

# What is cost effectiveness and cost benefit analysis?

## A focus on healthcare

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*“The drug itself has no side effects - but the number of health economists needed to prove its value may cause dizziness and nausea.”*

# The health economist's challenge...

...to provide information (for decision-makers) about how to allocate scarce healthcare resources such that maximum patient benefit is obtained from every pound (dollar/euro) spent

# The Context

- NHS takes 8.2% of GDP
- NHS budget in England: funding from taxation
  - £33 bn in 1996/7
  - £96 bn in 2008/9
  - ~£108.9 bn for 2012/2013
- NHS is free at the point of use for everyone resident in the UK = 63.2 m people
- Average expenditure per person
  - £426 in 1996 compared with £1,612 in 2010
- NHS employs some 1.7 m people of which ~50% clinically qualified



# Efficiency

- **How do we allocate scarce resources so that benefit is maximised?**
- *Allocative*: using resources, across the whole economy, so that benefit is maximised (or the opportunity cost is minimised).
- *Technical*: means producing a given output for the least cost, or maximising output for a given cost.

Q: How do we measure 'efficiency'?



# *Choices about allocation of resources have to be made and are being made by....*

- the Department of Health
- the National Institute of Health and Clinical Excellence
- Service commissioners eg. GP commissioning groups
- Individual clinicians

# .....Opportunity Cost

Is the benefit that would be derived from using a resource in its best alternative use



