

BoneCast

CSA Edition

How can health economics help us to optimize osteoporosis management?

Assoc. Prof. Mickaël Hiligsmann

How can health economics help us to optimise osteoporosis management?

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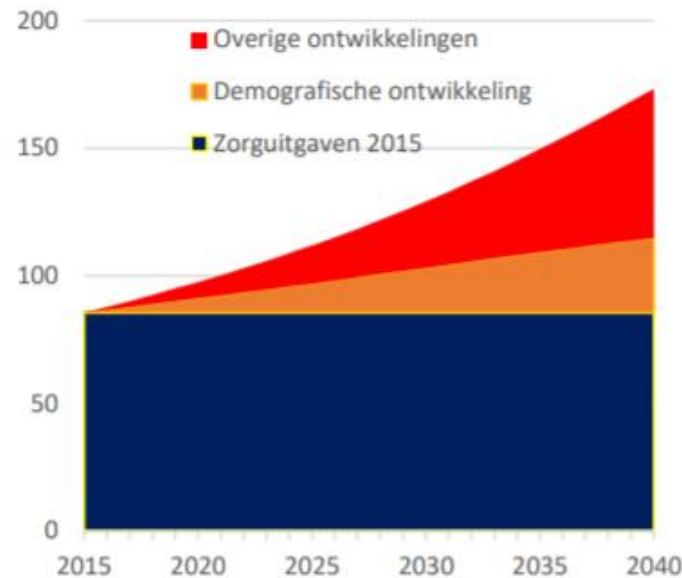
Key learning objectives

- To get a general overview of health technology assessment and economic evaluation, and of their relevance in **healthcare policymaking**
- To understand the **roles and impacts** of health economic evaluations on the management of osteoporosis
- To review recent **economic evaluations** on therapeutic options and prevention programs for osteoporosis and provide recommendations for osteoporosis-specific health economic evaluation studies

Rationale, roles and definition of economic evaluation (in osteoporosis)

Why Health Economics?

- Rising demand of health care (**unlimited needs**)
 - *Rapid development of (**expensive**) medical technological possibilities*
- Budget constraints (**scarce resources**)



Health expenditures up to 2040 (RIVM)

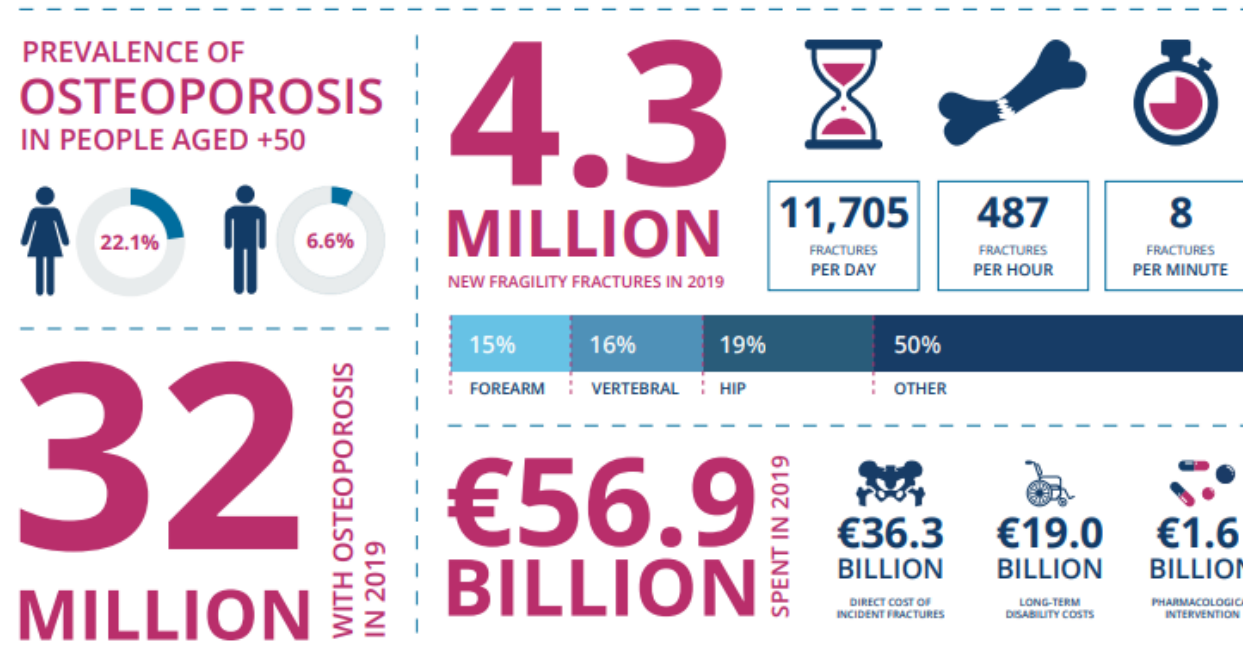
Source: Naar een toekomstbestendig zorgstelsel. Brede maatschappelijke heroverweging. Rijksoverheid, 20 april 2020

