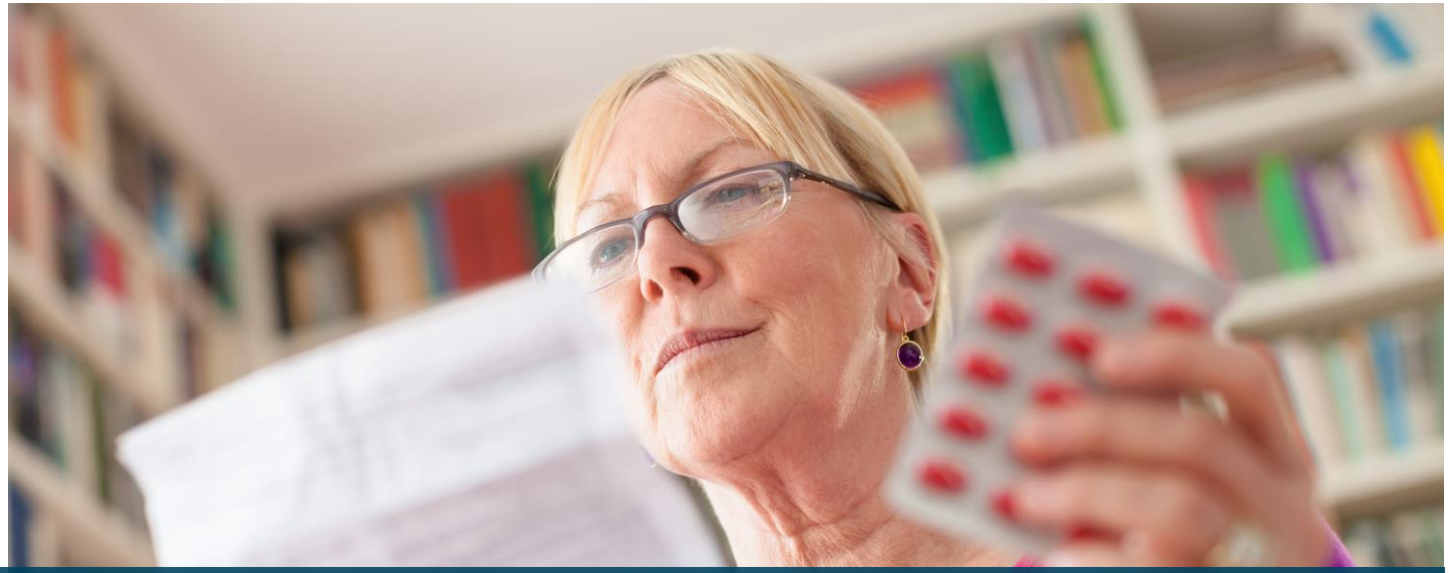

Challenges on access to medicines in Europe and policy solutions

Ancel.la Santos, Senior Policy Advisor, Health Action International
22 January 2019

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HAI HEALTH ACTION INTERNATIONAL

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- Jaume Vidal, Policy Advisor EU projects

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Health Action International (HAI)

- Non-profit organisation
- Vision: Access to safe, effective, affordable and quality-assured medicines for everyone, everywhere
- Advocacy, policy, research
- Global network – HAI Europe Association
- Funding from governments and foundations
- No funding from the pharmaceutical industry

http://haiweb.org/

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HAI's European projects

Goal

- Advance policies for improved access to medicines and rational medicines use in the European Union

Challenge

- High prices of newly marketed medicines increasingly threaten:
  - ➢ Affordability
  - ➢ Sustainability of healthcare systems

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Medicines price hikes

FINANCIAL TIMES

US COMPANIES TECH MARKETS GRAPHICS OPINION WORK & CAREERS LIFE & ARTS HOW TO SPEND IT

Make informed decisions. Try the FT. Choose your FT trial

prices

anofi and Novo Nordisk raise US nsulin prices

Celgene deal a 'catalyst' for pharma takeovers

Pharma companies put faith in AI for breakthroughs

Drug prices + Add to myFT

Dutch doctors fight pharma company's 500-fold drug price rise

Regulator urged to probe Leadiant Biosciences' alleged abuse of market power

Andrew Jack SEPTEMBER 2, 2018 16

A group of Dutch doctors and health advocates are taking action against an Italian pharmaceutical company, which has pushed through a 500-fold price increase of a life-saving drug.

