Pharma sector challenges and the health system

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Terms of reference (for today's presentation)

- Health systems face challenges in reaching their aims
  - Financial sustainability
  - Access to health technologies
  - Equity in health, in access to health care, and in funding of health expenditures
- Infarmed contributes with its role in the regulation of pharmaceuticals and medical devices: ensuring efficacy, safety, and affordability
- With 30 minutes, my choice of focus is on the pharma sector (only part of Infarmed activities) and on the first two objectives.

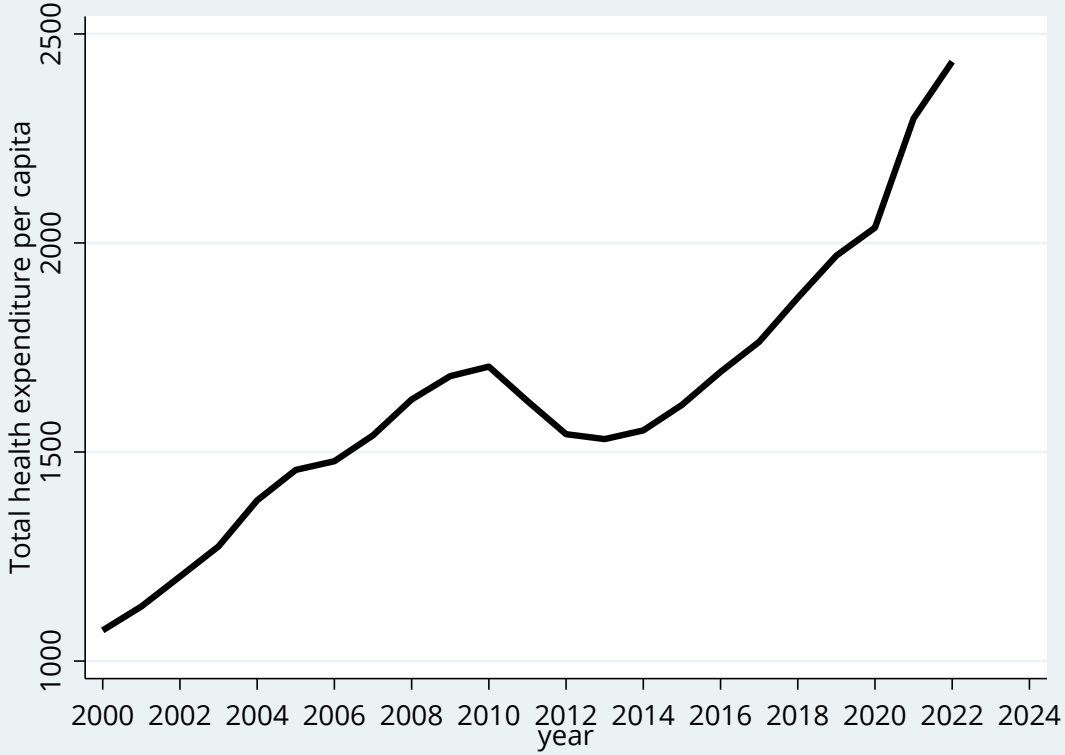
The agenda for today

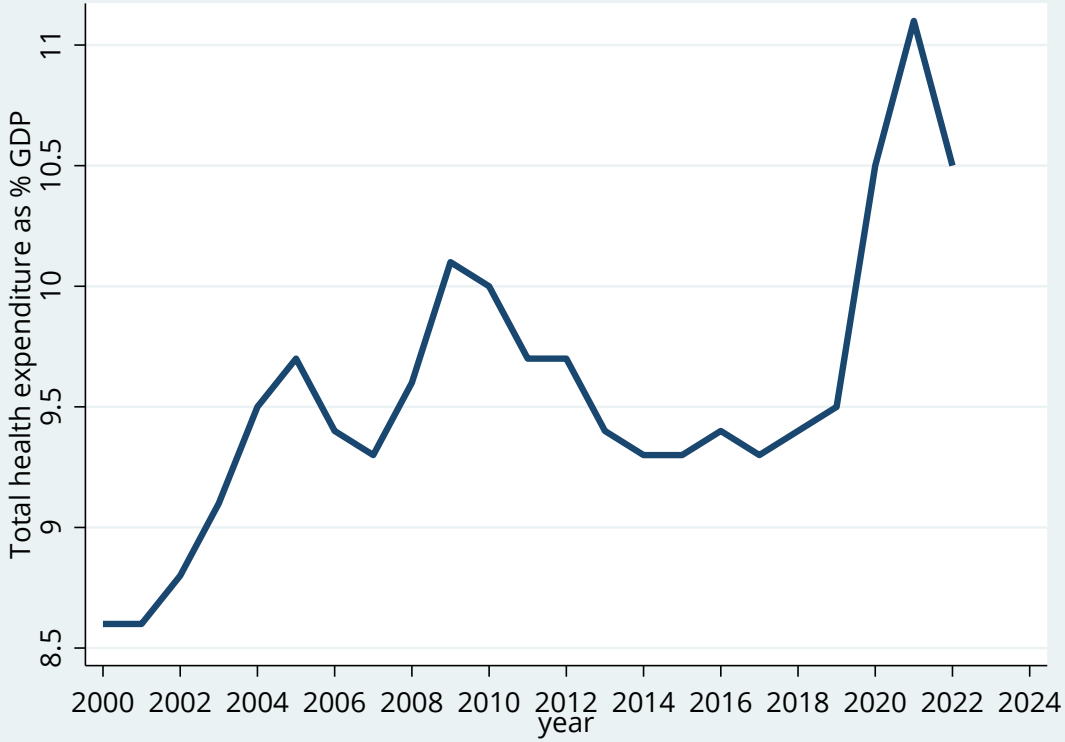
- Current challenges faced by Infarmed (and similar agencies in other countries): balancing cost and access and innovation incentives, dealing with complex new therapies; all in a multi-country setting, at least European Union.
- Key “tension points” of some of the challenges:
  - Role of Health Technology Assessment (HTA)
  - Demands for cost transparency
  - Use of real-world evidence
  - Pricing for combination therapies
  - Advance purchase agreements
  - International cooperation and action across countries
- Several criticisms and controversies are present: slow approval processes, influence of pharma lobby – I will not address those today, leave it for the discussion period (eventually)

