

**Evidence of AT outcomes:  
what can we learn from pharmacoeconomics**

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# Economics is...

“... study of how societies use scarce resources to produce valuable commodities and distribute them among different people”

*Paul A Samuelson, Nobel Laureate 1970*

# Health Economics is...

“Health care differs from other goods and services ...  
The output ... is less well defined... unpredictable and  
imperfectly understood ... third-party payment and  
government intervention are pervasive. Nevertheless,  
... economic analysis is essential in appraising public  
policy”

*Patricia M Danzon, 1991*

# Problem

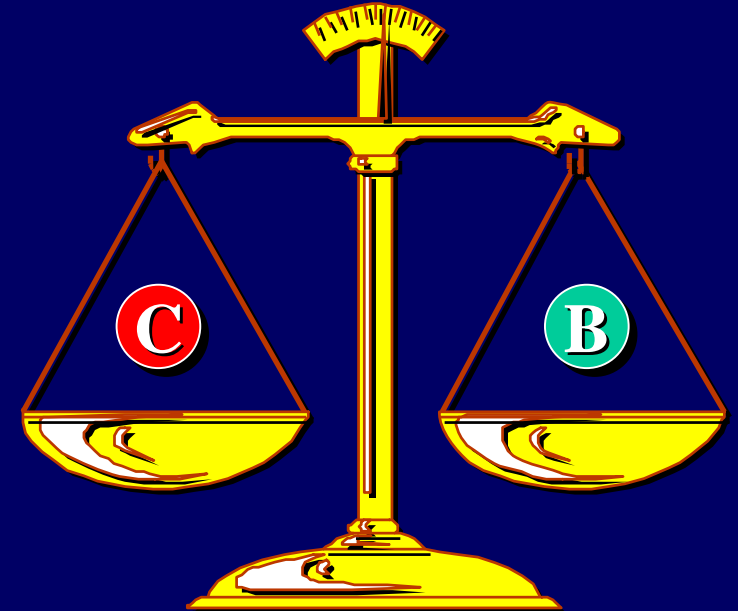
- **There are more beneficial interventions than those that we can actually finance**
- **Health care resources are limited**
- **Resources allocated (devoted) to an interventions can not be allocated to another one**
- **Somebody has to decide which interventions will be financed and the priorities**

# **Rational decision-making**

- **Requires information on benefits (quality, safety, effectiveness)**
- **Requires information on costs (potential benefits from other interventions forgone)**
- **Requires comparisons (bench-marking)**

# Health economics is...

- An aid to decision makers, not the choice itself
- Complementary, not alternative to quality, safety and effectiveness
- Not cost containment, but applications of principles and concepts of economics to health
  - maximising
  - utility (well-being)
  - resources
  - limited
  - applicable to alternative uses (interventions)



# Which Costs and Effects?

## COSTS

- **Direct, health-related** (drugs, diagnostics, hospitalization)
- **Indirect** (reduced working ability)
- **Intangible** (impaired quality of life)