The Dutch Pharmaceutical Accountability Foundation, a non-profit group set up to tackle high drug prices, is set to file a claim this week with The Netherlands' Authority for Consumers and Markets to probe alleged abuse of

https://www.dutchnews.nl/news/2019/01/minister-to-talk-to-novartis-after-five-fold-cancer-drug-price-hike/

https://www.ft.com/content/e394d54e-ae16-11e8-8d14-6f049d06439c

The case of Lutetium-octreotaat:

- Cancer drug developed by researchers from the Erasmus Medical Center Rotterdam
- Produced at the hospital pharmacy
- Novartis starts marketing it as an orphan drug
- Five-fold price increase

The CDCA case (chenodeoxycholic acid):

- Available for long time at a low cost to treat gallstones
- In 2017 Leadiant gets approval to market it as an orphan drug for CTX
- 500-fold price hike
- Pharmaceutical Accountability Foundation files a complaint

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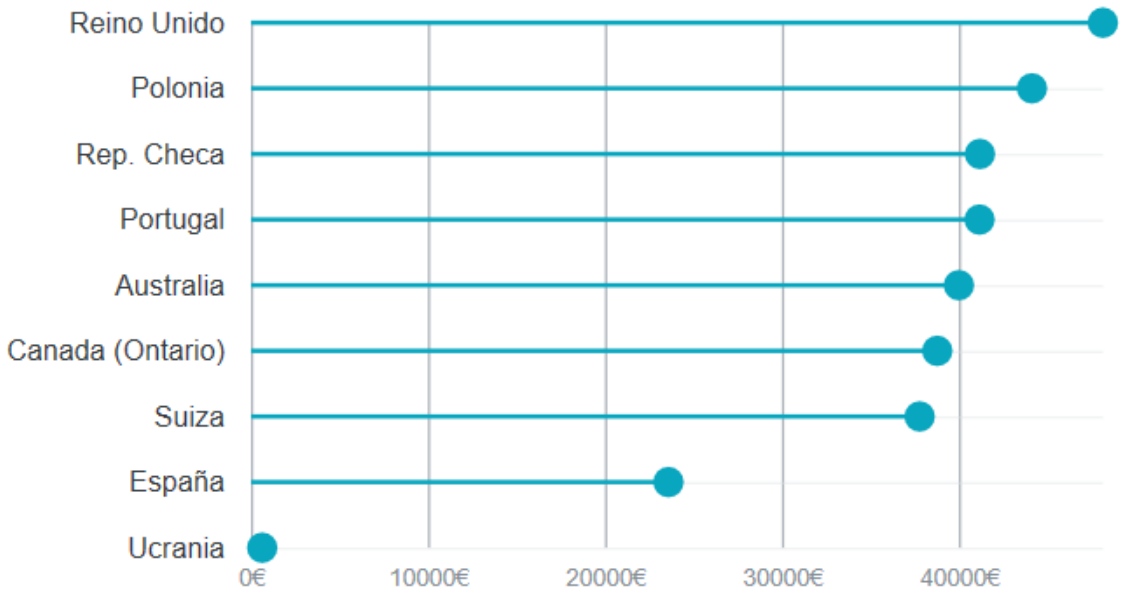
Sovaldi: “The $1.000 Pill”

Official prices of a 12 week treatment



Research by Civio, published in Oct 2017 https://civio.es/medicamentalia/2017/10/25/sovaldi-4-anos-despues-de-la-revolucion-contra-la-hepatitis-c-cuanto-cuestan-los-nuevos-farmacos/