A fabulous family holiday?  
A flashy sports car?  
Lifetimes supply of pizza

*Over one-year.....*

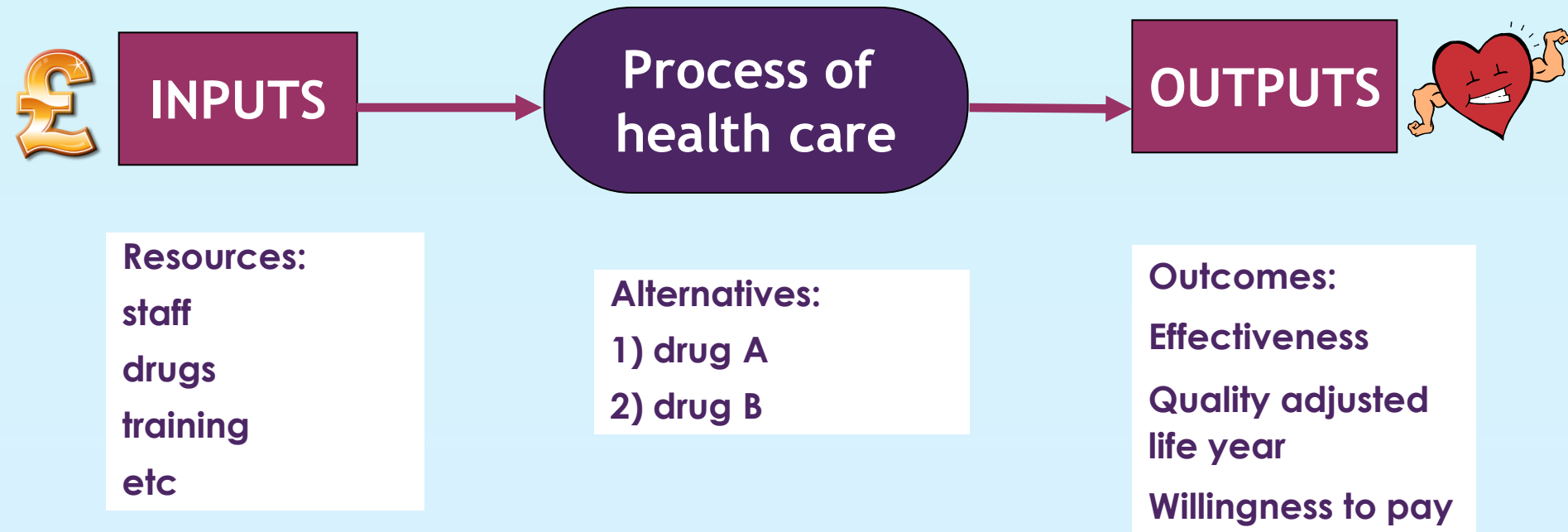
57 people offered genetic test

6 people have hip replacements

1 person gets Herceptin for breast cancer

# Economic evaluations

- One of a number of methods used by health economists
- An evaluative framework



## Vehicles for Economic Evaluation

- Prospective – alongside RCT
- Retrospective - modelling



# What should be costed?

- True economic cost takes into account all the cost associated with an intervention.
- In an economic evaluation, the costs included depend on:
  - the perspective (viewpoint) of the evaluation
    - Society (everyone)
    - NHS and Personal Social Services
    - Hospital
  - the time horizon (follow-up) of the evaluation
    - 1 year
    - 10 years
    - Lifetime

# Measuring the benefit of interventions

Benefits, outcomes and consequences refer to the effect on the patient, not the effect on the people providing the service.

Cost is not an outcome measure.

The principal outcome categories used in economic evaluation are:

- effectiveness
- utility
- quality of life
- expressing benefits as monetary values

# Methods of economic evaluation

- Cost Minimisation Analysis (CMA). The outcome of the service/treatment being compared is **assumed (based on evidence) to be the same**

- Cost Effectiveness Analysis (CEA). The outcome of the service/treatment being compared is measured in a **single, natural unit**
- Cost Utility Analysis (CUA). The outcome of the service/treatment being compared is measured using **utility values**

- Cost Benefit Analysis (CBA). The outcome of the service/treatment being compared is measured using **monetary units**

# Cost Effectiveness (Utility) Analysis

- The extra-welfarist perspective provides the theoretical foundation for the use of CEA
- Deciding how best to spend the ‘healthcare’ budget therefore benefit to be maximised is health (technical efficiency)
- CEA requires an instrument to describe and value ‘health’
- The majority of published economic evaluations are CEA/CUA – see NHS Economic Evaluation Database

# Measuring Effectiveness

## Effectiveness: outcome measured in natural units

General outcome measures:

- Number of cases successfully diagnosed
- Number of cases successfully treated;
- life years gained.

Clinical indicators:

- pain-free days;
- Improvement in CRP levels

**Data source: RCTs and meta-analyses**

Q: What is the problem with using these measures?



# Measuring Health

- Modern medicine improves quality, rather than quantity of life
- Using clinical (effectiveness) indicators implies that changes in these will link directly to an effect on the patient's health related quality of life.
- Many functional, social, psychological, cognitive and subjective factors that impact on quality of life.
- Quality of life measures can be divided into generic and disease-specific measures.

# Measuring health status: The EQ-5D

## Mobility

- I have no problems 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)

