

# Personalized Medicine\*: a health economist's view

Lieven Annemans

Ghent University

Lieven.annemans@ugent.be

\* or stratified , or targeted, or precision, .....



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# Starting point

*We need to stimulate and make available those innovative technologies that*

- *offer a **benefit** to patients and/or the health care system*
- *are cost-effective,*
- *are affordable,*
- *and fill unmet medical needs*

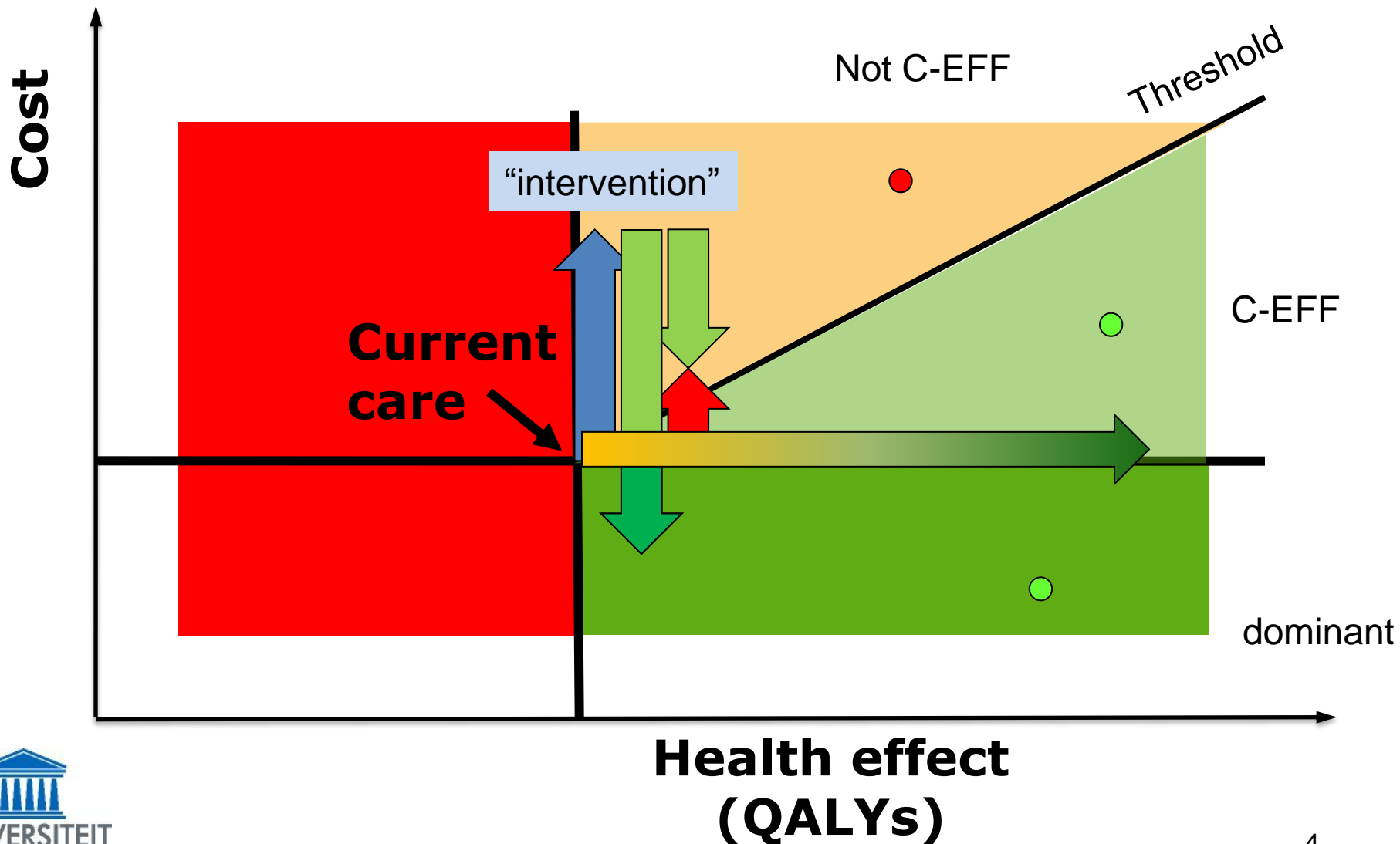
Based on Report of the Belgian EU Presidency,  
endorsed by the EU Council of Ministers of Health in Dec 2010

