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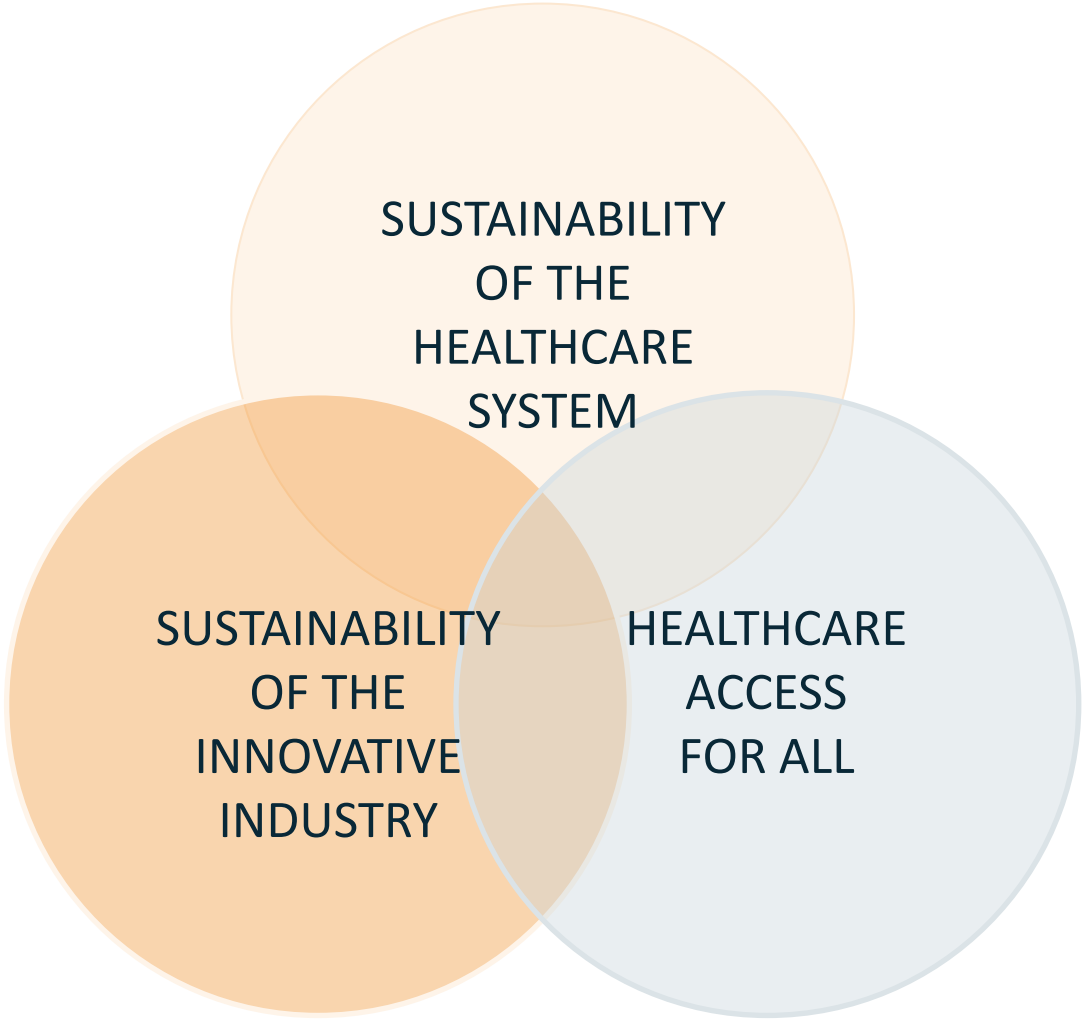
Challenges and solutions for patient access to highly specialized innovative therapies