## EFFECTS

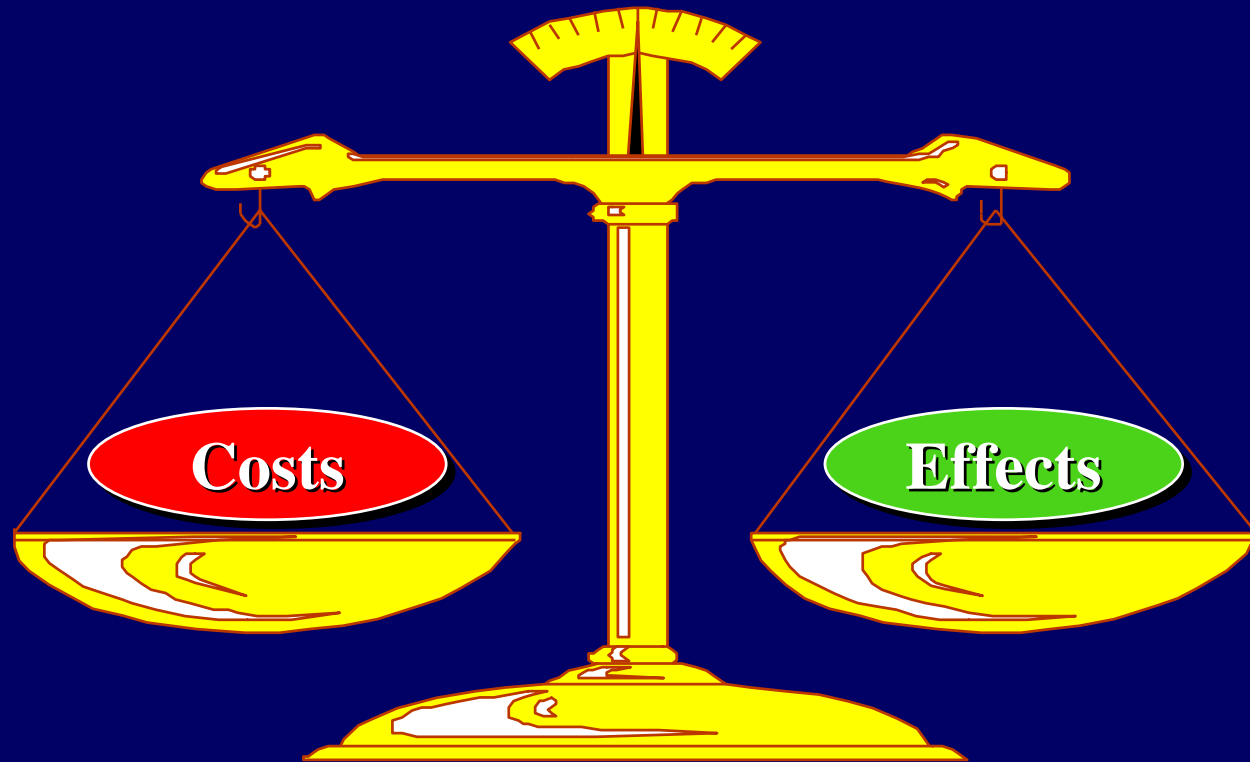
- **Direct, health-related** (biochemical parameters, morbidity, mortality)
- **Indirect** (improved working ability)
- **Intangible** (improved quality of life)

# Health economic evaluation

It is a research method that estimates the **costs** and the **consequences** of medical technologies to **compare alternative** approaches



# Health economic evaluation



# Evidence of AT outcomes: what can we learn from pharmacoeconomics

Public health



orphan treatments

individualized medicine

# What can pharmacoeconomics learn from AT-economics?

Public health



orphan treatments

individualized medicine

# What can pharmacoeconomics learn from AT-economics?

Public health

“common” treatments

orphan treatments

AT-Ec

individualized medicine

# What can pharmacoeconomics learn from AT-economics?

Public health

“common” treatments



**orphan treatments**

individualized medicine

## Cost of care and quality of life for patients with hemophilia complicated by inhibitors: the COCIS Study Group

Alessandro Gringeri, Lorenzo G. Mantovani, Luciana Scalone, and Pier Mannuccio Mannucci, for the COCIS Study Group

Inhibitors in patients with hemophilia are a rare complication of a rare disease causing pain and disability in patients and impairment to the quality of their lives. Recent advances in treatment have brought improvements, but they have done so by absorbing larger amounts of financial resources. This study involved 52 Italian patients with hemophilia with high-responding inhibitors who were longitudinally observed for 18 months to evaluate concomitantly cost of care and quality of life. Overall, 0.6 bleeding epi-

sodes per patient per month were recorded. This frequency of events was lower than that reported in other cohorts of patients with hemophilia who were not taking inhibitors. The average monthly cost of care was, in euros, €18 000 (US \$18 000) per patient, mainly because of treatment products. Recombinant activated factor VII, mostly used for orthopedic surgery, represented 50% of the expenses. Quality of life, measured through validated questionnaires, was similar to that of patients with severe hemophilia

without inhibitors. In particular, physical quality of life was similar to that in patients with diabetes and on dialysis, whereas mental quality of life was comparable to that in the general population. This study shows that hemophilia complicated by inhibitors, a prototype of rare disease, requires high amounts of resources for management that provides a satisfactory quality of life. (Blood. 2003; 102:2358-2363)

