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HEADS PhD PROGRAMME IN HEALTH DATA SCIENCE

DECIDE

Introduction to Health Interventions, Policy and Services

Coordinator:

Luís Filipe Azevedo

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DECIDE

Introduction to Health Interventions, Policy and Services

Methods in Decision Analysis and Decision Modelling – Part I
Valuing Outcomes, Preferences and Utilities

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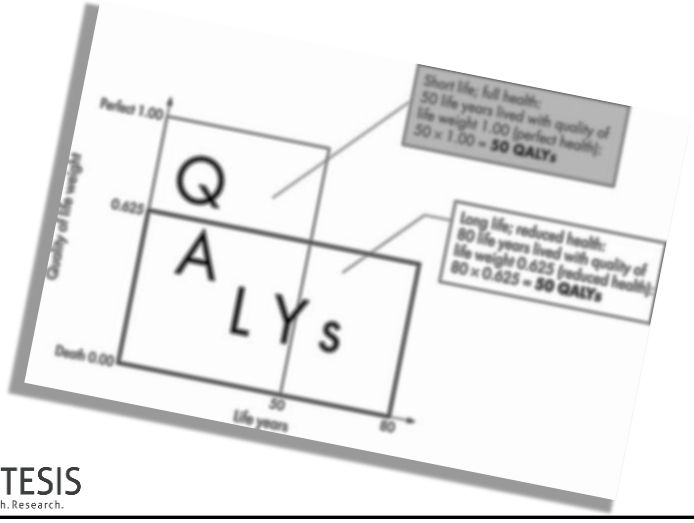
Methods in Decision Analysis and Decision Modelling – Part I:

Valuing Outcomes, Preferences and Utilities



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Summary

- Contexts and paradigms of decision making
- Preferences and utilities
  - Theoretical foundations of the concept of Utility
  - QALYs and other mixed measures
- Theoretical foundations of the concept of utility
- Methods to measure preferences and utilities
  - Direct elicitation methods
  - Indirect methods

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Contexts and paradigms of decision making

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Contexts and Paradigms

- Decision making in health and medicine should take into account a **diverse set of health outcome dimensions** that should be **tailored** to the **clinical setting, population and patient´s characteristics**

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Contexts and Paradigms

- Some of the most relevant **types of health outcome dimensions**:
  - Mortality
  - Survival
  - Morbidity
  - Functional disability
  - Quality of life
  - Satisfaction
- Some of these outcome dimensions are **difficult to measure** and are based on **value judgments** and **preferences**, which are dependent upon **subjective criteria of individuals, groups or the society where they belong**

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Contexts and Paradigms

A patient's history as an example…

- **A woman 28 years old**, with a **heavy family history of breast cancer** (including a sister with breast cancer diagnosed at 35 years old)
- Genetic testing revealed that **her sister** was **carrier of a mutation in the BRCA1 gene**
- She was **worried about her individual risk** of developing breast cancer and she decides to **perform the genetic testing** herself and **ends up finding she is also a carrier of the same mutation**
- She was informed that **the life-time risk of being affected by breast cancer in carriers of the mutation in the BRCA1 gene is estimated to be around 50%**

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Contexts and Paradigms

- With a **tight follow-up**, including periodical mammograms, it is probable that, if she is affected by breast cancer, the tumour **could be detected in a relatively initial phase** (she has a **probability of having a stage I disease of 80%**)
- With an **adequate treatment**, including surgery, radiotherapy and chemotherapy, the **probability of disease recurrence in a stage I disease is 30% at 10 years, and 1% each year after that**. Three in four **(75%) women with disease recurrence will have metastatic invasion of the tumour**, and those will have a **mortality rate of 40%** per year
- Alternatively, this woman **may choose to perform a bilateral prophylactic mastectomy** and **reduce her risk of breast cancer by nearly 90%**

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Contexts and Paradigms

- The sensible decision this woman is facing involves a **complex and difficult weighting** of the **benefits associated with a prophylactic mastectomy** against the **huge and relevant impact of this radical and eventually unnecessary intervention** in her **quality of life, functional capacity, social life, sexual life, body self-image, self-esteem, satisfaction and breast-feeding ability**

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Contexts and Paradigms

- There are contexts and situations where we are faced with temporal periods of **survival in health states that may be considerably worse than a hypothetical perfect state of health**, not affecting survival itself but the quality of life within the survival time
- And this lead us to an important question…

  How to value different health states and define a formal method and measure allowing us to value and compare the quality of life associated with different health states?

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Contexts and Paradigms

- **Decision Making Perspectives**
  - **Individual perspective**
  - **Society and/or group perspective**

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Decision Making Perspectives

- **Individual perspective**
  - **The paramount significance in these settings of the doctor–patient relationship**
  - In scenarios like these the physician and the patient are faced with a need to make a **decision**, **choosing among a set of different treatment and management strategies**
  - The **best choice** will always be the one that **best fit the needs and preferences of the patient**

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Decision Making Perspectives

- **Individual perspective**
  - **Most patients prefer to be informed** about their health problems and the therapeutic and clinical management options
  - However, although most of them want to be informed, their **willingness and preferred degree of autonomy in the decision-making process is highly variable**

Degner LF, Sloan JA. **Decision making during serious illness: what role do patients really want to play?** *J Clin Epidemiol* 1992;45(9):941-50.

