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# Trends and ways forward in policy

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# Cost-effectiveness at ZIN: a short history

1980s First three analysis: heart and liver transplantation, IVF

1990s National fund for investigative medicine (Ontwikkelingsgeneeskunde)

1999 First pharmacoeconomic guidelines (revised in 2006 and 2016)

2000 First costing guidance (revised in 2004, 2010 and 2016)

2005 Pharmacoeconomic analyses mandatory by selection of pharmaceuticals

2012 Discussion following reassessment pharmaceuticals Pompe and Fabry

2015 Report CE in practice: introduction of reference values for ICER

# CE in reimbursement decisions: 2000s

- Early 2000s: Introduction CE expertise in 'Medicinal products reimbursement committee'

- No judgement cost-effectiveness (ICER)
  - Technical assessment of the analysis

- Question answered: Is the cost-effectiveness analysis valid?
  - Answer: yes or no (voldoende of onvoldoende onderbouwd)

    Example: the ICER of €250,000/QALY is valid

- Models part of the analysis, no strict assessment done
  - Trial based evaluations also allowed

# Typical models in 2000s

- Decision trees

- Markov models
  - Relatively simple models
  - Limited number of calculations
  - Running time
  - Small number of tabs in Excel

- Assessment of models by ZIN
  - Quick look, no in depth assessment

# Criticism in CE reports Jan 2005 – Oct 2008

Analysis of 21 CE reports

- Sound methodology in 8/21 reports
- Points of criticisms:
  - Time horizon too short 4/21
  - Not all relevant costs considered 7/21
  - Not all relevant effects considered 7/19

Total of 19 analyses including models

- Nine models described in sufficient detail
- Not all relevant deterministic sensitivity analyses performed
- Probabilistic sensitivity analysis lacking in 6 analysis

# Developments since 2010

- Developments in methodology analyses, more CE research

- Societal discussion about cost-effectiveness changing
  - Discussion Pompe and Fabry (2012) vs discussion Orkambi (2017)
  - High (very) ICERs
  - High prices of pharmaceuticals

- Changes at ZIN
  - Discussion on importance of CE
  - More advisors with CE expertise
  - More CE expertise in advisory committees

# Development in models since 2010s

- More complicated Markov models
  - Large amount of tabs in Excel
  - Increasing number of calculations
  - Impacting running time

- Other types of models
  - Patient-level models
  - Discrete event simulation

- Assessment of models by ZIN
  - Checking models extensively
  - Own adaptations / analysis

# Criticism in CE reports in 2010 till 2016

Analysis of 18 reports not valid/insufficient methodological quality

More explicit criticism

Major points of criticism:

- Methodological/model:
  - extrapolation, model structure (too simple/relative health states)
- Input parameters:
  - treatment effect, calculation utilities, costs (e.g. HCA vs FCM)
- Sensitivity analysis:
  - small ranges, not all parameters included

# Current challenges with models

- Increasingly complex models
  - More time consuming to critically assess the model
- Web-based models
  - Working in interface, not possible to assess model engine
  - Transparency issues
- Software
  - Only Excel accepted at the moment
- Important: Good documentation (technical report) and transparency

# Trends in pharmaceuticals impacting models

Different types of clinical studies

- Single arm/ phase II trials
- Surrogate outcomes, e.g. registration on PFS or tumor response

Different types of therapy

- Tumor agnostic: different comparators
- Gene therapies: duration of effect
- Small patient groups (orphans/subgroups)

Looking at treatment pathways

# Concluding remarks

- Increasing role of cost-effectiveness in decision-making
- Complexity increasing and other model types available
- Criticism changing, more advanced methodological and input parameters issues
- Several challenges with new pharmaceuticals
  - E.g. Available data and type of models
- Working on CE-analysis by non-pharmaceuticals: new challenges