How can health economics help us to optimize osteoporosis management?

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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)

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

1. Expected number of adults aged 75 and above between 2019 and 2034:
   - Men: +42.6%
   - Women: +29.6%

2. Increased incidence of fragility fractures:
   - 2019: 4.28 million
   - 2034: 5.34 million
   - Total fragility fractures: +24.8%
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 have 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 (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.

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 A → New device A → Effects A

Cost B → Old situation B → Effects B

Difference in societal costs? → relationship? → Difference in effects QALY?
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.
Outcomes

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
  \[ \text{QALY} = \text{life years gained} \times \text{utility} \]

**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

**Comparison between diseases**

**Sensitive to multiple aspects of treatments**

**Recommended for economic evaluations**

**Insensitive to small changes**

**Diagram:**
- "Health profile" without intervention
- "Health profile" with intervention
- Area under the curve: QUALITY ADJUSTED LIFE YEARS
- Time in years
- Quality of life valuation
- QALYs gained
- Time in years
QALY measurement: EQ-5D

Patient profile: e.g. 12112

Valuation set:
Utility score e.g. 0.723
Costs and utilities related to fractures

- Substantial costs
- Excess mortality
- ↓ quality of life

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

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)

ICER = \( \frac{C_A - C_B}{E_A - E_B} = \frac{\Delta C}{\Delta E} \)

2 X Gross Domestic Product
US: $100,000 or $150,000
Cost-effectiveness plane

- Cost difference
- Effect difference

REJECT

ADOPT

Willingness to pay

Dominance -
By example: OP treatment in patients aged over 80 years
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
Budget impact analysis

- 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

- 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

- 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

Namini Li, Charlotte Brosdahl, Jane A. Caudle, Steven W. Ing, Nancy E. Lane, Jean-Yves Reginster, Stuart Silverman, Andrea J. Singer, Michael Hiligsmann

Accepted for publication in PharmacoEconomics 2023

- 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

✓ 25 articles (up to June 2022)
✓ Drugs/nutrition, intervention thresholds, screening, post-fracture care program
Cost-effectiveness of fracture liaison services (FLS)

- 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

✓ 33 articles (2000-2016)
✓ 7 countries
Cost-effectiveness and intervention thresholds

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
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

https://www.osteoporosis.foundation/sites/iofbonehealth/files/2023-01/2022_country_profile_sp.pdf
How to conduct economic evaluations in osteoporosis?
Conduct of economic evaluations in OP

- Incidence data
- Fracture costs
- Fracture impact on quality of life
- Excess mortality
- Treatment effects
- (Sensitivity) analyses

Simulation model
Recommendations for economic evaluation in OP

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 disability
- National ICUOS 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

References

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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

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**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
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/maastrichtheta/self-learning-course](https://www.maastrichtuniversity.nl/maastrichtheta/self-learning-course)

- Capture the Fracture® Partnership Policy Group
  WCO-ESCEO 2023: Non-sponsored Symposium
Thank you for your attention!

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