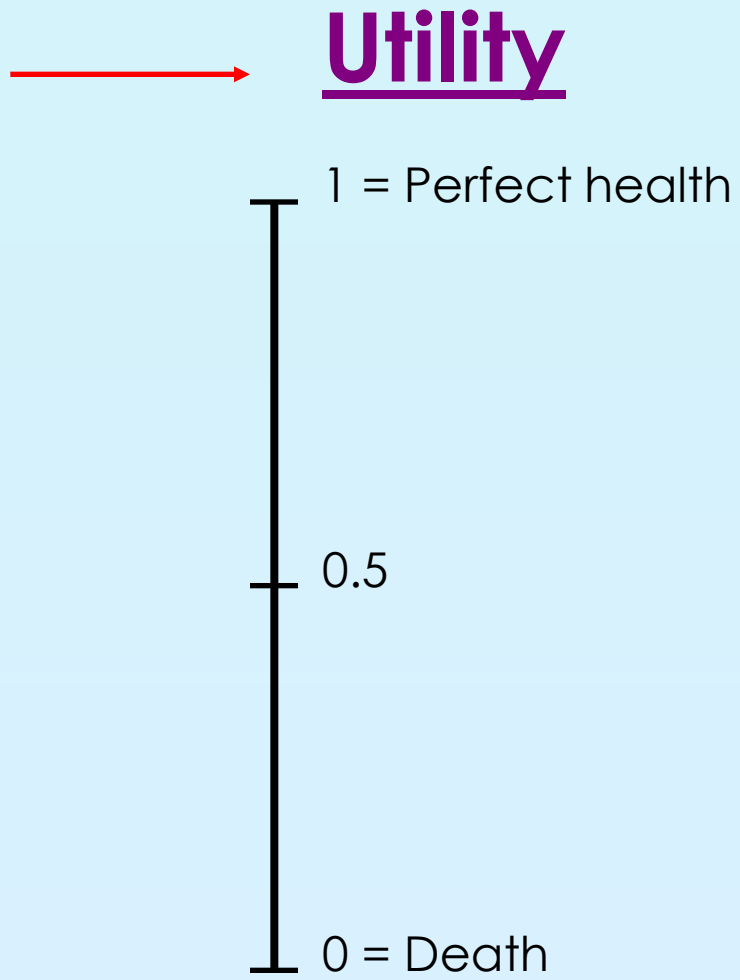
- I have no problems with performing my usual activities
- I have some problems with performing my usual activities
- I am unable to perform my usual activities