Efficiency

High importance of health economics in OP

1. Major public health problem

- Huge cost burden for osteoporosis-related healthcare



High importance of health economics in OP

2. A problem on the rise

NUMBER OF ADULTS +75
EXPECTED TO
INCREASE

BETWEEN 2019 AND 2034

+42.6%

MEN

+29.6%

WOMEN



INCREASED INCIDENCE OF
FRAGILITY FRACTURES

2019
4.28
MILLION



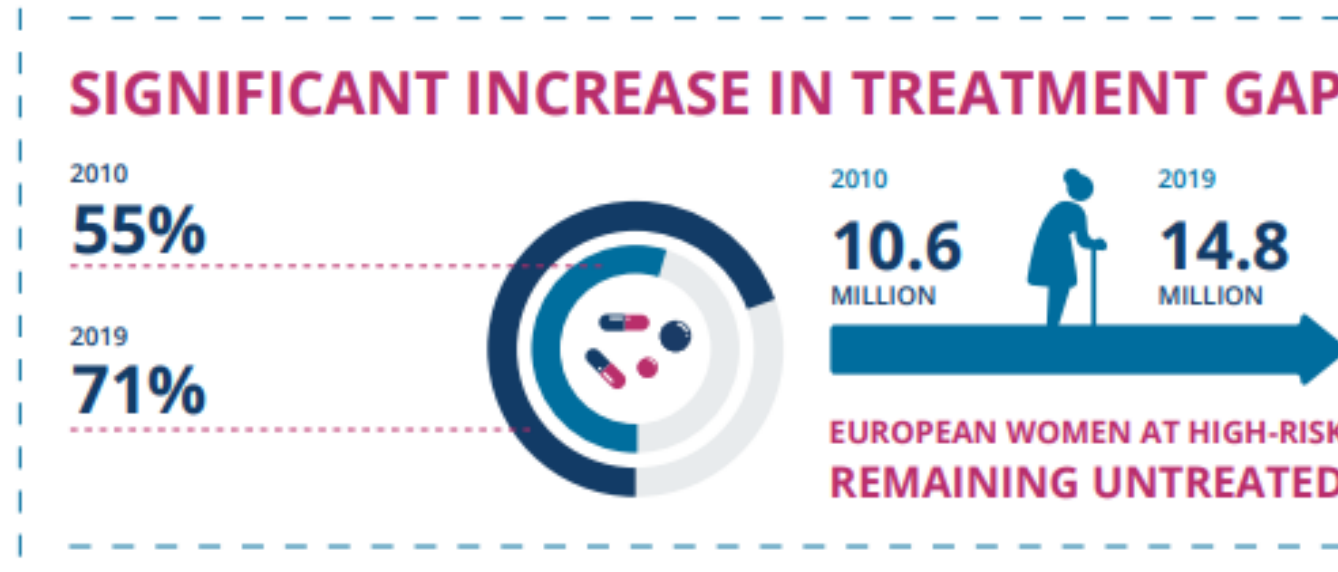
2034
5.34
MILLION

+24.8%

TOTAL FRAGILITY FRACTURES

High importance of health economics in OP

3. Treatment gap



Needs

Efficient allocation of scarce healthcare resources

Solutions for fracture prevention

Convince policy makers about the (economic) value of osteoporosis management

HEALTH
ECONOMICS

Health interventions requirements

Value

- **Clinical** (How much better does the innovation **perform compared** to Standard of Care (efficacy, safety)?)
- **Economic** (Is the added value of the innovation **worth its price**?)



Affordability

- **Budget impact** (Does the payer has the **budget** to pay the innovation?)

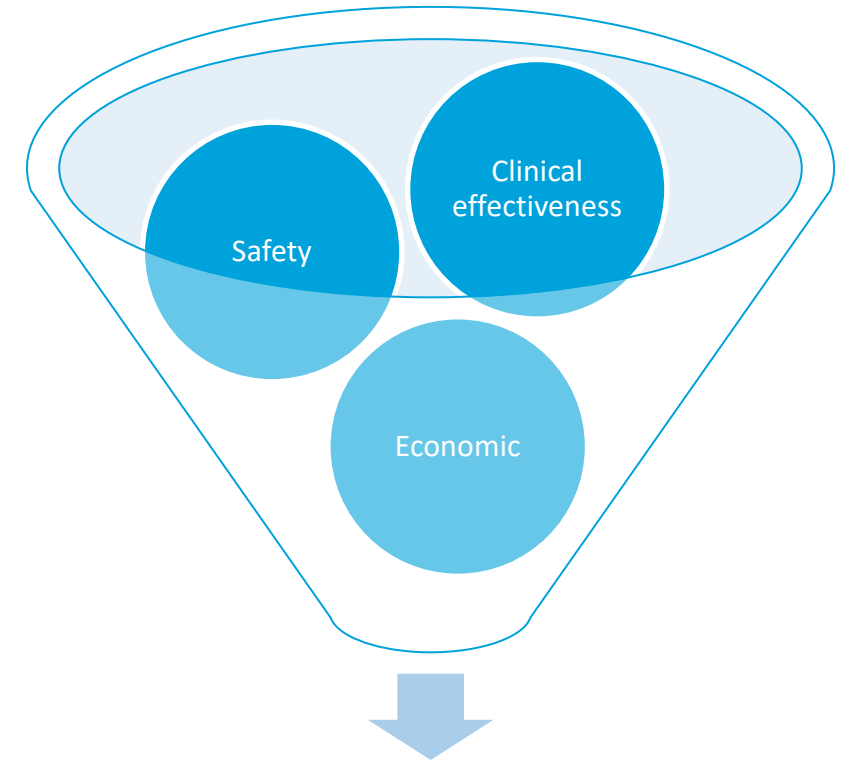


Examples of policy questions

Is it worth to invest money to tackle osteoporosis?

Are anti-osteoporosis medications cost-effective?

Are Fracture Liaison Services an efficient way of allocating scarce resources?



Health technology assessment

Health Technology Assessment (HTA)



HTA is a multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. The purpose is to inform decision-making in order to promote an equitable, efficient, and high-quality health system.

Note 3: The dimensions of value for a health technology may be assessed by examining the intended and unintended consequences of using a health technology compared to existing alternatives. These dimensions often include clinical effectiveness, safety, costs and economic implications, ethical, social, cultural and legal issues, organizational and environmental aspects, as well as wider implications for the patient, relatives, caregivers, and the population. The overall value may vary depending on the perspective taken, the stakeholders involved, and the decision context.

O'Rourke et al. Int J Technol Assess Health Care. 2020 Jun;36(3):187-190

Overview of HTA activity in Europe



Key: N=31 countries with England, Scotland and Wales counted separately; red = no current HTA procedure; blue = pharmaceuticals only; yellow = both pharmaceuticals and non-pharmaceuticals

EuNetHTA, 2017

Roles of Health Economics in decision-making



- Drug reimbursement and pricing
- Public health programs
- Value-based pricing of experimental technologies
- Funding agencies

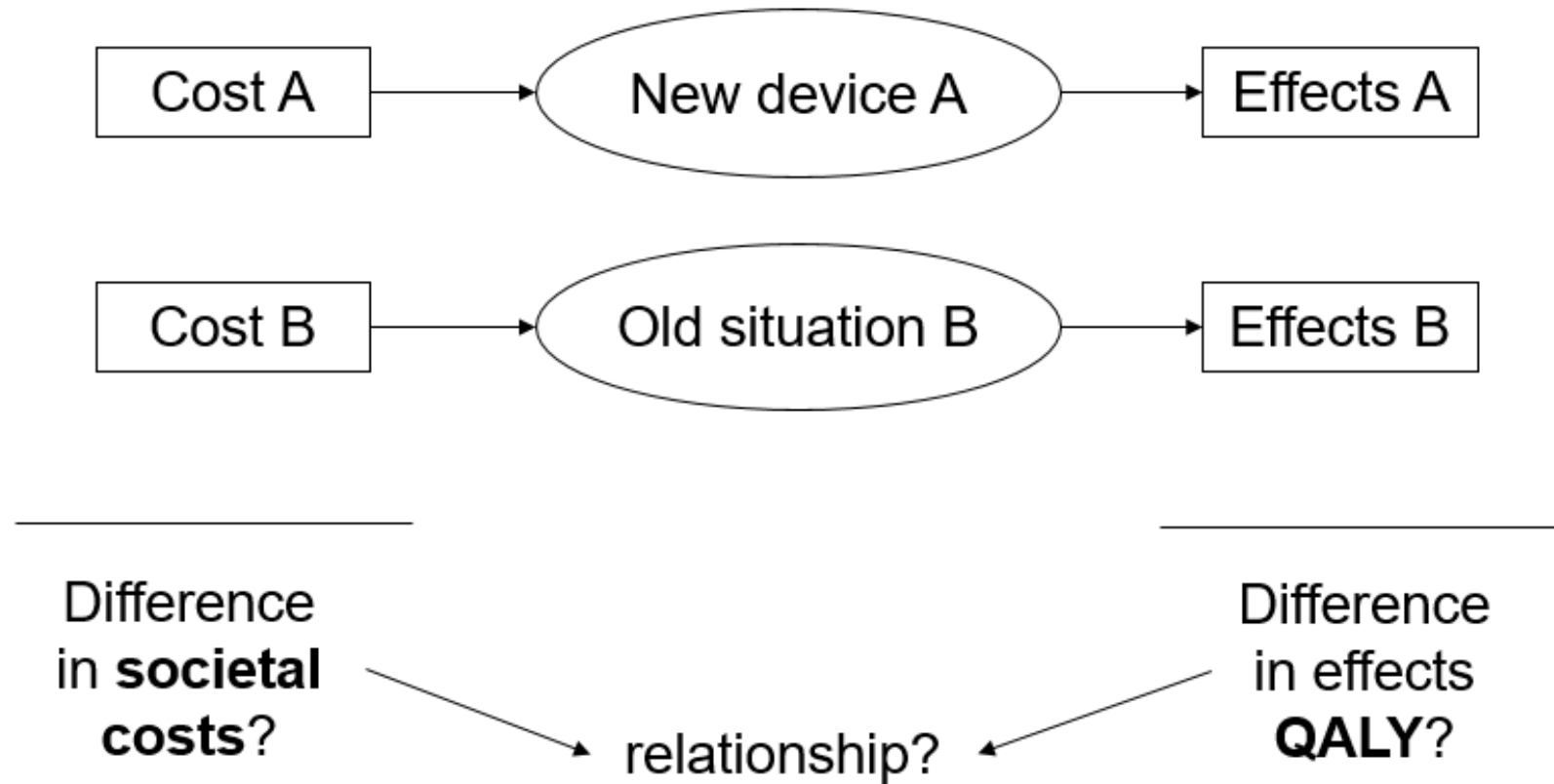
Economic evaluation / cost-effectiveness



