

THE CONCEPT OF FAIR PRICING OF MEDICINES

DEVELOPING COUNTRY VACCINE MANUFACTURERS' NETWORK

WEBINAR
21 JULY 2020

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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: Outside the box pricing: Hepatitis C treatment in Australia
- Case study 3: Outside the box innovation: DNDi Hepatitis C drug development

3. 3 Conclusions on fair pricing

4. Reflections on developing country vaccine manufacturers and Covid-19

CONCERN RE FAIRNESS OF MEDICINES PRICING

€ 48,000
(2014)



€ 133,000
(2015)



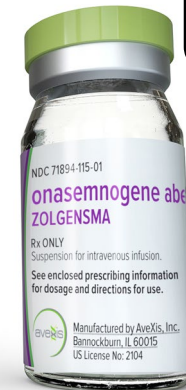
€ 320,000
(2018)



\$850,000
(2018)



\$2,100,000
(2019)



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

CASE STUDY 1: CYSTIC FIBROSIS DRUGS

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- Trikafta was FDA approved Oct 2019
- Development history:
 - 1989: Cystic fibrosis 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

6

- 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-elexacaftortezaftorivacaftor-ivacaftor-Treat>

FAIRNESS TO SELLERS AND BUYERS

A **SIMPLIFIED** MODEL

Sellers:

Small and large developers, manufacturers, distributors

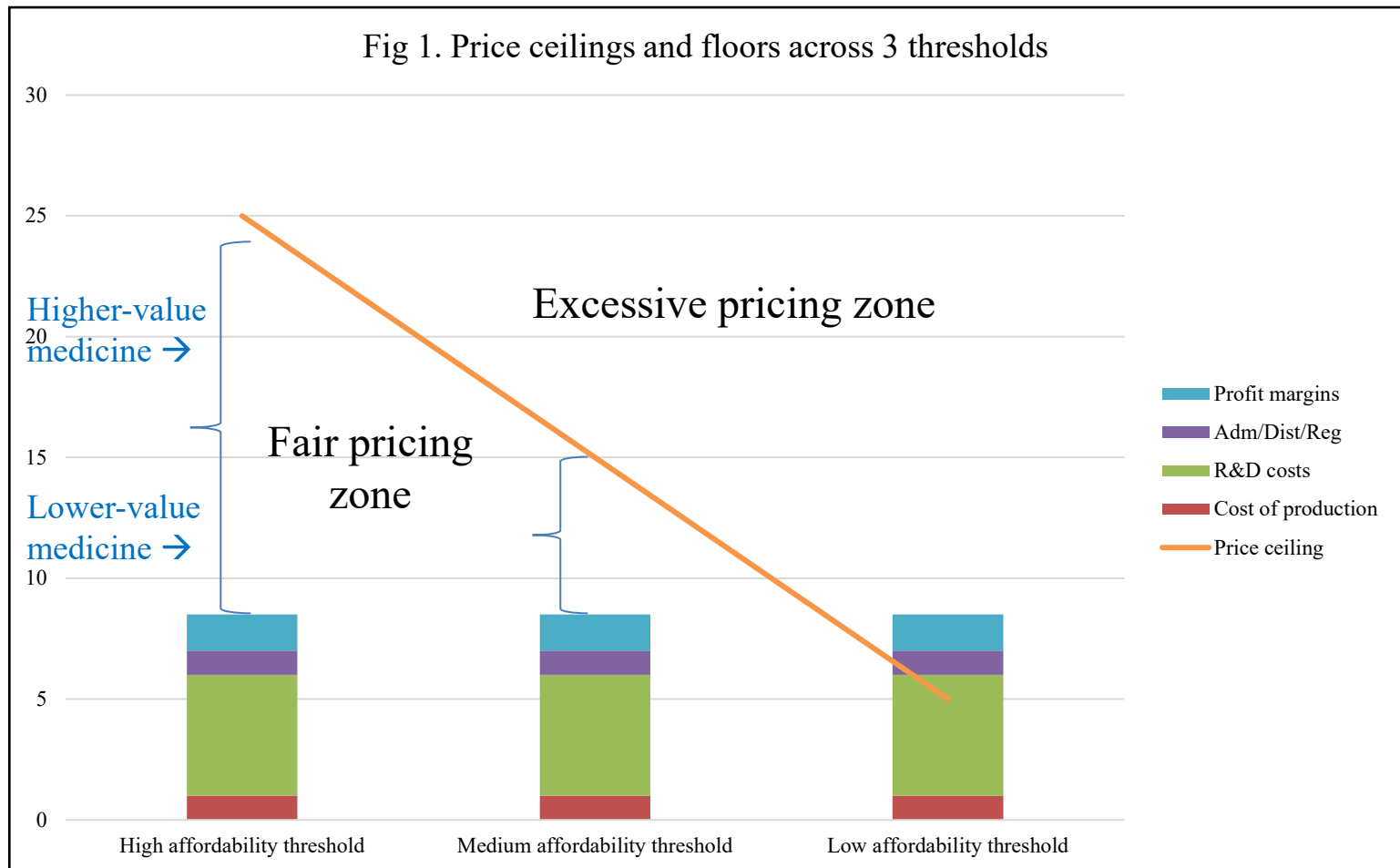
- Cost of R&D
- Cost of manufacturing and distribution
- Other related costs (e.g. registration, administration, pharmacovigilance)
- Fair profit

