

LECTURE 2

BASICS OF DM IN HEALTH CARE

Basics of DM in Health Care

- In the previous section we discussed different tools that help DMs responsible for health care resource allocation decisions
 - Cost minimisation
 - Cost Effectiveness Analysis
 - Cost-Benefit Analysis
 - Cost-Utility Analysis

Basics of DM in Health Care

- In this lecture we will discuss the following:
 - The five steps of evaluation of new or existing health care interventions from the clinical and policy perspectives
 - How do we calculate Incremental Cost-Effectiveness (Cost-Utility) ratios?
 - How do we use economic evaluation results (CEA, CUA, CBA) in funding decisions?
 - What are the theoretical foundations of such evaluations?
 - Things to bear in mind while comparing different studies
 - Other issues

The five steps of evaluation of HC interventions

1. Demonstration of efficacy:

Showing that the intervention can achieve its stated goal *under optimal circumstances*

Showing that a new antihypertensive agent lowers blood pressure when taken by patients in very controlled settings (where compliance is assured and salt intake is monitored)



The five steps of evaluation of HC interventions

2. Assessment of effectiveness:

Showing that the intervention does more good than harm when used *in usual circumstances*

Our antihypertensive agent (drug) would be considered effective if it achieved its goal in ordinary patients (whose compliance may not be as good and whose diet may not be controlled)

The five steps of evaluation of HC interventions

3. Assessment of efficiency (via economic analysis, CEA, CUA, CBA):

This considers both consequences of a health care intervention and the resources required to deliver the intervention

Efficiency: achieving the maximal increment in health benefit given a fixed amount of resources

Asking whether more good is done by adopting the new antihypertensive agent in place of an older one instead of devoting the extra resources (required for the new agent) to some alternative new health care programme (e.g. Screening hypercholesterolemia)



A new AHT agent



Screening hypercholesterolemia

The five steps of evaluation of HC interventions

4. Ensuring Availability:

Matching supply to locations such that they are accessible to persons who require them

<http://www.forbes.com/sites/larryhusten/2013/04/09/lifesaving-heart-rhythm-drug-unavailable-in-most-of-world/>

Lifesaving Heart Rhythm Drug Unavailable In Most Of World



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Quinidine— the only drug known to be effective in preventing lethal ventricular arrhythmias in people with several rare conditions, including Brugada syndrome, idiopathic ventricular fibrillation, and early repolarization syndrome— is no longer available in much of the world.

In a study published online in the *Journal of the American College of Cardiology*, Sami Viskin and colleagues surveyed physicians around the world about the availability of quinidine in their country and received responses from 273 physicians in 131 countries. In 76% of the countries quinidine was not available at all. In another 10% quinidine was available only through a regulatory process that can take several days to several months to obtain the drug. Quinidine was easily obtained in only 14% of countries.

The researchers also obtained information about 22 patients with serious arrhythmias that were probably or possibly related to the unavailability of quinidine.

In most other cases of drug shortages, the authors note, the unavailability of life-saving drugs is usually related to the high cost of the drugs. In this case, however, the lack of availability is due to the “low price and restricted indication for a low prevalence disease, rendering unfavorable pharmaceutical market forces from the perspective of industry.”