Lieven Annemans / Bas Amesz

Zeist, June 5, 2018

The difficult exercise



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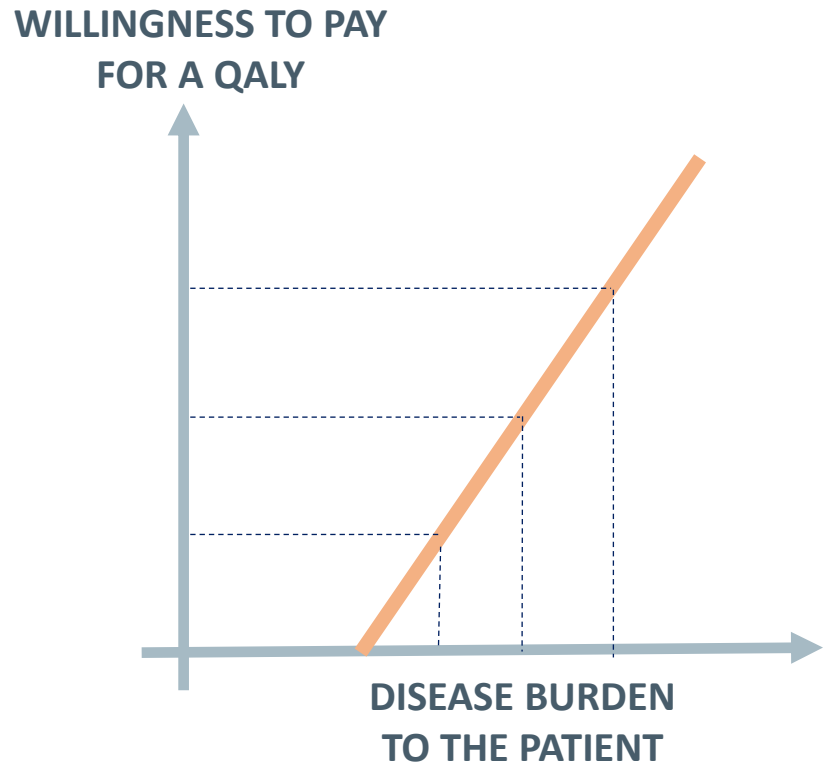
The two basic pricing options have too many drawbacks

Pricing options	Advantages	Disdvantages
 **"Cost+" price**	Acceptable mark-up as compensation for the costs of investment in R&D	Difficult to assess the true cost of R&D (what about failures?) Wrong incentives ('spend a lot on R&D') Added value not sufficiently recognized
 **Value-based pricing**	Better added-value is recognized by better rewarding	1 Not clear how much society should be willing to pay for health benefits 2 Evidence may not be sufficiently convincing at launch

1 ZIN has developed a more sophisticated approach, taking the patient burden in mind

Zorginstituut NL (ZIN): variable threshold

- **€80,000/QALY**
  for severe condition, even up to €100,000 at end-of life
- **€50,000/QALY**
  for moderate burden
- **€20,000/QALY**
  for mild burden