Buyers:

Payers, insurers, households, patients

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

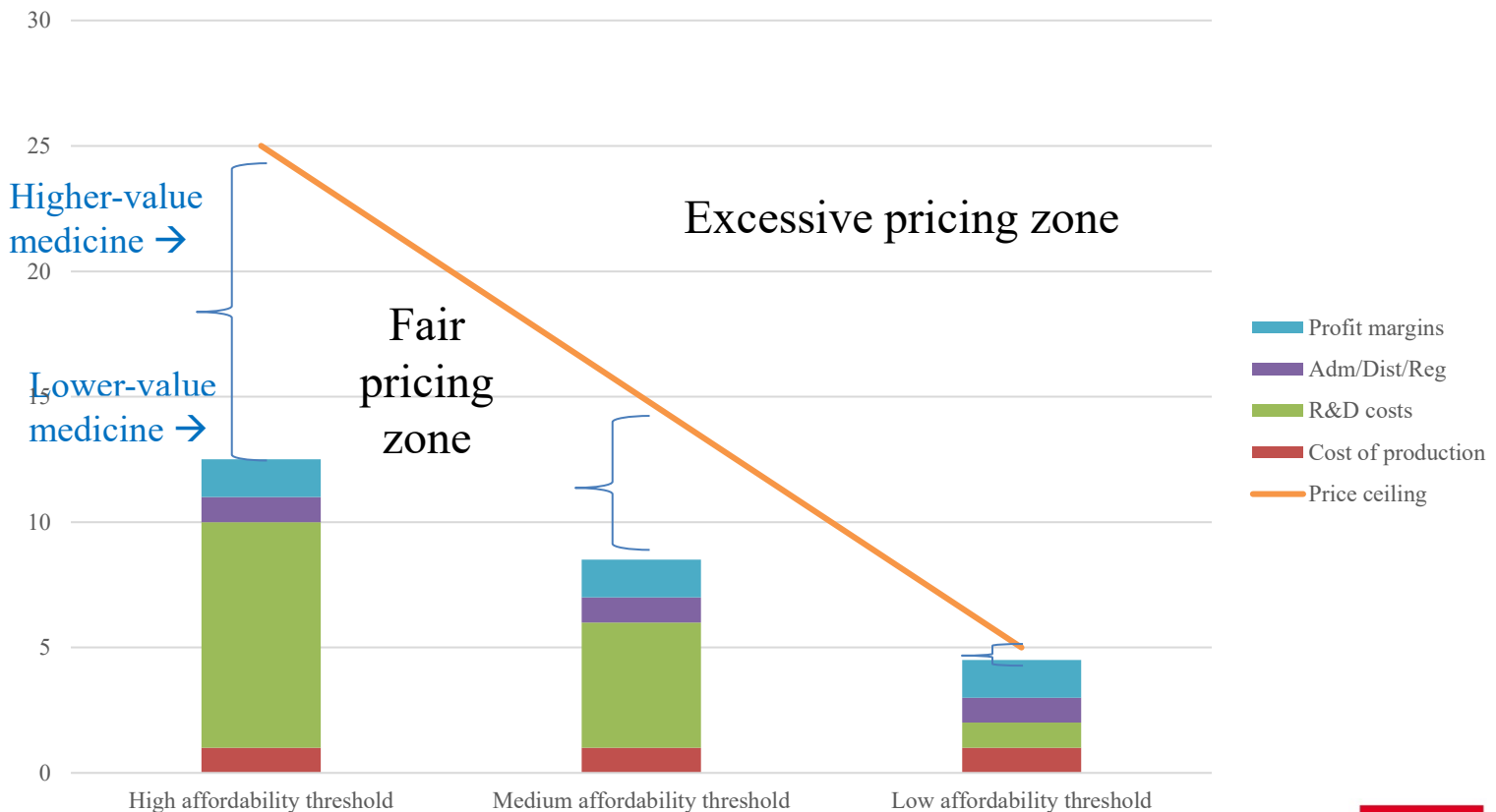
A ZONE OF FAIR PRICING: EQUALLY DISTRIBUTED R&D COSTS



Source: Moon Suerie, Mariat Stephanie, Kamae Isao, Pedersen Hanne Bak. Defining the concept of fair pricing for medicines *BMJ* 2020; 368 :l4726 <https://www.bmj.com/content/368/bmj.l4726>

