THE GRADUATE INSTITUTE GENEVA

INSTITUT DE HAUTES ÉTUDES INTERNATIONALES ET DU DÉVELOPPEMENT
GRADUATE INSTITUTE OF INTERNATIONAL AND DEVELOPMENT STUDIES

# FAIR PRICING IN THEORY AND PRACTICE: TRANSPARENCY, GOVERNANCE & POLITICAL WILL

## 4TH PHARMACEUTICAL PRICING AND REIMBURSEMENT INFORMATION (PPRI) CONFERENCE
## MEDICINES ACCESS CHALLENGE
## THE VALUE OF PRICING AND REIMBURSEMENT POLICIES

**VIENNA, AUSTRIA**
**24 OCTOBER 2019**

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# DECLARATION OF INTERESTS



1. Travel paid by conference organizers (thank you!)

2. Salary paid by Graduate Institute

3. Have received research grants and consulting fees from governments, intergovernmental organizations, and/or non-governmental organizations

4. Have not received research grant or consulting fees from pharmaceutical industry

# OVERVIEW



1. Fair pricing in theory
   - → Case study 1: Cystic fibrosis medicines
   - → Thinking outside the box
   - → Simplified model of fair pricing
2. Fair pricing in practice:
   - → Transparency, governance, and political will
   - → Calibrating incentives and prices
   - → Case study 2: Hepatitis C treatment in Australia
3. 3 Conclusions

# FAIR PRICING IN THEORY: WHAT IS FAIR? TO WHOM?

# CASE STUDY 1: CYSTIC FIBROSIS DRUGS



- Trikafta was FDA approved Tuesday (22 Oct 2019)
- Development history:
  - 1989: CF gene mutation identified by publicly-funded research
  - 2000: non-profit Cystic Fibrosis Foundation grants Aurora Biosciences $47m for drug discovery
  - 2001: Vertex Pharmaceuticals acquires Aurora
    - 2013: ivacaftor (Kalydeco)
    - 2015: ivacaftor + lumacaftor (Orkambi)
    - 2018: ivacaftor + tezacaftor (Symdeko)
    - 2019: ivacaftor + tezacaftor + elexacaftor (Trikafta)
  - Trikafta: 3 years from synthesis to approval
  - 2 clinical trials: 24 & 4 weeks; total 510 patients
  - US FDA: Priority Review, Fast Track, Breakthrough Therapy, Orphan drug designation, Priority Review Voucher


Sources: https://www.statnews.com/2019/10/23/we-conquered-a-disease-how-vertex-delivered-a-transformative-medicine-for-cystic-fibrosis/, https://www.fda.gov/news-events/press-announcements/fda-approves-new-breakthrough-therapy-cystic-fibrosis, https://www.businesswire.com/news/home/20191021005792/en/ADDING%C2%A0MULTIMEDIA-FDA-Approves-TRIKAFTA-elexacaftortezacaftorivacaftor-ivacaftor-Treat

# CASE STUDY 1: CYSTIC FIBROSIS DRUGS



- Market:
  - 70,000-100,000 globally
  - From 6% to 90% cystic fibrosis patients now treatment eligible
  - Vertex 2019 revenue: $3.7 billion
  - Projected 2024 revenue: $8 billion
  - US list price $311,000


Is this a fair price?

YES / NO / MAYBE

Sources: https://www.statnews.com/2019/10/23/we-conquered-a-disease-how-vertex-delivered-a-transformative-medicine-for-cystic-fibrosis/, https://www.fda.gov/news-events/press-announcements/fda-approves-new-breakthrough-therapy-cystic-fibrosis, https://www.businesswire.com/news/home/20191021005792/en/ADDING%C2%A0MULTIMEDIA-FDA-Approves-TRIKAFTA-elexacaftortezacaftorivacaftor-ivacaftor-Treat

# FAIRNESS TO SELLERS AND BUYERS
# A SIMPLIFIED MODEL

**Sellers:**

Small and large developers, manufacturers, distributors

- Cost of R&D
- Cost of manufacturing and distribution
- Other related costs (e.g. registration, administration, pharmacovigilance)
- Fair profit

