

# HEALTH ECONOMICS WORKSHOP

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## Assessment of Healthcare Technologies: The Third Generation Tools

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# How to Make Right Choices in Health?

A decision will be known as good one for Public Health if the difference between its advantages and its drawbacks in terms of population's health is strictly superior to the one that would have been observed had the decision not been taken.

Net gains in public health	=	Incremental population's health gains	-	Incremental population health losses subsequent to the additional investments
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# Implicit Health Sacrifices

## An Unavoidable Dimension of the Choice in Favor of a Technology

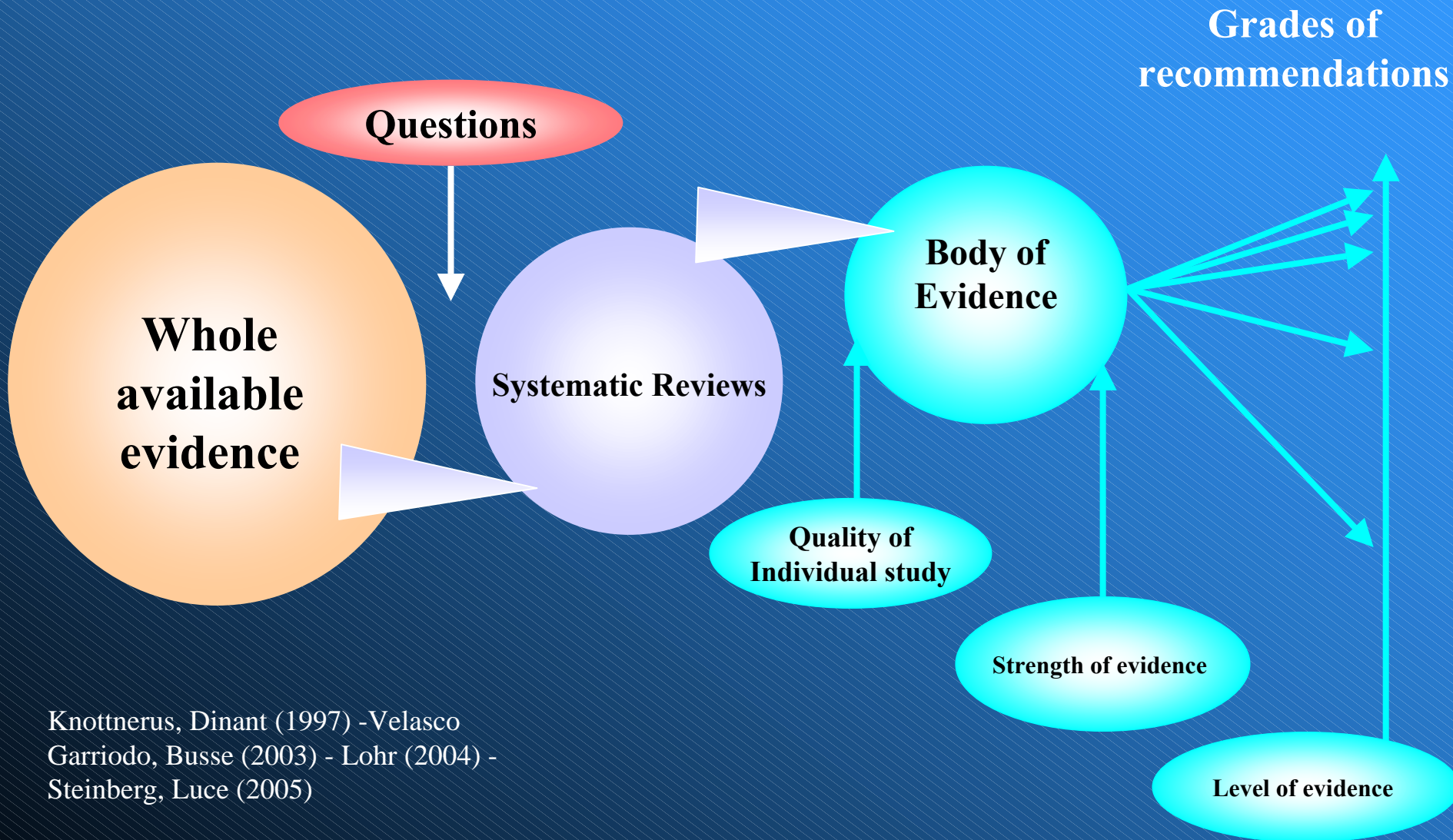
- The advantages are measured in terms of efficacy  $\Delta$  and quality of life  $\Delta$
- The drawbacks are measured in terms of risk differences and with the yardstick of the health actions that could have promoted and we have not been able to do considering what we have done ... i.e. in costs

Benefit – Risks – Costs profiles  
are at the heart of public health interest

- To make the concept operational, it is sufficient!!! to measure the realities it covers

# 1) Evidence based Medecine...

# Collecting and Weighing the Evidence



Knottnerus, Dinant (1997) -Velasco  
Garriodo, Busse (2003) - Lohr (2004) -  
Steinberg, Luce (2005)

# Search For « Proof »

- Search for proof in the form of statistically significant results is a Common tendency
- But « the absence of evidence is not the evidence of absence »
- Statistical significance does not specified the magnitude of an effect, or the comparison of benefits, harms and costs
- This approach should be avoided

# Effect Size

- Absolute Risk (AR)
- Relative Risk (RR)
- Odds Ratio (OR )
- Number Necessary to Treat (NNT)

# Hierarchy of Research Designs

- Randomised clinical trials, Non randomised trials
- Prospective et retrospective cohort
- Interrupted time series with comparison series
- Before-After study with control group
- Interrupted time series without comparison series
- Before-After study without control group
- Case Control study
- Cross sectional study
- Non comparative study: cases series, descriptive and normative study

**Greatest  
Suitability**

**Moderate  
Suitability**

**Least  
Suitability**

**Non  
Suitable**

# The Ideal Study

- **Randomization** → *Comparability of Populations*
  - Similar risk factor distribution
  - Not necessarily true in nature (e.g., new drug & new users)
- **Placebo arm** → *Comparability of Effects*
  - External conditions that might affect rate should be similar
  - Not just the drug – also the management, etc.
- **Blinding** → *Comparability of Information*
  - Avoid biased collection of information
  - Multiple levels: patient, doctor, assessor, analyst, etc.
- **But strong Selection Bias !**

# The Limits of Randomized Trials

- Impossible direct comparison between all therapeutic options
- Truncated vision of the illness's evolutionary genius
- Negation of epidemiologic and institutional local realities
- Scotomisation of decisive elements for the decision-makers  
*(adverse events, QoL, pathways and contacts, any information other than those relating to the size of effects )*

# Risk of Bias in Observational Studies

## Selection Bias

Case-mix

Regression to the Mean

Hawthorne Effect

Loss to Attrition

Measurement Error

New Technology

Secular Trends

Access

Seasonality



Unit Cost Increases

Maturation

Reimbursement

Benefit Design

Treatment Interference

# Bayesian Analysis: a New Approach To Synthesis

- Bayesian analysis focus not just on the question « what is the effect of a vs b » but « how this trial change your opinion about a vs b »
- The analyst is compelled to state the prior distribution excluding the evidence of the trial, the likelihood of different values based on the trial and to combine both sources to produce an overall synthesis
- Bayesian approach is thus an explicit quantitative use of external evidence in the interpretation of a study. It allows inference from observational data, experts views and values jugements

## **2) Why Consider Costs ?**

# The Economic Question

Where should we put our money  
to lighten the burden of illness?