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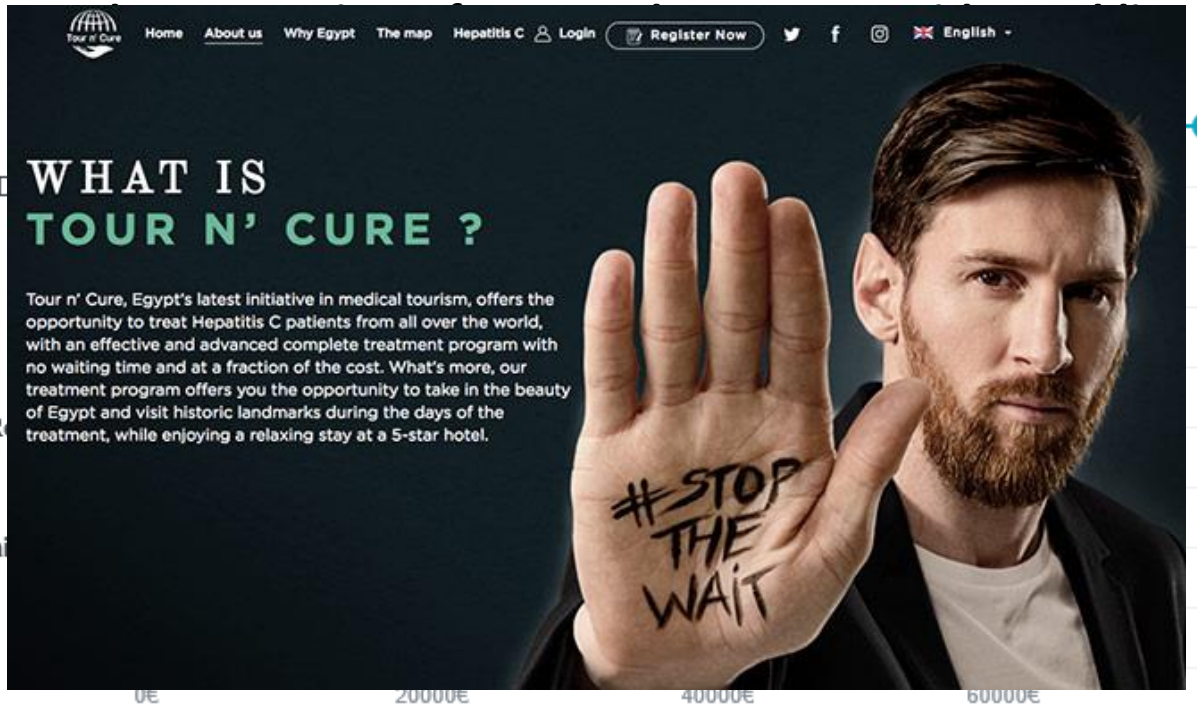
Unaffordable prices



Research by Civio, published in Oct 2017 https://civio.es/medicamentalia/2017/10/25/sovaldi-4-anos-despues-de-la-revolucion-contra-la-hepatitis-c-cuanto-cuestan-los-nuevos-farmacos/

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High prices for new cancer treatments

´´The launch prices of drugs for cancer and rare diseases are rising, sometimes without a commensurate increase in health benefits for patients.´´

OECD (2017). New Health Technologies Managing Access, Value and Sustainability

- What relation between R&D costs and prices?
- Innovation in the benefit of patients?

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R&D spending, how much?

Independent estimates on R&D spending on ten cancer drugs:

- Median cost of drug development was $648 million ($757.4 million for a 7% opportunity costs)
- While the median revenue since approval was $1658.4 million (range, 204.1 $million to $22 275 million)
- With a median of 4 years since approval, the *total* revenue from sales was $67 billion…

  … compared with total R&D spending of $7.2 billion ($9.1 billion, including 7% opportunity costs)

Prasad V, Mailankody S (2017). Research and Development Spending to Bring a Single Cancer Drug to Market and Revenues After Approval, *JAMA Intern Med*, 177(11)