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

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

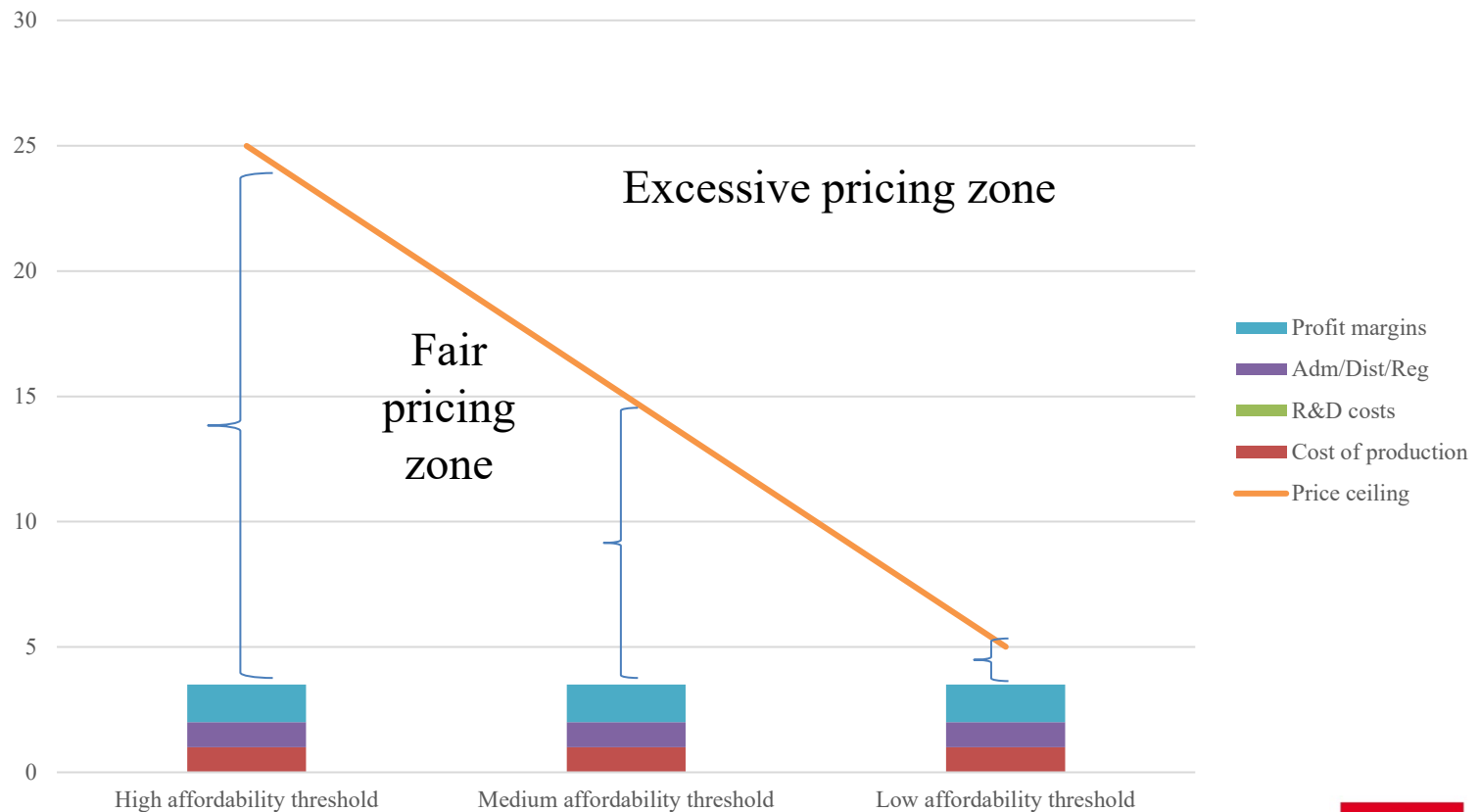


Source: Moon Suerie, Mariat Stephanie, Kamae Isao, Pedersen Hanne Bak. Defining the concept of fair pricing for medicines *BMJ* 2020; 368 :l4726 <https://www.bmj.com/content/368/bmj.l4726>

A ZONE OF FAIR PRICING

GENERIC MEDICINE

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

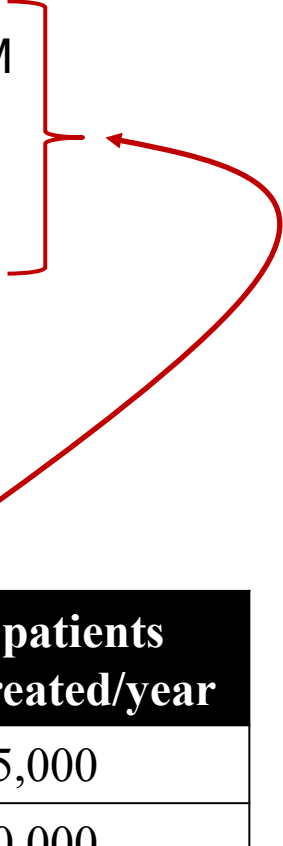


Source: Moon Suerie, Mariat Stephanie, Kamae Isao, Pedersen Hanne Bak. Defining the concept of fair pricing for medicines *BMJ* 2020; 368 :l4726 <https://www.bmj.com/content/368/bmj.l4726>