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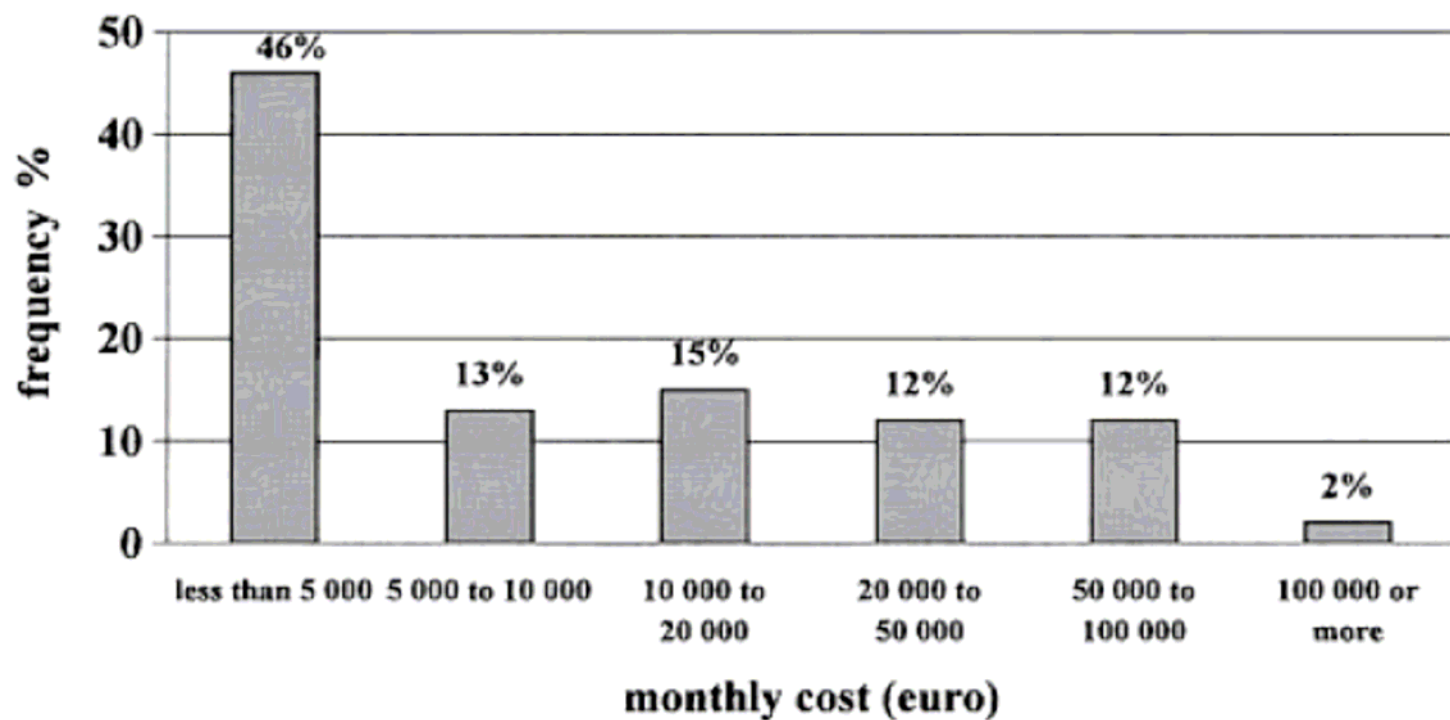
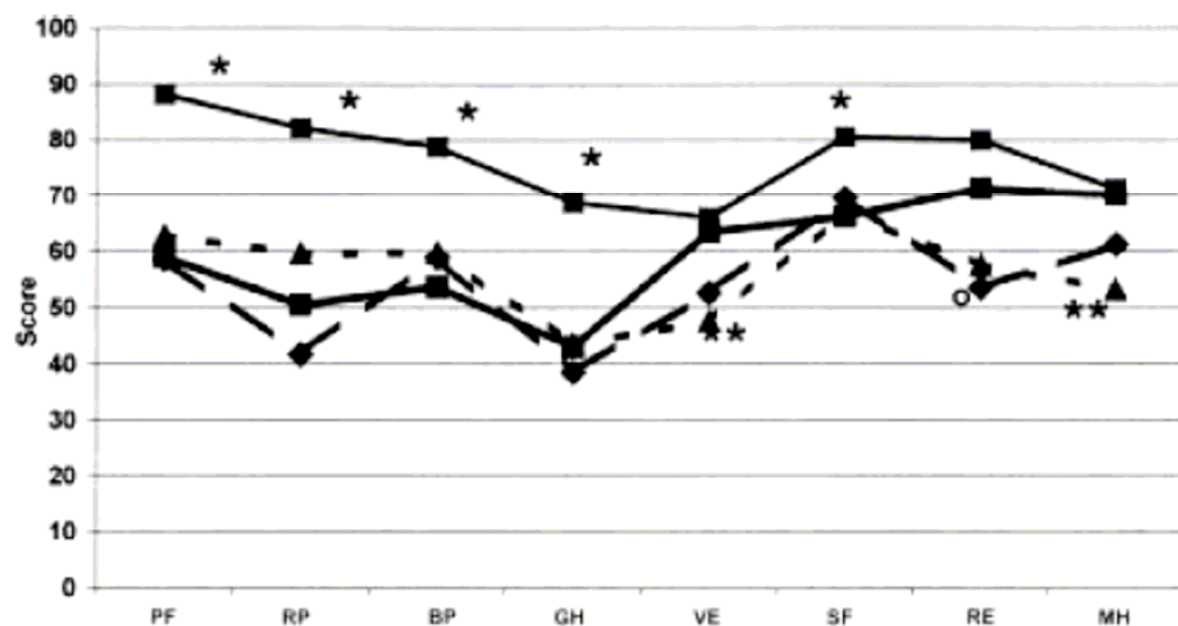