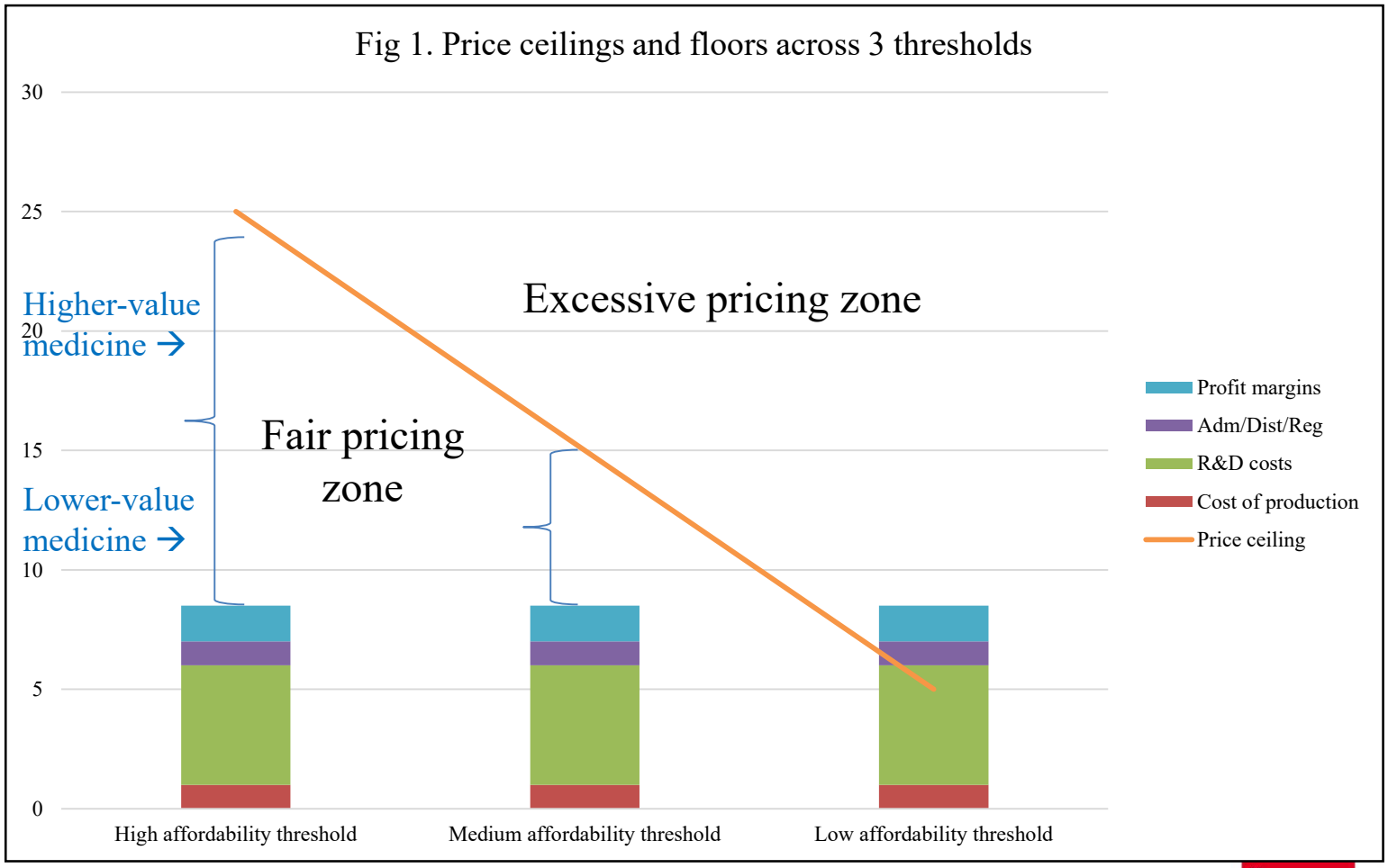
**Buyers:**

Payers, insurers, households, patients

- Present and future affordability (binding constraint)
- Value to the individual and health system
- Security of supply

Source: Moon et al. (in press) Defining the concept of fair pricing for medicines.

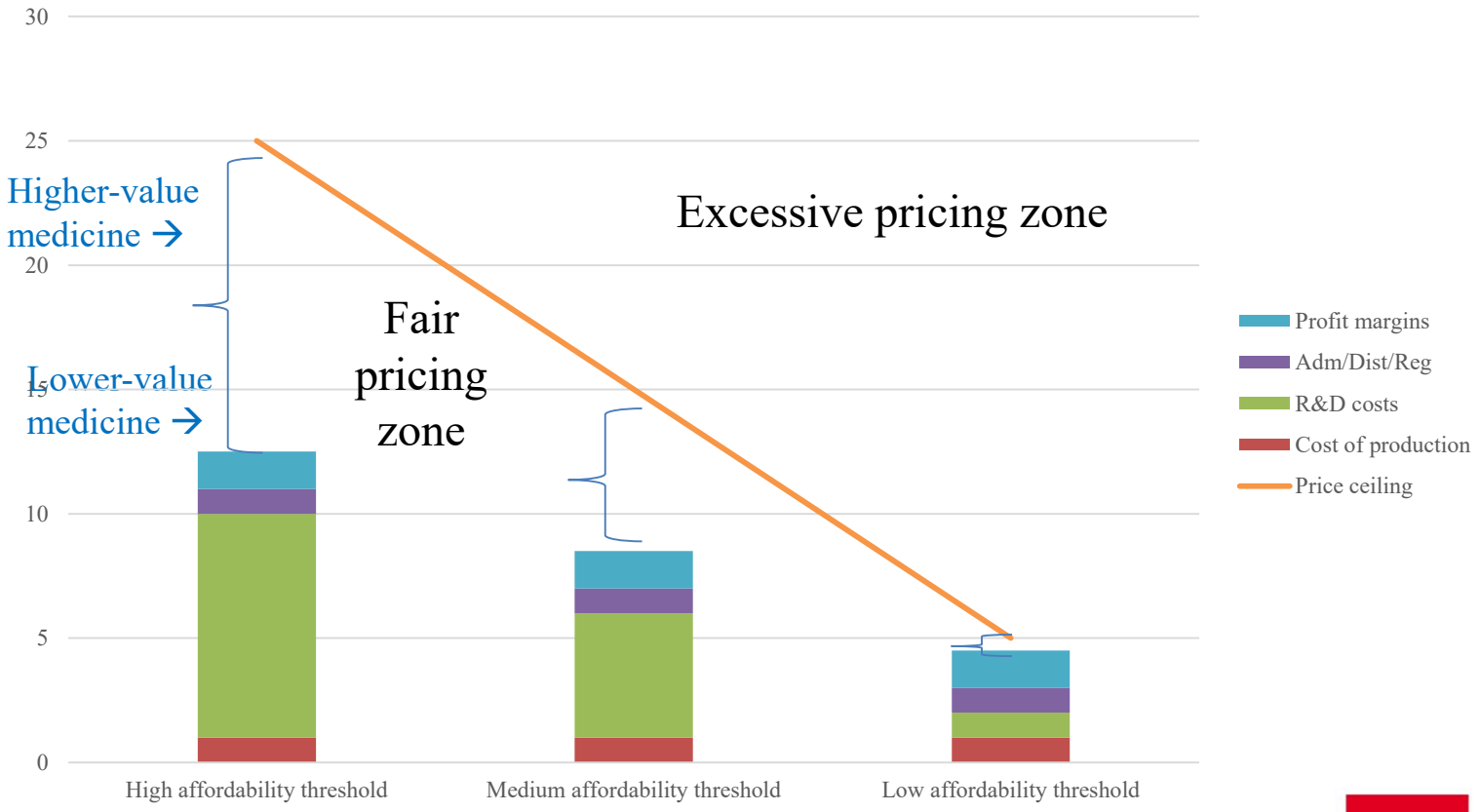
# A ZONE OF FAIR PRICING:
## EQUALLY DISTRIBUTED R&D COSTS




Source: Moon et al. (in press) Defining the concept of fair pricing for medicines.