ILLUSTRATION

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%

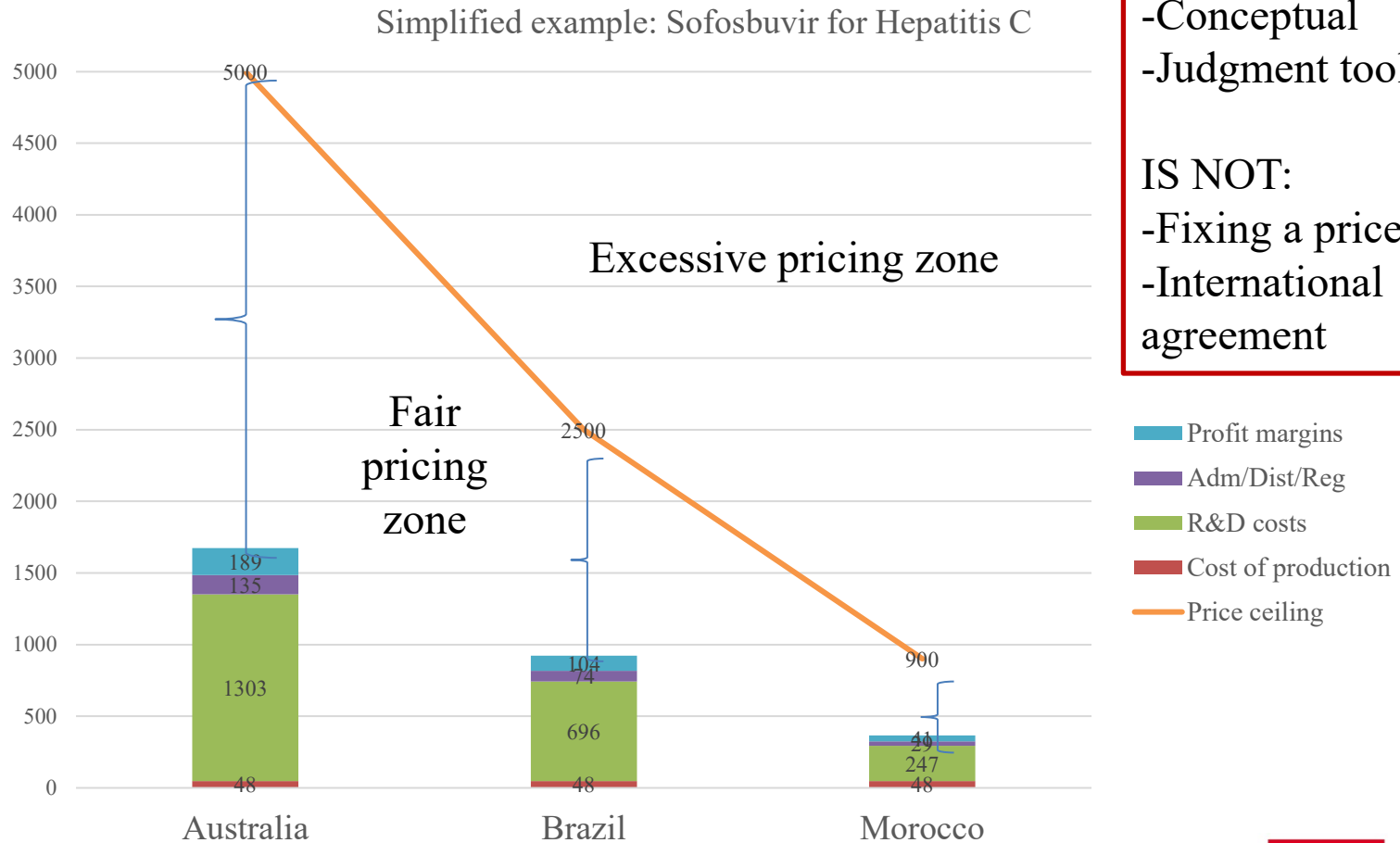


Capacity to pay	Country	% of global economy	GNI per capita	# patients treated/year
High	Australia	1.65	51,360	15,000
Medium	Brazil	2.35	8600	40,000
Low	Morocco	0.14	2860	6500

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



ILLUSTRATION

SOFOSBUVIR (HEPATITIS C)

- R&D costs:
 - Pharmasset (\$62 M) + Gilead (\$880 M) = \$943 M
- Gilead acquires Pharmasset for \$11,880 M
- Gilead outlay: \$11,880 M (including R&D cost)
- Recouped over 10 years (long term)
- Cost of production: \$400 M (low cost)
- Administration, distribution: 0%
- 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)

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



COMMENTS/QUESTIONS?