## Pain/Discomfort

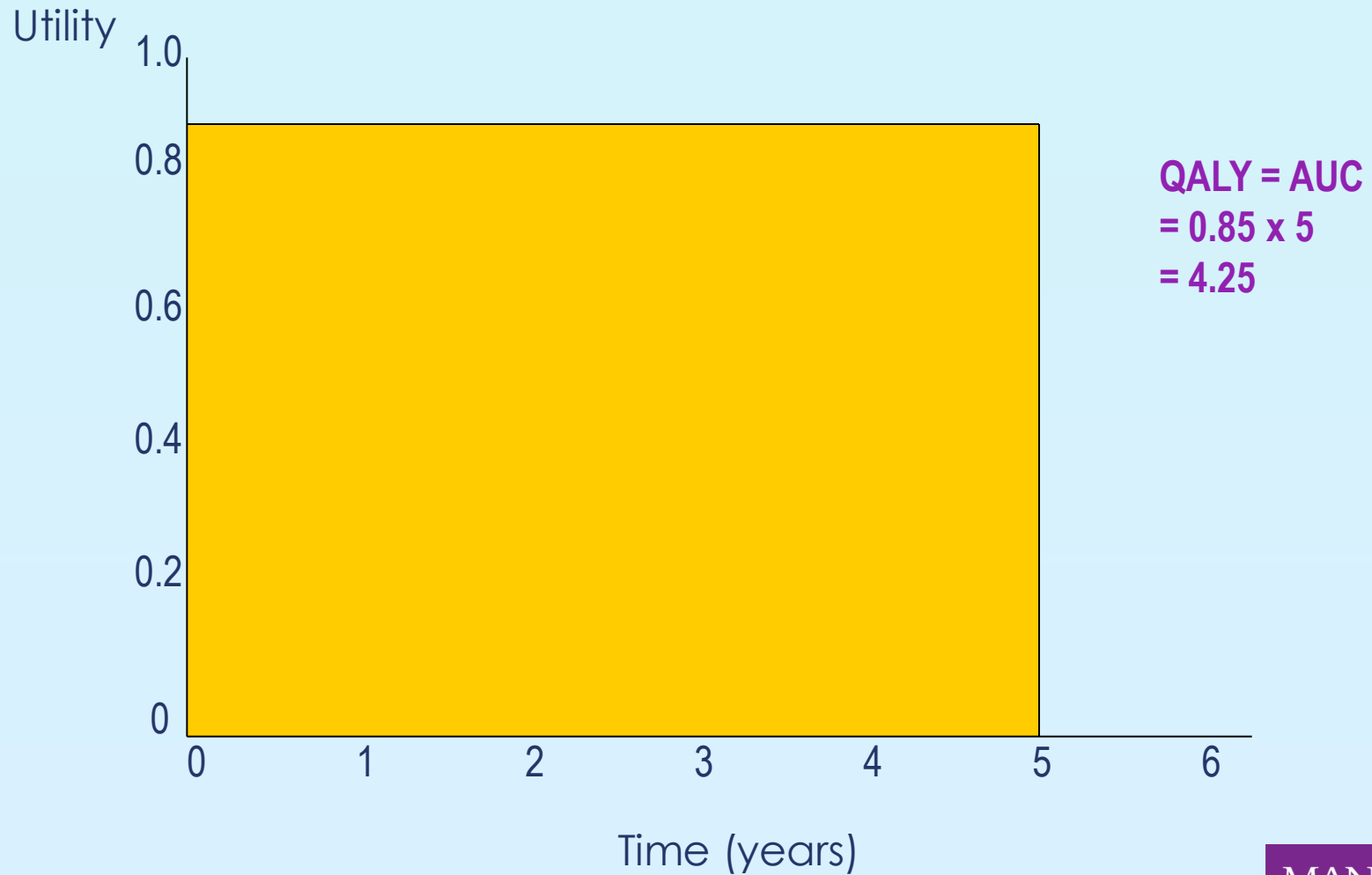
- I have no pain or discomfort
- I have moderate pain or discomfort
- I have extreme pain or discomfort

