Empirical evidence and future directions for equity weighting

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Proportional shortfall

Propective health

Remaining life expectancy

Quality of life

Absolute shortfall
Proportional shortfall

Quality of life

Prospective health

Remaining life expectancy

Absolute shortfall

‘Severity of illness’

‘Severity of illness’
Proportional shortfall

'Severity of illness'

Prospective health

Quality of life

Absolute shortfall

'Remaining life expectancy'

'Severity of illness' or 'Fair innings'
Why proportional shortfall?

Main reasons:
1. Balances concerns for ‘severity of illness’ and ‘fair innings’
2. Avoids ageism in reimbursement decisions (i.e. equal weight for younger and older patients)
A brief history of..

- Decision model introduced in NL, 2001
- Severity defined as proportional shortfall, 2002
- Severity used in various ways (e.g. qualitatively or DALYs), 2005
- Use of proportional shortfall formalized, 2015
- Severity classes and reference values introduced + proportional shortfall increasingly used, 2018
A brief history of..

Since 2001, seven empirical studies examined whether proportional shortfall is aligned with societal preferences.
## Support for proportional shortfall

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<thead>
<tr>
<th>Study</th>
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Level of support: -- = no, - = limited, + = modest, ++ = strong.
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Support for proportional shortfall (2)

- Public generally prefers prioritising younger over older patients
- Consequence of using proportional shortfall is that older patients may more frequently be prioritised
How to move forward?

Adjust proportional shortfall?
- To align proportional shortfall with preferences for prioritising younger patients
- To meet the objective of avoiding ageism (by giving older patients a lower weight)

Adjust monetary reference values?
- To reflect severity-related preferences within different age groups
Societal preferences for severity and age

Two stated-preference studies conducted to examine (the strength of) societal preferences for severity and age.

Choice- and person-trade-off tasks:
- Elicit preferences for priority setting based on severity, age, and combination of both (status: in press)

Contingent-valuation tasks:
- Estimate the severity-dependent willingness to pay per QALY at different ages (status: data collection)
Societal preferences

Difference in severity, same age:
- Preference for reimbursing treatment for more severely ill patients
Societal preferences

Difference in severity, same age:
- Preference for reimbursing treatment for more severely ill patients

Difference in age, same severity level:
- Preference for reimbursing treatment for younger patients
Societal preferences

Difference in severity, same age:
  ▪ Preference for reimbursing treatment for more severely ill patients

Difference in age, same severity level:
  ▪ Preference for reimbursing treatment for younger patients

Difference in severity and age:
  ▪ Preference for reimbursing treatment for younger patients, regardless of patients’ severity level
Strength of preferences

Equity weight (median)

Severity-based
Age-based
Severity- and Age-based

Small difference
Large difference

Equal weight
Strength of preferences

Equity weight (median)

Severity-based

Age-based

Severity- and Age-based

Equal weight

Small difference

Large difference
Current decision framework

Maximum reference value per QALY gained (in €)

Severity level

0 - 0.10
0.10 – 0.40
0.41 – 0.70
0.71 – 1.00

€ 0
€ 10,000
€ 20,000
€ 30,000
€ 40,000
€ 50,000
€ 60,000
€ 70,000
€ 80,000
**Severity-dependent WTP at different ages**

<table>
<thead>
<tr>
<th>Severity</th>
<th>10 years</th>
<th>20 years</th>
<th>40 years</th>
<th>70 years</th>
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<tr>
<td>10</td>
<td>€</td>
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<td>30</td>
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<td>10</td>
<td>€</td>
<td>€</td>
<td>€</td>
<td>€ LOWER</td>
</tr>
<tr>
<td>30</td>
<td>€</td>
<td>€</td>
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</tr>
<tr>
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<td>€ HIGHER</td>
<td>€</td>
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**Hypothesis:**
- Higher willingness to pay for relatively more severely ill and younger patients.
Future directions

- Severity and age may both be important, but age may be more important
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- Proportional shortfall or reference values may need to be adjusted to account for age-related societal preferences in society or to avoid ageism.
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- If severity is not ‘it’, what else may be relevant? Rarity of diseases? Prioritising patients at the end of life?
Future directions

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- If severity is not ‘it’, what else may be relevant? Rarity of diseases? Prioritising patients at the end of life?

- How to account for uncertainty in severity estimates that may impact the outcomes of reimbursement decisions?
Calculating the SAPCE

Versteegh et al. (2019) published a method and developed a tool for calculating the severity-adjusted probability of being cost effective.

By integrating:
- Uncertainty associated with patients’ QALE (obtained from PSA)
- Uncertainty associated with remaining QALE in absence of disease (based on age- and sex-adjusted population QALE)

And:
- Obtaining a distribution for (absolute and/or) proportional shortfall
- Calculating the probability a new technology is cost-effective given the different reference values that may apply
### Table 1: Example calculation of the severity–adjusted probability of being cost-effective

<table>
<thead>
<tr>
<th>Model run</th>
<th>Disease burden calculation</th>
<th>Model results</th>
<th>Combined results</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient QALE (Qd)</td>
<td>Population QALE (Qn)</td>
<td>AS</td>
<td>PS</td>
</tr>
<tr>
<td>-----------</td>
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<tr>
<td>1</td>
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Severity–adjusted probability of being cost–effective: 40%

ICER, incremental cost-effectiveness ratio; INMB = incremental net monetary benefit; QALE, quality-adjusted life expectancy; ^a Population QALE is age and sex specific; ^b 1 = Yes.

iMTA Disease Burden Calculator

iDBC tool (R based) available for:

- The Netherlands, Norway, USA, Spain, Germany, and the UK
- (Free) download from iMTAs website: https://imta.shinyapps.io/iDBC/
Want to discuss further?
Contact me

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Additional slides
Proportional shortfall – Calculations

Different calculations in context of (strong) heterogeneity, episodic disease course, and prevention.

Heterogeneity:
- Calculated as a weighted average

Episodic course:
- Calculated and presented per subgroup *during* episode
- Representative of shortfall during episode, but total shortfall is overestimated due to exclusion of disease-free period
Proportional shortfall – Calculations (2)

Prevention:
- Moment of treatment
- Subgroup of patients who actually fall ill

Rationale:
- Illustrates the sense of urgency/necessity of preventive treatment
- Avoids differences between patients who receive preventive or curative care for the same disease
- Avoids ‘double penalty’ as relatively higher costs and lower average proportional shortfall would lead to relatively less favourable ICERs for preventive treatments
- Better aligned with objective to prioritise the more severely ill