Source: ZIN. Kosteneffectiviteit in de praktijk | 26 juni 2015

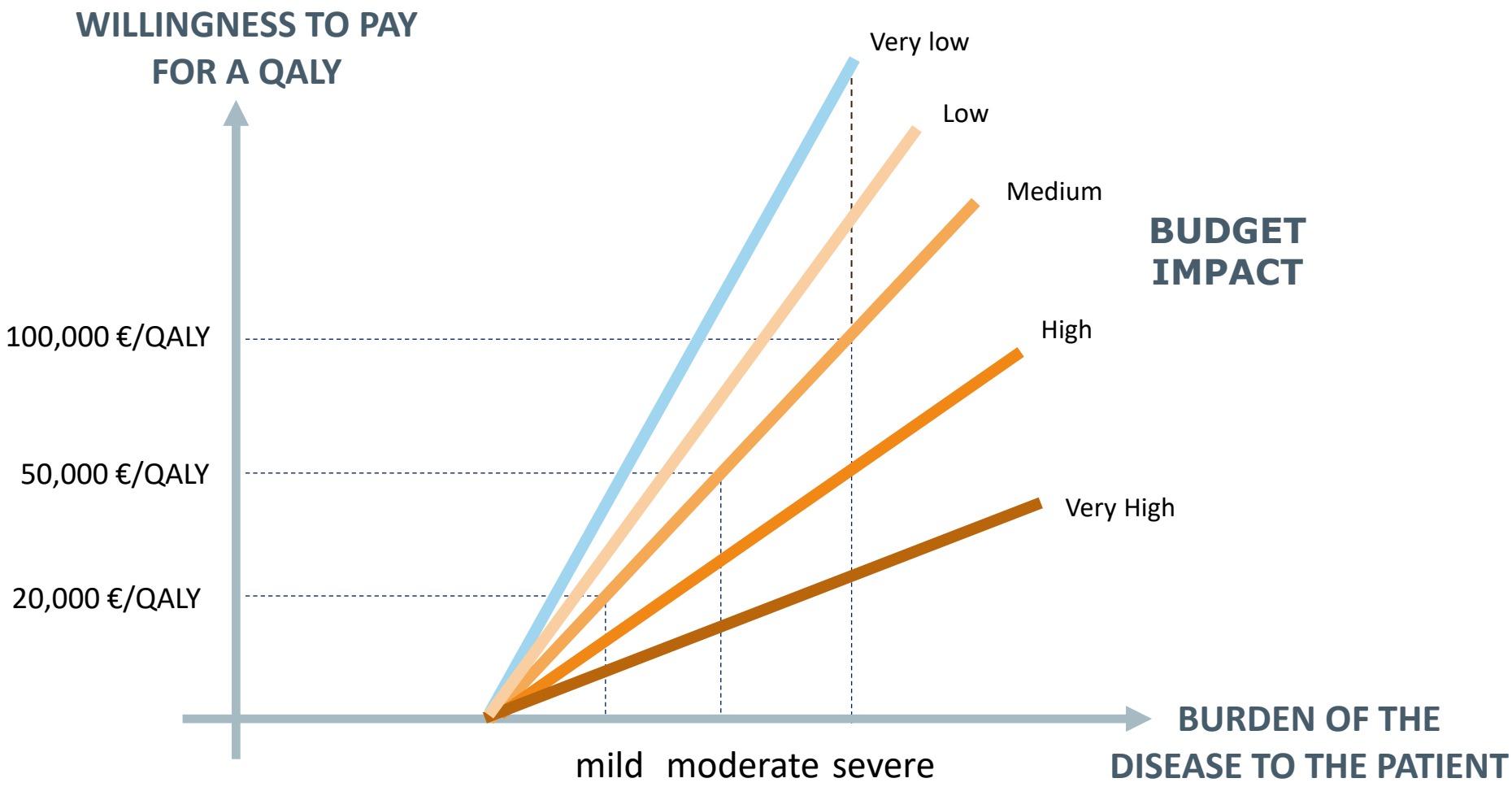
1 Budget impact needs a place in determining price levels

*"The economic and equity rationale for carrying out **budget impact analyses** is opportunity cost = benefits forgone by using resources in one way rather than another"* - Cohen et al (2008)

\ We need to address the issues of **efficient resource allocation** and **affordability**

- Need for well documented estimates at **population level**
- Need for very clear description of the **target population**
- Need for a **stratified approach** wherever possible

1 A new model takes a more integral view on pricing: **V**alue **I**nformed and **A**ffordable (VIA) pricing



Source: Annemans L, et al. Gezondheidseconomie voor niet-economen. Pelckmans Pro 2018.