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FAIR PRICING IN PRACTICE: TRANSPARENCY, GOVERNANCE & POLITICAL WILL

Reference
Pricing

Licensing -
compulsory or
voluntary

Competition
Law

Negotiation

Alternate R&D
models

Pooled
procurement

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

19



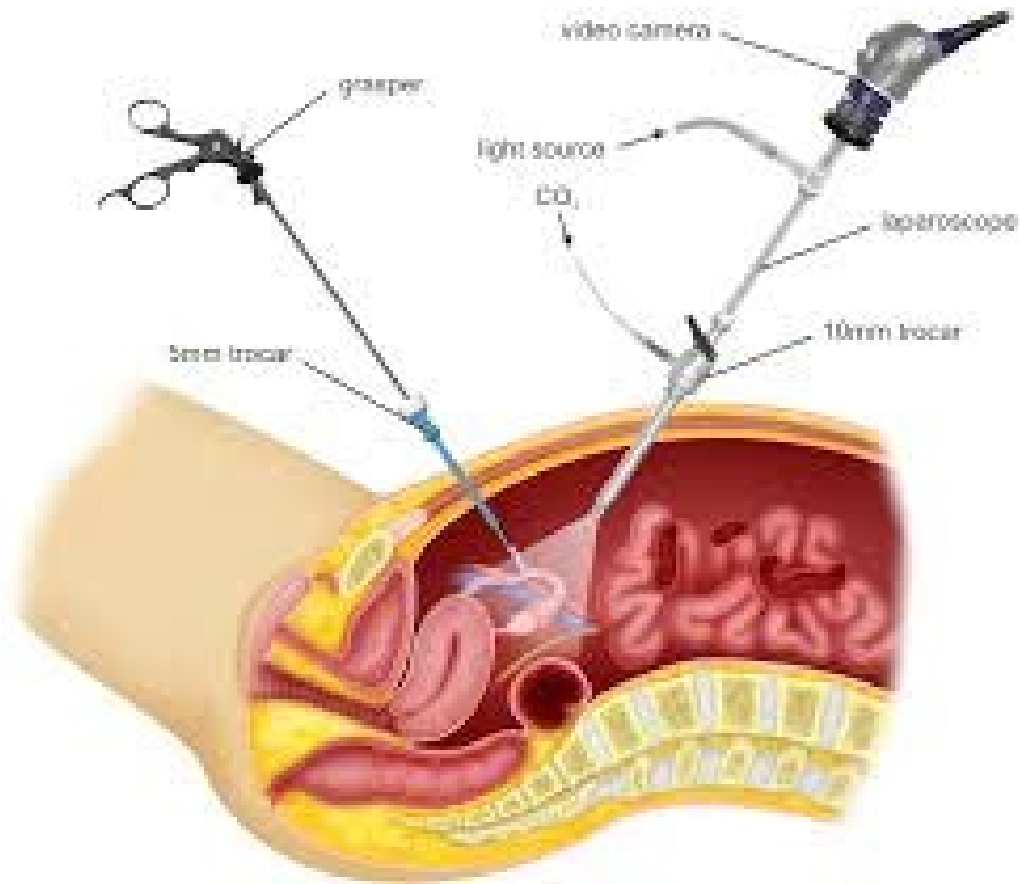
Ancient Roman surgical tools
(Pompeii)



Laparoscopic
surgical instrument

Source: <https://i.pinimg.com/originals/fe/3b/9f/fe3b9fd568c2b8fc28289d6e998a9c62.jpg>,
https://cdn11.bigcommerce.com/s-e6uiibqxy/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

21

- 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
-
- Would it harm innovation?



or



?

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

CASE STUDY 2: OUTSIDE THE BOX PRICING: 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