Conventional treatment or innovative treatment?

# The Answer

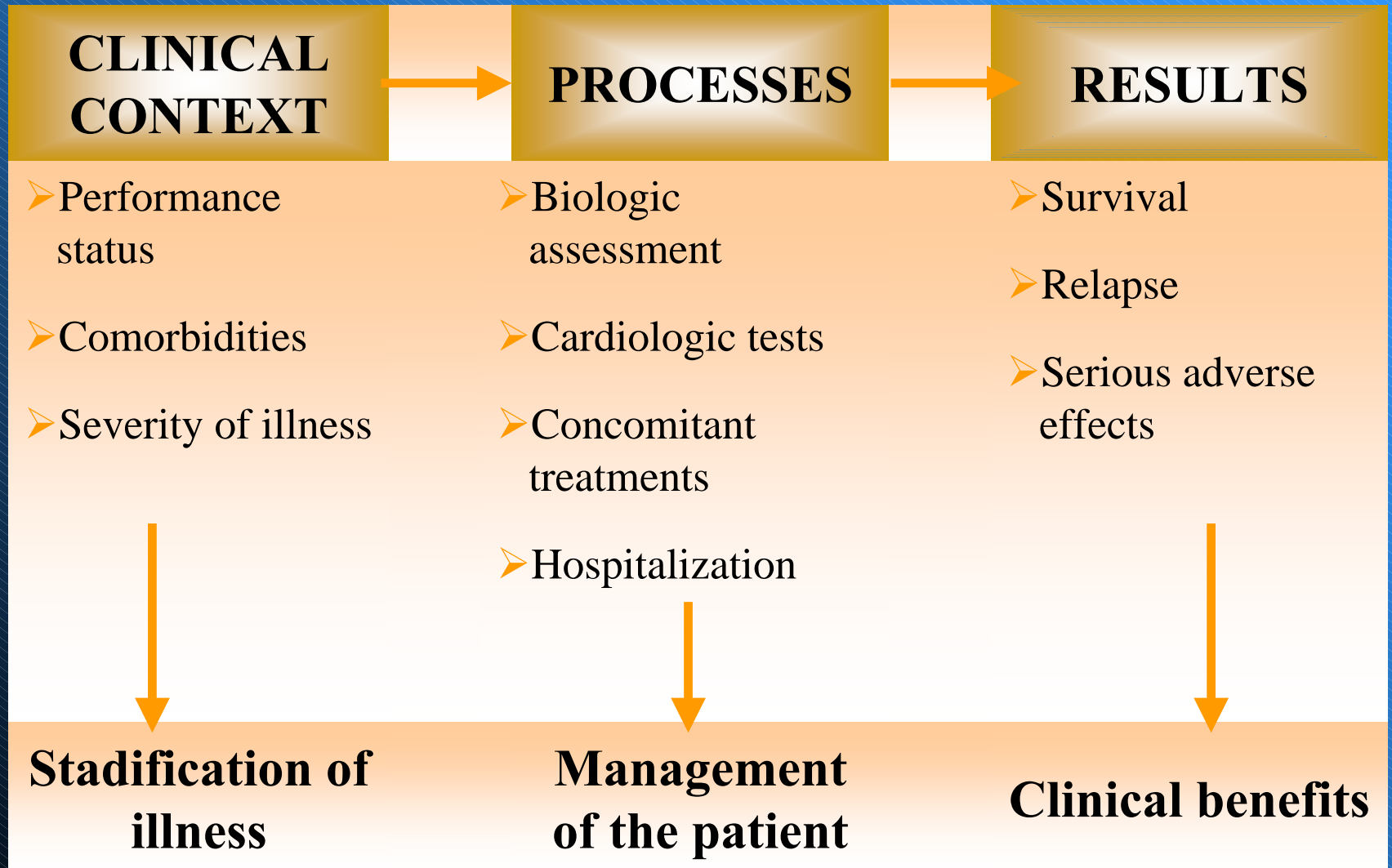
Choose the treatment which has the highest rate of return on the therapeutic, humanistic and financial aspects of the patient's life, per invested monetary unit.

# **Economic Analysis is a Subsidiary Downstream Discipline with respect to Medical Management**

Economic assessment is to science what dental care is to medicine!

- It takes the footprints of clinical path
- It makes a mould of it
- It casts the mould with Euros

# Clinical Parameters are Individual and Uncertain Data



# Tariffs are Deterministic Variables

They are available off the shelves of the public libraries and not included in any case report form

# Can we Shift the Boundaries?

## MEDICAL EXPERTISE

- « *SMR* » (absolute medical value with respect to placebo)

### **Target population and public health stakes :**

- ✓ Severity of illness
- ✓ Efficacy
- ✓ Security
- ✓ Position in the strategies
- ✓ Public health impact (QoL, impact on health organization, on practices)

- « *ASMR* » (added medical value with respect to comparators)

### **Effects size associated with innovation:**

- ✓ 5 levels

## DECISION-MAKERS

- **Drug budget impact model: CEPS**

- ✓ ASMR
- ✓ Target population
- ✓ European prices,
- ✓ Market shares,
- ✓ Price-volume agreements