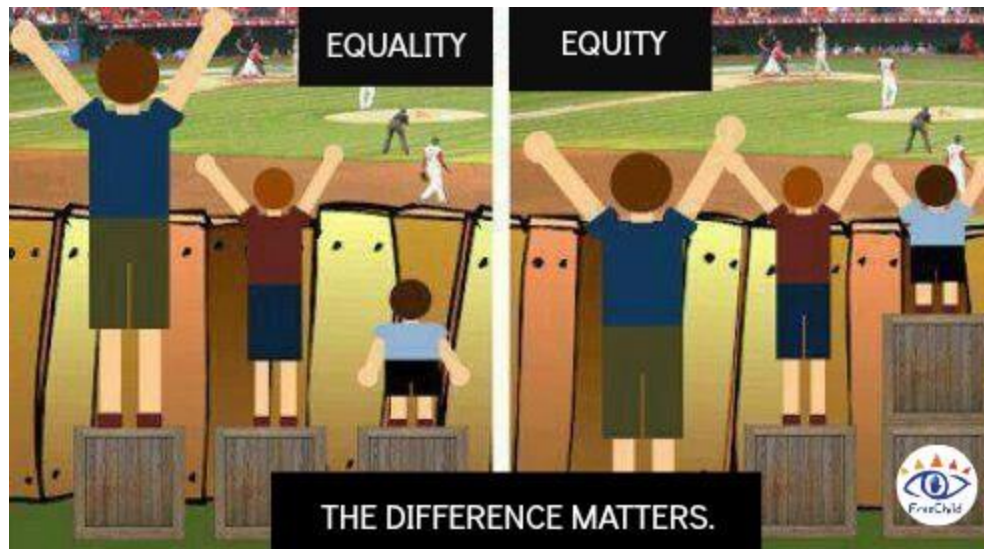


The five steps of evaluation of HC interventions

5. Distributional concerns:

Examining who gains and who loses as a result of allocation decisions

We will focus on Step 3. Efficiency analysis



Cost Effectiveness

- In order to assess the cost-effectiveness of a new intervention, it must be compared to at least one other intervention.
- Typically, the new intervention under study is compared to the commonly accepted intervention (e.g. “the usual care”, which may imply “doing nothing”)
- Two estimations must be made: the extra cost (ΔC) and the extra effect (ΔE) of the new treatment (in case of CUA, we estimate the extra utility, and in case of CBA we estimate extra benefit)

Average and Incremental Cost Effectiveness Ratio

Average C/E ratio: The total cost of a programme divided by the effectiveness of a programme compared to “doing nothing”

Implicit assumption: doing nothing has 0 cost and 0 benefit (effect)

Incremental C/E ratio: Incremental cost of a programme divided by the incremental effectiveness compared to the next most effective programme

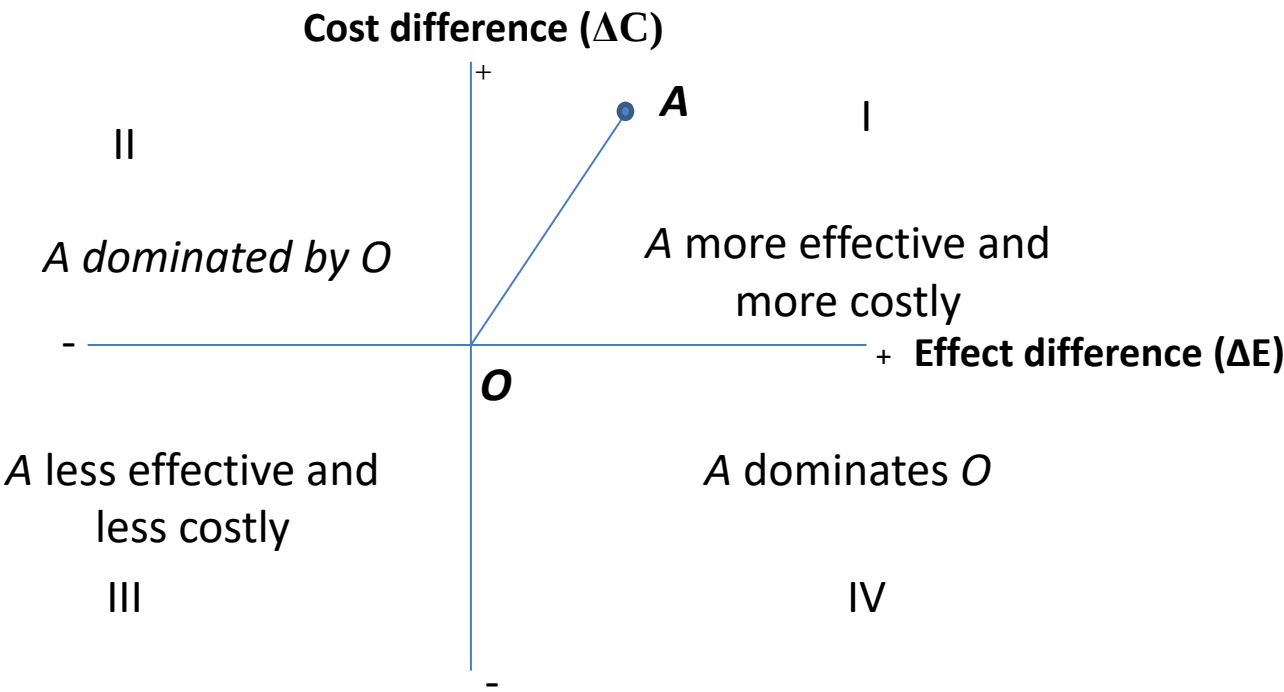
Same definition applies to C/U and C/B ratios!