Economic evaluation looks at the costs and effects of (new) interventions

- ⇒ Provide a framework for identifying and comparing the costs and benefits of different options
- ⇒ Inform decision makers about efficient healthcare allocation

Economic evaluation



Cost types



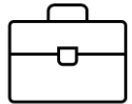
1) Medical costs

- e.g. costs for GP visits, hospitalizations, medications, etc.



2) Patient & family costs

- e.g. out-of-pocket payments, travel expenses, etc.



3) Productivity losses

- e.g. inability to work, reduced productivity at work, etc.



Clinical outcomes

- Surrogate parameter (e.g. fractures)
- Often taken out of trials, observational studies



Quality of life

- Patient reported outcome measures (PROs)
- QoL is multidimensional (physical, mental, social well-being,...)
- Increasingly measured within HRQoL studies

Quality-adjusted life years (QALY)

QALY:

One year in perfect health

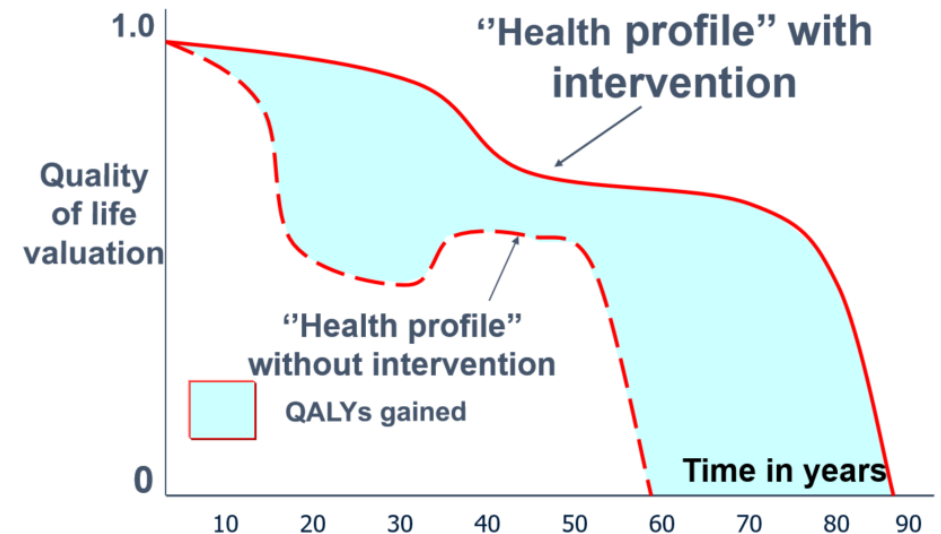
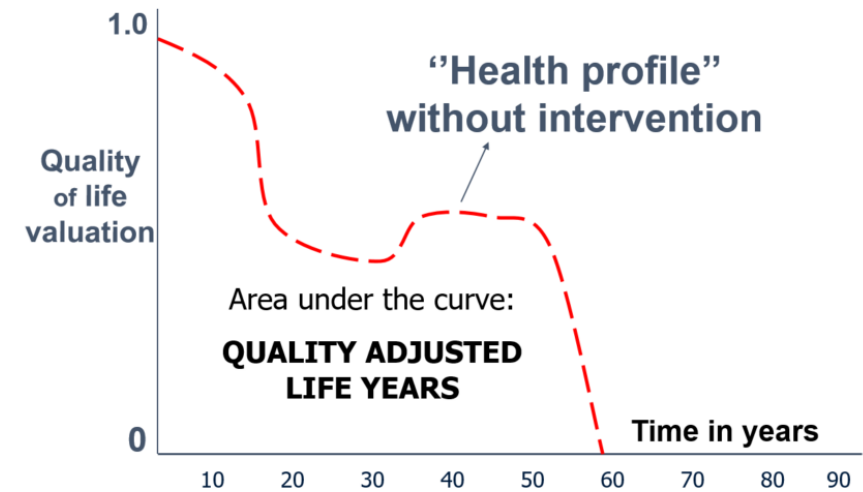
Considers length of life AND quality

= life years gained x utility

Utility:

= a number for your health state between 0 (worst health state or death) and 1 (best possible health state or full health)

- + Comparison between diseases
- + Sensitive to multiple aspects of treatments
- + Recommended for economic evaluations
- Insensitive to small changes