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Decision Making Perspectives

- **Individual perspective**
  - In these settings the healthcare professional needs to be able to **perceive and understand what are the patient's needs and wants regarding information and decision support**
  - The ideal situation would be one in which **the patient is able to apply his or her own sets of values and preferences to inform and weight the potential risks and benefits of each decision alternative**; and he or she would be able to **choose the option that best maximizes health outcomes taking into account his or her personal and subjective set of preferences**

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Decision Making Perspectives

- **Individual perspective**
  - However, in practice, often the patients end up asking the healthcare professional – "**What would you do if you were in my situation?**"
  - The healthcare professionals need to understand that only the **consideration of value judgments and preferences allow us in these cases to resolve the choice** between, for example, more survival time with worse quality of life versus less survival time with preserved or better quality of life

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Decision Making Perspectives

- **Individual perspective**
  - The healthcare professional should **try to perceive, understand, assess and apply the value judgments and preferences of that particular patient**, making a decision for the **alternative that maximizes the health outcomes for that particular patient taking into account his or her own preferences**
  - **The paramount importance of the doctor-patient relationship and its quality**
  - **This is usually obtained informally and qualitatively during the clinical interview**, through a series of questions and an effective communication with the patient

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Decision Making Perspectives

- **Perspective of the society/group**
  - Many times healthcare professionals and decision makers are faced with decisions that **involve not only the more restricted settings where individual perspectives should take primacy** but instead **involve broader settings where the perspectives of the society or groups of individuals should be taken into account**
  - In general, this is the relevant perspective in **health policy decision-making and decisions regarding healthcare technology**

Decision Making Perspectives

- **Perspective of the society/group**
  - The **decision makers, policy makers, administrators and managers of healthcare services and healthcare systems, and the healthcare professionals in general**, are faced with the **challenge of turning into a reality the evidence-based decision-making approach and systematically assessing the best treatment and clinical management alternatives for their patients/users/clients**, taking **into account not only their effectiveness, but also trying to allocate the available limited resources in a balanced and fare way and taking also into account the preferences and value judgments of individuals, groups and the society**

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Decision Making Perspectives

- **Perspective of the society/group**
  - Healthcare decision-making and evidence-based decision making should always take into account the **clinical effectiveness of alternatives** and the **value judgments and preferences of the patients/users/clients as a group** aiming to **maximize health outcomes and never look exclusively at managerial or cost-saving criteria**

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Preferences and utilities

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Preferences and Utility

- The questions arising when considering the need to assess and apply patient's preferences are: (1) **how to define and measure preferences?** and (2) **how to develop measures that could be able to take into account simultaneously quality and quantity of life?**
- **Probabilities of survival, expected survival and life expectancy** are **measures valued in accordance with their intrinsical meaning and significance**; thus, it is **easy to predict that individuals will prefer longer periods of survival and higher probability of survival**

Preferences and Utility

- Most frequently healthcare policies and interventions affect not only the probability and duration of survival but also the quality of life, functional capacity, disability and morbidity
- Whenever we are interested in systematically assessing and quantifying aspects associated with the quality of life, functional capacity, disability and morbidity there are two key concepts we should take into account:
  - **Preferences**
  - **Utility**

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Preferences and Utility

- **Preferences**
  - **Level of satisfaction or dissatisfaction, desire or rejection, that individuals, groups or populations associate to a given health state or health outcome**

Froberg DG, Kane RL. **Methodology for measuring health-state preferences--I: Measurement strategies.** *J Clin Epidemiol* 1989;42(4):345-54.

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Preferences and Utility

- **Utility**
  - **A quantitative measure of the degree of preference or level of satisfaction or dissatisfaction, desire or rejection, that individuals, groups or populations associate to a given health state or health outcome**
  - **An utility measure should follow a strict set of criteria and axioms that formally define a quantitative measure**

Torrance GW. **Utility approach to measuring health-related quality of life.** *J Chronic Dis* 1987;40(6):593-603.