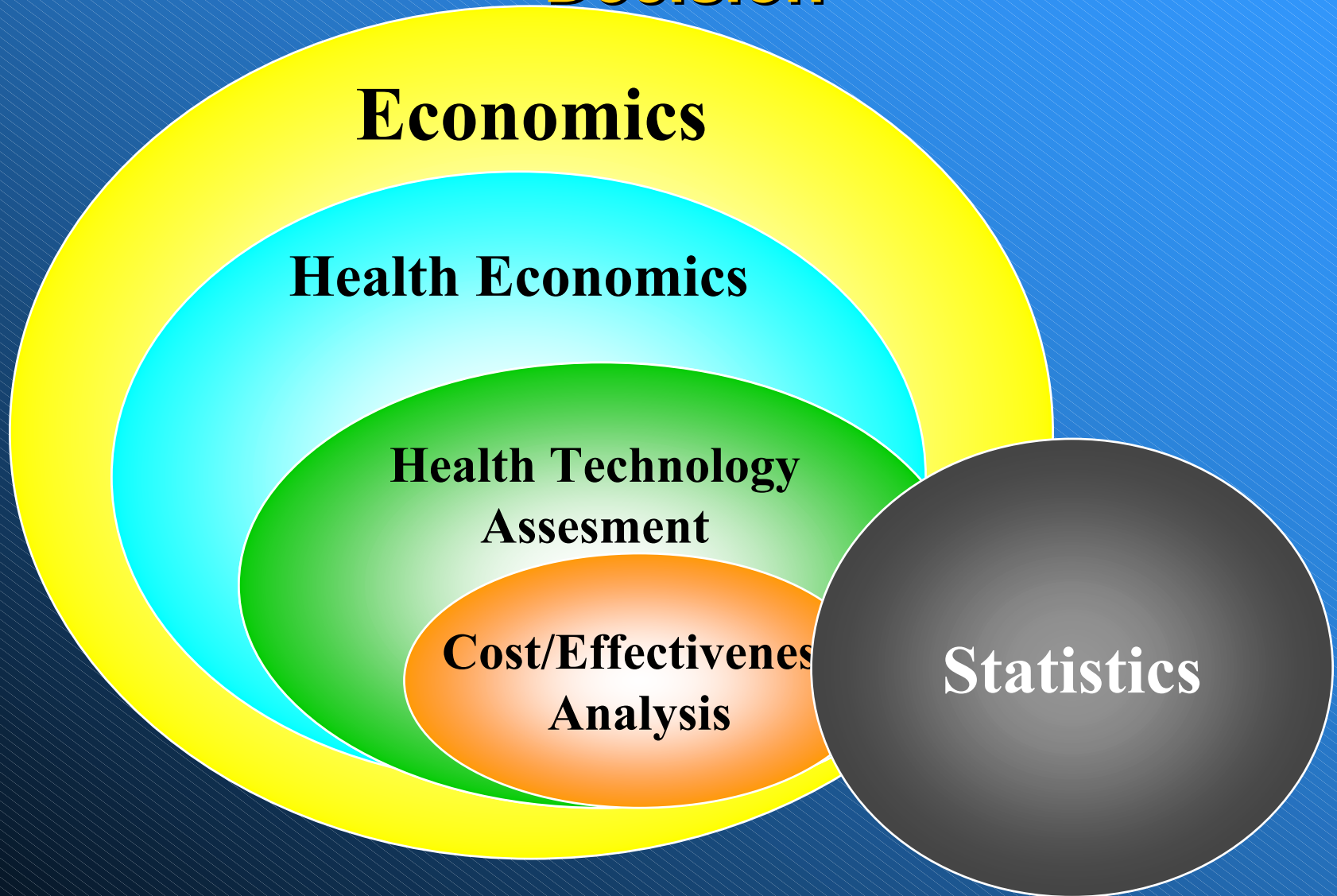
- **Regulatory rigidity; range of competence defined by decree:**

- ✓ Price negotiated with CEPS
- ✓ Coverage decided by MOH
- ✓ Rates of reimbursement fixed by UNCAM-UNOCAM

- **Influential groups reactions**

- ✓ Ministerial cabinets
- ✓ Congress
- ✓ Lobbies - Médias

# HTA: The Bridge Between Science and Decision



# A Systemic Approach to Gouvernance

## MEDICAL EXPERTISE

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## HTA EXPERTISE

### **Systemic approach**

Optimal allocation of resources between disciplines and treatments



What is the ROI in terms of clinical, humanistic and financial benefits?



Projects ranked according to their ROI (social utility)



Acceptability of the recommendations by the stakeholders

## DECISION-MAKERS

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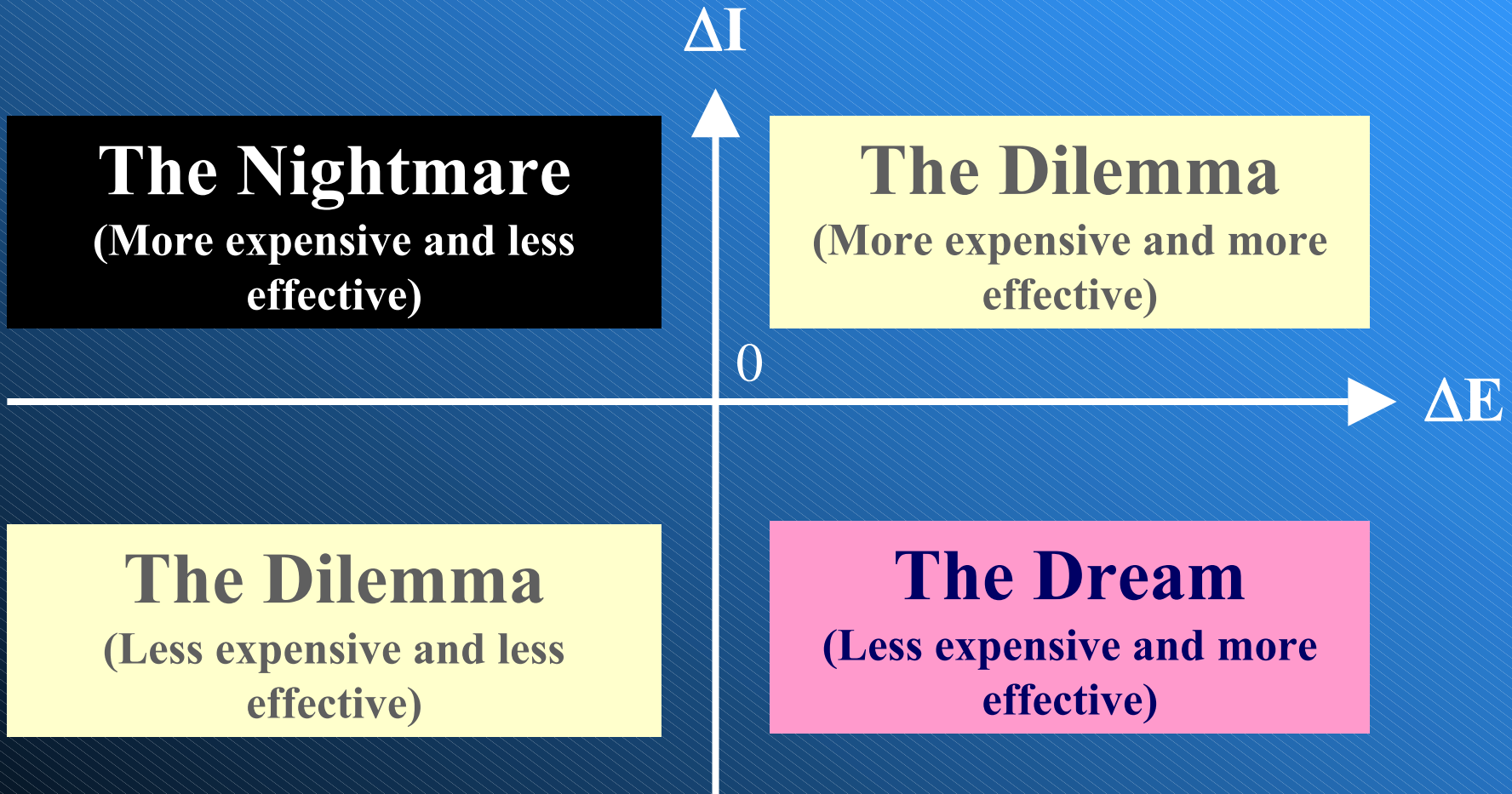
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**3) What Types of Economic Evaluation do we Use in HTA ?**