QALY measurement: EQ-5D

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

5 level

5 health dimensions

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

Level 1

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

Level 2

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

Level 1

PAIN / DISCOMFORT

I have no pain or discomfort

I have slight pain or discomfort

I have moderate pain or discomfort

I have severe pain or discomfort

I have extreme pain or discomfort

Level 1

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

Level 2

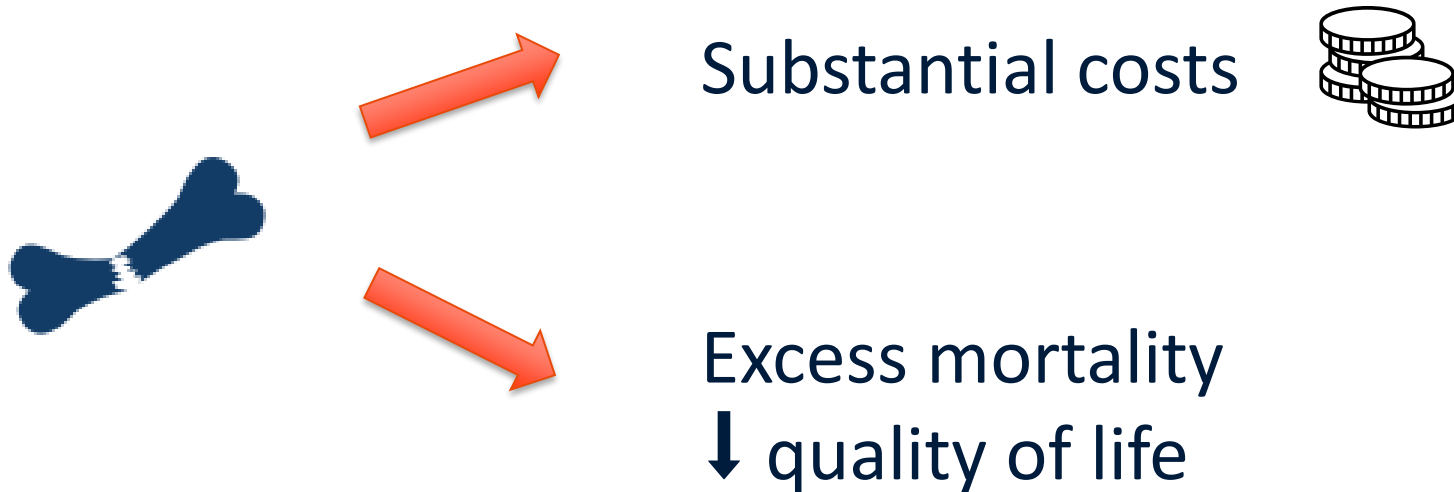


Patient profile:
e.g. 12112



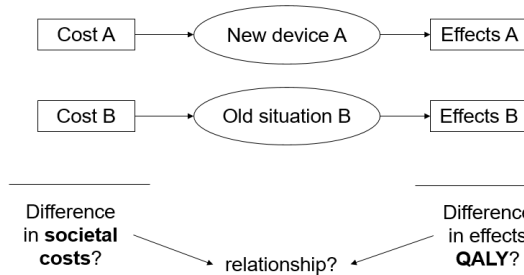
Valuation set:
Utility score
e.g. 0.723

Costs and utilities related to fractures



International Costs and Utilities Related to Osteoporotic fractures Study (ICUROS).
Multinational observational study that aims to describe costs and quality of life (QoL) consequences of osteoporotic fractures.
11 countries + 5,000 patients

Incremental cost-effectiveness ratio



$$\text{ICER} = (C_A - C_B) / (E_A - E_B) = \Delta C / \Delta E$$

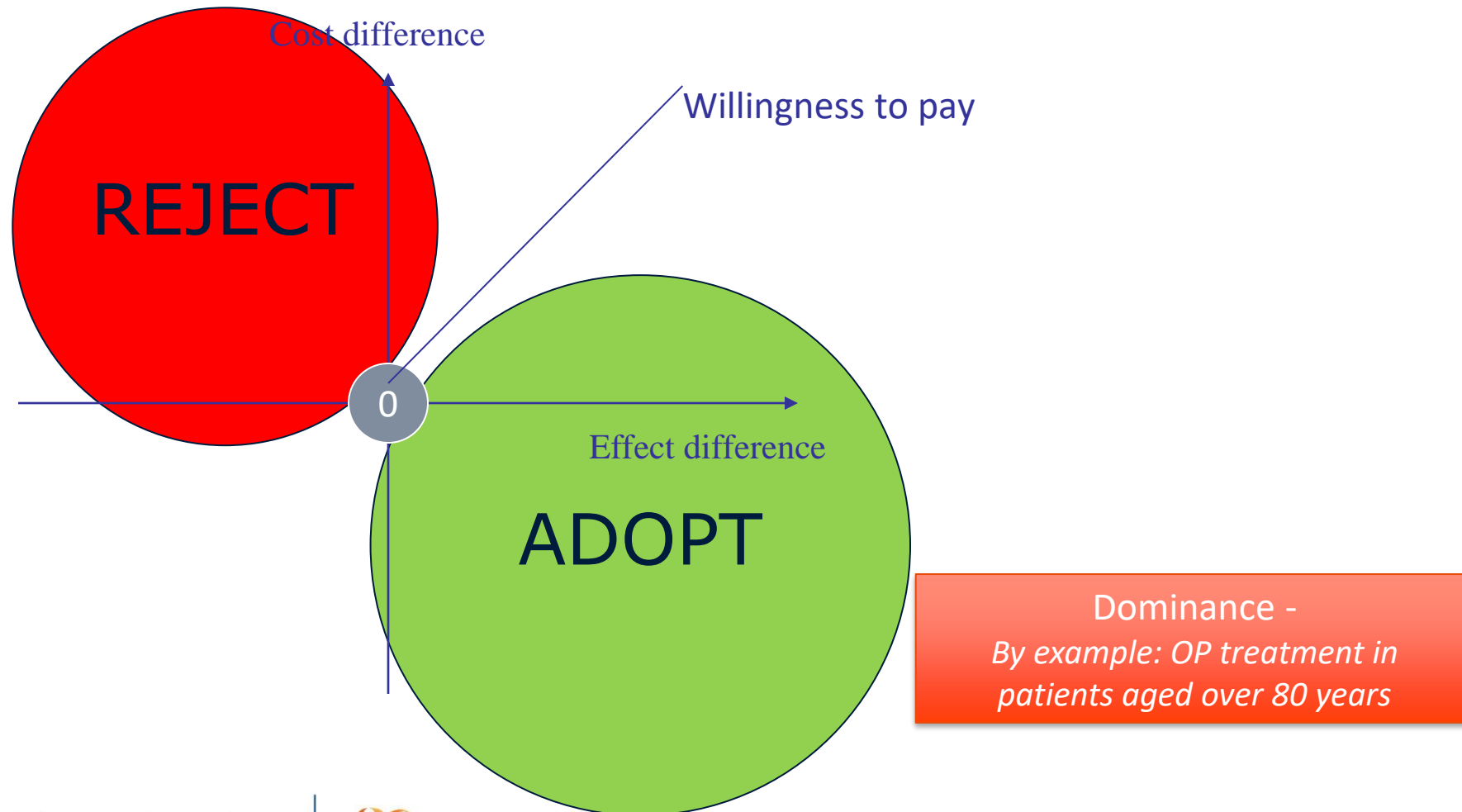
- ✓ *The additional cost per extra unit of effect from the comparator treatment*
- ✓ Additional cost per QALY gained (€/QALY)

The lower the ICER, the more cost-effective the intervention

Intervention adopted if **ICER < λ** (= willingness to pay per effectiveness unit)