# A ZONE OF FAIR PRICING:
## PROGRESSIVELY DISTRIBUTED R&D COSTS

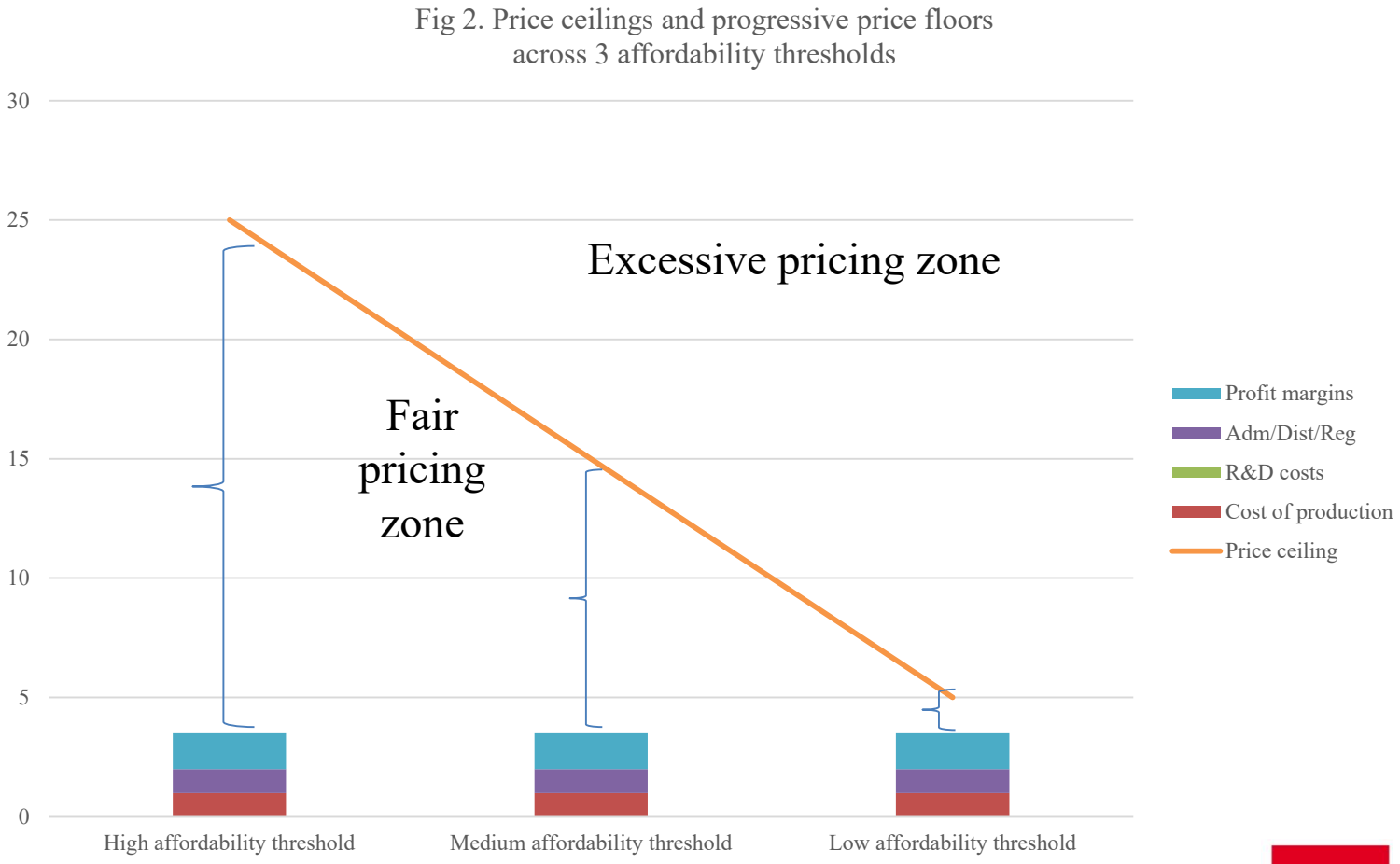
Fig 2. Price ceilings and progressive price floors across 3 affordability thresholds




Source: Moon et al. (in press) Defining the concept of fair pricing for medicines.

# A ZONE OF FAIR PRICING

## GENERIC MEDICINE




Source: Moon et al. (in press) Defining the concept of fair pricing for medicines.

# ILLUSTRATATION

## SOFOSBUVIR (HEPATITIS C)

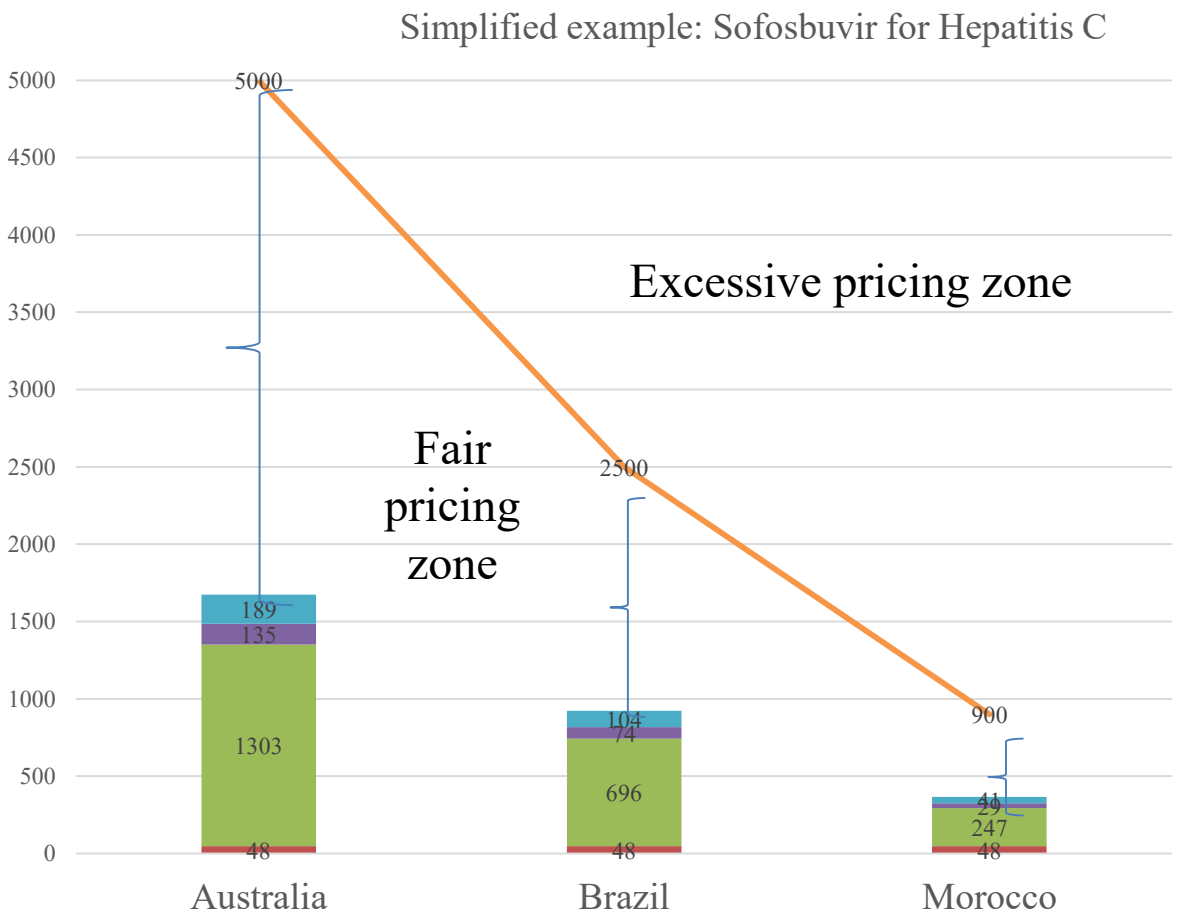
- R&D costs:
  - Pharmasset ($62 M) + Gilead ($880 M) = $943 M
- Gilead acquires Pharmasset: $11,000 M
- Gilead outlay: $11,880 M (R&D + acquisition cost)
- Recouped over 10 years (minimum) patent term
- Cost of production: $47 per treatment course
- Administration, distribution, registration: 10%
- Profit: 14%

| Capacity to pay | Country | % of global economy | GNI per capita | # patients treated/year |
|---|---|---|---|---|
| High | Australia | 1.65 | 51,360 | 15,000 |
| Medium | Brazil | 2.35 | 8600 | 40,000 |
| Low | Morocco | 0.14 | 2860 | 6500 |