- The tension between **access to new products** and **affordability** / financial sustainability of payers (of health systems) is not a new one – either in Portugal or elsewhere in modern health systems
- Why is it a permanent tension? (permanent reassessment, permanent search for new solutions)
  - Access to new products involves R&D – this requires a dynamic (over time) perspective of incentives (say, higher prices) to engage in such activities (intensity and direction)
  - Access to new products involves prices – this takes a static efficiency view – with health insurance (public or private) demanding lower prices

Sustainability of health systems

- **What does it mean**: ability to provide quality health services while remaining financially viable and responsive to changing needs of the population.
- In a National Health Service context – public spending associated with health care does not derail public finances (public debt is not explosive, as a criterion, for example)
- **What does it imply**: all components of public health spending must grow in a way compatible with available funds / fiscal revenues

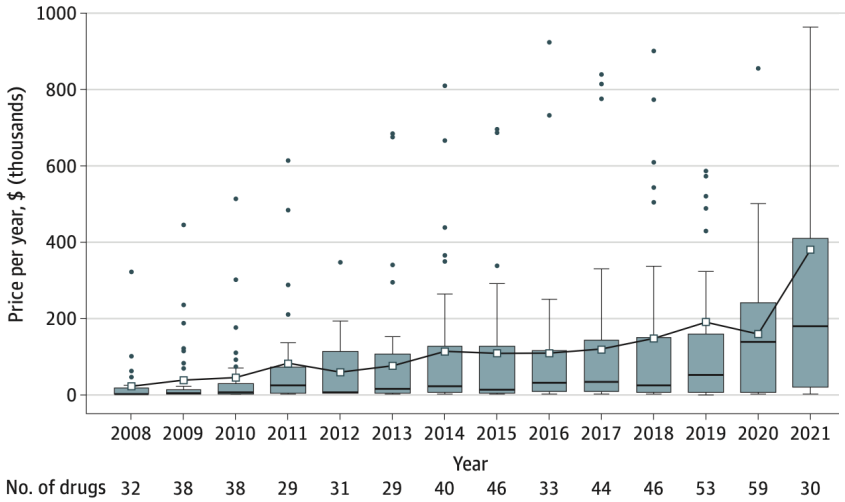




- Health expenditures have grown in the past decade (even after removal of pandemic year 2020)
- Pressures to lower spending will return
- Are pharmaceutical expenditures growing at a faster pace?