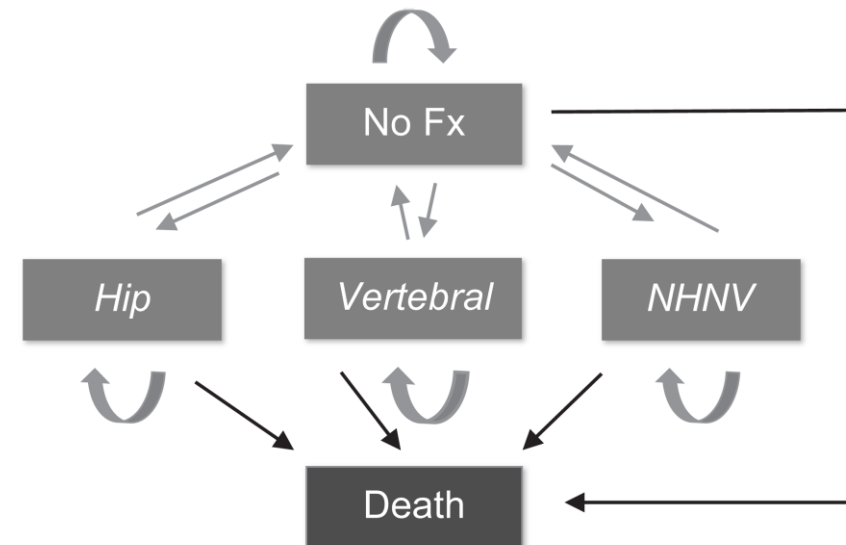
*2 X Gross Domestic Product
US: \$100,000 or \$150,000*

Cost-effectiveness plane



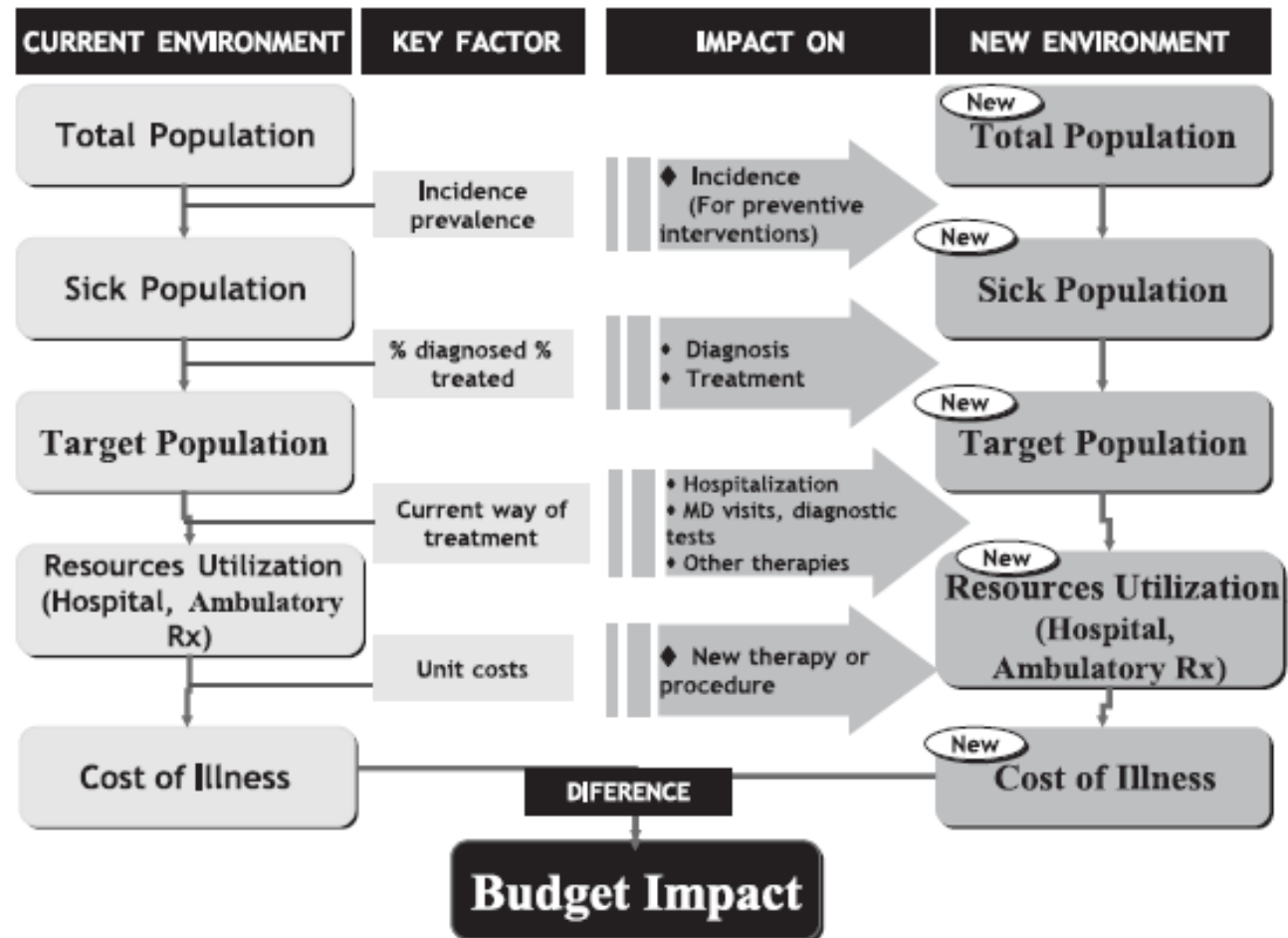
Methods for economic evaluation in osteoporosis

- Trial is not sufficient to capture all benefits and consequences of fracture prevention
- **Models can be used to**
 - Predict the health outcome and cost consequences of an intervention
 - Beyond the scope of available evidence - *extrapolation*
 - When interventions cannot be evaluated directly - *indirect comparison*
 - Generalise results to other settings or patient groups



- Essential part of a comprehensive **economic assessment** of health care technology
- Increasingly **required** in traditional HTA, along with CEA, prior to reimbursement
- To assess possible impact an **innovation** might have on current situation

*Budget impact analyses are used to estimate the **likely change in expenditure** to a specific budget holder resulting from a decision to reimburse a new healthcare intervention or some other change in policy at an aggregate population level. The budget (or financial) impact is usually calculated using a **budget impact model**, over a **period of 3 to 5 years**, at a national level or for more local healthcare payers and providers. In contrast to cost-effectiveness analyses, which are used to estimate value for money, analyses using budget impact models assess **affordability**. Two scenarios are usually compared: a world in which the new intervention or policy is implemented, and a counterfactual world without the new intervention. Each scenario takes into account population size, patient eligibility, speed of uptake and market share of the intervention, as well as many of the inputs associated with a model-based cost-effectiveness analysis. Budget impact models are commonly used by local or national-level decision makers for **planning purposes**, especially where (extra) expenditure in one budget is offset by savings in another.*



Key messages regarding economic evaluations on therapeutic options and prevention programs for osteoporosis

Cost-effectiveness of anti-osteoporosis medications



PharmacoEconomics (2021) 39:181–209
<https://doi.org/10.1007/s40273-020-00965-9>

SYSTEMATIC REVIEW



An Updated Systematic Review of Cost-Effectiveness Analyses of Drugs for Osteoporosis