Data Sources: US Senate Finance Committee (2015), WHO Progress Report on Access to Hepatitis C Treatment (2018), World Bank, MedsPAL, Hill, Barber, Gotham (2018)

# A ZONE OF FAIR PRICING

## SIMPLIFIED EXAMPLE: SOFOSBUVIR FOR HEP C




IS:
-Conceptual
-Judgment tool

IS NOT:
-Fixing a price
-International agreement

# ILLUSTRATATION

## SOFOSBUVIR (HEPATITIS C)

- R&D costs:
  - Pharmasset ($62 M) + Gilead ($880 M) = $943 M
- Gilead acquires Pharm
- Gilead outlay: $11,88
- Recouped over 10 yea
- Cost of production: $4
- Administration, distribu
- Profit: 14%

| Capacity to pay | Country | % of global economy | GNI per capita | # patients treated/year |
|---|---|---|---|---|
| High | Australia | 1.65 | 51,360 | 15,000 |
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Data Sources: US Senate Finance Committee (2015), WHO Progress Report on Access to Hepatitis C Treatment (2018), World Bank, MedsPAL, Hill, Barber, Gotham (2018)

# THINKING OUTSIDE-THE-BOX ABOUT MEDICINES PRICES

**Established:**

- How much do we pay, compared to others (like us)?
- How does it compare to prices of competing products?
- At that price, how many people can we afford to treat?
- How to achieve fairness in my country?
- What is the price per patient?

**Outside-the-box:**

- What price is affordable & allows for universal access?
- How much did it cost? (to develop, produce and distribute)
- How much profit has been earned? What's fair?
- How to achieve fairness in my country and globally?
- How else can we pay for innovation, apart from prices per patient?

Need some combination of established and outside-the-box…but more outside-the-box

# FAIR PRICING IN PRACTICE: TRANSPARENCY, GOVERNANCE & POLITICAL WILL

Reference Pricing

Licensing - compulsory or voluntary

Competition Law

Negotiation

Pooled procurement

Alternate R&D models

Health Technology Assessment

Conditions on public R&D funding & incentives

Address regulatory barriers to competition

Mandate Information Disclosure

Publicly-mandated production

Import for Personal Use ("Buyers clubs")

"Netflix" model

Patentability criteria

Medical Tourism

Pharmacist compounding

# CALIBRATING INCENTIVES



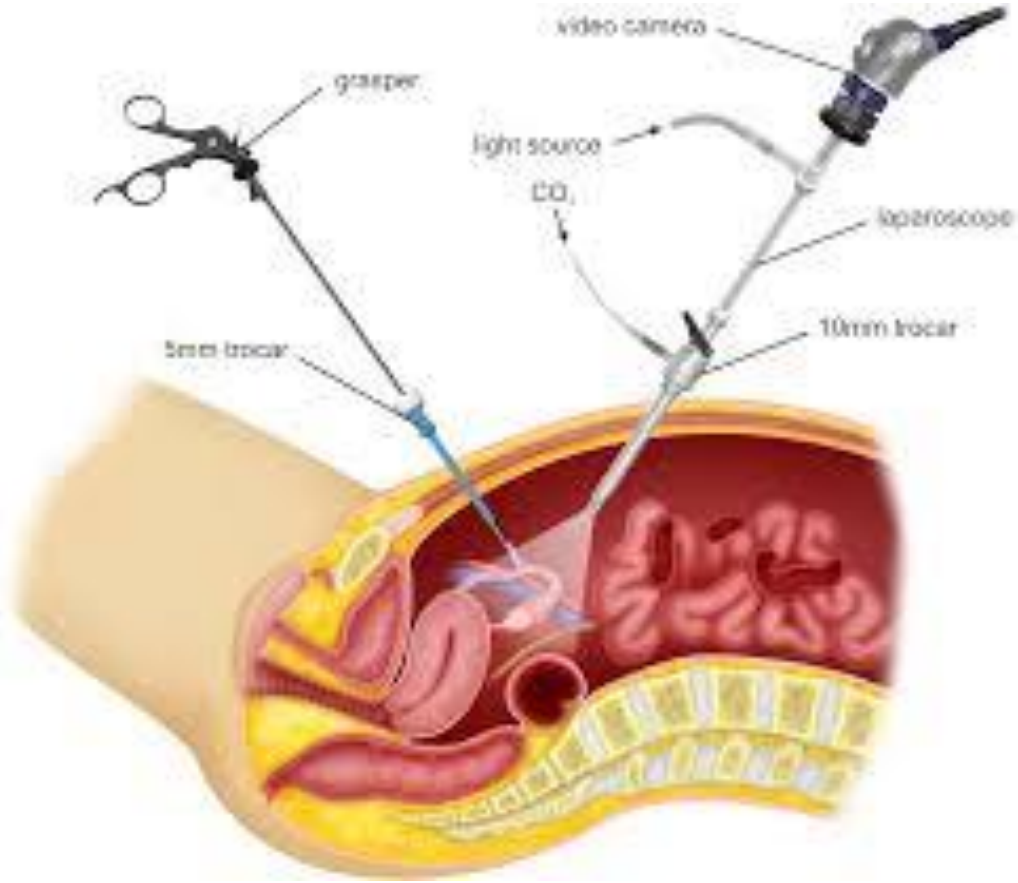

Ancient Roman surgical tools
(Pompeii)

Laparoscopic
surgical instrument

Source: https://i.pinimg.com/originals/fe/3b/9f/fe3b9fd568c2b8fc28289d6e998a9c62.jpg,
https://cdn11.bigcommerce.com/s-e6uiibqxty/images/stencil/500x659/products/375/772/40.069.20_8__96582.1495821288.jpg?c=2

# LAPAROSCOPIC SURGERY

(MINIMALLY INVASIVE)



# CASE STUDY 1: CYSTIC FIBROSIS DRUGS



- 2015: Orkambi EMA approved
- Vertex UK list price ~$135,000
- ~10,000 CF patients in UK
- NICE: not cost-effective
- NHS-Vertex negotiations ~3 years
- Vertex rejects $6.5 billion, 5 year offer
- Vertex destroys 8000 packs of UK stock of expired drug
- UK considers compulsory license


or

?

- Would it harm innovation?