Cost-Effectiveness

- CEA (CUA, CBA) helps with the allocation decisions by estimating the additional cost per one unit of additional gain.
- The extra cost per extra unit of outcome ($\Delta C/\Delta E$, $\Delta C/\Delta U$, $\Delta C/\Delta B$) represents the incremental (cost-effectiveness, cost-utility, cost-benefit) ratio for strategy A compared to B
- $ICER = (Cost(A) - Cost(B)) / (Effect(A) - Effect(B))$
- ICERs can be compared with those of other interventions or with a threshold value representing what is considered cost-effective.

Recall that :

When comparing two treatments, four possibilities exist for the intervention under study:



Use of Economic Evaluation

Economic evaluation is useful when:

- Choosing between **mutually exclusive interventions**, that is implementation of one means that the other will not be implemented or implementing one will change the cost and/or effectiveness of the other
- Allocating a fixed budget across **independent** programmes
- Two programmes 1 and 2 are independent if the costs and effectiveness of programme 1 (2) is not affected by whether programme 2 (1) is implemented or not

Dominance

Strong Dominance:

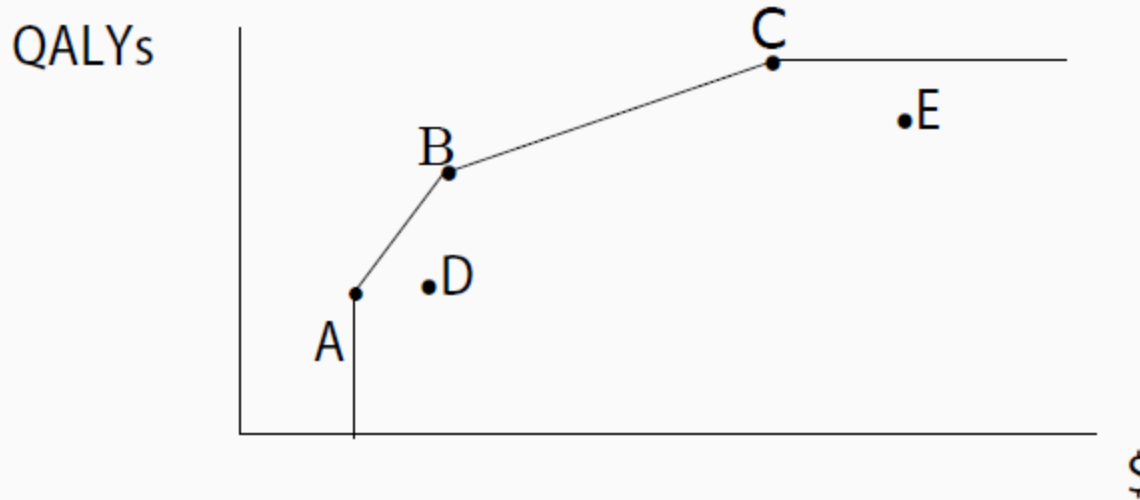
Programme A dominates Programme B if it is **less costly** and **more effective**