Nannan Li¹ · Dennis Cornelissen¹ · Stuart Silverman² · Daniel Pinto³ · Lei Si^{4,5} · Ingrid Kremer¹ · Sandrine Bours⁶ · Robin de Bot^{1,7} · Annelies Boonen⁶ · Silvia Evers¹ · Joop van den Bergh^{8,9,10} · Jean-Yves Reginster¹¹ · Mickaël Hiligsmann¹

- ✓ 27 articles (2013-2019)
- ✓ 15 countries
- ✓ 12 active comparators
- ✓ Sequential therapy

- Cost effective in postmenopausal women aged over 60-65 years with low bone mass, especially with prior (vertebral) fractures
- Dominant in women aged 80 years and over
- Active agents cost-effective or dominant compared to traditional oral bisphosphonates
- It is difficult to make clear recommendations between drugs in terms of cost-effectiveness

Cost-effectiveness of sequential therapies



Osteoporosis International
<https://doi.org/10.1007/s00198-022-06626-1>

REVIEW

A systematic review of cost-effectiveness analyses of sequential treatment for osteoporosis

Guangyi Yu¹ · Suiju Tong¹ · Jinyu Liu² · Yuansheng Wan³ · Min Wan¹ · Sujuan Li¹ · Ruxu You³

- ✓ 10 articles (up to June 2022)
- ✓ 4 countries

- Better health outcomes with sequential therapies
- Cost-effectiveness or dominance of sequential therapies with an anabolic first followed by antiresorptive compared to bisphosphonate monotherapy (75% of studies)

Cost-effectiveness in men with osteoporosis



Cost-Effectiveness Analyses of Interventions for Osteoporosis in Men: a Systematic Literature Review

Nannan Li¹, Charlotte Beaudart², Jane A Cauley³, Steven W Ing⁴, Nancy E Lane⁵, Jean-Yves Reginster^{2,6},
Stuart Silverman⁷, Andrea J Singer⁸, Mickaël Hiligsmann¹

Accepted for publication in *Pharmacoeconomics* 2023

- ✓ 25 articles (up to June 2022)
- ✓ Drugs/nutrition, intervention thresholds, screening, post-fracture care program

- Cost-effectiveness of anti-osteoporosis drugs and nutrition supplements in men with osteoporosis
- Economic benefits of screening strategies and post-fracture care programs for men
- Cost-effectiveness and intervention thresholds generally similar in studies conducted in both men and women, with slightly greater ICERs in men

Cost-effectiveness of fracture liaison services (FLS)



Osteoporosis International (2018) 29:1227–1242
<https://doi.org/10.1007/s00198-018-4411-2>

REVIEW



Economic impact and cost-effectiveness of fracture liaison services: a systematic review of the literature

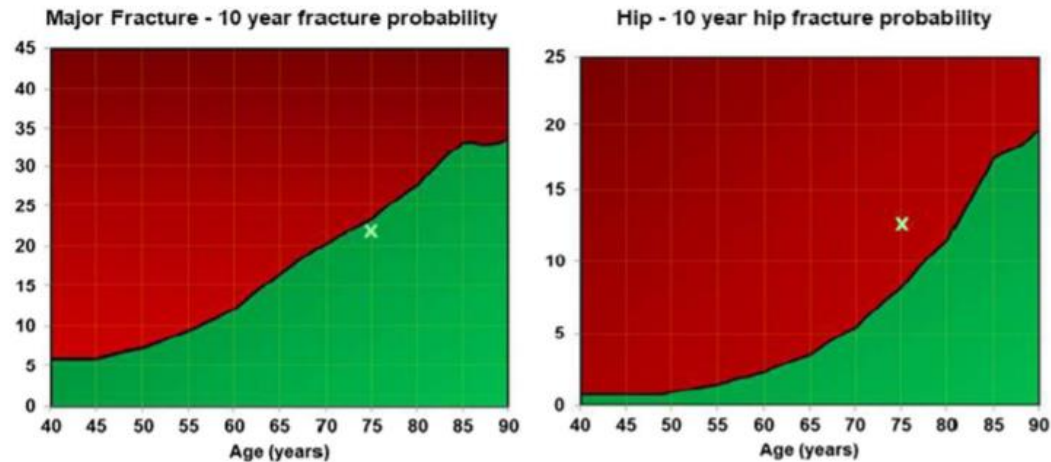
C.-H. Wu¹ · I.-J. Kao² · W.-C. Hung³ · S.-C. Lin⁴ · H.-C. Liu² · M.-H. Hsieh⁵ · S. Bagga⁶ · M. Achra⁶ · T.-T. Cheng⁷ · R.-S. Yang⁸

- ✓ 33 articles (2000-2016)
- ✓ 7 countries

- FLS was cost-effective in comparisons with usual care or no treatment, regardless of the program intensity or the country
- Dominance (more QALYs, less costs) in numerous studies

Cost-effectiveness and intervention thresholds

Intervention Threshold




Kanis JA et al. Arch Osteoporos (2016) 11: 25.

Osteoporosis International (2021) 32:133–144
<https://doi.org/10.1007/s00198-020-05536-4>

ORIGINAL ARTICLE



Cost-effectiveness of FRAX[®]-based intervention thresholds for management of osteoporosis in Singaporean women

M. Chandran¹  · G. Ganesan² · K.B. Tan^{2,3} · J.-Y. Reginster⁴ · M. Hiligsmann⁵

Health economic assessment

- to determine at which fracture risk it is cost-effective to treat patients
- to assess the cost-effectiveness of FRAX-based intervention thresholds

Other applications



Osteoporosis International (2020) 31:1499–1506
<https://doi.org/10.1007/s00198-020-05372-6>

ORIGINAL ARTICLE



Long-term cost-effectiveness of screening for fracture risk in a UK primary care setting: the SCOOP study

E. Söreskog¹ · F. Borgström^{1,2} · L. Shepstone³ · S. Clarke⁴ · C. Cooper^{5,6,7} · I. Harvey³ · N. C. Harvey^{5,6} · A. Howe³ · H. Johansson^{8,9,10} · T. Marshall¹¹ · T. W. O'Neill^{12,13} · T. J. Peters¹⁴ · N. M. Redmond^{14,15} · D. Turner³ · R. Holland¹⁶ · E. McCloskey^{8,17,18} · J. A. Kanis^{8,10} · SCOOP study team

	Usual management	Screening	Screening vs. usual management
Total cost (£), per patient	9596	9355	– 241
QALYs, per patient	7.359	7.369	0.011
Cost/QALY			Cost-saving

Osteoporos Int (2016) 27:2697–2707
DOI 10.1007/s00198-016-3596-5



ORIGINAL ARTICLE

Potential cost-effectiveness for using patient decision aids to guide osteoporosis treatment