Sources: https://www.statnews.com/2019/10/23/we-conquered-a-disease-how-vertex-delivered-a-transformative-medicine-for-cystic-fibrosis/, https://www.fda.gov/news-events/press-announcements/fda-approves-new-breakthrough-therapy-cystic-fibrosis, https://www.businesswire.com/news/home/20191021005792/en/ADDING%C2%A0MULTIMEDIA-FDA-Approves-TRIKAFTA-elexacaftortezacaftorivacaftor-ivacaftor-Treat

# PRICE REGULATION AND INNOVATION

## A COMPLEX RELATIONSHIP

Does regulating prices mean less innovation?

- R&D costs money
- High prices do not necessarily maximize revenue
  - Price x volume = revenue, or
  - Prizes (like “Netflix” model) = revenue
- High prices are inefficient way to generate R&D investment
  - Pharma & biotech R&D as % of sales: 18-21.6%*
- Regulating prices can send healthy signals to market, that:
  - Price must be justified by value, costs and risk
  - Public and private risk-taking will be rewarded
  - Price must be affordable to health systems
  - Time limit on price negotiations
  - Innovation across therapeutic areas is needed

Source:* 2010-2024 estimates. https://info.evaluate.com/rs/607-YGS-364/images/EvaluatePharma_World_Preview_2019.pdf

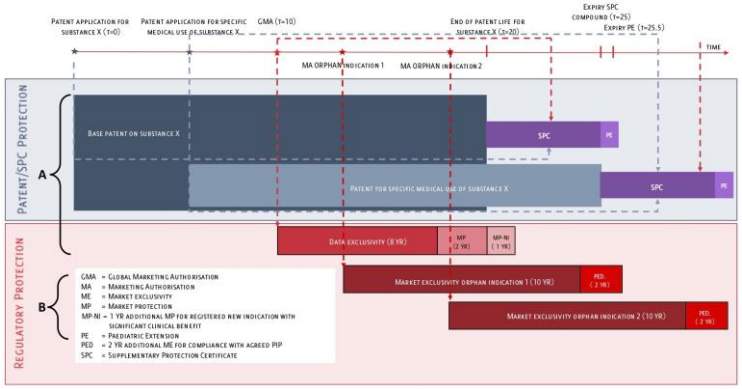




Image sources: Ellen 't Hoen slides (www.medicineslawandpolicy.org) from http://www.technopolis-group.com/report/effects-of-supplementary-protection-mechanisms-for-pharmaceutical-products/; https://i.pinimg.com/originals/fe/3b/9f/fe3b9fd568c2b8fc28289d6e998a9c62.jpg, https://cdn11.bigcommerce.com/s-e6uiibqxty/images/stencil/500x659/products/375/772/40.069.20_8__96582.1495821288.jpg?c=2

# CASE STUDY 2: AUSTRALIA & HEPATITIS C




Image Sources: https://www.thrillist.com/eat/nation/buffet-restaurant-food-service-jobs-explained; http://grmdaily.com/netflix-wide-magazine

# AUSTRALIA'S "NETFLIX" MODEL HEPATITIS C

- 2014:
  - ~230,000 people with Hepatitis C
  - Hep C drugs: AU$ 71,400 ($54,000) per patient
  - Rationing to most severely ill
- 2015:
  - Lump-sum "prize" of ~AU$ 1 billion ($766m) over 5 years
  - Unlimited medicines supply → universal access offered
  - Initial government estimate: 61,500 patients
  - Effective per-patient price: AU$ 16,260 ($12,460)

- Our estimate 2016-21: 104,000 patients
  - 87% drop in per-patient price: AU$ 9600 ($ 7352)
- Savings: AU$ 6.4 billion or 93,000 patients
- Australia world leader in HCV treatment and control

Source: Moon S and Erickson E. (2019) Universal access through lump-sum remuneration – Australia's approach to Hepatitis C. N Engl J Med 2019; 380:607-610. DOI: 10.1056/NEJMp1813728

# AUSTRALIA'S "NETFLIX" MODEL HEPATITIS C

- Universal access policy:
  - All major regimens included – clinician choice based on medical considerations
  - No restrictions on patient access based on stage of liver disease, ongoing drug or alcohol use
  - General practitioners & specialists can prescribe
  - Low out-of-pocket cost to patients ($7-$37/month)

- Public policy and public health benefit:
  - Lower price and budget certainty
  - Each person = no marginal cost
  - Incentive to treat early
  - + Society's willingness to treat and re-treat
  - + Society's willingness to treat marginalized populations (e.g. IDUs, prison population)
  - Treatment as prevention

Source: Moon S and Erickson E. (2019) Universal access through lump-sum remuneration – Australia's approach to Hepatitis C. N Engl J Med 2019; 380:607-610. DOI: 10.1056/NEJMp1813728

# AUSTRALIA'S "NETFLIX" MODEL HEPATITIS C

- Seller benefits:
  - Sizeable reward;
  - Revenue certainty;
  - Wide profit margin: Production cost << revenue
    - Production: ~$50-$100 per patient
    - Cost ~$10 M vs ~$766 M Revenue

- Largest real-world implementation of "delinkage": reward innovation separately from price