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Preferences and Utility

- When decision analysis takes into account preference-based measures and utilities is called an **utility analysis**
- Following the same principle, when an economic evaluation, for example, a cost-effectiveness andalysis takes into account preference-based measures and utilities is called a **cost-utility analysis**
- Preference-based measures and utilities are incorporated in decision analysis or economic evaluations as measures used to **weight and value preferences of individuals, groups or the society for certain health states or health outcomes**

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Preferences and Utility

- A good example are measures such as ***QALYs***
  - A measure that allows to **calculate the survival time adjusted for and weighted by the preferences of individuals or the society for certain health states or health outcomes**
  - We intrinsically accept the **trade-off between survival time and quality of life; thus, with higher preferences**
- To be able to calculate QALYs we will have first to **be able to formally and systematically measure preferences and utilities**, using **explicit, valid and reliable measurement methods and instruments**

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Preferences and Utility

- **Appropriate methods and instruments should:**
  - Adequately **describe a set of possible health states**, from the worst imaginable (defined as death as an anchor) to the best health state possible
  - In those descriptions **all relevant attributes should be included** (difficult task to accomplish)
  - Quantify preferences of individuals or populations for each health state or health outcome using **appropriate preference-based measures with adequate properties (utility measures)**

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Preferences and Utility

- **Utilities**
  - **Should vary in a continuous numerical scale in the interval 0 to 1**, where 0 represents the worst health state possible (assumed to be death as na anchor), with the lesser preference; and 1 represents the best health state imaginable, with the highest preference
  - Utilities should be **measured in interval scales**, meaning that, for example, the difference between preference of a health state with utility of 0.5 and another with 0.4 should be the equal to the difference between a health state utility of 0.3 and another of 0.2

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Preferences and Utility

- **Utility**
  - We will end up with a continuous numerical scale between 0 and 1!
  - An example of how to calculate QALYs:
    - Living during 10 years in a health state with 0.5 utility
    - This should be equivalent to live 5 years in a health state with perfect health (utility of 1)
    - QALYs are calculated as follows:

$$0.5 \times 10 = 5$$

In this case this euqals 5 QALYs; thus, 5 quality adjusted life-years

This is to say that living 10 years in a health state with utility 0.5 is equivalent to live 5 years in a health state with perfect health

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QALYs and other similar measures

- ***Quality adjusted life-years (QALY)***
  - As previously stated, the great advantage of QALYs as a health outcome measure is to be able to **capture and accommodate simultaneously health gains in terms of mortality and survival time (quantity of life) and quality of life/disability/morbidity (quality of life)**; and combine them in a **single measure based on the relative preferences of individuals or the society for different health states or health outcomes**

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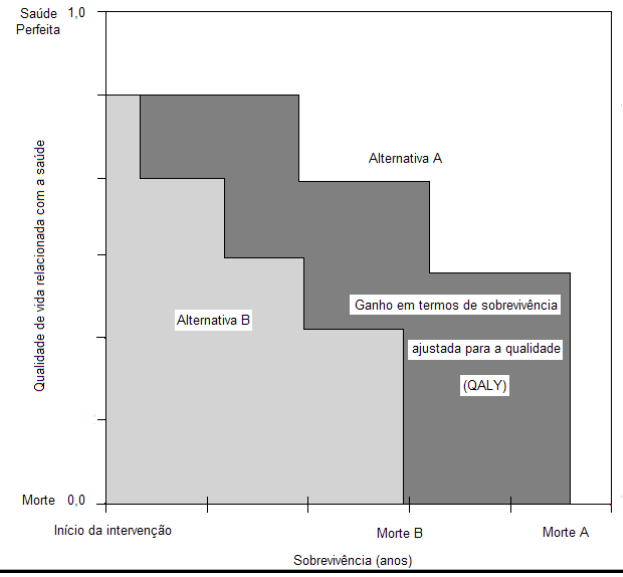
Graphical representation of the health related quality of life as a function of survival time for two alternatives being compared



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QALYs and other similar measures

- ***Quality adjusted life-years (QALY)***
  - To be able to calculate QALYs we should have **appropriate preference-based measures, methods and instruments to measure utilities, so as to be used as weights when calculating QALYs, allowing the adjustment for the quality of life of the survival time**
  - The **measures, methods and instruments described in the subsequent sections** are precisely the type of tools used to appropriately generate utilities and adequately adjust for the health related quality of life

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QALYs and other similar measures

- ***Quality adjusted life-years (QALY)***
  - To calculate QALYs we should always use adequate preference-based utility measures with the following criteria:
    (1) Adequately **preference-based** following criteria previously discussed
    (2) Be measured in an appropriately **calibrated numerically continuous scale in the interval 0 to 1** (with the anchors 0 and 1 as death and the best health state imaginable, respectively)
    (3) To be **measured in an interval scale**

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QALYs and other similar measures

- ***Quality adjusted life-years (QALY)***
  - To calculate QALYs we simply **multiply utility of agiven health state by the expected survival time in that health state**
  - Other similar measures with different designations:
    - *The US National Centre for Health Statistics* uses the term ***Years of Healthy Life*** **– YHL**
    - *The Statistics Canada* uses the terms ***Health-Adjusted Person-Years*** **(HAPY)** and ***Health Adjusted Life Expectancy*** **(HALE)**

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QALYs and other similar measures

- ***Other analogous mixed measures (measuring simultaneously quality and quantity of life)***
  - **Healthy-Years Equivalents – HYEs**
  - **Saved-Young-Life Equivalents – SAVEs**
  - **Disability-Adjusted Life-Years – DALYs**
    - **DALYs** were developed in the context of the **Global Burden of Disease and Injury Study** of **WHO**
    - **DALYs** have been **recommended** by WHO in the context of the so called **generalised cost-effectiveness analysis**