Weak Dominance: Extended dominance

A combination of two programmes shows greater cost-effectiveness than other

Eliminates a programme that has an incremental cost effectiveness ratio that is greater than that of a more effective intervention

Dominance



Cost on X axis, Effect on Y axis

Connect every two non-dominated alternatives in a series of line segments

It gets increasingly flatter as we move from less expensive to more expensive alternatives

All dominated alternatives are **below and to the right** of the series of line segment

Example 1: Mutually exclusive interventions

Two methods of treating patients with disease X

(Based on Detsky and Naglie, 1990)

Strategy	Treatment Costs	Effectiveness	Utility (Quality of Life)	Utility (Quality-Adjusted Life Expectancy)	Benefits
Treatment A	\$20000	4.5 years	0.8		\$4000
Treatment B	\$10000	3.5 years	0.9		\$2000
Incremental CER					
Incremental CUR					
Incremental CBR					

Example 1

Strategy	Treatment Costs	Effectiveness	Utility (Quality of Life)	Utility (Quality-Adjusted Life Expectancy)	Benefits
Treatment A	\$20000	4.5 years	0.8	3.6 QALYs	\$4000
Treatment B	\$10000	3.5 years	0.9	3.15 QALYs	\$2000
Incremental CER					
Incremental CUR					
Incremental CBR					

For treatment A: $QALE = 0.8 * 4.5 = 3.6$ QALYs

For treatment B: $QALE = 0.9 * 3.5 = 3.15$ QALYs

Example 1

Strategy	Treatment Costs	Effectiveness	Utility (Quality of Life)	Utility (Quality-Adjusted Life Expectancy)	Benefits
Treatment A	\$20000	4.5 years	0.8	3.6 QALYs	\$4000
Treatment B	\$10000	3.5 years	0.9	3.15 QALYs	\$2000

Incremental CER $\frac{\$20\,000 - \$10\,000}{4.5 \text{ years} - 3.5 \text{ years}} = \$10\,000 \text{ per life-year gained}$

Incremental CUR

Incremental CBR

Example 1

Strategy	Treatment Costs	Effectiveness	Utility (Quality of Life)	Utility (Quality-Adjusted Life Expectancy)	Benefits
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Incremental CER	$\frac{\$20\,000 - \$10\,000}{4.5 \text{ years} - 3.5 \text{ years}} = \$10\,000 \text{ per life-year gained}$				
Incremental CUR	$\frac{\$20\,000 - \$10\,000}{3.6 \text{ QALYs} - 3.15 \text{ QALYs}} = \$22\,222 \text{ per QALY gained}$				
Incremental CBR					

Example 1

Strategy	Treatment Costs	Effectiveness	Utility (Quality of Life)	Utility (Quality-Adjusted Life Expectancy)	Benefits
Treatment A	\$20000	4.5 years	0.8	3.6 QALYs	\$4000
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Incremental CUR	$\frac{\$20\,000 - \$10\,000}{3.6 \text{ QALYs} - 3.15 \text{ QALYs}} = \$22\,222 \text{ per QALY gained}$				
Incremental CBR	$\frac{\$20\,000 - \$10\,000}{\$4000 - \$2000} = 5$				

Example 2

Suppose that you want to choose between the following treatment strategies, which are **mutually exclusive**

Strategy	Cost	Effect (Life years gained)
A	12,500	1,300
B	10,000	1,500
C	14,000	2,200
D	17,000	2,600

Step 1. Arrange alternatives from least expensive to most expensive

Example 2

Strategy	Cost	Effect (Life years gained)
B	10000	1500
A	12500	1300
C	14000	2200
D	17000	2600

Step 2. Eliminate any dominated strategies

Example 2

Strategy	Cost	Effect (Life years gained)
B	10000	1500
A	12500	1300
C	14000	2200
D	17000	2600

Step 2. Eliminate any dominated strategies

A is dominated by B (Why?)