# Cost-effectiveness

doit être stimulée. L'offre de soins doit faire l'objet d'une évaluation permanente en fonction de critères scientifiquement étayés relatifs à la nécessité, l'efficacité et le rapport coût/efficacité. Le processus de soins doit être piloté et contrôlé d'une façon moderne et informatisée, avec davantage d'attention pour la qualité, tout en maîtrisant la complexité du

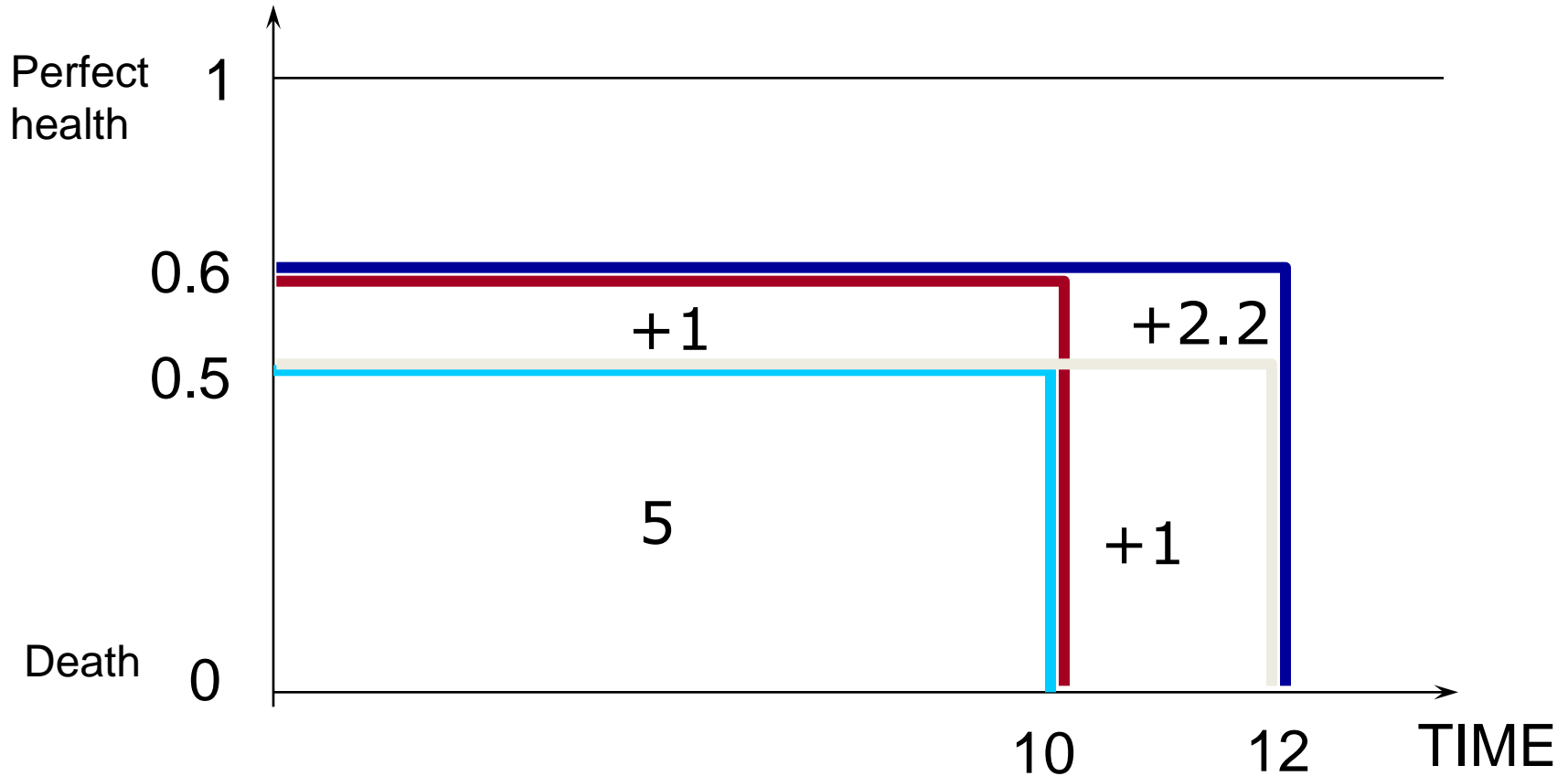
(federal government agreement Oct 2014)