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Theoretical foundations of the concept of utility

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Preferences and Utility

- **Theoretical foundations of the concept of utility**
  - This concept is primarily defined at the beginning of the XXth century, in the context of theoreticl developments in game theory, with works focused on decision making under uncertainty currently known as the **Theory of Utility of von Neumann and Morgenstern**
  - They have defined and proposed a set of **rules, criteria and axioms** establishing how a **rational individual** should **behave when making decisions under uncertainty**

Von Neumann J, Morgenstern O. ***Theory of Games and Economic Behaviour.*** Princeton, NJ: Princeton University Press, 1944.

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The four axioms of rational behaviour of von Neumann Morgenstern

Axiom 1 (Completeness) For any lotteries L, M, exactly one of the following holds:

$$L \prec M,\ M \prec L,\ \text{or}\ L \sim M$$

(either M is preferred, L is preferred, or the individual is indifferent[5]).

Transitivity assumes that preferences are consistent across any three options:

Axiom 2 (Transitivity) If $L \prec M$ and $M \prec N$, then $L \prec N$, and similarly for $\sim$.

Continuity assumes that there is a "tipping point" between being *better than* and *worse than* a given middle option:

Axiom 3 (Continuity): If $L \preceq M \preceq N$, then there exists a probability $p \in [0, 1]$ such that

$$pL + (1 - p)N \sim M$$

where the notation on the left side refers to a situation in which L is received with probability p and N is received with probability $(1-p)$.

Instead of continuity, an alternative axiom can be assumed that does not involve a precise equality, called the Archimedean property.[4] It says that any separation in preference can be maintained under a sufficiently small deviation in probabilities:

Axiom 3' (Archimedean property): If $L \prec M \prec N$, then there exists a probability $\varepsilon \in (0, 1)$ such that

$$(1 - \varepsilon)L + \varepsilon N \prec M \prec \varepsilon L + (1 - \varepsilon)N.$$

Only one of (3) and (3′) need be assumed, and the other will be implied by the theorem.

Independence of irrelevant alternatives assumes that a preference holds independently of the possibility of another outcome:

Axiom 4 (Independence): If $L \preceq M$, then for any N and $p \in [0, 1]$,

$$pL + (1 - p)N \preceq pM + (1 - p)N.$$

The independence axiom implies the axiom on reduction of compound lotteries:[6]

Axiom 4' (Reduction of compound lotteries): For any lotteries L, L', N, N' and any $p, q \in [0, 1]$,

$$\begin{array}{ll} \textit{if} & L \sim qL' + (1 - q)N', \\ \textit{then} & pL + (1 - p)N \sim pqL' + p(1 - q)N' + (1 - p)N. \end{array}$$

To see how Axiom 4 implies Axiom 4', set $M = qL' + (1 - q)N'$ in the expression in Axiom 4, and expand.

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Rules of Actional Thought

- **Ronald Howard's version of the decision making axioms proposed by John von Neumann and Oscar Morgenstern** in their classic work on game theory (1944, 1947)
- Simple, intuitive guidelines to follow when making decisions
- A set of five rational, consistent rules for a normative decision maker to follow

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The Probability Rule

- Decision makers use elemental and compound possibilities and probabilities to provide distinctions and information that characterize deals (choices) and the tradeoffs they entail
  - The clarity test*: Crucial for making clairvoyance meaningful and useful
  - Relevance of events
  - Mutual exclusion of elemental possibilities
  - Collective exhaustion of elemental possibilities

* In decision analysis, the clarity test (or clairvoyant test) is a test of how well a model element is defined. Although nothing (outside a formal system) can be completely defined, the clarity test allows the decision participants to determine whether such elements as variables, events, outcomes, and alternatives are sufficiently well defined to make the decision at hand. In general, a model element is well defined if a knowledgeable individual can answer questions about the model element without asking further clarifying questions.

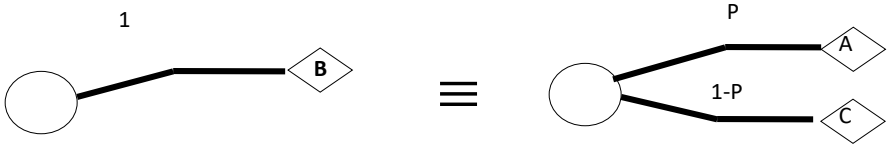
The Order Rule

- Prospects (values of outcomes of deals) can be arranged in a (weakly) descending **order from best to worst**
- The **order of prospects is consistent and transitive**
  - e.g. A>B, B>C => A>C
  - Nontransitive orders lead to a "money pump" (see Davidson, McKinsey and Suppes, 1955)

The Equivalence Rule

- If **A>B>C, then there is a number 0<p<1 such that the decision maker is indifferent between getting prospect B for sure, and receiving a deal with probability p of getting A and probability 1-p of getting C**
  - P is the **preference probability** of this model
  - B is the **certain equivalent** of the A,C deal