# Types of analysis

- Cost-of-Illness Analysis (COI)
- Comparative Cost analysis (CCA)
- Cost Minimization Analysis (CMA)
- Cost Benefit Analysis (CBA)
- Cost Effectiveness Analysis (CEA)
- Cost Utility Analysis (CUA)
- Budget Impact Analysis (BIA)

# Ranking Treatments According to Their Incremental Cost-Effectiveness Ratio



# How to Decide If

# The Outcomes are Worth the Effort ?

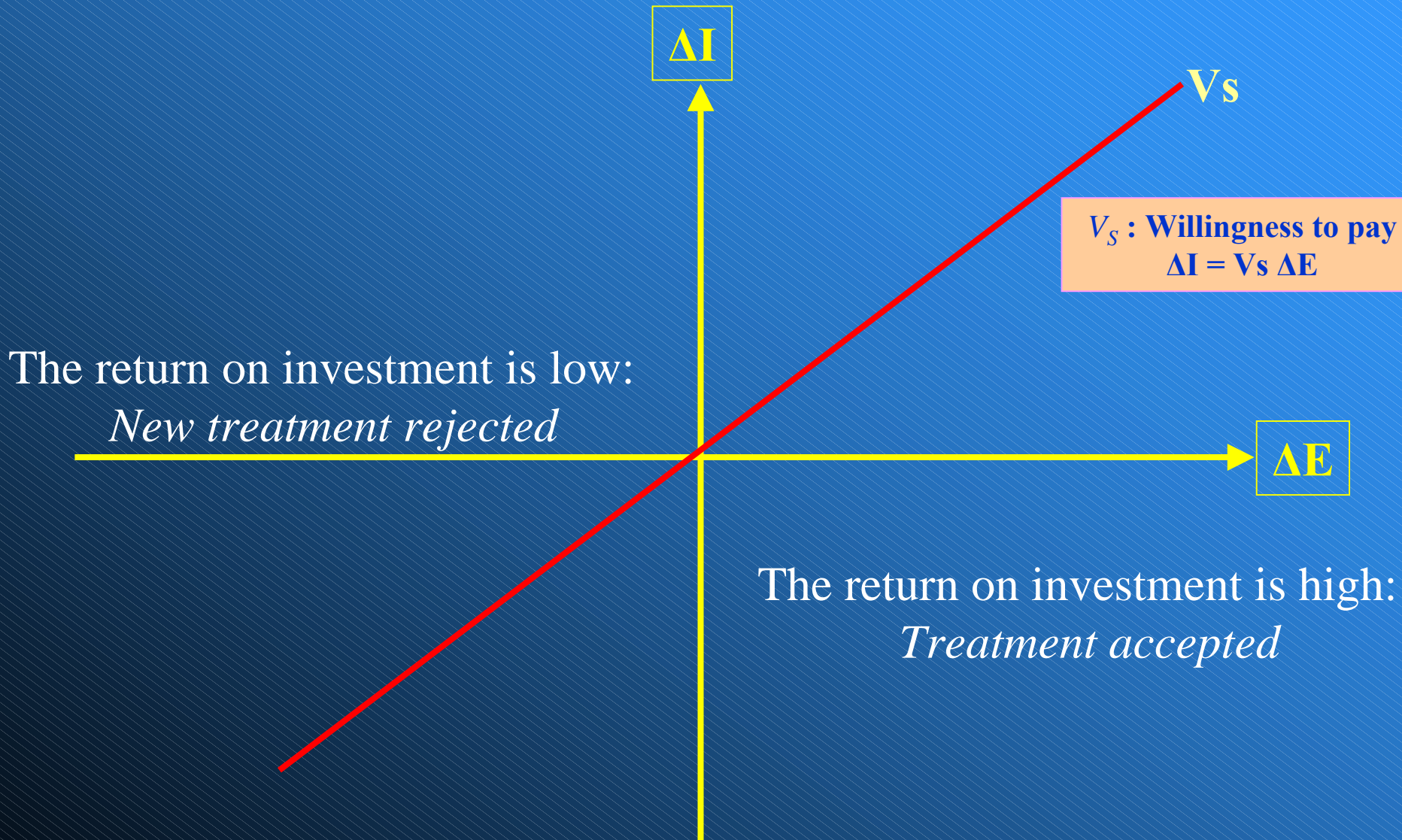
*Two possible reference criteria:*

- **MARGINAL WILLINGNESS TO PAY:** the maximum amount which the community is willing to pay to gain one unit of effectiveness
- **PRECEDENTS:** the cost-effectiveness ratios of new or established drugs which have been accepted for reimbursement or re-evaluated in the recent past