# Cost-effectiveness explained



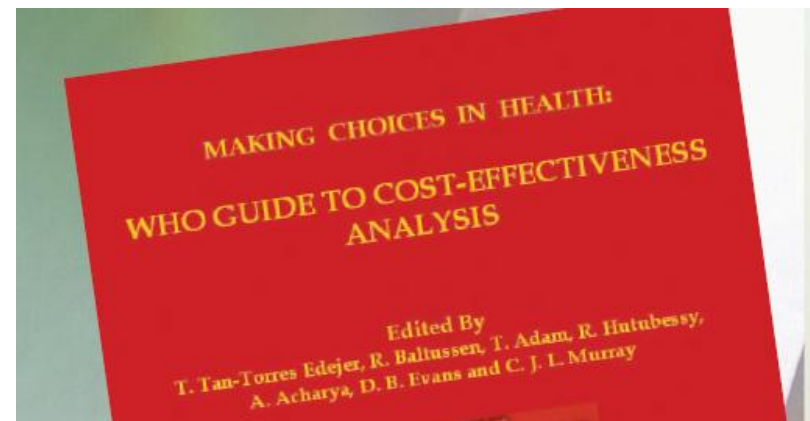
# QALY = Quality Adjusted Life Years

INDEX ('utility level') → Measured via EQ5D or SF36



# PROBLEM: where is the threshold?

- HISTORICAL BENCHMARK +/- 50,000€ per QALY:  
= cost effectiveness of caring for a dialysis patient  
(+/- 4 QALYs gained for an investment of +/- 200,000€)
- Desaigues et al (2007): willingness to pay  
→ €40,000 per Healthy Life Year (for EU25 countries)
- WHO (2003): <1 GDP per capita (e.g. Belgium = +/- €34000) (exceptionally up to 3x GDP per capita)



# Some examples: “league table”

Treatment	Cost per QALY gained (€)
Cardiac rehabilitation and prevention program	dominant
Helpline for suicide prevention	dominant
New anticoagulants for stroke prevention in atrial fibrillation	5,000
Intensive secondary prevention after a heart attack	12,000
New generation drugs in MS	35,000
<b>Kidney dialysis</b>	<b>50,000</b>
Annual mammography for women aged 40-50yrs	70,000
Bevacuzumab in first line advanced ovarian cancer	200,000
Biannual screening for prostate cancer in all men 40-80 yrs	500,000
Annual CT in former heavy smokers to detect lung cancer	1,000,000

