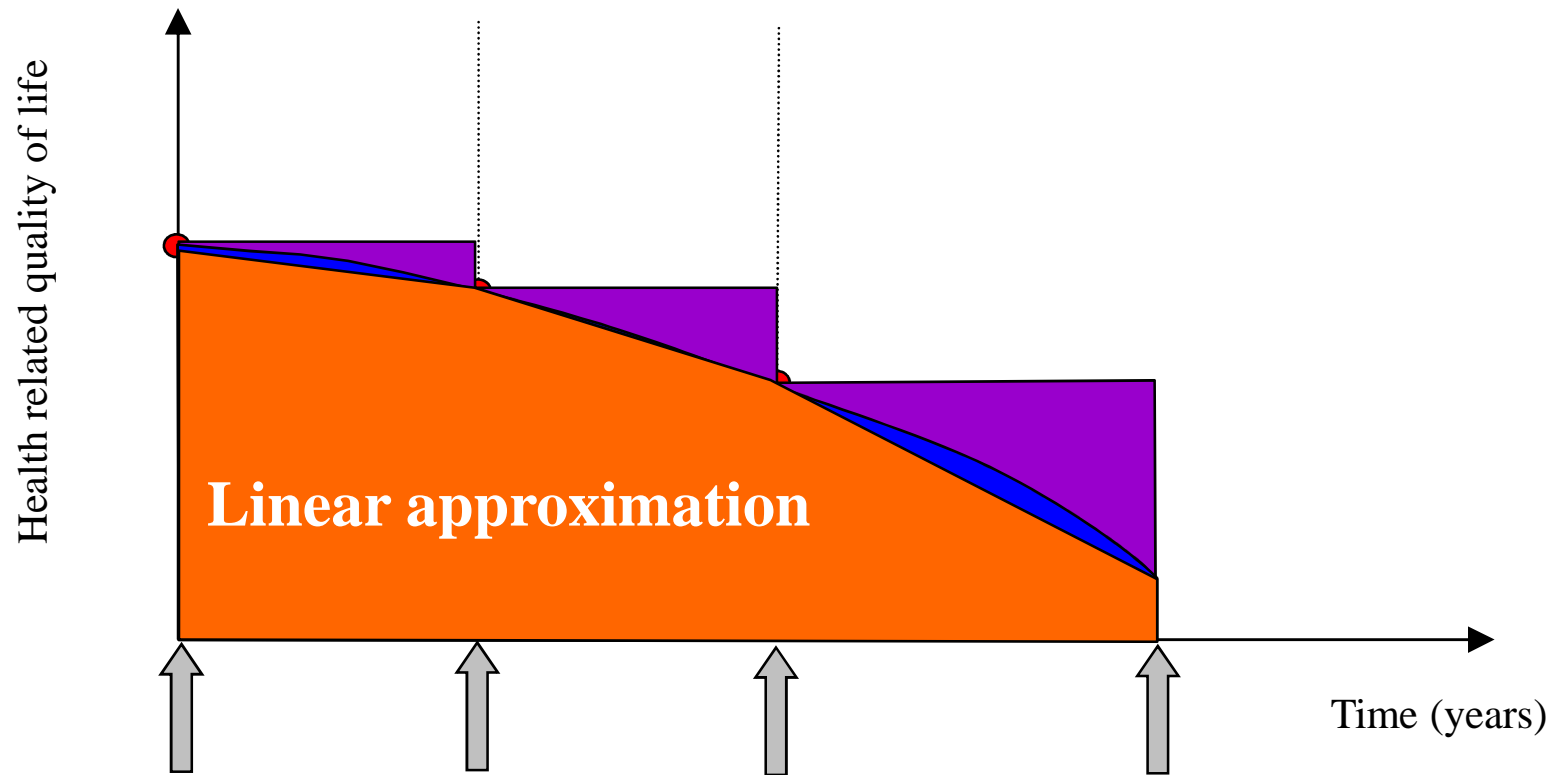
# NICE view on the limitations of EQ-5D

- If the EQ-5D is considered inappropriate, empirical evidence should be provided on why the properties of the EQ-5D are not suitable for the particular patient population.
  - These properties may include the content validity, construct validity, responsiveness and reliability of EQ-5D.
  - If direct valuations of descriptions of health states based on HRQL measures other than the EQ-5D are used, the valuation methods must be comparable to those used for the EQ-5D.
- Consider using alternative standardised and validated preference-based measures of HRQL, such as the Health Utility Index 2 (HUI 2), that have been designed specifically for use in children.