Preference Probabilities



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The Substitution Rule

- The decision maker has to be **indifferent between receiving a prospect and any deal for which that prospect is a certain equivalent**
  - B can be substituted for the A,C deal in any situation
  - Implies treatment of preference probabilities as probabilities that might lead to action

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The Choice Rule

- If the prospect ordering **includes D>E, and there are two deals with outcomes D,E, the decision maker must prefer the deal in which the probability of getting D is higher**
  - The only specific-action rule
  - Simply states that decision makers follow their preferences, whatever these are

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Lotteries and Normative Axioms

- John von Neumann and Oscar Morgenstern (VNM) in their classic work on game theory (1944, 1947) **defined several axioms a rational (normative) decision maker might follow with respect to preference among *lotteries***
- The VNM axioms **state our rules of actional thought more formally with respect to preferring one lottery over another**
- A **lottery** is a **probability function from a set of states *S* of the world into a set *X* of possible prizes**

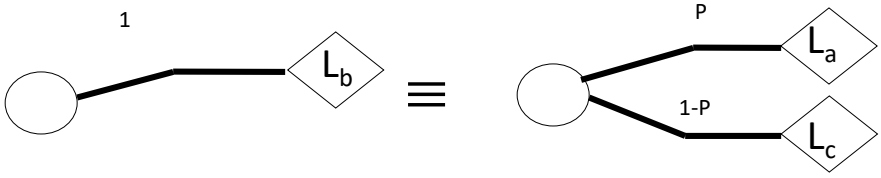
Utility Functions

- Assuming a **lottery *f* with a set of states *S* and a set of prizes *X*, a utility function is any function *u*:*X* x *S* -> R** (that is, into the real numbers)
- One important utility function of an outcome *x* is the one assessed by asking the decision maker to assign a **preference probability** among the worst outcome X_0 and the best outcome X_1
  - Note: There *must* be such a probability, due to the **continuity axiom** (our *equivalence rule*)

The Continuity Axiom

- If there are **lotteries L_a, L_b, L_c; $L_a > L_b > L_c$ (preference relation),** then there is a **number 0<p<1 such that the decision maker is indifferent between getting lottery L_b for sure, and receiving a compound lottery with probability p of getting lottery L_a and probability 1-p of getting lottery L_c**
  - P is the **preference probability** of this model
  - B is the **certain equivalent** of the L_a, L_c deal

Preference Probabilities



B is the **Certain Equivalent** of the lottery < L_a, p; L_c, 1-p>

The Expected-Utility Maximization Theorem

- Theorem: The **VNM axioms are jointly satisfied iff there exists a utility function *U* in the range [0..1] such that lottery *f* is (weakly) preferred to lottery *g* iff the expected value of the utility of lottery *f* is greater or equal to that of lottery *g***
  - Note: The **proof shows that the preference probability (and its linear combinations) in fact satisfies the requirements**

Implications of Utility Maximization to Decision Making

- Starting from relatively very weak assumptions, VNM showed that there is **always a utility measure that is maximized, given a *normative* decision maker that follows intuitively highly plausible behavior rules**
- **Maximization of expected utility could even be viewed as an evolutionary law** of maximizing some survival function
- However, **in reality (*descriptive behavior*) people often violate each and every one of the axioms**!

The Allais Paradox (Cancellation)

- **1 – What would you prefer:**
  - A: $1M for sure
  - B: a 10% chance of $5M, an 89% chance of $1M, and a 1 % chance of getting $0 ?
- **2 – And which would you like better:**
  - A: an 11% chance of $1M and an 89% of $0
  - B: a 10% chance of $5M and a 90% chance of $0

Using the values above and a utility function *U*(*W*), we can demonstrate exactly how the paradox manifests.
Because the typical individual prefers 1A to 1B and 2B to 2A, we can conclude that the expected utilities of the preferred is greater than the expected utilities of the second choices, or,

Experiment 1

$$1U(\$1\text{ M}) > 0.89U(\$1\text{ M}) + 0.01U(\$0\text{ M}) + 0.1U(\$5\text{ M})$$

Experiment 2

$$0.89U(\$0\text{ M}) + 0.11U(\$1\text{ M}) < 0.9U(\$0\text{ M}) + 0.1U(\$5\text{ M})$$

We can rewrite the latter equation (Experiment 2) as

$$0.11U(\$1\text{ M}) < 0.01U(\$0\text{ M}) + 0.1U(\$5\text{ M})$$

$$1U(\$1\text{ M}) - 0.89U(\$1\text{ M}) < 0.01U(\$0\text{ M}) + 0.1U(\$5\text{ M})$$

$$1U(\$1\text{ M}) < 0.89U(\$1\text{ M}) + 0.01U(\$0\text{ M}) + 0.1U(\$5\text{ M}),$$

which contradicts the first bet (Experiment 1), which shows the player prefers the sure thing over the gamble.

