Zorginstituut Nederland

Towards Sustainable Access to Orphan Therapies: HTA persepctives & RWE

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| Van goede zorg verzekerd |

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

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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.

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Expenditure on drugs in the Netherlands

	2015	2016	Mutatie '15-'16
Intramuraal, add-ons	1.662	1.809	8,8%
Intramuraal, stollingsfactoren	131	132	0,9%
Extramuraal, WMG geneesmiddelen	2.819	2.881	2,2%
Totaal	4.612	4.822	4,6%
Intramuraal, procentueel aandeel	39%	40%	

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

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

Annual shortage : 50 – 100 million

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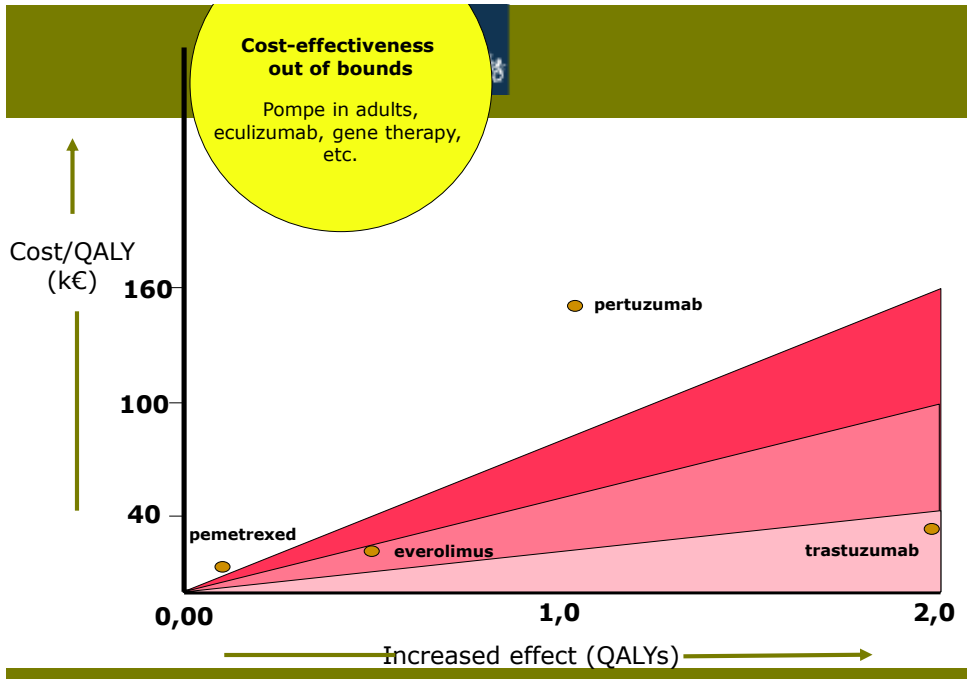

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Negotiations are getting more complicated:

- 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.

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Do not let empathy for the few guide your reimbursement decisions for expensive drugs (Matthijs 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.

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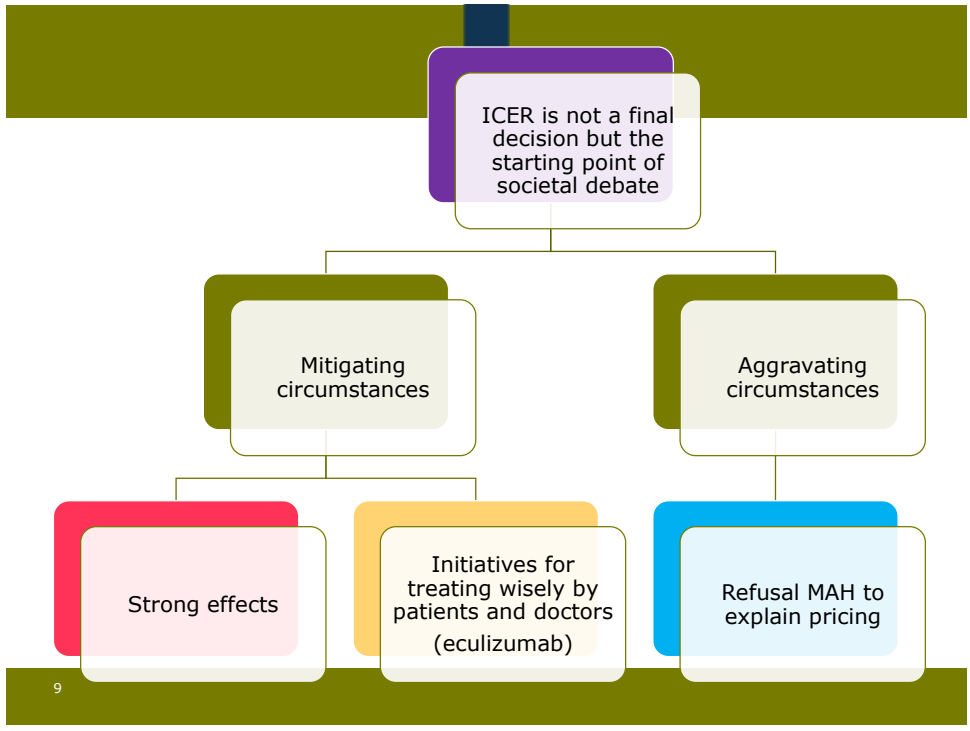

What is "value-based" healthcare?