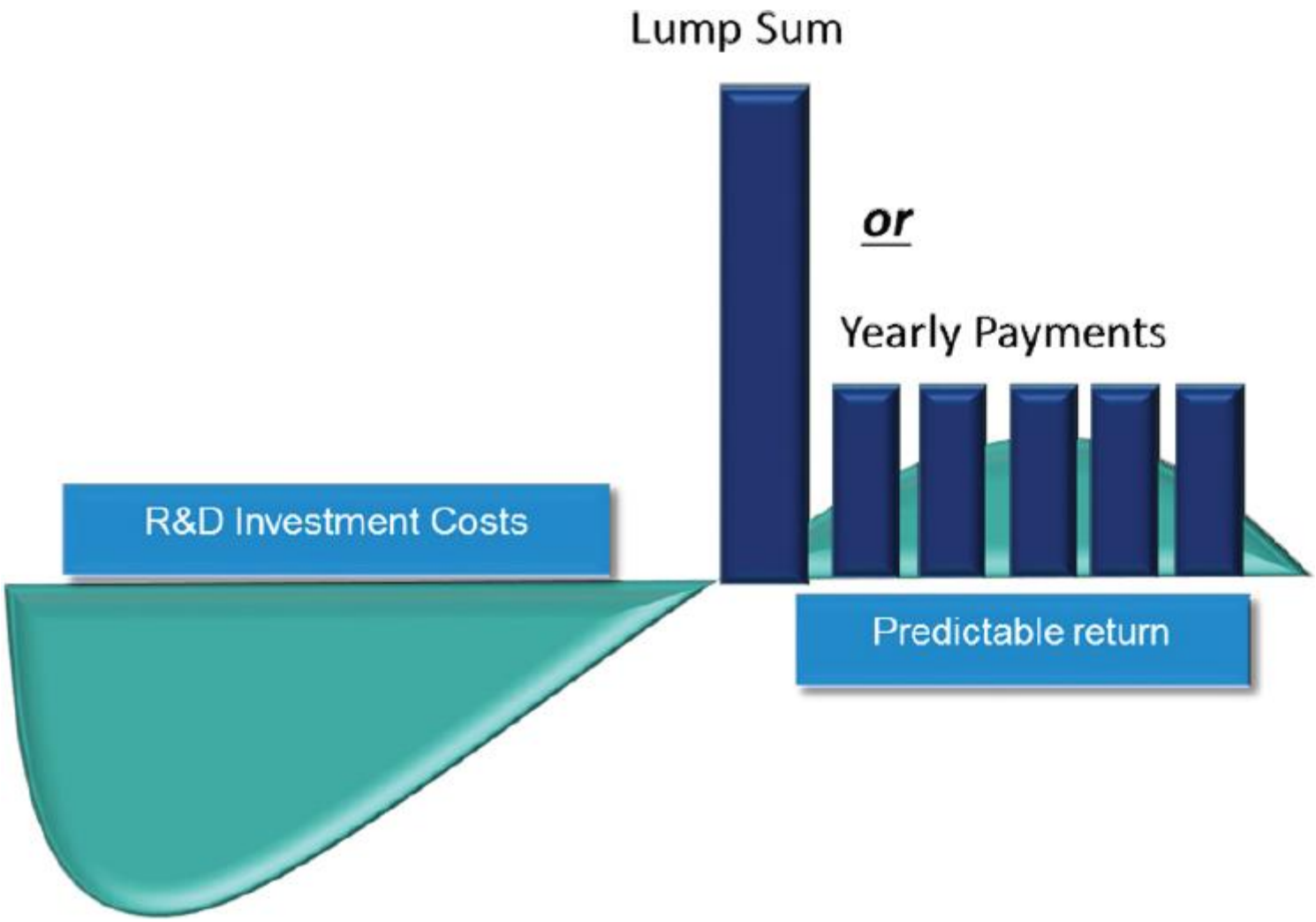



**FIGURE 5-4** Market entry reward model. SOURCES: Daniel presentation, June 21, 2017; adapted from DiMasi, 2016. Available: https://www.nap.edu/read/24914/chapter/6#81

# AUSTRALIA'S "NETFLIX" MODEL HEPATITIS C

- Broader use? Yes, when:
  - Cost of production is small % of price
  - Payer can reasonably estimate volume needed
  - Supplier willing and able to meet volume of demand
- Other health systems adopt Netflix for Hep C in 2019:
  - Louisiana state (US): $35 million, 18 months, 10,000 patients
  - Washington state (US): elimination by 2030
  - NHS England (UK): £1 billion over 3 years, 113,000 potential patients

- NHS England: Vertex rejected $660 M, 5 year offer for CF

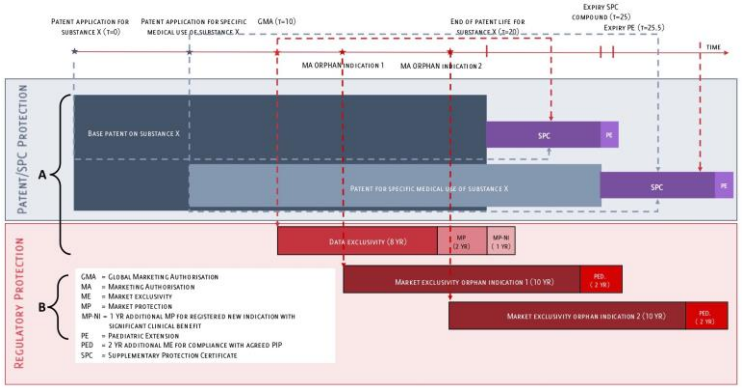



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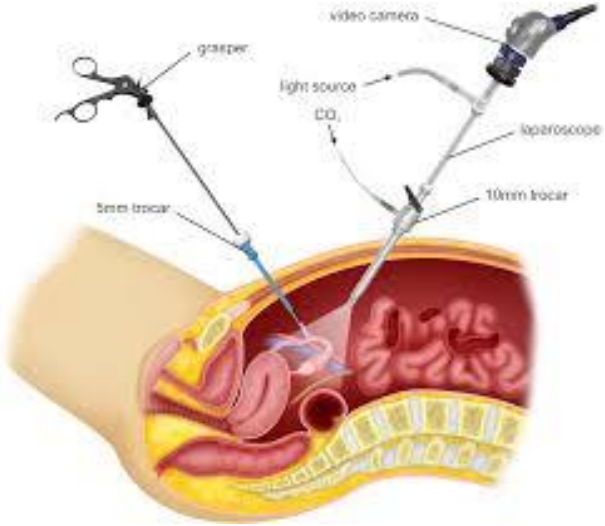
Image sources: Ellen 't Hoen slides (www.medicineslawandpolicy.org) from http://www.technopolis-group.com/report/effects-of-supplementary-protection-mechanisms-for-pharmaceutical-products/; https://i.pinimg.com/originals/fe/3b/9f/fe3b9fd568c2b8fc28289d6e998a9c62.jpg, https://cdn11.bigcommerce.com/s-e6uiibqxty/images/stencil/500x659/products/375/772/40.069.20_8__96582.1495821288.jpg?c=2

# TRANSPARENCY

Calibrated intervention requires understanding the system.




Information needed on:

- Net Prices
- Net R&D costs
  - Private investment
  - Public R&D funds
  - Tax breaks
- Patent status
- Data on safety, efficacy, health system effects



Source: Moon S. (2018) Removing the blindfold on medicines pricing.*BMJ* ; 360 doi: https://doi.org/10.1136/bmj.k840

# WORLD HEALTH ASSEMBLY 2019 TRANSPARENCY RESOLUTION

# WORLD HEALTH ASSEMBLY 2019 TRANSPARENCY RESOLUTION

- May 2019: WHA resolution approved: “Improving the transparency of markets for medicines, vaccines, and other health products”
- 19 co-sponsors: Europe, Latin America, Africa, Asia
  - Andorra, Brazil, Egypt, Eswatini, Greece, India, Italy, Kenya, Luxembourg, Malaysia, Malta, Portugal, Russian Federation, Serbia, Slovenia, South Africa, Spain, Sri Lanka, Uganda
- Endorses increased transparency on:
  - Net medicines prices
  - Net R&D costs
  - Clinical trial outcomes
  - Revenues, units sold, marketing cost
  - Patent and registration status

- August 2019: Italian decree requiring information disclosure to medicines agency
- October 2019: French parliament debates price and R&D transparency proposals