Methods for Measuring Preferences and Utilities

Measuring Preferences and Utilities

- **Methods for Measuring Preferences and Utilities**
  - **It is common to use instruments or questionnaires designed to measure health related quality of life (HRQoL) as indirect measures of preferences and utilities; however, not all instruments designed to measure HRQoL are appropriate to measure preferences and utilities**; thus, not all of them are useful in the context of utility analysis, cost-utility analysis or to calculate QALYs

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Measuring Preferences and Utilities

- **Methods for Measuring Preferences and Utilities**
  - Preference-based measures may be categorized in two groups – **direct measures** and **indirect measures**
    - In **direct measures** the elicitation of preferences for a given set of health states is performed directly in individuals or samples from the target population using one of the direct methods of preferences elicitation (utility estimation) – **standard gamble**, **time trade-off** or **direct measurement or classification scales**
    - In **indirect measures** the elicitation of preferences is performed **based on the set of individual responses or calculated indices from an HRQoL instrument (health profile)** linked to a **set of weights coming from an independent source where they have been defined as to appropriately represent the preferences (utilities) of the target population** for each health state

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Measuring Preferences and Utilities

- **Direct Methods**
  - Standard gamble
  - Time trade-off
  - Direct measurement or classification scales
  - Other methods

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Measuring Preferences and Utilities

- **Standard Gamble**
  - This is a method suggested by **the utility theory of von Neumann and Morgenstern** itself and it is considered as the reference gold standard method for measuring preferences and utilities; because it **adequately represents the conceptual framework and the experimental operational conditions of decision making under uncertainty**
  - The practical application of this method **puts the individuals in an hypothetical situation where he has to choose between two alternatives**
  - One of the alternatives is to **stay permanently in a given health state i** and the other alternative entails **a sort of gamble with two possible outcomes – full health state and death – with certain known probabilities of occurrence – p and 1-p, respectively**

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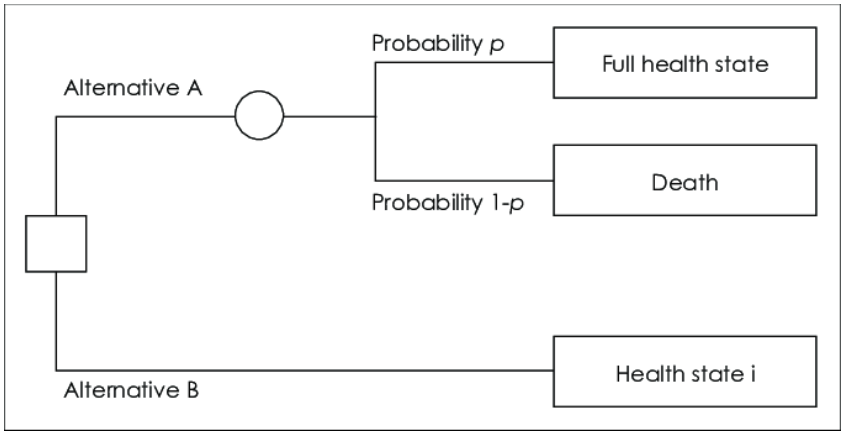
Measuring Preferences and Utilities

- **Standard Gamble**



- **Where full health ≥ Hi ≥ death**
- **Vary 'p' until the respondent is indifferent between the alternatives A and B**
- **The 'p' of indifference = the utility of Health state i**

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Measuring Preferences and Utilities

- **Standard Gamble**
  - In order to **elicit the subject's preference and utility of a given health state i**, the participant is subjected **repeatedly to this hypothetical decision scenario with varying values of 'p', and is asked to make a decision as to what alternative he prefers**
  - The utility elicitation exercise ends when the **subject reveals his point of indifference**, the **value of 'p' for which he is indifferent between the two alternatives available**
  - The **value 'p' revealed as the point of indifference corresponds to the utility of the health state i**

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Measuring Preferences and Utilities

• Time trade-off

- The **time trade-off (TTO) method** was proposed as an **alternative to the standard gamble** by Torrance, Thomas and Sackett in 1972
- The participant is also **subjected to an hypothetical decision making scenario with two alternatives**:
  1 – The **health state i during a period of time t** (e.g. expected survival of subjects with a given chronic disease), followed by death
  2 – The **full health state during a period of time x < t, followed by death**
- The duration of the period of time x is repeatedly changed until a point of indifference is defined where the individual has equal preference or is indifferent between the alternatives
- **The preference and utility of health state i given by the time trade-off method is calculated as the ratio x / t at the point of indifference**

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Measuring Preferences and Utilities

• Time trade-off

- The underlying concept is the **trade off between quantity and quality of life as measured by the preference of individuals for shorter or longer periods of time in a given health state**
- As an example, an **individual may be willing to accept 9 years of expected survival time without angina pectoris** in **exchange for 10 years of expected survival with angina pectoris**
- The preference or utility for this health state is given by the ratio between the time period associated with the full health state and the time in the health state under evaluation, at the indifference point; thus, in this case the **utility of angina pectoris as measured by the time trade-off method would be 9/10 or 0.9**