# An EQ-5D value set for Colombia?

- Zarete et al (2008)
  - National population survey conducted in the US in 2002, based on a sample of 1603 non-Hispanic nonblacks and 1115 Hispanics
  - Participants provided TTO utilities for a subset of 42 EQ-5D health states
  - A population value set for all 243 EQ-5D health states was developed using only the data from Spanish-speaking Hispanics
  - Authors say that their Hispanic model for EQ-5D valuations provide a “set of social preference weights for use in Latin American countries that presently lack their own domestic value set.”
- Argentine value set also now available (2009)

# Estimating QALYs from RCT data



Linear approximation

Baseline    Six-month follow-up    One-year follow-up    Expected survival?

# QALYs – “a measure of convenience”

- “Reasonably informative” single index measure of health gain, but not a ‘true’ measure of health gain
- Role for cost consequence analysis in addition to cost utility? (Drummond & Rutten, 2008)
- NICE position: “If the assumptions underlying QALYs (for example, constant proportional trade-off and additive independence between health states) are considered inappropriate in a particular case, then evidence to this effect should be produced and analyses using alternative measures may be presented as an additional non-reference-case analysis”.

# Supplementary Guidance for 'End of Life' Therapies (NICE, 2009)

- “Supplementary advice to the Appraisal Committees, to be taken into account when appraising treatments which may be life-extending for patients with short life expectancy, and which are licensed for indications affecting small numbers of patients with incurable illnesses”
- Additional advice will apply when such treatments have an ICER in excess of the upper end of the range normally approved by the Appraisal Committees, using the 'reference case' (i.e. > £30,000 per QALY)

# Supplementary Guidance for 'End of Life' Therapies (NICE, 2009) contd

- Advice should be applied in the following circumstances and when all the criteria referred to below are satisfied:
  - The treatment is indicated for patients with a short life expectancy, normally less than 24 months and;
  - There is sufficient evidence to indicate that the treatment offers an extension to life, normally of at least an additional 3 months, compared to current NHS treatment, and;
  - The treatment is licensed or otherwise indicated, for small patient populations.
- When criteria met, explore “giving greater weight to QALYs achieved in the later stages of terminal diseases”

# Disability Adjusted Life Years (DALYs)

- DALYs offer a combined index of quality adjusted life expectancy
- Two basic components which are summed
  - Years of Life Lost (YLL) due to premature mortality
  - Years Lost due to Disability (YLD) for incident cases of the health condition
- One DALY = one lost year of “healthy” life
- Aims to measure the gap between current health status and an ideal situation where the “entire population lives to advanced age, free of disease and disability”
- In contrast to QALYs, you want less DALYs not more.
- DALYs can be used to measure the ‘burden of disease’ (a “positive” function)
- DALYs can have a normative function – inform resource allocation

# DALYs - calculation

- YLL → calculated from number of deaths at each age multiplied by a *global standard of life expectancy* for each age
- YLD for a particular cause in a particular time period → calculation based on the number of incident case in that period, the average duration of disease and a disability weighting factor
- Disability weight factor – 0 (perfect health) to 1 (death)
- Time discounting (3%)
- Non uniform age weights (less weight to years lived at young and older ages)

# DALYs - issues

- Concerns about its equity implications – Anand & Hanson (1997), Arnesen & Nord (1999)
- Concerns about the role of burden of disease approaches in decision making – Williams (1999,2000) → Should “start with people not diseases”
- Expert driven valuations – Williams (1999,2000)
- Its actual application in cost-effectiveness analysis, e.g. selection of assumptions, transparency (Fox-Rushby & Hanson, 2001)
- For cost effectiveness analysis, local life expectancy is recommended, not the use of ‘standard expected years of life lost’
- Important when presenting DALYs to include full calculation and sensitivity analyses around weighting, discount rates etc
- *Has a role in economic evaluation in estimating the benefits of interventions*

# QALYs & DALYs (1)

- QALYs are associated with a number of important assumptions but they can potentially be made more sophisticated if the empirical evidence becomes available, e.g. through the use of weights
  - Can also deal with limitations through the deliberative process and when necessary by means of “non-reference case” analyses
- QALYs are a year of life in good health → they do not depend in any way on the number of such years one may have had or may expect
- QALYs – at least when used by NICE – serve to inform decision making over competing interventions → they are not used to judge the ‘worthiness’ of any life

## QALYs & DALYs (2)

- DALYs involve important assumptions too, and their 'quality of life' component involves valuations taken from experts
- DALYs when used with age and disability weighting (& discounting too?) may have unappealing ethical consequences, e.g. the DALYs prevented by an intervention which extends the life of a disabled person will be less than those prevented for an able-bodied person
  - Societal preference appears to want to give more weight to individuals with a worse initial health state!