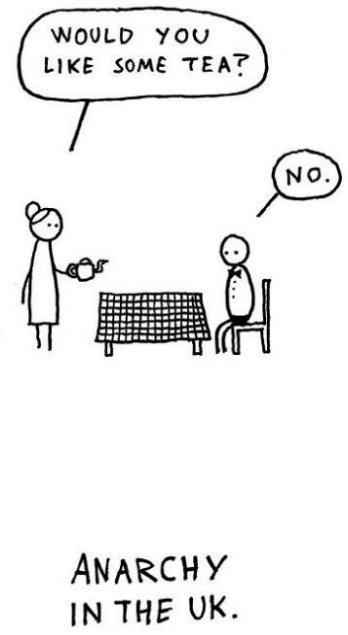
1 NICE introduces flexibility in evaluation of treatments for very rare conditions

£300,000 per QALY

for treatments deemed to provide significant QALY benefits

10x the standard threshold

is being considered in order to reflect the transformational health benefits they can offer to patients.



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1 The orphan disease price seem to be agreed, with the implicit VIA model in mind

Table 1. Preliminary cost per quality-adjusted life year incremental cost–effectiveness ratio estimates by NICE (2008).

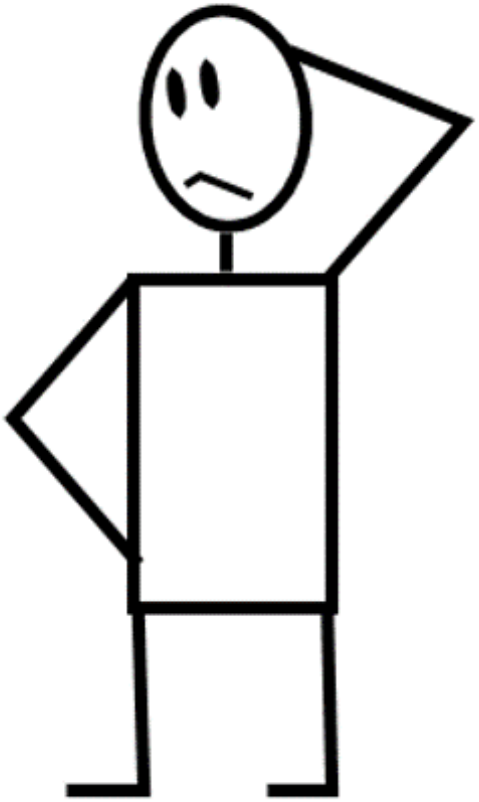
Condition	Prevalence (England)	Product	ICER (preliminary estimated £ per QALY)
M. Gaucher type I and III	270	Imiglucerase (Ceredase®)	391,200
MPS type 1	130	Laronidase (Aldurazyme®)	334,900
M. Fabry	200	Agalsidase beta (Fabrazyme®)	203,000
Hemophilia B	350	Nonacog alpha (BeneFIX®)	172,500
M. Gaucher type I	270	Miglustat (Zavesca®)	116,800

These examples from England illustrate the mismatch between ultra-orphan drug cost and conventional cost–effectiveness benchmarks as adopted by NICE (i.e., £20,000 to £30,000 per QALY gained) [8].
ICER: Incremental cost–effectiveness ratio; MPS: Mucopolysaccharidosis; QALY: Quality-adjusted life year.

Source: Schlander et al, J. Comp. Eff. Res. (2014) 3(4), 399–422

"Risk is measurable uncertainty"

"Uncertainty is unmeasurable risk"



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2 There are three key types of uncertainty

Medicine

- The exact magnitude of the treatment effect
- The possibility of a diminishing effect
- Adverse events and safety
- The dose required for optimal effect
- …

Disease

- The natural course of the disease
- Relation between surrogate and hard endpoint
- The incidence and prevalence (affecting the budget impact)
- …

Healthcare system

- patient adherence and acceptability
- provider prescription patterns
- consequences to the health care system (such as cost offsets)
- ….