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

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

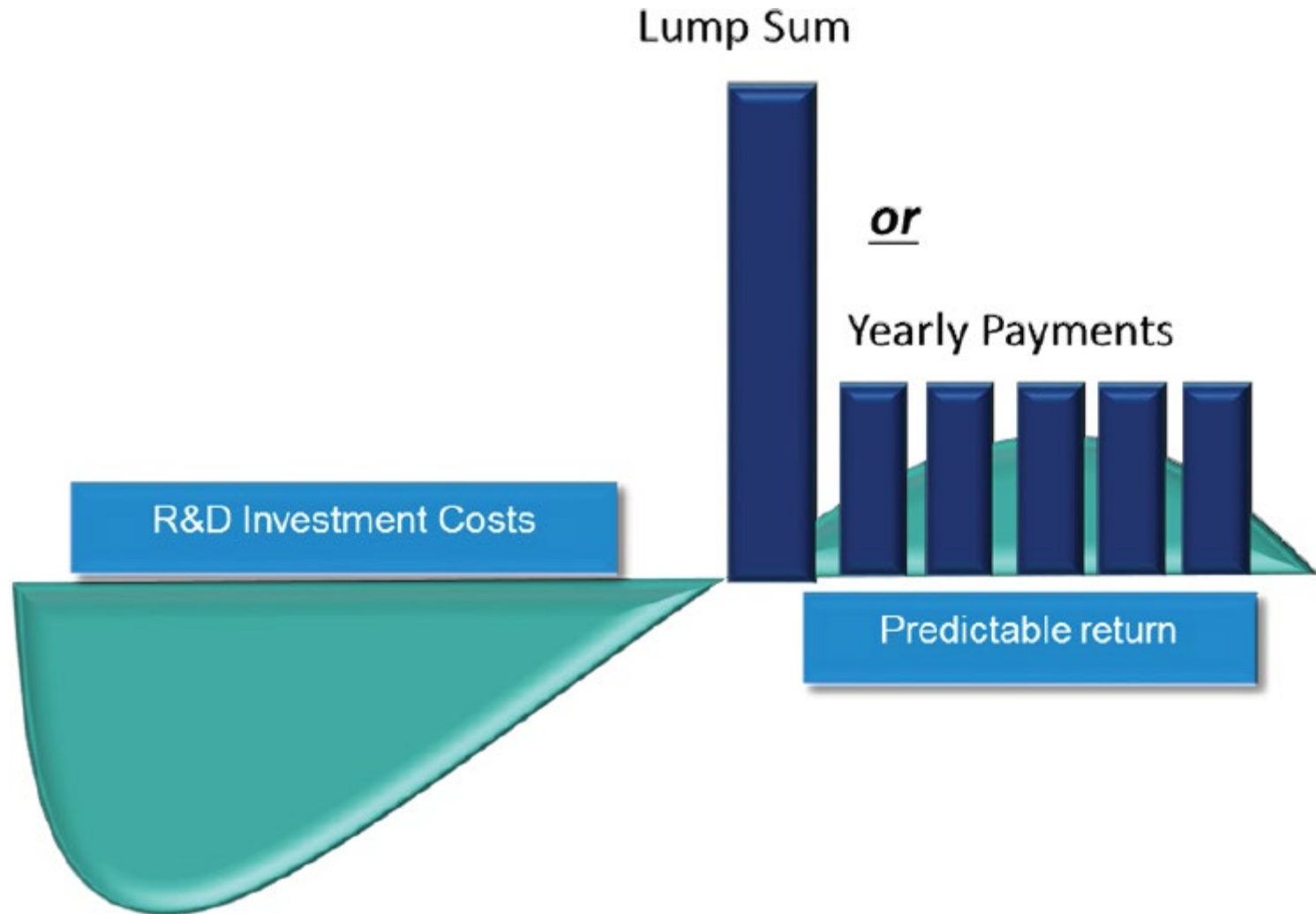


FIGURE 5-4 Market entry reward model. SOURCES: Daniel presentation, June 21, 2017; adapted from [DINAB, 2016](https://www.nap.edu/read/24914/chapter/6#81). Available: <https://www.nap.edu/read/24914/chapter/6#81>

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

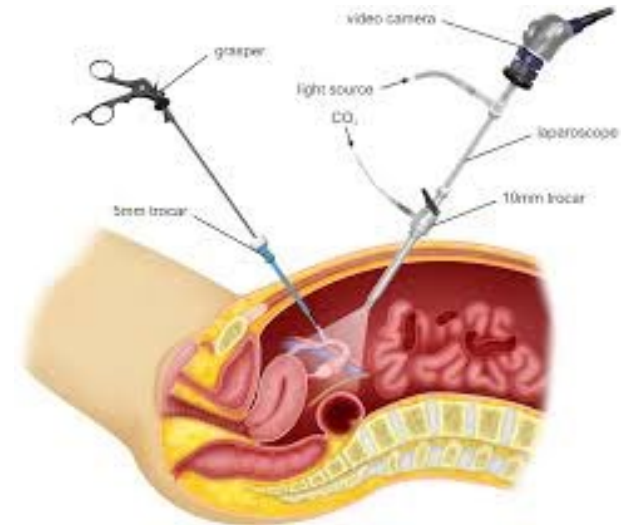


TRANSPARENCY

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



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

CASE STUDY 3: 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>

- 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
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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