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Measuring Preferences and Utilities

- **Direct measurement or classification scales and visual analogue scales**
  - **Qualitative ordinal rating scales** (ordinal rating scales with descriptors assumed to be equally spaced along the scale construct)
  - **Numeric rating scales** (numeric rating scale – NRS, for example, 0-100)
  - **Visual analogue scales** (visual analogue scales – VAS, usually a 10 centimetres line in a blank page with clear limit lines)
  - **Combined methods**, as an example, McMaster university uses a "feeling thermometer", resulting from the combination of a VAS and a 0-100 NRS

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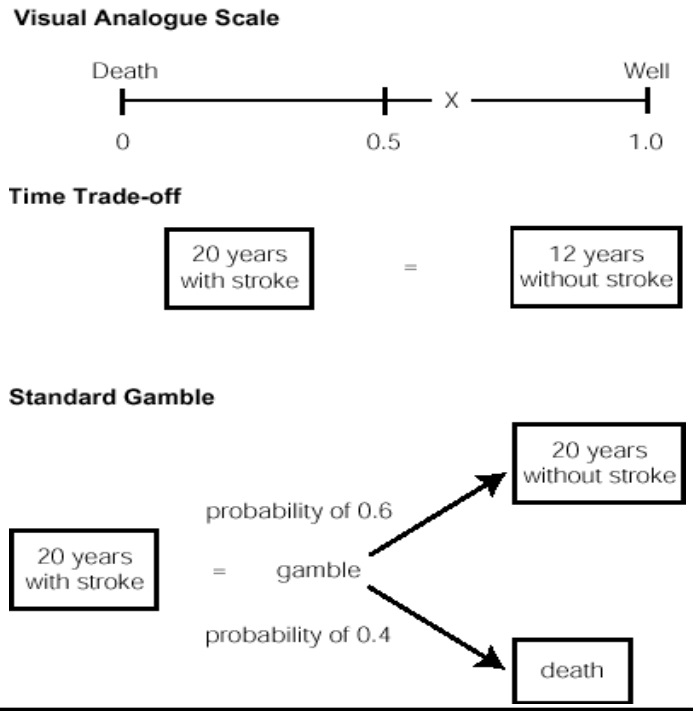
A practical example of elicitation for a given health state with utility of 0.6:



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Measuring Preferences and Utilities

- **Indirect Methods**
  - Measuring preferences and utilities using the direct methods just described may be a difficult and time consuming task
  - A more practical alternative often used is the elicitation of preferences and utilities using **indirect methods based on the classifications or ratings of generic HRQoL measures associated with a set of preference weights obtained independently that allow the indirect estimation of preferences and utilities of different health states**

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Measuring Preferences and Utilities

- **Methods for Measuring Preferences and Utilities**
  - **It is common to use instruments or questionnaires designed to measure health related quality of life (HRQoL) as indirect measures of preferences and utilities; however, not all instruments designed to measure HRQoL are appropriate to measure preferences and utilities**; thus, not all of them are useful in the context of utility analysis, cost-utility analysis or to calculate QALYs

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Measuring Preferences and Utilities

- **Methods for Measuring Preferences and Utilities**
  - Instruments or questionnaires designed to measure health related quality of life (HRQoL) may be generically categorized as:
    - **Health profiles** – Generic measures cover all relevant areas of HRQoL (including, for example, mobility, self-care, and physical, emotional, and social function), and specific measures also cover dimensions that are specific to a given clinical context. Health profiles give rise to numerical indices or qualitative descriptors for each relevant HRQoL dimension; however, they are merely descriptive of a given health state and do not measure the preferences of individuals or the society for those health states.
    - **Utility measures** – These are preference-based measures and allow the estimation of preferences of individuals or the society for a given health state based on the description of health states, eventually linked to a health profile, and a direct or indirect estimate of preferences associated with that health state in the form of utilities. Only these instruments may be used to calculate QALYs.

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Measuring Preferences and Utilities

- **Multi-attribute health status classifications systems associated with preference measures**
  - Within the broader set of all the generic HRQoL measures there are a subgroup of **instruments including multi-attribute health status classification systems linked to preference weights algorithms allowing the estimation of preferences and utilities for different health states**
  - These subgroup of instruments allow the **characterization of each health state using a multi-attribute classification system** (e.g. mobility, personal care, activities of daily living, social function, anxiety-depression, pain, etc.) and **estimate preferences and utilities using preference weights algorithms**, developed through **direct valuation exercises – valuation experiments – in representative samples from the target population**

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Measuring Preferences and Utilities