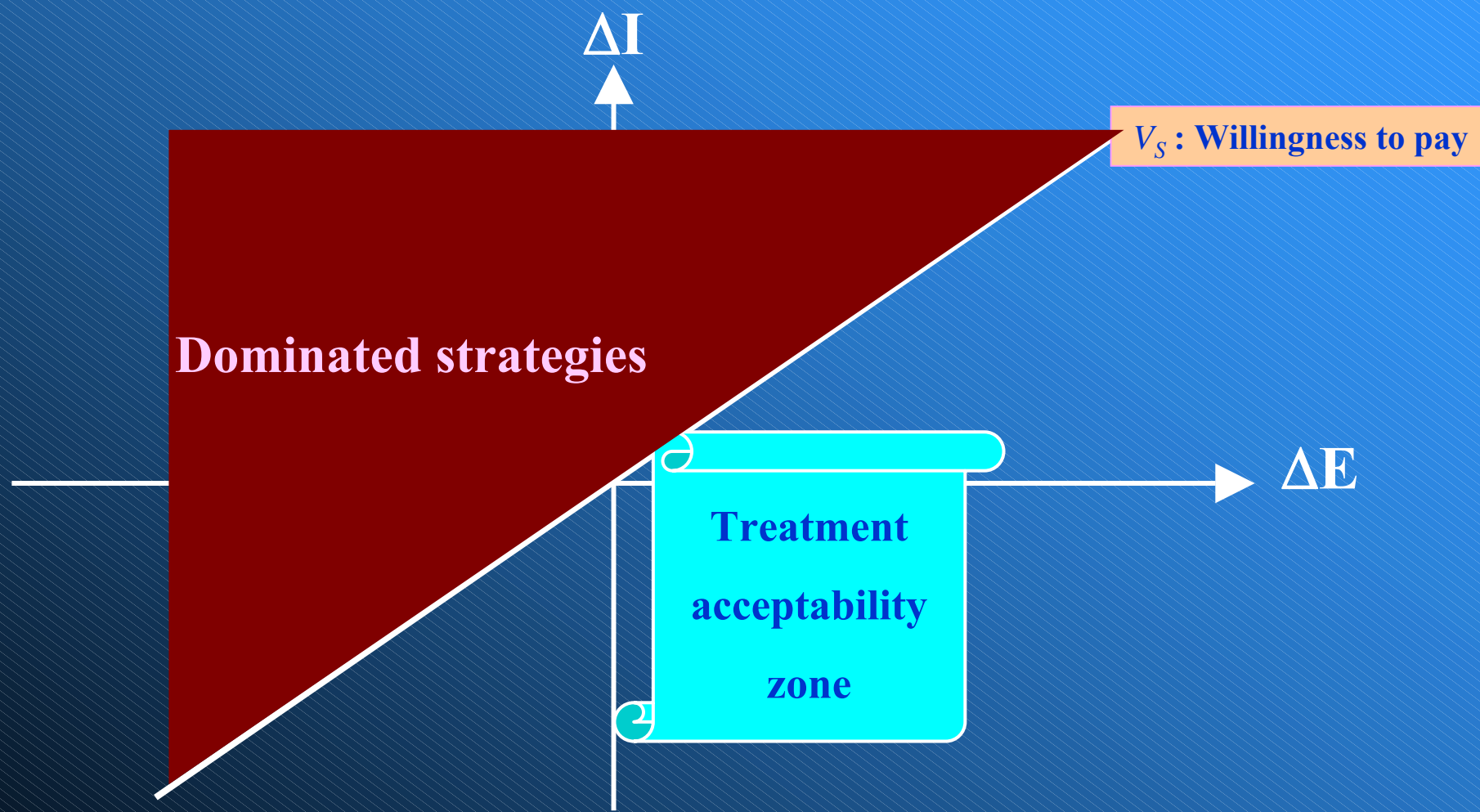
# Making Decisions Using ICER

- If the ICER doesn't fall into the quadrant of dominated or dominating strategy, then decision makings based on CE-ratio become a bit tricky.
- Rule 1: value judgement specified by an organization
  - \$20,000 per QALY used in Ontario guidelines
- Problems?

# Limits of Solidarity



# How Much are the Fit Willing to Pay?

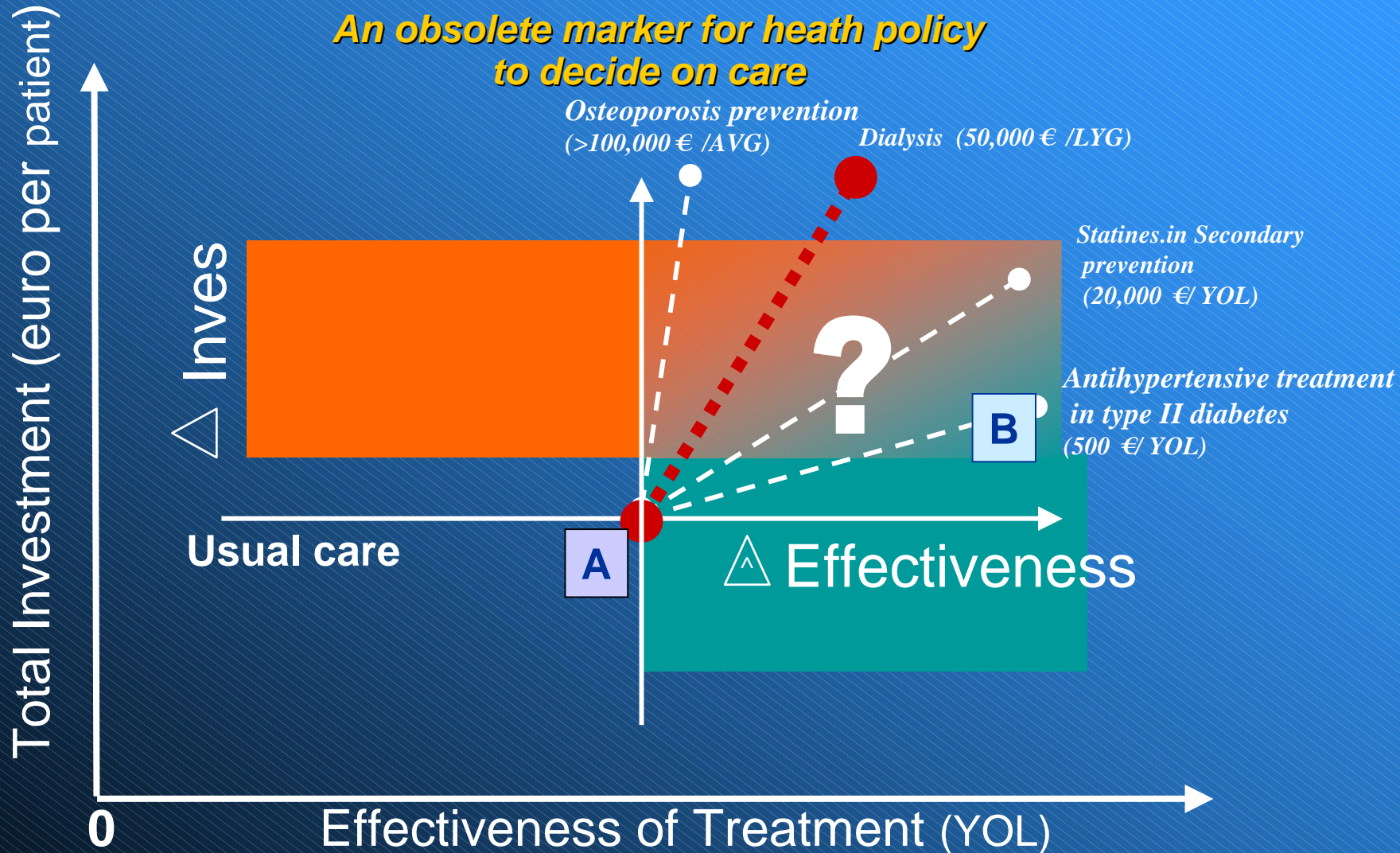


# Making Decisions Using ICER (cont.)

- Rule 2: comparison with the commonly used medical procedures.
- Rationale: Society should be willing to pay as much for new procedures/technologies as it does for procedures that are currently in common use.
  - League tables
- Problems?