# Is personalized medicine the golden duck?



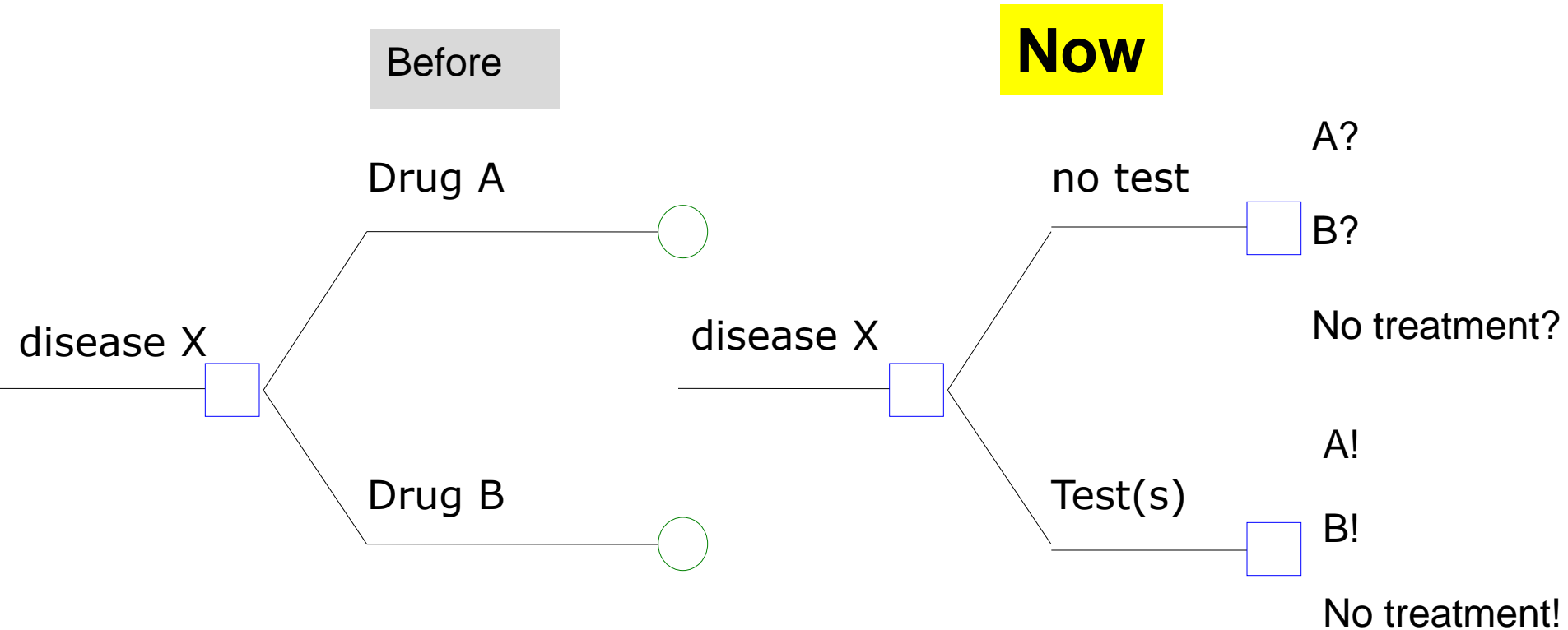


# On first sight, personalized medicine is better for all

- Patients
  - Reduced uncertainty, improved care and less exposure to ineffective treatments
- Physicians
  - More effective options and outcomes for patients
- Regulators
  - Increased efficacy and safety
- Industry
  - Innovative products that offer a clear improvement for patients
- **Payers & policy makers**
  - **More cost-effective use of our healthcare Euros**



# But despite the new paradigm, the same questions need to be addressed



## NEW ELEMENTS

- Cost of test
- Performance of test
- False positives and false negatives
- ....

# Better outcomes not guaranteed!!

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## POLICY PERSPECTIVES

### Challenges in the Development and Reimbursement of Personalized Medicine—Payer and Manufacturer Perspectives and Implications for Health Economics and Outcomes Research: A Report of the ISPOR Personalized Medicine Special Interest Group

Eric Faulkner, MPH<sup>1,2,3,4,\*</sup>, Lieven Annemans, PhD, MSc<sup>5</sup>, Lou Garrison, PhD<sup>6</sup>, Mark Helfand, MD, MPH<sup>7</sup>, Anke-Peggy Holtorf, PhD, MBA<sup>8</sup>, John Hornberger, MD, MS<sup>9,10</sup>, Dyfrig Hughes, PhD, MRPharmS<sup>11</sup>, Tracy Li, PhD<sup>12</sup>, Daniel Malone, PhD<sup>13</sup>, Katherine Payne, PhD<sup>14</sup>, Uwe Siebert, MD, MPH, MSc, ScD<sup>15,16,17</sup>, Adrian Towse, MA<sup>18</sup>, David Veenstra, PhD, PharmD<sup>6</sup>, John Watkins, PharmD, MPH, BCPS<sup>19</sup>, for Personalized Medicine Development and Reimbursement Working Group