- **Multi-attribute health status classifications systems associated with preference measures**
  - **Valuation research** aims to measure people's preferences with respect to health. This involves the **participation of a representative sample of people from the target population** in a **standardized valuation experiment**
  - In the experiment, **participants are asked to value health by reviewing health states described by the multi-attribute classification system**
  - The **result from the valuation study** is a **value set for that target population**
    - This is essentially a **set of weights for each of the levels** of the multiple dimensions. A large weight means that people believe that a particular level of severity has a large impact on health-related quality of life.
    - **Using the set of weights**, one can **convert each health state described by the multi-attribute system** into a single **summary index value** corresponding to the **estimate of the preference or utility of that health state according to the target population valuations**

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Measuring Preferences and Utilities

- ***Multi-attribute health status classifications systems associated with preference measures***
  - **The most popular systems or instruments of this kind are:**
    - Quality of Well-Being (QWB)
    - Health Utility Index (HUI)
    - EQ-5D do EuroQoL Group
    - Short Form 6D (SF-6D) associated with the Medical Outcomes Study – Short Form 36 (SF-36)

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Measuring Preferences and Utilities

- ***EQ-5D***
  - **The EuroQoL Group** initially developed a multi-attribute classification system with 6 attributes: mobility, self-care, usual activities, social interactions, pain/discomfort, and anxiety/depression
  - Subsequently a revised version was developed with the **current five dimensions**:

    -**mobility,**
    -**self-care,**
    -**usual activities**
    -**pain/discomfort**
    -**anxiety/depression**

  - The most current version has five levels for each dimension (no problems, slight problems, moderate problems, severe problems and being unable to do/extreme problems), defining 3,125 possible health states. The original version had 3 levels in each dimension
  - **The models or algorithms available to calculate estimates of preferences or utilities for each health state form 0 (death) to 1 (full health) were developed form representative samples of several different populations using valuation experiments based on TTO or SG**

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Under each heading, please tick the **ONE** box that best describes your health **TODAY**

MOBILITY
- I have no problems in walking about ❑
- I have slight problems in walking about ❑
- I have moderate problems in walking about ❑
- I have severe problems in walking about ❑
- I am unable to walk about ❑

SELF-CARE
- I have no problems washing or dressing myself ❑
- I have slight problems washing or dressing myself ❑
- I have moderate problems washing or dressing myself ❑
- I have severe 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 doing my usual activities ❑
- I have slight problems doing my usual activities ❑
- I have moderate problems doing my usual activities ❑
- I have severe problems doing my usual activities ❑
- I am unable to do my usual activities ❑

PAIN / DISCOMFORT
- I have no pain or discomfort ❑
- I have slight pain or discomfort ❑
- I have moderate pain or discom ❑
- I have severe pain or discomfort ❑
- I have extreme pain or discomfort ❑

ANXIETY / DEPRESSION
- I am not anxious or depressed ❑
- I am slightly anxious or depressed ❑
- I am moderately anxious or depressed ❑
- I am severely anxious or depressed ❑
- I am extremely anxious or depressed ❑

Current version with five levels in each dimension – EQ-5D-5L

Classic older version with three levels in each dimension – EQ-5D-3L

By placing a tick in one box in each group below, please indicate which statements best describe your own health state today.

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 ❑

Measuring Preferences and Utilities

- ***EQ-5D (an example with the UK model – Dolan D1)***

Full health	= 1.000
Constant term (for any dysfunctional state)	- 0.081
Mobility (level 1)	- 0
Self-care (level 1)	- 0
Usual activities (level 2)	- 0.036
Pain or discomfort (level 2)	- 0.123
Anxiety or depression (level 3)	- 0.236
N3 (level 3 occurs within at least one dimension)	- 0.269
Therefore, the estimated value for 11223	= 0.255

DIMENSION	COEFFICIENT
Constant (a)	0.081
Mobility	
level 2	0.069
level 3	0.314
Self-care	
level 2	0.104
level 3	0.214
Usual activity	
level 2	0.036
level 3	0.094
Pain/discomfort	
level 2	0.123
level 3	0.386
Anxiety/depression	
level 2	0.071
level 3	0.236
N3	0.269
adjusted r^2	0.46

Paul Dolan, Claire Gudex, Paul Kind, Alan Williams, ***A social tariff for EuroQol: results from a UK general population survey***, September 1995

Recommended readings

Highly recommended

- Capítulo 4 ("**Valuing outcomes**") from the book "Hunink MGM, Weinstein MC, Wittenberg E, Drummond MF, Pliskin JS, Wong JB, Glasziou PP. **Decision making in health and medicine : integrating evidence and values**. Cambridge ; New York: Cambridge University Press, 2014."

Additional papers

- Neumann PJ, Goldie SJ, Weinstein MC. **Preference-based measures in economic evaluation in health care**. Annual review of public health. 2000 May;21(1):587-611.
- Ferreira LN, Ferreira PL, Pereira LN, Oppe M. **The valuation of the EQ-5D in Portugal**. Quality of Life Research. 2014 Mar 1;23(2):413-23.
- Ferreira LN, Ferreira PL, Pereira LN, Brazier J, Rowen D. A **Portuguese Value Set for the SF-6D**. Value in Health. 2010 Aug 1;13(5):624-30.