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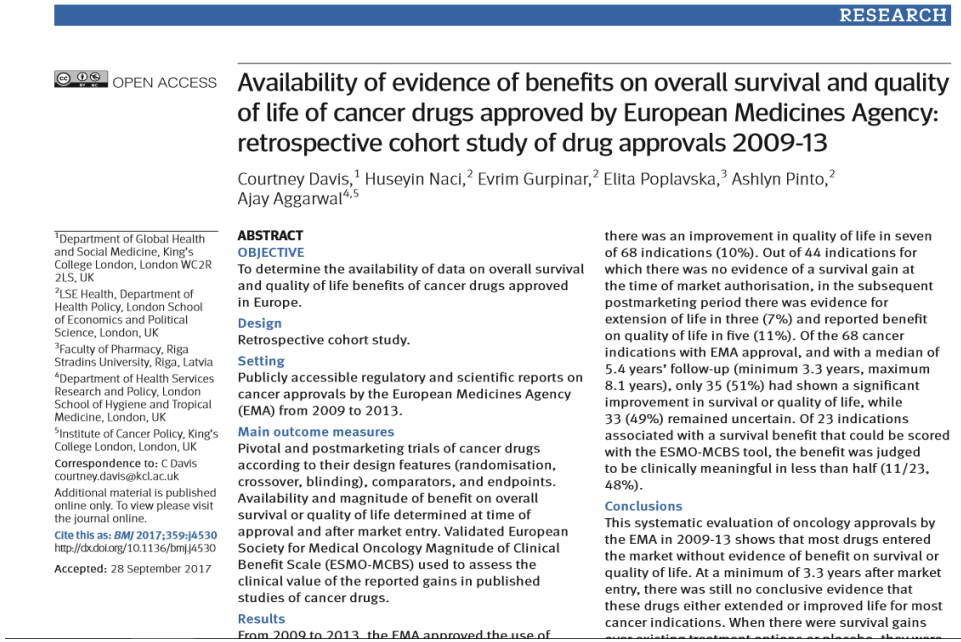
Evidence of benefit (or lack of)

- Published study at BMJ on cancer indications approved by EMA 2009-2013
- Research supported by HAI
- Results: Of the 68 cancer indications with EMA approval, and with a median of 5.4 years' follow-up, only 35 (51%) had shown improvement in survival or quality of life, while 33 (49%) remained uncertain
- Survival gains over existing treatment options or placebo were often marginal



RESEARCH

OPEN ACCESS

Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13

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Additional material is published online only. To view please visit the journal online.
Cite this as: *BMJ* 2017;359:j4530 http://dx.doi.org/10.1136/bmj.j4530
Accepted: 28 September 2017

ABSTRACT

OBJECTIVE
To determine the availability of data on overall survival and quality of life benefits of cancer drugs approved in Europe.

Design
Retrospective cohort study.

Setting
Publicly accessible regulatory and scientific reports on cancer approvals by the European Medicines Agency (EMA) from 2009 to 2013.

Main outcome measures
Pivotal and postmarketing trials of cancer drugs according to their design features (randomisation, crossover, blinding), comparators, and endpoints. Availability and magnitude of benefit on overall survival or quality of life determined at time of approval and after market entry. Validated European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS) used to assess the clinical value of the reported gains in published studies of cancer drugs.

Results
From 2009 to 2013, the EMA approved the use of

there was an improvement in quality of life in seven of 68 indications (10%). Out of 44 indications for which there was no evidence of a survival gain at the time of market authorisation, in the subsequent postmarketing period there was evidence for extension of life in three (7%) and reported benefit on quality of life in five (11%). Of the 68 cancer indications with EMA approval, and with a median of 5.4 years' follow-up (minimum 3.3 years, maximum 8.1 years), only 35 (51%) had shown a significant improvement in survival or quality of life, while 33 (49%) remained uncertain. Of 23 indications associated with a survival benefit that could be scored with the ESMO-MCBS tool, the benefit was judged to be clinically meaningful in less than half (11/23, 48%).