Why the prices of pharmaceuticals are on the spotlight? (international context)

Figure. Prices for Newly Marketed Drugs, 2008-2021



Median launch prices increased from $2115 per year (IQR, $928-$17 866) per year in 2008 to $180 007 (IQR, $20 236-409 732) per year in 2021 (**Figure**). The proportion of drugs priced at $150 000 per year or more was 9% (18/197) in 2008-2013 and 47% (42/89) in 2020-2021. Unadjusted mean launch prices increased exponentially by 20.4% per year (95% CI, 15.3%-25.8% per year). Adjusting for drug characteristics, mean prices increased exponentially by 13.0% per year (95% CI, 9.4%-16.7% per year). Most drug characteristics were indepen-

Research Letter FREE

June 7, 2022

Trends in Prescription Drug Launch Prices, 2008-2021

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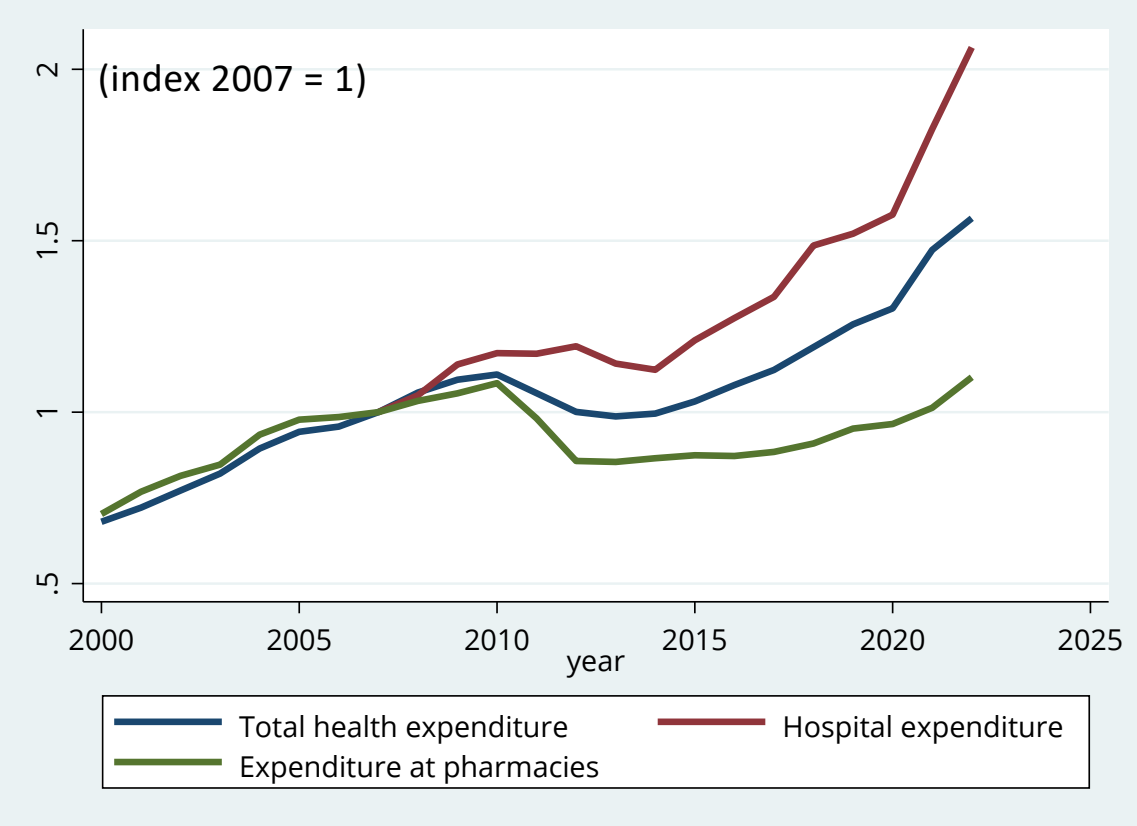
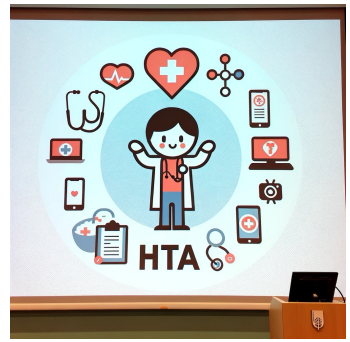
Why the prices of pharmaceuticals are on the spotlight? (Portugal)