Eliminate A

Is it a strong dominance or a weak dominance?

Example 2

Strategy	Cost	Effect (Life years gained)	Incremental Cost	Incremental Effect	ICER
B	10000	1500			
C	14000	2200			
D	17000	2600			

Step 3. Calculate ICERs
Check Weak Dominance

Example 2

Strategy	Cost	Effect (Life years gained)	Incremental Cost	Incremental Effect	ICER
B	10000	1500	10000	1500	6.67
C	14000	2200	4000	700	5.71
D	17000	2600	3000	400	7.5

Step 3. Calculate ICERs
Check Weak Dominance

Example 2

Strategy	Cost	Effect (Life years gained)	Incremental Cost	Incremental Effect	ICER
B	10000	1500	10000	1500	6.67
C	14000	2200	4000	700	5.71
D	17000	2600	3000	400	7.5

Step 3. Calculate ICERs

Check Weak Dominance

ICER of B > ICER of C (a more effective intervention)

Eliminate B

Example 2

Strategy	Cost	Effect (Life years gained)	Incremental Cost	Incremental Effect	ICER
C	14000	2200	14000	2200	6.36
D	17000	2600	3000	400	7.5

Step 4. If any elimination is made at Step 3, recalculate ICERs and repeat Step 3. Otherwise, go to Step 5.

Step 5. Stop

Both alternatives are cost-effective.

Choice will depend on the budget and maybe some other factors.

What to do next?

Cost-effectiveness is only one of a number of criteria that should be employed in determining whether interventions are made available.

There will be other factors unrelated to C/E ratios that are important to consider.

The DM should decide whether the extra spending on the next most expensive alternative is worthwhile

Example 3

Two screening studies for complications of diabetes



1. A more intensive screening strategy targeted to diabetics with hypertension: This program's expected cost is \$15000 and results in an average of 2.5 life years, with the patients experiencing an average of 80% of full health
2. The usual screening practice costs \$10 000 and yields 2 life years with patients experiencing 75% full health over the time period.

Calculate ICUR

Example 3

Strategy	Expected Cost	Utility (in QALYs)
Hypertension	\$15000	2
Usual Screening	\$10000	1.5

Calculate the utilities in QALYs

Strategy 1: $0.8 * 2.5 = 2$ QALYs

Strategy 2: $0.75 * 2 = 1.5$ QALYs

ICUR = $5000 / 0.5 = \$10000$ per QALY

Funding Decisions

- Economic evaluation of health care programmes can be used to set priorities for funding health care programmes
- The problem is allocating a fixed amount of resources across programmes that compete for these scarce resources

Assumption: the objective is maximizing the net health benefit to a target population using these fixed resources

Target population: the community served by a hospital, or a segment of that community

Using C/E or C/U ratios to set priorities in resource allocation

The ratios of various programmes can be ranked to set funding priorities

The resulting priority list helps DMs maximize the net health benefit that can be achieved from a fixed amount of resources

Resource Allocation to Independent programs

- Suppose that there are three interventions for different patient groups
- Each intervention is **compared with** a “doing nothing” alternative
- Table shows the **net** costs and effects

Intervention	Inc. Cost (\$) (compared to doing nothing)	Inc. Effect (life years gained) (compared to doing nothing)
A	15000	185
B	10000	120
C	12000	135

Resource Allocation to Independent programs

Intervention	Cost(\$)	Effect (life years gained)	Avg C/E
A	15000	185	81.08
B	10000	120	83.33
C	12000	135	88.89

Calculate average C/E

The priority order: A, B, C

Go shopping until your budget is exhausted!

Budget (\$)	Programmes to be implemented
10000	
15000	
20000	
25000	

Resource Allocation to Independent programs

Intervention	Cost(\$)	Effect (life years gained)	Avg C/E
A	15000	185	81.08
B	10000	120	83.33
C	12000	135	88.89

Calculate average C/E

The priority order: A, B, C

Go shopping until your budget is exhausted!