# KINGDON (1984) MULTIPLE STREAMS FRAMEWORK

Problem Stream

Policy Stream

Politics Stream

Policy Window

Policy Change

Policy Entrepreneurs

# CONCLUSIONS

# 3 CONCLUSIONS

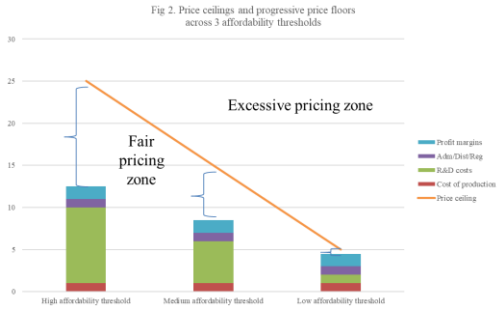
1. A clear concept of “fairness” in medicines pricing can help
   - → To achieve it in practice
   - → To justify it publicly

2. More information transparency can help to:
   1. Assess fairness objectively
   2. Calibrate incentives and price regulation

3. Governments have many tools available to make prices fair(er) in practice, if political will to use them

Thank you, Vielen Dank

Comments welcome:
suerie.moon@graduateinstitute.ch




# EXTRA SLIDES

# PUBLIC RETURN ON PUBLIC INVESTMENT: CASE STUDY DAA FOR HEPATITIS C

- 1974: Non-A, Non-B Hepatitis identified by US NIH scientists
- 1989: Hepatitis C virus identified (US CDC, US NIH, Chiron)
- 1999: Replicon isolated by R. Bartenschlager (Heidelberg University, funded by German Ministry for Research & Technology, German Society for Research)
- 2002: Replicon improved by C. Rice (Rockefeller University, funded by US NIH)
- 1999-2008: Apath (SME) distributes replicon to drug developers (funded by US Small Business Innovation Research program)
- 2001-11: Pharmasset (SME) develops sofosbuvir
  - 2004-8: PS-6130 adapted with McGuigan method (UK Medical Research Council, European Commission, Belgium)
- 2011: Gilead acquires Pharmasset for $11 billion
- 2012-5: Merck, Bristol Myers Squibb, J&J acquire Hep C SMEs
- 2013: US FDA approves Gilead's sofosbuvir
- 2013-7: Gilead HepC revenues >$50 billion

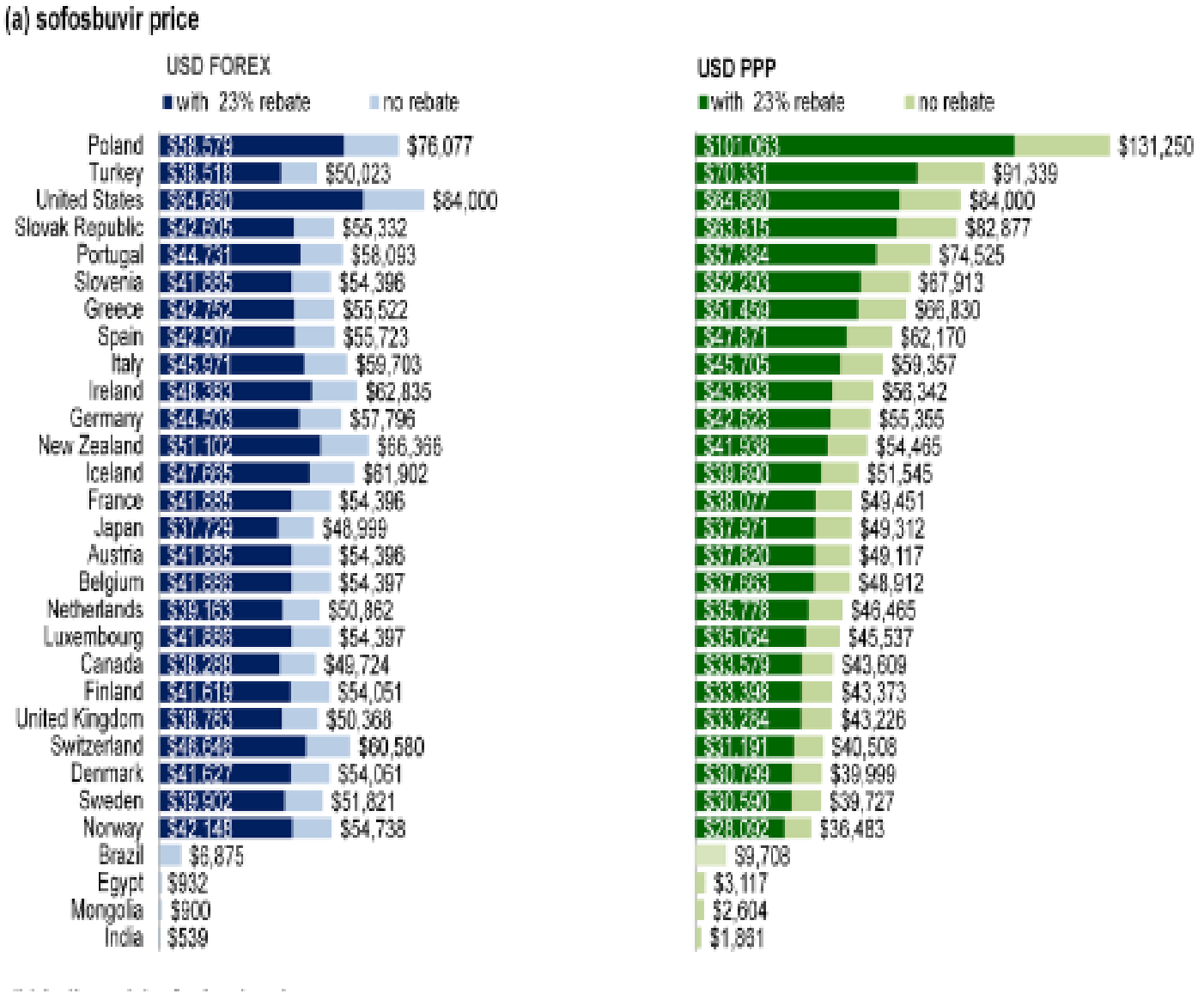
Sources: Roy, V. (2017). *The Financialization of a Cure: A Political Economy of Biomedical Innovation, Pricing, and Public Health* (Doctoral thesis). https://doi.org/10.17863/CAM.13671.

# PUBLIC RETURN ON PUBLIC INVESTMENT



- Sampat & Lichtenberg (2011):
  - Patents on 478 FDA-approved medicines 1988-2005
  - About ½ approved medicines benefits from publicly-financed research
  - 2/3 for priority review medicines
- Cleary et al (2018):
  - Publications relating to 210 new molecular entities FDA-approved (2010-6)
  - 100% benefited from US NIH funding
- Areas of market failure:
  - Neglected disease: 84% public (64%) & philanthropic (21%)
  - Antibiotics, Outbreak-prone pathogens?