Conclusions
This systematic evaluation of oncology approvals by the EMA in 2009-13 shows that most drugs entered the market without evidence of benefit on survival or quality of life. At a minimum of 3.3 years after market entry, there was still no conclusive evidence that these drugs either extended or improved life for most cancer indications. When there were survival gains

https://www.bmj.com/content/359/bmj.j4530

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Measures to improve affordability & sustainability of health systems

- Health technology assessment
- Joint price negotiation
- Transparency of R&D costs and medicines prices
- Pro-public health innovation framework
- ‘Sunshine act’ legislation

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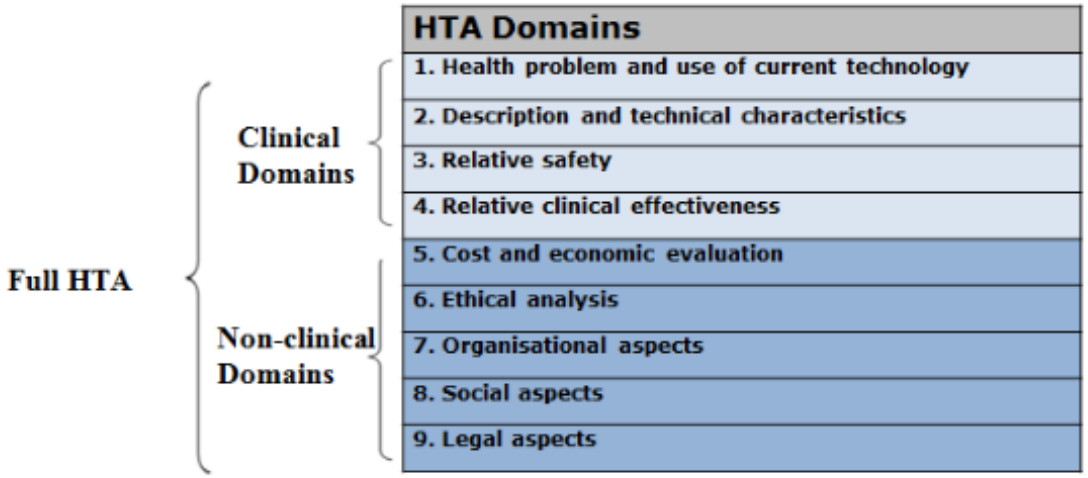
Health Technology Assessment

- What added value of a new health technology compared to existing ones?

Based on EUnetHTA Core Model



https://ec.europa.eu/health/sites/health/files/technology_assessment/docs/2018_ia_final_en.pdf

- Informs price and reimbursement decisions

EUnetHTA

- Network of 80+ organisations in 30 European countries
- Joint Action 3 (2016-2020)
- Joint assessments, early dialogues, horizon scanning

Challenges:

Low uptake joint outputs
Sustainability issues

- HTA Network
- HTA Network Stakeholder Pool (HAI, EFA)

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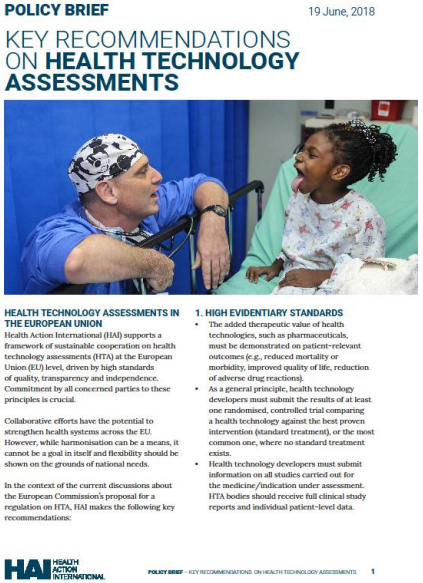
EU Regulation on HTA

- 2018 Commission proposal for a Regulation on HTA
  - Mandatory joint clinical assessments
  - Joint scientific consultations
  - Horizon scanning
  - Voluntary cooperation in non-clinical HTA domains

- Important principles for HAI:
  - High quality assessments
  - Flexibility
  - Transparency
  - Independence



POLICY BRIEF 19 June, 2018

KEY RECOMMENDATIONS ON **HEALTH TECHNOLOGY ASSESSMENTS**

HEALTH TECHNOLOGY ASSESSMENTS IN THE EUROPEAN UNION

Health Action International (HAI) supports a framework of sustainable cooperation on health technology assessments (HTA) at the European Union (EU) level, driven by high standards of quality, transparency and independence. Commitment by all concerned parties to these principles is crucial.

