



Zorginstituut Nederland

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

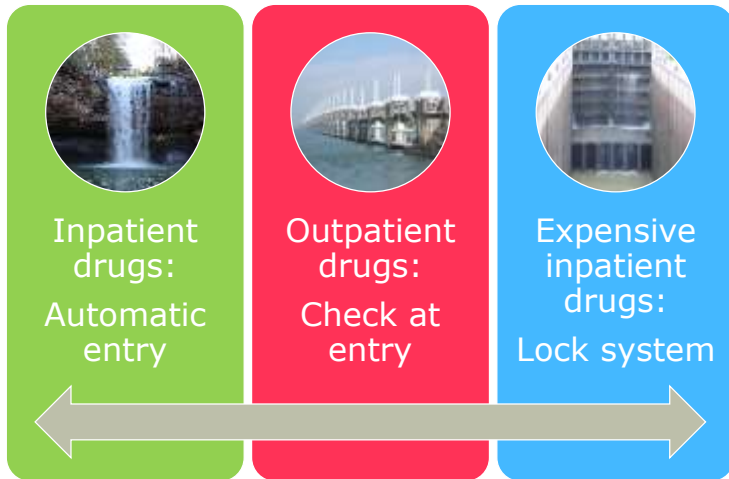
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| Van goede zorg verzekerd |



### 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



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## 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

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## 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

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### Burden of disease

0.1 – 0.4

0.4 – 0.7

0.7 – 1.0

### Maximum ICER

€20,000

€50,000

€80,000

Burden of disease on a scale of 0 to 1

Using proportional shortfall

Absolute shortfall also presented

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## 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...)

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Products with reference value €80.000/QALY	ICER (€/QALY)	Chance (%) Cost-effective	Budget impact in million €	Costs in € per patient per year
trastuzumab Herceptin® breast	15,000	100	28	24,000
nivolumab Opdivo® lung	134,000	3	46 - 203	46,000
pertuzumab Perjeta® breast	149,000	2	39.5	78,500
iva- & lumacaftor Orkambi® Cystic fibrosis	400,000	0	84 (- 125)	170,000
agalsidase α, β o.a. Fabrazyme® Fabry	3,300,000	-	15	195,000
alglucosidase α Myozyme® Pompe	300,000- 900,000	-	44	700,000
eculizumab Soliris® PNH	>480,000	0	25	360,000
everolimus Afinitor® breast	~60,000	67	20 - 57	60,000
pemetrexed Alimta® lung	92,000 - 116,000	20 - 40	24	11,000