## Anxiety/Depression

- I am not anxious or depressed
- I am moderately anxious or depressed
- I am extremely anxious or depressed



# The Quality Adjusted Life Year (QALY)



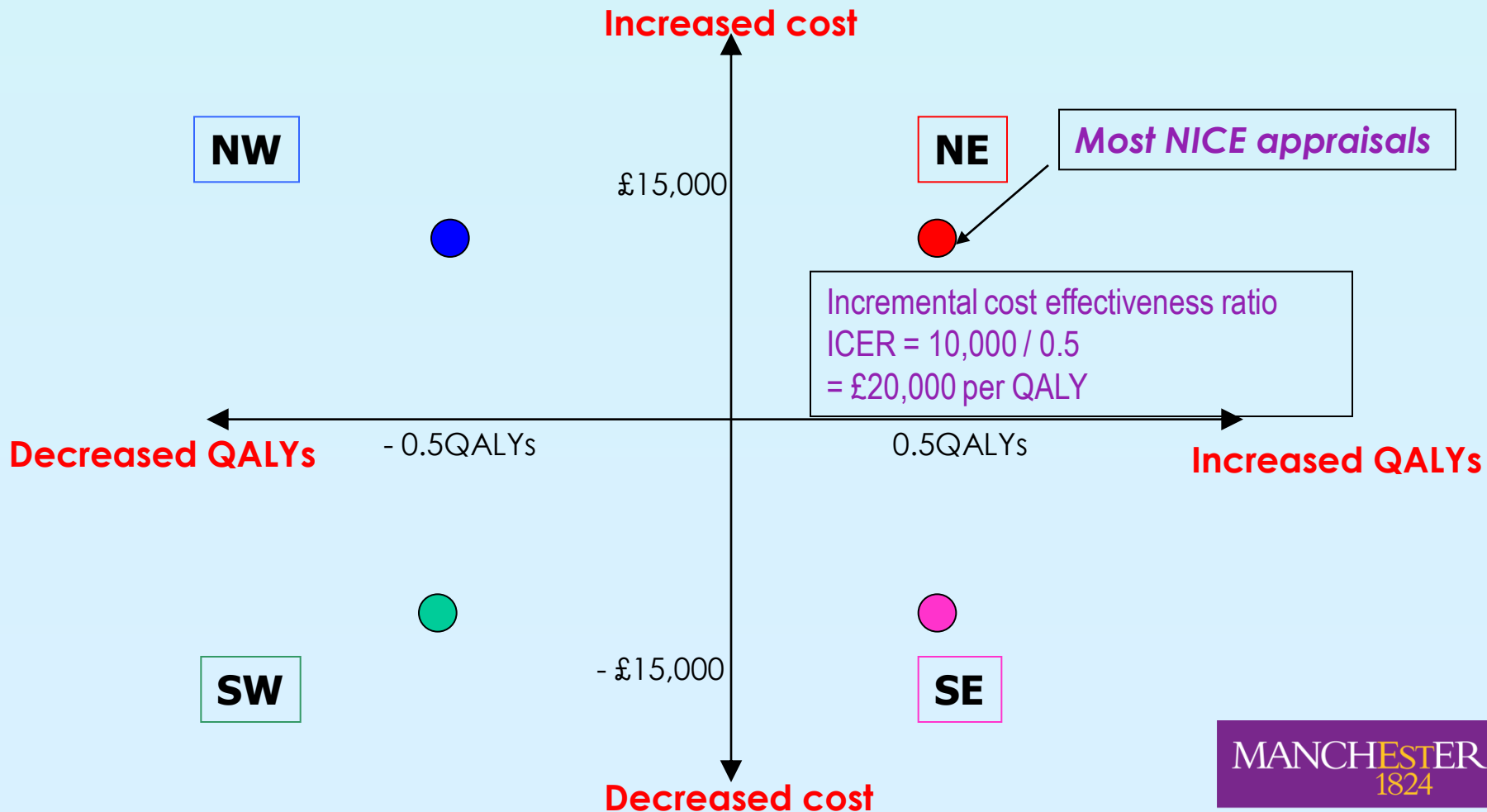


# The cost-effectiveness plane

**Difference in cost** = £Drug A – £Drug B

**Difference in QALYs** = QALYs Drug A – QALYs Drug B

**ICER** = Difference in cost / difference in QALYs



# Interpreting the results

- Should we recommend the intervention – as a cost effective use of resources?
- What is the quality of the evaluation?
  - Need a ‘consistent’ approach to inform decision-making
  - Uncertainty in the technical details and values (inputs)
  - Critical appraisal tools
    - NHS Economic Evaluation Database (NHS EED)
    - BMJ Guidelines for authors/reviewers
    - NICE: Methods Guides (Reference Case)
- What is the ‘threshold’ for cost effectiveness?
  - NICE range: £20,000 to £30,000 per QALY

Issue date: October 2009

**Guide to the single  
technology appraisal  
process**

Issue date: October 2009

**Guide to the multiple  
technology appraisal  
process**



**National Institute for  
Health and Clinical Excellence**

Issue date: December 2011

**Diagnostics Assessment  
Programme manual**

Issue Date: April 2009

**Methods for the  
development of NICE  
public health guidance  
(second edition)**

# Cost Benefit Analysis

- The welfarist perspective provides the theoretical foundation for the use of CBA
- Utility (of individuals) is assumed to represent the total benefit of an intervention
- Utility = ‘desirability’ or ‘preference’ in the context of CBA
- Very few examples of published CBA in health  
[Smith R & Sach T. Pharmacoeconomics 2007; 25: 107-127]

# Expressing benefits as monetary values

- Contingent valuation method
- Methods to elicit willingness to pay (or accept)
- The stated WTP (of a population) is assumed to represent how much individuals who make up society value an intervention
- Stated WTP could represent monetary value to avoid an illness or obtain the benefits of a treatment
  - health benefits
  - non-health benefits
  - process benefits

# Valuing the benefits of genetic testing

## R<sup>eye</sup>gard study

### **Background**

We want to know how much you value healthcare services for inherited eye conditions such as retinal dystrophy. One way of finding out the value of things, like genetic counselling or testing, is to ask people what they are willing to pay for it. Of course, in this country genetic services are free and available on the NHS. It will remain free and available on the NHS in the future. However, for the purpose of this survey, we would like you to imagine that you are in a country, like the USA, where you do have to pay. Remember, that by spending this amount, you will have less to spend on other goods or items you use in your life.