Figure 2. Frequency distribution of patients according to monthly cost of clotting factors used.



**Figure 4. Comparison of SF-36 profile.** Measured at the baseline for each dimension with other profiles in healthy men from the general Italian population (■, thin solid line; n = 999), patients with diabetes (▲, dotted line; n = 98), and patients on dialysis (◆, dashed line; n = 87).<sup>38</sup> ■, thick line indicates COCIS (n = 50). \*Significant difference ( $P < .05$ ), as computed by Student  $t$  test, between our hemophilia patients with inhibitors and the Italian male population. \*\*Italian patients with diabetes and on dialysis. ○ indicates Italian patients with diabetes; PF, physical functioning; BP, bodily pain; RP, role-physical; GH, general health; VT, energy/vitality; RE, role-emotional; SF, social functioning; and MH, mental health.



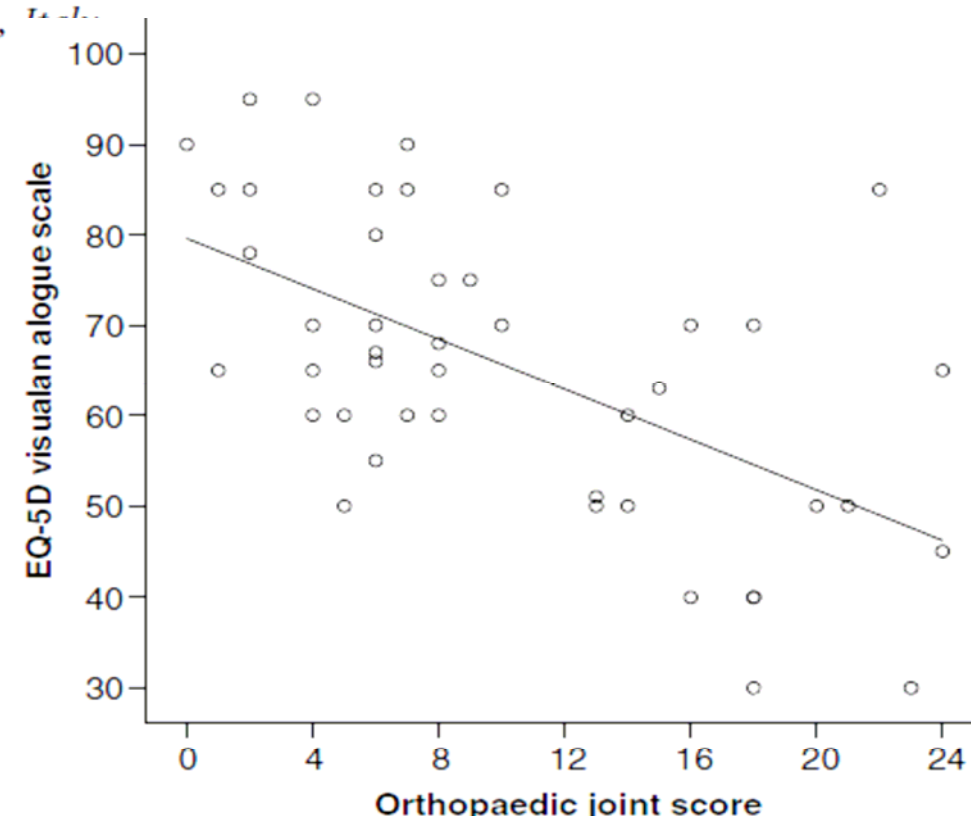
In conclusion, hemophilia with inhibitors represents an example of a rare disease that requires high amounts of resources, but it also demonstrates that effective care provides a satisfactory quality of life. The costs, which look exorbitant in absolute terms, are small when ascribed to individual citizens. Moreover, it is likely that therapeutic strategies aimed at preventing severe arthropathy, and consequently the need for surgery, are likely to dramatically affect health care costs, disability, and a patient's perception of health.

# Quality of life is associated to the orthopaedic status in haemophilic patients with inhibitors

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# Common Issues with Orphan Drugs

- Alternative treatments:
  - Often non-existing
  - Often heterogeneous
- Effectiveness and costs
  - Preventive and curative vs assistive
  - HR-QoL
  - Small samples
  - Difficult data collection/pooling
  - Generalizability
  - Social impact
  - Effect of environment