Budget (\$)	Programmes to be implemented
10000	As much of A as budget allows (treat 2/3 of patients)
15000	All of A
20000	All of A +As much of B as remaining budget allows (1/2 of patients)
25000	Implement both A and B fully

Independent programs

Example: Allocation decision of a health planner

Health planner responsible for allocation of resources in a hospital

Initial allocation decisions have been made

An additional \$2.4 million to be allocated to new programmes within the facility

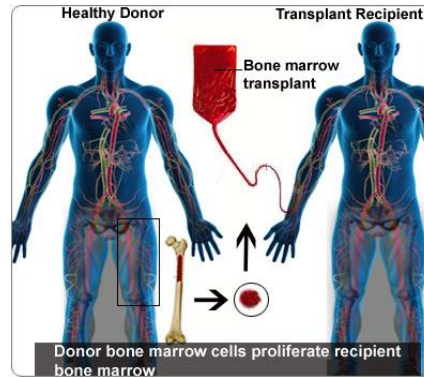
Three groups with new programmes

Programmes have already shown to be effective in increasing QALYs

Using CE or CU ratios to set priorities in resource allocation

Group 1: Hematologists (study blood and blood diseases)

Proposal: Bone marrow transplantation for acute nonlymphocytic leukemia (BMT)



Group 2: Radiologists (use radiation to diagnose or treat diseases)

Proposal: converting from high osmolar to low osmolar contrast agents (LOCA)

Group3 : Neonatologists (concerned with the care and diseases of newborn infants)

Proposal: Expanding the neonatal intensive care unit (NICU)



Resource allocation example

- Each group submits a report documenting the economic consequences of funding their program
- The neonatologists and radiologists asked to stratify their data:
 - Neonatologists: Two strata (low birth weight babies, and very low birth babies)
 - The radiologists: Two strata: patients with high risk (for developing an adverse reaction to contrast media) and patients with low risk

Resource allocation example

Program	ICUR ($\Delta C/\Delta Q$)	Incremental Costs per Patient ($\Delta C/\text{Patient}$)	Incremental QALYs per Patient ($\Delta Q/\text{Patient}$)	Total # of Patients in the Facility	Total Incremental Cost per Year	Incremental QALYs gained if funded
NICU for low birth weight babies	5100	8600	1.7	200		
LOCA for high-risk patients	22 600	22.6	0.0010	30000		
NICU for very low birth weight babies	30 900	17900	0.58	100		
BMT	59 300	57000	1.12	50		
LOCA for low- risk patients	220 000	60.55	0.0003	70000		

Note that the values are **incremental**, i.e., each program **compares** the use of the strategy listed in the table **with an alternative strategy treating the same patients!**

Resource allocation example

Program	ICUR ($\Delta C/\Delta Q$)	Incremental Costs per Patient ($\Delta C/\text{Patient}$)	Incremental QALYs per Patient ($\Delta Q/\text{Patient}$)	Total # of Patients in the Facility (per year)	Total Incremental Cost per Year	Incremental QALYs gained if funded
NICU for low birth weight babies	5100	8600	1.7	200	1 720 000 (=8600*200)	
LOCA for high-risk patients	22 600	22.6	0.0010	30000	678000	
NICU for very low birth weight babies	30 900	17900	0.58	100	1 790 000	
BMT	59 300	57000	1.12	50	2 850 000	
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Resource allocation example

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NICU for low birth weight babies	5100	8600	1.7	200	1 720 000	340 (=1.7*200)
LOCA for high-risk patients	22 600	22.6	0.0010	30000	678 000	30
NICU for very low birth weight babies	30 900	17900	0.58	100	1 790 000	58
BMT	59 300	57000	1.12	50	2 850 000	56
LOCA for low- risk patients	220 000	60.55	0.0003	70000	4 238 500	21