# Pitfalls of personalized medicine

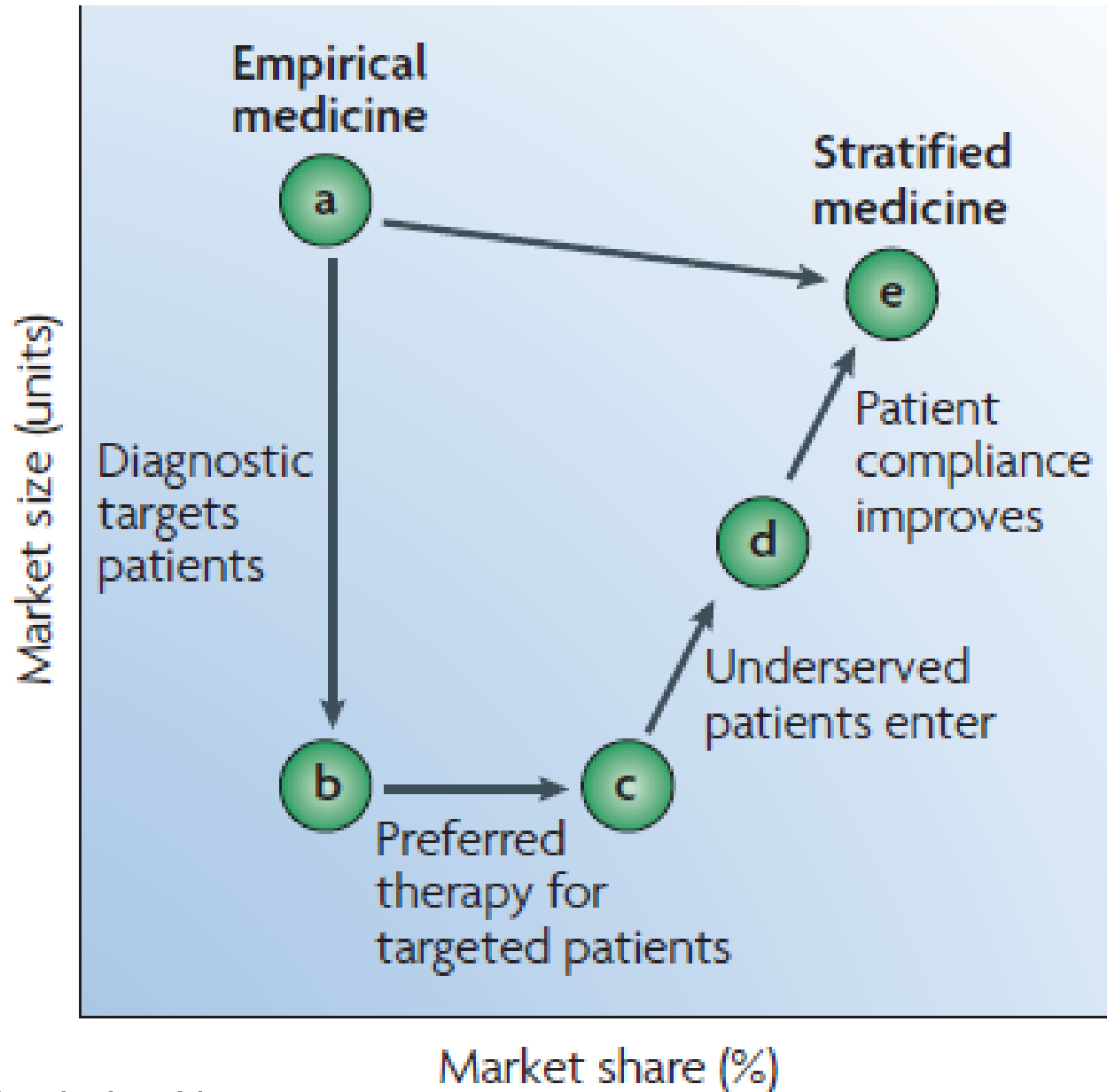
Downside potential / risks



- Additional cost for true and false positive patients
- Expanded patient populations for drugs (e.g., by screening and prevention)
- Increased diagnostics budgets
- Enforcement of privacy safeguards
- Extended patent protection by secondary test-treat product
  - **Consequences of false negatives**

Faulkner et al, 2012, adapted

# improved business for companies promised



“If 100,000 cancer patients will all receive a personalized treatment at 50,000€ the budget impact will be 5 Bln €”  
(J. De Grève – VUB)

# HiScreenDiag – Project

Building a Tool to Evaluate and Improve  
Health Investments in Screening and  
Diagnosis of Disease

Focus on biomarkers as companion to drugs

Lieven Annemans, Fernando Antoñanzas, Cornelis Boersma, Katharina Fischer, Dolores Ibarreta,  
Ian Jacob, Reiner Leidl, Daniele Paci, Katherine Payne, Maarten J. Postma, Roberto Rodriguez,  
Wolf Rogowski, William Sullivan, Dominique Vandijck