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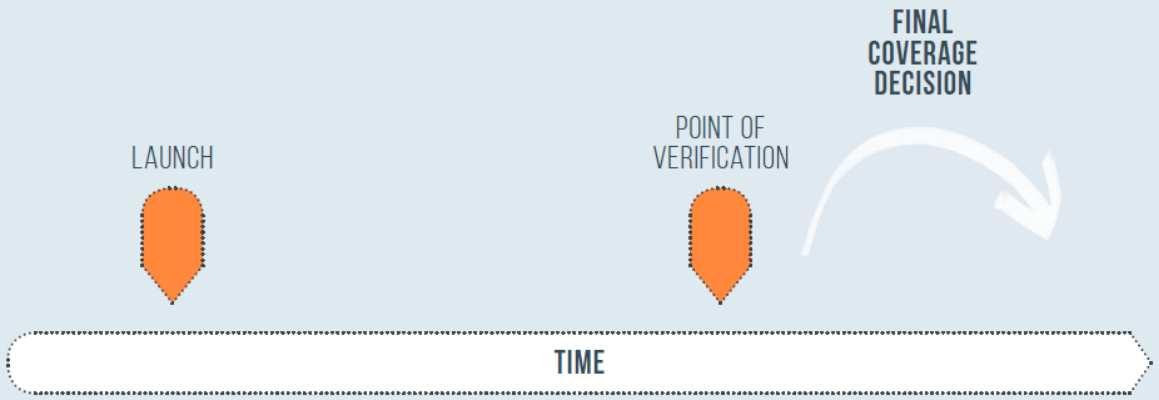
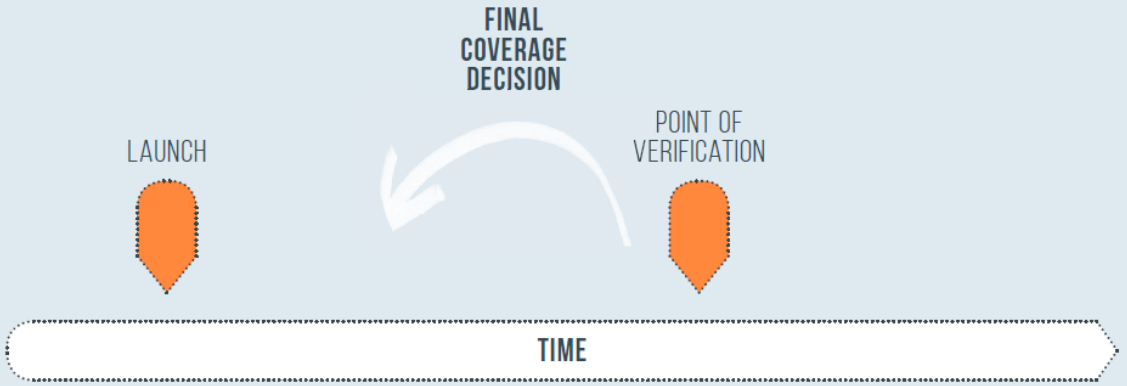
2 Outcomes based managed entry agreements: "*Do you keep your promises?*"

COVERAGE UPON EVIDENCE DEVELOPMENT



- Temporary approval, then final decision

PERFORMANCE LINKED REIMBURSEMENT



- Outcomes guarantee
- Not as good as promised: industry pays back

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2 Dynamic outcomes-based approaches to pricing reimbursement of innovative medicines - *When does it work?*

Five key considerations for outcomes-based approaches

1	Make clear what (adjusted) evidence is required for the next stage.
2	Match the appropriate study and research design to the uncertainties being addressed.
3	Implications of not meeting the requirements and expectations should also be agreed upon at the start of each step.
4	Ensure fast collection of high quality and accessible real life data.
5	Data governance and transparent public private partnerships between the health care system and the industry.

Dynamic outcomes based approaches to pricing and reimbursement of innovative medicines

A discussion document[1]

Professor Lieven Annemans and Professor Luca Pani* • February 2017

1. Introduction

Health policies in the EU aim to increase the healthy life expectancy of citizens within the limits of the available public resources. In order to achieve this objective, there is a need to improve the quality, effectiveness, and efficiency of EU health systems.[2]

In addition, there is a continuous need for innovative health technologies, such as medicines, that help to substantially reduce morbidity and mortality, and improve quality of life.[3] However, these truly innovative technologies[4] usually come at an extra cost, and – given the requirement for efficiency and sustainability – it is of key importance to establish appropriate methods and procedures for pricing and reimbursement (P&R) of these technologies.