Sources: Sampat, Bhaven N., and Frank R. Lichtenberg. "What are the respective roles of the public and private sectors in pharmaceutical innovation?." *Health Affairs* 30.2 (2011): 332-339. Cleary, E.G., Beierlein, J.M., Khanuja, N.S., McNamee, L.M. and Ledley, F.D. (2018) 'Contribution of NIH Funding to New Drug Approvals 2010–2016', Proceedings of the National Academy of Sciences, 115(10), pp. 2329-2334




Iyengar et al. 2016. Prices, Costs, and Affordability of New Medicines for Hepatitis C in 30 Countries: An Economic Analysis. Available : https://journals.plos.org/plosmedicine/article/file?id=10.1371/journal.pmed.1002032&type=printable

# OUTSIDE THE BOX R&D: DNDI'S HEPATITIS C STRATEGY

**Traditional** pharmaceutical business model

Innovation **"balanced"** against affordability

**New** pharmaceutical business model?

Innovation **with** affordability

Image sources: https://associationsnow.com/2015/04/talking-tech-balancing-it-security/; https://www.amazon.com/Garden-Decoration-Natural-Septuple-Stacked/dp/B0093QN1IK

# DNDI'S HEPATITIS C STRATEGY

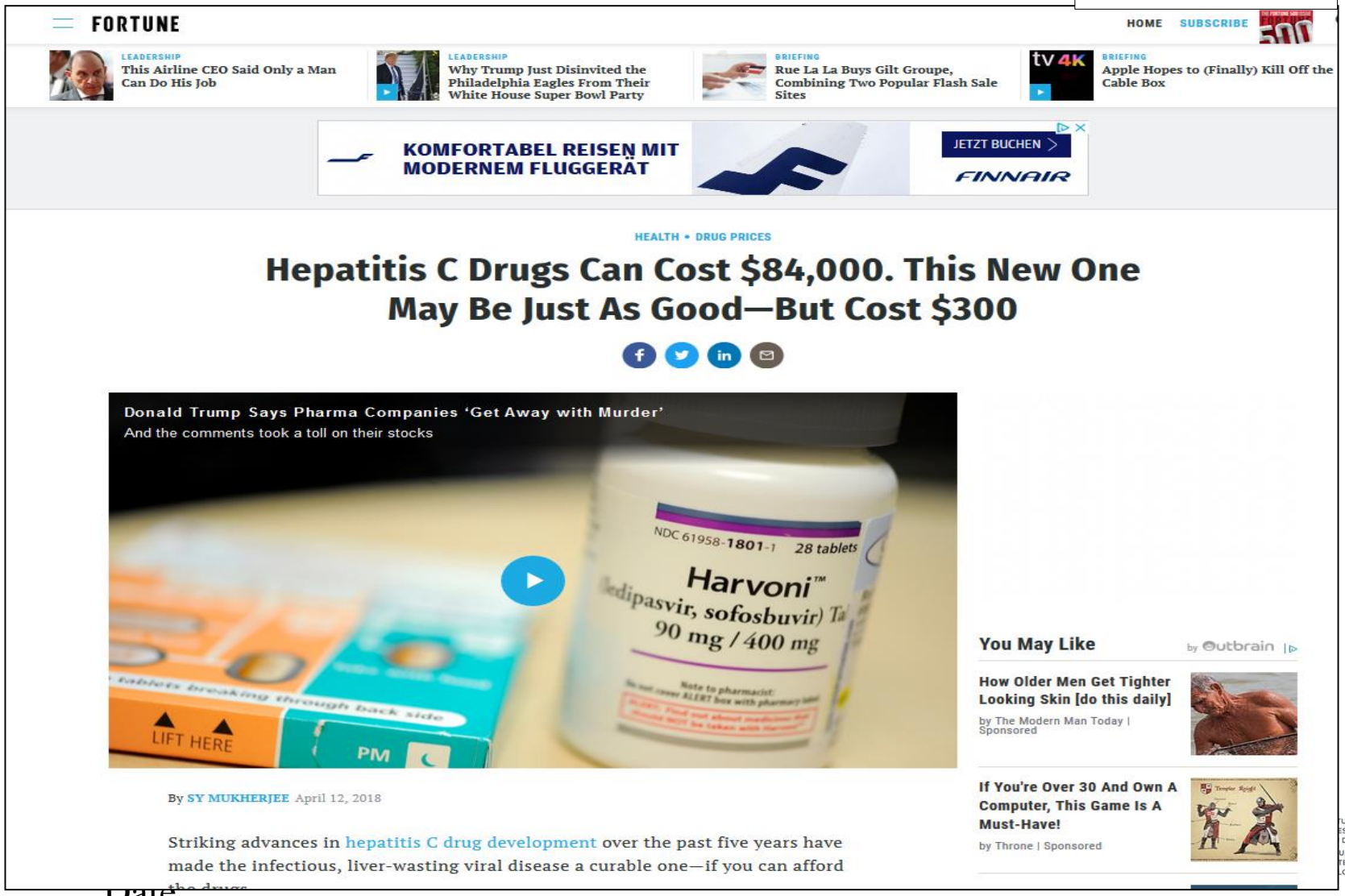


- Hep C DAA race: Gilead, Merck BMS, J&J, AbbVie
- Slower: Presidio Pharmaceuticals (SME): ravidasvir
- Multiple firms, parallel DAA R&D on public knowledge base

- Drugs for Neglected Diseases initiative (DNDi)
  - 2016 launches ravidasvir+sofosbuvir development
  - Especially relevant for middle-income countries
  - Medicines Patent Pool license: 4% LIC royalty, 7% MICs
  - High-income countries: why not?

# OUTSIDE THE BOX R&D: DNDI'S HEPATITIS C STRATEGY

April 2018



FORTUNE

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This Airline CEO Said Only a Man Can Do His Job

LEADERSHIP
Why Trump Just Disinvited the Philadelphia Eagles From Their White House Super Bowl Party

BRIEFING
Rue La La Buys Gilt Groupe, Combining Two Popular Flash Sale Sites

BRIEFING
Apple Hopes to (Finally) Kill Off the Cable Box



HEALTH • DRUG PRICES

## Hepatitis C Drugs Can Cost $84,000. This New One May Be Just As Good—But Cost $300

Donald Trump Says Pharma Companies 'Get Away with Murder'
And the comments took a toll on their stocks



By SY MUKHERJEE April 12, 2018

Striking advances in hepatitis C drug development over the past five years have made the infectious, liver-wasting viral disease a curable one—if you can afford the drugs



Date