Collaborative efforts have the potential to strengthen health systems across the EU. However, while harmonisation can be a means, it cannot be a goal in itself and flexibility should be shown on the grounds of national needs.

In the context of the current discussions about the European Commission's proposal for a regulation on HTA, HAI makes the following key recommendations:

1. HIGH EVIDENTIARY STANDARDS

- The added therapeutic value of health technologies, such as pharmaceuticals, must be demonstrated on patient-relevant outcomes (e.g., reduced mortality or morbidity, improved quality of life, reduction of adverse drug reactions).
- As a general principle, health technology developers must submit the results of at least one randomised, controlled trial comparing a health technology against the best proven intervention (standard treatment), or the most common one, where no standard treatment exists.
- Health technology developers must submit information on all studies carried out for the medicine/indication under assessment. HTA bodies should receive full clinical study reports and individual patient-level data.

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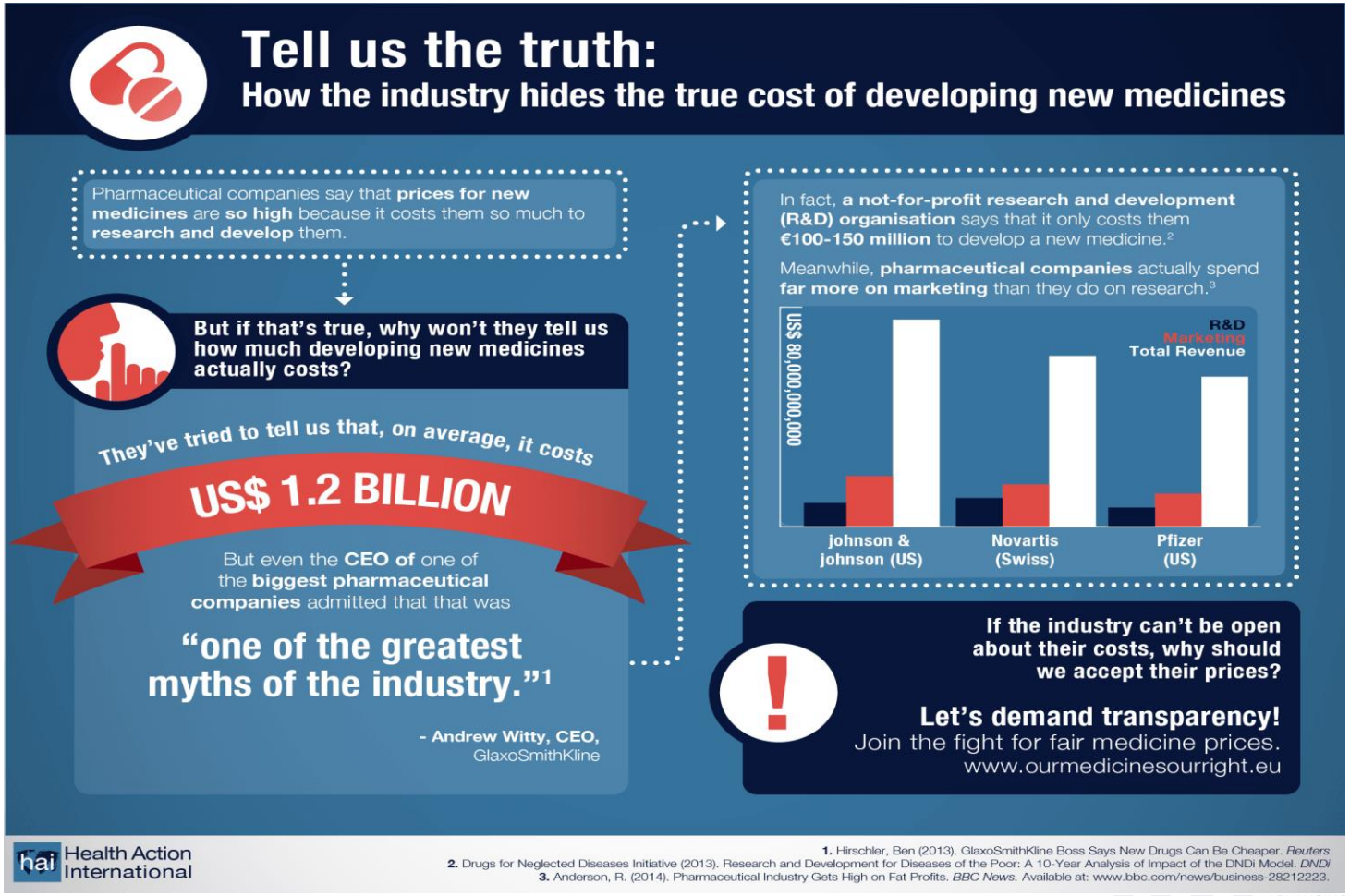
Joint price negotiation