**KOMFORTABEL REISEN MIT
MODERNEM FLUGGERÄT**



JETZT BUCHEN >

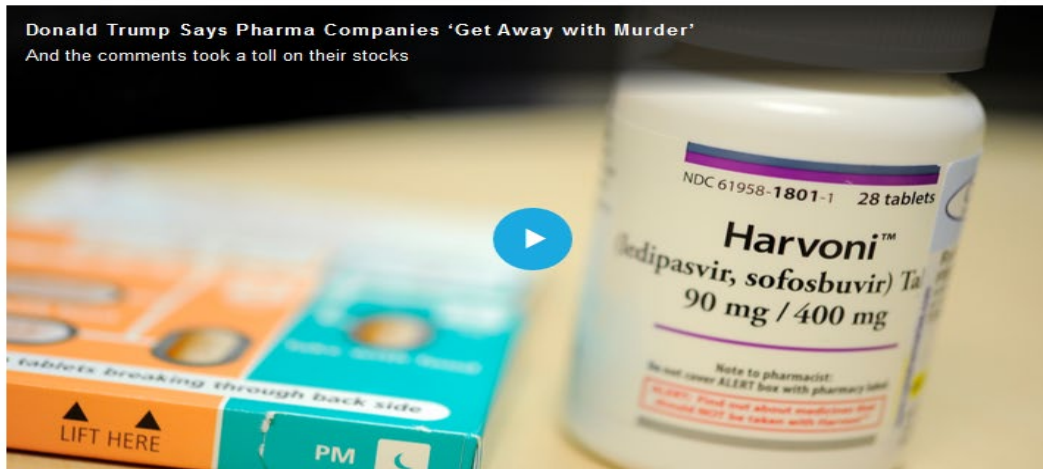
FINNAIR

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

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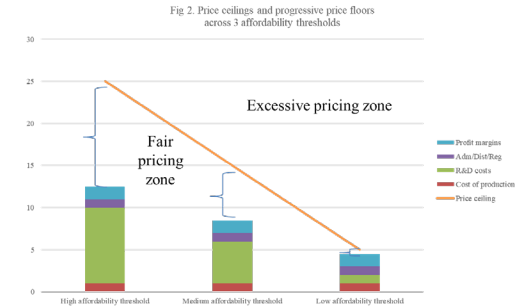
4. Reflections on developing country vaccine manufacturers and Covid-19

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. Many tools available to make prices fair(er) in practice, if political will to use them



REFLECTIONS ON DEVELOPING COUNTRY VACCINE MANUFACTURERS, FAIR PRICING AND COVID-19

- Vaccines affordability threshold: ~1% government health expenditure* (~0.05% per capita GNI)
- Globally-distributed vaccine production capacity essential for health, economic and national security
- But only if producers commit to equitable allocation & export
- Opportunity to adopt new regional models of cooperation
- Opportunity to adopt new business models of innovation
 - DCVMN members developing many vaccine candidates
 - Radical transparency?
- Access & affordability concerns across high, middle, low-income countries – global health 2.0

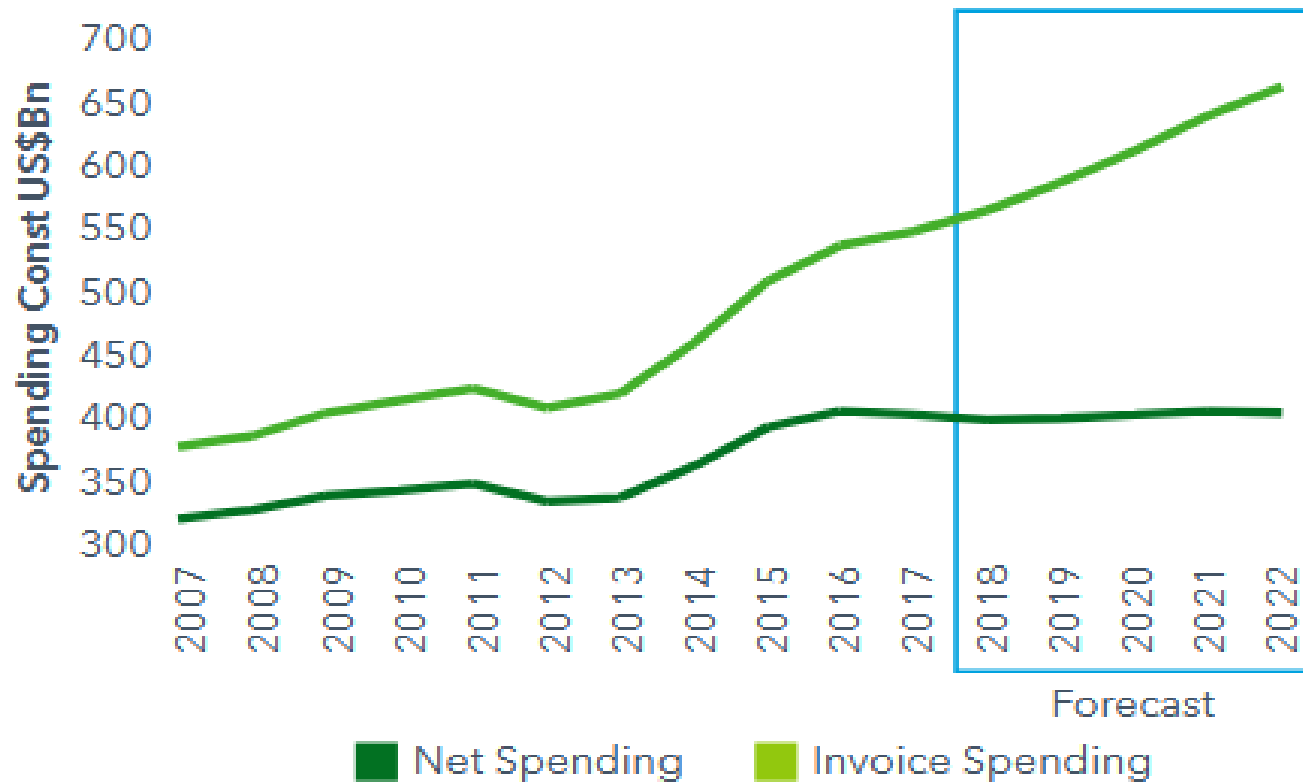
*Saxenian et al (2015)