Institutional response to expenditure pressure:

- HTA development + rules related to ICER + value-based pricing - how much part of the problem and how much part of the solution?
- Institutional mechanisms of approval, relying on HTA providing estimatives of value that support for very high prices that create too much transfer of value
- “Too much” has the sense that lower prices would still lead to the desired effort of R&D

The HTA role

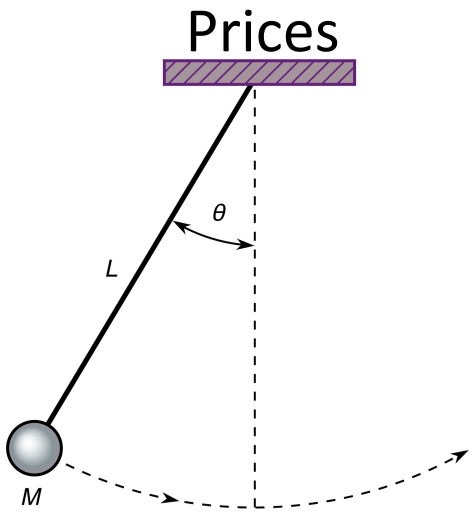


- HTA contributes to sustainability: provides evidence to inform policy and decision making, in a structured way
- It is an input to cost-effectiveness analysis; only technologies that are proven to be effective and safe are adopted
- HTA also addresses ethical and equity considerations (fair and equitable access to health care can be defined using HTA results, impact of technology on population groups)
- It creates the necessary basis to define “value” of a new product

Building on HTA

- Value-based decisions and value-based pricing - the use of information from HTA is a choice, how much it has become part of the problem (of sustainability / affordability of new products)?
- Cost transparency requests - how much part of the problem and how much part of the solution?
- What about the challenges of combination therapies?
- Real-word evidence - how much part of the problem and how much part of the solution?
- Will come to these again below

Pricing dilemmas: **market power** and **uncertainty**



High prices: good for investment

Low prices: good for affordability

Objectives and instruments

- Different problems may require different solutions
- Two major problems:
  - Returns to innovation as incentive but also as exercise of market power – requires two different roles for prices, and affects affordability – **return from investment** (allow for market power to recover investment) and **guide R&D effort** (invest more where the expected return is higher)
  - **Uncertainty about the value of the product** at the moment of launch – how a new product will work in the real population may be different than how it works in clinical trials. Companies may know more about the product than payers at the moment of decision about prices and reimbursement.

When solving a problem creates another one

- HTA – it is an instrument to gather information about the new products
- Using ICER on top of HTA – allows for prices close to threshold of value
- Value-based pricing using results from HTA, and setting price = value – in solving the problem of incentives, it may exhacerbate the problem of market power
- Important observation – the incentives to guide for R&D areas of interest are linked to relative prices of new products with different value – this is the part to retain from value-based pricing principles

When solving a problem creates another one

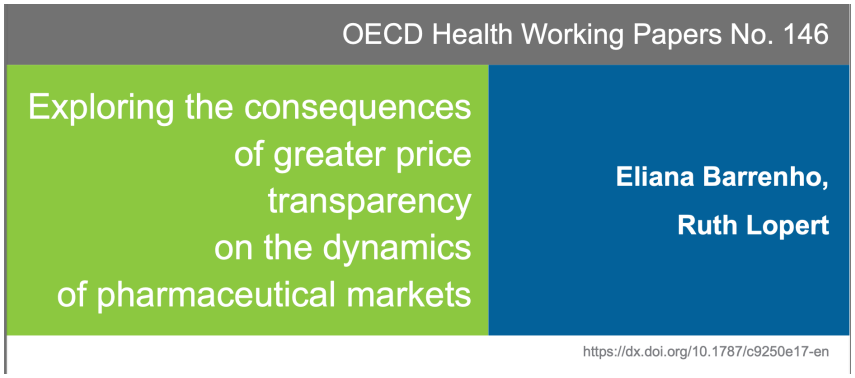
- Price = value increases problems with affordability (transfers all value to innovators), it alleviates problems with returns on investiment and with guiding R&D to more valuable needs
- It does not solve the uncertainty about the value / quality of the product
- Real-world evidence addresses uncertainy about the value (contingent prices, with distinct credibility regarding increase vs decrease of prices in the future upon information), does not address the market power problem (of too high prices).