“surprising” finding : the current decision processes in the EU are not transparent, fragmented and highly different

Building a Tool to Evaluate and Improve Health Investments in Screening and Diagnosis of Disease

Lieven Annemans, Fernando Antónanzas, Cornelis Boersma, Katharina Fischer, Dolores Ibarreta, Ian Jacob, Reiner Leidl, Daniele Paci, Katherine Payne, Maarten J. Postma, Roberto Rodriguez, Wolf Rogowski, William Sullivan, Dominique Vandijck

- Enormous differences in
  - who triggers the health economic evaluation of tests
  - who participates in the assessment
  - the criteria for assessment
  - the way they are conducted
- *“coverage decisions about biomarkers frequently appear to be made outside of the scope of national decision making bodies, presumably on a local decision making level”*

# HISCREENDIAG Project resulted in a common HTA toolkit

- To be applied for those screening/diagnostics that claim to have an added value
- toolkit built around 3 main elements:
  - Ten criteria for decision-making
  - Quality assessment
    - To assess the quality of the evidence that is submitted for each of the 10 criteria
  - Process of HTA
    - Step by step description of the proces for submitting, assessing and appraising the genetic diagnostic

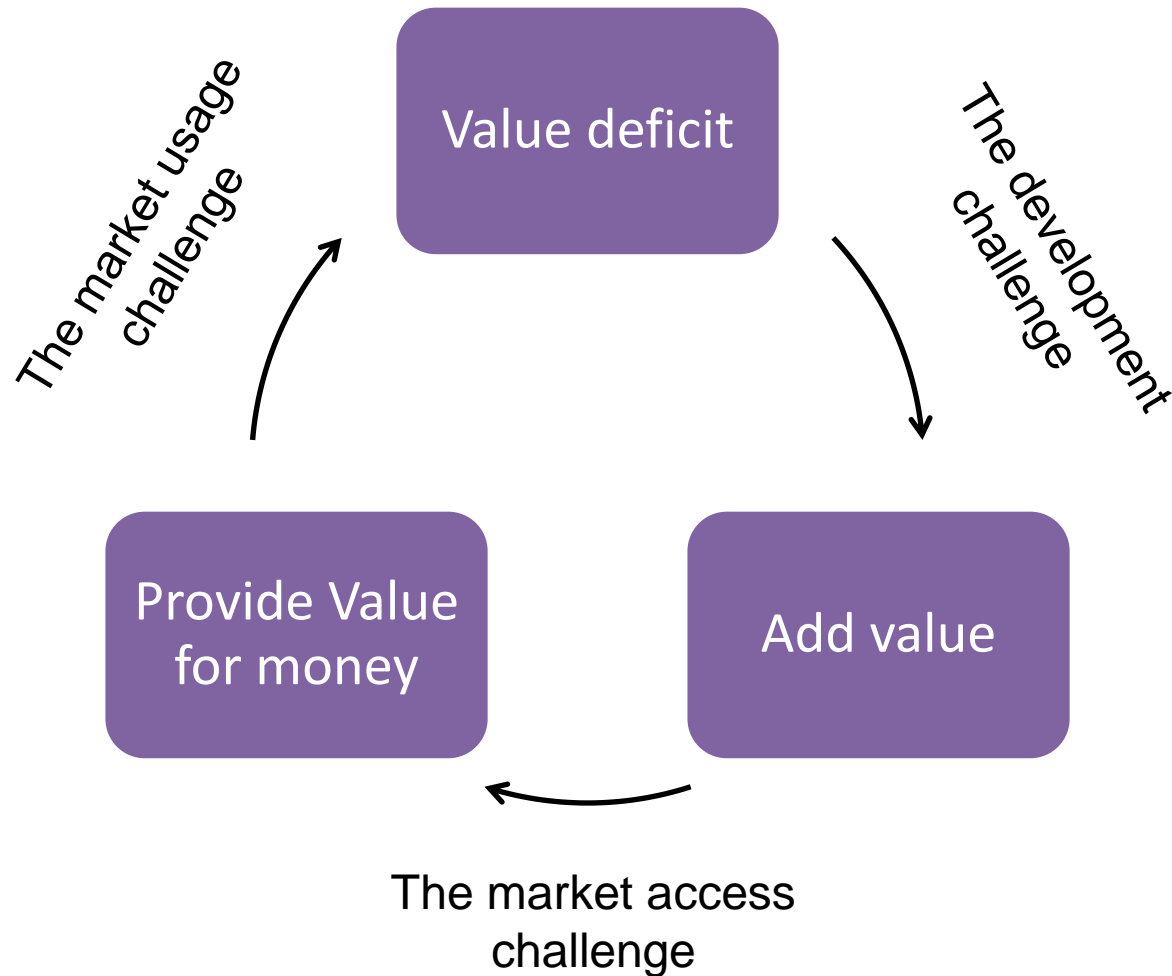




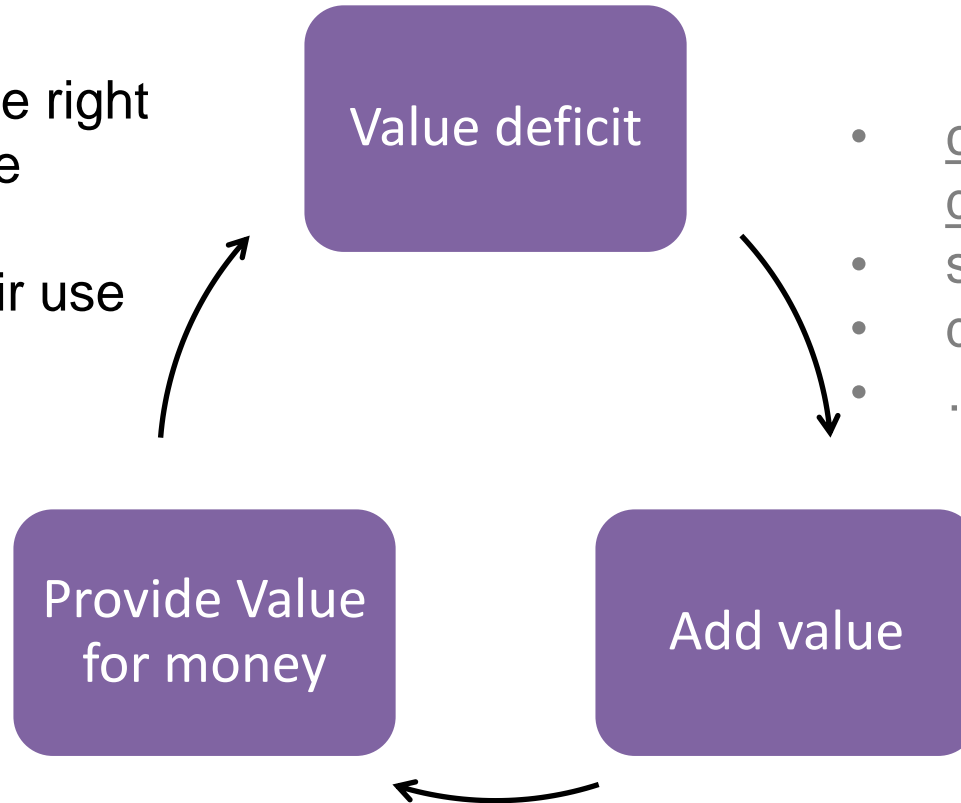
# 10 criteria

1. Current use of the technology (dissemination so far)
2. Epidemiology and management of relevant disease(s)
3. The exact technology and its characteristics
4. Safety/toxicity
5. Accuracy
6. Efficacy and (modelled)effectiveness
7. Costs & economic evaluation
8. Ethical aspects
9. Organisational aspects
10. Psychosocial and legal aspects

# Presenting solutions using the innovation cycle



# Approach to solutions using the innovation cycle



- stimulate the right usage of the innovations
- monitor their use

- creative clinical trial designs
- standardisation
- data sharing
- ...

- Stable, transparent and integrated reimbursement processes & algorithms (not to apply a full HTA each time!)
- harmonisation of criteria for reimbursement
- specific outcomes based agreements
  - Real world evidence

# Personalized Medicine: a health economist's view

**THANK YOU**

Lieven.annemans@ugent.be