Sustainable funding and fair pricing for orphan drugs. What are the solutions? – the Dutch approach

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History of orphan drug availability in NL (inpatient drugs)

-2006 : earmarked open-ended budget (subsidy scheme MoH)

2006-2012 : policy rules (reimbursement in return for observational studies of 4 years followed by reassessment (eg Pompe & Fabry))

2013- now : automatic influx in system for inpatient orphans; selection of drugs assessed
Inpatient drugs: Automatic entry

Outpatient drugs: Check at entry

Expensive inpatient drugs: Lock system

Policy on orphan drugs in the Netherlands

- Same decision criteria as for common drugs
  - In general assessment of CE further developed
  - Introduction of references values
  - Specification of CE conclusions

- In 2015: introduction of ‘orphan drug policy’
  - Special arrangements
  - Indication committee
  - Start & stop criteria
Assessment of orphan drugs

• Often only one drug available for indication, comparator best supportive care

• Limited evidence available, eg
  • Number of patients
  • Short follow-up period, surrogate endpoints
  • Heterogeneous effect
  • Single arm trials or phase 2 studies

• Cost-effectiveness models
  • Uncertainty
  • Effectiveness: extrapolation and endpoints
  • High costs of drugs

Burden of disease on a scale of 0 to 1

Using proportional shortfall

Absolute shortfall also presented
Appraisal: ICER and other criteria

- Reference value not only criterion, also other criteria considered
- Mitigating circumstances by high ICER
  - Strong effects
  - Treating wisely initiatives
- Aggravating circumstances by high ICER
  - Refusal MAH to explain pricing
- Displacement arguments vs serious orphan disease
- Advise to MoH to negotiate with MAH (No, unless…)

<table>
<thead>
<tr>
<th>Products with reference value €80,000/QALY</th>
<th>ICER (€/QALY)</th>
<th>Chance (%)</th>
<th>Cost-effective</th>
<th>Budget impact in million €</th>
<th>Costs in € per patient per year</th>
</tr>
</thead>
<tbody>
<tr>
<td>trastuzumab Herceptin® breast</td>
<td>15,000</td>
<td>100</td>
<td></td>
<td>28</td>
<td>24,000</td>
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<tr>
<td>nivolumab Opdivo® lung</td>
<td>134,000</td>
<td>3</td>
<td></td>
<td>46 - 203</td>
<td>46,000</td>
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<tr>
<td>pertuzumab Perjeta® breast</td>
<td>149,000</td>
<td>2</td>
<td></td>
<td>39.5</td>
<td>78,500</td>
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<tr>
<td>iva- &amp; lumacaftor Orkambi® Cystic fibrosis</td>
<td>400,000</td>
<td>0</td>
<td></td>
<td>84 (- 125)</td>
<td>170,000</td>
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<tr>
<td>agelesidase α, β o-a. Fabrazyme® Fabry</td>
<td>3,300,000</td>
<td>-</td>
<td></td>
<td>15</td>
<td>195,000</td>
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<tr>
<td>alglucosidase α Myozyme® Pompe</td>
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<td>44</td>
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<td>eculizumab Soliris® PNH</td>
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<td></td>
<td>25</td>
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<td></td>
<td>20 - 57</td>
<td>60,000</td>
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<tr>
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<td>92,000 - 116,000</td>
<td>20 - 40</td>
<td></td>
<td>24</td>
<td>11,000</td>
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