### **We want you to consider the following situation.**

You are experiencing some loss of vision. Your eye doctor suspects that your loss of vision may be because of a condition called retinal dystrophy. You have been invited to attend an outpatient appointment in a genetic clinic at the hospital. Inherited eye conditions, such as retinal dystrophy, can lead to severe impairment in your vision. There are many different types of retinal dystrophy and over 100 genes have now been identified. If you have got retinal dystrophy then you will continue to lose your vision and may at some point be registered as blind. At the moment there is no effective prevention or cure.

# Scenario 2: genetic counselling & testing

## R<sup>o</sup>gard study

**We now want you to think about this scenario** and how much you value genetic testing with genetic counselling for retinal dystrophy. In this scenario, assume that you get genetic counselling and genetic testing. The genetic counsellor tells you about a genetic test that is available. If you decide to take the test then a blood sample will be taken, which is used to obtain a sample of your DNA.

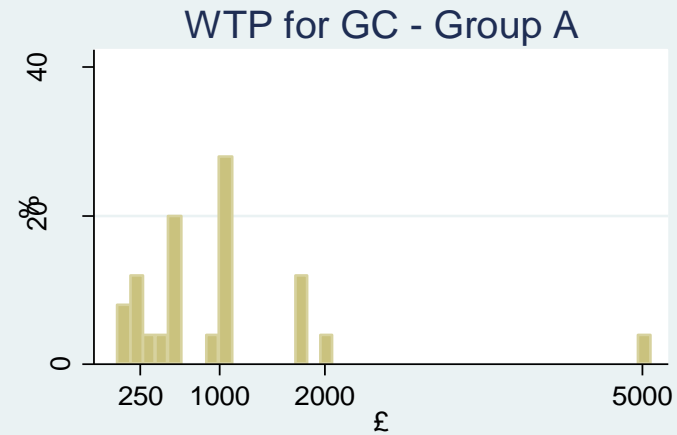
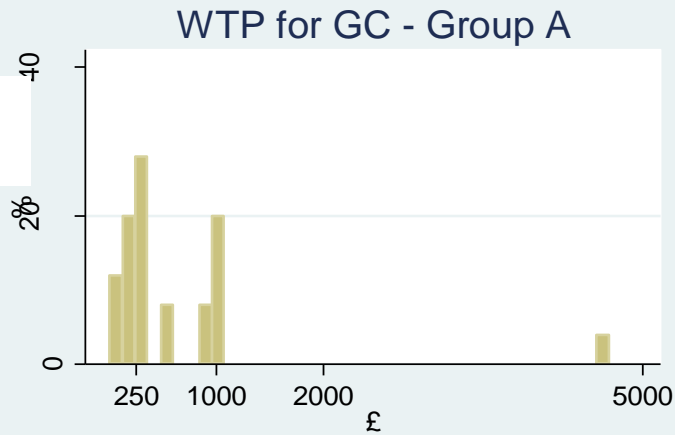
Genes are the instructions present in the cells of our body that control how our body is made. The genetic test can confirm your diagnosis and tell you what type of faulty gene is causing your retinal dystrophy. The test can tell you how the faulty gene is running in the family and the exact risks of other family members, such as your children, inheriting retinal dystrophy and developing the condition. The technology is not perfect and we cannot find the faulty gene in everyone who has retinal dystrophy. There is a 50% chance (like tossing a coin) that the genetic test will find the faulty gene and confirm your diagnosis and a 50% chance that it will not find it.