Resource allocation example

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Fund the programs from the top of list down until the budget is exhausted

Resource allocation example

Program	ICUR ($\Delta C/\Delta Q$)	Incremental Costs per Patient ($\Delta C/\text{Patient}$)	Incremental QALYs per Patient ($\Delta Q/\text{Patient}$)	Total # of Patients in the Facility (per year)	Total Incremental Cost per Year	Incremental QALYs gained per year if funded
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BMT	59 300	57000	1.12	50	2 850 000	56
LOCA for low- risk patients	220 000	60.55	0.0003	70000	4 238 500	21



1. Fund NICU for low birth babies first: Used budget so far = 1 720 000,
 Remaining budget = 680000
 QALY gain so far = 340

Resource allocation example

Program	ICUR ($\Delta C/\Delta Q$)	Incremental Costs per Patient ($\Delta C/\text{Patient}$)	Incremental QALYs per Patient ($\Delta Q/\text{Patient}$)	Total # of Patients in the Facility (per year)	Total Incremental Cost per Year	Incremental QALYs gained per year if funded
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LOCA for high-risk patients	22 600	22.6	0.0010	30000	678000	30
NICU for very low birth weight babies	30 900	17900	0.58	100	1 790 000	58
BMT	59 300	57000	1.12	50	2 850 000	56
LOCA for low- risk patients	220 000	60.55	0.0003	70000	4 238 500	21



2. Fund LOCA for high risk patients Used budget so far=2 398 000,
 Remaining budget= 2000
 QALY gain so far= 370

Why to use incremental values?

The values are **incremental**, i.e., each program **compares** the use of the strategy listed in the table **with an alternative strategy treating the same patients!**

Ex: LOCA: converting from **high** osmolar to **low** osmolar contrast agents (LOCA)

Average vs incremental ratios

Program	Average cost (\$)	Average QALY	Average Cost/QALY Ratio	Incremental Cost/QALY Ratio	Rank according to Average Cost/QALY	Rank According to Incremental Cost/QALY
NICU for low birth weight babies	20 700	8.1	2555 (=20 700/8.1)	5100	3	1
LOCA for high-risk patients	36.98	29.996	1.23	22 600	1	2
NICU for very low birth weight babies	19 900	1.8	11 055	30 900	4	3
BMT	193 000	3.32	58 132	59 300	5	4
LOCA for low-risk patients	97.53	29.999	3.25	220 000	2	5

If we use **average cost/QALYs** to prioritize programs then we would fund the following:

- LOCA for high risk patients (\$678 000)
- LOCA for low risk patients (\$ 4 238 000) (we are exceeding the budget and also the total QALYs gained would be 51 (compared to 340)!

Some Issues

- The ratios should be valid and reliable
- Often the studies lack methodological consistency and hence comparability
 - different measurement techniques used for utility or quality of life assessment
 - different methods used for estimating costs
 - different time horizons (the length of time into the future considered in the analysis)
- Distributional consequences of allocation decisions
- Relevance to clinicians

How do we use economic evaluation results

Note that this tool is suitable for DMs responsible for allocation decisions: a clinician's objective may be different as s/he may have a different perspective

(e.g. Maximize their patient's health regardless of the effect of this decision on other patients)

Theoretical Foundations

- No single theoretical foundation
- Contributions involve researchers from diverse backgrounds such as decision analysis and operations research
- CEA is an **engineering technique** for allocating resources
- Recently economists have started to graft CEA to theoretical roots in welfare economics

Theoretical Foundations

Some attempts to base CEA to theoretical roots in welfare economics (when societal perspective is used)

Welfare economics is concerned with the means by which we can assess the desirability- **from the societal point of view**-of alternative allocations of resources

Assumptions:

- Individuals maximize a well defined preference function (utility) (individuals are best judges of their own welfare)
- The overall welfare of society is a function of these individual preferences

$$W=f(u_1(x), u_2(x),...,u_n(x))$$

Much of the literature is concerned with developing criteria to determine whether a program improves the welfare of the affected population

To determine this, we should first measure welfare **at the individual level** and then aggregate individual well-being to measure welfare at the **societal level**.