Value means something different to every stakeholder.

It's thus only helpful is effects are translated into…

measurable and objectifiable outcomes that mean something to patiens.

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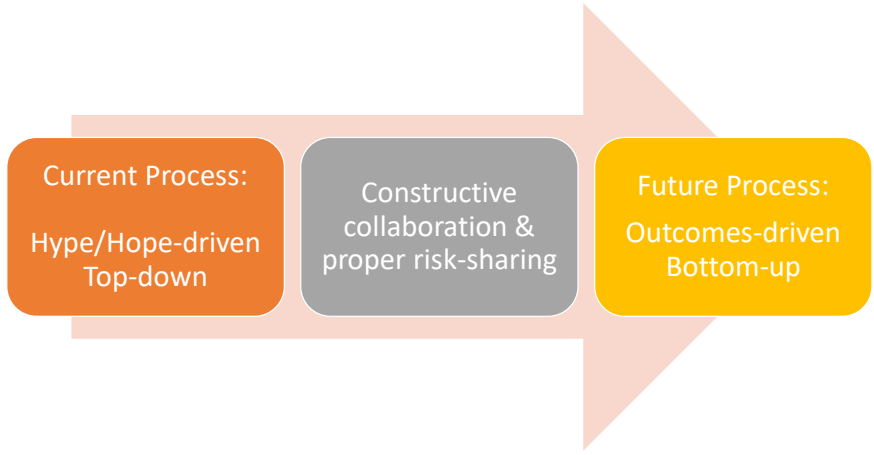
A transition is needed!



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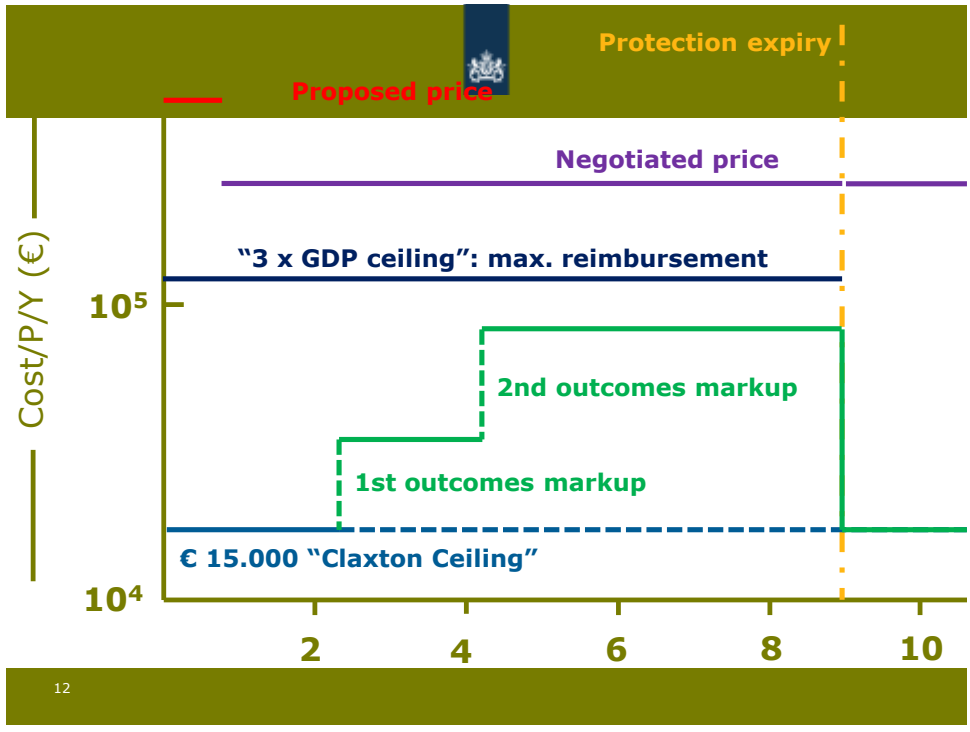

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Role of registries

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

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RWE for New HTA Methodology

Indicati… …static melanoma
…st-)effe…tiveness of
…muno… …apies

Proa…
Elec…
Rec…
…C

Patien…
asses…
effectiveness

• Prelimin… …etc.

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Thank you for your attention.

Questions?

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