The increasing focus in our healthcare systems on outcomes that matter for patients may create new opportunities in this regard. P&R decisions for innovative technologies that account for the added value that those technologies deliver for patients and society overall, will encourage the continued search for truly innovative technologies. Value can thereby be defined as "the importance, worth, or usefulness of something".[5] It is recognised that the value of a new medicine is determined by both disease and treatment related characteristics.[6] Indeed, if the impact of a disease on patients is high (severe symptoms, disability, reduced life expectancy etc.) and the medicine provides a

[1] This discussion paper has been commissioned by the Belgian INAMI/RIZIV and authored by Professor Lieven Annemans (University of Ghent) and Professor Luca Pani (CHMP and SAWP Member, European Medicines Agency and Department of Psychiatry and Behavioral Sciences, Leonard M. Miller School of Medicine, University of Miami). It is intended to contribute to consideration of new approaches by payers to ensure the appropriate use of health technologies. Individuals and stakeholder representatives from patient groups (The European Patients Forum, Eurordis, European Aids Treatment Group), healthcare professional organisations, government bodies (representatives from national HTA authorities and payers) and industry have participated in a series of roundtable discussions in the course of 2016 which have contributed to the development of the paper. The discussion paper is not a statement of consensus between the various stakeholders. It represents the authors' views on the topic which have been informed by their participation in the roundtable discussions. The discussions were facilitated and chaired by FIPRA and sponsored by the European Federation of Pharmaceutical Industries Association (EFPIA), Novartis, Sanofi, GSK and Roche.

[2] EU Communication on effective, accessible and resilient healthcare systems April 2014

[3] Council Conclusions on Innovation for the Benefit of Patients, December 2014

[4] Annemans L et al. Valorising and creating access to innovative medicines in the European Union. Frontiers in Pharmacology, 2011.

[5] https://en.oxforddictionaries.com/definition/value

[6] Moreno S and Ray J. The value of innovation under value-based pricing. Journal of Market access and health policy. 2014

Source: Prof. Lieven Annemans and prof. Luca Pani – Feb. 2018

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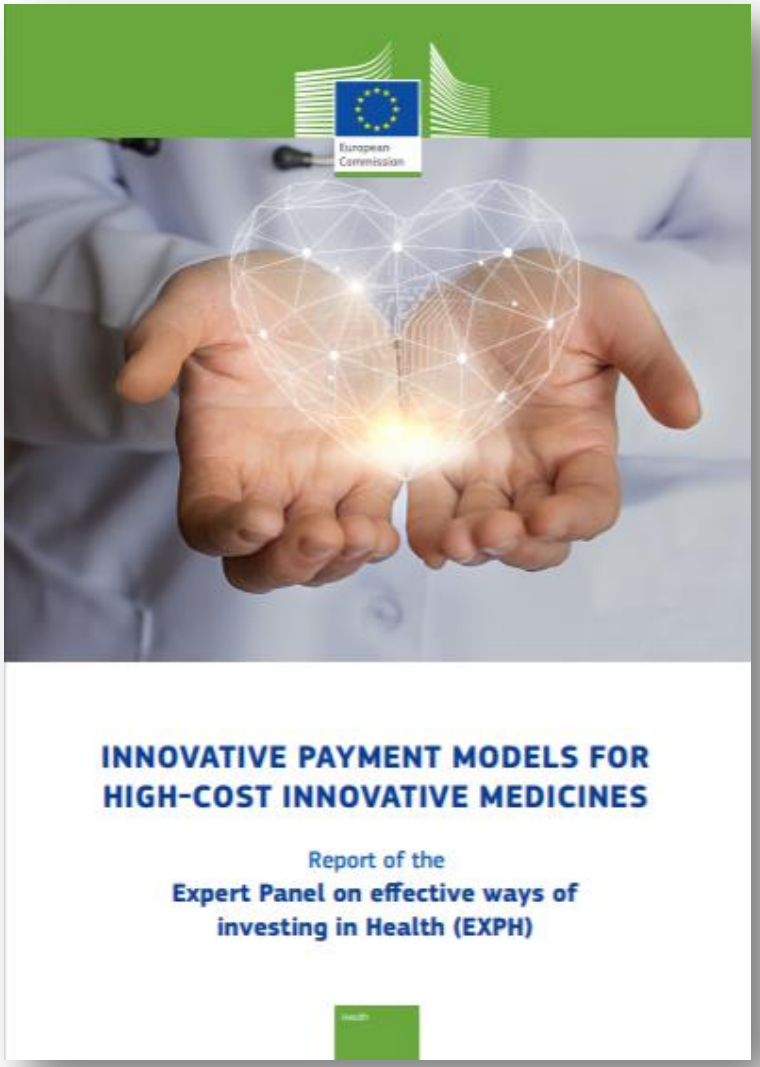
One step further…