# Threshold : 50,000 € per Year of Life Saved

*An obsolete marker for health policy  
to decide on care*

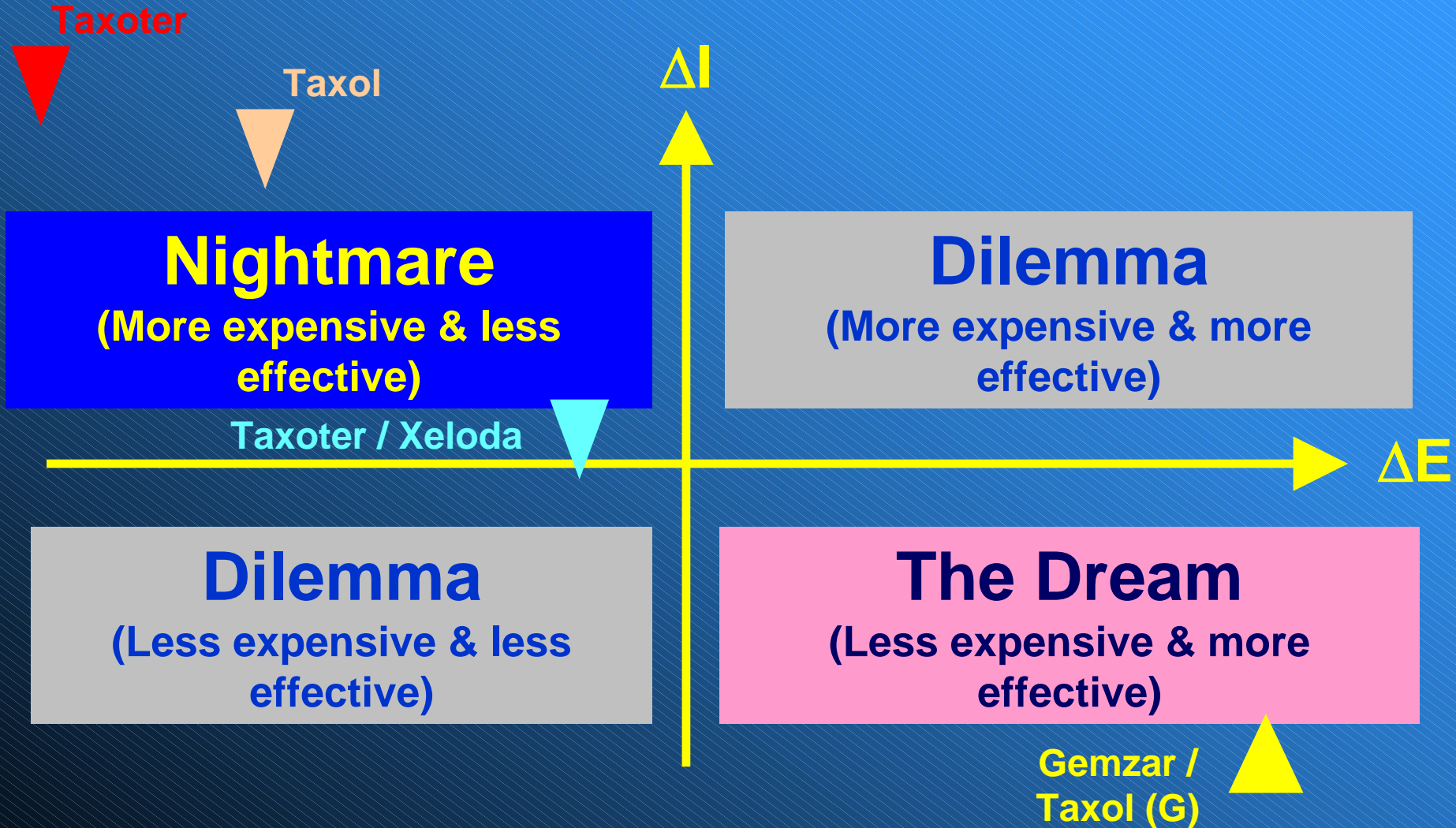


YOL = year of life saved ; QALY = Quality adjusted life Years

# League Table Example

<b>Treatment</b>	<b>\$ QALY</b>
Coronary artery bypass surgery for left main coronary artery	\$ 4,200
Treatment of severe hypertension in males age 40	\$ 9,400
Treatment of mild hypertension in males age 40	\$ 19,100
Estrogen therapy for postmenopausal symptoms	\$ 27,000
Hospital dialysis	\$ 54,000

# An Example in Metastatic Breast Cancer



# A Need: To Take Hold of the Uncertainty Inherent to the Rules of the Game

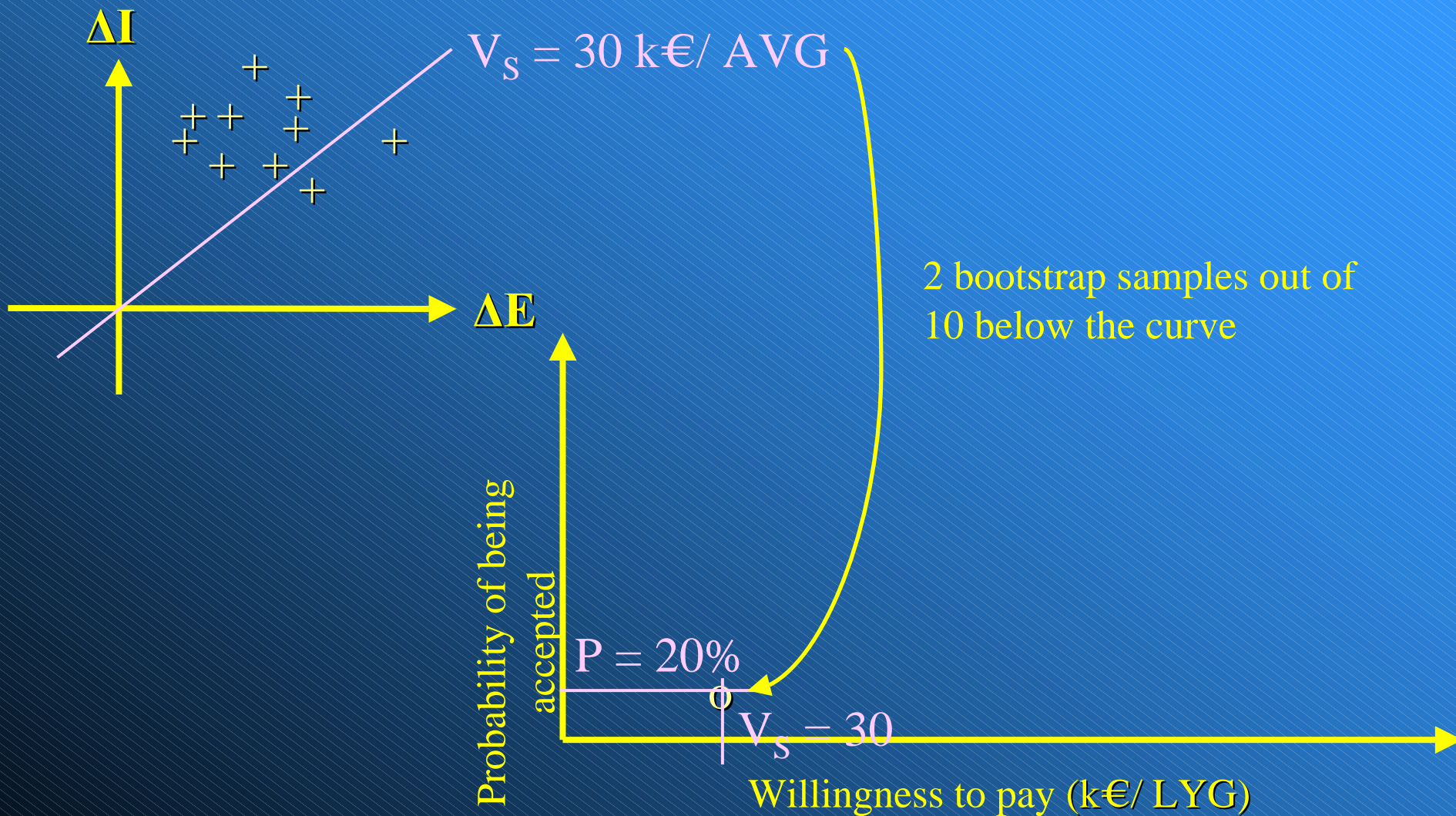
- The value ( $V_s$ ) given by the Society to an additional unit of effect is a **socio-political value** which the evaluator cannot judge.
- The results must be analysed in light of the different **willingness to pay** from the purchaser by constructing an acceptability curve for the treatment by the statutory authorities.
- This curve shows the probability that this treatment will be considered to be **efficient** by the authorities for all possible values of  $V_s$ .
- **Estimation procedure:** generation of  $\Delta E$ ,  $\Delta I$  couples bootstrap – by the proportion of points beneath the line for all values of  $V_s$ .