**Quality of life** refers to emotional, social and physical wellbeing of an individual, and her/his ability to *function* in the ordinary tasks of living

**Health-related quality of life analyses** measure the impact of treatments and disease **Quality of Life**

# Differences between patients', physicians' and pharmacists' preferences for treatment products in haemophilia: a discrete choice experiment

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Table 7. Effects of employment status on marginal effects of the distribution mode of clotting factors.

Employment status	Home		Community pharmacy	
	Marginal effects	95% CI	Marginal effects	95% CI
Employed	0.256	0.182 to 0.331	0.444	0.315 to 0.573
Unemployed	0.172	0.009 to 0.335	0.159	−0.117 to 0.435
Self-employed	0.093	−0.080 to 0.265	0.015	−0.287 to 0.318

# Value of networking in rare diseases:

1) better evidence

2) stronger recommendations

*Haemophilia* (2006), 12, 363–371

DOI: 10.1111/j.1365-2516.2006.01296.x

## Current European practice in immune tolerance induction therapy in patients with haemophilia and inhibitors

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## Value of networking in rare diseases:

1) better evidence

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*Haemophilia* (2007), 13, 38–45

DOI: 10.1111/j.1365-2516.2006.01403.x

# Current use of by-passing agents in Europe in the management of acute bleeds in patients with haemophilia and inhibitors

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# Economics is...

“The decision maker has a choice between optimal decisions for an imaginary simplified world or decisions that are "good enough, that satisfice", for a world approximating the complex real one more closely.”

*Herbert A Simon, Nobel Laureate 1978*



# Conclusion

*“...please do not be discouraged. Our experience shows that these measurements are not as onerous as they may at first appear and is our belief that these measurements are often essential*

*as it is better to have an approximate measure of the right factors than a precise measure of the wrong ones.”*

*Drummond, Stoddard, Torrance, 1987*