THANK YOU
COMMENTS WELCOME
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EXTRA SLIDES

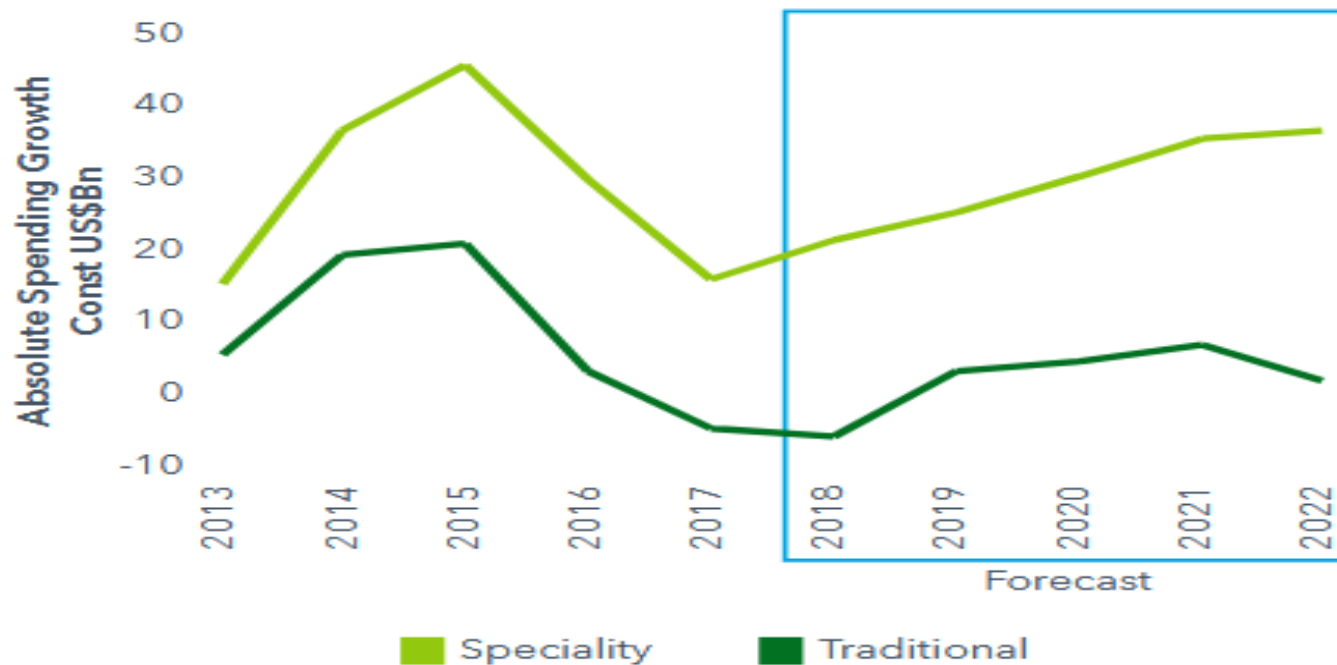
PRICE TRENDS

Exhibit 8: Developed Market Brand Invoice and Net Spending 2007-2022



Source: IQVIA 2018. 2018 and Beyond: Outlook and Turning Points. Available : https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/2018-and-beyond-outlook-and-turning-points.pdf?_=1540209266492.

Exhibit 10: Brand Spending Growth of Specialty and Traditional Drugs 2013-2022 in the Developed Markets



Source : IQVIA 2018. 2018 and Beyond: Outlook and Turning Points. Available : https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/2018-and-beyond-outlook-and-turning-points.pdf?_=1540209266492

- Specialty: ~40% total spending (2018) → ~50% by 2022
- Includes cancer, HIV, Hepatitis C, autoimmune, others

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

- 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

(a) sofosbuvir price