# Bootstrap World

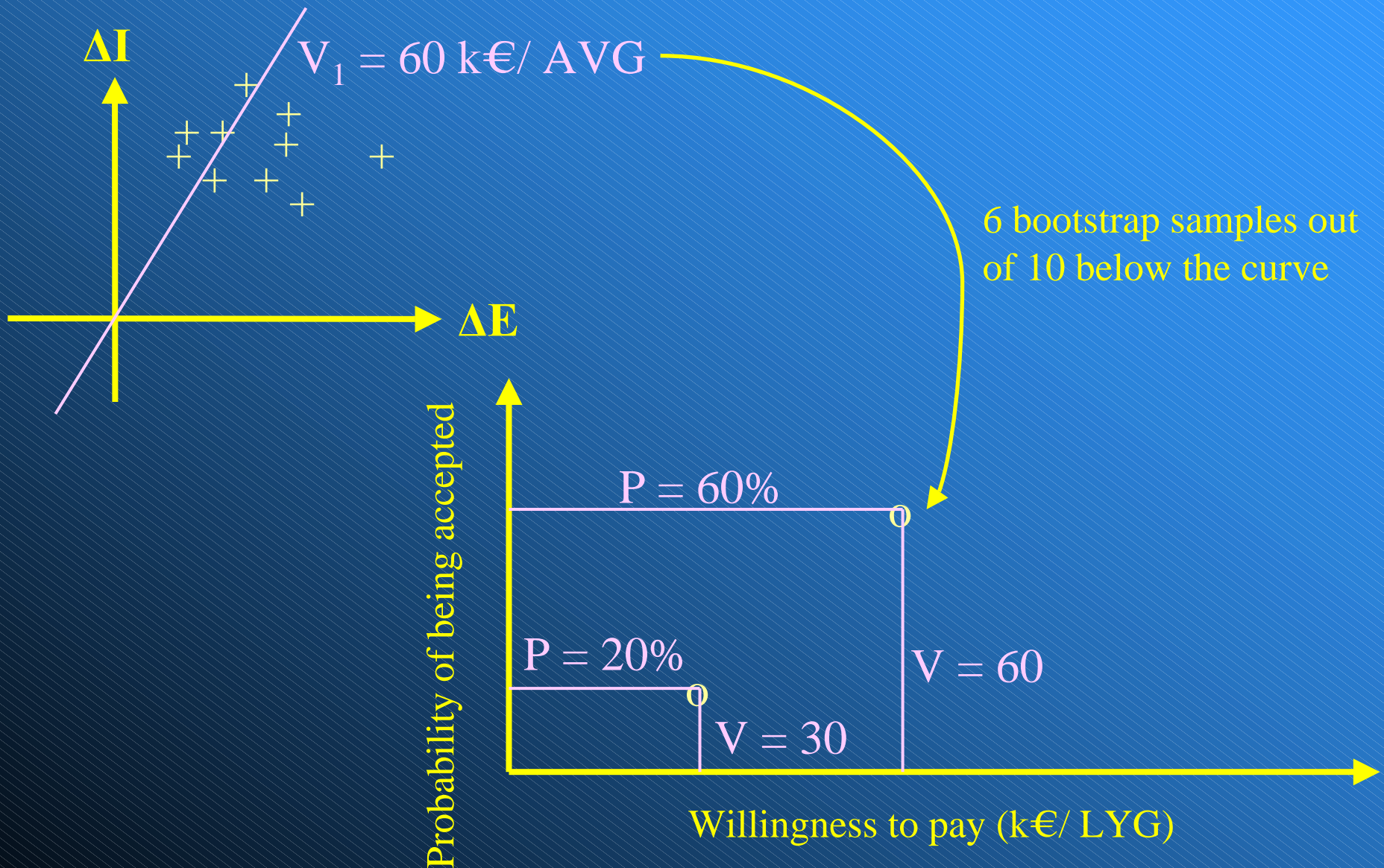
Four stage process:

1. Bootstrap  $n_c$  cost/effect pairs from the control group: calculate means
2. Bootstrapp  $n_T$  cost/effect pairs from the treatment group: calculate means
3. Calculate the bootstrapped ICER from these bootstrapped means
4. Repeat many times to create the bootstrap estimate of the ICER sampling distribution