- Increased bargaining power
- Beneluxa initiative: Belgium, The Netherlands, Luxembourg, Austria, Ireland
  - Orkambi
  - Spinraza
- Other less advanced initiatives (e.g. Valletta Declaration, Visegrad group)

Transparency
R&D costs and medicines prices



https://www.ourmedicinesourright.eu/take_action

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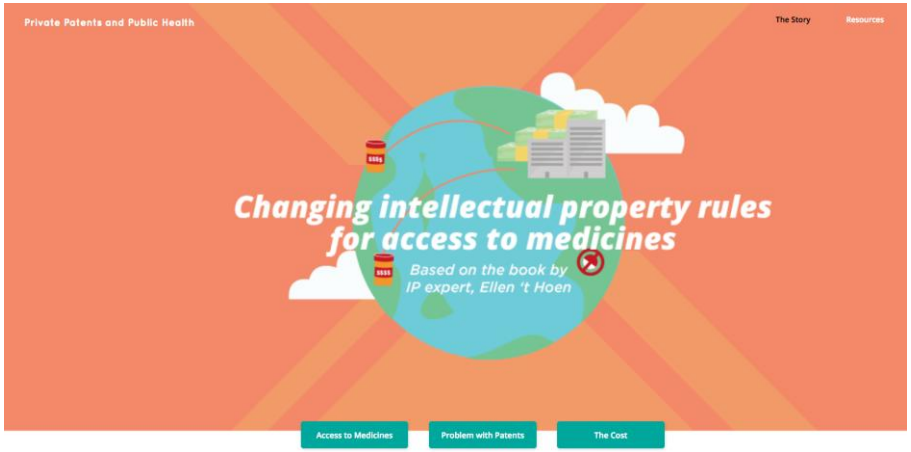
Pro-public health innovation framework

- Public health sensitive IP rules
- Full use of TRIPS flexibilities
- Conditionalities to publicly funded R&D (Horizon 2020 & FP9)



http://accesstomedicines.org/

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‘Sunshine act’ legislation

- Industry payments to physicians associated with higher rates of brand-name drug prescription and prescription costs (1, 2)
- Transparency of financial relationships can contribute to:
  - Better informed decisions about HCPs and treatment
  - Lower healthcare costs
- EU countries with legislated transparency: Belgium, Denmark, Greece, France, Latvia, Portugal, Romania, Slovakia

1. Jones RG, Ornetein C (2016). Matching Industry payments to Medicare prescribing patterns: an analyisis. Pro Publica.

2. Perlis RH, Perlis CS (2016) Physician Payments from Industry Are Associated with Greater Medicare Part D Prescribing Costs. PLoS ONE, 11(5): e0155474. doi:10.1371/ journal.pone.0155474

Thank you!

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