Core questions of HTA:

How does a product perform as compared to other existing options?

• Different from registration questions that deal with efficacy and safety

How well has cost-effectiveness been demonstrated?

• What do we get in return for the money?
• Is CE of treatment close to relevant CE reference levels?
Why explore Real-World Evidence (RWE)?

• RCTs: a golden standard?
  • Short duration
  • Ethical issues
  • Surrogate outcomes
  • Is there a comparator?
  • Etc.

• Expansion of mandates:
  • Questions go beyond treatment X vs. Y (or placebo)
  • Societal perspective of analyses
  • Different evidence is also needed

Problems of new, expensive drugs

Frequently in-patient.

Use unevenly spread over hospitals.

High price, made known only shortly before launch.

Difficult to budget for upfront.

Rapidly-changing insights, e.g. combination treatments in oncology.

Impossible to negotiate over each and every product/indication.
Expenditure on drugs in the Netherlands

<table>
<thead>
<tr>
<th></th>
<th>2015</th>
<th>2016</th>
<th>Mutation 15–16</th>
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<tbody>
<tr>
<td>Intramural, add-ons</td>
<td>1,662</td>
<td>1,809</td>
<td>8,8%</td>
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<tr>
<td>Intramural, stollingsfactoren</td>
<td>131</td>
<td>132</td>
<td>0,9%</td>
</tr>
<tr>
<td>Extramural, WMG genemiddelen</td>
<td>2,819</td>
<td>2,881</td>
<td>2,2%</td>
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<tr>
<td>Total</td>
<td>4,612</td>
<td>4,822</td>
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<tr>
<td>Intramural, percentage cambio</td>
<td>39%</td>
<td>40%</td>
<td></td>
</tr>
</tbody>
</table>

Annual in-patient cost increase: 5–10% (~170 million)

Political agreement 2018: 1,6% (~90 million)

Annual shortage: 50 – 100 million

Cost-effectiveness out of bounds
Pompe in adults, eculizumab, gene therapy, etc.

Increase effect (QALYs)
Negotiations are getting more complicated:

- Increased number of drugs for small groups enter the system.
- The manufacturer has a monopoly position.
- Heavy pressure is orchestrated through the media.
- Hype-, hope- and belief-based medicine replace EBM/GRADE.
- An extravagant price with a rebate is still extravagant.

Do not let empathy for the few guide your reimbursement decisions for expensive drugs  
(Mathijs Versteegh, NRC, 13 oktober 2017)

With Orkambi, used in cystic fibrosis, we win 3.48 healthy life years at a cost of 1.5 billion euro.

In cardiology, that amount of money would generate much more health: 36.59 QALYs.
ICER is not a final decision but the starting point of societal debate.

Mitigating circumstances:
- Strong effects
- Initiatives for treating wisely by patients and doctors (eculizumab)

Aggravating circumstances:
- Refusal MAH to explain pricing

What is “value-based” healthcare?

Value means something different to every stakeholder.

It’s thus only helpful if effects are translated into...

measurable and objectifiable outcomes that mean something to patients.
A transition is needed!

Current Process:
Hype/Hope-driven
Top-down

Constructive collaboration & proper risk-sharing

Future Process:
Outcomes-driven
Bottom-up

1st outcomes markup

2nd outcomes markup

€ 15.000 “Claxton Ceiling”

“3 x GDP ceiling”: max. reimbursement

Negotiated price

Proposed price

Protection expiry

Cost/Per Y (€)
Role of registries

Public access to registries.
Filled with data paid for by public money.
EU registry cooperation should be obligatory.

When relying on registry data for rapid reimbursement
Data collection, evaluation & interpretation not in one hand

RWE for New HTA Methodology

Indication - metastatic melanoma
Post-)effectiveness of immunotherapies

Program - Electronic Health Records (EHR’s)

Patient registries to assess effectiveness

Preliminary discussions on scope etc.
Thank you for your attention.

Questions?

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