# Acceptability for Reimbursement by the Legal Authorities, *depending on the financial effort are willing to employ*

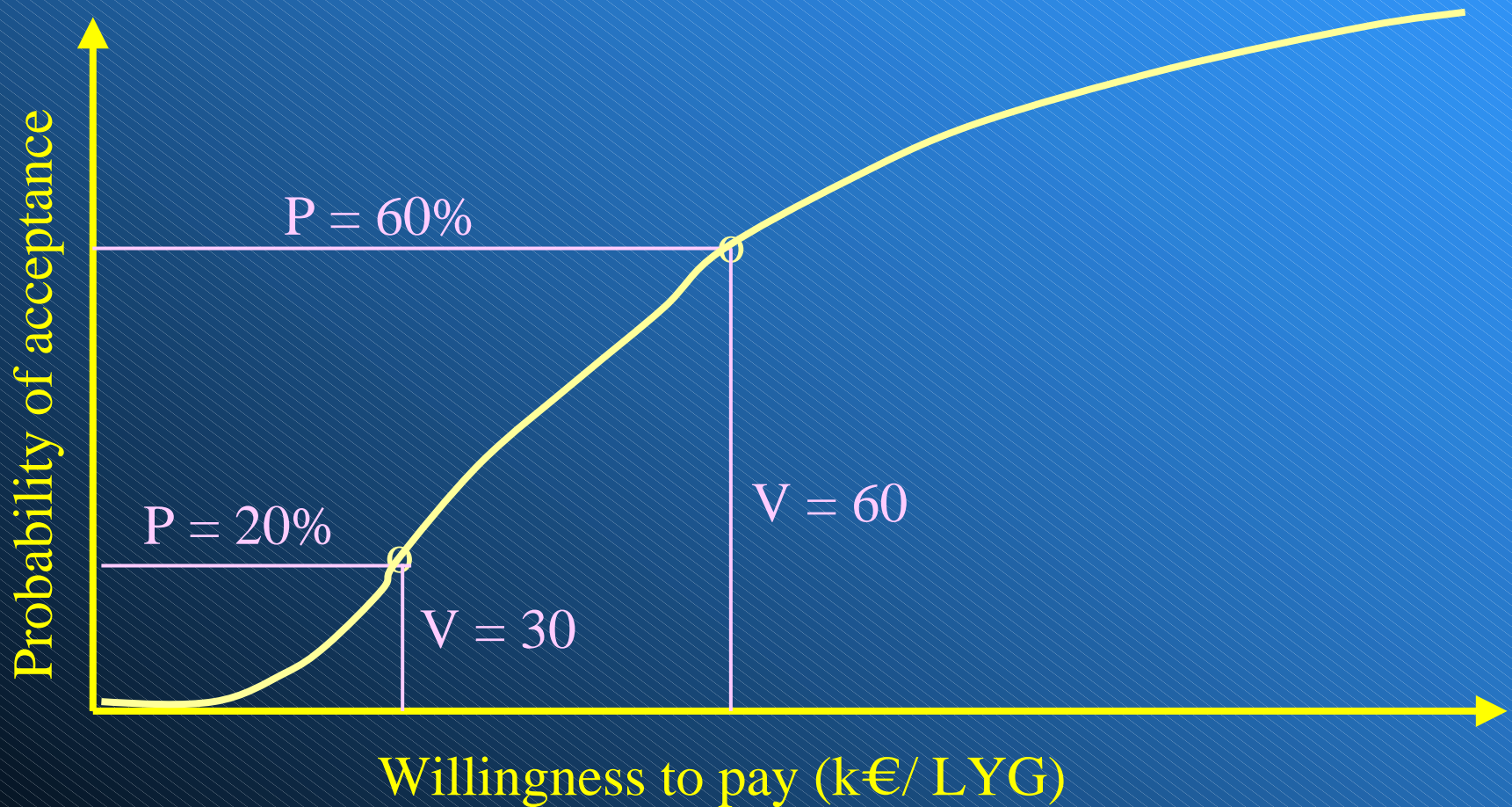


# Acceptability for Reimbursement by the Legal Authorities, depending on the financial effort are willing to employ



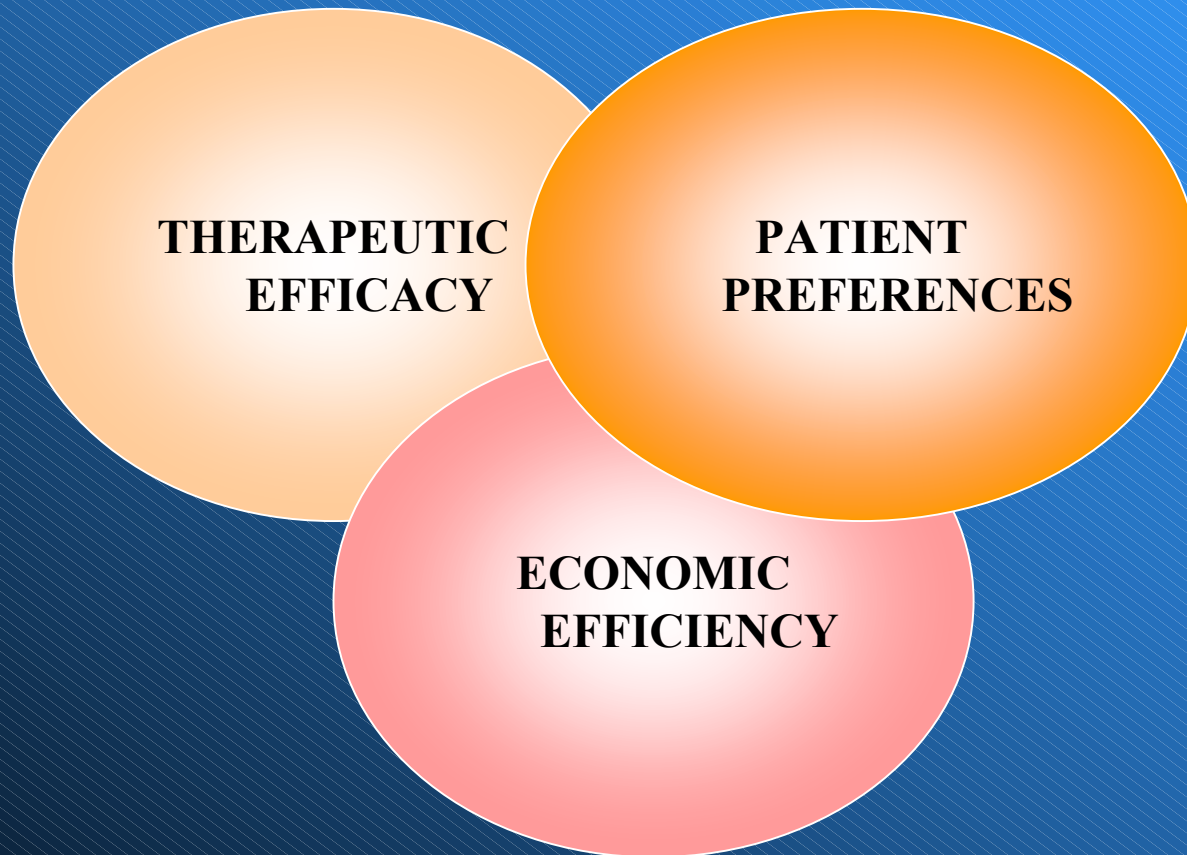
# Reimbursement Acceptability Curve for the Statutory Authorities:

## A coherent tool for the public bodies



# **4) How to inform the political decision making process?**

# **An Imperative : Collect All Information Which Contribute to the Decision Making**



# Generalised Review of Probing Data



# Meta Decision Analysis: A Tool to be Used in First Line

- To **structure** the information in a single analytical framework
- To **integrate** simultaneously benefits, risks and costs
- To **estimate** quantitatively the frequency of evolutionary events and adverse effects
- To **identify** the pathways of the patient's management and to link the costs

# **... To Collect the Evidence and Estimate the Expected Efficacy and the Actual Effectiveness**

- **To synthesise heterogeneous clinical endpoints with a composite morbid-mortality index**
- **To reintroduce patients preferences or citizen wills in the decisional process at an individual or collective level**
- **To extrapolate the results to different populations or settings**
- **To isolate the key variables and to specify the uncertainty surrounding them**
- **To present the results to decision makers as probabilities for the intervention to be cost effective given a maximum willingness to pay per unit of effect**

# Conclusion

*The implementation of databases fed by professionals based on individual data, deeply upsets the assessment methods.*

- New endpoints are introduced
  - QoL assessment
  - Estimates of the additional investments required to obtain the expected or actual clinical benefits
- A new ethic of our duties arises:

*« prodigate the best » per euro invested*