H. Penton^{1,2} · M. Hiligsmann³ · M. Harrison^{2,4} · J.-Y. Reginster⁵ · A. Boonen⁶ · N. Bansback^{4,7}

Cost-effectiveness: some key messages

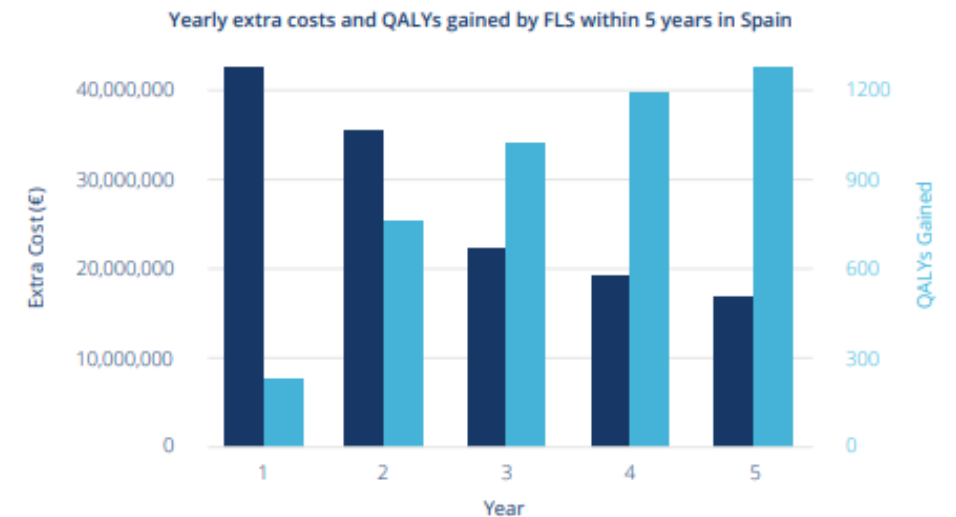
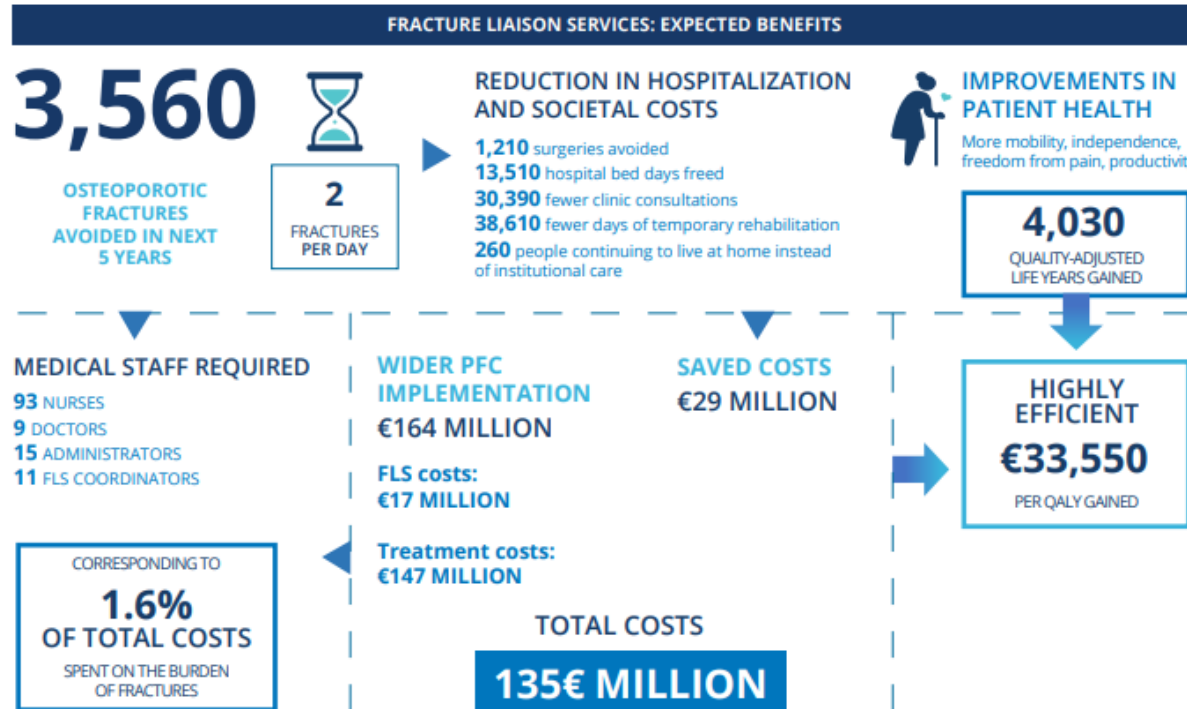
- Anti-osteoporosis medications
 - Cost-effective in women and men at risk for fractures
 - Dominance in those aged 80 years and over
 - Sequential therapies (anabolic/antiresorptive) cost-effective in high risk
- Post-fracture care programs (FLS)
 - Highly cost-effective
- (FRAX) intervention thresholds
 - Cost-effective



Arguments to convince policy makers

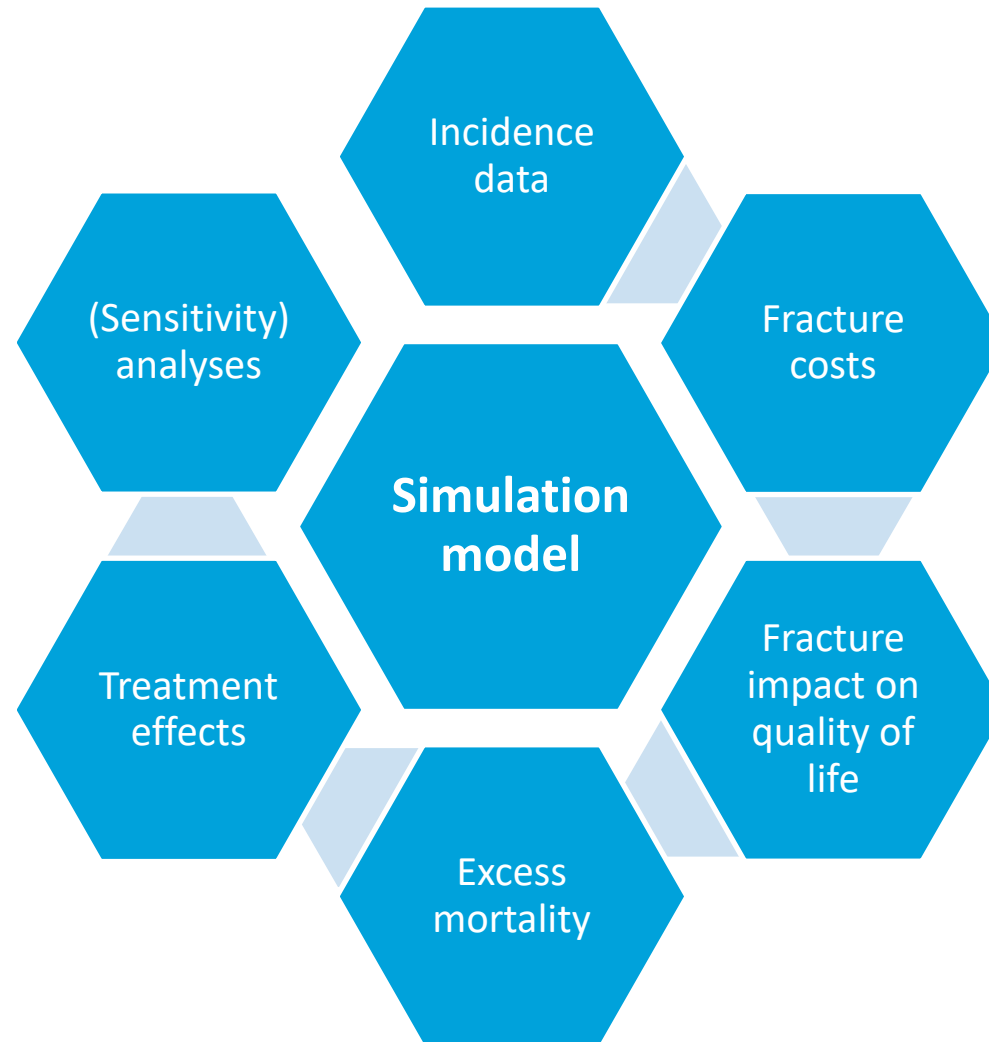
Capture the Fracture® Partnership

Example of budget calculator for FLS in Spain



How to conduct economic evaluations in osteoporosis?