The genetic test will not change your own treatment, but will confirm the risks to family members of inheriting the condition. Other family members may choose to have a genetic test. The genetic test may provide information to use in making decisions about, for example, future life choices such as career, hobbies or having children.

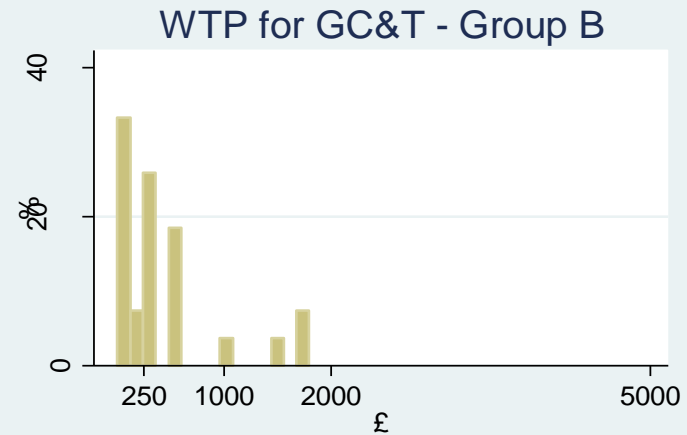
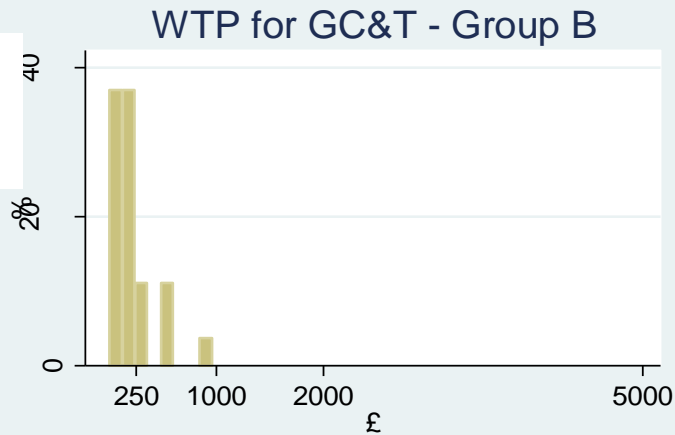
The information from the gene test may make you feel anxious. The genetic counsellor will check that you understand the information and support you in making an informed choice about whether to take the test. The genetic counsellor will offer you support in talking with other family members and, if appropriate, they will arrange for some of your family members to come and see a genetic counsellor.

# Valuing genetic testing: early results

Genetic counselling



Genetic counselling & testing



Group A: experience of retinal dystrophy (n=25)  
Group B: general population (n=27)



# Using CBA in Practice: the theory

- Only introduce interventions that provide a potential Pareto improvement
- The gainers can compensate the losers and still be better off
- Use WTP to elicit a monetary benefit
- Monetary metric allows comparison within & across public sector budgets (allocative efficiency)
  - Treasury Green Book
- Introduce technologies with positive benefits
  - $\text{£ benefit} - \text{£ cost} > 0$

# Using CBA in the UK Context

- Very few examples of published CBAs or use of CBA in national decision making
- ....despite some economists stating that there is no reason why health is different to other public sectors  
[Pauly MV in Sloan F. Valuing healthcare. Cambridge University Press]
- Methodological and ethical concerns  
[Smith R & Sach T. Health Economics Policy & Law 2010; 5: 91-111]
- Current policy makers taking a pragmatic 'social decision makers' perspective

*'it is a healthcare budget; so maximise health'*

# Concluding Remarks

- Why use economic evaluation?
- Source of evidence to inform resource allocation decision making: national; regional; local
- Model-based CEA has become an integral part of health technology assessment and appraisal (by NICE)
- CEA or CBA?

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**See <http://economicsnetwork.ac.uk/health>**