- Expert panel on effective ways of investing in health
- Opinion on Innovative payment models for high-cost innovative medicines

"Payment systems should evolve in the direction of paying for acquisition of a service and not for a product"



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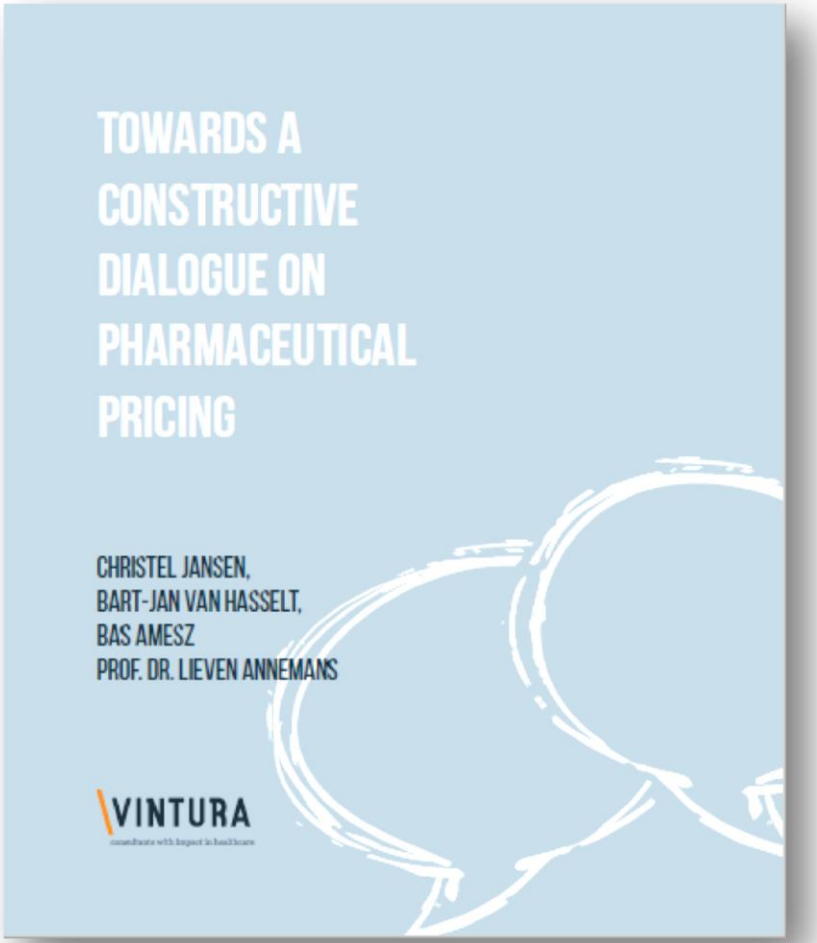
Read more about health economics and pharmaceutical pricing



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