THE CONCEPT OF FAIR PRICING OF MEDICINES

DEVELOPING COUNTRY VACCINE MANUFACTURERS’ NETWORK

WEBINAR
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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: 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)

€ 320,000 (2018)

$850,000 (2018)

$2,100,000 (2019)

€ 133,000 (2015)

Image Sources:
https://www.consumerreports.org/drug-prices/kymrdollars-childhood-cancer/
https://www.orkambihcp.com/administration;
http://www.multivu.com/players/English/8548551-avexis-fda-approval-zolgensma/
FAIR PRICING IN THEORY: WHAT IS FAIR? TO WHOM?
CASE STUDY 1: CYSTIC FIBROSIS DRUGS

- 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

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

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 Suerie, Mariat Stephanie, Kamae Isao, Pedersen Hanne Bak. Defining the concept of fair pricing for medicines *BMJ* 2020; 368 :i4726 [https://www.bmj.com/content/368/bmj.i4726](https://www.bmj.com/content/368/bmj.i4726)
**A ZONE OF FAIR PRICING:**
*EQUALLY DISTRIBUTED R&D COSTS*

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**Fig 1. Price ceilings and floors across 3 thresholds**

- **Higher-value medicine → Excessive pricing zone**
- **Lower-value medicine → Fair pricing zone**

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**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](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 :I4726 https://www.bmj.com/content/368/bmj.i4726
A ZONE OF FAIR PRICING
GENERIC MEDICINE

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

Excessive pricing zone
Fair pricing zone

Source: Moon Suerie, Mariat Stephanie, Kamae Isao, Pedersen Hanne Bak. Defining the concept of fair pricing for medicines *BMJ* 2020; 368 :i4726 [https://www.bmj.com/content/368/bmj.i4726](https://www.bmj.com/content/368/bmj.i4726)
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%

<table>
<thead>
<tr>
<th>Capacity to pay</th>
<th>Country</th>
<th>% of global economy</th>
<th>GNI per capita</th>
<th># patients treated/year</th>
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A ZONE OF FAIR PRICING

SIMPLIFIED EXAMPLE: SOFOSBUVIR FOR HEP C

Excessive pricing zone

Fair pricing zone

IS:
- Conceptual
- Judgment tool

IS NOT:
- Fixing a price
- International agreement
ILLUSTRATION
SOFOSBUVIR (HEPATITIS C)

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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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3. Conclusions on fair pricing
4. Reflections on developing country vaccine manufacturers and Covid-19
FAIR PRICING IN PRACTICE:
TRANSPARENCY, GOVERNANCE &
POLITICAL WILL
Address regulatory barriers to competition

Pooled procurement

Health Technology Assessment

Import for Personal Use ("Buyers clubs")

Reference Pricing

Licensing - compulsory or voluntary

Competition Law

Alternate R&D models

Conditions on public R&D funding & incentives

Mandate Information Disclosure

Publicly-mandated production

"Netflix" model

Patentability criteria

Medical Tourism

Pharmacist compounding

Negotiation
CALIBRATING INCENTIVES

Ancient Roman surgical tools (Pompeii)

Laparoscopic surgical instrument

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
  - Would it harm innovation?

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

CASE STUDY 2: OUTSIDE THE BOX
PRICING: AUSTRALIA & HEPATITIS C

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

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

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 Drive-AB, 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
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](https://doi.org/10.1136/bmj.k840)
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

New pharmaceutical business model?

Innovation “balanced” against affordability

Innovation with affordability

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

### PRICE TRENDS

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

- **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?