Theoretical Foundations

Individual utility maximization

Uncertainty inherent in the risk of disease and the uncertainty related to treatment

Hence **expected utility maximization** is mostly used (will be discussed later in detail)

Expected utility theory supplies a theoretical foundation for the quantification of effectiveness **at the individual level**.

- The measure of health benefit should take individual preference under uncertainty into account (if exists) (gain in expected utility for that individual)

Quality adjusted life years are one such measure

Theoretical Foundations

QALYs can be used to represent utility only if individuals are

- willing to trade off years of life in a given health state for fewer years in an ideal health state (e.g. perfect health) at a constant rate
- indifferent among various survival curves that have the same life expectancy (risk neutral)

Even if these two do not hold in practice QALYs may still offer a close enough approximation of health-related utility

So, one can define individual health-related utility in terms of quality-adjusted life expectancy

Theoretical Foundations

Aggregating individual utilities to determine social welfare

Two different perspectives

1. Output of health care: contribution to overall well being

Allowing exchange of health care and other good and services (food, shelter, clothing) between people such that it increases the overall well being

A poor person may be willing to give up some of his/her health care (100 QALYs worth) for some cash \$X

A rich person would be willing to give \$X and gain 90 QALYs worth health care

Both are better off according to their preferences however the total health decreases (from 100 QALYs to 90 QALYs)

Theoretical Foundations

Aggregating individual utilities to determine social welfare

Two different perspectives

2. Output of health care: contribution to health itself (Extra-welfarist approach)

Health is a fundamental good

Such trades make society worse off

Maximize ***health*** subject to resource constraints

Theoretical Foundations

W: social utility function, aggregate of individual utilities

$$\text{Maximize } W=f(u_1(x), u_2(x),\dots,u_n(x))$$

According to economists, the ultimate goal of any resource allocation scheme is maximization of the social utility

The form of the aggregation function $f(.)$ might change

Utilitarianism :

$$W = \sum_i u_i$$

Other form:
$$W = \sum_i \overleftarrow{w}_i \overrightarrow{u}_i$$

Give more weight to worse off

No consensus on which form to use for the social utility (welfare) function

Difficult to select a specific weighting scheme in the second form (almost impossible)

No consensus, how will we decide whether a resource reallocation improves the social welfare?

The compensation test

Pareto criterion can be used to test whether social welfare is improved by a reallocation of resources

Actual Pareto improvement: A program (reallocation, policy) that makes at least one person better off and makes no person worse off

Rarely observed in real life: Not all winners! Some win, some lose...

The compensation test

Potential Pareto improvement (Kaldor- Hicks criterion, compensation test):

A program is welfare enhancing if:

- The gainers are willing to pay enough for their gains in order to compensate the losers (*in theory*)
- If there **were** a mechanism for such a compensation to occur, actual Pareto improvement would occur

Does not require compensation actually be paid, merely that the possibility for compensation exists

A more “efficient” outcome can leave some people worse off ! (equity issues)

Cost benefit analysis is directly based on this principle. If a program is undertaken whose benefits exceed its costs, a potential Pareto improvement will occur

Maximizing aggregate QALYs

As an alternative to defining social utility as an aggregate of individual utilities, special status might be given to health

2. Output of health care: contribution to health itself

Health is a fundamental good

Maximize health subject to resource constraints

Maximize aggregate QALYs

Utilitarian Approach: sum of QALYs (Harsanyi 1953, 1955)

Imagine a citizen in a state prior to birth uncertain of which possible health scenarios await him/her

Assumes a rational individual would opt for maximizing aggregate (average) utility

Rawlsian Approach: the “maximin rule”, maximize the well-being of the worst-off member of the society

Research on scales of measurement