When solving a problem creates another one

SEVENTY-SECOND WORLD HEALTH ASSEMBLY **WHA72.8**

Agenda item 11.7 **28 May 2019**

Improving the transparency of markets for medicines, vaccines, and other health products[1]



"Lack of transparency of research costs or return on investment can influence decisions that impact affordability and ultimately access for patients." (p. 19)

When solving a problem creates another one

- Cost transparency regarding R&D costs provide information on market power exercise, but create “temptation” for cost-based prices
- The (positive) role of deterrence of very high prices is to be balanced against potential drawbacks
- Cost-based prices may easily give wrong signals for investment (invest more in areas with higher cost, even if of smaller impact) (invest more to have more costs, as this will bring higher prices).
- It does not necessarily to higher-value innovation, it is very likely to lead to more costly innovation
- A key question is: how to best use the information from cost transparency efforts?

(note: cost transparency is distinct from the sister issue of price transparency)

Combination therapy and value-based pricing

- New thinking and new ideas are required, as different challenges are emerging and do not have an obvious answer from existing tools
- Example 1: Combination therapy – puts together product *A* and product *B*.
- HTA has defined value V_A for product *A* and V_B for product *B*.
- Combination therapy holds $V_{A+B} < V_B + V_A$
- Value-based pricing (in the formulation price = value) for the combination gives how much for each product?
- Different proposals will lead to different incentives and returns to R&D

Main challenge: development of new tools

- Proposal 1: getting the proportion $V_i/(V_A+V_B)$, $i=A, B$
- Proposal 2: if B is the ”backbone”, then $p_B= V_B$, $p_A = V_{A+B} – V_B$
- Proposal 3: other rule?
- Each rule will lead to different results of prices but also of initial investment given the expected prices.
- There is a need to define generally accepted principles and then take a rule that respects those principles (current discussions provide examples, not a general rule)

Advance purchase agreements

- Example 2: Different ways to guide investment decisions – advance purchase agreements
- It can be another way to guide investment for identified unmet needs, it may also lead to higher investment as it changes the risk associated with investment (influence on market dynamics).
- It is not without problems – the product may not materialize (R&D is risky)
- It requires coordination with a set of countries significant enough to raise a payment that matters for R&D – brings in the problems of joint purchases (what to buy, added complexity to negotiations)
- Does not address market power concerns.

Real-world evidence

- Real-world evidence (RWE): many sources (electronic health records, insurance claims, patient registries, etc).
- The quality and completeness of these data sources can vary greatly, affecting the reliability of the evidence derived from them, on which we may want to condition future price changes
- Its use is likely to require standardized methods for data collection and analysis – are we doing it?
- RWE and patients with rare diseases or of underrepresented groups
- RWE and observational studies potential bias
- Ethical and Privacy Concerns – consent, anonymity of data, cybersecurity, etc.
- Political economy: credibility of conditional changes and litigation over the evidence provided
- Deals with the uncertainty challenge, not the market power challenge

Road ahead? My conjecture…

- Use of HTA as first step
- Engage in negotiations – strike balance between division of value and rewarding better innovations – affordability matters
- Look for new ways to guide and pay for innovation addressing identified unmet needs
- Use conditional payments for uncertainty whenever credible
- Learn from experience, revise and fine tune
- Collaborate internationally for best results

Infarmed at 31: 5 challenges

- Revisiting old rules that fail to address emerging issues
- Understanding market power and market uncertainty as separate issues in the pricing and reimbursement decisions
- Developing innovative approaches for new challenges, such as combination therapies
- Addressing demands for action cautiously to avoid unintended consequences, particularly in cost and price transparency
- Participating (and leading) in multi-country coordination settings