Conduct of economic evaluations in OP



Recommendations for economic evaluation in OP



Osteoporosis International (2019) 30:45–57
<https://doi.org/10.1007/s00198-018-4744-x>

CONSENSUS STATEMENT



Recommendations for the conduct of economic evaluations in osteoporosis: outcomes of an experts' consensus meeting organized by the European Society for Clinical and Economic Aspects of Osteoporosis, Osteoarthritis and Musculoskeletal Diseases (ESCEO) and the US branch of the International Osteoporosis Foundation

M. Hilgsmann¹ · J.-Y. Reginster^{2,3} · A.N.A. Tosteson⁴ · S.V. Bukata⁵ · K.G. Saag⁶ · D.T. Gold⁷ · P. Halbout⁸ · F. Jiwa⁹ · E.M. Lewiecki¹⁰ · D. Pinto^{11,12} · J.D. Adachi¹³ · N. Al-Daghri³ · O. Bruyère² · M. Chandran¹⁴ · C. Cooper^{15,16} · N.C. Harvey¹⁵ · T.A. Einhorn¹⁷ · J.A. Kanis^{18,19,20} · D.L. Kendler²¹ · O.D. Messina²² · R. Rizzoli²³ · L. Si^{24,25} · S. Silverman²⁶

Received: 23 August 2018 / Accepted: 16 October 2018 / Published online: 31 October 2018
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Type of economic evaluation

- Cost-utility analysis using QALY as outcome

Method for the conduct of economic evaluation

- A model-based economic evaluation

Modeling technique

- Lifetime horizon
- Markov model is appropriate (6 months/1 year cycle length)
- Avoid hierarchy of fractures and restrictions after fracture events
- Hip, clinical vertebral, and non-vertebral non-hip fracture

Base-case analysis and population

- Multiple scenarios: age range, BMD, and fracture risk scenarios
- At least a scenario including a 10-year risk of a major osteoporotic fracture equal to 20% or with a BMD T-score ≤ -2.5 with or without fractures
- The FRAX® or GARVAN® tools can be used to model fracture risk
- Increased risk after fracture events within the model

Mortality

- Excess mortality after hip fractures
- Proportion attribute to the fracture (e.g., 25–30%)

Fracture costs and utility

- Societal and/or healthcare payer perspective
- Acute fracture costs
- Long-term costs after hip fracture (attributable to the fracture)
- First year and subsequent years' effects of fractures on disutility
- National ICUROS data if available
- An additional effect (on costs and/or utility) after multiple fractures

Treatment characteristics

- Treatment duration similar to guidelines or RCTs
- Comparators: no treatment and relevant active osteoporotic agent(s)
- Sequential therapy may be considered as intervention/comparators
- Efficacy data from RCTs, (network) meta-analysis
- In the absence of hip/wrist specific efficacy data, use of non-vertebral or clinical fracture efficacy data
- Treatment effects after discontinuation depending on treatment
- Medication adherence as sensitivity analysis
- Drug costs and administration/monitoring costs
- Adverse events

Recommendations for

- the design and conduct of economic evaluations in osteoporosis
- regarding the reporting of economic evaluations in osteoporosis, as a complement to the CHEERS 2022 checklist

+ **osteoporosis-specific reference case** to serve a minimum standard for all economic analyses in osteoporosis

⇒ To improve the transparency, quality, and comparability of economic evaluations in osteoporosis

Promoting high-quality methodology standards has the potential to increase their use by decision-makers and to lead to a more effective allocation of resources

Challenges of economic evaluation in osteoporosis



- Differences in fracture risk, comparators, costs between countries => national study
- Lack of head to head comparisons => network meta-analysis
- Quality of model structure => study reliability
- Poor reporting => CHEERS 2022 + ESCEO/IOF guideline

Conclusion

New developments in health economics

- Real-world data / evidence (ESCEO working group at WCO-ESCEO 2023)
- Investigating patient preferences and values
- Early health economics
- New elements of value

Osteoporosis International
<https://doi.org/10.1007/s00198-022-06310-4>

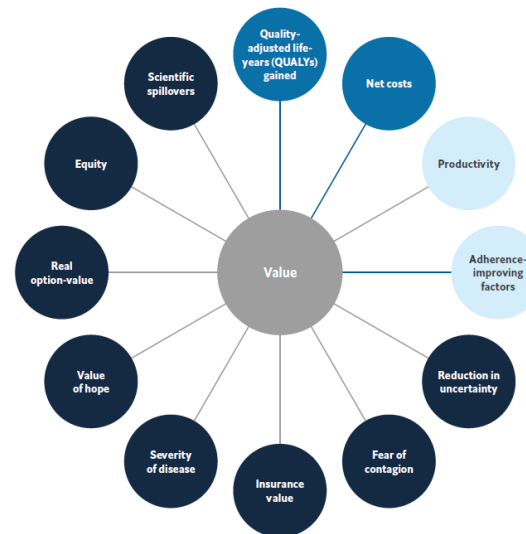
ORIGINAL ARTICLE



Patient preferences for lifestyle behaviours in osteoporotic fracture prevention: a cross-European discrete choice experiment

C. Beaudart¹ · A. Boonen² · N. Li¹ · S. Bours¹ · S. Goemaere³ · J.-Y. Reginster⁴ · C. Roux⁵ · B. McGowan⁶ · A. Diez-Perez⁷ · R. Rizzoli⁸ · C. Cooper⁹ · M. Hiligsmann¹

Figure 4: The ISPOR special task force's elements of value "flower"
The mid-blue circles are core elements of value. Light blue circles are common but inconsistently used elements of value. The dark blue circles are potential novel elements of value.



Final key messages

Health economics is unavoidable ...
... because **scarcity** is unavoidable

But we must not focus narrowly on cost
... but on **cost-effectiveness**

So ... importance of taken **economic arguments** into consideration in (policy) decision-making!

Extra resources

- <https://www.maastrichtuniversity.nl/maastrichteta/self-learning-course>



- Capture the Fracture® Partnership Policy Group

WCO-ESCEO 2023: Non-sponsored Symposium



Thank you for your attention!

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